



**First Quarter 2017 Financial and Operational Results**  
May 8, 2017

**Accelerating Drug Discovery to Transform Patients' Lives**

# Earnings Call Agenda

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## Introduction

- Todd James, IRC  
Senior Director, Investor Relations and Corporate Communications

## Q1 2017 Overview

- Habib Dable  
President and Chief Executive Officer

## International MDS Symposium Highlights

- Matthew Sherman, M.D.  
Chief Medical Officer

## Financial Results

- Kevin McLaughlin  
Chief Financial Officer

## Upcoming Milestones and Events

- Habib Dable  
President and Chief Executive Office

## Question & Answer Session

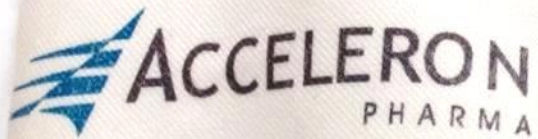
# Acceleron Forward-Looking Statements



This presentation contains forward-looking statements about the Company's strategy, future plans and prospects, including statements regarding the development of the Company's compounds, including sotatercept, luspatercept, dalantercept, ACE-083, ACE-2494, the Company's IntelliTrap™ drug discovery platform, and the Company's TGF-beta superfamily program generally, the timeline for clinical development and regulatory approval of the Company's compounds, the expected timing for the reporting of data from ongoing trials, and the structure of the Company's planned or pending clinical trials. The words "anticipate," "appear," "believe," "continue," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "would," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement. Applicable risks and uncertainties include the risks that the Company's cash, cash equivalents and investments will be insufficient to fund operations into the second half of 2019, that preclinical testing of the Company's compounds and data from clinical trials may not be predictive of the results or success of ongoing or later clinical trials, that data may not be available when the Company expects it to be, that the Company or its collaboration partner, Celgene, will be unable to successfully complete the clinical development of the Company's compounds, that the development of the Company's compounds will take longer or cost more than planned, that the Company or Celgene may be delayed in initiating, enrolling or completing any clinical trials, and that the Company's compounds will not receive regulatory approval or become commercially successful products.

Other risks and uncertainties include those identified under the heading "Risk Factors" included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 1, 2017, and other filings that the Company has made and may make with the SEC in the future. The forward-looking statements contained in this presentation reflect the Company's current views with respect to future events, and the Company does not undertake and specifically disclaims any obligation to update any forward-looking statements.



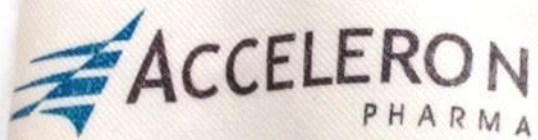
**Habib Dable**  
*President and CEO*

# Operational Clinical Pipeline Performance

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- Enrolling Phase 3 studies with luspatercept
  - MEDALIST study in lower-risk myelodysplastic syndromes (MDS)
  - BELIEVE study in transfusion dependent beta-thalassemia
- 14<sup>th</sup> International Symposium on MDS Phase 2 Clinical Presentation:
  - First-line, Lower-risk MDS Phase 2 results
- Announced ACE-083 Phase 2 trial planned in CMT patients
- FSHD Phase 2 Part 1 enrollment and treatment ongoing



**Matthew Sherman, M.D.**  
*Chief Medical Officer*

# 14<sup>th</sup> International Symposium on MDS



## Oral presentation:

- Luspatercept Response in New Subpopulations of Patients with Lower-Risk Myelodysplastic Syndromes (MDS): Update of the PACE Study

## Poster presentation:

- Pharmacokinetics and Exposure-Response Relationship of Luspatercept in Patients with Anemia Due to Lower-Risk MDS: Preliminary Results from Phase 2 Studies

# Luspatercept Lower-Risk MDS Clinical Trials Overview



## Phase 2

### PACE-MDS

Current Phase 2 study has been expanded to include lower-risk MDS patient subgroups excluded from MEDALIST including ESA-naïve who are:

