# Open-label Extension Study of WVE-N531 in Patients with Duchenne Muscular Dystrophy

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### Hub Summary

This is a Phase 2 open-label extension (OLE) study to evaluate the long-term safety, tolerability, efficacy, pharmacokinetics (how the drug is absorbed and metabolised in the body) and pharmacodynamics (how the drug affects the body) through potential exploratory biomarker(s) of intravenous (IV) WVE-N531, an exon skipping investigational therapy, in patients with DMD who participated in FORWARD-53.Only patients who have a mutation amenable to exon 53 skipping are able to participate.

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### Study Number: WVE-N531-002

### Description by Wave Life Sciences

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The study will include up to 175 patients from planned and ongoing WVE-N531 studies globally. All patients will continue to receive 10 mg/kg WVE-N531 IV every 4 weeks (Q4W), through Week 96. Safety monitoring will continue for approximately 11 months after the final dose. The primary objective of the study is to evaluate the long-term safety and tolerability of WVE-N531.

All participants and investigators will know that the WVE-N531 is being administered. This clinical trial is not placebo-controlled, meaning there will be no dummy treatment.

## Primary Outcome Measures

Safety and tolerability:

- Adverse Events (AEs)
- Physical examination findings
- Vital signs
- Safety laboratory tests
- ECGs and ECHO
- Pulmonary function tests

## Secondary Outcome Measures

Long-term functional outcomes of WVE-N531 by assessing changes in functional assessments and pulmonary function:

- Time to stand as assessed in North Star Ambulatory Assessment (NSAA) (Version 2.0)
- NSAA (Version 2.0) composite score
- Timed 10-meter walk/run as assessed in NSAA (Version 2.0)
- Four-stair climb time
- Performance of the Upper Limb (PUL) (Version 2.0)
- Stride Velocity 95th Centile (SV95C)/upper limb outcome for nonambulatory patients
- Upper limb proximal strength assessed by handheld myometer

### Can I take part?

## Inclusion Criteria

## Trial Status
Recruiting

**UK Locations**
Oxford, Enrolling by invitation

**Trial Sponsor**
Wave Life Sciences

**Phase**
2

**Length Of Participation**
96 weeks + safety follow-up; approx. 2.5 years

**Recruitment Target**
175

**Ambulatory**
Ambulant and non-ambulant

**Therapeutic Category**
Exon Skipping

**Age**
4 and up

**Mutation Specific**
Mutation specific therapies, Exon 53

**Muscle Biopsy**
No Muscle Biopsy Required

**MRI**
No

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1. Participated in a prior study of WVE-N531 and has not experienced any significant toxicities due to WVE-N531 or significant clinical deterioration of general health since the last dose or early discontinuation visit.

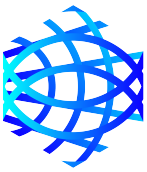
2. Of note: if there will be a greater than 31-day gap between the last dose on the prior study and the first dose on this study, the case should be discussed between the Investigator and Medical Monitor.

## Exclusion Criteria

1. Any clinically significant medical finding or change during or following participation in the prior WVE-N531 study, other than DMD that, in the judgment of the Investigator, would affect the potential safety of the patient to receive WVE-N531 or interfere with participation in the study.

2. Any recreational substance use (including prescribed cannabinoids), other than alcohol and nicotine which are not exclusion criteria, irrespective of legality, within two months prior to first dose and/or unwilling to refrain from such use for the duration of the study.

For contact details and to find out more, please refer to dmdhub.org.



Duchenne UK

PDF created on 07/11/2026.