

September 2026

An Update on the DNL952 Clinical Study in Late-onset Pompe Disease

Dear Pompe Disease Community,

Denali Therapeutics is dedicated to working in partnership with the lysosomal storage disease community to advance research and develop potential new treatment options.

We are using Denali's TransportVehicle™ platform to develop an investigational enzyme replacement therapy (ERT) for Pompe disease, called DNL952, that is designed to improve delivery of the acid alpha-glucosidase (GAA) enzyme to muscle tissue and the brain by crossing the blood-brain barrier.

We are pleased to share that recruitment has begun for Denali's first clinical study of DNL952 in adults living with late-onset Pompe disease (LOPD).

About the study

The study will evaluate DNL952 at several dose levels. Researchers will assess:

- The safety and tolerability of DNL952, including any side effects.
- How the body absorbs and processes DNL952, known as pharmacokinetics or PK.
- How DNL952 affects the body, known as pharmacodynamics or PD.
- The study plans to enroll up to approximately 32 participants.

DNL952 is being studied for its potential to address both the muscle-related and possible neurological causes of motor and respiratory symptoms in people living with LOPD.

Study locations and additional information

Study sites are currently open in the United States. Denali plans to open additional sites in the United States, United Kingdom, Germany, and the Netherlands within the next three to six months.

Additional information about the study, including eligibility criteria and recruiting site information, as well as up-to-date information on the study sites, **can be found on [ClinicalTrials.gov](https://clinicaltrials.gov) using the study identifier: NCT07354724.**

We recognize the importance of continued progress in Pompe disease research and are grateful to the individuals, families, advocates, clinicians, and researchers whose partnership and commitment make studies like this possible. We are committed to keeping the Pompe community informed and will continue to share updates as the DNL952 program progresses. If you have questions, please contact our Patient Advocacy team at patientadvocacy@dnli.com.

Thank you on behalf of the Denali team,

Kim Ramsey
Executive Director, Patient Advocacy

DNL952 is an investigational medicine not approved by any Health Authority.