



## Accelerating Drug Discovery to Transform Patients' Lives

4<sup>th</sup> Quarter and Full Year 2016 Financial and Operational Results

March 1, 2017

# Earnings Call Agenda

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## Introduction

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

## Q4 and FY 2016 Overview

- Habib Dable  
President and Chief Executive Officer

## Financial Results

- Kevin McLaughlin  
Chief Financial Officer

## Upcoming Milestones and Events

- Habib Dable  
President and Chief Executive Office

## Question & Answer Session

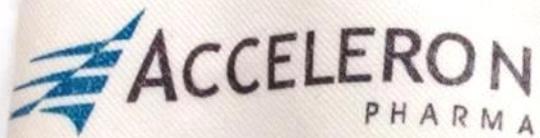
# Acceleron Forward-Looking Statements



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 RS+ myelodysplastic syndromes (MDS)
  - BELIEVE study in transfusion dependent beta-thalassemia
- ASH 2016 Phase 2 Clinical Presentations:
  - Lower-risk MDS Phase 2 extension study
  - First-line ESA naïve MDS Phase 2 data
  - Beta-thalassemia Phase 2 extension study
  - Myelofibrosis investigator sponsored trial results
- ACE-083 Phase 2 trial initiated in FSH muscular dystrophy patients
  - Preparing to initiate second neuromuscular disease trial
- Treatment and enrollment ongoing in Phase 2 trial with dalantercept in renal cell carcinoma

# Q4 and FY 2016 Financial Results



<b>Cash</b>	
Cash, cash equivalents and investments	\$234.4M
<b>Revenue</b>	
Collaboration Revenue	\$27.8M
<b>Costs, Expenses and Other Income</b>	
Total Costs and Expenses	\$93.9M
R&D Expenses	\$68.6M
G&A Expenses	\$25.3M
Total other income	\$9.1M
<b>Net Loss</b>	
Net Loss	\$57.0M

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 **2H 2017**
  - Develop clinical and regulatory strategy in first-line lower-risk MDS in **2017**
  - Initiate new Phase 2 trials in myelofibrosis and NTD beta-thalassemia by **YE 2017**
  - Additional Phase 2 extension study results at medical conferences in **2017**
  
- **ACE-083**
  - Initial FSHD Phase 2 Part 1 dose-escalation results by **late 2017**
  - Initiate Phase 2 trial in a second neuromuscular disease in **2017**
  
- **Dalantercept**
  - Report topline results in the DART Phase 2 study in RCC in **2H 2017**
  
- **ACE-2494**
  - Initiate Phase 1 healthy volunteer study in **2017**
  
- Host investor and analyst Acceleron Research Day in **2017**

## Q4 and FY 2016 Results Q&A Session

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**Habib Dable**

President and Chief Executive Officer

**Steven Ertel**

Chief Operating Officer

**Matthew Sherman, M.D.**

Chief Medical Officer

**Kevin McLaughlin**

Chief Financial Officer

**Todd James, IRC**

Investor Relations and Corp. Comm.



# Thank You

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NASDAQ: XLRN