FDA Grants Orphan Drug Designation to Allena Pharmaceuticals' Investigational Therapy for the Treatment of Primary Hyperoxaluria

July 13, 2017

Newton, Mass - July 13, 2017 - Allena Pharmaceuticals, Inc., a specialty biopharmaceutical company dedicated to bringing first in class, specific, non-absorbed, oral protein therapeutics to patients with serious renal, urologic and orphan diseases, announced today that the US Food and Drug Administration (FDA) has granted orphan drug designation to Allena's investigational product ALLN-177, an oral formulation of oxalate decarboxylase, for the treatment of primary hyperoxaluria (PH).

Allena's lead compound ALLN-177, is being developed to treat patients with severe hyperoxaluria, or patients with markedly elevated urinary oxalate excretion. PH, a type of severe hyperoxaluria, is a rare genetic disorder caused by endogenous overproduction of oxalate by the liver that can result in kidney stone disease, kidney damage, and kidney failure, which may lead to death. PH has three main types, PH1, PH2 and PH3, and is estimated to affect approximately 1 in 58,000 based on population analysis.1 The most severe and most common type among genetically characterized patients is PH1. These patients typically develop recurrent kidney stones with progressive nephrocalcinosis and end stage renal disease by 20-30 years of age.1 There are no FDA approved therapies for PH, and the most severe patients may be treated with liver and/or kidney transplant.

ALLN-177 is a first-in-class therapeutic being developed to treat patients with severe hyperoxaluria using an oral, non-absorbed enzyme that works in the gastrointestinal (GI) tract, where it can degrade both dietary and endogenously produced oxalate. GI elimination of oxalate may help alleviate the chronic systemic oxalate burden on PH patients.

"Primary Hyperoxaluria is a devastating disease for patients and their families. We desperately need better therapeutic options to treat this disease," said Craig B. Langman, M.D., the Issac A. Abt M.D. Professor of Kidney Diseases at Feinberg School of Medicine, Northwestern University and Head, Kidney Diseases at Lurie Children's Hospital Chicago.

The preclinical data supporting the FDA orphan designation demonstrated oxalate decarboxylase significantly reduced urinary and plasma oxalate in multiple animal models including a rodent disease model of PH and a porcine model with urine oxalate in the range seen in PH.

The Orphan Drug Designation Program is administered by the FDA's Office of Orphan Products Development, which grants orphan status to drugs intended to treat rare diseases that affect fewer than 200,000 people in the U.S. The program provides incentives for sponsors and has enabled the development and marketing of more than 400 products for rare diseases since 1983.

"This is an important regulatory designation to advance the development of ALLN-177 for patients who could benefit from novel therapeutic options that directly address excess oxalate," said Louis Brenner, M.D., President and Chief Operating Officer of Allena Pharmaceuticals. "We are committed to the development of ALLN-177 for the treatment of patients with severe hyperoxaluria disorders."

About Hyperoxaluria and ALLN-177

Hyperoxaluria is a metabolic disorder resulting from high oxalate levels in the urine due to either hyper-absorption of oxalate from the diet (secondary) or from overproduction of oxalate by the liver due to a genetic defect (primary). Kidney stones are typically the first sign of hyperoxaluria, are often painful, and may require interventional procedures. Severe hyperoxaluria in settings of enteric and primary hyperoxaluria may also lead to kidney damage (nephrocalcinosis), chronic kidney disease and end-stage renal disease, which may lead to death.

ALLN-177 is an orally-administered, recombinant oxalate-degrading enzyme in development for the treatment of severe hyperoxaluria. ALLN-177 targets oxalate in the GI tract in an effort to reduce the burden of both dietary and endogenously produced oxalate. ALLN-177 has the potential to decrease the oxalate available systemically for deposition as calcium oxalate crystals or stones in the kidneys, as well as reduce long-term kidney complications.

About Allena Pharmaceuticals

Allena Pharmaceuticals, Inc. is a specialty biopharmaceutical company dedicated to bringing first in class, specific, non-absorbed, oral protein therapeutics to patients with serious renal, urologic and orphan diseases. Allena is completing a Phase 2 program in secondary hyperoxaluria. The company's technological approach enables the design and development of oral protein therapies that remain in the gastrointestinal (GI) tract, where the protein exerts its therapeutic effect by degrading certain nephrotoxic metabolites, without being absorbed into the bloodstream. Led by a proven management team with deep expertise in protein therapeutic design, development, and commercialization, Allena is committed to bringing breakthrough new treatments to patients with unmet medical needs. Based in Newton, MA, the company is supported by a top-tier investor syndicate including Frazier Healthcare, Third Rock Ventures, Bessemer Venture Partners, HBM Partners, Pharmstandard International S.A., Partner Fund Management, Fidelity Management & Research Company, and other investors. For more information, please visit www.allenapharma.com (http://www.allenapharma.com).

References:
1. Hopp, Katharina et al. "Phenotype-Genotype Correlations and Estimated Carrier Frequencies of Primary Hyperoxaluria." J Am Soc Nephrol. 26.10 (2015): 2559-2570.

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