

117TH CONGRESS
1ST SESSION

H. R. 3537

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 HOUSE OF REPRESENTATIVES

MAY 25, 2021

Mr. QUIGLEY (for himself, Mr. FORTENBERRY, Mr. BRENDAN F. BOYLE of Pennsylvania, Mr. MOULTON, Mr. GARCIA of California, Mr. CARBAJAL, Mr. LARSON of Connecticut, Ms. JACKSON LEE, Ms. DEAN, Mr. SUOZZI, Ms. VELÁZQUEZ, Mr. LEVIN of California, Mr. DEUTCH, Ms. NORTON, Mr. TIMMONS, Mr. BRADY, Mr. MCKINLEY, Mr. VAN DREW, Mr. CALVERT, Mr. KEATING, Mr. DIAZ-BALART, Mr. CARTER of Georgia, Mrs. MCBATH, Mr. SMITH of Missouri, Mr. TURNER, Mr. DUNCAN, Mr. HICE of Georgia, Mr. YOUNG, Mr. SMITH of Nebraska, Mr. GROTHMAN, Mr. RUPPERSBERGER, Mr. RUTHERFORD, Mr. SCHWEIKERT, Mr. RYAN, Mr. CROW, Mr. GUTHRIE, Mr. FITZPATRICK, Ms. MCCOLLUM, Mr. AUSTIN SCOTT of Georgia, Mr. BAIRD, Mr. RODNEY DAVIS of Illinois, Mr. VALADAO, Mr. MOOLENAAR, Mr. MALINOWSKI, Ms. ROYBAL-ALLARD, Mr. PAYNE, Mr. LYNCH, Ms. HERRERA BEUTLER, Mr. BUCK, Mr. MULLIN, Mr. GRIJALVA, Mr. COOPER, Mr. PANETTA, Mr. KIM of New Jersey, Mr. SIRES, Ms. LEE of California, Ms. MOORE of Wisconsin, Ms. SCHAKOWSKY, Mr. THOMPSON of California, Mr. GALLEG0, Mrs. AXNE, Mrs. NAPOLITANO, Mr. ESPAILLAT, Ms. PRESSLEY, Mr. FLEISCHMANN, Mr. RESCHENTHALER, Mr. CICILLINE, Ms. DEGETTE, Mr. BURCHETT, Mr. LAMALFA, Ms. MENG, Ms. BROWNLEY, Mr. TRONE, Ms. KUSTER, Mr. CONNOLLY, Mr. MEEKS, Mrs. KIRKPATRICK, Mrs. DEMINGS, Mr. O'HALLERAN, Mr. LIEU, Mr. DESAULNIER, Mr. GARAMENDI, Mr. KILMER, Mr. RUSH, Mr. MCCAUL, Mr. MCCLINTOCK, Mr. MFUME, Mr. LAMB, Mr. GREEN of Texas, Mr. SWALWELL, Mr. GOTTHEIMER, Ms. PINGREE, Ms. KAPTUR, Mr. FERGUSON, Ms. SCANLON, Mr. BACON, Mr. WITTMAN, Mr. MORELLE, Mr. AMODEI, and Mr. WALTZ) introduced the following bill; which was referred to the Committee on Energy and Commerce

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.

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

3 **SECTION 1. SHORT TITLE.**

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

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

7 (a) IN GENERAL.—The Secretary of Health and
8 Human Services (referred to in this section as the Sec-
9 retary) shall award grants to participating entities for pur-
10 poses of expanded access for individuals to investigational
11 drugs for the prevention, diagnosis, mitigation, treatment,
12 or cure of amyotrophic lateral sclerosis. In the case of an
13 applicant seeking such a grant, an expanded access re-
14 quest must be submitted, and allowed to proceed by the
15 Secretary, under section 561 of the Federal Food, Drug,
16 and Cosmetic Act (21 U.S.C. 360bbb) and part 312 of
17 title 21, Code of Federal Regulations (or any successor
18 regulations), before the application for such grant is sub-
19 mitted.

20 (b) APPLICATION.—

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

6 (2) USE OF DATA.—An application submitted
7 under paragraph (1) shall include a description of
8 how data generated through an expanded access re-
9 quest under section 561 of the Federal Food, Drug,
10 and Cosmetic Act (21 U.S.C. 360bbb) with respect
11 to the investigational drug involved may be used by
12 the Secretary to support research or development re-
13 lated to the prevention, diagnosis, mitigation, treat-
14 ment, or cure of amyotrophic lateral sclerosis or
15 other rare neurodegenerative diseases.

16 (c) SELECTION.—Not later than 120 days after the
17 date of submission of an application for a grant under this
18 section, the Secretary shall determine whether to award
19 the grant, taking into consideration—

20 (1) whether awarding such grant will support a
21 research objective relating to expanding access to in-
22 vestigational drugs (as described in subsection (a));
23 and

1 (2) whether awarding such a grant may have
2 the effect of diminishing eligibility for, or impeding
3 enrollment of, ongoing clinical investigations.

