Eidos Therapeutics Announces Publication in the Journal of the American College of Cardiology (JACC) and Presentation at American College of Cardiology (ACC) Scientific Sessions

March 18, 2019

AG10 demonstrated equivalent, near-complete stabilization of TTR in wild-type and mutant subjects with TTR amyloid cardiomyopathy (ATTR-CM).

AG10 treatment normalized serum TTR levels, a prognostic indicator of survival in ATTR-CM patients, in all mutant and wild-type subjects.

AG10 has the potential to be a safe and effective treatment for patients with ATTR-CM, and is being evaluated in the global Phase 3 ATTRibute-CM study.

SAN FRANCISCO, March 18, 2019 (GLOBE NEWSWIRE) -- Eidos Therapeutics, Inc. (Eidos) (Nasdaq:EIDX), a clinical stage biopharmaceutical company focused on addressing the large and growing unmet need in diseases caused by transthyretin (TTR) amyloidosis (ATTR), today announced the publication of its Phase 2 clinical trial studying AG10 in subjects with symptomatic TTR amyloid cardiomyopathy (ATTR-CM) in the Journal of the American College of Cardiology (JACC) entitled "Transthyretin Stabilization by AG10 in Symptomatic Transthyretin Amyloid Cardiomyopathy".

Eidos also presented subgroup analyses from the same Phase 2 clinical study in an oral presentation at this year's American College of Cardiology (ACC) Scientific Sessions. Stephen Heitner, M.D., director of the hypertrophic cardiomyopathy clinic at the Oregon Health & Science University, presented these findings in an oral presentation entitled "AG10 Consistently Stabilizes Transthyretin to a High Level in Both Wild Type and Mutant Amyloid Cardiomyopathy: Responder Analyses From a Phase 2 Clinical Trial".

"These data confirm the potential ability of AG10 to treat ATTR-CM, regardless of mutational status," said Jonathan Fox, MD, PhD, president and chief medical officer of Eidos. "The observed near-complete stabilization of TTR in all actively treated subjects and across the dosing interval provide clinical proof-of-concept for AG10 in both wild-type and mutant ATTR-CM. These Phase 2 results support further investigation of AG10 in the ongoing ATTRibute-CM Phase 3 study."

The primary results from the Phase 2 study were presented at the 2018 American Heart Association (AHA) Annual Meeting in Chicago, IL on November 10, 2018.

About ATTRibute-CM

ATTRibute-CM (NCT03860935) is expected to 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 six-minute walk distance 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. The design of ATTRibute-CM was reviewed with the U.S. Food and Drug Administration (FDA) and with the European Medicines Agency (EMA).

About AG10

AG10 is an investigational, orally-administered small molecule designed to potently stabilize TTR thereby halting at its outset the series of molecular events that give rise to 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.

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 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 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 for AG10 to be a safe and effective treatment for all forms of ATTR-CM, the potential registrational endpoints in the ATTRibute-CM trial, the design of the ATTRibute-CM trial, our ability to enroll patients in and conduct the ATTRibute-CM trial in accordance with our plans, 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 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.

Media Contact:
Carolyn Hawley
Canale Communications
619-849-5382
Carolyn@canalecomm.com

Investor Contact:
Alex Gray
Burns McClellan
212-213-0006
agray@burnsmc.com

Source: Eidos Therapeutics, Inc.