- RS+ and EPO  $\leq$ 200 IU/L
- RS- and any EPO level

NCT01749514; NCT02268383

## Phase 3



### MEDALIST

An ongoing Phase 3 study of lower-risk MDS patients who are:

- Regularly transfused
- RS+
- ESA refractory or ineligible (ESA-naïve and EPO >200 IU/L)

NCT02631070

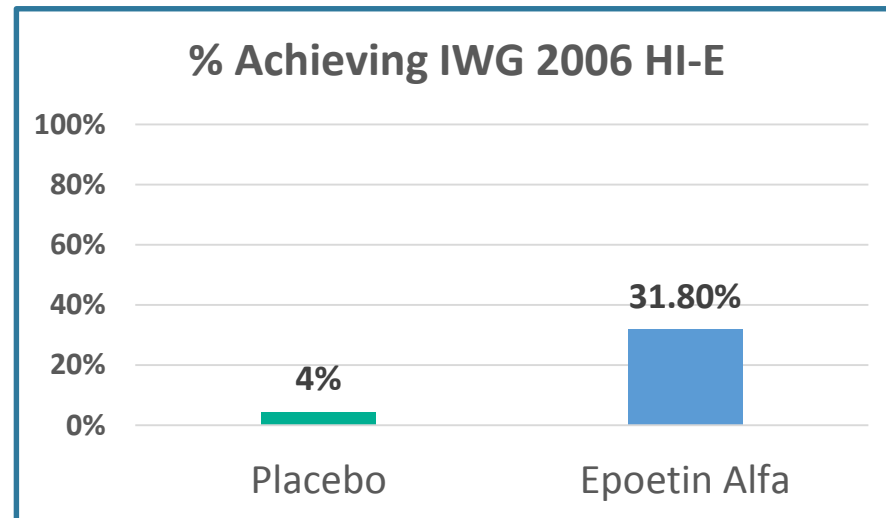
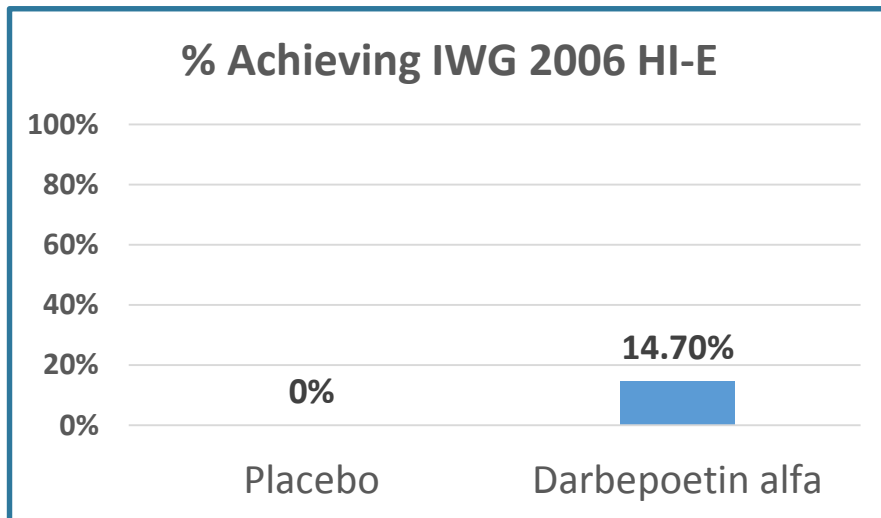


# Phase 3 Response Rates in Lower-Risk MDS with ESAs



## darbepoetin alfa<sup>1</sup> Phase 3 trial in lower-risk MDS

## epoetin alfa<sup>2</sup> Phase 3 trial in lower-risk MDS



### ■ 24-week Double-blind Period:

- All HI-E responders (n=11/75) had baseline EPO levels < 100 mU/mL
- 1/11 responders had a baseline transfusion burden prior to randomization

