LEADING THE
FIGHT TO
END DUCHENNE

September 8, 2015

The Honorable Michael Bennet
Comm. On Health, Education, Labor & Pensions
S261 Russell Senate Office Building
Washington, DC 20510

The Honorable Elizabeth Warren
Comm. On Health, Education, Labor & Pensions
317 Hart Senate Office Building
Washington, DC 20515

The Honorable Richard Burr
Comm. on Health, Education, Labor & Pensions
217 Russell Senate Office Building
Washington, DC 20515

The Honorable Orrin Hatch
Comm. on Health, Education, Labor & Pensions
104 Hart Senate Office Building
Washington, DC 20510

Dear Senator Bennet, Senator Burr, Senator Warren, and Senator Hatch:

On behalf of Parent Project Muscular Dystrophy (PPMD) and the Duchenne muscular dystrophy (Duchenne) community, we are pleased to endorse the Advancing Targeted Drugs for Rare Diseases Act. With two rolling New Drug Applications (NDAs) for Duchenne candidate therapies submitted to the Food and Drug Administration (FDA), 2 Food and Drug Administration (FDA) Advisory Committee meetings anticipated this fall, and a robust therapeutic pipeline with other applications expected over the coming months, this legislation comes at a crucial time for the Duchenne community.

PPMD is strongly supportive of the bill aimed at clarifying FDA's discretionary authority to expedite development of targeted drugs for rare diseases for serious conditions by relying upon extrapolated data from previously approved drugs that use the same or similar approach. It is our expectation that this bill will enable sponsors developing therapies for Duchenne –and other devastating rare diseases and disorders with profound unmet medical needs – to leverage similar or closely related underlying technologies and/or data to accelerate the development pathway for additional targeted therapies. Statutory language giving the FDA the explicit authority to consider such data is necessary given the lack of clarity in current law and regulation. Without this clarity, industry sponsors may be unlikely to pursue development of therapies to treat a number of the more rare Duchenne mutations.

We are pleased to endorse this important legislation and look forward to working to support this critical effort going forward. Thank you for your innovation, leadership and effort.

Sincerely,

Pat Furlong
Founding President

Parent Project
Muscular Dystrophy

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