February 23, 2017

# Intercept Pharmaceuticals Reports Full Year 2016 Financial Results and Provides Business Update

- ***Net Ocaliva® (obeticholic acid or OCA) 4Q16 sales of $13.4 million, full year 2016 net sales of $18.2 million***
- ***Enrollment of Phase 3 REGENERATE interim analysis cohort anticipated completion by mid-2017***
- ***Data expected from two Phase 2 trials for OCA in 2017***
- ***Planned initiation of Phase 2 trial of INT-767 in 2017***

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

NEW YORK, Feb. 23, 2017 (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 reported financial results for the three months and full year ended December 31, 2016 and provided other general business updates.

"2016 was a truly momentous year for Intercept, marked by the U.S. and European approvals of Ocaliva for the treatment of primary biliary cholangitis (PBC)," said Mark Pruzanski, M.D., President and CEO of Intercept. "We successfully transitioned to a commercial organization with our U.S. launch for PBC in June 2016 that generated net sales of $18.2 million for the year, while making solid progress in our clinical development programs."

"We believe 2017 has the potential to be another transformative year for Intercept," added Dr. Pruzanski. "We are dedicated to bringing Ocaliva to more PBC patients in need, not only in the U.S, but now also internationally. We intend to complete enrollment of the interim analysis cohort in our Phase 3 REGENERATE trial in nonalcoholic steatohepatitis (NASH) patients with fibrosis by mid-2017, an important milestone given our intention to seek regulatory approval based on its results. In addition, we plan to report data for two placebo-controlled Phase 2 trials of OCA, one designed to advance our understanding of OCA's lipid effects in NASH patients taking statins and the second to evaluate OCA in patients with primary sclerosing cholangitis, a devastating cholestatic liver disease with no approved treatment options. Finally, we will be initiating a Phase 2 trial of our second product candidate, INT-767, in NASH patients with fibrosis."

**Ocaliva Commercial Update**

Net U.S. Ocaliva sales were $18.2 million for the full year 2016, and $13.4 million for the fourth quarter.

Ocaliva was approved by the U.S. Food and Drug Administration (FDA) in May 2016 for the treatment of PBC in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA. Intercept commercially launched Ocaliva in the United States in June 2016 and in conjunction launched Interconnect®, a comprehensive, personalized program that connects patients with dedicated care coordinators who help them understand their disease and provides treatment support and, for eligible patients, financial assistance options.

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. We commenced our European commercial launch in January 2017.

**Anticipated 2017 Milestones**

- PBC Program
  - Ongoing U.S. Ocaliva launch
  - Launch Ocaliva in key European markets and seek regulatory approval in other target international markets
  - Continue enrollment in COBALT Phase 4 trial evaluating the effect of Ocaliva on clinical outcomes in PBC

- NASH Program
  - Complete enrollment of interim analysis cohort for Phase 3 REGENERATE trial by mid-2017
  - Report top-line results from the Phase 2 CONTROL trial
  - Initiate Phase 3 trial in NASH patients with liver cirrhosis

- Pipeline
  - Report top-line results from the Phase 2 AESOP trial for OCA in primary sclerosing cholangitis (PSC)
  - Initiate Phase 2 trial for INT-767, a dual FXR/TGR5 agonist, in NASH patients with fibrosis

**Personnel Update**

Intercept announced today that Jerry Durso has been appointed as Chief Operating Officer.

"We are very excited to welcome Jerry as our new COO," said Dr. Pruzanski. "This is a role that will augment our capabilities in managing the increasing complexity of our business as we continue to grow in size and geography, and broaden our commercial horizons beyond PBC with a view to NASH and other indications."

