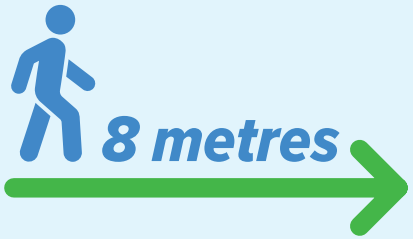
Who can take part?

You may be able to take part if you:

- are 18–65 years of age, inclusive
- have been diagnosed with SCA2
- can walk without help from another person or an assistive device for 8 metres.



What else do I need to consider?

- The study team will explain the possible benefits and risks of the study.
- You can stop participating at any time.
- Reasonable study-related travel costs, all study medications, and study-related tests will be provided at no cost to you.
- A team of doctors and nurses will monitor your health carefully during the study.

What is a clinical research study?

A clinical research study helps to answer questions about investigational study drugs, such as:

- Are they safe?
- Are they effective?

All medications must be researched in people before they can be approved for doctors to prescribe.

An Institutional Review Board (IRB)/Ethics Committee (EC) has reviewed the study. An IRB/EC protects the rights, safety, and well-being of participants.

Learn more

Please contact the study team below. By contacting us, there is no obligation to take part in the study.

AROATXN2-1001

Patient Information

This brochure contains information about the AROATXN2-1001 Study for people with spinocerebellar ataxia type 2 (SCA2).

This information should help you decide whether you, or someone you know, may want to take part in the study.

arrowhead pharmaceuticals

Patient Brochure, 12 Sep 2024 [V02 CAN(en)]

About the AROATXN2-1001 Study

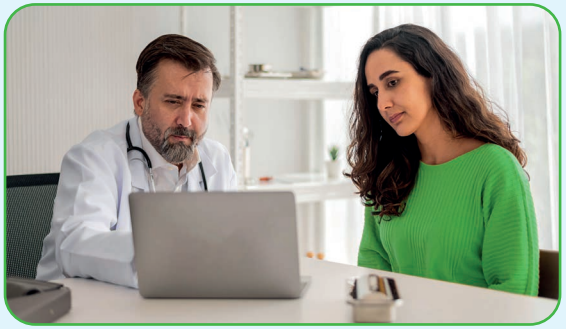
Current therapies for SCA2 are supportive and aimed at helping ease symptoms. However, there are no available treatments that delay, slow, or reverse the progress of the condition.

The AROATXN2-1001 Study will look at a potential new medication (study drug) in adults with SCA2. This study drug may lower the amount of a protein called ATXN2 in the body, which may stop or slow SCA2 disease progression.

The study will look at:

- how safe the study drug is
- what side effects it may cause
- what is the most suitable dose for further research.

The study drug will be compared with a placebo, which looks like the study drug but contains no actual medicine.



What will this study involve?

- Up to 36 people will take part.
- Participants will receive either the study drug at 1 of 4 dose levels or the placebo. Participants are 2 times more likely to receive the study drug than the placebo.
- Participants will receive the study medication (study drug or placebo) once, as an injection in the fluid around the spine.
- Participants will have 5 lumbar punctures (a procedure to collect spinal fluid) throughout the study.
- Participants will have 2 magnetic resonance imaging (MRI) brain scans. One scan will be at screening and another 6 months after their first study medication dose.
- An overnight stay at the study centre for 1 night.
- 12 study centre visits for study assessments.

The study is made up of 3 periods and will last for about 10 months.

Screening period
(up to 2 months)

- You will have at least 1 study centre visit to see if the study is suitable for you and whether you want to take part.

Study treatment period
(about 24 hours, overnight)

- You will receive the study medication and have study assessments.
- This will include 2 of the 12 study centre visits – 1 visit before and 1 visit after the study medication dose.

Follow-up period
(about 8 months)

- You will have 9 study centre visits for study assessments.
- At the end of 8 months, follow up may be extended with further study centre visits every 2 months.