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Rigel Q4 & YE 2017 Conference Call

March 6, 2018

2:00pm PT / 5:00pm ET

Introduction & Safe Harbor Statement

Dolly Vance

Executive Vice President, Corporate Affairs and General Counsel

Participants

Rigel Senior Management:

- **Raul Rodriguez** – President and Chief Executive Officer
- **Anne-Marie Duliege, MD** – Executive Vice President and Chief Medical Officer
- **Eldon Mayer** – Executive Vice President and Chief Commercial Officer
- **Dolly Vance** – Executive Vice President, Corporate Affairs and General Counsel
- **Nelson Cabatuan** – Vice President, Finance

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Forward Looking Statement

In both these slides and during the conference call accompanying these slides, Rigel management will be making some forward-looking statements, including statements relating to the timing of initiation, enrollment and results of clinical trials; the results of the FDA's review of Rigel's NDA for fostamatinib in patients with chronic or persistent ITP; Rigel's ability to transition to an organization prepared to launch its first commercial product; Rigel's belief that fostamatinib may be an important alternative for patients with ITP or AIHA; Rigel's evaluation of ex-U.S. partnerships for fostamatinib and other partnering opportunities across its pipeline; the timing and outcome of Rigel's interactions with the FDA and other regulatory agencies; the sufficiency of Rigel's cash, cash equivalents, and short-term investments; and the management and advancement of Rigel's clinical programs.

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 Annual Report on Form 10-K for the year ended December 31, 2017. Rigel expressly disclaims any obligation or undertaking to update the forward-looking statements discussed in this call.

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Company Overview

Raul Rodriguez
President and Chief Executive Officer

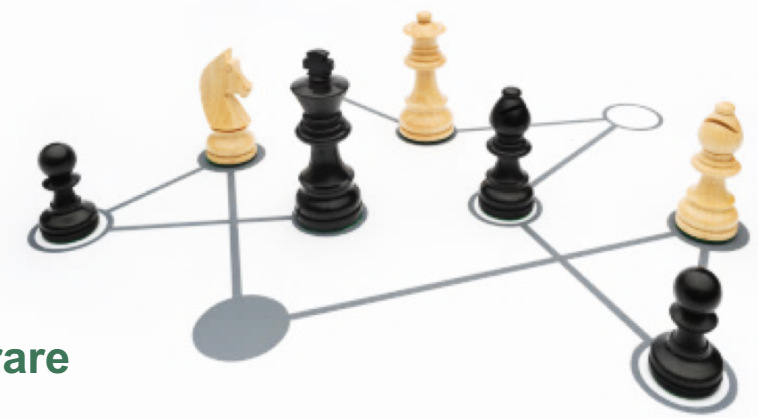
Agenda

1. Regulatory and Clinical Update
2. Commercial Readiness
3. Financial Update

Executing Our Business Strategy

Deliver Commercial Product: Fostamatinib

- Phase 3 Results in ITP ✓
- Submit US NDA in ITP ✓
- Manage US NDA to Approval
- Commercialize in US
- Expand Opportunity – AIHA ✓ and IgAN



Therapeutic focus on immune, heme-onc, and rare diseases: Next up IRAK1/4

Monetize select pipeline assets via partnerships

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Fostamatinib: Fundamental Target → Broad Opportunity

Fundamental Target → Broad Opportunity

- An oral inhibitor of SYK, a fundamental signaling target in many autoimmune diseases
- Addresses the underlying aberrant antibody destruction which is the basis of many of autoimmune diseases
- A first in class!

