

Time Matters in A-T

Treatment is Here[™]

**AQNEURSA is the first and only
FDA-approved prescription treatment
clinically proven to fight ataxia in A-T.**



Actual patients not shown.



Learn more about AQNEURSA and support resources for patients and families. Visit [AQNEURSA.com/AT](https://aqneursa.com/AT) or scan the QR code to get started.

Approved Use

AQNEURSA[®] (levacetylleucine) is indicated for the treatment of ataxia in adults and children patients with ataxia-telangiectasia (A-T) weighing ≥ 15 kg.

Important Safety Information

Before taking AQNEURSA, tell your healthcare provider about all your medical conditions, including if you are pregnant or plan to become pregnant, or are breastfeeding or plan to breastfeed.

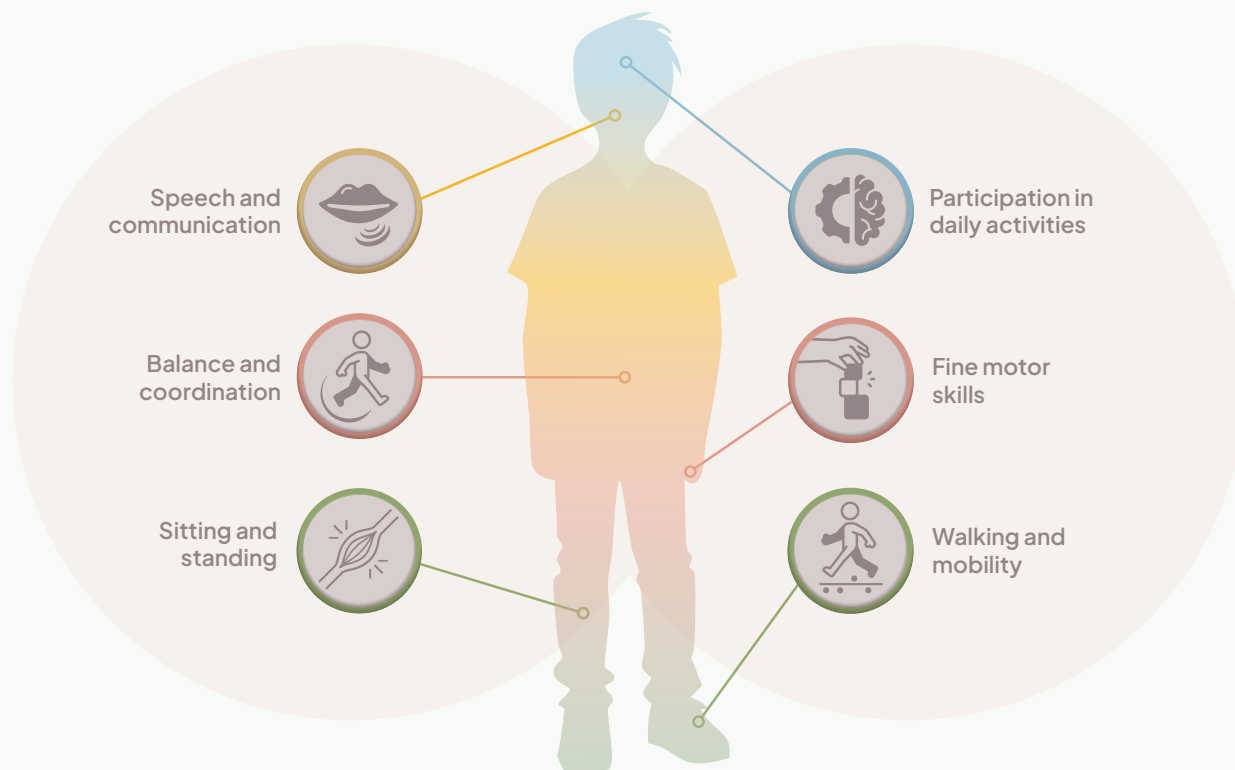
- Based on findings from animal reproduction studies, AQNEURSA may cause harm to your unborn baby when administered during pregnancy.
- Your healthcare provider will check to make sure you are not pregnant before starting treatment with AQNEURSA.
- Use effective birth control (contraception) during treatment with AQNEURSA and for 7 days after stopping treatment to avoid pregnancy.
- If you become pregnant while taking AQNEURSA, inform your healthcare provider immediately to discuss potential risks and alternative treatments.
- It is not known if AQNEURSA or its metabolites pass into human or animal milk or effect breastmilk production.

