Eiger Announces Publication of Positive Phase 2 Results of Avexitide in Children with Congenital Hyperinsulinism and Initiation of Phase 3 Program

- Phase 2 study demonstrating avexitide significantly reduced hypoglycemia in children with congenital hyperinsulinism published in *Diabetes Care*
- Eiger plans to initiate avexitide Phase 3 registrational program by year-end 2022

Palo Alto, Calif., April 25, 2022 -- Eiger BioPharmaceuticals, Inc. (Nasdaq: EIGR), a commercial-stage biopharmaceutical company focused on the development of innovative therapies to treat and cure hepatitis delta virus (HDV) and other serious diseases, today announced the publication of data demonstrating that treatment with avexitide significantly reduced the likelihood of fasting and protein-induced hypoglycemia in patients with congenital hyperinsulinism (HI).

The investigator sponsored study conducted at Children's Hospital of Philadelphia (CHOP) provides further evidence of avexitide's targeted therapeutic approach to treat HI and supports advancing avexitide into Phase 3 development.

"CHOP has been a strong supporter and development partner for avexitide, and we are pleased to see the publication of these positive results," said David Cory, President and CEO, Eiger. "Their early investigation of avexitide in congenital hyperinsulinism generated important foundational proof of concept data in this patient population. Armed with these data, we are moving forward into Phase 3 this year as we believe avexitide has the potential to transform the lives of children with this life-threatening condition."

Eiger has obtained alignment with FDA on the avexitide HI registration program. Avexitide has been granted orphan drug designation in the U.S. by FDA for the treatment of hyperinsulinemic hypoglycemia (which includes HI) and has also been granted rare pediatric disease designation making it eligible for a priority review voucher upon regulatory approval. Avexitide is the only investigational therapy for HI granted breakthrough therapy designation by FDA.

"The impact of this disease on patients and families can be devastating, with newborns often requiring intensive care hospitalization, surgery, and years of intensive management," said senior study author Diva D. De León-Crutchlow, MD, Chief of the Division of Endocrinology and Diabetes and Director of the Congenital Hyperinsulinism Center at Children's Hospital of Philadelphia. "With no approved treatments for HI currently available, new, effective therapies are urgently needed. Eiger's initiation of the avexitide Phase 3 program is therefore an important step for this community."

Summary of Phase 2 Study Results[1]

This open-label, crossover study (N=16) was designed to test the effects of avexitide (referred to as exendin (9-39) in the study) compared to a saline control on fasting- and protein-induced hypoglycemia in children with HI. Compared to control, avexitide treatment resulted in a 76% reduction in the likelihood of fasting hypoglycemia in the mid-dose (0.44 mg/kg) group and an 84% reduction in the likelihood of hypoglycemia in the high dose (0.6 mg/kg) group. Avexitide treatment (0.6 mg/kg) during protein tolerance testing resulted in an 82% reduction in the likelihood of hypoglycemia. The mid-dose group also demonstrated a 20% increase in fasting glucose, while the high dose resulted in a 28% increase in glucose after a meal and a 30% increase in glucose after protein challenge.

About Congenital Hyperinsulinism (HI)

Congenital hyperinsulinism (HI), a rare genetic disease, is the most common cause of persistent hypoglycemia (low blood sugar) in infants and children. The estimated incidence of between 1 in 2,500 and 1 in 50,000 live births[2-3] results in approximately 80-100 new cases of HI in the U.S. each year, 60% of which are diagnosed within the first month of life. It is characterized by inappropriate and persistent insulin secretion from the beta-cells of the pancreas, and results in permanent brain damage with neurodevelopmental deficits in up to 50% of patients. Near-total pancreatectomy is often indicated and leads to life-long insulin-dependent diabetes (IDDM). There are no approved therapies for HI; safe and effective therapies are urgently needed to prevent brain damage, IDDM, and death.

About Avexitide

Avexitide is an investigational, first-in-class glucagon-like peptide-1 receptor (GLP-1r) antagonist in development for the treatment of congenital hyperinsulinism (HI) and post-bariatric hypoglycemia (PBH). Avexitide has been granted breakthrough therapy designation for both HI and PBH.

By binding to the GLP-1r, avexitide inhibits GLP-1r signaling, thereby reducing dysregulated insulin secretion and preventing fasting- and protein-induced hypoglycemia. By addressing underlying disease mechanisms, avexitide may offer the first targeted approach to treatment of hypoglycemia in patients with HI. Eiger has developed a novel formulation of avexitide for subcutaneous injection for its Phase 3 registrational program.

About Eiger

Eiger is a commercial-stage biopharmaceutical company focused on the development of innovative therapies to treat and cure hepatitis delta virus (HDV) and other serious diseases. The Eiger HDV platform includes two first-in-class therapies in Phase 3 that target critical host processes involved in viral replication. Eiger is also developing peginterferon lambda as a therapeutic for COVID-19 and is planning to submit an emergency use authorization application to FDA based on positive results from the investigator sponsored Phase 3 TOGETHER study.

All five Eiger rare disease programs have been granted FDA breakthrough therapy designation: lonafarnib and peginterferon lambda for HDV, Zokinvy for progeria, and avexitide for both HI and PBH.

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

FORWARD-LOOKING STATEMENTS

This press release contains forward-looking statements within the meaning of the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. All statements other than statements of historical facts, including statements regarding our future financial condition, timing for and outcomes of clinical results, regulatory objectives, business strategy and plans and objectives for future operations, are forward-looking statements. Forward-looking statements are our current statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our anticipated significant milestones in 2022; the timing of our ongoing and planned clinical development; the sufficiency of our cash, cash equivalents and investments to fund our operations; our ability to obtain an Emergency Use Authorization from the FDA for peginterferon lambda for COVID-19; our capability to provide sufficient quantities of any of our product candidates, including peginterferon lambda, to meet anticipated full-scale commercial demands; our ability to finance the continued advancement of our development pipeline products; and the potential for success of any of our product candidates. Various important factors could cause actual results or events to differ materially from the forward-looking statements that Eiger makes, including additional applicable risks and uncertainties described in the "Risk Factors" sections in the Annual Report on Form 10-K for the year ended December 31, 2021 and Eiger's subsequent filings with the SEC. The forward-looking statements contained in this press release are based on information currently available to Eiger and speak only as of the date on which they are made. Eiger does not undertake and specifically disclaims any obligation to update any forward-looking statements, whether as a result of any new information, future events, changed circumstances or otherwise.

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1. Stefanovski D, Vajravelu M, Givler S, De León D. *Diabetes Care*, 2022; DOI: 10.2337/dc21-2009; 2. Bruining GJ. *Curr Opin Pediatr, 1990;* 2:758–765; 3. Otonkoski T et al. *Diabetes, 1999;* 48:408–415.