RIGEL PHARMACEUTICALS INC
Form 10-K
March 08, 2016
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10 K

(Mark One)

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

For the fiscal year ended December 31, 2015

or

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

Commission file number 0 29889

RIGEL PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	94 3248524
(State or other jurisdiction of incorporation or organization)	(IRS Employer Identification No.)
1180 Veterans Blvd. South San Francisco, California	94080
(Address of principal executive offices)	(Zip Code)

(650) 624 1100

(Registrant’s telephone number, including area code)

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

Title of each class:	Name of each exchange on which registered:
Common Stock, par value $.001 per share	The Nasdaq Global 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 (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10 K or any amendment to this Form 10 K.

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

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

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

The approximate aggregate market value of the Common Stock held by non affiliates of the registrant, based upon the closing price of the registrant's Common Stock as reported on the Nasdaq Global Market on June 30, 2015, the last business day of the registrant's most recently completed second fiscal quarter, was $283,029,401. Shares of the registrant's outstanding Common Stock held by each executive officer, director and affiliates of the registrant's outstanding Common Stock have been excluded. The determination of affiliate status for the purposes of this calculation is not necessarily a conclusive determination for other purposes.

As of February 29, 2016, there were 90,556,255 shares of the registrant's Common Stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Items 10, 11, 12, 13 and 14 of Part III of this Annual Report on Form 10 K incorporate information by reference from the definitive proxy statement for the registrant's 2016 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission pursuant to Regulation 14A not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10 K.

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FORWARD LOOKING STATEMENTS

This Annual Report on Form 10 K contains statements indicating expectations about future performance and other forward looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act), and the Private Securities Litigation Reform Act of 1995, that involve risks and uncertainties. We usually use words such as “may,” “will,” “should,” “could,” “expect,” “plan,” “anticipate,” “might,” “believe,” “estimate,” “predict,” “intend” or the negative of similar expressions to identify these forward looking statements. These statements appear throughout this Annual Report on Form 10 K and are statements regarding our current intent, belief or expectation, primarily with respect to our operations and related industry developments. Examples of these statements include, but are not limited to, statements regarding the following: our business and scientific strategies; the progress of our product development programs, including clinical testing, and the timing of commencement and results thereof; our corporate collaborations, and revenues that may be received from collaborations and the timing of those potential payments; our drug discovery technologies; our research and development expenses; protection of our intellectual property; and sufficiency of our cash resources and need for additional capital. You should not place undue reliance on these forward looking statements. Our actual results could differ materially from those anticipated in these forward looking statements for many reasons, including as a result of the risks and uncertainties discussed under the heading “Risk Factors” in Part I, Item 1A of this Annual Report on Form 10 K. A forward looking statement speaks only as of the date on which it is made, and, except as required by law, we undertake no obligation to update any forward looking statement to reflect events or circumstances after the date on which the statement is made or to reflect the occurrence of unanticipated events. New factors emerge from time to time, and it is not possible for us to predict which factors will arise. In addition, we cannot assess the impact of each factor on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward looking statements.

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PART I

Item 1. Business

Overview

Rigel Pharmaceuticals, Inc. was incorporated in Delaware in June 1996, and is based in South San Francisco, California. We are a clinical stage biotechnology company dedicated to the discovery and development of novel, targeted drugs in the therapeutic areas of immunology, oncology and immuno-oncology. Our pioneering research focuses on signaling pathways that are critical to disease mechanisms. Our current clinical programs include fostamatinib, an oral spleen tyrosine kinase (SYK) inhibitor, which is in Phase 3 clinical trials for immune thrombocytopenic purpura (ITP); a Phase 2 clinical trial for autoimmune hemolytic anemia (AIHA); and a Phase 2 clinical trial for IgA nephropathy (IgAN). In addition, we have two oncology product candidates in Phase 1 development with partners BerGenBio AS (BergenBio) and Daiichi Sankyo (Daiichi).

