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Intercept Announces Positive Results from Phase 2 AESOP Trial Evaluating OCA for the Treatment of Patients with Primary Sclerosing Cholangitis at The Liver Meeting® 2017

- *OCA met the primary endpoint of alkaline phosphatase (ALP) reduction at 24 weeks*
- *Results presented in a late-breaking oral session*

NEW YORK, Oct. 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 announced additional results from the Phase 2 AESOP trial evaluating the investigational therapy obeticholic acid (OCA) for the treatment of patients with primary sclerosing cholangitis (PSC). These data were presented by lead investigator Kris Kowdley, M.D., Director, Liver Care Network and Organ Care Research, Swedish Medical Center, Seattle, in a late-breaking oral session at The Liver Meeting® 2017, the Annual Meeting of the American Association for the Study of Liver Diseases (AASLD), in Washington, D.C.

AESOP is a 24-week, double-blind, placebo-controlled, dose-ranging trial evaluating the efficacy and safety of OCA compared to placebo in 77 patients with PSC. Patients were randomized to one of three treatment groups: placebo, OCA 1.5-3 mg, and OCA 5-10 mg (with dose titration occurring at the 12-week midpoint).

OCA achieved the primary endpoint of the AESOP trial: patients receiving 5 mg of OCA daily with the option to titrate to 10 mg achieved a statistically significant reduction in alkaline phosphatase (ALP) as compared to placebo at week 24 ($p<0.05$). The results from this dose-ranging study suggest that 5 mg may be the optimal titrated dose of OCA in this patient population.

(U/L)	Placebo (N = 25)	OCA 1.5-3 mg (N = 25)	OCA 5-10 mg (N = 26)
Mean Baseline ALP	563	423	429
Least Squares (LS) Mean Change from Baseline in ALP at Week 12	-53	-57	-135*
LS Mean Change from Baseline in ALP at Week 24	-27	-105	-110*†
LS Mean Percent Change from Baseline at Week 24	+1%	-22%*	-22%*

* $p<0.05$
† Primary endpoint was ALP change for OCA 5-10 mg compared to placebo at week 24.

Patients in the OCA 1.5-3 mg group also achieved statistically significant reductions in ALP versus placebo as measured by LS mean percent change from baseline at week 24. By week 24, ALP increased 1% in the placebo group and decreased by 22% in both the OCA 1.5-3 mg and OCA 5-10 mg groups ($p<0.05$).

There are currently no approved medications for PSC. Some patients are treated with ursodeoxycholic acid (UDCA) even though the AASLD treatment guidelines for PSC recommend against its use. In AESOP, a significant proportion of patients used UDCA, with 48%, 48% and 46% of patients on placebo, OCA 1.5-3 mg and OCA 5-10 mg, respectively, receiving UDCA at baseline.

In a post-hoc analysis examining the effects of OCA in the presence and absence of UDCA, ALP reductions were observed with OCA regardless of treatment with UDCA. Patients receiving OCA monotherapy had greater reductions in ALP at week 12 and week 24 as compared to patients who received OCA in addition to UDCA. At week 12, patients in the OCA 5-10 mg group receiving OCA monotherapy achieved a 30% LS mean reduction in ALP as compared to a 16% reduction in patients receiving OCA in combination with UDCA. At week 24, LS mean reductions in ALP in the OCA 5-10 mg group were 25% for patients receiving OCA monotherapy and 14% for patients receiving OCA in combination with UDCA.

	- UDCA			+ UDCA		
	Placebo	OCA 1.5-3 mg	OCA 5-10 mg	Placebo	OCA 1.5-3 mg	OCA 5-10 mg
LS Mean Percent Change from Baseline in ALP at						

Week 12	-5%	-12%	-30%	-1%	-1%	-16%
LS Mean Percent Change from Baseline in ALP at Week 24	-7%	-19%	-25%	19%	-15%	-14%

Pruritus is a common symptom of PSC and was the most common adverse event observed in AESOP, occurring in 46%, 60% and 67% of patients in the placebo, OCA 1.5-3 mg and OCA 5-10 mg groups, respectively.

A two-year open-label extension of AESOP remains ongoing. Of those patients who completed the double-blind phase of the AESOP trial, 97% chose to participate in the open-label extension phase.

"There is an urgent need for effective therapies in PSC, a rare cholestatic liver disease which can lead to cirrhosis, cholangiocarcinoma and premature mortality," said Dr. Kowdley. "These proof-of-concept results from the AESOP trial are encouraging and represent an important contribution to the growing momentum in PSC research. Further analyses of the AESOP results will help us better understand the effects of OCA in key subpopulations of interest."

About AESOP
AESOP is a Phase 2 randomized, double-blind, placebo-controlled, dose-finding evaluation of the efficacy and safety of 24 weeks of treatment with obeticholic acid (OCA) compared to placebo in 77 patients with PSC. The primary endpoint of the AESOP trial is the LS mean change in serum alkaline phosphatase (ALP) levels, as compared to placebo. Patients with well-controlled irritable bowel disease (IBD) at baseline were permitted to enroll in the AESOP trial and patients receiving ursodeoxycholic acid (UDCA) treatment at baseline (approximately 50%) were permitted to continue on a stable dose.

About Primary Sclerosing Cholangitis
Primary sclerosing cholangitis (PSC) is a rare, life-threatening, chronic cholestatic liver disease characterized by progressive destruction of bile ducts that leads to the development of cirrhosis and end-stage liver disease or cancer in a majority of patients. There are no approved therapies for PSC, and estimated survival time from PSC diagnosis to death or liver transplant is 14.5 years. Approximately 65% of PSC patients are male, and 60%-80% of patients have concomitant inflammatory bowel disease (IBD), most often ulcerative colitis. Although it is a rare disease, PSC is the seventh leading indication for liver transplant in adults in the United States.

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 now 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.

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, regarding the epidemiology and prevalence of PSC, the potential of OCA to treat patients with PSC, the potential utility of the data from the Phase 2 AESOP trial and the endpoints used in the trial, and our 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: the potential benefit and commercial potential of 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; 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 its products and 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 received from payors; the success of competing drugs that are or become available; regulatory developments in the United States and other countries; the performance of third-party suppliers and manufacturers; 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; Intercept's need for and ability to obtain additional financing; Intercept's estimates regarding expenses, revenues and capital requirements and the accuracy thereof; Intercept's use of cash and short-term investments; 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, 2016 filed on March 1, 2017 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.

Contact
For more information about Intercept Pharmaceuticals, please contact:

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

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