



3Q19 Financial Results Conference Call & Webcast

November 11, 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. In addition to financial information prepared in accordance with U.S. GAAP, this press release also contains adjusted financial measures that we believe provide investors and management with supplemental information relating to operating performance and trends that facilitate comparisons between periods and with respect to projected information. These adjusted financial measures are non-GAAP measures and should be considered in addition to, but not as a substitute for, the information prepared in accordance with U.S. GAAP. 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. 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.

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A RARE COMPANY.

A Leading Fully-Integrated, Global Rare Disease Biotechnology Company

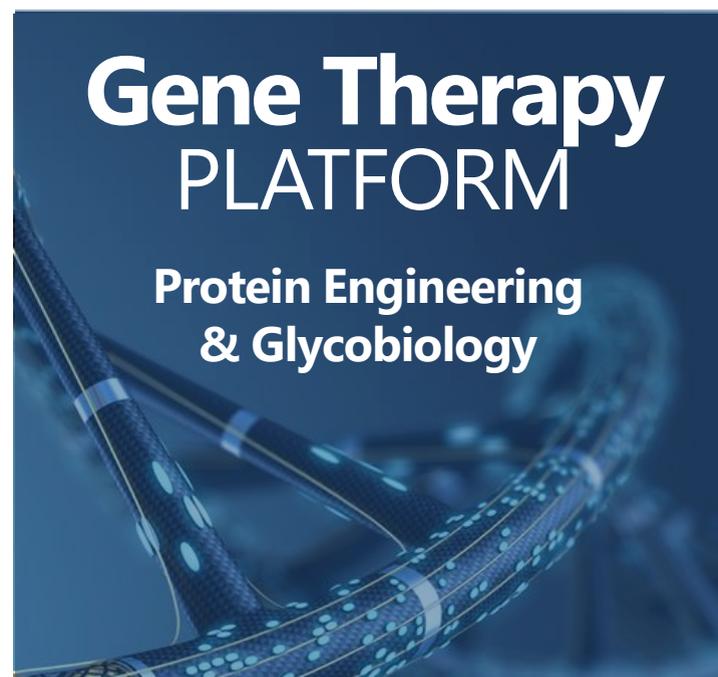


First Oral Precision Medicine for Fabry Disease



Gene Therapy PLATFORM

Protein Engineering & Glycobiology



World Class BIOLOGICS Capabilities

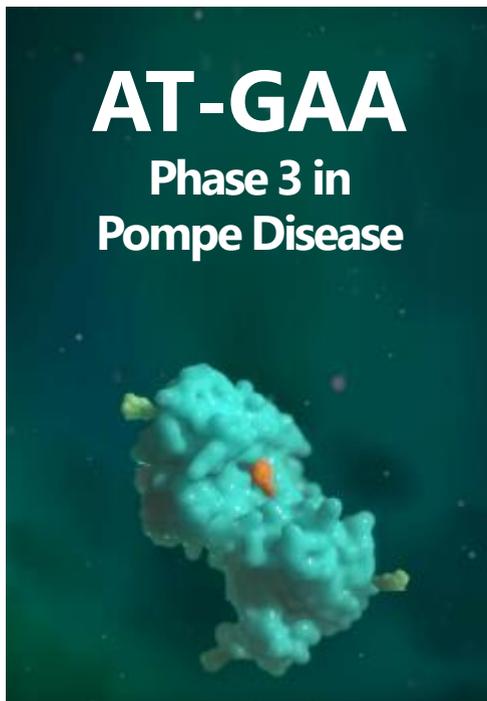


Global Footprint in 27 Countries



AT-GAA

Phase 3 in Pompe Disease



\$514M+ Cash as of 9/30/19

Two Clinical-Stage Gene Therapies

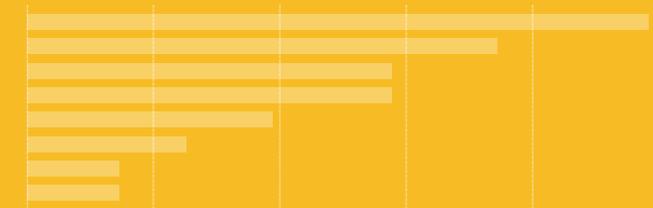


GLOBAL COMMERCIAL ORGANIZATION



Robust R&D Engine

Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases



Key Takeaways for 3Q19 Results

Today's Conference Call and Recent Analyst Day Highlight our Success and Outlook Across our Science, Clinical, Regulatory and Commercial Efforts



