

CORCEPT THERAPEUTICS INC  
Form 10-K  
March 15, 2013  
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**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM 10-K**

x **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

**For the fiscal year ended December 31, 2012**

**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)*

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**Delaware**  
(State or other jurisdiction of incorporation or organization)

**77-0487658**  
(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:**

<b>Title of Each Class:</b>	<b>Name of Each Exchange on which Registered:</b>
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 (§232.405 of this chapter) 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   
Non-accelerated filer  (Do not check if a smaller reporting company) Accelerated filer   
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

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The aggregate market value of voting and non-voting common equity held by non-affiliates of the Registrant was \$241,382,000 as of June 30, 2012 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 March 1, 2013 there were 99,814,250 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 2013 Annual Meeting of Stockholders are incorporated by reference in Items 10, 11, 12, 13 and 14 of Part III.

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**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 from time to time, the words believe, anticipate, intend, plan, estimate, expect, may, will, should, seeks and similar forward-looking statements. Such forward-looking statements are based on current expectations, but the absence of these words does not necessarily 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) 300mg Tablets;*

*our ability to realize the benefits of Orphan Drug Designation of Korlym in the United States;*

*the progress and timing of our research, development and clinical programs and the timing of regulatory activities for mifepristone for the treatment of the psychotic features of psychotic depression;*

*our estimates regarding enrollment in and the dates by which we expect to report results of our clinical trials and the anticipated results of these trials;*

*our ability to achieve marketing approval of Korlym in the European Union (EU) and realize the benefits of Orphan Drug Designation there;*

*the timing of the market introduction of future product candidates, including any compound in our families of selective GR-II antagonists;*

*our ability to manufacture, market, commercialize and achieve market acceptance for our future product candidates, including mifepristone for the treatment of the psychotic features of psychotic depression and any compound in our families of selective GR-II antagonists;*

*uncertainties associated with obtaining and enforcing patents;*

*our estimates for future performance, including revenue and profits; and*

*our estimates regarding our capital requirements.*

*Forward-looking statements are not guarantees of future performance and involve risks and uncertainties. Actual events or results may differ materially from those discussed in the forward-looking statements as a result of various factors. For a more detailed discussion of such forward-looking statements and the potential risks and uncertainties that may impact upon their accuracy, see the Risk Factors section of this Form 10-K and the 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. These forward-looking statements reflect our view only as of the date of this report. Except as required by law, we undertake no obligations to update any forward-looking statements. Accordingly, you should also carefully consider the factors set forth in other reports or documents that we file from time to time with the Securities and Exchange Commission (SEC).*

**ITEM 1. BUSINESS**

**Overview**

We are a pharmaceutical company engaged in the discovery, development and commercialization of drugs for the treatment of severe metabolic and psychiatric disorders. Our focus is on those disorders that are associated with a steroid hormone called cortisol. Elevated levels and abnormal release patterns of cortisol have been implicated in a broad range of human disorders. Since our inception in May 1998, we have been developing mifepristone a potent glucocorticoid receptor II (GR-II) antagonist that blocks the activity of cortisol for the treatment of a number of severe metabolic and psychiatric disorders. We have also discovered three series of novel selective GR-II antagonists and have moved a compound from one of these series into clinical development.

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On February 17, 2012, the United States Food and Drug Administration (FDA) approved Korlym (mifepristone) 300 mg Tablets as a once-daily oral medication for treatment of hyperglycemia secondary to hypercortisolism in adult patients with endogenous Cushing's syndrome who have type 2 diabetes mellitus or glucose intolerance and have failed surgery or are not candidates for surgery. FDA approval means that we can market the drug for the approved indication in the United States. We made Korlym available to patients in April 2012 and continue to develop the sales, marketing, medical affairs and logistical infrastructure needed to commercialize the drug. We also have an on-going Phase 3 study of mifepristone, the active ingredient in Korlym, for the psychotic features of psychotic depression.

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

*Cushing's Syndrome.* Cushing's syndrome is a disorder caused by prolonged exposure of the body's tissues to high levels of the hormone cortisol. Sometimes called hypercortisolism, 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 prevalence of 20,000 patients with Cushing's syndrome in the United States.

We received Orphan Drug Designation from the FDA in July 2007 for Korlym for the treatment of endogenous Cushing's syndrome. In the United States, Orphan Drug Designation is a special status granted by the FDA to encourage the development of treatments for diseases or conditions that affect fewer than 200,000 patients. Drugs that receive Orphan Drug Designation obtain seven years of marketing exclusivity for the approved indication from the date of drug approval, as well as tax credits for clinical trial costs, marketing application filing fee waivers and assistance from the FDA in the drug development process.

In October 2011 we received Orphan Drug Designation in the EU. Orphan Drug Designation in the EU confers benefits similar to those in the U.S., but includes ten years of marketing exclusivity for the approved indication in all 27 member states, free scientific advice during drug development, access to a centralized review process and a reduction or complete waiver of fees levied by the European Medicines Agency (EMA). We are working with the EMA now to prepare a Marketing Authorization Application (MAA) that, subject to filing with and review by the EMA, could serve as the basis for Korlym's approval in the EU.

As discussed above, in February 2012, the FDA approved our New Drug Application (NDA) for Korlym as a once-daily oral medication for treatment of hyperglycemia secondary to hypercortisolism in adult patients with endogenous Cushing's syndrome who have type 2 diabetes mellitus or glucose intolerance and have failed surgery or are not candidates for surgery.

*Psychotic Depression.* We are also developing mifepristone, Korlym's active ingredient, for treatment of the psychotic features of psychotic depression under an exclusive patent license from Stanford University. The FDA has granted fast track status to evaluate the safety and efficacy of mifepristone for the treatment of the psychotic features of psychotic depression.

In March 2008, we began enrollment in Study 14, our ongoing Phase 3 trial in psychotic depression. The protocol for this trial incorporates what we have learned from our three previously completed Phase 3 trials. It attempts to address the established relationship between increased drug plasma levels and clinical response and attempts to decrease the random variability observed in the results of the psychometric instruments used to measure efficacy. In one of the previously completed Phase 3 trials, Study 06, we prospectively tested and confirmed that patients whose plasma levels rose above a predetermined threshold statistically separated from both those patients whose plasma levels were below the threshold and those patients who received placebo; this threshold was established from data produced in earlier studies.

As expected, the group of patients who took 1200 milligrams (mg) of mifepristone in Study 06 developed higher drug plasma levels than did the groups of patients who received lower doses. Further, there was no

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discernible difference in the incidence of adverse events between patients who received placebo in Study 06 and those who received 300 mg, 600 mg or 1200 mg of mifepristone in that study. In August 2011, we published our analysis of these data in *The Journal of Clinical Psychopharmacology*. Based on this information, we are using a mifepristone dose of 1200 mg once per day for seven days in Study 14.

In addition, we are utilizing a third party centralized rating service to independently evaluate patients for entry into the study as well as to evaluate their level of response throughout their participation. We believe the centralization of this process will improve the consistency of rating across clinical trial sites and reduce the background noise that was experienced in earlier studies and is endemic to psychopharmacologic studies. We believe that this change in dose, as well as the other modifications to the protocol, should allow us to demonstrate the efficacy of mifepristone in the treatment of the psychotic symptoms of psychotic depression. In mid-2009, in order to conserve financial resources, we reduced the number of clinical sites to eight and extended the timeline for the study's completion. To increase the pace of patient enrollment, we began adding clinical sites in the fourth quarter of 2012 and plan to have 20 clinical sites participating by the end of the first quarter of 2013. Our goal is to enroll a sufficient number of patients by the end of 2013 to be able to perform a successful interim analysis.

*Antipsychotic-induced Weight Gain Mitigation.* In 2005, we published the results of studies in rats that demonstrated that mifepristone both reversed the weight gain associated with the ongoing use of olanzapine and mitigated the weight gain associated with the initiation of treatment with olanzapine (the active ingredient in Zyprexa®). The results from this study were published in the journal *Brain Behavioral Research* in early 2006. The study was paid for by Eli Lilly and Company (Eli Lilly).

During 2007 we announced positive results from our clinical proof-of-concept study in lean healthy male volunteers evaluating the ability of mifepristone to mitigate weight gain associated with the use of Zyprexa. The results show a statistically significant reduction in weight gain in those subjects who took Zyprexa plus mifepristone compared to those who took Zyprexa plus placebo. Also, the addition of mifepristone to treatment with Zyprexa had a beneficial impact on secondary metabolic measures such as fasting insulin, triglycerides and abdominal fat, as indicated by waist circumference. Eli Lilly provided Zyprexa and financial support for this study and its results were published in the journal *Advances in Therapy* in 2009. In January 2009, we announced positive results from a similar proof-of-concept study evaluating the ability of mifepristone to mitigate weight gain associated with the use of Johnson & Johnson's Risperdal. This study confirmed and extended the earlier results seen with mifepristone and Zyprexa, demonstrating a statistically significant reduction in weight and secondary metabolic endpoints of fasting insulin, triglycerides and abdominal fat, as indicated by waist circumference. The results from the study of mifepristone and Risperdal were presented at several scientific conferences, including the American Diabetes Association meeting in June 2009, and were published in the journal *Obesity* in 2010.

The combination of Zyprexa or Risperdal and mifepristone is not approved for any indication. The purpose of these studies was to explore the hypothesis that GR-II antagonists, such as mifepristone and our next generation of selective GR-II antagonists, would mitigate weight gain associated with antipsychotic medications. The group of medications known as second generation antipsychotic medication, including Zyprexa, Risperdal, Clozaril® and Seroquel®, are widely used to treat schizophrenia and bipolar disorder. All medications in this group are associated with treatment-emergent weight gain of varying degrees and carry a warning in their labels relating to treatment-emergent hyperglycemia and diabetes mellitus.

*Selective GR-II Receptor Antagonists.* In 2003, we initiated a discovery research program to identify and patent selective GR-II antagonists. Our intent is to develop a pipeline of products for proprietary use. Three distinct series of selective GR-II antagonists have been identified. These compounds, like the active ingredient in our lead product Korlym, potently block the cortisol receptor (GR-II) but, unlike Korlym, do not appear to block the PR (progesterone), ER (estrogen), AR (androgen) or GR-I (mineralocorticoid) receptors. Both the United



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States Patent & Trademark Office (USPTO) and the European Patent Office (EPO) have issued to us composition of matter patents in each of the three series. Two additional composition of matter patent applications are pending. See Business Intellectual Property.

Several of our new compounds have demonstrated positive results in animal models for the prevention and reversal of anti-psychotic-induced weight gain, as well as animal and *in vitro* models of other metabolic and common central nervous system disorders. One of these new compounds, CORT 108297, is in exploratory Phase 2 clinical trials and we plan to explore its potential use in several indications. *See Business Next-Generation Selective GR-II Antagonists for the Prevention and Reversal of Anti-Psychotic-Induced Weight Gain.* We have identified other selective GR-II antagonists from our proprietary series that we believe may have utility as therapeutic agents in a variety of diseases. We intend to continue our discovery research program with the goal of identifying new selective GR-II antagonists and to perform manufacturing and pre-clinical development on several of these compounds and to submit Investigational New Drug applications (INDs) with respect to the most promising of them, as we deem appropriate.

### **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 and is essential for survival. Cortisol significantly influences metabolism, exerts a clinically useful anti-inflammatory effect and contributes to emotional stability. Insufficient levels of cortisol may lead to dehydration, hypotension, shock, fatigue, low resistance to infection, trauma, stress and hypoglycemia. Excessive levels of cortisol may lead to impaired glucose tolerance, diabetes, obesity, depressed mood, psychosis, wasting of the arms and legs, edema, fatigue, hypertension, and other problems.

Elevated levels and abnormal release patterns of cortisol have also been linked to a broad range of metabolic and psychiatric conditions, such as weight gain, diabetes, hypertension, mood changes, psychosis and cognitive impairment.

While excess cortisol may play a role in numerous diseases, Cushing's syndrome (sometimes called hypercortisolism) is the archetypal disease of excess cortisol, as Cushing's syndrome patients have tumors that produce excess levels of cortisol or adrenocorticotropic hormone (ACTH), which stimulates the production of cortisol. Exposure to high levels of cortisol can result in weight gain, diabetes, hypertension, infections, severe fatigue and psychosis.

Many studies have shown that patients with psychotic depression have elevated levels and abnormal release patterns of cortisol. This abnormal cortisol activity is not usually present in patients with nonpsychotic depression. More than 20 years ago, one of our scientific co-founders postulated that elevated levels of cortisol in patients with psychotic depression lead to elevated levels of dopamine, an important chemical substance found in the brain. Elevated levels of dopamine have been implicated in both delusional thinking and hallucinations. This hypothesis led to the concept that, by regulating the level and release patterns of cortisol, one could normalize dopamine levels in the brain, which may, in turn, ameliorate the symptoms of psychotic depression. In addition to cortisol's effect on dopamine levels, research has shown that prolonged elevated cortisol may also play a direct role in causing the symptoms of psychotic depression.

The challenge in regulating levels of cortisol is that cortisol is needed for natural processes in the human body. Destroying the ability of the body to make cortisol or to drastically reduce its presence would result in serious detrimental effects. To have a viable therapeutic effect, a compound must be able to selectively modulate cortisol's effects.

### **Glucocorticoid Receptor Antagonists**

Cortisol is produced by the adrenal glands and is carried via the bloodstream throughout the body, including to the brain, where it directly influences neuronal function. In the brain, cortisol binds to two receptors,

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Glucocorticoid Receptor I and Glucocorticoid Receptor II, also known as GR-I and GR-II. GR-I is a high-affinity receptor that is involved in the routine functions of cortisol in the brain. It has approximately ten times the affinity of GR-II for cortisol and its binding sites are filled with cortisol nearly all the time. In general, GR-II binding sites do not fill until levels of cortisol become elevated. Short-term activation of GR-II has benefits, which include helping the individual to be more alert and better able to function under stressful conditions. Long-term activation of GR-II, however, has been shown to have significant toxicity and appears to be linked to multiple metabolic and psychiatric disease states, such as Cushing's syndrome and psychotic depression. The action of cortisol can be moderated by the use of blockers, or antagonists, that prevent the binding of the hormone to its receptors. These antagonists, referred to as glucocorticoid or cortisol receptor antagonists, may prevent the undesirable effects of elevated levels and abnormal release patterns of cortisol.

Mifepristone, the active ingredient in Korlym, works by selectively blocking the binding of cortisol to GR-II. It is neither an antagonist nor agonist of GR-I. It also blocks the binding of progesterone to the progesterone receptor (PR). Because of its selective affinity, we believe that mifepristone can have a therapeutic benefit by modulating the effects of abnormal levels and release patterns of cortisol without compromising the necessary normal functions of cortisol. We have also discovered three series of additional compounds that, like mifepristone, potentially block the GR-II receptor, but, unlike mifepristone, do not block the progesterone receptor. One of these compounds, CORT 108297, is now being studied in the clinic. We have identified other compounds suitable for advancement and plan to begin pre-clinical work on several of them in 2013.

### **Overview of Cushing's Syndrome**

Endogenous Cushing's syndrome is caused by prolonged exposure of the body's tissues to high levels of the hormone cortisol produced by a tumor or tumors. In endogenous Cushing's syndrome, the excess cortisol is stimulated or directly produced by pituitary, adrenal or ectopic tumors. Cushing's syndrome is an orphan indication which most commonly 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 in the United States. An estimated 20,000 patients in the United States have been diagnosed with Cushing's syndrome. Symptoms vary, but most people 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's syndrome can affect every organ system in the body and can be lethal if not treated effectively.

The preferred treatment for Cushing's 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, either because the tumor cannot be removed completely or the disease returns.

### **Commercialization of Korlym**

Korlym is the first approved therapy for patients with endogenous Cushing's syndrome. On February 17, 2012, Korlym was approved by the FDA for hyperglycemia secondary to hypercortisolism in adult patients with endogenous Cushing's syndrome who have type 2 diabetes mellitus or glucose intolerance and have failed surgery or are not candidates for surgery. As indicated in the medicine's prescribing information, physicians prescribing Korlym may determine the appropriate dose for each patient by assessing tolerability and degree of improvement in manifestations of Cushing's syndrome. In the first six weeks, these manifestations may include changes in glucose control, anti-diabetic medication requirements, insulin levels and psychiatric symptoms. After two months, assessment may also be based on improvements in cushingoid appearance, acne, hirsutism, striae, decreased body weight, along with further changes in glucose control.

We have begun marketing Korlym in the United States, without a partner, because we believe that the market is highly concentrated and accessible. Following the drug's approval by the FDA in February 2012,

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we began hiring a small number of experienced medical science liaisons (MSLs), supported by medical affairs and other infrastructure, to educate health care providers about Korlym. To reach more physicians, in October 2012 we began deploying a small force of experienced field sales personnel. We intend for our MSLs and sales representatives to focus on patients who are in the care of an endocrinologist and in active treatment for their disease. We estimate that we would need to target approximately 500 endocrinologists to reach a large portion of the Cushing's syndrome population in active treatment. We also reach patients directly through web-based initiatives and interactions with patient groups. We have executed agreements with a specialty pharmacy, a specialty distributor, a contract sales organization, and a third-party logistics company to distribute Korlym and provide logistical support.

A large percentage of the people who suffer from Cushing's syndrome remain undiagnosed or inadequately treated. We intend to develop programs to educate the medical community and patients about early diagnosis of this syndrome and to increase awareness regarding the role of GR-II antagonists for this syndrome. We have retained a vendor to help patients with the reimbursement process. This vendor also administers our financial assistance programs for uninsured or under-insured patients who cannot otherwise afford the cost of Korlym.

Both the FDA and the European Commission have granted Orphan Drug Designation for Korlym. In the United States, Orphan drugs receive seven years of marketing exclusivity for the approved indication from the date of approval, as well as tax credits for clinical trial costs, marketing application filing fee waivers and assistance from the FDA in the drug development process. Benefits of Orphan Drug Designation in the EU are similar to those in the U.S., but include ten years of marketing exclusivity for the approved indication in all 27 member states, free scientific advice during drug development, access to a centralized review process and a reduction or complete waiver of fees levied by the European Medicines Agency (EMA). In 2013, we plan to commence a study of Korlym in pediatric Cushing's syndrome patients. If we complete the study and submit the data to the FDA and EMA pursuant to protocols and within timelines that we may agree upon with these agencies, our Orphan Drug marketing exclusivity period will be extended by six months in the United States and two years in the EU.

## **Additional Trials and Preclinical Studies**

As part of its approval for Korlym, the FDA has required us to study the interactions, if any, between Korlym and ketoconazole, an anti-fungal agent that is sometimes used to treat Cushing's syndrome, although it is not approved by the FDA for that purpose. Further, the FDA has required us to perform a drug utilization study to better characterize the reporting rates of adverse events associated with the long-term use of Korlym. On our own initiative, we conducted a long-term extension study in patients who completed the Phase 3 trial to assess safety of chronic dosing. Upon the approval of Korlym we transitioned study patients to commercial product and terminated the study.

## **Overview of Psychotic Depression**

Psychotic depression is a serious psychiatric disease in which a patient suffers from severe depression accompanied by delusions, hallucinations or both. These psychotic features typically develop after the onset of a depressed mood, but may develop concurrently as well. Once psychotic symptoms occur, they usually reappear with each subsequent depressive episode. Of particular importance, when the patient's mood returns to normal the psychosis also resolves.

Data from the National Institute of Mental Health published in 2005 indicate that depressive disorders affect an estimated 9.5% of adults in the United States, or about 19 million people each year. Of these 19 million people, many published studies show that approximately 15-20%, or about three million people, have psychotic depression. Most patients with psychotic depression suffer their first episode of major depression between the ages of 30 and 40 and the majority will experience more than one episode in their lifetime. People with psychotic depression are approximately 70 times more likely to commit suicide in their lifetime than the general population and often require lengthy and expensive hospital stays.

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### **Current Treatments for Psychotic Depression**

There are two treatment approaches for the psychotic features of psychotic depression currently used by psychiatrists: electroconvulsive therapy (ECT) and combination drug therapy, which is a combination of antidepressant and antipsychotic medication. Neither of these treatments has been approved by the FDA for the psychotic features of psychotic depression and both approaches almost always have a slow onset of action, which may result in lengthy and costly hospitalization. Each of these treatments can have debilitating side effects. Of the two treatments, ECT is generally considered to be more effective.

ECT involves passing an electrical current through the brain until the patient has a seizure. At least 100,000 patients receive ECT each year in the United States, with each patient requiring approximately six to twelve procedures over a period of three to five weeks.

