November 3, 2025

Dear Duchenne community,

At Sarepta, we are dedicated to advancing science and therapies to serve individuals impacted by rare disease, and our mission is rooted in a deep commitment to the Duchenne community.

Clinical study update

Today, we announced topline results from ESSENCE, a global, randomized, double-blind, placebo-controlled, Phase 3 study of golodirsen and casimersen in 225 patients, ages 6 to 13 years old. The topline results, which were just announced, offer an initial view of safety and efficacy data from the study.

Gratitude and ongoing commitment

Golodirsen and casimersen were approved in 2019 and 2021, respectively, under FDA accelerated approval pathway. Completing the ESSENCE study aims to fulfill the FDA post-marketing requirement to verify the predicted clinical benefits of these therapies through a confirmatory trial.

This trial – one of the largest placebo-controlled studies ever conducted for Duchenne – spanned nearly nine years, the majority of which was spent enrolling the large number of participants across the world. The duration reflects the challenges of the ultra-rare populations eligible for this study, the unexpected constraints associated with the global pandemic, and the ethical considerations of a required 2-year placebo design in a progressively debilitating disease with unmet need.

We understand that asking families to participate in a long-term study with a required placebo control for a progressive disease is no small request, and completing the trial was possible only through the extraordinary dedication of the Duchenne community. We are deeply grateful for the courage and commitment of the families who participated in this study. Your contributions are providing valuable knowledge aimed at making a meaningful impact on patients' lives.

With nearly a decade of real-world evidence, and the lived experience of you in the community, we remain confident in the role of exon-skipping therapies for the treatment of Duchenne.

Open exchange and transparency

We look forward to engaging with the FDA on the next steps. Our commitment to transparency and communication remains steadfast, and we will continue to provide updates as appropriate.

We know there is much more to do in Duchenne and will continue to support the community.

Sincerely,

Wendy Erler
Senior Vice President, Patient Affairs