March 16, 2016

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

Dear Dr. Woodcock:

In 2012, Congress provided additional tools to facilitate new therapies intended to treat persons with life-threatening and severely-debilitating illnesses, especially when no satisfactory alternative exists.\(^1\) Thank you for your ongoing commitment to use these tools and authorities to expeditiously review candidate therapies. We write to urge the U.S. Food and Drug Administration (FDA) to continue exercising flexibility in the review and approval of all candidate therapies to better serve patients battling rare diseases with unmet medical needs.

The Committee on Homeland Security and Governmental Affairs recently held a hearing titled “Connecting Patients to New and Potential Life Saving Treatments.”\(^2\) During that hearing, one of the witnesses, Laura McLinn, testified about her experiences following her son’s diagnosis of Duchenne muscular dystrophy a few months before his fourth birthday.\(^3\) She explained that Duchenne is 100 percent fatal, effects 1 in 3,500 boys, and without a miracle, her son Jordan will lose the ability to walk, climb, dress himself, feed himself and even hug her. Ms. McLinn spoke of the promising treatments in the pipeline as well as the FDA’s ability to expedite review of therapies for “serious conditions” that either meet an “unmet medical need” or for which there is initial evidence of effectiveness.\(^4\) The hearing also highlighted the requirement that FDA “consider the perspectives of patients during regulatory discussions.”\(^5\)

We write today to underscore the focused efforts of Congress to provide for and encourage accelerated review of promising therapies, prioritize the patient perspective in evaluating new drugs and treatments, and provide regulators with flexibility to expedite evaluation of drugs for a life-threatening illness for not only Duchenne, but all rare and severe

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\(^2\) Connecting Patients to New and Potential Life Saving Treatments. Hearing before the Senate Committee on Homeland Security and Governmental Affairs, Feb. 25, 2016. 114th Cong.

\(^3\) Id.


\(^5\) 21 U.S.C. Sec. 360bbb.
diseases.\textsuperscript{6} One of the accelerated approval pathways in current law recognizes the inherent difficulty of developing drugs for rare diseases and therefore affords 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 post-approval confirmatory trial to confirm efficacy.\textsuperscript{7} This accelerated process allows demonstrably safe therapies that treat an unmet medical need to be made accessible earlier to patients who otherwise have no other option.

FDA regulations state that it is “appropriate 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.\textsuperscript{8} The regulations further explain that “the benefits of the drug need to be evaluated in light of the severity of the disease being treated.”\textsuperscript{9} As Members of Congress representing constituents who are battling rare and severe diseases with unmet medical needs, we wholeheartedly agree with this viewpoint and we urge the FDA to ensure this flexibility is applied in reviewing all candidate therapies.

We also urge the FDA to continue to develop and implement its Patient-Focused Drug Development initiative.\textsuperscript{10} This initiative will better incorporate the perspective of patients and other stakeholders into agency processes and decision-making, especially with respect to the agency’s proper contextual assessment of expected benefits and costs.

The cost of unnecessary delays manifests in terms of human lives and therefore the urgency on this matter, to patients and their families, is absolute. Thank you for your attention to this important matter.

Sincerely,

Ron Johnson
Chairman
Committee on Homeland Security and Governmental Affairs

Tom Carper
Ranking Member
Committee on Homeland Security and Governmental Affairs


\textsuperscript{7} Supra, note 1, section 901.

\textsuperscript{8} 21 C.F.R. § 312.80.

\textsuperscript{9} Id.
