August 30, 2016

Rigel's Fostamatinib Meets Primary Endpoint in Phase 3 Study in Chronic ITP

Conference call and webcast today at 8:00 AM Eastern Time

SOUTH SAN FRANCISCO, Calif., Aug. 30, 2016 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL) today announced that fostamatinib, its oral spleen tyrosine kinase (SYK) inhibitor, met the primary endpoint in the first of two double-blind studies in the FIT Phase 3 clinical program for the treatment of adult chronic/persistent immune thrombocytopenia (ITP). The study (n=76) showed that 18% of patients receiving fostamatinib achieved a stable platelet response compared to none receiving a placebo control (p=0.0261). A stable platelet response was defined as achieving greater than 50,000 platelets per uL of blood on at least four of the last six scheduled visits between weeks 14 and 24 of treatment. The results from the second FIT Phase 3 study are expected in October/November 2016.

The most frequent adverse events were gastrointestinal-related, and the safety profile of the product was consistent with prior clinical experience, and no new or unusual safety issues were discovered.

"These data demonstrate the potential benefit of fostamatinib for chronic ITP patients who are in need of new treatment options," said Raul Rodriguez, president and chief executive officer of Rigel. "We believe that fostamatinib has significant commercial potential given that it has a unique mechanism of action that may work where other products have failed."

"We are very encouraged by these results," said Anne-Marie Duliege, M.D., executive vice president and chief medical officer of Rigel. "Consistent with the prior clinical study of fostamatinib in ITP, this FIT Phase 3 study demonstrated that fostamatinib provided a robust and enduring benefit for those patients who responded to the drug candidate."

Patients who met the primary endpoint of this study typically had an increase in platelet counts to a level above 50,000/uL within the initial weeks of treatment, providing early feedback as to whether it was a viable option for treating their ITP.

In general, the clinical goal of ITP treatment is to raise platelet counts to more than 50,000/uL. Patients who met the primary endpoint in this study had their platelet counts increase from a median of 16,000/uL at baseline to a median of more than 100,000/uL at week 24, a robust response that potentially allows patients to remain above 50,000/uL more

consistently.

All of the patients from this study who met the stable platelet response endpoint enrolled in the long-term, Phase 3 extension study and continued to maintain their platelet levels for months past the initial study period of 24 weeks. These data affirm similar results observed in two patients from the Rigel Phase 2 study of fostamatinib in ITP who have been taking fostamatinib for more than seven years and have maintained stable platelet levels over this extended time period.

Fostamatinib's clinical safety profile includes more than 5,000 patient years of data across multiple autoimmune indications and has a well-defined and manageable safety profile, providing data that it may be suitable for long-term maintenance therapy in chronic ITP.

If these results are reproduced in the second Phase 3 study and are supported by the results of a planned interim analysis of the Phase 3 extension study, the company expects to submit a New Drug Application with the U.S. Food and Drug Administration in the first quarter of 2017. Further results from the FIT Phase 3 studies and long-term extension will be presented at future medical meetings.

FIT Phase 3 Program

The FIT program consists of two identical multi-center, randomized, double-blind, placebo-controlled studies of approximately 75 adult patients each. The patients have been diagnosed with persistent or chronic ITP, and have blood platelet counts consistently below 30,000/uL of blood. The patients all had experience with at least one other ITP treatment such as steroids, Rituxan, splenectomy and/or TPO mimetics. Patients were randomized in a 2:1 ratio to receive either fostamatinib or placebo twice a day to be taken for up to six months. Study subjects remained on treatment for up to 24 weeks. The primary efficacy endpoint of this program is a stable platelet response defined as achieving platelet counts at or above 50,000/uL of blood for at least four of the last six clinic visits of the study. Patients were subsequently offered to enroll in an open-label, Phase 3, long-term extension study, which is ongoing.

Fostamatinib and 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. There are approximately 50-60 thousand adult patients in the U.S. living with primary chronic ITP. ITP patients can suffer extraordinary bruising, bleeding and fatigue as a result of low platelet counts. Further, people suffering with chronic ITP live with increased risk of severe bleeding events that can result in serious medical complications or even death. Current therapies for ITP include steroids, blood platelet production boosters (TPOs) and splenectomy. While these treatment options can be effective in treating ITP symptoms, given the heterogeneity of the disease, each has significant limitations. It can be difficult to predict which approved treatments are going to be effective.

Fostamatinib is an oral investigational drug 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 U.S. Food and Drug Administration 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 basis of ITP by impeding platelet destruction. Fostamatinib

potentially offers a compelling addition to the treatment options available for ITP patients.

Conference Call and Webcast Presentation Today at 8:00AM Eastern Time
Rigel will hold a live conference call and webcast today at 8:00am Eastern Time (5:00am Pacific Time). Participants can access the live conference call by dialing 855-892-1489 (domestic) or 720-634-2939 (international) and using the Conference ID number 72149873.

The conference call and accompanying slide presentation will also be webcast live and can be accessed from Rigel's website at www.rigel.com. The webcast will be archived and available for replay after the call via the Rigel website.

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 fostamatinib, an oral spleen tyrosine kinase (SYK) inhibitor, which is in Phase 3 clinical trials for immune thrombocytopenia (ITP); a Phase 2 clinical trial for 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.

This press release contains "forward-looking" statements, including, without limitation, statements related to Rigel's clinical development plans, including the timing, design and nature of planned clinical trials and the timing and nature of results of those trials, as well as the potential activity of fostamatinib with respect to ITP. 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, risks associated with and Rigel's dependence on Rigel's corporate partnerships, as well as other risks detailed from time to time in Rigel's reports filed with the Securities and Exchange Commission, including its Annual Report on Form 10-Q for the year ended June 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.

Contacts:

Raul Rodriguez
Phone: 650.624.1302
Email: invrel@rigel.com

Jessica Daitch
Chandler Chicco Agency

Phone: 917.816.6712
Email: jessica.daitch@inventivhealth.com

Logo - https://photos.prnewswire.com/prnh/20030226/RIGLLOGO

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/rigels-fostamatinib-meets-primary-endpoint-in-phase-3-study-in-chronic-itp-300319730.html

SOURCE Rigel Pharmaceuticals, Inc.