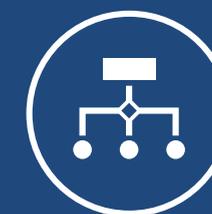
Galafold Continues Strong Launch Performance & Cornerstone of Amicus Success



Amicus Financial Outlook Strengthened with Current Cash Revised Now Well into 1H2022



AT-GAA for Pompe Advances Toward Approval as "Crown Jewel" of Amicus Portfolio



Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future



Financial Summary

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement

3Q19 Select Financial Results

3Q19 Revenue of \$48.8M Primarily from Global Galafold Sales

<i>(in thousands, except per share data)</i>	Sept. 30, 2019	Sept. 30, 2018
Product Revenue	48,768	20,596
Cost of Goods Sold	5,596	4,310
R&D Expense¹	58,892	138,227
SG&A Expense	39,680	31,867
Changes in Fair Value of Contingent Consideration	789	1,300
Depreciation and Amortization	1,116	1,073
Loss from Operations	(57,305)	(156,181)
Income Tax Benefit	251	51
Net Loss	(61,089)	(159,214)
Net Loss Per Share	(0.24)	(0.84)

¹Inclusive of the 2018 upfront payment of \$100 million for the Celenex asst acquisition.

Cash Runway Now Well into 1H2022 (2.5+ years)

Fully Funded Through Major Milestones in Portfolio and Continued Global Growth

Fabry Franchise

Galafold®(migalastat) Monotherapy
 Fabry Gene Therapy

PENN

Pompe Franchise

AT-GAA (Novel ERT) (Gene)
 Pompe Gene Therapy

PENN

Batten Franchise

CLN6 Batten
 CLN3 Batten
 CLN8 Batten
 CLN1 Batten

NCH

NCH

NCH

NCH

Next-generation Ophthalmics and CNS Gene Therapies

CDKL5 Deficiency
 Niemann-Pick
 Tay-Sachs Disease
 Other

PENN

NCH / PENN

NCH

NCH / PENN

MPS Franchise

Mepsevii™ (vestronidase alfa) (Japan Only)*
 Next Generation MPSIIIA
 MPSIIIB

PENN

PENN

\$420M+
Cash
YE2019

2.5+ Years Cash Runway →

Well into
1H2022

Financial Summary & Guidance

Strong Balance Sheet with \$514M+ Cash at 9/30/19 – Cash Runway Well into 1H2022

FINANCIAL POSITION

Cash	\$514M
Cash Runway¹	Well Into 1H2022
Debt²	\$152.8M

CAPITALIZATION

Shares Outstanding	254,772,163
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FINANCIAL GUIDANCE

FY19 Galafold Revenue Guidance	\$170M-\$180M
FY19 Non-GAAP OpEx Guidance	\$410M-\$420M
YE19 Cash Balance	\$420M+

¹Based on existing operating plan ²Includes \$2.8 million of convertible debt and \$150 million of straight debt

Financial Outlook: Key Takeaways



Amicus Financial
Outlook Strengthened
with Current Cash
Revised to 1H2022

- Company now fully funded through major milestones in portfolio and continued global growth
- Cumulative Galafold projected revenues of \$1B+ in 2020-2022 offset significant majority of company spend/investments
- Achieved through OpEx savings, CapEx phasing, program prioritization and increased Galafold revenue projections
- Under current operating plan, 2019 is peak year for non-GAAP operating expense on path to profitability
- No material business development planned or needed in next several years
- Only modest additional capital required to extend runway into profitability with multiple non-equity sources available as/when needed



Galafold[®] (migalastat) Global Launch...

