INTERCEPT PHARMACEUTICALS INC
Form 8-K
January 10, 2014

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the

Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): January 10, 2014

INTERCEPT PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**001-35668**	**22-3868459**
(state or other jurisdiction of incorporation)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**
450 W. 15th Street, Suite 505 New York, New York		**10011**
(Address of principal executive offices)		**(Zip Code)**

Registrant's telephone number, including area code: (646) 747-1000

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

¨Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
¨Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

¨Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
¨Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Item 7.01 Regulation FD Disclosure.

On January 9, 2014, Intercept Pharmaceuticals, Inc. ("Intercept" or the "Company") provided an update on the Phase 2b clinical trial of obeticholic acid ("OCA") in non-alcoholic steatohepatitis ("NASH"), its clinical programs for OCA in general and certain other matters. The press releases are attached hereto as Exhibits 99.1 and 99.2 and are incorporated by reference into this Item 7.01.

In accordance with General Instruction B-2 of Form 8-K, the information set forth in or incorporated by reference into this Item 7.01 shall not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

Item 8.01 Other Events.

The Company provided the following updates to its development programs.

Primary Endpoint Met in Interim Analysis of Phase 2b Clinical Trial of OCA in NASH

On January 9, 2014, the Company announced that the Phase 2b clinical trial of OCA for the treatment of NASH ("FLINT") has been stopped early for efficacy based on a planned interim analysis showing that the primary endpoint of the trial has been met.

The decision to stop FLINT has been based on the recommendation of the Data Safety Monitoring Board of the National Institute of Diabetes and Digestive and Kidney Diseases ("NIDDK"), which reviewed liver biopsy data from before and at the end of the treatment period in approximately half of the 283 randomized patients in accordance with a planned interim efficacy analysis. This analysis demonstrated that OCA treatment resulted in a highly statistically significant improvement ($p=0.0024$ on an intention-to-treat basis) in the primary histological endpoint, defined as a decrease in the NAFLD Activity Score, a system of scoring the histopathological features in the liver ("NAS"), of at least two points with no worsening of fibrosis, as compared to placebo. Those patients who had not yet completed the trial and therefore did not have a second biopsy were treated as non-responders in the intention-to-treat analysis. The pre-defined threshold of statistical significance for stopping FLINT was $p<0.0031$.

FLINT is a multi-center, double-blind, placebo-controlled, Phase 2b clinical trial assessing the safety and efficacy of a 25 mg oral dose of OCA administered daily to adult NASH patients. FLINT enrolled 283 patients at eight leading academic hepatology centers in the United States comprising the NASH clinical research network. Patients were randomized to receive either a 25 mg dose of OCA or placebo for 72 weeks. Patients enrolled in the trial were qualified based on a diagnosis determined by liver biopsy at the start of the trial with a NAS of four or greater and with a score of at least one in each component of the NAS eight point scale (steatosis 0-3, lobular inflammation 0-3, ballooning 0-2). The primary endpoint in the FLINT trial is defined as an improvement of two or more points in the NAS with no worsening of liver fibrosis, as compared to placebo. End of study biopsies were conducted in patients after the 72-week treatment period, with all biopsies centrally scored in a blinded fashion. The trial has been sponsored and conducted by the NIDDK, a part of the National Institutes of Health, which submitted an IND to the FDA for OCA for the treatment of NASH in 2010.

NASH is a serious chronic liver disease caused by excessive fat accumulation in the liver that, for reasons that are still incompletely understood, induces chronic inflammation which leads to progressive fibrosis (scarring) that can lead to cirrhosis, eventual liver failure and death. There are currently no drugs approved for the treatment of NASH. Studies have shown that over a ten year period at least 10% of NASH patients will develop cirrhosis, and liver-related mortality due to this disease is ten-fold that of the general population. According to recent epidemiological studies, it is estimated that approximately 12% of the U.S. adult population has NASH, while 2.7% (potentially more than six million patients) are believed to have advanced liver fibrosis or cirrhosis due to progression of the disease. The proportion of liver transplants attributable to NASH has increased rapidly in past years, and over the next decade the disease is projected to become the leading indication for liver transplant ahead of chronic hepatitis C and alcoholic liver disease.

