Written Comments Guide

Additional Community Perspectives for FDA
following May 12 Advisory Committee Meeting for Sarepta Therapeutics SRP-9001

On May 12, 2023, the U.S. Food and Drug Administration (FDA) convened an Advisory Committee meeting of the Cellular, Tissue, and Gene Therapies Advisory Committee to discuss Sarepta Therapeutics' SRP-9001, an investigational gene therapy for the treatment of Duchenne muscular dystrophy. The Advisory Committee meeting was used by FDA to obtain independent expert advice on scientific, technical, and policy matters. The Duchenne community played a very active role, both in providing written comments on SRP-9001 and in testimony during the Open Public Hearing portion of the meeting.

The Duchenne Community is encouraged now to provide additional written comments to the FDA following the Advisory Committee discussion. This is an opportunity to reinforce any earlier comments and reflect on the topics that were covered in the Advisory Committee meeting as the FDA is making its decision. Comments should be submitted as soon as possible so they can be considered by FDA prior to the SRP-9001 expected action date of May 29, 2023.

Written comments should be submitted by email to ocod@fda.hhs.gov. Please put "DMD Gene Therapy" in the subject line of your email so it can be easily identified by FDA staff for this purpose. We recommend these emails be as brief as possible.

Suggested Topics

- Your name, when you/your family member was diagnosed with Duchenne, and if your family member participated in the SRP-9001 clinical trials.
- You/your child's current stage of progression of symptoms and the impact it is having on the individual and your family's life.
- How you view the importance of a gene therapy that offers the chance of benefits compared with the risks outlined in the Advisory Committee meeting.
- What a delay in accessing a new therapy, including for a few months, means in terms of preserving muscle function and quality of life (time is muscle).

Topics to Avoid

- Other unapproved therapies – please keep the focus on the therapy being reviewed.
- Cost and coverage of therapy – kindly avoid focusing on the cost or health plan coverage of the therapy. Although this is an important concern for many people, the FDA is not responsible for setting therapy costs or coverage.
- Criticism of FDA – these sessions are expected to be respectful and professionally appropriate.

Thank you again for your active participation in Duchenne advocacy. PPMD will continue to work alongside our community partners as we hope for a positive outcome on May 29th, advancing us toward the day when we can – and will – end Duchenne.

For additional information, please contact Lauren Stanford (lauren@parentprojectmd.org or 202-235-3207).