Amicus Therapeutics®

2Q19 Financial Results Conference Call & Webcast

August 8, 2019

Forward-Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. 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 presentation 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, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular 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 our business, including, without limitation: the potential that results of clinical or preclinical 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, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe, Japan, the US and other geographies or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. 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 revenue and 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, 2018 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2019. 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.

2Q19 and Early 3Q19 Highlights

- **2Q19 Galafold Revenue of $44.1M - Continued Global Strength for Successful Rare Disease Launch**
- **AT-GAA PROPEL Pivotal Study Now Majority Enrolled**
- **Positive 2-Year Interim Clinical Results in CLN6 Batten Disease - Potential to Halt Progression of Fatal Neurologic Disease**
- **Expanded Penn Collaboration Provides Industry's Largest Rare Disease Gene Therapy Pipeline**
- **Strengthened Balance Sheet with $575M+ Cash at 6/30/19**

Galafold® (migalastat) Global Launch…

…taking a leadership role in the treatment of Fabry disease

“We push ideas as far and as fast as possible”

- Amicus Belief Statement

Galafold Snapshot (as of June 30, 2019)

One of the Most Successful Rare Disease Launches

$44.1M
2Q19 Galafold Revenue

$160-180M
FY19 Global Galafold Rev. Guidance

Geographic Expansion in 2019

Galafold™ (migalastat)

24
Countries with Pricing & Reimbursement

9
Regulatory Approvals* :
Argentina, Australia, Canada, EU, Israel, Japan, S. Korea, Switzerland , U.S.

348
Amenable Variants in U.S. Label

Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including the full U.S. Prescribing Information, please visit https://www.amicusrx.com/pi/Galafold.pdf. For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at www.ema.europa.eu.

*As of July 31, 2019

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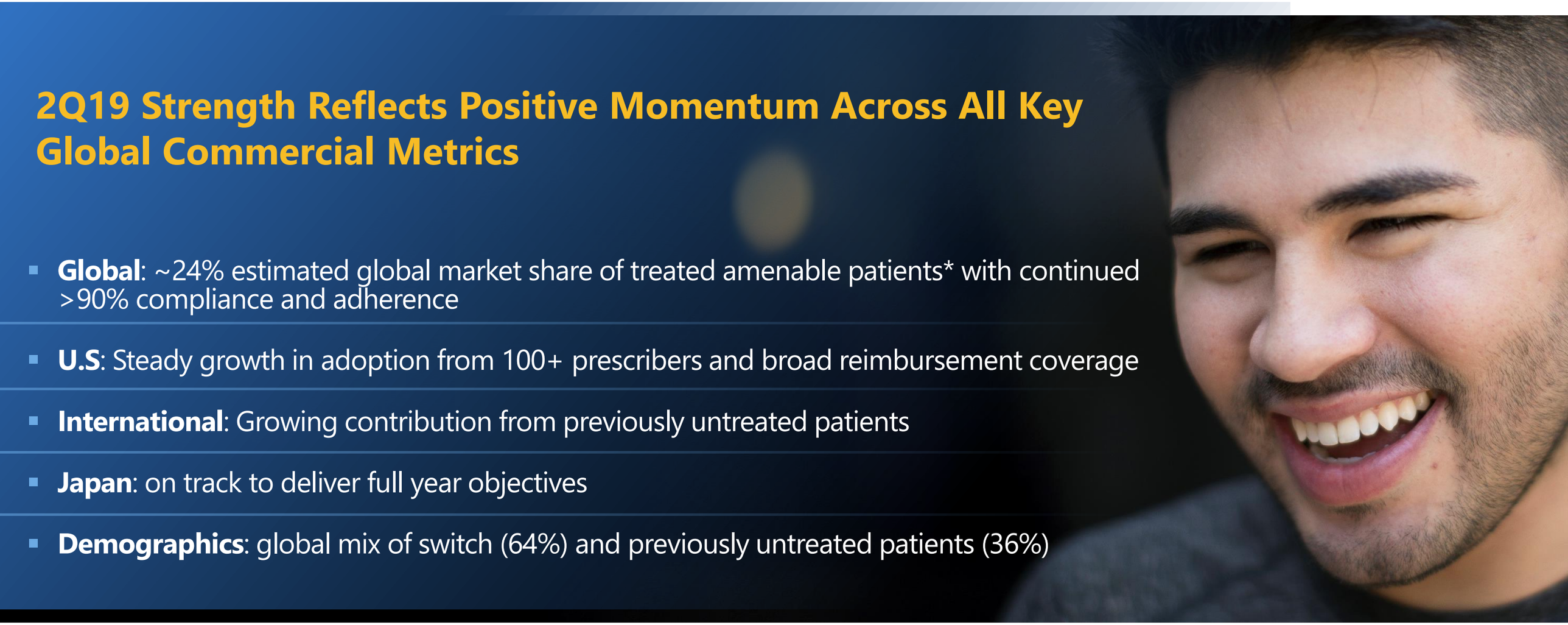
Galafold Global Launch Momentum (as of June 30, 2019)

