

## What are clinical research studies?

Clinical research studies test different things like whether a study drug is safe, and if the drug is effective against certain health conditions. Before any drug is made available to the public, it goes through several phases of clinical research.

Volunteer participants are very important to clinical research studies. Your study participation could possibly help doctors improve medical care and the quality of life for people with AATLD.



**For more information about the ESTRELLA study and the SHINE AATLD program, visit [estrellastudy.com](https://estrellastudy.com), or contact:**



## Do you have alpha-1 antitrypsin deficiency?

Learn about the ESTRELLA clinical research study and a potential risk of liver disease.

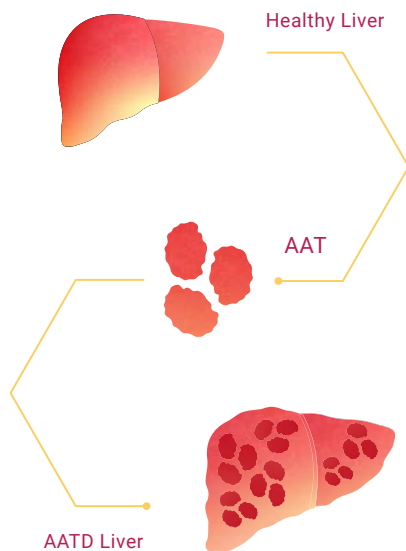


## What is the ESTRELLA study?

The goal of the ESTRELLA study is to find out whether a study drug is safe and does not cause severe side effects in participants with alpha-1 antitrypsin deficiency-associated liver disease (AATLD).

## How are alpha-1 antitrypsin deficiency and liver disease associated?

Alpha-1 antitrypsin is a protein that is made in the liver and then circulates in the bloodstream. People with alpha-1 antitrypsin deficiency (AATD) have a change or mutation in the gene that makes the alpha-1 antitrypsin (AAT) protein. This gene change can lead the AAT protein to build up in the liver, resulting in liver disease. Sometimes people with AATD do not know they have liver disease.



## What is the study drug?

The study drug is an **investigational** drug designed to reduce AAT protein build-up in the liver. **Investigational** means that the study drug has not been approved by the US Food and Drug Administration (FDA) or the European Medicines Agency (EMA) and can only be used in research studies.

## Who can participate in the study?

You may qualify to participate if you meet study requirements\*:

- Age 18 to 70 years
- Confirmed diagnosis of AATLD
- Able to have 2 required liver biopsies

\*Other study requirements will apply. Women able to have children do **NOT** qualify for study participation.

## What will happen if I join the study?

If you join the ESTRELLA study, you will participate in the following study periods:

- Study Screening (up to 42 days)
  - Complete health exams and procedures to see if you qualify for the study
  - If it is determined you have met all the requirements for the study, you will have the first liver biopsy
- Study Therapy (24 to 48 weeks)
  - Be randomly assigned to receive either the study drug or placebo (placebo has no active ingredients)
  - Visit the study centre and receive the study drug or placebo as an injection
  - Return to the study centre 3 times for health checks after receiving your first injections
  - Return to the study centre for 5 more, or 11 more, doses (depending on your enrolment group)
  - During this time frame, you will have the second liver biopsy
- Study Follow-up (up to 1 year\* after your last Study Therapy visit)
  - Come to the study centre every month for a health check and blood draw until the AAT protein in your blood reaches a specific level for 2 visits in a row

\* You may be asked to enrol in a separate follow-up study that includes additional visits for longer than 1 year