Combination drug therapy is an alternative treatment for the psychotic features of psychotic depression that involves taking antipsychotic drugs such as olanzapine, haloperidol or chlorpromazine in combination with antidepressant drugs, such as fluoxetine, imipramine or venlafaxine. Patients on combination drug therapy often require three weeks or more to show improvement in their symptoms and treatment can take months before the symptoms are resolved entirely. Antipsychotic drugs can cause significant adverse side effects, including weight gain, diabetes, sedation, permanent movement disorders and sexual dysfunction.

### **Mifepristone for the Psychotic Features of Psychotic Depression**

We are developing mifepristone as an oral medication to treat the psychotic features of psychotic depression. As a GR-II antagonist, mifepristone appears to mitigate the effects of the elevated and abnormal release patterns of cortisol in patients suffering from psychotic depression. We intend mifepristone to be a once-daily treatment given to patients with psychotic depression over seven consecutive days in a controlled setting, such as a hospital or physician's office.

We believe that mifepristone may significantly reduce psychotic symptoms of psychotic depression in many patients within one week and allow patients to be more easily maintained on antidepressant therapy alone without the need for ECT or antipsychotic medication. We believe that mifepristone may be superior to currently available treatments because we believe that mifepristone will enable patients with psychotic depression to improve their quality of life more quickly and with fewer side effects than with ECT or combination drug therapy.

### **Completed Clinical Trials of Mifepristone for Psychotic Depression**

We have completed seven prior clinical trials evaluating mifepristone for treatment of the psychotic features of psychotic depression, in addition to our ongoing Phase 3 trial. The trials include three Phase 3 trials conducted from 2004 through 2007, in addition to four earlier stage clinical trials with mifepristone. These completed trials generated important data confirming the safety profile of mifepristone (alone and in combination with commonly prescribed antipsychotic and antidepressant medications), demonstrated positive efficacy trends, and provided insights into the design of future clinical trials which might improve the probability of clinical success.

*Completed Phase 3 Clinical Trials.* In addition to Phase 1 and 2 studies, we have completed three randomized, double-blind, placebo-controlled Phase 3 clinical trials to further assess the safety and efficacy of mifepristone for the treatment of the psychotic features of psychotic depression. Two of these trials (Study 06 and Study 07) were conducted primarily in the United States. The third trial (Study 09) was conducted in Eastern Europe.

The primary endpoint for Study 06 and Study 07 was the proportion of patients with at least a 50% improvement in the Brief Psychiatric Rating Scale Positive Symptom Subscale (BPRS PSS) at both Day 7 and

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Day 56. The primary endpoint for Study 09 was the proportion of patients with at least a 50% improvement in the BPRS PSS, at both Day 7 and Day 28, with day 56 as a secondary endpoint. Patients must have had at least mild psychotic symptoms (BPRS PSS <sup>3</sup> 12) to enter the studies and were hospitalized if clinically necessary.

*Study 07:* The first of these trials, which began in September 2004, enrolled 257 patients randomized one-to-one to either treatment or placebo. Patients in the treatment arm received 600 mg of mifepristone once daily for seven days. Patients did not take any antidepressant or antipsychotic medication for at least one week before beginning the seven day treatment period. After the seven days of mifepristone treatment, all patients received antidepressant therapy through Day 56. Treatment with antipsychotic medications or ECT was not allowed at any time during the study.

In this study patients receiving mifepristone did not have a statistically significant difference in response rate at the primary endpoint than did the patients receiving placebo. A retrospective analysis of the data showed that patients achieving drug plasma levels higher than 1800 nanograms per milliliter (ng/ml) had a statistically significant greater response rate than placebo. There was also a statistically significant site by treatment effect in this trial. Among the twenty sites who participated from the trial onset, patients who were given mifepristone had a significantly higher response rate than patients who received placebo. Among the sites added later in the trial, there was no significant difference in response rate between mifepristone and placebo patients. These findings were published in 2009 by *Contemporary Clinical Trials*.

*Study 09:* This study, which commenced in May 2005, was a randomized, double-blind, placebo-controlled study in which 247 patients were enrolled at sites in Eastern Europe. Patients in the treatment arm received 600 mg of mifepristone once daily for seven days. The primary endpoint was the proportion of patients with at least a 50% improvement in the BPRS PSS score at both Day 7 and Day 28. The study did not demonstrate a significant difference in response between patients receiving mifepristone and patients receiving placebo as measured by the primary endpoint. The results at the two key secondary endpoints of Study 09 also were not statistically significant. Study 09 had an extremely high placebo response rate.

*Study 06:* This trial began in October 2004, and enrolled 443 patients. These patients were randomly assigned to three active dose groups (300 mg, 600 mg and 1200 mg) or a placebo group, with patients receiving once daily dosing for a period of seven days. The three dosing levels responded to the FDA's request to supplement data on a range of doses to augment the data provided by our open label dose ranging study completed in 2001.

The study did not achieve statistical significance with respect to the primary endpoint. However, there was a statistically significant correlation between plasma levels and clinical outcome achieved during treatment. Response rates for patients whose plasma levels rose above a predetermined threshold of 1661 ng/ml were statistically different than those patients whose plasma levels were below the threshold and those patients who received placebo. Further, the incidence of serious adverse events did not differ between placebo and any of the three mifepristone dose groups. In August 2011, we published an analysis of these results in *The Journal of Clinical Psychopharmacology*.

*Ongoing Phase 3 trial Study 14:* We believe that the confirmation of a correlation between drug concentration and clinical response, as well as other observations from Study 06 and our two other completed Phase 3 clinical trials, served as a strong basis for the design of our ongoing Phase 3 study, which commenced in March 2008. The protocol for this trial incorporates information learned from the three completed Phase 3 trials in that it addresses the established relationship between increased drug plasma levels and clinical response, and it attempts to decrease the random variability observed in the results of the psychometric instruments used to confirm diagnosis and measure efficacy.

Increased Signal : In this trial we are administering a mifepristone dose of 1200 mg once per day for seven days instead of 600 mg once per day for seven days.

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Decreased Noise : We also are utilizing a third party centralized rating service to independently evaluate the patient's diagnosis prior to entry into the study as well as to assess response. We believe the centralization of this process will improve the accuracy of diagnosis and the consistency of rating across clinical trial sites and reduce the background noise that is endemic to psychopharmacologic studies and clearly visible in our earlier studies.

We believe that these changes in the protocol should allow us to establish the efficacy of mifepristone in the treatment of the psychotic features of psychotic depression. Given the serious nature of psychotic depression, the lack of any approved drugs for the disorder and the data from our first clinical trial, the FDA granted a fast track designation for mifepristone for the treatment of the psychotic features of psychotic depression. In addition, the FDA has indicated that mifepristone will receive a priority review if no other treatment is approved for the psychotic features of psychotic depression at the time we submit our NDA.

Enrollment in Study 14 is ongoing. Our goal is to enroll a sufficient number of patients by the end of 2013 to be able to perform a successful interim analysis. To help reach this goal, we plan to increase the number of clinical sites from eight to approximately 20 by the end of the first quarter of 2013.

*Clinical Trial Agreements.* Many of our Phase 3 clinical trials are conducted through the use of clinical research organizations (CROs.) At our request, these organizations oversee clinical trials at various institutions to test the safety and efficacy of our product candidates for the targeted indications. Our ongoing Phase 3 clinical trial, Study 14, evaluating mifepristone for the treatment of the psychotic features of psychotic depression is being conducted under an agreement with ICON Clinical Research, LP (ICON). We may terminate this agreement with 60 days notice to ICON, or sooner based on mutual agreement of the parties. In addition, we entered into an agreement with MedAvante, Inc. (MedAvante), in March 2008, to provide the centralized psychiatric diagnosis and rating services for patients being screened and enrolled in Study 14. We may terminate this agreement with 30 days notice to MedAvante.

### **Discovery Research: Next-Generation Selective GR-II Antagonists**

In 2003, we initiated a discovery research program to identify and patent selective GR-II antagonists at a contract research organization in the United Kingdom. Through this program we have identified and filed patent applications for three distinct series of selective GR-II antagonists. These compounds appear to be as potent as our lead product mifepristone in blocking cortisol but, unlike mifepristone, they do not appear to block the progesterone or other steroid receptors. Currently, we are investigating several compounds in our research programs. We plan to submit INDs for such additional compounds as our research indicates may be promising and as we deem appropriate.

We have assembled a patent portfolio covering both a broad range of uses and the composition of our new chemical entities.

We have composition of matter claims on three patent families of novel selective glucocorticoid receptor (GR-II) antagonists. Applications for all three families have been allowed or issued in both the United States and Europe. Two additional composition of matter patent applications are pending in both the United States and Europe.

We also have a portfolio of patents describing the use of drugs that block the GR-II receptor for the treatment of metabolic and psychiatric disorders. In addition to psychotic depression, we own or have exclusively licensed issued patents for the use of GR-II antagonists for treatment and / or prevention of:

weight gain following treatment with antipsychotic medication;

mild cognitive impairment;

stress disorders;

early dementia, including early Alzheimer's disease;



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delirium;

gastroesophageal reflux disease;

cognitive deterioration in adults with Down's Syndrome;

psychosis associated with cocaine addiction;

catatonia; and

increased therapeutic response to ECT.

See Business Intellectual Property.

**Next-Generation Selective GR-II Antagonists for the Prevention and Reversal of Antipsychotic-Induced Weight Gain**

In January 2009, we announced results from two preclinical studies of our first next-generation selective GR-II receptor antagonist, CORT 108297, for the prevention and reversal of weight gain caused by olanzapine, a medication marketed by Eli Lilly as Zyprexa. Using the same experimental rat model used previously with mifepristone, the preclinical studies demonstrated that CORT 108297 1) reversed and 2) prevented the weight gain caused by olanzapine in rats. Eli Lilly provided olanzapine and funded the cost of the studies.

In the first of these two studies, seventy-two female rats (n=12 per group) were allowed to eat a normal diet for 56 days. During an induction phase of weight gain (study days 1-34), 12 rats were administered placebo, whereas 48 were administered olanzapine. Animals receiving olanzapine gained significantly more weight than animals receiving placebo (p<.000001). On Day 35, the 48 animals that had received olanzapine during the weight induction phase were randomized (n=12 per group) to receive one of the following regimens: placebo, CORT 108297 (20mg/kg), CORT 108297 (60mg/kg), CORT 108297 (120mg/kg) for the subsequent 21 days. There were robust, statistically significant, differences in weight between the olanzapine plus placebo and olanzapine plus CORT 108297 groups: Animals receiving olanzapine and placebo continued to gain significant body weight from day 35 to 56 (p<.0001) while animals receiving olanzapine plus CORT 108297 (all doses) exhibited significant weight reduction (p<.00001). At the highest dose tested (120 mg/kg), the animals' weight returned to levels observed prior to initial olanzapine ingestion. The results of this first study suggest that after significant weight gain from olanzapine has already occurred, CORT 108297 can be introduced while olanzapine is continued and reverse the weight gain caused by olanzapine.

In the second study, rats (n = 96) were dosed with placebo, olanzapine (2.4 mg/kg), or, olanzapine plus CORT 108297 (2, 6, 20, 60, or 120 mg/kg) for 21 days. From baseline to day 21, rats administered olanzapine plus CORT 108297 gained significantly less weight than rats receiving olanzapine and placebo (p <.00001). Larger doses of CORT 108297 were significantly correlated with greater weight reduction (p<.00001). This second study suggests that when CORT 108297 is administered concomitantly with olanzapine, weight gain associated with the use of olanzapine can be prevented or at least attenuated.

These first two studies used dose levels of 20 mg/kg, 60 mg/kg and 120 mg/kg of CORT 108297. The results of these two experiments replicated the findings from previous animal studies of mifepristone, and were also consistent with results from randomized trials conducted in humans. The results were presented at the International Society of Psychoneuroendocrinology and the World Congress of Biological Psychiatry conferences in July 2009 and were published in the peer-reviewed journal, *Diabetes Obesity and Metabolism* in 2010.

A third study in the rat further evaluated the dose response relationship of CORT 108297 in preventing olanzapine induced weight gain with doses from 2 mg/kg to 20 mg/kg.





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At the American Diabetes Association conference in June 2009 there was also a presentation of preclinical data from a study which demonstrated that CORT 108297 suppresses body weight gain and improves insulin sensitivity in healthy mice fed a 60% fat diet and high sucrose liquid. In 2011, these study results were published in the peer-reviewed publication, *The Journal of Nutrition and Metabolism*.

The manufacturing and preclinical development of CORT 108297 began late in 2008 and resulted in the submission of an IND to the FDA in December 2009. Dosing of healthy volunteers in the first Phase 1 study of CORT 108297 was completed in July 2010. This initial study was a single dose escalation study in healthy volunteers. We continue to evaluate CORT 108297 in exploratory Phase 2a studies in models of antipsychotic induced weight gain and changes in biomarkers induced by prednisone, a steroid.

If any of our selective GR-II antagonists prove to mitigate the weight gain and metabolic disturbances associated with the use of antipsychotic medication, they could potentially be of benefit to the millions of people currently taking this important pharmacotherapy.

## **Proof-of-Concept Studies**

### *Metabolic Disorders*

In April 2005, we announced results from two preclinical studies conducted in a rat model of olanzapine-induced weight gain. These studies demonstrated that mifepristone's GR-II antagonist action has the potential to both reverse the weight gain associated with olanzapine and to prevent the weight gain associated with the initiation of treatment with olanzapine, which led to our studies in humans.

In 2007, we announced results of our human clinical proof-of-concept study evaluating the ability of mifepristone to mitigate weight gain associated with the administration of Eli Lilly's Zyprexa (olanzapine). The results indicated a statistically significant reduction in weight gain in those subjects who took Zyprexa plus mifepristone compared to those who took Zyprexa plus placebo. Eli Lilly provided Zyprexa and financial support for this study. During 2009, we announced results from another proof-of-concept study evaluating the ability of mifepristone to mitigate weight gain associated with the administration of Johnson & Johnson's Risperdal (risperidone). The results indicated a statistically significant reduction in weight gain in those subjects who took Risperdal plus mifepristone compared to those who took Risperdal plus placebo. Both Zyprexa and Risperdal are indicated for the treatment of schizophrenia and bipolar disorder.

In the study of mifepristone and Zyprexa, 57 lean, healthy men (body mass index of 25 or less) were randomized to receive either Zyprexa plus placebo (n=22), Zyprexa plus mifepristone (n=24) or mifepristone plus placebo (n=11). This study took place in an institutional setting where daily weights were recorded and a range of metabolic parameters were measured. In the two week study, subjects in the Zyprexa plus placebo group gained an average of 7.0 pounds and subjects in the Zyprexa plus mifepristone group gained an average of 4.4 pounds; which is a statistically significant difference (p<.001). Subjects in the mifepristone plus placebo group gained an average of 4.4 pounds. The difference in weight gain trajectory was apparent in the first days of the study, reaching statistical significance during the first week. The increase in waist circumference, a surrogate for abdominal fat, in subjects who received Zyprexa plus placebo was also significantly greater than subjects who received Zyprexa plus mifepristone (p<.01). The study was not designed to enroll a sufficient number of patients to have statistical power to detect significant effects on metabolic measures; however, the effect of mifepristone in this model was greater than expected. In addition to the finding about waist circumference, notable additional non-statistically significant group differences were observed. Patients taking Zyprexa plus placebo experienced greater increases from baseline to end of study in both triglycerides and fasting insulin compared to patients taking Zyprexa plus mifepristone. No unexpected study drug related adverse events were observed. These results were published in *Advances in Therapy* in 2009.

In the study of mifepristone and Risperdal, 75 lean, healthy men (body mass index of 23 or less) were randomized to receive either Risperdal plus placebo (n=30), Risperdal plus mifepristone (n=30) or mifepristone plus placebo (n=15). This study also took place in an institutional setting where daily weights were recorded and

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a range of metabolic parameters were measured. In this four-week randomized double-blind controlled study, subjects in the Risperdal plus placebo group gained an average of 9.2 pounds, compared to a gain of 5.1 pounds in the Risperdal plus mifepristone group. This difference was statistically significant ( $p < 0.0001$ ). Additional important metabolic parameters, including fasting insulin, triglycerides and abdominal fat, as reflected by waist circumference, were also measured. The addition of mifepristone to Risperdal resulted in a statistically significant reduction in fasting insulin levels, triglyceride levels, and abdominal fat (as measured by waist circumference). Consistent with prior studies, mifepristone appeared to be well tolerated. These results were published in *Obesity* in 2010.

The combinations of Zyprexa and mifepristone or Risperdal and mifepristone are not approved for any indication. The purpose of these studies was to explore the hypothesis that GR-II antagonists would mitigate weight gain and other metabolic effects associated with antipsychotic medications. The group of medications sometimes referred to as atypical antipsychotics, including Zyprexa, Risperdal, Clozaril (clozapine) and Seroquel (quetiapine), are widely used to treat schizophrenia and bipolar disorder. All medications in this group are associated with treatment-emergent weight gain of varying degrees and carry a warning in the label relating to treatment-emergent hyperglycemia and diabetes mellitus.

### *Other Disorders*

We have collaborated with researchers investigating the utility of mifepristone and some of our next-generation selective GR-II antagonists in pre-clinical and human proof-of-concept studies in a wide range of disorders, including alcoholism, post-traumatic stress disorder, Alzheimer's disease, and ovarian cancer.

### **Research and Development**

We incurred approximately \$14.1 million, \$21.0 million and \$18.9 million of research and development expenses in the years ended December 31, 2012, 2011 and 2010, respectively, which accounted for approximately 36%, 65% and 69% of our total operating expenses in these respective fiscal years. For a further discussion, see Part II, Item 7, Management's Discussion and Analysis of Financial Conditions and Results of Operations – Results of Operations.

### **Manufacturing Korlym**

As a drug discovery, development and commercialization company, we intend to continue to utilize our financial resources to commercialize Korlym and advance other product candidates rather than diverting resources to establishing our own manufacturing facilities.

We intend to continue to rely on experienced contract manufacturers to produce our product candidates. We have entered into a manufacturing agreement with one contract manufacturer, Produits Chimiques Auxiliaires et de Synthèse SA (PCAS), to produce the active pharmaceutical ingredient (API) for Korlym. The FDA approved our commercial use of material produced by PCAS as part of our NDA submission for Korlym. The agreement with PCAS, which was executed in November 2006, was for an initial period of five years with an automatic extension for one additional year and has been extended to June 2013. We are pursuing discussions to continue the relationship thereafter. The agreement calls for us to purchase from PCAS at least 75 percent of our requirements until the expiration of the agreement. If PCAS is unable to manufacture the product for a consecutive six-month period, we have the right to terminate the agreement, without penalty.

We have also entered into an agreement with another contract manufacturer, PharmaForm, L.L.C. (PharmaForm), for the production of Korlym tablets. The agreement with PharmaForm was executed in March 2012 for an initial period of two years. The agreement will be automatically extended for additional one year periods unless one Party gives six months' prior written notice that it does not want such an extension. The agreement with PharmaForm may be terminated by either party upon 180 days written notice; we may terminate projects initiated under this agreement with 30 days written notice. There are no minimum purchase amounts under this agreement.

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We are currently in negotiations for a commercial manufacturing agreement with AAI Pharma, our second tablet manufacturer whose facility was approved by the FDA for manufacture of our commercial Korlym tablets in November 2012.

**Competition for Korlym**

Korlym competes with established treatments, including surgery, radiation, and approved medicines prescribed off-label. Korlym will also compete with Novartis' drug, Signifor® (pasireotide) Injection, which the FDA approved in December 2012 for the treatment of adult patients with Cushing's disease (a subset of Cushing's syndrome) who are not candidates for pituitary surgery or for whom surgery did not work. In April 2012, Signifor received marketing approval in the EU. It has Orphan Drug designation in both the United States and the EU. Signifor is a somatostatin analogue that inhibits ACTH production by the pituitary, which leads to reduced cortisol production. In the Phase 3 study that served as the basis for Novartis' NDA, the drug normalized cortisol levels in 26 percent of patients. Sixty-seven percent of patients developed hyperglycemia or diabetes. Signifor must be taken twice daily, by injection.

Korlym may also experience competition from compounds under development for Cushing's syndrome. We are aware that Laboratoire HRA Pharma (HRA) has received an Orphan Drug Designation in the United States and Europe for the use of mifepristone to treat a subtype of Cushing's syndrome and has begun a clinical trial in Europe and the United States. If this product is approved for commercialization in the United States and the EU, our potential future revenue could be reduced. We are also aware that Exelgyn Laboratories, which operates as a subsidiary of Medi Challenge (Pty) Ltd., received Orphan Drug Designation for endogenous Cushing's syndrome in Europe, but they have stated that they have not yet conducted any clinical trials.

