

116TH CONGRESS
1ST SESSION

H. R. 4228

To expand and improve the programs and activities of the Department of Health and Human Services for awareness, education, research, surveillance, diagnosis, and treatment concerning rare diseases and conditions.

IN THE HOUSE OF REPRESENTATIVES

SEPTEMBER 6, 2019

Mr. CARSON of Indiana (for himself, Mr. HUDSON, Ms. BROWNLEY of California, Mr. BUCSHON, Mr. DEFazio, Mr. DEUTCH, Mr. FITZPATRICK, Ms. JACKSON LEE, Mr. LUJÁN, Mrs. CAROLYN B. MALONEY of New York, Ms. MOORE, Mrs. MURPHY, Ms. NORTON, Mr. RASKIN, Mr. ROUDA, Mr. SUOZZI, and Mrs. WALORSKI) introduced the following bill; which was referred to the Committee on Energy and Commerce

A BILL

To expand and improve the programs and activities of the Department of Health and Human Services for awareness, education, research, surveillance, diagnosis, and treatment concerning rare diseases and conditions.

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 “Rare disease Advance-
5 ment, surveillance Research, and Education Act of 2019”
6 or the “RARE Act of 2019”.

1 **SEC. 2. NIH RARE DISEASE REGIONAL CENTERS OF EXCEL-**
2 **LENCE.**

3 Paragraph (1) of section 402A(a) of the Public
4 Health Service Act (42 U.S.C. 282a(a)) is amended by
5 adding at the end the following: “In addition to the
6 amounts authorized to be appropriated by the preceding
7 sentence, there are authorized to be appropriated such
8 sums as may be necessary for each of fiscal years 2020
9 through 2024 for carrying out section 481A (relating to
10 rare disease regional centers of excellence).”.

11 **SEC. 3. CDC SURVEILLANCE OF RARE DISEASES AND CON-**
12 **DITIONS.**

13 Title III of the Public Health Service Act is amended
14 by inserting after section 317T of such Act (42 U.S.C.
15 247b–22) the following:

16 **“SEC. 317U. CDC SURVEILLANCE OF RARE DISEASES AND**
17 **CONDITIONS.**

18 “(a) IN GENERAL.—The Secretary may, as appro-
19 priate—

20 “(1) enhance and expand infrastructure and ac-
21 tivities to track the epidemiology of up to 4 rare dis-
22 eases and conditions selected under subsection
23 (c)(1); and

24 “(2) incorporate information obtained through
25 such activities into an integrated surveillance system

1 to be known as the National Rare Disease or Condi-
2 tion Surveillance System.

3 “(b) RESEARCH.—The Secretary shall ensure that
4 the National Rare Disease or Condition Surveillance Sys-
5 tem is designed in a manner that provides information
6 that facilitates further research on rare diseases and con-
7 ditions.

8 “(c) CONTENT.—In carrying out subsection (a), the
9 Secretary—

10 “(1) shall select up to 4 rare diseases and con-
11 ditions that are determined by the Secretary to
12 have—

13 “(A) a high rate of mortality or morbidity;

14 or

15 “(B) potential for meaningful research and
16 treatment;

17 “(2) shall provide for the collection and storage
18 of information on the incidence and prevalence of
19 such rare diseases and conditions in the United
20 States;

21 “(3) to the extent practicable, shall provide for
22 the collection and storage of other available informa-
23 tion on such rare diseases and conditions, including
24 information related to persons living with such a
25 rare disease or condition who choose to participate

1 in surveillance activities, and including information
2 on—

3 “(A) demographics, such as age, race, eth-
4 nicity, sex, geographic location, family history,
5 and other information, as appropriate;

6 “(B) risk factors that may be associated
7 with the rare disease or condition, such as ge-
8 netic and environmental risk factors and other
9 information, as appropriate; and

10 “(C) diagnosis and progression markers;

11 “(4) to the extent practicable, shall provide for
12 the collection and storage of information relevant to
13 analysis on such rare diseases and conditions, such
14 as information concerning—

15 “(A) the natural history of the diseases
16 and conditions;

17 “(B) the prevention of the disease or con-
18 dition, including secondary diseases and condi-
19 tions;

20 “(C) the detection, management, and
21 treatment approaches for the diseases and con-
22 ditions; and

23 “(D) the development of outcomes meas-
24 ures; and

1 “(5) may address issues identified during the
2 consultation process under subsection (d).

3 “(d) CONSULTATION.—In carrying out this section,
4 the Secretary shall consult with individuals with appro-
5 priate expertise, which may include—

6 “(1) epidemiologists with experience in disease
7 surveillance;

8 “(2) representatives of national voluntary
9 health associations that—

10 “(A) focus on rare diseases or conditions;

11 and

12 “(B) have demonstrated experience in re-
13 search, care, or patient services;

14 “(3) health information technology experts or
15 other information management specialists;

16 “(4) clinicians with expertise in rare diseases or
17 conditions;

18 “(5) research scientists with expertise in rare
19 diseases or conditions, or experience conducting
20 translational research or utilizing surveillance sys-
21 tems for scientific research purposes; and

22 “(6) patients and caregivers of patients with
23 rare diseases or conditions.

