

---

---

**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): **October 20, 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 2.02 Results of Operations and Financial Condition**

On October 20, 2016, Rigel Pharmaceuticals, Inc. ("Rigel") announced that it expects to report that it had approximately \$85.3 million of cash, cash equivalents and short-term investments as of September 30, 2016. This amount is preliminary and is subject to change upon the completion of Rigel's financial closing procedures. Additional information and disclosures would be required for a more complete understanding of the Company's financial position and results of operations as of September 30, 2016.

**Item 7.01 Regulation FD Disclosure.**

On October 20, 2016, Rigel issued a press release, titled "Rigel Announces Results from the Second FIT Phase 3 Study and the Long-Term Open-Label Extension Study for Fostamatinib in ITP," a copy of which is attached as Exhibit 99.1 hereto and is incorporated herein by reference. In addition, on October 20, 2016, 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 98782744. 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 99.2 hereto and is incorporated herein by reference.

**Forward-Looking Statements**

This Current Report on Form 8-K contains forward-looking statements, including, without limitation, statements relating to Rigel's cash position as of September 30, 2016. These forward-looking statements are based upon Rigel's current expectations. Actual results could differ materially from these forward-looking statements as a result of certain factors, including, without limitation, risks related to changes in estimated cash position based on the completion of financial closing procedures and the audit of Rigel's financial statements, as well as other risks detailed in Rigel's reports filed with the Securities and Exchange Commission, including its Quarterly Report on Form 10-Q for the quarter 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. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this report.

This information, including Exhibits 99.1 and 99.2, is being furnished pursuant to Items 2.02 and 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.

**Item 9.01 Financial Statements and Exhibits.**

**(d) Exhibits.**

<b>Exhibit</b>	<b>Description</b>
99.1	Press Release, dated October 20, 2016, titled “Rigel Announces Results from the Second FIT Phase 3 Study and the Long-Term Open-Label Extension Study for Fostamatinib in ITP.”
99.2	Presentation, dated October 20, 2016.

2

**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: October 20, 2016

**RIGEL PHARMACEUTICALS, INC.**

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

3

**EXHIBIT INDEX**

<b>Exhibit</b>	<b>Description</b>
99.1	Press Release, dated October 20, 2016, titled “Rigel Announces Results from the Second FIT Phase 3 Study and the Long-Term Open-Label Extension Study for Fostamatinib in ITP.”
99.2	Presentation, dated October 20, 2016.

4



**Rigel Announces Results from the Second FIT Phase 3 Study and the Long-Term Open-Label Extension Study for Fostamatinib in ITP**

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

SOUTH SAN FRANCISCO, Calif., October 20, 2016—Rigel Pharmaceuticals, Inc. (Nasdaq:RIGL) today announced results from the second of two double-blind studies in the FIT Phase 3 clinical program for fostamatinib, an oral spleen tyrosine kinase (SYK) inhibitor, in adult chronic/persistent immune thrombocytopenia (ITP). The primary endpoint in the study was a stable platelet response, defined as platelet counts greater than 50,000/uL of blood on at least four of the last six scheduled clinic visits between weeks 14 and 24 of treatment. In the FIT 2 (Study 048) Phase 3 study, the fostamatinib response rate was 18%, consistent with the recently reported FIT 1 (Study 047) Phase 3 study. In Study 048, one patient in the placebo group (4%) achieved a stable platelet response; therefore the difference between those on treatment and those on placebo did not reach statistical significance ( $p=0.152$ ) and the study did not meet its primary endpoint. When the data from both studies are combined, however, this difference is statistically significant ( $p=0.007$ ). Data from both FIT Phase 3 studies and the open-label extension study demonstrates the consistent benefit of fostamatinib in ITP.

**Stable Platelet Responders / Total Patients**

	<b>FIT 1 — Study 047</b>		<b>FIT 2 — Study 048</b>		<b>Combined</b>	
Fostamatinib	9/51	18%	9/50	18%	18/101	18%
Placebo	0/25	0%	1/24	4%	1/49	2%
	$p=0.026$		$p=0.152$		$p=0.007$	

“We believe that the totality and consistency of data from the FIT Phase 3 program, which included two Phase 3 studies and one long-term extension study, strongly supports a clear treatment effect, with a sustained clinical benefit of fostamatinib,” said Raul Rodriguez, president and chief executive officer of Rigel. “We are encouraged by these results and believe that the risk/benefit ratio for fostamatinib is positive for patients with chronic/persistent ITP, a population with a serious unmet medical need. As a result, we will continue to pursue this opportunity. Our next step is to seek feedback from the FDA.”