Many colleges, universities and public and private research organizations are also active in the human health care field. While these entities focus on education, they may develop or acquire proprietary technology that we may require for the development of our product candidates. We may attempt to obtain licenses to this proprietary technology.

Our ability to compete successfully will be based on our ability to develop proprietary products, attract and retain scientific personnel, obtain patent or other protection for our product candidates, obtain required regulatory approvals and manufacture and successfully market Korlym and our future products either alone or through outside parties.

**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.

Under an agreement with Stanford University, we have licensed exclusive rights to the following issued U.S. patents and any corresponding foreign patents:

U.S. Patent Number	Subject Matter	Expiration Date
6,150,349	Use of GR-II antagonists in the treatment of psychotic major depression	October 5, 2018
6,362,173	Use of GR-II antagonists in the treatment of cocaine-induced psychosis	October 5, 2018
6,369,046	Use of GR-II antagonists in the treatment of early dementia	February 4, 2019

The corresponding foreign patents expire in 2018.

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We are required to make milestone payments and pay royalties to Stanford University on sales of products commercialized under any of the above patents. We are currently in compliance with our obligations under the agreement. If Stanford University were to terminate any of our exclusive licenses due to breach of the license on our part, we would not be able to commercialize mifepristone for the treatment of the psychotic features of psychotic depression, cocaine-induced psychosis or early dementia.

We also own issued U.S. patents for the use of GR-II antagonists in the treatment of mild cognitive impairment, for the treatment of weight gain following treatment with antipsychotic medication, for the prevention and treatment of stress disorders, for increasing the therapeutic response to ECT, for the treatment of delirium, for the treatment of catatonia, for the treatment of gastroesophageal reflux disease and for inhibiting cognitive deterioration in adults with Down's Syndrome. The expiration dates of these patents and their foreign counterparts range from 2020 to 2028.

In addition, we have nine U.S. method-of-use applications covering certain GR-II antagonists, including the treatment of:

patients suffering from mental disorders by optimizing mifepristone levels in plasma serum;

neurological damage in premature infants;

muscular dystrophy;

migraine headaches;

psychosis associated with interferon-alpha therapy;

depression in patients taking Interleukin-2 (IL-2); and

amyotrophic lateral sclerosis (ALS).

The approximate expiration dates of the patents that could issue from these applications and their foreign counterparts range from 2023 to 2032.

We have composition of matter claims on three patent families of novel selective GR-II antagonists. Applications for all of the three families have been allowed in both the United States and Europe. The expiration dates of these U.S. and European patents range from 2026 to 2027. Two additional composition of matter patent applications are pending.

We have also filed, where we deemed appropriate, foreign patent applications corresponding to our U.S. patents and applications.

However, we cannot assure you that any of our patent applications will result in the issuance of patents, that any issued patent will include claims of the breadth sought in these applications, or that competitors will not successfully challenge or circumvent our patents if they are issued.

Although three of our patents have claims directed to the composition of compounds, we do not have a patent with claims directed to the composition of mifepristone. Our rights under our issued patents related to mifepristone cover only the use of that compound in the treatment of specific diseases.

The composition of matter patent covering mifepristone has expired. The only previously 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 and EU, (2) the restrictions imposed by the FDA on the use of mifepristone to terminate pregnancy, (3) the different patient populations, administering

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physicians and treatment settings between the use of mifepristone to terminate pregnancy and to treat Cushing's syndrome and (4) our method of use patents described above.

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The patent positions of companies in the pharmaceutical industry are highly uncertain, involve complex legal and factual questions and have been and continue to be the subject of much litigation. Our product candidates may give rise to claims that we infringe on the products or proprietary rights of others. If it is determined that our drug candidates infringe on others' patent rights, we may be required to obtain licenses to those rights. If we fail to obtain licenses when necessary, we may experience delays in commercializing our product candidates while attempting to design around other patents, or determine that we are unable to commercialize our product candidates at all. If we do become involved in intellectual property litigation, we are likely to incur considerable costs in defending or prosecuting the litigation. We believe that we do not currently infringe any third party's patents or other proprietary rights, and we are not obligated to pay royalties relating to the use of intellectual property to any third party other than Stanford University.

In 2004 Akzo Nobel filed an observation to the grant of our exclusively licensed European patent application with claims directed to psychotic depression. In February 2006, the EPO allowed our patent application. We are not aware of any other disputes related to patent issues.

## **License Agreement**

Under our exclusive license agreement with Stanford University to patents covering the use of mifepristone to treat the psychotic features of psychotic depression and for the treatment of early dementia, we are required to pay Stanford \$50,000 annually as a nonrefundable royalty payment. This payment is creditable against future royalties. We are also obligated to pay Stanford a \$50,000 milestone upon the filing of the NDA for mifepristone for the treatment of psychotic depression and a further \$200,000 milestone payment upon FDA approval of mifepristone for that indication. The milestone payments are also creditable against future royalties. This license agreement expires upon expiration of the related patents or upon notification by us to Stanford relating to the use of intellectual property. See Intellectual Property.

## **Government Regulation**

Prescription pharmaceutical products are subject to extensive pre- and post-approval regulation, including regulations that govern the testing, manufacturing, safety, efficacy, labeling, storage, record keeping, advertising, and promotion of the products under the Federal Food, Drug and Cosmetic Act. All of our product candidates will require regulatory approval by government agencies prior to commercialization. The process required by the FDA before a new drug may be marketed in the United States generally involves the following: completion of preclinical laboratory and animal testing; submission of an IND, which must become effective before clinical trials may begin; performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed drug or biologic's intended use; and, in the case of a new drug, approval by the FDA of an NDA. The process of complying with these and other federal and state statutes and regulations in order to obtain the necessary approvals and subsequently complying with federal and state statutes and regulations involves significant time and expense.

Preclinical studies are generally conducted in laboratory animals to evaluate the potential safety and the efficacy of a product. Drug developers submit the results of preclinical studies to the FDA as a part of an IND, which must be approved before beginning clinical trials in humans. Typically, human clinical trials are conducted in three sequential phases that may overlap.

Phase 1. Clinical trials are conducted with a small number of subjects to determine the early safety profile, maximum tolerated dose and pharmacokinetics of the product candidate in human volunteers.

Phase 2. Clinical trials are conducted with groups of patients afflicted with a specific disease to determine preliminary efficacy, optimal dosages and expanded evidence of safety.

Phase 3. Large-scale, multi-center, comparative trials are conducted with patients afflicted with a target disease to establish the overall risk/benefit ratio of the drug and to provide enough data to demonstrate with substantial evidence the efficacy and safety of the product, as required by the FDA.

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The FDA and the Institutional Review Boards closely monitor the progress of each of the three phases of clinical trials that are conducted in the United States and may reevaluate, alter, suspend or terminate the testing at any time for various reasons, including a belief that the subjects are being exposed to an unacceptable health risk. The FDA may also require that additional studies be conducted, such as studies demonstrating that the drug being tested does not cause cancer.

After Phase 3 trials are completed, drug developers submit the results of preclinical studies, clinical trials, formulation studies and data supporting manufacturing to the FDA in the form of an NDA for approval to commence commercial sales. The FDA reviews all NDAs submitted before it accepts them for filing. The FDA may request additional information rather than accept an NDA for filing. If the FDA accepts an NDA for filing, it may grant marketing approval, request additional information or deny the application if it determines that the application does not meet regulatory approval criteria. Once an NDA has been accepted for filing, by law the FDA has 180 days to examine the application and respond to the applicant. However, the review process is often significantly extended by FDA requests for additional information or clarification. Under the Prescription Drug User Fee Act, the FDA has a goal of responding to NDAs within ten months of the filing date for standard review, and 6 months for priority review if a sponsor shows that its drug candidate provides a significant improvement compared to marketed drugs. The FDA has indicated to us that it will grant us a priority review of our NDA of mifepristone for the treatment of the psychotic features of psychotic depression if no other medications have been approved for this indication at the time of our submission. FDA approvals may not be granted on a timely basis, or at all.

If the FDA approves an NDA, the subject drug becomes available for physicians to prescribe in the United States. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-approval regulatory standards is not maintained. The drug developer must submit periodic reports to the FDA. Adverse experiences with the product must be reported to the FDA and could result in the imposition of marketing restrictions through labeling changes or product removal. Product approvals may be withdrawn if problems with safety or efficacy occur after the product reaches the marketplace. In addition, the FDA may require post-marketing studies, referred to as Phase 4 studies, to monitor the effect of approved products, and may limit further marketing of the product based on the results of these post-approval studies.

Facilities used to manufacture drugs are subject to periodic inspection by the FDA and other authorities where applicable, and must comply with current Good Manufacturing Practices regulations (cGMP). Failure to comply with the statutory and regulatory requirements subjects the manufacturer to possible legal or regulatory action, such as suspension of manufacturing, seizure of product or voluntary recall of a product.

With respect to post-approval product advertising and promotion, the FDA imposes a number of complex regulations on entities that advertise and promote pharmaceuticals, which include, among others, standards and regulations for direct-to-consumer advertising, off-label promotion, industry sponsored scientific and educational activities, and promotional activities involving the Internet. The FDA has very broad enforcement authority under the Federal Food, Drug and Cosmetic Act, and failure to abide by these regulations can result in penalties including the issuance of a warning letter directing a company to correct deviations from FDA standards, a requirement that future advertising and promotional materials be pre-cleared by the FDA, and state and federal civil and criminal investigations and prosecutions.

In addition to studies requested by the FDA after approval, a drug developer may conduct other studies to explore use of the approved compound for treatment of new indications. The purpose of these trials and studies and related publications is to broaden the application and use of the drug and its acceptance in the medical community. Data supporting the use of a drug for these new indications must be submitted to the FDA in a new or supplemental NDA that must be approved by the FDA before the drug can be marketed for the new indications.

*Orphan Drug Designation.* We have received Orphan Drug designation for Korlym for the treatment of endogenous Cushing's syndrome in both the United States and the EU. In the United States, Orphan Drug



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designation provides special status to a product to treat a rare disease or condition providing that the product meets certain criteria. Orphan designation qualifies the sponsor of the product for the tax credit and marketing incentives of the Orphan Drug Act, including seven years of exclusive marketing rights for the specific drug for the orphan indication, if it receives the first regulatory approval for that indication, with limited exceptions. A marketing application for a prescription drug product that has been designated as a drug for a rare disease or condition is not subject to a prescription drug user fee unless the application includes an indication for other than a rare disease or condition. Orphan Drug designation does not prevent competitors from developing or marketing different drugs for an indication. It also does not convey an advantage in, or shorten the duration of, the review and approval process for a drug by the applicable regulatory authority.

Benefits of Orphan Drug Designation in the EU are similar to those in the U.S., but include ten years of marketing exclusivity in all 27 member states, free scientific advice during drug development, access to a centralized review process and a reduction or complete waiver of fees levied by the European Medicines Agency.

*Approvals outside the United States.* Other than applying for and receiving Orphan Drug Designation for Korlym for Cushing's syndrome in the EU, we have not started the regulatory approval process in any jurisdiction other than the United States and we are unable to estimate when, if ever, we will commence the regulatory approval process in any foreign jurisdiction. We or our potential future partners will have to complete an approval process similar to the U.S. approval process in foreign target markets for our product candidates before we can commercialize our product candidates in those countries. The approval procedure and the time required for approval vary from country to country and can involve additional testing. Foreign approvals may not be granted on a timely basis, or at all. Regulatory approval of pricing is required in most countries other than the United States. The prices approved may be too low to generate an acceptable return to us.

*Fast Track Designation.* The FDA sometimes grants fast track status under the Food and Drug Administration Modernization Act of 1997. The fast track mechanism is intended to facilitate the development and approval of new drugs intended for the treatment of serious or life-threatening diseases or conditions and which demonstrate the potential to address unmet medical needs for the disease or condition. The fast track process includes scheduling of meetings to seek FDA input into development plans, the option of submitting an NDA serially in sections rather than submitting all components simultaneously, the option to request evaluation of studies using surrogate endpoints, and the potential for a priority review.

We have been granted fast track status for mifepristone for the treatment of the psychotic features of psychotic depression. However, the fast track designation may be withdrawn by the FDA at any time. The fast track designation does not guarantee that we will qualify for or be able to take advantage of the expedited review procedures and does not increase the likelihood that mifepristone will receive regulatory approval.

*Priority Review.* The FDA has indicated to us that it will grant us a priority review of our NDA of mifepristone for the treatment of the psychotic features of psychotic depression if no other medications have been approved for this indication at the time of our submission.

**Executive Officers**

The following table sets forth, as of March 1, 2013, information about our executive officers:

<b>Name</b>	<b>Age</b>	<b>Position</b>
Joseph K. Belanoff, M.D.	55	Chief Executive Officer and Director
Robert L. Roe, M.D.	72	President and Secretary
G. Charles Robb	50	Chief Financial Officer
Steven Lo	45	Vice President of Commercial Operations
Anne M. LeDoux	65	Vice President, Controller and Chief Accounting Officer

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*Joseph K. Belanoff, M.D.* is a co-founder of our company and has served as a member of our Board and as our Chief Executive Officer since 1999. Dr. Belanoff is currently a clinical faculty member and has held various positions in the Department of Psychiatry and Behavioral Sciences at Stanford University since 1992. Dr. Belanoff received his B.A. from Amherst College and his M.D. from Columbia University College of Physicians & Surgeons. Our Board selected Dr. Belanoff to serve as a director because, as our Chief Executive Officer, he brings expertise and knowledge regarding our business and operations to our Board of Directors. Dr. Belanoff also has expertise in clinical medicine and psychopharmacology.

*Robert L. Roe, M.D.* joined us as President in October 2001. Dr. Roe has spent more than 35 years in the pharmaceutical and biotechnology industries. From 1999 to 2001, he served as President and Chief Executive Officer of Allergenic, Inc. From 1996 to 1999, he was Executive Vice President, Chief Operating Officer and a director of Cytel Corporation. From 1995 to 1996, he was Executive Vice President, Chief Operating Officer and a director of Chugai Biopharmaceuticals, Inc. From 1992 to 1995, Dr. Roe served as President of the Development Research Division and Senior Vice President of Syntex Corporation. Dr. Roe received his B.A. from Stanford University and his M.D. from the University of California, San Francisco.

*G. Charles Robb* joined us as Chief Financial Officer in September 2011. Mr. Robb has more than 25 years of experience in executive management, operations and finance. From April 2005 through August 2011 Mr. Robb served as the Senior Vice President of Operations, Administration and Finance of Fitness Anywhere, Inc. (FAI), a private fitness equipment and training company with operations in the United States, Europe and Asia. From 2003 to 2005, Mr. Robb was engaged in the private practice of law. From 2000 to 2002 he was Senior Vice President of Citadon, Inc. He also held positions in business development for Normura Asset Capital Corporation from 1998 to 1999 and in sales and marketing for Legal Research Network, Inc. from 1996 to 1998. From 1992 to 1996 Mr. Robb practiced law at Howard, Rice, Nemerovski, Canady, Falk & Rabkin. Mr. Robb earned a B.A. in English and Political Philosophy from Yale and a J.D. from Harvard Law School, where he was a member of the Harvard Law Review.

*Steven Lo* joined us as Vice President of Commercial Operations in September 2010. Mr. Lo has more than 18 years of commercial experience in the pharmaceutical and biotechnology industry. From 1997 to 2010, Mr. Lo held various positions in marketing, sales and managed markets at Genentech, Inc., a biotechnology company that became a member of the Roche Group in March 2009, most recently as Franchise Head, leading that company's endocrinology marketing and sales organization. Mr. Lo received his B.S. degree from the University of California, Davis and his Master of Health Administration from the University of Southern California.

*Anne M. LeDoux* joined us as Controller in 2004 and was promoted to the position of Vice President, Controller and Chief Accounting Officer in April 2007. Ms. LeDoux has over 20 years of financial and accounting management experience with public pharmaceutical and biotechnology companies. Prior to joining Corcept in 2004, Ms. LeDoux served in various financial positions at Aviron, Roche Biosciences and Syntex Corporation. She was also Vice President and Chief Financial Officer at the Northern California Health Center and Vice President, Finance for the Children's Hospital of San Francisco. Ms. LeDoux is a Certified Public Accountant with over 13 years of experience in public accounting, primarily at Coopers and Lybrand. Ms. LeDoux received her Bachelor of Arts degree in Business from the University of Massachusetts and a law degree from Western New England College, School of Law.

## **Employees**

We are managed by a core group of experienced pharmaceutical executives with a track record of bringing new drugs to market. To facilitate advancement of development programs, we also enlist the expertise of associates and advisors with extensive pharmaceutical development experience.

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As of December 31, 2012, we had 38 full-time employees, six part-time employees, a contracted sales force of 13 sales representatives and 12 long-term contract staff. Four of our employees have M.D.s. We consider our employee relations to be good. None of our employees is covered by a collective bargaining agreement.

## **General**

We were incorporated in the State of Delaware on May 13, 1998. Our registered trademarks include Corcept® and CORLUX®. A trademark is pending with respect to Korlym. Other service marks, trademarks and trade names referred to in this document are the property of their respective owners.

## **Available Information**

We are subject to the information requirements of the Securities Exchange Act of 1934, as amended, and we therefore file periodic reports, proxy statements and other information with the SEC relating to our business, financial statements and other matters. The reports, proxy statements and other information we file may be inspected and copied at prescribed rates at the SEC's Public Reference Room, 100 F Street, N.E., Washington, D.C. 20549, on official business days during the hours of 10:00 A.M. to 3:00 P.M. You may obtain information on the operation of the SEC's Public Reference Room by calling the SEC at 1-800-SEC-0330. The SEC also maintains an Internet site that contains reports, proxy statements and other information regarding issuers like us that file electronically with the SEC. The address of the SEC's Internet site is [www.sec.gov](http://www.sec.gov). For more information about us, please visit our website at [www.corcept.com](http://www.corcept.com). You may also obtain a free copy of our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and amendments to those reports on the day the reports or amendments are filed with or furnished to the SEC by visiting our website at [www.corcept.com](http://www.corcept.com). The information found on, or otherwise accessible through, our website, is not incorporated information, and does not form a part of, this Form 10-K.

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### **ITEM 1A. RISK FACTORS**

*An investment in our common stock involves significant risks. You should carefully consider the risks described below and the other information in this Annual Report on Form 10-K, including our financial statements and related notes, before you decide to invest in our common stock. If any of the following risks or uncertainties actually occurs, our business, results of operations or financial condition could be materially harmed, the trading price of our common stock could decline and you could lose all or part of your investment. The risks and uncertainties described below are those that we currently believe may materially affect us; however, they may not be the only ones that we face. Additional risks and uncertainties of which we are unaware or currently deem immaterial may also become important factors that may harm our business. Except as required by law, we undertake no obligations to update any risk factors.*

#### **Risks Related to the Commercialization of Korlym**

##### **and Development of Mifepristone and Our Other Proprietary GR-II Antagonists**

**We depend heavily on the success of Korlym, which we only recently began to sell. If we are unable to commercialize Korlym successfully, or experience significant delays in doing so, we may not generate revenues as quickly as or at the levels that we or investors expect and our stock price will likely decline.**

We anticipate that for the foreseeable future our ability to generate meaningful revenues and achieve profitability will be solely dependent on the successful commercialization of Korlym. Many factors could harm our efforts to commercialize Korlym, including:

an inability to generate meaningful revenue due to low product usage, inadequate coverage and reimbursement or other factors;

competition from Novartis's Signifor and from other companies with greater financial, technical and marketing resources than ours.

an inability to manufacture Korlym or the active ingredient in Korlym in commercial quantities and at an acceptable cost;

political concerns relating to other uses of mifepristone, or RU-486, that could limit the market acceptance of Korlym;

negative, inconclusive or otherwise unfavorable results from any post-approval studies we conduct;

previously unknown, serious side effects that may be identified; and

rapid technological change making Korlym obsolete.

**Even if we are able to commercialize Korlym successfully, we cannot predict the rate at which success will occur.**

As our current ability to generate revenue is wholly dependent upon the commercialization of Korlym, its rate of sale will directly and materially affect our results of operations. There are inherent difficulties in predicting the volumes of Korlym that will be sold, which are heightened by our relative inexperience commercializing Korlym or other products. Failure of our revenue to meet the expectations of investors could cause our stock price to decline. See also the discussion below under "The failure of our financial results to meet estimates published by research analysts or other investor expectations could cause our stock price to decline."

