

Intercept Pharmaceuticals Announces Positive Phase II Results for INT-747 as a Treatment of Primary Biliary Cirrhosis

NEW YORK, Oct. 27 /PRNewswire/ -- Intercept Pharmaceuticals, Inc., today announced positive results from a 165 patient, placebo controlled, double-blind Phase II clinical trial of INT-747 in patients with primary biliary cirrhosis (PBC). The study evaluated the effects of adding one of three doses of INT-747 or placebo to ursodeoxycholic acid (UDCA) therapy in patients who did not respond adequately to UDCA therapy alone. All three doses of INT-747 added to UDCA produced a statistically highly significant reduction in alkaline phosphatase (Alk Phos) levels, the primary endpoint at the end of the 12-week treatment period, as compared to patients receiving placebo and UDCA. Alk Phos is a liver enzyme routinely used to evaluate the clinical status and disease progression of PBC patients. All the INT-747 doses added to UDCA produced 20% or greater reductions in Alk Phos, with similar significant falls in other clinical liver enzymes. With the exception of a higher incidence of pruritus (itching) in the two top INT-747 dose groups, adverse events were generally similar across all groups.

PBC is a chronic autoimmune disease of the liver marked by the slow progressive destruction of the small bile ducts within the liver, which may lead to liver failure and the need for liver transplantation. PBC primarily afflicts women with up to 300,000 patients estimated worldwide.

Keith Lindor, MD, Dean of the Medical School at Mayo Clinic and a key investigator in the study, commented, "UDCA is the only drug currently approved for PBC and it does not adequately treat the disease in many patients. There is a real need for novel therapies and the clinically meaningful results from this study are supportive of INT-747's potential as an effective drug for PBC."

Mark Pruzanski, MD, Intercept's President and Chief Executive Officer, added, "The remarkable performance of INT-747 in this study, together with the previously announced positive Phase II study results in type 2 diabetes patients with nonalcoholic fatty liver disease, supports INT-747's potential as a novel therapeutic across a range of chronic liver diseases. We intend to present the complete data set from the study at an appropriate scientific meeting in 2010."

Intercept's Chief Medical Officer, David Shapiro, MD, commented, "This study involved more than 30 centers in eight countries and has given us a better understanding of the needs of PBC patients, as well as a good working relationship with the hepatologists looking after them across North America and Europe. With this PBC network in place, we are well positioned to conduct a Phase III program. We intend to request an End of Phase II Meeting with FDA to review the results and plans for a Phase III program."

About INT-747 (first-in-class FXR agonist)

INT-747 is a potent, first-in-class farnesoid X receptor (FXR) agonist derived from the primary human bile acid chenodeoxycholic acid (CDCA), the natural endogenous FXR agonist. INT-747 is formulated as an orally bioavailable drug given once daily. Bile acid signaling through FXR has been shown to regulate the regenerative properties of the liver and, in numerous animal models of liver disease, INT-747 treatment prevents, and even reverses, liver damage caused by progressive fibrosis (scarring). FXR is also expressed in the intestine and kidney and INT-747 exhibits similar protective antifibrotic effects in models of inflammatory bowel disease and diabetic nephropathy.

About Intercept Pharmaceuticals

Intercept is a clinical stage biopharmaceutical company focused on discovering and developing small molecule drugs for the treatment of chronic fibrotic and metabolic diseases. The company's most advanced programs are focused on the development of modified bile acids that are selective for FXR, a nuclear receptor, and TGR5, a G protein-coupled receptor. Intercept's lead drug candidate, the FXR agonist INT-747, is currently being advanced for chronic liver disease indications and the company has been granted orphan status in the US for the PBC

indication. The company's next candidate in the pipeline, INT-777, is a selective TGR5 agonist being advanced to an IND, projected for the second quarter of 2010.

For more information about Intercept, please go to www.interceptpharma.com, and for information about Intercept's lead investor, Genextra S.p.A., please go to www.genextra.it.

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