2Q19 Strength Reflects Positive Momentum Across All Key Global Commercial Metrics



- **Global**: ~24% estimated global market share of treated amenable patients* with continued >90% compliance and adherence
- **U.S**: Steady growth in adoption from 100+ prescribers and broad reimbursement coverage
- **International**: Growing contribution from previously untreated patients
- **Japan**: on track to deliver full year objectives
- **Demographics**: global mix of switch (64%) and previously untreated patients (36%)

*Market share based on reported 2018 global Fabry sales and assumes a 35% amenability rate.

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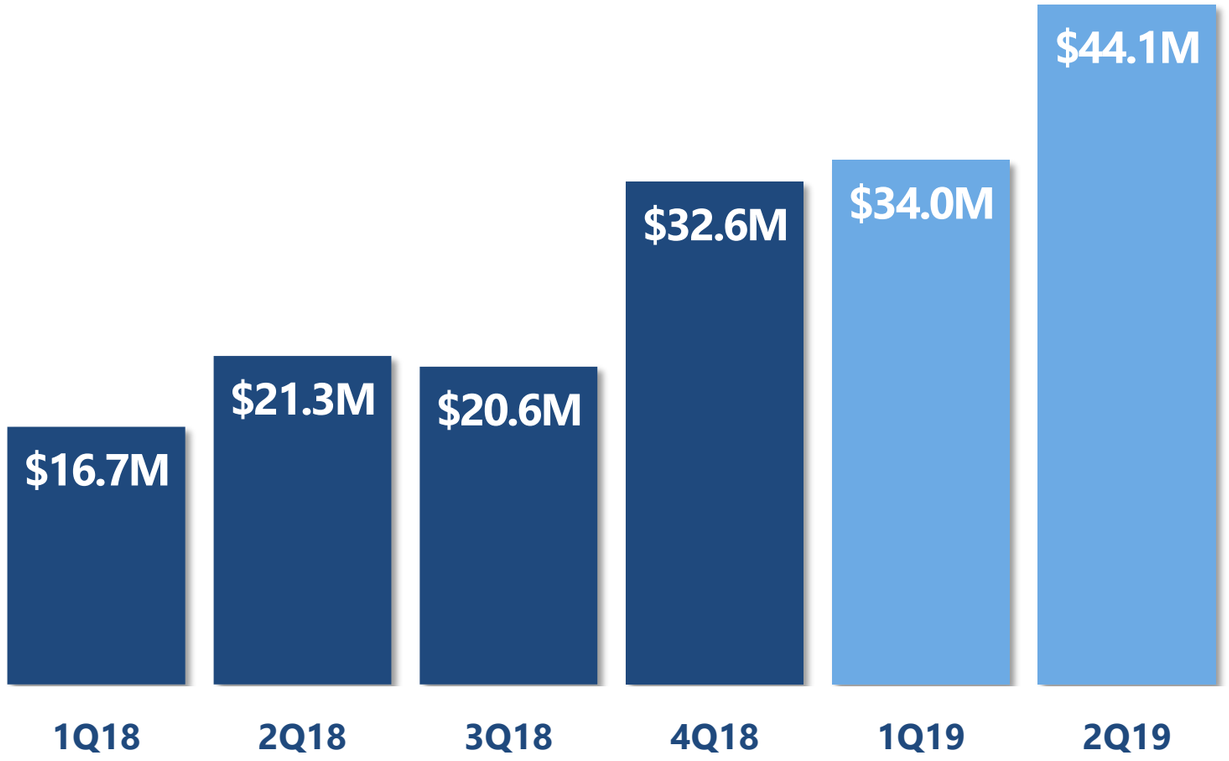
Galafold Quarterly Performance

