Eiger Announces FDA Breakthrough Therapy Designation for Avexitide for Treatment of Congenital Hyperinsulinism

- All Five Orphan Programs Now Have Breakthrough Therapy Designation

Palo Alto, Calif., August 5, 2021 /PRNewswire/ -- Eiger BioPharmaceuticals, Inc. (Nasdaq: EIGR), a commercial-stage biopharmaceutical company focused on the development and commercialization of targeted therapies for serious rare and ultra-rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for avexitide for the treatment of congenital hyperinsulism (HI).

Breakthrough Therapy Designation is a process designed to expedite the development and review of drugs that are intended to treat a serious condition and where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint(s). Eiger's application was supported by data from three completed Phase 2 studies in 39 neonates, children and adolescents with congenital hyperinsulinism. Avexitide is a targeted, first-in-class, GLP-1 antagonist in development for the treatment of metabolic disorders, including congential hyperinsulinism, an ultra-rare, life-threatening, pediatric disorder of persistent hypoglycemia that results in irreversible brain damage in up to 50% of children.

"Avexitide represents a promising, targeted approach for the treatment of congenital hyperinsulinism, an urgent, unmet medical need with no approved therapy," said Colleen Craig, MD, Vice President of Metabolic Diseases at Eiger. "We look forward to continued collaboration with the FDA."

About Avexitide

Avexitide is a 31-amino acid peptide that selectively targets and blocks GLP-1 receptors, reducing dysregulated insulin secretion by the pancreas, and thereby reducing fasting and postprandial hypoglycemia. Proof of concept has been demonstrated in 39 patients across three Phase 2 studies in neonates, children and adolescents with congenital hyperinsulinism. Avexitide has been granted Breakthrough Therapy Designation by the FDA for the treatment of congenital hyperinsulism, Orphan Drug Designation by the FDA for the treatment of hyperinsulinemic hypoglycemia (which includes congenital hyperinsulinism), Orphan Drug Designation by the EMA for the treatment of congenital hyperinsulinism and Rare Pediatric Disease Designation by the FDA.

Avexitide is also in development for post-bariatric hypoglycemia (PBH). Four clinical studies have been completed in 54 patients with PBH. Eiger has received concurrence from FDA and EMA on a single Phase 3 study for patients with PBH. Avexitide has been granted Breakthrough Therapy Designation by the FDA, as well as Orphan Drug Designation by the FDA for the treatment of hyperinsulinemic hypoglycemia and Orphan Drug Designation by the EMA for the treatment of non-insulinoma pancreatogenous hypoglycemia syndrome (NIPHS). Both of these broad orphan designations include PBH.

Avexitide is not approved or commercialized for any indication.

About Congenital Hyperinsulinism (HI)
Congenital hyperinsulinism (HI) is a rare, genetic, pediatric metabolic disorder characterized by severe fasting and protein-induced hypoglycemia due to dysregulated secretion of insulin by the pancreas. Repeat episodes and/or dangerously low blood sugars increase the risk of neurological and developmental complications, including persistent feeding problems, learning disabilities, recurrent seizures, brain damage, or even death. Existing medical options are often ineffective or are associated with substantial side effects that discourage compliance and lead to suboptimal treatment outcomes. Subtotal pancreatectomy is an option, but this approach can be associated with life-threatening complications, does not immediately fully resolve hypoglycemia in most patients, and ultimately leads to the development of lifelong insulin-dependent diabetes.

About Eiger
Eiger is a commercial-stage biopharmaceutical company focused on the development and commercialization of targeted therapies for serious rare and ultra-rare diseases.

Eiger's lead clinical programs are focused on the development of foundational therapies for Hepatitis Delta Virus (HDV) infection, the most serious form of viral hepatitis. Eiger’s HDV platform strategy includes two complementary HDV treatments. Lonafarnib is a first-in-class, oral prenylation inhibitor in a global Phase 3 trial. Peginterferon lambda is a first-in-class, type III, well-tolerated interferon entering Phase 3.

Zokinvy® for the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and processing-deficient progeroid laminopathies is the Company's first FDA approved product. A Marketing Authorization Application (MAA) is under review by the European Medicines Agency (EMA).

For additional information about Eiger and its clinical programs, please visit www.eigerbio.com

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