



Allena Pharmaceuticals Showcases Reloxaliase and Enteric Hyperoxaluria Program at OHF International Hyperoxaluria Workshop

June 21, 2019

NEWTON, Mass., June 21, 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 that it will present on its reloxaliase development program and the unmet need in patients with enteric hyperoxaluria (EH) in four sessions at the Oxalosis & Hyperoxaluria Foundation (OHF) International Hyperoxaluria Workshop, to be held June 21-22, 2019 in Boston, MA. The OHF International Hyperoxaluria Workshop is the largest gathering of hyperoxaluria patients, disease advocates and thought leaders worldwide.

"Allena's strong presence at the OHF International Hyperoxaluria Workshop highlights our progress in developing reloxaliase as a potential first-in-class therapy for patients suffering from enteric hyperoxaluria, as well as our ongoing efforts to work with advocacy groups and treating physicians to better characterize the progression of this debilitating disease," said Louis Brenner, M.D., President and Chief Executive Officer of Allena Pharmaceuticals. "We are encouraged by the growing awareness of severe oxalate disorders and the increasingly active community focused on treating patients with enteric hyperoxaluria. Our network of investigators and patient advocates has supported us throughout the development of reloxaliase. We look forward to sharing data from our pivotal URIROX-1 Phase 3 clinical trial in the second half of the year and to laying the foundation in support of the future launch of reloxaliase."

The four presentations are summarized below:

In the session on enteric hyperoxaluria, Allena's Chief Medical Officer, Dr. Annamaria Kausz, will present data on the burden of disease and the significant unmet need in this under-recognized patient population. In addition to recurrent kidney stones, EH patients can also suffer from progressive loss of kidney function and end stage renal disease. A recent review on clinical outcomes in EH patients with oxalate nephropathy found that renal replacement therapy was required in > 50% of patients.¹ Additionally, there is increasing awareness of the association between excess urinary oxalate excretion and the risk of chronic kidney disease progression.²

In the session on the industry pipeline, Allena's President and Chief Executive Officer, Dr. Louis Brenner, will present an overview of the development program for reloxaliase, including the ongoing URIROX Phase 3 program and Study 206. 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 EH. These trials represent the largest studies conducted to-date in patients with EH.

In the poster session, a case series of the first four subjects with EH and advanced CKD treated with reloxaliase in Study 206 will be presented. Study 206 is a multi-center, open-label, single-arm Phase 2 basket study, designed to evaluate reloxaliase in adult and pediatric patients suffering from the progression of enteric hyperoxaluria with advanced chronic kidney disease (CKD) or primary hyperoxaluria (PH), both of which can lead to systemic oxalosis.

In the family and patient advocacy panel, Allena will join other biotechnology companies developing potential therapies that target the common enemy of excess oxalate, which can lead to severe disease in high risk patient populations.

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. 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. The European Commission has granted orphan drug designation for reloxaliase for the treatment of primary hyperoxaluria.

About 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.

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. Patients will be randomized 1:1 to reloxaliase vs. placebo and will take 284 mg (equivalent to 7,500 units) of reloxaliase or placebo with each meal or snack up to five times per day, consistent with the eating patterns of patients with enteric hyperoxaluria. 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 is designed to enroll 400 patients with 24-hour urine 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 chronic kidney disease.

The primary efficacy endpoint of URIROX-2 is the percent change from baseline in 24-hour UOx excretion during Weeks 1-4, comparing reduction in the average UOx excretion across Weeks 1-4 with reloxaliase to placebo, the same primary endpoint as URIROX-1. Secondary endpoints in

URIROX-2 include the proportion of subjects with a $\geq 20\%$ reduction from baseline in 24-hour UOx excretion during Weeks 1-4 and percent change from baseline in 24-hour UOx excretion during Weeks 16 to 24. The primary long-term efficacy endpoint to confirm clinical benefit is the proportion of subjects with kidney stone disease progression, defined as a composite of either symptomatic kidney stones or finding of new or enlarged kidney stones using imaging, over a minimum treatment period of two years. Secondary long-term efficacy endpoints to confirm clinical benefit include change in eGFR from baseline and emergency room visits, hospitalizations or procedures for the management of kidney stones.

In January 2019, Allena announced that it reached alignment with the U.S. Food and Drug Administration (FDA) on both the design of URIROX-2 and its strategy to pursue a Biologics License Application (BLA) submission for reloxaliase in patients with enteric hyperoxaluria using the accelerated approval regulatory pathway.

In March 2019, Allena announced an agreement with the Duke Clinical Research Institute (DCRI), a leading academic research institute within Duke University School of Medicine, to establish and lead an Academic Coordinating Center (ACC) in support of the URIROX-2 Phase 3 clinical trial and preparation for the potential launch of reloxaliase.

About Study 206

Study 206 is a multi-center, open-label, single-arm Phase 2 basket study, designed to evaluate reloxaliase in adult and pediatric patients suffering from the progression of EH with advanced CKD or PH, both of which can lead to systemic oxalosis. The clinical trial is designed to enroll between 15 and 20 patients aged 12 and older. Patients orally administer 7,500 units of reloxaliase with each meal or snack five times a day, for 12 consecutive weeks. The primary endpoints of the trial are change from baseline in 24-hour UOx excretion and POx levels.

This basket study is seeking to identify an efficacy signal in several high risk patient populations including enteric hyperoxaluria patients with CKD, kidney transplantation or dialysis dependence or patients with primary hyperoxaluria types 1, 2 or 3. UOx is being collected for patients who are not on dialysis. Data has been released on the first seven patients from the study. This includes the first four EH patients who demonstrated plasma oxalate (POx) reductions of 28% and 16%, compared to baseline, in the two patients not on dialysis, and 49% and 45% in the two patients on dialysis. In addition, for the two patients not on dialysis, 24 hour UOx excretion was reduced by 29% and 42%. The summary data showed a range of POx reduction from 16% to 49% with an average reduction of 35%, compared to baseline. This summary POx data corrects an inadvertent error in a press release issued on June 4th, 2019. The individual patient data that was also publicly released on June 4th, 2019 is correct and unchanged. Three patients with PH type 2 and PH type 3 with preserved renal function were treated. These are the first patients with any form of PH treated with reloxaliase. One patient had a $> 20\%$ mean reduction in UOx excretion while the other two patients did not show a response to reloxaliase.

Based on the treatment effect observed in these four EH patients, coupled with the unmet need and high risk of morbidity and mortality in EH patients with advanced CKD, Allena intends to focus further enrollment in Study 206 on patients with EH and CKD, kidney transplantation or dialysis dependence and patients with PH and compromised renal function. Allena expects to announce topline results from the trial in the second half of 2019.

1. Lumlertgul et al, *Kidney Int Rep* 2018; 3:1363-1372.
2. Waikar SS et al, *JAMA Intern Med* 2019; 179(4):542-551.

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, without limitation, statements regarding the results of Study 206 and the clinical and commercial potential of reloxaliase for patients with primary hyperoxaluria or enteric 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 interim results or 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 in URIROX-2 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 related to Allena's use of UOx and/or POx as surrogate endpoints in its ongoing clinical trials, neither of which it believes have been previously utilized as biomarkers to support regulatory approval of other drug candidates, and the risks related to validating that reductions in UOx and/or POx correlate with meaningful clinical benefit; 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2019, 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.

Investor Contact

Hannah Deresiewicz
Stern Investor Relations, Inc.
212-362-1200

hannah.deresiewicz@sternir.com

Media Contact

Adam Daley

Berry & Company Public Relations

212-253-8881

adaley@berrypr.com



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