

September 5, 2013



## **Rigel To Focus On ITP, DLE And Dry Eye**

### **Strategy Provides Multiple Paths to Phase 3/NDA in Next 2-3 Years**

SOUTH SAN FRANCISCO, Calif., Sept. 5, 2013 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq: RIGL) today announced its plans to focus the Company's resources on the completion of three lead clinical programs. These efforts include commencing a Phase 3 clinical study of fostamatinib, an oral SYK inhibitor, in Immune Thrombocytopenic Purpura (ITP) pending discussions with regulatory agencies. Rigel believes that a Phase 3 clinical program would encompass approximately 150 patients and can be completed in 2015. Rigel's other two lead programs, R333, a topical dermatological JAK/SYK inhibitor for discoid lupus erythematosus, and R348, a topical ophthalmic JAK/SYK inhibitor for dry eye, are presently in Phase 2 studies, with results expected in Q4 2013 and Q2 2014, respectively. Rigel expects to advance one of these two molecules into a Phase 3 clinical program by 2014/15. The Company will not continue further development of fostamatinib for the treatment of rheumatoid arthritis or lymphoma due to insufficient efficacy findings from recent clinical trials and the competitive landscape for the agent in those indications.

"Strategically, we have made a decision to concentrate our resources on the programs that we believe hold the greatest potential for a near term path to market," said James M. Gower, chairman and chief executive officer of Rigel. "The size and scope of these clinical programs are such that we can fund and manage them in-house, thereby maintaining control and flexibility over their development."

As a consequence of prioritizing projects and looking to conserve the Company's cash resources, Rigel also announced that it has reduced its workforce by 18%, resulting in the elimination of 30 positions, mostly from the drug discovery area. The Company is still assessing the restructuring and other charges associated with this measure, which are expected to be recorded predominantly in the third quarter of 2013. As of June 30, 2013, the company had \$251 million in cash and equivalents, which Rigel believes is enough to maintain its current development priorities into 2016.

Rigel remains committed to its mission to identify and develop novel small molecule therapeutics and will maintain active programs including: R118, an oral AMPK activator being developed as a potential treatment for intermittent claudication (peripheral artery disease).

#### **Immune Thrombocytopenic Purpura (ITP)**

Chronic ITP affects approximately 100,000 people, with the majority of these cases being in women. ITP is a blood disorder in which the immune system attacks and destroys the body's own blood platelets, which have an important role in the clotting and healing process. ITP patients can suffer bruising, bleeding and fatigue as a result of their low blood platelet counts. Currently marketed therapies aim to raise blood platelet counts, but do not address the etiology of the disorder. The results of Rigel's Phase 2 study, published in *Blood* (volume 113, number 14), showed that fostamatinib may be effective in treating this rare autoimmune disorder.

### **Discoid Lupus Erythematosus (DLE)**

DLE is a chronic autoimmune disease of the skin, which affects approximately 300,000 Americans - with an estimated 16,000 additional new cases diagnosed in the US each year. Disc-shaped sores with inflammation, swelling, scaling, scarring, pigment discoloration, and even hair loss characterize the disease. The lesions most commonly appear in sun-exposed areas, predominantly on the face, chest and scalp. Rigel's R333 is a potent, topical JAK and SYK inhibitor that is designed to interrupt pivotal inflammatory cascade signals with the potential to prevent or diminish both acute and chronic symptoms. Results from a Phase 2 clinical study of R333 in DLE are expected in Q4 2013.

### **Dry Eye**

Dry eye disease is an inflammatory disease of the eye that affects more than 5 million Americans. The disease targets the lacrimal (tear producing) glands of the eye and may be associated with other autoimmune conditions including Sjögren's syndrome, systemic lupus erythematosus and rheumatoid arthritis. Rigel has developed a topical ophthalmic (eye drop) formulation of R348, a JAK and SYK inhibitor, aimed at reducing the inflammation responsible for the symptoms of this disease. Results of a Phase 2 study of R348 in chronic dry eye are expected in Q2 2014.

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

Rigel Pharmaceuticals, Inc. is a clinical-stage drug development company that discovers and develops novel, small-molecule drugs for the treatment of inflammatory and autoimmune diseases, as well as muscle disorders. Rigel's pioneering research focuses on intracellular signaling pathways and related targets that are critical to disease mechanisms. The company currently has five product candidates in clinical development: fostamatinib, an oral SYK inhibitor for ITP; R333, a topical JAK/SYK inhibitor for discoid lupus, and R348, a topical JAK/SYK inhibitor for dry eye – both in Phase 2 clinical trials; and two oncology product candidates in Phase 1 development with partners BerGenBio and Daiichi Sankyo.

*This press release contains "forward-looking" statements, including, without limitation, statements related to development plans, the focus and direction of Rigel's resources, the timing of planned clinical trials and results, Rigel's ability to fund and manage its clinical programs in-house, Rigel's plans with respect to a reduction in force and restructuring charges associated with those reductions in force, and Rigel's ability to fund and maintain its current development plans into 2016. 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, our need for additional capital in the future to sufficiently fund our 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, 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, 2013. 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.*

Contact: Raul Rodriguez

Phone: 650.624.1302

Email: [invrel@rigel.com](mailto:invrel@rigel.com)

Media Contact: Susan C. Rogers, Alchemy Consulting, Inc.

Phone: 650.430.3777

Email: [susan@alchemyemail.com](mailto:susan@alchemyemail.com)

SOURCE Rigel Pharmaceuticals, Inc.