CORCEPT THERAPEUTICS INC
Form 10-K
March 06, 2017

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the fiscal year ended December 31, 2016

or

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from to

Commission File Number: 000-50679

CORCEPT THERAPEUTICS INCORPORATED

(Exact Name of Corporation as Specified in Its Charter)

Delaware	77-0487658
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

149 Commonwealth Drive

Menlo Park, CA 94025

(Address of principal executive offices) (zip code)

(650) 327-3270

(Registrant’s telephone number, including area code)

Securities registered pursuant to Section 12 (b) of the Act:

Title of Each Class:	Name of Each Exchange on which Registered:
Common Stock, $0.001 par value	The NASDAQ Capital Market

Securities registered pursuant to Section 12 (g) of the Act:

None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15 (d) of the Act. Yes No

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of the Registrant's knowledge, in definitive proxy or information statements incorporated by reference to Part III of this Form 10-K or any amendment to this Form 10 K.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer or a smaller reporting company. See the definitions of "large accelerated filer", "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer		Accelerated filer
Non-accelerated filer	(Do not check if a smaller reporting company)	Smaller reporting company

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes No

The aggregate market value of voting and non-voting common equity held by non-affiliates of the Registrant was $427,665,971 as of June 30, 2016 based upon the closing price on the NASDAQ Capital Market reported for such date. This calculation does not reflect a determination that certain persons are affiliates of the Registrant for any other purpose.

On February 28, 2017 there were 112,942,391 shares of common stock outstanding at a par value of $0.001 per share.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Registrant's definitive proxy statement for its 2016 Annual Meeting of Stockholders are incorporated by reference in Items 10, 11, 12, 13 and 14 of Part III.

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Form 10-K

For the year ended December 31, 2016

PART I

This Annual Report on Form 10-K (Form 10-K) contains forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended (Exchange Act), and Section 27A of the Securities Act of 1933, as amended (Securities Act). All statements contained in this Form 10-K, other than statements of historical fact, are forward-looking statements. When used in this report or elsewhere by management, the words "believe," "anticipate," "intend," "plan," "estimate," "expect," "may," "will," "should, "would," "could," "seek" and similar expressions a forward-looking statements. Such forward-looking statements are based on current expectations. The absence of these words does not mean that a statement is not forward-looking. Forward-looking statements made in this Form 10-K include, but are not limited to, statements about:

- our ability to manufacture, market and sell Korlym® (mifepristone) 300 mg Tablets;
- our estimates regarding enrollment in and the completion dates of our clinical trials and the anticipated results of these trials;
- the progress and timing of our research and development programs and the regulatory activities associated with them;
- our ability to realize the benefits of Orphan Drug designation for Korlym in the United States;
- our estimates for future performance, including revenue and profits;
- the timing of the market introduction of future product candidates, including new uses for Korlym and any compound in our families of selective cortisol modulators;
- our ability to manufacture, market, commercialize and achieve market acceptance for our future product candidates;
- uncertainties associated with obtaining and enforcing patents; and
- estimates regarding our capital requirements.

Forward-looking statements are not guarantees of future performance. They involve risks and uncertainties. Actual events or results may differ materially from those discussed in the forward-looking statements for many reasons. For a more detailed discussion of the risks and uncertainties that may affect the accuracy of our forward-looking statements, see the "Risk Factors," "Overview" and "Liquidity and Capital Resources" sections of the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this Form 10-K. Forward-looking statements reflect our view only as of the date of this report. Except as required by law, we undertake no obligation to update any forward-looking statement. You should carefully consider the other reports and documents that we file with the Securities and Exchange Commission (SEC).

Unless stated otherwise, all references in this document to "we," "us," "our," "Corcept," the "Company," "our company" and similar designations refer to Corcept Therapeutics Incorporated.