**Physicians may accept Korlym slowly or may never accept it, which would adversely affect our financial results.**

Many factors may affect the market acceptance and commercial success of Korlym.

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Even though the FDA has approved Korlym, physicians may not adopt it as a treatment for their eligible patients. Physicians will prescribe Korlym only if they determine, based on experience, clinical data, side effect

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profiles and other factors, that it is preferable to other products or treatments currently in use, even if those products are not approved for Cushing's syndrome. Because Cushing's syndrome is rare, most physicians are inexperienced in the care of patients with the illness and it may be difficult to persuade them to prescribe a new treatment, such as Korlym, even with clinical trial results that suggest that it may be a compelling treatment for them to consider.

Other factors that may affect the market acceptance and commercial success of Korlym include:

the effectiveness of Korlym, including any side effects, as compared to alternative treatment methods;

the rate of adoption of Korlym by physicians and by target patient populations;

the possible preference of some physicians for more familiar, long-standing off-label treatments for Cushing's syndrome or for Novartis' drug, Signifor, for the treatment of Cushing's disease;

the cost-effectiveness of Korlym and the availability of third-party insurance coverage and reimbursement, in particular from government payors such as Medicare and Medicaid, for patients using Korlym;

the product labeling required by the FDA for Korlym;

the extent and success of our efforts to manufacture, commercialize, market, distribute and sell Korlym; and

negative publicity concerning Korlym, RU-486, Mifeprex® or mifepristone.

The failure of Korlym to achieve market acceptance would prevent us from generating meaningful revenue.

### **The Orphan Drug Designation for Korlym may not provide protection from competition and other benefits as anticipated.**

In July 2007, we received Orphan Drug Designation from the FDA for Korlym for the treatment of hyperglycemia secondary to hypercortisolism in adult patients with endogenous Cushing's syndrome who have type 2 diabetes mellitus or glucose intolerance and have failed surgery or are not candidates for surgery. Drugs that receive Orphan Drug Designation are eligible to obtain seven years of marketing exclusivity for the approved indication from the date of drug approval, with limited exceptions, as well as tax credits for clinical trial costs, marketing application filing fee waivers and assistance from the FDA in the drug development process.

In October 2011, the European Commission granted us Orphan Drug Designation for Korlym for the treatment of endogenous Cushing's syndrome (hypercortisolism) in the EU. Benefits of Orphan Drug Designation in the EU are similar to those in the United States, but include ten years of marketing exclusivity for the approved indication in all 27 member states, free scientific advice during drug development, access to a centralized review process and a reduction or complete waiver of fees levied by the European Medicines Agency.

Although we have received Orphan Drug Designation in both the United States and the EU, we cannot be assured that we will recognize the potential benefits of these designations. Even after an orphan drug is approved for its orphan indication, the FDA can subsequently approve a different drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. In addition, the FDA may, during the seven-year orphan drug exclusivity period, approve the same drug for a different indication.

Notwithstanding Korlym's Orphan status in both the United States and the EU, in 2012 Novartis received approval in both jurisdictions to market its somatostatin analogue Signifor for adult patients with Cushing's disease (a subset of Cushing's syndrome that afflicts approximately 70 percent of all Cushing's syndrome patients) for whom pituitary surgery is not an option or has not been curative. Novartis has substantially more resources and experience than Corcept and may provide significant competition.



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Further, we are aware that Laboratoire HRA Pharma has received Orphan Drug Designation in the United States and the EU for the use of mifepristone to treat a subtype of Cushing's syndrome and has begun a Phase 2 clinical trial in Europe and the United States for this indication. We are also aware that Exelgyn Laboratories, which operates as a subsidiary of Medi Challenge (Pty) Ltd., received Orphan Drug Designation for Cushing's syndrome in the EU, but it has stated that it has not yet conducted any clinical trials.

If another drug with mifepristone as its active ingredient is approved in the EU for Cushing's syndrome before Korlym, we will not receive the ten years of marketing exclusivity from the date of drug approval in the EU and other potential benefits. Any delay in our commercialization of Korlym may have a negative impact on the revenue that we might be able to realize from the exclusivity provided during the applicable periods.

**We will face competition from companies that attempt to develop mifepristone or other compounds for the treatment of Cushing's syndrome, which could limit our future revenues from the commercialization of Korlym and which could have a negative impact on future revenues from the commercialization of Korlym for any indication. These companies may have significantly more resources than we do.**

We will experience competition from Novartis, which has received approval in the United States and the EU to market Signifor (pasireotide), an injectable somatostatin analogue, for the treatment of adult patients with Cushing's disease (a subset of the patients with Cushing's syndrome) for whom pituitary surgery is not an option or has not been curative. We expect Novartis to begin promoting Signifor in the United States.

In addition, we are aware that Laboratoire HRA Pharma has begun a Phase 2 clinical trial in Europe and the United States evaluating the use of mifepristone to treat a subtype of Cushing's syndrome, and that Exelgyn Laboratories may be planning to develop a Cushing's syndrome product, although it has stated that it has not conducted any clinical trials to date. See also the discussion above under "The Orphan Drug Designation for Korlym may not provide protection from competition and other benefits as anticipated. If another product for treatment of Cushing's syndrome or Cushing's disease is approved for commercialization, our potential future revenue could be reduced.

**If we are unable to continue to obtain acceptable prices or adequate coverage and reimbursement for Korlym from third-party payors, we will be unable to generate significant revenues.**

There is significant uncertainty related to the availability of third-party insurance coverage and reimbursement for newly approved medications. The commercial success of our medications in both domestic and international markets depends on whether third-party coverage and reimbursement is available for them. Government payors, including Medicare and Medicaid, health maintenance organizations and other third-party payors are increasingly attempting to contain healthcare costs by limiting both coverage and the level of reimbursement of new medicines, and, as a result, they may not cover or provide adequate payment for our medications. Our near-term dependence on the commercial success of Korlym makes us particularly susceptible to any such cost containment or reduction efforts. Accordingly, even though Korlym has been approved for commercial sale, unless government and other third-party payors continue to provide adequate and timely coverage and reimbursement, physicians may not prescribe it and patients may not purchase it. In addition, meaningful delays in insurance coverage for individual patients may increase our costs and reduce our revenues. Further, we will need to obtain approvals from hospital formularies before Korlym can be reimbursed for in-patient treatment. If we fail to obtain such approvals, this will reduce the level of revenues that we are able to attain.

In some foreign markets, pricing and profitability of prescription pharmaceuticals are subject to government control. In the United States, we expect that there will continue to be federal and state proposals for similar controls. Also, the trends toward managed health care in the United States and recent laws and legislation intended to reduce the cost of government insurance programs could significantly influence the purchase of health care services and products and may result in lower prices for our products or the exclusion of such products from reimbursement programs.



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In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, collectively referred to as the PPACA, was passed. The PPACA included, among other things, the following measures:

annual, non-deductible fees on any entity that manufactures or imports certain prescription drugs and biologics;

increases in Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program for both branded and generic drugs;

a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in and conduct comparative clinical research;

new requirements for manufacturers to discount drug prices to eligible patients by 50 percent at the pharmacy level and for mail order services in order for their outpatient drugs to be covered under Medicare Part D;

an increase in the number of entities eligible for discounts under the Public Health Service pharmaceutical pricing program; and

establishment of a licensure framework for follow-on biologic products.

The PPACA provisions on comparative clinical effectiveness research extended the initiatives of the American Recovery and Reinvestment Act of 2009, also known as the stimulus package, which included \$1.1 billion in funding to study the comparative effectiveness of health care treatments. This stimulus funding was designated for, among other things, conducting, supporting or synthesizing research that compares and evaluates the risks and benefits, clinical outcomes, effectiveness and appropriateness of products. The PPACA also appropriated additional funding to comparative clinical effectiveness research. Although Congress has indicated that this funding is intended to improve the quality of health care, it remains unclear how the research will impact current Medicare coverage and reimbursement or how new information will influence other third-party payor policies.

Since its passage, a number of state governors have strenuously opposed the mandatory purchase of insurance, referred to as the individual mandate, and aspects of voluntary Medicaid expansion under PPACA, and initiated lawsuits challenging its constitutionality. On June 28, 2012, the United States Supreme Court upheld the constitutionality of the individual mandate, and invalidated requirements that states forfeit certain federal funding if they do not expand Medicaid coverage as prescribed by PPACA. The Court left the remainder of PPACA intact.

In addition, other legislative changes have been proposed and adopted in the United States since the PPACA was enacted. Most recently, on August 2, 2011, the Budget Control Act of 2011 among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee did not achieve its targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2 percent per fiscal year, starting in 2013. On January 2, 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, or the ATRA, which, among other things, delayed for another two months the budget cuts mandated by these sequestration provisions of the Budget Control Act of 2011. The ATRA also reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other health care funding, which could have a material adverse effect on our customers and accordingly, our financial operations.

These new laws and the regulations and policies implementing them, as well as other healthcare reform measures that may be adopted in the future, may have a material adverse effect on our industry generally and on our ability to successfully develop and commercialize our products.

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### **We will need to continue to develop our medical education, sales and marketing capabilities to successfully commercialize Korlym and our other proprietary, selective GR-II antagonists.**

To achieve commercial success for any approved product, we must either develop sales and marketing capabilities internally or enter into arrangements with third parties to market and sell our current and future products, and we may not be successful in doing so. We continue to hire experienced field and internal personnel to commercialize Korlym in the United States, which is expensive and time consuming. Although we received approval to market and sell Korlym in February 2012, our efforts to staff, deploy and train a marketing and medical education organization remain in an early stage. Any failure or delay in the development or failure to maintain effectively our internal capabilities for the marketing and sales of Korlym would adversely impact the commercialization of the product. If our efforts to develop an internal commercial marketing and sales team are not successful, cost-effective and timely, we may not achieve profitability.

### **We will need to increase the size of our organization, and we may experience difficulties in managing growth.**

We expect that the development of our commercial organization and the likely future expansion of our research and development efforts will strain our administrative, operational and management resources. Future growth will impose significant added responsibilities on members of management, including the need to identify, recruit, maintain and integrate additional employees. To date, we have relied on a small management team, including a number of part-time contributors. Our future financial performance and our ability to compete effectively will depend, in part, on our ability to manage any future growth effectively.

To that end, we must be able to:

integrate additional management, clinical development, administrative and sales and marketing personnel;

expand the size and composition of our management team;

develop our administrative, accounting and management information systems and controls;

hire and train additional qualified personnel;

manage our sales and marketing efforts effectively;

maintain our supply chain effectively;

manage our clinical trials effectively; and

manage our research and development efforts effectively.

We may not be able to accomplish these tasks, and our failure to accomplish any of them could harm our business.

### **Public perception of the active ingredient in Korlym, mifepristone (also known as RU-486 ), may limit our ability to market and sell Korlym.**

The active ingredient in Korlym, mifepristone (RU-486), is approved by the FDA in another drug for the termination of early pregnancy. As a result, mifepristone has been and continues to be the subject of considerable ethical and political debate in the United States and elsewhere. Public perception of mifepristone may limit our ability to engage alternative manufacturers and may limit the commercial acceptance of Korlym

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by patients and physicians. Even though we have taken measures to minimize the likelihood of the prescribing of Korlym to a pregnant woman, physicians may choose not to prescribe Korlym to a woman simply to avoid any risk of unintentionally terminating a pregnancy. We have taken measures to control the distribution of Korlym to reduce the potential for diversion and this controlled distribution may negatively impact sales of Korlym.

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**We have no manufacturing capabilities and we currently depend on third parties to manufacture the active ingredient and the tablets for Korlym, both of which are single-source suppliers. If these suppliers are unable or unwilling to continue manufacturing Korlym and we are unable to contract quickly with alternative sources, or if these third-party manufacturers fail to comply with FDA regulations or otherwise fail to meet our requirements, our business will be harmed.**

We currently have no experience in, and we do not own facilities for, nor do we plan to develop facilities for, manufacturing any products. We depend on third-party contract manufacturers to supply the active pharmaceutical ingredient, or API, in Korlym and to manufacture the Korlym tablet. In addition, we expect to use third-party manufacturers and suppliers if and when our product candidates are approved. The facilities used by our contract manufacturers to manufacture our products must be approved by the FDA pursuant to inspections. We do not control the manufacturing processes of, and are completely dependent on, our contract manufacturing partners for compliance with the regulatory requirements, known as current good manufacturing practices, or cGMPs. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. In addition, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or a comparable foreign regulatory authority does not approve these facilities for the manufacture of our products or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market our products. In addition, sanctions could be imposed on us, including fines, injunctions, civil penalties, failure of regulatory authorities to grant marketing approval of our product candidates, delays, suspension or withdrawal of approvals, seizures or recalls of products, operating restrictions and criminal prosecutions, any of which could harm our business.

We have only one approved manufacturer of the API in Korlym, with whom our agreement expires in June 2013. We intend to continue the relationship and are in the process of negotiating a new agreement. We have a memorandum of understanding with a second API manufacturer. However, there are no activities currently being conducted at this second manufacturer's site to develop or qualify the manufacturing processes or facilities and we did not request approval of material produced by this second manufacturer when we submitted our NDA for Cushing's syndrome.

We have an agreement with a tablet manufacturer that we included in our NDA submission for Korlym. This tablet manufacturer has temporarily suspended commercial production while it relocates to, and seeks regulatory approval to begin operation of, a new facility. On November 1, 2012, the FDA approved our second Korlym tablet manufacturer as a qualified site for the manufacture of Korlym tablets, with whom we are now negotiating a commercial supply agreement. We cannot assure you that our tablet suppliers will be able or willing to meet our future demands. If our original tablet manufacturer is not able to qualify its new site or if our second Korlym tablet manufacturer is unable to prepare Korlym tablets in the quantities and time frame required, we may not be able to manufacture our product in a timely manner. If our suppliers were to fail to manufacture tablets on a timely basis in the quantities that we require, or fail to maintain manufacturing capabilities that meet FDA standards, we would likely experience a lengthy delay in our manufacturing processes.

Our current arrangements with these manufacturers are terminable by such manufacturers. If we are unable, for whatever reason, to obtain the API or Korlym tablets from our contract manufacturers, we may not be able to manufacture our required quantities or identify alternate manufacturers of mifepristone or Korlym tablets in a timely manner or on reasonable terms, if at all, which would harm our business.

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**If we or others identify previously unknown, serious side effects of mifepristone, we may be required to perform lengthy additional clinical trials, change the labeling of Korlym or withdraw it from the market, any of which would hinder or preclude our ability to generate revenues.**

The FDA's approval of Korlym requires that we conduct a study of the interactions between Korlym and ketoconazole, an anti-fungal agent sometimes used to treat patients with Cushing's syndrome. It also requires us to study drug utilization to better characterize the reporting rates of adverse events associated with the long-term use of Korlym. If we or others identify previously unknown, serious side effects of mifepristone:

regulatory authorities may withdraw their approvals;

we may be required to conduct additional clinical trials, make changes in labeling, implement changes to or obtain re-approvals of our manufacturing facilities;

we may experience a significant drop in the sales of Korlym;

our reputation in the marketplace may suffer; and

we may become the target of lawsuits, including class action lawsuits.

Any of these events could harm or prevent sales of the affected products or could increase the costs and expenses of commercializing and marketing Korlym.

**We may have substantial exposure to product liability claims and may not have adequate insurance to cover those claims.**

We may be subject to product liability or other claims based on allegations that the use of our products has resulted in adverse effects or that our product candidates are not effective, whether by participants in our clinical trials for Korlym or other product candidates, or by patients using Korlym. A product liability claim may damage our reputation by raising questions about Korlym or any of our product candidates' safety or efficacy and could limit our ability to sell a product by preventing or interfering with product commercialization. In some cases, less common adverse effects of a pharmaceutical product are not known until long after the FDA approves the product for marketing. The active ingredient in Korlym is used to terminate pregnancy. Therefore, clinicians using the medicine in our clinical trials and physicians prescribing the medicine to women with childbearing potential must take necessary and strict precautions to ensure that the medicine is not administered to pregnant women. The failure to observe these precautions could result in significant product claims.

We have only limited product liability insurance coverage, with limits that we believe to be customary for a company beginning to commercialize its first pharmaceutical product. We intend to expand our product liability insurance coverage to any product candidates for which we obtain marketing approval. However, this insurance may be prohibitively expensive or may not fully cover our potential liabilities. Our inability to obtain adequate insurance coverage at an acceptable cost could prevent or inhibit the commercialization of Korlym or any of our product candidates, or result in meaningful underinsured or uninsured liability. Defending a lawsuit could be costly and significantly divert management's attention from conducting our business. If a third party successfully sues us for any injury caused by our product candidates, our liability could exceed our total assets.

**Even if we receive regulatory approval for our product candidates, we will be subject to ongoing and continued regulatory review, and if we are unable to maintain regulatory approval of Korlym, or if we fail to comply with regulatory requirements, we will be unable to generate revenue or may be subject to penalties and our business will be harmed.**

Even after we obtain U.S. regulatory approval for a product, the FDA may still impose significant restrictions on the approved indicated uses for which the product may be marketed or on the conditions of approval. For example, a product's approval may contain requirements for potentially costly post-approval studies and surveillance, including phase 4 clinical trials, to monitor the safety and efficacy of the product. The



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FDA's approval of Korlym was subject to limitations on the indicated uses for which the product may be marketed and requirements for post-marketing follow-up studies and information reporting. In addition, the FDA's approval of Korlym requires that we conduct a study of the interactions between Korlym and ketoconazole, an anti-fungal agent sometimes used to treat patients with Cushing's syndrome. It also requires us to conduct a drug utilization study to better characterize the reporting rates of adverse events associated with the long-term use of Korlym.

We will also be subject to ongoing obligations and continued regulatory review by the FDA and other regulatory authorities in the United States and other countries with respect to the research, testing, manufacturing, labeling, distribution, adverse event reporting, storage, selling, advertising, promotion, recordkeeping and marketing of products. These requirements include submissions of safety and other post-marketing information and reports, annual updates on manufacturing activities and continued compliance with cGMPs and cGCPs for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with our third-party manufacturers or manufacturing processes, or failure to comply with FDA regulations and other applicable foreign and U.S. regulatory requirements may result in, among other things, warning letters, civil and criminal penalties, injunctions, holds on clinical trials, product seizure or detention, refusal to permit the import or export of products, restrictions on product marketing, withdrawal of the product from the market, voluntary or mandatory product recalls, total or partial suspension of production, refusal to approve pending NDAs or supplements to approved NDAs, and suspension or revocation of product approvals.

The FDA's policies may change and additional governmental regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may place at risk any marketing approval that we may have obtained, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

**The sale of our products is subject to regulatory approvals, and our business is subject to extensive regulatory requirements, and if we are unable to obtain regulatory approval for future product candidates, including mifepristone for the treatment of the psychotic features of psychotic depression, we will be limited in our ability to commercialize such product candidates and our business will be harmed.**

We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates. Obtaining regulatory approval of a new drug is an uncertain, lengthy and expensive process, and success is never guaranteed. Despite the time, resources and effort expended, failure can occur at any stage. In order to receive approval from the FDA for each product candidate, we must demonstrate that the new drug product is safe and effective for its intended use and that our manufacturing processes for the product candidate comply with the FDA's cGMPs. cGMPs include requirements related to production processes, quality control and assurance, and recordkeeping. The FDA has substantial discretion in the approval process for human medicines. The FDA may require substantial additional clinical testing or find our drug products do not satisfy the standards for approval. Our inability or the inability of our suppliers to comply with applicable FDA and other regulatory requirements can result in, among other things, delays in or denials of new product approvals, warning letters, fines, consent decrees restricting or suspending manufacturing operations, injunctions, civil penalties, recall or seizure of products, total or partial suspension of sales, and/or criminal prosecution. Any of these or other regulatory actions could materially adversely affect our business and our financial condition.

Future governmental action or changes in FDA law, policy or personnel may also result in delays or rejection of an NDA in the United States. In addition, because the only other currently FDA-approved use of mifepristone is the termination of pregnancy, we expect that the label for mifepristone for any indication will include, as Korlym's does, some limitations, including a so-called "black-box" warning that it should not be used by pregnant women or women seeking to become pregnant.

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If we receive regulatory approval for our future product candidates, including mifepristone for the treatment of psychotic depression, we will be subject to ongoing FDA obligations and continued regulatory oversight and review, such as continued safety reporting requirements; and we may also be subject to additional FDA post-marketing restrictions and obligations, such as a Risk Evaluation and Mitigation Strategy. If we are not able to maintain regulatory compliance, we may not be permitted to market our product candidates and/or may be subject to product recalls or seizures.