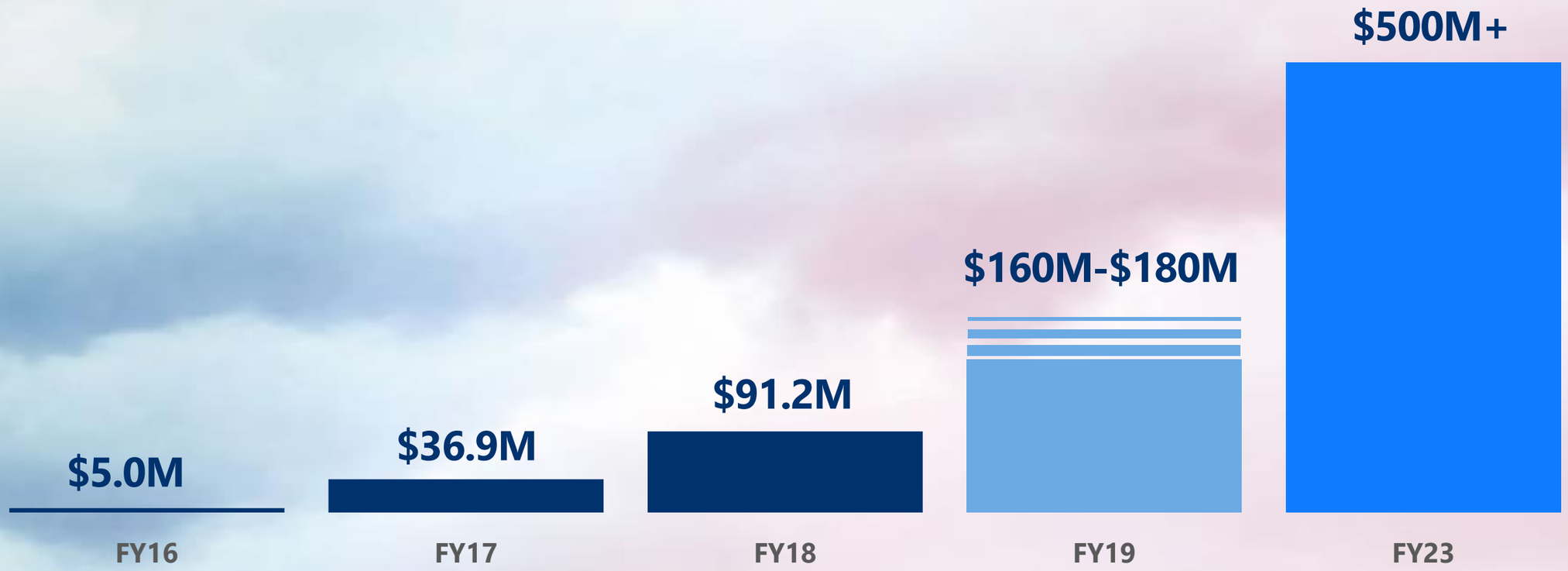
2Q19 Revenue of $44.1M Grew 107% Year-over-Year Reflecting Continued Strong Growth in Global Adoption of First Fabry Oral Precision Medicine