ITEM 1. BUSINESS

Overview

We are a pharmaceutical company engaged in the discovery, development and commercialization of drugs that treat severe metabolic, oncologic and psychiatric disorders by modulating the effects of cortisol. Elevated levels and abnormal release patterns of cortisol are implicated in a broad range of human disorders. Since our inception in 1998, we have been developing mifepristone, a compound that modulates the effects of cortisol by acting as a competitive antagonist at the glucocorticoid receptor (GR). We have also discovered three structurally distinct series of proprietary, selective cortisol modulators, all of which share mifepristone's affinity for GR but, unlike mifepristone, do not bind to the progesterone receptor and so do not cause effects associated with progesterone receptor affinity. Development of the lead compounds from these series is in progress.

In 2012, the United States Food and Drug Administration (FDA) approved Korlym® (mifepristone) 300 mg Tablets as a once-daily oral medication for the treatment of hyperglycemia secondary to hypercortisolism in adult patients with endogenous Cushing syndrome who have type 2 diabetes mellitus or glucose intolerance and have failed surgery or are not candidates for surgery.

We are conducting two clinical trials of our proprietary selective cortisol modulator, CORT125134. One trial is investigating CORT125134 as a potential treatment for patients with Cushing syndrome. The second trial is investigating the combination of CORT125134 and nab-paclitaxel (Celgene Corporation's Abraxane®) to treat patients with solid-tumor cancers. These trials are enrolling patients.

We plan to begin clinical trials of two other selective cortisol modulators in 2017.

The Role of Cortisol in Disease

Cortisol is a steroid hormone that plays a significant role in the way the body reacts to stressful conditions. It influences metabolism and the immune system and contributes to emotional stability. It is essential for survival. Insufficient cortisol activity may lead to dehydration, hypotension, shock, fatigue, low resistance to infection, trauma, stress and hypoglycemia. Excessive cortisol activity may lead to a suppressed immune response, impaired glucose tolerance, diabetes, obesity, fatty liver disease, depressed mood, psychosis, wasting of the arms and legs, edema, fatigue, hypertension and other problems. Pre-clinical and clinical data suggest that cortisol may reduce a patient's immune response to oncogenesis and shield certain cancer cells from the apoptotic effects of chemotherapy.

The challenge in regulating excessive levels of cortisol is that destroying the ability of the body to make cortisol can cause serious harm. An effective medication must modulate cortisol's effects without suppressing them below normal levels or disrupting the body's normal cortisol rhythm, in which cortisol levels rise at awakening and decrease during the day. The action of cortisol can effectively be modulated by the use of compounds that compete with cortisol as it attempts to bind to GR. Mifepristone, the active ingredient in Korlym, is a competitive GR antagonist, as are Corcept's proprietary compounds.

Because mifepristone works by reducing the binding of excess cortisol to GR, it can modulate the effects of abnormal levels and release patterns of cortisol without compromising cortisol's necessary, normal functions and rhythms. However, mifepristone also binds to the progesterone receptor and thereby terminates pregnancy and sometimes causes other side effects, including irregular vaginal bleeding. Our selective cortisol modulators block GR as potently as mifepristone does, but have no affinity for the progesterone receptor and so do not cause progesterone receptor-related side effects.

Cushing Syndrome

Background. Cushing syndrome is caused by prolonged exposure of the body's tissues to high levels of cortisol. It is relatively uncommon and most often affects adults aged 20 to 50. An estimated 10 to 15 of every one million people are newly diagnosed with this syndrome each year, resulting in approximately 3,000 new patients and an estimated total of 20,000 patients with Cushing syndrome in the United States.

Symptoms vary, but most people with Cushing syndrome have one or more of the following manifestations: high blood sugar, diabetes, high blood pressure, upper body obesity, rounded face, increased fat around the neck, thinning arms and legs, severe fatigue and weak muscles. Irritability, anxiety, cognitive disturbances and depression are also common. Cushing syndrome can affect every organ system in the body and can be lethal if not treated. The preferred treatment for Cushing syndrome patients is surgery, which, if successful, can cure the disease. Depending on the type of tumor, surgery can result in a range of complications and has varying rates of success. In approximately half of the patients, surgery is not successful because the tumor cannot be located or removed completely.

Korlym to Treat Patients with Cushing Syndrome. We have received Orphan Drug designation from the FDA for Korlym in the treatment of patients with endogenous Cushing syndrome. Drugs that receive Orphan Drug

designation receive seven years of marketing exclusivity for the approved indication, as well as tax credits for clinical trial costs, marketing application filing fee waivers and assistance from the FDA in the drug development process.

