



## Intercept Pharmaceuticals Provides Regulatory and Business Update

March 26, 2020

NEW YORK, March 26, 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 the rescheduling of its advisory committee meeting (AdCom) by the U.S. Food and Drug Administration (FDA), provided an update on expected first quarter 2020 financial performance, and announced new initiatives intended to ensure business continuity and support its employees amid the evolving COVID-19 pandemic, all while continuing the critical work necessary to bring its approved medicines to the patients who need them.

**Regulatory Update.** Amid the evolving COVID-19 pandemic, the FDA has notified us that it has rescheduled the previously announced AdCom related to our New Drug Application (NDA) for obeticholic acid (OCA) in liver fibrosis due to nonalcoholic steatohepatitis (NASH). The AdCom which had previously been tentatively scheduled for April 22, 2020, is now tentatively scheduled for June 9, 2020. We continue to work closely with the FDA on our priority review application and our Prescription Drug User Fee Act (PDUFA) target action date for the NDA remains June 26, 2020. We also continue to work collaboratively with the European Medicines Agency (EMA) on its review of our Marketing Authorization Application (MAA) for liver fibrosis due to NASH submitted in December 2019.

**Clinical Trials Update.** We are focused on ensuring patient safety and maintaining the integrity of our ongoing clinical trials. We are monitoring the COVID-19 situation very closely and together with our contract research organizations, study sites and other partners, have taken measures intended to minimize any disruption to our ongoing trials. Our pivotal Phase 3 NASH studies, REGENERATE and REVERSE, are fully enrolled and our focus is to provide needed support to participating sites and patients around the world. With respect to our ongoing PBC studies, under the current circumstances we are additionally temporarily pausing screening and randomization of patients in certain of our continuing studies of OCA in PBC.

**Financial Update.** Our PBC business has continued to perform well during the first quarter and our preliminary view indicates net sales are expected to be generally in line with those in the fourth quarter of 2019. In addition, we are reconfirming our previously announced non-GAAP adjusted operating expenses guidance for the full year 2020. We are investing to support the launch of OCA for liver fibrosis due to NASH, our commercial efforts in primary biliary cholangitis ("PBC"), our clinical development and pipeline programs and our other operating activities. Our non-GAAP adjusted operating expenses guidance continues to assume the approval of our NDA for liver fibrosis due to NASH by the FDA on or about our PDUFA target action date.

**Supply Chain Update.** Ensuring patient access to Ocaliva® (obeticholic acid or OCA) for patients with PBC is of paramount importance, particularly during these unprecedented times. We are working closely with our third-party manufacturers, distributors and other trusted partners to manage our supply chain activities and mitigate any potential disruptions to our product supplies as a result of COVID-19.

**Employee Update.** We support broader public health strategies designed to prevent the spread of COVID-19 and are focused on the health and welfare of our employees. In accordance with guidance issued by the Centers for Disease Control and Prevention (CDC), the World Health Organization (WHO) and local authorities, Intercept's global workforce, both office and field-based employees, are working remotely from home until further notice. We are now leveraging digital communication technologies to continue important interactions with healthcare professionals, patients and other stakeholders.

As we, along with the rest of the world, navigate these unprecedented circumstances, we are committed to continuing to implement measures intended to minimize any potential business impact from COVID-19 and will continue to closely monitor, assess and respond to the situation as it evolves.

### Non-GAAP Financial Measures

This press release presents non-GAAP adjusted operating expenses on projected basis. Non-GAAP adjusted operating expenses exclude from total operating expenses, as calculated and presented in accordance with GAAP, the effects of two non-cash items: stock-based compensation and depreciation and non-cash operating lease expense cost. Non-GAAP adjusted operating expenses is a financial measure that is not prepared in accordance with GAAP. Accordingly, investors should consider non-GAAP adjusted operating expenses in addition to, but not as a substitute for, total operating expenses that we calculate and present in accordance with GAAP. Among other things, our management uses non-GAAP adjusted operating expenses to establish budgets and operational goals and to manage our business. Other companies may define or use this measure in different ways. We believe that the presentation of non-GAAP adjusted operating expenses provides investors and management with helpful supplemental information relating to operating performance and trends. A quantitative reconciliation of projected non-GAAP adjusted operating expenses to total operating expenses is not available without unreasonable effort primarily due to our inability to predict with reasonable certainty the amount of future stock-based compensation expense.

