Amolyt Pharma Announces European Commission Has Granted Orphan Drug Designation to AZP-3601 for the Treatment of Hypoparathyroidism

Top-line data from ongoing clinical trial in patients with hypoparathyroidism expected mid-2022

LYON, France, and Cambridge, MA, March 1, 2022 — Amolyt Pharma, a global company specialized in developing therapeutic peptides for rare endocrine and related diseases, today announced that the European Commission has granted Orphan Drug Designation (ODD) to AZP-3601 for the treatment of hypoparathyroidism. Amolyt previously received ODD from the U.S. Food and Drug Administration for AZP-3601 for the same condition in January 2021.

"We are very pleased to have been granted Orphan Drug Designation in both the U.S. and now the E.U., something we believe reflects the need for new therapies to treat hypoparathyroidism," said Thierry Abribat, Ph.D., founder and chief executive officer of Amolyt Pharma. "This highlights the potential significant benefits that AZP-3601 may bring to patients versus currently approved therapies, including sustained serum calcium normalization for a full 24 hours, and reduced risk of hypercalciuria, a key contributing factor to chronic kidney disease."

"Additionally, we believe AZP-3601's unique mechanism of action may preserve bone integrity as demonstrated by the bone biomarker data from our Phase 1 clinical trial in healthy volunteers. Together, these clinical benefits are relevant for many patients with hypoparathyroidism, especially the 26% who have established chronic kidney disease and those at risk of developing kidney disease plus the 17% with osteopenia or osteoporosis. We are advancing AZP-3601 through an efficient clinical development plan and we look forward to announcing proof-of-concept data in patients with hypoparathyroidism mid-year."

Based on the Committee for Orphan Medicinal Products (COMP) positive opinion, the European Commission grants ODD to potential treatments for diseases that are life-threatening or chronically debilitating and have a prevalence of not more than 5 in 10,000. When granted, ODD carries a range of incentives for sponsors, including protocol assistance, reduced regulatory fees, access to a centralized marketing authorization, and ten years of market exclusivity.

About Hypoparathyroidism

Hypoparathyroidism is defined by a deficiency of parathyroid hormone (PTH) that results in decreased calcium and elevated phosphorus levels in the blood. About 80% of the approximately 80,000 people in the U.S. and 110,000 in the European Union with hypoparathyroidism are women. Despite available treatments, patients experience persistent, life-altering symptoms and often develop complications and comorbidities that diminish quality of life and create segments with unique clinical needs. Clinical manifestations of hypoparathyroidism impact a large number of tissues and organ systems, specifically the kidneys and bone. 17% of patients with hypoparathyroidism have osteopenia or osteoporosis and 53% are peri- or postmenopausal women with an increased risk of developing osteoporosis. Roughly 26% of people with

hypoparathyroidism have chronic kidney disease or failure, highlighting the importance of reducing urinary calcium excretion as a key treatment goal.

About AZP-3601

AZP-3601 is an investigational therapeutic peptide designed to target a specific conformation of the parathyroid hormone (PTH) receptor to safely produce sustained and stable levels of calcium in the blood and thereby manage the symptoms of hypoparathyroidism, and to limit urine calcium excretion by restoring calcium reabsorption by the kidney, with the goal of consequently preventing chronic kidney disease. In addition to its unique receptor profile, AZP-3601 is also designed to have a short half-life to potentially preserve bone integrity, an important benefit, since the majority of patients are peri- and postmenopausal women with an increased risk of developing osteoporosis.

About Amolyt Pharma

Amolyt Pharma, a clinical stage biotechnology company, is building on its team's established expertise in therapeutic peptides to deliver life-changing treatments to patients suffering from rare endocrine and related diseases. Its portfolio includes AZP-3601, a long-acting PTH analog as a potential treatment of hypoparathyroidism, AZP-3813, a peptide growth hormone receptor antagonist for the potential treatment of acromegaly, and AZP-3404, which is undergoing indication selection work. Amolyt Pharma aims to further expand and develop its portfolio by leveraging its global network in the field of endocrinology and with support from a strong syndicate of international investors. To learn more, visit https://amolytpharma.com/ or follow us on Twitter at @AmolytPharma.

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