AQNEURSA® is the first and only FDA-approved treatment for Ataxia-Telangiectasia (A-T)[1,2]

AQNEURSA® (levacetylleucine) for oral suspension

Only AQNEURSA meaningfully addresses ataxia in A-T.[2]

Actual patient not shown.

INDICATION

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

IMPORTANT SAFETY INFORMATION

Embryo-Fetal Toxicity

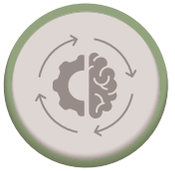
- Based on findings from animal reproduction studies, AQNEURSA may cause embryo-fetal harm when administered during pregnancy. The decision to continue or discontinue AQNEURSA treatment during pregnancy should consider the female's need for AQNEURSA, the potential drug-related risks to the fetus, and the potential adverse outcomes from untreated maternal disease.

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

Patients with A-T experience progressive ataxia and associated neurological deterioration, highlighting a significant unmet need for therapies targeting ataxia[3,4]

A-T is a relentlessly progressive, fatal disease that places a substantial burden on patients.[3,6,7]

Ataxia may be comprised of multiple signs and symptoms, including gait ataxia, truncal ataxia, oculomotor ataxia, and difficulties with balance, speech, and hand coordination.[1]



Ataxia is a progressive condition, with patients experiencing increasing disability and rarely regaining lost function.[3,6,7]

As deterioration progresses, patients face loss of independence requiring increasing levels of assistance and growing physical, emotional, and practical demands.[3,5,7-9]

Pediatric and adult patients with A-T may experience heterogeneous signs and symptoms of ataxia which may complicate recognition and contribute to delays in diagnosis. These diagnostic delays may contribute to ongoing disease burden.[3,10,11]

Historically, management has focused on supportive care rather than therapies that address ataxia, underscoring the importance of earlier diagnosis and proactive management to help optimize patient care and support.[3-5,10-12]

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

AQNEURSA™ (levacetylleucine)

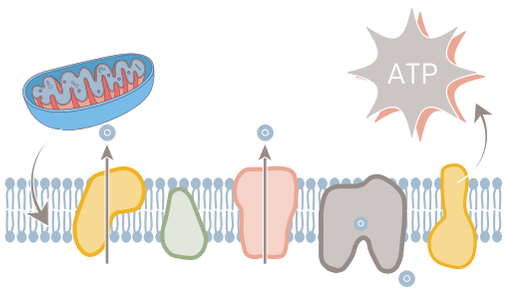
AQNEURSA is a first-in-class, chemically modified amino acid and the first FDA-approved treatment for ataxia in patients with A-T[1,2]

The distinct molecular target for AQNEURSA in the treatment of A-T is unknown.[1]

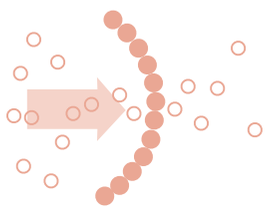
- The proposed mechanism of action for AQNEURSA is the activation of cerebellar glucose metabolism, correlated with enhanced cerebellar activity[2,13,14]
- AQNEURSA is designed to enter enzyme-controlled pathways to correct metabolic dysfunction, improve lysosomal function, and enhance mitochondrial function and ATP production[2,13,14]

Preclinical (murine and other animal) studies suggested AQNEURSA:

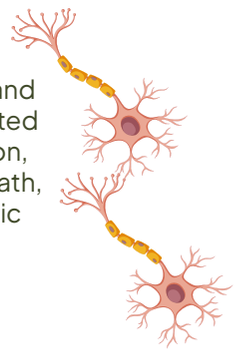
Improved mitochondrial function
Generated more energy (ATP) and helped restore metabolic balance through enhanced mitochondrial performance.[2,14]



Normalized lysosomal function and reduced substrate accumulation
Supported lysosomal function and improved cellular homeostasis by reducing accumulation of metabolic substrates.[2,14]



Restored cellular signaling and reduced neuroinflammation
Promoted cellular health and neuronal function, protected against cellular dysfunction, attenuated further cell death, and helped prevent chronic neurodegeneration.[2,13,14]



These results have not been confirmed in human trials.

ATP=adenosine triphosphate.

