

117TH CONGRESS
1ST SESSION

H. R. 3537

IN THE SENATE OF THE UNITED STATES

DECEMBER 9, 2021

Received

AN ACT

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.

1 *Be it enacted by the Senate and House of Representa-*
2 *tives of the United States of America in Congress assembled,*

1 **SECTION 1. SHORT TITLE.**

2 This Act may be cited as the “Accelerating Access
3 to Critical Therapies for ALS Act”.

4 **SEC. 2. GRANTS FOR RESEARCH ON THERAPIES FOR ALS.**

5 (a) IN GENERAL.—The Secretary of Health and
6 Human Services (referred to in this section as the “Sec-
7 retary”) shall award grants to participating entities for
8 purposes of scientific research utilizing data from ex-
9 panded access to investigational drugs for individuals who
10 are not otherwise eligible for clinical trials for the preven-
11 tion, diagnosis, mitigation, treatment, or cure of
12 amyotrophic lateral sclerosis. In the case of a participating
13 entity seeking such a grant, an expanded access request
14 must be submitted, and allowed to proceed by the Sec-
15 retary, under section 561 of the Federal Food, Drug, and
16 Cosmetic Act (21 U.S.C. 360bbb) and part 312 of title
17 21, Code of Federal Regulations (or any successor regula-
18 tions), before the application for such grant is submitted.

19 (b) APPLICATION.—

20 (1) IN GENERAL.—A participating entity seek-
21 ing a grant under this section shall submit to the
22 Secretary an application at such time, in such man-
23 ner, and containing such information as the Sec-
24 retary shall specify.

25 (2) USE OF DATA.—An application submitted
26 under paragraph (1) shall include a description of

1 how data generated through an expanded access re-
2 quest under section 561 of the Federal Food, Drug,
3 and Cosmetic Act (21 U.S.C. 360bbb) with respect
4 to the investigational drug involved will be used to
5 support research or development related to the pre-
6 vention, diagnosis, mitigation, treatment, or cure of
7 amyotrophic lateral sclerosis.

8 (3) NONINTERFERENCE WITH CLINICAL
9 TRIALS.—An application submitted under paragraph
10 (1) shall include a description of how the proposed
11 expanded access program will be designed so as not
12 to interfere with patient enrollment in ongoing clin-
13 ical trials for investigational therapies for the pre-
14 vention, diagnosis, mitigation, treatment, or cure of
15 amyotrophic lateral sclerosis.

16 (c) SELECTION.—Consistent with sections 406 and
17 492 of the Public Health Service Act (42 U.S.C. 284a,
18 289a), the Secretary shall, in determining whether to
19 award a grant under this section, confirm that—

20 (1) such grant will be used to support a sci-
21 entific research objective relating to the prevention,
22 diagnosis, mitigation, treatment, or cure of
23 amyotrophic lateral sclerosis (as described in sub-
24 section (a));

(2) such grant shall not have the effect of diminishing eligibility for, or impeding enrollment of, ongoing clinical trials for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis by determining that individuals who receive expanded access to investigational drugs through such a grant are not eligible for enrollment in—

(A) ongoing clinical trials that are registered on ClinicalTrials.gov (or successor website), with respect to a drug for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis; or

(B) clinical trials for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis for which an exemption under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)) has been granted by the Food and Drug Administration and which are expected to begin enrollment within one year; and

(3) the resulting project funded by such grant will allow for equitable access to investigational drugs by minority and underserved populations.

(d) USE OF FUNDS.—A participating entity shall use funds received through the grant—

1 (1) to pay the manufacturer or sponsor for the
2 direct costs of the investigational drug, as author-
3 ized under section 312.8(d) of title 21, Code of Fed-
4 eral Regulations (or successor regulations), to pre-
5 vent, diagnose, mitigate, treat, or cure amyotrophic
6 lateral sclerosis that is the subject of an expanded
7 access request described in subsection (a), if such
8 costs are justified as part of peer review of the
9 grant;

10 (2) for the entity's direct costs incurred in pro-
11 viding such drug consistent with the research mis-
12 sion of the grant; or

13 (3) for the direct and indirect costs of the enti-
14 ty in conducting research with respect to such drug.

15 (e) DEFINITIONS.—In this section:

16 (1) The term “participating entity” means a
17 participating clinical trial site or sites sponsored by
18 a small business concern (as defined in section 3(a)
19 of the Small Business Act (15 U.S.C. 632(a))) that
20 is the sponsor of a drug that is the subject of an in-
21 vestigational new drug application under section
22 505(i) of the Federal Food, Drug, and Cosmetic Act
23 (21 U.S.C. 355(i)) to prevent, diagnose, mitigate,
24 treat, or cure amyotrophic lateral sclerosis.