Mr. Durso brings nearly 25 years of experience in building and leading commercial and business operations at life sciences companies both in the United States and abroad. Mr. Durso has spent the majority of his career at Sanofi, a global pharmaceutical company, where he most recently served as senior vice president, chief commercial officer of the global diabetes division from 2011 through 2015. From 2010 to 2011, Mr. Durso was senior vice president, chief commercial officer of Sanofi's U.S. pharmaceuticals business. Prior to that, he served in a number of commercial leadership roles of increasing responsibility in business unit and brand management, marketing and sales since he first joined Sanofi in 1993. Mr. Durso currently serves as an advisory board member of the Robert Wood Johnson University Hospital Somerset in Somerville, New Jersey.

**Financial Results**

*Full Year 2016 Financial Results*

For the full year ended December 31, 2016, Intercept reported a net loss of $412.8 million. GAAP operating expense for the year ended December 31, 2016 was $427.5 million. Non-GAAP adjusted operating expense1 for the full year ended December 31, 2016 was $332.5 million, which excludes a one-time net expense of $45 million for the settlement of a purported class action lawsuit, non-cash stock-based compensation expense of $46.2 million and depreciation expense of $3.8 million.

***Revenues***

Intercept recognized $18.2 million of net sales of Ocaliva for the full year 2016. Intercept currently recognizes revenue using the sell-through method (i.e., when its specialty pharmacies dispense Ocaliva to patients, not when products are sold to the specialty pharmacies). Revenue recognition will transition from the sell-through method to the sell-in method once a sufficient period of commercial experience has occurred to enable Intercept to estimate product returns.

Intercept recognized $6.8 million and $2.8 million of license revenue related to the amortization of the up-front and milestone payments under the collaboration agreement with Sumitomo Dainippon for the year ended December 31, 2016 and 2015, respectively.

***Expenses***

Costs of goods sold (COGS) was negligible for 2016. Prior to the FDA approval of Ocaliva, Intercept had expensed costs related to the manufacturing and buildup of commercial launch supplies of OCA. Therefore, COGS was only reflective of packaging and labeling costs incurred during the period.
Intercept expects COGS to remain negligible until previously expensed supplies of OCA are sold.

Selling, general and administrative expenses increased to $273.6 million for the full year ended December 31, 2016, up from $119.2 million for the full year ended December 31, 2015. The increase over the prior period was driven by the U.S. commercial launch of Ocaliva in PBC, along with increased international infrastructure and pre-commercial activities to support the anticipated launch of Ocaliva in PBC outside the United States.

Research and development expenses increased to $153.9 million for the full year ended December 31, 2016, up from $112.7 million for the full year ended December 31, 2015. The increase over the prior period was primarily driven by increases in clinical development programs for OCA and infrastructure to support such programs.

Interest expense for the full year ended December 31, 2016 was $14.2 million, due to the issuance of the 3.25% convertible senior notes due 2023 (convertible notes) in July 2016.

*Three Months Ended December 31, 2016*

Intercept recognized $13.4 million of net sales of Ocaliva for the three months ended December 31, 2016.

Intercept reported a net loss of $120.0 million for the three months ended December 31, 2016, compared to a net loss of $88.3 million for the three months ended December 31, 2015. The net loss included $19.2 million and $12.2 million of non-cash stock-based compensation expenses for the three months ended December 31, 2016 and 2015, respectively.

**Cash Position**

As of December 31, 2016, Intercept had cash, cash equivalents and investment securities available for sale of

approximately $689.4 million, compared to $628.1 million as of December 31, 2015. In July 2016, Intercept completed an underwritten public offering of convertible notes. After deducting the underwriting discount and offering expenses, net proceeds from the convertible notes offering were approximately $447.7 million. Approximately $38.4 million of the net proceeds from the offering were used to fund the payment of the cost of capped call transactions entered into in connection with the issuance of the convertible notes.

**Financial guidance**

Intercept projects non-GAAP adjusted operating expenses of $380 million to $420 million for the fiscal year ending December 31, 2017. This guidance excludes non-cash items such as stock-based compensation and depreciation. These expenses are planned to support the continued commercialization of Ocaliva in PBC in the United States and other markets, continued clinical development for OCA in PBC and NASH and the continued development of INT-767 and other pipeline programs.

