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Rigel Provides Business Updates and Preliminary Data in IgA Nephropathy

Announces Presentation at the 35th Annual J.P. Morgan Healthcare Conference in San Francisco

SOUTH SAN FRANCISCO, Calif., Jan. 5, 2017 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL) today announced that Raul Rodriguez, the company's president and chief executive officer, will present a review of products in development and a financial overview at the upcoming 35th Annual J.P. Morgan Healthcare Conference in San Francisco on January 11, 2017, at 2pm PST (see webcast details below).



Rigel's presentation will review the fostamatinib clinical program, highlighting study data from the recently completed Phase 3 FIT clinical program in patients with immune thrombocytopenic purpura (ITP). Rigel will also present initial Phase 2 study results of fostamatinib in IgA nephropathy (IgAN), an autoimmune disease of the kidneys, and provide an update on its pipeline development.

"In 2016, Rigel completed its FIT Phase 3 clinical program in chronic ITP. Based on these results, we believe that fostamatinib, if approved by the FDA, could become an attractive new treatment option for some patients with this serious disease," said Mr. Rodriguez. "We're looking forward to submitting a New Drug Application to the U.S. Food and Drug Administration (FDA) for fostamatinib in chronic ITP in the first quarter of this year. In addition, our recent results in IgAN are encouraging and may provide an additional indication

for fostamatinib," he added.

Product Development Highlights

Fostamatinib in ITP

- In August and October 2016, Rigel reported results from the FIT Phase 3 studies of fostamatinib for the treatment of ITP. The results of the Phase 3 clinical program, including the long-term extension study, demonstrate that those patients who respond to fostamatinib have a timely, robust, and sustained response. Given the totality and consistency of these Phase 3 results, the company expects to file a New Drug Application in the first quarter of this year. Rigel has not received any comments or questions on this plan from the FDA.

Fostamatinib in IgAN

- The first cohort in the Phase 2 study of fostamatinib in IgAN was completed in various centers throughout Asia, the U.S. and Europe. This cohort evaluated the efficacy, safety, and tolerability of a low dose of fostamatinib (100mg BID, n=26; placebo n=12) as measured by change in proteinuria, renal function, and histology (comparing the pre- and post-study renal biopsies). The primary efficacy endpoint was the mean change of proteinuria from baseline at 24 weeks. The study found that at 24 weeks fostamatinib was well tolerated with a good safety profile. The initial data suggest a trend towards a greater reduction in proteinuria in fostamatinib treated patients relative to placebo. Further analysis of the Cohort 1 data, particularly the histology review of the renal biopsies, as well as other secondary efficacy endpoints, continues and will be presented later in 2017.

"IgA nephropathy, the most common glomerulonephritis worldwide, is a disease that causes inflammation and scarring of the kidney. Patients with IgA nephropathy are at risk of serious complications of kidney dysfunction including high blood pressure and renal failure. Currently, there is no specific approved or consensus treatment for IgA nephropathy," stated James A. Tumlin, M.D., Professor, Internal Medicine and Nephrology, The University of Tennessee Health Science Center at Chattanooga. "These Phase 2 results are very encouraging, particularly in that fostamatinib was well tolerated and demonstrated a trend towards improving proteinuria. We hope that future analyses and additional studies will confirm the effectiveness of fostamatinib for the treatment of IgAN."

- Rigel expects that the second cohort, evaluating a higher dose of fostamatinib (150 mg) for IgAN, will finish enrollment in 2017 with full results in 2018. Rigel plans to seek a pharmaceutical partner with a strong Asian market presence to collaborate in the design and conduct of follow-on Phase 3 studies, as well as take responsibility for the subsequent commercialization in that territory.

Additional Product Development

- In 2016, Rigel initiated stage 1 of a Phase 2 proof-of-concept study of fostamatinib in patients with autoimmune hemolytic anemia (AIHA). Rigel expects to have results in 2017.
- In the fourth quarter of 2016, as part of our immuno-oncology partnership, Bristol-

Myers Squibb identified a TGF beta-receptor kinase inhibitor molecule for IND-enabling toxicology studies, resulting in a \$3 million milestone payment to Rigel.