In the combined dataset for Study 047 and Study 048, patients who met the primary endpoint had their platelet counts increase from a median of 18,500/uL of blood at baseline to more than 100,000/uL at week 24 of treatment. These patients benefited substantially and typically did so within weeks of initiating treatment, providing early feedback as to whether fostamatinib may be a viable option for treating their ITP. In the combined data sets, the frequency of patients who achieved a stable platelet response was statistically superior in the fostamatinib group versus the placebo group in all subgroup analyses: prior splenectomy or not; prior exposure to TPO agents or not; platelet counts below or above 15,000/uL of blood at baseline, demonstrating that the effect of fostamatinib is consistent across various clinical and treatment backgrounds.

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

#### **FIT Phase 3 Long-Term Extension Study 049**

Patients from both the 047 and 048 Phase 3 studies were given the option to enroll in a long-term open-label extension study (Study 049) and receive treatment with fostamatinib. As of June 2016, 118 patients had been enrolled in this study. All the patients who responded to fostamatinib in the parent studies enrolled in Study 049 and had a median platelet count of 96,000/uL of blood in this study. These patients have been exposed to fostamatinib for a median of 13 months through the combined parent and 049 trials.

In addition, there were 43 placebo non-responders from the 047 and 048 studies that enrolled in the 049 study. 36 of these patients had at least 12 weeks of follow-up. Of these, 6 patients (17%,  $p=0.01$ ) achieved a prospectively defined stable platelet response in the 049 study.

“Given the heterogeneity of ITP, it is currently almost impossible to predict how patients will respond to available therapies, which is why it is so important for physicians and patients to have treatment options,” said James B. Bussel, M.D., professor of pediatrics, pediatrics in obstetrics and gynecology, and pediatrics in medicine at Weill Cornell Medicine, and the principal study investigator on the FIT Phase 3 program. Dr. Bussel is also a member of Rigel’s advisory/scientific board. “This heterogeneity means that treatments that work by different mechanisms can make important contributions in certain patients, such as those who might be especially responsive to fostamatinib because of its unique mechanism of action. The FIT Phase 3 studies have both demonstrated that fostamatinib provided a robust and enduring benefit for those patients who responded to the drug.”

#### **Statement on Financial Position**

Rigel expects to report that it ended the third quarter of 2016 with approximately \$85.3 million in cash, cash equivalents, and short-term investments, which Rigel expects will be sufficient to fund its operations through the end of 2017. In this forecast, Rigel has allocated substantial funds to continue efforts in preparation of the potential commercial launch of fostamatinib in ITP. Rigel is also continuing to evaluate ex-U.S. partnerships for fostamatinib and other partnering opportunities. As a result of the recent research restructuring, Rigel believes that it has created greater flexibility for its cash runway moving forward.

#### **About the 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, have failed at least one prior therapy for ITP, and have platelet counts consistently below 30,000/uL of blood. Patients were randomized in a 2:1 ratio to receive either fostamatinib or placebo orally twice a day for up to 24 weeks. The primary efficacy endpoint of this program is a stable platelet response defined as achieving platelet counts greater than 50,000/uL of blood for at least four of the six scheduled clinic visits between weeks 14 and 24 of treatment. Patients were subsequently offered to enroll in an open-label, long-term Phase 3 extension study, which is ongoing.

#### **About 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. ITP patients can suffer extraordinary bruising, bleeding and fatigue as a result of low platelet counts. Current therapies for ITP include steroids, blood platelet production boosters (TPOs) and splenectomy. However, a significant portion of patients do not do well on existing therapies. As a result, there remains a significant medical need for additional treatment options for patients with ITP.

Fostamatinib is an oral investigational candidate 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 FDA 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 cause of ITP by impeding platelet destruction.

#### **Conference Call and Webcast 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 1-855-892-1489 (domestic) or 1-720-634-2939 (international) and using the Conference ID number 98782744.

