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117TH CONGRESS
1ST SESSION

H. R. 254

To amend the Public Health Service Act to create a National Neuromyelitis Optica Spectrum Disorder Consortium to provide grants, coordinate synergistic research, and targeted therapy with respect to the causes of, and risk factors associated with, neuromyelitis optica spectrum disorder, and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

JANUARY 11, 2021

Ms. LEE of California introduced the following bill; which was referred to the Committee on Energy and Commerce

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A BILL

To amend the Public Health Service Act to create a National Neuromyelitis Optica Spectrum Disorder Consortium to provide grants, coordinate synergistic research, and targeted therapy with respect to the causes of, and risk factors associated with, neuromyelitis optica spectrum disorder, 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 ''Neuromyelitis Optica Spectrum Disorder Consortium Act''.

SEC. 2. FINDINGS.

Congress finds the following:

(1) Neuromyelitis optica spectrum disorder (in this section and section 3 referred to as ''NMOSD'') is a devastating neurologic disease leading to blindness, paralysis, and premature death.

(2) There are an estimated 16,000 to 17,000 people with NMOSD in the United States and more than a quarter-million patients worldwide.

(3) Women are affected up to 7 times more than men, and Afro-Caribbeans and Latino persons are about 2.5 times more predisposed to NMOSD than Caucasians. The reasons why Blacks and Hispanics are disproportionately affected cannot be fully understood without further studies. Furthermore, why NMOSD disproportionately occurs in females is unknown.

(4) The average age at diagnosis is approximately 35 to 45 years, a peak window of time that further compounds the burden of NMOSD on parenthood and careers of women and men. The age range of NMOSD patients is broad and includes children as young as 3 years of age and adults as old as 90.

(5) NMOSD imposes substantial costs for affected patients and their families both in financial

costs such those associated with medical care, prescription medicines, and emergency room visits, as well as in opportunity costs such as its negative impact on maintaining gainful employment or attending school or career development programs.

(6) The origins of NMOSD are unknown, but it is hypothesized to be autoimmune in nature. Collectively, autoimmune diseases currently affect approximately 1 in 10 Americans. Without a clear understanding of the causes of NMOSD, development of cures that save and improve lives and reduce the substantial associated health care costs will not be possible.

(7) Despite the recent Food and Drug Administration approval of three medications for NMOSD, there remains an unmet need for more effective and safe therapies to spare these patients from this recurrent disease with its accumulating neurologic disability.

(8) Because of their relatively low overall incidence, orphan diseases like NMOSD frequently do not receive sufficient attention and research funding. Of special importance is the opportunity for the remarkable progress made recently regarding NMOSD to serve as—

(A) a model for solutions to rare and immunologic diseases; and

(B) an exemplary therapeutic disease target for immunosuppressive therapies and for determining vaccination benefits and risks relative to COVID–19.

(9) No single institution has a sufficient number of patients to independently conduct research that will adequately address the cause, prevention, treatment, and potential cure of NMOSD. Furthermore, there is a paucity of resources available for regenerative medicine research in NMOSD that will be required to repair optic nerve and spinal cord damage caused by NMOSD and thus to restore health.

(10) There has been no comprehensive study analyzing all relevant clinical, biological, and epidemiological aspects of NMOSD to identify potential risk factors and biomarkers for NMOSD.

(11) We can apply our understanding of NMOSD to the study of other autoimmune diseases, including type 1 diabetes mellitus, rheumatoid arthritis, psoriasis, multiple sclerosis, systemic lupus erythematosus, and many others.

SEC. 3. SENSE OF CONGRESS.

It is the sense of Congress that—

(1) there is a need to establish and coordinate a synergistic, multicenter research effort based on collaboration between regional consortia and governmental and nongovernmental entities in order to—

(A) comprehensively study the causes of NMOSD;

(B) identify potential biomarkers of disease activity;

(C) leverage recent efforts in developing approved therapies for NMOSD as a model for developing breakthrough therapies for other autoimmune diseases; and

(D) highlight NMOSD as a model disease to better understand the potential benefits and risks of immunosuppressive therapy and innovative vaccine strategies targeting COVID–19;

(2) there is a need to encourage a collaborative effort among academic medical centers comprising epidemiological study groups capable of gathering comprehensive and detailed information for each patient enrolled in those groups; and

(3) the effort referred to in paragraph (2) should facilitate investigation of environmental, nutritional, genetic, and treatment factors with respect

to the pathological and epidemiological characteristics of NMOSD.