Since the beginning of 2015, we have experienced the following significant business events:

- In February 2016, we announced that we initiated a Phase 2 clinical trial to evaluate fostamatinib as a potential treatment for AIHA. The trial is a two-stage study and we expect to report the results of the Stage 1 segment by the end of 2016.
- In January 2016, we experienced the following fostamatinib events:

i.) we completed patient enrollment of the first of two Phase 3 studies with fostamatinib for the treatment of ITP and expect to report results from the first study in the middle of 2016. We expect to report results on the second study shortly thereafter;

ii.) we announced that the Phase 2 study of fostamatinib in IgAN continues to enroll patients for the first cohort in various centers throughout Asia, the U.S. and Europe, and that the study is on track to report top line results in the second half of 2016; and

- In September 2015, we announced that we entered into an exclusive, worldwide license agreement with Aclaris Therapeutics International Limited (Aclaris) for the development and commercialization of certain JAK inhibitors for the treatment of alopecia areata and other dermatological conditions.
- In September 2015, we announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug designation to fostamatinib for the treatment of ITP.
- In June 2015, we announced that Keith A. Katkin, president and chief executive officer of Avanir Pharmaceuticals, has been appointed to our board of directors.
- In February 2015, we announced that we entered into a collaboration agreement with Bristol-Myers Squibb Company (BMS) for the discovery, development and commercialization of cancer immunotherapies based on our extensive portfolio of small molecule TGF beta receptor kinase inhibitors, in which BMS paid us an upfront payment of $30.0 million.
- In January 2015, we announced that in December 2014 we earned a non refundable payment of $5.8 million from AZ resulting from AZ's continued development of R256 in asthma as of December 2014, which we received in the first quarter of 2015.

Strategy

Our research team is focused on creating a portfolio of product candidates that may be developed as therapeutics for our own proprietary programs or for development by potential collaborative partners. We recognize that the product development process is subject to both high costs and a high risk of failure. We believe that identifying a variety of product candidates and working in conjunction with other pharmaceutical partners may minimize the risk of failure, fill the product pipeline gap at major pharmaceutical companies, and ultimately increase the likelihood of advancing clinical development and potential commercialization of the product candidates.

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The key elements to our business and scientific strategy are to:

- develop and commercialize fostamatinib in the United States where we believe a company our size can successfully compete;
- outlicense European and Asian rights to fostamatinib with Phase 3 clinical data in hand;
- develop and commercialize fostamatinib for possible additional indications;
- develop a diverse portfolio of drug candidates that address a focused band of therapeutic indications or that represent significant market opportunities;
- utilize our robust discovery engine to rapidly discover and validate new product candidates in a focused range of therapeutic indications; and
- develop drug candidates and establish strategic collaborations with pharmaceutical and biotechnology companies to further develop and market our product candidates.

Product Development Programs

Our product development portfolio features multiple novel, targeted drug candidates in the therapeutic areas of immunology, oncology and immuno-oncology.

Pipeline	Current Stage	Status
Fostamatinib—Oral SYK Inhibitor		
Immune Thrombocytopenic Purpura (ITP)	Phase 3	We completed patient enrollment of the first of two Phase 3 studies with fostamatinib for the treatment of ITP in January 2016 and expect to report results from the first study in the middle of 2016. We expect to report results on the second study shortly thereafter.
IgA Nephropathy (IgAN)	Phase 2	The Phase 2 study of fostamatinib in IgAN continues to enroll patients for the first cohort in various centers throughout Asia, the U.S. and Europe, and that the study is on track to report top line results in the second half of 2016.
Autoimmune Hemolytic Anemia (AIHA)	Phase 2	We initiated a Phase 2 clinical trial in patients with AIHA in February 2016. The trial is a two-stage study and we expect to report the results of the Stage 1 segment by the end of 2016.
R348—Topical Ophthalmic JAK/SYK Inhibitor		
Dry Eye in Patients with Ocular Graft Versus Host Disease (GvHD)	Phase 2	R348 is being evaluated in a Phase 2 clinical trial of patients with ocular GvHD to determine if it reduces inflammation and limits the damage to the eye tissue caused by the disease. We expect results of this clinical trial in 2016.

