Gene Therapy Study for Late Juvenile Metachromatic Leukodystrophy (LJ MLD)

STUDY OVERVIEW

The purpose of this study is to gather information about how safe and effective a MLD gene therapy is in children with pre-symptomatic late juvenile MLD (LJ MLD) or early symptomatic LJ MLD

WHO CAN PARTICIPATE?

Children with a confirmed diagnosis of **pre-symptomatic LJ MLD**

- must be younger than 17 years of age at treatment **AND**
- must have a sibling with a confirmed diagnosis of LJ MLD based on age at disease onset (≥7 and <17 years of age)

OR

Children with a confirmed diagnosis of **early symptomatic LJ MLD**

- must have age of disease onset ≥7 and <17 years

STUDY LOCATION

The study is being conducted at Ospedale San Raffaele – Telethon Institute for Gene Therapy (OSR-TIGET) in Milan, Italy

QUESTIONS?

Please ask your child's physician to contact the study investigator Dr. Francesca Fumagalli at OSR-TIGET in Milan, Italy or Orchard's clinical trial team at medinfo@orchard-tx.com

More detailed information about this study (NCT04283227), including eligibility criteria, can found on clinicaltrials.gov

STUDY DESIGN

Screening Phase:
Potential study participants will undergo an initial evaluation to confirm they meet all requirements to receive gene therapy

Baseline Phase:
Additional procedures and tests will be done to gather information and to re-confirm eligibility

Treatment Phase:
Study participants will be admitted to the study site in Milan for a period of time before, during, and after gene therapy

Follow up Phase:
Frequent safety and efficacy assessment visits in the first year and then every six months, for up to 8 years after gene therapy

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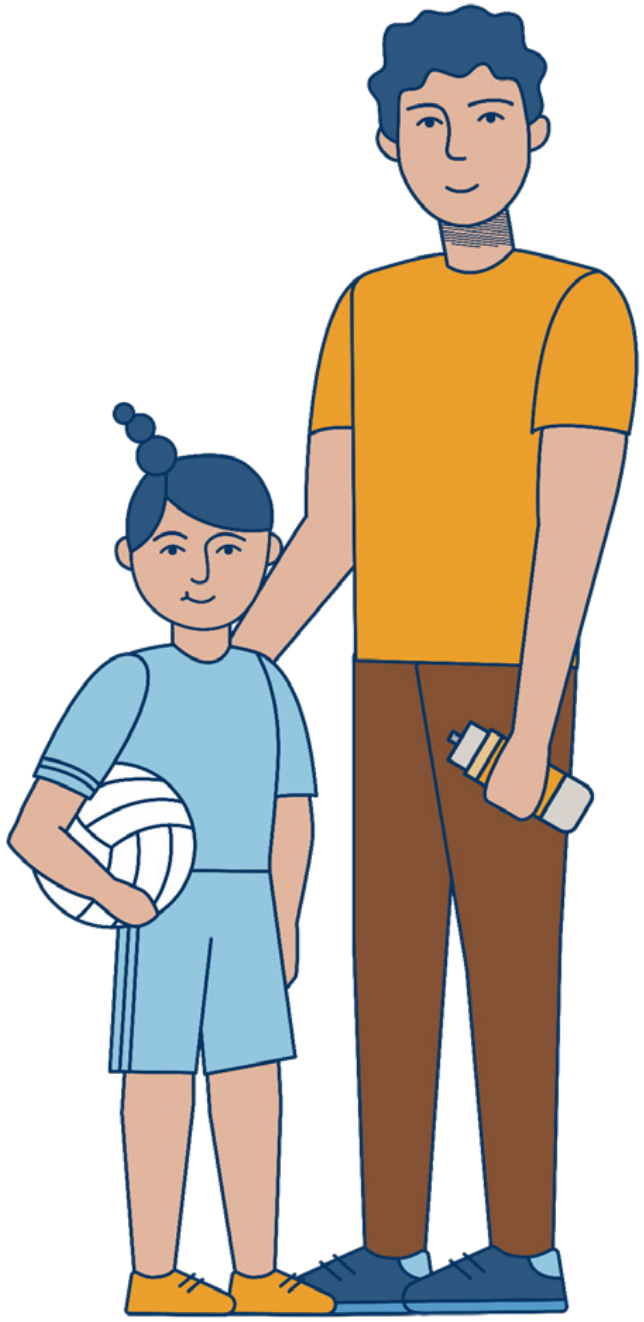
Learn more about a gene therapy study for children born with Late Juvenile MLD here



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