Aqneursa™ (levacetylleucine) – New drug approval

- On September 25, 2024, IntraBio announced the FDA approval of Aqneursa (levacetylleucine), for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adults and pediatric patients weighing ≥15 kg.

- NPC is a rare, inherited lysosomal disease that causes systemic, neurological and psychiatric symptoms.

    — NPC occurs in about 1 in 100,000 live births.

- The distinct molecular target for Aqneursa in the treatment of NPC is unknown.

- The efficacy of Aqneursa was established in a randomized, double-blind, placebo-controlled study in 60 patients with NPC. All patients received Aqneursa and placebo in a cross-over manner. 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 fSARA consists of gait, sitting, stance, and speech disturbance domains.

    — The estimated mean fSARA total score was 5.1 when patients were treated with Aqneursa and 5.6 when patients were treated with placebo. The estimated treatment difference for the fSARA total score was -0.4 (95% CI: -0.7, -0.2; $p < 0.001$).

- A warnings and precaution for Aqneursa includes embryo-fetal toxicity.

- The most common adverse reactions (≥ 5% and greater than placebo) with Aqneursa use were abdominal pain, dysphagia, upper respiratory tract infections, and vomiting.

- The recommended dose of Aqneursa is based on the patient's actual body weight (kg) to be administered orally up to three times daily.

Patient's Body Weight	Morning Dose	Afternoon Dose	Evening Dose
15 kg to less than 25 kg	1 gram	No dose	1 gram
25 kg to less than 35 kg	1 gram	1 gram	1 gram
35 kg or more	2 grams	1 gram	1 gram

- IntraBio has already launched Aqneursa. Aqneursa will be available as 1 gram granules in a unit-dose packet.