



Eiger BioPharmaceuticals Announces First Patient Dosed in Phase 2 LIBERTY Study of Ubenimex in Pulmonary Arterial Hypertension

Novel first-in-class inhibitor of LTB₄ targeting disease modification

PALO ALTO, Calif., July 18, 2016 /PRNewswire/ -- Eiger BioPharmaceuticals, Inc. (Nasdaq:EIGR), focused on the development and commercialization of targeted therapies for rare diseases, announced today that the first patient was dosed in the Phase 2 LIBERTY study. The LIBERTY study will evaluate the effects of ubenimex added to current standard of care in patients with pulmonary arterial hypertension (PAH). Despite multiple approved vasodilator therapies, PAH remains a progressive, life-threatening cardiovascular disease. Ubenimex is a well-characterized, oral, small-molecule inhibitor of leukotriene A₄ hydrolase, which blocks the production of leukotriene B₄ (LTB₄), an inflammatory mediator implicated in PAH disease.



"The LIBERTY study represents a transformative, clinical translational effort with potential to demonstrate, for the first time, disease modification in PAH," said Roham Zamanian, MD, Lead Investigator and Director of the Adult Pulmonary Hypertension Program at Stanford University School of Medicine. "While vasoactive agents have utility in the clinical management of the symptoms of PAH, they do not address the underlying inflammation which is an important signature of this cardiovascular disease. We have arrived at a moment of shift of therapeutic paradigm, where we may have a chance to realize a potentially disease modifying approach."

"The goal of the LIBERTY study is to block LTB₄ production with ubenimex as a novel and potentially disease modifying treatment for PAH," said Joanne Quan, MD, Chief Medical Officer at Eiger BioPharmaceuticals. "Inflammation, now recognized as an important component of PAH, is not addressed by currently available therapies. Recently published preclinical results of studies conducted at Stanford University suggest that elevated LTB₄ levels may play a role in the inflammatory component of PAH, which can lead to obstructed arterioles, vasoconstriction, and worsening cardiac function. Targeted LTB₄ blockade may represent an important new therapeutic approach to this disease."

About the LIBERTY Phase 2 Study

LIBERTY is a multi-center, randomized, double-blind, placebo-controlled Phase 2 study of ubenimex in patients with pulmonary arterial hypertension. Approximately forty-five patients will be randomized in a 2:1 ratio to receive ubenimex or matching placebo, administered orally for a total of 24 weeks. Patients who complete treatment through Week 24 will be eligible to enroll in an open-label extension study to receive continued treatment. This open-label extension will allow all patients the option to receive ubenimex for at least an additional 24 weeks and provide additional data on safety, tolerability and efficacy.

About LTB₄ and Ubenimex

LTB₄ is a naturally-occurring inflammatory mediator known to be elevated in both animal models of PAH as well as human PAH disease. In animal models, elevated LTB₄ causes inflammation resulting in arteriole occlusion, vasoconstriction and hypertension. Targeted pharmacologic inhibition of LTB₄ reversed PAH disease in treated animals as demonstrated by decreased obstruction of arterioles, improved cardiac function, and improved survival.

Ubenimex is a well-characterized, oral, small-molecule, inhibitor of LTA₄H, the enzyme responsible for the formation of the pro-inflammatory mediator, LTB₄.

Ubenimex is approved in Japan (brand name Bestatin™) as an adjunct to chemotherapy agents to extend survival and to maintain remission after treatment for acute non-lymphocytic leukemia in adults. Ubenimex has been used for over 25 years in Japan and remains commercially available through Nippon Kayaku. Ubenimex has been granted Orphan Drug Designation for treatment of PAH by the US FDA and European Medicines Agency (EMA). Ubenimex is not approved for any indication in the US or Europe.

About PAH

Pulmonary arterial hypertension (PAH) is a type of high blood pressure that affects the arteries in the lungs and the right side of

the heart. PAH begins when tiny arteries in the lungs, called pulmonary arterioles, become narrowed, blocked or destroyed. This makes it harder for blood to flow through the lungs, and raises pressure within the lungs' arteries. As the pressure builds, the heart's lower right chamber (right ventricle) must work harder to pump blood through the lungs, eventually causing the heart muscle to weaken and eventually fail. PAH is a progressive, life-threatening illness.

About Eiger

Eiger is a clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare diseases. The company has built a diverse portfolio of well-characterized product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and for which an effective therapy is urgently needed.

Note Regarding Forward-Looking Statements

This press release contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this press release regarding our strategy, future operations, future financial position, future revenue, projected expenses, prospects, plans and objectives, intentions, beliefs and expectations of management are forward-looking statements. These forward-looking statements may be accompanied by such words as "anticipate," "believe," "could," "estimate," "expect," "forecast," "intend," "may," "plan," "potential," "project," "target," "will" and other words and terms of similar meaning. Examples of such statements include, but are not limited to, whether or not pegylated interferon lambda-1a or lonafarnib or ubenimex or exendin (9-39) may be further developed and approved, statements relating to the availability of cash for Eiger's future operations, Eiger's ability to develop its drug candidates for potential commercialization, the timing of the commencement and number and completion of Phase 2 trials and whether the products can be successfully developed or commercialized. Various important factors could cause actual results or events to differ materially from the forward-looking statements that Eiger makes, including the risks described in the "Risk Factors" sections in the Annual Report on Form 10-K for the period ended December 31, 2015 and Eiger's periodic reports filed with the SEC. Eiger does not assume any obligation to update any forward-looking statements, except as required by law.

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