Any regulatory approvals that we receive for our future product candidates may also be subject to limitations on the indicated uses for which the medicine may be marketed or contain requirements for potentially costly post-marketing follow-up studies. In addition, if the FDA approves any of our product candidates, we will be subject to ongoing and continuing regulatory requirements. See also the discussion above under “Even if we receive regulatory approval for our product candidates, we will be subject to ongoing and continued regulatory review, and if we are unable to maintain regulatory approval of Korlym, or if we fail to comply with regulatory requirements, we will be unable to generate revenue or may be subject to penalties and our business will be harmed.”

### **If we market products in a manner that violates FDA regulations or health care fraud and abuse laws, we may be subject to civil or criminal penalties.**

In the United States, we are subject to FDA regulations governing the promotion of health care products. Although physicians are permitted, based on their medical judgment, to prescribe drugs for indications other than those approved by the FDA, manufacturers are prohibited from promoting their products for such off-label uses. In the United States, we are marketing Korlym for treatment of hyperglycemia secondary to hypercortisolism in adult patients with endogenous Cushing’s syndrome who have type 2 diabetes mellitus or glucose intolerance and have failed surgery or are not candidates for surgery and provide promotional materials and training programs to physicians regarding the use of Korlym for this indication. Although we believe our marketing materials and training programs for physicians do not constitute off-label promotion of Korlym, the FDA may disagree. If the FDA determines that our promotional materials, training or other activities by our employees or agents constitute off-label promotion of Korlym, it could request that we modify our training or promotional materials or other activities or subject us to regulatory enforcement actions, including the issuance of a warning letter, injunction, seizure, civil fine and criminal penalties. It is also possible that other federal or state enforcement authorities might take action if they believe that the alleged improper promotion led to the submission and payment of claims for an unapproved use, which could result in significant fines or penalties under other statutory authorities, such as laws prohibiting false claims for reimbursement. Even if it is later determined that we are not in violation of these laws, we may be faced with negative publicity, incur significant expenses defending our position and have to divert significant management resources from other matters.

In addition, there are health care fraud and abuse regulations and enforcement by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include:

the federal health care programs Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal health care programs such as the Medicare and Medicaid programs;

federal false claims laws, which prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to get a false claim paid. Pharmaceutical companies have been prosecuted under these laws for a variety of promotional and marketing activities, such as allegedly providing free product to or entering into sham consulting arrangements with customers to induce such customers to purchase, order or recommend the company’s products in violation of the Anti-Kickback Statute and



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federal false claims laws and regulations; reporting to pricing services inflated average wholesale prices that were then used by certain governmental programs to set reimbursement rates; engaging in the promotion of off-label uses that caused customers to submit claims to and obtain reimbursement from governmental payors for non-covered off-label uses; and submitting inflated best price information to the Medicaid Drug Rebate Program;

the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA), which created federal criminal laws that prohibit executing a scheme to defraud any health care benefit program or making false statements relating to health care matters;

federal sunshine laws that require transparency regarding financial arrangements with health care providers, such as the reporting and disclosure requirements imposed by the PPACA on drug manufacturers regarding any transfer of value made or distributed to prescribers and other health care providers. Manufacturers will be required to begin data collection on August 1, 2013 and report such data to the Centers for Medicare and Medicaid Services (known as CMS) by March 31, 2014; and

state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers.

Some states, such as California, Massachusetts, Minnesota, Nevada, Vermont and Washington, as well as the District of Columbia, mandate implementation of commercial compliance programs to ensure compliance with these laws.

The risk of our being found in violation of these laws and regulations is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Moreover, recent health care reform legislation has strengthened these laws. For example, the PPACA, among other things, amended the intent requirement of the federal anti-kickback and criminal health care fraud statutes; a person or entity no longer needs to have actual knowledge of these statutes or specific intent to violate them. In addition, the PPACA provided that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the false claims statutes. If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from governmental health care programs, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our financial results.

**Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and results of earlier studies and trials may not be predictive of future trial results.**

Clinical development is a long, expensive and uncertain process, and data obtained from clinical trials and supportive studies are susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The results from early clinical trials may not be predictive of results to be obtained in later clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Clinical trials may not demonstrate sufficient safety and efficacy to obtain the necessary regulatory approvals or a commercially viable product. To gain regulatory approval from the FDA to market mifepristone for the psychotic features of psychotic depression, our ongoing phase 3 clinical trial must demonstrate the safety and efficacy of mifepristone for that indication. The ongoing phase 3 clinical trial of mifepristone for the treatment of the psychotic features of psychotic depression may not demonstrate efficacy or safety results sufficient for approval, and we may need to conduct other studies in support of a potential NDA in that indication. If our ongoing phase 3 clinical trial is not

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completed or conducted as planned or if mifepristone does not prove to be safe and effective or does not receive required regulatory approvals, the commercialization of mifepristone for the psychotic features of psychotic depression would be delayed or prevented, and our ability to generate revenues would be impaired.

Moreover, the commencement and completion of clinical trials may be delayed by many factors that are beyond our control, including:

delays obtaining regulatory approval to commence a trial;

reaching agreement on acceptable terms with contract research organizations, or CROs, and clinical trial sites;

obtaining institutional review board, or IRB, approval at each site;

slower than anticipated patient enrollment;

scheduling conflicts with participating clinicians and clinical institutions;

lack of funding;

negative or inconclusive results;

patient noncompliance with the protocol;

adverse medical events or side effects among patients during the clinical trials;

negative or problematic FDA inspections of our clinical operations or manufacturing operations; and

real or perceived lack of effectiveness or safety of mifepristone.

We could encounter delays if a clinical trial is suspended or terminated by us, the IRBs of the clinical trial sites in which such trials are being conducted, by the Data Safety Monitoring Board, or DSMB, for such trial or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

We may decide, or the FDA or other regulatory authorities may require us, to pursue additional clinical or preclinical studies on mifepristone for the treatment of the psychotic features of psychotic depression. Additional trials or studies may require additional funding, the availability of which is not assured. Also, it is possible that additional trials or studies that we decide are necessary or desirable will delay or prevent the completion of the development of mifepristone for treating the psychotic features of psychotic depression. Even if we are able to conduct all of the clinical trials and supportive studies that we consider appropriate for an optimal NDA, we may never receive regulatory approval to market mifepristone for psychotic depression.

**Our use of MedAvante to provide centralized psychiatric rating services in Study 14, our ongoing clinical trial evaluating mifepristone for the psychotic features of psychotic depression, may not result in any improvement in the accuracy and consistency of the psychiatric assessments and may continue to slow the pace of enrollment in Study 14.**

In connection with our ongoing phase 3 trial evaluating mifepristone for the psychotic features of psychotic depression, Study 14, we engaged MedAvante to provide centralized psychiatric rating services. MedAvante is providing centralized psychometric assessments via high resolution video-conferencing. The use of MedAvante's centralized rating services is intended to increase the accuracy and consistency of the psychiatric assessments.

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MedAvante has provided similar centralized rating services to companies conducting clinical studies in various psychiatric disorders. However, they have not previously provided centralized rating services to any study in patients with psychotic depression. Although we and MedAvante conducted a small pilot evaluation in patients with psychotic depression to assess patient receptivity, we cannot be certain that centralized rating will be successful with the patients enrolled in our study.

If patients are uncomfortable or unwilling to participate in the centralized rating process or if MedAvante is unable to provide services in a satisfactory manner over the course of the trial, we may not see any improvement in the accuracy or reliability of the psychiatric assessments. Such a result might diminish the likelihood of a successful trial or a definitive demonstration of the efficacy of mifepristone in treating the psychotic features of psychotic depression.

During screening for Study 14, there has been a higher than anticipated incidence of potential patients who do not meet appropriate criteria for entrance into the trial for diagnostic and other clinical reasons. Although we believe that this is the result of improved accuracy in the screening process resulting from the use of the MedAvante centralized rating services as an additional step in the selection of patients appropriate for inclusion in the study, MedAvante's diagnostic screening may not actually improve trial performance. In addition, in mid-2009, in order to lower expenses and to conserve financial resources, we scaled back our planned rate of spending on this trial and extended the timeline for its completion. Our current plan is to increase the number of clinical sites from eight to approximately 20, which will increase our rate of spending on the trial, with an unknown effect on the likelihood of success.

**In order to meet our goal of enrolling a sufficient number of patients in Study 14 by the end of 2013 to perform a successful interim analysis, we will need to increase the pace of enrollment which will be costly and may not be successful.**

The pace of enrollment in Study 14 is subject to a number of factors, including our ability to identify, qualify and enlist new trial sites, our ability to identify potential study subjects and enroll them in the trial, and the ability and willingness of patients in the trial to complete the study protocol. Furthermore, we will need to work with our existing third-party service providers and may need to retain additional personnel to support our effort to increase the rate of Study 14 enrollment, which will be costly, and we may not be successful in these efforts. Finally, even if we succeed in increasing the rate of enrollment in Study 14, this will not necessarily allow us to demonstrate the efficacy of the medicine.

**If we conduct an interim analysis of the data from our on-going Phase 3 trial of mifepristone for the treatment of psychotic depression (Trial 14), the results may not show efficacy. If the interim results are inconclusive and we continue the study, conducting an interim analysis will make achieving a positive result more difficult.**

One of our goals is to enroll a sufficient number of patients in Trial 14 by the end of 2013 so that we can, if we choose, perform an interim analysis of the study's data. We have not yet determined whether we will perform such an analysis or what its parameters might be. Regardless of how many patients we enroll in Trial 14 by the end of 2013, there can be no assurance that an interim analysis will take place.

Even if we reach our patient enrollment goal and choose to perform an interim analysis, its results may be negative or inconclusive. If they are negative, then we will terminate the trial and either incur the substantial additional expense and delay of undertaking a new trial, or discontinue the study of mifepristone for the psychotic features of psychotic depression, which may reduce our future revenue. If the results are inconclusive, and we choose to continue the trial, we will incur additional expense and delay the possibility of our obtaining regulatory approval of a treatment for this disease.

In addition, performing an interim analysis makes the measure of statistical significance in any continued trial more rigorous and more difficult to meet. Therefore, a continued trial following an interim analysis is generally less likely to achieve a statistically meaningful positive outcome, making it more difficult to achieve a positive result.

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**We depend on third parties to conduct and manage many of our clinical trials and to perform related data collection and analysis and, if these third parties do not successfully carry out their contractual duties or meet expected timelines, we may face costs and delays that may prevent or delay us from obtaining regulatory approval for or commercializing our product candidates, which could substantially harm our business.**

We rely on clinical investigators and clinical sites to enroll patients and other third parties such as clinical research organizations, or CROs, to manage many of our trials and to perform related data collection and analysis. We control only certain aspects of these third parties' activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with current good clinical practices, or cGCP, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities through periodic inspections of trial sponsors, clinical investigators and clinical sites. If we or any of the third parties working on or conducting our trials fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approval of our marketing applications, if at all. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with cGCP requirements. In addition, our clinical trials must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, we may not be able to control the timing of identification and selection of appropriate sites for our planned trials and the amount and timing of resources that the clinical sites that conduct the clinical testing may devote to our clinical trials. If our clinical investigators and clinical sites fail to enroll a sufficient number of patients in our clinical trials or fail to enroll them on our planned schedules, we will be unable to complete our trials or to complete them as planned, which could delay or prevent us from completing the clinical development of mifepristone for the psychotic features of psychotic depression or other development programs.

We have an agreement with a CRO that is conducting our ongoing phase 3 trial evaluating mifepristone for the treatment of the psychotic features of psychotic depression (Study 14) to supervise and monitor clinical site performance and to perform investigator supervision, data collection and analysis for this trial. The conduct of future clinical trials may also be conducted through the use of CROs and third party clinical sites. We may not be able to maintain relationships with this or other CROs or with the clinical investigators and the clinical sites through the completion of all trial activities without delays in anticipated timing of trial activities or excessive expenditures. If any of our relationships with CROs or other third parties terminates, we may not be able to enter into arrangements with alternative CROs or third parties on commercially reasonable terms, or at all. If these CROs, clinical investigators, clinical sites or other third parties do not carry out their contractual duties or obligations or fail to meet expected deadlines, or if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to their failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may be unable to obtain regulatory approval for, or successfully commercialize, mifepristone for the psychotic features of psychotic depression.

**Failure to obtain regulatory approval in foreign jurisdictions will prevent us from commercializing Korlym and our other product candidates abroad.**

We may seek to commercialize our products and product candidates in international markets with the help of one or more partners or on our own. Outside the United States, we may commercialize a product only if we receive a marketing authorization and, in many cases, pricing approval, from the appropriate regulatory authorities, whose approval processes include all of the risks associated with the FDA approval process, and, in some cases, additional risks. The approval procedure varies among countries and can involve additional testing, and the time required to obtain approval may differ from that required to obtain FDA approval. Other than seeking and receiving Orphan Drug Designation in the EU, we have not taken any actions to obtain foreign approvals. We may not develop our product candidates in the clinic in order to obtain foreign regulatory approvals on a timely basis, if at all.

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Approval by the FDA does not ensure approval by regulatory authorities in other countries, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA, but a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. We may not be able to file for regulatory approvals and may not receive necessary approvals to commercialize our product candidates in any foreign market.

### **The fast track designation for the development program of mifepristone for the treatment of the psychotic features of psychotic depression may not lead to a faster development or regulatory review or approval process.**

If a human medicine is intended for the treatment of a serious or life-threatening disease or condition and the medicine demonstrates the potential to address unmet medical needs for this disease or condition, the sponsor of an IND may apply for FDA fast track designation for a particular indication. Marketing applications submitted by sponsors of product candidates in fast track development may qualify for expedited FDA review under the policies and procedures offered by the FDA, but the fast track designation does not assure any such qualification. Although we have obtained a fast track designation from the FDA for mifepristone for the treatment of the psychotic features of psychotic depression, we may not experience a faster development process, review or approval compared to applications considered for approval under conventional FDA procedures. In addition, the FDA may withdraw our fast track designation at any time. If we lose our fast track designation, the approval process may be delayed. In addition, our fast track designation does not guarantee that we will qualify for or be able to take advantage of the expedited review procedures and does not increase the likelihood that mifepristone will receive regulatory approval for the treatment of the psychotic features of psychotic depression.

### **We face competition from companies with substantial financial, technical and marketing resources, which could limit our future revenues from the commercialization of mifepristone for the treatment of the psychotic features of psychotic depression or for other indications.**

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. If approved for commercial use as a treatment for the psychotic features of psychotic depression, mifepristone will compete with established treatments, including ECT and combination medicinal therapy.

Combination medicinal therapy consists of the use of antipsychotic and antidepressant medicines not currently approved for the treatment of psychotic depression. The antipsychotics are prescribed by physicians for off-label use to treat the psychotic features of psychotic depression, which is the clinical target of mifepristone. Antipsychotics include Abilify® (Bristol-Myers Squibb), Clozaril® (Novartis), Geodon® and Navane® (Pfizer), Haldol® (Ortho-McNeil), Mellaril® (Mylan), Risperdal® (Janssen Pharmaceuticals), Seroquel® (AstraZeneca), Stelazine® and Thorazine® (GlaxoSmithKline) and Zyprexa® (Eli Lilly). Mifepristone may not compete effectively with these established treatments. We are aware of one clinical trial conducted by Organon, for a new chemical entity for the treatment of psychotic depression. Organon was the pharmaceutical division of Akzo Nobel, which was purchased by Schering Plough which was then subsequently acquired by Merck & Co. Organon's new chemical entity is a GR-II antagonist; we believe that its commercial use would be covered by our patent.

Our present and potential competitors include major pharmaceutical companies such as the makers of the drugs identified above, as well as specialized pharmaceutical firms, universities and public and private research institutions. Moreover, we expect competition to intensify as technical advances are made. These competitors, either alone or with collaborative parties, may succeed with the development and commercialization of medicinal products that are superior to and more cost-effective than mifepristone. Many of our competitors and related private and public research and academic institutions have greater experience, more financial and marketing resources and larger research and development staffs than we do. In addition, many of these competitors, either alone or together with their collaborative partners, have significantly greater experience than we do in developing human medicines, obtaining regulatory approvals, manufacturing and commercializing products.

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Accordingly, mifepristone may not be an effective competitor against established treatments and our present or potential competitors may succeed in developing medicinal products that are superior to mifepristone or render mifepristone obsolete or non-competitive. If we are unable to establish mifepristone as a superior and cost-effective treatment for the psychotic features of psychotic depression, or any future use, we may be unable to generate the revenues necessary to support our business.

**Our efforts to discover, develop and commercialize new product candidates beyond mifepristone are at a very early stage. If we fail to identify and develop additional uses for GR-II antagonists, we may be unable to market additional products.**

To develop additional potential sources of revenue, we believe that we must identify and develop additional product candidates. We own or have exclusively licensed issued U.S. patents covering the use of GR-II antagonists to treat psychotic depression, mild cognitive impairment, weight gain due to treatment with antipsychotic medication, stress disorders, early dementia, delirium, gastroesophageal reflux disease, Down's Syndrome, catatonia and psychosis associated with cocaine addiction, and to increase the therapeutic response to electroconvulsive therapy (ECT). In addition, we have nine U.S. method-of-use patent applications covering GR-II antagonists for the treatment of a number of other metabolic and psychiatric disorders, three U.S. composition of matter patents covering specific GR-II antagonists, and two additional U.S. composition of matter patents are pending. We have also filed patent applications in the major international markets.

The use of GR-II antagonists may not be effective to treat these conditions or any other indications. Moreover, we could discover that the use of GR-II antagonists in these patient populations has unacceptable side effects or is otherwise not safe. Due to the risks of efficacy and side effects inherent in developing novel compounds, we are likely to enter multiple compounds into development, which would increase our rate of spending with no assurance that we will be successful in developing new drugs that are safe and effective.

In addition, we may not develop or continue to develop product candidates for any of the indications or compounds covered by our patents and patent applications. Typically, there is a high rate of attrition for product candidates in preclinical and clinical trials, and our product development efforts may not lead to commercially viable products.

We may elect to enter into collaboration arrangements with respect to one or more of our product candidates. If we do enter into such an arrangement, we would be dependent on a collaborative partner for the success of the product candidates developed under the arrangement. Any future collaborative partner may fail to successfully develop or commercialize a product candidate under a collaborative arrangement.

We only have significant clinical experience with mifepristone and we may determine that mifepristone is not desirable for uses other than for the treatment of hyperglycemia secondary to hypercortisolism in adult patients with endogenous Cushing's syndrome who have type 2 diabetes mellitus or glucose intolerance and have failed surgery or are not candidates for surgery and, potentially, for the psychotic features of psychotic depression. For example, we do not intend to develop mifepristone for mitigation of the weight gain associated with the use of Zyprexa, Risperdal or other atypical antipsychotics, even though we have reported positive results in the proof of concept studies described in Part I, Item 1, Business - Overview - Mifepristone Proof-of-Concept Studies, *Other Metabolic Disorders* of our Annual Report on Form 10-K for the year ended December 31, 2012. We are pursuing other GR-II antagonists for this use. The compounds developed pursuant to our early clinical, preclinical and discovery research programs, including CORT 108297, may fail to become viable product candidates regardless of the resources we may dedicate to the program. Even if product candidates are identified, we may abandon further development efforts before we reach clinical trials or after expending significant expense and time conducting clinical trials due to financial constraints, concerns over the safety or efficacy of the product candidates, manufacturing difficulties or other reasons. Moreover, governmental authorities may enact new legislation or regulations that could limit or restrict our development efforts. If we are unable to successfully discover and commercialize new uses for GR-II antagonists, we may be unable to generate sufficient revenue to support our operations.

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### **If we lose our key personnel or are unable to attract and retain additional skilled personnel, we may be unable to pursue our product development and commercialization efforts.**

Our ability to operate successfully and manage our potential future growth depends significantly upon retaining key research, technical, sales, marketing, managerial and financial personnel, and attracting and retaining additional highly qualified personnel in these areas. We depend substantially on the principal members of our management and scientific staff. We do not have agreements with any of our executive officers that provide for their continued employment with us or employment insurance covering any of our key personnel. Any officer or employee can terminate his or her relationship with us at any time and work for one of our competitors. The loss of these key individuals could result in competitive harm because we could experience delays in our product research, development and commercialization efforts without their expertise.

