

Intercept Pharmaceuticals Announces Positive Phase II Results for Obeticholic Acid (INT-747) as Monotherapy in Primary Biliary Cirrhosis

NEW YORK, March 31, 2011, /PRNewswire/ -- Intercept Pharmaceuticals, Inc., today announced positive results from a 59 patient, placebo controlled, double-blind Phase II clinical trial of obeticholic acid (OCA) given as monotherapy to patients with primary biliary cirrhosis (PBC). The study evaluated the effects of 10 mg and 50 mg of OCA compared with placebo in patients with elevated alkaline phosphatase (AP). AP is a liver enzyme routinely used to evaluate the clinical status and disease progression of PBC patients.

At the end of the 12 week treatment period, both doses of OCA produced statistically highly significant reductions in AP, the primary endpoint, compared with the patients receiving placebo (changes: 10mg: -45%; 50mg: -38%; placebo: 0%; $p < 0.0001$ both doses versus placebo). There were also significant improvements in other liver enzymes, including gamma-glutamyl transferase. In addition, serum markers of inflammation and immunity also improved with significant reductions of C-reactive protein (CRP), and immunoglobulin M (IgM), which is closely associated with the autoimmune dysfunction in PBC. Pruritus (itch) was seen more commonly in the OCA treated patients and increased with dose; otherwise, all other adverse events were generally similar across the 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. There is only one drug approved to treat the disease, ursodeoxycholic acid (UDCA), and up to 50% of PBC patients on UDCA therapy continue to be at significant risk of progression to cirrhosis.

Kris V. Kowdley, MD, Director of the Center for Liver Disease at Virginia Mason Medical Center, in Seattle, Washington and a principal investigator in the study, commented, "There is a real need for novel therapies in PBC. The clinically meaningful results from this study are highly supportive of OCA's potential as an effective new drug. We know now that OCA is effective given both in addition to UDCA and, from this study, when given alone. The reductions in AP levels were very substantial and occurred within a month. The effects on IgM and inflammatory markers suggest that OCA may have disease modifying properties."

Intercept's Chief Medical Officer, David Shapiro, MD, commented, "This study involved 20 centers in 7 countries and has given us and the PBC thought leaders involved a better understanding of the potent mechanism of action of OCA. With the international PBC network we have established, we are well positioned to conduct a Phase III program. We are currently discussing requirements for approval with FDA and have received guidance from the EMA supportive of our proposed program."

Mark Pruzanski, MD, Intercept's Chief Executive Officer, added, "The remarkable results achieved with OCA treatment in this study, together with the previously announced positive Phase II results with our drug added to UDCA in refractory PBC patients and in diabetic patients with nonalcoholic fatty liver disease, supports OCA's potential as a novel hepatoprotective drug that may help preserve liver function in patients with a variety of chronic liver diseases."

About Obeticholic Acid (OCA or INT-747)

OCA is a potent, first-in-class farnesoid X receptor (FXR) agonist derived from the primary human bile acid chenodeoxycholic acid, the natural endogenous FXR agonist. Intercept has announced positive Phase II results from randomized clinical trials in patients with primary biliary cirrhosis (PBC), the most common autoimmune liver disease, and in type 2 diabetics with nonalcoholic fatty liver disease. These clinical data and OCA's mechanism of action support its potential as a novel hepatoprotective agent in a broad range of chronic liver diseases. Intercept is currently preparing for the initiation of a Phase III PBC program in the U.S. and Europe and OCA is also being evaluated in a large NIDDK-run placebo-controlled nonalcoholic steatohepatitis (NASH) trial that recently started enrolling in the US.

About Intercept Pharmaceuticals

Intercept is a biopharmaceutical company focused on discovering and developing small molecule drugs for the treatment of chronic liver 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. Bile acid signaling through these receptors regulates key aspects of lipid, glucose and overall energy metabolism, while also serving to maintain the functional integrity of the liver, intestine and kidneys, organs that are exposed to bile acid flux.

For more information about Intercept, please go to www.interceptpharma.com. CONTACT: Mark Pruzanski, M.D. or Barbara Duncan, both of Intercept, +1-646-747-1000. For information about Intercept's majority shareholder Genextra S.p.A., please go to www.genextra.it.