DUCHENNE ALLIANCE

Senator Michael Bennet
261 Russell Senate Office Building
Washington, DC 20510

Senator Richard Burr
217 Russell Senate Office Building
Washington, DC 20510

Senator Elizabeth Warren
317 Hart Senate Office Building

Senator Orrin Hatch
104 Hart Senate Office Building
Washington, DC 20510

Dear Senators Bennet, Burr, Warren and Hatch:

On behalf of the Duchenne Alliance, I am writing to strongly support the *Advancing Targeted Therapies for Rare Diseases Act of 2015*. This legislation will serve to clarify that the Food and Drug Administration (FDA) has the authority to allow drug sponsors to utilize data and information collected in previous trials of a targeted therapy to help support the approval of new targeted therapies that incorporate or utilize the same or similar genetically targeted technologies.

The Duchenne Alliance is a group of independent non-profit organizations dedicated to defeating Duchenne muscular dystrophy. The Duchenne Alliance member foundations collaborate to co-identify, co-review, and co-fund the most promising biomedical research. They have established the Duchenne Alliance Research Fund (DARF) to streamline their approach and to develop and support other initiatives to assist patients suffering from DMD and their families.

While we believe the FDA currently has the authority to consider such information as part of its review and approval process, the *Advancing Targeted Therapies for Rare Diseases Act of 2015* will erase any doubts that the agency and sponsors may have regarding FDA authority. Further, we believe your legislation will ensure the agency understands that Congress has not only granted the FDA the authority to rely upon data from earlier trials in considering successor genetically targeted drugs and treatments, but that when it is appropriate, Congress would like the agency to do so.

Thank you for your continued efforts to support, promote and facilitate the development and approval of treatments for rare diseases. This legislation will provide further support for the

development of genetically targeted treatments for the Duchenne community, to drug companies and clinical trial sponsors, and to the entire rare disease community.

Sincerely,

Christine McSherry
Co-Founder
Duchenne Alliance

Alison Willis
Co-Founder
Duchenne Alliance