



MBX Biosciences Receives Orphan Drug Designation for Lead Candidate MBX 2109 for the Treatment of Hypoparathyroidism

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CARMEL, Ind., July 26, 2022 — MBX Biosciences, Inc., a clinical stage biopharmaceutical company developing precision endocrine peptide therapeutics to treat endocrine disorders, today announced that the U.S. Food and Drug Administration (FDA) granted Orphan Drug designation to its lead therapeutic product candidate, MBX 2109, for the treatment of hypoparathyroidism.

"This is an exciting achievement for MBX and underscores the significant need to expand treatment options for hypoparathyroidism," said Kent Hawryluk, President and Chief Executive Officer of MBX Biosciences. "We believe MBX 2109, currently in a Phase 1 clinical trial, has the potential to overcome limitations of the current standard of care with fewer administrations necessary due to its long-acting profile. Our goal is to improve the outcomes and quality of life for patients with this chronic condition."

Orphan drug designation is designed to support the development of medicines addressing rare medical diseases or conditions that affect fewer than 200,000 people in the United States. Orphan drug designation qualifies the sponsor of the drug for certain development incentives, including tax credits for qualified clinical testing, prescription drug user fee exemptions and seven-year marketing exclusivity upon FDA approval.

MBX 2109 is an investigational long-acting parathyroid hormone (PTH) prodrug. It is currently undergoing evaluation in a Phase 1 randomized, double blind, placebo-controlled trial to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of single and multiple ascending doses of MBX 2109 in healthy adult subjects. For additional information about the trial, please visit clinicaltrials.gov using the identifier NCT05158335.

About Hypoparathyroidism

Hypoparathyroidism (HP) is a rare endocrine disorder caused by a deficiency of parathyroid hormone (PTH) that results in decreased calcium and increased phosphorus levels in the blood. There are approximately 200,000 individuals worldwide with HP. The clinical manifestations are broad affecting a large number of organ systems, especially the kidney and bone which are major target organs for PTH. The persistence of symptoms and development of complications often diminish a person's quality of life. In contrast to most hormone deficiencies for which hormone replacement therapy is the mainstay of therapy, HP has conventionally been treated with calcium supplements and active vitamin D. This approach can maintain the serum calcium level but may require high doses thereby contributing to the risk of long-term kidney complications.

About MBX 2109

MBX 2109 is an investigational long-acting parathyroid hormone (PTH) prodrug in development as a PTH replacement therapy. It was designed with the company's novel, proprietary prodrug chemistry platform to provide sustained PTH activity with a convenient once-weekly dosing regimen to simplify an individual's disease management, while relieving both the symptoms of the disorder and long-term complications.

About MBX Biosciences

MBX Biosciences, Inc. is a clinical stage biopharmaceutical company pioneering investigational Precision Endocrine Peptides (PEPs™). PEPs are designed to overcome key limitations of native peptide therapeutics to deliver superior pharmacologic properties. MBX is advancing a pipeline of PEP candidates to address the unmet needs of people with endocrine disorders. The company's lead product candidate, MBX 2109 for the treatment of hypoparathyroidism, is currently in a [Phase 1 clinical trial](#). Members of MBX's leadership team have collaborated over several decades on the discovery, development, and commercialization of highly successful endocrine therapeutics including Forteo® and Humalog®. The company is supported by leading life science investors including Frazier Life Sciences, New Enterprise Associates and OrbiMed. For more information, please visit the MBX [website](#) and follow us on [LinkedIn](#) and [Twitter](#).

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