- Consistent with Galafold adoption trends and ordering patterns in previous years, quarter to quarter growth will not be linear
- Higher revenue growth in 2Q19 as expected; +115% YoY constant currency revenue growth, offset by negative currency impact (-$1.7M, or -8%)

Galafold Success and FY19 Galafold Revenue Guidance

Galafold Sales in 2019 Represent the First Full Year of Launch in the Major Geographies in the World and will be between $160-$180M, with more than 1,000 Fabry Patients on Therapy by the End of 2019. Galafold Sales of $500M+ are Expected by 2023



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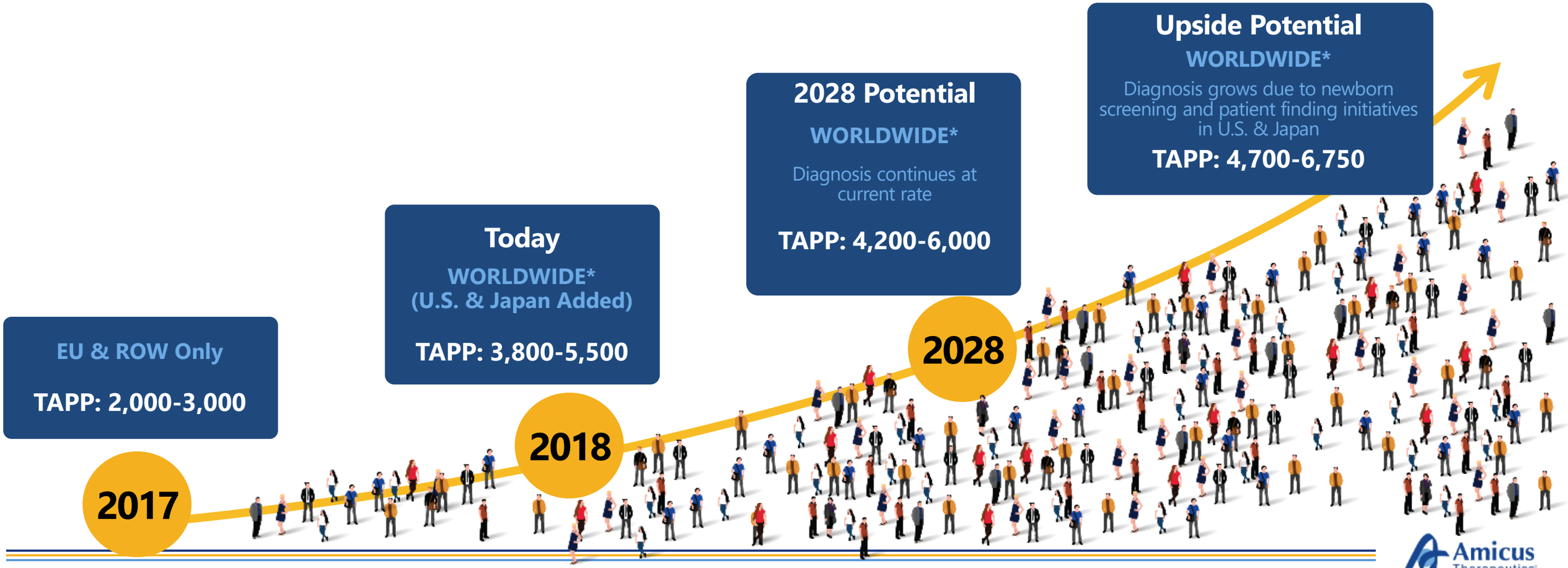
Total Amenable Patient Population ("TAPP")

Estimate based on 35% - 50% amenability

Fabry Disease is Increasingly Believed to be One of the Most Prevalent Human Genetic Diseases. The Total Amenable Patient Population ("TAPP") has the Potential to Make Galafold a $1B+ Annual Product



