



Alexion and BridgeBio Announce Japanese License Agreement for Eidos' Transthyretin Amyloidosis (ATTR) Investigational Medicine

September 9, 2019

- Eidos grants Alexion exclusive license to develop and commercialize AG10 in Japan -

- Phase 3 study of AG10 in ATTR cardiomyopathy underway in U.S. & Europe; Phase 3 trial in ATTR polyneuropathy planned to initiate in second half of 2019 -

- Agreement expands Alexion's amyloidosis portfolio -

- Eidos to receive upfront payment of \$25 million and equity investment of \$25 million, with potential for additional Japanese-based milestone- & royalty-dependent payments -

BOSTON & SAN FRANCISCO--(BUSINESS WIRE)--Sep. 9, 2019-- [Alexion Pharmaceuticals, Inc.](#) (NASDAQ:ALXN) and [BridgeBio Pharma, Inc.'s](#) (NASDAQ:BBIO) subsidiary [Eidos Therapeutics, Inc.](#) (NASDAQ:EIDX) today announced an agreement that grants Alexion an exclusive license to develop and commercialize AG10 in Japan. AG10 is a small molecule designed to treat the root cause of transthyretin amyloidosis (ATTR) – destabilized and misfolded transthyretin (TTR) protein – by binding and stabilizing TTR in the blood. Eidos is currently evaluating AG10 in a Phase 3 study in the U.S. and Europe for ATTR cardiomyopathy (ATTR-CM) – a progressive, fatal disease caused by the accumulation of misfolded TTR amyloid in the heart – and plans to begin a Phase 3 study in ATTR polyneuropathy (ATTR-PN) – a progressive, fatal disease caused by the accumulation of misfolded TTR amyloid in the peripheral nervous system.

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"There is a significant need for new treatments for TTR amyloidosis. We believe AG10 holds promise in its ability to stabilize TTR and halt disease progression," said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. "We are excited by the potential to grow our amyloidosis portfolio by partnering with Eidos to expand the development of AG10 to Japan. Alexion has more than 10 years of experience operating there, and we look forward to applying our expertise to bring AG10 to Japanese patients."

"The Phase 2 study in ATTR-CM suggested that AG10 has the potential to become an important treatment option for the underserved ATTR-CM population. The trial showed that AG10 was generally well-tolerated and resulted in near-complete stabilization of TTR, which is known to be correlated with disease severity in ATTR-CM. In the study, AG10 also normalized serum TTR levels, a prognostic indicator of survival in ATTR patients," said Jonathan Fox, M.D., Ph.D., President and Chief Medical Officer of Eidos. "We have now begun our Phase 3 program to evaluate the safety and efficacy of AG10 in larger studies. This agreement provides the potential opportunity to help even more patients globally by leveraging Alexion's significant development and commercial experience to expand the AG10 program into Japan."

Under the terms of the agreement, Alexion will acquire an exclusive license for the clinical development and commercialization of AG10 in Japan. Eidos will receive an upfront payment of \$25 million and an equity investment of \$25 million at a premium to the market price upon deal execution, with the potential for additional Japanese-based milestone- and royalty-dependent payments.

About AG10

AG10 is an investigational, orally-administered small molecule designed to potently stabilize tetrameric transthyretin, or TTR, thereby halting at its outset the series of molecular events that give rise to TTR amyloidosis, or ATTR. In a Phase 2 clinical trial in patients with symptomatic ATTR-CM, AG10 was generally well tolerated, demonstrated greater than 90 percent average TTR stabilization at Day 28, and increased serum TTR concentrations, a prognostic indicator of survival in a retrospective study of ATTR-CM patients, in a dose-dependent manner.

AG10 was designed to mimic a naturally-occurring variant of the TTR gene (T119M) that is considered a rescue mutation because co-inheritance has been shown to prevent or ameliorate ATTR in individuals also inheriting a pathogenic, or disease-causing, mutation in the TTR gene. To our knowledge, AG10 is the only TTR stabilizer in development that has been observed to mimic the stabilizing structure of this rescue mutation.

