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AQNEURSA® (levacetylleucine) Recommended for EU Approval by the CHMP to Treat Niemann-Pick Disease Type C

- **AQNEURSA® could offer a new frontline treatment for NPC patients in Europe**
- **CHMP recommendation based on Phase III pivotal trial data and extension**
- **phase data, showing rapid symptomatic improvement, long-term benefit, and disease-modifying, neuroprotective effects[1,2]**
- **European Commission decision will follow later this year**
- **AQNEURSA previously received U.S. FDA approval in September, 2024**

AUSTIN, Texas--(BUSINESS WIRE)--IntraBio Inc., a biopharmaceutical company focused on developing therapies for rare neurological diseases, today announced that the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has issued a positive opinion recommending approval of AQNEURSA® (levacetylleucine) for the treatment of Niemann-Pick disease type C (NPC).

"This positive CHMP opinion represents another important milestone in expanding access to AQNEURSA to the global NPC community," said Dr. Marc Patterson, Chief Medical Officer of IntraBio. "The recommendation reflects the strength of our clinical data and the potential for AQNEURSA to be a foundational therapy for NPC, delivering meaningful benefits for patients. We are proud to work alongside the NPC community to bring this long-awaited treatment option to even more families."

NPC is a rare, inherited lysosomal disorder characterized by progressive neurological deterioration, leading to loss of motor function, difficulties with speech and swallowing, and cognitive decline.[3] NPC affects both children and adults, significantly impacting quality of life and daily functioning.[3,4]

"For NPC families like mine, this positive opinion brings long-awaited hope for a treatment that can actually offer improvements," said Carmelo Fernández, President of Fundación Niemann-Pick de España. "We have waited years for a therapy that can make a meaningful difference in the lives of people with NPC, and today's announcement brings us one step closer."

The positive CHMP's opinion is based on results from IntraBio's pivotal Phase III randomized, placebo-controlled, clinical trial (IB1001-301; NCT05163288), which evaluated the impact of AQNEURSA on neurological symptoms and functioning in pediatric and adult patients (n=60) with a confirmed diagnosis of NPC. AQNEURSA significantly improved neurological symptoms and functional abilities across its primary and all secondary endpoints within 12 weeks of treatment versus placebo, and was well tolerated throughout the development program.[1] The CHMP opinion

was further supported by long-term extension phase data showing that treatment with AQNEURSA had disease-modifying and neuroprotective effects, helping to reverse disease progression over time.[2] Detailed results from the IB1001-301 trial were published in *The New England Journal of Medicine* in February 2024.[1]

About AQNEURSA

AQNEURSA was approved by the U.S. Food and Drug Administration (FDA) on 24 September 2024 for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing ≥15 kg.[5]

Since approval in the U.S., AQNEURSA has experienced rapid adoption as a frontline therapy for NPC with continued growth in demand.

IntraBio remains on track to proceed with additional global regulatory submissions for AQNEURSA in 2025 and beyond.

IntraBio's Phase III Pivotal trial investigating N-Acetyl-L-Leucine (levacetylleucine) for Ataxia- Telangiectasia has completed recruitment in under two months, ultimately over-enrolling the trial by over 167%. Data readout is expected in Q1 of 2026.

U.S. 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.

Please click here for Full Prescribing Information for AQNEURSA:
https://www.aqneursahcp.com/wp-content/prescribing-information.pdf

About Niemann-Pick Disease Type C

Niemann-Pick disease Type C (NPC) is a rare (1:100,000 live births), prematurely fatal, autosomal recessive, lysosomal storage disorder.[6] The disease presents with systemic, psychiatric, and neurological symptoms, including cerebellar ataxia. NPC is chronic and progressive in nature and is characterized by rapid degeneration of the cerebellum and major organ systems which severely impacts the quality of life.[3,4,7]

About IB1001-301

IB1001-301 (NCT05163288) is a multinational, randomized, placebo-controlled, crossover trial that evaluates the safety and efficacy of IB1001 (AQNEURSA, levacetylleucine) in pediatric and adult patients with NPC. Patients aged 4 years and older were screened at trial sites in Australia, Europe, the United Kingdom, and the United States.

Patients were assessed during a baseline period and then randomly assigned (1:1) to receive orally administered IB1001 or placebo for 12 weeks. At the end of the 12-week treatment period, patients crossed over and initiated therapy with the alternate study drug (IB1001 or placebo) over the subsequent 12-week period. Patients who completed the study had the option to participate in an open-label Extension Phase, with some patients having been dosed for over 5 years.

About IntraBio

IntraBio Inc. is a global biopharmaceutical company that develops and commercializes targeted therapies for rare and common neurological and neurodevelopmental diseases. IntraBio's platform technologies result from decades of research and collaboration with universities and institutions worldwide, and leverages the expertise of its scientific founders from the University of Oxford and the University of Munich.

For more information about IntraBio, please visit the company's website at intrabio.com and follow on LinkedIn (@IntraBio-Inc).

References

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