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

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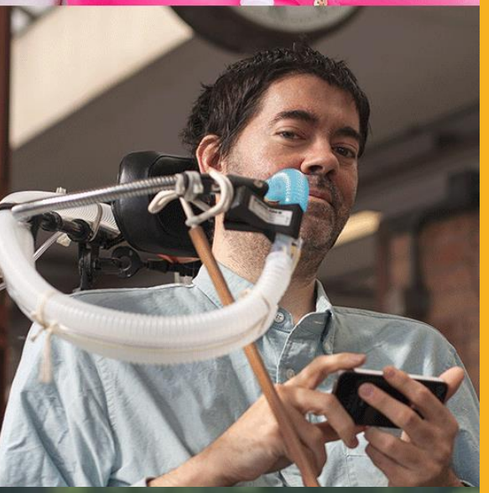


AT-GAA Novel ERT for Pompe Disease

“We encourage and embrace constant innovation”

- Amicus Belief Statement

PROPEL (ATB200-03) Study Design

AT-GAA is the First Ever Second-Generation Product for Any Lysosomal Disorder to Earn Breakthrough Therapy Designation ("BTD") from the FDA

52-Week Primary Treatment Period (Double-Blind)

2:1 Randomization

Participants with Late-Onset Pompe Disease

~100 Patients
90 WW Clinical Sites

ERT-Switch
ERT-Naïve

AT-GAA
Bi-Weekly

Standard of Care
Bi-Weekly

Long-Term Extension (Open-Label)

AT-GAA
Bi-weekly

Primary Endpoint: 6-Minute Walk Test at Week 52; Multiple Secondary Endpoints

Well-Powered for Superiority

BTD Criteria

- Preliminary clinical evidence indicates drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints

AT-GAA BTD Based on Ph 1/2 Clinical Efficacy

BTD Features

- Intensive guidance on an efficient drug development program
- Organizational commitment involving senior managers
- All Fast Track program features

PROPEL (ATB200-03) Study Global Enrollment

Sites in 29 Countries are Now Active to Ensure Broad Access and Experience with AT-GAA

Phase 3 Enrollment Expected to Complete in 2019 with Data in 1H 2021

Targeting 90 Sites to Include all Leading Global Pompe Treatment Centers

AT-GAA: 2019 Objectives

Advance AT-GAA for as Many Patients Worldwide as Quickly as Possible

- ✓ Additional Phase 1/2 Data (up to 24 Months)
- ✓ Breakthrough Therapy Designation
- Enroll PROPEL study (n=100)
- Present additional Phase 1/2 data at World Muscle Society (Oct. 1-5)
- Report natural history study data
- Initiate pediatric study
- Advance agreed upon CMC requirements to support BLA

Mepsevii™ for Mucopolysaccharidosis Type VII (MPS VII, Sly syndrome)

Licensed Exclusive Japanese Rights to Mepsevii to Leverage Existing Infrastructure, Relationships and Experience In Clinical Development, Regulatory Approvals and Commercialization within the Lysosomal Disorders Community in Japan

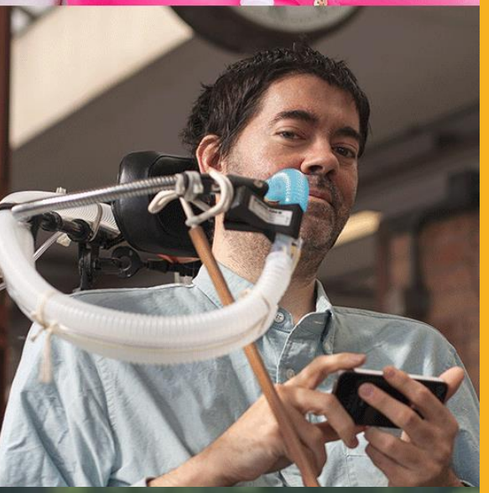
Amicus and Ultragenyx are Aligned on Strategy to Advance Mepsevii for People in Japan with MPS VII

Mepsevii™
(vestronidase alfa-vjbk)
injection, for intravenous use

Amicus Licensed Exclusive Japanese Rights to Mepsevii (vestronidase alfa) from Ultragenyx

Mepsevii is currently approved for the treatment of children and adults with MPS VII in U.S., EU and Brazil.

