

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM 8-K**

**CURRENT REPORT**

**Pursuant to Section 13 OR 15(d) of The Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported): **August 30, 2016**

**RIGEL PHARMACEUTICALS, INC.**

(Exact name of registrant as specified in its charter)

**Delaware**

(State or other jurisdiction of incorporation)

**0-29889**

(Commission File No.)

**94-3248524**

(IRS Employer Identification No.)

**1180 Veterans Boulevard**

**South San Francisco, CA**

(Address of principal executive offices)

**94080**

(Zip Code)

Registrant's telephone number, including area code: **(650) 624-1100**

**Not Applicable**

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

**Item 7.01. Regulation FD Disclosure.**

On August 30, 2016, Rigel Pharmaceuticals, Inc. ("Rigel") will host a live conference call at 8:00am Eastern Time (5:00am 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 72149873. The conference call will also be webcast live and can be accessed from Rigel's website at [www.rigel.com](http://www.rigel.com). The webcast will be archived and available for replay after the call via the Rigel website. A copy of the presentation for the conference call is attached as Exhibit 99.1 hereto and is incorporated herein by reference.

This information, including Exhibit 99.1, is being furnished pursuant to Item 7.01 of this Current Report on Form 8-K and shall not be deemed to be "filed" for the purposes of Section 18 of the Securities and Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section and will not be incorporated by reference into any other filing under the Exchange Act or under the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such a filing. This Current Report on Form 8-K will not be deemed an admission as to the materiality of any information in this Current Report on Form 8-K that is being disclosed pursuant to Regulation FD.

**Item 8.01. Other Information.**

On August 30, 2016, Rigel issued a press release, titled "Rigel's Fostamatinib Meets Primary Endpoint in Phase 3 Study in Chronic ITP," a copy of which is attached as Exhibit 99.2 hereto and is incorporated herein by reference.

**Item 9.01. Financial Statements and Exhibits.**

**(d) Exhibits.**

<u>Exhibit</u>	<u>Description</u>
99.1	Presentation, dated August 30, 2016.
99.2	Press Release, dated August 30, 2016, titled "Rigel's Fostamatinib Meets Primary Endpoint in Phase 3 Study in Chronic ITP."

## SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Dated: August 30, 2016

**RIGEL PHARMACEUTICALS, INC.**

By: /s/ Dolly A. Vance  
Dolly A. Vance  
*Executive Vice President, General Counsel and Corporate Secretary*

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## EXHIBIT INDEX

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# **Rigel Conference Call FIT Phase 3 Study in ITP**

August 30, 2016  
5:00am PT / 8:00am ET



# Agenda

Safe Harbor Statement	<i>D. Vance</i>
Introduction and Overview	<i>R. Rodriguez</i>
FIT 1 Phase 3 Results	<i>A. Duliege</i>
FIT 1 Phase 3 Commentary	<i>J. Bussel</i>
Q&A	

# Safe Harbor Statement

In the conference call accompanying these slides, Rigel management will be making some forward-looking statements, including statements relating to Rigel's plans for the future clinical development of fostamatinib and the timing of results thereof.

Any statements contained in this call that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "anticipates," "plans," "intends," "expects" and similar expressions are intended to identify these forward-looking statements. These forward-looking statements are based on Rigel's current expectations and involve risks and uncertainties.

There are a number of important factors that could cause Rigel's results to differ materially from those indicated by these forward-looking statements, including risks associated with the timing and success of clinical trials and other risks detailed in Rigel's SEC reports, including its Quarterly Report on Form 10-Q for the quarter ended June 30, 2016. Rigel expressly disclaims any obligation or undertaking to update the forward-looking statements discussed in this call.