IMPORTANT SAFETY INFORMATION

Pregnancy and Lactation

- For females of reproductive potential, verify that the patient is not pregnant prior to initiating treatment with AQNEURSA. Advise females of reproductive potential to use effective contraception during treatment with AQNEURSA and for 7 days after the last dose if AQNEURSA is discontinued.
- There are no data on the presence of levacetylleucine or its metabolites in either human or animal milk, the effects on the breastfed infant or the effects on milk production. The developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for AQNEURSA and any potential adverse effects on the breastfed infant from levacetylleucine or from the underlying maternal condition.

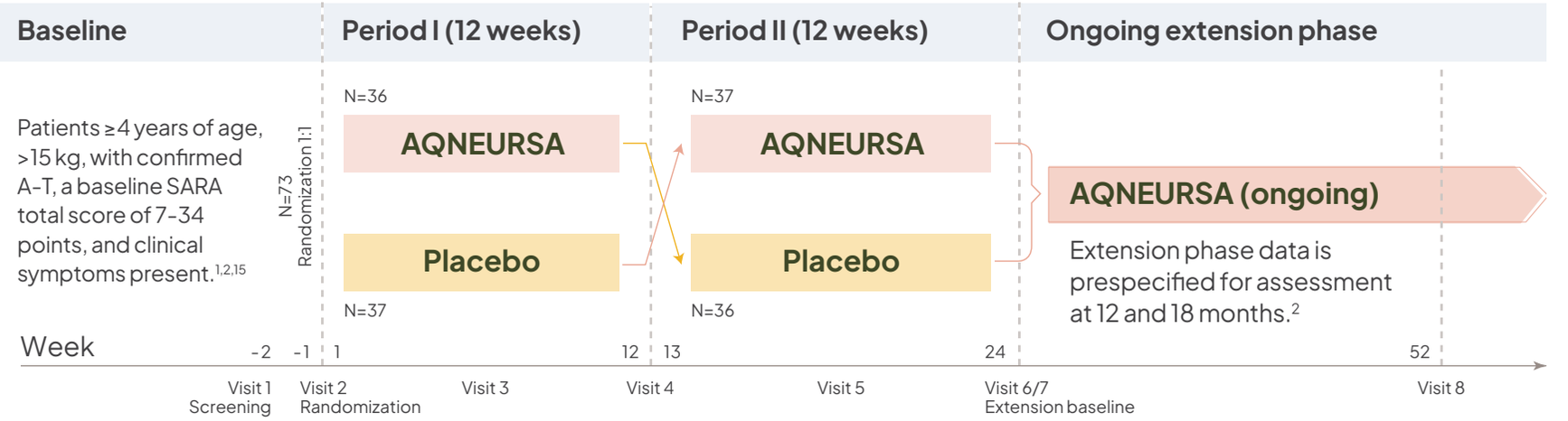
Please see additional Important Safety Information throughout and Full Prescribing Information.

AQNEURSA™ (levacetylleucine)

FDA approval of AQNEURSA for patients with A-T was granted based on a robust pivotal study[1,2]

AQNEURSA was evaluated in a phase 3, multinational, randomized (1:1), double-blind, placebo-controlled, crossover trial (N=73).[1,2]

The primary endpoint was the functional Scale for the Assessment and Rating of Ataxia (fSARA), and supported by the Scale for Assessment and Rating of Ataxia (SARA)[1,2]



- Patients received AQNEURSA for 12 weeks and placebo for 12 weeks, with patients randomized 1:1 to receive AQNEURSA or placebo first[1]
- AQNEURSA or placebo were administered orally during each treatment period[1,2,15]
- Seventy of 73 patients completed the study and received both placebo and AQNEURSA[2]
- Baseline characteristics included a median age of 13 years (range, 4–50 years); 52% of patients were female, and 47 of 73 patients were pediatric[1]
- The safety profile of AQNEURSA was evaluated across pediatric and adult patient populations[1]
- Patients weighing ≥35 kg received 4 g/day (2 g in the morning, 1 g in the afternoon, and 1 g in the evening); patients weighing <35 kg received weight-based dosing[1]

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

AQNEURSA™ (levacetylleucine)

AQNEURSA significantly improved signs and symptoms of ataxia and functional outcomes vs placebo as measured by fSARA[1,2*]

