Allena Pharmaceuticals Announces Positive Phase 2a Data of ALLN-177 for the Treatment of Hyperoxaluria

Initiating Two Phase 2b Studies in 2015

NEWTON, Mass., March 18, 2015 – 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 positive results from a Phase 2a clinical trial of ALLN-177, an orally administered recombinant oxalate degrading enzyme being developed for the chronic management of hyperoxaluria and kidney stones. The multicenter, open-label study demonstrated a statistically significant reduction of urinary oxalate excretion in recurrent calcium oxalate kidney stone patients with hyperoxaluria treated with ALLN-177 relative to baseline (P=0.0084). ALLN-177 was well tolerated and no serious adverse events were reported.

Hyperoxaluria is excessive urinary excretion of oxalate, a condition resulting from either hyperabsorption of dietary oxalate or the overproduction of oxalate in the body due to one of several known genetic defects. Hyperoxaluria, if left untreated, can lead to kidney stones, and in more severe cases, can progress to chronic kidney disease, end-stage renal disease and transplantation.

"We are very pleased with the results of this trial of ALLN-177, Allena's first study in patients with recurrent calcium oxalate kidney stones due to hyperoxaluria," said Alexey Margolin, Ph.D., co-founder, president and CEO of Allena Pharmaceuticals. "Based on these positive Phase 2a results, we are initiating two randomized, placebo-controlled studies in patients with enteric and idiopathic hyperoxaluria, which we expect to start later this year."

"Sustained high levels of hyperoxaluria can lead to serious related conditions, including kidney stones and chronic kidney disease, and with time to oxalate nephropathy and kidney failure," said David S. Goldfarb, M.D., professor of medicine & physiology at NYU Langone Medical Center and chief of nephrology at NY Harbor VA Medical Center. "Once a patient with hyperoxaluria has been diagnosed, intervention is required to prevent the development of these serious complications. Presently, there are no effective oxalate-reducing therapies available – significantly limiting therapeutic options for many patients in need. The results of this clinical trial are very encouraging, and ALLN-177 could represent a significant breakthrough for these patients if confirmed in further trials."

About the Study

The multicenter, open-label, single-arm outpatient Phase 2a study evaluated the safety and efficacy of ALLN-177 in recurrent calcium oxalate kidney stone patients with hyperoxaluria. Patients (n=16) were on self-selected diets (typically low oxalate) as well as high calcium and high liquid intake, as well as their standard medications (potassium citrate, thiazides, calcium supplements) throughout the study period. Key findings included:

- The mean reduction of urinary oxalate excretion (UOx)/24-hours between baseline and treatment periods was 13.9±18.4 mg. The overall reduction was highly statistically significant ($p = 0.0084$);
- Eleven of 16 subjects had a mean UOx reduction of >5 mg/24 hours on ALLN-177 (range: 5-60.8 mg; mean 20.8 mg);
- 30% of patients in this study exhibited extremely high urinary oxalate levels at baseline (>80 mg range: 87-231 mg UOx/24-hours). Typically these levels are consistent with patients who have known genetic defects (primary hyperoxaluria); and
- ALLN-177 was well tolerated and no significant adverse events were reported.

About Hyperoxaluria and Kidney Stones
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 (primary) due to a genetic defect. Hyperoxaluria can initially cause the development of kidney stones or can lead to kidney damage (nephrocalcinosis), chronic kidney disease, end-stage renal disease and dialysis. There are currently no effective pharmacologic treatments for hyperoxaluria.

The incidence of kidney stones has increased dramatically in the last 10 to 20 years, affecting one in 11 people in the U.S. in 2010, compared to one in 20 in 1994. An estimated four million people in the U.S. suffer from a kidney stone annually, and 25 percent of these are frequent or intermittent stone formers. In 2009, there were 1.3 million visits to an emergency department (ED) in the U.S. for kidney stones, accounting for approximately 1% of all ED visits. Approximately 20% of all ED visits for kidney stones resulted in hospitalization.

Kidney stones, while episodic, are also associated with a two-fold higher risk of chronic kidney disease and end-stage renal disease, as well as a higher risk of atherosclerosis and cardiovascular events. The prevalence of kidney stones is greater in patients with intestinal disease or fat malabsorption who are at higher risk for hyperoxaluria due to hyper-absorption of oxalate. This high-risk population includes those who have undergone bowel resection, bariatric or gastric surgery, and people with fat malabsorption due to conditions like Crohn's disease, cystic fibrosis, short bowel syndrome and liver disease.

About ALLN-177
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, reducing 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 kidney stones and 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 such as emergency room visits, hospital admissions, extractions and lithotripsy.

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's lead program, ALLN-177, is expected to enter a Phase 2b clinical trial in patients with hyperoxaluria in 2015. The company's proven 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 reducing 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, Mass., the company is backed by top-tier venture investors including Frazier Healthcare, Third Rock Ventures, HBM Partners, Bessemer Venture Partners and other investors. For more information, please visit www.allenapharma.com.

Company Contact:
Robert Gallotto
Chief Operating Officer
(617) 467-4577

Media Contact:
Katie Engleman
Pure Communications
(910) 509-3977

###