Eiger BioPharmaceuticals Announces U.S. Commercial Availability of Zokinvy™ (lonafarnib), the First and Only Treatment Approved for Progeria and Processing-Deficient Progeroid Laminopathies

- The Eiger OneCare™ Program Provides Ongoing Patient Support Services

Palo Alto, Calif., January 25, 2021 /PRNewswire/ -- Eiger BioPharmaceuticals, Inc (Nasdaq:EIGR), a commercial-stage biopharmaceutical company focused on the development and commercialization of foundational therapies for Hepatitis Delta Virus (HDV) infection, today announced the commercial launch and availability of Zokinvy™ (lonafarnib) in the United States. Zokinvy was approved by the U.S. Food and Drug Administration (FDA) in November 2020 to reduce the risk of death in patients with Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and for the treatment of processing-deficient progeroid laminopathies.

“Eiger sought regulatory approval of Zokinvy to ensure continued access to the only drug proven to reduce the risk of death in patients with Progeria,” said Eldon Mayer, Chief Commercial Officer of Eiger. “To fulfill our commitment to patients, we established Eiger OneCare™. This comprehensive program includes personalized support by specialized care managers, reimbursement experts, co-pay assistance for eligible patients and other patient support services designed to assist patients seeking access to Zokinvy.”

Most of the identified Zokinvy-eligible patients in the U.S. have received Zokinvy through Eiger’s global expanded access program and are now enrolled in Eiger OneCare which will facilitate a smooth transition to Zokinvy commercial supply.

Eiger OneCare will be available Monday through Friday from 9 AM to 5 PM Eastern Time at 1-833-MYEIGER (1-833-693-4437).

For more information, visit www.Zokinvy.com.

For healthcare professionals with questions about Zokinvy, call Eiger’s Medical Information department at 833-267-0545.

About Zokinvy (lonafarnib)

Zokinvy blocks the accumulation of defective, farnesylated proteins which form tight associations with the nuclear envelope, leading to cellular instability and the process of premature aging in children and young adults with Progeria and processing-deficient Progeroid Laminopathies.

Eiger licensed exclusive worldwide rights to lonafarnib from Merck, known as MSD outside of the United States and Canada. Merck will not receive any milestone payments for the development of lonafarnib for the treatment of Progeria and has waived royalty obligations from Eiger for a specified quantity of lonafarnib.

About Progeria and Progeroid Laminopathies

Progeria, also known as Hutchinson-Gilford Progeria Syndrome (HGPS), and Progeroid Laminopathies are separate and distinct ultra-rare, fatal, genetic premature aging diseases that accelerate mortality in young patients.

Progeria is caused by a point mutation in the *LMNA* gene, yielding the farnesylated aberrant protein, progerin. Progeroid Laminopathies are genetic conditions of accelerated aging caused by a constellation of mutations in the *LMNA* and/or Zmpste24 genes yielding farnesylated proteins that are distinct from progerin. While non-progerin producing, these genetic mutations result in disease manifestations with phenotypes that have overlap with, but are distinct from, Progeria.

Without Zokinvy therapy, children with Progeria commonly die of the same heart disease that affects millions of normally aging adults (arteriosclerosis), by an average age of 14.5 years. Disease manifestations include severe failure to thrive, scleroderma-like skin, global lipodystrophy, alopecia, joint contractures, skeletal dysplasia, global accelerated atherosclerosis with cardiovascular decline, and debilitating strokes. It is estimated that there are 400 children worldwide with Progeria and 200 children with Progeroid Laminopathies. Of these patients, approximately 180 children and young adults have been identified by The Progeria Research Foundation, including approximately 20 in the U.S. and 23 in Europe.

INDICATION

ZOKINVY is indicated in adult and pediatric patients 12 months of age and older with a body surface area (BSA) of 0.39 m^2 and above:

- To reduce the risk of mortality in Hutchinson-Gilford Progeria Syndrome (HGPS)
- For the treatment of processing-deficient Progeroid Laminopathies with either:
    - Heterozygous LMNA mutation with progerin-like protein accumulation
    - Homozygous or compound heterozygous ZMPSTE24 mutations

Limitations of Use

ZOKINVY is not indicated for use in patients with non-HGPS Progeroid Syndromes or with Progeroid Laminopathies known to be processing-proficient. Based upon its mechanism of action, ZOKINVY would not be expected to be effective in these populations.

Contraindications

- Strong or moderate CYP3A inhibitors or inducers

- Midazolam
- Lovastatin, simvastatin, and atorvastatin

IMPORTANT SAFETY INFORMATION

- The most common adverse reactions are vomiting (90%), diarrhea (81%), infection (78%), nausea (56%), decreased appetite (53%), fatigue (51%), upper respiratory tract infection (51%), abdominal pain (48%), musculoskeletal pain (48%), electrolyte abnormalities (43%), headache (37%), decreased weight (37%), increased aspartate aminotransferase (35%), myelosuppression (35%), cough (33%), decreased blood bicarbonate (33%), hypertension (29%), and increased alanine aminotransferase (27%).

Gastrointestinal Adverse Reactions

- Gastrointestinal adverse reactions were the most frequently reported adverse reactions. Of the 57 patients (90%) that experienced vomiting, 30 (53%) patients had mild vomiting, 26 (46%) patients had moderate vomiting, and 1 (2%) patient had severe vomiting.
- Of the 35 patients (56%) that experienced nausea, 34 (97%) patients had mild nausea and 1 (3%) patient had moderate nausea.
- Of the 51 patients (81%) that experienced diarrhea, the majority of patients (92%) experienced mild or moderate diarrhea; 38 (75%) patients reported mild diarrhea and 9 (18%) patients reported moderate diarrhea. Four (8%) patients reported severe diarrhea.
- Loss of fluids and dehydration can be severe, leading to hospitalization. As a result, patients should receive therapy for diarrhea at the earliest signs in order to avoid possible severe complications.

