



**First Quarter 2019 Financial Results**

**May 9, 2019**

# 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, the timeline for clinical development and regulatory approval of the Company's compounds and the expected timing for reporting of data from ongoing clinical trials. The words "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "may," "plan," "potential," "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.

## **ACTUAL RESULTS COULD DIFFER MATERIALLY FROM THOSE INCLUDED IN THE FORWARD-LOOKING STATEMENTS DUE**

to various factors, risks and uncertainties, including, but not limited to, 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 the results of any clinical trial may not be predictive of the results or success of other clinical trials of the same product candidate, that the development of the Company's compounds will take longer and/or cost more than planned, that the Company will be unable to successfully complete the clinical development of the Company's compounds, that the Company 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. These and other risks and uncertainties are identified under the heading "Risk Factors" included in the Company's most recent Annual Report on Form 10-K, 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 ARE BASED ON MANAGEMENT'S CURRENT**

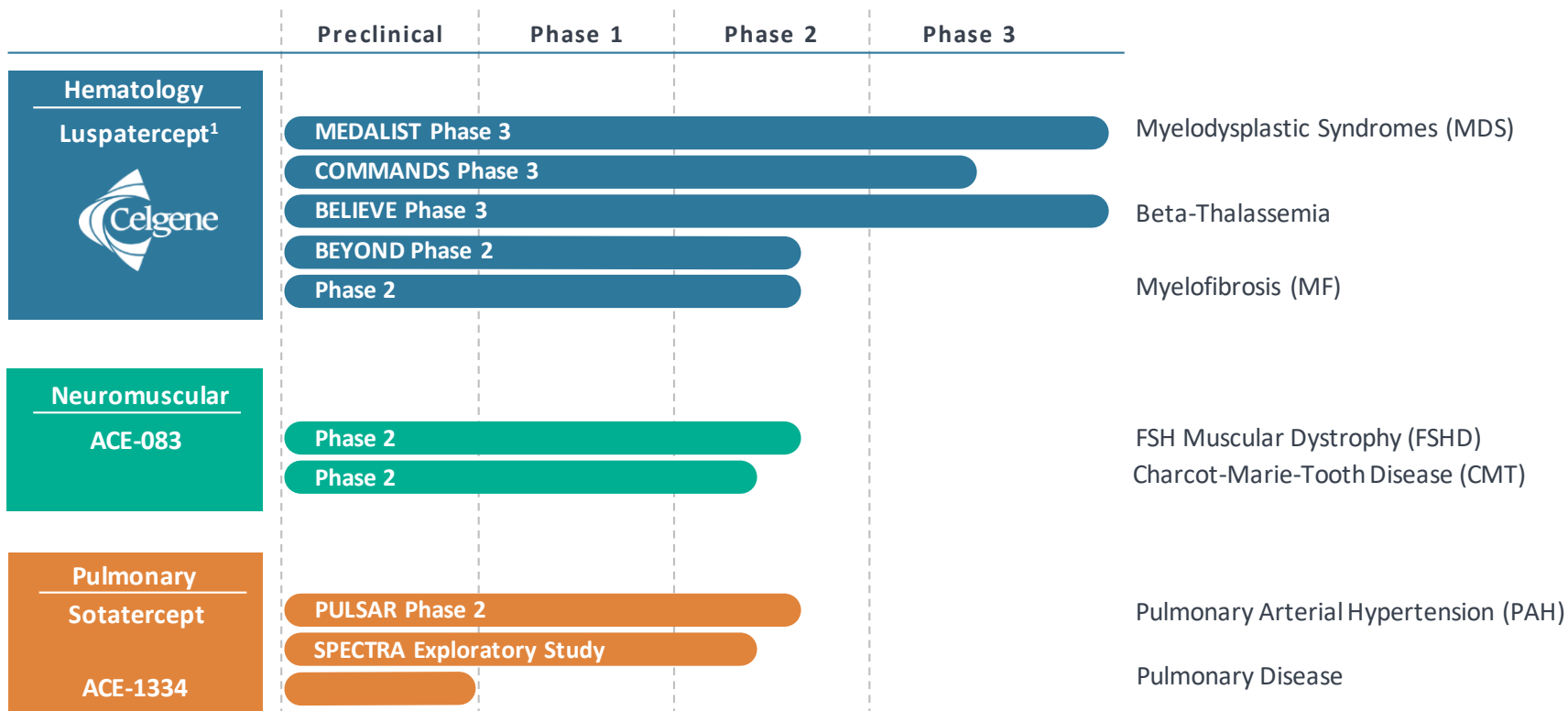
views, plans, estimates, assumptions and projections with respect to future events, and the Company does not undertake and specifically disclaims any obligation to update any forward-looking statements.