We first made Korlym available to patients on a commercial basis in April 2012. We sell Korlym using experienced sales representatives, who target U.S. endocrinologists who care for a large portion of the patients with Cushing syndrome. In addition, we have a field-based force of medical science liaisons. We also reach patients directly through web-based initiatives and interactions with patient groups. Because a large percentage of the people who suffer from Cushing syndrome remain undiagnosed or are inadequately treated, we have developed and continue to refine and expand programs to educate the medical community and patients about diagnosis of this syndrome and to increase awareness regarding the role of cortisol modulators to treat the disease.

We use a specialty pharmacy and a specialty distributor to distribute Korlym and provide logistical support. We have retained a vendor to help patients with the reimbursement process and to administer our financial assistance programs for uninsured or under-insured patients. We also donate money to independent charitable foundations. These organizations, along with our own programs, help us ensure that no Cushing syndrome patient is denied access to Korlym for financial reasons.

CORT125134 to Treat Patients with Cushing Syndrome. We are enrolling patients in a Phase 2 trial of our proprietary, selective cortisol modulator, CORT125134, to treat patients with Cushing syndrome. CORT125134 shares Korlym's affinity for GR. Data from the compound's Phase 1 trial showed that it potently modulates the effects of the steroid prednisone, a commonly-used GR agonist. Modulating the effect of prednisone is important because it is a strong surrogate for Korlym's modulation of cortisol – the essential quality of an effective treatment for patients with Cushing syndrome. Pharmacokinetic data indicate that CORT125134 is suitable for once-daily oral dosing. We expect to have data from this trial by the end of 2017.

FKBP5 Gene Expression. We are developing a CLIA-validated assay to measure expression of the gene FKBP5, which is stimulated by cortisol activity at GR. Our hypothesis is that FKBP5 expression increases in patients with Cushing syndrome and falls as their disease is treated. If our hypothesis is correct, our assay would allow physicians to measure the degree to which their patients suffer from excess cortisol activity – the cause of Cushing syndrome – which would help them more easily identify patients with the disease and better treat those already in their care.

Oncology

There is substantial in vitro, in vivo and clinical evidence that cortisol's activity allows certain solid-tumor cancers to resist treatment. In some cancers, cortisol activity promotes tumor growth. Cortisol also stimulates genes that retard cellular apoptosis.

Our oncology development program also seeks to exploit a second mechanism. Cortisol suppresses the body's immune response. Suppression of the immune response is often beneficial, as it lessens the frequency of autoimmune diseases. However, activating, not suppressing, the body's immune system is beneficial in fighting certain cancers. Our hypothesis is that adding a cortisol modulator to a treatment regimen will help the patient's immune system combat the disease.

A range of tumor-types express GR and are potential targets for cortisol modulation therapy, among them triple-negative breast, ovarian, castration-resistant prostate, cervical and pancreatic cancer, as well as sarcoma and melanoma.

Korlym to Treat Patients with Solid-Tumor Cancers. In December 2016, we announced the results of our Phase 1/2 trial of Korlym in combination with eribulin (Eisai's Inc.'s drug, Halaven®) to treat patients with metastatic triple-negative breast cancer. The trial studied 21 patients with GR positive tumors, one with GR negative tumors and one with tumors whose GR status was not known. As determined using the Response Evaluation Criteria in Solid

Tumors (RECIST), efficacy results were as follows: four patients exhibited a partial response, defined as a 30 percent or greater reduction in tumor size; eight had stable disease; and 11 had progressive

disease. Six patients achieved progression-free survival (PFS) longer than the upper bound of the 95% confidence interval for PFS (15 weeks) in patients receiving Halaven® monotherapy in a comparable population (Aogi et al., Annals of Oncology 23: 1441-1448, 2012). Median PFS in the trial was 11.1 weeks – compared to 7.2 weeks in the Halaven monotherapy study reported by Aogi. We believe that the addition of Korlym to chemotherapy warrants further study, such as the double-blind, placebo-controlled, multicenter, University of Chicago-led trial described above that Celgene is funding.

