AMENDMENT IN THE NATURE OF A SUBSTITUTE TO H.R. 3537

OFFERED BY M__. _____

Strike all after the enacting clause and insert the following:

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 scientific research utilizing data from expanded access to investigational drugs for individuals who are not otherwise eligible for clinical trials for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis. In the case of a participating entity 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 will be used to support research or development related to the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis.

(3) NONINTERFERENCE WITH CLINICAL TRIALS.—An application submitted under paragraph (1) shall include a description of how the proposed expanded access program will be designed so as not to interfere with patient enrollment in ongoing clinical trials for investigational therapies for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis.

(c) SELECTION.—Consistent with sections 406 and 492 of the Public Health Service Act (42 U.S.C. 284a,

289a), the Secretary shall, in determining whether to award a grant under this section, confirm that—

(1) such grant will be used to support a scientific research objective relating to the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis (as described in subsection (a));

(2) such grant shall not have the effect of diminishing eligibility for, or impeding enrollment of, ongoing clinical trials for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis by determining that individuals who receive expanded access to investigational drugs through such a grant are not eligible for enrollment in—

(A) ongoing clinical trials that are registered on ClinicalTrials.gov (or successor website), with respect to a drug for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis; or

(B) clinical trials for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis for which an exemption under section 505(i) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(i)) has been granted by the Food and Drug

Administration and which are expected to begin enrollment within one year; and

(3) the resulting project funded by such grant will allow for equitable access to investigational drugs by minority and underserved populations.

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

(1) to pay the manufacturer or sponsor for the direct costs of the investigational drug, as authorized under section 312.8(d) of title 21, Code of Federal Regulations (or successor regulations), to prevent, diagnose, mitigate, treat, or cure amyotrophic lateral sclerosis that is the subject of an expanded access request described in subsection (a), 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 such drug.

(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)) to prevent, diagnose, mitigate, treat, or cure amyotrophic lateral sclerosis.

(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 health care facility, or network of facilities, at which patients participating in a participating clinical trial receive an investigational drug through such trial.

(f) SUNSET.—The Secretary may not award grants under this section on or after September 30, 2026.

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 mechanisms with such eligible entities, for the purpose of advancing the understanding of neurodegenerative diseases and fostering the development of treatments for amytrophic lateral sclerosis and other rare neurodegenerative diseases. The Partnership shall—

(1) establish partnerships and consortia 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 with clinical and other technical expertise in the field of biomedical sciences 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 molecular 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 sup-

port of basic research and research associated with phase 3 clinical trials conducted with respect to investigational drugs that are the subjects of expanded access requests 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 Commissioner of Food and Drugs shall publish on the website of the Food and Drug Administration 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; 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 of the relevant centers and 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, acting through the Commissioner of Food and Drugs, shall 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 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 through—

(A) the use of master protocols and adaptive and add-on clinical trial designs; and

(B) efforts to establish new or leverage existing clinical trial networks.

SEC. 6. GAO REPORT.

Not later than 4 years after the date of the enactment of this Act, the Comptroller General of the United States shall submit to the Committee on Energy and Commerce of the House of Representatives and the Committee on Health, Education, Labor, and Pensions of the Senate a report containing—

(1) with respect to grants awarded under the program established under section 2—

(A) an analysis of what is known about the impact of such grants on research or development related to the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis; and

(B) data concerning such grants, including—

(i) the number of grants awarded;

(ii) the participating entities to whom grants were awarded;

(iii) the value of each such grant;

(iv) a description of the research each such grant was used to further;

(v) the number of patients who received expanded access to an investigational drug to prevent, diagnose, mitigate, treat, or cure amyotrophic lateral sclerosis under each grant;

(vi) whether the investigational drug that was the subject of such a grant was approved by the Food and Drug Administration; and

(vii) the average number of days between when a grant application is submitted and when a grant is awarded; and

(2) with respect to grants awarded under the program established under section 5—

(A) an analysis of what is known about the impact of such grants on research or development related to the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis;

(B) an analysis of what is known about how such grants increased efficiency and productivity of the clinical development of therapies, including through the use of clinical trials that operated with common master protocols, or

had adaptive or add-on clinical trial designs; and

(C) data concerning such grants, including—

(i) the number of grants awarded;

(ii) the participating entities to whom grants were awarded;

(iii) the value of each such grant;

(iv) a description of the research each such grant was used to further; and

(v) whether the investigational drug that was the subject of such a grant received approval by the Food and Drug Administration.

SEC. 7. 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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