



## **BridgeBio and Eidos Present Data from Phase 2 Open Label Extension Suggesting Long-term Tolerability of AG10 and Stabilization of Transthyretin Amyloid Cardiomyopathy Disease Measures**

November 16, 2019

*AG10 was Well Tolerated with Median 65 Weeks Follow-up since Phase 2 Initiation*

*Rates of All-Cause Mortality (Including Either Death or Cardiac Transplantation, 8.5%) and Cardiovascular Hospitalization (25.5%) Observed in Exploratory Analysis Were Lower than Rates Observed in Placebo-treated Participants in the ATTR-ACT Study*

*Near-complete Stabilization of TTR Maintained in Participants Throughout Duration of Study*

*Serum TTR levels, a Prognostic Indicator of Survival in ATTR-CM Patients, Were Elevated and Maintained in the Normal Range Throughout the Study Duration*

*Cardiac Biomarkers and Echocardiographic Parameters Were Stable Throughout Trial Duration*

*Presentation from AHA 2019 Scientific Sessions Available on Company Websites (eidostx.com and bridgebio.com)*

SAN FRANCISCO, Nov. 16, 2019 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (BridgeBio) (Nasdaq:BBIO) and Eidos Therapeutics, Inc. (Eidos) (Nasdaq:EIDX), today presented positive data from the companies' ongoing Open Label Extension (OLE) of the Phase 2 clinical trial studying AG10 in patients with symptomatic transthyretin (TTR) amyloidosis cardiomyopathy (ATTR-CM). ATTR-CM is a progressive and fatal disease that is an under recognized cause of heart failure. The data were presented in a late-breaking featured science oral presentation at the American Heart Association (AHA) Scientific Sessions in Philadelphia, Pennsylvania.

Study participants received 800mg of AG10 twice daily during the OLE and were followed for a median of 65 weeks since Phase 2 initiation. AG10 treatment was generally well-tolerated and resulted in near-complete TTR stabilization as measured using established ex vivo assays. Lower rates of mortality (death or cardiac transplantation) and cardiovascular-related hospitalizations were observed in AG10 Phase 2 OLE participants than were reported in a similar population of ATTR-CM patients who received placebo for 15 months in the ATTR-ACT study. Cardiac biomarkers and echocardiographic parameters were stable in patients treated with AG10 in the Phase 2 OLE.

"This update from our Phase 2 OLE demonstrated continued tolerability of AG10 in patients with advanced ATTR-CM. We also observed meaningfully lower rates of mortality and cardiovascular hospitalizations than what would be expected for untreated ATTR-CM patients with similar disease severity," said Jonathan Fox, M.D., Ph.D., FACC, president and chief medical officer of Eidos. "These encouraging data continue to support the development of AG10 as a potentially best-in-class treatment for ATTR-CM patients."

### **AG10 Phase 2 Open Label Extension Results**

The ongoing OLE study enrolled 47 of 49 participants (96%) from the 28-day randomized, placebo-controlled, Phase 2 study of AG10 in ATTR-CM patients with New York Heart Association (NYHA) Class II or III symptoms. Interim analysis of the ongoing study was completed on August 31, 2019 in conjunction with annual regulatory reporting and review, at which time 41 participants remained in the study. Three (6.4%) participants in the OLE had died, two due to disease progression and one due to cervical cancer. Three (6.4%) additional patients enrolled in the study had discontinued treatment, including one participant who underwent cardiac transplantation for their disease.

- Adverse events reported in the OLE study were generally consistent with the underlying ATTR-CM disease state and no safety signals of potential clinical concern were associated with the administration of AG10 in the study. Forty-six (97.9%) patients experienced a treatment-emergent adverse event reported during the study, with falls, congestive cardiac failure, dyspnea, and acute kidney injury the most commonly reported adverse events. Nineteen (40.4%) participants experienced a treatment-emergent serious adverse event reported during the study, with congestive cardiac failure (10.6%) and acute kidney injury (8.5%) the most commonly reported serious adverse events.
- The rate of all-cause mortality (including either death or cardiac transplantation, 8.5%) and cardiovascular-related hospitalizations (25.5%) observed in an exploratory analysis of participants in this study following a median of 15 months since Phase 2 initiation were lower than those observed at 15 months in placebo-treated patients in the ATTR-ACT study (all-cause mortality including death or cardiac transplantation, 15.3%; cardiovascular-related hospitalizations, 41.8%).
- Stabilization of TTR, as measured using established ex vivo assays, was maintained >90% on average at all study visits in actively treated patients.

