Alexion and Stealth Announce Agreement for Option to Co-Develop and Commercialize Late-Stage Therapy for Mitochondrial Diseases

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- Elamipretide in Phase 3 development for primary mitochondrial myopathy -

- If option is exercised, Alexion & Stealth will co-develop and co-promote subcutaneous elamipretide in U.S. & Alexion will receive exclusive ex-U.S. license -

- Stealth to receive initial payment of $30 million for equity investment, option fee and development funding, with potential for additional option-related & milestone-dependent payments if option is exercised -

BOSTON--(BUSINESS WIRE)--Oct. 10, 2019-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) and Stealth BioTherapeutics Corp (NASDAQ:MITO) today announced an agreement for an option to co-develop and commercialize elamipretide for mitochondrial diseases. Currently being evaluated in a Phase 3 study in people with primary mitochondrial myopathy (PMM) - a genetic mitochondrial disease - elamipretide is a novel, potential first-in-class therapy that targets mitochondrial dysfunction. There are currently no therapies approved to treat PMM, which is characterized by debilitating skeletal muscle weakness, chronic fatigue and exercise intolerance. Under the terms of the agreement, Alexion will have the opportunity to exercise the option following the delivery of results from the Phase 3 study currently underway in PMM.

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“Mitochondria play a critical role in normal organ function and, when dysfunctional, can have devastating consequences on multiple organ systems, leading to many serious diseases, such as primary mitochondrial myopathy,” said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. “Given our strong existing relationships with neuromuscular specialists – who play a critical role in treating PMM – we believe this is an exciting potential opportunity to further expand our rare neurology portfolio and look forward to the possibility of working with Stealth to realize the promise of elamipretide for patients.”

“Our mission is to deliver therapies to patients suffering from devastating mitochondrial diseases, and this partnership will enhance our ability to rapidly deliver on that mission,” said Stealth Chief Executive Officer Reenie McCarthy. “Together with Alexion, which is widely recognized for its demonstrated ability to bring important new therapies for rare diseases to children and adults in need, we believe we can achieve synergies of execution that will both expedite and increase patient access following achievement of key upcoming clinical and regulatory milestones for our PMM and Barth syndrome programs.”

Under the terms of the agreement, Alexion will receive an exclusive option to partner with Stealth in the development of subcutaneous elamipretide based on final results from the Phase 3 study currently underway in PMM. If Alexion chooses to exercise the option, the companies will co-develop subcutaneous elamipretide in the U.S. for PMM and Barth syndrome, as well as Leber’s hereditary optic neuropathy (LHON), which is currently in earlier stage clinical development. Upon commercialization, the agreement would provide for a 50-50 co-promote between the two companies in the U.S. and Alexion would receive exclusive rights to develop and commercialize subcutaneous elamipretide outside the U.S. Alexion will make initial payments to Stealth totaling $30 million, including an option fee, an equity investment and development funding. If the option is exercised, the agreement provides for additional payments, including an option exercise fee, an additional equity investment, development funding and potential regulatory and commercial milestone payments. Stealth’s other pipeline assets, including SBT-272, are not included in the option.

Stealth will host a conference call and webcast at 8:30 a.m. ET today to review this partnership and the potential opportunity for elamipretide. To access the call please dial 866-939-3921 (domestic) and 678-302-3550 (international) and provide the passcode 49106405. A live audio webcast of the call will be available on the Investors & News section of Stealth’s website at https://investor.stealthbt.com/. The archived webcast will be available approximately two hours after the conference call and will be available for 30 days following the call.

About Primary Mitochondrial Myopathy

Primary mitochondrial myopathy (PMM) affects patients afflicted with a heterogenous group of genetic disorders whose disease predominantly impairs skeletal muscle function, with many patients suffering from lifelong functional impairment. PMM is among the most common forms of mitochondrial disease; patients must have a genetically confirmed diagnosis of primary mitochondrial disease plus a predominantly myopathic clinical presentation to have PMM. Signs, symptoms and disease severity vary significantly among patients, but the most common symptoms include progressively debilitating skeletal muscle weakness, chronic fatigue and exercise intolerance. As a result, the diagnostic process can be challenging, requiring a multi-disciplinary approach, most frequently involving neuromuscular specialists and geneticists. There are currently no approved treatments for PMM, and standard-of-care is supportive, typically focusing on the specific symptoms experienced by each individual patient.

About Elamipretide

Elamipretide, a mitochondria-targeted therapeutic, is in clinical development for a variety of diseases caused by mitochondrial dysfunction. Elamipretide targets the inner mitochondrial membrane where it associates with cardiolipin – the signature phospholipid of the inner mitochondrial membrane, which plays a role in many mitochondrial processes, including respiration and energy conversion. This elamipretide-cardiolipin association has been shown to normalize the structure of the inner mitochondrial membrane, thereby improving mitochondrial function. In preclinical and clinical studies, elamipretide was shown to increase mitochondrial respiration, improve the electron transport chain function and ATP production, and reduce formation of pathogenic reactive oxygen species levels. Functional benefit is believed to be achieved through improvement of ATP production and interruption and potential reversal of damaging oxidative stress.