# Introduction and Overview

Raul Rodriguez  
President and Chief Executive Officer



# Participants

## Rigel Senior Management:

- **Raul Rodriguez** – President and Chief Executive Officer
- **Anne-Marie Duliege, MD** – Executive Vice President and Chief Medical Officer
- **Donald G. Payan, MD** – Executive Vice President and Chief Scientific Officer
- **Dolly Vance** – Executive Vice President, Corporate Affairs and General Counsel
- **Ryan Maynard** – Executive Vice President and Chief Financial Officer

## Principal Investigator:

- **James Bussel, MD** – Professor of Pediatrics, Pediatrics in Obstetrics and Gynecology, and Pediatrics in Medicine at Weill Cornell Medical College





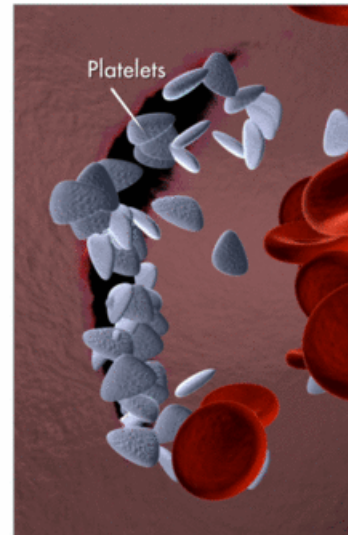
# ITP Background

## Significant unmet need:

- Characterized by the destruction of platelets by the body's own immune system
- Increased risk of severe bleeding events
  - Can result in serious medical complications or even death
- Heterogeneous patient population, difficult to predict which of available therapies will work, potentially including splenectomy
- **Approximately 50,000-60,000 adult primary ITP patients in the United States (Orphan disease)**

## Attractive market dynamics:

- Niche market
- Focused and identifiable prescriber base
- Need for new agents



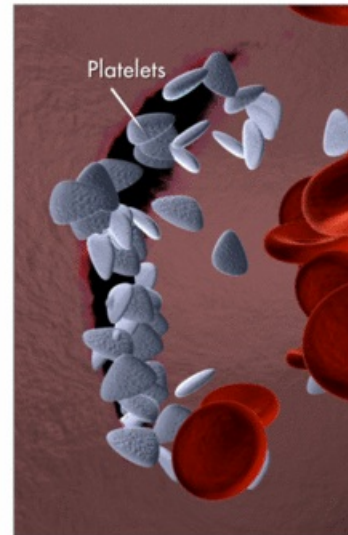
Neunert C, et al. *Blood*. 2011;117:4190-4207.  
Provan D and Newland AC. *Adv Ther*. 2015;32:875-887.  
Provan D, et al. *Blood*. 2010;115:168-186.



# Fostamatinib in ITP

## Why Fostamatinib?

- **Novel mechanism of action**
  - Oral inhibitor of SYK, a key player in platelet destruction in ITP
  - May uniquely address the underlying autoimmune basis of ITP by impeding platelet destruction
- **Based on Phase 2 study: timely, substantial, and enduring benefit**
  - Primary endpoint responders do so within weeks of initiating treatment
  - Initial platelet counts <20K increase to >100K
  - Two patients taking Fostamatinib for 7+ years maintain attractive platelet levels over an extended period of time
- **Safety**
  - Large safety database, primarily in patients with autoimmune disease (>5000 patient-years)



Brasemann S, et al. *J Pharmacol Exp Ther*. 2006;319:998-1008.  
Podolanczuk A, et al. *Blood*. 2009;113:3154-3160.

