

October 4, 2021

Dear Duchenne Community,

Today, Sarepta Therapeutics announced the initiation, in partnership with Roche, in the United States, and countries around the world, of study SRP-9001-301, also known as EMBARK, a pivotal study of SRP-9001 (rAAVrh74.MHCK7. micro-dystrophin) for the treatment of Duchenne muscular dystrophy. SRP-9001 is an investigational gene transfer therapy intended to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein.

Initiating a clinical trial means that the study sponsor(s) and clinical trial sites take the necessary steps required to enable dosing of the investigational therapy. These initiating steps happen prior to the first patient being recruited into a study.

We have received many inquiries in past months, and we recognize the interest and urgency expressed by the Duchenne community. Parent Project Muscular Dystrophy (PPMD) has invited us to share details of the EMBARK clinical trial on a community webinar, which we are targeting for the week of October 11th. Please stay tuned for an announcement from PPMD.

Many individuals with Duchenne, families, and Patient Advocacy Organizations have contributed time and effort to support early studies of this investigational therapy. We thank you for your dedication, without which this milestone in therapeutic development efforts would not be possible.

With our kind regards,

Siobhan Fitzgerald

Siobhan Fitzgerald Executive Director, Patient Affairs Sarepta Therapeutics