Intercept Announces NASH and PBC Program Updates

January 7, 2019

Top-line data from the interim analysis of the Phase 3 REGENERATE trial of obeticholic acid (OCA) in nonalcoholic steatohepatitis (NASH) patients with advanced fibrosis anticipated in Q1 2019

Phase 3 REVERSE trial of OCA in NASH patients with compensated cirrhosis projected to complete enrollment in 2019

Intercept acquires license to U.S. development and commercialization rights to pan-PPAR agonist bezafibrate with the goal of evaluating the potential for a fixed dose combination regimen with OCA for patients with primary biliary cholangitis (PBC) and other liver diseases

NEW YORK, Jan. 07, 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 two updates to its NASH development program. Intercept anticipates that it will read-out top-line data from the interim analysis of its Phase 3 REGENERATE trial evaluating obeticholic acid (OCA) in non-cirrhotic NASH patients with advanced liver fibrosis in the first quarter of 2019. Intercept also expects to complete enrollment of its Phase 3 REVERSE trial evaluating OCA in NASH patients with compensated cirrhosis in 2019.

“2019 is expected to be a transformative year for Intercept, as we look forward to announcing top-line results from the REGENERATE interim analysis this quarter and continuing to advance our leading Phase 3 development program for the treatment of NASH patients with advanced liver fibrosis and compensated cirrhosis where we believe there is the greatest unmet medical need,” said Mark Pruzanski, M.D., Intercept’s President and Chief Executive Officer.

In addition, Intercept announced that it has acquired from Aralez Pharmaceuticals Inc. its license to develop and commercialize bezafibrate in the U.S., its IND on file with the FDA and other associated regulatory documentation, and a non-exclusive license to certain of Aralez’s intellectual property. Bezafibrate, a pan-peroxisome proliferator-activated receptor (PPAR) agonist that has been studied in PBC, is not approved in the U.S. for any indication. Intercept intends to initiate a Phase 2 study to evaluate the efficacy, safety and tolerability of bezafibrate in combination with OCA in patients with PBC, with the longer term goal to develop and seek regulatory approval for a fixed dose combination regimen in this indication and potentially other liver diseases.

“We are pleased to have acquired the U.S. rights to bezafibrate and look forward to studying it in combination with OCA as a potential treatment for progressive non-viral liver diseases. Based on the potential benefits of combining bezafibrate with OCA in PBC, we will initially study the combination in this indication,” said Christian Weyer, M.D., M.A.S., Intercept's Executive Vice President, Research & Development.

As previously announced, Intercept management will present a corporate update at the 37th Annual J.P. Morgan Healthcare Conference on Wednesday, January 9, 2019 at 2:30 p.m. PT. A live webcast of the presentation will be available on the investor page of our website at http://ir.interceptpharma.com. A replay of the event will be available on our website shortly following the completion of the presentation and will be available for two weeks.

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 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.

About Nonalcoholic Steatohepatitis

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. There are currently no medications approved for the treatment of NASH. The proportion of liver transplants attributable to NASH has increased rapidly in recent years and as early as 2020 the disease is projected to become the leading cause of liver transplants in the United States.

About Primary Biliary Cholangitis

Primary biliary cholangitis (PBC) is a chronic, progressive liver disorder that mostly affects women, afflicting approximately one in 1,000 women over the age of 40. If left untreated, survival of PBC patients is significantly worse than the general population.

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”), the potential approval of OCA for indications other than primary biliary cholangitis (“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 or completing and timely reporting the results of our NASH or PBC clinical trials; our ability to timely and cost-effectively obtain regulatory approval of our product candidates, including OCA for NASH; 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 maintain our relationships with, and the performance of, third-party vendors upon whom we are substantially dependent, including contract research organizations for our clinical trials and our third-party suppliers and manufacturers; our ability to identify, develop and commercialize our products and product candidates; our ability to obtain and maintain intellectual property protection for our products and product candidates; our ability to successfully commercialize our product candidates, if approved; 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, which may be affected by the ability of patients and healthcare providers to obtain coverage or reimbursement from payors for our products and the extent to which such coverage or reimbursement is provided; our ability to establish and maintain an effective sales and marketing infrastructure 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 attract and maintain collaborators with development, regulatory and commercialization expertise; our need for and ability to obtain additional financing; our estimates regarding 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; 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, 2017.

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