Written Comments Guide

FDA Advisory Committee Meeting for Sarepta Therapeutics SRP-9001

May 12, 9:00 a.m. – 6:00 p.m. ET

On **May 12, 2023**, the U.S. Food and Drug Administration will convene 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 (Duchenne). The Advisory Committee meeting will be used by FDA to obtain independent expert advice on scientific, technical, and policy matters. The meeting is public and includes an opportunity for voluntary community participation through written and oral remarks. While the main audience for comments will be the Advisory Committee members, FDA will be attending and will review all materials, remarks, and comments to support their evaluation of SRP-9001.

The public is invited to provide written comments on this important matter so that both the Advisory Committee and the FDA have the benefit of their perspective on whether this new drug should be approved for marketing. Comments submitted by **11:59 p.m. ET on May 5** will be provided to the Advisory Committee for consideration. Comments received between **May 6 – 11:59 p.m. ET on May 11** will be considered by FDA only. All submissions are public.

Written comments must be submitted here. All submissions received must include the Docket No. FDA-2023-N-1190 for "Cellular, Tissue, and Gene Therapies Advisory Committee; Notice of Meeting; Establishment of a Public Docket; Request for Comments."

Suggested Topics

- Your name, who you are representing, and disclosures – note when family member was diagnosed with Duchenne, if your family member participated in the SRP-9001 clinical trials, or if you have a conflict of interest.
- The current stage of the Duchenne and the impact it is having on the individual and your family's life.
- Unmet medical need – highlighting how current therapies are not enough.
- How you view the importance of a gene therapy that offers the chance of benefits, which you can further describe.
- How you weigh a new therapy that offers the chance of a benefit in balance with:
    - the risk that the therapy ends up having no benefit at all; or
    - the therapy may have unanticipated side effects, including those which could be serious.

Topics to Avoid

- Other unapproved therapies – Please keep the focus on the therapy being reviewed. This will make the information most useful to committee members, FDA and industry.
- 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 for being willing to take part in this important event. As you know, Parent Project Muscular Dystrophy and our partner organizations are keenly focused on helping to find better treatments and, eventually, a cure for Duchenne. By providing your written comments to the Advisory Committee members, you will have an opportunity to make sure that FDA understands the priorities of people with Duchenne and take those into account when reviewing SRP-9001. For additional information, please contact Lauren Stanford (lauren@parentprojectmd.org or 202-235-3207).