EXAMINING LEGISLATION TO IMPROVE PUBLIC HEALTH

HEARING

BEFORE THE

SUBCOMMITTEE ON HEALTH

OF THE

COMMITTEE ON ENERGY AND COMMERCE

HOUSE OF REPRESENTATIVES

ONE HUNDRED FOURTEENTH CONGRESS

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EXAMINING LEGISLATION TO IMPROVE PUBLIC HEALTH

THURSDAY, SEPTEMBER 8, 2016

HOUSE OF REPRESENTATIVES,
SUBCOMMITTEE ON HEALTH,
COMMITTEE ON ENERGY AND COMMERCE,
Washington, DC.

The subcommittee met, pursuant to call, at 10:00 a.m., in room 2322, Rayburn House Office Building, Hon. Joseph R. Pitts (chairman of the subcommittee) presiding.

Present: Representatives Pitts, Guthrie, Barton, Burgess, Blackburn, Lance, Bilirakis, Long, Ellmers, Bucshon, Brooks, Collins, Green, Engel, Schakowsky, Butterfield, Schrader, Kennedy, Cardenas, and Pallone (ex officio).

Also Present: Representative Roybal-Allard.

Staff Present: Paul Edattel, Chief Counsel, Health; Adrianna Simonelli, Professional Staff Member, Health; Heidi Stirrup, Health Policy Coordinator; Waverly Gordon, Minority Professional Staff Member; Tiffany Guarascio, Minority Deputy Staff Director and Chief Health Advisor; and Samantha Satchell, Minority Policy Analyst.

OPENING STATEMENT OF HON. JOSEPH R. PITTS, A REPRESENTATIVE IN CONGRESS FROM THE COMMONWEALTH OF PENNSYLVANIA

Mr. PITTS. The subcommittee will come to order. The chair will recognize himself for an opening statement. Today's hearing will examine several different legislative proposals that will address various aspects of the Public Health Service Act.

H.R. 1192, the National Diabetes Clinical Care Commission Act, amends the Public Health Service Act to foster more effective implementation and coordination of clinical care for people with prediabetes and the chronic diseases and conditions that result from diabetes.

Today, our witnesses will also be discussing potential changes to legislation that will make it less disease-specific, so the focus can be broader, to include related autoimmune and metabolic syndromes. According to the Centers for Disease Control and Prevention, CDC, almost 29 million Americans have diabetes, and an estimated 86 million American adults have prediabetes. Diabetes is the seventh leading cause of death in the United States. It is the leading cause of kidney failure. The total national cost associated with diabetes in 2012, according to the CDC, exceeded $245 billion. One in three Medicare dollars is currently spent upon people with

diabetes. There are 35 Federal departments, agencies, and offices involved with implementation of Federal diabetes activities. And this legislation will establish a commission to evaluate, recommend solutions for better coordination of patient care and ways to control costs across all of these offices.

And I thank my colleague, Representative Pete Olson, for sponsoring this important legislation, which will be welcome news for the over 100 million people afflicted with diabetes or prediabetes.

H.R. 1717, the Sober Truth on Preventing Underage Drinking Reauthorization Act, or the STOP Act, sponsored by Representative Roybal-Allard of California, provides for programs and activities to prevent underage drinking.

H.R. 1807, the Sickle Cell Disease Research, Surveillance, Prevention and Treatment Act of 2015, sponsored by Representative Danny Davis of Illinois and Dr. Michael Burgess of Texas, would reauthorize the Sickle Cell Disease Demonstration Program. Sickle cell disease has no cure. It leads to premature death. This legislation will hopefully move us one step closer to improving the quality of care and symptom management for those afflicted.

H.R. 3119, the Palliative Care and Hospice Education Training Act, sponsored by Representative Engel of New York, increases the number of permanent faculty in palliative care at accredited allopathic and osteopathic medical schools, nursing schools, social work schools, to promote education and research in palliative care and hospice and to support the development of faculty careers in academic palliative medicine.

H.R. 3952, the Congenital Heart Futures Reauthorization Act of 2015, sponsored by Representative Bilirakis of Florida, coordinates Federal congenital heart disease research efforts and improves public education and awareness of congenital heart disease.

Today, we will hear from one panel of experts and stakeholders as to their ideas and recommendations on these various bills.

We welcome all of you, and I now yield to Dr. Burgess.

[The prepared statement of Mr. Pitts follows:]

PREPARED STATEMENT OF HON. JOSEPH R. PITTS

The Subcommittee will come to order.

The Chairman will recognize himself for an opening statement.

Today's hearing will examine several different legislative proposals that will address various aspects of the Public Health Service Act.

H.R. 1192, the "National Diabetes Clinical Care Commission Act" amends the Public Health Service Act to foster more effective implementation and coordination of clinical care for people with pre-diabetes, diabetes, and the chronic diseases and conditions that result from diabetes. Today, our witness will also be discussing potential changes to the legislation that will make it less disease specific so the focus can be broader to include related autoimmune and metabolic syndromes.

According to the Centers for Disease Control and Prevention (CDC), almost 29,000,000 Americans have diabetes and an estimated 86,000,000 American adults have pre-diabetes. Diabetes is the seventh leading cause of death in the United States and is the leading cause of kidney failure.

Total national costs associated with diabetes in 2012 according to the CDC exceeded $245 billion. One in three Medicare dollars is currently spent on people with diabetes.

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Today we will hear from one panel of experts and stakeholders as to their ideas and recommendations on these various bills.

I will now yield to Dr. Burgess.

Mr. BURGESS. Thank you, Mr. Chairman.

And it is with a great deal of pleasure that I recognize from my neck of the woods, Dr. Leffert being here today. He is the president-elect of the American Association of Clinical Endocrinologists, and in another life, I used to refer patients to Dr. Leffert from my practice in Louisville, Texas, down to the big city specialist.

So, Jonathan, it is great to see you again. It is great to have you here as part of this committee.

And Ms. Banks, who is here on behalf of the Sickle Cell Disease Foundation, who will be talking about the disease that Danny Davis and I, Representative Davis and I, introduced a bill. We are going to be looking at legislation that seeks to identify and improve the overall public health of our country, and one of those bills is 1807, the Sickle Cell Disease Research, Surveillance, Prevention and Treatment Act of 2015. It was back in the middle 1970s when I was a resident at Parkland Hospital, our obstetric service there, Dr. Jack Pritchard, Dr. Eric Cunningham, sickle cell disease in pregnancy was a special project that they put a lot of effort into. And as a consequence, we ended up seeing a lot of patients referred from around the country to the program there at Parkland Hospital in the middle 1970s. It had been some time since I thought about it again, and then, with Representative Davis at one of your meetings here on the Hill, it really struck how there really hadn't been the advancements in this area that I thought there would have been by this time. So that is one of the things that this committee has been very active in the Cures for the 21st Century. We want those things that are supposed to be there by now, and I would include this as one of those things that we want to be there by now.

So thank you, Mr. Chairman. I will back and await the discussion.

Mr. PITTS. The chair thanks the gentlemen and now yields to the ranking member of the subcommittee, Mr. Green of Texas, 5 minutes for an opening statement.

OPENING STATEMENT OF HON. GENE GREEN, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF TEXAS

Mr. GREEN. Thank you, Mr. Chairman.

I want to welcome our panel today, and thank you for taking your time to come before us. We are examining five pieces of legislation that will improve public health and build on this committee's record of advancing and enacting very bipartisan bills. I want to thank the chairman for calling the hearing and our witnesses for being here with us this morning.

H.R. 1192, the National Diabetes Clinical Care Commission Act, was introduced and championed by my colleague on our committee but also my neighbor in Houston, Pete Olson, Congressman Olson, and Dave Loebsack, who is also a member of our committee. We would be considering a manager's amendment on H.R. 1192, which would establish a national clinical care commission to evaluate and offer recommendations to improve care, leverage resources, and coordinate efforts around complex metabolic, autoimmune, and insulin-related diseases. Through innovation and collaboration and maximizing return on investment, this important legislation provides the opportunity to address the enormous economic and human impact caused by diabetes and other disorders, and I am proud to be a cosponsor of this legislation.

H.R. 1717, the Sober Truth on Preventing Underage Drinking and Reauthorization Act, or the STOP Act, was introduced by Representative Lucille Roybal-Allard. She has been a tireless champion for this issue. In fact, she has talked to me—I think we came to Congress in 1993—and she has talked to me all of the time since then about trying to deal with drunk driving. I want to recognize her, as she is here, and thank her for her leadership.

The STOP Act will build on successful efforts to reduce underage drinking by reauthorizing a number of important public health programs and add an additional component of screening and intervention.

H.R. 1807, the Sickle Cell Disease Research, Surveillance, Prevention and Treatment Act, will enhance our ability to understand and survey and treat sickle cell disease. Sickle cell disease is a group of inherited red blood disorders that affect approximately 100,000 Americans. Unfortunately, it is difficult to diagnose, as symptoms can be severe, and the treatment requires comprehensive and complex care.

H.R. 1807, introduced by Representative Danny Davis and Mike Burgess, also on our committee, will promote research and prevention and treatment and emphasizes collaboration of community-based entities focusing on sickle cell disease.

H.R. 3119, Palliative Care and Hospice Education and Training Act, is an important bill to improve palliative care. Representative Eliot Engel, also a member of our committee, introduced this legislation, recognizing that palliative care enhances the quality of life for individuals with serious and life-threatening disease by treating the symptoms, the side effects, and emotional pain experienced by patients. H.R. 3119 would improve training for health professionals, enhance research in palliative and hospice care, and support projects to fund the training of physicians and nonphysician healthcare professionals entering the field of palliative care.

Finally, we are considering H.R. 3952, the Congenital Heart Futures Reauthorization Act. Each year, approximately 4,000 babies are born with congenital heart defects, making it the most common type of birth defect in the United States. It is estimated that 1 million children and 1.4 million adults live with congenital heart disorders. They require specialized care and face a lifelong risk of disability and premature death. The cause is unknown, but several genetic and environmental factors have been linked in the diseases. H.R. 3952, introduced by Representative Gus Bilirakis, also from our committee, and Adam Schiff and Eleanor Holmes Norton, builds on existing efforts by requiring the Centers for Disease Control and Prevention to enhance and expand its research, surveillance, and education outreach to providers and the public about congenital heart diseases. Under this legislation, the CDC would report to Congress on a cohort study to improve the knowledge of epidemiology of the disease across lifespans and implement an awareness campaign. I am proud to support each of these important bills and thank our sponsors and our committee for their commitment to improving public health and look forward to hearing from our witnesses and learning more about each of these bipartisan pieces of legislation.

Mr. Chairman, unless someone else wants my last 30 or 40 seconds, I will yield back.

Mr. PITTS. The chair thanks the gentleman.

I now recognize the vice chair of the full committee, the gentlelady from Tennessee, Ms. Marcia Blackburn, for 5 minutes for opening statement.

OPENING STATEMENT OF HON. MARSHA BLACKBURN, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF TENNESSEE

Mrs. BLACKBURN. Thank you, Mr. Chairman.

I want to thank our witnesses for being here.

And I am so pleased that we are taking some time to go through these bills. They all have a significance to public health. The diabetes bill, I had the opportunity before coming to Congress to serve on the Diabetes Association Board in my state, and I appreciate Representative Olson's good work on looking at this issue.

Of course, the underage drinking bill, when I was in the state senate, this is something at the state level where we put a tremendous amount of effort and energy, and Tennessee, my home State, continues to do so. I know that Reps. Davis and Burgess have worked hard on the sickle cell disease. It does need more attention. It needs more research. It needs more focus, so we are pleased to see that.

We all have heard about palliative care, the importance of that, as we have been at home and in our districts and talked to families and to healthcare providers and beginning to think this through and look for new models. And certainly, in Nashville, we have a tremendous amount of research and new focus that has come to bear on the delivery of palliative care. So I am pleased that we are moving forward there. You know, I have to say that H.R. 3952, going back to my days as being on the Board of Friends of Children's Hospital in Nashville and dealing with congenital heart dis-

ease, and as a mom and a grandmom now, having friends and knowing of families, individuals from church, that have a baby that is born with CHD, and just seeing firsthand, living through the anguish and the desperate reach for resources that can help with this, that can extend the life of that child. I am so pleased that we are moving this forward. I do hope that we will see NIH and other research entities focus on how we deal with this so that these precious children will live long past that 18th birthday and will be able to move toward enjoying a full and productive life.

Mr. Chairman, I will yield my time to whomever would like it.

Mr. PITTS. Anyone on the majority seeking time?

If not, the gentlelady yields back.

The chair now recognizes Judge Butterfield, North Carolina, 5 minutes for opening statement in place of Mr. Pallone.

OPENING STATEMENT OF HON. G.K. BUTTERFIELD, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF NORTH CAROLINA

Mr. BUTTERFIELD. Thank you. I too want to thank you, Chairman Pitts, for holding this important hearing on reauthorizing important programs to combat the sickle cell disease and on improving diabetes awareness and care. Let me join my colleagues in thanking the five witnesses for coming forward today to testify and to give us the benefit of your expertise. I know you just didn't wake up this morning and come to this room. You have been preparing for this day, and we thank you for your work.

Sickle cell disease and diabetes disproportionately affect African American citizens, including many in my congressional district in North Carolina. In fact, more than 30 years ago, I lost a first cousin to the disease. The two diseases, if not properly managed, can land people in the hospital multiple times. In fact, a 2010 study published in the journal of the American Medical Association shows that people with SCD are hospitalized nearly three times per year. Many people who have SCD are unaware of it before tests can confirm the illness. Even those who know they have SCD find themselves back in the hospital with problems with pain or other morbidities. SCD is a serious disease which can dramatically reduce life expectancy. A study in the New England Journal of Medicine found that the median age for men with SCD is only 42 years old. For women, it is 48 years. The disease is caused by a small genetic abnormality that deforms blood cells and causes them to block blood flow. SCD can lead to the development of other conditions, ranging from heart disease and stroke to kidney or liver problems.

It is estimated, Mr. Chairman, that 100,000 Americans have SCD. Many more have sickle cell trait, although they never experience symptoms, may not even know that they have the trait. Children can inherent SCD if both of their parents have SCD or sickle cell trait and certain genes are passed on to them. Many of those who have SCD are African Americans. More than 1 out of every 400 African Americans have SCD. That is 1 out of 400, and 1 of every 13 has the sickle cell trait.

And so I applaud my friend and colleague Congressman Danny Davis and other colleagues that have done likewise—from Chi-

cago—for his longtime advocacy for SCD health programs and his reintroduction of H.R. 1807, the Sickle Cell Disease Research, Surveillance, Prevention and Treatment Act of 2015. This legislation is a priority for many members of the Congressional Black Caucus, and I am proud to support it. And this important bill would reauthorize the sickle cell disease Treatment Demonstration Program, improve research and surveillance of the disease, and support a grant program for states to develop and implement prevention and treatment strategies.

This bill, Mr. Chairman, is a clean reauthorization and would not increase government spending. It is a meaningful first step to help prevent and treat SCD, and I urge my colleagues to support this important bill. We can, we must do more to support those with this disease. SCD does not currently have a cure. No treatments have been approved since 1998. For that reason, I have long advocated to include SCD in the Pediatric Priority Review Voucher Program, the PRV, run by the Food and Drug Administration. I am encouraged that there is currently a viable treatment in clinical trials at the FDA, but we must do all we can to spur innovation in rare pediatric disease spaces. More than 100,000 Americans are counting on us to support sickle cell disease prevention and treatment programs and need our help to find a cure. I urge my colleagues to support 1807, and I yield back. Thank you.

Mr. PITTS. The chair thanks the gentleman.

That concludes the opening statements. As usual, the written opening statements of all members will be made a part of the record.

And we welcome, as was noted previously, Congresswoman Roybal-Allard to sit with the committee today.

Without objection, so ordered.

And I ask unanimous consent to submit the following for the record: a letter from 43 organizations representing physicians, allied health professionals, patients, community health organizers, and industry; as well as statements from the Academy of Nutrition and Dietetics, Novo Nordisk, and Diabetes Advocacy Alliance, all regarding H.R. 1192; and both a statement from the American Society of Hematology regarding H.R. 1807 along with their State of Sickle Cell Disease 2016 Report.

Without objection, so ordered.

[The information appears at the conclusion of the hearing.]

Mr. PITTS. I will now introduce the panelists in the order of which they will speak, and as usual, your written statements will be made a part of the record, and you will each be recognized for 5 minutes for a summary.

So, first, Dr. Jonathan Leffert, Acting Director and President-elect of the American Association of Clinical Endocrinologists; secondly, General Arthur Dean, Chairman and CEO, Community Anti-Drug Coalitions of America; and Sonja L. Banks, President and COO of Sickle Cell Disease Association of America, Inc.; then Dr. Sean Morrison, Professor and Vice Chair, Brookdale Department of Geriatrics and Palliative Medicine, Icahn School of Medicine at Mount Sinai, and Director of the National Palliative Care Research Center; and, finally, Dr. Brad Marino, Chair, Pediatric Congenital Heart Association.

Thank you for coming today.

And, Dr. Leffert, you are recognized 5 minutes for your summary at this time.

STATEMENTS OF JONATHAN LEFFERT, M.D., FACP, FACE, ECNU, PRESIDENT-ELECT, AMERICAN ASSOCIATION OF CLINICAL ENDOCRINOLOGISTS; ARTHUR DEAN, CHAIRMAN AND CEO, COMMUNITY ANTI-DRUG COALITIONS OF AMERICA; SONJA L. BANKS, PRESIDENT AND COO, SICKLE CELL DISEASE ASSOCIATION OF AMERICA, INC.; R. SEAN MORRISON, M.D., PROFESSOR AND VICE CHAIR, BROOKDALE DEPARTMENT OF GERIATRICS AND PALLIATIVE MEDICINE, ICAHN SCHOOL OF MEDICINE AT MOUNT SINAI, AND DIRECTOR, NATIONAL PALLIATIVE CARE RESEARCH CENTER; AND BRAD MARINO, M.D., MPP, MSCE, CHAIR PEDIATRIC CONGENITAL HEART ASSOCIATION

STATEMENT OF JONATHAN LEFFERT

Dr. LEFFERT. Thank you, Mr. Chairman, and, Dr. Burgess, for your kind words. My name is Jonathan Leffert, and I am a clinical endocrinologist from Dallas, Texas, and the current president-elect of the American Association of Clinical Endocrinologists. On behalf of our 7,000 members, I would like to thank you for this opportunity to testify about H.R. 1192, the National Diabetes Clinical Care Commission Act. The subcommittee should be commended for addressing diabetes and recommending to expand the scope of H.R. 1192 to include other metabolic and autoimmune diseases and diseases resulting from insulin deficiency and insulin resistance. We appreciate the opportunity to work with the bill's sponsors, Representative Pete Olson and Representative Dave Loebsack, in this subcommittee on consensus language to amend H.R. 1192.

I will focus my comments today on diabetes, which represents a significant part of my medical practice as a clinical endocrinologist and is the most prevalent of the diseases that will be addressed by an amended H.R. 1192.

According to the Centers for Disease Control and Prevention, the number of Americans diagnosed with diabetes over the course of the last 35 years has increased more than fivefold, from 5.5 million Americans in 1980 to 29.1 million in 2014. The CDC estimates that there are 86 million Americans with prediabetes, a condition known to progress to diabetes without appropriate intervention. Diabetes is also the catalyst for many other diseases. Diabetes is the leading cause of new cases of blindness among adults. Diabetes is the leading cause of kidney failure. Diabetes causes increased death rates from cardiovascular disease and higher rates of hospitalization from heart attack and stroke. Diabetes is the seventh leading cause of death in the United States. The total cost of diabetes to the Nation in 2012 exceeded $322 billion. Sixty-two percent of this cost is borne by the U.S. Government through programs like Medicare and Medicaid. By 2025, the total cost of diabetes is projected to reach $514 billion, a level comparable to the entire Medicare budget. Our Nation cannot afford for the current diabetes prevalence and cost trends to continue. Congress should not let another session go by without addressing this critical health crisis.

H.R. 1192 provides a cost-effective approach to begin to address diabetes and the many other diseases and diagnoses encompassed by this legislation. The commission established in H.R. 1192 will provide a venue where the expertise of specialists, primary care physicians, allied healthcare professionals, and patient advocates will help our Federal Government partner to evaluate current programs so they are meeting the goal of improving the quality of patient care.

The commission will also facilitate improved coordination and communication among Federal agencies. Consider the example of the FDA approved continuous glucose monitors, referred to as CGMs. These devices are indispensable to patients with type 1 diabetes by allowing them to constantly monitor blood glucose levels. Patients with this device no longer fear losing consciousness from low blood sugar or enduring complications from constantly high blood sugar levels. Nearly all private insurance plans cover CGMs. However, once a patient turns 65 and enrolls in the Medicare program, coverage for this lifesaving device is no longer available. Remedies to fix these issues often require an act of Congress, which places Congress in a position to do the job of the regulatory agencies that failed to work together.

Having the commission available to work through issues such as this will help all parties to find and implement meaningful solutions. The expertise on the commission would also be utilized to prioritize the clinician training and deployment of new revolutionary technologies, such as the artificial pancreas, to ensure patient access to these medical innovations is not comprised.

Agencies can and must work together in a coordinated national response driven by research experts, specialists, healthcare professionals, and people living with diabetes. The commission established under H.R. 1192 will help achieve this important objective.

On behalf of the American Association of Clinical Endocrinologists, I would like to thank the members of the committee for the opportunity to testify today on H.R. 1192, and I urge you to act now and move this bill forward, ensuring its passage by the U.S. House of Representatives as soon as possible.

In addition to the 220 Members of Congress who have cosponsored H.R. 1192, including many who are members of this committee, I would like to thank the 45 organizations representing the patients, physicians, allied health professionals, community organizations, and industry, and the Diabetes Advocacy Alliance, who have helped to advance this legislation.

[The prepared statement of Dr. Leffert follows:]

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TESTIMONY

of

Jonathan D. Leffert, MD, FACP, FACE, ECNU

on Behalf of

American Association of Clinical Endocrinologists

before the

U.S. House of Representatives Committee on Energy & Commerce
Subcommittee on Health

September 8, 2016

Thank you Mr. Chairman. My name is Jonathan Leffert, and I am a clinical endocrinologist from Dallas, Texas and the current President-Elect of the American Association of Clinical Endocrinologists (AACE). On behalf of the 7,000 members of the AACE, I would like to thank you for this opportunity to testify about H.R. 1192, the National Diabetes Clinical Care Commission Act, which is sponsored by Representatives Pete Olson (R-TX) and Dave Loebsack (D-IA), and the necessity that this bipartisan legislation be enacted as soon as possible. The Subcommittee should be commended for addressing this devastating gateway disease and for broadening the scope of this bill by offering an amendment, should it be considered by this Subcommittee later this year, to include other metabolic and autoimmune disease, diseases resulting from insulin deficiency and insulin resistance and their complications. We appreciated the opportunity to work with our sponsors and this Subcommittee on the language for this amendment.

However, I am going to focus my comments today on diabetes, which represents a significant part of my medical practice as a clinical endocrinologist and is one of the most prevalent of the diseases that will be addressed by an amended H.R. 1192.

According to the Centers for Disease Control and Prevention (CDC), the number of Americans with diagnosed diabetes over the course of the last 35 years has increased more than five-fold, from 5.5 million Americans in 1980 to 29.1 million in 2014. The CDC estimates there are 86 million Americans with pre-diabetes, a condition known to progress to diabetes without appropriate intervention.

Diabetes is also the catalyst for many of the other diseases in these disease clusters that will be addressed by this legislation as amended, such as cardiovascular disease and chronic kidney disease.

The National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) defines diabetes as "*a complex group of diseases with a variety of causes. People with diabetes have high blood glucose, also called high blood sugar or hyperglycemia. Over time, high blood glucose damages nerves and blood vessels, leading to complications such as heart disease, stroke, kidney disease, blindness, dental disease and amputations.*

The role that diabetes plays in the onset of many other diseases and debilitating conditions cannot be understated.

- Diabetes is the leading cause of new cases of blindness among adults
- Diabetes is the leading cause of kidney failure
- 60% of all non-traumatic lower limb amputations in the U.S. occur in individuals with diabetes
- 60-70% of individuals with diabetes have neuropathies or nerve disorders

- Individuals with diagnosed diabetes have increased death rates from cardiovascular disease and higher rates of hospitalization for heart attack and stroke

Although many of the disease complications of diabetes are preventable, millions of Americans suffer the devastating consequences of disease progression, experiencing reduced quality of life, productivity and even death. Diabetes is the 7th leading cause of death in the United States.

As stewards of taxpayer funds and the federal budget, you are probably aware of the economic impact of the diabetes epidemic. The total cost of diabetes to the nation in 2012 exceeded $322 billion and 62% of this cost is borne by the U.S. government through programs like Medicare and Medicaid. By 2025, the total cost of diabetes is projected to reach $514 billion – a level comparable to the entire Medicare budget. Currently, $1 out of every $3 Medicare dollars is spent on people with diabetes.

It is not surprising that global diabetes trends are mirroring the trajectory of the disease in the United States. An estimated 422 million adults were living with diabetes in 2014, compared to 108 million in 1980. So concerning is this trend that the World Health Organization's *Global Report on Diabetes* released earlier this year called on governments around the globe to take more aggressive steps in fighting the rising number of diabetes cases worldwide.

I am sure you will agree with me that our nation cannot afford for the current diabetes prevalence and cost trends to continue. Maintaining the status quo with respect to this disease is not an option. Our country needs to better leverage the resources spent on medical research and patient care and use them more effectively to address diseases and disorders of the metabolic system, the autoimmune system and those due to insulin resistance. If not addressed, diabetes

and its disease complications have the very real potential of bankrupting the federal health programs that provide care to over 125 million Americans.

While there is no "cure" at present for diabetes and its devastating complications and no single solution for addressing the disease, we have many tools that can improve the quality of life for people with diabetes and its complications. Our healthcare system can and should do better for patients. Congress should not let another session go by without addressing this critical health crisis.

By passing H.R. 1192, Congress will advance legislation that provides a cost-effective approach to begin to address diabetes and the many other disease diagnoses encompassed in this legislation. Through innovation, collaboration, and application of advances in care called for in this bill, the federal government can better leverage taxpayer dollars invested in diabetes research to reduce the staggering impact of diabetes and its disease complications on health care spending. At the same time, care will be improved for the tens of millions of Americans living with diabetes.

The Commission established under this legislation will represent a partnership between the private sector experts who work with patients with diabetes on a daily basis and Federal agency representatives who are active in clinical diabetes care. The Commission will provide a venue where the expertise of specialists, primary care physicians, allied healthcare professionals and patient advocates will help our federal government partners to evaluate current programs to ensure they are meeting the goal of improving the quality of patient care delivered to people with diseases and disorders of the metabolic system, the autoimmune system and those due to insulin resistance. For example, improved coordination among agencies would make a difference in the lives of Medicare patients with Type 1 diabetes mellitus and their use of the continuous glucose

monitoring device (CGMs). CGMs detect and display real-time glucose levels in five minute intervals. This data is transmitted wirelessly, allowing individuals and those who care for them to constantly monitor glucose levels. This technology enables better blood glucose control and helps users live their life without the fear of losing consciousness from low blood sugar or enduring complications from constantly high blood sugar levels. These FDA-approved devices are indispensable for the patient with type 1 diabetes. Nearly all private insurance plans cover CGMs; however, once a patient turns 65 and enrolls in the Medicare program, coverage for the life-saving device is no longer available. A key tool in the patient's care plan "tool box" is suddenly removed, which places the patient's health and safety in jeopardy. Such short-sighted policies reflect poor communication and coordination of federal diabetes activities and a broken system that does not work for patients. Remedies to fix these issues often require an act of Congress, which places patients in an abyss and Congress in a position to do the job of the regulatory agencies that failed to work together.

The CGM example suggests that the health care system must be better-prepared for the deployment of new technologies so they are accessible to patients as they are approved by the FDA. The coming years promise new device and medication innovations, such as the artificial pancreas, which will be pivotal for patients with diabetes. Having an effective, well-organized federal response to activate these innovations will help millions of Americans with this devastating disease. This will require the government to change the status quo of agencies working in silos to a coordinated national response, driven by research experts, specialists, health care professionals and people living with diabetes.

The Commission will not only help improve patient care through better communication and coordination among federal agencies, but it will also help facilitate the dissemination of innovative clinical practices and resources resulting from federal research to the physician practice at the local level, so patients have access to high-quality, optimal care.

The Commission established under H.R. 1192 can play an important role in this effort as it provides the solution to the dual problem of cost and access in the federal government's response to patients with diabetes. We can attain this goal without allocating any new funds, as the cost of this Commission is to be paid out of the existing HHS budget. The bill language explicitly states that the cost of convening the Commission shall come from existing HHS funds.

In addition to the 220 Members of Congress who have supported H.R. 1192, including many who are members of this Committee, I would like to thank the 45 organizations representing the patients, physicians, allied health professionals, community organizations and industry, including the Juvenile Diabetes Research Fund (JDRF) and the American Diabetes Association (ADA), and the Diabetes Advocacy Alliance (DAA) who have helped to advance this legislation.

On behalf of AACE, I would like to thank the Members of this Committee for the opportunity to testify today on H.R. 1192 and I urge you to act now and move this bill forward, ensuring its passage by the U.S. House of Representatives as soon as possible. Please do not let the opportunity pass to do something to reverse the trajectory of these expensive and debilitating diseases and to improve the health and quality of life for patients with diabetes and its associated diseases and complications.

Mr. PITTS. The chair thanks the gentleman for his summary and now recognizes General Dean 5 minutes for his summary.

STATEMENT OF ARTHUR DEAN

Mr. DEAN. Chairman Pitts, Ranking Member Green, and esteemed members of the subcommittee on Health, it is my pleasure to testify in support of H.R. 1717, the Sober Truth on Preventing Underage Drinking Act, so-called the STOP Act.

I would also like to thank the bill's sponsor, Congresswoman Lucille Roybal-Allard, for her steadfast leadership on this issue, and I thank you for the support and leadership you provided to us.

I am General Arthur Dean, currently serving as the Chairman and CEO of Community Anti-Drug Coalitions of America, a membership based, not-for-profit organization, commonly called CADCA. CADCA is a national nonprofit organization, and our mission is to build and strengthen the capacity of local citizens, and we put them into what we call community coalitions designed for them to have the capacity and the skills to build safe, healthy, and drug-free communities in the U.S. and around the world. We work with more than 5,000 communities in the U.S., and it is our role to be here. And they are very, very concerned about the prevention and the reduction and combatting underage drinking. Back in 2003, the National Research Council and the Institute of Medicine published a report titled "Reducing Underage Drinking: A Collective Response." This report cited serious underage drinking and recommended critical components for a national strategy to reduce alcohol consumption by minors. The original STOP Act builds upon these recommendations, and in 2016, the bill passed the House of Congress with a unanimous bipartisan support. Authorization for the law expired in 2010, as you know, which makes it urgent that Congress pass a reauthorization as soon as possible.

H.R. 1717, the STOP Act reauthorization bill, would maintain and enhance the original provisions of the STOP Act, and quickly I would describe some of those things that we are concerned about. It reauthorizes the highly successful community-based coalition enhancement grants. We take trained coalitions and give them a small grant to work this issue. Provides grants for current and former drug-free community grantees and partners and allows them to partner with higher education to prevent underage drinking on college campuses. It reauthorizes the Interagency Coordinating Committee to Prevent Underage Drinking—we call that ICCPUD—which coordinates the efforts of 16 Federal agencies to combat this problem. It reauthorizes a highly visible national adult-oriented media campaign to raise the awareness of this issue and provide education. It reauthorizes epidemiology studies on excessive drinking and analyzes how young people drink and how they obtain alcohol in the relationship associated with that.

And, lastly, 1717 creates a new grant program for pediatric healthcare providers. We think it is important that those that are treating our youth understand and focus on best practices around screening, brief intervention, and referral as appropriate.

In the 10 years that have passed since STOP Act was created, it is clear that law's coordinated provisions have effectively been reducing underage drinking. As we look at the most recent Moni-

toring the Future study, it shows that lifetime alcohol use by those in the 8th grade, 10th grade, and 12th grade is currently at the lowest level since each of these grades were included in the study. While this is welcome news—it is important news—underage drinking continues to be a very serious problem that is faced in this country: 17.2 percent, or nearly one in six, high school seniors still binge drink, which is unfortunate. Between 2006 and 2010, approximately 4,300 young people under the age of 21 died from excessive drinking, which is critical. And the total annual economic costs of underage drinking are estimated at $24.6 billion.

So H.R. 1717 builds upon the effective data-driven, Drug-Free Communities Program as the most cost-effective way to prevent and reduce underage drinking. The community-based coalition enhancement grants included in the STOP Act are just one vital component of a comprehensive approach to improve public health and address underage drinking.

I respectfully urge the committee to support swift passage of H.R. 1717, the Sober Truth on Preventing Underage Drinking Act. I will be submitting a detailed statement for the record, which includes information for your consideration.

And we ask that, as in the past, this bill be unanimously supported and passed quickly so that it can become law during this session of Congress. I thank you so very much for your attention to this issue, and we understand that underage drinking, although data says we have made progress, there is much progress that still needs to be made, and many, many young people will be better served if this passes, takes place quickly. Thank you very much.

[The prepared statement of Mr. Dean follows:]

CADCA®

Written Statement of

General Arthur T. Dean

Chairman and CEO, Community Anti-Drug Coalitions of America (CADCA)

for

House Energy and Commerce Committee Hearing

in support of H.R. 1717, the Sober Truth on Preventing Underage Drinking (STOP) Act

September 8, 2016

Chairman Upton, Ranking Member Pallone, and esteemed Members of the Committee, it is my pleasure to testify today in support of H.R. 1717, the Sober Truth on Preventing Underage Drinking Act, also called the STOP Act. I am General Arthur Dean, Chairman and CEO of Community Anti-Drug Coalitions of America (CADCA).

CADCA is a national non-profit whose mission is to build and strengthen the capacity of community coalitions to create safe, healthy, and drug-free communities globally. Working with the more than 5,000 member coalitions around the United States, CADCA has for a very long time been at the forefront of efforts to prevent, reduce, and combat underage drinking.

Back in 2003, the National Research Council and Institute of Medicine published a report titled, "Reducing Underage Drinking: A Collective Responsibility." This report placed the seriousness of underage drinking into context for the American public, and recommended a number of critical components as part of a national strategy to reduce alcohol consumption by minors. The original STOP Act built upon these recommendations, and included all of them in the final bill that passed both Houses of Congress in an overwhelmingly bipartisan basis in 2006.

CADCA, as well as other organizations as part of the National Alliance to Prevent Underage Drinking, were key supporters of getting the STOP Act passed at that time. Authorization for the law expired in 2010, making it urgent that Congress pass a reauthorization as soon as possible, and the 21 national organizations that are members of the National Alliance to Prevent Underage Drinking have come out in strong support of this bill (see attachment 1).

H.R. 1717, the STOP Act reauthorization bill, proposes to maintain and enhance the areas of policy development contained in the original STOP Act and adds an additional area, screening and brief intervention. It does all of this while staying within the existing overall authorized level of $18 million. Below are detailed descriptions of each of the components of H.R. 1717 (see attachment 2 for a detailed breakdown of comparisons of original authorization vs. reauthorization in H.R. 1717).

COMMUNITY-BASED PROGRAMS TO PREVENT UNDERAGE DRINKING

Community-Based Coalition Enhancement Grants ($6 million for each of FY 2016-2020)

These are grants of up to $50,000 for up to four years for current and former Drug-Free Communities (DFC) grantees to enhance underage drinking prevention efforts. DFC grantees are well-placed to handle such enhancement grants because they are data-driven, they understand their community epidemiology, and are capable of implementing multi-sector interventions at preventing and reducing alcohol use. H.R. 1717 builds upon the effective, data-driven infrastructure of the Drug-Free Communities program as the most cost-effective [1] way to invest minimal federal dollars to prevent and reduce the number of youth who drink alcohol at the community level. As a condition of their grant, DFC grantees are required to engage in a tremendous amount of strategic planning, and therefore have identified the best practices and

[1] Swisher, J.D., Scherer and Yin, K. The Journal of Primary Prevention. "Cost-Benefit Estimates in Prevention Research." 25:2, October 2004

comprehensive, community-wide strategies that could be implemented with a minimal amount of resources. DFC grantees, and therefore STOP Act grantees, primarily focus on implementing strategies to ensure the entire community is targeted with comprehensive, multi-component strategies across all community sectors to change norms and reshape community environments to achieve population-level decreases in underage drinking rates.

