116TH CONGRESS
2D SESSION

S. 4867

To direct the Secretary of Health and Human Services to support research on, and expanded access to, investigational drugs for amyotrophic lateral sclerosis, and for other purposes.

IN THE SENATE OF THE UNITED STATES

OCTOBER 26 (legislative day, OCTOBER 19), 2020

Mr. COONS (for himself and Ms. MURKOWSKI) introduced the following bill; which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

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A BILL

To direct the Secretary of Health and Human Services to support research on, and expanded access to, investigational drugs for amyotrophic lateral sclerosis, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Accelerating Access to Critical Therapies for ALS Act''.

SEC. 2. GRANTS FOR RESEARCH ON AND EXPANDED ACCESS TO EXPERIMENTAL THERAPIES FOR ALS.

(a) IN GENERAL.—The Secretary of Health and Human Services shall award grants to eligible entities for purposes of supporting research on, and expanded access for individuals to, investigational drugs for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis pursuant to an expanded access request submitted, and allowed to proceed by the Secretary, under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb).

(b) APPLICATION.—An eligible entity seeking a grant under this section shall submit to the Secretary an application at such time, in such manner, and containing such information as the Secretary shall specify. Such application shall specify a research objective relating to expanding access to investigational drugs (as described in subsection (a)) that would be supported by the award of such grant.

(c) SELECTION.—Not later than 90 days after the date of submission of an application for a grant under this section, the Secretary shall determine whether to award the grant, taking into consideration whether awarding such grant will support a research objective relating to expanding access to investigational drugs (as described in

subsection (a)), consistent with the mission of the National Institutes of Health.

(d) USE OF FUNDS.—An eligible entity may use funds received through the grant—

(1) to pay the manufacturer or sponsor for the direct costs of such drug (as authorized under section 312.8(d) of title 21, Code of Federal Regulations (or successor regulations)), if such costs are justified as part of peer review of the grant;

(2) for the entity's direct costs incurred in providing such drug consistent with the research mission of the grant; or

(3) for the direct and indirect costs of the entity in conducting research with respect to the drug involved.

(e) DEFINITIONS.—In this section:

(1) The term ''eligible entity'' means a participating clinical trial site or sites sponsored by a small business concern (as defined in section 3(a) of the Small Business Act (15 U.S.C. 632(a))) that is the sponsor of a drug that is the subject of an investigational new drug application under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)).

(2) The term ''participating clinical trial'' means a phase 3 clinical trial conducted pursuant to an exemption under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)) or section 351(a) of the Public Health Service Act (42 U.S.C. 262(a)) to investigate a drug intended to prevent, diagnose, mitigate, treat, or cure amyotrophic lateral sclerosis.

(3) The term ''participating clinical trial site'' means a nonprofit or public health care facility, or network of facilities, at which patients participating in a participating clinical trial receive an investigational drug through such trial.

SEC. 3. HHS COLLABORATIVE FOR NEURODEGENERATIVE DISEASES.

(a) ESTABLISHMENT.—Not later than one year after the date of the enactment of this Act, the Secretary of Health and Human Services shall establish and implement a Collaborative for Neurodegenerative Diseases between the National Institutes of Health and the Food and Drug Administration (to be known and referred to in this section as the ''Collaborative''), which shall—

(1) enter into a cooperative agreement, contract, or other instrument with a private entity or

entities under which such private entity or entities will operate the Collaborative;

(2) focus on advancing regulatory improvements and scientific research that will support and accelerate the development and approval of drugs for patients with amyotrophic lateral sclerosis;

(3) foster the development of effective drugs that improve the lives of people that suffer from rare neurodegenerative diseases; and

(4) share information with the Secretary to advance the purposes specified in paragraph (3), such as through carrying out the grant programs under sections 2 and 5 and developing the action plan under section 4.

(b) GIFTS.—

(1) IN GENERAL.—The Collaborative may solicit and accept gifts, grants, and other donations, establish accounts, and invest and expend funds in support of pre-competitive research and research associated with phase 3 and phase 4 clinical trials conducted with respect to investigational drugs that are the subjects of expanded access applications under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb).

(2) USE.—In addition to any amounts appropriated for purposes of carrying out this section, the Collaborative may use, without further appropriation, any funds derived from a gift, grant, or other donation accepted pursuant to paragraph (1).

