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David Zaccardelli, PharmD
Chief Executive Officer

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New Team Members

- David Zaccardelli, PharmD joins as President & CEO (12/17/18)

United Therapeutics | cempra | GlaxoWellcome | BAUSCH + LOMB

- Jason Hoitt joins as Chief Commercial Officer (12/17/18)

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Key Business Highlights At-A-Glance

DOPTELET® 	✓ DOPTELET®, a second generation thrombopoietin receptor agonist used in the treatment of thrombocytopenia (i.e., low platelet counts) ✓ DOPTELET has demonstrated robust efficacy in both the acute and the chronic setting ✓ Patent until May 2025; pending patent term ext. app. to extend patent until 10/2029
LAUNCH 	✓ DOPTELET approved May 21, 2018 for the treatment of thrombocytopenia in adult patients with chronic liver disease (CLD) scheduled to undergo a procedure ✓ DOPTELET launched in June 2018 ✓ Partnership with Salix positions DOPTELET for significantly increased market presence in 2019
PIPELINE 	✓ Supplemental NDA accepted for review by the FDA for the treatment of Chronic Immune Thrombocytopenia (ITP) with a target PDUFA date of June 30, 2019 ✓ Well differentiated vs Promacta® (eltrombopag), Nplate® (romiplostim) and Tavalisse™ (fostamatinib) ✓ Chemotherapy Induced Thrombocytopenia (CIT) study remains on track for 1H 2020
FINANCIALS 	✓ $122.0M cash and equivalents on hand (*as of September 30, 2018*) ✓ $12.6M used to fund clinical and commercial operations for Q3, 2018 ✓ $20M long-term debt

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May 21st, 2018: DOPTELET Receives FDA Approval

DOPTELET (avatrombopag) is a thrombopoietin receptor agonist indicated for the treatment of thrombocytopenia in adult patients with chronic liver disease who are scheduled to undergo a procedure.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS
DOPTELET is a thrombopoietin (TPO) receptor agonist and TPO receptor agonists have been associated with thrombotic and thromboembolic complications in patients with chronic liver disease. Portal vein thrombosis has been reported in patients with chronic liver disease treated with TPO receptor agonists. In the ADAPT-1 and ADAPT-2 clinical trials, there was 1 treatment-emergent event of portal vein thrombosis in a patient (n=1/430) with chronic liver disease and thrombocytopenia treated with DOPTELET.
Consider the potential increased thrombotic risk when administering DOPTELET to patients with known risk factors for thromboembolism, including genetic prothrombotic conditions (Factor V Leiden, Prothrombin 20210A, Antithrombin deficiency or Protein C or S deficiency).
DOPTELET should not be administered to patients with chronic liver disease in an attempt to normalize platelet counts.

CONTRAINDICATIONS:
None

ADVERSE REACTIONS
Most common adverse reactions (≥ 3%) are: pyrexia, abdominal pain, nausea, headache, fatigue, and edema peripheral.
Please see full Prescribing Information for DOPTELET (avatrombopag) www.doptelet.com

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Initial Observations on Opportunities, Improvements and Next Steps

- Gained substantial insight into the CLD market

- ITP Large Commercial Opportunity
  - Established/well-defined market: $800M U.S. ($1.5B globally)
  - Target PDUFA date for ITP sNDA (6/30/19)