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.

#### **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. The company completed and reported results from two Phase 3 clinical studies of fostamatinib in chronic immune thrombocytopenia (ITP) in August and October 2016. Rigel is also conducting a Phase 2 clinical trial with fostamatinib in autoimmune hemolytic anemia (AIHA) and a Phase 2 clinical trial for IgA nephropathy (IgAN). 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, as well as Rigel's cash position as of September 30, 2016 and sufficiency of cash resources. 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, change in Rigel's estimated cash position based on the completion of its financial closing procedures, 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 Quarterly Report on Form 10-Q for the quarter 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.*

---

Contacts:  
Raul Rodriguez  
Phone: 650.624.1302  
Email: [invrel@rigel.com](mailto:invrel@rigel.com)

Jessica Daitch  
Chandler Chicco Agency  
Phone: 917.816.6712  
Email: [jessica.daitch@inventivhealth.com](mailto:jessica.daitch@inventivhealth.com)

---



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

October 20, 2016

5:00am PT / 8:00am ET



# Agenda

Safe Harbor Statement	<i>D. Vance</i>
Introduction and Overview	<i>R. Rodriguez</i>
FIT Phase 3 Program Results	<i>A. Duliege</i>
FIT 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
- **Dolly Vance** – Executive Vice President, Corporate Affairs and General Counsel
- **Ryan Maynard** – Executive Vice President and Chief Financial Officer
- **Eldon Mayer** – Executive Vice President and Chief Commercial Officer
- **Esteban Masuda, PhD** – Senior Vice President, Research