Rigel Currently Focused on 3 Orphan Autoimmune Diseases

- ITP: Antibody-mediated platelet destruction
- AIHA: Antibody-mediated red blood cell destruction
- IgA Nephropathy: Antibody creation of IgA1 immune complexes

Opportunities in Numerous Additional Autoimmune Diseases

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Fostamatinib: Building and Leveraging Opportunities

Orphan Autoimmune Diseases with Unmet Medical Need and/or Limited Treatment Options

- ITP: Only TPO-RA agents approved
- AIHA: none approved
- IgAN: none approved
- Exploring others

Evidence of clinical efficacy:

- ITP: Timely, Robust and Enduring Platelet Response in Phase 3
  - Stable Response = 18%
  - Clinically-Relevant Platelet Response Rate = 43% (post-hoc)
- AIHA: Response Rate = 53% in Stage 1 of Phase 2 POC study
- IgAN: Interim results suggest potential clinical benefit measured by proteinuria and histology

Leverage our commercial capabilities broadly

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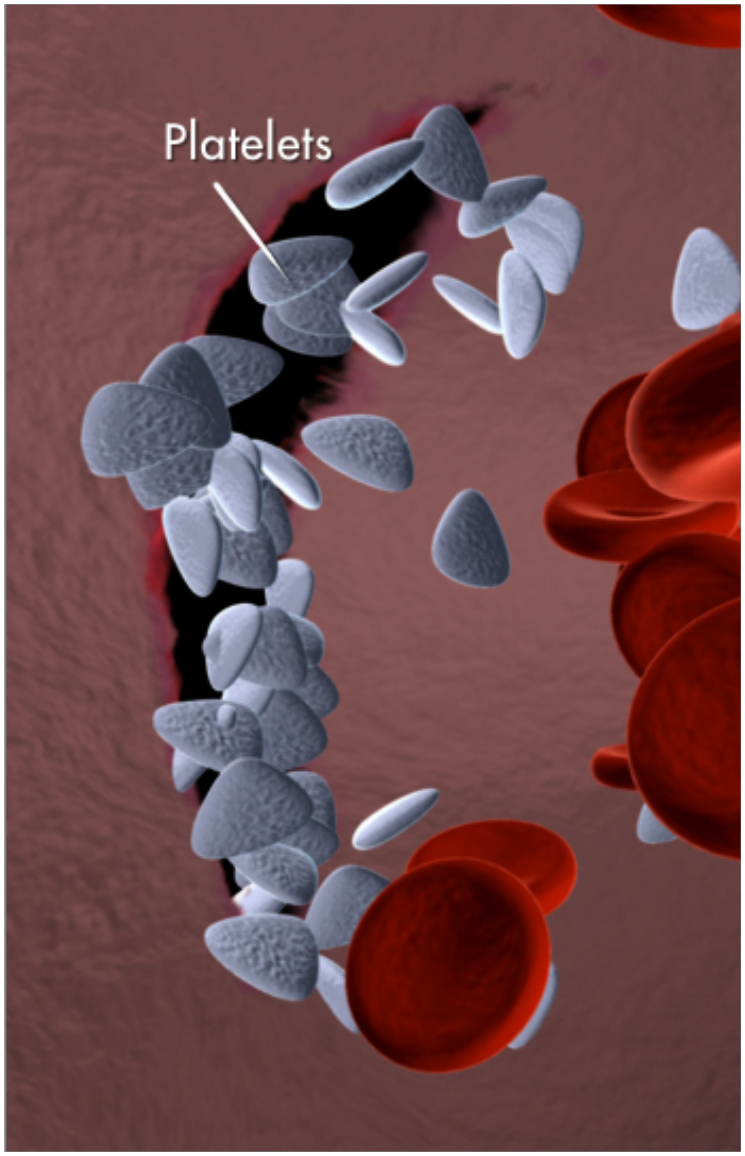
Clinical & Regulatory Update

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

ITP Background

Significant unmet need:

- Characterized primarily by destruction of platelets
- Increased risk of severe bleeding events
  - Can result in serious medical complications or even death
- Heterogeneous patient population, difficult to predict effective therapies
- Limited treatment options
  - No treatment option available to treat the underlying disease



Clinical Summary

The combined Phase 3 studies with fostamatinib demonstrate consistent clinical benefit in treating ITP

- Stable response = ~ 18%
- Clinically-relevant platelet response rate = 43% (post-hoc)

For patients with platelet increase, the response was:

- Rapid
- Robust
- Enduring

Safety profile consistent with prior experience. AEs were generally mild or moderate. AEs related to GI, hypertension and liver toxicity were most frequent.

