



TO: US Niemann-Pick Disease type C Patient Community

FROM: Taylor Fields, Chief Development Officer & President Product Development IntraBio

SUBJECT: AQNEURSA™ (levacetylleucine) is Now FDA Approved and Available for Eligible Patients

Dear NPC Community,

IntraBio is pleased to announce that as of September 25th, 2024, the U.S. Food and Drug Administration (FDA) has approved AQNEURSA™ a new therapeutic treatment for children and adults living with Niemann-Pick Disease (NPC) weighing ≥ 15 kg.

We at IntraBio have long understood the NPC community's unmet need and the long delay in the availability of FDA-approved treatment options. We are extremely excited to provide families with a new stand-alone FDA-approved treatment for NPC. We would like to express our deepest gratitude to the individuals, families, advocates, clinicians, and organizations who have supported, collaborated, and contributed to the development of AQNEURSA. Your courage and commitment have been instrumental in this journey to approval.

IntraBio is honored to be part of such a passionate and dedicated community and looks forward to continuing to learn from and collaborate with you to make a difference in the lives of individuals and families living with NPC. We aim to champion research that improves the diagnosis and detection of NPC, partner to advance the care and management of NPC, and support and empower individuals and families affected by NPC while providing the best therapeutic options for this community.

You can find more information about AQNEURSA, including important safety information at [AQNEURSA.com](https://www.intrabio.com/aqneurasa). A full press release can be found [here](#).

INDICATION & IMPORTANT SAFETY 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.

IMPORTANT SAFETY INFORMATION

Do not take AQNEURSA if you:

- have not had a negative pregnancy test. If you become pregnant while taking AQNEURSA, inform your doctor immediately to discuss potential risks and alternative treatments

Before taking AQNEURSA, discuss with your doctor if you:

- are pregnant or planning to become pregnant. Based on findings from animal reproduction studies, AQNEURSA may cause harm to your unborn baby when administered during pregnancy. If you become pregnant while taking AQNEURSA, inform your doctor immediately to discuss potential risks and alternative treatments.
- require the need for effective contraception during treatment with AQNEURSA and for 7 days after stopping treatment to avoid pregnancy.
- are breastfeeding or plan to breastfeed. It is not known if AQNEURSA or its metabolites pass into human or animal milk or effects on breastmilk production
- are taking P-gp substrate. Your doctor will monitor you for related adverse reactions
- have any other medical conditions
- are taking any medications and supplements, including *N-acetyl-DL-leucine* or *N-acetyl-D-leucine*, as some of these may impact the effectiveness of AQNEURSA

The most common side effects of AQNEURSA include abdominal pain, dysphagia, upper respiratory tract infections and vomiting. Speak with your doctor if these side effects persist or worsen.

Take AQNEURSA exactly as prescribed by your doctor.

If a dose of AQNEURSA is missed, skip the missed dose and take the next dose at the scheduled time. Do not take 2 doses at the same time to make up for a missed dose. AQNEURSA can be taken with or without food.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call [1-800-FDA-1088](tel:1-800-FDA-1088).

Please click [here for full Prescribing Information for AQNEURSA](#).

How can patients access AQNEURSA?

The best way to way to access AQNEURSA is to speak with your prescribing physician to determine if AQNEURSA is right for you. If so, your doctor will fill out an Enrollment Form and return it to our specialty pharmacy, Curant Health. This can be done online, and the Enrollment Form is available at <https://aqneursa.com/wp-content/enrollment-form.pdf>. Filling out an Enrollment Form is one of the first steps in the path to accessing AQNEURSA and the full suite of support services.

Below are answers to common questions we've been hearing from the community, which should help you and your family members further understand next steps:

ENROLLMENT FORM

Where does the physician send the Enrollment Form?

The prescribing physician is required to fax the signed and completed Enrollment Form to our Patient Support Services, AQNEURSA Cares™, at 470-751-8639. The form must be signed by the physician and patient for the patient to be considered for patient support services and financial programs.

What happens after AQNEURSA Cares receives an Enrollment Form?

A Care Coordinator will contact the patient or family member first about next steps, including:

- Benefits investigation
- Prior authorization and/or appeal
- Working to assist the physician if a claim is denied
- Referral to a financial assistance program if needed
- If applicable, updates about AQNEURSA availability

How do patients receive AQNEURSA?

AQNEURSA™ will be mailed directly to the patient's home by Curant, our rare disease-experienced specialty pharmacy. The pharmacist will call prior to shipping the medicine to ensure that all mailing information is correct and that someone will be home to accept the delivery the next day. You will not be able to get or pick up AQNEURSA at a retail or neighborhood pharmacy.

PATIENT SUPPORT SERVICES

What patient support services are offered?

IntraBio offers support programs to eligible patients through our Patient Support Service, AQNEURSA Cares™. This program includes financial support to reduce or eliminate out-of-pocket costs for qualifying patients and also connects patients with third-party resources.

AQNEURSA Cares includes access to financial and educational resources and a dedicated team of specialists. The team is available to help with individuals' unique challenges including starting treatment, questions about taking the medication, and navigating insurance coverage. You can contact 866-200-0419 to speak to an AQNEURSA Cares team representative to assist with any questions or concerns you may have about your access to AQNEURSA.

Do I need to enroll in AQNEURSA Cares?

Enrolling in AQNEURSA Cares is important to helping ensure eligible patients have access to our robust support offerings, including prescription confirmations, verification of insurance coverage, and medication shipments. Patients can still receive their medication without enrolling in the program.

As we deliver AQNEURSA to the NPC community, we are committed to helping individuals understand the process and address questions and issues. Please visit AQNEURSA.com to learn more information.

Sincerely,

Taylor Fields
Chief Development Officer & President Product Development
IntraBio

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