Intercept anticipates that stock-based compensation expense will represent the most significant non- cash item that will be excluded in adjusted operating expenses as compared to operating expenses under GAAP. Adjusted operating expense is a financial measure not calculated in accordance with GAAP. A reconciliation of projected operating expense calculated in accordance with GAAP to non- GAAP adjusted operating expense is not available on a forward-looking basis without unreasonable effort due to an inability to make accurate projections and estimates related to certain information needed to calculate, for example, future stock-based compensation expense.

**Conference Call on February 23rd at 8:30 a.m. ET**

Intercept will hold its 2016 full year financial results conference call and webcast on Thursday, February 23rd at 8:30 a.m. ET. The live event will be available on the investor page of the Intercept 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 the Intercept website approximately two hours after the completion of the call and will be archived for two weeks.

***About Intercept***

Intercept is a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat non-viral, progressive liver diseases, including primary biliary cholangitis (PBC), nonalcoholic steatohepatitis (NASH), primary sclerosing cholangitis (PSC) and biliary atresia. Founded in 2002 in New York, Intercept now has operations in the United States, Europe and Canada.

***Non-GAAP Financial Measures***

This press release presents adjusted operating expense, which is a non-GAAP measure, both on a historical and projected basis. Adjusted operating expense should be considered in addition to, but not as a substitute for, operating expense that Intercept prepares and announces in accordance with GAAP. Intercept excludes certain items from adjusted operating expense, such as stock-based compensation and depreciation, that management does not believe affect Intercept's basic operations and that do not meet the GAAP definition of unusual or nonrecurring items. For the year ended December 31, 2016, adjusted operating expense also excludes the one-time $45 million net expense for the settlement of the purported class action lawsuit.

A table reconciling historical GAAP operating expense to non-GAAP adjusted operating expense is included below under the heading "Reconciliation of GAAP to Non-GAAP Operating Expense." A reconciliation of projected operating expense calculated in accordance with GAAP to non-GAAP adjusted operating expense is not available on a forward-looking basis without unreasonable effort due to an inability to make accurate projections and estimates related to certain information needed to calculate, for example, future stock-based compensation expense. Management also uses adjusted operating expense to establish budgets and operational goals and to manage Intercept's business.
Other companies may define this measure in different ways. Intercept believes this presentation provides investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information.

***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. Intercept is currently enrolling COBALT, a Phase 4 clinical outcomes trial of Ocaliva 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, conditional to the company providing further data post-approval to confirm benefit. For detailed safety information for Ocaliva (obeticholic
acid) 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**

**Contraindications**

Ocaliva is contraindicated in patients with complete biliary obstruction.

**Warnings and Precautions**

**Liver-Related Adverse Reactions**

In two 3-month, placebo-controlled clinical trials a dose-response relationship was observed for the occurrence of liver-related adverse reactions including jaundice, ascites and primary biliary cholangitis flare with dosages of Ocaliva of 10 mg once daily to 50 mg once daily (up to 5-times the highest recommended dosage), as early as one month after starting treatment with Ocaliva.

In a pooled analysis of three placebo-controlled trials in patients with PBC, the exposure-adjusted incidence rates for all serious and otherwise clinically significant liver-related adverse reactions, and isolated elevations in liver biochemical tests, per 100 patient exposure years (PEY) were: 5.2 in the Ocaliva 10 mg group (highest recommended dosage), 19.8 in the Ocaliva 25 mg group (2.5 times the highest recommended dosage) and 54.5 in the Ocaliva 50 mg group (5 times the highest recommended dosage) compared to 2.4 in the placebo group.

Monitor patients during treatment with Ocaliva for elevations in liver biochemical tests and for the development of liver-related adverse reactions. Weigh the potential risks against the benefits of continuing treatment with Ocaliva in patients who have experienced clinically significant liver-related adverse reactions. The maximum recommended dosage of Ocaliva is 10 mg once daily. Adjust the dosage for patients with moderate or severe hepatic impairment.

