

.....  
(Original Signature of Member)

117TH CONGRESS  
1ST SESSION

**H. R.** \_\_\_\_\_

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

Mr. QUIGLEY introduced the following bill; which was referred to the Committee on \_\_\_\_\_

\_\_\_\_\_  
**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 THERAPIES FOR ALS.**

2 (a) IN GENERAL.—The Secretary of Health and  
3 Human Services shall award grants to participating enti-  
4 ties for purposes of expanded access for individuals to in-  
5 vestigational drugs for the prevention, diagnosis, mitiga-  
6 tion, treatment, or cure of amyotrophic lateral sclerosis.  
7 In the case of an applicant seeking such a grant, an ex-  
8 panded access request must be submitted, and allowed to  
9 proceed by the Secretary, under section 561 of the Federal  
10 Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) and  
11 part 312 of title 21, Code of Federal Regulations, before  
12 the application for such grant is submitted.

13 (b) APPLICATION.—

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

19 (2) USE OF DATA.—An application submitted  
20 under paragraph (1) shall include a description of  
21 how data generated through an expanded access re-  
22 quest under section 561 of the Federal Food, Drug,  
23 and Cosmetic Act (21 U.S.C. 360bbb) with respect  
24 to the investigational drug involved may be used by  
25 the Secretary to support research or development re-  
26 lated to the prevention, diagnosis, mitigation, treat-

1       ment, or cure of amyotrophic lateral sclerosis or  
2       other rare neurodegenerative diseases.

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

7           (1) whether awarding such grant will support a  
8       research objective relating to expanding access to in-  
9       vestigational drugs (as described in subsection (a));  
10      and

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

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

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

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

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

4           (e) DEFINITIONS.—In this section:

5           (1) The term “participating entity” means a  
6           participating clinical trial site or sites sponsored by  
7           a small business concern (as defined in section 3(a)  
8           of the Small Business Act (15 U.S.C. 632(a)) that  
9           is the sponsor of a drug that is the subject of an in-  
10          vestigational new drug application under section  
11          505(i) of the Federal Food, Drug, and Cosmetic Act  
12          (21 U.S.C. 355(i)).

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

21          (3) The term “participating clinical trial site”  
22          means a nonprofit or public health care facility, or  
23          network of facilities, at which patients participating  
24          in a participating clinical trial receive an investiga-  
25          tional drug through such trial.

1 **SEC. 3. HHS PUBLIC-PRIVATE PARTNERSHIP FOR RARE**  
2 **NEURODEGENERATIVE DISEASES.**

3 (a) ESTABLISHMENT.—Not later than one year after  
4 the date of the enactment of this Act, the Secretary of  
5 Health and Human Services shall establish and implement  
6 a Public-Private Partnership for Neurodegenerative Dis-  
7 eases between the National Institutes of Health, the Food  
8 and Drug Administration, and one or more eligible entities  
9 (to be known and referred to in this section as the “Part-  
10 nership”) through cooperative agreements, contracts, or  
11 other appropriate instruments with such eligible entities,  
12 for the purpose of developing treatments for amyotrophic  
13 lateral sclerosis and other rare neurodegenerative diseases.  
14 The Partnership shall—

15 (1) establish partnerships, consortia, and col-  
16 laborations with other public and private entities  
17 and individuals with expertise in amyotrophic lateral  
18 sclerosis and other rare neurodegenerative diseases  
19 for the purposes described in this subsection;

20 (2) focus on advancing regulatory science and  
21 scientific research that will support and accelerate  
22 the development and review of drugs for patients  
23 with amyotrophic lateral sclerosis and other rare  
24 neurodegenerative diseases; and

25 (3) foster the development of effective drugs  
26 that improve the lives of people that suffer from

1       amyotrophic lateral sclerosis and other rare  
2       neurodegenerative diseases.

