August 4, 2020

Rigel Reports Second Quarter 2020 Financial Results, Provides Business Update, and Overviews COVID-19 Program

Second quarter net product sales of $15.0 million, a year-over-year increase of 47%

University of Amsterdam in vitro studies showed fostamatinib blocked macrophage hyper-inflammatory response to serum from severe COVID-19 patients

Appointed 30-year industry veteran David Santos as Chief Commercial Officer

Conference call and webcast today at 4:30PM Eastern Time

SOUTH SAN FRANCISCO, Calif., Aug. 4, 2020 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq: RIGL) today reported financial results for the second quarter ended June 30, 2020, including sales of TAVALISSE® (fostamatinib disodium hexahydrate) tablets, for the treatment of adults with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment.

"During the second quarter we achieved significant progress across all aspects of our business, despite the challenges resulting from the COVID-19 pandemic," said Raul Rodriguez, Rigel's president and CEO. "Sales of TAVALISSE increased during the quarter and in July, the product became available in initial European markets. We continued to advance our pipeline, most recently with the launch of an investigator-sponsored trial in COVID-19 pneumonia. We believe there is clear scientific rationale to explore the potential of SYK-inhibition to treat these patients and possibly prevent severe respiratory conditions resulting from COVID-19. We will continue to pursue opportunities to expand these efforts."

COVID-19 Program Highlights

Rigel announced a Phase 2 investigator-sponsored trial (IST) with Imperial College London to evaluate the efficacy of fostamatinib for the treatment of COVID-19 pneumonia. The IST is a two-stage, open label, controlled clinical trial with patients randomized (1:1:1) to

fostamatinib, ruxolitinib, or standard of care. Treatment will be administered twice daily for 14 days and patients will receive a follow-up assessment at day 14 and day 28 after the first dose. The primary objective will be to determine the efficacy of fostamatinib and the efficacy of ruxolitinib compared to standard of care to reduce the proportion of hospitalized patients progressing from mild or moderate to severe COVID-19 pneumonia. Rigel will provide support for this trial along with Novartis.

Recent in vitro studies led by the Amsterdam University Medical Center at the University of Amsterdam, showed that R406, the active metabolite of fostamatinib, blocked macrophage hyperinflammatory responses to a combination of immune complexes formed by anti-Spike IgG in serum from severe COVID-19 patients. Anti-Spike IgG levels are known to correlate with the severity of COVID-19. These results suggest that by inhibiting anti-Spike IgG-mediated hyperinflammation, R406 could potentially play a role in the prevention of cytokine storms as well as pulmonary edema and thrombosis associated with severe COVID-19.[1]

In addition, researchers at The Broad Institute of the Massachusetts Institute of Technology (MIT) and Harvard led a recent screen to identify FDA-approved compounds that reduce mucin-1 (MUC1) protein abundance. MUC1 is a biomarker used to predict the development of ALI and ARDS and correlates with poor clinical outcomes. Of the 3,713 compounds that were screened, fostamatinib was the only compound identified which both decreased expression of MUC1 and is FDA approved. Fostamatinib demonstrated preferential depletion of MUC1 from epithelial cells without affecting cell viability. The research was focused on drug repurposing for the much lower risk of toxicity and the ability of FDA-approved treatments to be delivered on a shortened timescale, which is critical for patients afflicted with lung disease resulting from COVID-19.[2]

Business Update

Post-hoc data analysis from Rigel's previous Phase 3 clinical program of TAVALISSE in adult patients with chronic ITP, which highlights the potential benefit of use in earlier lines of therapy, was published in the *British Journal of Haematology.* Inclusion in one of the leading peer-reviewed journals in the field of hematology underscores the significance of the 78% (25/32) response rate, defined as at least 1 platelet count of at least 50,000/μL, when TAVALISSE was used as a second-line therapy in Rigel's Phase 3 clinical program. Adverse events were manageable and consistent with those previously reported with fostamatinib. Rigel's field teams are sharing this analysis with physicians.

Grifols S.A., Rigel's collaborator in Europe, launched fostamatinib disodium hexahydrate in Germany and the United Kingdom in July, under the European brand name TAVLESSE. TAVLESSE is indicated in Europe for the treatment of chronic ITP in adult patients who are refractory to other treatments.

Clinical trial sites in Rigel's FORWARD study, a Phase 3 pivotal trial of TAVALISSE in warm autoimmune hemolytic anemia (wAIHA), are now mostly open and enrolling patients after a temporary postponement due to the COVID-19 pandemic. Currently, the FORWARD study has enrolled 44 of the 90 patients targeted for enrollment and has over 90 active clinical trial sites established across 22 countries with incremental sites being added.

At the European League Against Rheumatism (EULAR) 2020 E-Congress in June, Rigel presented two oral and two poster presentations highlighting its investigational compound

R835, a potent and selective inhibitor of both interleukin receptor associated kinase (IRAK)1 and IRAK4. In multiple pre-clinical rodent models of acute and chronic inflammation, R835 administration resulted in reduced inflammation, and in Phase 1 human studies it showed encouraging pharmacokinetic properties.

