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

Review this resource to help understand potential payor coverage requirements

<table>
<tr><td>Drug information[1]</td><td>AQNEURSA is supplied in unit dose packets, each containing 1 g of levacetylleucine as granules for oral suspension.
Dosing:
<table>
<tr><th>Patient Body Weight</th><th>Morning Dose</th><th>Afternoon Dose</th><th>Evening Dose</th></tr>
<tr><td>15 to <25 kg</td><td>1 g</td><td>No Dose</td><td>1 g</td></tr>
<tr><td>25 to <35 kg</td><td>1 g</td><td>1 g</td><td>1 g</td></tr>
<tr><td>35 kg or more</td><td>2 g</td><td>1 g</td><td>1 g</td></tr>
</table></td></tr>
<tr><td>ICD-10-CM diagnosis code[2]</td><td>E75.242 Niemann-Pick disease type C (NPC)</td></tr>
<tr><td>Diagnostic information</td><td>• 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 as[3]:
- 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</td></tr>
<tr><td>Patient condition and medical history</td><td>• 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</td></tr>
<tr><td>Additional information</td><td>• 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.aqneursa.com or contact AQNEURSA Cares at 866-200-0419 for additional support</td></tr>
</table>

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

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.

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 on Page 2 and click here for Full Prescribing Information.

IMPORTANT SAFETY INFORMATION (continued)

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

- The most common adverse reactions (incidence ≥5% and greater than placebo) are abdominal pain, dysphagia, upper respiratory tract infections, and vomiting.

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

1. AQNEURSA. Prescribing information. IntraBio.
2. Centers for Medicare & Medicaid Services (CMS). 2024 International Classification of Diseases, 10th Revision, Clinical Modification ICD10-CM tabular list of diseases and injuries. Updated February 1, 2024. Accessed June 5, 2024 https://www.cms.gov/medicare/coding-billing/icd-10-codes
3. 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