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

5               (1) is—

6                       (A) an institution of higher education (as  
7                       such term is defined in section 1001 of the  
8                       Higher Education Act of 1965 (20 U.S.C.  
9                       1001)) or a consortium of such institutions; or

10                      (B) an organization described in section  
11                      501(c)(3) of the Internal Revenue Code of 1986  
12                      and exempt from tax under subsection (a) of  
13                      such section;

14               (2) has experienced personnel and demonstrated  
15       connection to the patient population;

16               (3) demonstrates to the Secretary’s satisfaction  
17       that the entity is capable of identifying and estab-  
18       lishing collaborations between public and private en-  
19       tities and individuals with expertise in  
20       neurodegenerative diseases, including patients, in  
21       order to facilitate—

22                      (A) development and critical evaluation of  
23                      tools, methods, and processes—

24                               (i) to characterize neurodegenerative  
25                               diseases and their natural history;

1 (ii) to identify drug targets for  
2 neurodegenerative diseases; and

3 (iii) to increase efficiency, predict-  
4 ability, and productivity of clinical develop-  
5 ment of therapies, including advancement  
6 of rational therapeutic development and es-  
7 tablishment of clinical trial networks; and

8 (B) securing funding for the Partnership  
9 from Federal and non-Federal governmental  
10 sources, foundations, and private individuals;  
11 and

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

20 (c) GIFTS.—

21 (1) IN GENERAL.—The Partnership may solicit  
22 and accept gifts, grants, and other donations, estab-  
23 lish accounts, and invest and expend funds in sup-  
24 port of pre-competitive research and research associ-  
25 ated with phase 3 clinical trials conducted with re-

1       spect to investigational drugs that are the subjects  
2       of expanded access applications under section 561 of  
3       the Federal Food, Drug, and Cosmetic Act (21  
4       U.S.C. 360bbb).

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

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

12       (a) IN GENERAL.—Not later than 6 months after the  
13       date of the enactment of this Act, the Secretary of Health  
14       and Human Services shall publish on the website of the  
15       Department of Health and Human Services an action plan  
16       describing actions the Food and Drug Administration in-  
17       tends to take during the 5-year period following publica-  
18       tion of the plan with respect to program enhancements,  
19       policy development, regulatory science initiatives, and  
20       other appropriate initiatives to—

21           (1) foster the development of safe and effective  
22       drugs that improve or extend, or both, the lives of  
23       people living with amyotrophic lateral sclerosis and  
24       other rare neurodegenerative diseases as quickly as  
25       possible; and

1           (2) facilitate access to investigational drugs for  
2           amyotrophic lateral sclerosis and other rare  
3           neurodegenerative diseases.

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

6           (1) identify appropriate representation from  
7           within the Food and Drug Administration to be re-  
8           sponsible for implementation of such action plan;  
9           and

10          (2) include elements to facilitate—

11           (A) interactions and collaboration between  
12           the Food and Drug Administration, including  
13           the review centers thereof, and stakeholders in-  
14           cluding patients, sponsors, and the external bio-  
15           medical research community;

16           (B) consideration of cross-cutting clinical  
17           and regulatory policy issues, including consist-  
18           ency of regulatory advice and decision-making;

19           (C) identification of key regulatory science  
20           and policy issues critical to advancing develop-  
21           ment of safe and effective drugs; and

22           (D) enhancement of collaboration and en-  
23           gagement by staff of the relevant centers of the  
24           Food and Drug Administration and other rel-  
25           evant offices of the Food and Drug Administra-

1           tion with other operating divisions within the  
2           Department of Health and Human Services, the  
3           Partnership, and the broader neurodegenerative  
4           disease community.

5           (3) be subject to revision, as determined appro-  
6           priate by the Secretary of Health and Human Serv-  
7           ices.

8   **SEC. 5. FDA RARE NEURODEGENERATIVE DISEASE GRANT**  
9                           **PROGRAM.**

10          The Secretary of Health and Human Services shall  
11          use funds made available under section 6 to award grants  
12          and contracts to 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 rare life-threat-  
16          ening or severely debilitating neurodegenerative diseases  
17          in adults and children, including costs incurred with re-  
18          spect to the development and critical evaluation of tools,  
19          methods, and processes—

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

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

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

1       ing rational therapeutic development and working to  
2       establish new or leverage existing clinical trial net-  
3       works.

4 **SEC. 6. AUTHORIZATION OF APPROPRIATIONS.**

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