



# Galafold™ (Migalastat) U.S. Approval Call



August 13, 2018

# Safe Harbor

*This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to approval and commercialization plans for Galafold in the United States. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that we may not be successful in commercializing Galafold in the United States, the potential that public and commercial payors will not reimburse Galafold, the potential that we may not be able to manufacture or supply sufficient commercial products; and the potential that we will need additional funding to complete all of our commercialization and manufacturing activities. 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, 2017 as well as our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018 filed August 7, 2018 with the Securities and Exchange Commission. 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 we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.*

# Our Passion for Making a Difference Unites Us



# Galafold (Migalastat) Launch Progress (8/10/18)

**Patients have Reimbursed Access in 20 Countries**



# U.S. Label Highlights

## Galafold Approved under Subpart H Accelerated Approval Pathway



- Galafold is indicated for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (*GLA*) variant based on in vitro assay data
- This indication is approved under Accelerated Approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.
- Approved for 348 amenable variants
- The most common side effects: headache, nasopharyngitis, urinary tract infection, nausea and pyrexia



# U.S. Demographics for Galafold



## Launch Underway

**NDA Approved (Priority Review)**

**Ahead of August 13, 2018 PDUFA**

**Full launch team hired and trained**

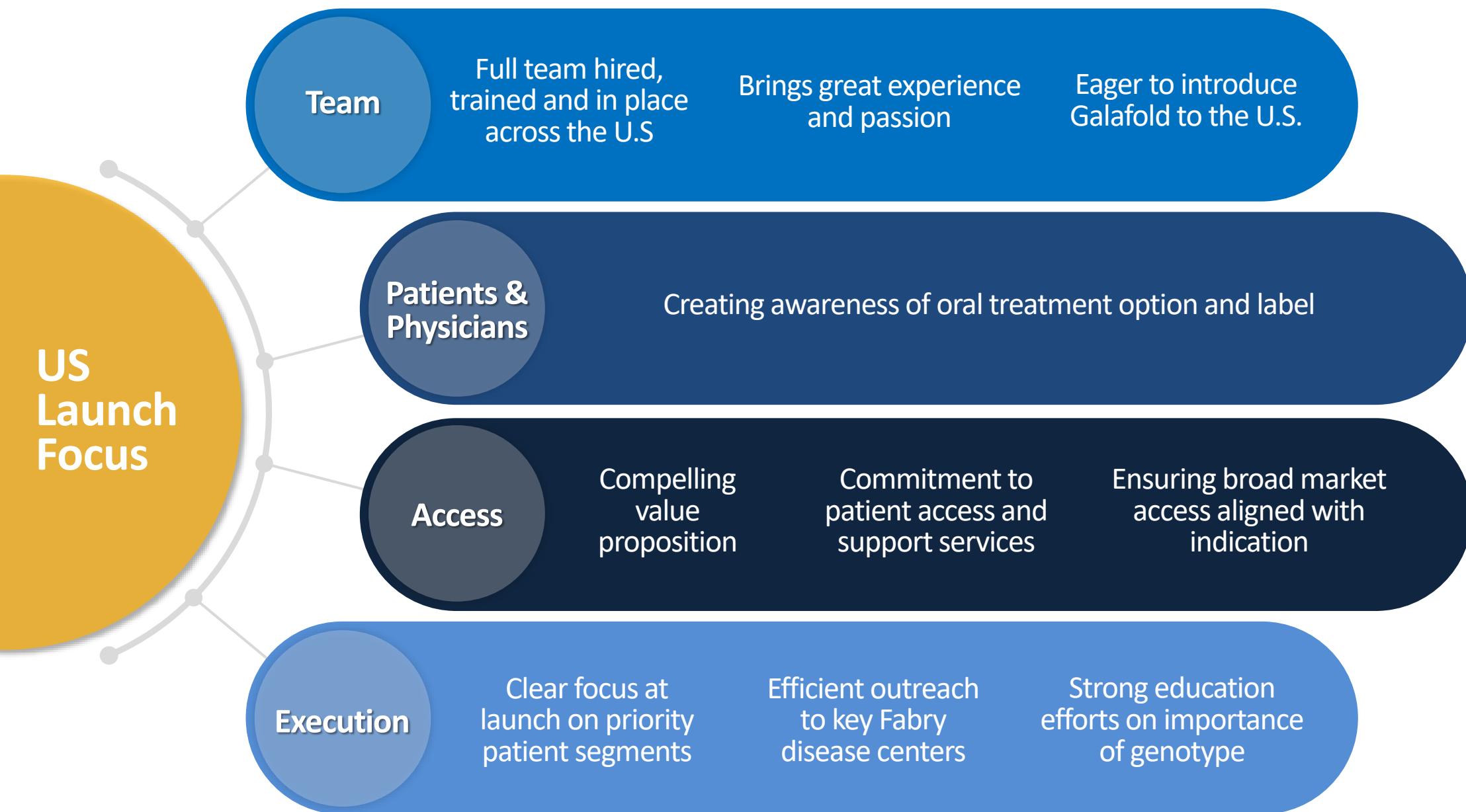
**Appropriate stakeholder engagement ongoing**

