

May 1, 2018



Rigel Announces First Quarter 2018 Financial Results and Provides Company Update

Rigel Expected to Launch TAVALISSE™ (fostamatinib disodium hexahydrate) in the U.S. in late May 2018

Conference Call and Webcast Today at 5:00PM Eastern Time

SOUTH SAN FRANCISCO, Calif., May 1, 2018 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL), today reported financial results for the first quarter ended March 31, 2018.

TAVALISSE Launch Update

During the conference call today, Rigel will discuss the TAVALISSE market access plans, including the wholesale acquisition cost of TAVALISSE, and provide additional detail regarding RIGEL ONECARE™, its patient and practice support center featuring a suite of services.

Recent Achievements

- On April 30th, Rigel announced that the *American Journal of Hematology* published positive results from the Fostamatinib in Thrombocytopenia (FIT) Phase 3 clinical program. 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 24, Rigel completed an underwritten public offering of common stock with proceeds of approximately \$58.4 million, net of underwriting discounts and commissions and other estimated offering expenses.
- On April 17, Rigel announced the FDA approval of TAVALISSE™ (fostamatinib disodium hexahydrate) for the treatment of thrombocytopenia in adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment. TAVALISSE is an oral spleen tyrosine kinase (SYK) inhibitor that targets the underlying autoimmune cause of the disease by impeding platelet destruction, providing an important new treatment option for adult patients with chronic ITP.
- On April 3, the company announced topline data from its proof-of-concept Phase 2

study of fostamatinib in patients with IgA nephropathy (IgAN), an orphan autoimmune disease of the kidneys.

- On March 8, Rigel presented data from Stage 1 of the Phase 2 study of fostamatinib in patients with warm antibody autoimmune hemolytic anemia (AIHA) at the Thrombosis and Hemostasis Societies of North America.

"The FDA's approval of TAVALISSE for chronic ITP represents a huge milestone for Rigel and allows us to advance our efforts to transition the company into commercialization, so that we may bring this therapy to a patient population in need of treatment options," stated Raul Rodriguez, president and CEO of Rigel. "In just a few weeks, TAVALISSE will be available as the first therapy that targets the mechanism for platelet destruction in ITP. We believe that it provides physicians and patients with a new treatment option with the potential for a rapid, robust and durable response, along with the convenience of oral dosing and a manageable safety profile."

For the first quarter of 2018, Rigel reported a net loss of \$24.4 million, or \$0.17 per share, compared to a net loss of \$15.3 million, or \$0.13 per share, in the first quarter of 2017.

There were no contract revenues from collaborations in the first quarter of 2018. Contract revenues from collaborations of \$3.6 million in the first quarter of 2017 was comprised primarily of the \$3.3 million payment from BerGenBio AS as a result of advancing BGB324, a selective, potent and orally available small molecule AXL kinase inhibitor, to a Phase 2 clinical study.

Rigel reported total costs and expenses of \$24.7 million in the first quarter of 2018, compared to \$19.8 million in the first quarter of 2017. The increase in costs and expenses was primarily due to the increases in personnel costs, as well as costs in preparation for the commercial launch of TAVALISSE in chronic ITP.

As of March 31, 2018, Rigel had cash, cash equivalents and short-term investments of \$94.3 million, compared to \$115.8 million as of December 31, 2017. In April 2018, Rigel completed an underwritten public offering in which it received proceeds of approximately \$58.4 million, net of underwriting discounts and commissions and estimated offering expenses. Rigel expects that its cash, cash equivalents and short-term investments will be sufficient to support its current and projected funding requirements, including the launch of fostamatinib for chronic ITP in the U.S., through at least the next 12 months.

Corporate Update

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

Rigel anticipates that TAVALISSE will be available to patients in the U.S. beginning in late May 2018. In the interim, Rigel continues to execute on its commercial readiness plan to support this proposed launch, including establishing distribution channels with external partners, developing the systems needed to provide medication access, and hiring all key personnel. Already, sales force recruitment is well underway to support launch activities. The commercial team, including Business Operations, Market Access, Marketing, and Sales, has extensive experience in rare diseases, hematology and oncology as well as launch experience in both small and large companies. In addition, a highly experienced Medical Science Liaison team is available to support medical inquiries from health care practitioners

about TAVALISSE.

Portfolio Update

Fostamatinib in Autoimmune Hemolytic Anemia (AIHA)

Rigel is evaluating the safety and efficacy of fostamatinib in patients with warm antibody AIHA. On January 31, 2018, the FDA granted Orphan Drug designation to fostamatinib for the treatment of patients with AIHA.

The Phase 2, open-label, multi-center, Simon two-stage study completed enrollment of Stage 1 in 2017. A clinical response in this trial was defined as achieving a hemoglobin level of greater than 10 g/dl and at least a 2 g/dl increase from baseline. In February 2018, an additional patient in the Stage 1 extension study met the response criteria bringing the total to 9 of 17 (53%) evaluable patients achieving a response to fostamatinib treatment. Six patients achieved a response during the 12-week evaluation period, and an additional three patients met the response criteria in the extension study after 12 weeks of dosing. 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. A third patient experienced a non-treatment related SAE as determined by the investigator, recovered and continued on treatment.

