Intercept Announces Phase 3 REVERSE Trial Evaluating OCA for the Treatment of NASH Patients with Compensated Cirrhosis

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- *Results from REVERSE are intended to serve as the basis for seeking marketing approval of OCA for the treatment of NASH patients with compensated cirrhosis*
- *REGENERATE and REVERSE pivotal trials expand Intercept's leadership's position in NASH*

NEW YORK, Feb. 12, 2018 (GLOBE NEWSWIRE) -- Intercept Pharmaceuticals, Inc. (Nasdaq:ICPT) (Intercept), a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, today announced the REVERSE trial (Randomized Phase 3 Study Evaluating the Efficacy and Safety of Obeticholic Acid in Subjects with Compensated Cirrhosis Due to Nonalcoholic Steatohepatitis). REVERSE is a randomized, double-blind, placebo-controlled, multi-center study that will evaluate the efficacy and safety of the investigational therapy obeticholic acid (OCA) in approximately 540 patients with a biopsy-confirmed diagnosis of cirrhosis due to NASH.

"NASH is poised to soon eclipse hepatitis C as the leading reason for liver transplants in the U.S. and Europe, so there is an urgent need for effective therapies that can reverse fibrosis and cirrhosis," said Mark Pruzanski, M.D., President and CEO of Intercept. "OCA is currently the only FDA-designated Breakthrough Therapy in development for NASH and, with our Phase 3 trials REGENERATE and REVERSE underway, we are on track to bring the first approved therapy to NASH patients with fibrosis and cirrhosis who are at greatest risk of liver failure."

The REVERSE trial will be conducted at sites in North America, Europe, Australia and New Zealand. The primary endpoint is the percentage of subjects with histological improvement in fibrosis by at least one stage using the NASH Clinical Research Network (CRN) scoring system after 12 months of treatment. Patients are being randomized in a 1:1:1 ratio to one of the three treatment arms: once-daily dosing of OCA 10 mg, once-daily OCA 10 mg with titration to 25 mg at three months, or placebo. Patients who successfully complete the double-blind phase of REVERSE will be eligible to enroll in an open-label extension phase for up to 12 additional months.

Results from the double-blind phase of the study are intended to serve as the basis for seeking initial U.S. and international marketing authorizations of OCA for the treatment of NASH patients with compensated cirrhosis. Consistent with regulatory requirements, a subsequent outcomes trial will be planned with the goal of confirming clinical benefit on a post-marketing basis in a broader population of NASH patients with cirrhosis.

About NASH with Cirrhosis
NASH is a serious progressive non-viral liver disease caused by excessive fat accumulation in the liver that induces chronic inflammation, resulting in progressive fibrosis (scarring) that can lead to cirrhosis, eventual liver failure, cancer and death. There are currently no medications approved for the treatment of NASH. The proportion of liver transplants attributable to NASH has increased rapidly in past years and by 2020 the disease is projected to become the leading indication for liver transplant.[1]

Approximately 25% to 50% of patients with NASH develop fibrosis rapidly (over a period of 4 years to 6 years).[2] Of these patients, approximately 25% will develop cirrhosis in approximately 8 years, eventually resulting in decompensated liver disease associated with complications of ascites, variceal bleeding and hepatic encephalopathy.[2] Liver failure is the main cause of morbidity and mortality in patients with NASH-associated cirrhosis.[3] It has been estimated that 40% to 60% of NASH patients with cirrhosis will develop liver failure after 5 to 7 years, with one-third of these patients either dying or requiring liver transplantation.[4]

About Intercept
Intercept is a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, including primary biliary cholangitis (PBC), nonalcoholic steatohepatitis (NASH), primary sclerosing cholangitis (PSC) and biliary atresia. Founded in 2002 in New York, Intercept now has operations in the United States, Europe and Canada. For more information about Intercept, please visit www.interceptpharma.com or connect with the company on Twitter and LinkedIn.

Safe Harbor Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements the potential utility of the results from REVERSE and REGENERATE, the prevalence of NASH, the rate at which NASH leads to liver transplant, a subsequent outcomes trial, the potential of OCA to treat patients with NASH, and our strategic directives under the caption "About Intercept." These "forward-looking statements" are based on management's current expectations of future events and are subject to a number of important risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: the potential benefit and commercial potential of OCA in NASH, the potential benefit and commercial potential of Ocaliva in PBC, and Intercept's ability to maintain its regulatory approval in jurisdictions in which Ocaliva is approved for use in PBC; the initiation, cost, timing, progress and results of Intercept's development activities, preclinical studies and clinical trials; the timing of and Intercept's ability to obtain and maintain regulatory approval of OCA in PBC in countries outside the ones in which it is approved and in indications other than PBC and any other product candidates it may develop; conditions that may be imposed by regulatory authorities on Intercept's marketing approvals for its products and product candidates such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), and any related restrictions, limitations, and/or warnings in the label of any approved products and product candidates; Intercept's plans to research, develop and commercialize its product candidates; Intercept's ability to obtain and maintain intellectual property protection for its products and product candidates; Intercept's ability to successfully commercialize its products and product candidates; the size and growth of the markets for Intercept's products and product candidates and its ability to serve those markets; the rate and degree of market acceptance of any of Intercept's products, which may be affected by the reimbursement received from payors; the success of competing drugs that are or become available; regulatory developments in the United States and other countries; the performance of third-party suppliers and manufacturers; the election by Intercept's collaborators to pursue research, development and commercialization activities; Intercept's ability to attract collaborators with development, regulatory and commercialization expertise; Intercept's need for and ability to obtain additional financing; Intercept's estimates regarding expenses, revenues and capital requirements and the accuracy thereof; Intercept's use of cash and short-term investments; Intercept's ability to attract and retain key scientific or management personnel; and other factors discussed under the heading "Risk Factors" contained in our annual report on Form 10-K for the year ended December 31, 2016 filed on March 1, 2017 as well as any updates to these risk factors filed from time to time in our other filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Intercept undertakes no duty to update this information unless required by law.

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References

1. Wong RJ, Aguilar M, Cheung R, et al. Nonalcoholic Steatohepatitis is the Second Leading Etiology of Liver Disease Among Adults Awaiting Liver Transplantation in the U.S. Gastroenterology. 2014 Nov 25.
2. Loomba R and Sanyal AJ. The global NAFLD epidemic. Nat Rev Gastroenterol Hepatol. 2013 Nov;10(11):686-90.
3. Hui JM, Kench JG, Chitturi S, et al. Long-term outcomes of cirrhosis in nonalcoholic steatohepatitis compared with hepatitis C. Hepatology. 2003 Aug;38(2):420-7.
4. Ong JP, Younossi ZM. Epidemiology and natural history of NAFLD and NASH. Clinics in liver disease. 2007 Feb;11(1):1-16, vii.

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