

# We welcome individuals with LGMD2A/R1 to join our journey



JOURNEY is a clinical outcomes assessment study, also referred to as a natural history study. The study does not involve the use of an investigational study drug, but instead studies the natural progression of LGMD subtypes over a period of time (~3 years). The data collected from JOURNEY will help Sarepta learn more about the condition and how muscle strength, breathing, and heart function can change over time. This information is critically important for researchers to design clinical trials for future therapies. Individuals affected with limb-girdle muscular dystrophy type 2A/R1 (LGMD2A/R1, calpainopathy) are now invited to participate.



## The journey to uncover your potential treatment options

Currently there are no treatments approved for individuals with a limb-girdle muscular dystrophy. Your participation in JOURNEY will not hinder your ability to participate in future clinical studies, including those with investigational study drugs.

### Who may be eligible

- Male or Female age 4 years and older
- Genetic diagnosis of LGMD2A/R1. Enrollment of individuals with LGMD2E/R4, LGMD2D/R3, or LGMD2C/R5 has been met and is closed for future enrollment

\*Other inclusion/exclusion may apply and will be discussed with a study doctor at screening.



## JOURNEY Participation

Study participants will have access to highly experienced physicians and undergo medical and functional procedures during the screening visit, and at scheduled study visits throughout the lifetime of the study.



### On-site visits: For screening and every 6-month visit:

- meet with the study doctor and research team
- complete motor assessments and laboratory tests.



### In between on-site visits: For every 3-month visit:

- study research team will contact you every 3 months by phone
- ask you questions on your health and well being.



## To learn more about the study and how you can join the journey, go to [journeyLGMD.com](https://www.journeylgmd.com)

You can also find more information about this study and participating locations, by visiting [ClinicalTrials.gov](https://ClinicalTrials.gov) and searching NCT04475926.

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