This community-based approach has been very successful (see attachment 3). The most recent independent national evaluation of the DFC program showed that, among all DFC grantees ever funded from first report to latest report, 30-day use of alcohol declined by 24.4%[2], with past 30-day use of alcohol by high school students declining by 15.5%. [3] Individual STOP Act grantees, as well, have shown remarkable results: North Coastal Prevention Coalition, from the Tri-City area of San Diego County in California, markedly decreased past 30-day alcohol use and binge drinking 11th grade students between 2008 and 2011 (see attachment 4). The Coalition for Healthy and Safe Families, another STOP Act grantee, from Newton, New Jersey, showed a decrease in past 30-day use of alcohol among high school seniors from 63% in 2010 to 51.9% in 2012 (see attachment 5).

Some example strategies that grantees have engaged in include:

- Conducting community-wide education campaigns
- Conducting vendor and server training

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[2] Available here: https://www.whitehouse.gov/sites/default/files/DFC2014Interim%20ReportExecutiveSummaryFinal.pdf.

[3] Ibid.

- Conducting compliance checks
- Mobilizing the community to restrict youth access to alcohol
- Strengthening and enforcing underage sales/services laws

There is tremendous interest in and demand for these grants by the substance abuse prevention field, as can be seen from the chart below:

Number of Coalition Enhancement Grants Awarded			
Fiscal Year	Appropriated Level	Number of Applicants for New Grants	Number of New Grants Awarded
2008	$4 million	419	79
2009	$5 million	427	23
2010	$5 million	0	0
2011	$5 million	0	0
2012	$5 million	364	81
2013	$5 million	247	17
2014	$5 million	0	0
2015	$5 million	0	0
2016	$5 million	0	0

GRANTS FOR PARTNERSHIPS BETWEEN COMMUNITY COALITIONS AND INSTITUTIONS OF HIGHER EDUCATION ($2.5 million for each of FY 2016-2020)

This provision provides grants to current and former DFC grantees to partner with institutions of higher education to prevent and reduce the rate of underage alcohol consumption, including binge drinking on college campuses. Grants may be awarded up to $100,000 per year for up to four years. Coalitions will work hand in hand with institutions of higher education to reduce the consumption and abuse of alcohol through comprehensive, community-wide and evidence-based strategies that change environments and norms. Specific focus is on reducing incidents of alcohol use and abuse for students under 21 and to include the identification of incidents of violations, physical assaults, sexual assaults, reports of intimidation, disruption of school functions, disruption of students' studies, mental health referrals, illnesses, alcohol-related visits to emergency departments and deaths. Note that this modifies the existing authorization by changing eligible applicants from a State, institution of higher education, or non-profit entity to DFC coalitions, and cuts the funding level from $5 million to $2.5 million (see attachment 2 for more information).

INTERAGENCY COORDINATING COMMITTEE TO PREVENT UNDERAGE DRINKING (ICCPUD); ANNUAL REPORT ON UNDERAGE DRINKING PREVENTION AND ENFORCEMENT ACTIVITIES ($1 million for each of FY 2016-2020)

H.R. 1717 reauthorizes the Interagency Coordinating Committee to Prevent Underage Drinking (ICCPUD), which coordinates the efforts and expertise of sixteen federal agencies to combat

underage drinking. It was formed in 2004 to assist in the development of" A Comprehensive Plan for Preventing and Reducing Underage Drinking", which Congress called for in 2004. The STOP Act, enacted in 2006, formally established ICCPUD and called for enhancing its prevention efforts. Its purpose is, in addition to coordinating federal efforts, to provide ongoing, high-level leadership and guidance on policy and program developments across the federal government with respect to underage drinking; and also writes an Annual Report to Congress with a comprehensive assessment of federal efforts and the status of underage drinking prevention in the country, including state-specific information on reducing underage drinking (see attachment 6 and 7).

NATIONAL ADULT-ORIENTED MEDIA CAMPAIGN TO PREVENT UNDERAGE DRINKING ($1 million for each FY 2016-2017 and such sums as necessary for FY 2018-2020)

The highly-visible National Adult-Oriented Media Campaign is meant to raise awareness about the issue of underage drinking. Carried out through the Substance Abuse and Mental Health Services Administration (SAMHSA), the online presence of the media campaign can be found on www.samhsa.gov/under-drinking. The current campaign, titled, "Talk. They Hear You", aims to increase parents' awareness of the prevalence and risk of underage drinking; equip parents with skills and understanding to prevent underage drinking; and to increase parents' taking action to prevent underage drinking. (see attachment 8)

EPIDEMIOLOGICAL STUDIES ON EXCESSIVE AND UNDERAGE DRINKING ($4.5 million for each of FY 2016-2020)

Reauthorizes epidemiological studies on excessive drinking and underage alcohol use to provide better data in support of evaluation of community and other efforts to reduce and prevent underage drinking. The work, done at the CDC, is improving the collection and usefulness of data in support of effective and comprehensive community-based approaches to underage drinking by funding dedicated alcohol epidemiologists in at least five states. Drawing on its Youth Risk Behavior Surveillance System, it is analyzing and reporting more precise information than is currently available on how young people drink, where they obtain alcohol, and the relationship between youth alcohol use and a wide range of risk behaviors including suicide, drinking-driving, physical fighting, and risky sexual behavior. CDC uniquely tracks the cost of underage drinking at the federal and state levels, and monitors youth exposure to alcohol advertising to show how alcohol industry voluntary standards may be more effectively applied to reduce youth exposure. It also tracks at the national and state level the deaths and years of life among persons under age 21 because of excessive alcohol use. (see attachment 9).

REDUCING UNDERAGE DRINKING THROUGH SCREENING AND BRIEF INTERVENTION ($3 million for each of FY 2016-2020)

In a change from the original authorization, H.R. 1717 provides a new grant program for pediatric health care providers on best practices for screening children and adolescents for alcohol use, offering brief interventions to discourage drinking, and referring those in need to treatment. By training appropriate health care providers in the Screening, Brief Intervention, and Referral to Treatment (SBIRT) model and disseminating best practices, SBIRT widens the net to help youth who have started misusing substances and has been shown to be effective at reducing alcohol consumption. A 2009 study on SBIRT found that individuals who were screened positive for hazardous drug and alcohol use noticed a 39% reduction in heavy alcohol consumption six months later.[4] There is a great need for SBIRT for those youth who have initiated substance use in order to stop the trajectory towards addiction as early as possible. This new funding will provide critical support to the remaining provisions of the STOP Act.

In the 10 years since the initial STOP Act passed, it has become clear that the law's coordinated provisions have worked effectively to reduce underage drinking, as the most recent Monitoring the Future study showed that lifetime alcohol use by 8th, 10th, and 12th graders is currently at the lowest levels since each grade was included in the study.[5] While this is very welcome news, underage drinking still remains a serious public health issue facing the country. Here are some facts on underage drinking that will show you just how serious this crisis is:

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[4] Madras BK, Compton WM, Avula D, Stegbauer T, Stein JB, Clark HW. Screening, brief interventions, referral to treatment for illicit drug and alcohol use at multiple healthcare sites: Comparison at intake and 6 months. Drug and Alcohol Dependence. 2009;99(1-3):280-295.

[5] 2015 Monitoring the Future Survey.

- Despite the major declines in youth underage drinking rates, alcohol remains by far the preferred substance of choice for young people, with 35.3% of high school seniors drinking in the past month, compared to 11.4% for cigarettes and 23.6% for illicit drugs.[6]
- 17.2%, or nearly 1 in 6, high school seniors still binge drink on at least one occasion in the past two weeks.[7]
- The total annual economic costs of underage drinking are estimated at $24.6 billion, with $3.7 billion in health care expenditures and other impacts on society, such as loss of productivity, car crashes, and more, at $20.9 billion.[8]
- Between 2006 and 2010, approximately 4,300 young people under the age of 21 died due to excessive drinking.[9]
- Youth who use alcohol may remember 10% less of what they have learned than those who don't drink.[10] According to recent research, 16% to 18% of teen drinkers have missed school or work because of alcohol use.[11]
- Among college students, underage drinking was involved in 97,000 incidents of alcohol related sexual assault or date rape and 690,000 students were assaulted by students who were drinking.[12]

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6 Ibid.

7 Ibid.

8 Sacks, J. J., J. Roeber, E. E. Bouchery, K. Gonzales, F. J. Chaloupka and R. D. Brewer (2013). "State costs of excessive alcohol consumption, 2006." American Journal of Preventive Medicine 45(4): 474-485.

9 Centers for Disease Control and Prevention. Alcohol Related Disease Impact (ARDI) application, 2013. Available at http://apps.nccd.cdc.gov/DACH_ARDI/Default.aspx.

10 Brown, S.A., Tapert, S.F., Granholm, E., et al. (2000). Neurocognitive functioning of adolescents: Effects of protracted alcohol use. Alcoholism: Clinical and experimental research, (24)(2).

11 "Teenagers and alcohol misuse in the United States: By any definition, it's a big problem." Addiction, {91)(10), 1489-1503.

12 National Institutes of Health. National Institute on Alcohol Abuse and Alcoholism. 2013} College Drinking. Available at http:Uwww.niaaa.nih.gov/alcohol-health/special-populations-co-occurring-disorders/college-drinking.

The stakes are high to maintain national efforts at reducing underage drinking. While the rate and number of young people drinking alcohol has come down in the past several years, it is still the main substance of choice for youths. Underage drinking has devastating consequences not only for the children who suffer the health, safety, and mortality consequences, but for their families, communities, and our country as a whole. I respectfully urge you to support passage of H.R. 1717, the Sober Truth on Preventing Underage Drinking Act.

Thank you for the opportunity to present CADCA's views on this important piece of legislation today.

Mr. PITTS. The chair thanks the gentleman.

I now recognize Ms. Banks 5 minutes for your summary.

STATEMENT OF SONJA L. BANKS

Ms. BANKS. Good morning, Chairman Pitts and Ranking Member Green——

Mr. PITTS. Could you turn on the microphone now? The light should be on.

Ms. BANKS. Good morning. Chairman Pitts, Ranking Member Green, this distinguished committee, thank you for holding this hearing and allowing me the opportunity to testify in support of H.R. 1807, the Sickle Cell Disease Research, Surveillance, Prevention and Treatment Act. I also would like to thank Dr. Burgess, the lead Republican cosponsor of this legislation, and Representative Butterfield, for their leadership on this important legislation. We would also be remiss if we did not recognize Representative Danny Davis for not only leading this important legislation but remaining one of the sickle cell community's key champions.

I am here representing the Sickle Cell Disease Association of America, the Nation's only organization working full-time on a national level to resolve issues surrounding sickle cell disease and sickle cell trait. Since 1971, SCDA has been on the forefront for improving the quality of life, health, and services for individuals and families impacted by sickle cell disease, promoting policies and research and fighting for a universal cure.

Now allow me to take you on a journey. I want you to think about the worst pain that you have ever experienced in your life, a broken bone, a stomach virus, a flu, or maybe for you women in here who have children, labor pains. Now I want you to take that pain, and I want you to magnify it by two. Now magnify it by five. Now magnify it by 10. Now imagine the pain hitting you at any time, anywhere, with no control, no treatment, and no way to manage it. Now imagine it rearing its ugly head monthly, weekly, and even daily. Though not the journey we all long for, it is one that is a reality. These unpredictable pain episodes are the hallmark of sickle cell disease and the reality for those who are afflicted with it. They can start as early as 6 months of age and span throughout the lifetime, impacting school, work, and ordinary daily living.

Sickle cell disease is an inherited blood disorder affecting approximately 100,000 Americans. This disease causes the destruction and deformation of red blood cells, producing extreme complications that could include stroke in children and adults, lung problems, chronic damage to organs, including kidneys, liver and spleen, and, yes, severe painful episodes, and even death. One in every 400 African American newborns have sickle cell disease, as does 1 in every 1,200 newborns in Hispanic descent.

Despite its first noted discovery well over 100 years ago, progress has been relatively slow, and the sickle cell community still faces numerous challenges. For instance, the average life expectancy of a person with sickle cell disease is relative young, age 40 to 45. Presently, there is only one medication that has been FDA approved to treat this disease. There is an overwhelming shortage of physicians that treat or specialize in sickle cell disease, which makes it very difficult for patients to have a primary care physi-

cian or medical home. A vast majority of our patients make the emergency room their medical home. There is no comprehensive model here to help reduce the major healthcare complexities that SCD patients encounter.

It is because of these challenges and more that H.R. 1807 is so crucial for the sickle cell community. Reauthorization is needed to assure program stability, establish more effective care coordination, set in motion a model of care, and allow for a broader reach into areas of the country where people with sickle cell disease are not adequately served. This legislation will allow states to receive Federal funding for patient counseling, education initiatives, and community outreach programs, set the groundwork for 25 sickle cell treatment centers across the country to treat our patients, support the continuance of a national coordinating and evaluation center, allow the Centers for Disease Control to establish and continue its surveillance program.

Through this initiative, we are hopeful that data collected would help us to understand and improve current estimates about the incidence and prevalence of sickle cell disease. Distinguished leaders, I humbly stand—and I know I am sitting—before you as an advocate. No, I do not have sickle cell disease. I am not personally affected by it. I do not have anyone in my family with it, but I am an advocate. I, like you, believe that every American deserves equitable quality of life. Individuals with sickle cell disease deserve better treatment. They deserve better access to care, and more importantly, they deserve a better quality of life. So will you stand with me and support this legislation? It can and it will change many lives for the better. Thank you.

[The prepared statement of Ms. Banks follows:]

Testimony of Sonja L. Banks

President, Sickle Cell Disease Association of America

"Examining Legislation to Improve Public Health"

Energy & Commerce Subcommittee on Health

September 8, 2016

Summary

Historical Background

I. Current State of Federal Law: Congress enacted and President Nixon signed into law the *Sickle Cell Anemia Control Act* in 1972. At the time, President Nixon pledged that his Administration would "reverse the record of neglect on the dreaded disease." Under that first law, Congress authorized $10 million to expand sickle cell programs in fiscal year 1972, with the goal of increasing funding in FY1973 and expanding SCD-related activities at the Veterans Administration.

Over 30 years later, Congress and President George W. Bush enacted the *Sickle Cell Treatment Act of 2003* (SCTA) as an amendment to the *American Jobs Creation Act of 2004* (P.L. 108-357). Original cosponsors Senator Charles Schumer (D-NY), Senator Richard Burr (R-NC) and Representative Danny Davis (D-IL) were instrumental in introducing the SCTA within the Jobs Creation Act.

II. Problem: Authorization for the SCTA expired on September 30, 2009. As a result, funding for SCTA programs, while not entirely gone, has diminished in the more than seven years that the reauthorization for the Act's programs has not occurred. The reach and scope of current activities suffer significantly from under-funding at the national level, with only a maximum of 9-10 treatments centers/sites were ever funded.

H.R. 1807

While work is still progressing, the HRSA grant programs need to be reauthorized to assure program stability and allow for a broader reach into areas of the country where people with sickle cell disease are not being adequately served. HR 1807 will also authorize a much needed surveillance program in SCD and other outreach and education efforts through the CDC.

Key Points:

- New name "The Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2013"
- Allows states to receive federal funding for patient counseling, educational initiatives and community outreach programs
- Sets the groundwork for the development of up to 25 sickle cell treatment centers located across the country and establishes a National Coordinating Center for Sickle Cell Disease.
- Supports the continuance of a National Coordinating and Evaluation Center and (6) community-based demonstration sites that provide SCD follow-up and other services to support comprehensive care for newborns diagnosed with SCD.
- Expands the development of transition services for adolescents to adult health care
- Seeks authorization of Centers for Disease Control (CDC) to continue and establish a Hemoglobinopathies Surveillance System program and SCD public health promotion initiatives.

Testimony of Sonja L. Banks

President, Sickle Cell Disease Association of America

"Examining Legislation to Improve Public Health"

Energy & Commerce Subcommittee on Health

September 8, 2016

Chairman Pitts and Ranking Member Green, on behalf of the Sickle Cell Disease Association of America, Inc. (SCDAA) it 45 member organizations, over 35 affiliate partners and 100,000 plus patients and families we represent I want to thank you for holding this hearing and allowing me the opportunity to testify in support of H.R. 1807, the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act. I also would like to thank Dr. Burgess, the lead Republican cosponsor of this legislation, and Rep. Butterfield, for their leadership on this important legislation. We would also be remiss if we did not recognize Rep. Danny Davis for not only leading this important legislation but remaining one of the sickle cell community's key champions.

I am here today representing the nation's only organization working full time on a national level to resolve issues surrounding sickle cell disease and sickle cell trait. Since 1971, the SCDAA has been on the forefront of improving the quality of health, life and services for individuals, families and communities impacted by sickle cell disease. Additionally, SCDAA has been and remains

instrumental in promoting policies and research facilitating the continued search for a "universal" cure for sickle cell disease.

Sickle cell disease is an inherited blood disorder affecting approximately 100,000 Americans. The disease causes the destruction and deformation of red blood cells due to the presence of abnormal hemoglobin, which results in anemia and vaso-occlusion, which essentially means the blocking of blood vessels due to sticky, inflexible, sickled - shaped red blood cells. One in every 400 African American newborns has sickle cell disease, as does one in every 1,200 newborns of Hispanic descent.

The consequences and complications of this disease are extreme. Common complications include early childhood death from infection, stroke in young children and adults, lung problems similar to pneumonia, chronic damage to organs including the kidney, leading to kidney failure, and to the lungs causing pulmonary hypertension, and severe painful episodes. In fact pain episodes are a hallmark of sickle cell disease. They are unpredictable in many ways, both the timing of when they occur, how severe they will be and how long they will last. For those with the disease, these devastating pain episodes can start as early as six months of age and can span a lifetime, impacting school attendance and participation in the workforce. In fact, these pain crises contribute significantly to the 200,000 emergency room visits collectively made by sufferers of sickle cell disease each

year in our country. A typical crisis will result in a hospital stay of seven to ten days. Due to the lack of effective treatment options to treat the disease, patients are given medication to ensure hydration and opioids to manage the pain. Although we have known for over five decades the gene responsible for sickle cell disease, we have no medications to alter the course of a pain episode. Sadly, people with the most severe form of the disease may have a two to three decade shorter life expectancy.

In 2004, the Congress recognized the need to do more to improve the treatment and prevention of sickle cell disease. Specifically, as part of the American Jobs Creation Act, a Sickle Cell Disease Prevention and Treatment Demonstration Program was enacted. The demonstration program required the Health Resources and Services Administration to make grants to up to 40 eligible entities for the purposes of developing and establishing systemic mechanisms to improve the prevention and treatment of sickle cell disease. Specifically, grantees were charged with improving the coordination of service delivery for patients; providing genetic counseling; training health professionals; working to ensure greater newborn screening; and working with state health departments to ensure access to care, among other things. To ensure this was done most effectively and comprehensively, the grants were to be awarded to heighten geographic diversity and grantee collaboration with sickle cell disease treatment centers. Additionally,

the legislation called for the creation of a National Coordinating Center for the demonstration program, and this Center is administered by the National Institute for Children's Health Quality.

While the enactment of the Sickle Cell Treatment and Prevention Demonstration Program was a major step forward for the individuals and families suffering with this disease, since that time the program has not been fully-funded and opportunities for advancement have been lost. As stated, while the law envisioned the creation of 40 eligible entities to develop and establish systemic mechanisms to improve the prevention and treatment of sickle cell disease, to date only nine grants have been given to eligible entities.

H.R. 1807 reauthorizes and improves upon the HRSA demonstration program. The treatment and prevention component reauthorization, contained within section 4 of the bill, sets a more realistic number of eligible entities which can be funded. The original law specified 40 eligible entities, H.R. 1807 sets that number at 25 eligible entities.

Importantly, a major advancement made in H.R. 1807 would place a duty on these grantees to "expand, coordinate, and implement transition services for adolescents with sickle cell disease making the transition to adult-focused health care." Today, so many young people fall through the cracks as they transition

from childhood to adolescence and young adulthood. This very important change would make it a requirement for grantees to adopt strategies to ensure that these individuals transition appropriately, minimizing the disruption of care and resulting in better health outcomes.

Additionally, H.R. 1807 establishes a new surveillance grant program for states, wherein grants would be authorized to up to 20 states representing a majority of the sickle cell disease patient population. The current surveillance conducted by the CDC is limited to the state of California and the data collected is general in nature. The data which would be accumulated under this grant program authorized by HR 1807 would cover associated health outcomes, complications and treatments, and would result in public health initiatives and strategies which would improve current estimates about the incidence and prevalence of the disease, would identify health disparities, would assess the utilization therapies and strategies to prevent complications from the disease, and would evaluate the impact of genetic, environmental, behavioral and other risk factors that may impact health outcomes.

SCDAA has been working in partnership with the American Society of Hematology, the world's largest society of professional Hematologists, in support of the reauthorization of the sickle cell disease programs in HR 1807 and on other legislative and regulatory initiatives to better meet the needs of this underserved

community. Earlier this week ASH released a report supported by more than 20 organizations evaluating and making recommendations related to multiple aspects of sickle cell disease, including access to care in the US, training and professional education, research and clinical trials, and global health. Together, private sector organizations along with federal and state government agencies working in a coalition will make a real difference in improving the lives of people with sickle cell disease.

Finally, SCDAA wants to recognize the important advancements being made to not only treat the symptoms of those suffering from sickle cell, but to treat the disease itself. It's been decades since the FDA has approved new treatments for the benefit of patients with sickle cell disease. During the debate on the 21st Century Cures Act, Rep. Rush noted the historic underrepresentation of minority communities in clinical trials, and the fact that the nation needs to do a better job of supporting institutions that educate medical professionals with an interest in working in minority communities. By doing this, we could make advancements in the development of therapies for diseases like sickle cell which primarily impact minority populations.

Nearly 80% of hospitalizations for sickle cell disease occur among those covered by public payers Medicaid and Medicare." The good news is, as it relates specifically to sickle cell disease, we are on the precipice of exciting new therapies which hold the promise of lessening patient suffering and reliance on expensive acute care. These therapies could be approved by FDA as soon as 2018. More importantly these therapies may improve the quality of life of those living with sickle cell disease. The SCDAA would like to work with this committee and the Congress to ensure that when these therapies, which will alleviate so much pain and suffering for patients with sickle cell disease, are approved, there are incentives for hospitals to provide these drugs to patients. We cannot have a situation develop where a breakthrough sickle cell disease drug is approved for the benefit of patients in crisis, but there are disincentives for hospitals to use these new therapies. There are government programs, such as the New Technology Add-On Payment program in Medicare, which attempts to ensure that new therapies are available for needy, hospitalized patients, but only a handful of drug therapies have ever been approved for this program. And some have found that the program doesn't do enough to incentivize hospitals to use the new therapies. And no such program exists under the Medicaid program, which provides coverage for so many sickle cell disease patients. The presence of new, breakthrough therapies

is a tremendous advance, but we must ensure that disease sufferers have access to these therapies when they are in need.

Thank you very much for allowing me to testify before you today.

Mr. PITTS. The chair thanks the gentlelady and now recognizes Dr. Morrison 5 minutes for your summary.

STATEMENT OF R. SEAN MORRISON, M.D.

Dr. MORRISON. Chairman Pitts, Ranking Member Green, and members of the Energy and Commerce Health subcommittee, good morning, and thank you for the opportunity to address H.R. 3119, the Palliative Care and Hospice Education and Training Act. My name is Sean Morrison, and I am professor and vice chair of geriatrics and palliative medicine and director of Palliative Care at the Mount Sinai Health System New York City. I am a former president of the American Academy of Hospice and Palliative Medicine, and I am here today representing the Patient Quality of Life Coalition, a group of over 40 patient, provider, and health systems focused on improving the quality of life for persons living with serious illness and their families. I would also like to thank Representative Engel, a fellow New Yorker, for his continued leadership, and the 33 other bipartisan members of this committee who have signed this bill.

As a practicing physician, health services researcher, and teacher, I am acutely aware of the challenges faced by the seriously ill in this country. Multiple studies have demonstrated that inadequately treated systems, fragmented care systems, poor communication between patients, families and their physicians, strains on caregivers, and escalating healthcare use all characterize the experience of living with a serious illness in this country. Five percent of seriously ill Medicare beneficiaries account for over 50 percent of spending, and contrary to the popular perception, only 11 percent of these persons are in the last year of life. The majority live for many years with progressively debilitating illness that interferes with their quality of life and ability to work and live independently.

Palliative care is team-based care: doctors, nurses, social workers, and chaplains, focused on relief of pain and other symptoms and support for the best quality of life for patients and families in the setting of a serious illness. It should be provided at any age at the time of diagnosis of a serious illness and concurrently with all other appropriate medical treatment, including those directed at cure and life prolongation. Palliative care has been shown to enhance quality of life, doctor-patient family communication, satisfaction with care, reduce healthcare costs, and in cancer, improve survival. Over 95 percent of mid- to large-size hospitals now have palliative care teams, and palliative care is being rapidly integrated into the nonhospital settings.

Yet three major challenges remain if palliative care is to become universally accessible and, indeed, inserted into the genome of American medicine. First, based on a recent national survey, over three-quarters of patients and families who could benefit from palliative care don't know what it is and thus cannot request it when it would be most beneficial. Yet when read a definition of palliative care, more than 90 percent said they would want it for themselves or their family members and that it should be universally available throughout the country. Targeted educational efforts to increase

patient, family, and provider awareness about palliative care and its benefits are appropriately recommended in H.R. 3119.

Workforce shortages prevent patients from accessing palliative care. There are simply too few palliative care specialists to meet the needs of the population. The 134 existing palliative medicine fellowship programs graduate fewer than 300 new doctors a year, less than a 10th of what is needed. Because palliative care was recognized as a subspecialty, after the Balanced Budget Act of 1997 limited the number of Medicare-supported residency spots, training in palliative medicine is now supported only by private sector philanthropy and variable and inconsistent institutional support.

H.R. 3119 would support specialist training in palliative care, palliative care education for students and trainees, and mid-career training in the core palliative care knowledge and skills for nonpalliative-care practicing healthcare professionals.

Finally, the knowledge base to support palliative care is inadequate. Treatment for symptoms, such as breathlessness, fatigue, itching, and pain, are primitive compared to the science underlying most disease treatments. Despite four reports from the Institute of Medicine calling for major Federal investment in palliative care research, a recent study found that less than 1/100th of a percent of the NIH budget is focused on improving quality of life in the setting of serious illness.

I struggle daily with the fact that opioids with all of their attendant risks remain the most effective treatment for my patients in severe pain. H.R. 3119 would require the Director of the National Institutes of Health to expand and intensify research specific to palliative care.

To close, H.R. 3119, the Palliative Care and Hospice Education and Training Act will help address the barriers preventing all Americans from enjoying the highest quality of life in the setting of serious illness. I would like to again express my sincere thanks for the opportunity to address this important issue and legislation on behalf of the Patient Quality of Life Coalition with you this morning. Thank you again.

[The prepared statement of Dr. Morrison follows:]

Statement by

R. Sean Morrison
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Before the Subcommittee on Health
Energy and Commerce Committee
U.S. House of Representatives
September 8, 2016

Hearing: *Examining Legislation to Improve Public Health*

Chairman Pitts, Ranking Member Green and Members of the Energy and Commerce Committee Health Subcommittee. Good morning and thank you for the opportunity to address the Subcommittee. My name is Sean Morrison and I am a physician, Professor and Vice-Chair of Geriatrics and Palliative Medicine, and Director of the Hertzberg Palliative Care Institute at the Icahn School of Medicine at Mount Sinai. I also direct the National Palliative Care Research Center in New York City – a philanthropically funded organization dedicated to improving the evidence base for the care of persons living with serious illness and their families. I am a former President of the American Academy of Hospice and Palliative Medicine and am here today representing the Patient Quality of Life Coalition; a group of over 40 patient, provider and health system organizations including the Academy. The Patient Quality of Life Coalition was established in 2013 and is focused on improving quality of life for patients with serious illness and their families. Thank you for the opportunity to testify before the Subcommittee in support of H.R. 3119, the Palliative Care and Hospice Education

and Training Act. I'd also like to thank Representative Engel, particularly, for his leadership and sponsorship of this important legislation, as well as note the strong bipartisan support the legislation has from over half of the full committee Membership of the Energy and Commerce Committee signed on as co-sponsors.

Palliative care is a relatively new medical and team-based specialty devoted to improving the quality of life – through expert pain and symptom management; skilled communication about what matters most to patients and their families; and well-coordinated and communicated care over the course of a serious illness. Palliative care is delivered at the same time as curative or disease-directed treatments and eligibility is based on patient need and not on prognosis. H.R. 3119 would address three important public policy issues that have been identified as necessary to provide patients with serious illness better access to palliative care services throughout the continuum of their care: Public and Professional Education, Workforce Development, and Research.

Since completing my training in 1995, my research, clinical care, and teaching activities have focused exclusively on improving quality of life for persons living with serous illness and their families. With my colleague Dr. Diane Meier, I established one of the first five palliative care programs at an academic medical center. I have served on national committees that have focused on enhancing research and clinical care for the seriously ill for the National Institutes of Health, Institute of Medicine, and National Quality Forum. I have been continuously funded by the National Institutes of Health for the past 20 years and my research has focused exclusively on improving care for persons

with serious illness and their families. I am an active clinician caring for seriously ill patients and families in both inpatient and outpatient settings.

The elimination of suffering and the cure of disease are the fundamental goals of medicine.[1] Although medical advances have transformed previously fatal conditions such as cancer and heart disease into illnesses that people can live with for many years, they have not been accompanied by corresponding improvements in the quality of life for these patients and their families.[2] Living with a serious illness should not mean living in pain or experiencing symptoms like shortness of breath, nausea, or fatigue. Yet, multiple studies over the past two decades suggest that medical care for patients with advanced illness is characterized by inadequately treated pain and other physical distress; fragmented care systems; poor communication between doctors, patients, and families; enormous strains on family caregiver and support systems and escalating health care resource use.[2] Five percent of Medicare enrollees account for over 50% of Medicare spending and, contrary to popular perception, only 11% of these persons are in the last year of life.[3] The majority of high cost beneficiaries live for multiple years with progressively debilitating illness.[3] Over the next decades most health care professionals will be caring for seriously ill older adults and their families with multiple chronic conditions, multi-year illnesses, and intermittent crises interspersed with periods of relative stability.[4,5]

What is Palliative Care and Why Is It Needed?

Palliative care is interdisciplinary team based medical care focused on relief of pain and other symptoms and support for the best possible quality of life for patients

with serious illness and their families. Palliative care should be initiated starting at point of diagnosis of a serious illness and is provided alongside all other appropriate medical therapies including those directed at life prolongation and cure throughout the entire course of illness.[5] Palliative care programs have been shown to reduce symptoms and enhance quality of life, improve doctor-patient-family, satisfaction with care, enhance the efficiency and effectiveness of hospital services, reduce healthcare costs, and in cancer patients, improve survival.[2,6-8] Over 95% of all mid-large size hospitals in the United States now have palliative care teams and models of palliative care delivery are being rapidly created and disseminated in non-hospital care settings.[9] This growth is in response to the increasing numbers and needs of Americans living with serious, complex, and chronic illnesses, and the realities of the care responsibilities faced by their families.

The development of the specialty of palliative care has been a critical step in addressing the unmet needs of patients with serious illness and their families and the growth of this field has been remarkable. Nevertheless, challenges remain if care for seriously ill patients and their families is to improve in the United States. Most patients and families who could benefit from palliative care do not know of its existence or equate palliative care with end-of-life care or hospice and thus cannot or do not request palliative care when they can most benefit from it: early and throughout the course of a serious illness[10] Because of lack of investment, and unlike other areas of healthcare, the knowledge base to support the core elements of palliative care clinical practice (i.e., pain and symptom management, communication skills, spiritual support, practical

support for patients and family caregivers, care coordination) is inadequate and care models developed to support the needs of patients and families have yet to be evaluated.[2] That is, the evidence base to assure high quality clinical care and guide appropriate institutional and system benchmarks is lacking. Finally, although progress has been made in hospitals such that 95% of mid-large size hospitals have palliative care teams and two thirds of all hospitals now provide palliative care, many of these programs are understaffed and only able to care for a fraction of eligible patients.[9] Furthermore, the majority of nursing homes and community healthcare settings outside of hospitals lack integrated and adequately supported palliative care programs.

Ensuring Access to High Quality Palliative Care

A number of key initiatives need to be undertaken for palliative care to be accessible to all patients with serious illness. First, there need to be patient and provider educational initiatives to increase awareness of the benefits of this care, in the setting of a serious illness and the difference between palliative care and end-of-life care or hospice. Second, there need to be work force initiatives to ensure sufficient numbers of palliative care specialists to teach healthcare trainees and practicing clinicians in the core knowledge and skills of palliative care, conduct the needed research to enhance the evidence base, and provide appropriate care for the most complex population of those with serious illness and their families. Similarly, non-palliative care specialists need to learn core palliative care knowledge and skills given that there will never be enough specialists to provide this type of care. Third, research initiatives are necessary to augment the current inadequate evidence base. H.R. 3119,

the Palliative Care Hospice Education and Training Act would address all three of these important policy changes, and allow for greater patient access to palliative care services for patients and their families.

Public and professional misperceptions

A major issue impeding access to palliative care is the perceptions among doctors and other healthcare professionals that palliative care is appropriate only at the end of life, that palliative care is synonymous with hospice, and that patients will react negatively and lose all hope if palliative care referral is discussed.[11,12] This is perhaps not surprising given that clinicians, particularly those trained more than 10 years ago, received little to no training in the core knowledge and skills of palliative care nor were they exposed to modern palliative care teams during their educational training. While many physicians have misperceptions about palliative care, most patients do not know anything about it. A recent survey showed that almost 90% of adults in the United States had either no knowledge or limited knowledge of palliative care. However, when read a definition for palliative care, more than 90% of the respondents stated that they would want palliative care for themselves or their family member and that it should be universally available.[10] Targeted social marketing and educational efforts must be directed both to the public and to medical professionals. A national educational campaign to increase public and professional awareness about palliative care and its quality of life, family, and survival benefits, as called for in H.R. 3119, is critically needed. Such a campaign would define palliative care as appropriate care for persons with any serious illness throughout the course of their disease, encourage patients and families

to seek high quality palliative care early in the course of illness, and educate healthcare professionals as to the appropriate role of palliative care in the care of their patients.

Workforce Initiatives

Workforce shortages also prevent many patients from accessing or using palliative care services. The number of palliative care specialists falls far short of what is necessary to serve the current population in need despite the fact that the American Academy of Hospice and Palliative Medicine and the Hospice and Palliative Nurses Association are among the fastest growing professional membership organizations in medicine and nursing respectively. A 2010 study estimated that over 12,000 full time palliative care physicians were needed at that time just to meet current demand for palliative care services in the United States.[13] The demand for palliative care professionals in 2016 is even greater. Similar shortages exist across the other core palliative care disciplines of nursing, social work, and chaplaincy. The demand for the expansion of palliative care services in community care settings that was created by incentives under the Affordable Care Act, the Joint Commission Advanced Certification for Palliative Care, and the increasing palliative care infrastructure in both public and private sectors of healthcare is further straining the limited specialist-level palliative care workforce.

A major reason for this shortage is the "newness of the field". Palliative medicine was recognized as a subspecialty by the American Board of Medical Specialties only in 2008. In 2016, only 119 fellowship programs accredited by Accreditation Council for Graduate Medical Education and 15 fellowship programs accredited by the American

Osteopathic Association existed in this country, together graduating a total of 296 new palliative care physicians each year (personal communication, Steven Smith, American Academy of Hospice and Palliative Medicine). Furthermore, because the Balanced Budget Act of 1997 placed a limit on the number of Medicare-supported residency slots before palliative medicine was formally recognized as a medical subspecialty by the American Board of Medical Specialties, specialty training in palliative medicine is entirely dependent on private sector philanthropy or variable and inconsistent institutional support and not by Medicare funding, as is the case with all other medical training in this country. Palliative care specialists are critically needed to teach and mentor healthcare trainees and practicing clinicians in the core knowledge and skills of palliative care, conduct the needed research to enhance the evidence base in order to provide the highest quality care to patients with serious illness and their families, and provide appropriate care for the sickest and most complex population of those with serious illness and their families.

Creating a specialist workforce is not enough, however, to ensure that patients with serious illness and their families receive the care that they deserve. As noted above, the specialist workforce will never be large enough to meet the needs of those Americans with serious illness and their families. Enhancing the palliative care knowledge and skills of ALL front-line clinicians must occur if care for the seriously ill is to improve. Expanding core palliative care knowledge and skills of all clinicians will be a key step toward resolving the shortage in the palliative care specialist workforce. The core palliative care competencies of skilled communication, expert pain and symptom

management, and psychosocial assessment remain, at best, a small part of most medical school and residency training programs. The vast majority of practicing physicians and trainees have either rudimentary or no skills in these areas, which negatively affects patient and family outcomes. Indeed, after 10 years of graduate and post-graduate medical education from 1986-1996 at the University of Chicago, the New York Hospital Cornell Medical Center, and the Mount Sinai School of Medicine, I had received only a thirty-minute lecture on pain management – it occurred in my first year pharmacology course and focused on how opioids bind to nerve cells, are metabolized in the liver, and excreted in the kidney. How to approach and treat the patient in pain or how to effectively communicate to patients a serious diagnosis, discuss goals of care, address prognosis, or facilitate complicated decision making in the setting of serious illness was never covered during my education. From research conducted over the past ten years, we now have a body of evidence that demonstrates that these skills (particularly communication skills) can be effectively learned and developed and are associated with improved outcomes.[14-16] Strategies to expand training in core palliative care knowledge and skills to all clinicians – those in training and those already in practice are needed. H.R. 3119 would establish an education and training program modeled after the successful geriatric education and training programs created a number of years ago, that allow for support of palliative care curriculum development in medical schools and training programs in palliative care for all key healthcare professionals required to provide palliative care – doctors, nurses, social workers and certified healthcare chaplains.