### ■ 24-week Primary Analysis Period:

- All HI-E responders (n=27/85) had baseline EPO levels < 200 mU/mL
- 18/27 responders had no transfusion burden at baseline (9 had transfusions ≤ 4 RBC units in the 8 weeks prior)

1. ARCADE (20090160): A Phase 3 Randomized Placebo-Controlled Double-Blind Trial of Darbepoetin Alfa in the Treatment of Anemia in Patients With Low or Intermediate-1 Risk Myelodysplastic Syndromes

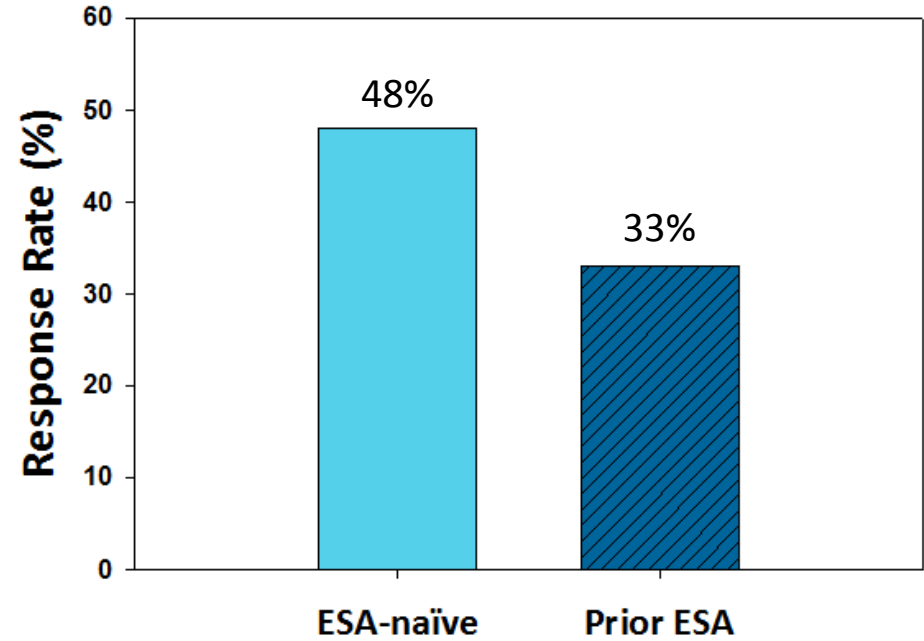
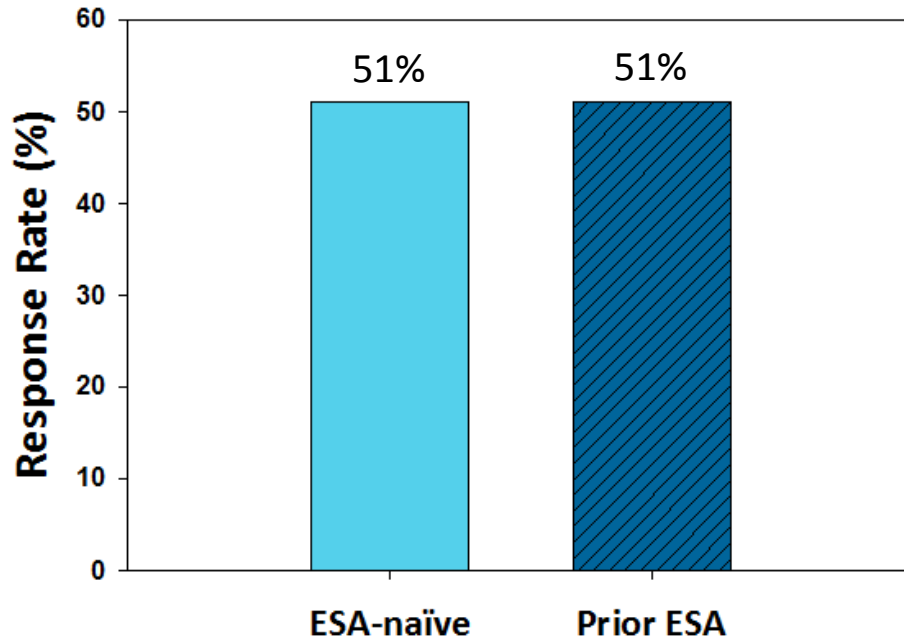
2. Randomized, double-blind, placebo-controlled, multicenter study evaluating epoetin alfa versus placebo in anemic patients with IPSS low-INT1 risk MDS