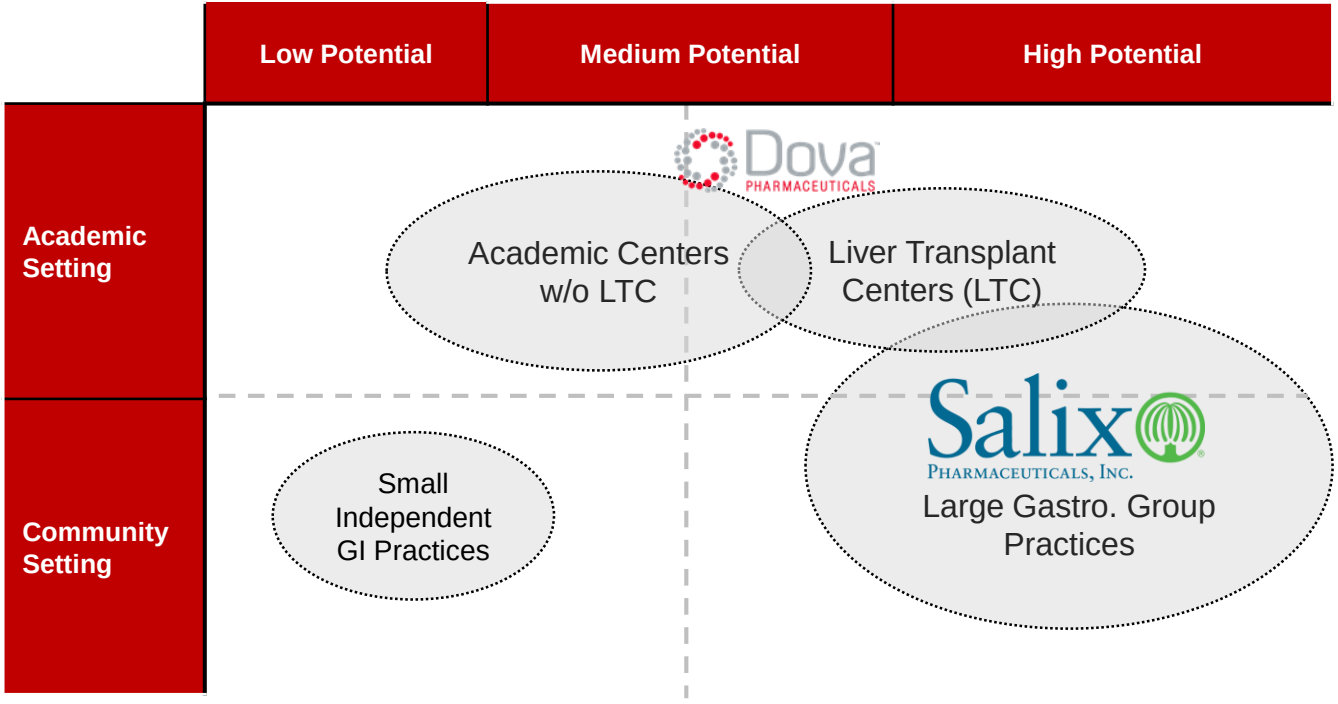
- CIT offers notable, differentiated market opportunity

- Key near-term objectives:
  - Improve CLD commercial performance
  - ITP approval/launch readiness
  - Complete CIT clinical trial by 1H 2020

Significant Upside Potential for DOPTELET in CLD

- Unmet medical need for DOPTELET
- Shift in physician mindset/treatment paradigm
- Requires change in practice/behavior
  - Lead-time to procedure
  - Platelet transfusion → tablet administration
  - New treatment paradigm for payors
- Comprehensive commercial review underway
  - Sales force alignment/deployment
  - Customer call strategy/Salix alignment
  - Marketing
  - Market access, pricing strategy
- Salix partnership has potential to provide significant launch momentum

Market Segmentation



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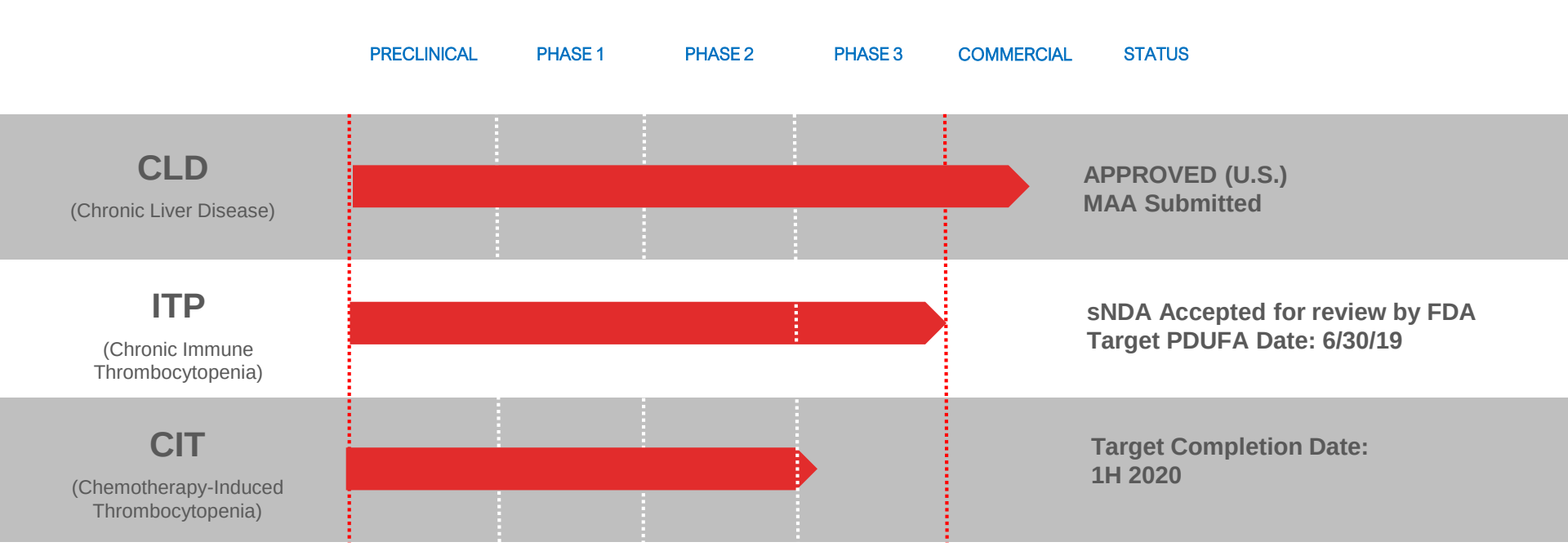
DOPTELET: Potential to Address Various Types of Thrombocytopenia



