



FDA Grants Orphan Drug Designation to Eidos Therapeutics' Product Candidate, AG10, for Treatment of Transthyretin Amyloidosis

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SAN FRANCISCO, Oct. 03, 2018 (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), announced today that the U.S. Food and Drug Administration (FDA) has granted the company Orphan Drug Designation for AG10 for the treatment of ATTR.

TTR normally circulates in the blood as a four-part molecule or tetramer, but in ATTR the tetramer destabilizes and dissociates into individual monomers which aggregate as amyloid fibrils. AG10, Eidos' lead product candidate, is designed to target ATTR at its source by stabilizing tetrameric TTR in the blood. Eidos is investigating AG10 for the treatment of ATTR cardiomyopathy (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.

"The granting of Orphan Drug status to Eidos' lead development candidate, AG10, for treating transthyretin amyloidosis is an important step forward for the product and our company," said Jonathan Fox, M.D., Ph.D., president and chief medical officer of Eidos. "We believe that AG10 holds great promise as a disease-modifying therapy for patients with ATTR, and Orphan status will help us develop it as quickly as possible."

Orphan Drug Designation provides incentives and support for the development of drugs for patients with rare diseases, specifically to drugs that are intended for the treatment of diseases or conditions that affect fewer than 200,000 people in the United States. Orphan Drug Designation provides certain benefits, including seven years of market exclusivity upon regulatory approval, exemption from FDA application user fees and tax credits for qualified clinical trial expenses.

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 examined in a Phase 2 clinical trial in patients with ATTR cardiomyopathy. Top-line results from this trial are expected to be reported by the end 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 the clinical and therapeutic potential and future clinical milestones of AG10, including the initiation of Phase 3 clinical trials, the potential for AG10 to capture any expected benefits from Orphan Drug Designation, the indications we intend to pursue and our possible clinical or other business strategies, and the timing of these events, 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 our Quarterly Report on Form 10-Q for the quarter ended June 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, agrav@burnsmc.com



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