# Luspatercept Response Rates in Patients by ESA Exposure



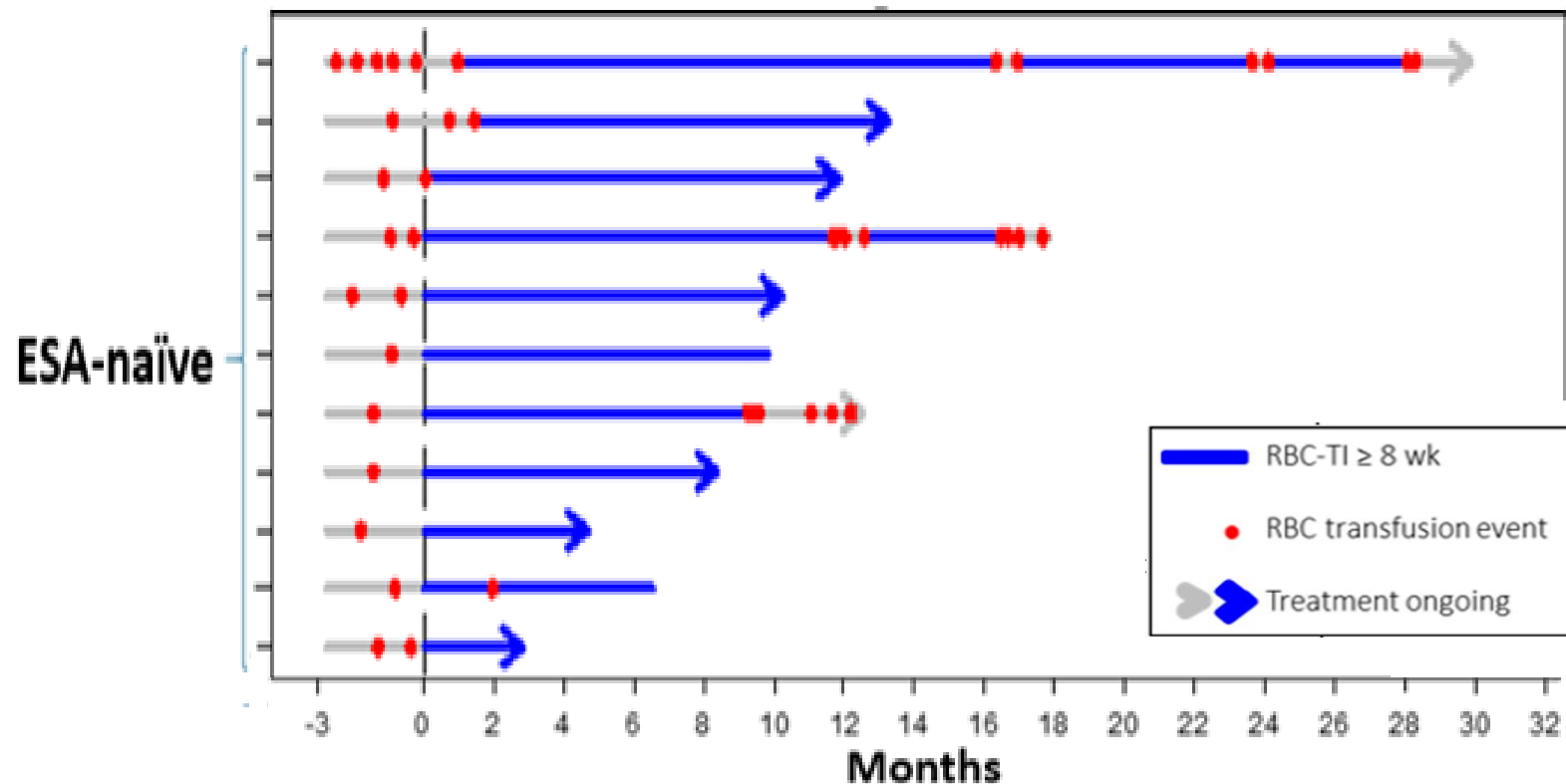
## IWG HI-E

## RBC-TI



	IWG HI-E, n/N N=82	RBC-TI, n/N N=56
<b>All patients</b>	42/82 (51%)	22/56 (39%)
<b>ESA-naïve</b>	20/39 (51%)	11/23 (48%)
<b>Prior ESA</b>	22/43 (51%)	11/33 (33%)

