



## **Eidos Therapeutics Initiates ATTRibute-CM, a Phase 3 Study of AG10 in ATTR-CM with Registrational 12-month Endpoint**

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*ATTRibute-CM study design, which incorporates feedback from the FDA, has the potential to accelerate registration with a 12-month primary endpoint of change in 6-minute walk distance (6MWD), followed by a 30-month endpoint of mortality and cardiovascular-related hospitalizations*

*Trial sites activated in global Phase 3 study (ATTRibute-CM) of AG10 in patients with transthyretin amyloidosis cardiomyopathy*

*Eidos to Host Conference Call and Webcast at 8am ET*

SAN FRANCISCO, Feb. 27, 2019 (GLOBE NEWSWIRE) -- Eidos Therapeutics, Inc. (Eidos) (Nasdaq:EIDX), today announced the initiation and design of its pivotal global Phase 3 trial (ATTRibute-CM) of AG10 in patients with transthyretin (TTR) amyloid cardiomyopathy (ATTR-CM). The design of the ATTRibute-CM study, which incorporates feedback from FDA, includes two potentially registrational endpoints. In Part A, benefit in change from baseline in 6-minute walk distance (6MWD) will be evaluated at 12 months, potentially accelerating the time to registration. In Part B, reduction in all-cause mortality and frequency of cardiovascular-related hospitalizations will be evaluated at 30 months.

"ATTRibute-CM aims to generate registrational evidence that AG10 provides meaningful benefit to ATTR-CM patients. The trial is designed to evaluate preservation of function and quality of life on an accelerated timeframe in addition to evaluating longer-term benefit on mortality and cardiovascular-related hospitalizations," said Jonathan Fox, MD, PhD, president and chief medical officer of Eidos. "If successful, this trial could lead to an approval of AG10 for the treatment of ATTR-CM, a disease with significant unmet medical need and limited treatment options."

The ATTRibute-CM study advances clinical development of AG10 following positive results from a Phase 2 trial in subjects with symptomatic ATTR-CM. In that study, AG10 was shown to be generally well-tolerated and significantly increase serum transthyretin, a biomarker associated with survival in a retrospective study of ATTR-CM patients, in a dose-dependent manner. All subjects treated with AG10 achieved normal serum transthyretin levels within 28 days of treatment, even those whose baseline levels were well below normal. Results of this study were presented in a late-breaking Featured Science oral presentation at the Annual Scientific Sessions of the American Heart Association in November 2018. An open-label extension of this study is ongoing.

### **ATTRibute-CM Design**

ATTRibute-CM will enroll approximately 510 subjects with symptomatic ATTR-CM, associated with either wild-type or mutant TTR, with New York Heart Association Class I-III symptoms. Subjects will be randomized 2:1 between treatment (AG10 800 mg) and placebo twice daily. In Part A, change in 6MWD at 12 months will be compared between treatment and placebo groups as the first registrational primary endpoint. In Part B, the study will continue for a total duration of 30 months, at which point all-cause mortality and frequency of cardiovascular-related hospitalizations will be compared between treatment and control groups. Secondary endpoints include quality of life as assessed by the Kansas City Cardiomyopathy Questionnaire, safety parameters, serum TTR levels, and TTR stabilization. In Part B, concomitant use of approved, indicated therapies may be allowed.

### **Investor Conference Call and Webcast Details**

Eidos management will host an investor conference call and webcast 8 am ET to review the Phase 3 trial design. To participate in the conference call, dial +1-844-293-0174 (U.S. toll free) or +1-916-582-3546 (international), conference ID 7365928. The webcast will be available live and for replay on the company's website at [ir.eidostx.com](http://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. In a Phase 2 clinical trial in subjects with symptomatic ATTR-CM, AG10 was generally well tolerated, demonstrated >90% 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 is currently being studied in an open-label extension of a Phase 2 clinical trial in patients with ATTR-CM and sites are currently being activated for a Phase 3 clinical trial of AG10 in patients with ATTR-CM (ATTRibute-CM).

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 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.

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

ATTR represents a significant unmet medical need with a 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 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). 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. All statements other than statements of historical facts, including the statements about the potential therapeutic and clinical benefits of AG10, the potential registrational endpoints in the ATTRibute-CM trial, the potential to accelerate registration of AG10, the design of the ATTRibute-CM trial, our ability to enroll patients in and conduct the ATTRibute-CM trial in accordance with our plans, future clinical and regulatory 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 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, 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](http://www.sec.gov) and our website at [eidostx.com](http://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.

### **Media Contact:**

Carolyn Hawley  
Canale Communications  
619-849-5382  
[Carolyn@canalecomm.com](mailto:Carolyn@canalecomm.com)

### **Investor Contact:**

Alex Gray  
Burns McClellan  
212-213-0006  
[agray@burnsmc.com](mailto:agray@burnsmc.com)



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