A Letter to the Duchenne Community

June 30, 2026

Dear Duchenne Community,

We are pleased to share an important regulatory update. The U.S. Food and Drug Administration (FDA) has accepted our applications to review two of Sarepta's exon-skipping therapies, AMONDYS 45® (casimersen) and VYONDYS 53® (golodirsen) for traditional approval. This means the FDA has determined the company's applications are complete and ready to be reviewed. The applications include additional evidence that we believe support converting both therapies from accelerated approval to traditional approval. The FDA is conducting its review process and has set a target decision date of February 28, 2027. This coincidentally happens to also be Rare Disease Day!

In support of the applications, we have submitted findings from the ESSENCE confirmatory study, years of real-world clinical experience, and the information about the safety profiles of both therapies.

For families, the journey with Duchenne is marked by perseverance, resilience, and hope. We recognize that every milestone in research and regulatory review carries deep meaning because it represents the collective efforts of patients, families, advocates, clinicians, researchers, and study participants who have helped advance our understanding of this disease and the delivery of approved therapies to patients.

This milestone would not have been possible without the families and individuals who participated in clinical trials and confirmatory studies, joined natural history research studies and registries, shared their experiences, and tirelessly advocated for continued progress in Duchenne. For all of you, your commitment has helped build the body of evidence that is informing this critical review process.

Notably, the FDA accelerated approval pathway enabled earlier treatment access where no existing options were available while additional evidence continues to be collected. The experiences shared by individuals living with Duchenne and their caregivers continue to play a critical role in shaping our understanding of the disease and the impact that treatments may have over time.

For more than a decade, we have remained committed to listening, learning, and continuing to deepen our knowledge through clinical research, real-world experience, the expertise of healthcare providers, and—most importantly—the lived experiences shared by the Duchenne community. Your partnership shapes our work every step of the way.

We understand that regulatory reviews can bring both anticipation and questions. As the FDA review progresses, we remain committed to keeping the community informed and sharing updates when we can.

Thank you for your partnership, your advocacy, and the trust you place in all those working to advance the future of Duchenne care and research. We are honored to be part of this community and remain steadfast in our commitment to all of you.

Please do not hesitate to reach out to us at any time: advocacy@sarepta.com.

With gratitude,

Wendy

Wendy Erler

Senior Vice President, Patient Affairs

Frequently Asked Questions:

1. **What is accelerated approval?**

   Accelerated approval is a critical regulatory pathway developed by FDA and later codified by Congress to speed therapies to patients with serious, life-threatening conditions with unmet medical need. FDA can grant accelerated approval based on a surrogate endpoint that is reasonably likely to predict clinical benefit. This allows patients to have earlier access to treatment while drug developers conduct clinical studies to confirm clinical benefit.

2. What is a supplemental New Drug Application (sNDA) and how does it compare to a New Drug Application (NDA)?

When a drug developer is seeking FDA approval for a new drug that has not been previously approved, a New Drug Application (NDA) is submitted to request approval. A supplemental NDA (sNDA) is used by a drug developer to request FDA approval to modify or update a drug that is already FDA-approved. In the context of accelerated approval, the sNDA is submitted by the drug developer to request conversion from accelerated to traditional approval. The sNDA contains safety and efficacy data, including data from the confirmatory study. FDA reviews the sNDA to determine if clinical benefit has been confirmed in support of conversion to traditional approval.

3. How is a confirmatory study different from a pivotal/registrational study?

Both pivotal/registrational and confirmatory studies are designed to demonstrate the safety and efficacy of a drug. If the initial approval was under accelerated approval, demonstration of efficacy from a pivotal/registrational trial may be based on a surrogate or intermediate clinical endpoint, with a confirmatory study then required to verify clinical benefit post-approval.