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Eneboparatide met primary endpoint of normalising serum calcium in adults with hypoparathyroidism at 24 weeks in CALYPSO Phase III trial

Trial continues as planned to 52 weeks to further characterise the risk-benefit profile

High-level results from the CALYPSO Phase III trial showed that eneboparatide (AZP-3601), an investigational parathyroid hormone (PTH) receptor 1 agonist, met its primary endpoint with statistical significance in adults with chronic hypoparathyroidism (HypoPT) at 24 weeks, compared to placebo. The primary endpoint is a composite of normalisation of albumin-adjusted serum calcium levels and independence from active vitamin D and oral calcium therapy.

HypoPT is a rare endocrine disease caused by a deficiency of PTH and characterised by impaired regulation of calcium and phosphate levels in the blood. This dysregulation of the physiological action of PTH can lead to clinical manifestations, including negative impact on the kidney and bone.[1] HypoPT is one of the largest known rare diseases, affecting over 200,000 people in the United States and the European Union, approximately 80% of whom are women.[2-4]

Marc Dunoyer, Chief Executive Officer, Alexion, AstraZeneca Rare Disease, said: "People living with HypoPT, a rare endocrine disease, are often at increased risk of hypercalciuria, osteopenia and osteoporosis, and these results from the CALYPSO trial underscore eneboparatide's potential to be another option for these patients. We look forward to reviewing clinical results at 52 weeks to fully characterise the risk-benefit profile."

Eneboparatide was well tolerated. After the 24-week randomised treatment period, all patients receive eneboparatide in the ongoing long-term extension period until 52 weeks. Full efficacy and safety data will be analysed at 52 weeks. Alexion plans to share these data with global health authorities and present them at forthcoming medical meetings.

Notes

Hypoparathyroidism
Hypoparathyroidism (HypoPT) is a rare endocrine disease caused by a deficiency of parathyroid hormone (PTH) and characterised by decreased calcium and elevated phosphate levels in the blood, which can lead to a variety of neuromuscular, renal and skeletal manifestations.[1,5,6] The primary cause in approximately 75% of people with HypoPT is injury to or removal of the parathyroid glands during neck surgery.[5,6] There are over 200,000 people in the United States and the European Union living with HypoPT, approximately 80% of whom are women.[2-4] Despite available treatments, people living with HypoPT continue to experience a significant unmet need.[1,8]

CALYPSO
CALYPSO is a global Phase III, randomised, double-blind, placebo-controlled, multicentre trial designed to evaluate the efficacy and safety of eneboparatide in adults with chronic hypoparathyroidism. A total of 202 patients treated with standard of care (active vitamin D and oral calcium supplementation) were randomised in a 2:1 ratio to receive eneboparatide or placebo.[9]

The primary efficacy endpoint is a composite of the proportion of patients that achieve albumin-adjusted serum calcium within the normal range and independence from standard of care after 24 weeks of treatment. The key secondary efficacy endpoints include normalisation of 24-hour urinary calcium in patients with hypercalciuria at baseline and assessment of patient-reported outcomes that reflect physical symptoms and impact on quality of life.[9]

After the 24-week randomised main treatment period, all patients receive eneboparatide treatment in the ongoing long-term extension period.[9]

Eneboparatide (AZP-3601)
Eneboparatide (AZP-3601) is an investigational parathyroid hormone (PTH) receptor 1 agonist for the treatment of chronic hypoparathyroidism (HypoPT). It is designed to bind with high affinity to a specific conformation of the PTH receptor 1 to restore PTH function to manage the symptoms of HypoPT, while preserving kidney function and bone health.[6] Eneboparatide has been granted fast track designation and orphan drug designation by the US Food and Drug Administration and orphan designation by the European Medicines Agency for the treatment of HypoPT.

Alexion
Alexion, AstraZeneca Rare Disease, is focused on serving patients and families affected by rare diseases and devastating conditions through the discovery, development and delivery of life-changing medicines. A pioneering leader in rare disease for more than three decades, Alexion was the first to translate the complex biology of the complement system into transformative medicines, and today it continues to build a diversified pipeline across disease areas with significant unmet need, using an array of innovative modalities. As part of AstraZeneca, Alexion is continually expanding its global geographic footprint to serve more rare disease patients around the world. It is headquartered in Boston, US.

AstraZeneca
AstraZeneca (LSE/STO/Nasdaq: AZN) is a global, science-led biopharmaceutical company that focuses on the discovery, development, and commercialisation of prescription medicines in Oncology, Rare Diseases, and BioPharmaceuticals, including Cardiovascular, Renal & Metabolism, and Respiratory & Immunology. Based in Cambridge, UK, AstraZeneca's innovative medicines are sold in more than 125 countries and used by millions of patients worldwide. Please visit astrazeneca.com and follow the Company on social media @AstraZeneca.

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