Knowledge and Evidence Base

Reports from the Institute of Medicine in 1997, 2001, 2003, 2015[4,17-19] have consistently called for major federal investments in palliative care research and yet to date, these calls have remained unanswered. Unlike other areas of medical research traditionally funded by the NIH, the knowledge and evidence base to support core elements of palliative care clinical practice (i.e., pain and symptom management, communication skills, care coordination) is inadequate. The reasons for this distressing state of affairs are many, but almost all stem from a philosophy of medical research that has traditionally viewed symptoms and suffering as unimportant in themselves and interesting only insofar as they guide the physician to a correct diagnosis.[20] The prevailing philosophy dictates that once the diagnosis is made (e.g., cancer) and the disease is treated (e.g., chemotherapy), the symptoms (e.g., breathlessness, pain) will dissipate. What is left unsaid is what happens when the disease can't be cured, or is only partially treated or managed, or the treatment itself results in temporary or permanent distress and disability.[20] It is not surprising, perhaps, that a comprehensive review of research in palliative medicine supported by the National Institutes of Health (NIH) revealed the data that should guide the treatment of human suffering associated with serious illness of all kinds are not only inadequate but in many instances are completely absent. As a result, current clinical practice regarding symptoms is driven not by evidence but by extrapolation from other situations, small and underpowered or single site studies, and is often anecdotal or based on hearsay and therefore not based on valid science.[20]

Key research needs to be funded and performed if palliative care is going to achieve its potential to enhance value throughout the health care system. First, important gaps in clinical evidence need to be addressed so that persons with serious illness can receive the best available care. For example, because the mechanisms underlying symptoms are poorly understood, treatments for symptoms such as breathlessness, fatigue, itching, delirium, anxiety, and even pain are primitive compared the science underlying many disease treatments. Indeed, it is almost beyond comprehension that the most effective treatment for severe pain in the setting of most serious illnesses remains opioids with all of their attendant complications and risks – a fact that has not changed since the 1600s. Treatment for nerve (neuropathic) pain, a common complication of diabetes and cancer, is even less effective. Fewer then 2/3rds of patients with severe neuropathy have responses to the best available treatment and only 20% of these patients report fifty percent improvement in pain relief.[21] Although a recent report points to the success of non-pharmacologic measures in the treatment of chronic pain (such as low back pain or migraines), none of these treatments act immediately and most are ineffective for the most severe pain syndromes associated with a serious illness like cancer. It is hard to imagine that we would tolerate this state of affairs if we were considering conditions of high blood pressure or diabetes or cholesterol, rather than pain and other sources of human suffering.

Second, the needs of older adults with serious illness and their caregivers and the long term and changing nature of those needs are not well described. In particular, the complex care needs of patients with multiple coexisting conditions and functional

dependency must be investigated. The ability to identify and support the population at risk requires moving beyond prognosis and diagnoses to include powerful predictive factors such as needing another person to get through the day, and prior need for institutional care.[22]

Third, data to guide care for seriously ill children are needed. Although the numbers of children living with serious illness are notably smaller than those of adults, in 2010, 45,000 children died in the United States, over 25,000 thousand children are living with a serious illness at any given time, and nearly 17 million adults are serving as caregivers to a seriously ill child.[19,23,24] Despite the need, the evidence on how best to deliver palliative care for seriously ill children is almost non-existent. Finally, the development and evaluation of palliative care–delivery models outside hospitals (in people's homes, nursing homes, office practices) is essential. To achieve this goal research funding for palliative care will need to be increased beyond the 0.01% of the National Institutes of Health budget that currently supports research on palliative care.[25] H.R. 3119 would require the director of the National Institutes of Health to expand and intensify research on palliative care, and pain and symptom management across institutes at the NIH.

Conclusion

Research has conclusively demonstrated that most seriously ill Americans experience treatable suffering and many are impoverished because of uncompensated medical care.[26] At the same time, rising government healthcare expenditures threaten to bankrupt Medicare.[27] Palliative care offers a rational solution to this problem by

improving quality of life and quality of care and in so doing substantially reducing need for costly crisis care for the highest risk and highest need patient population. The evolution and growth of palliative care in the United States has resulted from the combined investments of both the public and the private sectors. Substantial private-sector contributions exceeding $300 million in the last twenty-five years[28] have created the new field of palliative care and are reflected in the growth of hospital palliative care services, education and training for health professionals, and formal recognition of subspecialty status for physicians and nurses. Commercial and integrated health plans are experimenting with creative benefit design supporting palliative care delivery resulting in better value.[29-32] The combined and sustained commitment of both the private and the public sectors will be necessary to bring the palliative care innovation to scale in the United States. As Hubert H. Humphrey said at the dedication of the Humphrey Building in 1977 "the moral test of government is how that government treats those who are in the dawn of life, the children; those who are in the twilight of life, the elderly; and those who are in the shadows of life; the sick, the needy and the handicapped."[33] The provisions in the bill under consideration, H.R. 3119, the Palliative Care and Hospice Education and Training Act will contribute significantly to addressing the barriers preventing all Americans from enjoying the highest quality of life in the setting of serious illness. In closing, I'd like to express again my thanks to Chairman Pitts and Ranking Member Green, as well as to all of you on the sub-committee for allowing me to address this issue with you today.

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Mr. PITTS. The chair thanks the gentleman and now recognizes Dr. Marino 5 minutes for your summary.

STATEMENT OF BRAD MARINO, M.D., MPP, MSCE

Dr. MARINO. Good morning. My name is Brad Marino. I am a pediatric cardiologist at Ann & Robert H. Lurie Children's Hospital of Chicago. I am a professor of pediatrics at Northwestern University Feinberg School of Medicine. I am the chair of the Medical Advisory Board for the Pediatric Congenital Heart Association, and I currently chair the Council for Cardiovascular Disease in the Young for the American Heart Association.

Thank you very much for the opportunity to offer testimony today in support of H.R. 3952, the Congenital Heart Futures Reauthorization Act of 2015. I wish to thank Chairman Pitts and Ranking Member Green for holding this hearing and Representative Bilirakis and Representative Schiff and the dozens of congressional cosponsors for the bipartisan effort to build upon existing programs which promote lifelong research, track epidemiology, and raise awareness for congenital heart disease, or CHD, the most common birth defect.

On behalf of Lurie Children's, the Pediatric Congenital Heart Association, the American Heart Association, the Children's Heart Foundation, and the Adult Congenital Heart Association, and the millions of individuals with CHD, I want to offer my strongest support for this very important legislation. Lurie Children's, the sixth ranked children's hospital nationally by U.S. News and World Report, is the largest provider of pediatric specialty care in Illinois, as well as serving children from all 50 States and 46 countries, many of whom have congenital heart disease.

As a practicing pediatric cardiac intensivist, epidemiologist, and outcomes researcher, for more than 20 years, I have borne witness to the catastrophic results of CHD on affected children and their families that last a lifetime. Critical information about the epidemiology of CHD, the effectiveness of treatments, and lifelong outcomes is seriously lacking at best and nonexistent in specific areas such as secondary sequelae of CHD.

Over the last several decades, tremendous advances in care have dramatically reduced mortality rates for children with the most complex congenital heart disease and increased life expectancy of adults with CHD. In the absence of U.S. data, extrapolation of Canadian data suggests that there are currently more than 2.4 million individuals living in the United States with CHD, half of whom are adults. However, while survival has improved, the reality is that complex CHD and its treatments may result in significant cardiovascular complications and organ-specific comorbidities, including kidney and liver disease and brain injuries, that significantly impact health status, physical, and psychosocial functioning, and quality of life.

Early intervention for CHD is not a cure, underscoring the need for those with CHD to have lifelong care by expert providers. We need to better understand and improve the transition from pediatric to specialized adult cardiovascular care. Estimates suggest that less than 25 percent, one out of four, adults with complex congenital heart disease are receiving appropriate subspecialty care.

People born with CHD require lifelong, costly specialized cardiac care. As a result, healthcare utilization among the CHD population is disproportionately higher than the general population.

It is estimated that compared to the medical costs of care for the general population, the medical costs for individuals with CHD are 10 to 20 times greater. Around half of all dollars spent on pediatric CHD-related inpatient admissions is paid by Medicaid.

To improve care and reduce costs, it is essential that Congress enacts legislation supporting increased understanding of CHD across the lifespan. The Congenital Heart Futures Reauthorization Act of 2015 calls for the robust public health research and surveillance that will help us better understand and improve long-term outcomes for the more than 40,000 babies born each year with CHD.

Since the enactment of the Congenital Heart Futures Act of 2010, Congress has appropriated nearly $15 million to support CHD activities at the CDC, which has led to the standardization of research methods and an increased understanding of the public health burden that CHD poses. Continued Federal investment is desperately needed to better understand CHD across the lifespan, improve outcomes, and reduce costs. The Congenital Heart Futures Reauthorization Act of 2015 does just that. This legislation assesses the current state of biomedical research for CHD across the lifespan by directing the NIH to provide a status report on its current research on CHD. This will improve an understanding of the causes of CHD and drive innovation and effective treatments for CHD and related disease processes. H.R. 3952 expands public health research on CHD by directing the CDC to plan, develop, and implement a representative cohort study. The data from this cohort will help to describe basic U.S. demographics of CHD, assess healthcare utilization, and develop evidence-based practices and guidelines for CHD care, eliminating our reliance on statistics from Canada to describe the burden of CHD in the United States.

This bill also directs the CDC to establish and implement an outreach, education, and awareness campaign, and ensuring that those with CHD receive appropriate care across their lifespan. With this critical education campaign, individuals with CHD and their families will better understand their lifelong healthcare needs and the necessity of receiving appropriate lifelong specialized cardiac care.

Congenital heart disease is common. It is costly, and it is a critical public health issue. In enclosing, on behalf of the Ann & Robert H. Lurie Children's Hospital of Chicago, the Pediatric Congenital Heart Association, the American Heart Association, the Children's Heart Foundation, and the Adult Congenital Heart Association, I urge you to take swift action to enact H.R. 3952, the Congenital Heart Futures Reauthorization Act of 2015. It is essential that Congress pass this legislation to provide improved care, outcomes, and quality of life for the millions of individuals in the United States with CHD.

Thank you for your time and consideration.

[The prepared statement of Dr. Marino follows:]

Statement for the Record
In support of
H.R. 3952
"The Congenital Heart Futures Reauthorization Act of 2015"

Submitted to the Subcommittee on Health
House Committee on Energy and Commerce

Submitted by:

Bradley S. Marino, MD, MPP, MSCE
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Ann & Robert H. Lurie Children's Hospital of Chicago, IL

September 8, 2016

On behalf of the Ann & Robert H. Lurie Children's Hospital of Chicago, the millions of people with congenital heart disease, or CHD, the health professionals who care for them, and the Pediatric Congenital Heart Association, Children's Heart Foundation, American Heart Association and Adult Congenital Heart Association that represent them, I want to offer my strong support for H.R. 3952, the Congenital Heart Futures Reauthorization Act of 2015.

I wish to thank Representative Bilirakis and Representative Schiff for their steadfast leadership as sponsors of this bipartisan effort to build upon existing programs which track the epidemiology, raise the awareness of and promote lifelong research on the most common birth defect. Thank you, also, to the dozens of congressional cosponsors committed to this goal. I also want to express my appreciation to Chairman Pitts and Ranking Member Green for holding this hearing, providing the opportunity to move this important bill toward enactment.

Lurie Children's, the 6th ranked Children's Hospital nationally by US News and World Report, is the largest provider of pediatric specialty care in Illinois, as well as serving children from all fifty states and 46 countries. The Heart Center at Lurie Children's provides the highest quality care for patients with the most complex and serious heart conditions. As the region's largest children's heart center, we care for more patients with cardiac conditions than any other hospital in the state of Illinois, treating more than 10,000 patients with congenital and acquired heart disease each year. Lurie Children's also provides more pediatric Medicaid services than any other hospital in the State of

Illinois. On average, 55 to 60% of the beds in our hospital hold a child that is insured by Medicaid.

As a practicing pediatric cardiac intensivist, epidemiologist, and outcomes researcher for more than 20 years, I have borne witness to the catastrophic results of CHD on affected children and their families. As the national Chair for the American Heart Association Council for Cardiovascular Disease in the Young, Chair for the Pediatric Congenital Heart Association Medical Advisory Board and the Chair for the 36 institution Cardiac Neurodevelopmental Outcome Collaborative, I can attest that CHD has a lifelong impact. However, critical information about the epidemiology of CHD, the effectiveness of treatments, and lifelong outcomes is seriously lacking, at best, and non-existent in specific areas such as secondary sequelae of CHD.

Over the last several decades, advances in surgical techniques, intensive care, imaging, and medical therapies have dramatically lowered mortality rates for children with the most complex CHD and increased the life expectancy of adults with CHD. Survival is now expected, with long-term survival rates (>20 years) estimated at 80% in the current era. In the absence of US data, which the Congenital Heart Futures Reauthorization Act will address, extrapolation of Canadian data suggests that there are currently more than 1.3 million adults with CHD in the US. This prevalence is expected to increase 5% annually, resulting in a burgeoning population of CHD survivors who need life-long specialized cardiac care. Although the growing population of individuals with CHD is a testament to important innovations in CHD care, the reality is that

complex CHD and its treatments may result in important complications including persistent valvular or heart muscle issues that may require further surgery, catheter-based intervention, or heart transplantation, abnormal heart rhythms that may require the placement of a pacemaker, and important kidney, liver, and neurodevelopmental problems. In short, the interventions children with CHD receive are not cures, underscoring the need for children and adults with CHD to have lifelong care by expert providers to avoid health complications later in life.

One of the biggest issues we face is transitioning care for individuals with CHD from pediatric to specialized adult cardiovascular care. As the Director of the Cardiovascular Bridge Programs at Lurie Children's and Northwestern Memorial Hospital, I see first-hand, each day, the lack of infrastructure, systems, providers and payors required to care for the ever increasing adult congenital heart disease population. Adding to this challenge are those patients who no longer seek care believing their childhood intervention has "cured" them, or that they are well enough to no longer need specialized cardiac care. Estimates suggest less than 25% of adults with CHD are receiving appropriate subspecialty care. This population of patients who are lost to follow-up care present a critical public health issue, which will be addressed by this important legislation.

The incidence and prevalence of heart failure in the CHD survivor population is dramatically increasing with a dearth of well-tested and approved heart failure medications, mechanical ventricular assist devices, devices specifically sized and tested

for use in the cardiac catheterization laboratory, and organs for heart transplantation. In addition, congenital heart survivors can face debilitating, ongoing neurodevelopmental issues. Cardiac surgery and perioperative treatments in the infant put the developing brain at tremendous risk for injury and long-term neurodevelopmental complications. Survivors often suffer injury to the brain due to chronic or intermittent low oxygen levels, decreased brain blood flow, and/or reperfusion injury related to the abnormalities of their circulatory systems and the medical and surgical therapies they have received. These brain injuries result in worse neurodevelopmental, psychosocial, and physical functioning, and can significantly negatively impact the individual's quality of life.

In fact, recent studies have shown that individuals with complex CHD have an increased risk for neurodevelopmental impairment across a broad range of domains, including intelligence, school achievement, language, visual processing, memory, attention, executive functioning (organization, planning, and task-management), and fine and gross motor skills. In addition, a disproportionate number of these patients have significant behavioral or emotional problems, including ADHD, anxiety, depression, and post-traumatic stress symptomatology. Many school-age survivors of infant cardiac surgery require remedial services including tutoring, special education, and physical, occupational, and speech therapy. Up to 1/3 of complex CHD survivors will require some form of special education or learning supports. As adults, individuals with CHD are less likely to be employed and reach economic self-sufficiency than the general population.

People born with CHD require lifelong, costly, specialized cardiac care, and face an ongoing risk of permanent disability and premature death. As a result, healthcare utilization among the CHD population is disproportionately higher than the general population. It is estimated that compared to medical costs of care for their peers, the medical costs for individuals with CHD are 10 to 20 times as great. Inpatient care costs (not including costs of physician care) for patients < 21 years old with CHD alone totaled more than $5.6 billion in 2009, representing 15 percent of hospitalization costs for all patients in this age range. Around half of all dollars spent on pediatric CHD related inpatient stays is paid by Medicaid.

Hospital admissions for adults with CHD roughly doubled between 1998 and 2005. Nearly 20% of these admissions were for cardiac surgery or catheter-based intervention. Healthcare utilization and costs continue to rise, due to hospital admissions, cardiac and non-cardiac surgery, and emergency room visits. For example, with improved longevity to childbearing age, the number of high risk annual births in women with CHD is increasing. Childbearing women with CHD are fourteen times as likely to experience cardiovascular complications during pregnancy and are eighteen times as likely to die from such complications as are women without CHD. They also have longer hospital stays and incur higher hospital charges.

With disproportionate medical costs, it is critical for us to understand the life-course of those living with CHD, their health care utilization and potential cost reduction strategies.

Before us, we have a reasonable and meaningful solution. To improve care and reduce costs, it is essential that Congress enacts legislation supporting improved understanding of CHD across the lifespan. The Congenital Heart Futures Reauthorization Act of 2015 (CHFRA) calls for improved public health research and surveillance that will help us better understand and improve long-term outcomes for the more than 40,000 babies born with CHD each year.

Previous Congressional investment for CHD activities supported by the Centers for Disease Control and Prevention's (CDC's) National Center on Birth Defects and Developmental Disabilities (NCBDDD) has funded the development of innovative surveillance strategies among children, adolescents and adults with CHD. This has led to the standardization of research methods and an increased understanding of the public health burden of this condition. Since the enactment of the Congenital Heart Futures Act in 2010, Congress has appropriated nearly $15 million to NCBDDD for these activities. Continued federal investment is necessary to provide rigorous epidemiological and longitudinal public health surveillance and public health research on individuals across the lifespan to better understand CHD at every age, improve outcomes and reduce costs.

The Congenital Heart Futures Reauthorization Act of 2015 (CHFRA) continues and builds upon these important activities.

The reauthorization bill assesses the current bio-medical research needs and projects related to CHD across the lifespan. It directs the National Institutes of Health (NIH) to provide a report on its current research into CHD. This will provide a better understanding of the state of bio-medical research to improve understanding of causes and drive innovative and effective treatments for CHD and related disease processes.

The Congenital Heart Futures Reauthorization Act fills a critical gap and expands public health research on CHD. The legislation directs the CDC to plan, develop and implement a representative cohort study to help describe basic U.S. demographics of the disease, assess healthcare utilization, and develop evidence-based practices and guidelines for CHD care. Once this data is collected, we will no longer have to rely on statistics from Canada to describe the burden of CHD in the United States.

The Congenital Heart Futures Reauthorization Act raises awareness about the specialized cardiac care needs of those with CHD across the lifespan. The bill directs the CDC to establish and implement an awareness, outreach and education campaign aimed at ensuring that those with CHD receive appropriate care across the lifespan. With this critical educational campaign, individuals with CHD and their families will better understand their life-long healthcare needs and the necessity of receiving appropriate lifelong specialized cardiac care.

Congenital heart disease is common, costly and a critical public health issue. On behalf of Ann & Robert H. Lurie Children's Hospital of Chicago, the Pediatric Congenital Heart Association, the Children's Heart Foundation, the American Heart Association, and the Adult Congenital Heart Association, I want to thank the many members of Congress who join me in support of H.R. 3952, the Congenital Heart Futures Reauthorization Act 2015. It is essential that Congress pass this legislation, to provide improved care, outcomes and quality of life for the millions of individuals with CHD.

Thank you for your time and consideration.

Summary of Written Testimony for Dr. Bradley Marino:

Congenital heart disease (CHD) is the most common birth defect, for which there is no cure. Advances in early detection and intervention are resulting in increased survival. However, individuals born with CHD require lifelong, costly, specialized cardiac care, and face an ongoing risk of permanent disability and premature death.

With disproportionate medical costs and nearly half of all dollars spent on inpatient stays paid by Medicaid, we must take measures to understand the life-course of CHD, health care utilization and cost management opportunities.

Before us, we have a reasonable and meaningful solution. This legislation will support improved understanding of CHD across the lifespan, which will improve care and reduce costs.

The Congenital Heart Futures Act Reauthorization builds upon previous Federal investment to:

1. Assess current bio-medical research needs and projects related to CHD across the lifespan at the NIH;
2. Authorize rigorous epidemiological and longitudinal surveillance of people with CHD throughout the lifespan at the CDC;
3. Lead an education and awareness campaign to raise attention to the public health burden of CHD across the lifespan.

CHD is common, costly and a critical public health issue. It is essential that Congress pass H.R. 3952, the Congenital Heart Futures Reauthorization Act 2015, to provide improved care, outcomes and quality of life for the millions of individuals with CHD.

Mr. PITTS. The chair thanks the gentleman.

And I will begin the questioning and recognize myself for 5 minutes for that purpose.

Dr. Leffert, we will just go down the line. Thank you for highlighting the impact that metabolic diseases, such as diabetes, have on our healthcare system. Can you talk specifically about some of the issues or problems with the way the Federal Government currently administers programs for diabetes and related diseases that the commission is intended to address?

Dr. LEFFERT. Thank you, Mr. Chairman.

We have over 30 agencies across the Federal Government landscape that are engaged in either diabetes research or clinical care. And over the 5-year timeframe from 2007 to 2012, there has been a 48 percent increase in the money spent on diabetes care and lost productivity due to diabetes. The commission will recommend programs and activities to affect the quality of life, productivity, cost to society of patients with diabetes, prediabetes, and related conditions. Additionally, there are examples of inconsistent and sometimes counterproductive policies emanating from Federal agencies that reflect a lack of communication and coordination in the administration of Federal diabetes activities. The commission is intended to provide a venue that brings these agencies to the table on a consistent basis to improve upon those issues.

Mr. PITTS. Thank you.

General Dean, in your written testimony, you state that the incidence of underage drinking has been going down in each year among 8th, 10th, and 12th graders. If this is true, why do we need to continue investment in this STOP Act, and what work is still left to be done?

Mr. DEAN. In fact, it is true, but there is much work that needs to be done to address underage drinking in the country. And we believe that the STOP Act has been a catalyst for these improvements, and without it, we don't believe these improvements will continue. But we need to keep the pressure up to maintain these efforts. Despite our progress, in response to our 2015 survey of coalitions, alcohol continues to be the number one problem they face in their communities, and also we know that students that are underage in universities are significantly abusing these as well. The restructuring of the STOP Act will cause our coalitions to work directly with higher institutions of education to address these problems as well.

Mr. PITTS. Thank you.

Ms. Banks, can you elaborate on the biggest barriers to quality health care for those suffering from sickle cell disease?

Ms. BANKS. Well, I think some of the largest barriers for individuals with sickle cell disease, first and foremost is the lack of access to care. As mentioned, there is a shortage of primary care physicians, and therefore, our patients do not have a medical home, which means that they frequent the emergency room often, so that is a huge deficit for us. Also, there is no comprehensive model of care and what our patients lack and what we do not have in our community is a care coordination program where someone is actually providing care coordination with our patients. Our patients are born with sickle cell disease, so we know throughout the lifespan

that they are going to have it. There is no cure for it. So the goal would be for us to coordinate their care throughout the lifespan, and that is what is really missing in the sickle cell community.

Mr. PITTS. Thank you.

Dr. Morrison, how does palliative care specifically help those individuals and families of those who are suffering from a serious but not necessarily terminal illness?

Dr. MORRISON. Living with a serious illness in this country, such as congestive heart failure, chronic obstructive pulmonary disease, cancer, Alzheimer's disease, is associated with a number of distressing symptoms—pain, breathlessness, fatigue, nausea, anxiety—which people live with on a daily basis. It provides an enormous strain on family caregivers, who often give up their jobs, their work, to care for a seriously ill older relative. Palliative care addresses these needs by providing an added layer of support to patients, their families, and doctors. It treats the pain and symptoms of a distressing illness. It helps facilitate communication and provides support to patients and families. It addresses psychological, emotional, and spiritual needs, and it allows them to obtain the best quality of life possible in the setting of a serious illness. And it is absent from our American healthcare system at this point.

Mr. PITTS. Thank you.

Dr. Marino, why is it so difficult to retain patients in followup care for their congenital heart disease, and what does H.R. 3952 do to help change that?

Dr. MARINO. So one of the programs that I have been spearheading at Northwestern is something called the cardiovascular bridge programs. So typically in the U.S. today, when you are 18 years and 364 days, your doctor will say: It has been great taking care of you. Here is the name of a doctor in the city. Have him call for the records. Good luck.

Only one out of four patients that need ongoing cardiovascular care actually get that cardiovascular care. What this bill is going to do is create awareness among patients and parents that when your child has surgery as a baby, it is not curative. There are ongoing specific cardiac issues. There are developmental issues, kidney and liver issues, that have to be dealt with as that child ages and then gets transitioned into adulthood.

In our bridge programs at Northwestern, we literally have a team of adult and pediatric providers, social workers, and advanced practice nurses that basically work with patients 16 to 26 to allow these patients to have a graded transition instead of an abrupt transition at 18 that will keep them in care.

Beside the awareness, by having the cohort study that is put in 3952, that the CDC would put together, we would know much more specifically which patients are at the highest risk for not following up, which patients are at the highest risk for having complications. That will then tell us, of those patients that we know we need to follow up, which are the most critical to make sure they stay in care.

And then, lastly, with the NIH putting forth a status report on the biomedical research, there is so much research that still needs to be done on how best to care for these patients, what inter-

ventional procedures might result in a better quality of life as they transition from an adolescent to an adult. By having that new research and that priority for that research in place with NIH, we will be able to put new care models in place and new treatment models that will help these patients transition more effectively from adolescence into adulthood.

Mr. PITTS. The chair thanks the gentleman.

I now recognize Mr. Green 5 minutes for questions.

Mr. GREEN. Thank you, Mr. Chairman.

Dr. Leffert, diabetes can be effectively managed through evidence-based treatments, as well as through behavioral changes, including changes in diet, increasing physical activity. Some patients still experience devastating complications from diabetes, including blindness, kidney failure, and limb amputation. Why do these complications occur in spite of the availability of the treatments we have?

Dr. LEFFERT. Representative Green, the issue really is that we have a limited number of endocrinologists who are able to take care of patients with diabetes. We have a primary care base of physicians who take care of diabetes, but oftentimes, they are not all given the tools or the experience to be able to take care of these patients in the appropriate way. They need a lot of help, and our commission bill would do that.

In addition, our patients need education. Education is the key because this is a self-managed disease, and this bill would also help that in relationship to many of the programs that are currently being projected by the National Diabetes Education Program, which gives patients education towards diabetes.

Mr. GREEN. Thank you. It sounds like this commission would help us explaining to physicians how we can treat diabetes, again, with medication. And I always tell people it is much better to have prediabetes than diabetes so that you can manage it much better. Thank you.

The STOP Act became law in 2006, and almost 30 percent of the underage individuals who were alcohol users and 19 percent were binge users that year. This legislation marked the first national comprehensive effort to combat underage drinking. And, again, I want to recognize my colleague and classmate, Congresswoman Roybal-Allard, for her diligent effort. And like I said—before you were here, Lucille—she has worried me about this bill for a number of months.

General Dean, can you talk about the progress you have made since 2006 and why reauthorization of these programs is so important?

Mr. DEAN. Thank you very much, Ranking Member Green, for your interest, your leadership, and your support. We have made tremendous progress. Monitoring the Future cites that underage drinking percentages are down. They are the lowest they have been for years, but we still need to continue to work diligently. And what this reauthorization is going to do is not only will we be able to provide enhancement grants to community-based coalitions that have been trained and understand how to tackle and resolve problems in their communities, but it is also going to give them a few dollars, allow them to work with higher education, colleges and

universities as well, where we know there is a serious problem there too. And we think there are too many losses of life. Certainly, we can prevent that. We also can continue to tackle the violence and the unfortunate incidents that are taking place on our universities as a result of drinking, so there is still much work needed to be done, and we believe the reauthorization and the way it has been restructured will allow us to continue to make progress around this serious underage drinking problem.

Mr. GREEN. Thank you. Thank you for your effort on that.

Palliative care is a critically important aspect of healthcare system. It does not always garner the attention that it warrants.

Dr. Morrison, can you help this committee understand that palliative care, both from its impact on a patient's quality of life and the workforce involved, and how does this legislation improve the palliative care?

Dr. MORRISON. Thank you for the question. Palliative care is a relatively new specialty. It began in the 1990s when a number of us said: Why do you have to be dying in order to have a good quality of life? And back before palliative care, the only real area that focused on improving patient's quality of life was hospice. And as we all know, you have to have a prognosis of 6 months or less to be able to access hospice in this country.

So it is a relatively new specialty, and it is one of the fastest growing specialties in the United States, but we still have a workforce issue. We have one palliative medicine physician for every 13,000 people with serious illness, and this bill addresses this in three ways.

First of all, it does create a specialist workforce that will provide the research, the teaching, and take care of the most complex patients and families. But it also provides the core knowledge and skills of palliative care to those in training and those in practice. I spent 4 years at the University of Chicago, 3 years at New York Hospital Cornell Medical Center, another 3 years at Mount Sinai, and in that entire 10 years of education, had a 30-minute lecture about pain management that happened in my first year pharmacology course. And it dealt with how drugs like morphine are broken down in the liver and excreted in the kidneys. That was the extent of my education in how to treat distressing symptoms, and we have a generation of healthcare providers with that base fund of knowledge.

So this bill will address that as well by training those doctors, nurses, social workers, chaplains, who care for the seriously ill in the core knowledge and skills of palliative care: pain and symptom management, communication, care coordination.

And, finally, it addresses the evidence gap. We have all seen the problem in this country of inappropriate prescribing of opioids because we have a generation of doctors who do not know how to assess pain, how to manage pain, how to appropriately use opioids, and how to identify the problems of addiction. I have never had a patient come to me in serious pain and say: I would like my pain treated and, oh, by the way, I would like to be addicted to the medication afterwards. That can be addressed through outreach, and it can be addressed through appropriate knowledge and teaching. And that is what this bill addresses as well.

Mr. GREEN. Mr. Chairman, thank you. And I have some questions we will submit. But our committee and the subcommittee has actually passed a number of opioid bills. And I think you are correct. We need to have training for the physicians who are actually prescribing, and hopefully, this bill along with the package of bills we passed out.

Thank you, Mr. Chairman. I yield back.

Mr. PITTS. The chair thanks the gentleman.

I now recognize the vice chair of the subcommittee, the gentleman from Kentucky, Mr. Guthrie, 5 minutes for questions.

Mr. GUTHRIE. Thank you very. I am going to try to get as many questions, so if answers could be kind of brief.

But first, Ms. Banks, I have a friend of mine whose son has sickle cell. And I didn't really know that much about it. Dr. Burgess has been helping me with it sitting here. But we hired him so he would be gainfully employed. And just working with his schedule was about my only experience with it.

So, with this bill, are there any other efforts existing within the sickle cell community that would complement this bill and allow it to be more expansive to the patient population.

Ms. BANKS. Absolutely. Currently, we are in the process—and when I say "we," I mean SCDAA and many office treatment centers or hospitals—are working with community health workers. So we have actually instituted what is called a community health worker program. What we feel like in the sickle cell community is it is going to take a concerted effort throughout the community. So we are utilizing not only the providers but also community-based organizations and utilizing community health workers to actually go into the field, find these patients, because a lot of our patients are lost, meaning they haven't been to a physician in a year or so. And that is not good for them and their health. And then not only are they finding these individuals, but they are getting them into a medical home. So the goal is that you find the patient, but then you enroll the patient or you help that patient find medical care.

In addition, SCDAA has launched a national patient registry, which is the first ever of its kind. It is a patient-powered registry. We know that it is long overdue in the sickle cell community. We know that it is going to help us to collect the data that we need. But also, along with collecting that data, this is patient-powered. So it allows the patient to communicate with the physicians. It allows the patient to actually manage their care via technology. And also it allows the patient to be up-to-date on treatments and research that is going on in the community for sickle cell patients.

Mr. GUTHRIE. OK. Thank you very much.

Dr. Marino, great to see that Northwestern is doing good work. I am moving my daughter there Monday. So she will be on the Evanston campus. So, in your testimony, you mentioned the use of Canadian data. What type of U.S. data do we have, and why do have to use the Canadian data? I would just let you expand on that.

Dr. MARINO. Mr. Guthrie, first, congratulations to your daughter. Northwestern is a very, very tough school to get into. So congratulations to her.

There is no data in the U.S. What we have is single-center data of very small numbers of individuals that we can't extrapolate to national data. And because the data is collected very differently at the different centers, with variable definitions that don't match, you can't take 10 or 15 centers and put the data together. Because the Canadians have a national health system, they actually have a national data set that allows them in a very specific way, like Denmark as well, to gather this longitudinal date on the congenital heart disease patients. So 3952 would specifically have the CDC create a cohort study in the United States that would follow this very high-risk complex CHD population over time to collect that similar data. We don't know if what we have in the U.S. is different than Canada or if it is the same. There is just no data.

Mr. GUTHRIE. OK. Well, maybe on one of my visits over the next little while, I will be able to see what you guys are doing. That would be interesting to see.

So, General, I just want to ask you a question. And congratulations on your service and obviously reaching one of the top ranks in the military. That says a lot about your ability. Why is training pediatric healthcare providers in screening—let me start over. Why is training pediatric healthcare providers in screening, brief intervention, and referral to treatment important? And why is the provision on this being added to the STOP Act? And can you discuss how this new provision's authorization will work within the overall authorization for the bill?

Mr. DEAN. Thank you for your question. It is a very important question. It is a, we believe, significant change in the restructure of this reauthorization. And we are very excited about the inclusion of—we call it SBIRT for pediatric healthcare providers. It is a strong complement to the universal prevention as it allows youth who have been misusing substances to be identified more readily and to get effective intervention in a larger number of community settings. We also know it is effective. And a 6-month followup with SBIRT participants found that heavy alcohol consumption was 39 percent lower among individuals who initially screened positive for hazardous drugs and alcohol use. So screening early, training pediatric providers to do this, we have found already it is reducing the consumption by young people.

Mr. GUTHRIE. OK. Thank you.

And, Dr. Leffert, what are the expectations that the clinical care commission can establish within the next 3 years? I guess you have 3 seconds to tell me that. So I apologize.

Dr. LEFFERT. Well, as you know—thank you for the question, Representative Guthrie. As you know, diabetes is a big issue, and there are a lot of issues going along with it but. But we would hope that the process would allow for federally funded research initiatives from the bench to the bedside so that patients could have access to 21st century cures and innovations, and that these would become more coherent and synergistic, and that there would be better communication and coordination among the agencies, specifically NIH, FDA, and CMS. The commission would also help focus the efforts of the government research community toward improving clinical care for people with diabetes and to slow the incremental rise in diabetes and its associated complications.

Mr. GUTHRIE. Thank you very much.

My time has expired, so I yield back.

Mr. PITTS. The chair thanks the gentleman.

I now recognize the ranking member of the full committee, Mr. Pallone, 5 minutes for questions.

Mr. PALLONE. Thank you, Mr. Chairman.

I wanted to ask Dr. Morrison to focus on the palliative care legislation, you know, both the access to treatment but also the services available to those individuals to cope with their conditions. First, from diagnosis, what is the most common diagnosis for individuals who receive palliative care services?

Dr. MORRISON. The common diagnoses are what we consider to be serious illness. So it is cancer, heart disease, advanced lung disease, Alzheimer's disease and related dementias, and neuromuscular diseases such as Lou Gehrig's disease or amyotrophic lateral sclerosis. In children, it is really two large groups of diseases. One is obviously cancer, and the other is congenital or genetic diseases. And because of advances in pediatrics, we have a whole population now of kids who are living long, long periods of time. And that is wonderful. However, they are living with multiple symptoms and high caregiver burden on their families.

Mr. PALLONE. Well, you mention symptoms. What are the most common symptoms that are treated with palliative care?

Dr. MORRISON. They are what you would anticipate: pain, breathlessness, fatigue, nausea, anxiety. In children, it is primarily fatigue. And it is the one symptom for which we have really no effective treatments as of yet because of the lack of the evidence base and the lack of the research science.

