March 2, 2017

# NICE Recommends Ocaliva® (obeticholic acid) for the Treatment of Patients with Primary Biliary Cholangitis in England, Wales and Northern Ireland

*First new medication for primary biliary cholangitis in nearly 20 years*

*Rapid NICE approval only two months after marketing authorization in the EU; one of the fastest approvals to date for an orphan medicine*

NEW YORK, March 02, 2017 (GLOBE NEWSWIRE) -- Intercept Pharmaceuticals, Inc. (Nasdaq:ICPT) (Intercept) today announced that the National Institute for Health and Care Excellence (NICE) has approved Ocaliva (obeticholic acid) for routine use by the National Health Service (NHS) in England, Wales and Northern Ireland. Ocaliva has been conditionally approved in the European Union for the treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA. The NHS is expected to make Ocaliva available to patients with PBC within 90 days of NICE's final appraisal publication and Intercept will work with local reimbursement authorities to help ensure eligible patients obtain access.

Although it is a rare disease, PBC is a leading cause of liver transplantation in adult women in the UK. Ocaliva is a new treatment option for patients with PBC who do not fully respond to, or are intolerant to, current treatment and remain at risk of their disease progressing toward cirrhosis, liver transplantation or death.

"I am excited to see that the substantial group of PBC patients who are not achieving treatment goals with UDCA alone or who cannot tolerate UDCA will soon be able to access the first new therapeutic option in nearly 20 years. This truly is good news for our PBC patients," said David Jones, M.D., Ph.D., Professor of Liver Immunology at Newcastle University and Consultant Hepatologist at Newcastle upon Tyne Hospitals Trust, which hosts one of Europe's leading clinical services in the disease. "The development of new treatments for PBC is a powerful example of the medical innovation that can occur when government, industry, academia, community clinicians and, most importantly, patients come together to address an unmet need."

Ocaliva is a potent and selective agonist of the farnesoid X receptor (FXR), which is expressed at high levels in the liver and intestine and thought to be a key regulator of bile acid, inflammatory, fibrotic and metabolic pathways. In December 2016, Ocaliva received conditional marketing authorization in Europe based on efficacy and safety data derived from three randomized double-blind, placebo-controlled clinical trials evaluating the effect of Ocaliva on alkaline phosphatase (ALP) and bilirubin in patients with PBC. The marketing authorization was also supported by two clinical databases that include more than 10,000 patients from the Global PBC Study Group and UK-PBC Group, both independently confirming that achieving lower ALP and/or bilirubin levels is significantly correlated with increased transplant-free survival.

The most commonly reported adverse reactions were pruritus (63%) and fatigue (22%). Adverse reactions leading to discontinuation were 1% in the Ocaliva titration arm and 11% in the Ocaliva 10 mg arm. The most common adverse reaction leading to discontinuation was pruritus. The majority of pruritus occurred within the first month of treatment and tended to resolve over time with continued dosing.

"This very rapid decision by NICE, one of the fastest approvals to date for an orphan medication, is an important affirmation of the scientific innovation, clinical value and cost-effectiveness of Ocaliva by one of the most respected health technology assessment bodies," said Lisa Bright, Intercept's President, International. "We welcome NICE's decision to provide broad access to Ocaliva and we owe a tremendous debt to people living with PBC and the clinical groups who helped us to achieve this milestone for the PBC community."

"It is exciting news for PBC patients that this new treatment option will now be routinely available in England, Wales and Northern Ireland," said Collette Thain MBE, CEO of The PBC Foundation. "When I was diagnosed with PBC, UDCA was the only approved treatment option and PBC wasn't a major priority for many researchers. Thankfully, so much has changed for people living with PBC since then. After decades of advocacy from the PBC community, we have a new treatment option, a growing awareness of the disease among the general public, greater expertise amongst clinicians and an acceleration of PBC research in the UK and around the globe."

**About Primary Biliary Cholangitis**

Primary biliary cholangitis (PBC) is a rare, autoimmune cholestatic liver disease that puts patients at risk for life-threatening complications. PBC is primarily a disease of women, afflicting approximately one in 1,000 women over the age of 40. If left untreated, survival of PBC patients is significantly worse than the general population.

