December 15, 2023

Dear Duchenne community,

We are pleased to announce the initiation of dosing in our Phase 2 FORWARD-53 study, which is evaluating WVE-N531 as a treatment for boys with Duchenne muscular dystrophy (DMD) who are amenable to exon 53 skipping.

What do we know about WVE-N531?

- In preclinical animal studies, WVE-N531 reached high concentrations in skeletal muscle, and higher concentrations in heart and diaphragm, and generated dystrophin protein.
- In our proof-of-concept clinical trial with 3 boys with DMD, WVE-N531 reached high concentrations in skeletal muscle and produced 53% exon skipping following 3 doses of 10 mg/kg every other week. WVE-N531 was generally safe and well tolerated.

What is FORWARD-53?

FORWARD-53 is a potentially registrational, open-label Phase 2 clinical trial that is now fully enrolled with 10 boys with DMD who are amenable to exon 53 skipping. The primary endpoint is functional dystrophin protein levels which will be measured following 24 and 48 weeks of every other week dosing at 10 mg/kg. The trial is also evaluating safety and tolerability, digital and functional endpoints.

Next steps

We expect to deliver data, including dystrophin expression from muscle biopsies, in 2024.

Gratitude

Over the past few years, many of us at Wave have been privileged to meet families living with Duchenne and to work globally with the advocacy organizations that support you. All of us are enormously grateful - especially to the boys, the families and the investigators who are participating in our study. We recognize the personal sacrifices made by each family involved in these studies. Their participation, along with the support of the entire DMD community, is critical to advancing the scientific and medical understanding required to defeat this devastating disease.

We look forward to sharing more updates on WVE-N531 in 2024.

Sincerely,

Chelley Casey
VP, Patient Advocacy