

October xx, 2015

The Honorable Lamar Alexander, Chairman
Senate Committee on Health, Education,
Labor and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

The Honorable Patty Murray, Ranking Member
Senate Committee on Health, Education
Labor and Pensions
428 Dirksen Senate Office Building
Washington, D.C. 20510

Dear Chairman Alexander and Ranking Member Murray:

The undersigned organizations, representing millions of Americans living with a rare disease, write today to express our strong support for the *Advancing Targeted Therapies for Rare Diseases Act of 2015* (S. 2030). If enacted, this legislation could greatly accelerate the pace of therapeutic development for rare diseases and their sub-populations by streamlining the regulatory review process, without compromising safety and efficacy standards.

This legislation clarifies FDA's existing authority to leverage data previously used in the approval of a targeted drug when approving a new therapy that "incorporates or utilizes the same or similar genetically targeted technology, or the same variant protein targeted technology".

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. By clarifying that the FDA is allowed to use previously accepted data in certain circumstances, this provision will help facilitate the development and approval of therapies for these diseases.

This bill does not jeopardize intellectual property or patent protections, as only the sponsor that generated the original data may leverage that same data for future uses, unless the sponsor grants permission otherwise. Additionally, this legislation does not bind the FDA in its review processes, and instead reiterates existing flexibility in the data they are permitted to accept.

This bill is supported throughout the rare disease stakeholder community, and we urge the Senate Committee on Health, Education, Labor and Pensions to include this bill in the Senate Innovation for Healthier Americans Initiative.

Thank you for your continued dedication to the rare disease community, and we look forward to working with you to ensure this provision is enacted.

Sincerely,