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

6 (1) to pay the manufacturer or sponsor for the
7 direct costs of such drug (as authorized under sec-
8 tion 312.8(d) of title 21, Code of Federal Regula-
9 tions (or successor regulations)), if such costs are
10 justified as part of peer review of the grant;

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

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

17 (e) DEFINITIONS.—In this section:

18 (1) The term “participating entity” means a
19 participating clinical trial site or sites sponsored by
20 a small business concern (as defined in section 3(a)
21 of the Small Business Act (15 U.S.C. 632(a)) that
22 is the sponsor of a drug that is the subject of an in-
23 vestigational new drug application under section
24 505(i) of the Federal Food, Drug, and Cosmetic Act
25 (21 U.S.C. 355(i)).

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

9 (3) The term “participating clinical trial site”
10 means a nonprofit or public health care facility, or
11 network of facilities, at which patients participating
12 in a participating clinical trial receive an investiga-
13 tional drug through such trial.

14 **SEC. 3. HHS PUBLIC-PRIVATE PARTNERSHIP FOR RARE**
15 **NEURODEGENERATIVE DISEASES.**

16 (a) ESTABLISHMENT.—Not later than one year after
17 the date of enactment of this Act, the Secretary of Health
18 and Human Services (referred to in this section as the
19 “Secretary”) shall establish and implement a Public-Pri-
20 vate Partnership for Neurodegenerative Diseases between
21 the National Institutes of Health, the Food and Drug Ad-
22 ministration, and one or more eligible entities (to be
23 known and referred to in this section as the “Partner-
24 ship”) through cooperative agreements, contracts, or other
25 appropriate instruments with such eligible entities, for the

1 purpose of developing treatments for amyotrophic lateral
2 sclerosis and other rare neurodegenerative diseases. The
3 Partnership shall—

4 (1) establish partnerships, consortia, and col-
5 laborations with other public and private entities
6 and individuals with expertise in amyotrophic lateral
7 sclerosis and other rare neurodegenerative diseases
8 for the purposes described in this subsection;

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

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

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

20 (1) is—

21 (A) an institution of higher education (as
22 such term is defined in section 1001 of the
23 Higher Education Act of 1965 (20 U.S.C.
24 1001)) or a consortium of such institutions; or

1 (B) an organization described in section
2 501(c)(3) of the Internal Revenue Code of 1986
3 and exempt from tax under subsection (a) of
4 such section;

5 (2) has experienced personnel and demonstrated
6 connection to the patient population;

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

13 (A) development and critical evaluation of
14 tools, methods, and processes—

15 (i) to characterize neurodegenerative
16 diseases and their natural history;

17 (ii) to identify drug targets for
18 neurodegenerative diseases; and

19 (iii) to increase efficiency, predict-
20 ability, and productivity of clinical develop-
21 ment of therapies, including advancement
22 of rational therapeutic development and es-
23 tablishment of clinical trial networks; and

24 (B) securing funding for the Partnership
25 from Federal and non-Federal governmental

1 sources, foundations, and private individuals;
2 and

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

11 (c) GIFTS.—

12 (1) IN GENERAL.—The Partnership may solicit
13 and accept gifts, grants, and other donations, estab-
14 lish accounts, and invest and expend funds in sup-
15 port of pre-competitive research and research associ-
16 ated with phase 3 clinical trials conducted with re-
17 spect to investigational drugs that are the subjects
18 of expanded access applications under section 561 of
19 the Federal Food, Drug, and Cosmetic Act (21
20 U.S.C. 360bbb).

21 (2) USE.—In addition to any amounts appro-
22 priated for purposes of carrying out this section, the
23 Partnership may use, without further appropriation,
24 any funds derived from a gift, grant, or other dona-
25 tion accepted pursuant to paragraph (1).

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

3 (a) IN GENERAL.—Not later than 6 months after the
4 date of enactment of this Act, the Secretary of Health and
5 Human Services shall publish on the website of the De-
6 partment of Health and Human Services an action plan
7 describing actions the Food and Drug Administration in-
8 tends to take during the 5-year period following publica-
9 tion of the plan with respect to program enhancements,
10 policy development, regulatory science initiatives, and
11 other appropriate initiatives to—

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

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

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

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

25 (2) include elements to facilitate—

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

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

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

12 (D) enhancement of collaboration and en-
13 gagement by staff of the relevant centers of the
14 Food and Drug Administration and other rel-
15 evant offices of the Food and Drug Administra-
16 tion with other operating divisions within the
17 Department of Health and Human Services, the
18 Partnership, and the broader neurodegenerative
19 disease community; 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 shall
4 use funds made available under section 6 to award grants
5 and contracts to public and private entities to cover the
6 costs of research on, and development of interventions in-
7 tended to prevent, diagnose, mitigate, treat, or cure,
8 amyotrophic lateral sclerosis and other rare life-threat-
9 ening or severely debilitating neurodegenerative diseases
10 in adults and children, including costs incurred with re-
11 spect to the development and critical evaluation of tools,
12 methods, and processes—

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

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

17 (3) to increase efficiency and productivity of
18 clinical development of therapies, including advanc-
19 ing rational therapeutic development and working to
20 establish new or leverage existing clinical trial net-
21 works.

22 **SEC. 6. AUTHORIZATION OF APPROPRIATIONS.**

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

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