24 “(e) GRANTS.—The Secretary may award grants to,
25 or enter into contracts or cooperative agreements with,

1 public or private nonprofit entities to carry out activities
2 under this section.

3 “(f) COORDINATION WITH OTHER FEDERAL, STATE,
4 AND LOCAL AGENCIES.—Subject to subsection (h), the
5 Secretary shall—

6 “(1) make information and analysis in the Na-
7 tional Rare Disease or Condition Surveillance Sys-
8 tem available, as appropriate—

9 “(A) to Federal departments and agencies,
10 such as the National Institutes of Health and
11 the Department of Veterans Affairs; and

12 “(B) to State and local agencies; and

13 “(2) identify, build upon, leverage, and coordi-
14 nate among existing data and surveillance systems,
15 surveys, registries, and other Federal public health
16 infrastructure, wherever practicable.

17 “(g) PUBLIC ACCESS.—Subject to subsection (h), the
18 Secretary shall ensure that information and analysis in the
19 National Rare Disease or Conditions Surveillance System
20 are available, as appropriate, to the public, including re-
21 searchers.

22 “(h) PRIVACY.—The Secretary shall ensure that in-
23 formation and analysis in the National Rare Disease or
24 Condition Surveillance System are made available only to
25 the extent permitted by applicable Federal and State law,

1 and in a manner that protects personal privacy, to the ex-
2 tent required by applicable Federal and State privacy law,
3 at a minimum.

4 “(i) SUPPLEMENT NOT SUPPLANT.—The activities
5 under this section may supplement, but shall not supplant,
6 any activities with respect to spina bifida, muscular dys-
7 trophy, or fragile X syndrome that are ongoing as of the
8 date of enactment of this section.

9 “(j) REPORTS.—

10 “(1) REPORT ON INFORMATION AND ANAL-
11 YSES.—Not later than 2 years after the date on
12 which any system is established under this section,
13 the Secretary shall submit an interim report to the
14 Committee on Health, Education, Labor, and Pen-
15 sions of the Senate and the Committee on Energy
16 and Commerce of the House of Representatives re-
17 garding aggregate information collected pursuant to
18 this section and epidemiological analyses, as appro-
19 priate. Such report shall be posted on the Internet
20 website of the Department of Health and Human
21 Services and shall be updated biennially.

22 “(2) IMPLEMENTATION REPORT.—Not later
23 than 4 years after the date of the enactment of this
24 section, the Secretary shall submit a report to the

1 Congress concerning the implementation of this sec-
2 tion. Such report shall include information on—

3 “(A) the development and maintenance of
4 the National Rare Disease or Condition Surveil-
5 lance System;

6 “(B) the type of information collected and
7 stored in the surveillance system;

8 “(C) the use and availability of such infor-
9 mation, including guidelines for such use; and

10 “(D) the use and coordination of databases
11 that collect or maintain information on rare dis-
12 eases or conditions.

13 “(k) DEFINITIONS.—In this section:

14 “(1) NATIONAL VOLUNTARY HEALTH ASSOCIA-
15 TION.—The term ‘national voluntary health associa-
16 tion’ means a national nonprofit organization with
17 chapters, other affiliated organizations, or networks
18 in States throughout the United States with experi-
19 ence serving the population of individuals with a
20 rare disease or condition and have demonstrated ex-
21 perience in rare disease or condition research, care,
22 and patient services.

23 “(2) RARE.—The term ‘rare’, with respect to a
24 disease or condition, means having a prevalence of
25 fewer than 200,000 individuals in the United States.

1 “(1) AUTHORIZATION OF APPROPRIATIONS.—To
2 carry out this section, there are authorized to be appro-
3 priated \$10,000,000 for each of fiscal years 2020 through
4 2025.”.

5 **SEC. 4. INCREASING HEALTH PROFESSIONALS’ AWARENESS**
6 **OF RARE DISEASES.**

7 (a) IN GENERAL.—The Director of the Agency for
8 Healthcare Research and Quality shall expand and inten-
9 sify the activities of the Agency to increase the awareness
10 and knowledge of health care providers about rare diseases
11 and conditions.

12 (b) DEFINITION.—In this section, the term “rare dis-
13 eases and conditions”, with respect to a disease or condi-
14 tion, means having a prevalence of fewer than 200,000
15 individuals in the United States.

16 **SEC. 5. REPORT.**

17 (a) IN GENERAL.—The Secretary of Health and
18 Human Services shall seek to enter into an arrangement
19 with the National Academies (or another appropriate enti-
20 ty if the National Academies decline) to update and repub-
21 lish, by not later than 3 years after the date of enactment
22 of this Act, the 2010 report of the National Academies
23 entitled “Rare Diseases and Orphan Products: Accel-
24 erating Research and Development”.

1 (b) AUTHORIZATION OF APPROPRIATIONS.—To carry
2 out this section, there is authorized to be appropriated
3 \$1,000,000.

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