**Habib Dable**  
Chief Executive Officer




# Building Therapeutic Area Leadership



# US and EU Regulatory Applications Submitted in April

---



**MEDALIST**

Phase 3

**Lower-Risk MDS,  
RS+**



**BELIEVE**

Phase 3

**Beta-Thalassemia,  
Transfusion-Dependent**



**U.S. FOOD & DRUG  
ADMINISTRATION**

Biologics License Application (BLA)






**EUROPEAN MEDICINES AGENCY**  
SCIENCE · MEDICINES · HEALTH

An agency of the European Union 

Marketing Authorization Application (MAA)

# Additional Luspatercept<sup>1</sup> Trials Underway

	Disease Area	Topline Results Expected
 Phase 2	Myelofibrosis	2H:19
 Phase 2	Beta-Thalassemia, Non-Transfusion-Dependent	2020
 Phase 3	Lower-Risk MDS, First-line	TBD

# ACE-083 Phase 2 FSHD and CMT Program Highlights

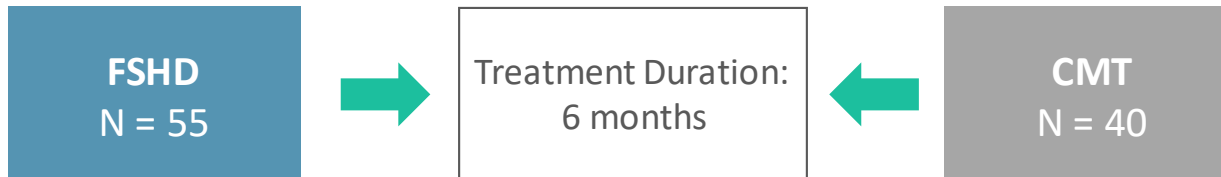
## FSHD Part 1 Results Encore Presentation



## CMT Part 1 Results Encore Presentation



## PART 2 OF EACH PHASE 2 TRIAL ONGOING



# Sotatercept PAH Program Highlights

---



Traditional Phase 2 Trial  
Randomized, double-blind, placebo-controlled



Exploratory Phase 2 Trial  
Open-label, single arm







**Kevin McLaughlin**  
Chief Financial Officer



# Q1 2019 Financial Results

---

<b>Cash</b>	
Cash, cash equivalents and investments	\$513.1M
<b>Revenue</b>	
Collaboration Revenue	\$2.8M
<b>Costs and Expenses</b>	
Total Costs and Expenses	\$43.6M
R&D Expenses	\$32.8M
G&A Expenses	\$10.8M
<b>Net Loss</b>	
Net Loss	\$38.1M

# Upcoming Corporate Priorities

---

## HEMATOLOGY

### ▪ Luspatercept

- MEDALIST and BELIEVE Phase 3 trial results planned to be submitted for publication in **2019**
- Myelofibrosis Phase 2 trial preliminary topline results expected in **2H 2019**
- BEYOND Phase 2 trial topline results expected in **2020**
- COMMANDS Phase 3 trial patient enrollment
- Potential expansion of clinical program into other indications in **2019**

## NEUROMUSCULAR

### ▪ ACE-083

- FSHD Part 2 of the Phase 2 trials topline results expected in **2H 2019**
- CMT Part 2 of the Phase 2 trials topline results expected in **Q1 2020**

## PULMONARY

### ▪ Sotatercept

- PULSAR Phase 2 trial topline results expected in **1H 2020**
- SPECTRA exploratory study preliminary results expected in **2020**

# Q1 2019: Financial Results Q&A Session

---

**Habib Dable**

Chief Executive Officer

**Kevin McLaughlin**

Chief Financial Officer

**John Quisel, Ph.D., J.D.**

Chief Business Officer

**Sujay Kango**

Chief Commercial Officer

**Todd James, IRC**

VP, Investor Relations and Corp. Comm.



# THANK YOU



[www.acceleronpharma.com](http://www.acceleronpharma.com)  
NASDAQ: XLRN