

NS-089/NCNP-02-201 in Boys With Duchenne Muscular Dystrophy (DMD)

Status: RECRUITING

Eligibility Criteria

Age: 4 years to 14 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

* Male \geq 4 years and \leq 15 years of age * Confirmed DMD mutation(s) in the dystrophin gene that is amenable to skipping of exon 44 to restore the dystrophin mRNA reading frame * Able to walk independently without assistive devices * Ability to complete the TTSTAND without assistance in \leq 20 seconds * Stable dose of glucocorticoid for at least 3 months and the dose is expected to remain on a stable dose for the duration of the study. * Other inclusion criteria may apply.

Exclusion Criteria:

* Has a body weight of \leq 20 kg at the time of informed consent (applies to participants screening for Part 1 only) * Evidence of symptomatic cardiomyopathy * Current or previous treatment with anabolic steroids (e.g., oxandrolone) or products containing resveratrol or adenosine triphosphate within 3 months prior to first dose of study drug * Current or previous treatment with any other investigational drug within 3 months prior to the first dose of study drug or within 5 times the half-life of a medication, whichever is longer * Surgery within the 3 months prior to the first dose of study drug or planned during the study duration * Previously treated in an interventional study of NS-089/NCNP-02 * Having taken any gene therapy. * Having received exon skipping oligonucleotide within 1 year prior to the first dose of IP or is expected to receive exon skipping oligonucleotide prior to completion of study. * Other exclusion criteria may apply.

Conditions & Interventions

Interventions:

DRUG: NS-089/NCNP-02

Conditions:

Duchenne Muscular Dystrophy, Exon 44, DMD

Keywords:

Exon 44 Skipping, DMD

More Information

Contact(s): Trial info - trialinfo@nspharma.com

Principal Investigator:

Phase: PHASE2

IRB

Number:

System ID: NCT05996003

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