



August 10, 2015

Division of Dockets Management (HFA-305)  
U.S. Food and Drug Administration  
5630 Fishers Lane, Room 1061  
Rockville, MD 20852

**Re: Docket No. FDA-2015-D-1884: Duchenne Muscular Dystrophy and Related Dystrophinopathies: Developing Drugs for Treatment; Draft Guidance for Industry**

Dear Sir or Madam:

On behalf of the 30 million Americans with one of the approximately 7,000 known rare diseases, NORD would like to thank the Food and Drug Administration (FDA) for the opportunity to provide comments on the Agency's Draft Guidance titled, "Duchenne Muscular Dystrophy and Related Dystrophinopathies: Developing Drugs for Treatment; Draft Guidance for Industry".

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

While we do not have any specific comments on the information that is unique to Duchenne Muscular Dystrophy (DMD) and the Duchenne community contained within the Draft Guidance, we would like to applaud the FDA for undertaking this collaborative process with the Duchenne community.

We believe the process that the FDA and the Duchenne Community embarked on together can serve as a model for other rare and common disease communities in ensuring the patient voice is included within the FDA review processes. This Draft Guidance should undoubtedly assist industry in developing innovative therapies for DMD, and facilitate thorough and expedient FDA review of therapies for DMD due to a heightened understanding of the disease experience.

Finally, we encourage the FDA to move forward in codifying the process the FDA and Duchenne Community has undertaken in order for patient organizations and the rare disease communities they represent to proactively engage with the FDA in the development of similar Draft Guidances for other rare, neglected, and often misunderstood diseases. We hope this model will be reproduced and will become a common practice in FDA engagement with rare disease patient organizations.

We thank FDA for the opportunity to comment, and we look forward to working with the FDA on duplicating this model for other rare diseases and their patient organizations. For questions regarding NORD or the above comments, please contact Paul Melmeyer, Associate Director of Public Policy, at [pmelmeyer@rarediseases.org](mailto:pmelmeyer@rarediseases.org) or (202) 588-5700, ext. 104.

Thank you in advance for your consideration.

Sincerely,

A handwritten signature in cursive script, appearing to read "Peter L. Saltonstall". The signature is written in dark ink on a light-colored background.

Peter L. Saltonstall  
President and CEO