Amolyt Pharma Granted FDA Fast Track Designation for Eneboparatide for the Treatment of Hypoparathyroidism

Designation reflects the seriousness of the disease and the potential for eneboparatide to address the urgent need for new treatment options that address key treatment goals in hypoparathyroidism

Lyon, France, and Cambridge, MA, May 2, 2024 — Amolyt Pharma, a global company specialized in developing therapeutic peptides for rare endocrine and related diseases, today announced that eneboparatide, its lead therapeutic peptide candidate in Phase 3 development for the treatment of hypoparathyroidism, has been granted fast track designation by the U.S. Food and Drug Administration (FDA). The FDA's Fast Track process is designed to facilitate the development and expedite the review of new drugs to treat serious conditions with unmet medical needs, with the goal of introducing new treatment options to patients faster.

"We believe FDA's granting of fast track designation to eneboparatide reflects the agency's recognition of the significant unmet needs that persist among patients suffering from hypoparathyroidism," stated Thierry Abribat, Ph.D., founder and chief executive officer of Amolyt Pharma. "We look forward to maintaining a constructive dialog with the agency as we work to bring new hope to patients suffering from this rare but challenging endocrine disorder as efficiently as possible."

"The current standard of care treatment, oral calcium and vitamin D supplementation seldom controls the life altering symptoms and complications of hypoparathyroidism, with many patients at risk of declining kidney function and diminished bone quality," stated Mark Sumeray, M.D., chief medical officer. "In studies to date, eneboparatide has been shown to normalize mean serum calcium and mean urinary calcium excretion while restoring balanced bone turnover. Building upon findings from our successful Phase 2 clinical trial, we are working diligently to execute our ongoing Calypso Phase 3 study and look forward to topline data in 2025."

About Eneboparatide Phase 3 Calypso Trial

Calypso is a Phase 3 multicenter, randomized, placebo-controlled, double-blind study designed to evaluate the efficacy and safety of eneboparatide in patients with chronic hypoparathyroidism. Approximately 165 patients treated with standard of care will be randomized in a 2:1 ratio to receive eneboparatide or placebo. The primary efficacy endpoint is 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 normalization of 24-hour urinary calcium in patients with hypercalciuria at baseline and assessment of patient-reported outcomes that reflect symptoms associated with physical and cognitive function and impact on quality of life. Additional exploratory endpoints assess bone quantity and quality using DXA scanning and high resolution peripheral quantitative CT scanning. After the initial 24-week placebo-controlled period, all patients will be treated with eneboparatide in an open-label extension phase for an additional 28 weeks.

The Calypso trial is being conducted in more than 50 centers in the United States, Europe, Canada, and the United Kingdom.

About Fast Track Designation
A drug that receives FDA Fast Track designation is eligible for some or all of the following[1]:

- More frequent meetings with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval;
- More frequent written communication from FDA about such things as the design of the proposed clinical trials and use of biomarkers;
- Eligibility for Accelerated Approval and Priority Review, if relevant criteria are met;
- Rolling Review, which means that a drug company can submit completed sections of its Biologic License Application (BLA) or New Drug Application (NDA) for review by FDA, rather than waiting until every section of the NDA is completed before the entire application can be reviewed. BLA or NDA review usually does not begin until the drug company has submitted the entire application to the FDA.

About Hypoparathyroidism
Hypoparathyroidism is a rare condition defined by a deficiency of parathyroid hormone (PTH) that results in decreased calcium and elevated phosphorus levels in the blood. Approximately 80% of the estimated 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 of the patient population with specific clinical needs. Clinical manifestations of hypoparathyroidism impact many tissues and organ systems, in particular, the kidneys and bone.

More than half of all patients are post-menopausal women who are at an increased risk for developing osteoporosis. In a prospective study of 101 adult cHP patients from the Canadian National Hypoparathyroidism Registry, 56.3% of menopausal women were diagnosed with osteopenia or osteoporosis, and 20.8% had fragility fractures. Approximately 26% of patients with hypoparathyroidism have chronic kidney disease or failure, highlighting the importance of reducing urinary calcium excretion as a key treatment goal.

About Eneboparatide
Eneboparatide is an investigational therapeutic peptide designed to bind with high affinity to a specific conformation of the parathyroid hormone (PTH) receptor to 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 progressive decline in kidney function and the development of chronic kidney disease. In addition to its unique receptor profile, eneboparatide is also designed to have a short half-life to potentially preserve bone integrity, an important potential benefit, since the majority of patients are peri- and postmenopausal women with an increased risk of developing osteoporosis.

[1] https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track

About Amolyt Pharma
Amolyt Pharma, a clinical stage biotechnology company, is building on its team's established expertise to deliver life-changing treatments to patients suffering from rare endocrine and related diseases. Its development portfolio includes eneboparatide (AZP-3601), a long-acting PTH1 receptor agonist as a potential treatment for hypoparathyroidism, and AZP-3813, a peptide growth hormone receptor antagonist for the potential treatment of acromegaly. 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 and LinkedIn.

Media Inquiries:
Kristin Politi
LifeSci Communications
kpoliti@lifescicomms.com

Investors Inquiries:
Ashley Robinson
LifeSci Advisors, LLC
arr@lifesciadvisors.com
+1.617.430.7577