If approved, fostamatinib would be an important treatment option for patients with ITP

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Fostamatinib in ITP
Regulatory Summary - US

Orphan designation granted in the US - August 2015

NDA submitted - April 2017

NDA submission accepted by FDA - June 2017

Mid-cycle communication: no advisory meeting planned, positive interactions - September 2017

Late-cycle communication - January 2018

PDUFA date - April 17, 2018

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Fostamatinib in ITP
Regulatory Summary - EU

National Scientific Advice meeting with MHRA – December 2017

- **Positive feedback: no objections to filing MAA**

Preparing MAA enabling submission to the EMA; plan to file an MAA in EU in Q4 2018

Autoimmune Hemolytic Anemia (AIHA) Opportunity

Significant unmet medical need:

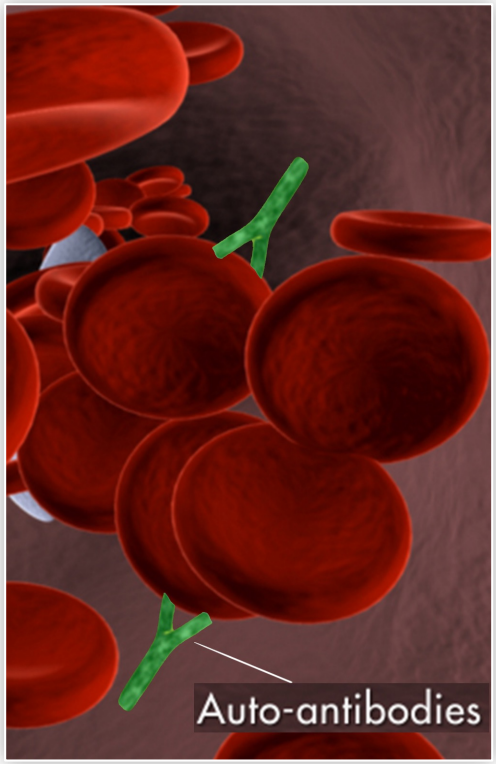
- Severe debilitating anemia
- ~40,000 adult patients in the US
- ***No approved therapies***

Unique MOA may address the cause of AIHA

- Clear treatment effect in ITP is an indicator of the value of SYK inhibition to treat AI cytolytic diseases

Objective endpoint (change in hemoglobin)

Large safety database (>5,000 patient-years)



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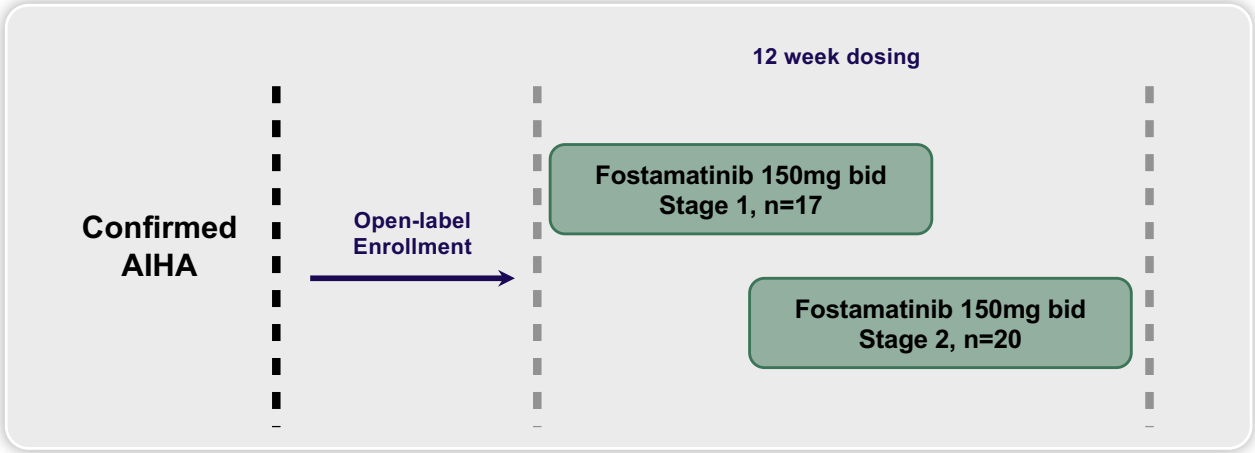
Fostamatinib - Phase 2
SOAR Clinical Trial in AIHA