...taking a leadership role in the
treatment of Fabry disease

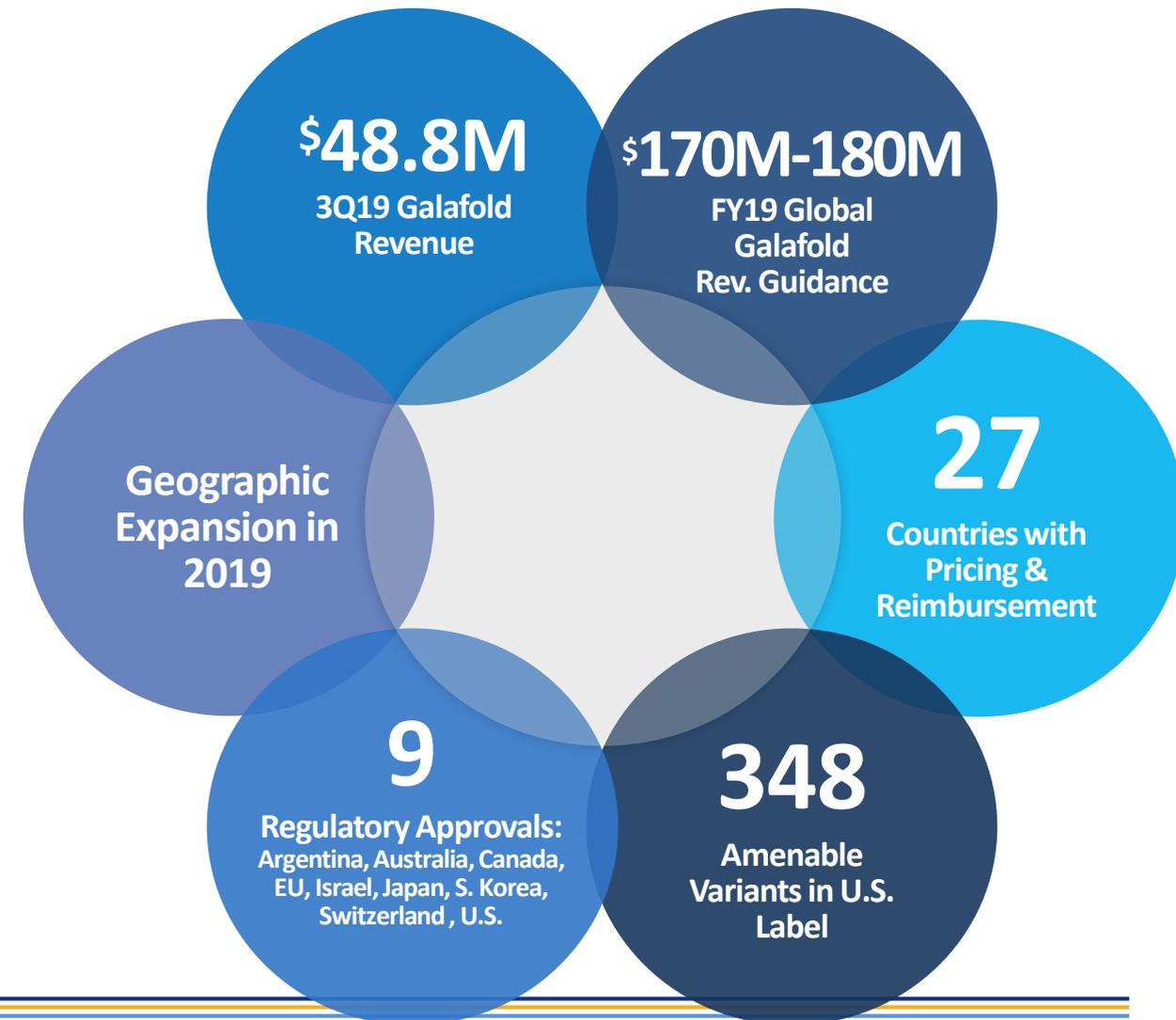
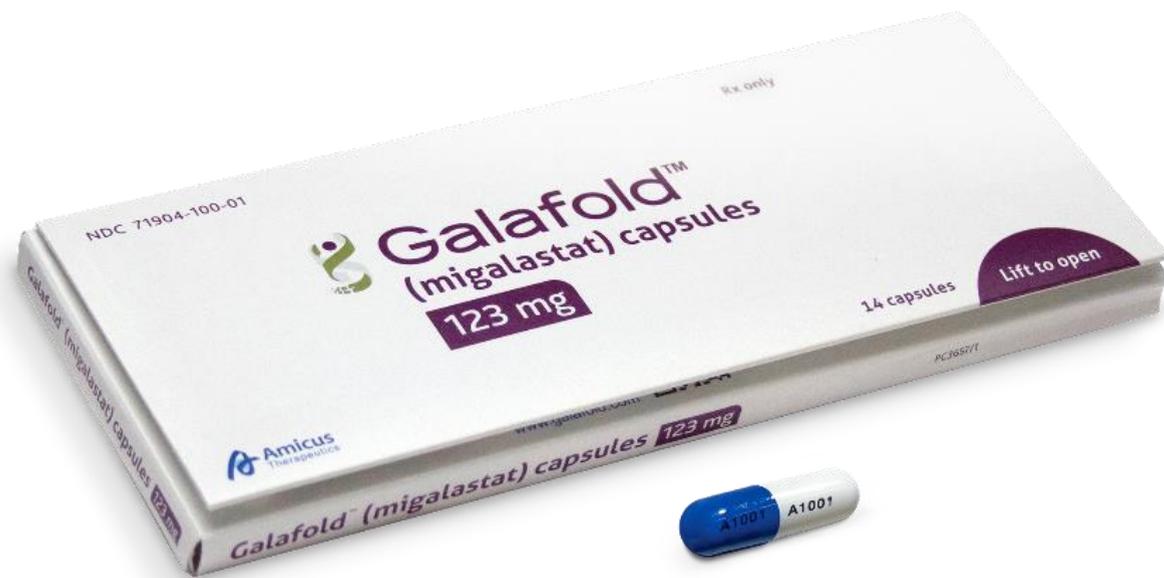
“We push ideas as far and as fast as possible”