Clinical Stage Programs

Fostamatinib—Immune Thrombocytopenic Purpura

Disease background. Chronic ITP affects an estimated 60,000 to 125,000 people in the U.S. In patients with ITP, the immune system attacks and destroys the body's own blood platelets, which play an active role in blood clotting and healing. ITP patients can suffer extraordinary bruising, bleeding and fatigue as a result of low platelet counts.

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Current therapies for ITP include steroids, blood platelet production boosters that imitate thrombopoietin (TPOs) and splenectomy.

Orally available SYK inhibitor program. Taken in tablet form, fostamatinib blocks the activation of SYK inside immune cells. ITP causes the body to produce antibodies that attach to healthy platelets in the blood stream. Immune cells recognize these antibodies and affix to them, which activates the SYK enzyme inside the immune cell, and triggers the destruction of the antibody and the attached platelet. When SYK is inhibited by fostamatinib, it interrupts this immune cell function and allows the platelets to escape destruction. The results of our Phase 2 clinical trial, in which fostamatinib was orally administered to sixteen adults with chronic ITP, published in Blood, showed that fostamatinib significantly increased the platelet counts of certain ITP patients, including those who had failed other currently available agents.

In October 2013, we met with the U.S. FDA for an end-of-Phase 2 meeting for fostamatinib in ITP. Based on that meeting, we designed a Phase 3 clinical program, called fostamatinib in thrombocytopenia (FIT), in which a total of 150 ITP patients will be randomized into two identical multi-center, double-blind, placebo-controlled clinical trials. The patients will have been diagnosed with persistent or chronic ITP, and have blood platelet counts consistently below 30,000 per microliter of blood. Two-thirds of the subjects will receive fostamatinib orally at 100 mg bid (twice daily) and the other third will receive placebo on the same schedule. Subjects are expected to remain on treatment for 24 weeks. At week four of treatment, subjects who meet certain platelet count and tolerability thresholds will have their dosage of fostamatinib (or corresponding placebo) increased to 150 mg bid. The primary efficacy endpoint of this program is a stable platelet response by week 24 with platelet counts at or above 50,000 per microliter of blood for at least four of the final six qualifying blood draws. In August 2015, the FDA granted our request for Orphan Drug designation to fostamatinib, our oral SYK inhibitor, for the treatment of ITP. The first trial of our Phase 3 clinical program for ITP completed patient enrollment in January 2016. Our second Phase 3 trial is currently actively enrolling patients. We expect to separately report top line results of the two Phase 3 trials, with the first trial reporting in the middle of 2016 and the other trial reporting shortly thereafter.

Fostamatinib—IgAN

Disease background. IgAN is an autoimmune disease that severely affects the functioning of the kidneys. An estimated 12,000 Americans are diagnosed with this type of glomerulonephritis each year, with 25% of its victims eventually requiring dialysis and/or kidney transplantation over time. IgAN is characterized by the deposition of IgA immune complexes in the glomeruli of the kidneys leading to an inflammatory response and subsequent tissue damage that ultimately disrupts the normal filtering function of the kidneys. By inhibiting SYK in kidney cells, fostamatinib may block the signaling of IgA immune complex receptors and arrest or slow destruction of the glomeruli.

Orally-available SYK inhibitor program. Our Phase 2 clinical trial in patients with IgAN, called SIGN (SYK Inhibition for Glomerulonephritis) continues to enroll patients for the first cohort. We expect to report top line results in the second half of 2016.

Fostamatinib—AIHA

Disease background.

AIHA is a rare, serious blood disorder where the immune system produces antibodies that result in the destruction of the body's own red blood cells. Symptoms can include fatigue, shortness of breath, rapid heartbeat, jaundice or enlarged spleen. While no medical treatments are currently approved for AIHA, physicians generally treat acute and chronic cases of the disorder with corticosteroids, other immuno-suppressants, or splenectomy. Research has shown that inhibiting SYK with fostamatinib may reduce the destruction of red blood cells. This disorder affects an

estimated 40,000 Americans, for whom no approved treatment options currently exist.

