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TAVALISSE™ (fostamatinib disodium hexahydrate) Phase 3 Data Published in the American Journal of Hematology Describes Pivotal Data and Overall Response Rate Versus Placebo

SOUTH SAN FRANCISCO, Calif., April 30, 2018 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL) today announced that the *American Journal of Hematology* has published positive results from the Fostamatinib in Thrombocytopenia (FIT) Phase 3 clinical program of TAVALISSE™ (fostamatinib disodium hexahydrate) for the treatment of adults with chronic immune thrombocytopenia (ITP). The study, "Fostamatinib for the Treatment of Adult Persistent and Chronic Immune Thrombocytopenia: Results of Two Phase 3, Randomized, Placebo-Controlled Trials," is available on the journal website.

On April 17, 2018, the FDA approved TAVALISSE™ for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment.

"These data demonstrate that TAVALISSE offers the potential for a rapid, robust and durable platelet response, which is why we are excited to soon make it available to the population of patients in need of alternate treatment options," said Anne-Marie Duliege, M.D., Chief Medical Officer of Rigel Pharmaceuticals, Inc. "TAVALISSE is the first approved treatment option that targets the SYK pathway, which is the main pathway involved in platelet destruction in ITP."

To view the TAVALISSE™ approval press release and multimedia assets, please visit: https://www.multivu.com/players/English/8297951-rigel-pharmaceuticals-itp-tavalisse-fda-approval/

About FIT-1 and FIT-2
FIT-1 and FIT-2 were randomized, double-blind, placebo-controlled phase 3 trials evaluating TAVALISSE, an oral spleen tyrosine kinase (SYK) inhibitor, in comparison with placebo in a total of 150 adult patients with persistent or (predominantly) chronic ITP. The studies were designed in accordance with FDA guidance and the efficacy endpoints were based on an objective laboratory assessment of platelet count. Patients who completed the 24-week

study treatment in either FIT-1 or FIT-2 could enroll in the long-term, open-label extension study (FIT-3); non-responders who discontinued the study after 12 weeks for lack of efficacy and had received 150 mg BID of study drug for ≥4 weeks could also enroll in FIT-3. These phase 3 studies were the first to evaluate second- or third-line treatment for ITP in the current era of widespread use of TPO-RA and rituximab.

TAVALISSE targets the underlying autoimmune cause of the disease by impeding platelet destruction, providing an important new treatment option for adult patients with chronic ITP.

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 an increased risk of severe bleeding events that can result in serious medical complications or even death. In addition to TAVALISSE, current therapies for ITP include steroids, blood platelet production boosters (TPOs) and splenectomy. However, not all patients are adequately treated with existing therapies. As a result, there remains a significant medical need for additional treatment options for patients with ITP.

About TAVALISSE

Indication

TAVALISSE™ (fostamatinib disodium hexahydrate) tablets is indicated for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment.

Important Safety Information

Warnings and Precautions

- Hypertension can occur with TAVALISSE treatment. Patients with pre-existing hypertension may be more susceptible to the hypertensive effects. Monitor blood pressure every 2 weeks until stable, then monthly, and adjust or initiate antihypertensive therapy for blood pressure control maintenance during therapy. If increased blood pressure persists, TAVALISSE interruption, reduction, or discontinuation may be required.
- Elevated liver function tests (LFTs), mainly ALT and AST, can occur with TAVALISSE. Monitor LFTs monthly during treatment. If ALT or AST increase to >3 x upper limit of normal, manage hepatotoxicity using TAVALISSE interruption, reduction, or discontinuation.
- Diarrhea occurred in 31% of patients and severe diarrhea occurred in 1% of patients treated with TAVALISSE. Monitor patients for the development of diarrhea and manage using supportive care measures early after the onset of symptoms. If diarrhea becomes severe (≥Grade 3), interrupt, reduce dose or discontinue TAVALISSE.
- Neutropenia occurred in 6% of patients treated with TAVALISSE; febrile neutropenia occurred in 1% of patients. Monitor the ANC monthly and for infection during treatment. Manage toxicity with TAVALISSE interruption, reduction, or discontinuation.
- TAVALISSE can cause fetal harm when administered to pregnant women. Advise pregnant women the potential risk to a fetus. Advise females of reproductive potential to use effective contraception during treatment and for at least 1 month after the last dose. Verify pregnancy status prior to initiating TAVALISSE. It is unknown if TAVALISSE or its metabolite is present in human milk. Because of the potential for

serious adverse reactions in a breastfed child, advise a lactating woman not to breastfeed during TAVALISSE treatment and for at least 1 month after the last dose.

Drug Interactions

- Concomitant use of TAVALISSE with strong CYP3A4 inhibitors increases exposure to the major active metabolite of TAVALISSE (R406), which may increase the risk of adverse reactions. Monitor for toxicities that may require a reduction in TAVALISSE dose.
- It is not recommended to use TAVALISSE with strong CYP3A4 inducers, as concomitant use reduces exposure to R406.
- Concomitant use of TAVALISSE may increase concentrations of some CYP3A4 substrate drugs and may require a dose reduction of the CYP3A4 substrate drug.
- Concomitant use of TAVALISSE may increase concentrations of BCRP substrate drugs (eg, rosuvastatin) and P-Glycoprotein (P-gp) substrate drugs (eg, digoxin), which may require a dose reduction of the BCRP and P-gp substrate drug.

Adverse Reactions

- Serious adverse drug reactions in the ITP double-blind studies were febrile neutropenia, diarrhea, pneumonia, and hypertensive crisis, which occurred in 1% of TAVALISSE patients. In addition, severe adverse reactions occurred including dyspnea and hypertension (both 2%), neutropenia, arthralgia, chest pain, diarrhea, dizziness, nephrolithiasis, pain in extremity, toothache, syncope, and hypoxia (all 1%).
- Common adverse reactions (≥5% and more common than placebo) from FIT-1 and FIT-2 included: diarrhea, hypertension, nausea, dizziness, ALT and AST increased, respiratory infection, rash, abdominal pain, fatigue, chest pain, and neutropenia.

Please see www.TAVALISSE.com for full Prescribing Information.

**To report side effects of prescription drugs to the FDA,
visit www.fda.gov/medwatch or call 1-800-FDA-1088 (800-332-1088).**

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 hematologic disorders, cancer and rare diseases. Rigel's pioneering research focuses on signaling pathways that are critical to disease mechanisms. The company's first FDA approved product is TAVALISSE™ (fostamatinib disodium hexahydrate), an oral spleen tyrosine kinase (SYK) inhibitor, for the treatment of adult patients with chronic immune thrombocytopenia who have had an insufficient response to a previous treatment. Rigel's current clinical programs include Phase 2 studies of fostamatinib in autoimmune hemolytic anemia and IgA nephropathy. 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, the

benefits and value to patients of TAVALISSE and Rigel's belief that TAVALISSE may be an important alternative for patients with 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," "should," "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, risks and uncertainties associated with the commercialization of TAVALISSE; risks that the FDA or other regulatory authorities may make adverse decisions regarding TAVALISSE; risks that TAVALISSE clinical trials may not be predictive of real-world results or of results in subsequent clinical trials; risks that TAVALISSE may have unintended side effects, adverse reactions or incidents of misuses; the availability of resources to develop Rigel's product candidates; market competition; 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-K for the period ended December 31, 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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