July 31, 2017

Intercept Announces Positive Results from the Phase 2 AESOP Trial Evaluating OCA for the Treatment of Patients with Primary Sclerosing Cholangitis

- *OCA met the primary endpoint of alkaline phosphatase (ALP) reduction at 24 weeks*
- *AESOP represents a successful proof of concept for OCA in a second cholestatic liver disease*

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

NEW YORK, July 31, 2017 (GLOBE NEWSWIRE) -- Intercept Pharmaceuticals, Inc. (Nasdaq:ICPT) (Intercept), a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, today announced that the Phase 2 AESOP trial evaluating obeticholic acid (OCA) for the treatment of patients with primary sclerosing cholangitis (PSC) met its primary endpoint. Patients who initiated OCA 5 mg with the option to titrate to 10 mg achieved a statistically significant reduction in alkaline phosphatase (ALP) as compared to placebo ($p<0.05$).

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, followed by a two-year long term safety extension (LTSE) open-label phase which is currently ongoing. 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). Approximately half the patients were receiving ursodeoxycholic acid (UDCA) treatment at baseline and continued on a stable dose during the trial. The primary endpoint of the study was the change in ALP relative to placebo at week 24 for the OCA 5 — 10 mg group. Results for the intent-to-treat population are shown below.

(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.

Pruritus is a common symptom of PSC and was the most common adverse event observed in the AESOP trial, occurring in 46%, 60% and 67% of patients in the placebo, OCA 1.5 — 3 mg and OCA 5 — 10 mg groups, respectively. Pruritus severity increased with OCA treatment in a dose-dependent manner. One (4%) patient in the OCA 1.5 — 3 mg group and three (12%) in the 5 — 10 mg group discontinued treatment due to pruritus compared to none with placebo.

Other treatment emergent adverse events were similar across all three arms and the proportion of patients completing the double-blind period was similar across treatment groups (84%, 76% and 81% for placebo, OCA 1.5-3 mg and OCA 5-10 mg, respectively). Of these patients, 59 of 61 (97%) chose to participate in the LTSE phase.

"Currently there are no therapies proven to be effective in treating patients with PSC, a chronic liver disease that results in significant morbidity and mortality," said Kris V. Kowdley, MD, Director, Liver Care Network and Organ Care Research, Swedish Medical Center, Seattle, and one of the lead investigators of the trial. "The results of this Phase 2 study of OCA are encouraging because patients achieved improvement in ALP, a marker of chronic cholestasis. There is a pressing need for innovation in this disease and, given these results, additional studies are warranted to better define the role of OCA as a treatment for PSC."

"These results provide proof of concept for OCA in a second cholestatic liver disease with a very high unmet need," said David Shapiro, M.D., Chief Medical Officer of Intercept. "We look forward to sharing the complete results from AESOP with the hepatology community at an upcoming scientific congress. We believe these data warrant further investigation and look forward to speaking with PSC thought leaders and regulators to help inform our future development plans."

Conference Call on July 31st at 8:30 a.m. ET
Intercept will discuss the AESOP results during its second quarter 2017 financial results conference call and webcast on July 31st at 8:30 a.m. ET. The live event will be available on the investor page of Intercept's 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 Intercept's website approximately two hours after the completion of the call and will be archived for two weeks.

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% of patients) 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.[1-4] There are no approved therapies for PSC[5], and estimated survival time from PSC diagnosis to death or liver transplant is 14.5 years.[6] Approximately 65% of PSC patients are male[4], and 60%-80% of patients have concomitant inflammatory bowel disease (IBD), most often ulcerative colitis.[4,5,7] Although it is a rare disease, PSC is the seventh leading indication for liver transplant in adults in the United States.[8,9]

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 about Intercept, please visit www.interceptpharma.com.

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 the epidemiology and prevalence of PSC, the potential utility of the endpoints used in the AESOP trial, the potential of OCA to treat patients with PSC, 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.

References

1. Molodecky NA, Kareemi H, Parab R, et al. Incidence of primary sclerosing cholangitis: A systematic review and meta-

analysis. *Hepatology*. 2011;53:1590-1599.
2. Chapman R, Fevery J, Kalloo A, et al. Diagnosis and management of primary sclerosing cholangitis. *Hepatology*. 2010;51(2):660-678.
3. Karlsen TH, Boberg KM. Update on primary sclerosing cholangitis. *J Hepatol*. 2013;59:571-582.
4. Bowlus CL. In: Forman LM, ed. *Primary Sclerosing Cholangitis: Current Understanding, Management, and Future Developments*. Switzerland: Springer International Publishing; 2017:1-11.
5. Horsley-Silva JL, Rodriguez EA, Franco DL, Lindor KD. An update on cancer risk and surveillance in primary sclerosing cholangitis. *Liver Int.* 2016; doi:10.1111/liv.13354.
6. Weismüller TJ, Trivedi PJ, Bergquist A, et al. Patient age, sex, and inflammatory bowel disease phenotype associate with course of primary sclerosing cholangitis. *Gastroenterology*. 2017;152(8):1975-1984.
7. Hirschfield GM, Karlsen TH, Lindor KD, Adams DH. Primary sclerosing cholangitis. *Lancet*. 2013;382:1587-1599.
8. Adam R, et al. Evolution of indications and results of liver transplantation in Europe. A report from the European Liver Transplant Registry (ELTR). *J Hepatol*. 2012 Sep;57(3):675-88.
9. Singal AK, et al. Evolving frequency and outcomes of simultaneous liver kidney transplants based on liver disease etiology. *Transplantation*. 2014 Jul 27;98(2):216-21.

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