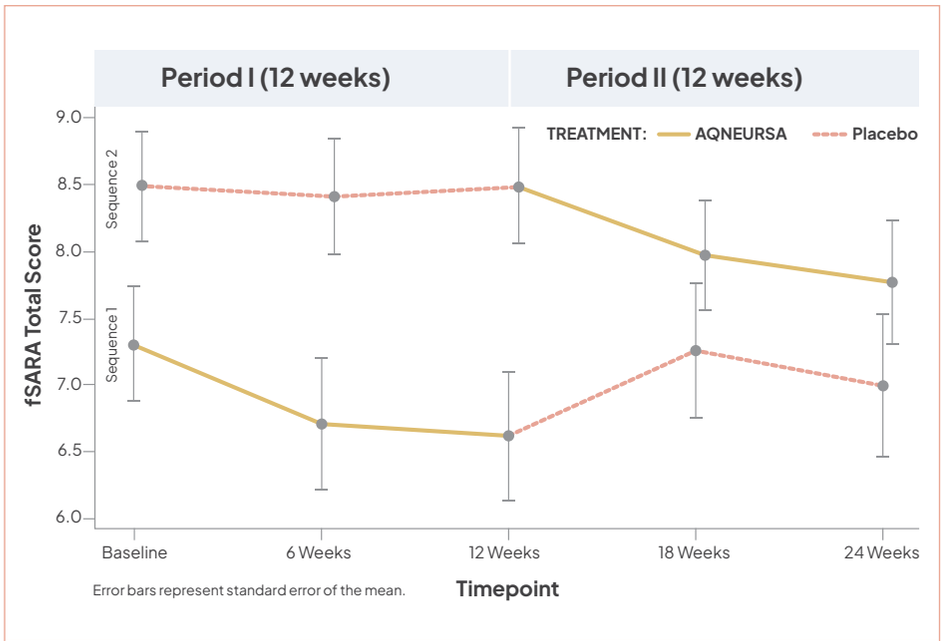
Estimated treatment benefit of **−0.6 points**

In mean total fSARA score with AQNEURSA vs placebo (95% CI: −0.9 to −0.2; two-sided *P*=0.0013).[1,2]

Consistent improvements in mean total fSARA score from baseline with AQNEURSA in both treatment sequences[1,2]:

- Sequence 1 (AQNEURSA in Period I; placebo in Period II): **−0.7 points (SD: 1.0) with AQNEURSA** vs -0.2 points (SD: 1.1) with placebo
- Sequence 2 (placebo in Period I; AQNEURSA in Period II): **−0.6 points (SD: 1.3) with AQNEURSA** vs 0.0 points (SD: 0.9) with placebo

These important benefits to everyday functions were evident within 12 weeks[16,17]



*The fSARA score was assessed at baseline, 6 weeks, 12 weeks (end of Period I), 18 weeks, and 24 weeks (end of Period II). Mean baseline fSARA scores were 7.3 (SD 2.6) for Sequence 1 (AQNEURSA-placebo) and 8.5 (SD 2.5) for Sequence 2 (placebo-AQNEURSA). The least squares mean fSARA total score was 7.2 with AQNEURSA and 7.8 with placebo. The least squares treatment effect was -0.6 points (95% CI, -0.9 to -0.2; *P*=0.0013). Patients receiving AQNEURSA demonstrated improvement, whereas those receiving placebo showed no improvement or worsening outcomes, supporting the treatment effect observed in the study.[1,2]

CI=confidence interval; SD=standard deviation.

IMPORTANT SAFETY INFORMATION

Adverse Reactions

- The most common adverse reactions (incidence ≥5% and greater than placebo) are fall, skin laceration, and urinary tract infection.

