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

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Newton, Mass. - July 27, 2017 - Allena Pharmaceuticals, Inc., a specialty biopharmaceutical company dedicated to bringing first in class, specific, non-absorbed, oral enzyme therapeutics to patients with serious renal, urologic and orphan diseases, announced today that the European Commission has granted orphan drug designation to Allena's investigational product ALLN-177, Bacillus subtilis oxalate decarboxylase, for the treatment of primary hyperoxaluria (PH). The orphan designation was granted to Allena Pharmaceuticals Ireland Limited, a subsidiary of Allena Pharmaceuticals, Inc.

Allena's lead compound ALLN-177, is being developed to treat patients with severe hyperoxaluria, a condition characterized by 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 0.1 in 10,000 people, which is roughly 5,000 people in the European Union (EU).1 The most severe and most common type among genetically characterized patients is PH1. These patients typically develop recurrent kidney stones, progressive renal damage, chronic kidney disease and end stage renal disease at a median age of 24 years of age.2 There are no EU 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 is designed to degrade both dietary and endogenously produced oxalate that is secreted into the GI tract. GI elimination of oxalate has potential to help alleviate the chronic systemic oxalate burden on PH patients.

"Primary Hyperoxaluria is a debilitating disease, which starts at a young age and leads to renal failure in young adulthood. It's imperative that we have better therapeutic options to treat this disease," said Eduardo Salido, M.D., Ph.D., Professor of Pathology at Universidad de La Laguna, Tenerife, Spain. Dr. Salido recently hosted the 12th International PH Workshop for Professionals, Patients and Families organized by the Oxalosis and Hyperoxaluria Foundation and is on the steering committee of Oxal Europe, the European Hyperoxaluria Consortium. Dr. Salido also co-authored a previous publication that demonstrated that oxalate decarboxylase significantly reduced urinary oxalate levels in a preclinical disease model of PH. This study was one of multiple animal studies including a rodent disease model and a porcine model of hyperoxaluria with hyperoxalemia that supported the ALLN-177 orphan drug designation application to the European Medicines Agency (EMA).

"This regulatory designation from the European Commission represents an expansion of our previous efforts in United States," said Louis Brenner, M.D., President and Chief Operating Officer of Allena Pharmaceuticals. "We are excited to develop ALLN-177 to address the oxalate disease burden for PH patients in both the US and EU, where there are no US Food and Drug Administration or European Commission approved therapies for this unmet medical need."

About Hyperoxaluria and ALLN-177

Hyperoxaluria is a metabolic disorder resulting from high oxalate levels in the urine due to either overproduction of oxalate by the liver due to a genetic defect (primary) or from hyper-absorption of oxalate from the diet (secondary). Secondary hyperoxaluria can be due to an unknown cause (idiopathic) or as a result of underlying GI disorders (enteric). 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 is being developed to target 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. ALLN-177 is an investigational product, and its safety and efficacy have not been evaluated by the EMA or any other health authority.

About Allena Pharmaceuticals

Allena Pharmaceuticals, Inc. is a specialty biopharmaceutical company dedicated to bringing first in class, specific, non-absorbed, oral enzyme 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 enzyme therapies that are designed to remain in the gastrointestinal (GI) tract, where the enzyme 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 enzyme 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. The European registry of orphan diseases (Orphanet 2013)
2. Cochat P., Rumsby G. Primary Hyperoxaluria. New England Journal of Medicine. 2013;369(7):649-658.

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