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Fostamatinib Meets Pre-Specified Primary Endpoint in Stage 1 of Autoimmune Hemolytic Anemia (AIHA) Phase 2 Study

Results Merit Initiation of Stage 2 of the Study

SOUTH SAN FRANCISCO, Calif., Oct. 3, 2017 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL) today announced that the company recently completed enrollment of Stage 1 of its Phase 2, open-label, multi-center, two-stage study of its investigational drug fostamatinib for the treatment of patients with warm antibody AIHA. On a top-line, preliminary basis, the Phase 2 study has achieved the pre-specified primary efficacy endpoint for Stage 1. A response was defined as achieving a hemoglobin level of greater than 10 g/dl and at least a 2 g/dl increase from baseline.

This Phase 2 study, also known as the SOAR study, is evaluating the safety and efficacy of fostamatinib in patients with warm antibody AIHA who have previously received at least one treatment for this disease, but did not have a meaningful benefit and are still anemic. The SOAR study utilizes an open-label, Simon two-stage design to evaluate fostamatinib at 150 mg BID (twice daily) in patients with warm antibody AIHA, a disease for which there is no available treatment.

Stage 1 of the SOAR study has enrolled 17 patients who have had at least one post-baseline hemoglobin measure. Of the 17 patients, 4 responded during the 12-week evaluation period and an additional 2 patients met the response criteria in the extension study after 12 weeks of dosing, for a response rate of 35% (6/17) on fostamatinib (these data are preliminary and require further verification). During the trial, 2 of the 17 patients withdrew early from the study due to non-safety-related reasons and will be replaced per the study protocol. A comprehensive analysis of the Phase 2 data will continue and will be presented at a future scientific conference.

The safety profile was consistent with the existing fostamatinib safety database, which comprises over 5,000 patient-years of exposure. Two deaths were reported during the trial due to non-treatment related serious adverse events (SAEs) as determined by the investigators (one patient with skin necrosis and infection, and an elderly patient with pneumonia who was immunosuppressed due to prior CLL and steroids). A third patient experienced a non-treatment related SAE as determined by the investigator, recovered and

continued on treatment. Having met the Stage 1 primary efficacy endpoint, Rigel intends to begin enrollment for Stage 2 of this study in which 20 patients will be enrolled under the same protocol.

"Many patients with AIHA suffer from severe, debilitating disease that negatively affects their quality of life," said David J. Kuter, M.D., the director for the Center of Hematology at Massachusetts General Hospital and the lead investigator of the SOAR study. "There are no FDA-approved medications for the treatment of AIHA, which means that those living with the condition are in need of new and effective therapeutic options."

About AIHA

Autoimmune hemolytic anemia (AIHA) is a rare, serious blood disorder where the immune system produces antibodies that result in the destruction of the body's own red blood cells. AIHA affects approximately 40,000 adult patients in the US and can be a severe, debilitating anemia. To date, there are no disease-targeted therapies for AIHA, despite the tremendous medical need that exists for these patients.

About Rigel (www.rigel.com)

Rigel Pharmaceuticals, Inc. is a biotechnology company dedicated to discovering, developing and providing novel small molecule drugs that significantly improve the lives of patients with immune and hematological disorders, cancer and rare diseases. 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. Rigel has submitted and the FDA has accepted for review, an NDA for fostamatinib in patients with chronic or persistent immune thrombocytopenia (ITP). In addition, Rigel has product candidates in development with partners BerGenBio AS, Daiichi Sankyo and Aclaris Therapeutics.

Forward Looking Statements

This release contains forward-looking statements relating to, among other things, Rigel's belief that fostamatinib may benefit patients with AIHA and the timing and nature of results of Rigel's clinical trials. 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," "hope" 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 top-line data Rigel has reported is based on preliminary analysis of key efficacy and safety data, and such data may change following a more comprehensive review of the data related to the clinical trial and such top-line data may not accurately reflect the complete results of the trial, and the FDA may interpret Rigel's findings differently, which could 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 studies; 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, 2017. 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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