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

## 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.

- 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 "Neuromyelitis Optica
- 5 Spectrum Disorder Consortium Act".

## SEC. 2. FINDINGS.

2	Congress	finde	tha	$\mathbf{f}_{\mathbf{O}}$	win o.
<u> </u>	Congress	mus	une	TOHC	wmg:

- 3 (1) Neuromyelitis optica spectrum disorder (in 4 this section and section 3 referred to as "NMOSD") 5 is a devastating neurologic disease leading to blind-6 ness, 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—

1	(A) a model for solutions to rare and
2	immunologic diseases; and
3	(B) an exemplary therapeutic disease tar-
4	get for immunosuppressive therapies and for
5	determining vaccination benefits and risks rel-
6	ative to COVID-19.
7	(9) No single institution has a sufficient num-
8	ber of patients to independently conduct research
9	that will adequately address the cause, prevention,
10	treatment, and potential cure of NMOSD. Further-
11	more, there is a paucity of resources available for re-
12	generative medicine research in NMOSD that will be
13	required to repair optic nerve and spinal cord dam-
14	age caused by NMOSD and thus to restore health.
15	(10) There has been no comprehensive study
16	analyzing all relevant clinical, biological, and epide-
17	miological aspects of NMOSD to identify potential
18	risk factors and biomarkers for NMOSD.
19	(11) We can apply our understanding of
20	NMOSD to the study of other autoimmune diseases,
21	including type 1 diabetes mellitus, rheumatoid ar-
22	thritis, psoriasis, multiple sclerosis, systemic lupus
23	erythematosus, and many others.
24	SEC. 3. SENSE OF CONGRESS.
25	It is the sense of Congress that—

1	(1) there is a need to establish and coordinate
2	a synergistic, multicenter research effort based or
3	collaboration between regional consortia and govern-
4	mental and nongovernmental entities in order to—
5	(A) comprehensively study the causes of
6	NMOSD;
7	(B) identify potential biomarkers of disease
8	activity;
9	(C) leverage recent efforts in developing
10	approved therapies for NMOSD as a model for
11	developing breakthrough therapies for other
12	autoimmune diseases; and
13	(D) highlight NMOSD as a model disease
14	to better understand the potential benefits and
15	risks of immunosuppressive therapy and innova-
16	tive vaccine strategies targeting COVID-19;
17	(2) there is a need to encourage a collaborative
18	effort among academic medical centers comprising
19	epidemiological study groups capable of gathering
20	comprehensive and detailed information for each pa-
21	tient enrolled in those groups; and
22	(3) the effort referred to in paragraph (2)
23	should facilitate investigation of environmental, nu-
24	tritional genetic and treatment factors with respect

1	to the pathological and epidemiological characteris-
2	ties of NMOSD.
3	SEC. 4. ESTABLISHMENT OF THE NATIONAL
4	NEUROMYELITIS OPTICA SPECTRUM DIS-
5	ORDER CONSORTIUM.
6	Part B of title IV of the Public Health Service Act
7	(42 U.S.C. 284 et seq.) is amended by adding after section
8	409J the following new section:
9	"SEC. 409K. NATIONAL NEUROMYELITIS OPTICA SPECTRUM
10	DISORDER CONSORTIUM.
11	"(a) Establishment of the National
12	NEUROMYELITIS OPTICA SPECTRUM DISORDER CONSOR-
13	TIUM.—
14	"(1) IN GENERAL.—Not later than 1 year after
15	the date of the enactment of this section, the Sec-
16	retary, acting through the Director of NIH, and in
17	coordination with the Director of the National Insti-
18	tute on Minority Health and Health Disparities,
19	shall establish, administer, and coordinate a Na-
20	tional Neuromyelitis Optica Spectrum Disorder Con-
21	sortium (in this section referred to as the 'NMOSD
22	Consortium') for the purposes described in para-
23	graph (2).

