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IntraBio Receives European Commission Approval of AQNEURSA® for the Treatment of Niemann-Pick Type C Disease

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AUSTIN, Texas--(BUSINESS WIRE)--IntraBio Inc. today announced that the European Commission granted marketing authorization to AQNEURSA® (levacetylleucine) for the treatment of neurological manifestations of Niemann-Pick Type C (NPC) disease, following a positive opinion from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA).

AQNEURSA® is approved in the European Union for use in adults and children aged 6 years and older weighing at least 20 kg. The approved indication includes use in combination with miglustat, or as monotherapy in patients where miglustat is not tolerated.

“This approval represents a significant milestone for the NPC community in Europe,” said Mallory Factor, Chief Executive Officer of IntraBio. “We are grateful to the EMA and European Commission for their thorough review and for recognizing the clinical value of AQNEURSA®. This decision reflects years of breakthrough scientific work and collaboration with clinicians and patient organizations, and it marks an important step toward expanding access to this therapy for people living with NPC.”

The approval is based on results from a Phase III randomized, double-blind, placebo-controlled, crossover study in patients with NPC. In the study, treatment with AQNEURSA® demonstrated a statistically significant and clinically meaningful improvement in neurological signs, symptoms, and functioning after 12 weeks of

treatment, as measured by the Scale for the Assessment and Rating of Ataxia (SARA), compared with placebo.[1]

In the ongoing open-label extension phase of the trial, improvements observed during the initial 12-week study have been sustained,[2,3] consistent with a neuroprotective, disease modifying effect over time. Observational comparisons with a natural history control cohort show that treatment with AQNEURSA® was associated with a 118% reduction in annual disease progression after 1 year, as measured by the 5-domain NPC Clinical Severity Scale (NPC-CSS), and a similar reduction after two-years.[2,3]

Professor Kyriakos Martakis, Associate Professor in Pediatrics at Justus Liebig University Giessen in Germany, a Principal Investigator for the trial, commented, "Niemann-Pick disease Type C is a rare and relentlessly progressive neurological disorder associated with substantial burden for patients and their families. The availability of an approved treatment addressing the neurological manifestations of NPC represents an important development for clinicians, but most of all, for individuals affected by this disease across Europe."

AQNEURSA® is available as 1 g granules for oral suspension. The active substance, levacetylleucine, is a first-in-class modified amino acid delivered to all tissues, including the central nervous system, designed to correct metabolic dysfunction, improve cellular energy production, and target the underlying processes of neurological dysfunction.[1,4]

Detailed recommendations for the use of AQNEURSA® are described in the Summary of Product Characteristics (SmPC), which is available on the EMA website in all official European Union languages.

AQNEURSA® was designated as an orphan medicinal product during its development.

About AQNEURSA®

AQNEURSA® (levacetylleucine) is approved in the United States, indicated for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing ≥15 kg, and in the European Union for the treatment of neurological manifestations of Niemann-Pick disease Type C, in combination with miglustat or as monotherapy in patients where miglustat is not

tolerated, in adults and children aged 6 years and older weighing at least 20 kg.[5,6] The most commonly reported adverse reaction with AQNEURSA® was flatulence.

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.[7] 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.[8-10]

About IntraBio

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

Today, IntraBio also separately announced the results of a Phase III randomized, double-blind, placebo-controlled, crossover study in patients with Ataxia-Telangiectasia (A-T), a neurodegenerative disease without any approved treatment and impacting approximately 1 in 70,000 people. Based on these results, IntraBio plans to immediately advance regulatory submissions to the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA), and additional global regulatory authorities.

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

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 US Prescribing Information for AQNEURSA: https://www.aqneursahcp.com/wp-content/prescribing-information.pdf

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