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

(f) SUNSET.—The Secretary may not award grants under this section on or after September 30, 2026.

16 SEC. 3. HHS PUBLIC-PRIVATE PARTNERSHIP FOR RARE
17 NEURODEGENERATIVE DISEASES.

(a) ESTABLISHMENT.—Not later than one year after the date of enactment of this Act, the Secretary of Health and Human Services (referred to in this section as the “Secretary”) shall establish and implement a Public-Private Partnership for Neurodegenerative Diseases between the National Institutes of Health, the Food and Drug Administration, and one or more eligible entities (to be known and referred to in this section as the “Partner-

1 ship”) through cooperative agreements, contracts, or other
2 appropriate mechanisms with such eligible entities, for the
3 purpose of advancing the understanding of
4 neurodegenerative diseases and fostering the development
5 of treatments for amyotrophic lateral sclerosis and other
6 rare neurodegenerative diseases. The Partnership shall—

7 (1) establish partnerships and consortia with
8 other public and private entities and individuals with
9 expertise in amyotrophic lateral sclerosis and other
10 rare neurodegenerative diseases for the purposes de-
11 scribed in this subsection;

12 (2) focus on advancing regulatory science and
13 scientific research that will support and accelerate
14 the development and review of drugs for patients
15 with amyotrophic lateral sclerosis and other rare
16 neurodegenerative diseases; and

17 (3) foster the development of effective drugs
18 that improve the lives of people that suffer from
19 amyotrophic lateral sclerosis and other rare
20 neurodegenerative diseases.

21 (b) ELIGIBLE ENTITY.—In this section, the term “el-
22 igible entity” means an entity that—

23 (1) is—

24 (A) an institution of higher education (as
25 such term is defined in section 1001 of the

1 Higher Education Act of 1965 (20 U.S.C.
2 1001)) or a consortium of such institutions; or

3 (B) an organization described in section
4 501(c)(3) of the Internal Revenue Code of 1986
5 and exempt from tax under subsection (a) of
6 such section;

7 (2) has experienced personnel with clinical and
8 other technical expertise in the field of biomedical
9 sciences and demonstrated connection to the patient
10 population;

11 (3) demonstrates to the Secretary's satisfaction
12 that the entity is capable of identifying and estab-
13 lishing collaborations between public and private en-
14 tities and individuals with expertise in
15 neurodegenerative diseases, including patients, in
16 order to facilitate—

17 (A) development and critical evaluation of
18 tools, methods, and processes—

19 (i) to characterize neurodegenerative
20 diseases and their natural history;

21 (ii) to identify molecular targets for
22 neurodegenerative diseases; and

23 (iii) to increase efficiency, predict-
24 ability, and productivity of clinical develop-
25 ment of therapies, including advancement

1 of rational therapeutic development and es-
2 tablishment of clinical trial networks; and
3 (B) securing funding for the Partnership
4 from Federal and non-Federal governmental
5 sources, foundations, and private individuals;
6 and

7 (4) provides an assurance that the entity will
8 not accept funding for a Partnership project from
9 any organization that manufactures or distributes
10 products regulated by the Food and Drug Adminis-
11 tration unless the entity provides assurances in its
12 agreement with the Secretary that the results of the
13 project will not be influenced by any source of fund-
14 ing.

15 (c) GIFTS.—

16 (1) IN GENERAL.—The Partnership may solicit
17 and accept gifts, grants, and other donations, estab-
18 lish accounts, and invest and expend funds in sup-
19 port of basic research and research associated with
20 phase 3 clinical trials conducted with respect to in-
21 vestigational drugs that are the subjects of expanded
22 access requests under section 561 of the Federal
23 Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb).

24 (2) USE.—In addition to any amounts appro-
25 priated for purposes of carrying out this section, the

1 Partnership may use, without further appropriation,
2 any funds derived from a gift, grant, or other dona-
3 tion accepted pursuant to paragraph (1).

4 **SEC. 4. ALS AND OTHER RARE NEURODEGENERATIVE DIS-**
5 **EASE ACTION PLAN.**

6 (a) IN GENERAL.—Not later than 6 months after the
7 date of enactment of this Act, the Commissioner of Food
8 and Drugs shall publish on the website of the Food and
9 Drug Administration an action plan describing actions the
10 Food and Drug Administration intends to take during the
11 5-year period following publication of the plan with respect
12 to program enhancements, policy development, regulatory
13 science initiatives, and other appropriate initiatives to—

14 (1) foster the development of safe and effective
15 drugs that improve or extend, or both, the lives of
16 people living with amyotrophic lateral sclerosis and
17 other rare neurodegenerative diseases; and

18 (2) facilitate access to investigational drugs for
19 amyotrophic lateral sclerosis and other rare
20 neurodegenerative diseases.

