



NEWS RELEASE

eidos therapeutics receives positive opinion for orphan designation from the european medicines agency for ag10, a potent oral stabilizer for the treatment of transthyretin amyloidosis

2018-10-26

SAN FRANCISCO, Oct. 26, 2018 (GLOBE NEWSWIRE) — Eidos Therapeutics, Inc. (Eidos) (Nasdaq:**EIDX**), announced today that the Committee for Orphan Medicinal Products (COMP) within the European Medicines Agency (EMA) adopted a positive opinion for the designation of Eidos' product candidate, AG10, as an orphan medicinal product for the treatment of transthyretin (TTR) amyloidosis (ATTR). In addition, the EMA also granted a product-specific pediatric investigational plan waiver to Eidos for AG10.

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

"We are pleased to have received Orphan Drug Designation from the FDA and now a positive opinion from the EMA as well. Eidos is excited about the opportunity to work with the ATTR patient and provider community on the further development of AG10 for ATTR," noted Jonathan Fox, M.D., Ph.D., president and chief medical officer of Eidos. "Importantly, AG10 has demonstrated potent stabilization of tetrameric TTR in our clinical program, and we believe maximizing the level of TTR stabilization will lead to optimal clinical benefit for patients living with this devastating disease."

Orphan Designation by the European Commission provides regulatory and financial incentives for the development



of medicines that treat a

life-threatening or chronically debilitating condition affecting less than five in 10,000 people in the European Union (EU). Orphan Designation provides Eidos with certain benefits, including EU market exclusivity upon regulatory approval, if received, reductions in EMA application fees, and access to protocol assistance. Eidos is pursuing 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.

about ag10

AG10 is an investigational, orally administered small molecule designed to potentially 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 and symptomatic heart failure. Results from this trial will be presented on November 10, 2018 at the American Heart Association's Annual Scientific Sessions.

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 also carrying a pathogenic, or disease-causing, mutation in the other copy of their 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 data therefrom, the initiation of Phase 3 clinical trials, the timing of these events, the indications we intend to pursue, the potential benefits available to us from orphan drug designations for AG10, 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 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

for investors

Alex Gray, Burns McClellan, (212) 213-0006, agray@burnsmc.com