The Phase 3 ATTRibute-CM study of AG10 in patients with ATTR-CM is underway in the United States and Europe. Part A of the study will assess the change from baseline in 6-minute walk distance (6MWD) at 12 months. Part B of the study will evaluate reduction in all-cause mortality and frequency of cardiovascular-related hospitalizations will be evaluated at 30 months. In addition, Eidos plans to initiate a Phase 3 study of AG10 in ATTR polyneuropathy (ATTR-PN) in the second half of 2019.

About Transthyretin Amyloidosis (ATTR)

There is significant medical need in transthyretin amyloidosis (ATTR) given the large patient population and an inadequate current standard of care. ATTR is caused by the destabilization of TTR due to inherited mutations or aging and is commonly divided into three distinct categories: wild-type ATTR cardiomyopathy (ATTRwt-CM), mutant ATTR cardiomyopathy (ATTRm-CM), and ATTR polyneuropathy (ATTR-PN). The worldwide prevalence of each disease is approximately 400,000 patients, 40,000 patients and 10,000 patients, respectively.

All three forms of ATTR are progressive and fatal. For patients with untreated ATTRwt-CM and ATTRm-CM, symptoms usually manifest later in life (age 50+), with median survival of three to five years from diagnosis. ATTR-PN either presents in a patient's early 30s or later (age 50+), and results in a median life expectancy of five to ten years from diagnosis for untreated patients. Progression of all forms of ATTR causes significant morbidity, impacts productivity and quality of life, and creates a significant economic burden due to the costs associated with progressively greater patient needs for supportive care.

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 BridgeBio and Eidos

BridgeBio is a team of experienced drug discoverers, developers and innovators working to create life-altering medicines that target well-characterized genetic diseases at their source. BridgeBio was founded in 2015 to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. BridgeBio's pipeline of over 15 development programs includes product candidates ranging from early discovery to late-stage development. For more information, please visit www.bridgebio.com.

Eidos is a BridgeBio Pharma subsidiary focused on addressing the large and growing unmet need in diseases caused by transthyretin (TTR) amyloidosis (ATTR). Eidos is developing AG10, a potentially disease-modifying therapy for the treatment of ATTR. For more information, please visit www.eidostx.com.

Forward-Looking Statements

This press release includes forward-looking statements. 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. Examples of forward-looking statements include, among others, statements we make regarding: (i) the therapeutic benefits and commercial potential of AG10 in Japan and elsewhere; (ii) development plans and clinical trial plans related to AG10; (iii) the potential of AG10 for the treatment of ATTR cardiomyopathy, ATTR polyneuropathy and other conditions; (iv) that the rights of Alexion under the license agreement will result in growth of Alexion's amyloidosis portfolio by partnering with Eidos to expand the development of AG10 to Japan; and (v) the likelihood that AG10 will be approved for commercial sale in Japan. The process by which products such as AG10 could potentially be developed and approved for commercial sale is long and subject to highly significant risks. Applicable risks and uncertainties include: results in early stage clinical trials may not be indicative of full results or results from later stage or larger clinical trials (or in broader patient populations) and do not ensure regulatory approval; the possibility that results of clinical trials are not predictive of safety and efficacy and potency of products (or the failure to adequately operate or manage our clinical trials) which could cause the halt of trials, delays or prevention from making regulatory approval filings or result in denial of regulatory approval of product candidates; unexpected delays in clinical trials; unexpected concerns that may arise from additional data or analysis obtained during clinical trials; delays or failure of product candidates to obtain regulatory approval due to clinical trial results, issues with clinical trial products, unexpected expense or otherwise; as well as those additional risks relating to product development and approval and other risks identified under the heading "Risk Factors" included in Alexion's, BridgeBio's and Eidos' most recent Form 10-Q filings and in their respective other future filings with the SEC. The forward-looking statements contained in this press release reflect Alexion's, BridgeBio's and Eidos' current views with respect to future events. Alexion, BridgeBio and Eidos do not undertake and each specifically disclaims any obligation to update any forward-looking statements, except as required by law.

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