Existing Mepsevii studies and investigator-initiated study in Japan to support future J-NDA submission



Gene Therapy Pipeline

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement

A RARE PORTFOLIO.

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMMERCIAL
Fabry Franchise						
Galafold® (migalastat) monotherapy						
Fabry Gene Therapy	PENN					
Pompe Franchise						
AT-GAA (Novel ERT + Chaperone)						
Pompe Gene Therapy	PENN					
Batten Franchise – Gene Therapies						
CLN6 Batten Disease	NCH					
CLN3 Batten Disease	NCH					
CLN8 Batten Disease	NCH					
CLN1 Batten Disease	NCH					
Next Generation Research Programs and CNS Gene Therapies						
CDKL5 Deficiency Disorder GTx / ERT	PENN					
Niemann-Pick Type C (NPC)	NCH / PENN					
Tay-Sachs Disease	NCH					
Other	NCH / PENN					
MPS Franchise - Gene Therapies						
Next Generation MPSIIIA	PENN					
MPSIIIB	PENN					

In less than a year, Amicus assembled the **largest portfolio of gene therapy programs for rare diseases** in the entire industry

Amicus-Penn Gene Therapy Collaboration

New Indications and Next Generation Research Program to Harness Combination of Amicus-Penn Technologies

Penn

GENE THERAPY PROGRAM

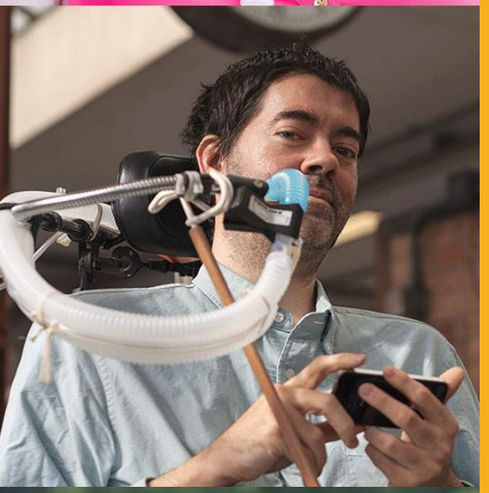
Amicus Therapeutics®

- Worldwide Rights to Penn's Next Generation Gene Therapy Technologies for the Majority of Lysosomal Disorders
- Six Active Preclinical Programs: Fabry, Pompe, CDKL5 Deficiency Disorder (CDD), Niemann-Pick Type C, Next Generation MPS IIIA, and MPS IIIB
- 12 Additional Rare Diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and Select Other Muscular Dystrophies
- Research Program to Improve Safety, Efficacy and Manufacturability of Next Generation Vectors ($10M / Year for 5 Years; Option to Extend)

5 Key Takeaways for AAV-CLN6 Gene Therapy

Interim Safety and Efficacy Data Demonstrate the Potential for AAV-CLN6 Gene Therapy to Stabilize Progression of a Devastating Disease

- **Meaningful impact on motor and language function** in children with a fatal neurologic disease that destroys brain function
- **Evidence of disease stabilization** in seven out of the eight children following AAV-CLN6 gene transfer
- **Natural history cohort shows progressive loss of language and motor function** in all untreated patients
- **Sibling comparisons (in-study and natural history) provide further support** for AAV-CLN6 gene therapy and early intervention
- **Favorable safety profile** with intrathecal administration of AAV in all study participants



Financial Summary

“We are business led and science driven”

