



May 20, 2024

A Letter to the Duchenne Muscular Dystrophy Community From Dyne Therapeutics

At Dyne we are working to advance our proprietary FORCE™ platform to overcome the limitations of muscle tissue delivery, with the stated mission to deliver life-transforming therapies for people living with serious muscle diseases. Our initial focus includes programs for myotonic dystrophy type 1 (DM1), Duchenne muscular dystrophy (DMD) and facioscapulohumeral muscular dystrophy (FSHD), with the hope of delivering on a shared goal with the community- stopping or reversing disease progression. You can learn more about the FORCE™ platform and our pipeline priorities here: <https://www.dyne-tx.com/our-forcetm-platform/>. Our purpose and passion are fueled by our continuous engagement and active dialogue with the communities we serve.

In January we shared initial clinical data for DYNE-251 from the earliest cohorts (study groups) as part of the Phase 1/2 DELIVER multiple ascending dose (MAD) trial design. Upon the release of that early data, we partnered with patient advocacy organizations and the patient community to share our data and our program-progress, as well as answer questions. We value transparency to build trust.

Recognizing a shared sense of urgency with the DMD community to accelerate potential therapies, we are pleased to now share new clinical data from the ongoing DYNE-251 DELIVER trial. This is in advance of our anticipated plan to provide data in the second half of 2024. Our clinical data continues to demonstrate the promise of the FORCE™ platform. The most recent data shares efficacy and safety data from patients with DMD amenable to exon 51 skipping enrolled in the 10 mg/kg (approximate PMO dose) cohort of the randomized, placebo-controlled MAD portion of the DYNE-251 DELIVER trial. Patients were randomized to receive either DYNE-251 or placebo once every four weeks for 6 months.

Key findings from DELIVER include:

Dystrophin expression measured by Western blot

- Patients treated with 10 mg/kg of DYNE-251 Q4W had a mean absolute dystrophin level of 3.22% of normal and a 2.97% change (unadjusted for muscle content) from baseline at 6 months. When adjusting for muscle content, the DYNE-251 treated group reached 7.64% mean absolute dystrophin.

Function

- Trends in improvement were observed in multiple functional endpoints in the 10 mg/kg DYNE-251 Q4W group at 6 months, including North Star Ambulatory

Assessment (NSAA), Stride Velocity 95th Centile (SV95C), 10-Meter Walk/Run Time, and Time to Rise from Floor.

Safety & Tolerability Data

- DYNE-251 has demonstrated a favorable safety profile¹, inclusive of the 48 patients enrolled through the 40 mg/kg Q8W cohort of the MAD portion of the DELIVER trial. The majority of treatment-emergent adverse events were mild or moderate and no related serious treatment-emergent adverse events have been identified.

We look forward to sharing this data and our program updates in more detail with the community over the coming weeks. Similarly, as the study progresses and additional data report out, we anticipate additional opportunities to collectively advance our understanding of DYNE-251 as a potential therapy for DMD.

Thank You

Our therapy development efforts and the other critical studies being conducted to better understand serious muscle diseases, progression and symptom burden would not be possible without the generous participation of community members like you. The insights and expertise provided by you have contributed to the development of our science and the design of our programs. Our recent publication in the journal Research Involvement and Engagement, *Patient Engagement in Clinical Trial Design for Rare Neuromuscular Disorders: Impact on the DELIVER and ACHIEVE Clinical Trials* (<https://rdcu.be/du0ID>), delivered on this commitment by documenting how your input enabled our DELIVER trial of DYNE-251 for DMD. We recognize and acknowledge the time, hard work and thought provided by community participants and we are deeply grateful. Thank you.

With gratitude,
Dyne Therapeutics

Q4W: dosing once every four weeks; Q8W: dosing once every eight weeks

¹ DYNE-251 safety data as of April 30, 2024