- Rigel plans on selecting a molecule from its IRAK program for preclinical development in 2017. It is expected that the program will include clinical evaluation in immunology areas, such as for lupus, gout and/or psoriasis.

Financial Update

Based upon preliminary estimates, Rigel expects to end 2016 with approximately \$74.8 million in cash, cash equivalents, and short-term investments, which it believes will be sufficient to fund its operations into 2018. These operations include funding the NDA submission and activities supporting the commercial launch of fostamatinib in the U.S. in 2018.

Rigel expanded its commercialization and operational capabilities with the hiring and promotion of key executives. Joining Rigel in 2016 are Eldon Mayer, Executive Vice President and Chief Commercial Officer, Ben Cadieux, Ph.D, Head of Medical Affairs, Esteban Masuda, Ph.D., Senior Vice President, Research, and Joseph Lasaga, Vice President, Business Development and Alliance Management.

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 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, a significant portion of patients do not do well on existing therapies. As a result, there remains a significant medical need for additional treatment options for patients with ITP.

Fostamatinib is an oral investigational candidate with a unique mechanism of action designed to inhibit SYK kinase, a key player in the immune process that leads to platelet destruction in ITP. The FDA has granted Orphan Drug designation to fostamatinib for the treatment of patients with ITP. Unlike other therapies that modulate the immune system in different ways or stimulate platelet production, fostamatinib may address the underlying autoimmune cause of ITP by impeding platelet destruction.

About IgAN

IgA Nephropathy (IgAN) (also known as Berger's disease) is a chronic autoimmune disease associated with inflammation in the kidneys that diminishes their ability to filter blood. It is the most common primary glomerular disease affecting an estimated 82,500 - 165,000 cases in the US, with a higher prevalence in Asia. For as many as 25 percent of those living with IgAN, the disease results in end-stage renal failure requiring dialysis or kidney transplantation. Outside of angiotensin blockade (primarily for blood-pressure control), there are no disease-targeted therapies for IgAN. Pre-clinical data show that fostamatinib decreases SYK activation in the kidney, reverses the inflammation in the glomeruli and

improves kidney function. The Phase 2 clinical evaluation continues in 2017.

About Rigel (www.rigel.com)

Rigel Pharmaceuticals, Inc. is 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. 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. The company completed and reported results from two Phase 3 clinical studies of fostamatinib in chronic immune thrombocytopenia (ITP) in August and October 2016. Rigel is also conducting a Phase 2 clinical trial with fostamatinib in autoimmune hemolytic anemia (AIHA) and a Phase 2 clinical trial for IgA nephropathy (IgAN). In addition, Rigel has two oncology product candidates in Phase 1 development with partners BerGenBio AS and Daiichi Sankyo.

Forward Looking Statements

This release contains forward-looking statements relating to, among other things, the progress, timely execution and timing of reporting topline data of the Phase 2 clinical study with fostamatinib in IgAN, the Phase 2 clinical study of fostamatinib in AIHA; the results of Rigel's discussions with the FDA regarding its plans to advance fostamatinib through the regulatory review process, including the timing of and Rigel's ability to file a New Drug Application; the management and advancement of Rigel's other clinical programs; Rigel's belief that fostamatinib may be an attractive alternative for patients with ITP; Rigel's ability to successfully seek a pharmaceutical partner to collaborate in the design and conduct of follow-on Phase 3 studies, as well as to commercialize in Asia; statements relating to Rigel's cash position as of December 31, 2016; the timing, amount and sufficiency of Rigel's cash, cash equivalents, and short-term investments; Rigel's ability to extend the value of Rigel's pipeline into fields that are beyond its therapeutic focus, the evaluation of fostamatinib for new treatment indications; and Rigel's product pipeline and development programs. 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," "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 availability of resources to develop Rigel's product candidates, Rigel's need for additional capital in the future to sufficiently fund Rigel's operations and research, the uncertain timing of completion of and the success of clinical trials, market competition, risks associated with and Rigel's dependence on Rigel's corporate partnerships, risks related to changes in estimated cash position based on the completion of financial closing procedures and the audit of Rigel's financial statements, 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 three months ended September 30, 2016. 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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