



Intercept Pharmaceuticals Reports First Quarter 2019 Financial Results and Provides Business Update

May 8, 2019

Positive results from pivotal Phase 3 REGENERATE study of OCA in patients with liver fibrosis due to NASH

New Drug Application (NDA) filing in the U.S. for OCA in NASH projected in the third quarter of 2019

Worldwide Ocaliva net sales of \$51.8 million in the first quarter of 2019 representing 47% growth versus the prior year quarter

Increasing 2019 Ocaliva net sales guidance range to between \$235 and \$245 million

Updating 2019 non-GAAP adjusted operating expenses guidance range to between \$470 and \$500 million in connection with acceleration of NASH NDA filing and launch preparation activities

Conference call scheduled for 8:30 a.m. ET today

NEW YORK, May 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 its financial results for the quarter ended March 31, 2019.

"Our Phase 3 REGENERATE readout demonstrating OCA's anti-fibrotic benefit in patients with NASH has been a watershed moment for the liver community and underscores our unique competitive opportunity to become the first approved NASH therapy," said Mark Pruzanski, M.D., President and Chief Executive Officer of Intercept. "I'm very pleased with the acceleration of our NASH NDA filing and launch preparations, as well as our continued strong commercial performance in PBC resulting in our increased 2019 Ocaliva net sales guidance."

OCA in NASH

In February 2019, we announced positive topline results from the pivotal Phase 3 REGENERATE study demonstrating that once-daily obeticholic acid ("OCA") 25 mg met the primary endpoint of 'fibrosis improvement by at least one stage with no worsening of NASH' in the planned 18-month interim analysis with high statistical significance ($p=0.0002$ vs placebo). Adverse events were generally mild-to-moderate in severity and the most common were consistent with the known profile of OCA. In April 2019, additional supportive REGENERATE data were presented during the Opening Ceremony of the International Liver Congress™ 2019, the 54th Annual Meeting of the European Association for the Study of the Liver ("EASL"). The data presented at EASL showed that OCA demonstrated robust efficacy across a range of additional histologic and biochemical parameters.

REGENERATE is the only successful Phase 3 study in NASH with liver fibrosis to date and will serve as the basis for our NDA filing with the U.S. Food and Drug Administration ("FDA") projected in the third quarter of this year seeking approval of OCA for NASH. OCA is the only investigational drug to have received Breakthrough Therapy designation from the FDA for NASH with fibrosis. We also intend to file a marketing authorization application ("MAA") with the European Medicines Agency ("EMA") in the fourth quarter of this year. REGENERATE remains ongoing through clinical outcomes and is the largest Phase 3 NASH study, with more than 2,000 patients enrolled to date. In addition, our Phase 3 REVERSE study of OCA in NASH patients with compensated cirrhosis is currently ongoing and projected to complete enrollment by the end of 2019.

Ocaliva® (obeticholic acid) Commercial Highlights

We recognized \$51.8 million of Ocaliva net sales in the first quarter of 2019, as compared to \$35.2 million in the prior year quarter. Ocaliva net sales in the first quarter of 2019 were comprised of U.S. net sales of \$38.0 million and ex-U.S. net sales of \$13.8 million, as compared to U.S. net sales of \$28.5 million and ex-U.S. net sales of \$6.7 million in the prior year quarter.

Ocaliva was approved in the United States by the FDA in May 2016 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. We commenced sales and marketing of Ocaliva in the United States shortly after receiving marketing approval, and Ocaliva is now available to patients primarily through a network of specialty pharmacy distributors.

Ocaliva was granted conditional approval by the European Commission in December 2016 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, and we commenced our European commercial launch in January 2017. Since January 2017, Ocaliva has also received regulatory approval in several of our target markets outside the United States and Europe, including Canada, Israel and Australia.

Selected First Quarter 2019 Financial Results

Revenues

We recognized \$52.2 million in total revenue in the first quarter of 2019, as compared to \$36.0 million in total revenue in the prior year quarter. Total revenue in the first quarter of 2019 included \$51.8 million of Ocaliva net sales and approximately \$0.4 million of licensing revenue, as compared to \$35.2 million and approximately \$0.8 million, respectively, in the prior year quarter.

Operating Expenses

Our cost of sales was \$0.6 million in the first quarter of 2019, as compared to \$0.3 million in the prior year quarter. Our cost of sales for the quarters

ended March 31, 2019 and 2018 consisted primarily of packaging, labeling, materials and related expenses.

Our selling, general and administrative expenses increased to \$77.2 million in the first quarter of 2019, up from \$62.5 million in the prior year quarter. The increase was primarily driven by increases in NASH pre-launch activities.

Our research and development expenses increased to \$58.4 million in the first quarter of 2019, up from \$48.7 million in the prior year quarter. The increase was primarily driven by increases in OCA NASH development program expenses, including costs associated with the REGENERATE readout in the first quarter.

In the quarters ended March 31, 2019 and 2018, we recorded \$136.2 million and \$111.4 million, respectively, in total operating expenses and \$118.9 million and \$97.8 million, respectively, in non-GAAP adjusted operating expenses, which excludes non-cash stock-based compensation expense of \$14.9 million and \$12.3 million, respectively, and depreciation and amortization expense of \$2.4 million and \$1.3 million, respectively.

