The Helping Experts Acclerate Rare Treatments (HEART) Act of 2022

S. 4071

U.S. Senators Bob Casey and Tim Scott

Approximately 30 million Americans live with a rare disease. Unfortunately, treatment options for the 7,000 known rare diseases are scarce; only a few hundred rare diseases have approved treatments. Drugs intended to treat a disease affecting less than 200,000 Americans—so-called “orphan drugs”—face many hurdles, ranging from limited economic incentives to limited available study populations. The latter obstacle can make a new drug’s safety and efficacy assessment particularly difficult for the U.S. Food and Drug Administration (FDA). The FDA currently has multiple programs and efforts focused on rare diseases, including the Office of Orphan Products Development and the Rare Diseases Program to tackle these challenges. Still, the expertise needed to comprehensively evaluate new submissions for orphan drugs, particularly in the science of small population studies and in the specific diseases under consideration, is sometimes not systematically included in the review process. Additionally, patients and caregivers in the rare disease community often feel excluded and in the dark about FDA’s decision-making processes and considerations.

The HEART Act of 2022

The HEART Act aims to further strengthen the FDA review process for drugs that treat rare and ultra-rare diseases by increasing direct involvement of patients, doctors, and scientists with specialized expertise throughout the review process. The goal is to ensure that the agency is including the appropriate subject-matter experts in those regulatory decisions and enhance transparency into how the FDA makes regulatory decisions on drugs to treat ultra-rare conditions. The bill includes four key provisions:

- Requires the FDA to report annually on their activities related to orphan drugs;
- Commissions a National Academies study on FDA and European best practices for such drug review;
- Encourages consultation with patients and patient groups before and throughout the review process; and
- Promotes consultation with disease and small population study experts and their inclusion in advisory committee meetings.

Background Information

The HEART Act amends section 569 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb–8), “Consultation with external experts on rare diseases, targeted therapies, and genetic targeting of treatments,” which was added in 2012 by Public Law 112-144, the Food and Drug Administration Safety and Innovation Act (FDASIA), and has not been substantially revised since. While FDASIA implemented important changes to allow consultations, experience in the ensuing decade should inform updates and revisions to this statute.

Designing, conducting, and evaluating studies of treatments for very small populations, like those for ultra-rare diseases, is notoriously challenging. Patients with rare diseases also have unique perspectives and experiences that should be considered when selecting clinical endpoints, interpreting outcomes, and assessing benefit-risk tradeoffs. All warrant specialized expertise that is currently not consistently incorporated.

Congressmen Paul Tonko (D-NY-20) and David McKinley (R-WV-1) have introduced two similarly motivated but slightly differing companion bills in the House of Representatives, H.R. 1184 and H.R. 6888.

For more information, or to cosponsor, please contact Max Olender at Max_Olender@help.senate.gov.
Supporting Organizations
Haystack Project
  Usher 1F Collaborative
  Soft Bones
  Alagille Syndrome Alliance
  NBIA Disorders Association
  Tuberous Sclerosis Alliance
  Alpha-1 Foundation
  The National Ataxia Foundation
  American Porphyria Foundation
  Cutaneous Lymphoma Foundation
  International Foundation for CDKL5 Research
  The Siegel Rare Neuroimmune Association
  International Pemphigus and Pemphigoid Foundation
  CDG Care
  Usher Syndrome Coalition
  Cure Cadasil
  Global DARE Foundation
  ICAN, International Cancer Advocacy Network
  National Niemann-Pick Disease Foundation
  International Fibrodysplasia Ossificans Progressiva Association
  Kids With Heart National Association for Children’s Heart Disorders
  The FCS Foundation
  National Lipid Association Foundation

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