Mr. PALLONE. All right. Now, let me ask a couple questions about access. Do most people battling serious health conditions, such as cancer, have access to palliative care services, and what are some of the consequences for individuals with serious illness who don't have access?

Dr. MORRISON. It is a very good question. What we know from a study actually we did earlier this year was that now 97 percent of mid- to large-size hospitals in the United States now have palliative care teams. And if we look at the Medicare population, about 75 percent of all Medicare beneficiaries who die live in an area where they could potentially access palliative care. The problem is that those hospital teams are relatively small and, because they are still understaffed, see only a small proportion of the number of patients and families who could truly benefit. What we have seen in the past 3 years also, however, is the expansion of palliative care into the community, particularly in Medicare Advantage plans and commercial plans, which aren't limited by the fee-for-service structure of the traditional Medicare program. So we are seeing some very, very new and exciting models of care happening in the community, particularly in Tennessee and Nashville, as was said earlier.

Mr. PALLONE. Well, in terms of increasing access to important services, how can we increase it? How does this bill help?

Dr. MORRISON. I think it helps in three ways. The first is that, because palliative care is a relatively new specialty, most people don't know what it is. Again, a survey that we did with the Amer-

ican Cancer Society several years ago showed that about 80 percent of a national representative sample had never heard of palliative care and didn't know what it was. And yet, when we read a definition to them, over 90 percent said that this is what they would want for themselves and their families. Providers too don't understand palliative care and too often confuse it with hospice and end-of-life care, when the reality is palliative care is for everybody. It is not dependent on prognosis. And in fact, we provide it to people we expect to be cured. So there is an educational campaign awareness that needs to happen.

And the second issue is we need to address the workforce gap. Every single clinician in this country who cares for somebody with serious illness needs to be able to treat pain appropriately, manage breathlessness. Talk to them about how a serious illness—I have probably as much training, sir, as you in how to talk to somebody and break bad news that you have cancer. When I finished medical school, we had the same amount of training in terms of how to have that conversation. And we need to address that through our medical schools and our training programs and to physicians like me who are in practice. This bill will do this as well.

Mr. PALLONE. Well, thank you. I am just trying to get in one question for General Dean about strategies for preventing underage drinking. I just want to learn more about the methods to prevent underage drinking. General Dean, what types of strategies and programs work best to prevent underage drinking, and what evidence is available to prove that these interventions work?

Mr. DEAN. OK. Thank you, Congressman. It is a great question. We believe that by mobilizing the entire community—what I mean by that is all of the sectors in the community: parents, teachers, youth, the police, business providers, faith community, civic business leaders all coming together. And we have built over the last 25 years a strategy, an academy type approach, to train the members of the community how to identify their problems, how to address their problems, and how to implement evidence-based strategies to reduce their problems. There have been evaluations done by the Office of National Drug Control Policy independent of CADCA that show that when these communities have been trained, the results are significant. And we have great examples. In the interest of time, I will not cover them with you. But I do have several examples here where communities have reduced their underage drinking by large percentages, 20, 30, 40, 50 percent, using these methods.

Mr. PALLONE. Well, through the chairman, if maybe we could ask him in writing to follow up with and give us those examples. Mr. Chairman, with your permission, he mentioned that he doesn't have the time to give some examples. So, with your permission, maybe we could have him follow up in writing and give us that information.

Mr. PITTS. I am sorry. I was talking.

Mr. PALLONE. No, I know. That is all right. He wanted to give me some examples, but in the interest of time, he is not doing it. I was going to ask if he could do it in writing.

Mr. PITTS. Yes. We will submit that to you in writing and ask you to please respond.

Mr. DEAN. I will be glad to. I have several examples of communities that have made significant progress, some exceeding 50 percent reduction using these community-based strategies to reduce underage drinking all across the country, both rural, urban, as well as suburban.

Mr. PALLONE. Thank you.

Mr. PITTS. Very good. Thank you.

The chair now recognizes Dr. Burgess 5 minutes for questions.

Mr. BURGESS. Well, thank you, Mr. Chairman.

And thanks to the panel for being here.

It has been a very interesting morning listening to you all.

Ms. Banks, let me just ask you, and I was struck in your testimony, both the written testimony and your testimony here this morning, you say it has been decades since there has been a new FDA-approved treatment for sickle cell. Is that correct?

Ms. BANKS. Correct.

Mr. BURGESS. So my recollection of Parkland Hospital in the 1970s is actually pretty much current therapy. Is that right?

Ms. BANKS. Correct.

Mr. BURGESS. I referenced this in my opening statement, this committee has spent a lot of time on a bill, H.R. 6, called Cures for the 21st Century. And although sickle cell—and we tried not to have disease-specific parts of the bill, I mean, this just strikes me as one of those areas where the type of translational research that would go across the National Institute of Health or would give the National Institute of Health Director much more discretion as to what they researched and what they funded, that this would be one of those areas.

And I just went on clinicaltrials.gov to look for the current clinical trials in sickle cell, and there weren't as many as I thought there ought to be for a disease of this magnitude that is so pervasive in the community. I mean, is that a reasonable assumption I have made looking at clinicaltrials.gov?

Ms. BANKS. Actually, you are correct. We are always wanting more research for sickle cell disease. I will say this.

Mr. PITTS. Poke your microphone.

Ms. BANKS. Oh, I am sorry. I will say this. About 7 years ago, when I started the Sickle Cell Disease Association of America, we literally had about two pharmaceutical companies in the space for sickle cell disease, and today we with about 16. So it is looking up for sickle cell disease. Of course, we still have to get over that hurdle of getting individuals in those trials and going through that. But it is hopeful.

But I totally agree with you. Sickle cell disease has long been forgotten. And over 100 years—this is probably one of the oldest diseases for its discovery out there—for there only to be one drug for treatment—and by the way, that drug is hydroxyurea. That drug was not approved for sickle cell disease. It is an actual cancer drug. So, really, when you look at those kinds of issues, it is long overdue in the sickle cell community.

Mr. BURGESS. Yes. And I appreciate your comments on that. And it is something we will keep an eye on in this committee because, of course, I am going to be optimistic that we are going to get Cures for the 21st Century done in this Congress. But there will

also be an FDA reauthorization that takes place in the next Congress. And that is another appropriate place to focus on this.

General Dean, I want to ask you a question. It is probably not fair because it is not on the bill that you came to testify on. But in your role as the CEO of Community Anti-Drug Coalitions of America, I got asked a question by a constituent, and I didn't know the answer. And I was a little bit embarrassed that I didn't know the answer. And if you don't know the answer, it is OK. You don't need to be embarrassed. Perhaps you can point me in the direction that I need to go. There is a woman who came into my office. She had lost her son in a—he was a pedestrian struck by a vehicle. He was in a crosswalk. The individual who was driving the vehicle was not issued a ticket or a citation. He did have alcohol in his system, but it was under the .08 limit in the State of Texas. But he also had a positive qualitative test for the active ingredient in marijuana. OK. It seems to me that—and obviously, this would be a state law, but does your group look at, now that there are more and more states that are providing a legal avenue for consumption of marijuana, does your group look at the additive multiplicative effects of drugs and alcohol? Do states need to perhaps reconsider what their limits are? This just struck me—of course, it is a very tragic and unfortunate case. But that just seemed like it was one of those things that cried out for something in addition to be done.

Now, law enforcement made, their position was, as far as laws of the State of Texas, we don't prosecute for having small amounts of marijuana in your blood. And this was a qualitative test, not a quantitative test, so we don't even know to what degree of intoxication there might have occurred from that, but from the alcohol standpoint, under the legal limit of intoxication.

Mr. DEAN. To answer your question, Congressman, we do care about this issue very much. We do watch and observe what is happening in states that have, first, through citizen votes decreed that marijuana is medicine. It obviously has not gone through the FDA process for that to be done. So we watch that carefully. We also are watching the states where they have—through citizens have passed it for recreational use. And we have seen, looking at data coming out of States like Colorado, Washington, and others, that there is a substantial increase in citizens, both young and old, driving under the influence of drugs versus alcohol. And in some cases, there are more impaired drivers on the streets these days in those locations from drugs than there are from alcohol.

So the law enforcement challenge is having the appropriate instruments and tests to test for it. It is not as simple as it is for alcohol. And, therefore, it becomes challenging for them to do that. So the answer is we are seeing the results. We are seeing the impact. We are concerned about it, and the law enforcement community is extremely concerned about it.

Mr. BURGESS. Well, I will have my office follow up with you. We may have further discussion about this. But you are the first person who has come to this committee who might know something about this. And I do want to follow up with you. And I would appreciate the opportunity to do so.

Mr. DEAN. It would be our pleasure.

Mr. BURGESS. And, Mr. Chairman, I also would ask unanimous consent, I have a 2013 article, but it is the most recent one I could find, "Current Management of Sickle Cell Anemia," and I would like to submit this for the record.

Mr. PITTS. Without objection, so ordered.

[The information appears at the conclusion of the hearing.]

Mr. PITTS. The gentleman yields back.

The chair now recognizes the gentleman from North Carolina, Judge Butterfield, 5 minutes for questions.

Mr. BUTTERFIELD. Thank you, Mr. Chairman.

Ms. Banks, I want to come back to you for just a couple of minutes, if you don't mind. Ms. Banks, can you describe for me how diminishing Federal funding, including lack of funding for treatment centers, has made it more difficult for people with sickle cell disease to get the care that they need?

Ms. BANKS. Sure. Of course, with every disease, especially with the complications in sickle cell disease, it is a challenge when you do not have the funding in place. Eventually, when the bill was first passed, because this is a reauthorization, there was a place in the bill where it said 40 treatment centers. To my knowledge, sickle cell only received 10 of those treatment centers or 10 of those treatment centers were funded. Because of that, our patients basically do not have a medical home. That is the reason why a lot of them frequent the emergency room. So, when you talk about diminishing Federal funding, you are looking at, where do our patients go for access to care? That is extremely, extremely important.

In this bill, we ask for 25 treatment centers, because we wanted to be very realistic in our ask and we wanted those 25 centers to be in areas where there are high populations of individuals with sickle cell disease. We are hoping that that would provide some coverage. We know it will not for 100,000 patients, but it would provide some coverage for our patients and somewhere to go.

It is very interesting, in comparable diseases, for instance, with cystic fibrosis, they have over 100 treatment centers. With hemophilia, I think they have over 40, 42. So, with sickle cell disease, only having 10 funded, you can see with 100,000 patients where we are at a huge deficit.

Mr. BUTTERFIELD. Is it true that African American children have higher rates of disease in trait? Is that an accurate statement?

Ms. BANKS. Say that again.

Mr. BUTTERFIELD. That black children, African American children, have higher rates of disease.

Ms. BANKS. Of sickle cell disease?

Mr. BUTTERFIELD. Yes, of sickle cell disease.

Ms. BANKS. Yes.

Mr. BUTTERFIELD. Yes. And what proportion would you say of African American babies are screened for this disease at birth?

Ms. BANKS. Well, actually, it is mandatory in every state. So, right now, every state screens for sickle cell disease when you are born.

Mr. BUTTERFIELD. It is a Federal mandate or a state mandate?

Ms. BANKS. It is Federal.

Mr. BUTTERFIELD. Yes. All right. Are there any barriers that prevent babies from being screened for sickle cell? Are there any bar-

riers that would prevent that from happening at birth? Or is it completely uniform across the board?

Ms. BANKS. To my knowledge, it is completely uniform across the board.

Mr. BUTTERFIELD. All right.

Ms. BANKS. Our issue, Congressman Butterfield, is that, years ago, when sickle cell was very prevalent and people heard about it, it was because babies were dying. And so, because of treatments, because of the newborn screening, babies are living well into adolescence. Our issue now is transition, and where do you go after you are 14, 15 and you begin to transition into young adult care? That is where we are having the shortage of adult hematologists or adult primary care physicians for those individuals. So now the challenge in sickle cell disease is where our babies are getting better, they are living through teens and young adult, and they don't have a place to go. So when you go to college, when you get to that college age, where do you start? I was talking to my chief medical officer, and it is interesting because there are individuals 24 and 25 years of age still going to pediatric physicians, a hematologist, because they do not have an adult physician that would treat their disease in a system in managing their disease.

Mr. BUTTERFIELD. Would you discuss the barriers, if any, facing African Americans from being screened or receiving treatment for sickle cell disease?

Ms. BANKS. I think the barriers for African Americans, particularly—or anyone with the disease pretty much is, again, the lack of a medical home. Our patients have been stigmatized mainly because the key issue is pain. And if you are frequenting the emergency room for pain, what are you going to be classified as? For most, our patients feel as if they are ostracized because when they go in, they are going for drugs. And it is because we do not have any drugs for treatment of the disease that we are treating our patients with opioids. So that is a huge barrier in the African American community. But it is a huge barrier in the sickle cell community as a whole.

Mr. BUTTERFIELD. Thank you. You are very kind.

I yield back, Mr. Chairman.

Mr. PITTS. The chair thanks the gentleman.

I now recognize the chair emeritus of the full committee, Mr. Barton, 5 minutes for questions.

Mr. BARTON. Mr. Chairman, I don't have any questions, and I am late arriving. So I am going to yield to the members who have been here.

Mr. PITTS. I recognize the gentleman from Florida. You are recognized for 5 minutes.

Mr. BILIRAKIS. Thank you, Mr. Chairman.

I appreciate it. Thank you, Mr. Chairman.

Again, I want to thank Chairman Pitts and Ranking Member Green for holding this very important hearing and including my bill, the Congenital Heart Futures Reauthorization Act. I appreciate it so very much. This legislation provided a 5-year reauthorization to the underlying law that I coauthored back in 2009. The Congenital Heart Futures Reauthorization Act will continue the CDC surveillance program, continue to provide NIH grants for fur-

ther congenital heart disease research, and require NIH to report on their ongoing research efforts. Congenital heart disease is the number one cause of birth defects related deaths. Twenty-five percent of children born with a congenital heart defect will need heart surgery or other interventions to survive. An estimated 2 to 3 million people are living with CHD. And individuals with CHD have an ER visitation rate of three to four times higher than the general population. The Congenital Heart Futures Reauthorization Act will continue our commitment to monitoring and increasing the available research and helping people born with a congenital heart defect. I would like to ask unanimous consent, Mr. Chairman, to introduce these letters of support: the Pediatric Congenital Heart Association, a letter from the Adult Congenital Heart Association, and a letter from the American Society of Echocardiography. I would like to ask unanimous consent.

Mr. PITTS. Without objection, so ordered.

[The information appears at the conclusion of the hearing.]

Mr. BILIRAKIS. Thank you very much.

And I have a couple questions for Dr. Marino. Dr. Marino, what are the biggest challenges facing children and adults with CHDs as they age, and how will the Congenital Heart Futures Reauthorization Act help meet these challenges?

Thank you again, sir, for testifying today.

Dr. MARINO. Congressman, thank you very much. We greatly appreciate your support.

It is a great question. Simply put, when you have your surgery as a baby, you are not cured. And we know that these patients have specific neurodevelopmental issues that come from brain injury from when they had their surgery as a baby. We know that they have specific cardiovascular complications. Many of them go on to heart failure and need heart transplantation. They might need a ventricular assist device, mechanical support device. They often have abnormal heart rhythms. They often have decreased exercise function. These patients will also—many of them have kidney or liver injury from their original therapies when they were a baby.

So, with this complex medical milieu of multisystem organ failure, they then now need to transition from an adolescent care model to an adult care model. You have heard from several of the other witnesses who are testifying today that there is lack of adult providers who can care for these types of patients that survive the childhood illness and then now move on to adult care. Adult congenital heart disease physicians are in—we have the same dearth of care providers. You have heard about palliative care and sickle cell disease. While there is now an accreditation for adult congenital heart disease care, there are still very few adult congenital heart disease care certified individuals nationally.

And then probably the last part which we discussed a little bit earlier was that, right now, there is no specific means by which we transfer these high-risk complex patients into adult care. And if you just hand off a patient at age 18, it is very unlikely, actually, that they are going to get followup care. And what we know—and I want to focus on cost for a second—if you get patients into appropriate followup care and you minimize secondary complications as

adult congenital patients, you will lower overall costs to the system. And given that more than 50 percent of these patients will be cared for through Medicaid as children and then Medicare as adults, if we can find ways to transition them better, not lose three out of four patients during that transition process, it will likely lower the overall costs for these 2.4 million individuals nationally who survive with adult congenital heart disease.

Mr. BILIRAKIS. A very good point. Thank you so much.

Next question, the Congenital Heart Futures Reauthorization Act specifically calls for a cohort study. How does this differ from current surveillance techniques being used to study CHD, and why is this needed?

Dr. MARINO. So, if you look over the last 20 years, there are lots of individual center studies trying to benchmark how these patients are doing. They don't talk to each other relative to a data standpoint. When I was at Harvard, working at Boston Children's Hospital or Children's Hospital Philadelphia or Cincinnati Children's or now at Lurie Children's during periods of my training and now my faculty positions, the data that is collected for these individual studies are all quite different. The definitions used to codify the patients and codify the complications that we have talked about are very, very different. So I can't take 10 different studies from 10 different centers and pull them together into a cohesive whole.

What 3952 will allow is for the CDC for the first time in the United States—and by the way, this has been done in at least a dozen other countries around the world—take the U.S. population, start collecting data at dozens of centers with the same variables, the same data set, and then follow them longitudinally, not just over 2 years, hopefully over decades, for us to get a much better sense for, what is the true incidence and prevalence of these complications that I have alluded to? What are the impacts of treatments that we then bring into the care models for these patients nationally? And then, more importantly, when it comes to transition, how do we best predict who is not going to have effective transition, who will, and then get the supports in place in adolescence to make sure we don't lose three out of four of these patients. Because I can tell you, in my work in Northwestern, there are literally dozens and dozens of patients each year that walk into us at Northwestern Memorial Hospital, who have been out of care, cardiac care, for 5 years, 10 years, who literally are near death. And I know for a fact if they had actually had appropriate transition and actually stayed in care, they likely would have survived multiple more decades with a great quality of life.

Mr. BILIRAKIS. Well, thank you very much, doctor.

I want to thank the entire panel for their testimony.

And I yield back. I appreciate it.

Mr. PITTS. The chair thanks the gentleman.

I now recognize the gentlelady from Illinois, Ms. Schakowsky, 5 minutes for questions.

Ms. SCHAKOWSKY. Well, first, let me just apologize. It is hard to be everywhere at once. I was looking forward to hearing from you. And I want to thank you so much for being here, all of you.

I want to say I am proud to see that Dr. Marino is here from Lurie Children's Hospital, and I just want to acknowledge the un-

paralleled care that you provide for not just the children of Chicago but many who come to the hospital.

I have really focused for much of my public career, both in the Illinois legislature and now here, on improving senior citizens' access to health care. Along with my colleague Doris Matsui, I have served as the co-chair of the House Seniors Task Force. Given that 10,000 people turn 65 every day, it is imperative that we really work to address the specific health needs of seniors.

So, Dr. Morrison, let me focus on that. I am interested in hearing how the aging of our population is going to affect the need for palliative care services moving forward, and by the way, you might want to distinguish between hospice and palliative care as you talk. Specifically, I am interested in hearing more about a statement you included in your written testimony that says: "Over the next decade, most healthcare professionals will be caring for seriously ill older adults and their families with multiple chronic conditions, multiyear illnesses, and intermittent crises interspersed with periods of relative stability." So how is this going to affect the need for palliative care services among the population, this population, as well as a workforce trained in palliative care?

Dr. MORRISON. Thank you, Congresswoman.

And as a geriatrician, thank you so much for your work for older adults in this country.

Let me take this in two ways. First, let me clearly differentiate between hospice and palliative care. Hospice was started in this country in the 1970s really as an alternative to life-prolonging curative treatment when it was recognized that many people near the end of life were experiencing distressing symptoms and very high care needs. And it has been a wonderful system of care since 1972, and even more so since Medicare covered hospice in 1982.

The problem with hospice is that you have to be dying to access it. And you to have a predictable prognosis of 6 months or less. And for those of us who began in palliative care, the question was, why should you be dying to have efforts focused on enhancing your quality of life?

When we look at the aging of the population, as you pointed out, it is the fastest growing segment in the United States. And for most of us, the time after the age of 65 or 70 is going to be many, many years of a very good quality of life. It will be time to integrate our work and life experiences. It will be time spent with our children and our grandchildren. But most of us, those of us who aren't killed crossing the street or have a sudden death, will develop a series of chronic ongoing progressive illnesses: heart disease, lung disease, even cancer, for which we have transitioned many cancers into chronic illnesses. And as we age, we will have more and more of those: diabetes, frailty, multiple chronic conditions. And the data that we have now suggests that most of us will spend at least 7 years of our life in that state. And the data that we have nationally suggests that 70 percent of older Americans with a serious illness have three or more distressing symptoms on a daily basis. And we can do better.

The last years of our life, the last 5, 10 years, should not mean living with daily symptoms. It should not mean tremendous burdens on our children and our grandchildren to care for us. And it

should not mean bankrupting Medicare to care for those. And palliative care, as a relatively new specialty, has demonstrated that it meets all those needs.

First of all, we have a wealth of data that palliative care teams improve symptoms. They make people feel better and their quality of life better. Secondly, it improves caregiver well-being and reduces burdens on caregivers. And, thirdly, by really providing the right care to the right people at the right time, it reduces costs largely by providing that added layer of support in caring for people where they want to be cared for, in the home. In New York City, if my 85-year-old patient falls in the middle of the night and his wife can't get him up and he is struggling to breathe because of heart failure, right now, she calls a doctor's office. And when you call the doctor's office in New York City, what do you get? If this is a medical emergency, please call 911. And maybe you will get a voice at the end of the phone. If you call our palliative care team, you get a real person at the end of the phone. You may get somebody to come into the home. And you will have in place a plan to deal with predictable crises for older adults. That is the added layer of support that palliative care can provide to our healthcare system.

Ms. SCHAKOWSKY. Perfect. Thank you.

Dr. MORRISON. Thank you.

Mr. PITTS. The gentlelady yields back.

The chair now recognizes the gentleman from Missouri, Mr. Long, 5 minutes for questions.

Mr. LONG. Thank you, Mr. Chairman.

And, Dr. Morrison, how long has palliative care been around?

Dr. MORRISON. Palliative care really developed as a specialty in about the mid-1990s, as I said, when we had this lightbulb go off that said: You don't have to be dying to have good quality of life.

But it only became a sub-specialty in 2008. So it has really only been since 2008 that the American Board of Medical Specialties has recognized palliative care as a specialty. And so it is a very young field.

Mr. LONG. My mom passed away in 2009. And I remember that when they came in and said, "We need to talk about palliative care," that was the first that I had really heard about it.

My series of questions are for you, Dr. Morrison. They focus on the care and support needs of individuals with Alzheimer's disease and other dementias. Could you elaborate on how palliative care could benefit people with Alzheimer's?

Dr. MORRISON. Yes. Absolutely. As you know, the prevalence of Alzheimer's disease is increasing rapidly in the United States, largely as we have made tremendous progress in treating other diseases. Alzheimer's disease fits in many respects perfectly within the paradigm of palliative care. It is a multiyear illness. Families, as cognitive status declines, patients are more and more dependent upon their families. It is a disease that is associated with a tremendous symptom burden. All of the diseases that people had before Alzheimer's disease, their osteoarthritis, their heart disease that causes breathlessness, their lung disease, don't go away in the setting of Alzheimer's disease. What happens, though, is people can't tell you that they are breathless. They can't tell you that they are

in pain. They can't tell you that they are hungry because of cognitive impairment. And so the suffering continues, but the suffering continues silently.

It is also a disease that has periods of stability where people will be the same for long periods of time and then there will be a crisis, an infection, a pneumonia, a urinary infection, a pressure ulcer. And so it doesn't fit well within our current model of hospice because people with Alzheimer's disease aren't dying quickly. They are actually living for a long period of time. And what they do is they need support, and they need then crisis intervention, which palliative care can provide, and then ongoing support after that throughout the course of that illness. And, as importantly, Alzheimer's disease is not just a disease that affects the patient. All of us who have had a loved one with dementia or know somebody know that it extends to the family as well, and that the burden on families is almost as great as the patient itself, and that, as a specialty, palliative care focuses on both the patient and the family as the unit of care.

Mr. LONG. OK. Thank you. My aunt passed away from Alzheimer's about 6 weeks ago. So I can relate to everything you are saying there.

How easy is it for individuals to gain access to palliative services today? Second part, are there enough providers offering these services across different settings? And are there enough new providers being trained in this space to meet patient needs?

Dr. MORRISON. Moderately easy, no, and no. And let me elaborate.

Mr. LONG. OK.

Dr. MORRISON. The first is that we have built over the past 20 years, largely because of private sector philanthropy and investment in infrastructure to support the development of palliative care. So, right now, 95 percent of our mid- to large-size hospitals have palliative care teams. And over two-thirds of all American hospitals have that infrastructure in place.

The issue is, as you pointed out, it is the workforce, that we actually don't have enough providers to be able to provide those services to everyone in need. And we really need two things. First of all, we need a specialist workforce not to take care of everybody with serious illness. That will never happen, and that should not be our goal. We need specialists to teach, to do the necessary research, and take care of the most complex patients and their families. And that is what the provisions of 3119 provide.

But, as importantly, we need to train every clinician who cares with somebody with serious illness in the core knowledge and skills of palliative care so that every doctor in this country knows how to treat pain effectively, every nurse knows how to communicate serious illness to somebody, and we have a care system that can provide that added layer of support for that very small but very expensive and very vulnerable patient population.

Mr. LONG. Lastly, I would like to know, how do the needs differ of the older patients from the needs of younger patients as relates to providing palliative services, and do current training opportunities address these differences?

Dr. MORRISON. It is a very good question. For most younger adults, most younger adults are typically living with a single illness. Is it cancer? Heart disease? For children, cystic fibrosis. For older adults, it is much more complex, because most of us, when we age, will develop multiple chronic conditions that all intersect and all affect our quality of life. So it is not just cancer. It is cancer. It is heart disease. It is debilitating arthritis. It is diabetes. And it is both cognitive impairment, Alzheimer's disease, and often functional impairment, difficulty walking. So it is a much more complex population in many respects. I think what we have done very well within our field is the collaboration with geriatrics. The recognition that we will never have enough geriatricians, we will never have enough palliative care physicians to treat the population, the older adult population that need, and it requires a collaboration and for us to break out beyond specialist-level care to think about population-related care. And that is one of the reasons that 3119, the bill before you, is modeled after the very successful Geriatric Academic Career Awards. Because that was such a good model for improving access to care for——

Mr. LONG. Speaking of 3119, that is how many minutes I am past time. So I yield back.

Dr. MORRISON. I apologize, sir.

Mr. PITTS. The chair thanks the gentleman and now recognizes the gentleman from California, Mr. Cardenas, 5 minutes for questions.

Mr. CARDENAS. Thank you very much, Mr. Chairman. And thank you for having this important hearing. First, I would like to recognize and thank my colleague Lucille Roybal-Allard for championing the legislation H.R. 1717, which is part of this hearing today, the Sober Truth in Preventing Underage Drinking Reauthorization Act. It is unfortunate that, being on Appropriations, you are not allowed to be on Energy and Commerce.

But thank you, colleague Lucille Roybal-Allard, for being here and for introducing that great bipartisan legislation.

My questions today are based on diabetes. And I would like to ask some questions to Dr. Leffert. If you don't mind explaining to us what prediabetes is and how it increases the risk of an individual developing type 2 diabetes.

Dr. LEFFERT. Thank you, Congressman.

Prediabetes is the process of developing diabetes but before that happens. So the genetic and environmental process results in a situation where what we call glucose intolerance or impaired fasting glucose. Both of those conditions are what we now have termed prediabetes. The issue with prediabetes is that prediabetes is a surrogate for cardiovascular disease. So, in people who have prediabetes, the risk for cardiovascular disease goes up, and then the disease process then may progress on to type 2 diabetes. So it has both an effect in and of itself and also as a progenitor towards type 2 diabetes. The data, I think, is that about a third of the patients will go on to type 2 diabetes, about a third of the patients will remain prediabetic, and about a third of the patients will regress if they start with diet and exercise. And so the main issue in prediabetes for our population and why it is such a huge issue is because of the fact that it is associated with obesity and genetic

factors, particularly among populations, like African Americans and Hispanics, in our country.

Mr. CARDENAS. And with proper education and cooperation with their health providers, et cetera, a person can decrease their chance of going from prediabetic to developing type 2 diabetes?

Dr. LEFFERT. That is absolutely correct. And I think that should be a big push of what we are doing in our healthcare prevention, meaning keeping people at the level of prediabetes or moving backward would be the most important aspect of what we do. And we can do that through a recognized approach toward nutrition therapy, toward dietary therapy, toward exercise. And there have been programs that have been done through the diabetes prevention program that have been successful in doing that particular thing itself.

Mr. CARDENAS. OK. How can the commission improve our ability to reduce the development of diabetes among individuals with prediabetes?

Dr. LEFFERT. Well, I think, again, it is a coordination issue. I think our Federal Government has a number of different agencies that are all working somewhat in silos. And I think our commission would allow us to have all of the organizations, including the private sector, industry, and other organizations together, to be able to coordinate that effort and prevent the onset of diabetes if we have people who have prediabetes.

Mr. CARDENAS. Now, obviously, there is a quality-of-life issue for somebody to not develop into having type 2 diabetes. But what quantifiable numbers when it comes to dollars would be saved if we were more successful in our efforts and coordinated better like you just described? Are we talking just a few million dollars a year to our economy, or are we talking billions of dollars?

Dr. LEFFERT. I think we are talking more like billions of dollars because I think the issue, when we go from prediabetes to diabetes and the hospitalizations that are associated with diabetes, the complications of diabetes related to kidney disease, heart disease, eye disease, is astronomical, and I think we could save a large amount of money of our Federal budget related to that.

Mr. CARDENAS. So diabetes-related illnesses like you just described I would imagine over lifetimes of tens and hundreds and millions of people would actually cost us trillions of dollars. Wouldn't it?

Dr. LEFFERT. Well, I think, right now, as Chairman Pitts said in his opening statement, one in three dollars of the Medicare budget is spent in diabetes.

Mr. CARDENAS. So it is in the trillions.

Dr. LEFFERT. It is a very, very significant amount of money that is being utilized in that regard.

Mr. CARDENAS. Thank you. I yield back.

Mr. PITTS. The chair thanks the gentleman.

And I now recognize the gentlelady from Indiana, Mrs. Brooks, 5 minutes for questions.

Mrs. BROOKS. Thank you, Mr. Chairman.

In my home State of Indiana, over 750,000 Hoosiers have type 1 or type 2, and nearly 2 million of about 6 ½ to 7 million Hoosiers have prediabetes. And so we know it is taking an immense toll on our State and on the Nation's healthcare system. But yet you

talked about some innovations, continuing glucose monitoring and artificial pancreases. Can you tell me, Dr. Leffert, how the new treatments, new devices that are on the horizon that can help bend the curve on both the incidence and the cost of the disease and help patients better manage their disease, how is this commission going to have a role in expediting patient access to these innovations?

Dr. LEFFERT. So you told us about a very big problem. And we talked about that already. I think the issue for us in terms of getting the treatments to the patients is really one, to some extent, of making sure that the cost is available, that we have treatments that are cost-effective and not so expensive that patients can afford them, but in addition, the commission will allow us to utilize the resources of multiple different agencies working together to be able to first move these new technologies forward—the artificial pancreas being one of them, particularly in type 1 diabetes—and then also help us with prioritization of the ability to educate the physician workforce.

We have 5,000 endocrinologists in the United States. That is not nearly enough to take care of diabetes. We have to educate and maintain that workforce at the primary care level and with all clinicians who are taking care of people with diabetes. So it is a tremendous opportunity here to utilize this commission to then focus our efforts at the level of the clinical physician and associated healthcare providers to be able to give these patients the best care.

Mrs. BROOKS. And is that being done? Because I am a huge believer in public/private partnerships, especially when it comes to commissions and government commission work, and so is involving the private sector clinicians on the commission the manner in which you are going to educate the agencies?

Dr. LEFFERT. To some extent I think that is exactly the reason for our bill. This bill started within our organization in being interested in trying to make sure that the flow of dollars coming from the Federal Government was adequately being utilized to take care of patients. Our organization, the American Association of Clinical Endocrinologists, are the physicians on the ground who take care of patients on a daily basis. We have the ability to see, though, not all of the patients that have diabetes. We focus, to a large extent, our efforts on complicated patients. And we want to be able to translate our knowledge and information throughout the system. And so that allows us to give primary care physicians the information and education, and we need the Federal Government to be able to push that through to the whole sector of physicians who take care of patients.

Mrs. BROOKS. Thank you.

Dr. Morrison, in your opening statement you talked about your concern, and there has been a lot of attention on opioids, and we just passed and had signed into law a very significant piece of opioid legislation. My involvement with that involved, actually, a task force focused on the prescribing practices of physicians. And can you please speak to the impact of that legislation or what your concern is? And we did add pain specialists to our task force because we want to ensure that patients who need opioids get them. But can you talk about that balance when we have an opioid epidemic happening in the country?

Dr. MORRISON. It is a challenge. And I recognize in many respects and I am envious I am not in your position about how to address it. But let me address a couple things.

First of all, there are really two populations of patients to think about when we talk about pain. First, there are the people who live with chronic pain and pain is their only symptom. The much larger population, and the one that we are focusing on here, is the patient population with serious illness where pain is just one of a number of distressing symptoms, so, for most people, pain, breathlessness, fatigue, anxiety. And it is a constellation. And yet the prescribing practices that we need to teach are very similar within both populations. And I often hear about: Well, we are going to carve out people with cancer or people at the end of life, and they are not going to be part of the legislation. The reality is, though, that that is only a small fragment of those with serious illness, that people are going to live for many years with pain, distressing symptoms, and they are not going to be treated by specialists. They are going to be treated by primary care physicians, cancer doctors.

And so what we need to do is we need an aggressive effort that is going to focus on teaching appropriate opioid prescribing. But, as importantly, it still strikes me as almost unimaginable that the drug we have for pain has not changed since the 1600s and that we need major investment in alternatives to a drug that we know has not only significant side effects but significant complications. So we need to teach appropriate opioid prescribing, appropriate recognition of the signs and symptoms of addiction, appropriate training into what are opioid-responsive pain syndromes and what are not, and we really need a critical investment in research to give us an alternative to opioids to treat pain and other symptoms.

Mrs. BROOKS. Thank you very much.

I yield back.

Mr. PITTS. The chair thanks the gentlelady and now recognizes the gentleman from New York, Mr. Engel, 5 minutes for questions.

Mr. ENGEL. Thank you, Mr. Chairman and Mr. Green, for convening this morning hearing. I am so pleased to have an opportunity to discuss H.R. 3119, the Palliative Care and Hospice Education and Training Act, a bill that I introduced with Congressman Tom Reed, my colleague from New York. Every one of us has been touched by serious illness, whether we have been affected personally or stood by a loved one grappling with critical illness. We all know how physically and emotionally trying situations can be for all those involved. Palliative care aims to relieve these stresses.

And thank you, Dr. Morrison, for everything you have been saying.

I am from New York City as well. Palliative care complements efforts to treat or cure illness by focusing on patients' quality of life. Palliative care is appropriate for patients with serious illness, starting at the point of diagnosis through treatment and onward through hospice and the end of life. It involves capable communication with patients and their families to coordinate care, determine preference, and help with medical decisionmaking throughout the care continuum. Despite the benefits of palliative care, many Americans aren't aware of the supports available to them. In addition,

there is a shortage of educated providers who can offer quality palliative care.

So my bill, H.R. 3119, aims to remedy these issues. My bill would expand opportunities for training in palliative and hospice care and offer incentives to attract and retain providers. In addition, through existing programs, my bill would create a national campaign to educate patients, families, and health professionals about the benefits of palliative care. And, finally, H.R. 3191 would expand critically needed research on palliative care at the National Institutes of Health.

I want to thank Chairman Upton, Chairman Pitts, Ranking Member Pallone, Ranking Member Green, for considering this important bill. And I would also like to thank the 200 Members, colleagues of House, who have cosponsored it, including several members of this committee. And I hope today's discussion, as it has been doing, will bring us one step closer to enacting this legislation and extraordinarily improvingpatients' quality of life.

So let me, Dr. Morrison, thank you again for being here today. During your testimony, you noted that palliative care has the potential to bring about long-term savings for the healthcare system. Would you explain exactly how improved access to palliative care and, specifically, this bill would produce these savings? And have there been studies that actually conclude that there are real savings?

Dr. MORRISON. Before answering your question, Mr. Engel, thank you, on behalf of the patients and families that I take care of. I live just south of your district.

Mr. ENGEL. Move on up.

