



## Intercept Receives Complete Response Letter from FDA for Obeticholic Acid for the Treatment of Fibrosis Due to NASH

June 29, 2020

**Conference call scheduled for 8:30 a.m. ET today**

NEW YORK, June 29, 2020 (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 issued a Complete Response Letter (CRL) regarding the New Drug Application (NDA) for obeticholic acid (OCA) for the treatment of fibrosis due to nonalcoholic steatohepatitis (NASH).

The CRL indicated that, based on the data the FDA has reviewed to date, the Agency has determined that the predicted benefit of OCA based on a surrogate histopathologic endpoint remains uncertain and does not sufficiently outweigh the potential risks to support accelerated approval for the treatment of patients with liver fibrosis due to NASH. The FDA recommends that Intercept submit additional post-interim analysis efficacy and safety data from the ongoing REGENERATE study in support of potential accelerated approval and that the long-term outcomes phase of the study should continue.

"At no point during the review did the FDA communicate that OCA was not approvable on an accelerated basis, and we strongly believe that the totality of data submitted to date both meet the requirements of the Agency's own guidance and clearly support the positive benefit-risk profile of OCA," said Mark Pruzanski, M.D., President and CEO of Intercept. "We are disappointed to see the determination the Agency has reached based on an apparently incomplete review, and without having provided medical experts and patients the opportunity to be heard at the anticipated Adcom on the merits of OCA, which is a designated Breakthrough Therapy. The FDA has progressively increased the complexity of the histologic endpoints, creating a very high bar that only OCA has so far met in a pivotal Phase 3 study. On behalf of the hepatology community, we are very concerned that the Agency's apparently still evolving expectations will make it exceedingly challenging to bring innovative therapies to NASH patients with high unmet medical need. We plan to meet as soon as possible with the FDA to review the CRL and discuss options for an efficient path forward to approval."

Intercept had previously disclosed that, based on the FDA's decision to postpone a tentatively scheduled advisory committee meeting (Adcom), it was expected that the Agency's review of its NDA would extend beyond the PDUFA goal date and that the FDA would move forward with rescheduling the Adcom.

The NDA submission for OCA is the first for NASH and was based on data from 35 clinical trials and more than 1,700 NASH patients treated with the drug. OCA is the only investigational NASH drug with Breakthrough Therapy designation and has uniquely demonstrated reproducible ability to reverse or otherwise stabilize liver fibrosis in patients with advanced fibrosis due to NASH. According to the FDA draft guidance for NASH fibrosis, of the histologic features of NASH, fibrosis is considered the strongest predictor of adverse clinical outcomes, including liver-related death. There is currently no approved therapy for this devastating disease, which has become a leading cause of liver failure and resulting poor clinical outcomes.

### **Conference Call on June 29, 2020 at 8:30 a.m. ET**

The Company will host a conference call today, June 29, at 8:30 a.m. ET to discuss the Complete Response Letter. The conference call will be available on the investor page of our website at <http://ir.interceptpharma.com> or by calling (855) 232-3919 (toll-free domestic) or (315) 625-6894 (international) passcode 5586301. A replay of the call will be available on our website shortly following the completion of the call and will be available for two weeks.

### **About Liver Fibrosis due to NASH**

Nonalcoholic steatohepatitis (NASH) is a serious progressive liver disease caused by excessive fat accumulation in the liver that induces chronic inflammation, resulting in progressive fibrosis (scarring) that can lead to cirrhosis, eventual liver failure, cancer and death. Advanced fibrosis is associated with a substantially higher risk of liver-related morbidity and mortality in patients with NASH. In the United States, NASH is currently the second leading cause for liver transplantation overall, and in females, the leading cause. NASH is anticipated to become the leading indication for liver transplantation in Europe within the next decade. There are currently no medications approved for the treatment of NASH.

### **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) and nonalcoholic steatohepatitis (NASH). Founded in 2002 in New York, Intercept has operations in the United States, Europe and Canada. For more information, please visit [www.interceptpharma.com](http://www.interceptpharma.com) or connect with the company on [Twitter](#) and [LinkedIn](#).