## Principal Investigator:

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



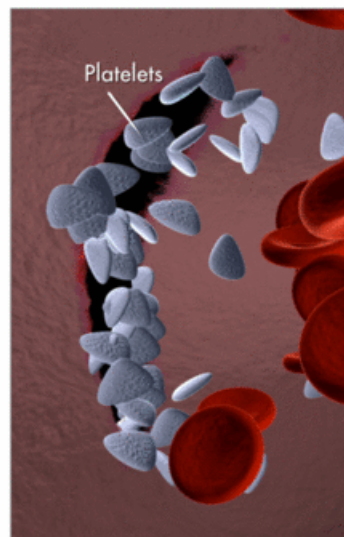
# ITP Background

## 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 (approx. 5000 patient-years)



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

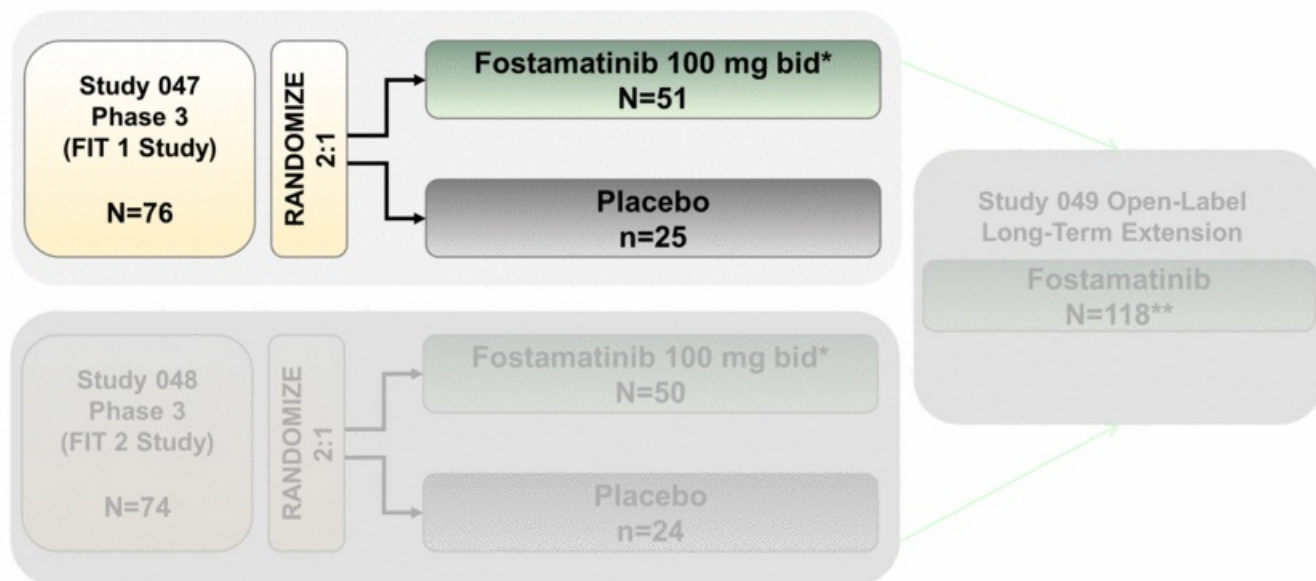


# **FIT Phase 3 Program Results**

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



# FIT Phase 3 Program



- **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

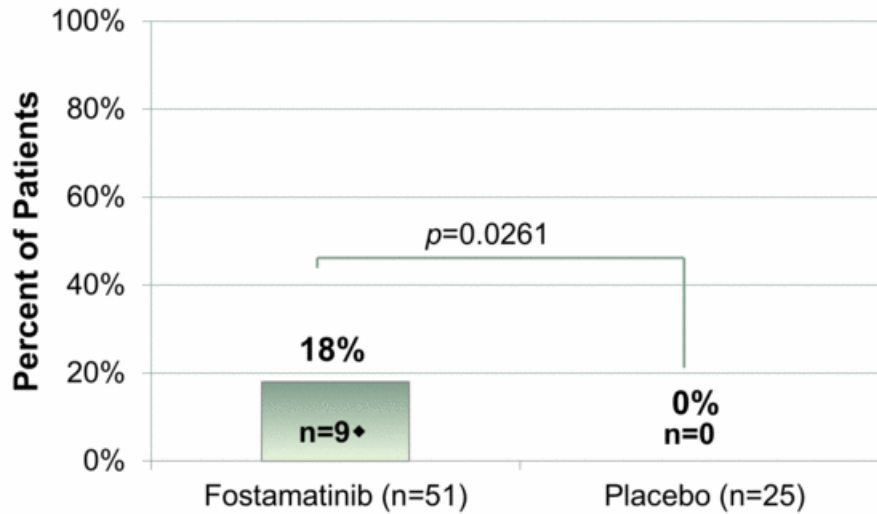
\*If  $<50,000$  platelets/ $\mu\text{L}$  at Week 3, then increase to 150 mg bid (bid = twice a day)

\*\* n=118 with monitored data as of June 2016



*From August announcement:*  
**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%



*From August announcement:*

## **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%)

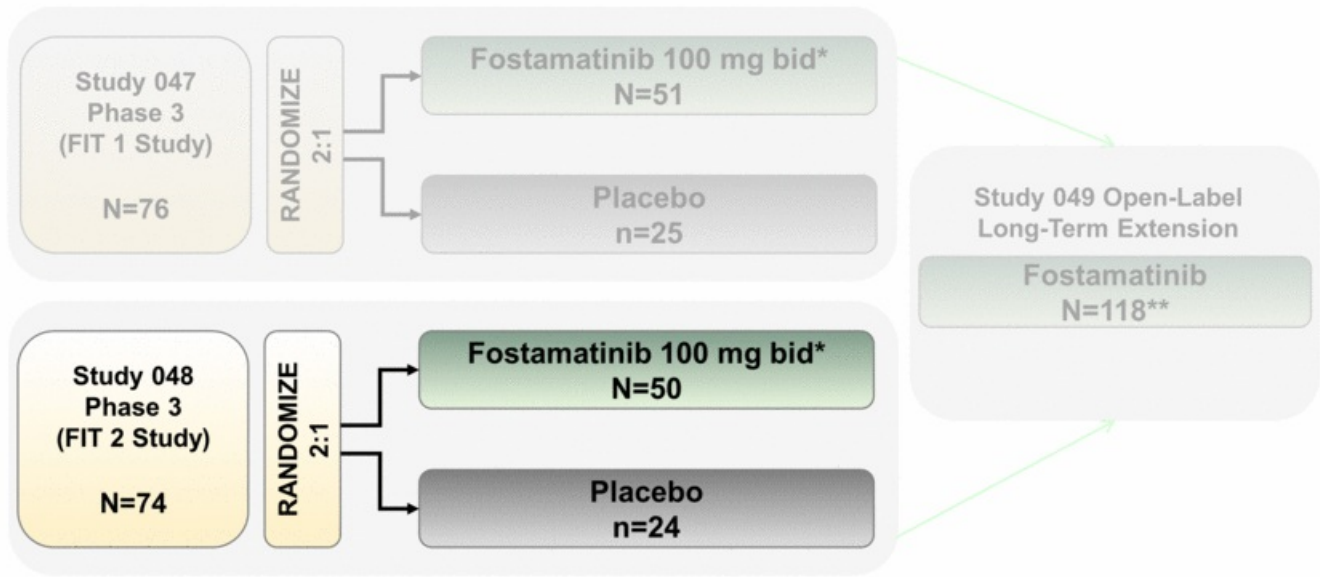
\* AEs in the fostamatinib group were generally mild (68%) or moderate (29%)