# Pattern of Responses in Patients Achieving at Least 8 Weeks of Transfusion Independence



Patients with Baseline RBC  $\geq$  2 Units

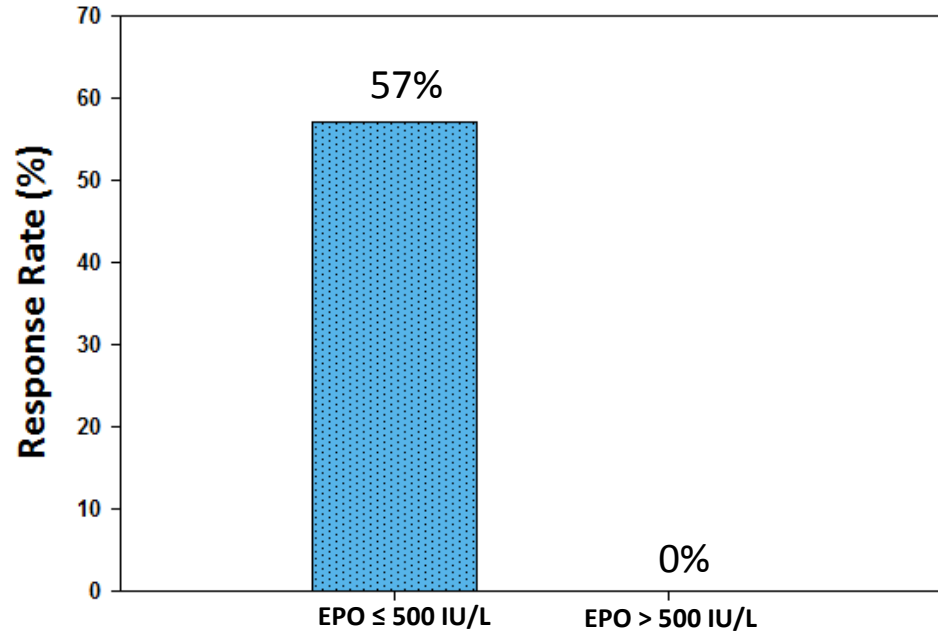
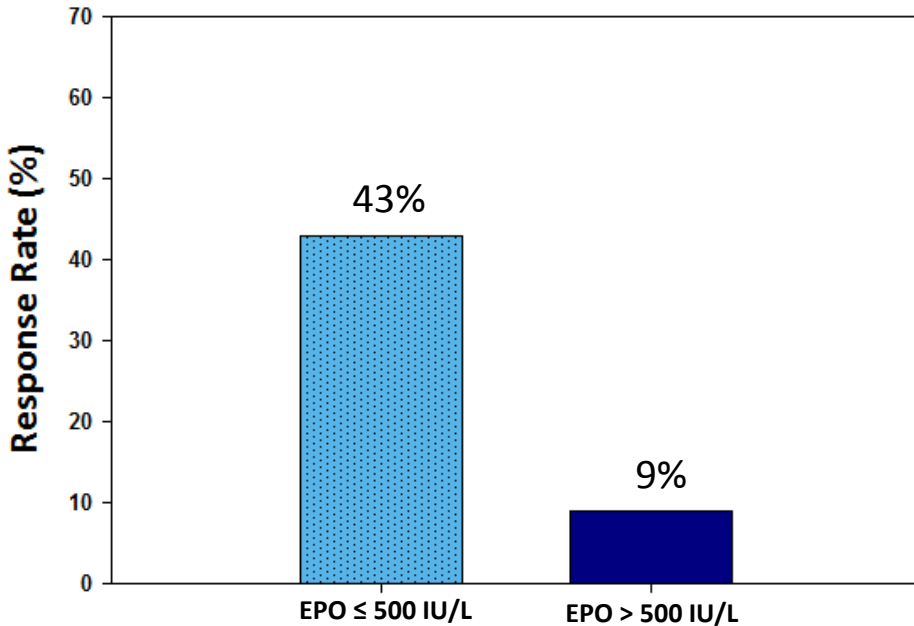
**RBC-TI:** RBC-transfusion independence  $\geq$  8 weeks