DOPTELET: A Differentiated Therapy for a Large Market--Chronic ITP

- Approved therapies have established annual cITP market at $800 million (U.S.)/$1.5 billion (global)
- Well-established treatment paradigm for TPO agonists
- DOPTELET effective and well tolerated in clinical trials
- Differentiated/preferred profile

DOPTELET Efficacy and Safety Data in Patients with ITP

- Pivotal Efficacy Data from Phase 3 Study 302 (n=49)
  - Primary endpoint: cumulative number of weeks of platelet response – avatrombopag superior relative to placebo ($p<0.0001$)
  - 1st secondary efficacy endpoint: superior platelet response at day 8 relative to placebo ($p<0.0001$)
  - Positive trend favoring avatrombopag for 2nd secondary endpoint, proportion of subjects with a reduction in use of concomitant ITP medications from baseline

- Primary Safety Data from studies of avatrombopag in patients with ITP
  - 128 patients with ITP treated with avatrombopag
  - Median duration of exposure 204 days, with 63% treated for at least 180 days
  - Exposure-adjusted adverse event rates comparable to placebo
    - No data clinically significant hepatotoxicity or increased incidence of thromboembolic or bleeding events

Jurczak W, et al. *Br J Haematol*. 2018. Published Online, https://doi.org/10.1111/bjh.15573
Internal Company data and analysis on file

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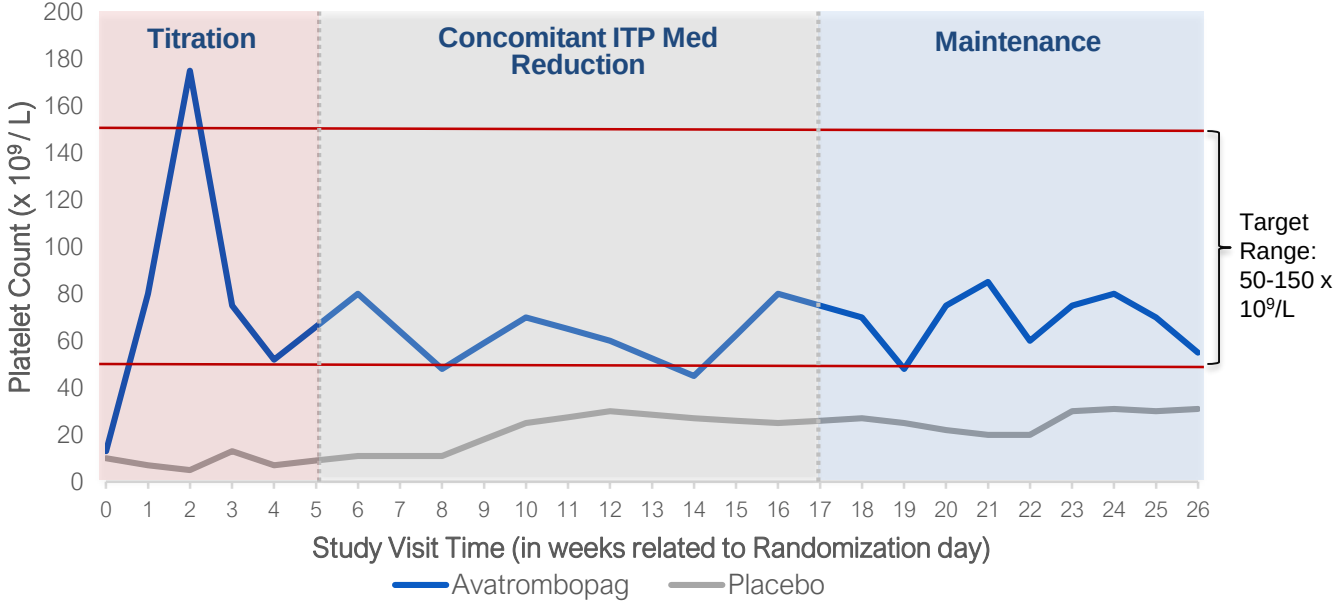
DOPTELET Phase 3 ITP Efficacy Data: Median Platelet Count Over Time

