

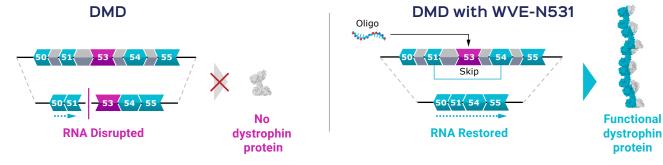
# Wave Life Sciences' Clinical Trial in Duchenne Muscular Dystrophy

#### What is FORWARD-53?

A potentially registrational Phase 2 clinical trial studying WVE-N531, an investigational exon skipping treatment for boys with Duchenne Muscular Dystrophy (DMD) amenable to exon 53 skipping.

## What is an exon skipping treatment?

WVE-N531 is designed to target dystrophin RNA (ribonucleic acid) to skip over the part that has the DMD causing mutation to restore the body's ability to create its own functional dystrophin protein.



### What has been done and what do we know about WVE-N531?

- √ Wave conducted studies looking at WVE-N531 in cells, animal models and in boys with DMD.
- √ In cell models, WVE-N531 restored dystrophin.
- ✓ In animal models, WVE-N531 reached high concentrations in the heart and diaphragm.
- ✓ In Part A of the Phase 1b/2 clinical trial with three boys with DMD, WVE-N531 reached high concentrations in skeletal muscle and led to 53% exon skipping following three doses of 10 mg/kg every other week.
- √ WVE-N531 was generally safe and well tolerated and the safety profile supports further clinical development.

Data so far supports dosing by intravenous infusion (IV) every other week or potentially less frequent dosing.

#### What does this mean?

- 1. Based on the levels of exon skipping and drug concentration in muscles observed in Part A of the study, we hope to see clinically meaningful levels of dystrophin protein with longer duration of treatment.
- 2. We are hopeful that we have identified a dose in Part B of the study that will both access muscle tissue and that will be well tolerated in patients.

For more information, please speak with your doctor, visit www.clinicaltrials.gov or contact patientadvocacy@wavelifesci.com.

Screening

#### Dosing Every Other Week (10 mg/kg IV)

#### Safety Follow-up

#### What's next?

Enrollment is complete and dosing is underway in the Phase 2 portion of FORWARD-53, an open-label clinical trial for boys with DMD amenable to exon 53 skipping.

#### Biopsy after 24 weeks of treatment

- ▶ 11 boys will receive 10 mg/kg every other week.
- Biopsies will take place after 24 and 48 weeks of treatment.

#### Biopsy after 48 weeks of treatment

- We will measure safety, tolerability, dystrophin protein levels and DMD-specific functional assessments.
- ▶ Data, including dystrophin protein levels from muscle biopsies after 24 weeks, is expected in the third quarter of 2024.