### About Ocaliva® (obeticholic acid)

Ocaliva is indicated in the United States for the treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA.

This indication is approved under the accelerated approval pathway based on a reduction in alkaline phosphatase (ALP) as a surrogate endpoint which is reasonably likely to predict clinical benefit, including an improvement in liver transplant free-survival. An improvement in survival or disease-related symptoms has not been established. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials. We are conducting a Phase 4 clinical outcomes trial, which we refer to as our COBALT trial, of OCA in patients with PBC with the goal of confirming clinical benefit on a post-marketing basis.

In December 2016, Ocaliva received conditional marketing authorization in Europe for the treatment of PBC in combination with UDCA in adults with

an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA, conditioned upon us providing further data post-approval to confirm benefit. For detailed safety information for Ocaliva 5 mg and 10 mg tablets including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European Summary of Product Characteristics that can be found on [www.ema.europa.eu](http://www.ema.europa.eu).

## **U.S. IMPORTANT SAFETY INFORMATION FOR OCALIVA IN PBC**

### **WARNING: HEPATIC DECOMPENSATION AND FAILURE IN INCORRECTLY DOSED PBC PATIENTS WITH CHILD-PUGH CLASS B OR C OR DECOMPENSATED CIRRHOSIS**

**In postmarketing reports, hepatic decompensation and failure, in some cases fatal, have been reported in patients with Primary Biliary Cholangitis (PBC) with decompensated cirrhosis or Child-Pugh Class B or C hepatic impairment when OCALIVA was dosed more frequently than recommended.**

**The recommended starting dosage of OCALIVA is 5 mg once weekly for patients with Child-Pugh Class B or C hepatic impairment or a prior decompensation event.**

#### **Contraindications**

OCALIVA is contraindicated in PBC patients with complete biliary obstruction.

#### **Warnings and Precautions**

##### ***Hepatic Decompensation and Failure in Incorrectly-Dosed PBC Patients with Child-Pugh Class B or C or Decompensated Cirrhosis***

In postmarketing reports, hepatic decompensation and failure, in some cases fatal, have been reported in PBC patients with decompensated cirrhosis or Child-Pugh B or C hepatic impairment when OCALIVA was dosed more frequently than the recommended starting dosage of 5 mg once weekly. Reported cases typically occurred within 2 to 5 weeks after starting OCALIVA and were characterized by an acute increase in total bilirubin and/or ALP concentrations in association with clinical signs and symptoms of hepatic decompensation (e.g., ascites, jaundice, gastrointestinal bleeding, worsening of hepatic encephalopathy).

Routinely monitor patients for progression of PBC disease, including liver-related complications, with laboratory and clinical assessments. Dosage adjustment, interruption or discontinuation may be required. Close monitoring is recommended for patients at an increased risk of hepatic decompensation. Severe intercurrent illnesses that may worsen renal function or cause dehydration (e.g., gastroenteritis), may exacerbate the risk of hepatic decompensation. Interrupt treatment with OCALIVA in patients with laboratory or clinical evidence of worsening liver function indicating risk of decompensation, and monitor the patient's liver function. Consider discontinuing OCALIVA in patients who have experienced clinically significant liver-related adverse reactions. Discontinue OCALIVA in patients who develop complete biliary obstruction.

##### ***Liver-Related Adverse Reactions***

Dose-related, liver-related adverse reactions including jaundice, worsening ascites and primary biliary cholangitis flare have been observed in clinical trials, as early as one month after starting treatment with OCALIVA 10 mg once daily up to 50 mg once daily (up to 5-times the highest recommended dosage). Monitor PBC patients during treatment with OCALIVA for elevations in liver biochemical tests and for the development of liver-related adverse reactions.