### **Cautionary Note Regarding Forward-Looking Statements**

This press release contains forward-looking statements, including, but not limited to, statements regarding the review of our New Drug Application for obeticholic acid (OCA) for the treatment of fibrosis due to nonalcoholic steatohepatitis (NASH) by the U.S. Food and Drug Administration (FDA), our intent to work with the FDA to address the issues raised in the complete response letter (CRL), as well as our strategy, future operations, future financial position, future revenue, projected costs, financial guidance, prospects, plans and objectives.

These statements constitute forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "possible," "continue" and similar expressions are intended to identify forward-looking

statements, although not all forward-looking statements contain these identifying words. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release, and we undertake no obligation to update any forward-looking statement except as required by law. These forward-looking statements are based on estimates and assumptions by our management that, although believed to be reasonable, are inherently uncertain and subject to a number of risks. The following represent some, but not necessarily all, of the factors that could cause actual results to differ materially from historical results or those anticipated or predicted by our forward-looking statements: our ability to successfully commercialize Ocaliva for PBC; our ability to maintain our regulatory approval of Ocaliva for PBC in the United States, Europe, Canada, Israel, Australia and other jurisdictions in which we have or may receive marketing authorization; our ability to timely and cost-effectively file for and obtain regulatory approval of our product candidates on an accelerated basis or at all, including OCA for liver fibrosis due to NASH following the issuance of the CRL; any advisory committee recommendation or dispute resolution determination that our product candidates, including OCA for liver fibrosis due to NASH, should not be approved or approved only under certain conditions; any future determination that the regulatory applications and subsequent information we submit for our product candidates, including OCA for liver fibrosis due to NASH, do not contain adequate clinical or other data or meet applicable regulatory requirements for approval; conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, including OCA for liver fibrosis due to NASH, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), any risk mitigation programs such as a REMS, and any related restrictions, limitations and/or warnings contained in the label of any of our products or product candidates; any potential side effects associated with Ocaliva for PBC, OCA for liver fibrosis due to NASH or our other 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 in the jurisdictions in which we intend to seek approval or completing and timely reporting the results of our NASH or PBC clinical trials; our ability to establish and maintain relationships with, and the performance of, third-party manufacturers, contract research organizations and other vendors upon whom we are substantially dependent for, among other things, the manufacture and supply of our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our clinical trial activities; our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to successfully launch OCA for liver fibrosis due to NASH, if approved; 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; the size and growth of the markets for our products and product candidates and our ability to serve those markets; the degree of market acceptance of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH or our other product candidates among physicians, patients and healthcare payors; the availability of adequate coverage and reimbursement from governmental and private healthcare payors for our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our ability to obtain adequate pricing for such products; our ability to establish and maintain effective sales, marketing and distribution capabilities, either directly or through collaborations with third parties; competition from existing drugs or new drugs that become available; our ability to prevent system failures, data breaches or violations of data protection laws; costs and outcomes relating to any disputes, governmental inquiries or investigations, regulatory proceedings, legal proceedings or litigation, including any securities, intellectual property, employment, product liability or other litigation; our collaborators' election to pursue research, development and commercialization activities; our ability to establish and maintain relationships with collaborators with development, regulatory and commercialization expertise; our need for and ability to generate or obtain additional financing; our estimates regarding future expenses, revenues and capital requirements and the accuracy thereof; our use of cash and short-term investments; our ability to acquire, license and invest in businesses, technologies, product candidates and products; our ability to attract and retain key personnel to manage our business effectively; our ability to manage the growth of our operations, infrastructure, personnel, systems and controls; our ability to obtain and maintain adequate insurance coverage; the impact of COVID-19, including any impact on our net sales, non-GAAP adjusted operating expenses or financial position, related quarantines and government actions, delays relating to our regulatory applications, disruptions relating to our ongoing clinical trials or involving our contract research organizations, study sites or other clinical partners, disruptions relating to our supply chain or involving our third-party manufacturers, distributors or other distribution partners, facility closures or other restrictions, and the extent and duration thereof; the impact of general U.S. and foreign economic, industry, market, regulatory or political conditions, including the potential impact of Brexit; and the other risks and uncertainties identified in our periodic filings filed with the U.S. Securities and Exchange Commission, including our Annual Report on Form 10-K for the year ended December 31, 2019 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2020.

## Contact

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