Dr. MORRISON. Thank you for sponsoring this legislation. The question is, will palliative care provide savings to the healthcare system? And the answer is, yes, it will. When we look at the population that palliative care provides for, it is the 5 percent of Medicare beneficiaries that are accounting for over 50 percent of spending. And what palliative care does is it provides the added layer of support that reduces the misutilization for that population. How does it do that? First of all, it provides a safe environment at home, so in the setting after crisis in the middle of the night, on a weekend, or any time that is not Monday to Friday, 9 to 5, it provides the added layer of support at home so that somebody doesn't have to go to the emergency department for care. Our modern-day hospitals are designed for the 95 percent of people who don't need palliative care, and it is a mismatch, a tremendous mismatch for somebody with multiple chronic conditions, cognitive impairment, frailty, in our modern hospitals. And what palliative care teams do is they provide that added layer of support and make the hospitals friendly to people with chronic illness. They address pain and other symptoms. They sit with patients and families and identify: What are their values? What are their goals for care? What are they hoping to accomplish? And then we match treatments to meet those goals, and in doing so, we reduce unnecessary and unwanted healthcare utilization. And conversely to hospice, we do this at the same time as all other appropriate life-prolonging treatments.

The question about cost and cost savings is an important one. We now have studies in general hospitals within the Medicaid popu-

lation, within the Veterans Administration, all that demonstrate that when palliative care is provided at the same time as other appropriate treatments, costs are dramatically reduced, and importantly, quality of life goes up, and survival is exactly the same, if not longer.

Mr. ENGEL. I think the point about survival certainly the same, if not longer, how do efforts to better patients' quality of life simultaneously enhance patients' clinical outcomes, you know, the tie-in between the two?

Dr. MORRISON. How does palliative care enhance clinical outcomes? We don't know for sure, but we have a very strong hypothesis why. First of all, we know that people living in pain, people who are depressed, people who are anxious, all contribute to increased medical complications. Pain is associated with delirium and confusion. Pain means that you can't get out of bed and walk, so you lose muscle mass. Pain prevents you from eating because you just don't feel hungry. Nausea does the same. So palliative care, by specifically focusing on distressing symptoms, allows people to get better.

The best example I can give is my 35-year-old who had a very aggressive lymphoma but because of the palliative care she received, she made every single one of her chemotherapy appointments on time because she wasn't too nauseated, too sick, too distressed, and subsequently, she made every single radiotherapy on time and completed her treatment, so the palliative care she provided allowed her to complete her curative treatments.

Mr. ENGEL. Thank you.

Mr. Chairman, can I please ask unanimous consent to enter into the record statements in support of H.R. 3119 from the Alzheimer's Association, the American Academy of Hospice and Palliative Medicine, the National Hospice and Palliative Care Organization, and the Oncology Nursing Society, as well as a letter of support from the 45 organizations on record in support of the bill?

And I want to thank Dr. Morrison for being the most eloquent speaker on this that I have heard. Thank you.

Mr. PITTS. Without objection, so ordered.

[The information appears at the conclusion of the hearing.]

Mr. ENGEL. Thank you, Mr. Chairman.

Mr. PITTS. The chair thanks the gentleman. That concludes the questions of the members of the committee present. We will have some followup questions, questions in writing. We will provide those to you. We ask that you please respond promptly.

I remind members that they have 10 business days to submit questions for the record, so members should submit their questions by the close of business on Thursday, September 22.

Very interesting, very important, very informative hearing. Thank you very much for your testimony.

And, with that, this hearing stands adjourned.

[Whereupon, at 12:05 p.m., the subcommittee was adjourned.]

[Material submitted for inclusion in the record follows:]

September 8, 2016

The Honorable Fred Upton
Chairman
House Energy & Commerce Committee
Washington, D.C. 20515

The Honorable Frank Pallone
Ranking Member
House Energy and Commerce Committee
Washington, D.C. 20515

The Honorable Joe Pitts
Chairman
Health Subcommittee
Washington, D.C. 20515

The Honorable Gene Green
Ranking Member
Health Subcommittee
Washington, D.C. 20515

The Honorable Pete Olson
U.S House of Representatives
2133 Rayburn House Office Bldg.
Washington, D.C. 20515

The Honorable Dave Loebsack
U.S. House of Representatives
1527 Longworth House Office Bldg.
Washington, D.C. 20515

Dear Representatives Upton, Pallone, Pitts, Green, Olson and Loebsack,

The undersigned organizations, representing physicians, allied health professionals, patients, community health organizations and industry, commend you for holding a hearing on September 8th regarding H.R. 1192, "The National Diabetes Clinical Care Commission Act." We urge passage by the Energy and Commerce Committee as soon as possible to facilitate expedited approval by the House and subsequent approval by the Senate this year.

We are very hopeful that the Committee's action will allow for enactment of H.R. 1192 this year and help the nation pivot from continuing the status quo to instead undertaking more proactive and innovative approaches to metabolic, autoimmune and insulin resistant diseases, which include the escalating diabetes epidemic and the chronic disease complications of diabetes. The United States spends $322 billion annually controlling the high blood glucose levels that characterize diabetes or the cardiac, nerve, kidney and newly recognized cancer-related complications of the disease. At this level of investment, the nation cannot afford to be complacent and accept inefficiencies and inconsistent policies as part of the federal response to a disease that continues to grow unchecked.

As you know, H.R. 1192 creates a commission which would include leaders of the federal government as well as several private sector experts in diabetes and its disease complications. The commission would review the myriad of ways the government currently spends money on activities related to the diagnosis and treatment of diabetes and other metabolic, autoimmune and insulin resistant diseases, and make recommendations on how these funds could be utilized more effectively.

Through innovation, collaboration, and application of advances in care called for in this important legislation, H.R. 1192 provides the opportunity to begin to reduce the staggering impact of diabetes on health care spending, while simultaneously improving care for the tens of millions of Americans living with diabetes, and the many more tens of millions of Americans impacted by diabetes.

We thank you and the Members of the Energy and Commerce Committee for taking action to advance H.R. 1192 and we are gratified by the support of the 220 co-sponsors of this bill. We look forward to working with you to secure its passage by the House of Representatives and the Senate this year.

Sincerely,

Abbott
Academy of Nutrition and Dietetics
American Academy of Family Physicians
American Academy of Ophthalmology
American Association of Clinical Endocrinologists
American Association of Diabetes Educators
American Association of Kidney Patients
American Clinical Laboratory Association
American College of Cardiology
American Diabetes Association
American Medical Association
American Optometric Association
American Pharmacists Association
American Society for Metabolic and Bariatric Surgery
American Society of Endocrine Physician Assistants
American Society of Nephrology
AstraZeneca
Becton Dickinson and Company
Board for Certification of Nutrition Specialists
Children with Diabetes
Dexcom, Inc.
Diabetes Hands Foundation
Diabetes Patient Advocacy Coalition
diaTribe Foundation
Eli Lilly and Company
Endocrine Society
Healthcare Leadership Council
Health Monitor Network
Johnson & Johnson
JDRF
Medtronic
National Kidney Foundation
Novo Nordisk, Inc.
Obesity Action Coalition
Obesity Medicine Association
Omada Health, Inc.
Renal Physicians Association
Roche Diabetes Care, Inc.
Sanofi
The Obesity Society
Vivus, Inc.
Weight Watchers International
YMCA of the USA

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September 8, 2016

The Honorable Fred Upton
Chairman
House Energy & Commerce Committee
Washington, D.C. 20515

The Honorable Frank Pallone
Ranking Member
House Energy and Commerce Committee
Washington, D.C. 20515

The Honorable Joe Pitts
Chairman
Health Subcommittee
Washington, D.C. 20515

The Honorable Gene Green
Ranking Member
Health Subcommittee
Washington, D.C. 20515

The Honorable Pete Olson
U.S House of Representatives
2133 Rayburn House Office Bldg.
Washington, D.C. 20515

The Honorable Dave Loebsack
U.S. House of Representatives
1527 Longworth House Office Bldg.
Washington, D.C. 20515

Dear Representatives Upton, Pallone, Pitts, Green, Olson and Loebsack:

The Academy of Nutrition and Dietetics commends you for holding a hearing on September 8, 2016, regarding H.R. 1192, "The National Diabetes Clinical Care Commission Act." Representing more than 100,000 registered dietitian nutritionists (RDNs), nutrition and dietetic technicians, registered (NDTRs) and advanced-degree nutritionists, the Academy is the largest association of food and nutrition professionals in the United States. Academy members play an important role in the prevention and treatment of diabetes and we support this important legislation. We urge passage by the Energy and Commerce Committee as soon as possible to facilitate expedited approval by the House and subsequent approval by the Senate this year.

The Academy is hopeful the Committee's action will allow for enactment of H.R. 1192 this year and help the nation pivot from continuing the status quo to instead undertaking more proactive and innovative approaches to metabolic, autoimmune and insulin resistant diseases, which include the escalating diabetes epidemic and the chronic disease complications of diabetes. The total cost of diabetes to the United States has increased nearly 50 percent in five years, totally $322 billion annually. This cost includes not only diabetes care, but also the myriad of complications that occur with diabetes. Furthermore, there are 29.1 million people in the U.S. with diabetes and 86 million with prediabetes. As the Medicare population continues to grow at a rapid pace, it is critical that Congress act now to ensure our health care dollars are invested in the best treatment and prevention options for people with prediabetes and diabetes.

As you know, H.R. 1192 creates a commission that includes leaders of the federal government, as well as private sector health care professionals, including registered dietitian nutritionists (RDNs) who provide care for patients with prediabetes and diabetes. The commission would review the myriad of ways the government currently spends money on activities related to the diagnosis and treatment of diabetes and other metabolic, autoimmune and insulin resistant diseases and make recommendations on how these funds could be utilized more effectively.

Through innovation, collaboration and application of advances in care called for in this important legislation, H.R. 1192 provides the opportunity to address the enormous economic and human toll caused by diabetes and complications of the disease.

The Academy of Nutrition and Dietetics thanks you and the Members of the Energy and Commerce Committee for taking action to advance H.R. 1192 and we again offer the Academy's support for this critical legislation. We look forward to working with you to secure its passage by the House of Representatives and the Senate this year.

Sincerely,

Lucille Beseler, MS, RDN, LDN, CDE, FAND
President 2016-2017
Academy of Nutrition and Dietetics

Statement of Novo Nordisk
In Support of H.R.1192, the National Diabetes Clinical Care Commission Act

To the Subcommittee on Health
Committee on Energy and Commerce
United States House of Representatives

Hearing on "Examining Legislation to Improve Public Health"

September 8, 2016

Thank you for giving us the opportunity to provide a statement in support of H.R. 1192, the National Diabetes Clinical Care Commission Act. Novo Nordisk applauds the House Energy and Commerce Committee for taking up this important legislation, which could help improve care for people living with diabetes.

Novo Nordisk is a healthcare company with more than 90 years of history in innovation and achievement in diabetes care. In addition to diabetes care, Novo Nordisk is a leader in obesity, hemostasis management, growth hormone therapy, and hormone therapy for women. Novo Nordisk's business is driven by our Triple Bottom Line: a commitment to economic success, environmental soundness, and social responsibility to our employees and customers. Our global headquarters are in Denmark and our US headquarters is in Princeton, New Jersey. We conduct research and manufacturing in the US, and have over 6,000 US-based employees in all 50 states. Here in the US, Novo Nordisk is also proud to be a Co-Chair of the Diabetes Advocacy Alliance, a coalition of 20 members representing patients, professional and trade associations, other nonprofit organizations, and corporations, all united in the desire to change the way diabetes is viewed and treated in America.

We agree that it is time to look at how the Federal Government can best manage its programs and spending on diabetes, so that we can meaningfully impact the numbers of people living with these diseases, and also improve the care and quality of life for patients with chronic conditions.

The epidemic of diabetes is devastating our healthcare system and economy. Today, more than 29 million people in the U.S. are living with diabetes, and another 86 million are at high risk for the disease.[i] During the past 30 years, the percentage of Americans diagnosed with diabetes has more than doubled; and by 2050, one in three will be living with diabetes.[ii] In the Medicare population, the statistics are just as concerning. Over one quarter of the Medicare-eligible population (nearly 11 million seniors) have diabetes.[iii] Furthermore, the longer people live with diabetes, the more likely it is they will suffer from related complications, including heart disease, blindness, lower limb amputations, and kidney failure, among others.[i] These largely preventable problems will add a human and economic burden not only to the individual and their families, but also to the federal government.

This chronic disease continues to be one of the largest healthcare threats to our nation's economy. Currently, one in three Medicare dollars is spent on people with diabetes, and the cost of diabetes has continued to increase significantly, with the true total cost of diabetes rising to $322 billion per year in 2012, up 48 percent ($100 billion) in just five years from $218 billion in 2007.[iv] For individuals, the financial burden is taking a toll. People diagnosed with diabetes have healthcare costs 2.3 times higher than if they didn't have the disease.[i]

We have the real potential to bend the Medicare and Medicaid cost curve if we can begin to develop effective strategies to reduce the onset of diabetes and the costly but preventable complications of the disease. Currently there are 35 different agencies that have programs or policies related to diabetes. However, they are largely uncoordinated and likely redundant. This bill would create a commission for the purpose of improving the implementation and coordination of clinical care for patients with pre-diabetes, diabetes, and the chronic diseases and conditions that result from diabetes. The Commission, representing a partnership between private sector experts and specialists in the Federal agencies most active in clinical care, will issue recommendations to Congress and to the Secretary of HHS on new approaches to improve patient care, such as getting information and resources to clinicians on best practices for delivering high quality care, and effectively deploying new treatments and technologies, such as the artificial pancreas. The bill requires no new money because the legislation specifies that the Commission meetings will be supported through existing HHS funds.

Several states have already recognized the toll of diabetes on their state budgets and have passed Diabetes Action Plans in their state to address this epidemic. We believe strongly there should be a similar response at the federal level and that is why we urge for passage of the National Diabetes Clinical Care Commission Act.

According to the Novo Nordisk Way, our contribution to society comes from having a patient-centered approach. Given this bill's potential to make a difference in the lives of millions of people with diabetes and prediabetes, we strongly support its passage and look forward to working with you as this bill progresses.

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[i] Centers for Disease Control and Prevention. National Diabetes Statistics Report 2014. Accessed June 10, 2015.
[ii] Centers for Disease Control and Prevention. Projection of the year 2050 burden of diabetes in the US adult population: dynamic modeling of incidence, mortality, and prediabetes prevalence Report 2010. Accessed June 10, 2015.
[iii] Center for Medicare and Medicaid Services, Chronic Conditions Among Medicare Beneficiaries Chartbook: 2012 Edition.
[iv] ADA Cost of Diabetes in 2012.

Diabetes Advocacy Alliance™

September 6, 2016

The Honorable Fred Upton
Chairman
U.S. House of Representatives
Energy and Commerce Committee
Washington, D.C. 20515

The Honorable Frank Pallone
Ranking Member
U.S. House of Representatives
Energy and Commerce Committee
Washington, D.C. 20515

The Honorable Joe Pitts
Chairman
U.S. House of Representatives
Health Subcommittee
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The Honorable Gene Green
Ranking Member
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The Honorable Pete Olson
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The Honorable Dave Loebsack
U.S. House of Representatives
1527 Longworth House Office Bldg.
Washington, D.C. 20515

Dear Representatives Upton, Pallone, Pitts, Green, Olson and Loebsack:

The Diabetes Advocacy Alliance™ (DAA) would like to express our support and appreciation for the public health hearing the House Energy and Commerce Committee's Subcommittee on Health has scheduled for September 8th, 2016 regarding H.R. 1192, the *"National Diabetes Clinical Care Commission Act."* The DAA strongly supports this legislation and urges the Energy and Commerce Committee to pass the legislation as soon as possible so it can move expeditiously to the House and Senate floors for approval.

The DAA is a coalition of 21 diverse member organizations, representing patient, professional and trade associations, other non-profit organizations, and corporations, all united in the desire to change the way diabetes is viewed and treated in America. Since 2010, the DAA has worked to increase awareness of, and action on, the diabetes epidemic among legislators and policymakers. The organizations that comprise the DAA share a common goal of elevating diabetes on the national agenda so we may ultimately defeat diabetes.

The *"National Diabetes Clinical Care Commission Act"* creates a commission comprised of federal government leaders and private sector experts in diabetes and its disease complications to review how the government is currently spending resources on activities related to the diagnosis and treatment of diabetes and other metabolic, autoimmune and insulin resistant diseases. The commission would then make critically important recommendations on how to utilize these funds more effectively. The legislation has the potential to reduce the overwhelming impact diabetes has on health spending while at the same time improving care for some of the nearly 30 million Americans living with diabetes and the many more tens of millions of Americans

impacted by the disease.

Again, we thank you and the Members of the Energy and Commerce Committee for taking action to advance H.R. 1192 which has very strong, bipartisan support. The DAA looks forward to working with you to secure its passage by the House of Representatives and Senate this year.

Sincerely,

Karin Gillespie	Meghan Riley	Henry Rodriguez, MD
Novo Nordisk	American Diabetes Association	Pediatric Endocrine Society
DAA Co-chair	DAA Co-chair	DAA Co-chair

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American Society of Hematology
Statement to the
Committee on Energy and Commerce
Subcommittee on Health

Hearing on Examining Legislation to Improve Public Health

September 8, 2016

Chairman Pitts, Ranking Member Green, and members of the Energy and Commerce Subcommittee on Health, the American Society of Hematology (ASH) is extremely grateful for the opportunity to provide written testimony for the record on H.R. 1807, the *Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2015*, which was introduced last year by Representatives Danny Davis and Michael Burgess. We applaud their efforts along with those of members of the Congressional Sickle Cell Disease Caucus.

ASH represents more than 16,000 physicians, researchers, and medical trainees committed to the study and treatment of blood and blood-related diseases such as leukemia, lymphoma, and myeloma; non-malignant conditions, including anemia and hemophilia; and congenital disorders, such as thalassemia. ASH members also include clinicians who specialize in treating children and adults with sickle cell disease (SCD) and researchers who investigate the causes and potential treatments of sickle cell disease manifestations.

SCD is an inherited, lifelong blood disorder that causes individuals to produce abnormal hemoglobin, causing their red blood cells to become rigid and sickle-shaped. These sickled cells have a shortened lifespan, resulting in a constant shortage of red blood cells. When these sickled cells travel through the blood, they often get stuck in the smaller blood vessels, blocking other oxygen-rich red blood cells from freely flowing throughout the body. This leads to complications ranging in severity, including severe pain, acute chest syndrome (a condition that lowers the level of oxygen in the blood), stroke, organ damage, and even premature death. In the United States, approximately 100,000 individuals have SCD. The Centers for Disease Control and Prevention (CDC) estimates that SCD affects 1 out of every 365 black or African-American births, and 1 out of every 16,300 Hispanic-American births.

Although the molecular basis of SCD was established five decades ago, it has been challenging to translate this knowledge into the development of novel targeted therapies. Many important discoveries have been made, and some treatments developed. These discoveries have identified innumerable questions and opportunities to better understand and treat this complex disease. Yet, many basic scientific processes are still not fully understood, too few treatments have been developed, and care that could improve the duration and quality of life for individuals with SCD is inconsistent in the United States and wholly absent in large parts of the world.

Currently, Hydroxyurea, the only FDA-approved drug for adults with SCD (often used off-label in children), improves the course of SCD and might lead to significant health care cost reductions. In a two year pediatric study, overall health care costs for children on hydroxyurea were $1.8 million, compared with $2.5 million for those who did not receive this treatment.

Unfortunately, hydroxyurea is not regularly prescribed and adherence to the therapy is poor. In addition to hydroxyurea, blood transfusions can help to manage SCD, but they can lead to abnormally high levels of iron in the blood, which can cause long-term organ damage and reactions due to a mismatch between the donors and recipients. There has also been early success in curing SCD through bone marrow transplant, but this process is costly, dangerous, and is not widely available.

In an effort to identify unmet medical needs for people with SCD, ASH, along with other groups, earlier this week issued the *State of Sickle Cell Disease: 2016 Report*, evaluating the disease in four priority areas — access to care, training and professional education, research and clinical trials, and global health. The report shows that significant improvements are needed across all areas and that, though patients are living longer, the system of care needs to change to ensure a better quality of life. To address these challenges, ASH launched the Sickle Cell Disease Coalition along with more than 20 other organizations who are issuing a call to action that will amplify the voice of the SCD community, promote SCD awareness, and transform SCD care both in the United States and around the globe.

ASH thanks the Subcommittee for focusing needed attention on SCD and supports *The Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2015* (H.R. 1807). However, the Society believes there is the need for additional legislation to enhance current federal activities in SCD research, training and services.

Strengthening and expanding current efforts will help ensure that individuals living with this disease receive adequate care and treatment. In fact, a multi-pronged approach by key Federal agencies in collaboration with private sector sickle cell stakeholder groups could more efficiently, effectively and economically improve the comorbidities and health disparities faced by individuals with SCD and their families. Specifically, ASH believes the following priorities are important components of any legislative package addressing SCD:

- Authorization of the Department of Health and Human Services' Interagency Working Group on SCD to coordinate efforts among federal agencies.

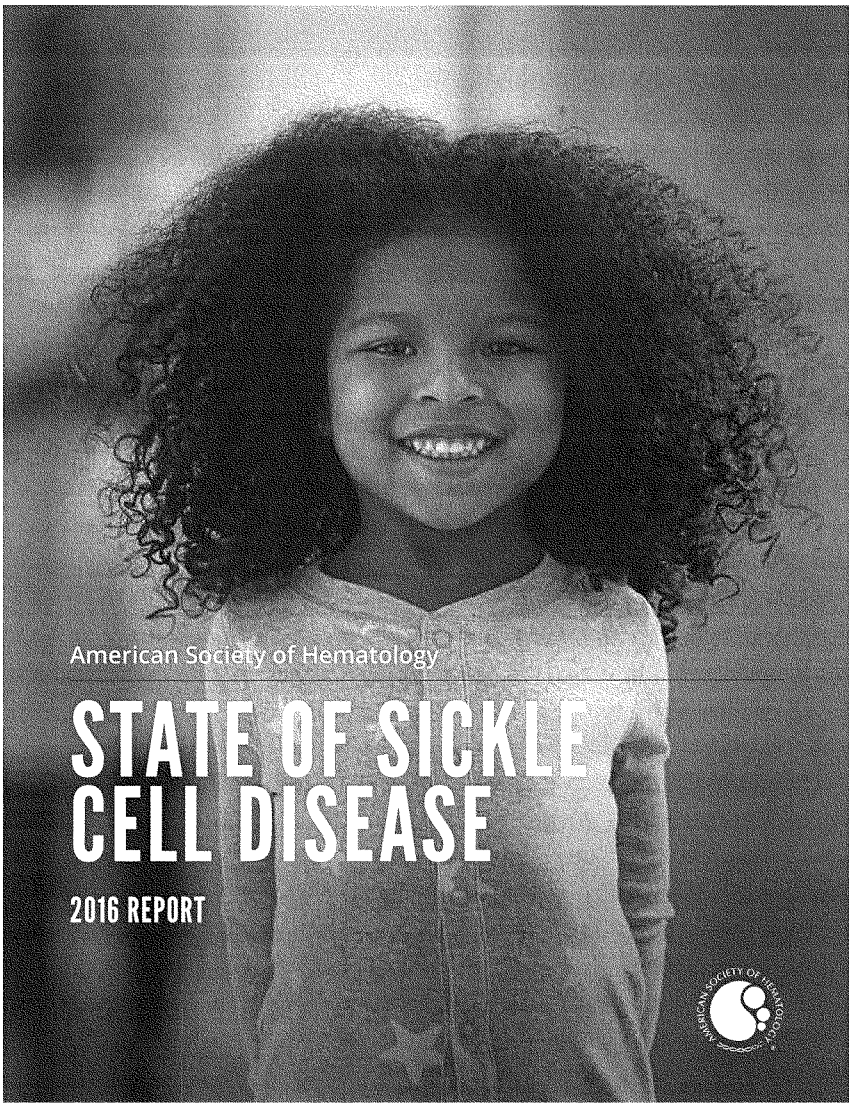
- Enhancement of the Centers for Disease Control and Prevention's SCD outreach and education programs on SCD and SCD trait for patients and providers.

- Improvement of access to high quality care via demonstration projects at CMS and development of best practices.

- Provision of incentives for drug development for SCD within the Food and Drug Administration (H.R. 1537, Advancing Hope Act of 2015).

The status quo is unacceptable, and we must improve the circumstances under which care for individuals with SCD is provided. Now is the perfect time – today, we have opportunities to improve overall treatment, care, and quality of life for millions of people, especially young children. We are calling for action in sickle cell.

Thank you again for the opportunity to submit testimony. Please contact Stephanie Kaplan, ASH Senior Manager of Government Relations and Public Health, at 202-776-0544 or *skaplan@hematology.org*, or Tracy Roades, ASH Legislative Advocacy Manager, at 202-776-0544 or *troades@hematology.org*, if you have any questions or need further information.


American Society of Hematology
STATE OF SICKLE CELL DISEASE
2016 REPORT

The organizations that endorse the *State of Sickle Cell Disease: 2016 Report* include:

American Academy of Pediatrics

American College of Emergency Physicians

APHA

STRONGER

aspho

APHL

The Emergency Department Sickle Cell Care Collaborative*

ENA

Emmaus Life Sciences Inc.

gbt

GLOBAL SICKLE CELL DISEASE NETWORK

GlycoMimetics, Inc.

IASCNAPA

MAST THERAPEUTICS, INC.

BE THE MATCH

National Medical Association

Pfizer

Prolong Pharmaceuticals

Sickle Cell Adult Provider Network

SMDA

Sickle Cell Warriors Inc

*The Emergency Department Sickle Cell Care Collaborative (EDSC3) endorses the State of Sickle Cell Disease: 2016 Report. The endorsement by EDSC3 does not represent or obligate in any way any of the other organizations that are represented by EDSC3 members.

Summary

Over the past century, great advances have been made in the understanding and treatment of sickle cell disease (SCD). This first "molecular disease," caused by a single gene mutation, has advanced the field of modern human molecular biology. Many important discoveries have been made, and some treatments developed. These discoveries have identified innumerable questions and opportunities to better understand and treat this complex disease. Yet, many basic scientific processes are still not fully understood, too few treatments have been developed, and most of the people who have SCD do not have access to these treatments which could improve the duration and quality of their lives.

With profound concern about the unmet scientific opportunities and lack of access to high-quality care, a group of SCD stakeholders has convened and is eager to find remedies. This document outlines their analysis and details a comprehensive plan to address these issues.

The plan encompasses four priority areas – Access to Care in the United States, Training and Professional Education, Research and Clinical Trials, and Global Issues Related to SCD – which will be the focus of our collective efforts toward ultimately advancing SCD care, early diagnosis, treatment, and research.

For a detailed list of strategies to address these issues visit ***scdcoalition.org***

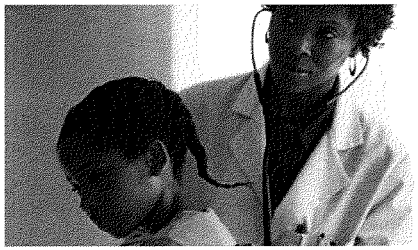
Introduction

Sickle cell disease (SCD), which causes a wide range of severe and even life-threatening consequences, is caused by a single misspelling in the DNA instructions for hemoglobin, a protein vital for carrying oxygen in the blood. As a result of this mutation, individuals with SCD experience lifelong complications including anemia, infections, stroke, tissue damage, organ failure, intense painful episodes, and premature death. These debilitating symptoms and the complex treatment needs of people living with SCD often limit their education, career opportunities, and quality of life.

The many complications of SCD can make every stage of life extremely challenging for individuals with the disease. For example, approximately 10 percent of children with SCD will have a symptomatic stroke, which can cause learning problems and lifelong disabilities. Pain is the most common clinical manifestation of SCD and results in tremendous suffering, prolonged absences from school, and difficulty maintaining full employment. Individuals with SCD experience chronic pain compounded with acute pain episodes. In a six-month study of adults with SCD, half of the respondents reported experiencing some level of pain for half of the days, while nearly a third noted having pain the majority of the time.[1] Acute pain episodes can occur multiple times per year and may result in long hospital stays, leading to a complete disruption of the person's life and his or her family's life.

SCD is the first known molecular disorder. However, advances in treatment have been limited compared with later discovered molecular diseases – such as cystic fibrosis – where multiple treatments have benefited patient populations. Over the past century, several important discoveries have been made in the understanding and treatment of SCD. Yet, many very basic scientific processes are not understood, and far too few treatments have been developed. In addition, most people with SCD do not have access to treatments that could improve the duration and quality of their lives.

We know that more can be done for people with SCD to provide improved access to high quality care. While a cure currently exists, it is available to only a small portion of the patient population and limited primarily to developed countries. The health outcomes and treatment disparities related to SCD make it a public health priority both in the United States and globally.



There is enormous opportunity to improve the state of SCD. There are actions we can take today to address unmet needs – both in the United States and around the world.

In the United States, it is estimated that:			
SCD affects approximately **100,000 people**	SCD occurs in about **1 in every 365** African-American births	SCD occurs in about **1 in every 16,300** Hispanic-American births	**About 1 in 13** African-American babies is born with sickle cell trait (SCT)

Source: Centers for Disease Control and Prevention, World Health Organization. Telfer et al. 2013. Lancet 583: 1427-31

There is enormous opportunity to improve the state of SCD. There are actions we can take today to address unmet needs – both in the United States and around the world.

We must address these disparities by increasing access to diagnostic and therapeutic interventions, and providing comprehensive care and appropriate support services to people living with SCD. Emerging therapies and promising new insights in the treatment of SCD represent a significant step toward improving outcomes and reducing the treatment burden for affected children and adults.

Alleviating the pain and suffering caused by this disease, as well as the socioeconomic costs, is entirely within our grasp. Although conquering SCD is doable, it requires a well-orchestrated plan and a coordinated effort from a range of partners including government agencies, patient advocacy organizations, health care providers, public health organizations, researchers, foundations, pharmaceutical and biotech companies, and other stakeholders.

In an effort to advance a forward-looking and comprehensive agenda that will make a significant difference, a group of SCD researchers, clinicians, individuals with the disease, and policymakers have come together to develop an organized approach to improving outcomes for people with SCD. From these meetings, four priority areas were identified to focus our collective efforts toward ultimately advancing SCD care, early diagnosis, treatment, and research.

Globally, it is estimated that:

SCD occurs in approximately **300,000 births** annually	**SCD is most prevalent in malaria endemic parts of the world,** primarily Africa, the Middle East, and South Asia	In many African countries, **10% to 40% of the population** carries the sickle-cell gene, resulting in estimated SCD prevalence of at least 2%

Source: Centers for Disease Control and Prevention; World Health Organization, Piel et al. 2013 Lancet 381: 142-51.

THE FOUR PRIORITIES

to improving outcomes for people with sickle cell disease

Access to Care in the U.S.

Training and Professional Education

Research and Clinical Trials

Global Issues

ACCESS TO CARE
IN THE UNITED STATES

CURRENT STATE

75%

More than 75 percent of adults with SCD with frequent pain crises fail to get hydroxyurea, which is the recommended treatment.[2]

31%

Despite universal newborn screening for SCD in the United States, one study found that long-term follow-up after diagnosis was not performed in nearly one-third (30.8%) of cases.[3]

SCD is also associated with high treatment costs. For an average person with SCD reaching age 45, total lifetime health care costs were estimated to be nearly $1 million, with annual costs ranging from over $10,000 for children to over $30,000 for adults.[4]

Hydroxyurea, the only FDA-approved drug for adults with SCD (often used off-label in children), improves the course of SCD and might lead to significant health care cost reductions. In a two year pediatric study, overall health care costs for children on hydroxyurea were $1.8 million, compared with $2.5 million for those who did not receive this treatment.[5] Unfortunately, despite the National Heart, Lung, and Blood Institute's (NHLBI) recommendations, hydroxyurea is not regularly prescribed and adherence to the therapy is poor.

Though the mortality rate for children with SCD decreased by 3 percent each year in a study from 1979 to 2005, the mortality rate for adults with SCD increased by 1 percent each year during the same period.[6]

People with SCD in the emergency department for pain experienced longer delays to administration of the initial analgesic compared with control patients, despite higher arrival pain scores and triage acuity levels.[7]

- The lack of available specialized providers (e.g., hematologists) plays a significant role in an over-reliance on emergency departments care for some individuals with SCD, especially adults.[8,9]

Many children with SCD do not receive the necessary services to prevent serious complications from the disease.

- Only one-third of children with SCD receive appropriate monitoring for stroke risk.
- 25 percent of children with SCD do not receive pneumococcal vaccination, which is recommended for all children younger than five years of age.[10]
- Children not receiving these necessary services are at greater risk of dying or suffering from cognitive defects as a result of stroke and invasive pneumococcal infections.

MILESTONES IN SCD

SCD has been known for hundreds of years by the people of Africa by different tribal names, including nwiiwii, nuiduidui, ahotuto, and chwechweechwe

1846 First formal case of sickle cell disease reported.

1910 Herrick provides the first formal description of sickle cell anemia, reporting a "pear-shaped and elongated" blood cell from a Chicago dental student.

1922 Mason observes fourth reported case of sickled blood and names the disease "sickle cell anemia."

1927 Hahn & Gillespie describe the pathophysiology of the disease noting an association between the sickling of red blood cells and low oxygen conditions.

1933 In Memphis, Tenn., 2,500 African Americans were tested to distinguish between sickle cell trait and sickle cell disease.

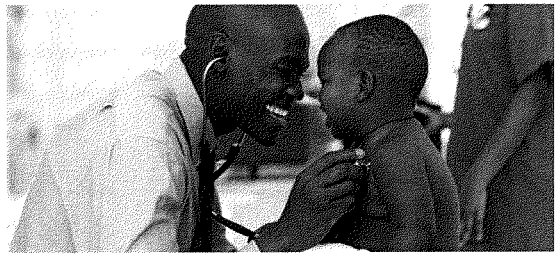
1934 The painful sickle "crises" are suggested to be the result of blockages of small blood vessels.

Today, individuals with SCD are living longer, but the system of care needs to change to ensure a better quality of life.

In the United States, access to appropriate care is influenced by a number of factors, including health insurance, the availability of health care providers, and provider experience treating people with SCD. Individuals with SCD reported experiencing poor communication between primary-care physicians and hematologists, providers with limited knowledge and experience with pain management, difficulty in scheduling urgent appointments, and lack of follow-up after hospitalization.[11] People with SCD and their families have expressed a need for better information, shared decision-making, and strengthened communication with those providing their medical care.

Other factors, such as geography, economic status, and co-existing conditions have an impact on access to care. Those living in or around metropolitan areas are more likely to have access to knowledgeable providers than those living in rural communities; however, people with access to SCD treatment centers may have difficulty affording frequent and expensive treatments. Additionally, people with conditions related to SCD such as strokes or cognitive impairment struggle to navigate the health care system without assistance.

The transition from pediatric to adult care can be particularly challenging as the focus of care differs. In the United States, children with SCD are more likely than adults to receive care in academic medical centers, which have SCD-specific expertise. Most of these children are seen by primary-care providers – pediatricians, family medicine doctors, and nurse practitioners – and many have access to pediatric hematologists.[12] In contrast, adults with SCD are more likely to receive care in community hospitals, where SCD-specific expertise is much less common.[13] As more people with SCD are living into adulthood, disease management needs to shift from acute care of complications to a chronic care model that focuses on prevention of crises, and intervention and relief for common complications. A coordinated health care delivery system for adults with SCD is a first step to improve health outcomes.[14]



GOALS FOR THE FUTURE

Ensure implementation of existing standard-of-care guidelines and best practices in disease management as well as develop new resources to help all physicians make the best decisions in treating people with SCD

Implement coordinated care models that incorporate community health workers, ensuring more equal quality of care regardless of where an individual with SCD receives care.

Improve the pediatric-to-adult care transition by ensuring there are qualified physicians in both pediatric and adult care, through creation of training incentives and retention of providers who treat SCD.

Improve access to evidence-based care through innovative health care delivery models and incorporate SCD care into the health care system and delivery.

Read more about the goals for the future at ***scdcoalition.org***

1940 Sherman confirms that sickling of red blood cells in the absence of oxygen is caused by a molecular change in hemoglobin structure.

1948 In *Blood*, Watson suggests a link between HbF levels and the presence of SCD symptoms.

1948 Pauling & Itano showed with protein electrophoresis that hemoglobin from people with SCD is abnormal. SCD is the first disorder described to be the result of a protein abnormality.

1949 Two teams – Neel and Beet – independently publish on SCD inheritance, stating individuals with sickle trait were heterozygous for the gene, whereas those with SCD were homozygous.

1951 Pauling discovers the molecular nature of SCD by showing that the red, oxygen-carrying protein called "hemoglobin" has a different chemical structure in persons with SCD, making SCD the first "molecular disease" discovered.