Orally available SYK inhibitor program. We initiated a Phase 2 clinical trial in patients with AIHA in February 2016. The trial is an open-label, multi-center, two-stage study that will evaluate the efficacy and safety of fostamatinib in patients with warm antibody AIHA who have previously received treatment for the disorder, but have relapsed. Stage 1

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will enroll 17 patients who will receive 150 mg of fostamatinib orally twice a day for a period of 12 weeks. The patients will return to the clinic every two weeks for blood draws and medical assessment. The primary efficacy endpoint of this study is to achieve increased hemoglobin levels by week 12 of greater than 10 g/dL, and greater than or equal to 2 g/dL higher than baseline. Stage 2 will begin after enrollment in Stage 1 has been completed and will include an additional 20 patients who will receive the same treatment protocol as Stage 1. We expect to have results of the Stage 1 segment of the trial by the end of 2016.

R348—Dry Eye in Patients with Ocular Graft Versus Host Disease (GvHD)

Disease background. According to an article published by the American Academy of Ophthalmology, a significant number (22% to 80%) of patients with acute or chronic GvHD develop a secondary incidence of dry eye (keratoconjunctivitis sicca). In general, these patients are severely ill and have a great medical need for a topical therapy that may better manage their symptoms.

Topical Ophthalmic JAK/SYK inhibitor program. R348, an ophthalmic JAK/SYK inhibitor, is being evaluated in a Phase 2 study of patients with ocular GvHD to determine if it reduces inflammation and limits the damage to the eye tissue caused by the disease. We expect results of this clinical trial in 2016.

Research/Preclinical Programs

We are conducting proprietary research in the broad disease areas of inflammation/immunology, immuno-oncology, cancers and muscle wasting/muscle endurance. Within each disease area, our researchers are investigating mechanisms of action as well as screening compounds against potential novel targets and optimizing those leads that appear to have the greatest potential.

We are conducting preclinical studies to identify a lead molecule for our IRAK program. This program may provide opportunities in both the oncology and immunology areas, including acute myeloid leukemia (AML). We are currently targeting AML and MDS with different mechanisms of action in various preclinical projects.

Leveraging our extensive immunology expertise, we are continuing to explore novel immuno-oncology approaches to treating various oncology indications. The first of these resulted in a collaboration with BMS for TGF beta receptor kinase inhibitors. Several other projects are currently underway.

Sponsored Research and License Agreements

We conduct research and development programs independently and in connection with our corporate collaborators. We are a participant in our collaboration agreement with BMS for the discovery, development and commercialization of cancer immunotherapies based on our small molecule TGF beta receptor kinase inhibitors, as discussed below. Our participation is limited to the Joint Research Committee and the performance of research activities based on billable full-time equivalent fees as specified in the agreement. We do not have ongoing participation obligations under our agreements with Aclaris for the development and commercialization of certain JAK inhibitors for the treatment of alopecia areata and other dermatological conditions, AZ for the development and commercialization of R256, an inhaled JAK inhibitor, BerGenBio for the development and commercialization of an oncology program, and Daiichi to pursue research related to a specific target from a novel class of drug targets called ligases. Under these agreements, which we entered into in the ordinary course of business, we received or may be entitled to receive upfront cash payments, progress dependent contingent payments on events achieved by such partners and royalties on any net sales of products sold by such partners under the agreements. Total future contingent payments to us under all of these current agreements could exceed $533.6 million if all potential product candidates achieved all of the payment triggering events under all of our current agreements (based on a single product candidate under each agreement). Of

this amount, up to $150.5 million relates to the achievement of development events, up to $345.6 million relates to the achievement of regulatory events and up to $37.5 million relates to the achievement of certain commercial or launch events. This estimated future contingent amount does not include any estimated royalties that could be due to us if the partners successfully commercialize any of the licensed products. Future events that may trigger payments to us under the

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agreements are based solely on our partners' future efforts and achievements of specified development, regulatory and/or commercial events.

