Introducing

AQNEURSA™
(levacetylleucine)

Act Today, Improve Tomorrow.

AQNEURSA™ is the only FDA-approved stand-alone therapy for the treatment of Niemann-Pick Disease Type C (NPC) that improved neurological symptoms and functional abilities within 12 weeks in the pivotal trial.*

Indication & Important Safety Information

AQNEURSA™ (levacetylleuchine) is indicated for the treatment of neurological manifestations of Niemann-Pick Disease Type C (NPC) in adults and pediatric patients weighing ≥15 kg.

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

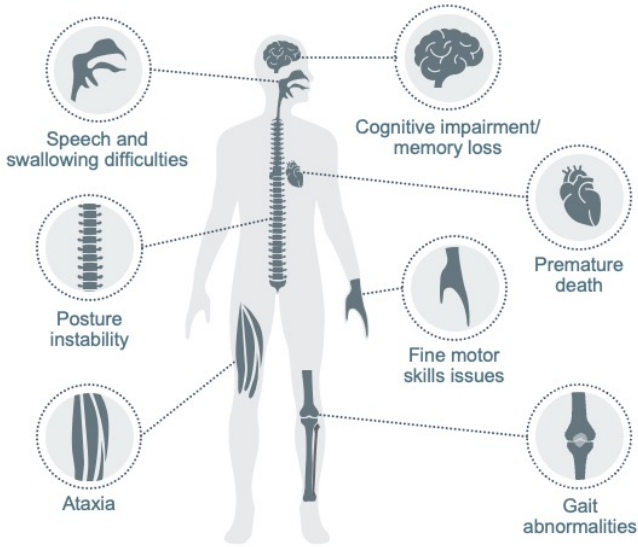
Please see additional Important Safety Information throughout and click here for Full Prescribing Information for AQNEURSA

What is Niemann-Pick Disease Type C (NPC)?

Niemann-Pick Disease Type C (NPC) is an ultra-rare progressive neurodegenerative disease caused by mutations in the NPC1 or NPC2 genes that lead to neurological and physical symptoms. The condition can also get worse quickly, causing life-threatening complications.[1,2]

NPC can be identified by the following symptoms[3,4]:

The most common symptoms of NPC include walking and fine-motor difficulties, problems with speech and swallowing, cognitive impairment, and seizures. People may also have trouble eating, drinking, and taking care of themselves.



Areas affected in individuals living with NPC

NPC is defined by neurological, systemic, and psychiatric challenges that ultimately result in serious functional and cognitive decline. It can have a profound impact on a person's functional abilities. It may also impact that person's parent, caregiver, or family, and people with NPC may require full-time care.[1,3,4]

What is AQNEURSA™?

AQNEURSA (levacetylleucine) is an approved treatment for people living with Niemann-Pick Disease Type C (NPC). AQNEURSA is an orally administered, modified amino acid. Its safety and tolerability profile have been studied extensively in clinical trials.

AQNEURSA is administered orally and should be taken 1–3 times per day as directed by your doctor by dissolving and mixing it in a liquid like water, orange juice, or almond milk and drinking it. It can also be given through a gastrostomy tube (G-tube).

Could AQNEURSA be right for you?

Finding the right treatment is important. Speak with your doctor to see if AQNEURSA is right for you.

How AQNEURSA can help

In clinical trials, AQNEURSA patients showed improvement in many activities of daily living that support everyday functional independence, including walking, balance, speech, and fine motor skills.

The primary efficacy outcome was assessed using a modified version of the Scale for Assessment and Rating of Ataxia (SARA), referred to as the functional SARA (fSARA). The SARA is a clinical assessment tool that assesses gait, stability, speech, and upper and lower limb coordination across 8 individual domains. The fSARA consists only of gait, sitting, stance, and speech disturbance domains of the original SARA with modifications to the scoring responses. Results on the fSARA were supported by consistent results demonstrated on the original SARA.

Important Safety Information

What are the possible side effects of AQNEURSA?

The most common reactions while patients were on AQNEURSA in the clinical trial were abdominal pain, dysphagia (difficulty swallowing), upper respiratory tract infections and vomiting.

Please see additional Important Safety Information throughout and **click here** for Full Prescribing Information for AQNEURSA

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 AQNEURS

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.

Please click here for Full Prescribing Information for AQNEURSA.

Where do I get AQNEURSA?

A doctor can write you a prescription for AQNEURSA. AQNEURSA is not available at a retail or neighborhood pharmacy. AQNEURSA is distributed by Curant Health, our rare disease-experienced specialty pharmacy. In order to receive access to AQNEURSA have your doctor visit www.aqneursa.com and complete the enrollment form.

AQNEURSA Cares Program

Patients with commercial insurance may be eligible for assistance with their co-pay. If you are unable to afford your co-pay and you have commercial insurance (employer-sponsored or individual), you may be eligible for co-pay assistance where allowed by law. For more information visit www.aqneursa.com or scan the QR Code below.

AQNEURSA™ CARES

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To see the full prescribing information, please visit https://intrabio.com/wp-content/aqneursa-prescribing-information.pdf or scan the QR code

*Neurological symptoms and functional outcomes were assessed using a modified version of the Scale for Assessment and Rating of Ataxia (SARA), referred to as the functional SARA (fSARA). Patients who received AQNEURSA first in Period I followed by placebo second in Period II (Treatment Sequence 1) showed a greater improvement in the fSARA score in Period I, with a mean change from baseline of -0.5 (standard deviation: 1.2), compared to Period II with a mean change from baseline of 0 (1.5). Similarly, patients who received placebo first in Period I followed by AQNEURSA second in Period II (Treatment Sequence 2) experienced greater improvement in the fSARA score while receiving AQNEURSA in Period II, with a mean change of -0.7 (0.9), compared to a mean change of -0.3 (0.9) in Period 1.

References

1. Burton BK, Ellis AG, Orr B, et al. Estimating the prevalence of Niemann-Pick disease Type C (NPC) in the United States. Mol Genet Metab 2021:134:182–187. **2.** Bremova-Ertl T, Ramaswami U, Brands M, et al. Trial of N-Acetyl-l-Leucine in Niemann-Pick Disease Type C. N Engl J Med. 2024;390:421–431. **3.** Vanier MT. Niemann-Pick disease type C. Orphanet J Rare Dis. 2010;5:16. **4.** Geberhiwot T, Moro A, Dardis A, et al. Consensus clinical management guidelines for Niemann-Pick disease type C. Orphanet J Rare Dis. 2018;13(1):50

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