

Prior Authorization (PA) Checklist For AQNEURSA® (levacetylleucine)

Review this resource to help understand potential payor coverage requirements

Drug information ¹	AQNEURSA is supplied in unit dose packets, each containing 1 g of levacetylleucine as granules for oral suspension. Dosing:			
	Patient Body Weight	Morning Dose	Afternoon Dose	Evening Dose
	15 to <25 kg	1 g	No Dose	1 g
	25 to <35 kg	1 g	1 g	1 g
	35 kg or more	2 g	1 g	1 g
ICD-10-CM diagnosis code ²	E75.242 Niemann-Pick disease type C (NPC)		G11.3 Ataxia-Telangiectasia (A-T)* *Official descriptor: Cerebellar ataxia with defective DNA repair	
Diagnostic information	• Confirmation of NPC diagnosis by genetic testing identifying disease-causing alleles in NPC1 or NPC2 • Biomarker screening • Filipin test • Observation of the clinical features of NPC, such as³: - Neurologic and psychiatric – eg, developmental delay/regression, ataxia, VSGP, hearing loss, cataplexy, seizures, motor-function decline, movement disorders, ocular motor impairment, cognitive impairment, dysphagia, tremors, speech impairments - Systemic – eg, hepatosplenomegaly or splenomegaly (isolated or with neurological manifestations), history of prolonged neonatal cholestatic jaundice - NOTE to HCP: not all clinical features are listed here so please include any additional clinical features observed		• Confirmation of A-T diagnosis by genetic testing identifying biallelic pathogenic variants in the ATM gene • Elevated serum alpha-fetoprotein (AFP) • Observation of the clinical features of A-T, such as⁴: - Neurologic features – e.g., progressive cerebellar ataxia, which may include gait ataxia, truncal ataxia, oculomotor ataxia, difficulties with balance, trouble with speech, and hand incoordination; dysphagia; movement disorders (chorea, dystonia, athetosis, and myoclonic jerks); peripheral neuropathy; and cognitive decline - Systemic and Other clinical features – oculocutaneous telangiectasia, immunodeficiency, pulmonary disease, endocrine abnormalities - NOTE to HCP: not all clinical features are listed here so please include any additional clinical features observed	
Patient condition and medical history	• Patient weight, growth, and development • Neurological and/or psychological testing results • Symptoms and quality of life (physician observation) • Medication history – dose, dates of use, side effects, etc • Chart notes • Laboratory test results • Verification that the patient is not pregnant • Details on hospital admissions and discharges			
Additional information	• The following materials may help payors learn more about AQNEURSA and can be submitted with a PA or formulary exception request: - Prescribing Information - Clinical studies published in peer-reviewed journals (Available from Medical Information at medinfo@intrabio.com or phone (833) 306-9677) • Visit www.aqneursahcp.com or contact AQNEURSA Cares at 866-200-0419 for additional support			

Key: ICD-10-CM – International Classification of Diseases, 10th Revision, Clinical Modification; VSGP – vertical supranuclear gaze palsy.

Please see Indication and Important Safety Information on Page 2 and click [here for Full Prescribing Information](#).

INDICATION

AQNEURSA[®] (levacetylleucine) is indicated for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing ≥ 15 kg.

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

- 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.

Adverse Reactions

- In Niemann-Pick disease type C, the most common adverse reactions (incidence $\geq 5\%$ and greater than placebo) are abdominal pain, dysphagia, upper respiratory tract infections, and vomiting.
- In Ataxia-Telangiectasia, the most common adverse reactions (incidence $\geq 5\%$ and greater than placebo) are fall, skin laceration, and urinary tract infection.

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.

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

- AQNEURSA. Prescribing information. IntraBio.
- Centers for Medicare & Medicaid Services (CMS). International Classification of Diseases, 10th Revision, Clinical Modification ICD10-CM tabular list of diseases and injuries. <https://www.cms.gov/medicare/coding-billing/icd-10-codes>
- Geberhiwot T, Moro A, Dardis A, et al; International Niemann-Pick Disease Registry (INPDR). Consensus clinical management guidelines for Niemann-Pick disease type C. *Orphanet J Rare Dis*. 2018;13(1):50. doi:10.1186/s13023-018-0785-7
- 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

Disclaimer: This information is provided for informational purposes only. IntraBio makes no representation or guarantee concerning coverage or reimbursement. Please check individual payor policies for plan-specific coverage information and requirements. This is not a comprehensive description of potential payor access and coverage requirements. The prescriber is solely responsible for determining coverage and reimbursement requirements and submitting the necessary information to payors. Nothing within this resource is intended to be a substitute for, or influence on, prescribers' independent medical judgement.

AQNEURSA[®], AQNEURSA Cares[®], and their respective logos are registered trademarks of IntraBio Inc. ©2026 IntraBio. All rights reserved. MAT-0037 v2 09/26