New REGENERATE Interim Analysis Data Presented at The Liver Meeting® Report OCA Improved Multiple Noninvasive Markers of Liver Fibrosis in Patients with NASH

November 8, 2019

Treatment with OCA resulted in early and sustained improvements of commercially available noninvasive biomarker and imaging assessments of fibrosis

Additional patient-reported outcomes data demonstrate that pruritus in patients treated with OCA 25 mg did not have an impact on measures of quality of life

NEW YORK, Nov. 08, 2019 (GLOBE NEWSWIRE) -- Intercept Pharmaceuticals, Inc. (Nasdaq:ICPT), a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, today announced additional supportive data from the first reported analyses of the Phase 3 REGENERATE study examining the effects of obeticholic acid (OCA) on noninvasive assessments of liver fibrosis and patient-reported outcomes (PROs). The new data will be presented at The Liver Meeting® 2019, the Annual Meeting of the American Association for the Study of Liver Diseases (AASLD), in Boston, Massachusetts from November 8, 2019 through November 12, 2019.

Patients in the REGENERATE primary intent-to-treat (ITT) population (n=931) with stage 2 and 3 fibrosis were randomized 1:1:1 to receive OCA 25 mg (n=308), OCA 10 mg (n=312) or placebo (n=311) once daily over 18 months. As previously reported, in the primary efficacy analysis, once-daily OCA 25 mg met the primary endpoint of fibrosis improvement (≥1 stage) with no worsening of NASH in 23.1% of patients compared to 11.9% of placebo patients at the planned 18-month interim analysis (p=0.0002 vs. placebo).

In a new analysis of the interim data presented at The Liver Meeting, OCA-treated patients in the primary ITT group showed time- and dose-dependent improvements compared to placebo across commercially available noninvasive tests, including blood tests of fibrosis (Fibrosis-4 [FIB-4] index, AST to platelet ratio index [APRI], and FibroSURE®) as early as three months after treatment initiation. In addition, vibration-controlled transient elastography [VCTE], an imaging assessment of liver stiffness and a surrogate of fibrosis, decreased from baseline in both OCA groups but increased with placebo at 18 months.

In a responder analysis, improvements in noninvasive tests mirrored shifts in fibrosis stage, with the greatest improvements observed in patients achieving >1 fibrosis stage reduction. In contrast to patients treated with placebo, OCA-treated patients with no change in fibrosis stage also had marked improvement in noninvasive tests. The authors concluded that the improvements observed in histologic non-responders suggest OCA's therapeutic benefit may not be adequately captured by categorical histologic fibrosis staging at 18 months.

"These results are important because they provide insight into OCA's ability to improve noninvasive measures of fibrosis that may be used to evaluate patients in clinical practice," said Rohit Loomba, M.D., MHSc, Director, NAFLD Research Center, and Professor of Medicine, University of California at San Diego. "Given the invasive and expensive nature of liver biopsy, it is encouraging to see the NASH field moving so rapidly to embrace noninvasive tests and transient elastography. Positive data from robust, well-controlled Phase 3 studies like REGENERATE are expected to further accelerate validation and adoption of noninvasive tests."

Additional REGENERATE Data at The Liver Meeting 2019: Patient-Reported Outcomes

Two new analyses being presented at The Liver Meeting focused on PROs in REGENERATE's expanded ITT population, which consisted of the primary ITT population (n=931), plus an exploratory cohort of 287 NASH patients with stage 1 liver fibrosis and additional risk factors who were at increased risk of progression to cirrhosis (n=1,218). PROs related to pruritus were assessed using the chronic liver disease questionnaire for NASH (CLDQ-NASH), Work Productivity and Activity Impairment (WPAI) and EQ-5D tools. At the start of study treatment, 21% of patients reported significant pruritus (CLDQ Itch score≤4) at baseline, which negatively impacted all PRO domains (all p≤0.01). Following randomization, PROs were measured every six months through the 18-month interim analysis. Despite a dose-dependent increase in pruritus in the OCA 10 mg and 25 mg arms, pruritus with OCA 25 mg did not have a detectable negative impact on PROs.

"These initial PRO data from REGENERATE are surprising in two ways," said Zobair M. Younossi, M.D., MPH, Professor and Chairman of the Department of Medicine at Inova Fairfax Medical Campus and the Chair of the REGENERATE Steering Committee. "First, the finding that PRO scores for patients with NASH are below population norms suggests NASH is not an asymptomatic disease and that effective treatment of NASH can potentially improve PROs. Second, we have learned that pruritus reported in patients receiving the OCA 25 mg dose does not seem to have a major impact on patient experience. The peak severity of pruritus was observed early in the treatment course without subsequent worsening, and pruritus seemed to be relatively mild overall without detectable negative impact on PROs."

About Liver Fibrosis due to NASH

Nonalcoholic steatohepatitis (NASH) is a serious progressive 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. Advanced fibrosis is associated with a substantially higher risk of liver-related morbidity and mortality in patients with NASH, and as early as 2020, the disease is projected to become the leading cause of liver transplants in the United States. There are currently no medications approved for the treatment of NASH.