(c) ADVISORY PANEL.—

(1) IN GENERAL.—The Collaborative shall convene an advisory panel to conduct a review of the design and implementation of the programs authorized by this Act. Such panel shall include representatives of patients, treating physicians, national organizations that facilitate provision of care services, researchers, drug sponsors, drug manufacturers, and Federal agencies.

(2) REPORT.—The advisory panel convened under paragraph (1) shall, not later than 18 months after the date of the enactment of this Act, submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the Senate a report that contains—

(A) the findings and conclusions of the review conducted under paragraph (1); and

(B) recommendations for carrying out the programs under this Act during the 2-year pe-

riod following the submission of such report, including recommendations relating to the inclusion of additional neurodegenerative diseases or disease areas within the grant programs under sections 2 and 5.

(d) DUTIES AND AUTHORITIES.—The Collaborative shall identify and implement strategies for the Secretary—

(1) for purposes of expediting the approval of drugs to treat amyotrophic lateral sclerosis, including through coordination among the centers of the Food and Drug Administration to achieve the goals specified in the draft guidance for drug sponsors entitled ''Amyotrophic Lateral Sclerosis: Developing Drugs for Treatment Guidance for Industry'' published in September 2019;

(2) to facilitate access to investigational drugs for amyotrophic lateral sclerosis pursuant to an expanded access request under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) in a similar manner as investigational drugs for cancer are provided through Project Facilitate of the Center of Excellence for Oncology;

(3) with respect to the rare neurodegenerative disease grant program established under section 5;

(4) to define or develop the regulatory and translational pathway for emerging therapeutic categories;

(5) to share, within the Collaborative, findings and insights related to pre-competitive research and research associated with phase 3 and phase 4 clinical trials conducted with respect to investigational drugs that are the subjects of expanded access applications under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb); and

(6) to develop and implement an ongoing mechanism to share feedback and information and develop strategies with the neurodegenerative disease community, including patients, treating physicians, national organizations that facilitate provision of care services, access, and research, researchers, drug sponsors, drug manufacturers, and Federal agencies.

SEC. 4. NEURODEGENERATIVE DISEASE ACTION PLAN.

(a) IN GENERAL.—Not later than 6 months after the date of the enactment of this Act, the Secretary of Health and Human Services shall publish an action plan describing actions the Department of Health and Human Services, through the National Institutes of Health and Food and Drug Administration, intend to take during the 5-year period following publication of the plan with respect to

program enhancements, policy development, regulatory science initiatives, and other appropriate initiatives to—

(1) foster the development of safe and effective drugs that improve the lives of people living with rare neurodegenerative diseases as quickly as possible; and

(2) facilitate access to investigational drugs.

(b) CONTENTS.—The action plan published under subsection (a) shall—

(1) identify appropriate representation from within the Food and Drug Administration and National Institutes of Health to be responsible for implementation of such action plan; and

(2) include elements to facilitate—

(A) interactions and collaboration between the Food and Drug Administration, including the review centers thereof, and stakeholders including patients, sponsors, and external biomedical community;

(B) consideration of cross-cutting clinical and regulatory policy issues, including consistency of regulatory advice and decision making;

(C) identification of key regulatory science and policy issues critical to advancing development of safe and effective drugs; and

(D) engagement by staff of the relevant centers of the Food and Drug Administration and other relevant offices of the Food and Drug Administration and National Institutes of Health with the designated leadership of the Collaborative.

SEC. 5. RARE NEURODEGENERATIVE DISEASE GRANT PROGRAM.

The Secretary of Health and Human Services, acting through the Commissioner of Food and Drugs, shall carry out a program of awarding grants to, and contracts entered into with, public and private entities to cover the costs of research on and development of interventions intended to prevent, diagnose, mitigate, treat, or cure amyotrophic lateral sclerosis and other life-threatening or severely debilitating neurodegenerative diseases, including costs incurred with respect to the development and critical evaluation of tools, methods, and processes—

(1) to characterize such neurodegenerative diseases and their natural history;

(2) to identify molecular targets for such neurodegenerative diseases; and

(3) to increase efficiency and productivity of clinical development of therapies, including advanc-

ing rational therapeutic development and working to establish clinical trial networks.

SEC. 6. AUTHORIZATION OF APPROPRIATIONS.

For purposes of carrying out this Act, there are authorized to be appropriated $100,000,000 for each of fiscal years 2022 through 2026.

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