Allena Pharmaceuticals Announces Agreement with Duke Clinical Research Institute for URIROX-2 Pivotal Phase 3 Trial

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Duke to Establish and Lead an Academic Coordinating Center for Phase 3 URIROX-2 Trial of Reloxaliase in Patients with Enteric Hyperoxaluria

NEWTON, Mass., March 27, 2019 (GLOBE NEWSWIRE) -- Allena Pharmaceuticals, Inc. (NASDAQ:ALNA), a late-stage, biopharmaceutical company dedicated to developing and commercializing first-in-class, oral enzyme therapeutics to treat patients with rare and severe metabolic and kidney disorders, today announced an agreement with the Duke Clinical Research Institute (DCRI), a leading academic research institute within Duke University School of Medicine, to support the URIROX-2 Phase 3 clinical trial of reloxaliase in enteric hyperoxaluria.

DCRI will establish and lead an Academic Coordinating Center (ACC) for Allena's URIROX-2, one of two ongoing pivotal Phase 3 clinical trials evaluating the safety and efficacy of reloxaliase as a novel therapy for patients with enteric hyperoxaluria. The DCRI's Dr. Charles Scales, Dr. Myles Wolf, and Dr. Christina Wyatt will direct this new ACC. Dr. Scales, Associate Professor of Surgery (Urology) and Population Health Sciences, is a recognized leader in the epidemiology and care of patients with kidney stone disease. Dr. Wolf, the Charles Johnson M.D. Professor of Medicine and Chief of Nephrology, is a renowned expert in disordered mineral metabolism across the spectrum of patients with chronic kidney disease. Dr. Wyatt, Associate Professor of Medicine (Nephrology), is an established expert in the epidemiology and management of kidney disease in unique patient populations.

The DCRI ACC will establish an Academic Steering Committee (ASC) to provide independent oversight and support investigator engagement for URIROX-2. The ACC will also contribute scientific expertise and thought leadership to the data analysis and publication strategy, including health economics outcomes research (HEOR), in support of the development of reloxaliase as a novel therapeutic candidate for patients with enteric hyperoxaluria. Dr. Scales will serve as the chair of the ASC.

"We are pleased to collaborate with the DCRI, a renowned research organization that is uniquely positioned to help advance URIROX-2, the second pivotal Phase 3 study we are conducting for patients with enteric hyperoxaluria," said Louis Brenner, M.D., President and Chief Executive Officer of Allena Pharmaceuticals. "Alongside our work with patient advocacy groups, we expect that this partnership with the DCRI will increase the awareness of enteric hyperoxaluria in the medical community, enhance identification of patients for our clinical program, and begin laying the foundation to support the potential future launch of reloxaliase. We look forward to working closely with DCRI as we seek to develop the first therapeutic for patients with this serious disorder."

"Allena's URIROX-2 trial will be the largest and most rigorous trial conducted to date to evaluate kidney stone disease progression and kidney function in patients with enteric hyperoxaluria," said Dr. Scales. "This trial will provide a rich source of data to evaluate the clinical benefit of reloxaliase and the health economic impact of reducing oxalate burden in this high-risk population. We look forward to coordinating with investigators, patient advocacy groups, industry partners, academic researchers and the Allena team on this new ACC."

Pivotal Phase 3 URIROX Program

Allena's URIROX program consists of two pivotal Phase 3 trials, URIROX-1 and URIROX-2, which are designed to evaluate the safety and efficacy of reloxaliase in patients with enteric hyperoxaluria. Allena plans to pursue a Biologics License Application (BLA) submission for reloxaliase using the accelerated approval regulatory pathway.

URIROX-1 is a multicenter, global, randomized, double-blind, placebo-controlled study evaluating the safety and efficacy of reloxaliase in an expected 124 patients for a four-week treatment period. It has the same primary and key secondary efficacy endpoints as URIROX-2. Based on enrollment progress to date, Allena expects to report topline data from URIROX-1 in the second half of 2019.

URIROX-2 is a multicenter, global, randomized, double-blind, placebo-controlled study designed to evaluate the safety and efficacy of reloxaliase in patients with enteric hyperoxaluria, over a minimum treatment period of two years. The trial will enroll 400 patients with 24-hour urinary oxalate (UOx) excretion greater than or equal to 50 mg and a history of kidney stones, and will include patients with normal kidney function as well as with chronic kidney disease. The primary efficacy endpoint for URIROX-2 is the percent change from baseline in 24-hour UOx excretion during Weeks 1-4, comparing mean reduction in the average UOx excretion across Weeks 1-4 with reloxaliase to placebo. The primary long-term efficacy endpoint is the proportion of subjects with kidney stone disease progression. Secondary long-term efficacy endpoints include change in kidney function, and emergency room visits, hospitalizations or, procedures for the management of kidney stones. Allena initiated the study in the fourth quarter of 2018.