We face intense competition for qualified personnel from numerous companies, as well as universities and nonprofit research organizations in the highly competitive San Francisco Bay Area. Although we believe that we have been successful in attracting and retaining qualified personnel to date, we may not be able to attract and retain sufficient qualified personnel in the future. The inability to attract and retain these personnel could result in delays in the research, development and commercialization of our potential products.

### **Rapid technological change could make our product candidates obsolete.**

Pharmaceutical technologies have undergone rapid and significant change and we expect that they will continue to do so. Our future will depend in large part on our ability to maintain a competitive position with respect to these technologies. Any products and processes that we develop may become obsolete or uneconomical before we recover any or all expenses incurred in connection with their development. Rapid technological change could make our product candidates obsolete or uneconomical, which could materially adversely affect our business, financial condition and results of operations.

### **The occurrence of a catastrophic disaster or other similar events could cause damage to our own or our manufacturers facilities and equipment, which could require us to cease or curtail operations.**

Because our executive offices are located in the San Francisco Bay Area and some of our current manufacturers are also located in earthquake-prone areas, our business is vulnerable to damage from various types of disasters or other similarly disruptive events, including earthquake, fire, flood, power loss and communications failures. In addition, political considerations relating to mifepristone may put us and our manufacturers at increased risk for terrorist attacks, protests or other disruptive events. If any disaster or other similar event were to occur, we may not be able to operate our business and our manufacturers may not be able to produce our product candidates. Our insurance may not be adequate to cover, and our insurance policies may exclude coverage for, our losses resulting from disasters or other business interruptions.

### **We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cyber security incidents, could harm our ability to operate our business effectively.**

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our clinical and commercialization activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. The loss of clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and our product research, development and commercialization efforts could be delayed.



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**Risks Related to Our Capital Needs and Financial Results**

**We may need additional capital in order to complete the development and commercialization of our proprietary, selective GR-II antagonists. Additional capital may not be available to us at all or on favorable terms, which could adversely affect our business.**

We may have to perform more clinical trials, in addition to our ongoing phase 3 trial, prior to submitting an NDA for mifepristone for the treatment of the psychotic features of psychotic depression. If so, we may need to raise additional funds to complete the development of mifepristone for that indication. In addition, we may need to raise additional funds to continue and expand the development of our proprietary, selective GR-II antagonists in various indications. We may also raise additional funds for other research and development activities, including clinical trials, and working capital and for other general corporate purposes, or to acquire or invest in businesses, products and technologies that are complementary to our own.

Factors impacting our cash position and future prospects of liquidity include the following:

the amount and timing of revenues from the commercialization of Korlym;

the pace at which physicians adopt Korlym as a treatment;

the willingness of insurance companies, the government and other third-party payors to provide coverage for Korlym at reasonable rates;

changes in the coverage and reimbursement policies of third-party insurance companies or government agencies;

the costs, timing of site selection and enrollment of our clinical trials;

the results of our research efforts and clinical trials;

the need to perform additional clinical trials and other supportive studies;

the timing of the submission of an NDA to the FDA, the acceptance of the NDA submission, and the outcome of the FDA approval process for the marketing of mifepristone for the treatment of the psychotic features of psychotic depression;

the timing of commercialization of mifepristone for the treatment of psychotic depression;

developments or disputes concerning patents or proprietary rights, including announcements of claims of infringement, interference or litigation against us or our licensors;

actual or anticipated fluctuations in our operating results;

changes in our growth rates; and

changes in our research and development plans for our proprietary, selective GR-II antagonists.

Consequently, we may need additional funding sooner than anticipated. In addition, we may choose to raise additional capital due to market conditions or strategic considerations even if we believe we have sufficient funds for our current and future operating plans.

We cannot be certain that additional funding will be available on acceptable terms or at all. Even though we have raised funds a number of times in the past, market and economic conditions may make it difficult for us to raise any or sufficient additional capital. Our sales of common stock and warrants and the exercises of warrants have been dilutive to stockholders and any exercise of outstanding warrants and additional equity financing could cause further dilution to stockholders. Debt financing, if available, may involve restrictive covenants. If we obtain funds through collaborations with others, these arrangements may be on unfavorable terms or may require us to relinquish certain rights to Korlym, our technologies or product candidates, which we would otherwise seek to develop on our own. If adequate funds are not available, we may be required to delay, reduce the scope of or eliminate one or more of our research or development programs or we may be required to discontinue operations.

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### **We have incurred losses since inception and anticipate that we will incur continued losses for at least the next few years.**

We have a limited history of operations and have focused primarily on clinical trials. We have begun to commercialize Korlym and, if the outcome of our clinical trials supports it, we plan to seek FDA regulatory clearance to market mifepristone for the treatment of the psychotic features of psychotic depression. Historically, we have funded our operations primarily from the sale of our equity securities. We have incurred losses in each year since our inception in 1998. As of December 31, 2012, we had an accumulated deficit of \$246.6 million. We only began to sell our first commercial product, Korlym, in the United States in April 2012. Based on this limited experience marketing Korlym, it is difficult for us to predict the magnitude or timing of future product sales. We expect our research and development expenses to increase in connection with the clinical trials and other development activities for mifepristone for the psychotic features of psychotic depression and for other product candidates. We expect to incur significant expenses related to commercializing Korlym. As a result, we expect that our losses will increase at least until Korlym is generating material amounts of revenue. We are unable to predict the extent of any future losses or whether or when we will become profitable.

### **We may not be able to pursue all of our product research and development opportunities if we are unable to generate sufficient revenue or secure adequate funding for these programs.**

The costs required to start or continue many of the programs that our intellectual property allows us to consider for further development are collectively greater than the funds currently available to us. For example, we have successfully discovered three series of compounds that are selective GR-II antagonists but, unlike mifepristone, do not appear to block the progesterone receptor. Further development of these proprietary compounds or any further development stemming from our method-of-use patents may be delayed or cancelled if we determine that such development may jeopardize our ability to complete the clinical development of mifepristone for the treatment of psychotic depression.

### **Global economic conditions could adversely affect our liquidity and financial condition.**

In the United States and globally, market and economic conditions have been volatile over the past few years, with significantly tighter credit conditions in the markets in which we conduct our operations. The U.S. and global economies have experienced a recession and face continued concerns about the systemic impact of adverse economic conditions, such as unstable global financial markets, adverse effects on the cost and availability of capital, high corporate, consumer and governmental debt levels and high unemployment. Concern about the stability of the markets generally, and the strength of counterparties specifically, has led and may again lead many lenders and institutional investors to reduce, and in some cases, cease, to provide credit to businesses. Renewed or increased turbulence in the global markets and economies may adversely affect our liquidity and financial condition.

In addition, our access to funds under any credit facility into which we may enter depends on the ability of the counterparties to such facilities to meet their funding commitments to us. We cannot assure you that long-term disruptions in the global economy and the return of tighter credit conditions among, and potential failures of, third party financial institutions as a result of such disruptions will not have an adverse effect on such counterparties.

If we do not have sufficient cash flow to continue operating our business and are unable to borrow funds or raise equity or debt capital, we may need to find alternative ways to increase our liquidity. Such alternatives may include, without limitation, curtailing clinical or drug development activity, or limiting our commercial efforts, product manufacturing or sales and marketing support, which would have an adverse effect on our business, results of operations, cash flows and financial condition.

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**If we acquire other GR-II antagonists or other technologies or potential products, we will incur a variety of costs and may never realize the anticipated benefits of the acquisition.**

If appropriate opportunities become available, we may attempt to acquire other GR-II antagonists, particularly GR-II antagonists that do not terminate pregnancy. We may also be able to acquire other technologies or potential products that are complementary to our operating plan. We currently have no commitments, agreements or plans for any acquisitions. The process of acquiring rights to another GR-II antagonist or any other potential product or technology may result in unforeseen difficulties and expenditures and may absorb significant management attention that would otherwise be available for ongoing development of our business. In addition, we may fail to realize the anticipated benefits of any acquired potential product or technology. Future acquisitions could dilute our stockholders' ownership interest in us and could cause us to incur debt, expose us to future liabilities and result in amortization or other expenses related to goodwill and other intangible assets.

**Failure to meet our obligations under our Financing Agreement with Biopharma Secured Debt Fund II Sub, S.à.r.l, could adversely affect our financial results and liquidity.**

Pursuant to our Financing Agreement with Biopharma entered into in August 2012, we are obligated to make payments to Biopharma equal to 20 percent of our net product sales of Korlym, any future mifepristone-based products and our next-generation selective GR-II antagonists (Covered Products), subject to certain quarterly caps, as well as an un-capped 20 percent of any upfront, milestone or other contingent payments we receive with respect to Covered Products, until such payments to Biopharma total \$45 million.

Pursuant to this agreement, we may not: (i) incur indebtedness greater than the sum of earnings before interest, taxes, depreciation and amortization, including such items as non-cash stock-based compensation, (EBITDA) for the four calendar quarters preceding such incurrence, which we refer to as the Indebtedness Covenant; (ii) pay a dividend or other cash distribution, unless we have cash and cash equivalents in excess of \$50 million after such payment; (iii) amend or restate our certificate of incorporation or bylaws unless such amendments or restatements do not affect Biopharma's interests under the transaction; and (iv) encumber any of the collateral securing our performance under the agreement.

The percentage used to calculate our payments to Biopharma would increase to 50 percent and any applicable payment caps would lapse if we (i) fail to provide Biopharma with certain information regarding our promotion and sales of Covered Products, (ii) do not devote a commercially reasonable amount of resources to the promotion and marketing of the Covered Products or (iii) violate the Indebtedness Covenant and, in each case, fail to cure within the applicable cure period.

Upon a Corcept change of control transaction, as defined in the agreement, Biopharma will be automatically entitled to receive any amounts not previously paid, up to our maximum repayment obligation of \$45 million. As defined in the agreement, Change of Control includes, among other things, (i) a greater than 50 percent change in the ownership of Corcept, (ii) certain changes in Board composition of Corcept and (iii) the licensing of Korlym to a third party for sale in the United States.

To secure our obligations under the agreement, we granted Biopharma a security interest in our rights in patents, trademarks, trade names, domain names, copyrights, know-how and regulatory approvals related to the Covered Products, all books and records relating to the foregoing and all proceeds of the foregoing, which we refer to as the Collateral. If we (i) fail to deliver a royalty payment when due and do not remedy that failure within 30 days, (ii) fail to maintain a first-priority perfected security interest in the Collateral in the United States and do not remedy that failure within five business days of receiving notice of such failure or (iii) become subject to an event of bankruptcy, then Biopharma may attempt to recover up to \$45 million (after deducting any payments we have already made).

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There can be no assurance that we will not breach the covenants or other terms of, or that an event of default will not occur under this agreement and, if a breach or event of default occurs, there can be no assurance that we will be able to cure the event within the time permitted. Any failure to pay our obligations when due, any breach or default of our covenants or other obligations, or any other event that causes an acceleration of payment at a time when we do not have sufficient resources to meet these obligations, could have a material adverse effect on our business, results of operations, financial condition and future viability.

The acceleration of the payment obligation in the event of a change of control transaction may make us less attractive to potential acquirers, and the payment of such funds out of our available cash or acquisition proceeds would reduce acquisition proceeds for our stockholders.

### **Risks Relating to Our Intellectual Property**

**If Korlym or future product candidates conflict with the patents of others or if we become involved in other intellectual property disputes, we may have to engage in costly litigation or obtain a license and we may be unable to commercialize our product candidates.**

Our success depends in part on our ability to obtain and maintain adequate patent protection for the use of mifepristone for the treatment of the psychotic features of psychotic depression and other potential uses of GR-II antagonists. If we do not adequately protect our intellectual property, competitors may be able to use our intellectual property and erode our competitive advantage.

To date, we own eleven issued U.S. method of use patents and have exclusively licensed three issued U.S. method of use patents. We have nine U.S. method-of-use patent applications pending for GR-II antagonists. We own three composition-of-matter patents and have two composition of matter patent applications pending. We have applied, and will continue to apply, for patents covering our product candidates as we deem appropriate. We have also filed, where we deemed appropriate, foreign patent applications corresponding to our U.S. patents and applications.

We have exclusively licensed three issued U.S. patents from Stanford University for the use of GR-II antagonists in the treatment of psychotic major depression, which is commonly referred to as psychotic depression, cocaine-induced psychosis and early dementia, including early Alzheimer's disease. We bear the costs of protecting and defending the rights to these patents. In order to maintain the exclusive license to these patents until their expiration, we are obligated to make milestone and royalty payments to Stanford University. If we become noncompliant with our obligations under this agreement, we may lose the right to commercialize mifepristone for the treatment of psychotic depression, cocaine-induced psychosis and early dementia and our business would be materially harmed. In addition, if Stanford University were to terminate our mifepristone license due to breach of the license on our part, we would not be able to commercialize mifepristone for the treatment of the psychotic features of psychotic depression, cocaine-induced psychosis or early dementia.

Our patent applications and patents licensed or issued to us may be challenged by third parties and our patent applications may not result in issued patents. For example, in 2004, Akzo Nobel, which was subsequently acquired by Schering Plough which was then subsequently acquired by Merck & Co., filed an observation challenging the claims of our exclusively licensed European patent application with claims directed to psychotic depression. In this instance, the patent later issued and, in 2007, we received notice from the European Patent Office that there will be no opposition proceedings in Europe in regard to this patent.

Our presently pending and future patent applications may not issue as patents, and any patent issued to us may be challenged, invalidated, held unenforceable or circumvented. For example, the arguments presented by Akzo Nobel could be raised in the United States either before the U.S. Patent and Trademark Office or in a court of law. Furthermore, the claims in patents which have been issued to us, or which may be issued to us in the future, may not be sufficiently broad to prevent third parties from producing competing products. In addition, the laws of various foreign countries in which we compete may not protect our intellectual property to the same

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extent as do the laws of the United States. If we fail to obtain adequate patent protection for our proprietary technology, our competitors may produce competing products based on our technology, which would impair our ability to compete.

If a third party were successful in asserting an infringement claim against us, we could be forced to pay damages and prevented from developing, manufacturing or marketing our potential products. We do not have liability insurance for patent infringements. A third party could require us to obtain a license to continue to use their intellectual property, and we may not be able to do so on commercially acceptable terms, or at all. We believe that significant litigation will continue in our industry regarding patent and other intellectual property rights. If we become involved in litigation, it could consume a substantial portion of our resources. Regardless of the merit of any particular claim, defending a lawsuit takes significant time, is expensive and diverts management's attention from other business.

### **If we are unable to protect our trade secrets and proprietary information, our ability to compete in the market could be diminished.**

In addition to patents, we rely on a combination of confidentiality, nondisclosure and other contractual provisions, laws protecting trade secrets and security measures to protect our trade secrets and proprietary information. Nevertheless, these measures may not adequately protect our trade secrets or other proprietary information. If they do not adequately protect our rights, third parties could use our proprietary information, which could diminish our ability to compete in the market. In addition, employees, consultants and others who participate in the development of our product candidates may breach their agreements with us regarding our trade secrets and other proprietary information, and we may not have adequate remedies for the breach. We also realize that our trade secrets may become known through means not currently foreseen. Notwithstanding our efforts to protect our trade secrets and proprietary information, our competitors may independently develop similar or alternative products that are equal or superior to our product candidates without infringing on any of our proprietary information or trade secrets.

### **Our licensed patent covering the use of mifepristone to treat psychotic depression is a method of use patent rather than a composition of matter patent, which may make it more difficult for us to prove patent infringement if physicians prescribe another manufacturer's mifepristone for the treatment of Cushing's syndrome or psychotic depression or if patients acquire mifepristone from other sources, such as the internet or black market.**

We have an exclusive license from Stanford University to a patent covering the use of GR-II antagonists, including mifepristone, for the treatment of psychotic depression. A method of use patent covers only a specified use of a particular compound, not a particular composition of matter. Because none of our issued patents covers the composition of mifepristone, we cannot prevent others from commercializing mifepristone in indications not covered by our method of use patents. If others receive approval to manufacture and market mifepristone or any other GR-II antagonist, physicians could prescribe mifepristone or any other GR-II antagonist for patients with psychotic depression instead of mifepristone. Although any such off-label use would violate our licensed patent, effectively monitoring compliance with our licensed patent may be difficult and costly. In addition, if others develop a treatment for the psychotic features of psychotic depression that works through a mechanism which does not involve the GR-II receptor, physicians could prescribe that treatment instead of mifepristone.

In addition, we cannot be assured that patients will not obtain mifepristone from other sources. As with other pharmaceutical products, patients may be able to purchase mifepristone through the internet or black market. Mifepristone is also sold in the United States by Danco Laboratories for the termination of early pregnancy. While distribution is limited to a single dose provided in the physician's office and covered by other restrictions, we cannot be certain that Cushing's syndrome patients will not be able to obtain mifepristone from this source or others, should another company receive approval to market mifepristone for another indication.

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**Risks Related to Our Stock**

**The market price of our common stock has been and is likely to continue to be highly volatile due to the limited number of shares of our common stock held by non-affiliates or factors influencing the stock market and opportunities for sale at any given time may be limited.**

We cannot assure you that an active trading market for our common stock will exist at any time. Holders of our common stock may not be able to sell shares quickly or at the market price if trading in our common stock is not active. During the 52-week period ended March 1, 2013, our average daily trading volume was approximately 389,000 shares and the intra-day sales prices per share of our common stock on the NASDAQ Stock Market ranged from \$1.27 to \$4.55. As of March 1, 2013, our officers, directors and principal stockholders controlled approximately 36 percent of our common stock. The trading price of our common stock has been and is likely to continue to be highly volatile and could be subject to wide fluctuations in price in response to various factors, many of which are beyond our control, including:

the pace of market acceptance of Korlym or the timing and level of coverage and reimbursement attained;

our cash and short-term investment position;

actual or anticipated timing and results of our clinical trials;

new products or services introduced or announced by us or our competitors;

actual or anticipated regulatory approvals of our product candidates or of competing products;

changes in laws or regulations applicable to our product candidates or our competitors' products;

changes in the expected or actual timing of our development programs or our competitors' potential development programs;

actual or anticipated variations in quarterly operating results, including potential product returns and timing of revenue recognition;

announcements of technological innovations by us, our collaborators or our competitors;

general market and economic conditions, including those seen as a result of the recent worldwide financial credit crisis;

changes in financial estimates or recommendations by securities analysts;

conditions or trends in the biotechnology and pharmaceutical industries;

changes in the market valuations of similar companies;

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announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures or capital commitments;

additions or departures of key personnel;

disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;

developments concerning collaborations;

trading volume of our common stock;

limited number of shares of our common stock held by our non-affiliates;

maintaining compliance with the listing requirements of the stock exchange on which we are listed;

success of additional financing efforts; and

purchases or sales of our common stock by us, our officers, directors or our stockholders.



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In addition, the stock market in general, the NASDAQ Stock Market and the market for biotechnology and life sciences companies in particular have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of those companies. These broad market and industry factors may seriously harm the market price of our common stock, regardless of our operating performance. In the past, following periods of volatility in the market, securities class-action litigation has often been instituted against companies. Such litigation, if instituted against us, could result in substantial costs and diversion of management's attention and resources.

### **The failure of our financial results to meet estimates published by research analysts or other investor expectations could cause our stock price to decline.**

There are inherent difficulties in predicting the amount of Korlym that will be sold. For example, the rate of physician adoption of Korlym is uncertain. Furthermore, the timing of a single large order for Korlym could substantially affect our revenue, making our levels of revenue potentially volatile and the identification of revenue trends difficult. Due to such uncertainty, we have not provided any revenue forecasts to investors or research analysts. Research analysts who cover our business have, however, put forth a wide range of revenue estimates, based entirely on their own investigation and analysis. We have not guided or commented on these estimates and you should rely on them at your own discretion. Announcement of financial results that fail to meet analyst estimates or the expectations of investors could cause our stock price to decline.

### **Research analysts may not continue to provide or initiate coverage of our common stock or may issue negative reports, which may have a negative impact on our common stock's market price.**

Securities analysts currently covering our common stock may discontinue research coverage. Additional securities analysts may elect not to provide research coverage of our common stock. A lack of research coverage may adversely affect our common stock's market price. The trading market for our common stock may be affected in part by the research and reports that industry or financial analysts publish about us or our business. If one or more of the analysts who elects to cover us downgrades our stock, our stock price would likely decline rapidly and significantly. If one or more of these analysts ceases coverage of our company, we could lose visibility in the market, which in turn could cause our stock price to decline. In addition, rules mandated by the Sarbanes-Oxley Act of 2002, and a global settlement reached in 2003 between the SEC, other regulatory analysts and a number of investment banks have led to a number of fundamental changes in how analysts are reviewed and compensated. In particular, many investment banking firms are required to contract with independent financial analysts for their stock research. It may be difficult for companies such as ours with smaller market capitalizations to attract independent financial analysts that will cover our common stock. This could have a negative effect on our market price.

