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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. GALLEGO, 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.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Accelerating Access to Critical Therapies for ALS Act''.

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

(a) IN GENERAL.—The Secretary of Health and Human Services (referred to in this section as the Secretary) shall award grants to participating entities for purposes of expanded access for individuals to investigational drugs for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis. In the case of an applicant seeking such a grant, an expanded access request must be submitted, and allowed to proceed by the Secretary, under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) and part 312 of title 21, Code of Federal Regulations (or any successor regulations), before the application for such grant is submitted.

(b) APPLICATION.—

(1) IN GENERAL.—A participating entity seeking a grant under this section shall submit to the Secretary an application at such time, in such manner, and containing such information as the Secretary shall specify.

(2) USE OF DATA.—An application submitted under paragraph (1) shall include a description of how data generated through an expanded access request under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) with respect to the investigational drug involved may be used by the Secretary to support research or development related to the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis or other rare neurodegenerative diseases.

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

(1) whether awarding such grant will support a research objective relating to expanding access to investigational drugs (as described in subsection (a)); and

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

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

(1) to pay the manufacturer or sponsor for the direct costs of such drug (as authorized under section 312.8(d) of title 21, Code of Federal Regulations (or successor regulations)), if such costs are justified as part of peer review of the grant;

(2) for the entity's direct costs incurred in providing such drug consistent with the research mission of the grant; or

(3) for the direct and indirect costs of the entity in conducting research with respect to the drug involved.

(e) DEFINITIONS.—In this section:

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

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

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

SEC. 3. HHS PUBLIC-PRIVATE PARTNERSHIP FOR RARE 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 ''Partnership'') through cooperative agreements, contracts, or other appropriate instruments with such eligible entities, for the

purpose of developing treatments for amytrophic lateral sclerosis and other rare neurodegenerative diseases. The Partnership shall—

(1) establish partnerships, consortia, and collaborations with other public and private entities and individuals with expertise in amyotrophic lateral sclerosis and other rare neurodegenerative diseases for the purposes described in this subsection;

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

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

(b) ELIGIBLE ENTITY.—In this section, the term ''eligible entity'' means an entity that—

(1) is—

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

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

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

(3) demonstrates to the Secretary's satisfaction that the entity is capable of identifying and establishing collaborations between public and private entities and individuals with expertise in neurodegenerative diseases, including patients, in order to facilitate—

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

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

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

(iii) to increase efficiency, predictability, and productivity of clinical development of therapies, including advancement of rational therapeutic development and establishment of clinical trial networks; and

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

sources, foundations, and private individuals; and

(4) provides an assurance that the entity will not accept funding for a Partnership project from any organization that manufactures or distributes products regulated by the Food and Drug Administration unless the entity provides assurances in its agreement with the Secretary that the results of the project will not be influenced by any source of funding.

(c) GIFTS.—

(1) IN GENERAL.—The Partnership may solicit and accept gifts, grants, and other donations, establish accounts, and invest and expend funds in support of pre-competitive research and research associated with phase 3 clinical trials conducted with respect to investigational drugs that are the subjects of expanded access applications under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb).

(2) USE.—In addition to any amounts appropriated for purposes of carrying out this section, the Partnership may use, without further appropriation, any funds derived from a gift, grant, or other donation accepted pursuant to paragraph (1).

SEC. 4. ALS AND OTHER RARE NEURODEGENERATIVE DISEASE ACTION PLAN.

(a) IN GENERAL.—Not later than 6 months after the date of enactment of this Act, the Secretary of Health and Human Services shall publish on the website of the Department of Health and Human Services an action plan describing actions the Food and Drug Administration intends to take during the 5-year period following publication of the plan with respect to program enhancements, policy development, regulatory science initiatives, and other appropriate initiatives to—

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

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

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

(1) identify appropriate representation from within the Food and Drug Administration to be responsible for implementation of such action plan;

(2) include elements to facilitate—

(A) interactions and collaboration between the Food and Drug Administration, including the review centers thereof, and stakeholders including patients, sponsors, and the external biomedical research community;

(B) consideration of cross-cutting clinical and regulatory policy issues, including consistency of regulatory advice and decision making;

(C) identification of key regulatory science and policy issues critical to advancing development of safe and effective drugs; and

(D) enhancement of collaboration and engagement by staff of the relevant centers of the Food and Drug Administration and other relevant offices of the Food and Drug Administration with other operating divisions within the Department of Health and Human Services, the Partnership, and the broader neurodegenerative disease community; and

(3) be subject to revision, as determined appropriate by the Secretary of Health and Human Services.

SEC. 5. FDA RARE NEURODEGENERATIVE DISEASE GRANT PROGRAM.

The Secretary of Health and Human Services shall use funds made available under section 6 to award grants and contracts to public and private entities to cover the costs of research on, and development of interventions intended to prevent, diagnose, mitigate, treat, or cure, amyotrophic lateral sclerosis and other rare life-threatening or severely debilitating neurodegenerative diseases in adults and children, including costs incurred with respect to the development and critical evaluation of tools, methods, and processes—

(1) to characterize such neurodegenerative diseases and their natural history;

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

(3) to increase efficiency and productivity of clinical development of therapies, including advancing rational therapeutic development and working to establish new or leverage existing clinical trial networks.

SEC. 6. AUTHORIZATION OF APPROPRIATIONS.

For purposes of carrying out this Act, there are authorized to be appropriated $100,000,000 for each of fiscal years 2022 through 2026.

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