

115TH CONGRESS
2D SESSION

H. R. 5115

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

FEBRUARY 27, 2018

Mr. CARSON of Indiana (for himself and Mr. COSTELLO of Pennsylvania) 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, Research, and Education Act of 2018” or the
6 “RARE Act of 2018”.

1 **SEC. 2. IMPROVING PROGRAMS AND ACTIVITIES OF HHS**
2 **CONCERNING RARE DISEASES AND CONDI-**
3 **TIONS.**

4 (a) NIH.—Paragraph (1) of section 402A(a) of the
5 Public Health Service Act (42 U.S.C. 282a(a)) is amended
6 by adding at the end the following:

7 “In addition to the amounts authorized to be appro-
8 priated by the preceding sentence, there is author-
9 ized to be appropriated \$10,000,000 for each of fis-
10 cal years 2019 through 2023 for carrying out sec-
11 tion 481A (relating to rare disease regional centers
12 of excellence).”.

13 (b) CDC SURVEILLANCE OF RARE DISEASES AND
14 CONDITIONS.—Title III of the Public Health Service Act
15 is amended by inserting after section 317T of such Act
16 (42 U.S.C. 247b–22) the following:

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

19 “(a) IN GENERAL.—The Secretary, acting through
20 the Director of the Centers for Disease Control and Pre-
21 vention and in coordination with other agencies as the Sec-
22 retary determines, shall, as appropriate—

23 “(1) enhance and expand infrastructure and ac-
24 tivities to track the epidemiology of rare diseases
25 and conditions; and

1 “(2) incorporate information obtained through
2 such activities into an integrated surveillance sys-
3 tem, which may consist of or include a registry, to
4 be known as the National Rare Disease or Condition
5 Surveillance System.

6 “(b) RESEARCH.—The Secretary shall ensure that
7 the National Rare Disease or Condition Surveillance Sys-
8 tem is designed in a manner that facilitates further re-
9 search on rare diseases and conditions.

10 “(c) CONTENT.—In carrying out subsection (a), the
11 Secretary—

12 “(1) shall provide for the collection and storage
13 of information on the incidence and prevalence of
14 rare diseases and conditions in the United States;

15 “(2) to the extent practicable, shall provide for
16 the collection and storage of other available informa-
17 tion on rare diseases and conditions, including infor-
18 mation related to persons living with a rare disease
19 or condition who choose to participate, such as—

20 “(A) demographics, such as age, race, eth-
21 nicity, sex, geographic location, family history,
22 and other information, as appropriate;

23 “(B) risk factors that may be associated
24 with a rare disease or condition, such as genetic

1 and environmental risk factors and other infor-
2 mation, as appropriate; and

3 “(C) diagnosis and progression markers;

4 “(3) to the extent practicable, shall provide for
5 the collection and storage of information relevant to
6 analysis on rare diseases and conditions, such as in-
7 formation concerning—

8 “(A) the natural history of the diseases
9 and conditions;

10 “(B) the prevention of the diseases and
11 conditions;

12 “(C) the detection, management, and
13 treatment approaches for the diseases and con-
14 ditions; and

15 “(D) the development of outcomes meas-
16 ures;

17 “(4) may address issues identified during the
18 consultation process under subsection (d); and

19 “(5) initially may address a limited number of
20 rare diseases or conditions.

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

24 “(1) epidemiologists with experience in disease
25 surveillance or registries;

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

3 “(A) focus on rare diseases or conditions;
4 and

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

7 “(3) health information technology experts or
8 other information management specialists;

9 “(4) clinicians with expertise in rare diseases or
10 conditions;

11 “(5) research scientists with expertise in rare
12 diseases or conditions, or experience conducting
13 translational research or utilizing surveillance sys-
14 tems for scientific research purposes; and

15 “(6) patients and caregivers of patients with
16 rare diseases or conditions.

17 “(e) GRANTS.—The Secretary may award grants to,
18 or enter into contracts or cooperative agreements with,
19 public or private nonprofit entities to carry out activities
20 under this section.

