Eiger and Partner, AnGes, Receive Approval for Zokinvy® (lonafarnib) for Hutchinson- Gilford Progeria Syndrome and Processing-Deficient Progeroid Laminopathies in Japan

- Clinical trial data demonstrated Zokinvy treatment extended life by an average of 4.3 years in children and young adults with Hutchinson-Gilford progeria
- Eiger to receive $500,000 approval milestone payment from AnGes
- Zokinvy approved in the U.S. (2020), 30 European countries (2022), and now Japan (2024)

PALO ALTO, Calif., Jan. 18, 2024 /PRNewswire/ -- Eiger BioPharmaceuticals, Inc. (Nasdaq:EIGR), a commercial-stage biopharmaceutical company focused on the development of innovative therapies for rare metabolic diseases, today announced that it and its partner AnGes, Inc. received marketing approval from the Ministry of Health, Labour and Welfare for Zokinvy (lonafarnib), a treatment for Hutchinson-Gilford progeria syndrome (HGPS) and processing deficient progeroid laminopathy (PDPL).

"We and our partner, AnGes, are pleased that Zokinvy is now approved in Japan for patients living with progeria, an ultra-rare and fatal pediatric disease that can result in premature death," said David Apelian, MD, PhD, MBA, CEO of Eiger. "We would like to thank the Progeria Research Foundation for their continued support of the regulatory submission as well as the patients and their families."

Collectively known as progeria, HGPS and PL are devastating ultra-rare and fatal pediatric diseases that cause dramatically accelerated aging and premature death. The main cause of death is heart attack or stroke due to severe hardening of the arteries.[3,4]

The approval was based on the positive results of two pivotal clinical trials demonstrating that Zokinvy, an oral disease-modifying agent which targets the cause of progeria, lowered the risk of death in children by 72% and extended life by an average of 4.3 years ($p<0.0001$) in children and young adults with HGPS.[2]

About Progeria
Collectively known as progeria, Hutchinson-Gilford progeria syndrome and progeroid laminopathies are ultra-rare, fatal, genetic premature aging diseases that accelerate mortality in young patients.

HGPS 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.[4,5]

Without Zokinvy therapy, children with HGPS 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.[3]

About Zokinvy® (lonafarnib)
Zokinvy is a first-in-class disease-modifying agent that blocks the accumulation of defective progerin and progerin-like proteins which leads to cellular instability and premature aging in children and young adults with progeria. Zokinvy has demonstrated a statistically significant survival benefit in children and young adults with HGPS.[1,4]

The most commonly reported adverse reactions were gastrointestinal (vomiting, diarrhea, nausea), and most were mild or moderate (Grade 1 or 2) in severity. Many progeria patients have received continuous Zokinvy therapy for more than 10 years.[1,2]

Zokinvy is FDA approved for the treatment of patients 12 months of age and older with a genetically confirmed diagnosis of Hutchinson-Gilford progeria syndrome or a processing-deficient progeroid laminopathy associated with either a heterozygous LMNA mutation with progerin-like protein accumulation or a homozygous or compound heterozygous ZMPSTE24 mutation.

For Important Safety Information and prescribing information for Zokinvy in the U.S., please visit www.zokinvy.com

Eiger and AnGes entered into an exclusive distribution agreement for the treatment of HGPS and PDPL indications, Zokinvy (Lonafarnib), in Japan on May 10, 2022. In March 2023, the Ministry of Health, Labour and Welfare designated Zokinvy as an

orphan drug.

About Eiger
Eiger is a commercial-stage biopharmaceutical company focused on the development of innovative therapies for rare metabolic diseases. Eiger's lead product candidate, avexitide, is a well characterized, first-in-class GLP-1 antagonist for the treatment of post-bariatric hypoglycemia (PBH) and congenital hyperinsulinism (HI). Avexitide is the only drug in development for PBH with Breakthrough Therapy designation from the FDA.

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 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, prospective products, preclinical and clinical pipelines, 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, the timing of our ongoing and planned clinical development; our capability to provide sufficient quantities of any of our products or product candidates for studies or to meet anticipated full-scale commercial demands; our ability to identify, pursue and enter into partnering opportunities for our virology assets; the sufficiency of our cash, cash equivalents and investments to fund our operations into the fourth quarter of 2024, including the scope and impact of any savings from our workforce reduction and cash conservation efforts; the revenue potential of avexitide in post-bariatric hypoglycemia and congenital hyperinsulinism; our ability to finance, independently or through collaborations, the continued advancement of our development pipeline; and the potential for success of any of our products or 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" section in Eiger's Quarterly Report on Form 10-Q for the quarter ended September 30, 2023 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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