Discontinue Ocaliva in patients who develop complete biliary obstruction.

**Severe Pruritus**

Severe pruritus was reported in 23% of patients in the Ocaliva 10 mg arm, 19% of patients in the Ocaliva titration arm and 7% of patients in the placebo arm in the POISE trial, a 12-month double- blind randomized controlled trial of 216 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. In the subgroup of patients in the Ocaliva titration arm who increased their dosage from 5 mg once daily to 10 mg once daily after 6 months of treatment (n=33), the incidence of severe pruritus was 0% from months 0 to 6 and 15% from months 6 to 12. The median time to onset of severe pruritus was 11, 158 and 75 days for patients in the Ocaliva 10 mg, Ocaliva titration and placebo arms, respectively.

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 (HDLC). In the POISE trial, dose-dependent reductions from baseline in mean HDL-C levels were observed at 2 weeks in Ocaliva-treated patients, 20% and 9% in the 10 mg and titration arms, respectively, compared to 2% in the placebo arm. At month 12, the reduction from baseline in mean HDL-C level was 19% in the Ocaliva 10 mg arm, 12% in the Ocaliva titration arm and 2% in the placebo arm. Nine patients in the Ocaliva 10 mg arm and six patients in the Ocaliva titration arm, versus three patients in the placebo arm had reductions in HDL-C to less than 40 mg/dL.

Monitor patients for changes in serum lipid levels during treatment. For patients who do not respond to Ocaliva after one 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 (≥5%) were pruritus, fatigue, abdominal pain and discomfort, rash, oropharyngeal pain, dizziness, constipation, arthralgia, thyroid function abnormality and eczema.

**Drug Interaction**

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

Please see the U.S. Full Prescribing Information for Ocaliva (obeticholic acid) 5 mg and 10 mg tablets.

***Safe Harbor Statements***

*This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding Intercept's financial position, including expected adjusted operating expenses; the activities anticipated to be undertaken by Intercept, including the anticipated progression of the U.S. and EU launches of Ocaliva® in PBC; the potential approval of OCA in PBC by regulatory bodies outside of the United States and the European Union and the timelines related thereto; the timelines for access to OCA for the treatment of PBC in Europe and other jurisdictions outside the United States and timelines related thereto; the initiation, enrollment, conduct and completion of clinical trials and the timelines related thereto, including the full enrollment of the interim analysis cohort for the Phase 3 REGENERATE trial of OCA in NASH patients with liver fibrosis; the anticipated regulatory process and timetable with respect to Intercept's product candidates; the continued development of OCA and Intercept's other product candidates; and Intercept's strategic directives under the caption "About Intercept." These "forward- looking statements" are based on management's current expectations of future events and are subject to a number of important risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to: Intercept's ability to successfully commercialize Ocaliva in PBC, and Intercept's ability to maintain its regulatory approval in jurisdictions in which Ocaliva is approved for use in PBC; the initiation, cost, timing, progress and results of Intercept's development activities, preclinical studies and clinical trials, including Intercept's development program in NASH; the timing of and Intercept's ability to obtain and maintain regulatory approval of OCA in PBC in countries outside the ones in which it is approved and in indications other than PBC and any other product candidates it may develop such as INT-767; conditions that may be imposed by regulatory authorities on Intercept's marketing approvals for its 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 in the label of any approved products and product candidates; Intercept's plans to research, develop and commercialize its product candidates; Intercept's ability to obtain and maintain intellectual property protection for its products and product candidates; Intercept's ability to successfully commercialize OCA in indications other than PBC and its other product candidates; the size and growth of the markets for Intercept's products and product candidates and its ability to serve those markets; the rate and degree of market acceptance of any of Intercept's products, which may be affected by the reimbursement that it may receive for its products from payors; the success of competing drugs that are or become available; the election by Intercept's collaborators to pursue research, development and commercialization activities; Intercept's ability to attract collaborators with development, regulatory and commercialization expertise; regulatory developments in the United States and other countries; the performance of third-party suppliers and manufacturers; Intercept's need for and ability to obtain additional financing; Intercept's estimates regarding expenses, future revenues and capital requirements and the accuracy thereof; Intercept's use of cash, short-term investments and the proceeds from the offering; Intercept's ability to attract and retain key scientific or management personnel; and other factors discussed under the heading "Risk Factors" contained in our annual report on Form 10-K for the year ended December 31, 2015 filed on February 29, 2016 as well as any updates to these risk factors filed from time to time in our other filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Intercept undertakes no duty to update this information unless required by law.*