Avatrombopag maintained the target platelet count (50 to <150×10^9/L) over the 6-month treatment period



Jurczak W, et al. *Br J Haematol*. 2018. Published Online, https://doi.org/10.1111/bjh.15573

If Approved by FDA, we Believe DOPTELET is Well-Differentiated

	HEPATOTOXICITY	DOSING	FOOD EFFECT
Doptelet (avatrombopag) tablets	 NO HEPATOTOXICTY	 ONCE DAILY ORAL DOSING	 CONVENIENT ADMINISTRATION WITH FOOD
PROMACTA® (eltrombopag)	 "PROMACTA MAY INCREASE THE RISK OF SEVERE AND POTENTIALLY LIFE-THREATENING HEPATOTOXICITY"	 ONCE DAILY ORAL DOSING	 TAKE ON AN EMPTY STOMACH (1 HOUR BEFORE OR 2 HOURS AFTER)
Nplate® romiplostim injection	 NO HEPATOTOXICTY	 SUBCUTANEOUS	 N/A
Tavalisse™ (fostamatinib disodium hexahydrate) tablets	 ELEVATED LIVER FUNCTION TESTS CAN OCCUR "MONITOR LIVER FUNCTION TESTS MONTHLY DURING TREATMENT".	 TWICE DAILY ORAL DOSING	 MAY BE TAKEN WITH OR WITHOUT FOOD

Note: DOPTELET's currently approved indication is the treatment of thrombocytopenia in adult patients with chronic liver disease scheduled to undergo a procedure; Promacta's indication is chronic ITP, pediatric chronic ITP, severe aplastic anemia and chronic hepatitis C; Nplate's indication is chronic ITP, Tavalisse's indication is chronic ITP DOPTELET has not yet been approved by the FDA for the treatment of chronic ITP. This represents data from the studies completed to date

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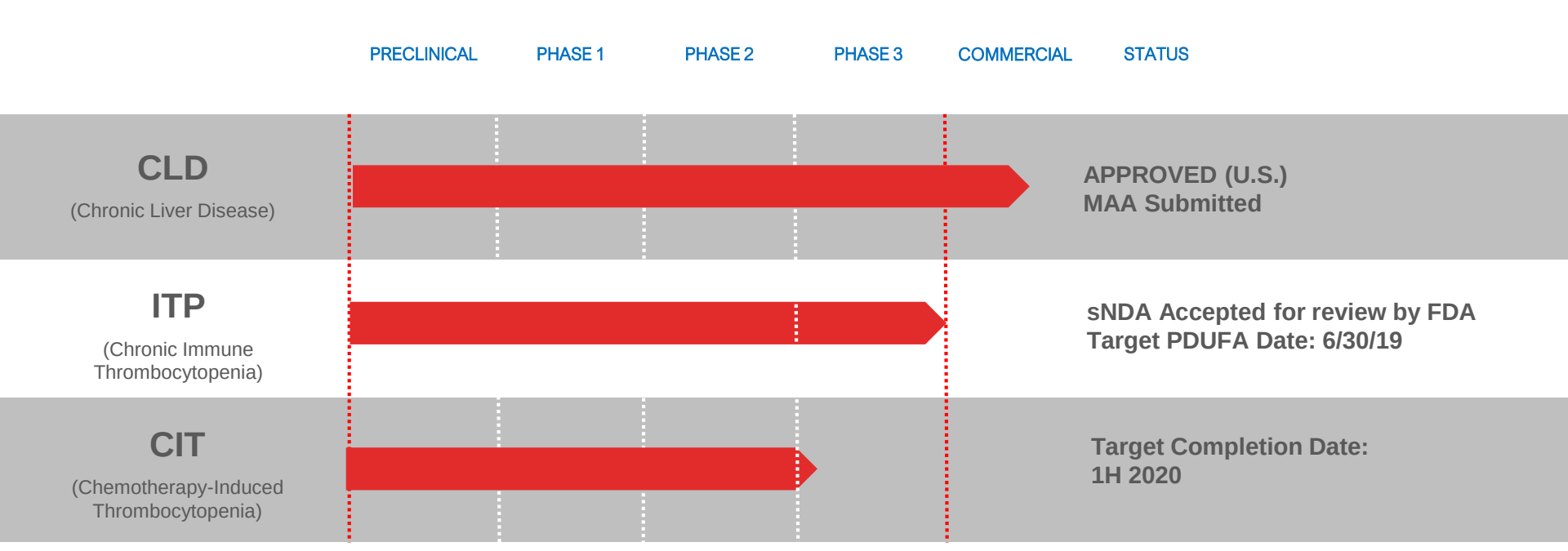
DOPTELET: Potential to Address Various Types of Thrombocytopenia