Please see additional Important Safety Information throughout and Full Prescribing Information for AQNEURSA.

A-T affects more than movement—it can impact everyday life

A-T is a progressive disease. As ataxia symptoms advance, skills and abilities that were once routine can become harder to maintain—and difficult to recover once lost.

Ataxia in A-T may affect:



As ataxia progresses, patients may experience increasing challenges with independence and daily routines.



Progressive ataxia can increase caregiving needs and burden, affecting families and caregivers across physical, emotional, and practical domains.

Improving function and addressing ataxia as early as possible can be important.

Important Safety Information (cont'd)

Tell your healthcare provider about all the medications you take and if you get any new medications, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Other medicines may cause side effects or affect how AQNEURSA works when used together.

- **Tell your healthcare provider if you are taking certain medications called P-gp substrates, *N-acetyl-DL-leucine* or *N-acetyl-D-leucine*.** If you are not sure if you are taking these medicines, talk to your healthcare provider or pharmacist.

 **AQNEURSA**
(levacetylleucine)

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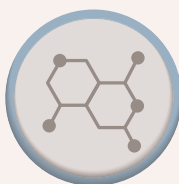
Proven to help people with A-T manage symptoms that affect movement, balance, and coordination

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FDA approval was based on a phase 3 clinical study involving 73 patients with A-T. In this randomized, placebo-controlled trial, researchers evaluated how AQNEURSA affected ataxia and function using established assessment tools.



First and only
FDA-approved treatment
for A-T



Studied in both
children and adults



Demonstrated improvements
in ataxia and function

Every participant helped researchers learn about AQNEURSA for A-T



This design helped researchers evaluate how AQNEURSA affects ataxia in people with A-T.

AQNEURSA was clinically proven to improve ataxia and daily function in patients with A-T.

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You are encouraged to report side effects of prescription drugs to the FDA.

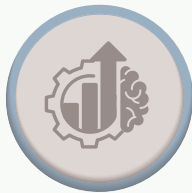
Visit www.fda.gov/medwatch, or call **1-800-FDA-1088**.



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AQNEURSA helps make daily activities more possible

In the clinical trial, AQNEURSA improved patients' symptoms compared with placebo.



WHAT THE RESULTS SHOW:

Patients taking AQNEURSA showed improvements in everyday abilities such as walking, sitting, and speaking.

In contrast, patients receiving placebo showed worsening in these everyday abilities over time.

What This Could Mean for Daily Life

Improvements in fSARA may relate to essential abilities used in daily routines:

walking | standing | sitting | speech

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Meaningful improvements in everyday activities are possible

What could this mean for you or your loved one?



AQNEURSA demonstrated meaningful **improvements in both symptoms and daily function** across pediatric and adult patients with A-T.

A-T can affect many aspects of daily life. The improvements seen on SARA suggest benefits across several important functions, including:

- Walking and getting around
- Maintaining balance while standing or sitting
- Speaking more clearly
- Using hands and arms for everyday tasks
- Coordinating leg movements



Lower scores
are better

UNDERSTANDING THE DATA

SARA (Scale for the Assessment and Rating of Ataxia)

- Measures coordination and movement during everyday activities
- **Even a 1-point change may reflect a meaningful change in daily function**

fSARA (Functional Scale for the Assessment and Rating of Ataxia)

- Measures 4 key areas that affect daily life: walking, sitting, standing, speaking

Since SARA evaluates more areas of neurological function than fSARA, these results may reflect improvements across a broader range of everyday activities affected by A-T.

“He could keep his balance better, fell less, and did not need that much support.”

— Parents of a child treated with AQNEURSA in the pivotal trial

Disclaimer: Quotes from the exit interviews reflect individual experiences with treatment with AQNEURSA. Results may not be typical.

Safety overview

AQNEURSA was generally well tolerated in the pivotal clinical study. The most common side effects were fall, skin laceration, and urinary tract infection.

Important Safety Information (cont'd)

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What to expect on AQNEURSA

Because A–T affects every individual differently, managing treatment is an ongoing partnership between you, your family, and your healthcare team.

A Unique Experience

No two people living with A–T experience symptoms at the exact same pace. Changes, progress, and response to treatment vary across individuals and age groups.

Staying Close to Your Healthcare Team

Regular check-ins with your doctor are key to tracking progress, discussing small daily observations, and making informed care decisions together.

Staying on Treatment

Do not stop without talking to your doctor. Taking AQNEURSA exactly as prescribed by your doctor supports ongoing treatment benefits.



