



NEWS RELEASE

preclinical data demonstrates potential of eidos therapeutics' lead compound for the treatment for transthyretin amyloidosis

2017-11-15

PALO ALTO, Calif., Nov. 15, 2017 /PRNewswire/ — **Eidos Therapeutics**, a subsidiary of **BridgeBio Pharma**, today announced that recently presented preclinical data highlight the potential of AG10 to treat TTR amyloid cardiomyopathy (ATTR-CM). These data were shared publicly for the first time yesterday as oral podium presentations at the American Heart Association's (AHA) 2017 Annual Scientific Sessions in Anaheim, CA. Eidos has recently initiated clinical trials to evaluate the potential of AG10 in treating this progressive, fatal disease.

ATTR-CM is a progressive, fatal disease caused by the accumulation of misfolded ATTR fibrils in the heart. Transthyretin (TTR) typically exists as a tetramer in the serum. With certain mutations, the four-part molecule can destabilize and dissociate into individual monomers. The monomers are unstable and can misfold and aggregate as amyloid fibrils. These deposits are toxic to cardiac muscle cells and limit the heart's ability to fill completely during relaxation, leading to progressive and fatal heart failure.

Data from Eidos' in vitro and animal studies of AG10 were presented in a **rapid-fire oral session** by Uma Sinha, Ph.D., Eidos' chief scientific officer. These experiments demonstrated that AG10 binds native, tetrameric TTR in human and animal serum and prevents its dissociation into disease-causing monomers. Dr. Sinha also presented a preclinical pharmacokinetic evaluation of AG10 that suggests the potential for once daily oral dosing in ATTR patients.

Additionally, in a full **oral presentation**, Isabella Graef, Eidos' co-founder and assistant professor of pathology at

Stanford University, described biochemical experiments that demonstrated the ability of AG10 to stabilize a series of mutant proteins associated with TTR-CM.

“Taken together, these nonclinical studies clearly demonstrate the potential for AG10 to be orally dosed in patients once daily and result in blood levels of AG10 that completely stabilize the native tetrameric form of TTR,” said Dr. Sinha. “It is well recognized that dissociation of TTR tetramers into monomers is the initial step in the development of ATTR, and that the stability of TTR tetramers is reduced by the disease-promoting mutations examined in Eidos’ studies. By stabilizing TTR tetramers in the blood, AG10 targets ATTR at its source and offers the potential to slow or halt progression of disease.”

About AG10

AG10 is a small molecule that selectively and potently binds to and stabilizes the tetrameric protein transthyretin, preventing its dissociation into disease-causing monomers. AG10 was discovered and initially developed by Eidos’ co-founders Isabella Graef, MD, and Mamoun Alhamadsheh, PhD, at Stanford University and the University of the Pacific, respectively.

About transthyretin amyloid cardiomyopathy (ATTR-CM)

ATTR-CM is a progressive, fatal disease caused by the accumulation of misfolded ATTR fibrils in the heart. These deposits are toxic to cardiac muscle cells and limit the heart’s ability to fill completely during relaxation, leading to progressive and fatal heart failure.

Approximately 240,000 people worldwide suffer from ATTR cardiomyopathy, though the patient population may be underestimated due to its rarity and confusion with more common forms of heart failure. Most patients are diagnosed after age 60, when the disease is already advanced, and may progress rapidly.

Some patients are predisposed to ATTR cardiomyopathy due to mutations in the gene that encodes TTR. The most prevalent pathogenic mutation, V122I TTR, is carried by 3.4% of African Americans.

There are no FDA-approved medications indicated for the treatment of ATTR cardiomyopathy.

About Eidos Therapeutics

Eidos Therapeutics is a clinical stage biopharmaceutical company based in the San Francisco Bay Area. Eidos is developing AG10 as a targeted therapeutic for transthyretin amyloidosis. The company’s singular mission is to improve the lives of patients suffering from this disease. Launched in 2016 after years of research supported by

Stanford's TRAM and SPARK programs, Eidos is led by a team of veteran biotechnology executives.

Together with patients and physicians, the company aims to bring a safe, effective treatment to market as quickly as possible.

About BridgeBio Pharma

BridgeBio is a clinical-stage biotechnology company developing novel, genetically targeted therapies to improve the lives of patients. The BridgeBio approach combines a traditional focus on drug development with a unique corporate model, allowing rapid translation of early stage science into medicines that treat rare diseases at their source.

Founded in 2015 by a team of industry veterans who previously brought more than a dozen products to market, BridgeBio has built a portfolio of ten transformative drug programs that address rare diseases across oncology, cardiology, dermatology and endocrinology. The drugs are in various phases of development, from discovery to late clinical stage.

With a commitment to scientific excellence and rapid execution, BridgeBio aims to translate today's discoveries into tomorrow's medicines.