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

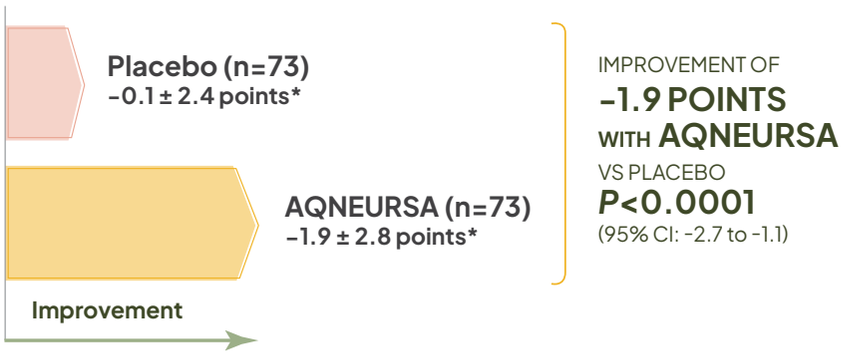
AQNEURSA™ (levacetylleucine)

AQNEURSA demonstrated clinically meaningful improvement in total SARA score vs placebo, reinforcing improvements in ataxia[1,2]

AQNEURSA significantly improved signs and symptoms of ataxia as assessed by SARA[1,2]

A 1-point change in total SARA score is considered clinically meaningful. At 12 weeks, AQNEURSA demonstrated a -1.9 point improvement, nearly double this threshold.[1,2]

Mean change from baseline in total SARA score at 12 weeks



IMPROVEMENT OF
-1.9 POINTS
WITH **AQNEURSA**
VS PLACEBO
***P*<0.0001**
(95% CI: -2.7 to -1.1)

Improvements in SARA and fSARA scores with AQNEURSA support meaningful improvements in ataxia. Worsening following AQNEURSA withdrawal further supports the treatment effect.[1,2]

"He could keep his balance better, fell less, and did not need that much support."[15†‡]

Parent of a child in the pivotal AQNEURSA trial.

*Baseline SARA scores averaged approximately 19.8. Mean changes over the course of the 2 treatment periods were consistent with a treatment effect, with improvement during AQNEURSA treatment and, among patients who received AQNEURSA first, worsening during the subsequent placebo period after AQNEURSA withdrawal.[2]

†70 exit interviews were conducted with patients, families, and caregivers following the pivotal trial. Quotes from the exit interviews reflect individual experiences of treatment with AQNEURSA. Results may not be typical.[2]

‡Quote taken from caregiver-reported qualitative findings in A-T phase 3 trial exit interviews, summarized in the Supplementary Appendix (Table S3).[15]

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

IMPORTANT SAFETY INFORMATION

Drug Interactions

- Avoid concomitant use of AQNEURSA with *N-acetyl-DL-leucine* or *N-acetyl-D-leucine*. The D-enantiomer, N-acetyl-D-leucine, competes with levacetylleucine for monocarboxylate transporter uptake, which may reduce the levacetylleucine efficacy.
- Monitor more frequently for P-gp substrate related adverse reactions when used concomitantly with AQNEURSA. AQNEURSA inhibits P-gp; however, the clinical significance of this finding has not been fully characterized.

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

AQNEURSA™ (levacetylleucine)

AQNEURSA has an established safety and tolerability profile in patients with A-T[1,2*]

Adverse reactions that occurred in adult and pediatric patients with A-T at an incidence of ≥5% in treatment Period I.[1†]

Adverse reaction	AQNEURSA (N=36) n (%)	Placebo (N=37) n (%)
Fall	2 (6)	1 (3)
Skin laceration	2 (6)	0 (0)
Urinary tract infection	2 (6)	1 (3)

Select safety observations

- **AQNEURSA was generally well tolerated in the pivotal phase 3 study[2]**
  Adverse reactions observed during Treatment Period II were similar to those observed during Treatment Period I[2]
- **No new safety signals were identified during the pivotal trial[2]**

*Patients were enrolled in the pivotal phase 3 study (IB1001-303), including both pediatric and adult patients with ataxia-telangiectasia.[2]
†Safety was evaluated in patients exposed to AQNEURSA across the clinical development program, including pediatric and adult patients with A-T.[1,2]

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

AQNEURSA™ (levacetylleucine)

AQNEURSA features oral dosing and administration that are designed to fit into patients' daily routines[1]

Weight-based dosing enables use across the broad age range of patients with A-T.[1]

AQNEURSA can be taken at home or on the go, offering convenience and flexibility, and is shipped directly to the patient's door.[1]

Recommended dosage and schedule of AQNEURSA based on body weight[1]

Body weight	Morning dose	Afternoon dose	Evening dose
15 kg to <25 kg	1 gram	No dose	1 gram
25 kg to <35 kg	1 gram	1 gram	1 gram
≥35 kg	2 grams	1 gram	1 gram