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

23 (1) identify appropriate representation from
24 within the Food and Drug Administration to be re-
25 sponsible for implementation of such action plan;

1 (2) include elements to facilitate—

2 (A) interactions and collaboration between
3 the Food and Drug Administration, including
4 the review centers thereof, and stakeholders in-
5 cluding patients, sponsors, and the external bio-
6 medical research community;

7 (B) consideration of cross-cutting clinical
8 and regulatory policy issues, including consist-
9 ency of regulatory advice and decisionmaking;

10 (C) identification of key regulatory science
11 and policy issues critical to advancing develop-
12 ment of safe and effective drugs; and

13 (D) enhancement of collaboration and en-
14 gagement of the relevant centers and offices of
15 the Food and Drug Administration with other
16 operating divisions within the Department of
17 Health and Human Services, the Partnership,
18 and the broader neurodegenerative disease com-
19 munity; and

20 (3) be subject to revision, as determined appro-
21 priate by the Secretary of Health and Human Serv-
22 ices.

1 **SEC. 5. FDA RARE NEURODEGENERATIVE DISEASE GRANT**
2 **PROGRAM.**

3 The Secretary of Health and Human Services, acting
4 through the Commissioner of Food and Drugs, shall
5 award grants and contracts to public and private entities
6 to cover the costs of research on, and development of inter-
7 ventions intended to prevent, diagnose, mitigate, treat, or
8 cure, amyotrophic lateral sclerosis and other rare
9 neurodegenerative diseases in adults and children, includ-
10 ing costs incurred with respect to the development and
11 critical evaluation of tools, methods, and processes—

12 (1) to characterize such neurodegenerative dis-
13 eases and their natural history;

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

16 (3) to increase efficiency and productivity of
17 clinical development of therapies, including
18 through—

19 (A) the use of master protocols and adapt-
20 ive and add-on clinical trial designs; and

21 (B) efforts to establish new or leverage ex-
22 isting clinical trial networks.

23 **SEC. 6. GAO REPORT.**

24 Not later than 4 years after the date of the enact-
25 ment of this Act, the Comptroller General of the United
26 States shall submit to the Committee on Energy and Com-

1 merce of the House of Representatives and the Committee
2 on Health, Education, Labor, and Pensions of the Senate
3 a report containing—

4 (1) with respect to grants awarded under the
5 program established under section 2—

6 (A) an analysis of what is known about the
7 impact of such grants on research or develop-
8 ment related to the prevention, diagnosis, miti-
9 gation, treatment, or cure of amyotrophic lat-
10 eral sclerosis; and

11 (B) data concerning such grants, includ-
12 ing—

13 (i) the number of grants awarded;

14 (ii) the participating entities to whom
15 grants were awarded;

16 (iii) the value of each such grant;

17 (iv) a description of the research each
18 such grant was used to further;

19 (v) the number of patients who re-
20 ceived expanded access to an investiga-
21 tional drug to prevent, diagnose, mitigate,
22 treat, or cure amyotrophic lateral sclerosis
23 under each grant;

24 (vi) whether the investigational drug
25 that was the subject of such a grant was

1 approved by the Food and Drug Adminis-
2 tration; and

3 (vii) the average number of days be-
4 tween when a grant application is sub-
5 mitted and when a grant is awarded; and

6 (2) with respect to grants awarded under the
7 program established under section 5—

8 (A) an analysis of what is known about the
9 impact of such grants on research or develop-
10 ment related to the prevention, diagnosis, miti-
11 gation, treatment, or cure of amyotrophic lat-
12 eral sclerosis;

13 (B) an analysis of what is known about
14 how such grants increased efficiency and pro-
15 ductivity of the clinical development of thera-
16 pies, including through the use of clinical trials
17 that operated with common master protocols, or
18 had adaptive or add-on clinical trial designs;
19 and

20 (C) data concerning such grants, includ-
21 ing—

22 (i) the number of grants awarded;

23 (ii) the participating entities to whom
24 grants were awarded;

25 (iii) the value of each such grant;

- 1 (iv) a description of the research each
2 such grant was used to further; and
3 (v) whether the investigational drug
4 that was the subject of such a grant re-
5 ceived approval by the Food and Drug Ad-
6 ministration.

7 **SEC. 7. AUTHORIZATION OF APPROPRIATIONS.**

8 For purposes of carrying out this Act, there are au-
9 thorized to be appropriated \$100,000,000 for each of fis-
10 cal years 2022 through 2026.

Passed the House of Representatives December 8,
2021.

Attest: CHERYL L. JOHNSON,
Clerk.