21 “(f) COORDINATION WITH OTHER FEDERAL, STATE,
22 AND LOCAL AGENCIES.—Subject to subsection (h), the
23 Secretary shall—

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

4 “(A) to Federal departments and agencies,
5 such as the National Institutes of Health and
6 the Department of Veterans Affairs; and

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

8 “(2) identify, build upon, leverage, and coordi-
9 nate among existing data and surveillance systems,
10 surveys, registries, and other Federal public health
11 infrastructure, wherever practicable.

12 “(g) PUBLIC ACCESS.—Subject to subsection (h), the
13 Secretary shall ensure that information and analysis in the
14 National Rare Disease or Conditions Surveillance System
15 are available, as appropriate, to the public, including re-
16 searchers.

17 “(h) PRIVACY.—The Secretary shall ensure that in-
18 formation and analysis in the National Rare Disease or
19 Condition Surveillance System are made available only to
20 the extent permitted by applicable Federal and State law,
21 and in a manner that protects personal privacy, to the ex-
22 tent required by applicable Federal and State privacy law,
23 at a minimum.

24 “(i) REPORTS.—

1 “(1) REPORT ON INFORMATION AND ANAL-
2 YSES.—Not later than 1 year after the date on
3 which any system is established under this section,
4 the Secretary shall submit an interim report to the
5 Committee on Health, Education, Labor, and Pen-
6 sions of the Senate and the Committee on Energy
7 and Commerce of the House of Representatives re-
8 garding aggregate information collected pursuant to
9 this section and epidemiological analyses, as appro-
10 pate. Such report shall be posted on the Internet
11 website of the Department of Health and Human
12 Services and shall be updated biennially.

13 “(2) IMPLEMENTATION REPORT.—Not later
14 than 4 years after the date of the enactment of this
15 section, the Secretary shall submit a report to the
16 Congress concerning the implementation of this sec-
17 tion. Such report shall include information on—

18 “(A) the development and maintenance of
19 the National Rare Disease or Condition Surveil-
20 lance System;

21 “(B) the type of information collected and
22 stored in the surveillance system;

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

1 “(D) the use and coordination of databases
2 that collect or maintain information on rare dis-
3 eases or conditions.

4 “(j) DEFINITIONS.—In this section:

5 “(1) NATIONAL VOLUNTARY HEALTH ASSOCIA-
6 TION.—The term ‘national voluntary health associa-
7 tion’ means a national nonprofit organization with
8 chapters, other affiliated organizations, or networks
9 in States throughout the United States with experi-
10 ence serving the population of individuals with a
11 rare disease or condition and have demonstrated ex-
12 perience in rare disease or condition research, care,
13 and patient services.

14 “(2) RARE.—The term ‘rare’, with respect to a
15 disease or condition, means affecting fewer than
16 200,000 individuals in the United States.

17 “(k) AUTHORIZATION OF APPROPRIATIONS.—To
18 carry out this section, there are authorized to be appro-
19 priated \$5,000,000 for each of fiscal years 2018 through
20 2023.”.

21 (c) AHRQ HEALTH CARE PROVIDER AWARENESS
22 ACTIVITIES.—

23 (1) IN GENERAL.—The Director of the Agency
24 for Healthcare Research and Quality shall expand
25 and intensify the activities of the Agency to increase

1 the awareness and knowledge of health care pro-
2 viders about rare diseases and conditions.

3 (2) DEFINITION.—In this subsection, the term
4 “rare diseases and conditions” refers to any disease
5 or condition affecting fewer than 200,000 individuals
6 in the United States.

7 (d) NATIONAL ACADEMIES REPORT.—

8 (1) IN GENERAL.—The Secretary of Health and
9 Human Services shall seek to enter into an arrange-
10 ment with the National Academies (or another ap-
11 propriate entity if the National Academies decline)
12 to update and republish, by not later than 3 years
13 after the date of enactment of this Act, the 2010 re-
14 port of the National Academies entitled “Rare Dis-
15 eases and Orphan Products: Accelerating Research
16 and Development”.

17 (2) AUTHORIZATION OF APPROPRIATIONS.—To
18 carry out this subsection, there is authorized to be
19 appropriated \$1,000,000.

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