- Amicus Belief Statement

Galafold Snapshot (as of September 30, 2019)

Galafold is the Cornerstone of Amicus' Success. It is an Orally Delivered Small Molecule Precision Medicine with a Unique Mechanism of Action for Fabry Patients with Amenable Variants that Replaces the Need for Intravenously Delivered Enzyme Replacement Therapy.

One of the Most Successful Rare Disease Launches



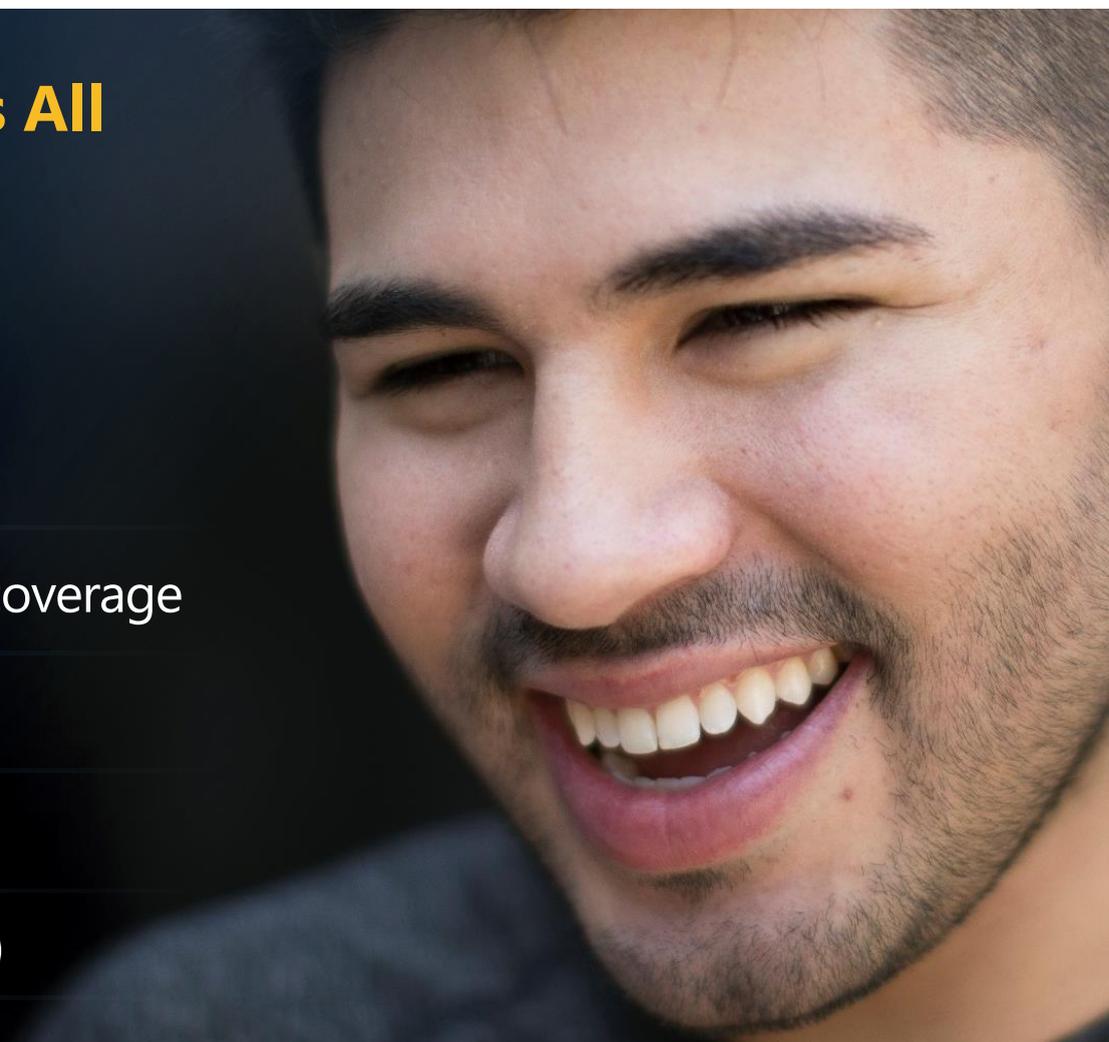
Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold ($\geq 10\%$) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, 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.

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

Global Commercial Metrics Continue to be Very Strong with >90% Compliance and Adherence, 30% Global Market Share of Treated Amenable Patients and Continued Broad Market Access

3Q19 Strength Continues to Reflect Positive Momentum Across All Key Global Commercial Metrics and 1,000+ Treated Patients