On a top-line, preliminary basis, the Phase 2 study achieved the pre-specified primary efficacy endpoint for Stage 1:

- Study design: open-label, Simon two-stage design
- Primary endpoint: response rate in the first 12 weeks without the need for rescue therapy
- Response: hemoglobin ≥ 10 g/dl and ≥ 2 g/dl increase from baseline

Fostamatinib - Phase 2 in AIHA
Demographic and Baseline Characteristics

	Fostamatinib 150 mg *bid* N=19
Age [years] – Median (Range)	58 (27, 88)
< 65 years	11 (58%)
Female	11 (58%)
White	15 (79%)
Body Mass Index – Median (Range)	26 (20, 46)
Hemoglobin [g/dL] – Median (Range)	9.1 (6.8, 9.9)

Fostamatinib - Phase 2 in AIHA
Hemoglobin Response

17 evaluable patients with at least one post-baseline hemoglobin*

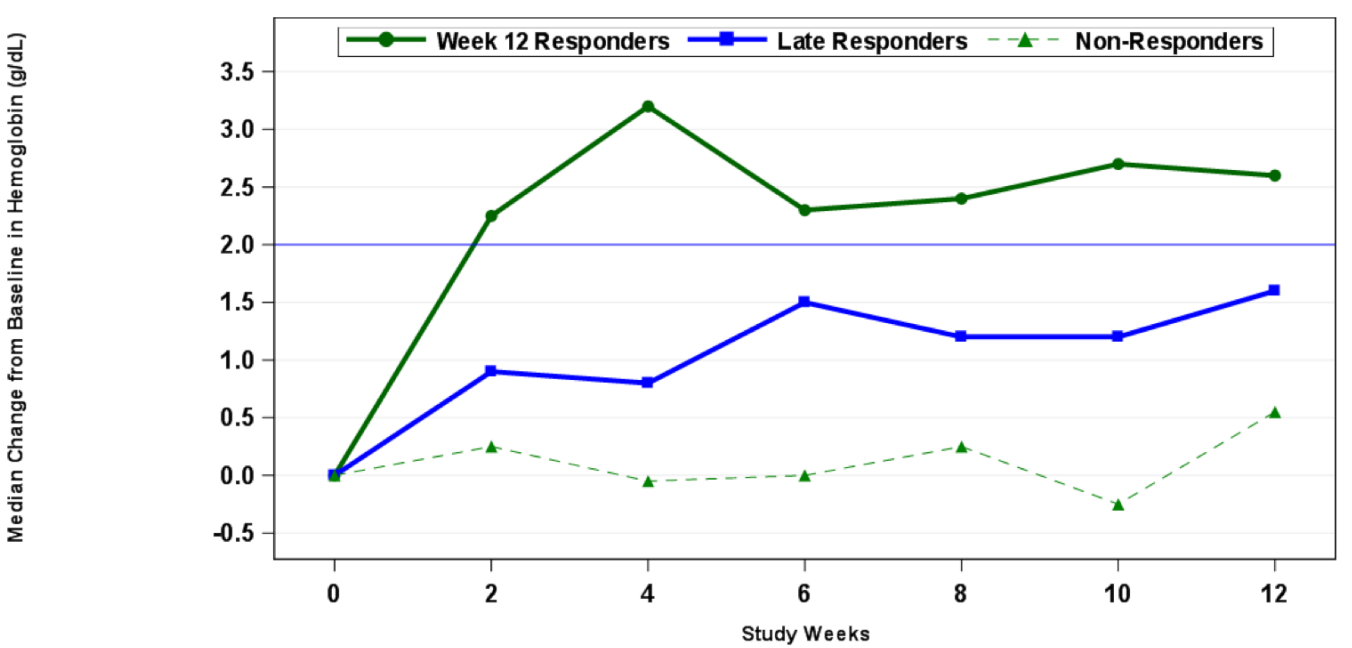
- **53%** of patients had a clinical response to fostamatinib treatment (9/17)
  - Six patients had a clinical response during the 12 week evaluation period
  - An additional three patients met the response criteria in the extension study (after 12 weeks of dosing)

