New Data from the Interim Analysis of REGENERATE Show that OCA Helped Patients with Liver Fibrosis Due to NASH Achieve Sustained Improvement in Noninvasive Markers of Fibrosis Over Two Years of Treatment

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NEW YORK, Aug. 27, 2020 (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 data indicating that obeticholic acid (OCA) helped patients with nonalcoholic steatohepatitis (NASH) achieve sustained improvements in liver biochemistry and noninvasive markers of liver fibrosis over two years of treatment. The new results based upon a post hoc review of the interim analysis data from the Phase 3 REGENERATE study are being presented at the virtual International Liver Congress™ 2020, the 55th Annual Meeting of the European Association for the Study of the Liver (EASL).

"The primary goal of a medicine to treat patients with advanced fibrosis due to NASH is to halt or reverse the progression to cirrhosis and its devastating complications," said Rohit Loomba, M.D., director, U.C. San Diego NAFLD Research Center and director of hepatology at U.C. San Diego School of Medicine. "These new noninvasive data from REGENERATE provide further evidence that OCA can help patients achieve this goal. It is encouraging to see a consistent and sustained effect across multiple noninvasive tests that clinicians use in practice every day to monitor and manage their patients. The marked improvement in measurements of liver stiffness observed with OCA therapy was particularly notable. Additionally, these data give us greater confidence that OCA continues to provide meaningful and durable benefit beyond the histologic benefit already established at 18 months."

As previously reported, once-daily OCA 25 mg met the primary endpoint of fibrosis improvement (≥1 stage) with no worsening of NASH at the planned 18-month interim analysis of REGENERATE with high statistical significance (p=0.0002 vs. placebo). The new analysis included patients from the interim analysis intent-to-treat (ITT) population randomized early enough to have both evaluable Month 18 biopsies (N = 251–263 per treatment arm) and Month 24 data at the time of the interim analysis (N = 120–125 per arm). Changes from baseline to Month 24 in alanine aminotransferase (ALT), aspartate aminotransferase (AST), gamma-glutamyl transferase (GGT), serum markers of fibrosis (FIB-4, AST to platelet ratio index [APRI]), and liver stiffness (FibroScan® vibration-controlled transient elastography [VCTE]; subset, N = 64–70 per arm) were analyzed.

Mean values of transaminases and other serum-based tests improved rapidly in patients treated with OCA and were sustained beyond 18 months of therapy compared with placebo. FibroScan VCTE also demonstrated improvement in liver stiffness in patients treated with OCA versus placebo after 24 months of therapy, with a mean difference of 2.7 kPa between OCA 25 mg and placebo. At baseline, median liver stiffness values were in the advanced fibrosis range; at 24 months, median values of patients treated with OCA 25 mg were below the threshold of 7.9 kPa, and most patients treated with OCA 25 mg had moved from advanced to moderate fibrosis.

Changes in transaminases and noninvasive markers of fibrosis were associated with changes in histologic fibrosis, with the greatest improvements observed in patients who had a ≥1 stage improvement in fibrosis stage at 18 months. Moreover, early changes in these markers were more pronounced in patients who had histologic fibrosis improvement at Month 18. Overall, these noninvasive data suggest that longer treatment duration with OCA will likely result in greater fibrosis reduction beyond 18 months.

The overall adverse event profile of OCA 25 mg in the subgroup of the ITT population with 24 months of follow-up at the time of the interim analysis was generally consistent with that observed in the overall ITT population. The most common adverse event was pruritus. The incidence of serious adverse events was balanced across the placebo and OCA 25 mg groups. Few serious adverse events occurred in more than one patient and no consistent pattern of serious adverse events was observed.

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. In the United States, NASH is currently the second leading cause for liver transplantation overall, and in females, the leading cause. NASH is anticipated to become the leading indication for liver transplantation in Europe within the next decade. 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 2,480 adult NASH patients randomized at over 300 qualified centers worldwide, and is expected to continue through clinical outcomes for verification and description of clinical benefit. The end-of-study analysis is designed to evaluate the effect of OCA on all-cause mortality and liver-related clinical outcomes, as well as 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 candidates, including OCA for liver fibrosis due to NASH, the timing and acceptance of our regulatory filings and the potential approval of OCA for liver fibrosis due to NASH, the review of our New Drug Application for OCA for the treatment of liver fibrosis due to NASH by the U.S. Food and Drug Administration (FDA), our intent to work with the FDA to address the issues raised in the complete response letter (CRL), the potential commercial success of OCA, as well as our strategy, future operations, future financial position, future revenue, projected costs, financial guidance, prospects, plans and objectives.

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; our ability to timely and cost-effectively file for and obtain regulatory approval of our product candidates on an accelerated basis or at all, including OCA for liver fibrosis due to NASH following the issuance of the CRL by the FDA; any advisory committee recommendation or dispute resolution determination that our product candidates, including OCA for liver fibrosis due to NASH, should not be approved or approved only under certain conditions; any future determination that the regulatory applications and subsequent information we submit for our product candidates, including OCA for liver fibrosis due to NASH, do not contain adequate clinical or other data or meet applicable regulatory requirements for approval; conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, including OCA for liver fibrosis due to NASH, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), any risk mitigation programs such as a REMS, 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 liver fibrosis due to 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; 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 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 liver fibrosis due to NASH, and our clinical trial activities; our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to successfully launch OCA for liver fibrosis due to 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 liver fibrosis due to 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 liver fibrosis due to 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, regulatory proceedings, 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 COVID-19, including any impact on our results of operations or financial position, related quarantines and government actions, delays relating to our regulatory applications, disruptions relating to our ongoing clinical trials or involving our contract research organizations, study sites or other clinical partners, disruptions relating to our supply chain or involving our third-party manufacturers, distributors or other distribution partners, facility closures or other restrictions, and the extent and duration thereof; 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, 2019 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2020.

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