

October 20, 2016



## Rigel Announces Results from the Second FIT Phase 3 Study and the Long-Term Open-Label Extension Study for Fostamatinib in ITP

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

SOUTH SAN FRANCISCO, Calif., Oct. 20, 2016 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL) today announced results for the second of two double-blind studies in the FIT Phase 3 clinical program for fostamatinib, an oral spleen tyrosine kinase (SYK) inhibitor, in adult chronic/persistent immune thrombocytopenia (ITP). The primary endpoint in the study was a stable platelet response, defined as platelet counts greater than 50,000/uL of blood on at least four of the last six scheduled clinic visits between weeks 14 and 24 of treatment. In the FIT 2 (Study 048) Phase 3 study, the fostamatinib response rate was 18%, consistent with the recently reported FIT 1 (Study 047) Phase 3 study. In Study 048, one patient in the placebo group (4%) achieved a stable platelet response; therefore the difference between those on treatment and those on placebo did not reach statistical significance ( $p=0.152$ ) and the study did not meet its primary endpoint. When the data from both studies are combined, however, this difference is statistically significant ( $p=0.007$ ). Data from both FIT Phase 3 studies and the open-label extension study demonstrates the consistent benefit of fostamatinib in ITP.

| Stable Platelet Responders / Total Patients |                   |     |                   |     |            |
|---------------------------------------------|-------------------|-----|-------------------|-----|------------|
|                                             | FIT 1 – Study 047 |     | FIT 2 – Study 048 |     | Combined   |
| Fostamatinib                                | 9/51              | 18% | 9/50              | 18% | 18/101 18% |
| Placebo                                     | 0/25              | 0%  | 1/24              | 4%  | 1/49 2%    |
|                                             | $p=0.026$         |     | $p=0.152$         |     | $p=0.007$  |

"We believe that the totality and consistency of data from the FIT Phase 3 program, which included two Phase 3 studies and one long-term extension study, strongly supports a clear treatment effect, with a sustained clinical benefit of fostamatinib," said Raul Rodriguez, president and chief executive officer of Rigel. "We are encouraged by these results and believe that the risk/benefit ratio for fostamatinib is positive for patients with chronic/persistent ITP, a population with a serious unmet medical need. As a result, we will continue to pursue this opportunity. Our next step is to seek feedback from the FDA."

In the combined dataset for Study 047 and Study 048, patients who met the primary

endpoint had their platelet counts increase from a median of 18,500/uL of blood at baseline to more than 100,000/uL at week 24 of treatment. These patients benefited substantially and typically did so within weeks of initiating treatment, providing early feedback as to whether fostamatinib may be a viable option for treating their ITP. In the combined data sets, the frequency of patients who achieved a stable platelet response was statistically superior in the fostamatinib group versus the placebo group in all subgroup analyses: prior splenectomy or not; prior exposure to TPO agents or not; platelet counts below or above 15,000/uL of blood at baseline, demonstrating that the effect of fostamatinib is consistent across various clinical and treatment backgrounds.

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

### **FIT Phase 3 Long-Term Extension Study 049**

Patients from both the 047 and 048 Phase 3 studies were given the option to enroll in a long-term open-label extension study (Study 049) and receive treatment with fostamatinib. As of June 2016, 118 patients had been enrolled in this study. All the patients who responded to fostamatinib in the parent studies enrolled in Study 049 and had a median platelet count of 96,000/uL of blood in this study. These patients have been exposed to fostamatinib for a median of 13 months through the combined parent and 049 trials.

In addition, there were 43 placebo non-responders from the 047 and 048 studies that enrolled in the 049 study. 36 of these patients had at least 12 weeks of follow-up. Of these, 6 patients (17%,  $p=0.01$ ) achieved a prospectively defined stable platelet response in the 049 study.

"Given the heterogeneity of ITP, it is currently almost impossible to predict how patients will respond to available therapies, which is why it is so important for physicians and patients to have treatment options," said James B. Bussel, M.D., professor of pediatrics, pediatrics in obstetrics and gynecology, and pediatrics in medicine at Weill Cornell Medicine, and the principal study investigator on the FIT Phase 3 program. Dr. Bussel is also a member of Rigel's advisory/scientific board. "This heterogeneity means that treatments that work by different mechanisms can make important contributions in certain patients, such as those who might be especially responsive to fostamatinib because of its unique mechanism of action. The FIT Phase 3 studies have both demonstrated that fostamatinib provided a robust and enduring benefit for those patients who responded to the drug."

### **Statement on Financial Position**

Rigel expects to report that it ended the third quarter of 2016 with approximately \$85.3 million in cash, cash equivalents, and short-term investments, which Rigel expects will be sufficient to fund its operations through the end of 2017. In this forecast, Rigel has allocated substantial funds to continue efforts in preparation of the potential commercial launch of fostamatinib in ITP. Rigel is also continuing to evaluate ex-U.S. partnerships for fostamatinib and other partnering opportunities. As a result of the recent research restructuring, Rigel believes that it has created greater flexibility for its cash runway moving forward.

### **About the 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, have failed at least one prior therapy for ITP, and have platelet counts consistently below 30,000/uL of blood. Patients were randomized in a 2:1 ratio to receive either fostamatinib or placebo orally twice a day for up to 24 weeks. The primary efficacy endpoint of this program is a stable platelet response defined as achieving platelet counts greater than 50,000/uL of blood for at least four of the six scheduled clinic visits between weeks 14 and 24 of treatment. Patients were subsequently offered to enroll in an open-label, long-term Phase 3 extension study, which is ongoing.

### **About 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. ITP patients can suffer extraordinary bruising, bleeding and fatigue as a result of low platelet counts. 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.

### **Conference Call and Webcast 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 1-855-892-1489 (domestic) or 1-720-634-2939 (international) and using the Conference ID number 98782744.

The conference call will also be webcast live and can be accessed from Rigel's website at [www.rigel.com](http://www.rigel.com). The webcast will be archived and available for replay after the call via the Rigel website.

### **About Rigel ([www.rigel.com](http://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. 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.

*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, as well as Rigel's cash position as of September 30, 2016 and sufficiency of cash resources. 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, change in Rigel's estimated cash position based on the completion of its financial closing procedures, 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 Quarterly Report on Form 10-Q for the quarter 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.*

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