About Hyperoxaluria

Hyperoxaluria is a metabolic disorder characterized by elevated urinary oxalate levels that may be due to either overproduction of oxalate by the liver from a genetic defect, known as primary hyperoxaluria, or from the excess absorption of oxalate from the diet, known as secondary hyperoxaluria. Secondary hyperoxaluria is further characterized either as enteric, resulting from a chronic and unremediable underlying GI disorder, or idiopathic, meaning the underlying cause is unknown. Kidney stones, 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.

Enteric hyperoxaluria is the more severe subset of secondary hyperoxaluria. Allena estimates that there are approximately 200,000 to 250,000 patients with enteric hyperoxaluria and kidney stones in the United States.

About Reloxaliase

Reloxaliase is an orally-administered, recombinant oxalate-degrading enzyme that is being developed for the treatment of severe hyperoxaluria. Reloxaliase targets oxalate in the GI tract in an effort to reduce the burden of both dietary and endogenously-produced oxalate. Reloxaliase 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. Reloxaliase is being evaluated in the ongoing pivotal Phase 3 URIROX-1 and URIROX-2 trials for patients with enteric hyperoxaluria. In addition, reloxaliase has been granted separate orphan drug designations by the U.S. Food and Drug Administration for the treatment of primary hyperoxaluria and for the treatment of pediatric hyperoxaluria. In addition, the European Commission has granted orphan drug designation for reloxaliase for the treatment of primary hyperoxaluria.

About Allena Pharmaceuticals

Allena Pharmaceuticals, Inc. is a late-stage biopharmaceutical company dedicated to developing and commercializing first-in-class, oral enzyme therapeutics to treat patients with rare and severe metabolic and kidney disorders. Allena's lead product candidate, reloxaliase, is a first-in-class, oral enzyme therapeutic for the treatment of hyperoxaluria, a metabolic disorder characterized by markedly elevated urinary oxalate levels and commonly associated with kidney stones, chronic kidney disease and other serious kidney disorders.

Forward-Looking Statements

This release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding Allena's URIROX clinical program and alignment with the FDA, statements regarding the timing of announcement of topline data from the URIROX-1 trial, statements regarding the design of the URIROX-2 trial, including the number of patients to be enrolled in the URIROX-2 trial, statements regarding Allena's ability to utilize the accelerated approval regulatory pathway for reloxaliase, and statements regarding the ability of reloxaliase to provide clinical benefit to patients with hyperoxaluria. Any forward- looking statements in this press release are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: the risk that results of earlier studies may not be predictive of future clinical trial results, and planned and ongoing studies may not establish an adequate safety or efficacy profile for reloxaliase to support regulatory approval or the use of the accelerated approval regulatory pathway; risks related to Allena's ability to utilize the accelerated approval pathway for reloxaliase, including the risk that available data at the time of any sample size re-estimation or interim analysis conducted during the URIROX-2 trial may not be sufficient to demonstrate an increased probability of kidney stone events in patients with enteric hyperoxaluria and increasing UOx levels; the risk that the FDA may require that Allena increase the sample size or duration of treatment following the sample size reassessments to be conducted in accordance with the adaptive design element of the trial or otherwise collect additional clinical data from the URIROX-2 or other clinical trials prior to submitting a BLA for reloxaliase; risks associated with Allena's ability to enroll a sufficient number of patients to adequately power URIROX-2 in order to achieve ultimate statistical success for kidney stone disease progression in the long-term follow-up phase of the trial; risks associated with obtaining, maintaining and protecting intellectual property; risks associated with Allena's ability to enforce its patents against infringers and defend its patent portfolio against challenges from third parties; the risk of competition from other companies developing products for similar uses; risk associated with Allena's ability to manage operating expenses and/or obtain additional funding to support its business activities; and risks associated with Allena's dependence on third parties. For a discussion of other risks and uncertainties, and other important factors, any of which could cause Allena's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in Item 1A of Part II of Allena's Annual Report on Form 10-K for the year ended December 31, 2018, as well as discussions of potential risks, uncertainties and other important factors in Allena's subsequent filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Allena undertakes no duty to update this information unless required by law.

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