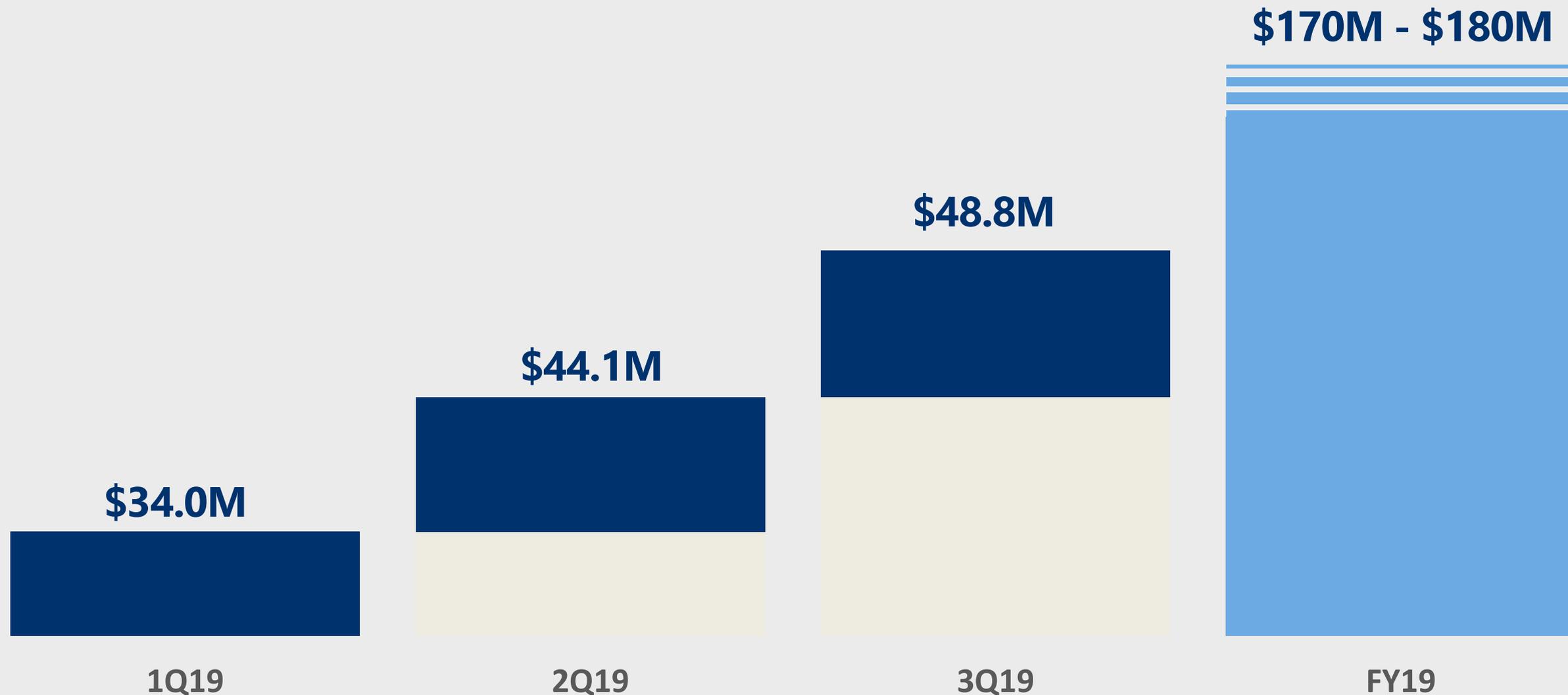
- **Global:** 30%+ estimated global market share of treated amenable patients*
- **U.S.:** Steady growth in adoption from 100+ prescribers and broad reimbursement coverage
- **International:** Growing contribution from previously untreated patients
- **Japan:** On track to deliver full year objectives