**PBC in the UK**

The estimated prevalence of PBC in the UK is approximately 3.9 per 10,000 population, equating to approximately 19,175 people in England.

Patients and medical leaders in the UK have played a critical role in accelerating PBC research, innovation and awareness globally. The patient community in the UK has guided Intercept's efforts to improve PBC education and understand the unmet needs of patients and their families. The UK-PBC Study Group, a research consortium funded by the UK government through the Medical Research Council and National Institute for Health Research, played a critical role in the development of Ocaliva and was one of two clinical databases to independently confirm that achieving lower ALP and/or bilirubin levels is significantly correlated with increased transplant-free survival.

**About Ocaliva® (obeticholic acid)**

Ocaliva (obeticholic acid) is a potent and highly selective agonist of the farnesoid X receptor (FXR), a nuclear receptor expressed in the liver and intestine. FXR is a key regulator of bile acid, inflammatory, fibrotic and metabolic pathways.

In December 2016, Ocaliva received conditional marketing authorization in Europe for the treatment of PBC in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA, conditional to the company providing further data post-approval to confirm benefit. In May 2016, the U.S. Food and Drug Administration granted accelerated approval to Ocaliva for the treatment of PBC. For full prescribing information in the U.S., visit Ocaliva.com.

**EU IMPORTANT SAFETY INFORMATION**

**Contraindications**

Hypersensitivity to the active substance or to any of the excipients and complete biliary obstruction.

**Warnings and Precautions**

Elevations in alanine amino transferase (ALT) and aspartate aminotransferase (AST) have been observed in patients taking obeticholic acid. Clinical signs and symptoms of hepatic decompensation have also been observed. These events have occurred as early as within the first month of treatment. Liver-related adverse events have primarily been observed at doses higher than the maximum recommended dose of 10 mg once daily. Patients should be monitored during treatment with Ocaliva for elevations in liver biochemical tests and for the development of liver-related adverse events. Dosage adjustments are needed for patients with moderate (Child-Pugh Class B) or severe (Child-Pugh Class C) hepatic impairment.

Severe pruritus was reported in 23% of patients treated with Ocaliva 10 mg arm, 19% of patients in the Ocaliva titration arm and 7% of patients in the placebo arms. The median time to onset of severe pruritus was 11, 158 and 75 days for patients in the Ocaliva 10 mg, Ocaliva titration and placebo arms, respectively. Management strategies include the addition of bile acid binding resins or antihistamines, dose reduction, reduced dosing frequency and/or temporary dose interruption.

**Adverse Reactions**

The most commonly reported adverse reactions were pruritus (63%) and fatigue (22%). Other common adverse reactions observed in clinical trials (> 5%) were abdominal pain and discomfort, rash, oropharyngeal pain, dizziness, constipation, arthralgia, thyroid function abnormality and eczema.

**Drug Interaction**

Bile acid binding resins such as cholestyramine, colestipol or colesevelam adsorb and reduce bile acid absorption and may reduce efficacy of obeticholic acid. When concomitant bile acid binding resins are administered, obeticholic acid should be taken at least 4-6 hours before or 4-6 hours after taking a bile acid binding resin, or at as great an interval as possible.

For detailed safety information for Ocaliva (obeticholic acid) 5 mg and 10 mg tablets including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European Summary of

Product Characteristics.

***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. Intercept's International headquarters are located in London. 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 clinical relevance and utility of ALP, bilirubin and the surrogate endpoint used in the Phase 3 POISE trial to predict clinical outcomes, the acceptance of Ocaliva® (obeticholic acid) as a treatment for PBC by healthcare providers, patients and payors, the commercial availability of OCA for the treatment of PBC and timelines related thereto, the anticipated prevalence of and other epidemiological estimates and market data related to PBC, the continued development of OCA and Intercept's other product candidates, 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: Intercept's ability to successfully commercialize 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, including Intercept's development program in NASH; 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 OCA in indications other than PBC and its other 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 that it may receive for its products from payors; the success of competing drugs that are or become available; 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; regulatory developments in the United States and other countries; the performance of third-party suppliers and manufacturers; Intercept's need for and ability to obtain additional financing; Intercept's estimates regarding expenses, future revenues and capital requirements and the accuracy thereof; Intercept's use of cash, short-term investments and the proceeds from the offering; 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.*

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