

Rare disease Advancement, Research, and Education (RARE) Act

Rep. Carson (IN-D-07) & Rep. Hudson (NC-R-08)

BACKGROUND

Nearly one in ten Americans live with one or more of the roughly 7,000 known rare diseases. These largely inherited diseases – defined as affecting 200,000 or fewer people – often lack substantive research investments and treatment options. While past Congressional action has helped support research at the National Institutes of Health (NIH) and the Centers for Disease Control and Prevention (CDC), much more work needs to be done to help these agencies improve rare disease awareness, education, research, surveillance, diagnosis, and treatment.

The Problem: Few rare diseases are tracked and studied upon diagnosis and throughout the disease’s progression, leading to research challenges and data shortfalls that impede treatment development.

The Solution: Rep. Carson and Rep. Hudson are introducing the *Rare disease Advancement, Research, and Education (RARE) Act* to improve NIH and CDC’s ability to study rare diseases by improving treatment, research, and diagnostics of rare diseases through existing and new programs.

Funding: The *RARE Act* would authorize “as such sums are necessary” for expanding the Rare Diseases Clinical Research Network (RDCRN) at NIH. The bill would authorize \$10 million each fiscal year from FY2020 to FY2024 for the creation of the National Rare Disease or Condition Surveillance System to facilitate rare disease research by tracking diseases at CDC. The bill would also authorize \$1 million for the updating of the Rare Diseases and Orphan Products: Accelerating Research and Development report at the National Academies of Sciences, Engineering, and Medicine.

SECTION-BY-SECTION

1. Short Title

2. Research: Enhance Rare Diseases Clinical Research Network/Centers of Excellence

The *RARE Act* would expand an existing and successful program at NIH: the Rare Diseases Clinical Research Network (RDCRN). The RDCRN’s 21 research “centers of excellence” support the research and clinical trials of over 190 rare diseases and increase the availability of rare disease information.

Action: Amends the *Public Health Service Act* to increase the authorization for the RDCRN by “as such sums as may be necessary” for each fiscal year from FY2020 through FY2024.

3. Data: Create a Rare Disease Surveillance System at the CDC

By creating a new CDC surveillance system, the *RARE Act* would help fill critical gaps in tracking rare disease epidemiology and data and further help researchers understand commonalities between diseases.

Action: Amends the *Public Health Service Act* to require the CDC to create a National Rare Disease or Condition Surveillance System to facilitate rare disease research by tracking disease prevalence, demographics, and risk factors. Authorizes the Secretary to select up to 4 diseases for the new surveillance system that have high mortality, high morbidity, and the potential for meaningful research and treatment. Authorizes \$10 million for each fiscal year from FY2020 to FY2024.

4. Awareness: Increase Health Professionals’ Awareness of Rare Diseases

The *RARE Act* would increase health care providers’ awareness and knowledge of rare diseases through added engagement from the Agency for Healthcare Research and Quality (AHRQ).

Action: Amends the *Public Health Service Act* to require AHRQ to expand and intensify the agency’s activities to increase providers’ awareness and knowledge about rare diseases.

5. Policies: Require an Updated Report from National Academics of Sciences, Engineering and Medicine

Action: Requires that the National Academies of Sciences, Engineering, and Medicine update its 2010 report “Rare Diseases and Orphan Products: Accelerating Research and Development” in order to further evaluate rare disease efforts and make further recommendations to policymakers. Authorizes \$1 million to carry out this requirement.