April 3, 2018

Rigel Announces Topline Data from Proof-of-Concept Phase 2 Study of Fostamatinib in IgA Nephropathy

Company to host a conference call today at 8:00AM EDT to Discuss the Study Results

SOUTH SAN FRANCISCO, Calif., April 3, 2018 /PRNewswire/ -- Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL), today 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. 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). Patients with greater than 1 gram/day of proteinuria have an increased risk of disease progression and represent an unmet medical need. Current guidance for clinical trials in IgAN recommends studying patients with greater than 1 gram/day of proteinuria at entry. Further analysis, including histology, are expected later in the year.

"We find the subgroup analysis encouraging because patients and physicians have been challenged to manage this serious disease that has no approved treatment options," stated Raul Rodriguez, president and CEO of Rigel. "This study has provided valuable information on the potential benefit of fostamatinib in IgA nephropathy patients with significant need, those with greater than 1 gram/day of proteinuria. We will continue to evaluate the data to determine the best path forward in this indication."

Fostamatinib in IgAN
The Phase 2 study of fostamatinib in IgAN enrolled patients in multiple centers throughout the U.S., Asia and Europe. For inclusion, patients were required to have a diagnosis of IgAN verified by biopsy and proteinuria greater than 500 mg/day. Patients (n=76) were randomized into one of three groups: placebo, fostamatinib at 100 mg bid or fostamatinib at 150 mg bid for 24 weeks. The study evaluated the safety and efficacy as measured by change in proteinuria and renal function. The mean change in proteinuria (sPCR) was -177, -577, and -158 mg/g for the placebo, 100 mg bid and 150 mg bid dose groups, respectively.

This result was not statistically significant.

Subgroup Analysis - Patients with >1 gram/day of Proteinuria
The pre-specified subgroup analysis showed that patients with a baseline proteinuria greater than 1 gram/day (sPCR>1000mg/g) who received fostamatinib had a dose-dependent trend towards a greater reduction in proteinuria from baseline compared to the placebo group at 24 weeks.

In patients with a baseline proteinuria > 1 gram/day (sPCR> 1000mg/g):

Treatment (bid)	Median Baseline sPCR (mg/g)	Median Change from Baseline in sPCR at Week 24	Median % Change from Baseline in sPCR at Week 24	# of patients at Week 24
Placebo	1,890	-177 mg/g	-14%	14
Fostamatinib 100 mg	2,232	-720 mg/g	-27%	16
Fostamatinib 150 mg	2,249	-803 mg/g	-36%	15

Consistently, in patients with a baseline proteinuria greater than 2 grams, fostamatinib treatment showed a similar trend toward a greater reduction in proteinuria as compared to placebo.

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. Two patients in the 100 mg bid dose group and four in the 150 mg bid dose group discontinued the study due to adverse events. There were six patients with serious adverse events (SAEs), two in each of the placebo, 100mg and 150mg dose groups. Of those six patients, one patient in each fostamatinib group had a drug related SAE. One patient had a fatal SAE, which was not drug related.

"There are no specific therapies or treatment algorithms for the thousands of patients that suffer from this disease. So, it is important to identify a possible treatment for IgA nephropathy to reduce the risk for serious complications of progressing kidney disease, which can culminate in the need for dialysis and kidney transplantation in the worst cases," stated Professor Frederick Tam, MBBChir PhD FRCP, the Ken and Mary Minton Chair of Renal Medicine, Department of Medicine at Imperial College London, U.K. "This is a very encouraging long term collaboration between Rigel pharmaceuticals and Imperial College London, resulting in translation from laboratory research to treating patients. In this Phase 2 study, the data showed a non-statistically significant trend for fostamatinib to reduce proteinuria within six months in patients with more advanced disease, which is important because there is a higher risk of kidney function loss when proteinuria increases and persists at high levels."

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-US territory.

Conference Call and Webcast Details
Rigel management will host a conference call and webcast today at 8:00am EDT to discuss the study results. Participants can access the live conference call by dialing 855-892-1489

(domestic) or 720-634-2939 (international) and using the Conference ID number 5669688. A slide presentation accompanying the conference call 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 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 percent 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, reverses the inflammation in the glomeruli and improves kidney function.

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 current programs include clinical studies of fostamatinib, an oral spleen tyrosine kinase (SYK) inhibitor, in a number of indications. Rigel has an NDA under review with the FDA for fostamatinib in patients with chronic 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, the timing of initiation, enrollment and results of clinical trials; Rigel's belief that fostamatinib may be an important alternative for patients with IgAN; Rigel's evaluation of ex-US partnerships for fostamatinib and other partnering opportunities across its pipeline; 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, the timing, completion and results of clinical trials; 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: David Polk
Phone: 310.309.1029
Email: david.polk@syneoshealth.com

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