

# The Advancing Targeted Therapies for Rare Diseases Act of 2015

*Senators Michael Bennet, Richard Burr, Elizabeth Warren, & Orrin Hatch*

## *Opportunities for advancing treatments for rare diseases*

Many rare diseases have genetic origins, and different mutations within a gene can result in the same disease. This means that some rare diseases are further fragmented into genetic subtypes. Fortunately, advances in medicine have made it possible to design customized treatments—‘personalized,’ ‘precision,’ or ‘targeted’ medicines – that work by targeting the genetic mutation causing the disease. These advances hold tremendous potential for patients battling rare diseases, such as Duchenne Muscular Dystrophy, Cystic Fibrosis, and certain cancers.

## *Challenges in developing targeted therapies*

Targeted therapies can impact a disease-related gene in order to change its effect, or can interact with a variant protein resulting from a mutated gene to alter its function. When a targeted therapy is developed to bind directly to a genetic sequence, it will only work for that exact mutation. Some of these targeted therapies can be easily altered to bind to different mutations or sequences without altering the overall chemistry of the drug. When a targeted therapy is developed to interact with a variant protein, it may only work for patients with a specific mutation, or it may provide some efficacy treating individuals with other mutations in the same gene.

While developing drugs for rare diseases is inherently difficult because of the small patient population available to conduct clinical trials, it is even more challenging to conduct trials in specific genetic subgroups of a rare disease. Because of this, targeted therapies are generally first developed for patients with the most frequent mutations that cause a disease. However, to provide therapies for the full spectrum of certain genetic rare diseases, there would need to be dozens of targeted therapies.

## *Advancing Targeted Therapies for Rare Diseases Act of 2015*

The Advancing Targeted Therapies for Rare Diseases Act of 2015 (S. 2030) would affirm the Food and Drug Administration’s (FDA’s) current authority to allow innovators to rely upon their own data supporting the approval of a targeted therapy to help facilitate the development and review of subsequent targeted therapies, based on the same or similar targeted technology, to treat patients with the same rare disease as the previously approved targeted therapy.

The Advancing Targeted Therapies for Rare Diseases Act would not change the FDA’s current approval standards or conditions of approval. The Advancing Targeted Therapies for Rare Diseases Act would help to advance much needed therapies to patients with serious or life-threatening rare genetic diseases by providing clarity to innovators developing targeted therapies that address these patients’ needs.

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