

Congress of the United States

Washington, DC 20510

May 2, 2023

The Honorable Robert M. Califf
Commissioner
U.S. Food and Drug Administration
10903 New Hampshire Ave.
Silver Spring, MD 20903

Dear Commissioner Califf,

We write to request your timely attention to a matter of critical importance to the Duchenne muscular dystrophy community, and to the patient community more broadly. More specifically, we write to ask that you ensure fulsome public input, especially from the patient and clinical community, during an upcoming Advisory Committee (“AdCom”) meeting to be held by the U.S. Food and Drug Administration (FDA).

The FDA recently announced plans to hold a Cellular, Tissue and Gene Therapies AdCom for the SRP-9001 (delandistrogene moxeparvovec) biologics license application on May 12, 2023.¹ SRP-9001 is an investigational gene therapy for the treatment of Duchenne muscular dystrophy. Given that this is a first-of-its-kind therapy for Duchenne muscular dystrophy, there is great interest from the patient and clinical community in participating in this AdCom meeting.

According to press reports, the AdCom meeting will be hosted as a *virtual* meeting, as opposed to an in-person meeting.² It is also our understanding that the open public hearing portion of the meeting will be time-limited, and that participants are being chosen via a lottery system that will leave some voices left out of the public input process. It is also our understanding that FDA participants and outside participants will not be allowed to have cameras on during this virtual AdCom. If this is true, we are concerned that the limitations the FDA is imposing upon this AdCom will dehumanize important patient voices, and will curtail vital input that outside stakeholders want and deserve to share with the FDA.

We respectfully urge you to use flexibility and discretion granted to you as Commissioner to ensure that this AdCom, and other AdCom meetings to follow in the future, are structured in a manner that will maximize public input, such that the perspectives of patient and clinical communities that have direct experience with the therapies under consideration can be accounted for in FDA regulatory decision making.

¹ Sarepta Therapeutics Press Release, “Sarepta Therapeutics Announces that the Date of the Advisory Committee Meeting for SRP-9001 will be May 12, 2023,” (April 10, 2023), <https://investorrelations.sarepta.com/news-releases/news-release-details/sarepta-therapeutics-announces-date-advisory-committee-meeting>.

² Ibid.

The Honorable Robert M. Califf

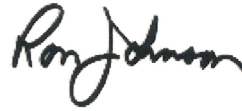
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We kindly request a response no later than Monday, May 8, 2023, at close of business. Thank you to you and your staff for your prompt attention to this matter.

A handwritten signature in blue ink that reads "Mike Braun". The signature is fluid and cursive.

Mike Braun
United States Senator
Indiana

A handwritten signature in black ink that reads "Ron Johnson". The signature is fluid and cursive.

Ron Johnson
United States Senator
Wisconsin