Eiger Announces Breakthrough Therapy Designation Granted by FDA for Lonafarnib for Treatment of Hepatitis Delta Virus (HDV) Infection

- Phase 3 HDV "D-LIVR" International Study Initiating

PALO ALTO, Calif., Dec. 17, 2018 /PRNewswire/ -- Eiger BioPharmaceuticals, Inc. (Nasdaq:EIGR), focused on the development and commercialization of targeted therapies for rare and ultra-rare diseases, today announced that the Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for lonafarnib for the treatment of hepatitis delta virus (HDV) infection. FDA Breakthrough Therapy designation involves a Fast Track development and FDA review process with guidance designed to expedite the development and review of medicines intended to treat serious or life-threatening diseases. HDV is the most severe form of human viral hepatitis and has no approved treatment. Lonafarnib is a first-in-class prenylation inhibitor for the treatment of HDV infection.

"There is an urgent medical need to treat HDV, the most serious form of viral hepatitis," said David Apelian, MD, PhD, MBA, Chief Operating Officer and Executive Medical Officer. "We look forward to collaborating with the FDA as we accelerate development of lonafarnib for HDV-infection. The Phase 3 D-LIVR Study is the first-ever, global registration trial in HDV, with the potential to bring two separate, lonafarnib-based treatment regimens to HDV patients."

This Breakthrough Therapy designation is supported by data from Phase 2 clinical studies of lonafarnib-based treatment regimens in HDV-infected patients, achieving combined primary endpoints of ≥ 2 $\log_{10}$ decline in HDV RNA and normalization of alanine aminotransferase (ALT) which reflect an improvement in liver condition and virologic response rarely observed in untreated HDV patients.

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 in Phase 3 development for HDV with a single, pivotal trial. Lonafarnib has been granted Orphan Drug designation by the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA), and Fast Track and now Breakthrough esignation 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 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 Eiger
Eiger is a late-stage biopharmaceutical company focused on the accelerated development and commercialization of a pipeline of

targeted, first-in-class therapies for rare and ultra-rare diseases. The company's lead program is in Phase 3, developing lonafarnib, a first-in-class prenylation inhibitor for the treatment of Hepatitis Delta Virus (HDV) infection. The company is also preparing an NDA with plans to file in 2019 for lonafarnib in the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and Progeroid Laminopathies. For additional information about Eiger, 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 timing expectations and whether larger studies will support the earlier study results identified, including whether the D-LIVR Phase 3 study as a single, pivotal study will be initiated by the end of 2018; whether the D-LIVR Phase 3 study results, if successful, will be sufficient to support registration; the timing of and our ability to initiate or enroll clinical trials, including whether our D-LIVR study can be initiated by the end of this year; our ability to complete and achieve successful clinical study results with any or all of our product candidates in order make timely regulatory filings and obtain and maintain regulatory approvals based on our expected timelines, including lonafarnib; our ability to move lonafarnib into potentially pivotal clinical studies and file an NDA for a separate progeria indication in a successful and timely manner; 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 September 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.

Investors:
Ingrid Choong, PhD
Email: ichoong@eigerbio.com
Phone: 1-650-619-6115

View original content to download multimedia:http://www.prnewswire.com/news-releases/eiger-announces-breakthrough-therapy-designation-granted-by-fda-for-lonafarnib-for-treatment-of-hepatitis-delta-virus-hdv-infection-300767216.html

SOURCE Eiger BioPharmaceuticals, Inc.