Eidos Therapeutics Presents Data from its Phase 1 Clinical Trial of AG10 at the 22nd Annual Scientific Meeting of the Heart Failure Society of America

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SAN FRANCISCO, Sept. 17, 2018 (GLOBE NEWSWIRE) -- Eidos Therapeutics, Inc. (Eidos) (Nasdaq:EIDX) announced the presentation of results from its Phase 1 clinical trial of AG10 during a poster session at the 22nd Annual Scientific Meeting of the Heart Failure Society of America (HFSA). The poster, entitled "AG10, A Novel, Potent, and Selective Transthyretin Stabilizer, Is Well-Tolerated at Doses Resulting in Target Therapeutic Blood Levels, and Demonstrates Clinical Proof-of-Concept in Healthy Volunteers," was presented on Saturday, September 15, and is accessible through the science section of the company's website.

In this initial Phase 1 study in healthy adult volunteers, AG10 was well-tolerated with no safety signals of potential clinical concern identified. At the highest tested dose, AG10 achieved 100% transthyretin (TTR) stabilization at peak concentration and over 95% TTR stabilization on average at steady state. Serum TTR concentrations, an in vivo indicator of TTR stability, were increased to a greater degree in AG10-treated patients than placebo-treated patients from baseline to Day 12.

"We believe that maximizing the level of TTR stabilization will lead to optimal clinical benefit for TTR Amyloidosis (ATTR) patients. This initial human trial demonstrated that AG10 stabilized TTR in established ex vivo assays and increased circulating TTR concentrations," noted Jonathan C. Fox, M.D., Ph.D., president and chief medical officer of Eidos. "Given that below normal levels of serum TTR are associated with poorer prognosis in ATTR patients, and stabilization of TTR by protective mutations or small molecule stabilizers increase serum TTR concentrations, we believe that these data provide clinical proof of concept. We are rapidly moving forward with additional studies of AG10 and have already completed enrollment in our Phase 2 trial in patients with symptomatic ATTR cardiomyopathy (ATTR-CM)."

TTR normally circulates in the blood as a four-part molecule, or tetramer. In ATTR, the tetramer is destabilized by inherited mutation or age-related factors and dissociates into individual monomers, which can aggregate and are deposited as amyloid fibrils in various tissues. AG10, Eidos' lead product candidate, is designed to target ATTR at its source by stabilizing tetrameric TTR in the blood. Eidos is pursuing AG10 for the treatment of ATTR-CM and ATTR polyneuropathy (ATTR-PN), both of which are progressive, fatal diseases. The company plans to initiate Phase 3 studies in each indication in the first half of 2019.

About AG10

AG10 is an 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 transthyretin amyloidosis, or ATTR. AG10 is currently being tested in a Phase 2 clinical trial in patients with symptomatic ATTR cardiomyopathy. Top-line results from this trial are expected to be reported in the fourth quarter of 2018.

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 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 future clinical milestones of AG10, including the completion of our ongoing Phase 2 clinical trial and availability of top-line data therefrom, the initiation of Phase 3 clinical 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 our other product candidates through current and future milestones, our ability to raise additional funding to complete the development and any commercialization of our product candidates, our dependence on the success of our lead product candidate, AG10, results from our clinical trials and pre-clinical studies and those of third parties working in the same area as our product candidate 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 the final

prospectus for our initial public offering filed with the SEC on June 21, 2018, and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, which are 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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