April 12, 2016

Dr. Janet Woodcock, M.D. Director, CDER
U.S. Food and Drug Administration
10903 New Hampshire Avenue Hillandale Building, 4th Floor
Silver Spring, MD 20993

Dear Dr. Woodcock,

Thank you for your ongoing commitment to the expeditious review of candidate therapies for Duchenne Muscular Dystrophy (DMD). In recent years, advancements in science have resulted in progress toward advancing the first-ever disease-modifying treatments for DMD, a goal we hope will be achieved soon.

As the FDA continues its review of potential new therapies for DMD, we urge the agency to utilize all available resources and authorities to accelerate the process of getting safe and effective treatments to patients diagnosed with this 100 percent fatal disease. The Food and Drug Administration Safety and Innovation Act (FDASIA) had a strong focus on accelerating the approval of drugs that treat unmet medical needs, prioritizing the patient perspective in evaluating new drugs and treatments and providing reviewers with flexibility when evaluating drugs for a life-threatening illness. We request you fully employ the tools Congress included in FDASIA and the broad regulatory flexibility the agency is granted through federal regulation[1] to help advance new DMD therapies.

The accelerated approval pathway outlined in Section 901(b) of FDASIA gives the agency the flexibility to grant approval to rare disease treatments that "have an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit," and allows the FDA to impose post-approval studies to confirm the clinical benefit. In FDA's draft Guidance, *Duchenne Muscular Dystrophy and Related Dystrophinopathies: Developing Drugs for Treatment Guidance for Industry*, the Agency expanded on this concept specifically in the context of DMD. We request that the agency consider surrogate endpoints and intermediate clinical endpoints to reduce the time and difficulty of performing clinical studies on treatments for rare diseases like DMD and help new therapies become accessible to patients who otherwise have no option as the agency has done with other deadly diseases such as HIV and cancer.

FDASIA includes multiple provisions focused on addressing the challenges of the rare disease patient community. The patient population of a rare disease is by definition small, meaning clinical trials will be conducted with fewer participants than trials for more prevalent conditions. We encourage the agency to utilize advances in regulatory science that can allow clinical trials in a small population able to provide the evidence necessary for accelerated approval of products that treat life-threatening, rare diseases.

[1] Code of Federal Regulations Title 21

FDASIA launched the Patient Focused Drug Development (PFDD) initiative and charged the agency to take into account the views and experiences of patients as part of the review process. As you know, the DMD community worked collaboratively with regulators and benefit-risk experts to ascertain patient-preference data, collect narratives from the community, and produce draft guidance that informed FDA's development of the draft *Duchenne Muscular Dystrophy and Related Dystrophinopathies: Developing Drugs for Treatment Guidance for Industry*. In addition, the experiences of patient representatives on the advisory committee and testimony of patients at the advisory committee meetings offer important perspectives and information. We urge the FDA to ensure that all of these perspectives are considered in regulatory review.

Patient perspectives are also important in conducting risk-benefit analyses. The FDA notes "that physicians and patients are generally willing to accept greater risks or side effects from products that treat life-threatening and severely debilitating illnesses, than they would accept from products that treat less serious illnesses" and "that the benefits of the drug need to be evaluated in light of the severity of the disease being treated." Under Title 21 regulations, it is appropriate for the FDA to exercise broad flexibility when reviewing drugs for certain disease types while ensuring safety and efficacy. We urge the FDA to ensure this flexibility is considered in the review of candidate therapies that meet regulatory requirements, while maintaining the rigor necessary to uphold safety and efficacy standards for new drugs.

The risk of doing nothing for a patient with DMD is their certain death. Treatments that are safe and reasonably likely to produce clinical benefit for DMD patients could meaningfully alter their lives.

Members of Congress remain committed to ensuring the FDA has the tools, authorities, and latitude necessary to review and approve safe and effective treatments for rare diseases as quickly as possible. We hope and expect that the agency will fully utilize these tools and authorities when appropriate to provide patients and physicians with new options to treat rare and deadly diseases like DMD.

We appreciate your prompt attention to this matter, and we look forward to hearing your reply. If you have any questions or need additional information please contact Sarah Lloyd Stevenson at 202-224-6253.

Sincerely,

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Roger F. Wicker
United States Senator

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Amy Klobuchar
United States Senator