 $\mbox{``(2)}$  Purposes.—The purposes of the NMOSD

Consortium shall be the following:

24

1	"(A) Providing grants of not less than 5-
2	years' duration to eligible consortia for the pur-
3	pose of conducting research with respect to the
4	causes of, risk factors and biomarkers associ-
5	ated with, and treatment of and comorbidities
6	associated with, NMOSD.
7	"(B) Assembling a panel of experts to pro-
8	vide, with respect to research funded by the
9	NMOSD Consortium, ongoing guidance and
10	recommendations for the development of the
11	following:
12	"(i) A standardized study design, in-
13	cluding adaptive clinical trial structures
14	that may quickly and efficiently evaluate
15	multiple treatment regimens to optimize
16	precision and effectively assess personal-
17	ized medicine in rare and immunologic dis-
18	eases.
19	"(ii) Standard protocols, methods,
20	procedures, and assays for collecting from
21	individuals enrolled as study participants a
22	minimum dataset that includes the fol-

lowing:

1	"(I) Complete medical history,
2	including autoimmune and nonauto-
3	immune comorbidities.
4	"(II) Neurologic examination and
5	standardization of critical clinical out-
6	comes such as the definition and adju-
7	dication of relapse in NMOSD.
8	"(III) Biospecimens, including
9	serum, blood cells, cerebrospinal fluid,
10	DNA, and RNA.
11	"(IV) Radiological data, includ-
12	ing magnetic resonance imaging
13	(MRI) and optical coherence tomog-
14	raphy, among other modalities.
15	"(iii) Specific analytical methods for
16	examining data, including bioinformatic
17	and computational modeling for deter-
18	ministic as well as predictive capabilities.
19	"(iv) Provisions for consensus review
20	of enrolled cases, including clinical trial
21	data as well as off-label drug use and epi-
22	demiologic studies that would be offer
23	greater insights if considered in aggregate
24	than alone.

1	"(v) An integrated data collection net-
2	work, including registry and other activi-
3	ties that improve scientific and clinical effi-
4	ciencies in achieving the purposes outlined
5	in this paragraph.
6	"(C) Designating a consortium-dedicated
7	laboratory to collect, analyze, and aggregate
8	data with respect to research funded by the
9	NMOSD Consortium and to make such data
10	and analysis available to researchers.
11	"(3) Eligible consortia.—To be eligible for
12	a grant under this section, a consortium shall dem-
13	onstrate the following:
14	"(A) The consortium has the capability to
15	enroll as research participants a minimum of 25
16	individuals with a diagnosis of NMO from the
17	consortium's designated catchment area.
18	"(B) The designated catchment area of the
19	consortium does not overlap with the designated
20	catchment area of another consortium already
21	receiving a grant under this section.
22	"(4) Report.—Not later than 1 year after the
23	date of the enactment of this section, and annually
24	thereafter, the Secretary, acting through the Direc-
25	tor of NIH, shall submit to Congress a report with

1	respect to the NMOSD Consortium, to be made pub-
2	licly available, including a summary of research
3	funded by the NMOSD Consortium and a list of
4	consortia receiving grants through the NMOSD Con-
5	sortium. At the discretion of the Secretary, such re-
6	port may be combined with other similar or existing
7	reports.
8	"(5) Authorization of appropriations.—
9	"(A) IN GENERAL.—There is authorized to
10	be appropriated \$25,000,000 for each of fiscal
11	years 2021 through 2024, to remain available
12	until expended, to carry out this section.
13	"(B) Sense of congress.—It is the
14	sense of Congress that funds appropriated to
15	carry out this section should be in addition to
16	funds otherwise available or appropriated to
17	carry out the activities described in this section.
18	"(b) Definitions.—For purposes of this section:
19	"(1) CATCHMENT AREA.—The term 'catchment
20	area' means a defined area for which population
21	data are available.
22	"(2) Consortium.—The term 'consortium'
23	means a partnership of two or more universities,

health care organizations, or government agencies,

- 1 or any combination of such entities, serving a des-
- 2 ignated catchment area.".

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