Double-Blind Phase of POISE Completed; 97% Enrollment into Long-Term Safety Extension Phase

In December 2013, the last patient follow-up visit was completed, marking the conclusion of the double-blind phase of POISE, the double-blind, placebo-controlled Phase 3 clinical trial studying the safety and efficacy of a once-daily dose of OCA in primary biliary cirrhosis ("PBC") patients. Of the 217 patients randomized, 19 patients (or approximately 9%) discontinued early, including seven patients (or approximately 3%) who did so due to pruritus. Top-line results from the double-blind phase of the POISE trial are expected to be available in the second quarter of 2014.

Patients completing the double-blind phase have had the option to continue in an open-label, long-term safety extension phase for another five years, during which all patients will receive OCA treatment with daily doses starting at 5 mg and potentially titrating up to 25 mg a day, as clinically indicated. Of the 198 patients who completed the double-blind phase, more than 95% continued in the long-term safety extension phase of the trial.

Global PBC Study Group

Data from over 6,100 PBC patients collected and pooled by an independent group of 15 academic medical centers across eight countries have been analyzed by the Global PBC Study Group ("Study Group"), and key data were presented at the 2013 AASLD conference by the Study Group. These and additional analyses confirm that the surrogate biochemical endpoint used in POISE (i.e., alkaline phosphatase < 1.67 times upper limit of normal and normal bilirubin) is strongly predictive of clinical outcomes in PBC patients. Specifically, the analyses demonstrated that patients who failed to meet the POISE trial primary endpoint after one year of ursodeoxycholic acid treatment had approximately two times greater chance of dying or requiring a liver transplant.

Based on these additional analyses, Intercept has submitted a design for its anticipated confirmatory trial to the U.S. Food and Drug Administration ("FDA") for review. If subsequent discussions result in general agreement concerning the appropriate design of the trial without undue delay, Intercept intends to initiate the confirmatory trial in the second quarter of 2014.

Completion of PESTO and Planned Initiation of Double-Blind Trial in Portal Hypertension

PESTO is an open-label, multi-center Phase 2a trial evaluating the safety and efficacy of OCA administered to alcoholic cirrhotic patients for approximately seven days at daily doses of 10 mg and 25 mg for the treatment of portal hypertension. Preliminary data from PESTO indicate that approximately 50% of patients evaluated for efficacy in the

combined dose groups demonstrated a clinically significant reduction in hepatic venous pressure gradient (“HVPG”) reflective of lowered risk of variceal bleeds. Systemic mean arterial blood pressure, already adversely low in cirrhotic patients, was unchanged in trial patients at the end of therapy. Detailed results obtained by the lead investigator, Raj Mookerjee, M.D., at the primary center (University College London) conducting the PESTO trial have been submitted for presentation at the upcoming International Liver Congress of the European Association for the Study of the Liver (EASL) in April 2014.

Intercept plans to initiate a multi-center, double-blind, placebo-controlled, randomized Phase 2b clinical trial focusing on HVPG as an endpoint in patients with liver cirrhosis and portal hypertension in the second half of 2014.

Completion of OBADIAH and Planned Initiation of Double-Blind Trial in Secondary Bile Acid Diarrhea

OBADIAH is an investigator-initiated, open-label Phase 2a trial evaluating the safety and efficacy of OCA in the treatment of primary and secondary bile acid diarrhea, with Professor Julian Walters at Imperial College London acting as Principal Investigator. The trial demonstrated that OCA increased levels of fibroblast growth factor 19 ("FGF19") with concomitant clinical improvement over a two-week treatment period in patients with primary bile acid diarrhea and in patients with secondary bile acid diarrhea due to Crohn's disease, with no response shown in a control group consisting of diarrhea-predominant irritable bowel syndrome patients with normal FGF19 levels. The data also show that increased length of prior ileal resection reduced response to OCA treatment in Crohn's patients suffering from secondary bile acid diarrhea. Detailed results from OBADIAH have been submitted for presentation at Digestive Disease Week in May 2014.

Intercept plans to initiate a multi-center, double-blind, placebo-controlled, randomized Phase 2b clinical trial of OCA in Crohn's patients with secondary bile acid diarrhea in the second half of 2014.

Phase 2 Trial in Primary Sclerosing Cholangitis

Intercept plans to initiate a multi-center, double-blind, placebo-controlled, randomized Phase 2 clinical trial of OCA in primary sclerosing cholangitis ("PSC") in the second half of 2014. PSC is a chronic autoimmune liver disease that could eventually lead to cirrhosis, liver failure and death. As with PBC, studies have shown that patients with PSC who have reduced or normal levels of ALP have significantly improved long-term clinical outcomes. Although there is no approved treatment of PSC, patients are commonly treated with ursodiol. The prevalence of PSC is estimated to be approximately one-third that of PBC, with approximately 60% of cases occurring in men and typically 75% of PSC patients also having associated ulcerative colitis.