A comprehensive analysis of the Phase 2 data will continue

*Preliminary data

Median Change from Baseline in Hemoglobin – 12 Week Duration

Efficacy Evaluable Population



Population By Week (N)							
Week 12 Responders	6	6	6	6	5	5	5
Late Responders	3	3	3	3	3	3	3
Non-Responders	8	8	8	7	8	8	8

Fostamatinib - Phase 2 in AIHA
No New Safety Signals

Adverse event profile consistent with fostamatinib safety database

Three patients with serious adverse events (SAEs), all assessed as non-treatment related by investigators

- One patient recovered and continued on treatment
- Two patients had SAEs resulting in fatalities
  - One with skin necrosis and infection (immunosuppressed due to steroids)
  - One elderly patient with pneumonia (immunosuppressed due to steroids and prior CLL)

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Summary and Next Steps

Summary:

- 53% response rate (9 out of 17 evaluable patients)
- No new safety signals: no drug-related SAEs
- Primary endpoint in Stage 1 achieved
- Orphan disease designation granted (Jan ’18)

Next Steps:

- Stage 2 enrollment in progress (20 patients)
- Regulatory strategy for optimal path to approval

IgA Nephropathy (IgAN) Opportunity

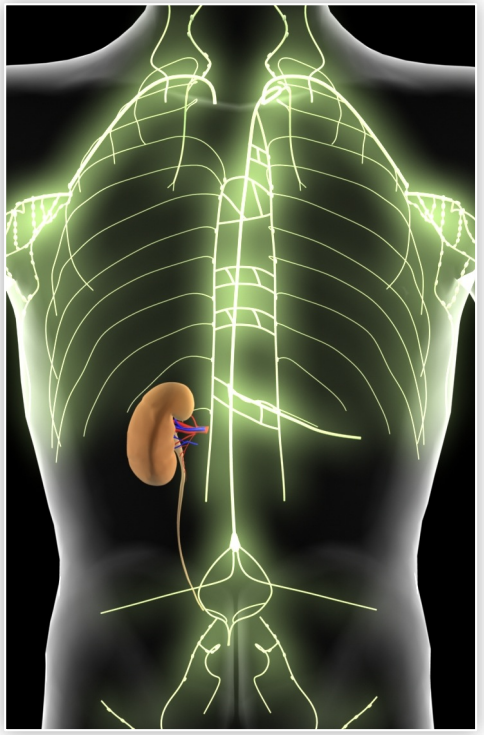
Significant medical need:

- Most common primary glomerulonephritis
- An estimated 82 – 165K cases in the US
- 25% progress to end stage renal failure
- No approved therapies

Strong preclinical data

Large safety database

Objective clinical endpoint (proteinuria)



