



# 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

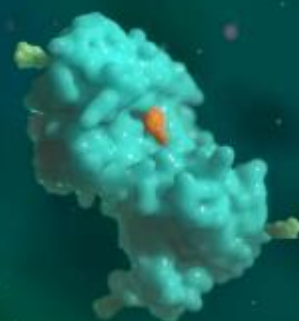


## Global Footprint in 27 Countries



## AT-GAA

Phase 3 in  
Pompe Disease



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

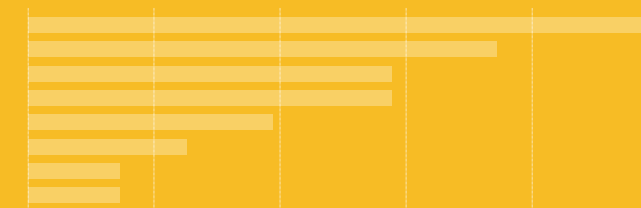
**Two Clinical-  
Stage Gene  
Therapies**

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

<sup>1</sup>Inclusive 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 (Enzyme Replacement Therapy))					
Pompe Gene Therapy	PENN				

**Batten Franchise**

CLN6 Batten	NCH			
CLN3 Batten	NCH			
CLN8 Batten	NCH			
CLN1 Batten	NCH			

**Next-generation Ophthalmic and CNS Gene Therapies**

CDKL5 Deficiency	PENN	
Niemann-Pick	NCH / PENN	
Tay-Sachs Disease	NCH	
Other	NCH / PENN	

**MPS Franchise**

Mepsevii™ (vestronidase alfa) (Japan Only)*				
Next Generation MPSIIIA	PENN			
MPSIIIB	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 Runway <sup>1</sup>	Well Into 1H2022
Debt <sup>2</sup>	\$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+

<sup>1</sup>Based on existing operating plan <sup>2</sup>Includes \$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<sup>®</sup> (migalastat) Global Launch...