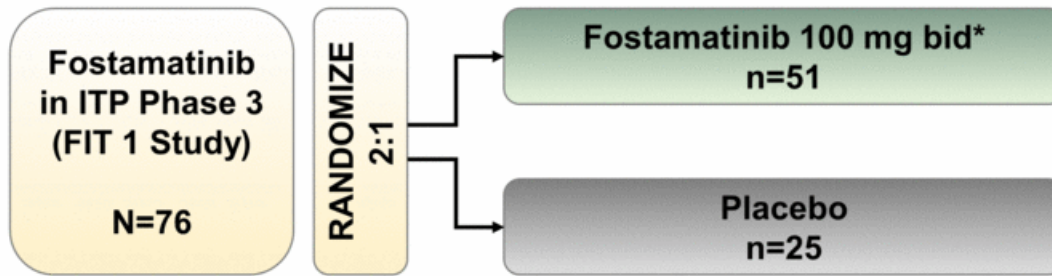


# **FIT 1 Phase 3 Program: Study 047 Results**

Anne-Marie Duliege, MD  
Executive Vice President and Chief Medical Officer



# FIT 1 Phase 3 - Study 047: Study Design



- **Primary endpoint:**
  - Stable platelet count, defined as platelet counts of  $\geq 50,000/\mu\text{L}$  on  $\geq 4$  of the 6 visits between Weeks 14 and 24
- **Secondary endpoints:**
  - Achievement of a platelet response (a platelet count of  $\geq 50,000/\mu\text{L}$ ) at Week 12 or Week 24
  - Additional measures of response for patients with baseline platelet count  $<15,000/\mu\text{L}$
  - Over the 24-week study period, frequency and severity of bleeding according to the ITP Bleeding Score (IBLS) and the World Health Organization (WHO) bleeding scale

\*If  $<50,000$  platelets/ $\mu\text{L}$  at week 3, then increase to 150 mg bid.  
bid=twice a day.



# FIT 1 Phase 3 - Study 047: Relevant Patient Baseline Characteristics

## Patient Population:

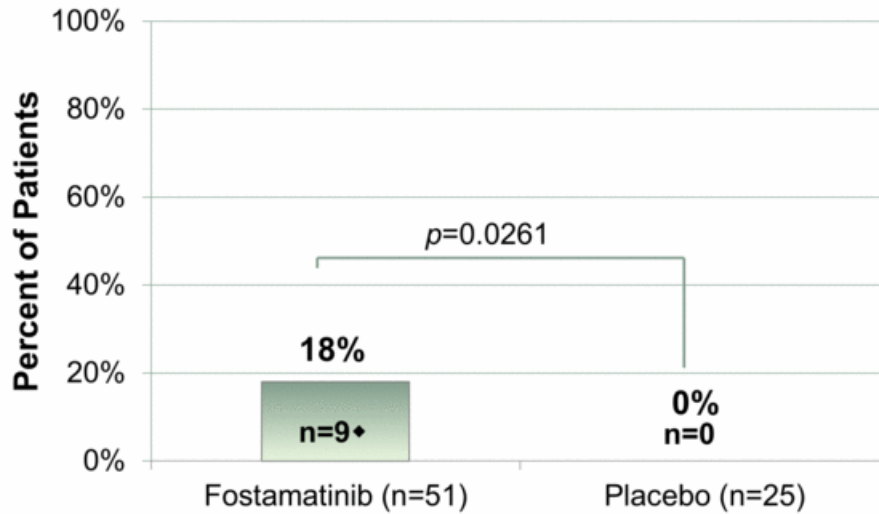
- Adults with chronic/persistent ITP, defined as having consistently low platelet levels of <30,000 platelets/ $\mu$ L of blood
- All subjects have received prior treatment for ITP

	Fostamatinib N=51	Placebo N=25	Total N=76
Age, median (years)	57	57	57
Gender, n (%)			
Female	30 (59%)	17 (68%)	47 (62%)
Male	21 (41%)	8 (32%)	29 (38%)
Duration of ITP (years)			
Median	7.5	5.5	7
Range	0.6 - 53	0.4 - 45	0.4 - 53
Prior treatments, n (%)			
Steroids	46 (90%)	25 (100%)	71 (93%)
Rituximab	26 (51%)	11 (44%)	37 (49%)
Thrombopoietic agents	26 (50%)	15 (60%)	41 (54%)
Splenectomy	20 (39%)	10 (40%)	30 (39%)
Median platelet count at Baseline	15,000	16,000	15,000