##### ***Severe Pruritus***

Severe pruritus was reported in 23% of PBC patients in the OCALIVA 10 mg arm, 19% of PBC patients in the OCALIVA titration arm, and 7% of PBC patients in the placebo arm in a 12-month double-blind randomized controlled trial of 216 PBC patients. Severe pruritus was defined as intense or widespread itching, interfering with activities of daily living, or causing severe sleep disturbance, or intolerable discomfort, and typically requiring medical interventions. Consider clinical evaluation of PBC patients with new onset or worsening severe pruritus. Management strategies include the addition of bile acid resins or antihistamines, OCALIVA dosage reduction, and/or temporary interruption of OCALIVA dosing.

##### ***Reduction in HDL-C***

Patients with PBC generally exhibit hyperlipidemia characterized by a significant elevation in total cholesterol primarily due to increased levels of high-density lipoprotein-cholesterol (HDL-C). Dose-dependent reductions from baseline in mean HDL-C levels were observed at 2 weeks in OCALIVA-treated PBC patients, 20% and 9% in the 10 mg and titration arms, respectively, compared to 2% in the placebo arm. Monitor PBC patients for changes in serum lipid levels during treatment. For PBC patients who do not respond to OCALIVA after 1 year at the highest recommended dosage that can be tolerated (maximum of 10 mg once daily), and who experience a reduction in HDL-C, weigh the potential risks against the benefits of continuing treatment.

#### **Adverse Reactions**

The most common adverse reactions from subjects taking OCALIVA for PBC were pruritus, fatigue, abdominal pain and discomfort, rash, oropharyngeal pain, dizziness, constipation, arthralgia, thyroid function abnormality, and eczema.

#### **Drug Interactions**

##### ***Bile Acid Binding Resins***

Bile acid binding resins such as cholestyramine, colestipol, or colesevelam adsorb and reduce bile acid absorption and may reduce the absorption, systemic exposure, and efficacy of OCALIVA. If taking a bile acid binding resin, take OCALIVA at least 4 hours before or 4 hours after taking the bile acid binding resin, or at as great an interval as possible.

##### ***Warfarin***

The International Normalized Ratio (INR) decreased following coadministration of warfarin and OCALIVA. Monitor INR and adjust the dose of warfarin,

as needed, to maintain the target INR range when coadministering OCALIVA and warfarin.

### **CYP1A2 Substrates with Narrow Therapeutic Index**

Obeticholic acid, the active ingredient in OCALIVA, may increase the exposure to concomitant drugs that are CYP1A2 substrates. Therapeutic monitoring of CYP1A2 substrates with a narrow therapeutic index (e.g. theophylline and tizanidine) is recommended when coadministered with OCALIVA.

### **Inhibitors of Bile Salt Efflux Pump**

Avoid concomitant use of inhibitors of the bile salt efflux pump (BSEP) such as cyclosporine. Concomitant medications that inhibit canalicular membrane bile acid transporters such as the BSEP may exacerbate accumulation of conjugated bile salts including taurine conjugate of obeticholic acid in the liver and result in clinical symptoms. If concomitant use is deemed necessary, monitor serum transaminases and bilirubin.

Please see [Full Prescribing Information, including Boxed WARNING](#) and [Medication Guide](#) for OCALIVA.

**To report SUSPECTED ADVERSE REACTIONS, contact Intercept Pharmaceuticals, Inc. at 1-844-782-ICPT or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).**

### **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](http://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 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 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: the impact of COVID-19, including any impact on our net sales, non-GAAP adjusted operating expenses 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; 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 or 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 NDA for liver fibrosis due to NASH; any advisory committee recommendation that our product candidates, including OCA for liver fibrosis due to 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 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, 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 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; 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 timely and 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, 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, 2019.

#### **Contact**

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