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





# FIT Phase 3 Program



- **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

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

\*\* n=118 with monitored data as of June 2016



# FIT 2 Phase 3 - Study 048: Relevant Patient Baseline Characteristics

## Patient Population:

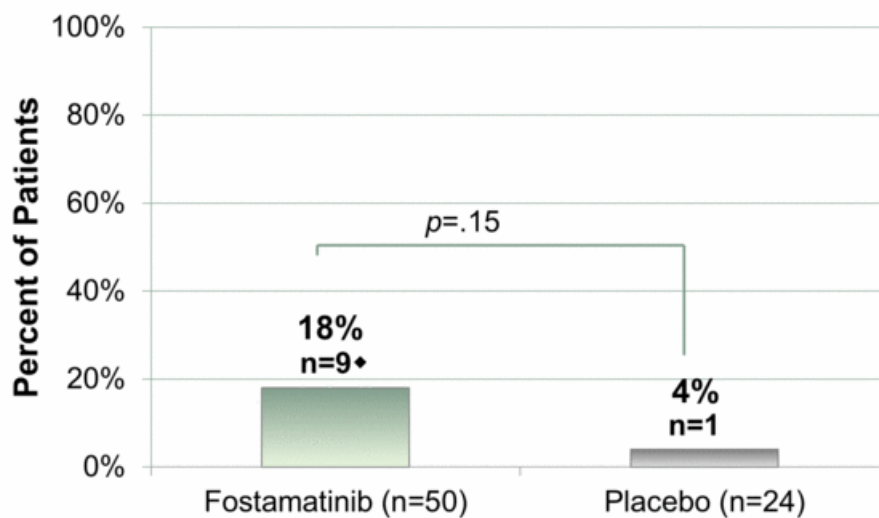
- Adults with chronic/persistent ITP, defined as having consistently low platelet levels of <30,000 platelets/ $\mu$ L of blood
- All subjects have received prior treatment for ITP

	Fostamatinib N=50	Placebo N=24	Total N=74
Age, median (years)	50	50	50
Gender, n (%)			
Female	31 (62%)	13 (54%)	44 (60%)
Male	19 (38%)	11 (46%)	30 (41%)
Duration of ITP (years)			
Median	8.8	10.8	9.6
Range	0.3 - 50	0.9 - 29	0.3 - 50
Prior treatments, n (%)			
Steroids	45 (90%)	22 (92%)	67 (91%)
Rituximab	8 (16%)	3 (13%)	11 (15%)
Thrombopoietic agents	20 (40%)	10 (42%)	30 (41%)
Splenectomy	14 (28%)	9 (38%)	23 (31%)
Median platelet count at Baseline	16,000	21,000	16,000



# FIT 2 Phase 3 – Study 048: Primary Endpoint

## Stable Platelet Response\* by Week 24



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

•  $9/50=18\%$



# FIT 2 Phase 3 - Study 048: Adverse Events

Number and % of Patients with ≥ one Adverse Event (AE)	Fostamatinib N=51	Placebo N=23
Any AE	36 (71%)*	18 (78%)
Serious AEs	5 (10%)	6 (26%)
Treatment-related AEs	20 (39%)	6 (26%)
Gastrointestinal complaints**	11 (22%)	5 (22%)
• Nausea	4 (8%)	3 (13%)
• Diarrhea	9 (18%)	3 (13%)
Infection	11 (22%)	5 (22%)
Hypertension during visit	10 (20%)	4 (17%)
Transaminase elevation	3 (6%)	0 (0%)

