FDA Grants Orphan Drug Designation to Allena's Investigational Therapy for the Treatment of Pediatric Hyperoxaluria

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NEWTON, Mass—(May 5th, 2016) Allena Pharmaceuticals, Inc., a specialty biopharmaceutical company focused on developing and commercializing innovative, non-systemic, oral protein therapeutics to treat metabolic and orphan diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to Allena's investigational therapy oxalate decarboxylase for the treatment of pediatric hyperoxaluria.

Allena's lead compound ALLN-177, an oral formulation of oxalate decarboxylase, is being developed to treat patients with disorders of oxalate metabolism. Hyperoxaluria is a metabolic disorder characterized by excess excretion of oxalate in the urine. Both primary and secondary pediatric hyperoxaluria can present as early as infancy. Pediatric hyperoxaluria has been associated with significant complications including kidney stones, nephrocalcinosis and kidney failure (end stage renal disease or ESRD).

"Presently there are no effective treatments for hyperoxaluria in children. The granting of this designation highlights the increasing recognition that kidney stones and other complications of hyperoxaluria are a pediatric problem," said Craig B. Langman, M.D., the Isaac A. Abt M.D. Professor of Kidney Diseases at Feinberg School of Medicine, Northwestern University and Head, Kidney Diseases at Lurie Children's Hospital of Chicago. "A recent study looking at kidney stone incidence rates found that since 1996, the rate of kidney stones has been increasing across the population, but most among children ages 10-19. Given the recent and substantial increase in kidney stones among children, it is important to develop new therapies targeting hyperoxaluria, one of the key risk factors for recurrent kidney stones in children," said Gregory Tasian, M.D. MSc, MSCE, Assistant Professor of Urology and Epidemiology at Perelman School of Medicine at the University of Pennsylvania and Attending Physician and co-Director of the Pediatric Kidney Stone Center at the Children's Hospital of Philadelphia.

The Orphan Drug Designation Program is administered by the FDA's Office of Orphan Products Development, which grants orphan status to drugs which are intended to treat rare diseases that affect fewer than 200,000 people in the U.S., or diseases that affect more than 200,000 people in the U.S. in circumstances where there is not expectation of recovering the costs of developing and marketing a therapeutic drug.

"We are encouraged by receiving this orphan designation from the FDA," said Louis Brenner, M.D., chief operating officer of Allena Pharmaceuticals. "This is an important step toward our goal of bringing ALLN-177 to pediatric and adult patients who suffer from oxalate disorders."

About Hyperoxaluria and ALLN-177

Hyperoxaluria is a condition 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). Oxalate is a terminal metabolite that cannot be further degraded by humans and is primarily excreted by the kidneys. Hyperoxaluria can initially cause the development of kidney stones, and may also lead to kidney damage (nephrocalcinosis), chronic kidney disease, end-stage renal disease and dialysis. Calcium oxalate is the most common constituent of kidney stones. There are currently no approved pharmacologic treatments for hyperoxaluria.

ALLN-177 is an orally-administered, recombinant oxalate-degrading enzyme in development for the chronic management of hyperoxaluria and kidney stones (nephrolithiasis). ALLN-177 targets oxalate in the gastrointestinal 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 the incidence of calcium oxalate related complications. Effective management of hyperoxaluria could reduce long-term kidney complications, as well as the number of interventions required for the management of kidney stones.

ALLN-177 is currently being tested in two clinical trials in adult patients. A Phase 2b, Multicenter, Randomized, Double-Blind, Placebo-controlled, Crossover Study is evaluating multiple doses of ALLN-177 in recurrent calcium oxalate kidney stone formers with hyperoxaluria (Clinicaltrials.gov identifier NCT 02503345). An additional Phase 2 Multicenter, Randomized, Double-Blind, Placebo-controlled Study (Clinicaltrials.gov identifier NCT 02547805) is evaluating the safety, tolerability and efficacy of 28 days of treatment with ALLN-177 for reducing urinary oxalate excretion in patients with secondary hyperoxaluria.

About Allena Pharmaceuticals

Allena Pharmaceuticals, Inc. is a specialty biopharmaceutical company focused on developing and commercializing non-systemic protein therapeutics to treat metabolic and orphan diseases. Allena is currently conducting two Phase 2 clinical trials of its lead product candidate, ALLN-177, in patients with 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 toxic metabolites, without being absorbed into the bloodstream. Led by a proven management team with deep expertise in protein therapeutic design and development, 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.