1953 A diagnostic tool is developed to identify SCD.

TRAINING AND
PROFESSIONAL EDUCATION

CURRENT STATE

In a national survey, only 20.4 percent of family physicians reported that they felt comfortable treating people with SCD.[13]

69.4 percent of family physicians reported that clinical decision support tools would be useful for helping to guide their treatment decisions for individuals with SCD.[16]

72.6 percent of family physicians believed that more education and clinical support tools would help avoid complications in managing the disease.[17]

Currently, there are not enough health care providers with the comprehensive knowledge and expertise to care for people with SCD. This knowledge gap not only exists within specialty (e.g., hematologists) and primary care, but also among other provider groups, such as clinicians who provide hospital-based and outpatient care. This contributes to an enormous unmet medical need, especially for adults with the disease who need coordinated chronic care and ongoing pain management. The unpredictable and often persistent nature of the pain and other complications associated with SCD poses a difficult challenge for providers, especially for those unfamiliar with treating people with this disease.

Although comprehensive programs exist for some children with SCD, many adults do not have access to preventive and comprehensive care. Their health care providers may be inexperienced in treating SCD and the coordination of their care may be insufficient. And while there has been increased access to health insurance in the last few years, the number of providers with SCD expertise has not increased. Consequently, health outcomes for people with the disease have not improved.[18]

The issues with access often relate to training and comfort with providing care. The number of hematologists trained and willing to care for children with SCD has increased, but still fewer are providing care for the more complex adults with SCD.[19] In fact, a survey of prominent pediatric SCD centers reported that the largest barrier to transition and continuity of care was the ability to identify an adult provider – specifically a hematologist – with the training, expertise, and comfort in treating this population.[20] As a result much of the care of adults with SCD takes place in emergency departments or other non-specialty settings.[8]

Some people with SCD rely exclusively on the hospital or emergency department for care, due to a lack of SCD providers, which can greatly disrupt the continuity of care that they receive. In fact, nearly three-quarters of hospitalization for SCD originates in the emergency department.[19] Frequency of emergency department visits is substantially higher for adults with SCD than for children. This is potentially problematic because people with SCD often warrant a comprehensive assessment related to their other medical complications (i.e., comorbidities). However, the emergency care system is not designed to care for the chronic problems of an individual with SCD. Relying on uncoordinated episodic care also leads to increased health care costs and the potential for inadequate or inappropriate treatment.[9]

Primary-care providers, such as family physicians, often provide care to individuals with SCD; however, they report feeling that they do not have adequate backgrounds in SCD management due to their lack of experience treating these individuals. Factors currently associated with primary-care physicians' comfort level in treating SCD is whether or not they typically see people with this disease, or from their previous training experiences.[22] Greater availability of clinical decision support tools

1954 It is discovered that sickle cell trait protects against malaria, explaining the prevalence of the sickle gene in regions where malaria is worldwide.

1955 A blood test is developed to identify abnormal hemoglobin, a method still used today to confirm SCD diagnosis.

1958 Ingram and Hunt sequence sickle hemoglobin showing that SCD occurs due to a change in a single amino acid, replacing a glutamic acid with a valine, in hemoglobin.

1963 Hemoglobin 3-D protein structure deciphered using x-ray crystallography.

1971 The National Association for Sickle Cell Disease is founded, now known as the Sickle Cell Disease Association of America (SCDAA).

1972 The *National Sickle Cell Anemia Control Act* is signed into law. It provides for establishment of voluntary SCD screening, education, research, and training.

Many adults are forced to rely on urgent care, leading to increased health care costs and the potential for inadequate or inappropriate treatment.

for health care providers could help those making treatment decisions and provide high-quality care to individuals with SCD.

Despite the NHLBI recommendations on the use of hydroxyurea in the management of SCD, providers are often reluctant to prescribe this therapy or may not be knowledgeable about its use in SCD.[23] As a result, patients are often not well educated on hydroxyurea, leading to misinformation and poor adherence.[24]

Increasing the focus of training programs on pathology, treatment, and research related to SCD will help expand the number of specialists who are qualified and willing to care for those with this disease. Increased emphasis on training providers who manage individuals with SCD, especially adults, and further implementation of coordinated care models may result in improved standards of care and greater availability of providers with SCD expertise.

GOALS FOR THE FUTURE

Develop an actionable plan to educate health care providers about best practices in caring for those with SCD, including hematologists, primary-care providers, hospitalists, and emergency department physicians.

Augment pain management expertise through use of best practices and a thorough assessment of reversible conditions known to precipitate pain crises, such as dehydration and infection.

Increase the number of providers who are able to care for those with SCD through training and certificate programs.

Develop clinical support tools to ensure quality of care for people with SCD.

Cultivate an interest in SCD care among clinicians in early medical training.

Read more about the goals for the future at ***scdcoalition.org***

1972 Researchers find a milder SCD variant that is associated with increased levels of fetal hemoglobin, suggesting that an increased fetal hemoglobin level could serve as a treatment target.

1973 The National Sickle Cell Disease Program is established and National Heart and the Lung Institute begins funding comprehensive sickle cell centers and establishes a sickle cell branch at NIH.

1975 New York becomes the first State to require newborn screening for SCD.

1977 Gilbert and Sanger's DNA sequencing method allows the mapping of the sickle cell gene.

1978 Nagel maps the human beta and delta hemoglobin genes, pinpointing the DNA mutation resulting in SCD.

Prenatal DNA method of SCD diagnosis reported.

RESEARCH AND
CLINICAL TRIALS

CURRENT STATE

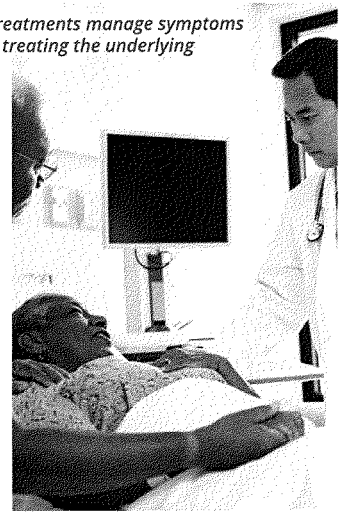
90%

SCD was once associated with early childhood mortality, but today in the United States more than 90 percent of people with SCD live into adulthood, which poses new issues and challenges.[25]

There is only one medication, hydroxyurea, currently approved by the FDA to treat adults with SCD.[26]

In the United States, there are currently 36 clinical trials underway related to SCD to evaluate novel agents and approaches. This includes several trials involving bone marrow transplantation and gene therapy, which are potentially curative.

The majority of available treatments manage symptoms of the disease, rather than treating the underlying cause of sickling.



Although the molecular basis of SCD was established more than half a century ago, it has been challenging to translate this research into the development of novel targeted therapies. While new approaches in managing this disease have improved diagnosis and supportive care over the last few decades, people with SCD still have severe complications to overcome.

There is no widely available cure for SCD and few effective treatments. Treatment for those with SCD focuses on disease and pain management, treatment of complications, and acute care during sickling crises, which occurs when red blood cells become rigid and sticky and block the flow through small blood vessels. Only one therapy, hydroxyurea, is approved by the U.S. Food and Drug Administration (FDA), but research shows that it is underused by health care providers and not taken consistently by individuals with SCD.

1910 The binding of sickled red blood cells inside blood vessels is shown to block blood flow, suggesting that the extent of stickiness may be a determinant of disease severity.

1984 Bone Marrow Transplant in child with SCD produces the first reported cure of the disease.

1986 Penicillin is recognized as an effective preventive measure in reducing infection and death in children with SCD.

1987 NIH panel recommends screening all newborns in the United States for SCD and giving penicillin prophylaxis to all affected infants by 3 months of age.

1995 Anticancer drug hydroxyurea shown to reduce the frequent, painful complications of SCD.

1996 A new method allowing the use of maternal blood samples for prenatal SCD diagnosis is developed.

Regular blood transfusions are sometimes used as a preventive measure; however, they can lead to abnormally high levels of iron in the blood, which can cause long-term organ damage and reactions due to a mismatch between the donors and recipients.

Currently there are a number of clinical trials underway; however, the majority are single agent trials sponsored by the pharmaceutical industry and it will be years before they will translate into widely available and affordable therapies. Now more than ever there is a need to develop a clinical trial network that could increase the number of patients recruited and allow for assessment of combination therapies. Furthermore, there is great need for increased pain research and exploration of additional curative therapies. While bone marrow and stem cell transplantation are potentially curative, their use is limited due to high cost, the difficulty of locating matched donors, and the high level of risk associated with the procedure. While still in an exploratory stage, gene therapy and genome editing hold the promise of a future approach for curing SCD and other hemoglobin disorders.

Today, there is an extraordinary opportunity to link research and care more closely. One way to achieve this is through a clinical research network. Some of the most pressing research questions that link to patient care include how to improve dosing and better anticipate response to treatments like hydroxyurea, investigating potential combinations of treatments, better defining the indications for transfusions in SCD, and improving the safety of transfusions by adopting accurate, state-of-the-art blood matching protocols. While clinical trial research helps to move the needle on improving SCD care, often these trials stagnate due to insufficient patient enrollment.[27,28] Therefore, it is imperative that individuals have a more active role in the clinical research process.

GOALS FOR THE FUTURE

Identify predictors of the severity of the disease, including optimizing dosing and treatment response predictors for hydroxyurea, biomarkers for SCD crisis and prognosis, and SCD diagnostic methods.

Optimize the use of existing therapies, better defining the indications for red blood cell transfusions, and identifying principles for accurate blood matching and developing longitudinal studies to determine long-term effectiveness of transfusions and hydroxyurea.

Develop novel therapies, including combination therapies, new drug-delivery modes, and new agents that can be used in combination with hydroxyurea.

Develop clinical trial networks to increase enrollment in clinical trials and share the data with interested stakeholders.

Strengthen curative therapies, such as bone marrow and stem cell transplantation, and support funding for research in SCD gene therapy and genome editing methods.

Enhance pain research for improved outcomes such as pain, fatigue, and infertility, and create a validated SCD-specific functional assessment tool for pain.

Enhance the participation of individuals with SCD in setting the research agenda and increase patient participation in clinical research.

Read more about the goals for the future at ***scdcoalition.org***

For the ASH Research Priorities for SCD and Sickle Cell Trait, click here.

1996 A study finds that bone marrow transplantation in children with SCD can be curative if their sibling donors share a specific protein.

1997 Periodic blood transfusions in children with SCD are shown to reduce the risk of stroke by 90 percent.

1998 The FDA approves hydroxyurea as the first drug proven to be effective in the treatement of adults with SCD.

2002 The Health Resources and Services Administration begins the SCD Newborn Screening Program.

2002 The Sickle Cell Adult Provider Network (SCAPN) is founded to establish and support an interactive network for health care providers and investigators who serve adults with SCD.

2003 The *Sickle Cell Treatment Act* of 2003 is signed into law by President George W. Bush.

GLOBAL ISSUES

CURRENT STATE

Prevalence of sickle cell trait varies greatly between different regions but reaches levels as high as 40 percent in some areas of sub-Saharan Africa, eastern Saudi Arabia, and central India.[29,30]

In resource-poor countries more than 90 percent of children with SCD do not survive to adulthood.[31]

Approximately 1,000 children in Africa are born with SCD every day and more than half will die before they reach five.[32]

SCD has a high prevalence in India, especially in the central and western regions. Approximately 20 percent of children with SCD die by the age of two.[32]

Organizations such as the World Health Organization (WHO) and United Nations (UN) have recognized SCD as a global health issue. In 2006, the World Health Assembly passed a resolution recognizing SCD as a public health priority and called on countries to tackle the disease. This resolution was also adopted by the United Nations in 2009.

The global picture of SCD is similar to that of the disease in the United States before 1970. In countries with poor public health systems and high levels of poverty, SCD remains a major killer of infants and children, similar to other diseases like malaria and HIV/AIDS. In resource-poor countries, 90 percent of children with SCD do not survive to adulthood. And the problem is growing; by 2050 the number of people with SCD is expected to increase by about 30 percent globally.[34]

Some middle-income countries are making advancements in both early diagnosis and management of SCD. Brazil, for example, has shown remarkable progress over the past two decades with newborn screening programs being offered across the country.[35]

2004 U.S. Postal Service issues an SCD awareness postage stamp.

2005 The African Union approves the decision to submit a report to the United Nations about sickle cell anemia.

2006 The NHLBI launches the Sickle Cell Disease Clinical Research Network.

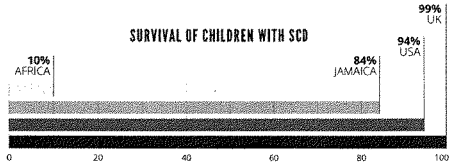
2007 ASH hosts workshop on SCD Research.

2007 The Global Congress on SCD was established by the WHO and the Thalassaemia International Federation with the aim of uniting SCD groups across the globe on SCD efforts.

2008 UN passes a resolution recognizing sickle cell anemia as a public health problem, declaring June 19 as World Sickle Cell Disease Day and urging increased awareness and improved care.

A 10-year study in Rio de Janeiro showed that diagnosis through newborn screening programs and treatment was associated with improved survival and quality of life of Brazilian children with SCD. Moreover, in Brazil, health care maintenance for SCD is seen as an essential component of primary care, and the government supplies hydroxyurea free of charge. This type of program has resulted in increased awareness and education for SCD, although there have been only modest improvements in mortality rates. Jamaica represents another example of a middle-income country with SCD screening and treatment approaches that have resulted in remarkable improvements in the median life expectancy.[36]

As newborn screening and treatment efforts are implemented around the world, higher survival rates will increase the need for improved treatment options for adults with SCD and ongoing care. In developing our goals for the future, one of our greatest priorities is designing, testing, and implementing sustainable care and pain management approaches for countries with limited resources.



Source: The National Institutes of Health, www.fic.nih.gov/News/GlobalHealthMatters/november-december-2014/Pages/sickle-cell-disease.aspx

GOALS FOR THE FUTURE

Establish and/or expand newborn screening and early intervention programs.

Increase education of governments and philanthropic groups about the importance of screening and caring for individuals with SCD in heavily burdened countries.

Improve global access to care providers, such as increasing the number of health care providers treating SCD and improving training for health care professionals.

Develop standard-of-care guidelines that apply to specific, low-resource areas globally including SCD adult care and enhanced use of community-based organizations.

Develop a structured approach to addressing pain in low-resource settings.

Read more about the goals for the future at ***scdcoalition.org***

2008 NHLBI convenes workshop of SCD stakeholders to discuss key public outreach issues.

2009 NHLBI study finds a modified blood adult stem cell transplant regimen reverses SCD in 90 percent of adults severely affected by disease.

2011 ASH hosts workshop on sickle cell trait (SCT) and athletic participation.

2012 ASH releases statement on screening for SCT and athletic participation.

2014 NHLBI releases guidelines for managing SCD, stressing the use of the drug hydroxyurea and transfusions for many with the genetic disorder.

2014 ASH releases a list of priorities for SCD and sickle cell trait that seeks to address remaining questions and specific research topics that could move the field forward.

Conclusion

Why is now the right time to focus on this disease?

SCD is a chronic disease that has been neglected for far too long. Those affected by this disease are among the most vulnerable and underserved, and the disease has a profound impact on their lives.

Currently, the only approved drug for adults with SCD – hydroxyurea – reduces the severity and frequency of painful episodes and is used for stroke prevention, but may not prevent acute complications or reverse organ damage that can result in early death or other health problems which affect their quality of life (i.e., comorbidities). There is a need for the development of new treatments and a widely available and affordable cure for this disease. Existing treatments and cures, including bone marrow and stem cell transplantation, are underutilized and do not reach the majority of individuals – especially adults with SCD who could benefit from them. Increased strategies to educate providers and people living with SCD are also needed to address issues of fragmented care. There is a tremendous disparity in SCD outcomes between individuals in low- and middle-income countries compared to high-income countries.

The status quo is unacceptable. It is imperative that we vastly improve the circumstances under which care is provided. There is opportunity to address these disparities and alter the course of SCD, by improving overall treatment, care, and quality of life for millions of people, especially young children. A number of organizations are coming together to address this issue and each has important assets to help advance a comprehensive SCD action plan. To make real change for those affected by SCD, we need participation from many different stakeholders – including government agencies, patient advocacy organizations, health care providers, researchers, foundations, and the private sector. Those with SCD are waiting, and their well-being depends on our help. Their future is in our hands.

The time is now to change the course of the disease.

Will you join us? For more information visit ***scdcoalition.org***

2015 → ASH hosts the ASH SCD Summit: A Call to Action as a first step to a broader initiative, to identify the highest priority actions needed to improve outcomes for individuals with SCD in the U.S. and globally.

2015 → 2015 Tennessee legislature passed Senate Bill 1074, which authorizes TennCare (Medicaid program) to provide medical assistance for SCD management services and for public education campaign activities related to the disease.

ASH released SCT Educational Toolkit with CDC and SCDAA

2018 → Sickle Cell Disease Coalition is established.

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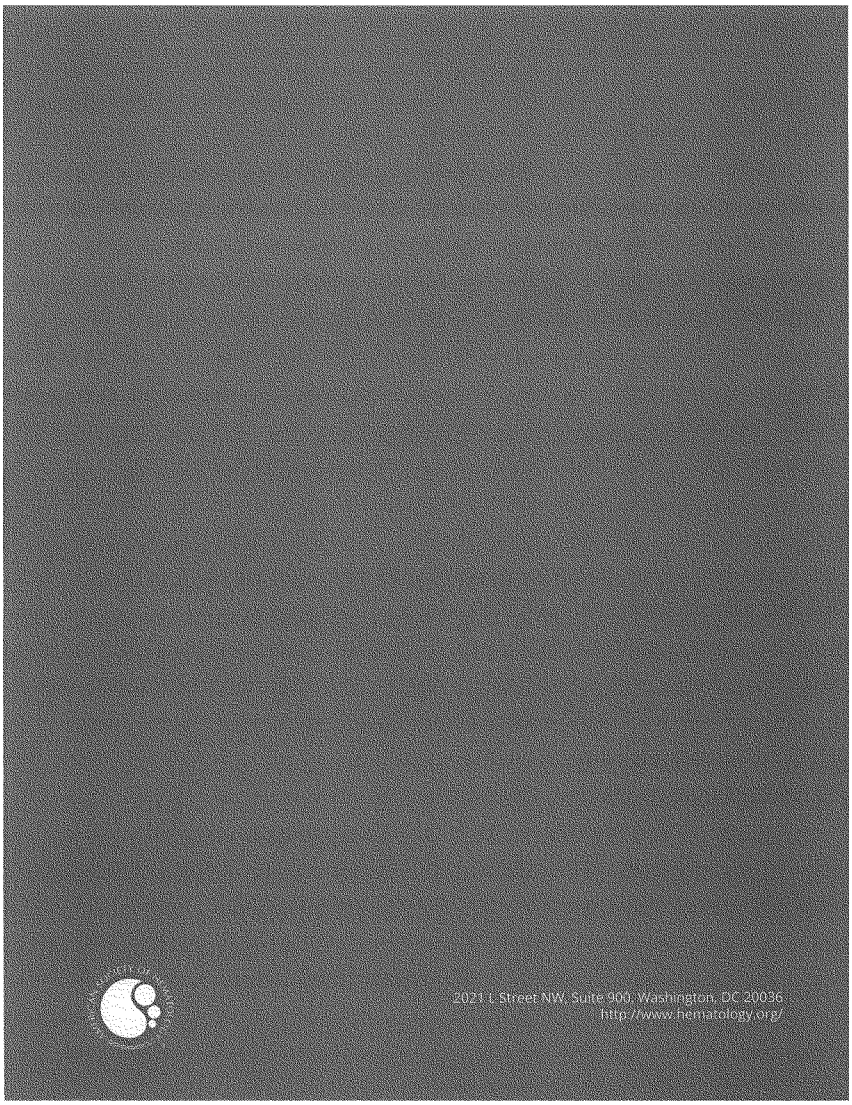
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Current Management of Sickle Cell Anemia

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Abstract

Go to:

Proper management of sickle cell anemia (SCA) begins with establishing the correct diagnosis early in life, ideally during the newborn period. The identification of affected infants by neonatal screening programs allows early initiation of prophylactic penicillin and pneumococcal immunizations, which help prevent overwhelming sepsis. Ongoing education of families promotes the early recognition of disease-released complications, which allows prompt and appropriate medical evaluation and therapeutic intervention. Periodic evaluation by trained specialists helps provide comprehensive care, including transcranial Doppler examinations to identify children at risk for primary stroke, plus assessments for other parenchymal organ damage as patients become teens and adults. Treatment approaches that previously highlighted acute vaso-occlusive events are now evolving to the concept of preventive therapy. Liberalized use of blood transfusions and early consideration of hydroxyurea treatment represent a new treatment paradigm for SCA management.

The natural history of untreated sickle cell anemia (SCA) is well described and documents serious morbidity and early mortality (Powars 1975; Platt et al. 1994; Serjeant 1995; Powars et al. 2005). Hemolytic anemia, acute vaso-occlusive events (VOEs), and chronic end-organ damage begin early in life, and complications accumulate throughout childhood. Without early identification or specific interventions, many patients with SCA have poor quality of life, and most die as young adults of SCA-related complications (Diggs 1973; Rogers et al. 1978).

Fortunately great strides have occurred over the past 40 years, and better management strategies have altered this previously bleak outlook. Despite the complexity and multifactorial pathophysiology of vaso-occlusion (Ware 2010a), relatively straightforward measures have greatly improved outcomes for children with SCA: (1) early identification by neonatal screening programs; (2) education of parents and patients about medical complications and early recognition; (3) preventive measures with prophylactic penicillin and pneumococcal immunizations; (4) aggressive treatment of acute VOEs including hydration, analgesics, antibiotics, and transfusions; (5) screening programs for early signs of organ damage, especially primary stroke risk using transcranial Doppler (TCD) examinations; and (6) therapeutic intervention with transfusions, hydroxyurea, or stem cell transplantation. For children receiving medical care at comprehensive care programs, 95%–99% survival rates into adulthood are documented (Telfer et al. 2007; Quinn et al. 2010). For adults with SCA, screening programs and anticipatory guidance are less standardized but still critical, and the benefits of preventive therapy using hydroxyurea are even more compelling.

Here we will focus primarily on the management of SCA (HbSS or HbS/β^0-thalassemia). We emphasize and summarize general principles of care and management, rather than discussing details of pathophysiology or mechanisms of disease. The management of specific examples of acute VOEs will be highlighted.

EARLY IDENTIFICATION

Go to:

Perhaps the most critical aspect of optimizing SCA management is early identification of affected patients, before the onset of signs and symptoms of disease. Without early diagnosis and intervention, SCA often acts as a swift and invisible killer, with many infants dying suddenly of bacterial sepsis or acute splenic sequestration crisis (ASSC) within the first few years of life (Pearson et al. 1969, 1977; Rogers et al. 1978; Powars et al. 1981). Sometimes fatal complications occur even before families or medical providers are aware the infants have SCA.

With the recognition that infants with SCA have greatly increased risk of bacterial sepsis, the landmark multicenter double-blinded placebo-controlled PROPS trial proved that penicillin prophylaxis significantly lowered the risk of bacteremia and death (Gaston et al. 1986). This simple intervention provided the justification needed for newborn screening of SCA, to identify affected infants soon after birth and to allow lifesaving prophylactic antibiotic therapy. Although a 1987 NIH Consensus Conference recommended newborn screening for SCA, universal screening was not accomplished in all U.S. states and territories until 2006.

Newborn screening programs in the United States, Jamaica, and Europe have documented the utility of early identification of SCA, with a marked reduction in morbidity and mortality, especially in the first 5 years of life (Rogers et al. 1978; Vichinsky et al. 1988; Almeida et al. 2001; Bardakdjian-Michau et al. 2001). Figure 1 illustrates that early identification of SCA through neonatal screening programs has contributed to the improved survival rates (Quinn et al. 2010).

Figure 1.
Survival curves of infants with SCA in the United States and Jamaica, by era. This research was originally published in *Blood*. (From Quinn et al. 2010; reprinted, with permission, © American Society of Hematology.)

Testing of newborns in the United States for SCA began with targeted screening, which involves selecting at-risk populations to screen, such as babies whose parents are African-American. Such an approach is problematic in several ways, and has evolved now to universal screening for all newborns. In contrast, most European countries still perform targeted screening for infants most likely to be affected, such as those of African ancestry. Although potentially cost-effective, targeted screening almost certainly misses some babies with SCA, and presents difficulties related to both equity and logistics (Grosse et al. 2005). Despite the high burden of disease, newborn screening has yet to be implemented systematically in Africa, although pilot studies document a high incidence of trait and disease.

Newborn screening for SCA requires a small dried blood spot (DBS) for analysis, collected from cord blood or the infant's heel/toe. Collection technique is important; DBS specimens have variable quality by the amount and distribution of blood on the filter paper. Specimens are most easily collected in the neonatal period for babies born in the hospital, or during initial immunization visits for babies born at home. Testing in the United States is most commonly performed by hemoglobin electrophoresis using isoelectric focusing (IEF), which easily distinguishes abnormal sickle hemoglobin (Hemoglobin S, HbS) from normal hemoglobin (HbA) and fetal hemoglobin (HbF), as illustrated in Figure 2. High-performance liquid chromatography (HPLC), capillary electrophoresis (CE) techniques, and even DNA-based laboratory diagnosis also can be used for accurate diagnosis. When possible, parental studies should also be performed to confirm the diagnosis of SCA.

Figure 2.
Isoelectric focusing (IEF) electrophoresis technique for identification of SCA. Blood specimens from AA, AS, and SS patient controls are shown on

the *left*, along with a manufactured Hb AFSC control in the *center*. Newborn samples, typically obtained from ...

EDUCATION

Go to:

Parental and family sickle cell education should begin once the diagnosis is made and continue throughout childhood. Given variable parental education and literacy, education should be provided in written and spoken form, and should be repeated with each visit to ensure information is comprehended. When possible, both parents should receive education, plus extended family members and other caregivers, to become knowledgeable about SCA.

Education in the early newborn period should focus on the basics of SCA, including its genetics and inheritance, need for penicillin prophylaxis, and benefits of protein-conjugated pneumococcal immunizations. At each visit these key points should be repeated to parents and caregivers. The importance of regular medical care should be emphasized, especially the need for prompt medical evaluation for fever. Antipyretics should never be given for fever at home, because this treatment can mask a serious infection. Education for young patients should also include signs and symptoms of ASSC: pallor, fussiness or irritability, and tender splenomegaly. Teaching parents to palpate their baby's spleen regularly, ideally several times a day during routine diaper changes, allows early diagnosis of ASSC and potentially prompt and lifesaving intervention.

As the child grows, education should focus more on recognition and early medical intervention for acute vaso-occlusive complications such as dactylitis and other painful events, respiratory distress, acute chest syndrome, and stroke. Parents should learn to manage mild pain at home with oral hydration and analgesia. At an early age, families should be introduced to possible treatment options including hydroxyurea and transfusions, and even stem cell transplantation, if available. As affected children grow up and enter adolescence, it is critical to provide ongoing education about SCA and its complications, to provide young patients with the skills necessary to understand and advocate for their own medical care. Such self-awareness and investment in their medical care becomes critically important on transition from pediatric to adult hematology care. Unfortunately, evidence suggests increased morbidity and mortality in late adolescence and early adulthood following this transition of care (Brousseau et al. 2010; Quinn et al. 2010).

PREVENTIVE MEASURES

Go to:

As the result of vascular congestion, intraparenchymal sickling, and hypoxic injury to the spleen, infants with SCA have early loss of filtrative splenic function and are susceptible to acute life-threatening infections, particularly from encapsulated bacteria such as *Streptococcus pneumoniae* and *Haemophilus influenzae* type b (Winkelstein and Drachman 1968; Pearson et al. 1969; Pearson 1977). This risk remains increased throughout life, but is most significantly increased in the first 5 years, when bacteremia incidence is the highest (Overturf et al. 1977; Powars et al. 1981; Gill et al. 1995).

The PROPS trial showed that prophylactic oral penicillin reduced the frequency of bacterial infection by 84% among young children (age 3–36 mo) with SCA (Gaston et al. 1986). However, the follow-up PROPS 2 study was unable to show benefit from penicillin after age 5 yr, primarily owing to their lower incidence of bacteremia (Falletta et al. 1995). After introduction of protein-conjugated pneumococcal vaccines, the incidence of invasive pneumococcal disease decreased by 93.4% among young children with SCA (Halasa et al. 2007). In Africa the dangers of pneumococcal sepsis for children with SCA have been questioned, but recent data confirm its prevalence and lethality (Williams et al. 2009).

Based on overwhelming evidence, early pneumococcal prophylaxis is recommended for all infants with SCA. Penicillin 125 mg by mouth twice daily should begin by 3–4 mo of age, as a liquid formulation or crushed tablet. The penicillin dose should be increased to 250 mg by mouth twice a day as the child grows, typically at 3 yr of age. Some international programs recommend monthly IM penicillin to help ensure compliance. Oral erythromycin can be used as a substitute if penicillin allergy or rash develops, but this is uncommon.

Pneumococcal immunization should begin with locally available pneumococcal conjugate vaccines (7, 10, or 13 valency) and supplemented at age 2 and 5–7 yr with the 23-valent pneumococcal polysaccharide vaccine (Pneumovax). Additional recommended vaccinations include the *H. influenzae* type b series, meningococcal conjugate vaccine (Menactra), and yearly influenza. When locally feasible, the published immunization schedule for high-risk children as recommended by the American Academy of Pediatrics (www2.aap.org/immunization/IZSchedule.html) should be followed.

Penicillin prophylaxis should continue through age 5, when the risk of invasive bacterial disease is lower. Once the immunization series is up to date, including the Pneumovax booster, children with SCA may discontinue penicillin prophylaxis. However, penicillin should be continued indefinitely if a child has had culture-positive sepsis or a surgical splenectomy.

The dramatically increased risk of overwhelming and rapidly fatal infection among young patients with SCA must be understood by all caregivers and medical providers. Fever >38.5°C is a medical emergency requiring prompt medical evaluation, including physical examination with vital signs and splenic palpation, blood culture, complete blood count, reticulocytes, urinalysis, and chest X-ray if clinically warranted. Type and crossmatch should be obtained if there is extreme pallor, splenomegaly, clinical instability, or acute respiratory or neurologic symptoms. After obtaining the blood culture, broad-spectrum antibiotics (e.g., ceftriaxone) should be administered intravenously. Addition of another broad-spectrum antibiotic (e.g., vancomycin) should be considered if the child appears toxic, has high fever, or suspicion of central nervous system (CNS) infection.

Hospitalization is recommended if clinical or laboratory indicators suggest sepsis, such as hemodynamic compromise including hypotension, child <1 yr of age, prior history of sepsis, temperature >40°C, WBC >30 × 10^9/L, or <3 × 10^9/L, concurrent symptoms such as pain or acute anemia, or if close follow-up is not reliable (Lane et al. 2001).

ACUTE VASO-OCCLUSION

Go to:

Pain Events

The sudden onset of pain that occurs frequently in patients with SCA results from acute intravascular sickling, so is often referred to as painful VOE or vaso-occlusive "crisis" (VOC). Although many providers and patients use the simple phrase "pain crisis," VOE is preferable because it broadly defines the process and avoids stigma about pain perception and management.

The VOE results from erythrocyte sickling, microvascular occlusion, and tissue ischemia/reperfusion, and is a hallmark clinical feature of SCA. Pain is the most common cause of acute morbidity of SCA, and is associated with severity of disease and early mortality among young adults (Platt et al. 1991). Pain often accompanies acute chest syndrome (ACS), a serious and potentially life-threatening complication of SCA, in 72% of cases (Platt et al. 1994; Vichinsky et al. 2000). This association usually follows sternal or truncal pain, which leads to splinting and poor inspiratory effort, and lack of active and complete inspiration following opioid-induced sedation. The frequency and severity of pain in SCA is more than just episodic and acute, however; pain in SCA is often chronic, underrecognized and underreported, and therefore undertreated (Solomon 2008). Pain diaries of 232 adult patients showed that SCA pain is common and often chronic; pain was present on 54.5% of days and 29.3% of patients reported pain on >95% of days (Smith et al. 2008).

The pathophysiology of vaso-occlusive pain is multifactorial and complex, and includes various blood cells including reticulocytes and neutrophils, plus plasma factors and vascular endothelium (Ware 2010a). Several factors have been identified as triggers of painful VOE, with individual patients often recognizing their own specific triggers. The most commonly described triggers include cold temperatures and especially cold water, as well as dehydration, overexertion, and menses (Redwood et al. 1976; Resar and Oski 1991; Yoong and Tuck 2002).

A combination approach of nonpharmacologic and pharmacologic agents should be used for acute management of vaso-occlusive painful events. Nonpharmacologic interventions with shown effectiveness include oral hydration, heat, massage, and various cognitive-behavioral and self-relaxation techniques (Rees et al. 2003; Dampier et al. 2004). Cold packs can increase local sickling and may exacerbate pain, so should be avoided. Pharmacologic interventions should begin at home with nonopioid analgesics, including acetaminophen and nonsteroidal anti-inflammatory drugs (NSAIDs); ibuprofen (10 mg/kg or 800 mg for adults >40 kg every 6–8 h) is an effective oral agent given its potent analgesic and anti-inflammatory properties. Corticosteroids may reduce the duration of painful VOE but on discontinuation, are associated with an increased frequency of rebound painful episodes requiring readmission (Griffin et al. 1994), and so are relatively contraindicated for routine pain management. If pain is not controlled with increased hydration, oral analgesia, and other conservative measures, opioids should be used. Oral narcotic therapy such as codeine and its derivatives can often be used effectively at home, in selected settings and patients.

In the event of severe vaso-occlusive pain requiring formal medical evaluation, aggressive pain management should be implemented promptly with intravenous hydration and opioid (morphine or hydromorphone) analgesia, and adjuvant intravenous NSAID therapy. Historically, the most painful VOEs have been evaluated and treated in the local emergency room, but given the relative lack of sickle cell knowledge and familiarity among some healthcare providers, such evaluations have delays in initiating appropriate analgesia, poor pain control, and a high rate of hospital admission. A sickle cell day hospital approach, which features staffing by experienced sickle cell providers, is increasingly used and results in improved pain management, better patient satisfaction, and decreased rates of hospitalization for both adults and children with SCA (Benjamin et al. 2000; Raphael et al. 2008).

During treatment of VOE, frequent evaluation of pain is important to assess the degree of relief and potential side effects of narcotic analgesia. When hospitalization is required, continuous opioid infusion by patient-controlled analgesia (PCA) is recommended (van Beers et al. 2007; Jacob et al. 2008). Figure 3 illustrates a convenient algorithm to consider for management of mild to severe painful VOE, but flexibility should exist for individual patient preferences. When opioids are used, an aggressive bowel regimen should be used concurrently to reduce gastrointestinal complications, especially hypomotility (O'Brien et al. 2010). Teaching and encouraging frequent incentive spirometry with ambulation can reduce the risk of developing complications including ACS (Bellet et al. 1995; Ahmad et al. 2011).

Figure 3.
Algorithm for the management of painful vaso-occlusive events.

Acute Splenic Sequestration Crisis

ASSC remains an important cause of morbidity and mortality for young children with SCA. Most ASSC events occur in infants or toddlers before age 2 yr. In some cases, ASSC may be the first clinical manifestation of SCA, and hence should be emphasized in the education of families during the first year of life.

The pathophysiology of ASSC involves erythrocyte sickling and rapid accumulation within the spleen. ASSC is clinically defined as a decrease in baseline hemoglobin concentration by ≥2 g/dL, in the presence of active reticulocytosis and splenomegaly; mild thrombocytopenia is common. The acute sequestration event can result in severe anemia, occasionally with hypovolemia, and even can evolve to circulatory shock or death (Topley et al. 1981; Emond et al. 1985; Powell et al. 1992).

Medical management of ASSC begins with early recognition and diagnosis; parents and caregivers must be educated about early signs and symptoms and the need to seek urgent medical evaluation. After initial assessment including vital signs and physical examination, laboratory studies should include complete blood count with

reticulocytes, blood culture if febrile, and type and crossmatch. Transfusion volumes should not be excessive because a transfusion "overshoot" phenomenon can occur when the spleen abruptly unloads trapped erythrocytes, raising the hemoglobin level above the target goal. Small aliquots (from the same unit) should be administered every 12–24 h to treat anemia and hypovolemia, while avoiding hyperviscosity following splenic release.