- AQNEURSA is administered by fully suspending a dose into 1 of a variety of cold or room-temperature liquids (water, orange juice, almond milk, or yogurt)[1]
- Swallow within 30 minutes. AQNEURSA can be taken with or without food[1]
- Each AQNEURSA packet contains 1 gram of levacetylleucine[1]
- For 2-gram doses in patients ≥35 kg, 2 packets of AQNEURSA should be prepared and taken individually[1]

In addition to oral administration, AQNEURSA may be administered via a gastrostomy tube, when appropriate.[1]

Please see the Full Prescribing Information and Instructions for Use for details on the administration of AQNEURSA

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

AQNEURSA™ (levacetylleucine)

Actual patient not shown.

The AQNEURSA Cares Program helps eligible patients access treatment

AQNEURSA™ CARES

Visit AQNEURSAhcp.com or scan the QR code to get started

Visit AQNEURSAhcp.com to download the Start AQNEURSA Prescription Form and begin the enrollment process:

Prescribe AQNEURSA by completing the form and including clinical information from the Prior Authorization Checklist

Fax the completed and signed form to AQNEURSA Cares at 470–751–8639

Inform the patient that AQNEURSA Cares will reach out to them

Please see Important Safety Information throughout and Full Prescribing Information.

AQNEURSA™ (levacetylleucine)

INDICATION

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

IMPORTANT SAFETY INFORMATION

Embryo-Fetal Toxicity

- Based on findings from animal reproduction studies, AQNEURSA may cause embryo-fetal harm when administered during pregnancy. The decision to continue or discontinue AQNEURSA treatment during pregnancy should consider the female's need for AQNEURSA, the potential drug-related risks to the fetus, and the potential adverse outcomes from untreated maternal disease.

Pregnancy and Lactation

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Adverse Reactions

- The most common adverse reactions (incidence ≥5% and greater than placebo) are fall, skin laceration, and urinary tract infection.

Drug Interactions

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To report SUSPECTED ADVERSE REACTIONS, contact IntraBio Inc. at **1-833-306-9677** or FDA at **1-800-FDA-1088** or **www.fda.gov/medwatch**.

Please click here for Full Prescribing Information for AQNEURSA.

