Stealth BioTherapeutics Announces PDUFA Action Date Extension for Elamipretide to Treat Patients with Barth Syndrome

– New PDUFA Action Date of April 29, 2025, Allows FDA Additional Time to Complete Review –

– Follows Positive FDA Advisory Committee Meeting Vote Concluding that Elamipretide is Effective for the Treatment of Barth Syndrome–

NEEDHAM, Mass., Jan. 23, 2025 /PRNewswire/ — Stealth BioTherapeutics Inc. (the "Company" or "Stealth"), a clinical-stage biotechnology company focused on the discovery, development and commercialization of novel therapies for diseases involving mitochondrial dysfunction, today announced that the U.S. Food and Drug Administration (FDA) has extended the Prescription Drug User Fee Act (PDUFA) action date for the New Drug Application (NDA) for elamipretide, a first-in-class mitochondria-targeted therapeutic in development for individuals with Barth syndrome. The new PDUFA target action date is April 29, 2025.

The FDA notified Stealth that the Agency requires additional time to conduct a full review of supplemental information provided in response to recent FDA requests that were received following the positive Cardiovascular and Renal Drugs Advisory Committee (CRDAC) meeting on October 10, 2024. The FDA advised that these submissions constitute Major Amendments to the NDA, resulting in a standard three-month extension from the original action date of January 29, 2025. Importantly, the FDA has not identified any safety issues and has not requested any new pre-marketing studies. In addition, the FDA has reconfirmed previously communicated post-marketing commitments and Stealth has addressed all information requests from the Agency to date.

"We appreciate the FDA's commitment to a thorough review of our NDA and the positive supplemental analyses submitted in response to its recent information requests, and remain confident in the robustness of this NDA package," said Reenie McCarthy, Chief Executive Officer. "We continue to work closely with the Agency as it completes its review of the elamipretide NDA and are actively preparing to support broad access to this therapy for individuals living with Barth syndrome as quickly as possible following potential approval."

If approved, this would be the first marketing authorization for elamipretide, a first-in-class mitochondria-targeted therapeutic, and the first FDA-approved therapy for Barth syndrome.

About Barth Syndrome

Barth syndrome is an ultra-rare genetic condition characterized by cardiac abnormalities leading to exercise intolerance, muscle weakness, debilitating fatigue, heart failure, recurrent infections, and delayed growth. The disease is associated with reduced life expectancy, with 85% of early deaths occurring by age 5. Barth syndrome occurs primarily in males and is estimated to affect one in 1,000,000 males worldwide or around 150 individuals in the United States. There are currently no FDA- or EMA-approved therapies for patients with Barth syndrome. Elamipretide has Orphan Drug, Fast Track, Priority

Review, and Rare Pediatric Designation from the FDA and Orphan Drug Designation from the EMA for the treatment of Barth syndrome.

About Stealth BioTherapeutics

Our mission is to develop novel therapies to improve the lives of patients living with diseases of mitochondrial dysfunction. Our lead product candidate, elamipretide, is under review for Barth syndrome and in late-stage development for primary mitochondrial myopathy and dry age-related macular degeneration. We are also evaluating a topical ophthalmic formulation of our second-generation clinical-stage candidate, bevemipretide (SBT-272), for dry age-related macular degeneration, and have a deep pipeline of novel compounds under evaluation for rare neurological and cardiac disease indications.

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