### **A sale of a substantial number of shares of our common stock may cause the price of our common stock to decline.**

Sales of a substantial number of shares of our common stock in the public market could harm the market price of our common stock. As additional shares of our common stock become available for resale in the public market, whether as a result of equity financings by us or due to the release of trading restrictions, the supply of our common stock will increase, which could decrease the price. Substantially all of the shares of our common stock are eligible for sale, subject to applicable volume and other resale restrictions.

### **We may be required to pay significant amounts if we are not able to meet our obligations under our outstanding registration rights agreements.**

The registration rights agreement covering the approximately 8.9 million shares of our common stock issued in a private offering in March 2008 and an additional approximately 4.5 million shares of common stock underlying warrants issued in connection with the offering provides that if we fail to file or cause to be declared effective the registration statement covering the resale of these shares prior to specified deadlines, or fail to

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maintain the effectiveness of such registration statement (subject to limited permissible suspension periods), we will be required to pay the holders of such shares and warrants liquidated damages at the rate of 1 percent of the purchase price of these shares and warrants per month, up to a total of 10 percent. The registration statement covering the resale of the shares and shares underlying the warrants sold in this transaction was declared effective by the SEC in November 2008. Since this registration statement was not declared effective within the time frame specified in the registration rights agreement, we became obligated to pay liquidated damages of approximately \$1.3 million in 2008 to the investors in this financing, which obligation was settled in the form of stock in lieu of cash in November 2008. As noted above, if we fail to maintain the effectiveness of this registration statement, we may be obligated to pay additional liquidated damage amounts in the future.

In addition, in March 2008, we entered into a CEFF with Kingsbridge, under which we granted to Kingsbridge a warrant for the purchase of 330,000 shares of common stock. We terminated our CEFF with Kingsbridge effective August 7, 2012 and no further securities will be sold thereunder. However, under the registration rights agreement issued in connection with the CEFF, we are required to continue to use commercially reasonable efforts to maintain the effectiveness of the registration statement covering the shares sold under this agreement and to be issued upon the exercise of the warrant for a period of up to two years following the termination of the CEFF, subject to earlier termination on certain events. During this period, if the effectiveness of the registration statement lapses through actions that were within our control, we may be obligated to pay Kingsbridge for all shares issued upon exercise of the warrant and still owned by Kingsbridge at any time during the period of ineffectiveness the difference between (a) the volume weighted average price as of the day prior to the period of ineffectiveness and (b) the volume weighted average price as of the day following the period of ineffectiveness.

If we are required to pay significant amounts under this or future registration rights agreements, it could have a material adverse effect on our financial condition and ability to finance our operations.

### **Our officers, directors and principal stockholders, acting as a group, will be able to significantly influence corporate actions.**

As of March 1, 2013, our officers, directors and principal stockholders control approximately 36 percent of our common stock. As a result, these stockholders, acting together, will be able to significantly influence all matters requiring approval by our stockholders, including the election of directors and the approval of mergers or other business combination transactions. The interests of this group of stockholders may not always coincide with our interests or the interests of other stockholders and may prevent or delay a change in control. This significant concentration of share ownership may adversely affect the trading price of our common stock because investors often perceive disadvantages to owning stock in companies with controlling stockholders.

### **Changes in laws and regulations may result in increased costs to us, which may harm our financial results.**

New laws and regulations, as well as changes to existing laws and regulations, affecting our company, including the provisions of the PPACA requiring the reporting of aggregate spending related to health care professionals, the provisions of the Sarbanes-Oxley Act of 2002 and rules adopted by the SEC and by The NASDAQ Stock Market have and will likely continue to result in increased costs to us as we respond to their requirements. We are investing resources to comply with evolving laws and regulations, and this investment may result in increased selling, general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities.

In addition, new rules and regulations could make it more difficult or costly for us to obtain certain types of insurance, including director and officer liability insurance, and we may be forced to accept reduced policy limits and coverage or incur higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our Board of Directors, or our board committees, or as executive officers. At present, we cannot predict or estimate the amount of the additional costs related to new rules and regulations or the timing of such costs.

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**Compliance with public company obligations, including the securities laws and regulations, is costly and requires significant management resources, and we may fail to comply.**

We are a small company with limited resources.

The federal securities laws and regulations, including the corporate governance and other requirements of the Sarbanes-Oxley Act of 2002, impose complex and continually changing regulatory requirements on our operations and reporting. These requirements impose comprehensive reporting and disclosure requirements, set stricter independence and financial expertise standards for audit committee members, and impose civil and criminal penalties for companies, their chief executive officers, principal financial officers and directors for securities law violations. These requirements have increased and will continue to increase our legal compliance costs, increase the difficulty and expense in obtaining director and officer liability insurance, and make it harder for us to attract and retain qualified members of our Board of Directors and/or qualified executive officers. Such developments could harm our results of operations and divert management's attention from business operations.

In addition, as directed by Section 404 of the Sarbanes-Oxley Act of 2002, the SEC adopted rules requiring public companies to include a report of management on the company's internal control over financial reporting in their annual reports on Form 10-K. This requirement first applied to our annual report on Form 10-K for the year ended December 31, 2007. This same legislation also requires that the independent registered public accounting firm auditing our financial statements must attest to and report on the effectiveness of our internal controls over financial reporting. This requirement first applied to our annual report on Form 10-K for the year ended December 31, 2010. Uncertainty exists regarding our ability to comply with these requirements by applicable deadlines and to maintain compliance in future years. If we are unable to complete the required assessment as to the adequacy of our internal control over financial reporting in future years or if our independent registered public accounting firm is unable to provide us with an unqualified report as to the effectiveness of our internal control over financial reporting as of future year ends, investors could lose confidence in the reliability of our financial reporting.

**Changes in or interpretations of accounting rules and regulations could result in unfavorable accounting charges or require us to change our accounting policies or operating practices.**

Accounting methods and policies for business and marketing practices of pharmaceutical companies are subject to continual review, interpretation and guidance from relevant accounting authorities, including the SEC. For example, in December 2004, the Financial Accounting Standards Board adopted a revised standard related to stock-based compensation. This standard, which we adopted in 2006, requires the recording of expense for stock options granted using fair value-based measurements. As a result, our operating expenses have increased and are likely to continue to increase. We rely heavily on stock options to compensate existing employees and attract new employees. Because we are now required to expense stock options using fair value-based measurements, we may choose to reduce our reliance on stock options as a compensation tool. If we reduce our use of stock options, it may be more difficult for us to attract and retain qualified employees. Although we believe that our accounting practices are consistent with current accounting pronouncements, changes to or interpretations of accounting methods or policies in the future may require us to reclassify, restate or otherwise change or revise our financial statements. Any such changes could result in corresponding changes to the amounts of assets, liabilities, revenues, expenses and income. Any such changes could have a material adverse effect on our business, financial position and results of operations and could cause the market value of our common stock to decline.

**If we fail to continue to meet all applicable NASDAQ Stock Market requirements, our stock could be delisted by The NASDAQ Stock Market. If delisting occurs, it would adversely affect the market liquidity of our common stock and harm our business.**

If we are unable to meet any of the NASDAQ listing requirements in the future, including, for example, if the closing bid price for our common stock is below \$1 per share for 30 consecutive trading days, The NASDAQ Stock Market could determine to delist our common stock, the delisting could adversely affect the market

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liquidity of our common stock and the market price of our common stock could decrease. Such delisting could also adversely affect our ability to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees.

**Anti-takeover provisions in our charter and bylaws and under Delaware law and payment acceleration provisions under the Biopharma Financing Agreement may make an acquisition of us or a change in our management more expensive or difficult, even if an acquisition or a management change would be beneficial to our stockholders.**

Provisions in our charter and bylaws may delay or prevent an acquisition of us or a change in our management. Some of these provisions allow us to issue preferred stock without any vote or further action by the stockholders, require advance notification of stockholder proposals and nominations of candidates for election as directors and prohibit stockholders from acting by written consent. In addition, a supermajority vote of stockholders is required to amend our bylaws. Our bylaws provide that special meetings of the stockholders may be called only by our Chairman, President or the Board of Directors and that the authorized number of directors may be changed only by resolution of the Board of Directors. These provisions may prevent or delay a change in our Board of Directors or our management, which is appointed by our Board of Directors. In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law. Section 203 may prohibit large stockholders, in particular those owning 15 percent or more of our outstanding voting stock, from merging or combining with us. In addition, our payment obligations to BioPharma accelerate in the event of a change of control transaction. See Risk Factors Failure to meet our obligations under our Financing Agreement with Biopharma Secured Debt Fund II Sub, S.à r.l, could adversely affect our financial results and liquidity. These provisions in our charter and bylaws under Delaware law could reduce the price that investors might be willing to pay for shares of our common stock in the future and result in the market price being lower than it would be without these provisions.

### **ITEM 1B. UNRESOLVED STAFF COMMENTS**

None.

### **ITEM 2. PROPERTIES**

We lease approximately 13,225 square feet of office space in Menlo Park, California for our corporate facilities. Our current lease extended our occupancy through December 2013 and provides us with an option to extend the lease for one additional year. We expect that these facilities will accommodate our operations for the next year.

### **ITEM 3. LEGAL PROCEEDINGS**

We are not currently involved in any material legal proceedings.

### **ITEM 4. MINE SAFETY DISCLOSURES**

Not applicable.

**Table of Contents****PART II****ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES****Market Information**

Our common stock is traded on The NASDAQ Capital Market under the symbol "CORT". The following table sets forth the high and low intra-day sale prices per share of our common stock on The NASDAQ Capital Market for the periods indicated. These prices represent quotations among dealers without adjustments for retail mark-ups, markdowns or commissions, and may not represent prices of actual transactions.

	<b>High</b>	<b>Low</b>
<b>2012</b>		
First Quarter	\$ 4.90	\$ 2.50
Second Quarter	\$ 4.55	\$ 3.49
Third Quarter	\$ 4.51	\$ 2.48
Fourth Quarter	\$ 2.84	\$ 1.27
<b>2011</b>		
First Quarter	\$ 4.51	\$ 3.43
Second Quarter	\$ 5.07	\$ 3.67
Third Quarter	\$ 4.02	\$ 2.51
Fourth Quarter	\$ 3.58	\$ 2.70

**Stockholders of Record and Dividends**

As of March 1, 2013, we had 99,814,250 shares of common stock outstanding held by 116 stockholders of record. We have never declared or paid cash dividends on our capital stock. We currently intend to retain any future earnings to finance the growth and development of our business and therefore, do not anticipate paying any cash dividends in the foreseeable future. In addition, the Biopharma Financing Agreement prohibits payment of dividends unless we have cash and cash equivalents in excess of \$50 million after such payment.

**Sale of Unregistered Securities**

All sales of unregistered securities during the year ended December 31, 2012 have previously been disclosed in filings with the SEC. We have used, or will use, the net proceeds from these transactions to fund our on-going operations, including the commercialization of Korlym 300 mg Tablets, continuation of our phase 3 clinical trial of mifepristone for the treatment of the psychotic features of psychotic depression, the further development of our portfolio of next-generation compounds, as well as to fund working capital and for general corporate purposes.

**Repurchases of Securities**

None.

**Market Performance Graph**

*The graph and the accompanying text below is not soliciting material, is not deemed filed with the SEC and is not to be incorporated by reference in any filings by us under the Securities Act or the Exchange Act, whether made before or after the date hereof and irrespective of any general incorporation language in such filing.*

The rules of the SEC require that we include a line-graph comparing cumulative stockholder returns on our common stock with the NASDAQ Composite Index (which tracks the aggregate price performance of equity



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securities of companies traded on NASDAQ) and either a published industry or line-of-business standard index or an index of peer companies selected by us. We have elected to use the NASDAQ Biotechnology Index (consisting of a group of approximately 120 companies in the biotechnology sector, including us) for purposes of the performance comparison that appears below.

The graph shows the cumulative total stockholder return assuming the investment of \$100.00 and the reinvestment of dividends and is based on the returns of the component companies weighted according to their market capitalizations as of the end of the period for which returns are indicated. No dividends have been declared on our common stock.

The stockholder return shown on the graph below is not necessarily indicative of future performance, and we do not make or endorse any predictions as to future stockholder returns.

COMPARISON OF 5 YEAR CUMULATIVE TOTAL RETURN\* AMONG  
CORCEPT THERAPEUTICS, THE NASDAQ CAPITAL MARKET (U.S.) INDEX  
AND THE NASDAQ BIOTECHNOLOGY INDEX

\* \$100 invested on December 31, 2007 including reinvestment of dividends. Fiscal year ended December 31.

**Table of Contents****ITEM 6. SELECTED FINANCIAL DATA****SELECTED FINANCIAL DATA****(in thousands, except per share data)**

The selected financial data set forth below are derived from our financial statements. The statement of operations data for the years ended December 31, 2012, 2011, and 2010 and the balance sheet data as of December 31, 2012 and 2011 are derived from our audited financial statements included in this Annual Report on Form 10-K (Form 10-K). The statements of operations data for the years ended December 31, 2009 and 2008, and the balance sheet data as of December 31, 2010, 2009 and 2008 have been derived from our audited financial statements, which are not included in this Form 10-K. The selected financial data set forth below should be read in conjunction with our financial statements, the related notes and Management's Discussion and Analysis of Financial Condition and Results of Operations included elsewhere in this Form 10-K.

	2012	Year Ended December 31,			2008
		2011	2010	2009	
<i>(In thousands, except per share data)</i>					
<b>Statement of Operations Data:</b>					
Revenues:					
Product sales, net	\$ 3,307	\$	\$	\$	\$
Collaboration revenue				29	209
<b>Total revenues</b>	<b>3,307</b>			<b>29</b>	<b>209</b>
Operating expenses:					
Cost of sales	91				
Research and development*	14,074	21,001	18,949	14,402	14,152
Selling, general and administrative*	25,414	11,331	8,488	5,877	5,746
<b>Total operating expenses</b>	<b>39,579</b>	<b>32,332</b>	<b>27,437</b>	<b>20,279</b>	<b>19,898</b>
<b>Loss from operations</b>	<b>(36,272)</b>	<b>(32,332)</b>	<b>(27,437)</b>	<b>(20,250)</b>	<b>(19,689)</b>
Non-operating income (expense), net*	(1,776)	(22)	1,471	84	(372)
<b>Net loss</b>	<b>\$ (38,048)</b>	<b>\$ (32,354)</b>	<b>\$ (25,966)</b>	<b>\$ (20,166)</b>	<b>\$ (20,061)</b>
Net loss per share:					
Basic and diluted	\$ (0.41)	\$ (0.39)	\$ (0.38)	\$ (0.38)	\$ (0.43)
Weighted average shares basic and diluted	93,015	83,309	68,336	52,443	46,721
* Includes significant non-cash expenses, of the following:					
Stock-based compensation					
Research and development	\$ 546	\$ 547	\$ 220	\$ 263	\$ 268
Selling, general and administrative	4,764	2,888	1,896	1,552	1,360
<b>Total stock-based compensation</b>	<b>5,310</b>	<b>\$ 3,435</b>	<b>2,116</b>	<b>1,815</b>	<b>1,628</b>
Non-operating expense related to accretion of interest on long-term obligation	1,680				
<b>Total significant non-cash expenses</b>	<b>\$ 6,990</b>	<b>\$ 3,435</b>	<b>\$ 2,116</b>	<b>\$ 1,815</b>	<b>\$ 1,628</b>



	2012	2011	As of December 31,		2008
			2010	2009	
			<i>(In thousands)</i>		
<b>Balance Sheet Data:</b>					
Cash, cash equivalents and investments	\$ 93,032	\$ 39,635	\$ 24,578	\$ 23,867	\$ 18,309
Working capital	86,703	34,749	21,136	22,001	16,717
Total assets	99,166	39,833	25,104	24,511	19,775
Long-term obligation - current portion	2,650				6
Long-term obligation, net of current portion	29,030				
Total stockholders' equity	61,777	34,807	21,244	22,092	16,907

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**ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS**

**Forward-Looking Statements**

*This Management Discussion should be read in conjunction with the financial statements and notes thereto appearing elsewhere in this report. We make statements in this section that are forward-looking statements within the meaning of the federal securities laws. For a complete discussion of such forward-looking statements and the potential risks and uncertainties that may impact upon their accuracy, see Forward-Looking Statements included in Part I, Risk Factors included in Part I of this Form 10-K and the Overview and Liquidity and Capital Resources sections of this Management's Discussion and Analysis of Financial Condition and Results of Operations.*

**Overview**

We are a pharmaceutical company engaged in the discovery, development and commercialization of drugs for the treatment of severe metabolic and psychiatric disorders. Our focus is on disorders associated with the steroid hormone cortisol. Elevated levels and abnormal release patterns of cortisol have been implicated in a broad range of human disorders.

Since our inception in May 1998, we have been developing mifepristone, a potent glucocorticoid receptor II (GR-II) antagonist. On February 17, 2012, the FDA approved Korlym (mifepristone) 300 mg Tablets in the United States as a once-daily oral medication for treatment of hyperglycemia secondary to hypercortisolism in adult patients with endogenous Cushing's syndrome who have type 2 diabetes mellitus or glucose intolerance and have failed surgery or are not candidates for surgery. We made Korlym available to patients in April 2012 and have begun marketing the drug in the United States. We continue to develop the sales, marketing, medical affairs and logistical infrastructure needed to commercialize the drug. We also have an on-going phase 3 study of mifepristone, the active ingredient in Korlym, for treatment of the psychotic features of psychotic depression. We have discovered three series of novel selective GR-II antagonists.

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

*Cushing's Syndrome.* Cushing's syndrome is a disorder caused by prolonged exposure of the body's tissues to high levels of the hormone cortisol. Sometimes called hypercortisolism, it is 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 prevalence of 20,000 patients with Cushing's syndrome in the United States.

The FDA approved our NDA for Korlym on February 17, 2012. This approval allows us to market Korlym in the United States for its approved indication. We are carrying out our commercialization plans, including hiring a small number of medical science liaisons (MSLs) and sales representatives. We have also developed internet marketing capabilities and patient assistance programs to support physicians and patients. We began shipping Korlym to our specialty pharmacy in early April 2012, and the medicine first became available to patients on April 10, 2012. We finished hiring our team of MSLs in the third quarter of 2012. Our sales representatives received their initial training and were deployed to the field in the fourth quarter of 2012.

We have Orphan Drug Designations for Korlym from the FDA for the approved indication and from the European Commission for the treatment of endogenous Cushing's syndrome. Orphan Drug Designation in the United States is a special status granted by the FDA to encourage the development of treatments for diseases or conditions that affect fewer than 200,000 patients in the United States. Drugs that receive Orphan Drug Designation obtain seven years of marketing exclusivity for the approved indication from the date of drug

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approval, as well as tax credits for clinical trial costs, marketing application filing fee waivers and assistance from the FDA in the drug development process. Benefits of Orphan Drug Designation in the EU are similar to those in the United States, but include ten years of marketing exclusivity for the approved indication in all 27 member states, free scientific advice during drug development, access to a centralized review process and a reduction or complete waiver of fees levied by the European Medicines Agency.

*Psychotic Depression.* We are also developing mifepristone, the active ingredient in Korlym, for the treatment of the psychotic features of psychotic depression under an exclusive patent license from Stanford University. The FDA has granted fast track status to evaluate the safety and efficacy of mifepristone for the treatment of the psychotic features of psychotic depression.

In March 2008, we began enrollment in Study 14, our ongoing phase 3 trial in psychotic depression. The protocol for this trial incorporates what we have learned from our three previously completed phase 3 trials. It attempts to address the established relationship between increased drug plasma levels and clinical response and attempts to decrease the random variability observed in the results of the psychometric instruments used to measure efficacy. In one of the previously completed phase 3 trials, Study 06, we prospectively tested and confirmed that patients whose plasma levels rose above a predetermined threshold statistically separated from both those patients whose plasma levels were below the threshold and those patients who received placebo; this threshold was established from data produced in earlier studies.

As expected, the group of patients who took 1200 milligrams (mg) of mifepristone in Study 06 developed higher drug plasma levels than did the groups of patients who received lower doses. Further, there was no discernable difference in the incidence of adverse events between patients who received placebo in Study 06 and those who received 300 mg, 600 mg or 1200 mg of mifepristone in that study. In August 2011, we published our analysis of these data in *The Journal of Clinical Psychopharmacology*. Based on this information, we are testing a mifepristone dose of 1200 mg once per day for seven days in Study 14.

