

Pompe Disease Glycogen Storage Disease Type 2 Lysosomal Storage Diseases

A Clinical Trial Looking at the Effects of a Single Dose of gene therapy SPK-3006 (Investigational Study Drug) Given Into a Vein ('Intravenous Infusion') of Adult Patients with Late-Onset Pompe Disease (LOPD) Who Have Been Receiving Enzyme Replacement Therapy (ERT).

A Gene Transfer Study for Late-Onset Pompe Disease (RESOLUTE SM)

Trial Status
Active, not recruiting

Trial Runs In
8 Countries

Trial Identifier
NCT04093349 2019-001283-30
SPK-3006-101

The source of the below information is the publicly available website ClinicalTrials.gov. It has been summarised and edited into simpler language.

Trial Summary:

The purpose of this study is to evaluate the safety, tolerability, and efficacy of a single intravenous infusion of SPK-3006 in adults with clinically moderate, late-onset Pompe disease receiving enzyme replacement therapy (ERT). Participants will be treated in sequential, dose-level cohorts.

Spark Therapeutics
Sponsor

Phase 1/Phase 2
Phase

NCT04093349 2019-001283-30 SPK-3006-101
Trial Identifiers

Eligibility Criteria:

Gender
All

Age
≥18 Years

Healthy Volunteers
No

How does the RESOLUTE# trial work?

The RESOLUTE clinical trial is recruiting men and women who have LOPD. In order to take part in the trial, people must have clinical symptoms of LOPD affecting daily living, must be 18 years of age or older, agree to use reliable birth control, have received ERT for at least the past 24 months (2 years), do not have any liver disease, and have not received

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any gene therapy before. *This is not a complete list of requirements to take part in the RESOLUTE clinical trial.*

The purpose of the RESOLUTE clinical trial is to study the effects of gene therapy *SPK-3006* (investigational study drug). For example, to see if it works to treat your Pompe disease and if you develop any serious and non-serious side effects.

How do I take part in the RESOLUTE clinical trial?

If you think the RESOLUTE clinical trial may be right for you and you would like to take part, please talk to your doctor. If your doctor thinks that you might be able to take part in this clinical trial, he/she may refer you to the closest clinical trial doctor who will give you all the information you need to make your decision about taking part in the clinical trial. You will also find the clinical trial locations at the top of this page. Before starting the RESOLUTE clinical trial, you will be told about any potential risks and benefits of taking part in the trial and will need to provide written informed consent to participate. You will also be told what other treatments are available so that you may decide if you still want to take part.

What treatment will be given if I participate in the RESOLUTE clinical trial?

Everyone who signs an approved informed consent form and is deemed eligible by the study doctor for the RESOLUTE clinical trial will receive the investigational study drug (gene therapy *SPK-3006*), which is given into a vein as a one-time intravenous infusion. You might receive other study treatments to help manage certain side effects.

How long is the RESOLUTE clinical trial?

After receiving the study drug, you will be monitored for up to 52 weeks (1 year).

What happens if I'm unable to take part in the RESOLUTE clinical trial?

If the RESOLUTE clinical trial is not suitable for you, you will not be able to take part. Your doctor may suggest other clinical trials that you may be able to take part in or other treatments that you can be given. You will not lose access to any of your regular care.

For more information about the RESOLUTE clinical trial follow this link to [ClinicalTrials.gov](https://clinicaltrials.gov).

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