Korlym to Treat Patients with Triple-Negative Breast Cancer and Castration-Resistant Prostate Cancer. Investigators at the University of Chicago have initiated a double-blind, placebo-controlled, multicenter Phase 2 study of Korlym in combination with Celgene's drug Abraxane to treat 64 patients with advanced, GR-positive triple-negative breast cancer. Celgene is funding the trial. We are providing Korlym. University of Chicago investigators are also leading a controlled, multicenter Phase 2 study of Korlym combined with the androgen deprivation agent enzalutamide (Astellas Pharma Inc.'s drug, Xtandi®) versus Xtandi monotherapy to treat 84 patients with metastatic, castration-resistant prostate cancer. The investigators' hypothesis is that adding cortisol modulation to androgen deprivation therapy will better suppress tumor growth. The Department of Defense and the Prostate Cancer Foundation are funding the trial. Astellas is providing Xtandi. We are providing Korlym.

We have exclusively licensed patents from the University of Chicago covering the use of cortisol modulators in combination with anti-cancer agents to treat triple-negative breast cancer and with androgen deprivation agents to treat castration-resistant prostate cancer.

CORT125134 to Treat Patients with Solid-Tumor Cancers. We are conducting a Phase 1/2 trial of Abraxane (nab-paclitaxel) in combination with CORT125134 to treat any solid-tumor cancer suitable for treatment with Abraxane. Once we identify a recommended dose of this combination, we will open 20-patient cohorts to test the combination's efficacy in one or more solid-tumor cancers. Our likely initial targets will be triple-negative breast cancer and ovarian cancer. Other possible indications include pancreatic cancer, cervical cancer and sarcoma. We may choose to open additional dose-finding cohorts to study CORT125134 in combination with different companion therapeutic agents, including immunotherapy, to treat other solid-tumor cancers.

Development of Our Other Selective Cortisol Modulators

CORT125134 is the lead compound in our portfolio of proprietary selective cortisol modulators, which consists of three structurally distinct series. All of these compounds, like Korlym, potently block GR but do not block the progesterone, estrogen or androgen receptors. In addition to our findings with CORT125134, several of our new compounds have demonstrated positive results in animal or in vitro models that test cortisol modulation. We are advancing the most promising of these compounds towards the clinic and expect to begin clinical trials of CORT118335 and CORT125281 in 2017. CORT118335 is a potential medication for fatty-liver disease, anti-psychotic-induced weight gain and other metabolic disorders. CORT125281 is a candidate for the treatment of castration-resistant prostate cancer (in combination with an androgen-deprivation agent such as Xtandi) and other indications.

The United States Patent & Trademark Office (USPTO) and the European Patent Office (EPO) have issued to us composition of matter patents related to our selective cortisol modulators. In addition, we own or have exclusively licensed patents for the use of all cortisol modulators (including Korlym) in a broad range of disorders. See "Business – Intellectual Property."

We intend to continue our discovery research program with the goal of identifying new selective cortisol modulators, to manufacture and conduct pre-clinical development of one or more of these compounds and to study the most promising of them in humans.

Studies by Independent Investigators

We have, for many years, sought to advance our understanding of cortisol modulation’s therapeutic potential by supporting the work of independent academic investigators. These researchers have studied the utility of our proprietary selective cortisol modulators in pre-clinical studies in a wide range of disorders, including post-traumatic

stress disorder, alcoholism, Alzheimer's disease, ALS, muscular dystrophy, Cushing syndrome, metabolic syndrome, fatty liver disease, ovarian cancer, castration-resistant prostate cancer and triple-negative breast cancer.

Clinical Trial Agreements

Some of our clinical trials are conducted through the use of clinical research organizations (CROs). Our Phase 2 trial of CORT125134 for the treatment of patients with Cushing syndrome is being conducted under an agreement with Chiltern International Limited (Chiltern). This agreement may be terminated by us upon 60-days written notice to Chiltern or sooner if the parties mutually agree.

Research and Development Spending

We incurred $23.8 million, $15.4 million and $18.4 million of research and development expenses in the years ended December 31, 2016, 2015 and 2014, which accounted for 33%, 29% and 34%, respectively of our total operating expenses in those years.