**>3,000 diagnosed (~1,500 treated) (US Estimates)**

**~35-50% amenable (Global Estimates)**

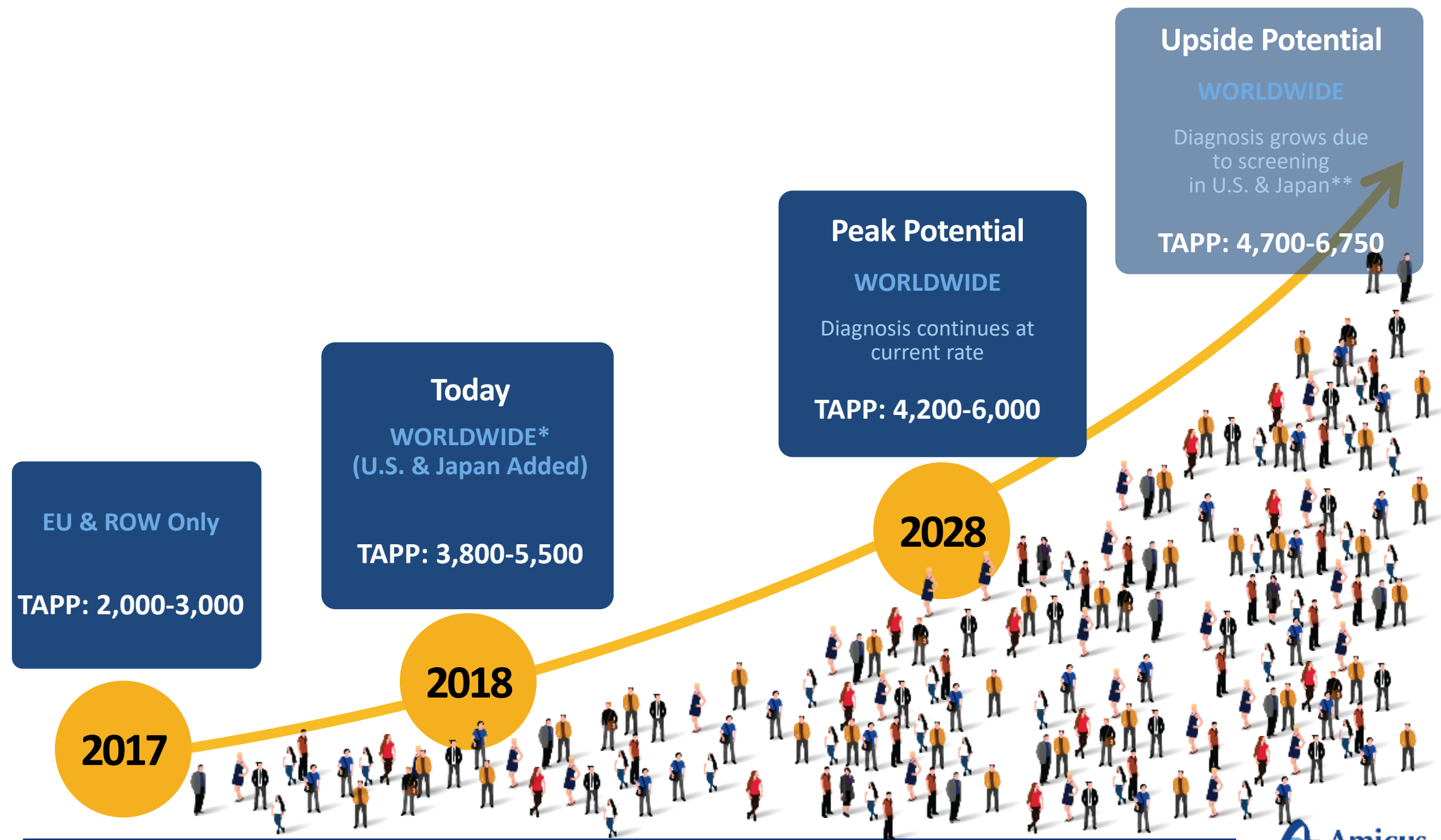
**Orphan Drug and Fast Track designations**

# Leveraging Our Operations Excellence



# Total Amenable Patient Population (“TAPP”)

Estimate based on 35% - 50% amenability



\*WORLDWIDE includes total amenable patient population in all Fabry ERT commercial markets today \*\*Estimated effect of newborn screening on adult diagnostic rate.

# Pricing Philosophy

“*Our medicines must be fairly priced and broadly accessible*”

- Amicus Founding Belief

## Galafold Pricing

- Galafold priced at parity or below to ERT\* (without infusion associated costs)
- Amicus will limit Galafold price increases to CPI (consumer price index)
- Amicus pledges to reinvest a portion of our profits into R&D of new treatments for Fabry disease until there's a cure



\*Average ERT price in the United States based on an average adult patient

# 2018 Key Strategic Priorities

*As of August 2018*

**Focused on FIVE Key Strategic Priorities in 2018**

**1 Double Galafold (migalastat) revenue to \$80-\$90M**

**2 Secure approvals for migalastat in Japan and the U.S.**



**3 Achieve clinical, manufacturing and regulatory milestones to advance AT-GAA toward global regulatory submissions and approvals**

**4 Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019**

**5 Maintain financial strength**

# Amicus Vision: Delivering for Patients and Shareholders

**To build a top-tier, fully integrated, global biotechnology company whose medicines treat 5,000+ patients with \$1B+ in worldwide sales revenue by 2023**



>350 Patients\* | \$36.9M Global Sales



5,000 Patients\* | \$1B Global Sales

YE17

2023

\*Clinical & commercial, all figures approximate

# Thank You

*“Our passion for making a difference unites us”*

*-Amicus Belief Statement*

