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Fostamatinib Study Results Continue to Trend Positive

Rigel Provides Update in ITP

SOUTH SAN FRANCISCO, Calif., Jan. 30, 2017 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL) today announced updates from the clinical program of fostamatinib in patients with chronic immune thrombocytopenic purpura (ITP).

Previously released results from the FIT Phase 3 clinical studies (047, 048) of fostamatinib in chronic ITP demonstrated that patients who responded to fostamatinib have a timely, robust, and sustained response to treatment. As of September 2016, the open-label, long-term extension study (049), was tracking the experience of 124 patients who opted to receive treatment with fostamatinib after their participation in either Study 047 or Study 048.

Seventeen patients who achieved a stable response to fostamatinib in the parent studies enrolled in Study 049. As of September 2016, responders who enrolled in the 049 study had maintained a median platelet count of 106,500/ μ L over this extended period of time. These patients have been on fostamatinib treatment for a median of 16 months as of September 2016. In addition, there were now 41 out of 44 former placebo patients who had been treated with fostamatinib for a minimum of 12 weeks. Of those, 22% (9/41, $p=0.0078$) achieved a prospectively defined stable platelet response, which Rigel believes further validates fostamatinib as a potential new treatment option for some patients with this serious disease.

Rigel also announced its calculations of an overall response rate for Study 047 and Study 048 by combining stable and transient responders. The overall response rate to fostamatinib is 29% (29/101) compared to 2% (1/49) for placebo ($p<0.0001$). A stable response was defined as a patient achieving platelet counts of $\geq 50,000/\mu$ L on ≥ 4 of the 6 visits between weeks 14 and 24. In the post-study analysis performed by Rigel, a transient response was defined to include patients achieving at least 2 consecutive median platelet counts over 50,000/ μ L during the trial without rescue, but who did not otherwise meet the stable response criteria.

Response Rates for Study 047 and Study 048

Response	Fostamatinib	Placebo	
Stable	18/101	1/49	
Transient	11/101	0/49	
Overall	29/101	1/49	
%; p	29%	2%	$p<0.0001$

"We now have over 16 months of FIT Phase 3 data to analyze and we're very encouraged that chronic ITP patients who respond to fostamatinib are able to maintain a median platelet count of over 100,000 platelets/uL," said Raul Rodriguez, Rigel's president and chief executive officer. "These data continue to support our plan to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for fostamatinib in chronic ITP in the first quarter of this year."

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 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 data of the Phase 3 clinical study with fostamatinib in ITP; 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; Rigel's belief that fostamatinib may be an attractive alternative for patients with ITP; 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 FDA may disagree with our approach of presenting an analysis of combined data from two trials, including one trial that did not meet its primary endpoint, or may interpret our findings differently, which could prevent us from submitting an NDA or result in the FDA not approving any submitted NDA; 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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