Manufacturing Korlym

We do not have manufacturing capabilities and intend to continue to rely on experienced contract manufacturers to produce Korlym and our product candidates. We have a long-term agreement with one contract manufacturer, Produits Chimiques Auxiliaires et de Synthese SA (PCAS), to produce mifepristone, the active pharmaceutical ingredient (API) for Korlym, pursuant to which we agree to purchase a minimum percentage of our mifepristone requirements. The initial term of the agreement is five years, with an automatic extension of one year, unless either party gives 12-months prior written termination notice. We have the right to terminate the agreement if PCAS is unable to manufacture mifepristone for nine consecutive months.

We have one tablet manufacturer for Korlym – Alcami Corporation (formerly known as AAI Pharma Services Corp., or AAI). In April 2014, we entered into an agreement with Alcami for the manufacture and packaging of Korlym tablets. The initial term of this agreement is three years, with consecutive automatic extensions of two years, unless either party gives written termination notice (in the case of Alcami, 18 months prior to the end of the applicable term; in our case, 12 months prior to the end of the applicable term). We have the right to terminate the agreement if (i) Alcami is unable to manufacture our product for four consecutive months or (ii) our product is withdrawn from the market. We have no minimum purchase obligations under this agreement.

Competition for Korlym

Korlym competes with established treatments, including surgery, radiation and other medications, including "off-label" uses of drugs such as ketoconazole, an anti-fungal medication. Korlym also competes with Novartis' drug, Signifor® (pasireotide) Injection, which the FDA approved in December 2012 for the treatment of adult patients with Cushing disease who are not candidates for pituitary surgery or for whom surgery did not work. (Cushing disease is a subset of Cushing syndrome that afflicts approximately 70 percent of patients with Cushing syndrome.)

Korlym may also experience competition from compounds under development for Cushing syndrome. For example, Strongbridge Biopharma plc has received Orphan Drug designation in the United States and the EU for the use of levoketoconazole, a chiral form of ketoconazole, to treat Cushing syndrome and has begun a Phase 3 clinical trial in Europe and the United States for this indication.

Intellectual Property

Patents and other proprietary rights are important to our business. It is our policy to seek patent protection for our inventions and to rely upon trade secrets, know-how, continuing technological innovations and licensing opportunities

to develop and maintain our competitive position.

Mifepristone. The composition of matter patent covering mifepristone has expired. The only other FDA-approved use of mifepristone is to terminate pregnancy. The FDA has imposed significant restrictions on the use of mifepristone to terminate pregnancy. To protect our market for Korlym we plan to rely on (1) the exclusive marketing rights conferred as a benefit of Orphan Drug designation in the United States, (2) the restrictions imposed by the FDA on the use of mifepristone to terminate pregnancy, (3) the different patient populations, administering physicians and treatment settings between the use of mifepristone to terminate pregnancy and to treat Cushing syndrome and (4) our method of use patents described below.

Oncology. We have an exclusive license agreement with the University of Chicago to patents covering the use of all cortisol modulators, including mifepristone, in the treatment of triple-negative breast cancer (in combination with anti-cancer agents) and castration-resistant prostate cancer (in combination with androgen deprivation agents). See “Business – License Agreements.”

Other Method of Use Patents. We own issued U.S. patents for the use of cortisol modulators in the treatment of mild cognitive impairment, the prevention and treatment of stress disorders, improving the therapeutic response to electroconvulsive therapy, the treatment of delirium, the treatment of catatonia, the treatment of psychosis with Interferon-Alpha therapy, inhibiting cognitive deterioration in adults with Down’s Syndrome, the treatment of weight gain following treatment with antipsychotic medication, the treatment of gastroesophageal reflux disease, the treatment of migraine headaches, the treatment of neurological damage in premature infants, and the treatment of diseases using combination steroid and GR antagonist therapy. We also own a method of use patent for optimizing mifepristone levels in plasma serum in patients suffering from mental disorders, including the mental disorders seen in Cushing syndrome. The expiration dates of these patents and their foreign counterparts range from 2020 to 2034.