IntraBio Announces U.S. FDA Approval of AQNEURSA for the Treatment of Niemann-Pick Disease Type C

- AQNEURSA™ (ak-nur-sah) is the only FDA-approved stand-alone therapy for the treatment of Niemann-Pick disease type C (NPC)
- Approval follows positive Phase III data demonstrating significant improvements in neurological symptoms and functional benefits that could be seen within 12 weeks in adult and pediatric NPC patients
- AQNEURSA provides a long-awaited approved treatment option for patients and families living with NPC
- AQNEURSA is available now

AUSTIN, TX, September 25, 2024– IntraBio Inc., a leader in the discovery and development of innovative drugs for rare neurodegenerative diseases, today announced that the U.S. Food and Drug Administration (FDA) has approved AQNEURSA™ (levacetylleucine) for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing ≥15 kg. AQNEURSA is the only FDA-approved stand-alone therapy indicated for the treatment of NPC.

"IntraBio has been dedicated to bringing novel treatments to patients with extremely high unmet medical needs like NPC, and today we celebrate a major milestone in this tremendous effort," said Mallory Factor, President and Chief Executive Officer of IntraBio. "Patients and families in the NPC community have long awaited an effective, FDA-approved treatment, and we are proud to bring hope to those affected by this devastating disease. We remain committed to ensuring that all patients who can benefit from this novel treatment will have the opportunity to do so. Based on our clinical research, we believe that AQNEURSA may hold potential for treating other rare and common neurodegenerative and neurodevelopmental disorders, and we will continue to rapidly develop AQNEURSA for these additional indications."

NPC is a rare, inherited lysosomal disease that occurs in about 1 in 100,000 live births. Patients with NPC typically experience systemic, neurological and psychiatric symptoms that can be debilitating and significantly impact functional abilities. Until now, current treatment approaches have not addressed the debilitating effects of NPC on patients' daily lives.

"The FDA approval of AQNEURSA marks a significant breakthrough for those living with Niemann-Pick disease type C," commented Laurie Turner, Family Services Manager at the National Niemann-Pick Disease Foundation. "For too long, our community has been without an approved therapy for the treatment of NPC. Today we celebrate this tremendous milestone for individuals and families living with NPC. We are immensely thankful for the dedication to innovative research that has led to this approval, and we are ready to help families embark on this new chapter of treatment."

The FDA approval is based on data from the IB1001-301 multinational, randomized, double-blind, placebo-controlled, pivotal clinical trial (NCT05163288), which evaluated the impact of AQNEURSA on neurological symptoms and functioning in pediatric (aged 4 years and older) and adult patients (n=60) with a confirmed diagnosis of NPC.

The trial met the primary efficacy endpoint and all secondary endpoints across all cohorts receiving AQNEURSA. Results from the study showed AQNEURSA significantly improved neurological signs and symptoms and demonstrated functional benefits important to everyday life that were evident within 12 weeks. These findings were published in the February 1, 2024 issue of the *New England Journal of Medicine.*

The primary outcome assessed by the FDA was a modified version of the Scale for the Assessment and Rating of Ataxia (SARA), referred to as the functional SARA (fSARA). SARA is a clinical assessment tool that assesses gait, stability, speech, and upper and lower limb coordination across eight individual domains. fSARA consists only of gait, sitting, stance, and speech disturbance domains of the original SARA with modifications to the scoring responses. The results in patients who received AQNEURSA compared to placebo showed a greater improvement in fSARA score with a mean treatment difference of -0.4 (95% CI: -0.7, -0.2) with a two-sided p-value of <0.001. Results on the fSARA were supported by consistent results demonstrated on the original SARA.

AQNEURSA was well tolerated in the trial with the most common adverse reactions (incidence ≥5% and greater than placebo in Period I of the trial) being abdominal pain, dysphagia, upper respiratory tract infections, and vomiting.

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.

U.S. full Prescribing Information for AQNEURSA is available at https://intrabio.com/wp-content/aqneursa-prescribing-information.pdf.

AQNEURSA Cares™ Support for Patients

IntraBio offers support programs to eligible patients through their 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. Contact 866-200-0419 to speak to an AQNEURSA Cares team representative to seek assistance with any questions or concerns about access to AQNEURSA.

About IntraBio

IntraBio Inc., a US biopharmaceutical company, is focused on the development of novel drugs addressing rare and common neurological diseases. IntraBio's platform technologies result from decades of research and collaboration with universities and institutions worldwide. Its clinical programs are based upon the expertise in lysosomal function and intracellular signaling of its scientific founders from the University of Oxford and the University of Munich.

Forward Looking Statement

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, which are subject to risks, uncertainties, and other factors. These statements include, but are not limited to, IntraBio's clinical development programs, regulatory submissions and approvals, and potential market opportunities. These forward-looking statements are based on current expectations and assumptions and involve risks and uncertainties that could cause actual results to differ materially.

Factors that may cause such differences include, among others, uncertainties related to the clinical trial process, regulatory approval scope, limitations and timelines, manufacturing, market acceptance, and the impact of competitive products.

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

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