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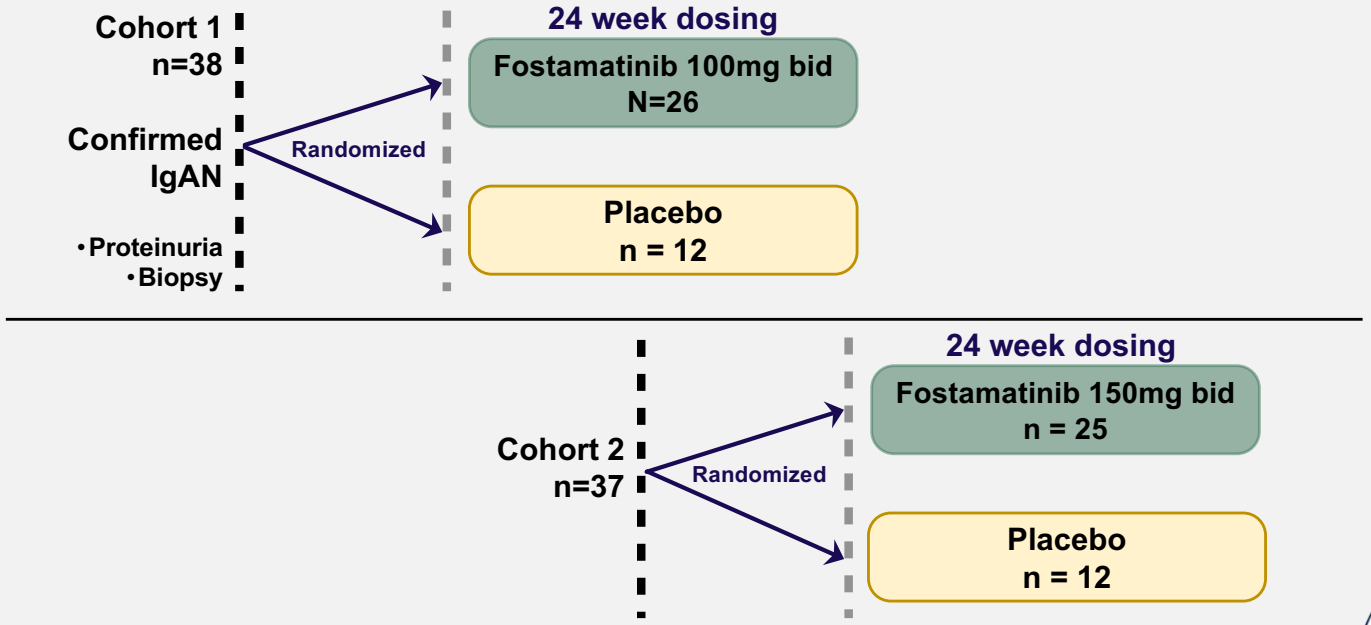
Fostamatinib - Phase 2
Clinical Trial in IgA Nephropathy



Primary endpoint:

Mean change of proteinuria from baseline to 24 weeks

Timeline:

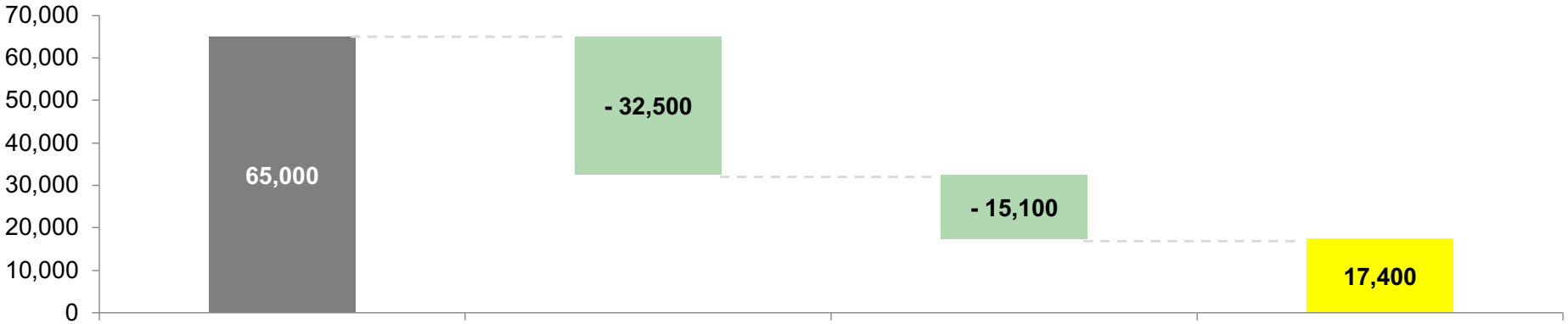
Cohort 1 results in 2017. Cohort 2 completed enrollment and results expected in April 2018