In addition, we are using a third-party centralized rating service to independently evaluate the patients for entry into the study as well as to evaluate their level of response throughout their participation in the study. We believe the centralization of this process will improve the consistency of rating across clinical trial sites and reduce the background statistical noise that was observed in earlier studies and is endemic to psychopharmacologic studies. We believe that the change in dose, as well as the other modifications to the protocol described above, should allow us to demonstrate the efficacy of mifepristone in the treatment of the psychotic symptoms of psychotic depression. In mid-2009, to conserve financial resources, we reduced the number of clinical sites in this study to eight and extended the timeline for its completion.

Enrollment in Study 14 is ongoing. Our goal is to enroll a sufficient number of patients by the end of 2013 to be able to perform a successful interim analysis. To help reach this goal, we began adding clinical sites in the fourth quarter of 2012 and plan to have 20 sites by the end of the first quarter of 2013.

*Antipsychotic-induced Weight Gain Mitigation.* In 2005, we announced the results of studies in rats that demonstrated that mifepristone both reversed the weight gain associated with the ongoing use of olanzapine and mitigated the weight gain associated with the initiation of treatment with olanzapine (the active ingredient in Zyprexa®). The results from this study were published in the journal *Brain Behavioral Research* in early 2006. This study was paid for by Eli Lilly and Company (Eli Lilly).

During 2007, we announced positive results from our clinical proof-of-concept study in lean healthy male volunteers evaluating the ability of mifepristone to mitigate weight gain associated with the use of Zyprexa. The results showed a statistically significant reduction in weight gain in those subjects who took Zyprexa plus mifepristone compared to those who took Zyprexa plus placebo. Also, the addition of mifepristone to treatment with Zyprexa had a beneficial impact on secondary metabolic measures such as fasting insulin, triglycerides and abdominal fat, as indicated by waist circumference. Eli Lilly provided Zyprexa and financial support for this

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study, the results of which were published in the journal *Advances in Therapy* in 2009. In January 2009, we announced positive results from a similar proof-of-concept study evaluating the ability of mifepristone to mitigate weight gain associated with the use of Johnson & Johnson's Risperdal®. This study confirmed and extended the earlier results seen with mifepristone and Zyprexa, demonstrating a statistically significant reduction in weight gain and in the secondary metabolic endpoints of fasting insulin, triglycerides and abdominal fat, as indicated by waist circumference. The results from the study of mifepristone and Risperdal were presented at several scientific conferences, including the American Diabetes Association meeting in June 2009, and were published in the journal *Obesity* in 2010.

The combination of Zyprexa or Risperdal and mifepristone is not approved for any indication. The purpose of these studies was to explore the hypothesis that GR-II antagonists, such as mifepristone and our next generation of selective GR-II antagonists, would mitigate weight gain associated with antipsychotic medications. The group of medications known as second generation antipsychotic medication, including Zyprexa, Risperdal, Clozaril® and Seroquel®, are widely used to treat schizophrenia and bipolar disorder. All medications in this group are associated with treatment-emergent weight gain of varying degrees and carry a warning in their labels relating to treatment-emergent hyperglycemia and diabetes mellitus.

*Selective GR-II Receptor Antagonists.* In 2003, we initiated a discovery research program to identify and patent selective GR-II antagonists with the intent of developing a pipeline of products for proprietary use. Three distinct series of GR-II antagonists were identified. These compounds, like mifepristone, potently block the cortisol receptor (GR-II) but, unlike mifepristone, they do not appear to block the PR (progesterone), ER (estrogen), AR (androgen) or GR-I (mineralocorticoid) receptors. Both the United States Patent & Trademark Office (USPTO) and the European Patent Office (EPO) have issued composition-of-matter patents to us on each of the three series. Two additional composition-of-matter patent applications are pending.

Several of our new compounds have demonstrated positive results in animal models for the prevention and reversal of anti-psychotic-induced weight gain. One of them, CORT 108297, is in exploratory phase 2a clinical trials and we plan to explore its potential use in other indications. We have identified other selective GR-II antagonists from our proprietary series that we believe may have utility as therapeutic agents in a variety of diseases. Our intent is to continue our discovery research program with the goal of identifying new selective GR-II antagonists and to manufacture and conduct pre-clinical development on one or more of these compounds and to submit Investigational New Drug (IND) applications with respect to the most promising of them, as we deem appropriate.

At the American Diabetes Association conference in June 2009, there was also a presentation of preclinical data from another study of CORT 108297 conducted at Stanford University. This study demonstrated that CORT 108297 suppresses body weight gain and improves insulin sensitivity in healthy mice fed a 60 percent fat diet and high sucrose liquid. The results of these preclinical data were published in April 2011 in the journal *Nutrition and Metabolism*.

In addition, we are continuing research and pre-clinical efforts to identify additional selective GR-II antagonists for clinical study.

### *General*

Our activities to date have included:

product development, including drug formulation and manufacturing, as well as designing, funding and overseeing clinical trials and conducting non-human clinical investigatory activities, such as toxicological testing;

commercialization of Korlym, including hiring and training medical science liaisons and sales representatives, retention and management of third-party distribution partners, establishment of third-party coverage and reimbursement and patient assistance programs and marketing activities.

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discovery research;

intellectual property prosecution and expansion; and

regulatory affairs.

Historically, we have financed our operations and internal growth primarily through private placements of our preferred and common stock, the public sale of common stock and our capped royalty financing transaction, rather than through collaborative or partnership agreements.

As of December 31, 2012, we had an accumulated deficit of \$246.6 million. Our historical operating losses have resulted principally from our research and development activities, including clinical trial activities for mifepristone, discovery research, non-clinical activities such as toxicology and carcinogenicity studies, manufacturing process development and regulatory activities, as well as selling, general and administrative expenses, including preparations for the commercial launch of Korlym. We may continue to incur net losses over at least the next few years as we continue our mifepristone and selective GR-II antagonist discovery and clinical development programs, apply for regulatory approvals, acquire and / or develop treatments in other therapeutic areas, establish sales and marketing capabilities and expand our operations.

Our business is subject to significant risks, including the risks inherent in our research and development efforts, the results of our mifepristone and other clinical trials, uncertainties associated with securing financing, uncertainties associated with obtaining and enforcing patents, our investment in manufacturing set-up, the management of our supply chain, the lengthy and expensive regulatory approval process and competition from other products. Our ability to successfully generate revenues in the foreseeable future is dependent upon our ability, alone or with others, to finance our operations and develop, obtain regulatory approval for, manufacture and market our products.

**Results of Operations**

*Net Product Sales* Net product sales includes product revenue resulting from sales to our customers, reduced by 1) trade allowances, such as discounts for prompt payment and distributor fees, 2) estimated government rebates and chargebacks, 3) reserves for expected product returns and 4) estimated costs of our patient assistance program.

In April 2012, we made Korlym commercially available in the United States through a specialty pharmacy that sells to individual patients and a specialty distributor that sells to hospital pharmacies. For the year ended December 31, 2012, we recognized \$3.3 million in net product sales. To calculate net product sales, we deducted from gross sales estimates of prompt-pay discounts, distribution service fees, rebates and chargebacks owed to government payors and patient assistance program costs, which amounts are not material for the year ended December 31, 2012.

Based on our limited experience marketing Korlym, it is difficult for us to forecast its sales for any future periods.

*Cost of sales* Cost of sales includes the cost to manufacture Korlym (which includes material, third-party manufacturing costs and indirect personnel and other overhead costs) based on units sold in the current period, as well as the cost of stability testing and distribution. We began capitalizing Korlym production costs as inventory following approval by the FDA to market Korlym on February 17, 2012. Prior to Korlym approval, we expensed all costs related to the manufacturing of product (including stability costs and manufacturing overhead) as incurred, classifying these costs as research and development expense. A portion of the product manufactured prior to FDA approval is available for us to use commercially.

Cost of sales was \$91,000 for the year ended December 31, 2012, which equals 2.8 percent of net product sales for the same period. The majority of these costs related to stability testing. The amount and timing of

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stability testing varies from period to period as determined by FDA regulations and our production schedule and is not a fixed percentage of our sales volumes. In addition, the cost of manufacturing Korlym reflected in our cost of sales in 2012, and for some period thereafter, will not reflect the full cost of production because we have previously expensed the majority of the raw materials, labor and overhead costs incurred to produce the product sold during this period. We expect that our cost of sales of Korlym as a percentage of net product sales will fluctuate from period to period during 2013 and for some time thereafter as product manufactured prior to FDA approval, which is already fully expensed, is consumed.

*Research and development expenses* Research and development expenses include 1) the personnel costs related to our development activities, including facilities costs and non-cash stock-based compensation, 2) the costs of discovery research, 3) costs associated with IND-enabling activities and pre-clinical studies, 4) costs of clinical trials, including trial preparation, enrollment, site monitoring and data management and analysis expenses, 5) regulatory costs, 6) the costs of manufacturing development, including the development and activities to qualify a second tablet manufacturing site, 7) the costs of manufacture and / or acquisition of clinical trial materials and material used in registration and validation batches included in the NDA submission for Korlym and 8) other costs associated with the preparation and prosecution of the Korlym NDA or other FDA submissions related to Korlym or other product candidates.

Research and development expenses decreased 33 percent to \$14.1 million for the year ended December 31, 2012 from \$21.0 million for the comparable period in 2011.

During the year ended December 31, 2012 as compared to the corresponding period in 2011, there was an increase of \$991,000 in staffing costs, which includes bonuses paid on FDA approval of Korlym in the amount of \$474,000, and an increase of \$164,000 for stock-based compensation expenses related to employees working in research and development functions. During the year ended December 31, 2012 as compared to the corresponding periods in 2011, there were decreases in consultancy costs of \$2.8 million due primarily to the additional resources required during 2011 for the preparation, submission and prosecution of the NDA, which was submitted in April 2011 and filed by the FDA in June 2011. For the year ended December 31, 2012, non-cash stock-based compensation expense related to consultant options decreased \$165,000 as compared to the corresponding period of 2011 due primarily to the inclusion in 2011 of costs related to a stock option award to a consultant that vested in its entirety on the acceptance of the NDA by the FDA in June 2011.

Korlym manufacturing costs categorized as research and development expense decreased \$3.8 million during the year ended December 31, 2012 as compared to the corresponding period in 2011, due primarily to capitalizing to inventory the costs of Korlym's active pharmaceutical ingredient and the manufacture of Korlym tablets for commercial sale following the date of FDA approval.

Clinical trial costs reflected a net decrease of \$1.6 million during the year ended December 31, 2012, as compared to the corresponding period of 2011. During the year ended December 31, 2012 as compared to the corresponding period in 2011, there were decreases of \$1.2 million related to clinical studies with CORT 108297 and \$917,000 related to the clinical trials with Korlym in the treatment of Cushing's syndrome. These decreases were partially offset by increases during year ended December 31, 2012, as compared to the corresponding period of 2011, of \$487,000 related to the psychotic depression study.

In addition, costs related to financial support for medical conferences and seminars in support of our Cushing's syndrome program decreased \$337,000 for the year ended December 31, 2012, as compared to the corresponding period of 2011, because, subsequent to product approval, the nature of our activities at medical meetings has changed, and now such costs relate to marketing activities that are classified as a component of selling, general and administrative expenses. Costs relating to IND-enabling activities and research efforts regarding our new GR-II antagonists increased \$363,000 during the year ended December 31, 2012, as compared to the corresponding period in 2011.

Research and development expenses increased 11% to \$21.0 million for the year ended December 31, 2011 from \$18.9 million for the comparable period in 2010. For the year ended December 31, 2011 as compared to the

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corresponding period in 2010, there were net increases of \$1.8 million in consultancy costs which included the following increases: a) \$588,000 related to the development of the Risk Evaluation and Mitigation System (REMS) that was included in the Korlym NDA submission, b) \$834,000 related to the preparation, submission and prosecution of the NDA, c) \$114,000 related to the development of a medical safety program, d) \$187,000 related to manufacturing and quality control activities to prepare for commercialization and e) \$192,000 in non-cash stock-based compensation costs related to a performance-based award to a consultant that vested in June 2011 upon the filing of our NDA for Korlym by the FDA, which were partially offset by the decrease in consulting fees in other clinical activities of \$78,000. For the year ended December 31, 2011, as compared to the corresponding period in 2010, there was also an increase of \$300,000 related to attendance of seminars in support of our Cushing's syndrome program.

Korlym manufacturing costs increased \$4.2 million during the year ended December 31, 2011, as compared to the corresponding period in 2010, due primarily to the acquisition of active pharmaceutical ingredient for Korlym and the initiation of manufacturing development work at a potential back-up site for the manufacture of Korlym that were only partially offset by a decrease in manufacturing activities related to our proprietary, selective new GR-II antagonists.

There were decreases in clinical trial costs of \$4.3 million during the year ended December 31, 2011, as compared to the corresponding period of 2010. Clinical trial cost decreases included (a) \$2.8 million related to drug-drug interaction and other NDA-supportive studies with Korlym that were substantially completed during 2010, (b) \$727,000 related to the clinical trials with Korlym for the treatment of Cushing's syndrome due to patients having completed the initial study and moving into the long-term extension study, (c) \$331,000 related to the clinical trial with mifepristone for the treatment of psychotic depression and (d) \$468,000 related to clinical studies activities with CORT 108297. During the year ended December 31, 2011, as compared to the corresponding period in 2010, there was also a decrease of \$244,000 related to the IND-enabling work on CORT 108297, which was partially offset by increases of \$168,000 related to research efforts on our other selective GR-II antagonists.

Below is a summary of our research and development expenses by major project:

Project	2012	Year Ended December 31,	
		2011	2010
		<i>(in thousands)</i>	
Development programs:			
Cushing's syndrome	\$ 4,093	\$ 10,925	\$ 5,075
Psychotic depression	2,613	1,779	2,567
Selective GR-II antagonists	4,249	4,546	5,100
Unallocated activities, including NDA supportive studies and manufacturing, regulatory and pre-clinical activities	2,573	3,204	5,987
Stock-based compensation	546	547	220
<b>Total research and development expense</b>	<b>\$ 14,074</b>	<b>\$ 21,001</b>	<b>\$ 18,949</b>

We expect that research and development expenditures in 2013 will likely be higher than they were in 2012, due to the cost of expanding enrollment in our phase 3 study of mifepristone in the treatment of psychotic depression and increased spending on the development of our next-generation selective GR-II antagonists. Research and development expenses in 2014 and beyond will depend on our strategic priorities. See also, Liquidity and Capital Resources .

Many factors can affect the cost and timing of our trials including inconclusive results requiring more clinical trials, slow patient enrollment, adverse side effects in study patients, insufficient supplies for our clinical trials and real or perceived lack of effectiveness or safety of the drug in our trials. The cost and timing of

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development of our selective GR-II antagonists will depend on the success of our efforts and any difficulties that we may encounter. In addition, the development of all of our product candidates will be subject to extensive governmental regulation. These factors make it difficult for us to predict the timing and costs of the further development and approval of our product candidates.

*Selling, general and administrative expenses* Selling, general and administrative expenses include 1) personnel and consultancy costs related to administrative and commercialization activities, including facilities costs and non-cash stock-based compensation, 2) expenses of third-party vendors that we engage to execute our commercial plans related to Korlym, including conducting market research, providing market analytics, developing reimbursement support services and, distribution and other logistical needs related to our commercialization of Korlym and 3) legal, accounting and other professional fees.

For the year ended December 31, 2012, selling, general and administrative expenses increased to \$25.4 million from \$11.3 million for the comparable period in 2011.

During the year ended December 31, 2012 as compared to the corresponding period in 2011, staffing and consultancy costs increased \$6.9 million due primarily to additional resources necessary to commercialize Korlym. The increase for the year ended December 31, 2012 included \$1.6 million in cash bonuses awarded in the first quarter of 2012 to employees working in selling, general and administrative functions in recognition of the FDA's approval of Korlym, \$1.3 million of non-cash stock-based compensation costs related to performance-based stock option awards to officers that vested in February 2012 upon the FDA approval of Korlym and \$600,000 of increases related to other stock options to directors, officers and employees working in selling, general and administrative functions.

In addition, other professional services costs related to commercialization activities and other corporate matters increased \$5.8 million during the year ended December 31, 2012 as compared to the corresponding period of 2011. These costs reflect increased vendor activities after FDA approval, pricing strategy and market analysis, patient registry and reimbursement programs, focus groups, internet marketing and communications.

There were also cost increases during the year ended December 31, 2012 as compared to the corresponding period of 2011 related to the infrastructure necessary to support the commercialization of Korlym including a) \$538,000 related to travel, b) 386,000 related to the expansion of facilities and information technology support, c) \$147,000 related to employee education primarily due to the training of the new medical science liaisons and d) \$95,000 related to donations.

For the year ended December 31, 2011, selling, general and administrative expenses increased 33% to \$11.3 million from \$8.5 million for the year ended December 31, 2010. During 2010, we had recorded an aggregate amount of \$1.3 million related to bonuses awarded to our officers and employees working in selling, general and administrative functions in recognition of significant company accomplishments during 2010. We did not award bonuses for 2011 performance to any officer or employee in these functions. In addition, during 2011, as compared to 2010, staffing and consultancy costs increased \$1.6 million due primarily to additional resources necessary to engage in preparations for the potential commercialization of Korlym, which included an increase of \$992,000 in noncash stock-based compensation costs related to stock options granted to employees, directors and consultants. There was also an increase of \$2.0 million during 2011, as compared to 2010, in market research and other commercialization preparation activities and an increase in legal costs related to patents, commercialization, compliance and other corporate matters during 2011, as compared to 2010, of \$456,000.

Selling, general and administrative expenses included stock-based compensation expense related to option grants to individuals performing these functions of \$4.8 million, \$2.9 million and \$1.9 million for the years ended December 31, 2012, 2011 and 2010, respectively.

We expect that selling, general and administrative expenses will increase during 2013 as compared to 2012 in regard to activities directly associated with product commercialization and the need to continue building our



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administrative infrastructure to support these activities. The level of selling, general and administrative activities and related expenses in 2014 and future years will be largely dependent on our assessment of the staff and other services necessary to support product commercialization and our continued clinical development activities and the availability of additional funds. See also, Liquidity and Capital Resources.

*Interest and other income, net* Interest and other income, net of investment management fees, was \$3,000 for the year ended December 31, 2011 as compared to \$1.5 million for the same period in 2010. Other income in 2011 was comprised of income on stockholder notes. Other income in 2010 had included \$750,000 in connection with the favorable settlement of a lawsuit brought on our behalf against an individual for defamation and harassment and \$733,000 in grants from the United States Treasury's Therapeutic Discovery Project Grant program.

*Interest and other expense* Interest and other expense for the year ended December 31, 2012 was \$1.8 million as compared to \$25,000 for the comparable period in 2011. This increase is primarily due to the inclusion in the current period of \$1.7 million of interest expense related to our Financing Agreement with Biopharma for the period from August 16, 2012, the date of funding of the agreement to December 31, 2012. Other expense for the year ended December 31, 2011 was \$25,000 in 2011 and in 2010 and consisted primarily of a state tax on capital, which is based on our capital and asset positions as of each year-end. Interest expense will increase during 2013, as compared to 2012, due to the inclusion of interest on the long-term obligation for a full year.

**Non-GAAP Financial Measures**

Our financial statements and footnotes thereto are prepared in accordance with U.S. Generally Accepted Accounting Principles (GAAP) and are included in Part IV, Item 15 of this Annual Report on Form 10-K. To supplement our financial results presented on a GAAP basis, we use non-GAAP measures of net loss that exclude significant non-cash expenses related to stock-based compensation expense and the accretion of interest expense under our capped royalty financing transaction. We use this non-GAAP measure of net loss to manage our business and believe that it may help investors better evaluate our past financial performance and potential future results. Non-GAAP measures should not be considered in isolation or as a substitute for comparable GAAP accounting and investors should read them in conjunction with our financial statements and notes thereto prepared in accordance with GAAP. The non-GAAP measure of net loss we use may be different from, and not directly comparable to, similarly titled measures used by other companies.

	<b>Year Ended December 31,</b>		
	<b>2012</b>	<b>2011</b>	<b>2010</b>
	<i>(in thousands)</i>		
GAAP net loss	\$ 38,048	\$ 32,354	\$ 25,966
Significant non-cash expenses:			
Stock-based compensation			
Research and development	546	547	220
Selling, general and administrative	4,764	2,888	1,896
Total stock-based compensation	5,310	3,435	2,1