Stage 2 enrollment of the Phase 2 study commenced in late 2017. Stage 2 follows the same protocol as Stage 1 and will include 20 patients. Rigel plans to meet with the FDA to determine the regulatory development pathway of fostamatinib in AIHA.

Fostamatinib in IgA Nephropathy (IgAN)

In April 2018, Rigel announced topline data from its proof-of-concept Phase 2 study of fostamatinib in patients with IgAN, an orphan autoimmune disease of the kidneys. The trial did not achieve statistical significance for its primary endpoint, which was mean change in proteinuria comparing fostamatinib dose groups to placebo controls in all patients studied. However, in a pre-specified subgroup analysis of patients with greater than 1 gram/day of proteinuria at baseline, the initial data showed a greater reduction in proteinuria in fostamatinib-treated patients relative to placebo patients (this finding did not reach statistical significance). Further analysis, including histology, are expected later in the year. Fostamatinib was well tolerated with mostly mild to moderate adverse events, and there were no new safety signals compared to the fostamatinib's safety database across all indications. The most frequent adverse events were diarrhea, nausea, headache, hypertension and vomiting. One patient had a fatal SAE, which was not drug related.

Rigel plans to seek a pharmaceutical partner to collaborate in the conduct of follow-on clinical studies in IgAN. This partner would take responsibility for the subsequent commercialization of fostamatinib if in an ex-U.S. territory.

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. 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 AIHA

Autoimmune hemolytic anemia (AIHA) is a rare, serious blood disorder in which 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 disease. To date, there are no disease-targeted therapies approved for AIHA, despite the tremendous medical need that exists for these patients.

About IgAN

IgA nephropathy (IgAN) (also known as Berger's disease) is a chronic autoimmune disease associated with inflammation in the kidneys that diminishes their ability to filter blood. It is the most common primary glomerular disease, affecting an estimated 82,500 - 165,000 patients in the US, with a higher prevalence in Asia. For as many as 25% of those living with IgAN, the disease results in end-stage renal failure requiring dialysis or kidney transplantation. There are no disease-targeted therapies approved for IgAN. Proteinuria is a sign and predictor of the severity of IgA nephropathy. Pre-clinical data show that fostamatinib decreases spleen tyrosine kinase (SYK) activation in the kidney, potentially reversing the inflammation in the glomeruli and improving kidney function.

Conference Call and Webcast Today at 5:00PM Eastern Time

Rigel will hold a live conference call and webcast today at 5:00pm Eastern Time (2:00pm Pacific Time).

Participants can access the live conference call by dialing 855-892-1489 (domestic) or 720-634-2939 (international) and using the Conference ID number 3691625. The webcast, with slide presentation, can be accessed from Rigel's website at www.rigel.com. The webcast will be archived and available for replay after the call via the Rigel website.

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 ($\geq$ 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 ($\geq$ 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).

Trademarks for TAVALISSE are owned by or licensed by Rigel.

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 timing and success of the U.S. commercial launch of TAVALISSE; the availability of TAVALISSE to patients; the benefits and value to patients of TAVALISSE; Rigel's ability to transition to an organization prepared to launch its first commercial product, including efforts to establish distribution channels with external partners, develop systems needed to provide medication access, and hiring key personnel; Rigel's belief that TAVALISSE may be an important alternative for patients with ITP; Rigel's ability to identify partners for commercialization of fostamatinib in ex-U.S. territories; Rigel's interactions with the FDA; and the timing and 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," "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.

Contact: Raul Rodriguez

Phone: 650.624.1302

Email: ir@rigel.com

Media Contact: Jessica Daitch

Phone: 917.816.6712

Email: jessica.daitch@syneoshealth.com



RIGEL PHARMACEUTICALS, INC.
STATEMENTS OF OPERATIONS
(in thousands, except per share amounts)

	Three Months Ended March 31, 2018	2017
	(unaudited)	
Revenues:		
Contract revenues from collaborations	\$ -	\$ 3,584
Costs and expenses:		
Research and development (see Note A)	11,242	12,376
General and administrative (see Note A)	13,492	7,410
Total costs and expenses	24,734	19,786
Loss from operations	(24,734)	(16,202)
Interest income	349	156
Gain on disposal of assets	-	732
Net loss	\$ (24,385)	\$ (15,314)
Net loss per share, basic and diluted	\$ (0.17)	\$ (0.13)
Weighted-average shares used in computing net loss per share, basic and diluted	147,114	113,598

Note A

Stock-based compensation expense included in:		
General and administrative	\$ 940	\$ 595
Research and development	600	360
	\$ 1,540	\$ 955

SUMMARY BALANCE SHEET DATA
(in thousands)

	March 31, 2018	December 31, 2017 ⁽¹⁾
	(unaudited)	
Cash, cash equivalents and short-term investments	\$ 94,300	\$ 115,751
Total assets	98,813	119,111
Stockholders' equity	79,807	100,646

(1) Derived from audited financial statements

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