Intercept Pharmaceuticals Receives FDA Orphan Drug Designation for the Fixed-Dose Combination of OCA and Bezafibrate for the Treatment of Primary Biliary Cholangitis (PBC)

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Company on track to complete planned interim analyses from two ongoing Phase 2 studies of the OCA-bezafibrate combination in 2023

Results from planned interim analysis of Phase 2 study evaluating the effects of OCA and bezafibrate on serum biomarkers in PBC to be presented at EASL Congress 2023

MORRISTOWN, N.J., May 16, 2023 (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 that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for the fixed-dose combination of obeticholic acid (OCA) and bezafibrate, a peroxisome proliferator-activated receptor (PPAR) agonist, for the potential treatment of individuals with primary biliary cholangitis (PBC).

“We are pleased that the FDA has granted orphan drug designation for the fixed-dose combination of OCA-bezafibrate, an important component of our long-term strategy and ongoing commitment to people living with PBC,” said M. Michelle Berrey, MD, MPH, President of Research & Development and Chief Medical Officer of Intercept. “This designation represents a milestone in the development of the OCA-bezafibrate fixed-dose combination, which we believe provides the potential to establish best-in-class clinical benefits and further improve the treatment of PBC.”

Intercept has two ongoing Phase 2 studies (747-213 / NCT04594694, 747-214 / NCT05239468) that are designed to explore a range of therapeutic doses for the combination of OCA and bezafibrate. The company expects to complete planned interim analyses from both ongoing Phase 2 studies in 2023, with the first data being presented at the 2023 European Association for the Study of the Liver (EASL) Congress, which will be held from June 21-24, 2023, in Vienna, Austria. The planned interim analyses from these Phase 2 studies, in addition to Phase 1 and preclinical data, will serve as the basis of a potential end-of-phase 2 meeting with the FDA.

FDA's Office of Orphan Drug Products grants orphan status to support the development of medicines for rare disorders that affect fewer than 200,000 people in the U.S. Orphan drug designation provides certain benefits, including market exclusivity upon regulatory approval, exemption of FDA application fees, and tax credits for qualified clinical trials.

About the Investigational OCA-Bezafibrate Fixed-Dose Combination
Intercept is investigating a fixed-dose combination of OCA and bezafibrate for the potential treatment of individuals with PBC. The OCA-bezafibrate combination is investigational; safety and efficacy have not been established. OCA, a farnesoid X receptor (FXR) agonist, is marketed by Intercept as Ocaliva in the United States for the treatment of PBC. Bezafibrate, a pan-peroxisome proliferator-activated receptor (pan-PPAR) agonist, is not approved in the United States for any indication.

FXR and PPAR are distinct pathways that each play a role in PBC. Simultaneously targeting both pathways may offer the greatest potential to impact bile acid synthesis, metabolism, and clearance that underlie cholestatic liver diseases. Published studies establish a clinical proof-of-concept that suggests that the combination of OCA and bezafibrate may provide additive clinical efficacy and tolerability benefits in the treatment of PBC.

About Primary Biliary Cholangitis
Primary biliary cholangitis (PBC) is a rare, progressive, and chronic autoimmune disease that affects the bile ducts in the liver and is most prevalent (approximately 1 in 10,000) in women over the age of 40. PBC causes bile acid to build up in the liver, resulting in inflammation and scarring (fibrosis), which, if left untreated, can lead to cirrhosis, a liver transplant, or death.

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), and severe alcohol-associated hepatitis (sAH). For more information, please visit www.interceptpharma.com or connect with the Company on Twitter and LinkedIn.

Forward Looking Statements
This press release contains forward-looking statements (“FLS”), including regarding:

- the progress, timing, and results of our clinical trials;
- the safety and efficacy of our products and product candidates; and
- the timing and results of our interactions with regulators, including meetings with regulators, and the contents and potential acceptance of our regulatory filings.

Important factors could cause actual results to differ materially from the FLS. For example:

- any future determination that the regulatory applications and subsequent information that we submit for our products or product candidates do not contain adequate clinical or other data or otherwise meet regulatory requirements to obtain or maintain approval;
- any potential side effects associated with our products or product candidates that could delay or prevent approval, require that an approved product be taken off the market, require the inclusion of safety warnings or precautions, or otherwise limit the sale of such product or product candidate;

- the initiation, timing, cost, conduct, progress, and results of our research and development activities, preclinical studies, and clinical trials, including any issues, delays, or failures in identifying patients, enrolling patients, treating patients, retaining patients, meeting specific endpoints, or completing and timely reporting clinical trial results;
- the outcomes of interactions with regulators regarding our clinical trials;
- our ability to identify, develop, and successfully commercialize our products and product candidates; and
- our ability to obtain and maintain intellectual property protection for our products and product candidates, including our ability to cost-effectively file, prosecute, defend, and enforce any patent claims or other intellectual property rights.

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