



Eidos Therapeutics Announces Positive Phase 2 Data for AG10 in Symptomatic Patients with Mutant or Wild-Type TTR Amyloid Cardiomyopathy

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AG10 Was Well Tolerated in Subjects Administered 400 mg or 800 mg Twice Daily for 28 Days

AG10 Resulted in Average TTR Stabilization Greater than 90% in All Actively Treated Subjects at Day 28

AG10 Significantly Raised Serum TTR Concentrations ($p < 0.0001$) by 50% and 36% in Subjects Administered 800 mg Twice Daily and 400 mg Twice Daily, Respectively, at Day 28

Normalized Serum TTR Levels Observed in All Actively Treated Subjects at Day 28

Eidos to Host Conference Call and Webcast on Monday, November 12th at 8am ET

SAN FRANCISCO, Nov. 10, 2018 (GLOBE NEWSWIRE) -- Eidos Therapeutics, Inc. (Eidos) (Nasdaq:EIDX), today announced positive results of its Phase 2 clinical trial studying AG10 in subjects with symptomatic transthyretin (TTR) amyloidosis cardiomyopathy (ATTR-CM). The data were presented in a late-breaking Featured Science oral presentation at the American Heart Association (AHA) Scientific Sessions. AG10 was well tolerated, demonstrated >90% TTR average stabilization at day 28, and increased serum TTR concentrations, a prognostic indicator of survival in ATTR-CM, in a dose-dependent manner. Subject to discussions with regulatory agencies, these data support the advancement of AG10 into Phase 3 pivotal trials planned to be initiated in the first half of 2019.

"These data demonstrate that AG10 is well tolerated in symptomatic patients with ATTR-CM with clear evidence of drug activity in all actively treated subjects," said Jonathan Fox, MD, PhD, president and chief medical officer of Eidos. "The consistently high levels of TTR stabilization, in all actively treated subjects and across the entire dosing interval, were correlated with statistically significant and dose-dependent increases in serum TTR concentrations. We observed normalized serum TTR levels in 100% of patients treated with AG10. We believe these data provide clinical proof-of-concept for AG10 in ATTR-CM patients. As reflected in these data, AG10's TTR-stabilizing properties continue to hold great promise that it could become a best-in-class treatment for ATTR-CM."

Phase 2 Clinical Trial

This Phase 2 clinical trial was a randomized, double-blind, placebo-controlled, multi-center study that enrolled patients with symptomatic ATTR-CM, both wild-type and mutant. Eligible patients had confirmed ATTR-CM and NYHA Class II or III symptoms, and at least one prior heart failure hospitalization or active treatment for chronic heart failure. The 49 enrolled subjects were randomly assigned in a 1:1:1 fashion to treatment with placebo, 400 mg AG10 twice daily, or 800 mg AG10 twice daily for 28 days. Results from the study showed the following:

- The study met its primary objective of establishing that AG10 was well tolerated with no safety signals of potential clinical concern related to the administration of AG10 in symptomatic ATTR-CM patients. One serious adverse event (SAE) of dyspnea deemed unrelated to study drug was observed in one actively-treated subject (3%) and SAEs of atrial fibrillation, congestive heart failure, and cellulitis were observed in two placebo-treated subjects (12%). The overall rate of adverse events (AEs) was 69% in subjects administered 800 mg bid AG10, 63% in subjects administered 400 mg bid AG10, and 88% in subjects administered placebo.
- As compared to placebo, subjects treated with AG10 demonstrated a statistically significant increase in serum TTR concentrations ($p < 0.0001$), a prognostic indicator of survival in ATTR-CM patients, in a dose-dependent manner. Subjects administered 800 mg AG10 twice daily, 400 mg AG10 twice daily, and placebo exhibited mean changes in TTR concentration from baseline of +50%, +36% and -7%, respectively, at day 28.
- All subjects administered AG10 had serum TTR concentrations within the normal range at day 28, whereas 31% of subjects administered placebo had serum TTR concentrations below the normal range on day 28.
- AG10 administration resulted in near-complete stabilization of TTR at day 28 (>90%, on average), across the dosing interval in all actively treated subjects as measured by established ex vivo assays.

"We know that stabilizing TTR can lead to clinical benefit in ATTR patients, that treatment with a stabilizer increases TTR levels in ATTR-CM patients, and that higher serum concentrations of TTR are associated with a better prognosis in ATTR-CM," said Dr. Daniel Judge, M.D., professor in the division of cardiology at the Medical University of South Carolina. "These Phase 2 data provide compelling evidence that AG10 stabilizes TTR to a high degree and restores serum TTR levels to normal even in patients carrying destabilizing mutations, suggesting the potential for the molecule to

become an effective disease-modifying therapy for ATTR patients.”

Investor Conference Call and Webcast Details

Eidos management will host an investor conference call and webcast on Monday, November 12 at 8am ET to review the Phase 2 data. To participate in the conference call, dial +1-844-293-0174 (U.S. toll free) or +1-916-582-3546 (international), conference ID 8594856. The webcast will be available live and for replay on the company's website at ir.eidostx.com.

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 amyloidosis, or ATTR. AG10 is currently being studied in an open-label extension of a Phase 2 clinical trial in patients with ATTR cardiomyopathy.

AG10 was designed to mimic a naturally-occurring variant of the TTR gene (T119M) that is considered a “rescue mutation” because it has been shown to prevent ATTR in individuals carrying pathogenic, or disease-causing, mutations in the TTR gene. To our knowledge, AG10 is the only TTR stabilizer in development that has been observed to mimic the “super-stabilizing” properties of this rescue mutation.

About transthyretin amyloidosis (ATTR)

ATTR represents a significant unmet need of a comparatively large patient population in the context of rare genetic diseases with an inadequate current standard of care. There are three distinct diseases that comprise the ATTR family: 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 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. 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 Eidos Therapeutics

Eidos Therapeutics is a clinical stage biopharmaceutical company focused on addressing the large and growing unmet need in diseases caused by transthyretin (TTR) amyloidosis (ATTR). For more information, please visit 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. All statements other than statements of historical facts, including the statements about the potential therapeutic and clinical benefits of AG10, its potential to become a best-in-class treatment for ATTR-CM, future clinical milestones of AG10, 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 our other product candidates through current and future milestones, our ability to raise additional funding to complete the development of AG10, our dependence on the success of AG10, 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, 2018, which is available on the SEC's website at www.sec.gov and our website at eidostx.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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