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

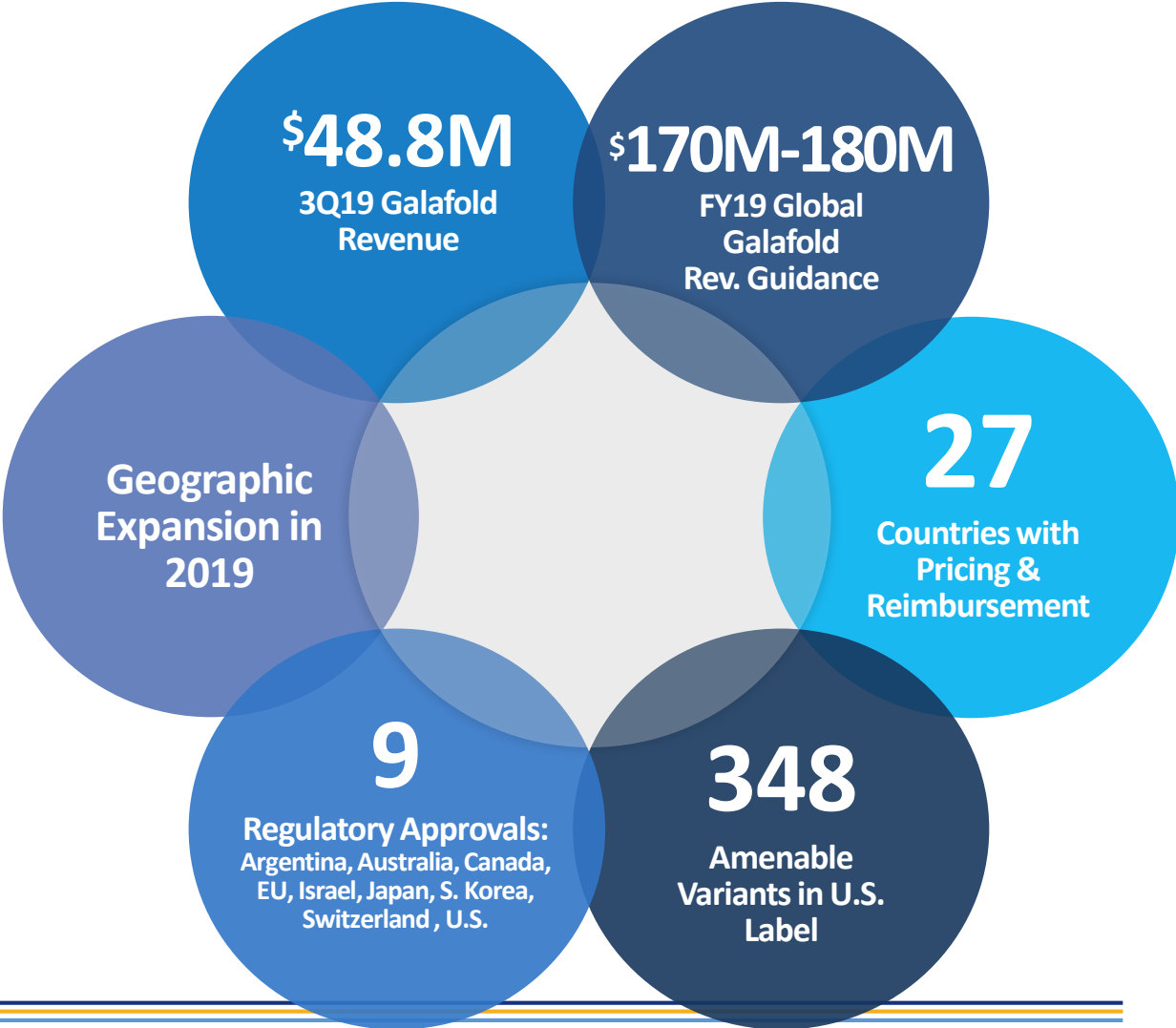
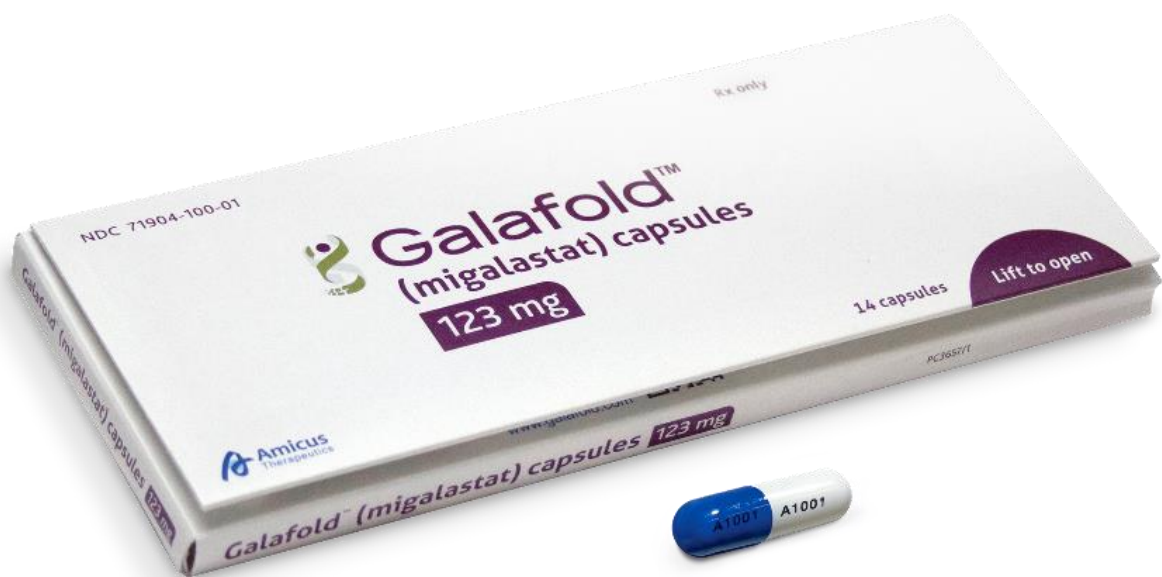
*“We push ideas as far and as fast as possible”*

- 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 (≥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](http://www.ema.europa.eu).