Phase 1 Trial of INT-767

INT-767 is an orally administered dual FXR and TGR5 agonist that, similar to OCA, is derived from the primary human bile acid chenodeoxycholic acid. Intercept is completing IND-enabling studies for INT-767, with the intention to submit an IND and initiate Phase 1 trials in the fourth quarter of 2014.

The Company is still awaiting detailed data in relation to the completed clinical trials discussed above.

The results of each of the clinical trials described above do not ensure that subsequent clinical trials will generate the same or similar results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. See the Company's risk factors as disclosed in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2012 together with any updates to these risk factors filed from time to time in Intercept's other filings with the Securities and Exchange Commission.

Impact on Capital Resources and Future Funding Requirements

Based upon the Company's current operating plan, after giving effect to the addition of the new development initiatives described above, Intercept's existing cash, cash equivalents and short-term investments are expected to fund operating expenses and capital expenditure requirements into the second half of 2015. This estimate reflects the planned expenditures relating to, among other items, nonclinical studies, clinical trials and other expenditures to support planned regulatory submissions for OCA in PBC at the end of 2014; the anticipated commencement of the open-label, safety extension phase of the Phase 3 trial in PBC in the second quarter of 2014; anticipated pre-commercial activities for OCA in PBC; the anticipated commencement of Phase 2 clinical studies of OCA in portal hypertension, secondary bile acid diarrhea and PSC; and IND-enabling studies and Phase 1 trial of INT-767.

These estimates are based on assumptions that may prove to be wrong and unexpected developments may cause the Company to use its available capital resources sooner than currently expected. Because of the numerous risks and uncertainties associated with the development and commercialization of the Company's product candidates, the Company is unable to estimate the amounts of increased capital outlays and operating expenditures necessary to complete the development of its product candidates.

Future capital requirements will depend on many factors, including:

- the results of the POISE trial and the Company's other clinical trials;
- the data from the POISE trial, the FLINT trial and the Company's other clinical trials;
- the willingness of the FDA and the European Medicines Agency ("EMA") to accept the POISE trial, as well as other completed and planned clinical and preclinical studies and other work, as the basis for review and approval of OCA for PBC;
- the clinical development of OCA for other potential indications;
- the outcome, costs and timing of seeking and obtaining FDA, EMA and any other regulatory approvals;

- the number and characteristics of product candidates that the Company pursues, including product candidates in preclinical development;
- the ability of the Company's product candidates to progress through clinical development successfully;
- the Company's need to expand research and development activities;
- the costs associated with securing and establishing commercialization and manufacturing capabilities;
- the costs of acquiring, licensing or investing in businesses, products, product candidates and technologies;
- the Company's ability to maintain, expand and defend the scope of its intellectual property portfolio, including the amount and timing of any payments the Company may be required to make, or that it may receive, in connection with the licensing, filing, prosecution, defense and enforcement of any patents or other intellectual property rights;
- the Company's need and ability to hire additional qualified management, scientific and medical, commercial and other personnel;
- the effect of competing technological and market developments;
- the Company's need to implement additional internal systems and infrastructure, including financial, reporting and security systems; and
- the economic and other terms, timing and success of the Company's existing licensing arrangements and any collaboration, licensing or other arrangements into which it may enter in the future.

Until such time, if ever, as the Company can generate substantial revenue from product sales, the Company expects to finance its cash needs through a combination of equity offerings, debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements. To the extent that the Company raises additional capital through the sale of equity or convertible debt securities, the ownership interests of common stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of common stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting the Company's ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If the Company raises additional funds through government or other third-party funding, marketing and distribution arrangements or other collaborations, strategic alliances or licensing arrangements with third parties, the Company may have to relinquish valuable rights to its technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to the Company.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

99.1 Press Release dated as of January 9, 2014 (NASH).

99.2 Press Release dated as of January 9, 2014 (general clinical update)

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

INTERCEPT PHARMACEUTICALS, INC.

Date: January 10, 2014 /s/ Mark Pruzanski
Mark Pruzanski, M.D.
President and Chief Executive Officer