# FIT 1 Phase 3 – Study 047: Primary Endpoint

## Stable Platelet Response\* by Week 24



\*Stable platelet response (primary endpoint): platelet count of  $\geq 50,000/\mu\text{L}$  on  $\geq 4$  of the last 6 visits between Week 14 and Week 24

•9/51=18%



# FIT 1 Phase 3 - Study 047: Adverse Events

Number (n) and % of Patients with $\geq$ one Adverse Event (AE)	Fostamatinib N=51	Placebo N=25
	n (%)	N (%)
Any AE*	49 (96%)	19 (76%)
Serious AEs	8 (16%)	5 (20%)
Treatment-related AEs	39 (77%)	7 (28%)
Gastrointestinal complaints**	31 (61%)	5 (20%)
Nausea	15 (29%)	1 (4%)
Diarrhea	23 (45%)	4 (16%)
Infection	17 (33%)	5 (20%)
Hypertension during visit	18 (35%)	2 (8%)
Transaminase elevation	11 (22%)	0 (0%)

\* AEs were generally mild (67%) or moderate (30%)

\*\* Nausea, vomiting, diarrhea, or abdominal pain



# **FIT 1 Phase 3 – Study 047 Commentary**

James Bussel, MD  
Principal Investigator





# Conclusions

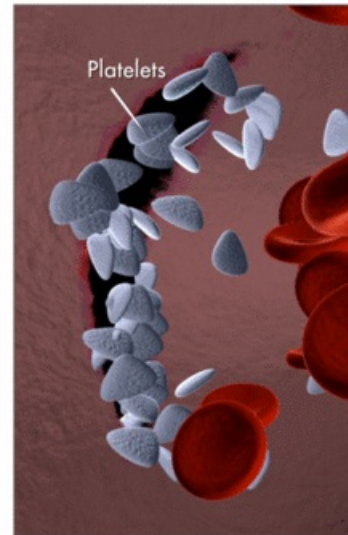
## Key Findings:

- Met study primary endpoint of stable platelet response
- Safety profile consistent with prior experience. AEs related to GI were most frequent. AEs were generally mild or moderate.

## For Patients who met Primary Endpoint:

- Timely platelet response
- Substantial increase in platelet counts
- Platelet response was enduring