Commercial & Market Perspective

Eldon Mayer

Executive Vice President & Chief Commercial Officer

Tavalisse™ Market Potential Opportunity



Total estimated adult chronic ITP patients

~50% cITP patients are treated in a given year. Patients cycle in and out of this group

~23% cITP patients are treated with steroids only

27% of patients are treated with later line therapies, e.g. rituximab, TPO's, splenectomy, immunosuppressants and cytotoxics

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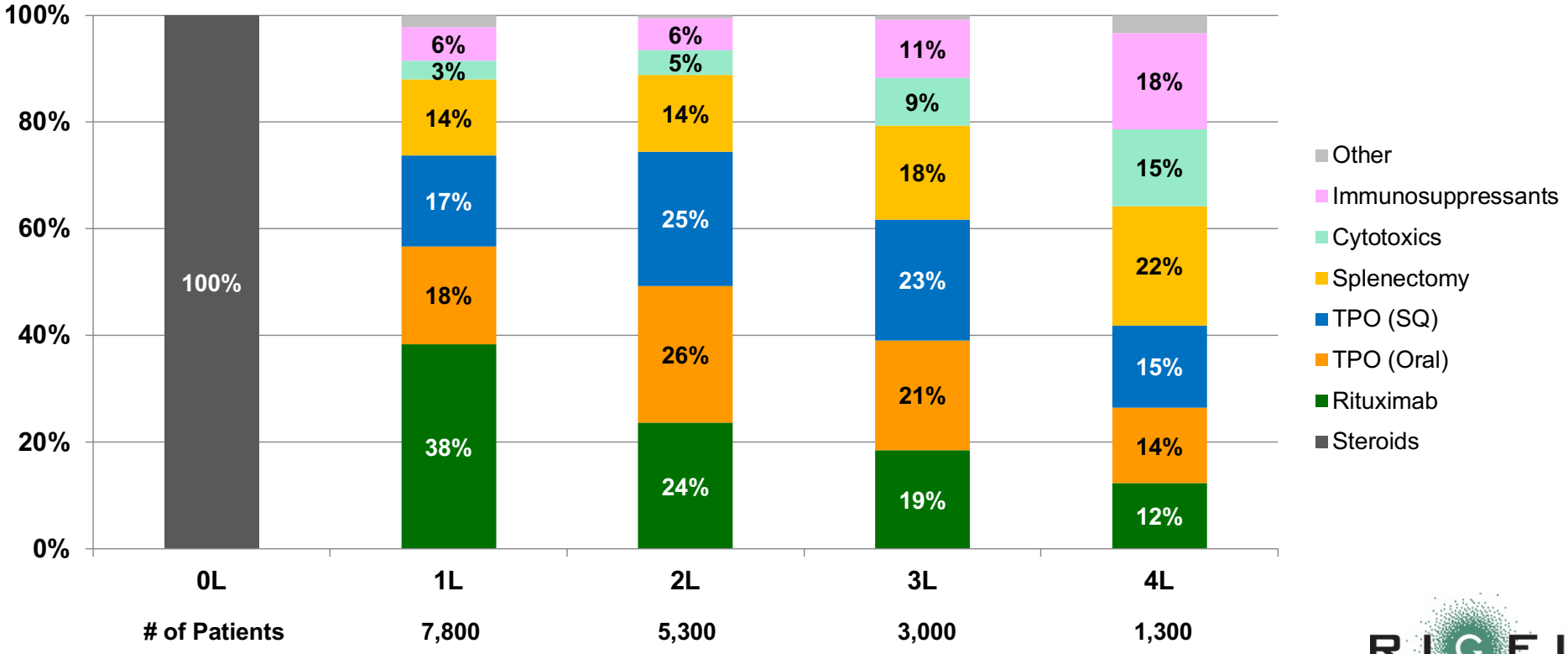
The ITP treatment paradigm is fragmented and sequencing of treatments is highly variable



Cycling on and off available treatments throughout all lines of therapy is common

