

116TH CONGRESS
2D SESSION

H. R. 8662

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

OCTOBER 23, 2020

Mr. FORTENBERRY (for himself, Mr. QUIGLEY, Mr. KING of New York, Mr. DIAZ-BALART, Mr. KEVIN HERN of Oklahoma, Mr. KATKO, Mr. GIANFORTE, Mr. HAGEDORN, Mr. BACON, Mr. FLEISCHMANN, Mrs. RODGERS of Washington, Mr. WESTERMAN, Mr. SMITH of Nebraska, Mr. BIGGS, Mr. MCKINLEY, Mr. SCHWEIKERT, Mr. UPTON, Mr. RUTHERFORD, Mr. MULLIN, Mr. KING of Iowa, Mr. AMODEI, Mr. MARSHALL, Mr. STIVERS, Mr. TIMMONS, Ms. VELÁZQUEZ, Ms. NORTON, Mr. COSTA, Mr. RUSH, Mr. ROUDA, Ms. SEWELL of Alabama, Mrs. BEATTY, Mr. O'HALLERAN, Mr. CISNEROS, Mr. LOWENTHAL, Mr. DAVID SCOTT of Georgia, Mr. CALVERT, Mr. WELCH, Mr. PETERSON, Mrs. DEMINGS, Ms. SCHAKOWSKY, Mr. SCHIFF, and Ms. BARRAGÁN) 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,*

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 AND EXPANDED AC-**
5 **CESS TO EXPERIMENTAL THERAPIES FOR**
6 **ALS.**

7 (a) IN GENERAL.—The Secretary of Health and
8 Human Services shall award grants to eligible entities for
9 purposes of supporting research on, and expanded access
10 for individuals to, investigational drugs for the prevention,
11 diagnosis, mitigation, treatment, or cure of amyotrophic
12 lateral sclerosis pursuant to an expanded access request
13 submitted, and allowed to proceed by the Secretary, under
14 section 561 of the Federal Food, Drug, and Cosmetic Act
15 (21 U.S.C. 360bbb).

16 (b) APPLICATION.—An eligible entity seeking a grant
17 under this section shall submit to the Secretary an appli-
18 cation at such time, in such manner, and containing such
19 information as the Secretary shall specify. Such applica-
20 tion shall specify a research objective relating to expand-
21 ing access to investigational drugs (as described in sub-
22 section (a)) that would be supported by the award of such
23 grant.

24 (c) SELECTION.—Not later than 90 days after the
25 date of submission of an application for a grant under this
26 section, the Secretary shall determine whether to award

1 the grant, taking into consideration whether awarding
2 such grant will support a research objective relating to ex-
3 panding access to investigational drugs (as described in
4 subsection (a)), consistent with the mission of the Na-
5 tional Institutes of Health.

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

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

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

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

19 (e) DEFINITIONS.—In this section:

20 (1) The term “eligible entity” means a partici-
21 pating clinical trial site or sites sponsored by a small
22 business concern (as defined in section 3(a) of the
23 Small Business Act (15 U.S.C. 632(a)) that is the
24 sponsor of a drug that is the subject of an investiga-
25 tional new drug application under section 505(i) of

1 the Federal Food, Drug, and Cosmetic Act (21
2 U.S.C. 355(i)).

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

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

16 **SEC. 3. HHS COLLABORATIVE FOR NEURODEGENERATIVE**
17 **DISEASES.**

18 (a) ESTABLISHMENT.—Not later than one year after
19 the date of the enactment of this Act, the Secretary of
20 Health and Human Services shall establish and implement
21 a Collaborative for Neurodegenerative Diseases between
22 the National Institutes of Health and the Food and Drug
23 Administration (to be known and referred to in this sec-
24 tion as the “Collaborative”), which shall—

1 (1) enter into a cooperative agreement, con-
2 tract, or other instrument with a private entity or
3 entities under which such private entity or entities
4 will operate the Collaborative;

5 (2) focus on advancing regulatory improvements
6 and scientific research that will support and accel-
7 erate the development and approval of drugs for pa-
8 tients with amyotrophic lateral sclerosis;

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

12 (4) share information with the Secretary to ad-
13 vance the purposes specified in paragraph (3), such
14 as through carrying out the grant programs under
15 sections 2 and 5 and developing the action plan
16 under section 4.

17 (b) GIFTS.—

18 (1) IN GENERAL.—The Collaborative may so-
19 licit and accept gifts, grants, and other donations,
20 establish accounts, and invest and expend funds in
21 support of pre-competitive research and research as-
22 sociated with phase 3 and phase 4 clinical trials con-
23 ducted with respect to investigational drugs that are
24 the subjects of expanded access applications under

1 section 561 of the Federal Food, Drug, and Cos-
2 metic Act (21 U.S.C. 360bbb).

