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Rigel to Provide Business Review and Updated Phase 2 Results for Fostamatinib in AIHA at the 36th Annual J.P. Morgan Healthcare Conference

SOUTH SAN FRANCISCO, Calif., Jan. 4, 2018 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL) today announced that Raul Rodriguez, the company's president and chief executive officer, will present an update on its product pipeline and a financial overview at the upcoming 36th Annual J.P. Morgan Healthcare Conference in San Francisco, California on January 10, 2018, at 1:30pm PST (see webcast details below).

Rigel's presentation will include a review of the company's New Drug Application (NDA) for fostamatinib, an oral spleen tyrosine kinase (SYK) inhibitor, in adult patients with immune thrombocytopenia (ITP). The FDA has confirmed that it does not plan to convene an Oncology Drugs Advisory Committee (ODAC) meeting to discuss the NDA. The action date for the FDA to complete its review is April 17, 2018 under the Prescription Drug User Fee Act (PDUFA).

"In 2017, we achieved the critical milestones necessary to transition Rigel into a fully integrated research, development and commercial company prepared to launch its first product into the US market," said Mr. Rodriguez. "2018 will be an exciting year. Fostamatinib, if approved by the FDA, could become an important alternative treatment option for patients with chronic ITP. Additionally, we have now shown in preliminary results that 47% of the patients treated with fostamatinib in our autoimmune hemolytic anemia study had a clinical response as measured by a defined increase in hemoglobin. There are currently no approved therapies for this indication."

Portfolio Update

Fostamatinib in ITP

In June, Rigel announced that the FDA had accepted for filing its NDA for fostamatinib for the treatment of adult patients with ITP. The NDA is supported by data from the FIT clinical program, which included three Phase 3 studies, two randomized placebo-controlled studies (Studies 047 and 048) and an open-label extension study (Study 049). Since the NDA filing, Rigel has worked closely with the FDA to address any questions and will continue to do so in 2018. Rigel has undergone two routine FDA inspections at its headquarters (BIMO and PAI)

which did not result in any FDA Form 483 observations. In addition, the FDA has inspected the two highest enrolling FIT clinical sites, both without 483 observations.

The organization is ramping up for a potential product launch, anticipated in the second quarter of 2018. In addition to hiring key personnel, Rigel is developing relationships with external partners to establish distribution channels and the systems needed to provide medication access.

Fostamatinib in Autoimmune Hemolytic Anemia (AIHA)
The Phase 2, open-label, multi-center, Simon two-stage study of fostamatinib for the treatment of warm AIHA completed enrollment of Stage 1. The study is evaluating the safety and efficacy of fostamatinib, at 150mg BID (twice daily), in patients with warm AIHA who have previously received at least one treatment for this disease, but did not have a meaningful benefit and are still anemic.

Stage 1 of the study enrolled 17 evaluable patients. 47% of these patients (8 patients out of 17) have responded to fostamatinib treatment as of December 2017. Six patients, including the last two patients enrolled, responded during the 12-week evaluation period and an additional two patients met the response criteria in the extension study after 12 weeks of dosing. A response was defined as achieving a hemoglobin level of greater than 10 g/dl and at least a 2 g/dl increase from baseline. The safety profile was consistent with the existing fostamatinib safety database. Stage 2 enrollment commenced in late 2017. Stage 2 follows the same protocol as Stage 1 and will include 20 patients.

These data are planned to be presented at a future medical meeting. Rigel plans to meet with the FDA in the first half of 2018 to determine the regulatory path for approval of fostamatinib in AIHA.

Additional Product Development

- Rigel has completed enrollment of the second cohort in its blinded Phase 2 study of fostamatinib in IgA Nephropathy (IgAN). The study is evaluating the efficacy, safety, and tolerability of fostamatinib as measured by change in proteinuria, renal function, and histology (comparing the pre- and post-study renal biopsies). The second cohort evaluates a higher dose of fostamatinib, 150mg BID, while the first cohort evaluated 100mg BID. The primary efficacy endpoint is the mean change of proteinuria from baseline at 24 weeks. Rigel expects the study to be complete at the end of the first quarter of 2018.
- During 2017, Rigel selected a molecule from its IRAK program for preclinical development. The molecule is differentiated in that it inhibits both the IRAK 1 and IRAK 4 signaling pathways, with potential to treat autoimmune and inflammatory diseases such as psoriasis, lupus, gout, psoriatic arthritis and multiple sclerosis. The company expects to initiate clinical trials in 2018.

Financial Update
Based upon preliminary estimates, Rigel expects to end 2017 with approximately $115.6 million in cash, cash equivalents, and short-term investments, which it believes will be sufficient to fund its operations into 2019. These operations include the potential commercial launch of fostamatinib in the U.S. in 2018 including hiring the sales force associated with the launch, the fostamatinib clinical trials described above for AIHA and IgAN, and continuing to

advance the research pipeline.

About ITP

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. Common symptoms of ITP are excessive bruising and bleeding. People suffering with chronic ITP may live with increased risk of severe bleeding events that can result in serious medical complication, or even death. Current therapies for ITP include steroids, blood platelet production boosters (TPOs) and splenectomy. However, not all patients are adequately treated with existing therapies. As a result, there remains a significant medical need for additional treatment options for patients with ITP.

About AIHA

Autoimmune hemolytic anemia (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. AIHA affects approximately 40,000 adult patients in the US and can be a severe, debilitating anemia. To date, there are no approved therapies disease-targeted therapies for AIHA, despite the tremendous medical need that exists for these patients.

Webcast Details

To access the live audio webcast or the subsequent archived recording, log on to www.rigel.com. Please connect to Rigel's website several minutes prior to the start of the live webcast to ensure adequate time for any software download that may be necessary.

About Rigel (www.rigel.com)

Rigel Pharmaceuticals, Inc. is a biotechnology company dedicated to discovering, developing and providing novel small molecule drugs that significantly improve the lives of patients with immune and hematological disorders, cancer and rare diseases. Rigel's pioneering research focuses on signaling pathways that are critical to disease mechanisms. The company's current clinical programs include clinical trials of fostamatinib, an oral spleen tyrosine kinase (SYK) inhibitor, in a number of indications. Rigel has submitted and the FDA has accepted for review, an NDA for fostamatinib in patients with chronic or persistent immune thrombocytopenia (ITP). In addition, Rigel has product candidates in development with partners BerGenBio AS, Daiichi Sankyo and Aclaris Therapeutics.

Forward Looking Statements

This release contains forward-looking statements relating to, among other things, the timing of enrollment and results of on-going clinical trials and the results of the FDA's review of Rigel's NDA for fostamatinib in patients with chronic or persistent ITP; Rigel's ability to transition to an organization prepared to launch its first commercial product; Rigel's belief that fostamatinib may be an important alternative for patients with ITP or AIHA; the management and advancement of Rigel's clinical programs; and the timing and results of Rigel's clinical trials. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "planned," "will," "may," "should," "expect," and similar expressions are intended to identify these forward-looking statements. These forward-looking statements are based on Rigel's current expectations and inherently involve significant risks and uncertainties. Actual results and the timing of events could differ materially from those anticipated in such forward looking statements as a result of these risks and uncertainties, which include, without limitation, the FDA may interpret Rigel's findings differently, which could result in

the FDA not approving the NDA; the availability of resources to develop Rigel's product candidates; market competition; as well as other risks detailed from time to time in Rigel's reports filed with the Securities and Exchange Commission, including its Quarterly Report on Form 10-Q for the period ended September 30, 2017. Rigel does not undertake any obligation to update forward-looking statements and expressly disclaims any obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein.

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