Rigel has appointed Dave Santos as executive vice president and chief commercial officer. Mr. Santos joins Rigel with over 30 years of commercial experience in the biopharmaceutical industry with companies such as Bristol-Myers Squibb, Lilly, Genentech, and most recently Jazz Pharmaceuticals, where he led the Hematology/Oncology Business Unit. He has a robust track record in sales and marketing leadership roles, building commercial capabilities, and growing brands in the hematology-oncology area, where he has spent most of his career.

Financial Update

For the second quarter of 2020, Rigel reported a net loss of $17.6 million, or $0.10 per share, compared to a net loss of $20.6 million, $0.12 per share, in the same period of 2019.

In the second quarter of 2020, total revenues were $16.0 million, consisting of $15.0 million in net product sales and $1.0 million in contract revenues from collaborations. Net product sales increased by 47% from $10.2 million in the second quarter of 2019.

Rigel reported total costs and expenses of $33.4 million in the second quarter of 2020, compared to $31.7 million for the same period in 2019. The increase in total costs and expenses was primarily due to the increase in third-party costs related to Rigel's ongoing pivotal Phase 3 study in warm AIHA, research and development costs related to other clinical programs and personnel-related costs, partially offset by stock-based compensation expense.

For the six months ended June 30, 2020, Rigel reported net income of $3.7 million, or $0.02 per share, compared to a net loss of $38.2 million, or $0.23 per share, in the same period of 2019.

Rigel reported total revenues of $71.8 million for the six months ended June 30, 2020, compared to $23.0 million for the same period in 2019. Total revenues for the six months ended June 30, 2020 consisted of $27.7 million in net product sales and $44.1 million in revenues related to Rigel's collaboration agreement with Grifols. Total revenues for the six months ended June 30, 2019 consisted of $18.2 million in net product sales and $4.8 million in revenues related to Rigel's collaboration agreements with Grifols and Kissei.

Total costs and expenses for the six months ended June 30, 2020 were $68.1 million, compared to $62.7 million, for the same period of 2019. The increase in total costs and expenses was primarily related to the research and development cost for its ongoing pivotal Phase 3 study in warm AIHA and research and development costs related to other clinical programs, partially offset by stock-based compensation expense.

As of June 30, 2020, Rigel had cash, cash equivalents and short-term investments of $92.5 million, compared to $98.1 million as of December 31, 2019.

Conference Call and Webcast with Slides Today at 4:30pm Eastern Time

Rigel will hold a live conference call and webcast today at 4:30pm Eastern Time (1:30pm

Pacific Time).

Participants can access the live conference call by dialing (877) 407-3088 (domestic) or (201) 389-0927 (international). The conference call and accompanying slides will also be webcast live and can be accessed from the Investor Relations section of the company's website at www.rigel.com. The webcast will be archived and available for replay after the call via the Rigel website.

About ITP

In patients with ITP (immune thrombocytopenia), 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 include fatigue, 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 fostamatinib, current therapies for ITP include steroids, blood platelet production boosters (TPO-RAs) and splenectomy. However, not all patients respond to 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 45,000 adult patients in the U.S. and can be a severe, debilitating disease. Warm AIHA (wAIHA), the most common form of AIHA, is characterized by the presence of antibodies that react with the red blood cell surface at body temperature. To date, there are no disease-targeted therapies approved for AIHA, despite the unmet medical need that exists for these patients.

About Coronavirus Disease 2019 (COVID-19) & SYK-Signaling

COVID-19 is caused by Severe Acute Respiratory Syndrome Coronavirus-2 (SARS-CoV-2). SARS-CoV-2 primarily infects the upper and lower respiratory tract and can lead to acute respiratory distress syndrome (ARDS). Additionally, some patients develop other organ dysfunction including myocardial injury, acute kidney injury, shock along with endothelial dysfunction and subsequently micro and macrovascular thrombosis.[3] Much of the underlying pathology of SARS-CoV-2 is thought to be secondary to a dysregulated immune response and more recently a hypercoagulable state leading to immunothrombosis.[4]

Spleen tyrosine kinase (SYK) is involved in the intracellular signaling pathways of many different immune cells. SYK inhibition may improve outcomes in patients with COVID-19 by biological mechanisms which include the inhibition of pro-inflammatory cytokines by monocytes and macrophages, decreased production of neutrophil extracellular traps (NETs) by neutrophils, and inhibition of platelet aggregation; three pathways that are mediated through Fc receptors (FcR) recognition of antigen-antibody complexes or activation of c-type lectin receptors (CLEC).[1] In monocytes and macrophages, SYK mediates the pro-inflammatory process through the production of pro-inflammatory cytokines. The production of pro-inflammatory cytokines has been reversed with the inhibition of SYK in MERS-CoV infected macrophages.[5]

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, visitwww.fda.gov/medwatch or call 1-800-FDA-1088 (800-332-1088).

TAVALISSE and TAVLESSE are registered trademarks of Rigel Pharmaceuticals, Inc.