References in this press release to “non-GAAP adjusted operating expenses” mean our total operating expenses, as calculated and presented in accordance with U.S. Generally Accepted Accounting Principles (“GAAP”), adjusted for the effects of two non-cash items: stock-based compensation and depreciation and amortization. See “Non-GAAP Financial Measures” below. A reconciliation of non-GAAP adjusted operating expenses to total operating expenses for all historical periods presented is included below under the heading “Reconciliation of Non-GAAP Adjusted Operating Expenses to Total Operating Expenses.”

Interest Expense

Interest expense in the quarters ended March 31, 2019 and 2018 was \$7.8 million and \$7.5 million, respectively. Our interest expense is related to the \$460.0 million aggregate principal amount of 3.25% Convertible Senior Notes due 2023 (the “Convertible Notes”) that we issued in July 2016.

Net Loss

In the first quarter of 2019, we reported a net loss of \$90.3 million, up from a net loss of \$81.6 million in the prior year quarter.

Cash Position

As of March 31, 2019, we had cash, cash equivalents and investment debt securities available for sale of approximately \$353.5 million. As of December 31, 2018, we had cash, cash equivalents and investment debt securities available for sale of approximately \$436.2 million.

2019 Financial Guidance

Based on our first quarter results and our current outlook for the remainder of 2019, we are increasing our 2019 Ocaliva net sales guidance range to between \$235 million and \$245 million. In addition, we are updating our 2019 non-GAAP adjusted operating expenses guidance range to between \$470 million and \$500 million in connection with the acceleration of our NASH NDA filing and launch preparation activities. See “Non-GAAP Financial Measures” below. 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.

Conference Call on May 8, 2019 at 8:30 a.m. ET

We are hosting our first quarter 2019 financial results conference call and webcast on Wednesday, May 8, 2019 at 8:30 a.m. ET. The conference call will be available on the investor page of our website at <http://ir.interceptpharma.com> or by calling (855) 232-3919 (toll-free domestic) or (315) 625-6894 (international) five minutes prior to the start time (no passcode is required). A replay of the call will be available on our website shortly following the completion of the call 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) and nonalcoholic steatohepatitis (NASH). Founded in 2002 in New York, Intercept has operations in the United States, Europe and Canada.

Non-GAAP Financial Measures

This press release presents non-GAAP adjusted operating expenses on a historical and projected basis. For the periods presented, 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 amortization. Non-GAAP adjusted operating expenses is a financial measure that has not been 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 table reconciling non-GAAP adjusted operating expenses to total operating expenses for all historical periods presented is included below under the heading “Reconciliation of Non-GAAP Adjusted Operating Expenses to Total Operating Expenses”. 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 accelerated approval 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.

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.

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, 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:

Justine O'Malley
+1-646-931-1180
investors@interceptpharma.com

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

Intercept Pharmaceuticals, Inc. Condensed Consolidated Statements of Operations (Unaudited) (In thousands, except per share data)

| | Three Months Ended | |
|--|--------------------|-------------|
| | March 31, | |
| | 2019 | 2018 |
| Revenue: | | |
| Product revenue, net | \$ 51,847 | \$ 35,158 |
| Licensing revenue | 405 | 805 |
| Total revenue | 52,252 | 35,963 |
| Operating expenses: | | |
| Cost of sales | 574 | 280 |
| Selling, general and administrative | 77,227 | 62,467 |
| Research and development | 58,396 | 48,672 |
| Total operating expenses | 136,197 | 111,419 |
| Operating loss | (83,945) | (75,456) |
| Other income (expense): | | |
| Interest expense | (7,839) | (7,509) |
| Other income, net | 1,514 | 1,375 |
| | (6,325) | (6,134) |
| Net loss | \$ (90,270) | \$ (81,590) |
| Net loss per common and potential common share: | | |
| Basic and diluted | \$ (3.03) | \$ (3.22) |
| Weighted average common and potential common shares outstanding: | | |
| Basic and diluted | 29,760 | 25,309 |

Condensed Consolidated Balance Sheet Information (In thousands)

| | March 31, 2019 (Unaudited) | December 31, 2018 (1) |
|---|----------------------------------|--------------------------|
| Cash, cash equivalents and investment debt securities | \$ 353,542 | \$ 436,160 |
| Total assets | \$ 438,258 | \$ 509,167 |
| Deferred revenue, total | \$ 3,122 | \$ 2,432 |
| Total liabilities (2) | \$ 493,259 | \$ 490,037 |
| Stockholders' (deficit) equity | \$ (55,001) | \$ 19,130 |

(1) Derived from the audited financial statements included in Intercept's Annual Report on Form 10-K for the year ended December 31, 2018.

(2) Includes \$375.4 million and \$371.2 million related to the Convertible Notes as of March 31, 2019 and December 31, 2018, respectively. Intercept separately accounts for the debt and equity components of the Convertible Notes. The aggregate outstanding principal amount of the Convertible Notes was \$460.0 million as of March 31, 2019 and December 31, 2018.

Reconciliation of Non-GAAP Adjusted Operating Expenses to Total Operating Expenses (Unaudited) (In thousands)

**Three Months Ended
March 31,**

| | 2019 | 2018 |
|--------------------------|-------------|-------------|
| Total operating expenses | \$ 136,197 | \$ 111,419 |

| | | |
|--------------------------------------|------------|-----------|
| Adjustments: | | |
| Stock-based compensation | 14,897 | 12,305 |
| Depreciation and amortization | 2,400 | 1,290 |
| Non-GAAP adjusted operating expenses | \$ 118,900 | \$ 97,824 |



Source: Intercept Pharmaceuticals, Inc.