- Serum TTR levels, a prognostic indicator of survival in a published cohort of wild-type ATTR-CM patients, were elevated upon AG10 treatment and were maintained in the normal range throughout the study duration. Mean serum TTR levels were increased from baseline by 39% and 56% in wild-type and variant-carrying ATTR-CM patients, respectively, at OLE Visit Day 180.
- Cardiac biomarkers and echocardiographic parameters were stable during the OLE study. NT-proBNP and Tnl were unchanged throughout the course of the study. Echocardiographic parameters, including left ventricular mass and left ventricular stroke volume index, were unchanged during the study.

The presentation of the Phase 2 open label extension data from the American Heart Association (AHA) Scientific Sessions will be available on the company website ([eidostx.com](http://eidostx.com) and [bridgebio.com](http://bridgebio.com)).

A Phase 3 study of AG10 in ATTR-CM patients (ATTRibute-CM) is currently ongoing. In Part A of the study, change in six-minute walk distance at 12 months will be compared between active treatment and placebo groups as the first registrational primary endpoint. In Part B, all-cause mortality and frequency of cardiovascular-related hospitalizations will be compared between treatment and control groups at 30 months total duration. In Part B, concomitant use of therapies indicated for the treatment of ATTR-CM may be allowed. The study is enrolling at 44 sites across six countries and enrollment for Part A is now projected to complete in the second half of 2020, with top-line data expected in 2021.

#### **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 randomized, placebo-controlled 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. The open label extension of this Phase 2 study identified no safety signals of potential clinical concern associated with administration of AG10 15 months after study initiation. In an exploratory analysis, lower rates of all-cause mortality (including death and cardiac transplantation) and cardiovascular hospitalizations were observed in study participants than in placebo-treated ATTR-CM patients in the ATTR-ACT study. Cardiac biomarkers and echocardiographic parameters were stable in the AG10 Phase 2 OLE.

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. 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 at 30 months. In addition, Eidos plans to initiate a Phase 3 study of AG10 in ATTR polyneuropathy (ATTR-PN) in Q1 2020.

#### **About transthyretin amyloidosis (ATTR)**

There is significant medical need in transthyretin amyloidosis (ATTR) given the large patient population and limited 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 estimated to be at least 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 BridgeBio Pharma**

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](http://www.bridgebio.com).

#### **About Eidos Therapeutics**

Eidos is a BridgeBio Pharma subsidiary focused on addressing the large and growing unmet need 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](http://www.eidostx.com).

#### **Forward-Looking Statements**

This release includes forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. All statements other than statements of historical facts, including the statements about the potential therapeutic and clinical benefits of AG10, the potential for AG10 to be a best-in-class treatment for ATTR-CM patients, the design of our ongoing Phase 3 ATTRibute-CM trial of AG10, our ability to enroll patients in and conduct the ATTRibute-CM trial and our planned Phase 3 clinical trial of AG10 in ATTR-PN in accordance with our plans, our ability to generate data from and to complete these trials, the timing of these events, the indications we intend to pursue and our possible clinical or other business strategies, are forward-looking statements. Forward-looking statements can be identified by terms such as "believes," "expects," "plans," "potential," "would" or similar expressions and the negative of those terms. These forward-looking statements are based on our management's current beliefs and assumptions about future events and on information currently available to management. Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be

materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. These risks include, but are not limited to, risks and uncertainties related to: our limited operating history and historical losses, our liquidity to fund the development of AG10 through current and future milestones, our ability to raise additional funding to complete the development of AG10, our dependence on the success of AG10, our ability to enroll patients in the ATTRibute-CM trial and our planned Phase 3 clinical trial of AG10 in ATTR-PN, results from our clinical trials and pre-clinical studies and those of third parties working in the same area as our product candidate, our ability to advance AG10 in clinical development in accordance with our plans, and our dependence on third parties in connection with our manufacturing, clinical trials and pre-clinical studies. Additional risks and uncertainties that could affect our future results are included in the section titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, which is available on the SEC's website at [www.sec.gov](http://www.sec.gov) and our websites at [eidostx.com](http://eidostx.com) and [bridgebio.com](http://bridgebio.com). Additional information on potential risks will be made available in other filings that we make from time to time with the SEC. In addition, any forward-looking statements contained in this press release are based on assumptions that we believe to be reasonable as of this date. Except as required by law, we assume no obligation to update these forward-looking statements, or to update the reasons if actual results differ materially from those anticipated in the forward-looking statements.

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