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) tablets, the only 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. The product has been approved by the European Commission for the treatment of chronic immune thrombocytopenia in adult patients who are refractory to other treatments and is marketed in Europe under the name TAVLESSE® (fostamatinib). Fostamatinib is currently being studied in an investigator-sponsored trial conducted by Imperial College London for the treatment of COVID-19 pneumonia[6].

Rigel's clinical programs include a Phase 3 study of fostamatinib in warm autoimmune hemolytic anemia (AIHA); a completed Phase 1 study of R835[6], a proprietary molecule from its interleukin receptor associated kinase (IRAK) inhibitor program; and an ongoing Phase 1 study of R552[6], a proprietary molecule from its receptor-interacting protein kinase (RIP) inhibitor program. In addition, Rigel has product candidates in clinical development with partners Aclaris Therapeutics, AstraZeneca, BerGenBio ASA, and Daiichi Sankyo.

1. Hoepel W. et al. Anti-SARS-CoV-2 IgG from severely ill COVID-19 patients promotes macrophage hyper-inflammatory responses. bioRxiv July 13, 2020. doi: https://doi.org/10.1101/2020.07.13.190140
2. Alimova M. et al. A High Content Screen for Mucin-1-Reducing Compounds Identifies Fostamatinib as a Candidate for Rapid Repurposing for Acute Lung Injury during the COVID-19 pandemic. bioRxiv June 30, 2020. doi: https://doi.org/10.1101/2020.06.30.180380
3. Berlin DA, Gulick RM, Martinez FJ. Severe Covid-19. N Engl J Med 2020
4. A.V. Rapkiewicz et al. Map-ftcm-otaaiC-Acs. EClinicalMedicine (2020). doi: https://doi.org/10.1016/j.eclinm.2020.100434
5. Zhao X, Chu H, Wong BH, et al. Activation of C-Type Lectin Receptor and (RIG)-I-Like Receptors Contributes to Proinflammatory Response in Middle East Respiratory Syndrome Coronavirus-Infected Macrophages. J Infect Dis 2020;221:647-59
6. *The product for this use or indication is investigational and has not been proven safe or effective by any regulatory authority.*

Forward Looking Statements

This release contains forward-looking statements relating to, among other things, the commercial success of TAVALISSE in the U.S.; the sufficiency of Rigel's supplies of TAVALISSE; the commercialization of TAVLESSE in Europe and the timing thereof; the utility of fostamatinib in warm autoimmune hemolytic anemia (AIHA); the impact of the COVID-19 pandemic on Rigel's results and operations; Rigel's ability to complete enrollment in its phase 3 clinical trial for AIHA and the timing thereof; Rigel's ability to further develop its clinical stage products; the scientific rationale for exploring use of fostamatinib to treat COVID-19 and related conditions; Rigel's plans to support Imperial College London's IST; the potential clinical benefit of fostamatinib in COVID-19 patients and the prevention of ARDS; role of SYK inhibition in potentially improving outcomes in COVID-19 patients; and Rigel's partnering efforts. 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 "potential," "may," "expects" 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 and marketing of TAVALISSE; risks that the FDA, EMA or other regulatory authorities may make adverse decisions regarding fostamatinib; 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 year ended December 31, 2019 and Quarterly Report on Form 10-Q for the quarter ended March 31, 2020. In addition, the COVID-19 pandemic may result in further delays in Rigel's studies, trials and sales, or impact Rigel's ability to obtain supply of TAVALISSE. 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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RIGEL PHARMACEUTICALS, INC.
STATEMENTS OF OPERATIONS
(in thousands, except per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
	(unaudited)		(unaudited)	
Revenues:				
Product sales, net	$ 14,974	$ 10,173	$ 27,654	$ 18,227
Contract revenues from collaborations	1,047	234	44,128	4,804
Total revenues	16,021	10,407	71,782	23,031
Costs and expenses:				
Cost of product sales	279	311	434	418
Research and development (see Note A)	14,214	13,226	30,363	24,175
Selling, general and administrative (see Note A)	18,920	18,209	37,350	38,155
Total costs and expenses	33,413	31,746	68,147	62,748
Loss from operations	(17,392)	(21,339)	3,635	(39,717)
Interest income	169	733	527	1,513
Interest expense	(353)	—	(495)	—
Net income (loss)	$ (17,576)	$ (20,606)	$ 3,667	$ (38,204)
Net income (loss) per share, basic and diluted	$ (0.10)	$ (0.12)	$ 0.02	$ (0.23)
Weighted-average shares used in computing net income (loss) per share				
Basic	168,570	167,191	168,519	167,182
Diluted	168,570	167,191	168,525	167,182

Note A

Stock-based compensation expense included in:				
Selling, general and administrative	$ 1,299	$ 1,742	$ 2,629	$ 3,908
Research and development	458	911	1,152	1,698
	$ 1,757	$ 2,653	$ 3,781	$ 5,606

SUMMARY BALANCE SHEET DATA
(in thousands)

	June 30, 2020	December 31, 2019 (1)
	(unaudited)	
Cash, cash equivalents and short-term investments	$ 92,497	$ 98,078
Total assets	138,035	147,569
Stockholders' equity	63,210	53,815

(1) Derived from audited financial statements

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