# Luspatercept Response Rates in RS- Patients by Baseline EPO Level Regardless of ESA Exposure



## IWG HI-E

## RBC-TI



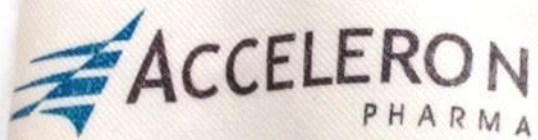
Baseline EPO Level (IU/L)	RS Status	IWG HI-E, n/N (%)	RBC-TI, n/N (%)
EPO ≤ 500	RS-	6/14 (43%)	4/7 (57%)
EPO > 500	RS-	1/11 (9%)	0/9 (0%)

## Phase 2 Update Summary

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- Meaningful responses seen in ESA-naïve patients
- Results in RS- patients are promising, especially in patients with baseline EPO  $\leq$  500 IU/L
- Luspatercept was generally well-tolerated for patients on treatment up to and greater than 24 months
- Phase 2 results support expanding luspatercept development into a Phase 3 trial in first-line, lower-risk MDS patients in early 2018



**Kevin McLaughlin**  
*Chief Financial Officer*

# Q1 2017 Financial Results



<b>Cash</b>	
Cash, cash equivalents and investments	\$213.2M
<b>Revenue</b>	
Collaboration Revenue	\$3.7M
<b>Costs, Expenses and Other Income</b>	
Total Costs and Expenses	\$29.5M
R&D Expenses	\$21.7M
G&A Expenses	\$7.8M
Total other income	\$0.5M
<b>Net Loss</b>	
Net Loss	\$25.4M

Current cash, cash equivalents and investments provide funding into **2H 2019**

# Acceleron Newsflow and Catalysts

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- **Luspatercept**
  - Complete enrollment in MEDALIST and BELIEVE Phase 3 trials in **Q2 2017**
  - Initiate Phase 3 trial in first-line, lower-risk MDS in **early 2018**
  - Initiate Phase 2 trial in myelofibrosis by **YE 2017**
  - Initiate Phase 2 trial in non-transfusion dependent beta-thalassemia by **YE 2017**
  - Additional Phase 2 results at medical conferences in **2017**
  
- **ACE-083**
  - Initial FSHD Phase 2 Part 1 dose-escalation results by **late 2017**
  - Initiate CMT Phase 2 Part 1 dose-escalation in **mid-2017**
  
- **Dalantercept**
  - Report top-line results in the DART Phase 2 study in RCC in **Q2 2017**
  
- **ACE-2494**
  - Initiate Phase 1 healthy volunteer study in **2017**
  
- **Host Research and Development Day in 2H 2017**



# Q1 2017 Results Q&A Session

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**Habib Dable**

President and Chief Executive Officer

**Matthew Sherman, M.D.**

Chief Medical Officer

**Kevin McLaughlin**

Chief Financial Officer

**Chris Rovaldi**

SVP, Operations and Program Mgmt.

**Todd James, IRC**

Investor Relations and Corp. Comm.



# Thank You

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NASDAQ: XLRN