Progress takes time

Improvements may happen gradually. Paying attention to small, meaningful changes in your regular routines helps build a complete picture of progress over time.

What changes should you and your doctor look for?

Use this checklist to notice changes and share them at your next appointment:

- ☐ **Walking and Mobility:**
Steadier movement, increased confidence moving around home or school.
- ☐ **Balance and Stability:**
Better balance while standing or sitting upright during meals.
- ☐ **Speech and Communication:**
Clearer articulation and easier communication with family and friends.
- ☐ **Hand and Arm Coordination:**
Greater control during everyday tasks like eating, writing, or handling objects.

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You are encouraged to report side effects of prescription drugs to the FDA.

Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.



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Dosing that fits into daily routines

AQNEURSA is taken by mouth and is dosed based on body weight, allowing treatment across pediatric and adult patients with A-T.

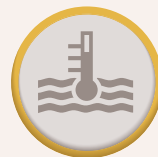
How AQNEURSA is taken



Easy Mixing: Oral powder mixed with water, orange juice, almond milk, or yogurt



Convenient Timing: Can be taken with or without food



G-Tube Friendly: Can also be administered through a gastrostomy (G) tube, when appropriate

How much AQNEURSA to take and how often to take it:

The amount of AQNEURSA taken and how many times it is taken each day is based on body weight.

2x
A DAY

15 kg (33 lb) or more, but less than 25 kg (55 lb)
1 packet every morning,
1 packet every evening

3x
A DAY

25 kg (55 lb) or more, but less than 35 kg (77 lb)
1 packet every morning,
1 packet every afternoon,
1 packet every evening

3x
A DAY

35 kg (77 lb) or more
2 packets every morning,
1 packet every afternoon,
1 packet every evening

Treatment made for real life, with easy-to-use, weight-based daily doses that can be taken at home or on the go and shipped directly to your doorstep.

Take AQNEURSA exactly as prescribed by your doctor.

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Start the conversation with your healthcare team

Before beginning treatment, it's important to understand what to expect and how your care will be managed. Use these questions to help guide a productive conversation with your healthcare team.

Consider asking:

- Is AQNEURSA appropriate for me or my loved one?
- What benefits were seen in the clinical study?
- When might treatment begin?
- How will we track changes in ataxia and daily activities over time?
- How is AQNEURSA taken?
- What should I expect during treatment?
- What support resources are available?

Additional questions:



Support Along the Way with AQNEURSA Cares

AQNEURSA Cares helps eligible patients and families navigate every step of the treatment journey through personalized support, coordination, and dedicated specialty pharmacy services through Curant Rare.

Support may include:

- Insurance benefits investigation and coverage support
- Prior authorization and treatment access assistance
- Personalized prescription coordination and refill support through Curant Rare
- Fast, free home delivery of AQNEURSA directly to patients and caregivers
- Ongoing patient outreach, education, and treatment support resources
- Co-pay assistance information for eligible commercially insured patients

Visit [AQNEURSA.com/AT](https://aqneurisa.com/AT) or scan the QR code to learn more about AQNEURSA Cares and available patient support services.



Scan to learn more

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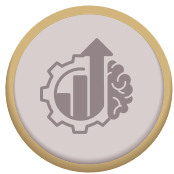


"This [treatment] made daily living activities easier for the patient to complete, particularly walking downstairs."

— Caregiver of a child treated with AQNEURSA in the pivotal trial

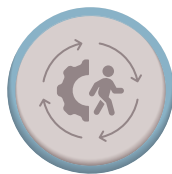
A breakthrough for your daily life

The first and only FDA-approved treatment for A-T



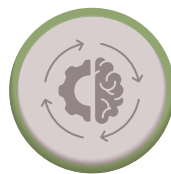
The first and only FDA-approved treatment for A-T

AQNEURSA is the first FDA-approved treatment for people living with A-T, offering a long-awaited option for children and adults affected by the condition.



Support for everyday activities

AQNEURSA may help improve abilities that matter most in everyday life, including walking, balance, coordination, speech, and other everyday tasks.



Proven safety and tolerability

In clinical studies, AQNEURSA was generally well-tolerated, with no treatment-related serious side effects reported.



Dosing designed to fit into daily life

AQNEURSA is an oral medication that can be taken with or without food, and administered through an existing G-tube, when appropriate.



Visit [AQNEURSA.com](https://www.aqneurisa.com) or scan the QR code to learn more and get started

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