References: 1. AQNEURSA. Prescribing Information. IntraBio. **2.** Martakis K, Bremova-Ertl T, Bolton C, et al. Safety and efficacy of levacetylleucine in ataxia-telangiectasia: a phase 3, randomized, double-blind, placebo-controlled crossover trial. *Lancet Neurol*. 2026;25(7):633–644. doi:10.1016/S1474–4422(26)00158–4 **3.** McGrath-Morrow SA, Rothblum-Oviatt C, Wright J, et al. Multidisciplinary management of ataxia telangiectasia: current perspectives. *J Multidiscip Healthc*. 2021;14:1637–1644. doi: 10.2147/JMDH.S295486 **4.** Rothblum-Oviatt C, Wright J, Lefton-Greif MA, et al. Ataxia telangiectasia: a review. *Orphanet J Rare Dis*. 2016;11(1):159. doi:10.1186/s13023–016–0543–7 **5.** Schoenaker MHD, Blom M, de Vries MC, Weemaes CMR, van der Burg M, Willemsen MAAP. Early diagnosis of ataxia telangiectasia in the neonatal phase: a parents' perspective. *Eur J Pediatr*. 2020;179(2):251–256. doi:10.1007/s00431–019–03479–5 **6.** Kiotchuoukova I, Foster D, Thakkar R, et al. Neurocutaneous diseases: diagnosis, management, and treatment. *J Clin Med*. 2024;13(6):1648. doi:10.3390/jcm13061648 **7.** Van Os NJH, van Deuren M, Weemaes CMR, et al. Classic ataxia-telangiectasia: the phenotype of long-term survivors. *J Neurol*. 2020;267(3):830–837. doi:10.1007/s00415–019–09641–1 **8.** Veenhuis S, van Os N, Weemaes C, Kamsteeg EJ, Willemsen M. Ataxia-Telangiectasia. In: Adam MP, Bick S, Mirzaa GM, Pagon RA, Wallace SE, Amemiya A, eds. *GeneReviews®* [Internet]. University of Washington, Seattle; 1993–2026. **9.** Kelly MJ, Bogdanova-Mihaylova P, Skeens J, et al. The cost of living with inherited ataxia in Ireland. *Cerebellum*. 2022;21(2):280–296. doi:10.1007/s12311–021–01271–6 **10.** Devaney R, Pasalodos S, Suri M, Bush A, Bhatt JM. Ataxia telangiectasia: presentation and diagnostic delay. *Arch Dis Child*. 2017;102(4):328–330. doi:10.1136/archdischild-2016–310477 **11.** Cabana MD, Crawford TO, Winkelstein JA, Christensen JR, Lederman HM. Consequences of the delayed diagnosis of ataxia-telangiectasia. *Pediatrics*. 1998;102(1 Pt 1):98–100. doi:10.1542/peds.102.1.98 **11.** Rothblum-Oviatt C, Wright J, Lefton-Greif MA, et al. Ataxia telangiectasia: a review. *Orphanet J Rare Dis*. 2016;11(1):159. doi:10.1186/s13023–016–0543–7 **12.** Petley E, Yule A, Alexander S, Ohja S, Whitehouse WP. The natural history of ataxia-telangiectasia (A-T): a systematic review. *PLoS One*. 2022;17(3):e0264177. doi:10.1371/journal.pone.0264177 **13.** Fields T, Patterson M, Bremova-Ertl T, et al. A master protocol to investigate a novel therapy acetyl-L-leucine for three ultra-rare neurodegenerative diseases: Niemann-Pick type C, the GM2 gangliosidoses, and ataxia telangiectasia. *Trials*. 2021;22(1):84. doi:10.1186/s13063–020–05009–3 **14.** Kaya E, Smith DA, Smith C, et al. Acetyl-leucine slows disease progression in lysosomal storage disorders. *Brain Commun*. 2020;3(1):fcaa148. doi:10.1093/braincomms/fcaa148 **15.** Martakis K, Bremova-Ertl T, Bolton C, et al. Safety and efficacy of levacetylleucine in ataxia-telangiectasia: a phase 3, randomized, double-blind, placebo-controlled crossover trial. *Lancet Neurol*. 2026;25(7):633–644. Supplementary appendix. doi:10.1016/S1474–4422(26)00158–4 **16.** Data on file. IntraBio Inc. **17.** Data on file. IntraBio Inc.

AQNEURSA is the first and only treatment for A-T and is approved for adult and pediatric patients[1,2]*

AQNEURSA™ (levacetylleucine)

The primary efficacy outcome was assessed using the functional Scale for Assessment and Rating of Ataxia (fSARA), as supported by the Scale for Assessment and Rating of Ataxia (SARA).[1]

Demonstrated Efficacy

Improvement demonstrated on the primary endpoint in the pivotal trial, supporting the clinical benefit of AQNEURSA.[1,2†]

Well Tolerated

AQNEURSA has an established safety and tolerability profile, supporting use in appropriate patients with A-T.[1,2]

Convenient Administration

Weight-based oral dosing designed to fit into patients' daily routines.[1]

Time Matters in A-T. Treatment is Here.™

Prescribe AQNEURSA to address the unmet need for a treatment that has demonstrated clinically meaningful benefit for patients living with A-T.[1-4]

"Small gains in everyday activities can make a meaningful difference."[15†‡]

Parent of a child in the pivotal AQNEURSA trial.

*AQNEURSA is indicated for the treatment of ataxia in adults and pediatric patients with ataxia-telangiectasia (A-T) weighing ≥15 kg.[1]

†The primary endpoint, functional SARA (fSARA), demonstrated a treatment difference of -0.6 (95% CI: -0.9, -0.2; *P*=0.0013).[1]

‡SARA total score at Week 12. Mean SARA score was 18.0 with AQNEURSA vs 19.7 with placebo (linear mixed model treatment effect: -1.9 [95% CI: -2.7, -1.1]; *P*<0.0001). Lower scores indicate better neurological status.[2]

§70 exit interviews were conducted with patients, families, and caregivers following the pivotal trial. Quotes from the exit interviews reflect individual experiences of treatment with AQNEURSA. Results may not be typical.[2]

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

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IntraBio