



May 25, 2017

## **Health Canada Grants Approval for Ocaliva™ (obeticholic acid) for the Treatment of Patients with Primary Biliary Cholangitis (PBC)**

**Ocaliva represents the first new treatment option in over 20 years for PBC, an autoimmune liver disease that is the leading reason for liver transplantation in women in Canada**

NEW YORK, May 25, 2017 (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 Health Canada has granted a conditional approval for Ocaliva (obeticholic acid) for the treatment of primary biliary cholangitis (PBC), when used in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA.

PBC is a rare, progressive, autoimmune cholestatic liver disease that puts patients at risk for life-threatening complications, affecting an estimated 11,000 Canadians. PBC impacts people in the prime of their lives and is the leading cause of liver transplantation among women in Canada.

Ocaliva is a farnesoid X receptor (FXR) agonist that will fill an important unmet need for patients who have an inadequate response to, or are unable to tolerate, the standard of care, UDCA, and therefore remain at significantly increased risk of liver failure, need for liver transplantation, or death.

"A substantial number of PBC patients are not achieving treatment goals with UDCA alone and a few cannot tolerate this standard of care. Until now we have only had experimental adjunctive therapies to help these non-responders with progressive disease," said Andrew Mason, MBS, FRCPI, Director of Research for the Division of Gastroenterology and Hepatology at the University of Alberta. "The introduction of Ocaliva will help to address this critical need and provide an opportunity for physicians to revisit treatment goals with their patients."

Ocaliva has been issued a marketing authorization with conditions (also known as a Notice of Compliance with Conditions or NOC/c) from Health Canada, pending the results of trials to verify its clinical benefit. Products approved under Health Canada's NOC/c policy have demonstrated promising benefit, are of high quality and possess an acceptable safety profile based on a benefit/risk assessment. Further, Health Canada approval follows an accelerated priority review of the Ocaliva New Drug Submission, recognizing the unmet need for new therapies in PBC.

"We are excited to be introducing the first new treatment option for PBC in over 20 years for Canadian patients so closely following regulatory approval in the U.S. and Europe," said Mark Pruzanski, M.D., President and CEO of Intercept. "Health Canada's approval is encouraging news for patients and represents another important step in Intercept's mission to improve the lives of people with progressive non-viral liver diseases."

Intercept is actively pursuing reimbursement of Ocaliva with private insurance carriers and public drug plans across Canada. Intercept is committed to ensuring patients with PBC can access Ocaliva as quickly and easily as possible and has launched the Navigate™ Patient Support Program to provide comprehensive and personalized support for eligible patients prescribed Ocaliva for PBC.

"We are very excited that Canadians living with PBC will now have an important new treatment option," said Gail Wright, President of the Canadian PBC Society. "It is such a promising time for PBC patients, and the community has been energized by new advances in research, growing disease awareness among the public and clinicians and now the introduction of a much-needed new therapy to help patients better manage their disease."

### **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.

### **About Ocaliva™ (obeticholic acid)**

Ocaliva (obeticholic acid) is an 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.

Ocaliva is indicated in Canada 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. Ocaliva has been issued a marketing authorization with conditions from Health Canada, pending the results of trials to verify its clinical benefit.

In May 2016, the U.S. Food and Drug Administration granted accelerated approval to Ocaliva for the treatment of PBC. In December 2016, Ocaliva received conditional marketing authorization in Europe from the European Medicines Authority.

## **CANADIAN IMPORTANT SAFETY INFORMATION**

### **CONTRAINDICATIONS**

Patients who are hypersensitive to this drug or to any ingredient in the formulation or component of the container. Ocaliva is contraindicated in patients with complete biliary obstruction.

### **WARNINGS AND PRECAUTIONS**

#### **Liver-Related Adverse Reactions**

In two 3-month, placebo-controlled clinical trials, a dose-response relationship was observed for the occurrence of liver-related adverse reactions including jaundice, worsening ascites and primary biliary cholangitis flare with dosages of Ocaliva of 10 mg once daily to 50 mg once daily (up to 5-times the highest recommended dosage), as early as one month after starting treatment with Ocaliva.

Monitor patients during treatment with Ocaliva for elevations in liver biochemical tests and for the development of liver-related adverse reactions. Weigh the potential risks against the benefits of continuing treatment with Ocaliva in patients who have experienced clinically significant liver-related adverse reactions. Discontinue Ocaliva in patients who develop complete biliary obstruction.

#### **Severe Pruritus**

Pruritus was mostly mild to moderate in severity and generally started within the first month following the initiation of treatment with Ocaliva and decreased in severity over time with continued dosing. Severe pruritus was reported in 23% of patients in the Ocaliva 10 mg arm, 19% of patients in the Ocaliva titration arm, and 7% of patients in the placebo arm, respectively. Management strategies include the addition of bile acid resins or antihistamines, Ocaliva dosage reduction, and/or temporary interruption of Ocaliva dosing.

### **ADVERSE REACTIONS**

The most common adverse drug reactions reported in double-blind clinical trials (frequency $\geq$ 5%) were pruritus, fatigue, constipation, oropharyngeal pain and arthralgia.

For detailed safety information for Ocaliva (obeticholic acid) 5 mg and 10 mg tablets please see the [Product Monograph](#).

#### **About Intercept Pharma Canada Inc.**

Intercept Pharma Canada Inc. is the Canadian subsidiary of Intercept Pharmaceuticals, Inc., founded in 2015 and based in Mississauga, Ontario. Intercept is a proud member of Ontario's biopharmaceutical community, and is committed to helping support the needs of Canada's liver health community.

#### **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](http://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<sup>TM</sup> (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, 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; 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.*

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