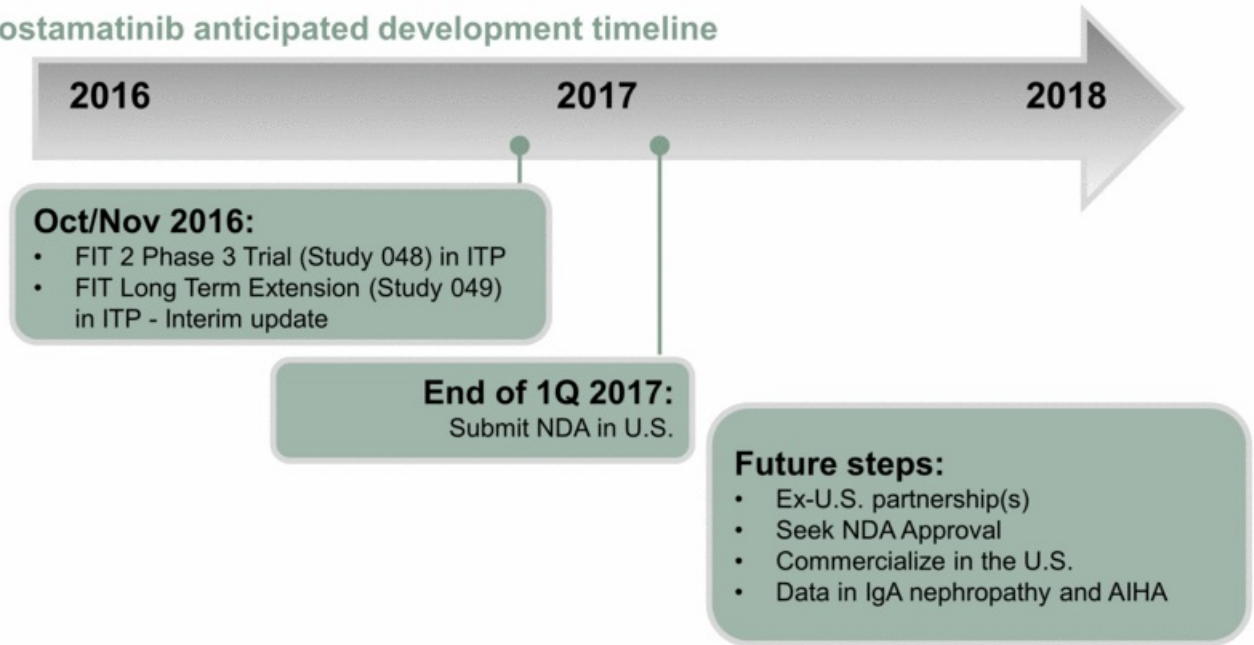
## Attractive opportunity for Rigel



# Q&A

# Fostamatinib Next Steps

## Fostamatinib anticipated development timeline



AIHA=autoimmune hemolytic anemia; IgA=immunoglobulin A; NDA=New Drug Application





## Rigel's Fostamatinib Meets Primary Endpoint in Phase 3 Study in Chronic ITP

*Conference call and webcast today at 8:00 AM Eastern Time*

SOUTH SAN FRANCISCO, Calif., August 30, 2016 — Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL) today announced that fostamatinib, its oral spleen tyrosine kinase (SYK) inhibitor, met the primary endpoint in the first of two double-blind studies in the FIT Phase 3 clinical program for the treatment of adult chronic/persistent immune thrombocytopenia (ITP). The study (n=76) showed that 18% of patients receiving fostamatinib achieved a stable platelet response compared to none receiving a placebo control (p=0.0261). A stable platelet response was defined as achieving greater than 50,000 platelets per uL of blood on at least four of the last six scheduled visits between weeks 14 and 24 of treatment. The results from the second FIT Phase 3 study are expected in October/November 2016.

The most frequent adverse events were gastrointestinal-related, and the safety profile of the product was consistent with prior clinical experience, and no new or unusual safety issues were discovered.

“These data demonstrate the potential benefit of fostamatinib for chronic ITP patients who are in need of new treatment options,” said Raul Rodriguez, president and chief executive officer of Rigel. “We believe that fostamatinib has significant commercial potential given that it has a unique mechanism of action that may work where other products have failed.”

“We are very encouraged by these results,” said Anne-Marie Duliege, M.D., executive vice president and chief medical officer of Rigel. “Consistent with the prior clinical study of fostamatinib in ITP, this FIT Phase 3 study demonstrated that fostamatinib provided a robust and enduring benefit for those patients who responded to the drug candidate.”

Patients who met the primary endpoint of this study typically had an increase in platelet counts to a level above 50,000/uL within the initial weeks of treatment, providing early feedback as to whether it was a viable option for treating their ITP.

In general, the clinical goal of ITP treatment is to raise platelet counts to more than 50,000/uL. Patients who met the primary endpoint in this study had their platelet counts increase from a median of 16,000/uL at baseline to a median of more than 100,000/uL at week 24, a robust response that potentially allows patients to remain above 50,000/uL more consistently.

All of the patients from this study who met the stable platelet response endpoint enrolled in the long-term, Phase 3 extension study and continued to maintain their platelet levels for months past the initial study period of 24 weeks. These data affirm similar results observed in two patients from the Rigel Phase 2 study of fostamatinib in ITP who have been taking fostamatinib for more than seven years and have maintained stable platelet levels over this extended time period.

Fostamatinib's clinical safety profile includes more than 5,000 patient years of data across multiple autoimmune indications and has a well-defined and manageable safety profile, providing data that it may be suitable for long-term maintenance therapy in chronic ITP.