- Amicus Belief Statement

2Q19 Select Financial Results

2Q19 Revenue of $44.1M Primarily from International Galafold Sales

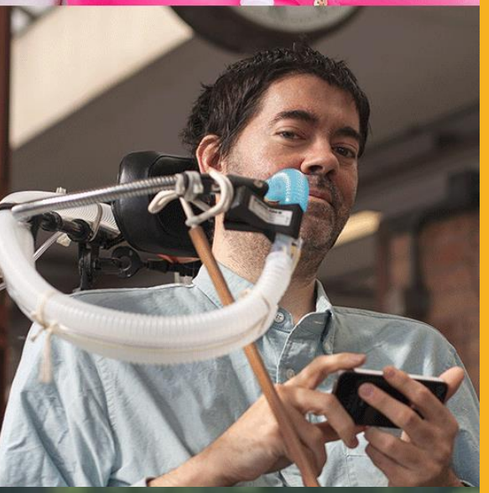
(in thousands, *except per share data)*	June 30, 2019	June 30, 2018
Product revenue	44,130	21,309
Cost of goods sold	5,367	3,135
R&D expense	70,981	34,660
SG&A expense	42,578	29,172
Changes in fair value of contingent consideration	480	300
Loss from operations	(76,430)	(46,931)
Income tax expense	(717)	(339)
Net loss	(84,551)	(61,833)
Net loss per share	(0.36)	(0.33)

Financial Summary and Guidance

Strong Balance Sheet with $575M+ Cash at 6/30/19 - Cash Runway into 2021

FINANCIAL POSITION	
Cash	$575.7M
Cash Runway[1]	Into 2021
Debt[2]	$152.8M
CAPITALIZATION	
Shares Outstanding[3]	254,513,522
FINANCIAL GUIDANCE	
FY19 Galafold Revenue Guidance	$160M-$180M

[1]Based on existing operating plan including proceeds from June 2019 equity offering to invest in manufacturing
[2]Includes $2.8 million of convertible debt and $150 million of straight debt
[3]Includes shares from June 2019 equity offering



Summary & Milestones

“We are business led and science driven”

- Amicus Belief Statement

2019 Key Strategic Priorities

1. **Nearly double annual revenue for Galafold® (guidance $160M-$180M)**
2. **Complete enrollment in AT-GAA Pivotal Study (PROPEL) and report additional Phase 1/2 data**
3. **Report additional 2-year clinical results in CLN6-Batten disease and complete enrollment in ongoing CLN3-Batten disease Phase 1/2 study**
4. **Establish preclinical proof of concept for Fabry and Pompe gene therapies**
5. **Maintain strong financial position**

Anticipated Milestones

Well-Positioned to Create Significant Value for Patients and Shareholders

Galafold: Fabry Disease

- FY19 revenue guidance $160M-$180M
- Growth in existing markets
- Expansion into new markets
- Diagnostic initiatives

AT-GAA: Pompe Disease

- ✓ Additional Phase 1/2 data (21 and 24 months)
- ✓ Breakthrough therapy designation (BTD) in LOPD
- ✓ Phase 1/2 study fully enrolled (Cohorts 1-4)
- PROPEL pivotal study enrollment (n=100)
- Additional Phase 1/2 data (Cohort 4)
- Natural history study data
- Additional supportive studies
- Advance CMC requirements to support BLA

Gene Therapy Programs

- ✓ Positive initial preclinical data for Pompe gene therapy
- ✓ Selection of CMO partners
- ✓ Complete enrollment in low-dose cohort in CLN3 Batten disease Phase 1/2 study
- ✓ Additional 2-year clinical data in CLN6 Batten disease
- Advance CLN6 Batten disease clinical, manufacturing and regulatory strategy
- Complete enrollment in high-dose cohort in CLN3 Batten disease Phase 1/2 study
- Selection of Amicus late process development and manufacturing facilities
- Additional preclinical data including next-generation gene therapies for Fabry and Pompe
- Selection of Pompe AAV gene therapy IND candidate

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

Amicus Therapeutics®