\* AEs in the fostamatinib group were generally mild (82%) or moderate (16%)

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



# FIT Phase 3 – Combined Study 047+048: Relevant Patient Baseline Characteristics

## Patient Population:

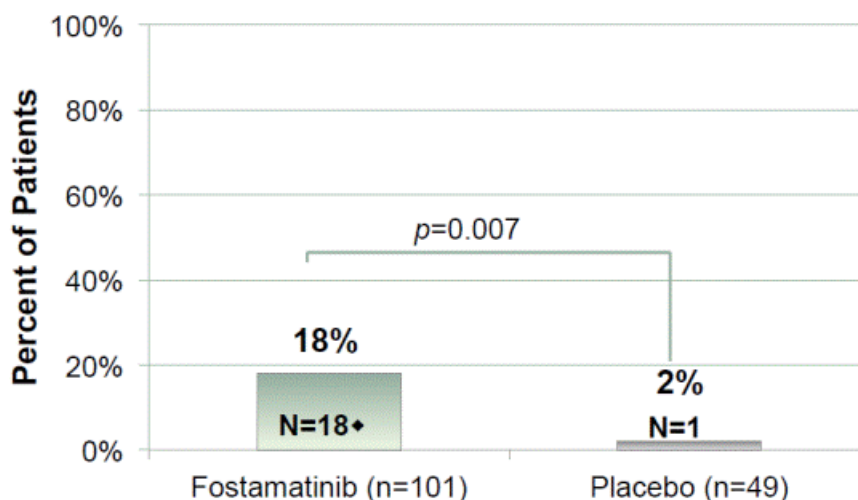
- Adults with chronic/persistent ITP, defined as having consistently low platelet levels of <30,000 platelets/ $\mu$ L of blood
- All subjects have received prior treatment for ITP

	Fostamatinib N=101	Placebo N=49	Total N=150
Age, median (years)	54	53	54
Gender, n (%)			
Female	61 (60%)	30 (61%)	91 (61%)
Male	40 (40%)	19 (39%)	59 (39%)
Duration of ITP (years)			
Median	8.7	7.8	8.5
Range	0.3-53	0.4-45	0.3-53
Prior treatments, n (%)			
Steroids	91 (90%)	47 (96%)	138 (92%)
Rituximab	34 (34%)	14 (29%)	48 (32%)
Thrombopoietic agents	46 (45%)	25 (51%)	71 (47%)
Splenectomy	34 (34%)	19 (39%)	53 (35%)
Median platelet count at Baseline	15,000	17,000	16,000



