Advancing Targeted Therapies for Rare Diseases Act of 2015 (S 2030, 114th Congress)

Aims to allow sponsors of new drugs to reuse relevant data from previously approved drugs in their applications. Passage would expedite applications for drugs aimed to target and treat genetic diseases.

Updated last April 21, 2016 for the 09/15/2015 version of S 2030.

WHAT IT DOES

Data collection on new drug performance is time-intensive and costly. Often, genetically-targeted drugs function by similar mechanisms and therefore, collecting new information and data may be redundant. The overall goal of this bill is to facilitate an expedited approval process for new drugs that would treat rare, life threatening genetic diseases. Compared to collecting all new data, allowing researchers and companies to use existing data for products that target similar genes, or use similar technology, can enable a new product to be brought to market faster and more cost efficiently.

The bill aims to expedite the development, review and approval process for new genetically-targeted drugs that treat rare and serious, life-threatening diseases. S 2030 adds a new section to the Federal Food, Drug and Cosmetic Act, which allows sponsors of new drugs to use information and data collected on other, already approved and similarly functioning drugs to support a new drug application. The data may be used if:

- The data and information was collected by the same sponsor that is submitting the new application; or
- The new sponsor has permission from the sponsor of an approved drug to use their data and information.

RELEVANT SCIENCE

- In the United States, a Rare disease designation is given to a condition affecting less than 200,000 people.
- There are more than 6,800 rare diseases. Altogether, rare diseases affect an estimated 25 million to 30 million Americans.
- The exact cause for many rare diseases remains unknown. For a significant portion, the problem can be traced to mutations (changes) in a single gene. Such diseases are referred to as rare, genetic diseases. Many of these genetic mutations can be passed on from one generation to the next, explaining why certain rare diseases run in families. Environmental factors, such as diet, smoking, or exposure to chemicals, also can play a role in rare diseases. Such factors may directly cause disease, or interact with genetic factors to cause or increase the severity of disease.
- Examples of rare diseases caused by mutations in single genes include:
  - Cystic fibrosis, which affects the respiratory and digestive;
  - Huntington's disease, which affects the brain and nervous system; and
  - Muscular dystrophies, which affect the muscles.
- Single genes are also responsible for some rare, inherited types of cancer. Examples of these are the BRCA1 and BRCA2 genes, in which certain mutations increase the risk for hereditary breast and ovarian cancers.
- Rare diseases related to environmental factors include uncommon types of anemia caused by vitamin-deficient diets or certain medications.

ENDORSEMENTS & OPPOSITION

Endorsements:
This bill is widely supported by rare disease awareness and advocacy groups. A strong letter of support was written by the National Organization for Rare Diseases (NORD), representing a coalition of many advocacy organizations. NORD states: “This authority is critically important to accelerating the development of treatments and cures for the numerous devastating rare diseases or subsets of rare diseases that otherwise have little hope of a treatment or cure due to their extremely small population size.”

Opposition:

- As of April 18, 2016, there are no known formal letters of opposition against S 2030.

STATUS

This bill was assigned to the Senate committee on Health, Education, Labor and Pensions. On February 9, 2016, the Committee sent it to the full Senate for approval.

RELATED POLICIES

The Food, Drug and Cosmetic Act contains a rare pediatric disease priority review voucher program, which incents pharmaceutical companies to develop drugs to treat or prevent rare pediatric diseases. This program was set to last for one year. S 1878, the Advancing Hope Act of 2015 would extend and expand the program to include sickle cell disease and pediatric cancers.

SPONSORS

S 2030 is sponsored by Sen. Michael Bennet (D-CO). The three original cosponsors are Richard Burr (R-NC), Orrin Hatch (R-UT), and Elizabeth Warren (D-MA).

PRIMARY AUTHOR

Allison Roder

EDITOR(S)

Elizabeth Cirulli, PhD, Thomas Williams, JD, MBE & Aubrey Incorvaia, MPP

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