- **Demographics:** Global mix of switch (66%) and previously untreated patients (34%)



*Market share based on reported global Fabry sales for the calendar year ending 3Q19 and assumes a 35% amenability rate.

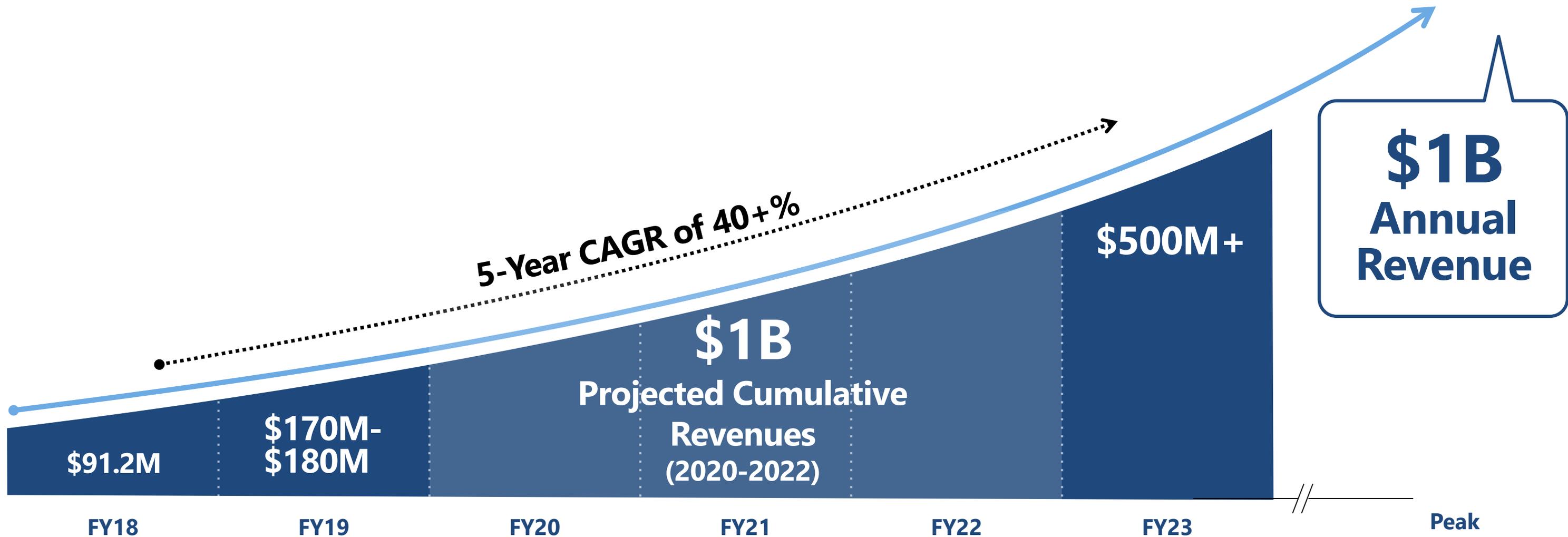
Galafold Success and FY19 Galafold Revenue Guidance