Alanine Aminotransferase and Aspartate Aminotransferase Elevations

- Increased alanine aminotransferase was commonly reported (17 [27%] patients). Of the 17 patients with increased alanine aminotransferase, 14 (82%) patients had mild increases, 1 (6%) patient had moderate increases, and 2 (12%) patients had severe increases.
- Increased aspartate aminotransferase was also commonly reported (22 [35%] patients). Of the 22 patients with increased aspartate aminotransferase, 21 (95%) patients had mild increases and 1 (5%) patient had a severe increase.

Hypertension

- Increases in blood pressure have been documented in patients treated with ZOKINVY. At baseline 22 (35%) patients had either a systolic blood pressure or a diastolic blood pressure or both above the 95th percentile. Over the course of

the trials, 18 (29%) patients had hypertension based on systolic blood pressure or diastolic blood pressure measurements above the 95th percentile on 3 or more occasions. Five (8%) patients who were normotensive at baseline had either systolic blood pressure or diastolic blood pressure above the 95th percentile at the end of treatment.

Ophthalmic Adverse Reactions

- Lonafarnib caused retinal toxicity in monkeys at 3.7 times the human dose based on plasma drug exposure, but not at 2.1 times the human dose.

Laboratory Abnormalities

Some patients treated with ZOKINVY developed laboratory abnormalities. These included:

- Electrolyte abnormalities (43%), such as hyperkalemia, hypokalemia, hyponatremia, or hypercalcemia
- Myelosuppression (35%), such as reductions in absolute neutrophil count, white blood cell counts, lymphopenia, hemoglobin, or hematocrit
- Increased liver enzymes, such as aspartate aminotransferase (35%), or alanine aminotransferase (27%)

These laboratory abnormalities often improved while continuing ZOKINVY, but it is not possible to exclude ZOKINVY as a cause of the abnormalities. Periodically monitor electrolytes, complete blood counts, and liver enzymes, and manage abnormalities accordingly.

Nephrotoxicity

- Lonafarnib caused nephrotoxicity in rats at plasma drug exposures approximately equal to that achieved with the human dose. Monitor renal function at regular intervals during ZOKINVY therapy.

Retinal Toxicity

- Lonafarnib caused rod-dependent, low-light vision decline in monkeys at plasma drug exposures similar to that achieved with the human dose. Perform ophthalmological evaluation at regular intervals and at the onset of any new visual changes during ZOKINVY therapy.

Impaired Fertility

- Lonafarnib caused impaired fertility in female rats at 1.2 times the human dose based on plasma drug exposure.
- Lonafarnib caused impaired fertility and testicular toxicity in male rats at 1.5 times the human dose based on plasma drug exposure, and toxicity in the male

reproductive tract in monkeys at doses lower than the human dose based on plasma drug exposure.

About Eiger

Eiger is a commercial-stage biopharmaceutical company focused on the development and commercialization of foundational therapies for Hepatitis Delta Virus (HDV) infection, the most serious form of human viral hepatitis.

Eiger is developing two complementary treatments for HDV. Lonafarnib is a first-in-class, oral prenylation inhibitor in a global Phase 3 trial. Peginterferon lambda is a first-in-class, well-tolerated type III 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 approval. A Marketing Authorization Application (MAA) is under review by the European Medicines Agency (EMA). Outside the U.S., Eiger's established global Managed Access Program, expected to span greater than 40 countries, ensures all children and young adults with Progeria and Progeroid Laminopathies have access to treatment.

For additional information about Eiger and its clinical programs, please visit 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, including statements regarding our future financial condition, timing for and outcomes of clinical results, business strategy and plans and objectives for future operations, are forward-looking statements. These forward-looking statements include terminology such as "believe," "will," "may," "estimate," "continue," "anticipate," "contemplate," "intend," "target," "project," "should," "plan," "expect," "predict," "could," "potentially" or the negative of these terms. Forward-looking statements are our current statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our anticipating significant milestones in 2021, the timing of our ongoing and planned clinical development, including our ability to support the launch of a new product and ship to specialty pharmacies; the sufficiency of our cash, cash equivalents and investments to fund our operations through at least Q4 2023; the expected closing of the sale of our PRV; our development programs for Zokinvy generally; and the potential approval of Zokinvy in jurisdictions outside of the U.S., including the EU; the risks related to the commercialization of Zokinvy, our ability to manufacture sufficient quantities of Zokinvy, and the commercial launch of Zokinvy in

the U.S., the market potential for Zokinvy as a treatment for Progeria and processing-deficient Progeroid Laminopathies; our progression and enrollment of our Phase 3 D-LIVR study in HDV; our ability to maintain supply of our commercial and clinical trial materials; our plans to advance Lambda in HDV in the U.S. and EU; our ability to transition into a commercial stage biopharmaceutical company; our ability to finance the continued advancement of our development pipeline products; and the potential for success of any of our product candidates. These statements concern product candidates that have not yet been approved for marketing by the U.S. Food and Drug Administration (FDA). No representation is made as to their safety or effectiveness for the purposes for which they are being investigated. 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2020 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.

SOURCE Eiger BioPharmaceuticals, Inc.

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