If these results are reproduced in the second Phase 3 study and are supported by the results of a planned interim analysis of the Phase 3 extension study, the company expects to submit a New Drug Application

with the U.S. Food and Drug Administration in the first quarter of 2017. Further results from the FIT Phase 3 studies and long-term extension will be presented at future medical meetings.

### **FIT Phase 3 Program**

The FIT program consists of two identical multi-center, randomized, double-blind, placebo-controlled studies of approximately 75 adult patients each. The patients have been diagnosed with persistent or chronic ITP, and have blood platelet counts consistently below 30,000/uL of blood. The patients all had experience with at least one other ITP treatment such as steroids, Rituxan, splenectomy and/or TPO mimetics. Patients were randomized in a 2:1 ratio to receive either fostamatinib or placebo twice a day to be taken for up to six months. Study subjects remained on treatment for up to 24 weeks. The primary efficacy endpoint of this program is a stable platelet response defined as achieving platelet counts at or above 50,000/uL of blood for at least four of the last six clinic visits of the study. Patients were subsequently offered to enroll in an open-label, Phase 3, long-term extension study, which is ongoing.

### **Fostamatinib and 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. There are approximately 50-60 thousand adult patients in the U.S. living with primary chronic ITP. ITP patients can suffer extraordinary bruising, bleeding and fatigue as a result of low platelet counts. Further, people suffering with chronic ITP live with 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. While these treatment options can be effective in treating ITP symptoms, given the heterogeneity of the disease, each has significant limitations. It can be difficult to predict which approved treatments are going to be effective.

Fostamatinib is an oral investigational drug with a unique mechanism of action designed to inhibit SYK kinase, a key player in the immune process that leads to platelet destruction in ITP. The U.S. Food and Drug Administration has granted Orphan Drug designation to fostamatinib for the treatment of patients with ITP. Unlike other therapies that modulate the immune system in different ways or stimulate platelet production, fostamatinib may address the underlying autoimmune basis of ITP by impeding platelet destruction. Fostamatinib potentially offers a compelling addition to the treatment options available for ITP patients.

### **Conference Call and Webcast Presentation Today at 8:00AM Eastern Time**

Rigel will hold a live conference call and webcast today at 8:00am Eastern Time (5:00am 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 72149873.

The conference call and accompanying slide presentation will also be webcast live and can be accessed from Rigel's website at [www.rigel.com](http://www.rigel.com). The webcast will be archived and available for replay after the call via the Rigel website.

### **About Rigel ([www.rigel.com](http://www.rigel.com))**

Rigel Pharmaceuticals, Inc. is a clinical-stage biotechnology company dedicated to the discovery and development of novel, targeted drugs in the therapeutic areas of immunology, oncology and immuno-oncology. Rigel's pioneering research focuses on signaling pathways that are critical to disease mechanisms. The company's current clinical programs include fostamatinib, an oral spleen tyrosine kinase (SYK) inhibitor, which is in Phase 3 clinical trials for immune thrombocytopenia (ITP); a Phase 2

clinical trial for autoimmune hemolytic anemia (AIHA); and a Phase 2 clinical trial for IgA nephropathy (IgAN).

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In addition, Rigel has two oncology product candidates in Phase 1 development with partners BerGenBio AS and Daiichi Sankyo.

*This press release contains “forward-looking” statements, including, without limitation, statements related to Rigel’s clinical development plans, including the timing, design and nature of planned clinical trials and the timing and nature of results of those trials, as well as the potential activity of fostamatinib with respect to 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,” “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 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 trials, 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 Annual Report on Form 10-Q for the year ended June 30, 2016. 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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Contacts:

Raul Rodriguez  
Phone: 650.624.1302  
Email: invrel@rigel.com

Jessica Daitch  
Chandler Chicco Agency  
Phone: 917.816.6712  
Email: jessica.daitch@inventivhealth.com

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