Because we do not control the research, development or commercialization of the product candidates generated under these agreements, we are not able to reasonably estimate when, if at all, any contingent payments would become payable to us. As such, the contingent payments we could receive thereunder involve a substantial degree of risk to achieve and may never be received in the next twelve months or thereafter. Accordingly, we do not expect, and investors should not assume, that we will receive all of the potential contingent payments provided for under these agreements and it is possible that we may never receive any additional significant contingent payments or royalties under these agreements.

In October 2015, we entered into a non-exclusive license agreement with a third party, pursuant to which we received a payment in the single-digit millions in exchange for granting a non-exclusive license to certain limited intellectual property rights. We concluded that the granting of the license, which was fully delivered to such third party in the fourth quarter of 2015, represents the sole deliverable under this agreement. Accordingly, we have recognized the payment as revenue during the year ended December 31, 2015.

In August 2015, we entered into a license agreement with Aclaris, pursuant to which Aclaris will have exclusive rights and will assume responsibility for the continued development of certain JAK inhibitor compounds for the treatment of alopecia areata and other dermatological conditions. Under the license agreement, we received a noncreditable and non-refundable upfront payment of $8.0 million in September 2015. We are also entitled to receive development and regulatory contingent fees that could exceed $80.0 million for a successful compound approved in certain indications. In addition, we are also eligible to receive tiered royalties on the net sales of any products under the agreement. We concluded that the granting of the license, which has been fully delivered to Aclaris in the third quarter of 2015, represents the sole deliverable under this agreement. Accordingly, we have recognized the $8.0 million payment as revenue during the year ended December 31, 2015.

In February 2015, we entered into a collaboration agreement with BMS for the discovery, development and commercialization of cancer immunotherapies based on our extensive portfolio of small molecule TGF beta receptor kinase inhibitors. Under the collaboration agreement, BMS will have exclusive rights and will be solely responsible for the clinical development and commercialization of any products. Pursuant to the collaboration agreement with BMS, we received a noncreditable and non-refundable upfront payment of $30.0 million in March 2015. We are also entitled to receive development and regulatory contingent fees that could exceed $309.0 million for a successful compound approved in certain indications. In addition, we are also eligible to receive tiered royalties on the net sales of any products from the collaboration. BMS shall also reimburse us for agreed upon costs based on a contractual cost per full-time equivalent employee in connection with the performance of research activities during the research term. Under the collaboration agreement, we were obligated to provide the following deliverables: (i) granting of license rights to our program, (ii) participation in the Joint Research Committee, and (iii) performance of research activities. We concluded that these deliverables are a single unit of accounting as the license does not have stand-alone value apart from the other deliverables. Accordingly, the $30.0 million upfront payment is being recognized ratably as revenue from the effective date of the agreement through September 2016, the end of the estimated research term. We believe that straight-line recognition of this revenue is appropriate as the research is expected to be performed ratably over the research period. During the year ended December 31, 2015, we recognized revenue of $16.6 million and $822,000 relating to the upfront payment and research activities we performed, respectively. As of December 31, 2015, deferred revenue related to the $30.0 million upfront payment was $13.4 million.

Our Discovery Engine

The approaches that we use in connection with both our proprietary product development programs and our corporate collaborations are designed to identify protein targets for compound screening and validate the role of those targets in the disease process. Unlike genomics based approaches, which begin by identifying genes and then searching for their functions, our approach identifies proteins that are demonstrated to have an important role in a specific disease pathway. By understanding the disease pathway, we attempt to avoid studying genes that will not make good drug

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targets and focus only on the subset of expressed proteins of genes that we believe are specifically implicated in the disease process.

We begin by developing assays that model the key events in a disease process at the cellular level. We then identify potential protein targets. In addition, we identify the proteins involved in the intracellular process and prepare a map of their interactions, thus giving us a comprehensive picture of the intracellular disease pathway. We believe that our approach has a number of advantages, including:

- improved target identification: it focuses only on the subset of expressed proteins of genes believed to be specifically implicated in the disease process;
- rapid validation of protein targets: it produces validated protein targets quickly because it uses key events in the disease process as the basis to design the functional, disease based screen;
- improved disease pathway mapping: it produces a comp