Eiger Announces First Patient Dosed in Open-Label Extension of Phase 2 LIBERTY Study of Ubenimex in Pulmonary Arterial Hypertension (PAH)

First-in-class inhibitor of LTB4 targeting disease modification in PAH

PALO ALTO, Calif., Jan. 3, 2017 /PRNewswire/ -- Eiger BioPharmaceuticals, Inc. (Nasdaq:EIGR), focused on the development and commercialization of therapies for rare diseases, today announced that the first patient has completed 24 weeks dosing in the double-blind Phase 2 LIBERTY study and has now received treatment with ubenimex in the open-label extension. LIBERTY is a randomized, double-blind, placebo-controlled study of ubenimex administered orally for a total of 24 weeks in patients with pulmonary arterial hypertension (PAH). After completing the blinded treatment period, each patient may be eligible to enroll in an open-label extension to receive ubenimex for at least 24 weeks. Ubenimex is a well-characterized, oral, small-molecule inhibitor of leukotriene A_4 hydrolase (LTA_4H), which blocks the production of leukotriene B_4 (LTB_4), an inflammatory mediator implicated in the pathogenesis of PAH.

"The LIBERTY study represents a potentially transformative, clinical translational effort with the goal 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 multiple approved 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."

"There has been tremendous enthusiasm in the entire community – patients, families, investigators, and study staff at pulmonary hypertension centers – for the LIBERTY study and a new approach to treating PAH. The extension study shows our commitment to the community by offering an option to receive open-label ubenimex for at least 24 weeks after completing the double-blind study," said Joanne Quan, MD, Chief Medical Officer at Eiger. "We look forward to completing enrollment in LIBERTY in mid-2017."

About the LIBERTY Phase 2 Study
LIBERTY is a multi-center, randomized, double-blind, placebo-controlled Phase 2 study of ubenimex in patients with PAH. 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 may 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 24 weeks and provide additional data on safety, tolerability and efficacy.

About LTB_4 and Ubenimex
LTB_4 is a naturally-occurring inflammatory mediator shown to be elevated in both animal models of PAH as well as human PAH disease. 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.

Ubenimex is a well-characterized, oral, small-molecule, inhibitor of LTA_4H, the enzyme responsible for the formation of the pro-inflammatory mediator, LTB_4.

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 identified, and for which an effective therapy is urgently needed. For more information, please visit the Company's website at www.eigerbio.com.

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 potential clinical development outcomes, impact on disease and ability to successfully achieve approval for our clinical candidate ubenimex, 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, including subcutaneous (SC) formulation, may be further developed and approved, whether Phase 2 studies of exendin 9-39 will show safety and activity consistent with early clinical results, including the interim results of the multiple-ascending dose (MAD) study, or that the SC formulation will be consistent with results seen with intravenous (IV) exendin 9-39, 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 our periodic reports filed with the Securities and Exchange Commission. Eiger assumes no obligation to update any forward-looking statements, except as required by law.

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