1 Adjusted operating expense, as presented above and elsewhere in this press release, is a non-GAAP financial measure. Adjusted operating expense excludes stock-based compensation and other non-cash items from GAAP operating expenses. For the year ended December 31, 2016, adjusted operating expense also excludes the one-time $45 million net expense for the settlement of the purported class action lawsuit. A table reconciling historical adjusted operating expense to GAAP operating expense is included below under the heading "Reconciliation of GAAP to Non-GAAP Operating Expense."

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

| | **Three Months Ended December** | **Twelve Months Ended** |
|---|---|---|

| | 31, | | December 31, | |
|---|---|---|---|---|
| | 2016 | 2015 | 2016 | 2015 |
| Revenue: | | | | |
| Product revenue, net | $ 13,364 | $ - | $ 18,169 | $ - |
| Licensing revenue | 445 | 445 | 6,782 | 2,782 |
| Total revenue | 13,809 | 445 | 24,951 | 2,782 |
| Operating expenses: | | | | |
| Selling, general and administrative | 73,995 | 53,260 | 273,596 | 119,242 |
| Research and development | 53,821 | 36,075 | 153,893 | 112,696 |
| Total operating expenses | 127,816 | 89,335 | 427,489 | 231,938 |
| Operating loss | (114,007) | (88,890) | (402,538) | (229,156) |
| Other income (expense): | | | | |
| Interest expense | (7,131) | - | (14,196) | - |
| Other income, net | 1,096 | 637 | 3,904 | 2,727 |
| Total other income (expense) | (6,035) | 637 | (10,292) | 2,727 |
| Net loss | $ (120,042) | $ (88,253) | $ (412,830) | $ (226,429) |
| Net loss per common and potential common share: | | | | |
| Basic and diluted | $ (4.84) | $ (3.62) | $ (16.74) | $ (9.56) |
| Weighted average common and potential common shares outstanding: | | | | |
| Basic and diluted | 24,812 | 24,351 | 24,663 | 23,694 |

**Condensed Consolidated Balance Sheet Information**
*(In thousands)*

| | December 31, 2016 | December 31, 2015 |
|---|---|---|
| Cash, cash equivalents and investment securities | $ 689,385 | $ 628,055 |
| Total assets | $ 739,253 | $ 655,758 |
| Deferred revenue, total | $ 10,147 | $ 8,018 |
| Total liabilities | $ 424,321 | $ 53,609 |
| Stockholders' equity | $ 314,932 | $ 602,149 |

**Reconciliation of GAAP to Non-GAAP Operating Expense**
*(In thousands)*

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | 2016 | 2015 | 2016 | 2015 |
| Total operating expense | $ 127,816 | $ 89,335 | $ 427,489 | $ 231,938 |
| Adjustments: | | | | |
| Stock based compensation | 19,164 | 12,151 | 46,205 | 34,189 |
| Depreciation | 1,644 | 632 | 3,831 | 1,691 |
| Litigation settlement | - | - | 45,000 | - |
| Adjusted operating expense | $ 107,008 | $ 76,552 | $ 332,453 | $ 196,058 |

CONTACT: For more information about Intercept Pharmaceuticals, please contact: Intercept Pharmaceuticals:
Mark Vignola

+1-646-747-1000
investors@interceptpharma.com

Media inquiries: media@interceptpharma.com Investor inquiries: investors@interceptpharma.com