Intercept Provides Regulatory Update

December 13, 2019

Company submits Marketing Authorization Application to the European Medicines Agency for obeticholic acid in patients with fibrosis due to NASH

FDA notifies Intercept of tentative date for previously announced Advisory Committee meeting

NEW YORK, Dec. 13, 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 that it has submitted its Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for obeticholic acid (OCA) for the treatment of fibrosis due to nonalcoholic steatohepatitis (NASH). The MAA submission is supported by the positive interim analysis results from the pivotal Phase 3 REGENERATE study in patients with liver fibrosis due to NASH.

Intercept also announced that the U.S. Food and Drug Administration (FDA) has notified the company of the tentative date for the previously announced advisory committee meeting (AdCom) related to Intercept's New Drug Application (NDA) for OCA in liver fibrosis due to NASH. The FDA has tentatively scheduled the AdCom for April 22, 2020. Intercept anticipates that the FDA accordingly will extend the recently announced March 26, 2020 Prescription Drug User Fee Act (PDUFA) target action date for Intercept's NDA. Intercept previously announced the FDA's acceptance of the NDA and granting of priority review.

"NASH is quickly becoming one of the most significant public health challenges in Europe and costs associated with advanced fibrosis and cirrhosis are estimated to represent approximately 95% of the total NASH related costs to health care systems," said Mark Pruzanski, M.D., President and Chief Executive Officer of Intercept. "We believe that OCA has the potential to become an essential treatment for people living with advanced fibrosis due to NASH and we look forward to working with the EMA during this review period. Separately, we are pleased that FDA has provided us with the tentative AdCom date and we look forward to working collaboratively with the agency during its review of the NDA as we continue to prepare for our anticipated NASH launch, if approved, within the first half of 2020."

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. 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 interim analysis was conducted to assess the effect of OCA on liver histology comparing month 18 biopsies with baseline. The intent-to-treat population for the interim analysis included 931 patients with stage 2 and 3 fibrosis (placebo, n=311; OCA 10 mg, n=312; OCA 25 mg, n=308). REGENERATE has completed target enrollment for the clinical outcomes cohort, with 2,480 adult NASH patients randomized at 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.

The safety population of the interim analysis included 1,968 randomized patients who received at least one dose of investigational product (OCA or placebo). Adverse events were generally mild to moderate in severity and the most common were consistent with the known profile of OCA. The frequency of serious adverse events was similar across treatment arms (11% in placebo, 11% in OCA 10 mg and 14% in OCA 25 mg). The most common adverse event reported was dose-related pruritus (placebo, 19%; OCA 10 mg, 28%; OCA 25 mg, 51%). The large majority of pruritus events were mild to moderate, with severe pruritus occurring in a small number of patients.

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 regulatory filings and the 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 the regulatory approval of our New Drug Application for NASH; any advisory committee recommendation that our product candidates, including OCA for NASH, should not be approved or approved only under certain conditions; any determination that the regulatory applications and subsequent information we submit for our product candidates, including OCA for 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, 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.

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