Allena Pharmaceuticals Announces Positive Phase 1 Data of ALLN-177 for the Treatment of Kidney Stones

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Company Rapidly Advancing ALLN-177 Into Phase 2 Study

NEWTON, Mass. – April 29, 2014 – 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 its Phase 1 clinical trial of ALLN-177, an orally administered recombinant oxalate degrading enzyme being developed for the chronic management of hyperoxaluria and kidney stones. The double-blind, placebo controlled crossover study demonstrated a statistically significant difference in the reduction of urinary oxalate levels in healthy subjects treated with ALLN-177 compared with placebo (P= 0.0002).

Hyperoxaluria is a condition resulting from high oxalate levels in the urine due to either hyper- absorption of oxalate from the diet or from overproduction of oxalate in the body due to one of several known genetic defects. Hyperoxaluria, regardless of cause, commonly leads to kidney stones, and may progress to chronic kidney disease, which if left untreated, results in end-stage renal disease and the need for dialysis.

“We are extremely encouraged by the results of this trial and by the overall progress of our development program,” said Alexey Margolin, Ph.D., co-founder, president and CEO of Allena Pharmaceuticals. “With no effective pharmacological treatments for hyperoxaluria available today, people who suffer from recurrent kidney stones could benefit tremendously from a safe and effective therapy that may treat or prevent hyperoxaluria and kidney stones. Based on the Phase 1 results, we are rapidly advancing ALLN-177 into a Phase 2 study in patients with kidney stones and hyperoxaluria.”

Clinical Study Summary

The Phase 1, double-blind, randomized, placebo controlled crossover study evaluated the safety and efficacy of ALLN-177 compared to placebo in 30 healthy volunteers who were placed on a high oxalate diet and showed a sustained increase in urinary oxalate levels, consistent with clinically meaningful hyperoxaluria.

For both the primary and secondary endpoints, ALLN-177 was shown to significantly and substantially reduce oxalate levels (mg/24 hours) compared with placebo (P= 0.0002). A pre- specified responder analysis was also performed comparing the number of responders for ALLN- 177 and placebo and showed that all responders were on ALLN-177 and none on placebo. Responders demonstrated mean reductions of oxalate excretion ranging from 8.3 mg/day to more than 40 mg/day. The onset of the effect of ALLN-177 was rapid and the effect was maintained during the duration of the trial as measured by a reduction in 24-hour urinary oxalate levels.

Importantly, ALLN-177 was well-tolerated and no safety signals were noted.

“Kidney stones are painful, debilitating and recurrent, resulting in significant social and financial burdens for patients,” said Craig B. Langman, M.D., professor of kidney diseases, Northwestern University. “Furthermore, the incidence of kidney stones has increased dramatically in the last 10 to 20 years. As the first well-controlled study demonstrating that a therapy can significantly reduce urinary oxalate levels, these positive data represent a significant milestone for patients suffering from this ailment.”

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 or from overproduction of oxalate by the liver. 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.

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. In 2009, there were 1.3 million visits to an emergency department (ED) in the U.S. for kidney stones, accounting for approximately 1 percent of all ED visits. Approximately 20 percent 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 malabsorption of nutrients.

Primary hyperoxaluria (PH) is a rare inherited autosomal genetic disorder affecting the liver, which leads to markedly elevated levels of oxalate in plasma and urine. Hyperoxaluria causes kidney stone formation and accumulation of oxalate in tissues, as well as calcification of the kidney, ultimately leading to kidney failure and premature death. PH affects approximately 5,000 patients worldwide.

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 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 2 clinical trial in patients with hyperoxaluria in 2014. 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 Bessemer Venture Partners, Frazier Healthcare and Third Rock Ventures. For more information, please visit www.allenapharma.com.

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