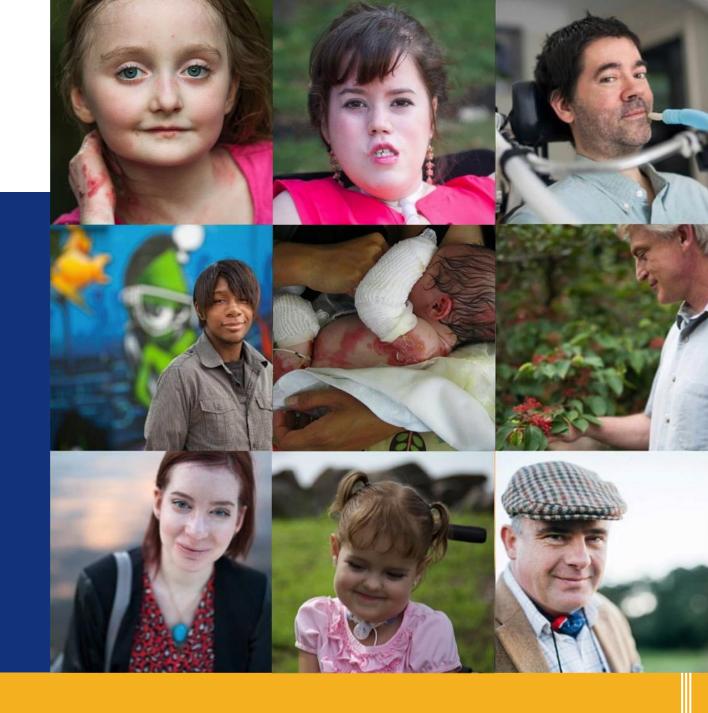


LEERINK Partners

5<sup>th</sup> Annual Global

Healthcare

Conference



John F. Crowley, Chairman and Chief Executive Officer February 10, 2016

Introduction

#### Safe Harbor

This presentation will contain, "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of Amicus' candidate drug products, the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products, financing plans, and the projected cash position for the Company. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing and outcomes of discussions with regulatory authorities, and in particular the timing of an NDA submission for migalastat monotherapy, and the potential goals, progress, timing and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we will need additional funding to complete all of our studies and, our dependence on third parties in the conduct of our clinical studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2014 and Form 10-Q for the guarter ended June 30, 2015. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

## Amicus 2016 – Looking Back

Amicus Has Greatly Expanded Product Pipeline, Technologies and Geographies

2014

2012

- Chaperone Technology for LSDs
- Small molecules
- U.S. rights to migalastat

 Callidus acquisition

2013

- Biologics
- Global rights to migalastat

- Positive Phase3 data formigalastat
- Biologics scaleup

International HQ

2015

- MAA Submission
- Scioderm acquisition
- Pompe ERT in clinic



## Amicus 2016 – Continuing the Momentum

# **Significant Milestones in 2016**

**2016**Anticipated Milestones

2014

2013

2012

- Chaperone Technology for LSDs
- Small molecules
- U.S. rights to migalastat

- Callidus acquisition
- Biologics
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- Positive Phase 3 data for migalastat
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International HQ

2015

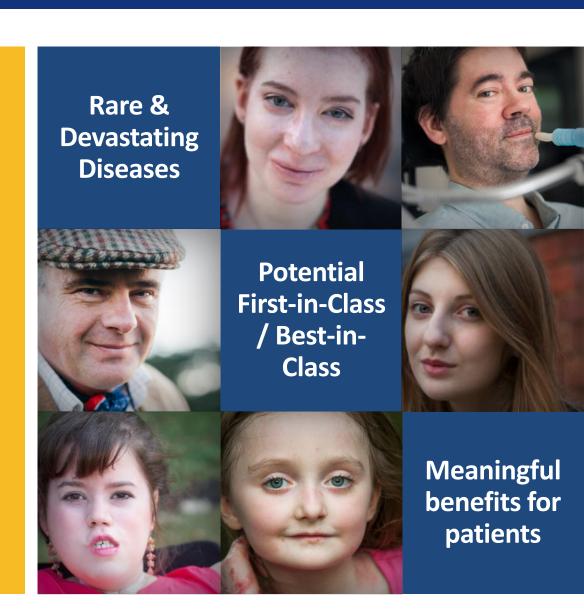
- MAA
   Submission
- Scioderm acquisition
- Pompe ERT in clinic

- CHMP opinion for migalastat for Fabry
- FDA regulatory clarity for migalastat
- EB Phase 3 data
- Pompe clinical data



#### **Amicus Vision**

Amicus Therapeutics is a global biotechnology company at the forefront of developing advanced therapies to treat a range of devastating rare and orphan diseases





### Key Drivers of Value

#### 3 Novel Product Candidates Each with \$500M to \$1B+ Market Potential

#### **Fabry**

- Migalastat
   Personalized Medicine
   (Small Molecule)
- MAA Submitted
- CHMP Opinion
   Anticipated Early 2016
- Prepared for EU Launch\*

# **Epidermolysis** Bullosa (EB)

- Phase 3 Novel Topical Cream (SD-101)
- U.S. Breakthrough Therapy Designation
- Rolling NDA
- Phase 3 Data
   Expected in 2H16

#### **Pompe**

- Novel ERT +
   Chaperone
   Treatment Paradigm
- BiologicsManufacturing
- Clinical Study
   Initiated with Data
   Anticipated in 2016

**R&D Engine and Continued Business Development Activity** 



**Financial Summary** 

# **Strong Balance Sheet**

#### Cash Position Provides Runway Under Current Operating Plan into 1H17

Financial Position	December 31, 2015
Current Cash:	\$214M
Current Debt	\$50M
FY16 Net Cash Spend Guidance:	\$135M-\$155M
Cash Runway	Mid-2017
Capitalization	
Shares Outstanding	125,027,034



# Thank You