3 (2) USE.—In addition to any amounts appro-
4 priated for purposes of carrying out this section, the
5 Collaborative may use, without further appropria-
6 tion, any funds derived from a gift, grant, or other
7 donation accepted pursuant to paragraph (1).

8 (c) ADVISORY PANEL.—

9 (1) IN GENERAL.—The Collaborative shall con-
10 vene an advisory panel to conduct a review of the de-
11 sign and implementation of the programs authorized
12 by this Act. Such panel shall include representatives
13 of patients, treating physicians, national organiza-
14 tions that facilitate provision of care services, re-
15 searchers, drug sponsors, drug manufacturers, and
16 Federal agencies.

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

24 (A) the findings and conclusions of the re-
25 view conducted under paragraph (1); and

1 (B) recommendations for carrying out the
2 programs under this Act during the 2-year pe-
3 riod following the submission of such report, in-
4 cluding recommendations relating to the inclu-
5 sion of additional neurodegenerative diseases or
6 disease areas within the grant programs under
7 sections 2 and 5.

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

10 (1) for purposes of expediting the approval of
11 drugs to treat amyotrophic lateral sclerosis, includ-
12 ing through coordination among the centers of the
13 Food and Drug Administration to achieve the goals
14 specified in the draft guidance for drug sponsors en-
15 titled “Amyotrophic Lateral Sclerosis: Developing
16 Drugs for Treatment Guidance for Industry” pub-
17 lished in September 2019;

18 (2) to facilitate access to investigational drugs
19 for amyotrophic lateral sclerosis pursuant to an ex-
20 panded access request under section 561 of the Fed-
21 eral Food, Drug, and Cosmetic Act (21 U.S.C.
22 360bbb) in a similar manner as investigational drugs
23 for cancer are provided through Project Facilitate of
24 the Center of Excellence for Oncology;

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

3 (4) to define or develop the regulatory and
4 translational pathway for emerging therapeutic cat-
5 egories;

6 (5) to share, within the Collaborative, findings
7 and insights related to pre-competitive research and
8 research associated with phase 3 and phase 4 clin-
9 ical trials conducted with respect to investigational
10 drugs that are the subjects of expanded access appli-
11 cations under section 561 of the Federal Food,
12 Drug, and Cosmetic Act (21 U.S.C. 360bbb); and

13 (6) to develop and implement an ongoing mech-
14 anism to share feedback and information and de-
15 velop strategies with the neurodegenerative disease
16 community, including patients, treating physicians,
17 national organizations that facilitate provision of
18 care services, access, and research, researchers, drug
19 sponsors, drug manufacturers, and Federal agencies.

20 **SEC. 4. NEURODEGENERATIVE DISEASE ACTION PLAN.**

21 (a) IN GENERAL.—Not later than 6 months after the
22 date of the enactment of this Act, the Secretary of Health
23 and Human Services shall publish an action plan describ-
24 ing actions the Department of Health and Human Serv-
25 ices, through the National Institutes of Health and Food

1 and Drug Administration, intend to take during the 5-year
2 period following publication of the plan with respect to
3 program enhancements, policy development, regulatory
4 science initiatives, and other appropriate initiatives to—

5 (1) foster the development of safe and effective
6 drugs that improve the lives of people living with
7 rare neurodegenerative diseases as quickly as pos-
8 sible; and

9 (2) facilitate access to investigational drugs.

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

12 (1) identify appropriate representation from
13 within the Food and Drug Administration and Na-
14 tional Institutes of Health to be responsible for im-
15 plementation of such action plan; and

16 (2) include elements to facilitate—

17 (A) interactions and collaboration between
18 the Food and Drug Administration, including
19 the review centers thereof, and stakeholders in-
20 cluding patients, sponsors, and external bio-
21 medical community;

22 (B) consideration of cross-cutting clinical
23 and regulatory policy issues, including consist-
24 ency of regulatory advice and decisionmaking;

1 (C) identification of key regulatory science
2 and policy issues critical to advancing develop-
3 ment of safe and effective drugs; and

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

10 **SEC. 5. RARE NEURODEGENERATIVE DISEASE GRANT PRO-**
11 **GRAM.**

12 The Secretary of Health and Human Services, acting
13 through the Commissioner of Food and Drugs, shall carry
14 out a program of awarding grants to, and contracts en-
15 tered into with, public and private entities to cover the
16 costs of research on and development of interventions in-
17 tended to prevent, diagnose, mitigate, treat, or cure
18 amyotrophic lateral sclerosis and other life-threatening or
19 severely debilitating neurodegenerative diseases, including
20 costs incurred with respect to the development and critical
21 evaluation of tools, methods, and processes—

22 (1) to characterize such neurodegenerative dis-
23 eases and their natural history;

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

1 (3) to increase efficiency and productivity of
2 clinical development of therapies, including advanc-
3 ing rational therapeutic development and working to
4 establish clinical trial networks.

5 **SEC. 6. AUTHORIZATION OF APPROPRIATIONS.**

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

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