Recurrent ASSC events are common, occurring in about half of the children who survive the first episode. Chronic transfusions can be implemented after the first episode, but their benefits on reducing recurrent events or avoiding splenectomy are limited (Kinney et al. 1990). The benefits of splenectomy for ASSC must be compared to its infectious and other postoperative risks; surgery is usually recommended only after one severe or life-threatening ASSC event, or after several recurrent ASSC events. Pneumococcal immunizations should be completed before surgery, and then lifelong penicillin prophylaxis is recommended (Ammann et al. 1977; Deodhar et al. 1993). Partial or subtotal splenectomy could potentially preserve some filtrative and immunological splenic function, but published reports in SCA are sparse and anecdotal experiences have been unsuccessful (Rice et al. 2003).

Acute Chest Syndrome

ACS is a common cause of morbidity and a leading cause of death among adults with SCA (Castro et al. 1994; Gill et al. 1995; Vichinsky et al. 2000; Powars et al. 2005). First described over 30 years ago (Charache et al. 1979), ACS has a complex pathophysiology that remains poorly defined. Numerous etiologies have been proposed including typical and atypical bacterial pathogens (Miller et al. 1991; Vichinsky et al. 2000; Neumayr et al. 2003), viral infection (Lowenthal et al. 1996), fat embolism (Vichinsky et al. 1994), intrapulmonary sequestration of erythrocytes (Vichinsky et al. 1994), and nitric oxide–hemoglobin interactions (Gladwin et al. 1999).

ACS is defined as a constellation of signs and symptoms including respiratory distress with tachypnea and dyspnea, hypoxemia, fever, elevated WBC count, mild anemia, and new infiltrate on chest X-ray (Castro et al. 1994; Vichinsky et al. 2000; Ballas et al. 2010). ACS is often characterized by rapid clinical decline, so a high index of suspicion is needed for early identification and intervention. The onset of ACS may be insidious, often including pain, with nearly 50% of patients admitted with a different diagnosis. Sternal and rib pain often leads to splinting and poor inspiration, which coupled with mild respiratory depression from opioids, can quickly deteriorate into a serious condition requiring aggressive respiratory and hematological support. To avoid this sequence of events, incentive spirometry can reduce the incidence of ACS and should be mandatory for all hospitalized patients with SCA (Bellet et al 1995; Ahmad et al. 2011).

Worsening hypoxemia or tachypnea, and early radiographic changes should lead to aggressive incentive spirometry, and oxygen therapy to correct hypoxemia. Close management of fluid status is warranted to prevent fluid overload. Although hyperhydration is typically recommended for painful VOE to improve blood flow, intravenous fluids should be limited to 50%–75% maintenance in the setting of evolving ACS, to reduce the risks of developing pulmonary edema or pleural effusions and worsening respiratory distress. Despite the lack of randomized clinical trials investigating the efficacy of antibiotics for ACS (Martí-Carvajal et al. 2007), coverage is provided for typical community-acquired and atypical pathogens, such as a broad-spectrum third-generation cephalosporin and macrolide (Lottenberg and Hassell 2005). Bronchodilators are not effective for all patients but are recommended for patients with ACS and concurrent reactive airways disease or asthma (Knight-Madden and Hambleton 2003; Knight-Madden et al. 2005).

Transfusions are often used for ACS (Lanzkowsky et al. 1978; Vichinsky et al. 2000), improving both anemia and oxygen-carrying capacity. Early simple transfusions are beneficial and can avert clinical deterioration that might warrant later exchange transfusion. Significant respiratory distress or clinical decompensation, hemoglobin ≥2 g/dL below baseline, and oxygen saturation >5% below baseline are all indications for packed red blood cell (PRBC) transfusion (Miller 2011). Automated erythrocytapheresis should be used for severe ACS associated with significant respiratory distress or hypoxia (Kleinman et al. 1984; Velasquez et al. 2009). There is no current evidence that inhaled nitric oxide (NO) has a beneficial role in the current management of ACS (Gladwin et al. 1999; Al Hajeri et al. 2008), but several clinical trials are ongoing. For recurrent ACS, both chronic transfusion

programs (Miller et al. 2001a; Hankins et al. 2005a) and hydroxyurea (Steinberg et al. 2003; Hankins et al. 2005b; Wang et al. 2011) can reduce the frequency and severity of additional ACS events.

Stroke

Cerebrovascular accidents are a relatively common and devastating complication of SCA, with an overt stroke incidence rate of 11% by age 20 years and 24% by age 45 (Ohene-Frempong et al. 1998). Clinical stroke events represent only a fraction of the cerebrovascular complications of SCA, which include silent cerebral infarctions (Miller et al. 2001b; Pegelow et al. 2001; DeBaun et al. 2012) and other neurocognitive deficits (Schatz et al. 2001; Thompson et al. 2002).

In the setting of acute clinical stroke, quickly reestablishing cerebral blood flow is crucial; new-onset weakness or aphasia suggests stroke and intervention should never depend on confirmatory radiological imaging. Modest IV hydration can help acutely, and should be provided while blood is being crossmatched for transfusion. Particularly for children with severe anemia, a simple PRBC transfusion can rapidly reduce intravascular sludging and help improve cerebral blood flow, which is critical to help reverse acute symptoms and prevent stroke progression. When available, exchange transfusion should be performed promptly to reduce HbS <30%, with a target hemoglobin concentration of ~10 g/dL (Swerdlow 2006). A retrospective analysis of children with overt stroke suggested children receiving exchange transfusion had a significantly lower risk of recurrent stroke, compared to children receiving only simple transfusion (Hulbert et al. 2006).

After an initial stroke, the risk of recurrent stroke events is 47%–93% without specific treatment (Powars et al. 1978; Balkaran et al. 1992; Pegelow et al. 1995). Chronic transfusions provided every 3–4 wk to maintain HbS of ~30% are recommended to prevent recurrent events (Lusher et al. 1976; Pegelow et al. 1995; Strater et al. 2002; Platt 2006). Once initiated, transfusions should be continued indefinitely, because discontinuation of transfusions is associated with increased risk of recurrent events (Wang et al. 1991; Adams and Bramilla 2005). Although efficacious, 10%–20% of patients will develop a second stroke despite transfusions (Pegelow et al. 1995; Scothorn et al 2002). Recent evidence further shows progression of vasculopathy and silent cerebral infarctions among chronically transfused patients (Hulbert et al. 2011). Hydroxyurea for the prevention of recurrent stroke, coupled with phlebotomy to remove iron overload, has shown efficacy (Ware et al. 1999, 2004), but in a phase III randomized clinical trial was inferior to transfusions and chelation therapy (Ware and Helms 2012). In certain clinical settings in which chronic transfusions are unsafe or otherwise not feasible, however, hydroxyurea may be a viable treatment option (Ali et al. 2011).

SCREENING PROGRAMS

Go to:

Prevention of complications is the ideal way to reduce the morbidity of acute VOE and potentially limit chronic organ damage; this is especially true when considering devastating neurologic events like stroke. Because blood flow velocity is inversely related to vessel diameter, the measurement of intracerebral arterial blood flow by transcranial Doppler ultrasonography (TCD) allows easy and noninvasive identification of large vessel stenosis and can accurately identify children at increased risk of developing primary stroke (Adams et al. 1992). Children with SCA have increased mean flow velocities when compared to children without anemia (Adams et al. 1989, 1992), and markedly elevated TCD values represent a biomarker of cerebrovascular disease and a significant risk factor for primary stroke.

Based on its utility of screening for stroke risk, both with efficacy in clinical trials (Adams et al. 1998) and effectiveness in clinical practice (McCarville et al. 2008; Enninful-Eghan et al. 2010; Bernaudin et al. 2011; Kwiatkowski et al. 2011), TCD screening is recommended annually for children with SCA starting at the age of 2–3 years. Figure 4 illustrates the recommended screening regimen and algorithm for management of TCD results (Platt 2006; McCarville et al. 2008). The risk of first stroke can be nearly eliminated by the initiation of a chronic transfusion program to decrease HbS <30% for time-averaged maximum blood flow velocities >200 cm/sec in the internal carotid or middle cerebral artery (Adams et al. 1998; Lee et al. 2006). Despite its clear efficacy for

prevention of primary stroke, the risks of chronic transfusion therapy are significant and include iron overload, alloimmunization, and cost (Rosse et al. 1990; Harmatz et al. 2000; Wayne et al. 2000). Hydroxyurea therapy at maximum tolerated dose (MTD) also can reduce TCD velocities (Zimmerman et al. 2007) and the ongoing TWiTCH trial (ClinicalTrials.gov NCT00122980) compares the efficacy of hydroxyurea versus transfusions for prevention of primary stroke in children with abnormal TCD velocities.

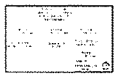

Figure 4.
Transcranial Doppler screening algorithm for children with SCA.

ADULT CARE

Go to:

Improved care of the child with SCA has led to increased survival rates into adulthood, and now attention must be focused on appropriate management of this older population. The success of neonatal and pediatric care leads to the formidable task of transitioning patients to the adult healthcare arena, in which providers often are neither trained adequately nor prepared to manage their unique needs. Written guidelines exist for managing adults with SCA (Lottenberg and Hassell 2005) but most of these recommendations do not derive from evidence-based research. The upcoming National Heart, Lung, and Blood Institute (NHLBI) guidelines document for SCA care, anticipated for release and distribution in 2013, will provide current evidence-based guidelines.

Adults with SCD should receive immunization boosters including pneumococcal, meningococcal, and even varicella vaccines based on age-specific recommendations. Management of acute VOEs such as pain, ACS, stroke, and priapism are similar for adults as for pediatric patients, but new issues may emerge related to chronic lung disease, especially restrictive pattern; leg ulcers that are difficult to manage and heal; and hepatic, endocrine, and even cardiac damage related to transfusional iron overload.

Additional screening for specific organ damage is recommended for teens and adults. Baseline pulse oximetry readings will help establish a baseline value and identify chronic hypoxemia. Blood pressure and serum creatinine are typically lower than age-related published values; hence, values at the upper limit of normal may indicate renal dysfunction. Dipstick urinalysis identifies gross proteinuria, but quantitative testing for microalbuminuria, using a 24-h urine collection if warranted, detects subclinical renal disease. Screening for hepatitis B, C, and HIV is warranted for patients who receive chronic blood transfusions; serum ferritin with review of transfusion history may identify patients who need iron chelation. Periodic screening for tricuspid regurgitation jet (TR jet) velocities can identify patients with pulmonary hypertension and possible early mortality (Gladwin et al. 2004). Ophthalmological screening for retinal disease is warranted every 1–3 yr with specialist referral for patients with abnormalities. Additional screening tests for the general adult population should also be provided, including strategies to prevent or identify cancer, hyperlipidemia, bone density loss, and even diabetes. (In the latter case, the lack of HbA makes screening with HbA1c innacurate, but serum fructosamine can be used.)

THERAPEUTIC INTERVENTION

Go to:

Urgent erythrocyte transfusions are indicated for many acute complications of SCA including ACS, ASSC, transient aplastic crisis owing to parvovirus B19 infection, and acute stroke. In these settings, transfused blood helps to alleviate anemia, improve circulating blood volume, increase oxygen-carrying capacity, and provide erythrocytes that cannot sickle. If the posttransfusion target is high enough, transfusions also help suppress endogenous sickle erythropoiesis. Elective transfusions are often given for preoperative management, to prevent perioperative sickle-related complications. Chronic transfusions given on a monthly basis are also highly efficacious for primary and secondary stroke prevention. In contrast, transfusions are not indicated for acute painful events or anemia per se (recognizing that almost all patients have a baseline steady-state partially compensated hemolytic anemia), and have little role in the management of standard VOE (Smith-Whitley and Thompson 2012).

In most acute settings, simple transfusions with packed erythrocytes (PRBC) should be administered. PRBC are readily available across the United States, and are routinely tested for HIV as well as hepatitis B and C. As a general principle, simple transfusions should be given with a target of alleviating anemia or treating the underlying condition; whole units (or half units for small pediatric patients) should be administered whenever possible, instead of fixed volumes (e.g., 10 mL/kg), to help limit foreign antigen exposure. The posttransfusion target hemoglobin concentration should not exceed 10–11 g/dL in the untreated patient because hyperviscosity can occur; in chronically transfused patients with low %HbS, however, the posttransfusion target can be raised to help suppress endogenous erythrocyte production. It is also important for the Blood Bank to be aware that the patient has SCA, because extending red blood cell (RBC) phenotype matching for minor blood group antigens is recommended to help prevent alloimmunization (Yazdanbakhsh et al. 2012).

For patients with neurological indications for chronic transfusion therapy such as abnormal TCD velocities or stroke, repeated simple transfusions are effective in preventing primary and secondary stroke, respectively, but ultimately result in transfusional iron overload. For this reason, partial exchange transfusions or isovolemic erythrocytapheresis is recommended to minimize iron accumulation. In most patients, intravenous access for exchange transfusions is facilitated by the placement of an implantable device. With chronic transfusions, the goal is typically HbS ≤30% as a pretransfusion value, which typically requires transfusion every 3–5 weeks depending on the type and volume of each transfusion, the patient's own erythropoietic drive, and the response to transfusion therapy. Chelation therapy for transfusional iron overload should be considered for all patients on chronic transfusions, but also for teens and adults who have a large cumulative number of episodic or sporadic transfusions.

HYDROXYUREA

Go to:

Increased fetal hemoglobin (HbF) levels have been associated with a less severe phenotype of SCA (Conley et al. 1963; Diggs 1973; Platt et al. 1991, 1994) and HbF induction has become a desired pharmacologic end point for SCA therapy (Charache 1990; Charache et al. 1992). Hydroxyurea has been shown to potently increase HbF and is currently the most effective disease-modifying therapy for both adults and children with SCA (Platt et al. 1984; Steinberg et al. 2003; Zimmerman et al. 2004; Hankins et al. 2005b). The first clinical experience with hydroxyurea for SCA was reported nearly 30 years ago in seminal proof-of-principles studies (Platt et al. 1984). Subsequently, a multicenter phase II study documented laboratory efficacy (increased Hb, %HbF, and MCV; decreased WBC, ANC, ARC, and platelets) of hydroxyurea using a dose escalation schedule to MTD (Charache et al. 1992). The Multi-Center Study of Hydroxyurea (MSH) double-blinded, placebo-controlled randomized clinical trial showed clinical efficacy of hydroxyurea for adults with severe SCA, with significantly reduced time to first painful event, plus fewer episodes of ACS, transfusions, and hospitalizations (Charache et al. 1995).

In children with SCA, similar laboratory and clinical efficacy have been shown in open-label trials (Kinney et al. 1999; Wang et al. 2001; Zimmerman et al. 2004; Hankins et al. 2005b; Thornburg et al. 2009). In hydroxyurea study of long-term effects (HUSTLE), all pediatric patients with medication adherence had HbF responses, although responses were variable and possibly related to differences in drug absorption, pharmacokinetics, and pharmacogenetics (Ware et al. 2011). The results from the double-blinded, placebo-controlled multicenter randomized BABY HUG study show the safety and clinical efficacy of hydroxyurea for young infants with SCA, regardless of previous clinical severity (Wang et al. 2011). The primary end point of BABY HUG was the ability of hydroxyurea to prevent chronic organ damage (kidney, spleen), and the short-term study results were equivocal. Anecdotal reports suggest prevention and even reversal of chronic organ damage with hydroxyurea therapy (Zimmerman et al. 2007; Hankins et al. 2008; Thornburg et al. 2009), so further investigation of the BABY HUG cohort is necessary.

Long-term follow up from MSH and the Greek Laikon Study of Hydroxyurea in Sickle Cell Syndromes documented reduced mortality for adult patients with SCA on hydroxyurea (Steinberg et al. 2010; Voskaridou et al. 2010). There is now indisputable evidence that hydroxyurea has laboratory and clinical efficacy for all ages; a growing body of evidence also supports the long-term safety of hydroxyurea and the ability of hydroxyurea to prevent chronic organ damage and reduce mortality (McGann et al. 2011). Whereas hydroxyurea previously has

been reserved for older patients with a severe clinical course, hydroxyurea use should be liberalized and offered to all adults with SCA. An increasing number of pediatric hematologists believe hydroxyurea should now be considered as a treatment option for all children with SCA, regardless of age or previous clinical course.

Hydroxyurea should be initiated by an experienced clinician familiar with laboratory monitoring and appropriate dose escalation to MTD. Hydroxyurea treatment should commence at ~20 mg/kg/d by mouth, once daily. Complete blood count (CBC) should be checked every 4 wk to monitor for myelosuppression, which is typically mild and dose dependent, and always reversible by holding the hydroxyurea dose temporarily (Heeney and Ware 2008). Dose adequacy and medication compliance can be assessed by reviewing changes in CBC parameters and reviewing the peripheral blood smear (Fig. 5). To reach MTD, the daily dose should be escalated by ~5 mg/kg every 8 wk until MTD is mild neutropenia (e.g., ANC of 1500–3000 × 10^6/L) or reticulocytopenia (ARC of 100–150 × 10^9/L) is reached on a stable dose. Drug toxicity is usually defined by cytopenias such as ANC <1.0 × 10^9/L, hemoglobin <7.0 g/dL with low reticulocyte count, ARC <80 × 10^9/L, and platelets <80 × 10^9/L (Heeney and Ware 2008). Given the unlikelihood of true hydroxyurea "nonresponders," efforts must be made to encourage medication compliance to reach and maintain a stable and efficacious hydroxyurea MTD (Ware 2010b).

Figure 5.
Blood smear changes with hydroxyurea therapy. *A* illustrates the untreated patient with anemia and numerous sickled forms; *B* is after initiation of hydroxyurea treatment with macrocytosis and more target cells; and *C* is after reaching a stable hydroxyurea ...

OTHER TREATMENTS

Go to:

HbF induction can be achieved by a group of short-chain fatty acids that inhibit the enzyme histone deacetylase; such HDAC inhibitors, primarily butyrate, can alter chromatin structure and induce HbF production by altering the transcription of the γ-globin gene (McCaffrey et al. 1997; Ataga 2009; Bradner et al. 2010). Clinical experience with HDAC inhibitors for SCA is limited but anecdotal reports suggest robust HbF induction in some patients with SCA (Atweh et al. 1999; Hines et al. 2008).

Decitabine is a nucleoside analog that induces HbF induction via epigenetic modulation, specifically hypomethylation of the γ-globin gene promoter. Experience with decitabine for SCA is also relatively limited, but several reports suggest clinical and laboratory efficacy of subcutaneously administered decitabine in adults who were not responsive to hydroxyurea (Creusot et al. 1982; DeSimone et al. 2002; Saunthararajah et al. 2003, 2008). Prospective trials of decitabine are warranted to determine if it has efficacy for a broad spectrum of patients with SCA.

Additional treatments that target specific pathways of the pathophysiology of SCA are just entering into clinical trials. One new promising inhibitor of the Gardos channel was found to have favorable effects on hemolysis and RBC survival, yet did not have clinical efficacy in a phase III randomized clinical trial (Ataga et al. 2011).

CONCLUDING REMARKS

Go to:

Decades of observational studies and therapeutic trials have contributed to a greater understanding of the pathophysiology and management of SCA. Based on these results, relatively simple interventions can substantially improve the survival of SCA, especially among children. Newborn screening, early preventive treatments, education about complications, and screening programs improve both the morbidity and mortality of SCA. Going forward, attention must focus on the care and management of teens and adults with SCA, and address quality of life as well as medical complications. More aggressive treatment of SCA is supported by current evidence, and therapeutic options with hydroxyurea should be considered early in life.

Footnotes

Go to:

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Pediatric Congenital Heart Association

Statement for the Record

In support of

H.R. 3952,

"The Congenital Heart Futures Reauthorization Act of 2015"

Submitted to the Subcommittee on Health,

House Committee on Energy and Commerce

September 8, 2016

On behalf of the estimated 2.4 million people living with congenital heart disease (CHD) in the United States, and the countless families who have lost loved ones to CHD, the Pediatric Congenital Heart Association is writing to offer our strong support for H.R. 3952, The Congenital Heart Futures Reauthorization Act of 2015, or CHFRA.

We are extremely grateful to Representative Bilirakis and Representative Schiff for their steadfast leadership as sponsors of this bipartisan effort to build upon existing programs which track the epidemiology, raise the awareness and promote lifelong research of the most common birth defect. Thank you, also, to the dozens of congressional cosponsors committed to this goal. We also want to express our appreciation to the leadership of the Energy and Commerce Committee and the Health Subcommittee for this opportunity to move this important bill toward enactment.

The Burden of Congenital Heart Disease

CHD, a structural abnormality of the heart present at birth, is the most common birth defect and the leading cause of birth defect-related infant mortality. Nearly 1 in 100 babies are born with CHD and more than five percent of those with CHD will not live to see their first birthday. Even for those who receive successful intervention, **it is not a cure**.

However, critical information about the epidemiology of CHD, the effectiveness of treatments, and lifelong outcomes is seriously lacking, at best, and non-existent in specific areas such as comorbidities that occur later in life.

Over the last several decades, advances in treatment have dramatically improved survival for children with the most complex CHD and increased the life expectancy of adults with CHD. More than 80% of children born with CHD are now expected to survive into adulthood. In the absence of US data, which the Congenital Heart Futures Reauthorization Act will address, extrapolation of Canadian data suggests that there are 2.4 million people with congenital heart disease, more than half of which are adults. This prevalence is expected to increase 5% annually, resulting in a surge of CHD survivors who need life-long specialized cardiac care. Although the growing population of individuals with CHD is a testament to important innovations in CHD care, the reality is that complex CHD and its treatments may result in chronic complications that may require further surgery, intervention, or heart transplantation. In addition to structural heart issues, individuals with CHD are at risk for secondary conditions including kidney, liver, and neurodevelopmental problems. In short, the interventions children with CHD receive are not cures, underscoring the need for lifelong care by expert providers to avoid and treat health complications later in life.

Another challenge faced by those living with CHD is the transition of care from pediatric to specialized adult heart care. In part, this is due to the lack of infrastructure, providers, and payors required to care for the ever increasing adult CHD population. Adding to this challenge are those patients who no longer seek care believing their childhood intervention has "cured" them, or that they are well enough to no longer need specialized cardiac care. Estimates suggest less than 25% of adults with CHD are

receiving appropriate subspecialty care. This population of patients who are lost to follow-up care present a critical public health issue, which will be addressed by this important legislation.

Neurological complications related to CHD pose another challenge. Cardiac surgery and perioperative treatments in the infant put the developing brain at tremendous risk for injury and long-term neurodevelopmental complications. Survivors often suffer injury to the brain due to low oxygen levels resulting from their CHD as well as operative and perioperative interventions. These brain injuries result in decreased neurodevelopmental, psychosocial, and physical functioning, and can significantly negatively impact the individual's growth, performance, and quality of life.

The Cost of Congenital Heart Disease

People born with CHD require lifelong, costly, specialized cardiac care, and face an ongoing risk of permanent disability and premature death. As a result, healthcare utilization among the CHD population is disproportionately higher than the general population. It is estimated that compared to medical costs of care for their peers, the medical costs for individuals with CHD are 10 to 20 times as great. Inpatient care costs (not including costs of physician care) for patients < 21 years old with CHD alone totaled more than $5.6 billion in 2009, representing 15 percent of hospitalization costs for all patients in this age range. Around half of all dollars spent on pediatric CHD related inpatient stays is paid by Medicaid.

Hospital admissions for adults with CHD roughly doubled between 1998 and 2005. Nearly 20% of these admissions were for cardiac surgery or catheter-based intervention. Healthcare utilization and costs continue to rise, due to hospital admissions, surgery and intervention, and emergency room visits. For example, with improved longevity to childbearing age, the number of high risk annual births in women with CHD is increasing. Childbearing women with CHD are fourteen times as likely to experience cardiovascular complications during pregnancy and are eighteen times as likely to die from such complications as are women without CHD. They also have longer hospital stays and incur higher hospital charges.

With disproportionate medical costs, it is critical for us to understand the life-course of those living with CHD, their health care utilization and potential cost reduction strategies.

Consider the story of Piper, who is one in 100. Piper was born on Independence Day in 2003 after a seemingly normal pregnancy. However, instead of rosy pink, Piper was born blue. Suddenly, the world was flipped upside down for her first-time parents, as they learned that there was something critically wrong with Piper's heart. She was rushed to the nearby children's hospital to receive life-saving treatment which included open heart surgery. Her recovery from 14 hours of surgery was challenging and Piper spent the next 6 weeks in the hospital. Imagine that she has since grown to become a vibrant adult having completed college and entering the work place. At her annual visit she expresses plans to become pregnant, soon. Her physician, after reviewing data,

refers her to a high risk obstetric center, and modifies her medications for heart rhythm problems. She delivers a healthy baby boy a year later, without complications for herself or her child. But, this data her physician referenced doesn't exist. Instead this current teenager is left with questions like can I travel to attend college? Will I able to have children? How long am I expected to live? Her life remains extraordinary with ongoing, costly, specialized care and concerns about a future where long-term outcomes are widely unknown.

A Critical Public Health Issue

The Congenital Heart Futures Act of 2015 (CHFRA) offers a reasonable and meaningful solution. To improve care and reduce costs, it is essential that Congress enacts legislation supporting improved understanding of CHD across the lifespan. The CHFRA calls for improved public health research and surveillance of those living with CHD, as well as providing for education and awareness, that will help us better understand and improve long-term outcomes for Piper and the more than 40,000 babies born with CHD each year.

Previous Congressional investment for CHD activities supported by the Centers for Disease Control and Prevention's (CDC's) National Center on Birth Defects and Developmental Disabilities (NCBDDD) has funded the development of innovative surveillance strategies among children, adolescents and adults with CHD. This has led to the standardization of research methods and an increased understanding of the public health burden of this condition. Since the enactment of the Congenital Heart

Futures Act in 2010, Congress has appropriated nearly $15 million to NCBDDD for these activities. Continued federal investment is necessary to provide surveillance and public health research on individuals across the lifespan to better understand CHD at every age, improve outcomes and reduce costs.

The Congenital Heart Futures Reauthorization Act of 2015 does just that. The legislation directs the Centers for Disease Control and Prevention (CDC) to plan, develop and implement a representative cohort study to help describe basic U.S. demographics of the disease, assess healthcare utilization, and lead to evidence-based practices and guidelines for CHD care. The bill also allows for the CDC to establish and implement an education, outreach and awareness campaign directed at CHD across the lifespan, to ensure that those who have a CHD, and their families, understand their life-long healthcare needs and maintain appropriate specialized care.

The CHFRA also directs the National Institutes of Health (NIH) to assess its current research into CHD so we can have a better understanding of the state of bio-medical research as it relates to improving understanding of causes and drive innovative and effective treatments for CHD and related disease processes.

Congenital heart disease is common and costly; a critical public health challenge. We are thankful for the many members of Congress and colleagues in the community who join us in support of support of H.R. 3952, the Congenital Heart Futures Reauthorization Act 2015. It is essential for Congress to pass this comprehensive

approach to CHD that will address a critical public health issue and lead to better care, outcomes and quality of life for the millions of individuals living with CHD.

The Pediatric Congenital Heart Association's mission is to "Conquer Congenital Heart Disease." We are founded on the key purpose to be the resounding voice of the pediatric patient population and are accomplishing this through collaboration with patients, parents, providers, and partner organizations in order to improve quality and outcomes through CHD education, research and awareness.

Adult Congenital Heart Association

https://www.achaheart.org/ * (888) 921-ACHA

Testimony of

Brian Altman

Adult Congenital Heart Association

Before the

Committee on Energy and Commerce; Subcommittee on Health

on

"Examining Legislation to Improve Public Health"

September 8, 2016

DISCLAIMER: The following testimony outlines my personal position and should not be attributed to my employer, the Substance Abuse and Mental Health Services Administration. This testimony was prepared on my personal time without using any of my employer's resources.

Chairman Pitts, Ranking Member Green and distinguished members of the Subcommittee. My name is Brian Altman and I am a 41 year old living with congenital heart disease (CHD). In addition to being an adult with CHD, I am a former member of the Board of Directors and Public Policy Chair of the Adult Congenital Heart Association (ACHA). I appreciate the opportunity to provide testimony on behalf of ACHA whose mission is to improve and extend the lives of the millions born with heart defects through education, advocacy and promoting research.

My Story

I was born in 1975 with transposition of the greater arties and a ventricular septal defect. To my parents, that was a diagnosis that brought tears to their eyes and at the time likely meant death for me at a very early age. In the short term, the diagnosis meant that I was a blue baby who needed to be rushed out of state for care. We lived in Tulsa, Oklahoma which at the time did not have highly skilled pediatric cardiologists. So, at two weeks old I was taken to Texas Children's Hospital where I received care until approximately 2003. During those years, I was very fortunate compared to many I know with CHD.

First, I had loving parents with private health insurance. Second, I "only" had three surgeries, "only" one of which was an open heart surgery. Third, by all accounts my open heart surgery, performed by the world renowned Dr. Denton Cooley, was a success and I was "fixed." Finally, at 28 years old as my pediatric cardiologist ensured that I was not lost to care and handed me over to a new type of provider, an adult congenital heart specialist. For the last 13 years, I have been fortunate to continue to receive specialty care at Children's National Medical Center and Boston Children's Hospital.

However, through ACHA and my doctors I know I was not "fixed" for life, my birth defect is a chronic illness and most individuals with my condition born in the 1970s are no longer alive. There are also many things which are unknown to me. First, because of a lack of research and surveillance about lifelong outcomes for adults with CHD until the Congenital Heart Futures Act passed in 2010, no doctor, researcher or epidemiologist can tell me the average lifespan of a person with my birth defects. Similarly, no one knows what types of other conditions are most prevalent among adults living with a "mustard procedure," the type of open heart surgery I had. So, years later when I developed generalized anxiety disorder, atrial tachycardia, and a leak in my tricuspid valve, these conditions were not ones that were easily predicable, despite CHD being the most common birth defect in America. As a result, I have been on costly medications and had a second open heart surgery last year.

For me, my CHD means I try to live each day to the fullest. I've studied hard, engaged in a career that advances public health policy and built a family I love with all my being. But it also means I have no idea if I will be alive to see my daughter graduate from high school or college, if I will get to walk her down the aisle when she gets married, or if I will get to hold hands with my husband in a nursing home.

ACHA

ACHA has been a life changing organization for me and so many other adults with CHD. From its founding, it has been dedicated to supporting individuals and families living with congenital heart disease and advancing the care and treatment available to our community. ACHA provides resources I did not even realize I needed such as a travel directory of Adult Congenital Heart specialty programs. I carry this with me wherever I go so if something

happens while away on work travel or vacation, I can be taken to specialists who know my conditions and the reason my EKG and heart look and sound the way they do. ACHA listens not just to patients or providers, but ensures that we are all working together to foster the best research, provide the best care and support each other through surgeries, rehabilitation and unfortunately too often death. ACHA thinks strategically about the next steps forward in care provision, patient support and advocacy. With this in mind, the ACHA provides webinars aimed at providers and patients and hosts a conference with tracks for both, but key sessions that bring the providers and patients together. ACHA formed the Heart to Heart Ambassadors program to help patients and families have a peer support network. And, ACHA is on the forefront of ensuring high quality care by recently launching an accreditation program. This program is crucial for advancing and standardizing the quality of care for ACHD. Every person with CHD needs access to specialized care regardless of where they live. Finally, ACHA has lead the way in advocacy for adults with CHD.

Congenital Heart Futures Act

Because of what I have lived through and the many amazing doctors, nurses, individuals living with CHD, those I have known and lost and their families, I dedicated many hours to ensuring passage of the Congenital Heart Futures Act. The law ensures that the federal government dedicates research, surveillance and awareness to CHD not just as a birth defect, but as a chronic disease that thankfully individuals live with into their forties, fifties and beyond. Even as I fought for passage six years ago, on every visit to offices such as yours I noted the bill and the vital programs it authorized were not for me. Rather, the law and hopefully reauthorization of the law will ensure that those younger than I will know and understand what

steps they and their health care providers can take to ensure they not just live as long as possible, but to thrive in their life.

I am thankful for the efforts the Centers for Disease Control and Prevention and the National Institutes for Health have taken so far and know so much more can be learned and so many more individuals can be reached. I am appreciative of the funding that Congress has provided for the programs authorized by the law and know the funding is vital to keep the research going and the registries up to date.

Conclusion

I appreciate the key members of Congress who have supported the Congenital Heart Futures Act including Representative Bilirakis, Representative Schiff and in the Senate the CHD's tireless champion, Senator Richard Durbin. Thank you for holding this legislative hearing on the Congenital Heart Futures Reauthorization Act. On behalf of ACHA and its members, I look forward to passage of this important bill so that we will have the science, surveillance, outreach and education to help individuals with CHD live and thrive.

ASE American Society of Echocardiography

June 1, 2016

Representative Gus Bilirakis
2112 Rayburn House Office Building
Washington, D.C. 2051

Representative Adam Schiff
2112 Rayburn House Office Building
Washington, D.C. 20515

Dear Representatives Bilirakis and Schiff:

On behalf of the American Society of Echocardiography (ASE), I am pleased to have the opportunity to offer our strong support for the Congenital Heart Futures Act Reauthorization Act of 2015. ASE is an organization of nearly 17,000 professionals committed to excellence in cardiovascular ultrasound and its application to patient care. Cardiovascular ultrasound is critical to the initial diagnosis and ongoing treatment of Congenital Heart Disease (CHD).

We very much appreciate your leadership in introducing this bipartisan legislation, which would affirm the importance of continued CHD research by directing the Centers for Disease Control (CDC) to conduct a cohort study examining the CHD epidemiology, authorize a campaign to raise public awareness, and promote biomedical research at the National Institutes of Health (NIH) on the diagnosis, treatment, prevention and long term outcomes of this serious and prevalent condition.

While we have made substantial progress in the treatment of heart disease, we appreciate your recognition that much work still needs to be done in the area of CHD, which remains the leading cause of infant mortality. Nearly 1 in 100 babies are born with CHD, and, while life expectancy of many infants born with heart defects has been extended, there is no cure. Moreover, those children who are accurately diagnosed and treated face life-long risks and potential complications, including heart failure, stroke, renal dysfunction, and neurocognitive problems. The epidemiological studies, research focus, and public awareness campaign required by the Congenital Heart Futures Reauthorization Act are desperately needed.

Again, the ASE very much appreciates your leadership on this issue, and looks forward to working with you to secure passage of this important legislation.

Sincerely yours,

Susan E. Wiegers, MD, FACC, FASE
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United States House of Representatives Committee on Energy and Commerce
Subcommittee on Health
Examining Legislation to Improve Public Health

Statement of the Alzheimer's Association on the Palliative Care and Hospice Education and Training Act

September 8, 2016

Thank you for holding today's hearing on legislation to improve public health. We applaud your leadership on issues important to Americans with Alzheimer's disease and other dementias and their caregivers. The Alzheimer's Association proudly supports the Palliative Care and Hospice Education and Training Act (H.R. 3119/S. 2748), which was introduced in the House of Representatives by Representative Eliot Engel (D-NY-16) and Representative Tom Reed (R-NY-23) in July 2015. This legislation, which currently has 200 bipartisan cosponsors in the House of Representatives, will ensure an adequate, well-trained palliative care workforce through workforce training, education and awareness, and enhanced research.

Alzheimer's disease is a progressive, neurodegenerative and fatal disease for which there is currently no treatment or cure. More than 5 million Americans are currently living with Alzheimer's disease and other dementias. In addition,15.9 million friends and family of those with Alzheimer's disease are also acting as uncompensated caregivers. In 2015, these individuals provided 18.1 billion hours of care, valued at more than $221 billion.[1] Alzheimer's disease was identified by the *New England Journal of Medicine* as the costliest disease in the United States.[2] As the baby boom generation continues to age, one in nine Americans above age 65 will develop Alzheimer's disease or another dementia. By 2050, the number of people in the United States with Alzheimer's disease may be as high as 16 million, representing an annual cost to the nation of nearly $1.1 trillion (in today's dollars).[3]

Palliative and hospice care – with a focus on managing and easing symptoms, reducing pain and stress, and increasing comfort – can improve both the quality of care and quality of life for those with advanced dementia. Observational studies have shown that as many as half of nursing home residents with advanced dementia have documented pain in the last weeks of life – and about a third have difficulty breathing or swallowing, or suffer from agitation. Individuals with advanced dementia who are enrolled in hospice have a lower rate of dying in the hospital, a lower rate of hospitalization in the last 30

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[1] Alzheimer's Association. (2016). *2016 Alzheimer's Disease Facts and Figures.*
[2] Michael D. Hurd, Ph.D., Paco Martorell, Ph.D., Adeline Delavande, Ph.D., Kathleen J. Mullen, Ph.D., and Kenneth M. Langa, M.D., Ph.D. Monetary Costs of Dementia in the United States. *N Engl J Med* 2013; 368:1326-1334April 4, 2013DOI: 10.1056/NEJMsa1204629.
[3] Alzheimer's Association. (2016). *2016 Alzheimer's Disease Facts and Figures.*

days of life, and better symptom management. Additionally, a recent study shows that nursing home residents with dementia who receive palliative care at the end of life, compared with those who do not receive such care, are up to 15 times less likely to die in a hospital, nearly 2.5 times less likely to have a hospitalization in the last 30 days of life, and are up to 4.6 times less likely to have an emergency room visit in the last week of life.[4] Families of individuals with dementia who are enrolled in hospice also have a greater satisfaction with patient care.

