Dear Duchenne muscular dystrophy Community,

We are excited to announce that Wave Life Sciences has initiated Part C of FORWARD-53, a clinical trial evaluating the investigational molecule WVE-N531 in boys with Duchenne muscular dystrophy (DMD) amenable to exon 53 skipping.

Part C of FORWARD-53 is an open-label Phase 1b/2 study of up to 15 ambulatory boys aged 4-10, to evaluate dystrophin expression, functional outcomes, muscle health and safety. Participants will receive a 10 mg/kg dose intravenously (IV) every four weeks following initial loading dose regimen and will undergo muscle biopsies before their first dose and following 24 weeks of treatment.

We are currently screening participants at the following sites in the US:

- Dr. Aravinddhan Veerapandiyan's – Little Rock, Arkansas – Mary-Kaylin Linch, linchm@archildrens.org
- Dr. Scott Batchelor's – Atlanta, Georgia – Kendra Askins, kendra.askins@rarediseaseresearch.com
- Dr. Tian's site – Columbus, Ohio – Angela Edmondson, angela.edmondson@cchmc.org
- Dr. Diana Castro's site – Dallas, Texas – Jennifer Avelar, jennifer.avelar@neuromdcenter.com

As other sites begin screening participants, they will be listed on www.clinicaltrials.gov (NCT04906460).

Key information related to Part C of FORWARD-53 includes:

- A10mg/kg intravenous (IV) dose of WVE-N531 every four weeks following initial loading dose regimen.
- Muscle biopsies are performed before the first dose and after 24 weeks of treatment.
- Safety monitoring will extend 10 months following the last dose.
- Participants will have the option of rolling into the WVE-N531 Open Label Extension study.
- The main outcomes being studied are the measurement of dystrophin protein levels in muscle tissue, safety, and functional assessments such as the North Star Ambulatory Assessment (NSAA), Performance of the Upper Limb (PUL), and Stride Velocity 95th Centile (SV95C).
- All participants must have stable pulmonary and cardiac function, be on stable corticosteroid therapy and have adequate muscle for biopsy.
- Some of the criteria that may exclude participants from the trial include significant medical conditions beyond DMD and/or recent major surgery.

For more information on the inclusion and exclusion criteria, please visit www.clinicaltrials.gov (NCT04906460). We encourage you to speak with your doctor about what may be best for you or your loved one.

We remain grateful to the entire patient community for your trust and engagement; you make it possible to move the development of new treatments forward. We will continue to keep you informed about our progress.

Sincerely,

Chelley Casey, VP Advocacy