Elamipretide is being investigated in late-stage clinical studies in primary mitochondrial myopathy (PMM) and Barth syndrome as well as in earlier

stage clinical studies in Leber's hereditary optic neuropathy (LHON) and geographic atrophy associated with dry age-related macular degeneration (GA). Elamipretide has received Fast Track and Orphan Drug designations for PMM, Barth syndrome and LHON, as well as Fast Track designation for GA.

About Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the discovery, development and commercialization of life-changing therapies. As the global leader in complement biology and inhibition for more than 20 years, Alexion has developed and commercializes two approved complement inhibitors to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) as well as the first and only approved complement inhibitor to treat atypical hemolytic uremic syndrome (aHUS), anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG) and neuromyelitis optica spectrum disorder (NMOSD). Alexion also has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). In addition, the company is developing several mid-to-late-stage therapies, including a second complement inhibitor, a copper-binding agent for Wilson disease and an anti-neonatal Fc receptor (FcRn) antibody for rare Immunoglobulin G (IgG)-mediated diseases as well as several early-stage therapies, including one for light chain (AL) amyloidosis and a second anti-FcRn therapy. Alexion focuses its research efforts on novel molecules and targets in the complement cascade and its development efforts on the core therapeutic areas of hematology, nephrology, neurology, and metabolic disorders. Alexion has been named to the *Forbes'* list of the World's Most Innovative Companies seven years in a row and is headquartered in Boston, Massachusetts' Innovation District. The company also has offices around the globe and serves patients in more than 50 countries. This press release and further information about Alexion can be found at: www.Alexion.com.

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About Stealth

Stealth is a clinical-stage biotechnology company focused on improving the lives of patients suffering from diseases involving mitochondrial dysfunction through the discovery, development and commercialization of novel mitochondrial medicines. Mitochondria, found in nearly every cell in the body, are the body's main source of energy production and are critical for normal organ function. Dysfunctional mitochondria characterize a number of rare genetic diseases, collectively known as primary mitochondrial diseases, and are also involved in many common age-related diseases. Stealth believes its lead product candidate, elamipretide, has the potential to treat both rare genetic and common age-related mitochondrial diseases. Stealth is studying elamipretide in the following primary mitochondrial diseases: primary mitochondrial myopathy, Barth syndrome and Leber's hereditary optic neuropathy. Stealth is also studying elamipretide in geographic atrophy associated with dry age-related macular degeneration. Stealth's other pipeline candidates include SBT-272, being evaluated for rare neurodegenerative disease indications, and SBT-20 and SBT-259, being evaluated for rare peripheral neuropathies. Stealth has optimized its discovery platform to identify novel mitochondria-targeted compounds, which may be nominated as therapeutic product candidates or utilized as scaffolds to deliver other compounds to mitochondria. Stealth has assembled a highly experienced management team, board of directors and group of scientific advisors to achieve its mission of leading mitochondrial medicine.

Forward-Looking Statements

This press release includes forward-looking statements, including statements related to the therapeutic benefits of elamipretide; the potential of elamipretide to target mitochondrial dysfunction (and to serve as a potential first-in-class therapy that targets mitochondrial dysfunction); the potential of elamipretide as a treatment for PMM and other indications; the agreement is a potential opportunity to further expand Alexion's rare neurology portfolio and offers the possibility of working with Stealth to realize the promise of elamipretide for patients; Stealth has optimized its discovery platform to identify novel mitochondria-targeted compounds, which may be nominated as therapeutic product candidates or utilized as scaffolds to deliver other compounds to mitochondria; Alexion's ability to use its relationship with neuromuscular specialists to bring elamipretide to patients suffering with PMM and other indications; the potential benefits of the collaboration, including the realization of synergies between Alexion and Stealth to accelerate the clinical development and commercial launch and patient access to elamipretide for children and adults; and the achievement of key upcoming clinical and regulatory milestones for Stealth's PMM and Barth syndrome programs. Such forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statements, including the process by which an early stage product such as elamipretide could potentially lead to an approved product for the treatment of PMM is long and subject to highly significant risks, including for example, decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products, delays, interruptions or failures in manufacture and supply, failure to satisfactorily address matters raised by the U.S. Food and Drug Administration and other regulatory agencies, the possibility that results of clinical trials are not predictive of safety and efficacy results in broader patient populations, the possibility that clinical trials could be delayed, the risk that anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with PMM are inaccurate; the collaboration may not achieve the anticipated benefits for Alexion, Stealth and for patients; the anticipated benefits of elamipretide may not be realized even if approved for sale; patients, physicians and payers may not accept elamipretide as a treatment for the indications being pursued; the discovery platform under development may not achieve the anticipated benefits due to technological challenges or otherwise; the anticipated contributions of the parties to the agreement (including the expected synergies) may not come to fruition due to technical and commercial limitations and other factors; and a variety of other risks set forth from time to time in Alexion's filings with the SEC, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended June 30, 2019 and in Alexion's other filings with the SEC, and in Stealth's filings with the SEC, including but not limited to the risks discussed in Stealth's Annual Report on Form 20-F for the period ended December 31, 2018. Alexion and Stealth disclaim any obligation to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

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