Potential Fit for DOPTELET in CIT Standard of Care

DOPTELET has the potential to address a significant unmet medical need for patients with CIT

Type of Cancer	Regime	Rate of TCP*
NSCLC	Platinum/Gemcitabine	50.5%
Ovarian	Platinum/Taxane	45.6%
Bladder	Platinum/Gemcitabine	57.0%

1. Chemotherapy Dose Reduction
2. Cycle Delay / Cancellation
3. Platelet Transfusion
4. Doptelet® (avatrombopag) tablets

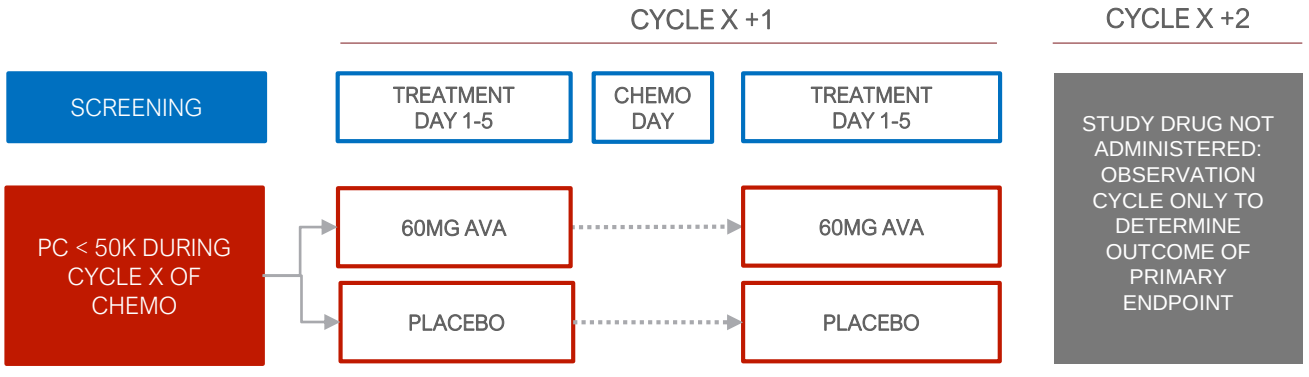
* Zhang X, et al. *Cochrane Database Syst Rev*. 2017;11:CD02035. Hitron A, et al. *J Oncol Pharm* Prac 2011 Dec;17(4):312-9

Chemotherapy Induced Thrombocytopenia (CIT) Phase 3 Design

STUDY DESIGN

Phase 3 randomized, double-blind, placebo-controlled study of the efficacy and safety of oral avatrombopag in subjects with active non-hematological cancers (i.e., ovarian, NSCLC, bladder) who develop CIT (platelet count <50K / µL in the previous cycle of chemo)

N = 120 (2:1 RDZ)



Global Rights + Significant Patent Life for DOPTELET

- MAA pending for the treatment of adults patients with CLD who are scheduled to undergo a procedure - expected action date in Q3 2019

- EU + RoW partnership opportunities across indications provide opportunities for additional revenue + non-dilutive capital

- Robust IP, including composition of matter through 2025 (U.S.)
  - Potential for patent term extension through 2029
  - EU patent protection into 2032

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Near-term Revenue Growth Potential + Label Expansion in 1H 2019

EXPERIENCED NEW LEADERSHIP

- David Zaccardelli, PharmD joins as CEO (12/17/18)
- Jason Hoitt joins as CCO (12/17/18)
- Proven leaders with track records of development and commercial success
- Comprehensive commercial review underway

ROBUST PIPELINE

- sNDA for ITP ($1.5 billion global market/$800 M U.S. market) accepted for review with a target PDUFA date of June 30, 2019
- CIT Phase 3 study initiated in 2Q 2018, expected completion 1H 2020

FAVORABLE IP

- Composition of matter patents expire in 2025 with potential patent term extension to 2029
- EU patent protection through August 2032

STRONG CASH POSITION

- $122.0 M cash and equivalents as of 9/30/18
- $20 M long-term debt
- $12.6 M used to fund clinical and commercial operations for Q3 2018

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