17

# FIT Phase 3 – Combined Study 047+048: Rate of Stable Platelet Response\*



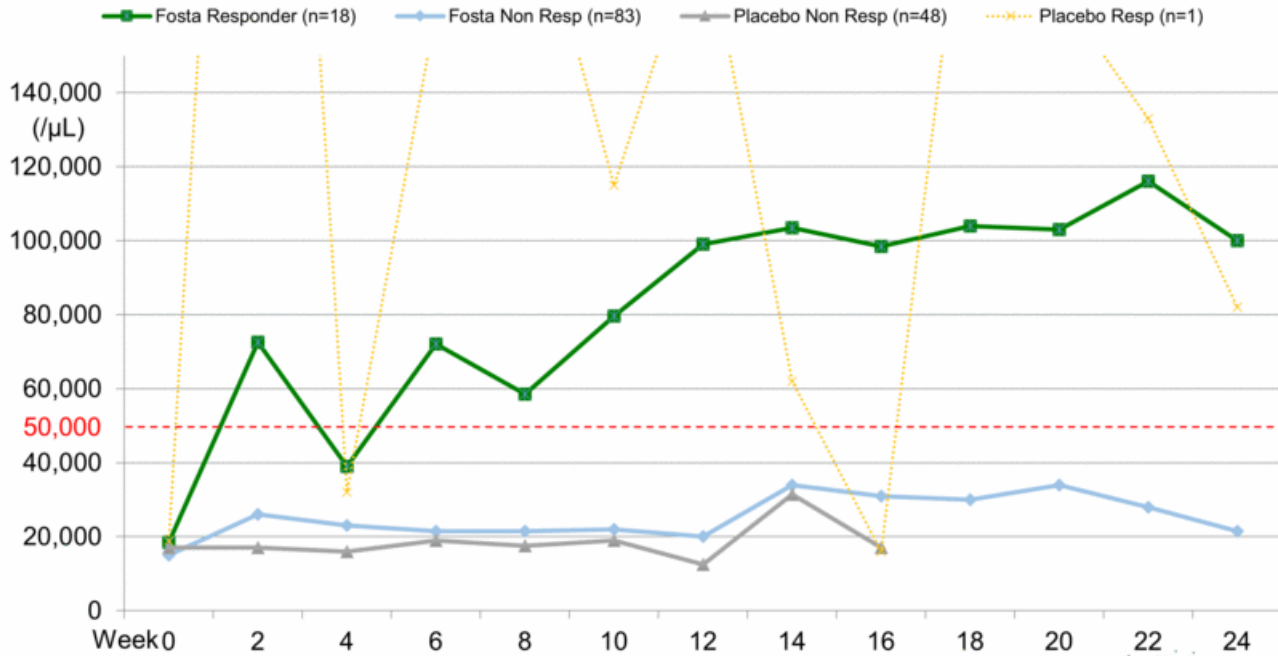
\*Stable platelet response (primary endpoint): platelet count of  $\geq 50,000/\mu$ L on  $\geq 4$  of the last 6 visits between Week 14 and Week 24

•18/101=18%



18

# FIT Phase 3 – Combined Study 047+048: Median Platelet Counts Over Time



\*Data not shown for time points with  $n \leq 3$  patients (except for the placebo non responder,  $n=1$ )



# FIT Phase 3 – Combined Study 047+048: Adverse Events

Number (n) and % of Patients with $\geq$ one Adverse Event (AE)	Fostamatinib N=102	Placebo N=48
	n (%)	n (%)
Any AE	85 (83%)*	37 (77%)
Serious AEs	13 (13%)	11 (23%)
Treatment-related AEs	59 (58%)	13 (27%)
Gastrointestinal complaints**	42 (41%)	10 (21%)
- Nausea	19 (19%)	4 (8%)
- Diarrhea	32 (31%)	7 (15%)
Infection	28 (28%)	10 (21%)
Hypertension during visit	28 (28%)	6 (13%)
Transaminase elevation	14 (14%)	0 (0%)

\* AEs in the fostamatinib group were generally mild (74%) or moderate (22%)