People with Alzheimer's and other dementias also rely heavily on hospice at the end of life. Of all people living with dementia, 18.6 percent are currently in hospice care – a higher percentage than other chronic conditions.[5] Among seniors in hospice care, one in every six has a primary hospice diagnosis of Alzheimer's or other dementia. Nearly half of all people with dementia die in hospice care. The population of persons with dementia using hospice has grown dramatically in recent years[6] – a trend that is likely to continue as the number of Americans with Alzheimer's disease and other dementias grows.[7]

Congress unanimously passed the bipartisan National Alzheimer's Project Act (P.L. 111-375) in 2010. The law instructs the Department of Health and Human Services (HHS) to develop a strategic plan to address the rapidly escalating Alzheimer's disease crisis. The annually updated *National Plan to Address Alzheimer's Disease* must be transmitted to Congress each year and is to include outcome-driven objectives, recommendations for priority actions and coordination of all federally funded programs in Alzheimer's disease research, care and services. The Alzheimer's Association recently convened an expert workgroup to develop milestones for the care and support goals under the *National Plan to Address Alzheimer's Disease*. Those milestones include actions that need to be taken by both the federal and state governments in order to create a high quality care and support system for individuals with the disease and their families. Specifically, the expert workgroup recommends action to outline comprehensive standards of dementia care for palliative and hospice care; to develop components of dementia-competent palliative care for persons with Alzheimer's disease and other dementias; to modify hospice criterion from end of life estimate to disability severity for persons with Alzheimer's disease and other dementias; and to define process components for clinicians to discuss end of life planning with individuals with Alzheimer's disease and key caregivers, which would include hospice care.[8]

The Palliative Care and Hospice Education and Training Act would ensure an adequate, well-trained palliative care workforce through workforce training, education and awareness, and enhanced research. Specifically, this legislation would establish palliative care and hospice workforce training programs for doctors, nurses, and other health professionals; create

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[4] Susan C. Miller et al. "The Effect Of Palliative Care Consults On Acute Care Use For Residents With Dementia In Nursing Homes". (November 2015).
[5] Unpublished tabulations based on data from the National 5% Sample Medicare Fee-for-Service Beneficiaries for 2014. Prepared under contract by Avalere Health, January 2016.
[6] National Hospice and Palliative Care Organization. (2014). *NHCPO's Facts and Figures: Hospice Care in America.*
[7] Alzheimer's Association. (2016). *2016 Alzheimer's Disease Facts and Figures.*
[8] Soo Borson et al., "Report on Milestones for Care and Support Under the U.S. National Plan to Address Alzheimer's Disease," *Alzheimer's & Dementia* 12, no. 3 (March 2016).

a national education and awareness campaign to inform patients, families, and health professionals about the benefits of palliative care and available services and supports; and enhance research on improving the delivery of palliative care. In addition, the legislation is consistent with findings and recommendations made by the Institute of Medicine expert panel on advanced dementia, as well recommendations made by the Advisory Council on Alzheimer's Care, Research, and Services.

Thank you for holding today's hearing and for your continued leadership on issues that are so important to individuals and families facing Alzheimer's disease. We look forward to working with you and your colleagues on efforts to improve care and support for individuals and families affected by this disease, including the Palliative Care and Hospice Education and Training Act.

American Academy of Hospice and Palliative Medicine

Testimony for the Record
Before the House Energy and Commerce Health Subcommittee
Hearing Entitled
"Examining Legislation to Improve Public Health"

Thursday, September 8, 2016

Chairman Pitts, Ranking Member Green, and members of the Subcommittee, the American Academy of Hospice and Palliative Medicine (AAHPM) would like to thank the House Energy and Commerce Health Subcommittee for the opportunity to share our Academy's views on **H.R. 3119, the Palliative Care and Hospice Education and Training Act (PCHETA)**. AAHPM particularly offers its gratitude to Congressman Engel for his strong leadership on this important legislation.

AAHPM is the professional organization for physicians practicing Hospice and Palliative Medicine. AAHPM's nearly 5,000 members also include nurses and other health and spiritual care providers who are committed to improving the quality of life of seriously ill patients and their families/caregivers. For close to 30 years AAHPM has been dedicated to expanding access of patients and families to high-quality palliative care and advancing the discipline of Hospice and Palliative Medicine through professional education and training, development of a specialist workforce, support for clinical practice standards, research, and public policy.

This written testimony discusses how Congress can help improve care for the expanding population of patients with serious illness or multiple chronic conditions. We encourage the Subcommittee to consider the needs of these patients and support PCHETA as part of its efforts to improve the nation's health.

H.R. 3119 would expand opportunities for interdisciplinary education and training in palliative care, including through new education centers and career incentive awards for physicians, nurses, physician assistants, social workers and other health professionals. The bill would also implement an

awareness campaign, to inform patients and health care providers about the benefits of palliative care and hospice and the services available to support individuals with serious or life-threatening illness, as well as direct funding toward palliative care research to strengthen clinical practice and healthcare delivery.

AAHPM's leadership stands ready to further discuss how PCHETA can help advance the Subcommittee's goals for the healthcare system and to answer any questions the Subcommittee has going forward with regard to improving care for patients and families/caregivers through the provision of high-quality palliative care.

BACKGROUND

Defining the problem

By 2050, the population aged 65 and over is projected by the U.S. Census Bureau to be 83.7 million, almost double that in 2012. As the population ages, an increasing number of people will be living with serious, complex and chronic illness. According to the Medicare Payment Advisory Commission (MedPAC), in 2010 more than two-thirds of Medicare beneficiaries had multiple chronic conditions while 14 percent had six or more. Treatment of chronic and serious illnesses, such as heart disease and cancer, now accounts for nearly 93 percent of Medicare spending.

Many of the problems of our health care system—high costs, overutilization, lack of coordination, preventable transitions between health care institutions, and poor quality—become particularly evident during extended chronic and serious illness. But a growing body of medical research has documented the benefits of high-quality palliative and hospice care for patients and families, for hospitals and payers, and for the health care system as a whole.

Palliative care is an essential part of the solution

AAHPM believes that palliative care providers and organizations, including hospices, are integral to meeting the "triple aim" of better care for individuals, improved health of populations, and lower

growth in health care expenditures. Indeed, the National Priorities Partnership has highlighted palliative and end-of-life care as one of six national health priorities that have the potential to create lasting change across the U.S. healthcare system.

Palliative care is an interdisciplinary model of care aimed at preventing and treating the debilitating effects of serious and chronic illness, such as cancer, cardiac disease, respiratory disease, kidney failure, Alzheimer's, AIDS, ALS, and MS. It can be provided from the time of diagnosis and involves the relief of pain and other symptoms that cause discomfort, such as shortness of breath, unrelenting nausea, etc. Palliative care can be offered alongside life-prolonging and curative therapies for individuals living with serious, complex, and potentially terminal illness and includes hospice care.

Palliative care focuses on matching treatments to achievable patient goals, in order to maximize quality of life from diagnosis to death. In practice, this involves detailed and skilled communication with patients and families to elicit goals and preferences, as well as expert assessment and management of physical, psychological and other sources of suffering across the multiple settings (hospital, post-acute care, ambulatory clinics, home) that patients traverse through the course of a serious illness. Evidence and experience show that seriously ill patients and those with multiple chronic conditions and functional impairment—many of whom are Medicare's highest need and highest cost beneficiaries—strain these systems significantly.

Recent studies have demonstrated that high-quality palliative care and hospice care not only improve quality of life and patient and family satisfaction, but can also prolong survival.[1-5] Furthermore, palliative care achieves these outcomes at a lower cost than usual care, by helping patients to better understand and address their needs, choose the most effective interventions, and avoid unnecessary/unwanted hospitalizations and interventions.

However, delivery of high-quality palliative care cannot take place without sufficient numbers of healthcare professionals with appropriate skills. By supporting H.R. 3119, AAHPM believes the Subcommittee can help build a healthcare workforce more closely aligned with the nation's evolving

healthcare needs. PCHETA will help close the large gap between the number of health care professionals with palliative care training and the number required to meet the needs of the expanding population of patients with serious illness or multiple chronic conditions.

Workforce Challenges

The reality today is healthcare providers need better education about pain management and palliative care. Students graduating from medical and nursing school have very little, if any, training in the core precepts of pain and symptom management, communication skills, and care coordination for patients with serious or life-threatening illness. The 2014 Institute of Medicine (IOM) report *Dying in America: Improving Quality and Honoring Individual Preferences Near the End of Life* noted that "major gains have been made in the knowledge base of palliative care."[6] The report documented, however, that "these knowledge gains have not necessarily been matched by the transfer of knowledge to most clinicians caring for people with advanced serious illnesses." Moreover, the IOM noted that an "overall pattern of inattention to palliative and end-of-life care ... still appears to predominate in the pediatric world." This lack of healthcare provider knowledge results in too many patients with serious illness receiving painful or ineffective treatments that do nothing to prolong or enhance their lives.

At the same time, despite the growing need for palliative care, the field has been unable to meet patient and health system demand because of a significant shortage of specialist providers. The current gap between those practicing in the field and the number of physicians required to meet current need is likely huge—possibly several thousand physicians. A 2010 article published in the *Journal of Pain and Symptom Management* provides the findings of an AAHPM task force established to assess whether a physician shortage existed and to develop an estimate of the optimal number of Hospice and Palliative Medicine physicians needed to meet current and future needs.[7] It was determined that an acute shortage of hospice and palliative medicine physicians exists, with the current capacity of fellowship programs insufficient to fill the gap.

As of August 2016, there were a total of 119 Hospice and Palliative Medicine training programs accredited by the Accreditation Council for Graduate Medical Education and 15 training programs accredited by the American Osteopathic Association. For the 2015-2016 academic year, these programs were training just 296 physicians in Hospice and Palliative Medicine. At this rate, today's training programs would produce fewer than 6,000 new Hospice and Palliative Medicine certified physicians *over the next 20 years.*

Yet AAHPM estimated 6,000+ full time equivalents—or 8,000 to 10,000 physicians—would have been required to meet the needs in hospice and palliative care programs back in 2010 (most recent workforce estimates), with up to 18,000 physicians needed if all hospices and palliative care programs were then using exemplary staffing models. These scenarios did not take into account future expansion of need due to population growth and aging or expansion of palliative care services into community settings such as nursing homes, home care, and office practices, all of which have exacerbated the hospice and palliative medicine workforce shortage.

The current Hospice and Palliative Medicine physician shortage can be attributed in large part to faulty Medicare policy. Despite the fact that the majority of patients receiving palliative care and hospice services are Medicare beneficiaries, and that palliative care has been repeatedly shown to increase value in health care by improving quality while reducing costs compared to usual care, Medicare does not invest in the training of Hospice and Palliative Medicine physicians. Instead, Hospice and Palliative Medicine specialty training is entirely dependent on private-sector philanthropy or institutional support because the Balanced Budget Act of 1997 placed a limit on the number of Medicare-supported residency slots before Hospice and Palliative Medicine was formally recognized as a medical subspecialty by the American Board of Medical Specialties. Given the instability of such funding, this is not a sustainable or rational way to train our nation's Hospice and Palliative Medicine physicians.

Nonetheless, noting that "hospice and palliative medicine specialists will never be sufficient in number to provide regular face-to-face treatment of every person with an advanced serious

illness," the IOM report recommends expanding training opportunities to ensure clinicians across disciplines and specialties who care for people with serious illness are competent in "basic palliative care," including communication skills, interprofessional collaboration, and symptom management.

Modeled after the existing geriatric education centers, PCHETA would establish Palliative Care and Hospice Education Centers to improve the training of interdisciplinary health professionals in palliative care, develop and disseminate curricula relating to palliative care; support the training and retraining of faculty; support continuing education; provide students with clinical training in appropriate sites of care; and provide traineeships for advanced practice nurses.

H.R. 3119 would also provide for training of physicians who plan to teach palliative medicine, academic career awards for junior medical faculty who commit to spend a majority of their funded time teaching and developing skills in interdisciplinary education in palliative care, and career incentive awards for other eligible health professionals who agree to teach or practice in the field of palliative care.

Finally, PCHETA would further provide supplemental training for faculty members in medical schools and other health professions schools (including pharmacy, nursing, social work, chaplaincy and other allied health disciplines in an accredited health professions school or program, such as a physician assistant education program) so healthcare providers who do not have formal training in palliative care can upgrade their knowledge and skills for the care of individuals with serious or life-threatening illness as well as enhance their interdisciplinary teaching skills.

Expanding Research to Improve Health Care Delivery

PCHETA also aims to strengthen clinical practice and improve health care delivery for patients living with serious or life-threatening illness, as well as their families, by directing funding toward palliative care research. Research funding for palliative care and pain and symptom management comprises less than 0.1 percent of the National Institutes of Health annual budget. From methods for improving communication and decision making to evidence-based treatments for relieving distressing

symptoms of serious illness such as fatigue, nausea, shortness of breath, pain, and confusion, PCHETA would direct an expansion and intensification of research in these important areas.

Raising Awareness

While building the workforce and research base for the field will address key barriers to accessing palliative care services, more must be done to ensure patients and providers are aware of the benefits of palliative care. According to the Institute of Medicine, there is a "need for better understanding of the role of palliative care among both the public and professionals across the continuum of care so that hospice and palliative care can achieve their full potential for patients and their families." PCHETA would direct the implementation of a national education and awareness campaign so that patients, families, and health professionals understand the essential role of palliative care in ensuring high-quality care for individuals facing serious or life threatening illness.

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AAHPM urges the Subcommittee to move swiftly to advance H.R. 3119 and improve the care of patients with serious or chronic conditions by expanding patient and family access to high-quality palliative care. AAHPM looks forward to working with the Subcommittee to improve public health and maximizing the contribution of hospice and palliative medicine physicians and the interdisciplinary palliative care team in that effort. Thank you again for taking the Academy's written comments into consideration. Please contact Jacqueline M. Kocinski, MPP, AAHPM Director of Health Policy and Government Relations, at jkocinski@aahpm.org if you have any questions.

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National Hospice and Palliative Care Organization

Hospice Action Network

Statement of Support for the Palliative Care and Hospice Education and Training Act (PCHETA)
National Hospice and Palliative Care Organization and Hospice Action Network
Before the House Energy and Commerce Committee
September 8, 2016

On behalf of the National Hospice and Palliative Care Organization (NHPCO), our 1,300 member organizations serving individuals in more than 3,800 locations nationwide, and the 1.6 million Americans served in hospice each year, we commend the Energy and Commerce Committee for its consideration of the Palliative Care and Hospice Education and Training Act (PCHETA). This important legislation will go a long way toward training the next generation of physicians and nurses who will care for many of us as we cope with serious illness and approach the end of our lives.

Hospice and palliative care are patient-centered models of care that take into account not only the patient's underlying disease state, but his or her emotional and spiritual needs, personal values and beliefs, care needs across settings, pain and symptoms, and family and caregiver needs. Hospice and palliative care professionals require a multi-dimensional set of skills, attitudes and competencies, including the ability to manage the myriad symptoms associated with advanced and comorbid illnesses; the ability to facilitate communication and problem solving between patients, their families and their care teams; and the ability to coordinate and provide team-based care across a range of settings.

A 2010 study published in the *Journal of Pain and Symptom Management* found a significant shortage in the number of adequately trained hospice and palliative care physicians and recommended an additional 6,000–18,000 physicians to meet existing hospice and palliative care needs. In the six years that have followed, that need has likely increased significantly. Further, palliative and end-of-life care must be better integrated into other specialties. A survey by the American Society of Clinical Oncology found that 65% of respondents felt that they had received inadequate education in controlling symptoms associated with cancer, and 81% felt they had inadequate mentoring in discussing a poor prognosis with their patients and families.

The Palliative Care and Hospice Education and Training Act will go a long way to correct these problems by supporting programs to provide clinical palliative medicine training in a variety of settings, including hospice, and developing specific measures to evaluate the competency of trainees. The Palliative Medicine and Hospice Academic Career Award program will enable hospice and palliative physicians to train members of interdisciplinary teams (IDTs) of healthcare professionals in palliative and hospice care techniques. This legislation will further expand the variety of professionals trained to provide hospice care, including nurses and clinical social workers. This program is especially important to the hospice community because of the reliance on the IDT to effectively care for patients.

As our health care delivery system prepares for the aging and decline of the 65 million baby boomers, it is imperative that we train physicians, nurses and other health care providers to better manage and deliver their care. We strongly support the Palliative Care and Hospice Education and Training Act, and encourage you to enact this important and much-needed legislation.

Oncology Nursing Society

Testimony for the Record in Support of
H.R. 3119, Palliative Care and Hospice Education and Training Act

Before the House Energy and Commerce Health Subcommittee
Hearing Titled
"Examining Legislation to Improve Public Health"

Thursday, September 8, 2016

Chairman Pitts, Ranking Member Green, and members of the Subcommittee, the Oncology Nursing Society (ONS) would like to thank the House Energy and Commerce Health Subcommittee for the opportunity to share ONS' views on H.R. 3119, the "Palliative Care and Hospice Education and Training Act" (PCHETA).

Our written testimony discusses the need to improve palliative care for patients with cancer or other serious or life-threatening illnesses. Oncology nurses have a pivotal role in offering palliative care for people living with a cancer diagnosis. Oncology nurses listen to what is important to patients as they assess how cancer and its treatment impact their physical, emotional, spiritual, and social well-being. Oncology nurses work with other health care providers to assist patients to manage symptoms, obtain community resources for care at home, and support patients in their final days with direct physical care at the end-of-life.

All patients with cancer can benefit from palliative care. Congress can improve access to palliative care by advancing the bipartisan PCHETA legislation introduced in the House by

Representative Eliot Engel and Representative Tom Reed and in the Senate by Senator Tammy Baldwin. ONS greatly appreciates the many members of the Energy and Commerce Committee who have demonstrated their support for this legislation by cosponsoring the bill.

PCHETA seeks to improve palliative care through education, awareness, and research. Not only do we need more trained interdisciplinary palliative care providers, but we need to expose more providers in general to palliative care training. The projected shortages of oncology providers are real and frightening. A shortage of oncologists will not only result in delays in treatment, but also delay conversations about goals of care. Increasingly, oncology nurses and primary care providers will need to be prepared to offer appropriate physical and behavioral symptom assessment and management and guide patients toward achieving their goals of care with effective communication skills and advanced care planning.

ONS applauds the committee for leading the way to realize the Cancer Moonshot and Precision Medicine initiatives through the passage last year of H.R. 6, the 21st Century Cures Act, and we hope Congress will soon complete its action on this legislation. However, health professionals must be ready to meet these needs. PCHETA will ensure we train more nurses, physicians, social workers, pharmacists and others in palliative care. Importantly, the bill follows the successful model of the geriatric education programs and ensures the training of teachers and that individuals are drawn to the field and retained.

Better awareness of palliative care by providers and the public also is necessary. PCHETA builds on existing authority at the Agency for Healthcare Research and Quality (AHRQ) and

provides for the establishment of a national campaign to inform patients, families, and health professionals about the benefits of palliative care and the services that are available to support patients with serious or life-threatening illness. The awareness campaign would include the dissemination of information, resources, and materials about palliative care services in a variety of formats. In planning for the campaign, AHRQ is required to consult with relevant stakeholders.

For most people, cancer will be a chronic illness that will require treatment and support through a journey that begins with a single step – usually a biopsy or an abnormal scan. Understanding that "wanting everything done" for a loved one will also need to include expert symptom management, emotional and spiritual support and assistance with identifying their "goals of care." To do this, there must be shared decision making in which there is a conversation with their providers about not only "what's wrong with you" but also "what matters to you"?

Additional research is needed to determine better ways to relieve suffering throughout the cancer care experience and at the end of life. This is especially true for symptoms such as shortness of breath, delirium, and pain control. Research demonstrates that palliative care works and its impact is measureable. It improves quality of life, results in less aggressive care at the end-of-life with fewer inpatient admissions and less ICU care, lowers emotional distress, and may in some cases actually prolong life. PCHETA, using existing authorities and funds, directs the National Institutes of Health (NIH) to expand national research programs to improve the delivery of palliative care to patients with serious illness. Oncology nurses are very active in palliative care research; the National Institute of Nursing Research funds much of the research in this area.

ONS views the promotion and improvement of cancer symptom management and pain control as a national priority for improving the care of persons with serious illness and their families. As both the medical literature and oncology nurses can attest to, far too often patients with serious illness experience unnecessary emergency department visits, hospitalization and re-hospitalizations, and other medical treatments because they lack the necessary integrated palliative care support needed in the setting of a serious illness.

Palliative care is integral to oncology and indicative of the importance of alleviating physical, psychological, social and spiritual pain and suffering. Oncology nurses stand at the front lines of cancer care and urge Congress to recognize the importance of better treatment and quality of life options, particularly for patients with a diagnosis of cancer. A proactive and integrated approach to palliative care, which incorporates a team-based approach that includes multiple health care providers, will help improve patients' quality of life across the care continuum.

The ONS has an important role in educating oncology nurses about palliative care and is actively educating its membership about emerging trends in palliative care.

The ONS is a professional organization of more than 37,000 registered nurses and other health care providers dedicated to excellence in patient care, education, research, and administration in oncology nursing. The ONS members are a diverse group of professionals representing a variety of professional roles, practice settings and subspecialty practice areas. Oncology nurses are leaders in health care committed to continuous learning and leading the transformation of cancer care by advocating for high-quality care for people with cancer.

The ONS is committed to maximizing the contribution that nurses -- the largest group of health care professionals -- have in reducing chronic illness. More importantly, the ONS is committed to maximizing the contribution of oncology nurses in palliative care and looks forward to working with the Subcommittee to advance this important patient-centered legislation.

September 1, 2016

The Honorable Eliot Engel
2161 Rayburn House Office Building
U.S. House of Representatives
Washington, DC 20515

The Honorable Tom Reed
1504 Longworth House Office Building
U.S. House of Representatives
Washington, DC 20515

Dear Representative Engel and Representative Reed:

The undersigned organizations write to express our support for H.R. 3119, the Palliative Care and Hospice Education and Training Act (PCHETA). This legislation will make a difference in the lives of millions of patients living with serious or life threatening illness and their caregivers.

Despite a high intensity of medical treatment, many seriously ill individuals still experience troubling symptoms, unmet psychological and personal care needs, fragmented care, poor communication with their health care providers, and enormous strains on their family caregivers. However, numerous studies have shown that adding palliative care can improve pain and symptom control, quality of life, and patient and family satisfaction.

Palliative care is an interdisciplinary model of care focused on relief of the pain, stress and other debilitating symptoms of serious illness, such as cancer, cardiac disease, respiratory disease, kidney failure, Alzheimer's, AIDS, ALS, and MS. Its goal is to relieve suffering and provide the best possible quality of life for patients and their families. Palliative care can be offered simultaneously with life-prolonging and curative therapies for persons living with serious, complex, and eventually terminal illness and includes hospice care. By its very nature, palliative care is patient-centered care — translating patient goals to appropriate treatments.

We appreciate your leadership in recognizing the significant role palliative care and hospice can play in creating lasting change across the health care system. With PCHETA's focus on expanding the palliative care workforce, promoting awareness of the benefits of palliative care among patient and providers, and improving the evidence base for this care, you have demonstrated a strong commitment to addressing key barriers to palliative care access for the growing number of Americans with serious or life-threatening illness.

Delivery of high-quality palliative care cannot take place without sufficient numbers of health care professionals with appropriate training and skills. Students graduating from medical school today have very little, if any, training in the core precepts of pain and symptom management, advance care planning, communication skills, and care coordination for patients with serious or life-threatening illness. Further, there is a large gap between the number of health care professionals with palliative care training and the number required to meet the needs of the expanding population of seriously ill patients. For example, 2010 estimates by the American Academy of Hospice and Palliative Medicine's Workforce Task Force calls for 6,000 or more full time equivalents to serve current needs in hospice and palliative care programs. At maximum capacity, however, the current system would produce only about 5,300 new hospice and palliative medicine certified physicians over the next 20 years. PCHETA would go a long way towards bridging this gap by establishing education centers and career incentive awards to improve the training of doctors, nurses, physician assistants, social workers and other health professionals in palliative care.

PCHETA also aims to strengthen clinical practice and improve health care delivery for patients living with serious or life-threatening illness, as well as their families, by directing funding toward palliative care

research. Research funding for palliative care and pain and symptom management comprises less than 0.1 percent of the National Institutes of Health annual budget. PCHETA would direct an expansion and intensification of research in these important areas.

At the same time, more must be done to ensure patients and providers are aware of the benefits of palliative care. According to the Institute of Medicine, there is a "need for better understanding of the role of palliative care among both the public and professionals across the continuum of care." PCHETA would direct the implementation of a national education and awareness campaign so that patients, families, and health professionals understand the essential role of palliative care in ensuring high-quality care for individuals facing serious or life threatening illness.

Thank you again for your support and leadership on this important issue. We look forward to working with you toward the passage of this legislation.

Sincerely,

Academy of Integrative Pain Management
Alzheimer's Association
American Academy of Hospice and Palliative Medicine
American Academy of Physician Assistants
American Cancer Society Cancer Action Network
American College of Surgeons Commission on Cancer
American Geriatrics Society
American Heart Association / American Stroke Association
American Psychosocial Oncology Society
American Society of Clinical Oncology
Association of Oncology Social Work
Association of Pediatric Hematology/Oncology Nurses
Association of Professional Chaplains
C-Change
California State University Institute for Palliative Care
Cambia Health Solutions
Cancer Support Community
Catholic Health Association of the United States
Center to Advance Palliative Care
Children's National Health System
Coalition for Compassionate Care of California
Colon Cancer Alliance
Courageous Parents Network
HealthCare Chaplaincy Network
Hospice and Palliative Nurses Association
Leukemia & Lymphoma Society
Lung Cancer Alliance
Motion Picture & Television Fund
National Alliance for Caregiving
National Association of Social Workers
National Coalition for Hospice and Palliative Care
National Coalition for Cancer Survivorship
National Hospice and Palliative Care Organization
National Palliative Care Research Center
Oncology Nursing Society
Partnership for Palliative Care
Pediatric Palliative Care Coalition
Physician Assistants in Hospice and Palliative Medicine
Social Work Hospice & Palliative Care Network
Society of Palliative Care Pharmacists
St. Baldrick's Foundation
Susan G. Komen
Supportive Care Coalition
Trinity Health
Visiting Nurse Associations of America

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Statement for the Record on Behalf of the
American Cancer Society Cancer Action Network

United States House of Representatives
Energy and Commerce Committee
Subcommittee on Health

Legislative Hearing: Examining Legislation to Improve Public Health

September 8, 2016

The American Cancer Society Cancer Action Network (ACS CAN) would like to thank Chairman Upton, Ranking Member Pallone, Chairman Pitts, Ranking Member Green, and Members of the Energy and Commerce Health Subcommittee for holding a hearing on *Examining Legislation to Improve Public Health*, that includes H.R. 3119, the Palliative Care Hospice Education and Training Act (PCHETA). We would also like to thank Congressman Eliot Engel and Tom Reed for their leadership and commitment as lead sponsors to move this important legislation forward.

ACS CAN, the nonprofit, nonpartisan advocacy affiliate of the American Cancer Society, supports evidence-based policy and legislative solutions designed to eliminate cancer as a major health problem. As the nation's leading advocate for public policies that are helping to defeat cancer, ACS CAN ensures that cancer patients, survivors, and their families have a voice in public policy matters at all levels of government.

During the 114th Congress, H.R. 3119 has garnered support from nearly 200 bi-partisan cosponsors in the House of Representatives, including over half of the full committee membership of the Energy and Commerce Committee. The legislation has also been endorsed by the Patient Quality of Life Coalition that was established in 2013 by ACS CAN, and has membership of over 40 patient, provider, and health system organizations.

H.R. 3119 would address three important public policy issues that have been identified as necessary to provide patients with serious illness better access to palliative care services throughout the continuum of their care: patient and provider education on palliative care as a delivery model of care for patients with serious illness; workforce development and training for health care providers on core competencies of palliative care; and expanded federal investment in palliative care research.

The goal of palliative care is to prevent and relieve suffering, and to support the best possible quality of life for patients and their families. Research suggests that palliative care should be made available to patients with serious illnesses upon diagnosis.[1]

Palliative care focuses on relief of the pain, symptoms, and stress of serious illness and on improving communication with patients and families. It is appropriate at any age and at any stage in a serious illness. Palliative care is provided by a team of providers that typically includes a palliative care doctor, nurse, social worker and a chaplain who work with the patient's other doctors to provide an extra layer of support for the patient and their family.

Evidence based research has concluded that patients with serious illness and their families receive poor-quality medical care that is characterized by inadequately treated symptoms, fragmented care, poor communication with health care providers, and enormous strains on family members or other caregivers.[2,3] By focusing on priorities that matter most to patients and their families, palliative care has been shown to improve both quality of care and quality of life for patients during and after treatment of a serious illness. In one study, patients with metastatic non-small-cell lung cancer who received palliative care services shortly after diagnosis enjoyed an improved quality of life compared to those who did not receive palliative care.[4] The American Heart Association / American Stroke Association have also stated that palliative care can be a helpful complement to current care practices and can improve quality of life for stroke patients, caregivers, and providers.[5]

Because their needs are met, patients receiving palliative care avoid crises, spend fewer days in the hospital, emergency department and intensive care unit, and have fewer re-admissions.[6] In fact, recent studies have demonstrated that high-quality palliative care not only improves quality of life and patient and family satisfaction, but it can also prolong survival.[7,8,9] Palliative care achieves these outcomes at a lower cost than usual by helping patients better understand their

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[1] Smith, TJ, Temin S, Alesi ER, Abernathy AP, Balboni TA, Basch EM, Ferrell BR, Loscalzo M, Meier DE, Paice JA, Peppercorn JM, Somerfield M, Stovall E, Von Roenn JH. American Society of Clinical Oncology Provisional Clinical Opinion: The Integration of Palliative Care Into Standard Oncology Care. J Clinical Oncol 2012; 30: 880-887.

[2] Teno JM, Clarridge BR, Casey V, Welch LC, Wetle T, Shield R, Mor V. Family perspectives on end-of-life care at the last place of care. JAMA. 2004 Jan 7; 291(1):88-93.

[3] Meier DE. Increased Access to Palliative Care and Hospice Services: Opportunities to Improve Value in Health Care. *The Milbank Quarterly*. 2011;89(3):343-380. doi:10.1111/j.1468-0009.2011.00632.x.

[4] Temel JS, Greer JA, Muzikansky A, et al. Early palliative care for patients with metastatic non-small-cell lung cancer. N Engl J Med. 2010;363:733-742.

[5] Palliative and End-of-Life Care in Stroke: A Statement for Healthcare Professionals From the American Heart Association/American Stroke Association http://stroke.ahajournals.org/content/early/2014/03/27/STR.0000000000000015 Mar 14.

[6] Meier DE. Increased Access to Palliative Care and Hospice Services: Opportunities to Improve Value in Health Care. *The Milbank Quarterly*. 2011;89(3):343-380. doi:10.1111/j.1468-0009.2011.00632.x.

[7] Temel JS, Greer JA, Muzikansky A, et al. Early palliative care for patients with metastatic non-small-cell lung cancer. N Engl J Med. 2010;363:733-742.

[8] Bakitas M, Lyons KD, Hegel MT, Balan S, Brokaw FC, Seville J, Hull JG, Li Z, Tosteson TD, Byock IR, Ahles TA Effects of a palliative care intervention on clinical outcomes in patients with advanced cancer: the Project ENABLE II randomized controlled trial. JAMA. 2009 Aug 19; 302(7):741-9.

[9] Connor SR, Pyenson B, Fitch K, Spence C, Iwasaki KJ Comparing hospice and non-hospice patient survival among patients who die within a three-year window. Pain Symptom Manage. 2007 Mar; 33(3):238-46.

needs, choose the most effective treatments and avoid unnecessary or unwanted hospitalizations and interventions.

In addition to important benefits to patient well-being and quality of life, palliative care has also been shown to reduce overall patient costs or to be cost neutral. One study of patients in Texas hospitals found that the provision of palliative care in the first 10 days after admission resulted in $9,689 savings per patient for those who died in the hospital and $2,696 savings per patient for those who were discharged alive.[10] A study of Medicaid patients in New York hospitals had similar findings, as the addition of palliative care resulted in $6,900 savings per patient – $7,563 per patient for those who died in the hospital and $4,098 per patient discharged alive.[11] The cost savings associated with palliative care in this study were estimated to save the New York Medicaid program an estimated $84-$252 million per year.[12] A study of patients in hospitals across multiple states also showed a $4,908 ($374/day) savings per patient for those who die in hospital and $1,696 ($279/day) savings per patient for those who were discharged alive.[13]

ACS CAN strongly supports H.R. 3119. If enacted the legislation would improve the lives of patients with serious illness such as cancer, and provide patients greater access to palliative care services that have been proven to provide patients greater quality of life, and positive health outcomes. The legislation accomplishes this goal through three main public policy priorities:

Patient and Provider Education

Despite the proven benefits of patient access to palliative care, many patients with serious illness who could benefit are unaware of the existence of palliative care services, or incorrectly equate palliative care with end-of-life or hospice care. H.R. 3119 would direct the Agency for Healthcare Research and Quality (AHRQ) to establish a national campaign to inform patients with serious illness, their caregivers, and providers about the benefits of palliative care services throughout the continuum of their care. A broad based campaign is necessary to appropriately define palliative care as care for patients with any serious illness that is made available throughout the continuum of their care, encourage patients and families to seek high quality palliative care early in the course of illness, and educate healthcare professionals as to the appropriate role of palliative care in the care of their patients.

Provider Education and Training Programs

Provider education is a critical issue that must be addressed before greater patient access to palliative care services can be achieved. There is currently a shortage of palliative care trained doctors. Palliative medicine is a relatively new medical subspecialty, recognized by the American Board of Medical Specialties only 15 years ago. Also, due to the caps placed on Graduate Medical Education in the 1997 Balanced Budget Act, as a practical matter specialty training in palliative medicine is essentially entirely dependent on private sector philanthropy.

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[10] McCarthy IM, Robinson C, Huq S, Philastre M, Fine RL, Cost savings from palliative care teams and guidance for a financially viable palliative care program, Health Serv Res. 2015 Feb;50(1):217-36, Epub 2014 Jul 15.

[11] Morrison RS, Dietrich J, Ladwig S, Quill T, Sacco J, Tangeman J, Meier DE., Palliative care consultation teams cut hospital costs for Medicaid beneficiaries, Health Aff (Millwood). 2011 Mar;30(3):454-63.

[12] Id.

[13] Morrison RS, Penrod JD, Cassel JB, Caust-Ellenbogen M, Litke A, Spragens L, Meier DE, Cost savings associated with US hospital palliative care consultation programs, Arch Intern Med. 2008 Sep 8; 168(16):1783-90.

Thus, palliative care training is not Medicare funded, unlike all other medical training in the United States. There is a grave need to train doctors in the medical subspecialty, as well as train all health care providers in core competencies of palliative care including pain and symptom management, psychosocial assessment and communication.

H.R. 3119 would establish an education and training program modeled after the successful Geriatric Education and Training Programs that currently exist. If enacted, education and training programs would support palliative care curriculum development in medical schools, as well as training programs in palliative care for all key healthcare professionals required to provide palliative care – doctors, nurses, social workers and other health care professionals.

Research

H.R. 3119 would also expand the federal investment in palliative care research at the National Institutes of Health (NIH). Historically, research focused on palliative care and symptom management has not been a priority across institutes at the NIH. A recent study found that less than one-one hundredth of one percent of the NIH budget is focused on improving quality of life in the setting of serious illness.[14] H.R. 3119 would address this important issue by requiring the Director of the NIH to develop and implement a strategy across all institutes within the NIH to expand and intensify research on palliative care and symptom management.

As the subcommittee examines ways that our current health care system can provide better quality, cost efficient care to patients with serious illness, we implore the subcommittee to closely examine the policy recommendations in H.R. 3119. On behalf of the millions of cancer patients, survivors and their families nationwide, thank you again to Chairman Upton, Ranking Member Pallone, Subcommittee Chairman Pitts, and Ranking Member Green for including H.R. 3119 in this important hearing on *Examining Legislation to Improve Public Health.* We look forward to continuing to work with the Energy and Commerce Committee to move this important legislation forward.

[14] Institute of Medicine. *Dying in America: Improving Quality and Honoring Individual Preferences Near the End-of-Life*. Washington D.C.: Institute of Medicine, 2015, available at http://nationalacademies.org/hmd/reports/2014/dying-in-america-improving-quality-and-honoring-individual-preferences-near-the-end-of-life.aspx.

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