**Sample Letter of Medical Necessity for
AQNEURSA™ (levacetylleucine)**

*This sample letter of medical necessity is designed to assist with* *access to AQNEURSA during the coverage review process.**This templated letter is designed as a framework only and should be customized appropriately by your office and for each patient.*

*As a healthcare professional, you are solely responsible for providing accurate information to payors. The information in the letter your office prepares should accurately reflect your patient’s history and your clinical rationale as to why AQNEURSA is medically necessary.*

**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 judgment.**

**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.

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](http://www.fda.gov/medwatch).

**Please click** [**here for Full Prescribing Information for AQNEURSA.**](https://intrabio.com/wp-content/aqneursa-prescribing-information.pdf)

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**AQNEURSA Letter of Medical Necessity Checklist**

Submitting a letter of medical necessity that includes the following elements may help inform the request for coverage review process. Please consider including the following information with the letter of medical necessity:

**Patient demographics**

* Patient name
* Date of birth
* Insurance policy/ID number
* Date of Niemann-Pick Type C (NPC) diagnosis

**Patient condition and medical history**

* Diagnostic information (see [Prior Authorization Checklist](https://aqneursa.com/wp-content/prior-authorization-checklist.pdf))
* 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

**Recommended treatment includes:** AQNEURSA

* 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 |

**Reasons for treatment selection**

* Prescribing Information
* Clinical studies published in peer-reviewed journals (Available from Medical Information at medinfo@intrabio.com or phone (833) 306-9677)

Retain a copy of the complete Letter of Medical Necessity for the patient’s records.

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**[This sample Letter of Medical Necessity template can be copied onto the prescriber’s letterhead, customized with patient-specific information,**

**edited as deemed appropriate by the prescriber, and signed]**

Attn: [Medical/Pharmacy Director] Re: [Patient Name]

[Insurance Company] [Date of Birth]

[Address] [Policy Number]

[City, State, ZIP code] [Authorization Request]

Therapy: AQNEURSA™ (levacetylleucine)

Expected start date of therapy: [Expected start date of therapy]

Dear Reviewer,

On behalf of [Patient Name], I am providing written documentation of the medical necessity of treatment with AQNEURSA (levacetylleucine) and am requesting approval for coverage. My patient, [Patient Name], was diagnosed with Niemann-Pick type C (NPC) (ICD-10-CM code E75.242) on [diagnosis date] via presentation of NPC clinical features such as [x, y, z] and [one of the following: positive biomarker screen and/or filipin test, or a positive genetic test for mutations in both copies of *NPC1* or *NPC2*]. My patient would benefit from treatment with AQNEURSA, which was FDA-approved for the treatment of adult and pediatric patients with neurological symptoms of Niemann-Pick type C (NPC) weighing ≥15 kg on September 24th, 2024. Please see the enclosed patient history documentation, which provides [Patient Name]’s medical history and supporting test results in more detail[, as well as clinically supportive information on the use of AQNEURSA for NPC].

The following items are enclosed:

* [Medical literature regarding the use of AQNEURSAfor the treatment of adult and pediatric patients with neurological symptoms of Niemann-Pick type C (NPC) weighing ≥15 kg
	+ Prescribing information for AQNEURSA1
	+ Bremova-Ertl T, et al. *N Engl J Med*. 2024;390:421-4312]
* Diagnostic information:
	+ ICD-10-CM code: E75.242 Niemann-Pick disease type C
	+ 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 – vertical supranuclear gaze palsy, 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
* [Patient condition and medical history
	+ Patient weight, growth, and development
	+ Neurological and/or psychological testing results
	+ Symptoms and quality of life (physician observed)
	+ 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]
* [Applicable coverage policies]

The enclosed information supports the medical necessity for treatment with AQNEURSA. I strongly believe this request should be covered for my patient based on the information provided. Due to the progressive nature of this disease, I would appreciate your prompt review of this request for coverage.

If you have any further questions regarding this matter or need additional information, please contact my office at [phone number].

Sincerely,

[Insert physician name and participating provider number]

[Include enclosures listed above, along with relevant patient demographic information]

**1.** AQNEURSA. Prescribing information. IntraBio. **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(5):421-431. doi: 10.1056/NEJMoa2310151. **3.** Geberhiwot T, Moro A, Dardis A, et al. Consensus clinical management guidelines for Niemann-Pick disease type C. *Orphanet J Rare Dis*. 2018 Apr 6;13(1):50. doi: 10.1186/s13023-018-0785-7.