SEC. 4. ESTABLISHMENT OF THE NATIONAL NEUROMYELITIS OPTICA SPECTRUM DISORDER CONSORTIUM.

Part B of title IV of the Public Health Service Act (42 U.S.C. 284 et seq.) is amended by adding after section 409J the following new section:

"SEC. 409K. NATIONAL NEUROMYELITIS OPTICA SPECTRUM DISORDER CONSORTIUM.

''(a) ESTABLISHMENT OF THE NATIONAL NEUROMYELITIS OPTICA SPECTRUM DISORDER CONSORTIUM.—

''(1) IN GENERAL.—Not later than 1 year after the date of the enactment of this section, the Secretary, acting through the Director of NIH, and in coordination with the Director of the National Institute on Minority Health and Health Disparities, shall establish, administer, and coordinate a National Neuromyelitis Optica Spectrum Disorder Consortium (in this section referred to as the 'NMOSD Consortium') for the purposes described in paragraph (2).

''(2) PURPOSES.—The purposes of the NMOSD Consortium shall be the following:

"(A) Providing grants of not less than 5-years' duration to eligible consortia for the purpose of conducting research with respect to the causes of, risk factors and biomarkers associated with, and treatment of and comorbidities associated with, NMOSD.

"(B) Assembling a panel of experts to provide, with respect to research funded by the NMOSD Consortium, ongoing guidance and recommendations for the development of the following:

"(i) A standardized study design, including adaptive clinical trial structures that may quickly and efficiently evaluate multiple treatment regimens to optimize precision and effectively assess personalized medicine in rare and immunologic diseases.

"(ii) Standard protocols, methods, procedures, and assays for collecting from individuals enrolled as study participants a minimum dataset that includes the following:

''(I) Complete medical history, including autoimmune and nonautoimmune comorbidities.

''(II) Neurologic examination and standardization of critical clinical outcomes such as the definition and adjudication of relapse in NMOSD.

''(III) Biospecimens, including serum, blood cells, cerebrospinal fluid, DNA, and RNA.

''(IV) Radiological data, including magnetic resonance imaging (MRI) and optical coherence tomography, among other modalities.

''(iii) Specific analytical methods for examining data, including bioinformatic and computational modeling for deterministic as well as predictive capabilities.

''(iv) Provisions for consensus review of enrolled cases, including clinical trial data as well as off-label drug use and epidemiologic studies that would be offer greater insights if considered in aggregate than alone.

"(v) An integrated data collection network, including registry and other activities that improve scientific and clinical efficiencies in achieving the purposes outlined in this paragraph.

"(C) Designating a consortium-dedicated laboratory to collect, analyze, and aggregate data with respect to research funded by the NMOSD Consortium and to make such data and analysis available to researchers.

"(3) ELIGIBLE CONSORTIA.—To be eligible for a grant under this section, a consortium shall demonstrate the following:

"(A) The consortium has the capability to enroll as research participants a minimum of 25 individuals with a diagnosis of NMO from the consortium's designated catchment area.

"(B) The designated catchment area of the consortium does not overlap with the designated catchment area of another consortium already receiving a grant under this section.

"(4) REPORT.—Not later than 1 year after the date of the enactment of this section, and annually thereafter, the Secretary, acting through the Director of NIH, shall submit to Congress a report with

respect to the NMOSD Consortium, to be made publicly available, including a summary of research funded by the NMOSD Consortium and a list of consortia receiving grants through the NMOSD Consortium. At the discretion of the Secretary, such report may be combined with other similar or existing reports.

''(5) AUTHORIZATION OF APPROPRIATIONS.—

''(A) IN GENERAL.—There is authorized to be appropriated $25,000,000 for each of fiscal years 2021 through 2024, to remain available until expended, to carry out this section.

''(B) SENSE OF CONGRESS.—It is the sense of Congress that funds appropriated to carry out this section should be in addition to funds otherwise available or appropriated to carry out the activities described in this section.

''(b) DEFINITIONS.—For purposes of this section:

''(1) CATCHMENT AREA.—The term 'catchment area' means a defined area for which population data are available.

''(2) CONSORTIUM.—The term 'consortium' means a partnership of two or more universities, health care organizations, or government agencies,

or any combination of such entities, serving a designated catchment area.''.

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