Strong Q3 Performance of \$48.8M Gives Confidence in Upwardly Revised Guidance of \$170M-\$180M. We Expect to Fall in the Midpoint of this Revised Guidance, Inclusive of FX



Galafold Growth Trajectory

Galafold is on Track to Generate \$1B+ in Projected Cumulative Revenues from 2020-2022 and is on an Anticipated Path to \$500M+ in Annual Sales in 2023 and \$1B+ Annual Sales at Peak





Program Updates

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement

AT-GAA: Updates & Key Takeaways



AT-GAA for Pompe
Advances Toward
Approval as “Crown
Jewel” of Amicus
Portfolio

- PROPEL pivotal study expected to over-enroll (~120 Patients) by YE2019
- Pediatric study underway
- Amicus natural history data (POM-002) generally consistent with declines in 6MWT in published literature
- Manufacturing PPQ runs at WuXi biologics initiated
- Phase 2 data and natural history published literature comparison continue to support potential to become Pompe standard of care
- Peak revenue potential of \$1B-\$2B, with exclusivity well into 2030s

Gene Therapy: Updates & Key Takeaways



Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

- CLN6 Phase 2 interim data shows profound impact with potential to become first ever approved gene therapy for fatal brain disease in children
- Additional patients to be dosed in Phase 2 study of CLN3 (largest cause of childhood neurodegeneration, 5,000+ children)
- Orphan drug designations granted in U.S. and EU for intrathecal AAV gene therapies for CLN6 and CLN3 Batten disease.
- Pompe gene therapy clinical candidate declared to move into IND-enabling studies
- Penn Collaboration is R&D engine, with rights to 50+ diseases
- 8 preclinical gene therapies in development



Closing Remarks

“We are business led and science driven”
- Amicus Belief Statement

2019 Key Strategic Priorities

- 1** **Nearly double annual revenue for Galafold[®] (guidance \$170M-\$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**

Our Passion for Making a Difference Unites Us

Amicus is Now at a Major Inflection Point and Positioned to Create Significant Shareholder Value Ahead while Advancing our Mission for Patients



Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement



Appendix

Reconciliation

Amicus Therapeutics, Inc.
Reconciliation of Non-GAAP Financial Measures
(in thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Total operating expenses - as reported GAAP	\$ 100,477	\$ 172,467	\$ 326,940	\$ 307,835
Research and development:				
Share-based compensation	3,106	2,905	12,090	8,603
Research and development asset acquisition expense	-	100,000	-	100,000
Selling, general and administrative:				
Share-based compensation	5,737	4,149	19,432	12,270
Changes in fair value of contingent consideration payable	789	1,300	2,652	2,700
Depreciation and amortization	1,116	1,073	3,261	3,015
Total operating expense adjustments to reported GAAP	10,748	109,427	37,435	126,588
Total operating expenses - as adjusted	\$ 89,729	\$ 63,040	\$ 289,505	\$ 181,247