About the REGENERATE Study

REGENERATE is a Phase 3, randomized, double-blind, placebo-controlled, multicenter study assessing the safety and efficacy of obeticholic acid (OCA) on clinical outcomes in patients with liver fibrosis due to NASH. A pre-specified 18-month analysis was conducted to assess the effect of OCA on liver histology comparing month 18 biopsies with baseline. REGENERATE has completed target enrollment for the clinical outcomes cohort, with more than 2,400 adult NASH patients randomized across 339 qualified centers worldwide, and will continue through clinical outcomes for verification

and description of clinical benefit. The end-of-study analysis will evaluate the effect of OCA on all-cause mortality and liver-related clinical outcomes, as well as its long-term safety.

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) and nonalcoholic steatohepatitis (NASH). Founded in 2002 in New York, Intercept has operations in the United States, Europe and Canada. For more information, please visit www.interceptpharma.com or connect with the company on Twitter and LinkedIn.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements, including, but not limited to, statements regarding the progress, timing and results of our clinical trials, including our clinical trials for the treatment of nonalcoholic steatohepatitis ("NASH"), the safety and efficacy of our approved product, Ocaliva (obeticholic acid or "OCA") for primary biliary cholangitis ("PBC"), and our product development candidates, including OCA for NASH, the timing and acceptance of our potential regulatory filings and potential approval of OCA for NASH or any other indications in addition to PBC, the timing and potential commercial success of OCA and any other product candidates we may develop and our strategy, future operations, future financial position, future revenue, projected costs, financial guidance, prospects, plans, objectives of management and expected market growth.

These statements constitute forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "possible," "continue" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release, and we undertake no obligation to update any forward-looking statement except as required by law. These forward-looking statements are based on estimates and assumptions by our management that, although believed to be reasonable, are inherently uncertain and subject to a number of risks. The following represent some, but not necessarily all, of the factors that could cause actual results to differ materially from historical results or those anticipated or predicted by our forward-looking statements: our ability to successfully commercialize Ocaliva for PBC; our ability to maintain our regulatory approval of Ocaliva for PBC in the United States, Europe, Canada, Israel, Australia and other jurisdictions in which we have or may receive marketing authorization; the initiation, timing, cost, conduct, progress and results of our research and development activities, preclinical studies and clinical trials, including any issues, delays or failures in identifying patients, enrolling patients, treating patients, retaining patients, meeting specific endpoints in the jurisdictions in which we intend to seek approval or completing and timely reporting the results of our NASH or PBC clinical trials; our ability to timely and cost-effectively file for and obtain regulatory approval of our product candidates, including OCA for NASH, in the United States, Europe and our other target markets; conditions that may be imposed by regulatory authorities on our marketing approvals for our 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 contained in the label of any of our products or product candidates; any potential side effects associated with Ocaliva for PBC, OCA for NASH or our other product candidates that could delay or prevent approval, require that an approved product be taken off the market, require the inclusion of safety warnings or precautions, or otherwise limit the sale of such product or product candidate; our ability to establish and maintain relationships with, and the performance of, third-party manufacturers, contract research organizations and other vendors upon whom we are substantially dependent for, among other things, the manufacture and supply of our products, including Ocaliva for PBC and, if approved, OCA for NASH, and our clinical trial activities; our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to timely and successfully launch OCA for NASH, if approved; our ability to obtain and maintain intellectual property protection for our products and product candidates, including our ability to cost-effectively file, prosecute, defend and enforce any patent claims or other intellectual property rights; the size and growth of the markets for our products and product candidates and our ability to serve those markets; the degree of market acceptance of Ocaliva for PBC and, if approved, OCA for NASH or our other product candidates among physicians, patients and healthcare payors; the availability of adequate coverage and reimbursement from governmental and private healthcare payors for our products, including Ocaliva for PBC and, if approved, OCA for NASH, and our ability to obtain adequate pricing for such products; our ability to establish and maintain effective sales, marketing and distribution capabilities, either directly or through collaborations with third parties; competition from existing drugs or new drugs that become available; our ability to prevent system failures, data breaches or violations of data protection laws; costs and outcomes relating to any disputes, governmental inquiries or investigations, legal proceedings or litigation, including any securities, intellectual property, employment, product liability or other litigation; our collaborators' election to pursue research, development and commercialization activities; our ability to establish and maintain relationships with collaborators with development, regulatory and commercialization expertise; our need for and ability to generate or obtain additional financing; our estimates regarding future expenses, revenues and capital requirements and the accuracy thereof; our use of cash and short-term investments; our ability to acquire, license and invest in businesses, technologies, product candidates and products; our ability to attract and retain key personnel to manage our business effectively; our ability to manage the growth of our operations, infrastructure, personnel, systems and controls; our ability to obtain and maintain adequate insurance coverage; the impact of general U.S. and foreign economic, industry, market, regulatory or political conditions, including the potential impact of Brexit; and the other risks and uncertainties identified in our periodic filings filed with the U.S. Securities and Exchange Commission, including our Annual Report on Form 10-K for the year ended December 31, 2018.

Contact

For more information about Intercept, please contact:

Lisa DeFrancesco
+1-646-565-4833
investors@interceptpharma.com

Christopher Frates
+1-646-757-2371
media@interceptpharma.com

Source: Intercept Pharmaceuticals, Inc.