We are actively preparing for launch with these ongoing activities

Marketing	Market Access	Business Operations	Sales
▪ Positioning and Messaging ▪ HCP/KOL Engagement ▪ Promotional materials	▪ Distribution ▪ Payer engagement ▪ Patient Services and Support Programs ▪ Pricing	▪ Market assessment ▪ Market Research ▪ KOL Landscape ▪ Systems & Data acquisition	▪ Customer and Market analysis ▪ Territory Structure ▪ Sales Force Recruiting, Hiring and Training

Financial Overview

Nelson Cabatuan

Vice President, Finance

Rigel Financial Overview

- **$115.8M cash as of December 31, 2017**
- **Sufficient cash to fund operations into 2019**

Rigel Opportunity (Near-Term)

- Fostamatinib
  - Broad, fundamental mechanism capable of addressing numerous autoimmune diseases
    - ITP at NDA review
    - AIHA POC results
    - Others underway/under evaluation
  - Provides pivot product to transition into commercial stage Company
    - Build and then leverage commercial organization across synergistic physician audiences

- Next up: R835, IRAK ¼ inhibitor to start clinical testing
  - Broad, fundamental immune signaling mechanism
  - Able to address numerous immune-based diseases

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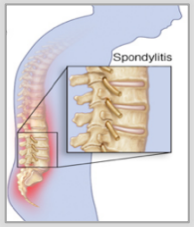
Next Up: IRAK1/4 Inhibitor

Potential Clinical Indications

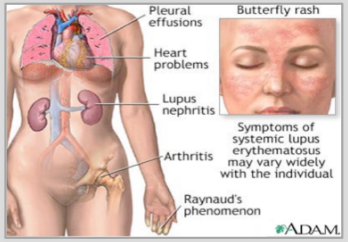
IRAK1/4 Inhibitor

Ankylosing Spondylitis



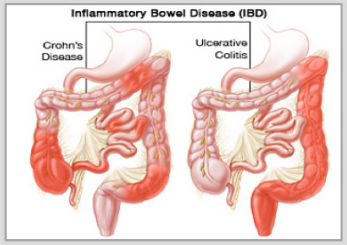
Anti-IL12/23

Lupus



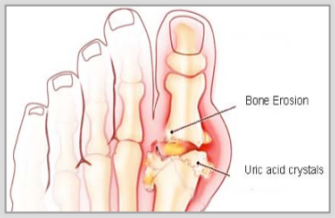
Myd88-/- protects

IBD



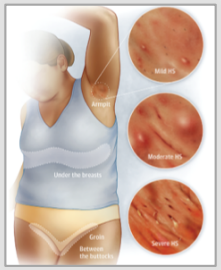
IL23-/- protects

Gouty Arthritis

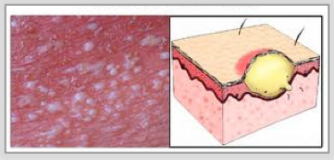


IL1 blockade

Hidradenitis suppurativa



Psoriasis

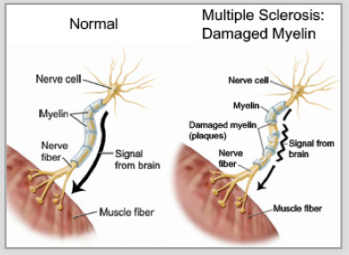


IL-36RN-/-

Psoriatic Arthritis

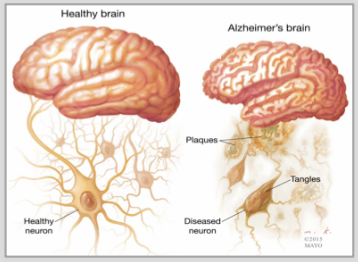
Anti-IL12/23

Multiple Sclerosis



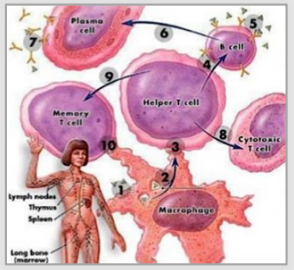
Myd88-/- protects

Alzheimers



IRAK4-KD protects

Dermatomyositis



Inflammasome

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Q&A


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