Eiger Announces FDA Guidance on HDV Phase 3 Study Design: Primary Endpoint Established; D-LIVR Study Initiating Q4 2018

D-LIVR Study: First-Ever Registration Study for Chronic HDV Infection

PALO ALTO, Calif., Sept. 24, 2018 /PRNewswire/ -- Eiger BioPharmaceuticals, Inc. (Nasdaq:EIGR), focused on the development and commercialization of targeted therapies for rare diseases, today announced the receipt of written guidance from the FDA, confirming concurrence on a pivotal trial design, including the primary endpoint for D-LIVR, the first-ever, registration study for Hepatitis Delta Virus (HDV) infection.

A combined primary endpoint of ≥ 2 $\log_{10}$ decline in HDV RNA and normalization of alanine aminotransferase (ALT) at the end of 48 weeks of treatment has been accepted by the FDA as the primary endpoint and would be supportive of an accelerated approval of two lonafarnib-based, ritonavir-boosted regimens. An all-oral arm of lonafarnib boosted with ritonavir and a combination arm of lonafarnib boosted with ritonavir combined with pegylated interferon-alfa will each be compared to placebo in the D-LIVR study. Accelerated approval could be based on successful achievement of this surrogate endpoint in this single pivotal study in addition to a post-marketing confirmatory trial to evaluate clinical benefit.

"We are very pleased with FDA acceptance of a combined endpoint of ≥ 2 $\log_{10}$ decline in HDV RNA and ALT normalization for the Phase 3 D-LIVR study and believe it serves as an excellent primary efficacy endpoint to support the accelerated approval of lonafarnib-based regimens for HDV," said David Apelian, MD, PhD, MBA, Chief Operating Officer and Executive Medical Officer. "This endpoint reflects an improvement in liver condition and virologic response rarely observed in untreated HDV patients, and we have previously demonstrated achievement of this combined endpoint in a substantial number of HDV patients with lonafarnib-based regimens in our Phase 2 program."

"HDV prevalence estimates in the U.S. and Western Europe now exceed a combined 300,000 infected, and 15-20 million in the rest of the world, with no approved treatment, making this a very large commercial opportunity," said David Cory, President and Chief Executive Officer. "The Phase 3 D-LIVR protocol is now finalized based on these recent discussions with FDA and will include two separate lonafarnib-based regimens, each to be compared to placebo, in a single pivotal trial. Eiger is about to begin the first-ever, registration trial with the potential to bring two separate, approved treatment options to HDV patients."

About D-LIVR Study

D-LIVR (**D**elta **L**iver **I**mprovement and **V**irologic **R**esponse in HDV) is an international, multi-center, Phase 3 study of approximately 300 lonafarnib (LNF)-treated patients (total N=400 patients including controls) to evaluate an all-oral arm of LNF boosted with ritonavir (RTV) and a combination arm of LNF boosted with RTV combined with pegylated interferon-alfa (PEG IFN-alfa), with each arm to be compared to a placebo arm (background HBV nucleos(t)ide only), in HDV-infected patients. A PEG IFN-alfa alone arm will be dosed to demonstrate contribution of effect only. The LNF containing arms will not be required to demonstrate superiority over PEG IFN-alfa alone. A combined primary endpoint of ≥ 2 $\log_{10}$ decline in HDV RNA and ALT normalization at end of 48 weeks of treatment will be used to assess activity of LNF based regimens versus placebo in the D-LIVR study.

About Lonafarnib

Lonafarnib is a well-characterized, late-stage, orally active inhibitor of farnesyl transferase, an enzyme involved in modification of proteins through a process called prenylation. HDV uses this host cell process inside liver cells to complete a key step in its life cycle. Lonafarnib inhibits the prenylation step of HDV replication inside liver cells and blocks the virus life cycle at the stage of assembly. Lonafarnib has been dosed in over 120 HDV-infected patients across international academic centers and is moving into Phase 3 development for HDV with a single, pivotal trial planned to initiate by the end of the year. Lonafarnib has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA), and Fast Track Designation by U.S. FDA. Lonafarnib is not approved for any indication, and is licensed from Merck Sharp & Dohme Corp. (known as MSD outside of the United States and Canada).

About Hepatitis Delta Virus (HDV)

Hepatitis Delta is caused by infection with HDV and is considered to be one of the most severe forms of viral hepatitis in humans. Hepatitis delta occurs only as a co-infection in individuals harboring Hepatitis B Virus (HBV). Hepatitis delta leads to more severe liver disease than HBV alone and is associated with accelerated liver fibrosis, liver cancer, and liver failure. Hepatitis delta is a disease with a significant impact on global health, which may affect up to approximately 15-20 million people worldwide. The

prevalence of HDV varies among different parts of the world. Globally, HDV infection is reported to be present in approximately 4.3% to 5.7% of chronic Hepatitis B carriers. The prevalence of HDV in patients infected with chronic HBV is even higher in certain regions, including certain parts of Mongolia, China, Russia, Central Asia, Pakistan, Turkey, Africa, Middle East and South America, with an HDV prevalence as high as 60% being reported in HBV-infected patients in Mongolia and Pakistan.

About Eiger

Eiger is a clinical-stage biopharmaceutical company focused on the accelerated development and commercialization of targeted therapies for rare and ultra-rare diseases. We innovate by developing well characterized drugs acting on newly identified or novel targets in rare diseases. Our mission is to systematically reduce the time and cost of the drug development process to more rapidly deliver important medicines to patients with rare diseases. Lonafarnib is our lead compound advancing into Phase 3 with a single, pivotal trial to treat HDV planned to initiate by the end of the year. Lonafarnib is also advancing toward an NDA for the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) in 2019. 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 ongoing and planned clinical development, including whether the D-LIVR study will be successful as a single, pivotal study to support registration; the timing of and our ability to initiate or enroll clinical trials, including whether our D-LIVR study can be advanced by the end of this year; the timing for completion and potential filing for registration for our clinical candidates; whether PREVENT Phase 2 study results will support further development of avexitide; our ability to make timely regulatory filings and obtain and maintain regulatory approvals for lonafarnib as a single agent or in combination, ubenimex, PEG IFN lambda, avexitide and our other product candidates; our intellectual property position; and the potential safety, efficacy, reimbursement, convenience clinical and pharmaco-economic benefits of our product candidates as well as the commercial opportunities, including potential market sizes and segments; our ability to finance the continued advancement of our development pipeline products, including our results of operations, cash available, financial condition, liquidity, prospects, growth and strategies; 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 the risks described in the "Risk Factors" sections in the Quarterly Report on Form 10-Q for the quarter ended June 30, 2018 and Eiger's periodic reports filed with the SEC. Eiger does not assume any obligation to update any forward-looking statements, except as required by law.

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