

# 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 ( $\geq 7$  and  $< 17$  years of age)

## OR

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

- ▶ must have age of disease onset  $\geq 7$  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](mailto:medinfo@orchard-tx.com)

*More detailed information about this study (NCT04283227), including eligibility criteria, can found on [clinicaltrials.gov](https://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:

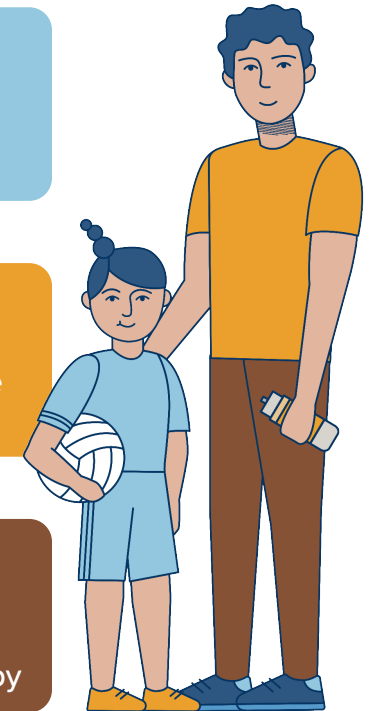
Additional procedures and tests will be done to gather information and to re-confirm eligibility

### 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  
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