January XX, 2015

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 use the tools and authorities of the Food and Drug Administration to expeditiously review candidate therapies for Duchenne muscular dystrophy. In recent years, Congress and the FDA have made tremendous progress toward achieving the goal of approving the first-ever disease-modifying treatments for Duchenne, an aim we hope will be achieved in early 2016.

As the FDA continues its review of a potential new therapy for Duchenne muscular dystrophy, we urge the agency to utilize all available tools, resources, and authorities to accelerate the process of delivering safe and effective treatments to patients diagnosed with this 100 percent fatal disease. In particular, we urge the agency to fully utilize its authorities and the tools Congress included in the Food and Drug Administration Safety and Innovation Act (FDASIA) to provide for new therapies intended to treat persons with life-threatening and severely-debilitating illnesses, especially when no satisfactory alternative therapy exists, as is the case in Duchenne.

The risk of doing nothing for a child with Duchenne is guaranteeing his or her death at a tragically young age. But the benefit of approving Duchenne therapies, seemingly efficacious drugs with clean safety profiles, could alter the lives of all Duchenne patients in a very positive way—by giving them a chance to live a longer, better life.

That is why we write to underscore the focus FDASIA has on accelerating the approval of drugs that treat unmet medical needs, prioritizing the patient perspective in evaluating new drugs and treatments, and providing regulators with flexibility when evaluating drugs for a life-threatening illness. The accelerated approval pathway outlined in Section 901 of FDASIA recognizes the limitations of developing drugs for rare diseases and gives the agency the flexibility to grant approval to rare disease treatments that have been shown to be safe and effective in fewer and smaller trials, while still requiring a larger confirmatory trial post-approval to confirm efficacy. This allows demonstrably safe therapies that treat an unmet medical need and appear to be efficacious, even with some uncertainty, to avoid the years of regulatory barriers and become accessible earlier to patients who otherwise have no other option.

Consistent with FDA regulations, we believe it is “appropriate [for the FDA] to exercise the broadest flexibility in applying the statutory standards, while preserving appropriate guarantees for safety and effectiveness” to new therapies intended to treat persons with life-threatening and severely-debilitating illnesses. As the FDA further notes, “the benefits of the drug need to be evaluated in light of the severity of the disease being treated.” As Members of Congress representing constituents battling Duchenne, we wholeheartedly agree with this viewpoint and urge the FDA to ensure this flexibility is applied in reviewing all Duchenne candidate therapies.

FDA has been successful at applying flexibility in oncology and HIV/AIDS to speed patient access to apparently safe treatments, and the need and opportunity to adopt innovative and flexible approaches to the review of rare disease drugs has never been greater than it is today. Patients are waiting.

Furthermore, it is critical the FDA take into account the views and experiences of patients as part of the review process. It is our understanding that the community worked collaboratively with regulators and benefit-risk experts to ascertain quantifiable patient-preference data, has collected narratives from a broad spectrum of the community that have been published and shared with the FDA, and produced a comprehensive draft guidance to industry that informed FDA’s development of its own draft guidance. We urge the FDA to consider the perspectives offered through these many Duchenne-specific patient-focused drug development tools, as well as the testimony and experiences of those in contact with the agency and your representatives, including patient representatives on the advisory committee and patients and expert clinicians who treat them as they testify during the open public hearing portion of the upcoming advisory committee meeting.

We remain committed to ensuring FDA has the tools, the authority, and the latitude necessary to speed treatments for rare disease to patients as quickly as possible, as was the intent expressed by Congress in passing FDASIA in 2012. We hope the agency will embrace the tools it has in order to provide patients and physicians with treatments for Duchenne.

Thank you for your attention to this important matter.

Sincerely,

Mike Fitzpatrick Bill Keating Pete King

Member of Congress Member of Congress Member of Congress