

Dyne Therapeutics Announces First Patient Dosed in Phase 1/2 DELIVER Clinical Trial of DYNE-251 for the Treatment of Duchenne Muscular Dystrophy

Hello all,

We are very pleased to announce that Dyne Therapeutics has announced achievement of first patient dosed in in our Phase 1/2 clinical trial, DELIVER, evaluating DYNE-251 for the treatment of DMD mutations amenable to exon 51 skipping, as well as of the initiation of our ACHIEVE Phase 1/2 clinical trial evaluating DYNE-101 for the treatment of myotonic dystrophy type 1 (DM1).

As you know, the DELIVER trial is a Phase 1/2 global clinical trial evaluating DYNE-251, consisting of a 24-week multiple ascending dose (MAD) randomized placebo-controlled period, a 24-week open-label extension and a 96-week long-term extension. The trial, which is designed to be registrational, is expected to enroll approximately 46 ambulant and nonambulant males with DMD who are ages 4 to 16 and have mutations amenable to exon 51 skipping therapy. The primary endpoints are safety, tolerability and change from baseline in dystrophin levels as measured by Western blot. Secondary endpoints include measures of muscle function, exon skipping, and pharmacokinetics. Dyne anticipates reporting data from the MAD placebo-controlled portion of the DELIVER trial on safety, tolerability and dystrophin in the second half of 2023.

In the MAD placebo-controlled portion of the DELIVER trial, patients will be randomized to receive DYNE-251 or placebo every four weeks intravenously based on a global protocol designed to incorporate feedback from multiple regulatory authorities, including on starting dose. Patient cohorts will be dosed from 0.7 mg/kg to 40 mg/kg (approximate PMO dose) in the U.S. Outside the U.S., starting doses and number of cohorts will vary by region. Following the placebo-controlled period, patients will transition to DYNE-251 treatment in the open-label portion of the trial and in the long-term extension.

Information on the DELIVER study is available at [clinicaltrials.gov](https://www.clinicaltrials.gov/): [https://www.clinicaltrials.gov/](https://www.clinicaltrials.gov/ct2/show/study/NCT05524883) (NCT05524883).