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

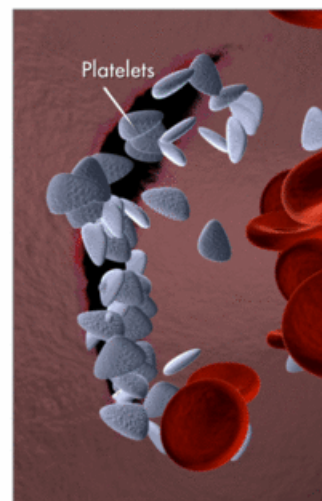


# FIT Phase 3 – Combined Study 047+048: Sub-Group Analysis

**The response rate was statistically superior in the fostamatinib group vs. placebo across all sub-groups:**

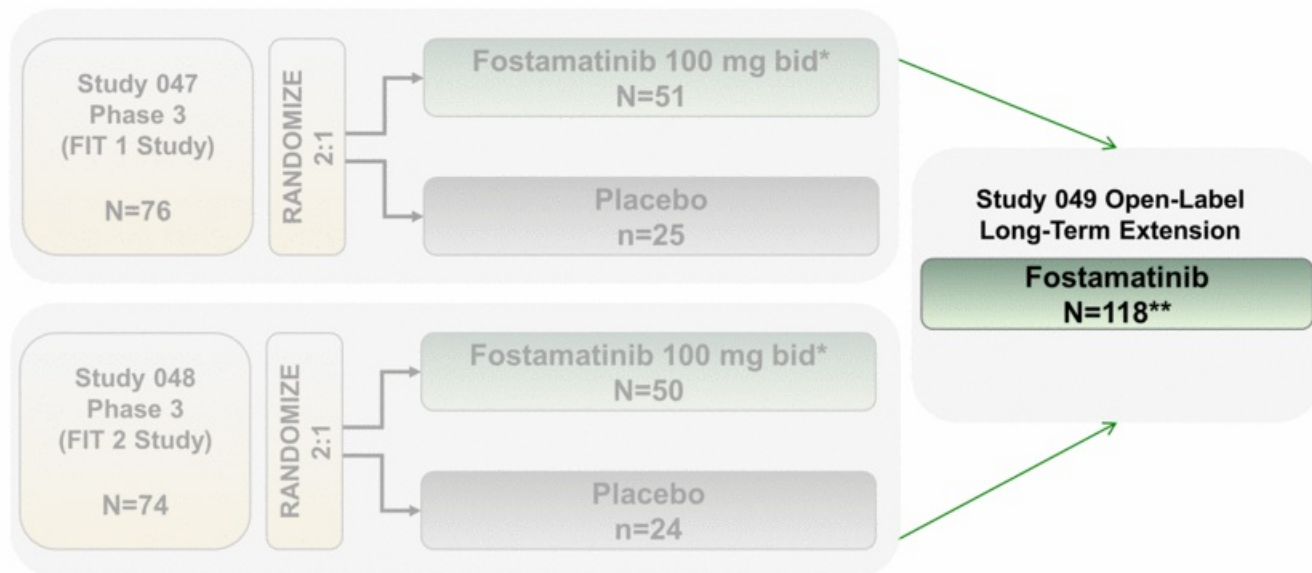
- Prior splenectomy / No prior splenectomy
- Prior TPO agent exposure / TPO naïve
- Baseline platelet count <15,000 /  $\geq 15,000$

**Fostamatinib benefit is consistent across patients with various clinical and treatment backgrounds**





# FIT Phase 3 Program



- **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

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

\*\*n=118 with monitored data as of June 2016



# FIT Phase 3 Long Term Extension Study 049

## 1. Enduring benefit in fostamatinib patients who had a stable platelet response in Study 047 and 048

- Have generally maintained platelet counts  $>50,000/\mu\text{L}$  in Study 049
- Median count =  $96,000/\mu\text{L}$ ; total median exposure of 13 months to fostamatinib

## 1. Response rate in patients newly exposed to fostamatinib in Study 049\* is consistent with Study 047 and 048:

- 43 placebo non-responders transitioned from Study 047 and Study 048
- Of those, 36 have been treated with fostamatinib for more than 12 weeks
- A stable platelet response was achieved by  $6/36 = 17\%$ ,  $p=0.01$
- This is consistent with the 18% response rate in Study 047 and 048

\*Data cut-off: June 2016



# **Fostamatinib FIT Phase 3 Program Commentary**

James Bussel, MD  
Principal Investigator



# Regulatory Path Forward

## In Next 3 Months

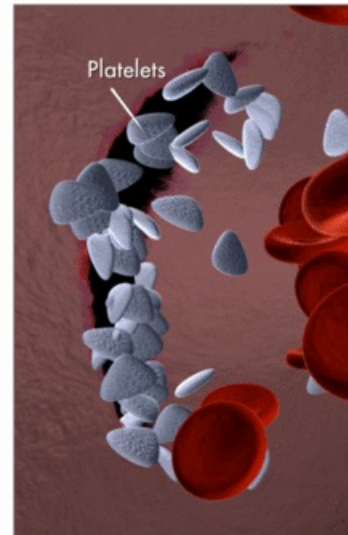
- Input from regulatory consultants
- Feed-back from FDA

## Pending FDA's Feed-back

- NDA submission still planned for Q1 2017

## Pending FDA's Acceptance of the NDA submission:

- Potential FDA Advisory Committee Review (Q4 2017)
- FDA decision on approval anticipated in Q1 2018



# Conclusions

## Key Findings:

- All FIT studies demonstrate consistent clinical benefit in treating ITP
- Safety profile consistent with prior experience. AEs related to GI were most frequent. AEs were generally mild or moderate.
- Positive Risk/Benefit

## For Patients who met Primary Endpoint:

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

## Attractive option for ITP patients

➤ Attractive opportunity for Rigel



# Q&A