

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.

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## 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.

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”.

1 **SEC. 2. GRANTS FOR RESEARCH ON AND EXPANDED AC-**  
2 **CESS TO EXPERIMENTAL THERAPIES FOR**  
3 **ALS.**

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

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

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

1 subsection (a)), consistent with the mission of the Na-  
2 tional Institutes of Health.

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

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

16 (e) DEFINITIONS.—In this section:

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

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

23           (1) enter into a cooperative agreement, con-  
24 tract, or other instrument with a private entity or

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

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

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

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

15 (b) GIFTS.—

16 (1) IN GENERAL.—The Collaborative may so-  
17 licit and accept gifts, grants, and other donations,  
18 establish accounts, and invest and expend funds in  
19 support of pre-competitive research and research as-  
20 sociated with phase 3 and phase 4 clinical trials con-  
21 ducted with respect to investigational drugs that are  
22 the subjects of expanded access applications under  
23 section 561 of the Federal Food, Drug, and Cos-  
24 metic Act (21 U.S.C. 360bbb).

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

6           (c) ADVISORY PANEL.—

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

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

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

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

1           riod following the submission of such report, in-  
2           cluding recommendations relating to the inclu-  
3           sion of additional neurodegenerative diseases or  
4           disease areas within the grant programs under  
5           sections 2 and 5.

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

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

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

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

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

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

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

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

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



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

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

7 (2) facilitate access to investigational drugs.

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

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

14 (2) include elements to facilitate—

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

20 (B) consideration of cross-cutting clinical  
21 and regulatory policy issues, including consist-  
22 ency of regulatory advice and decision making;

23 (C) identification of key regulatory science  
24 and policy issues critical to advancing develop-  
25 ment of safe and effective drugs; and

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

7 **SEC. 5. RARE NEURODEGENERATIVE DISEASE GRANT PRO-**  
8 **GRAM.**

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

19 (1) to characterize such neurodegenerative dis-  
20 eases and their natural history;

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

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

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

3 **SEC. 6. AUTHORIZATION OF APPROPRIATIONS.**

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

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