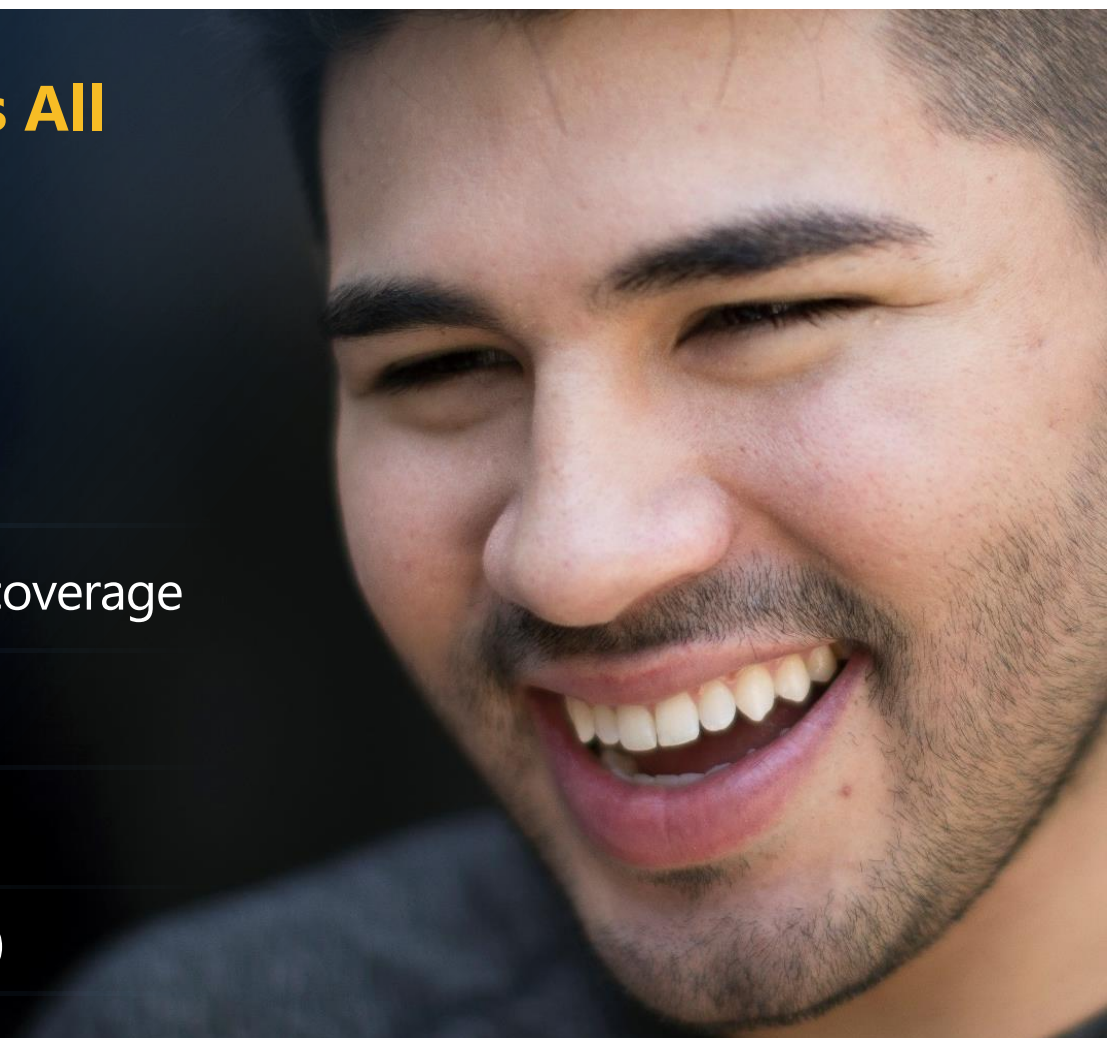


# Galafold Global Launch Momentum (as of September 30, 2019)

**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%)

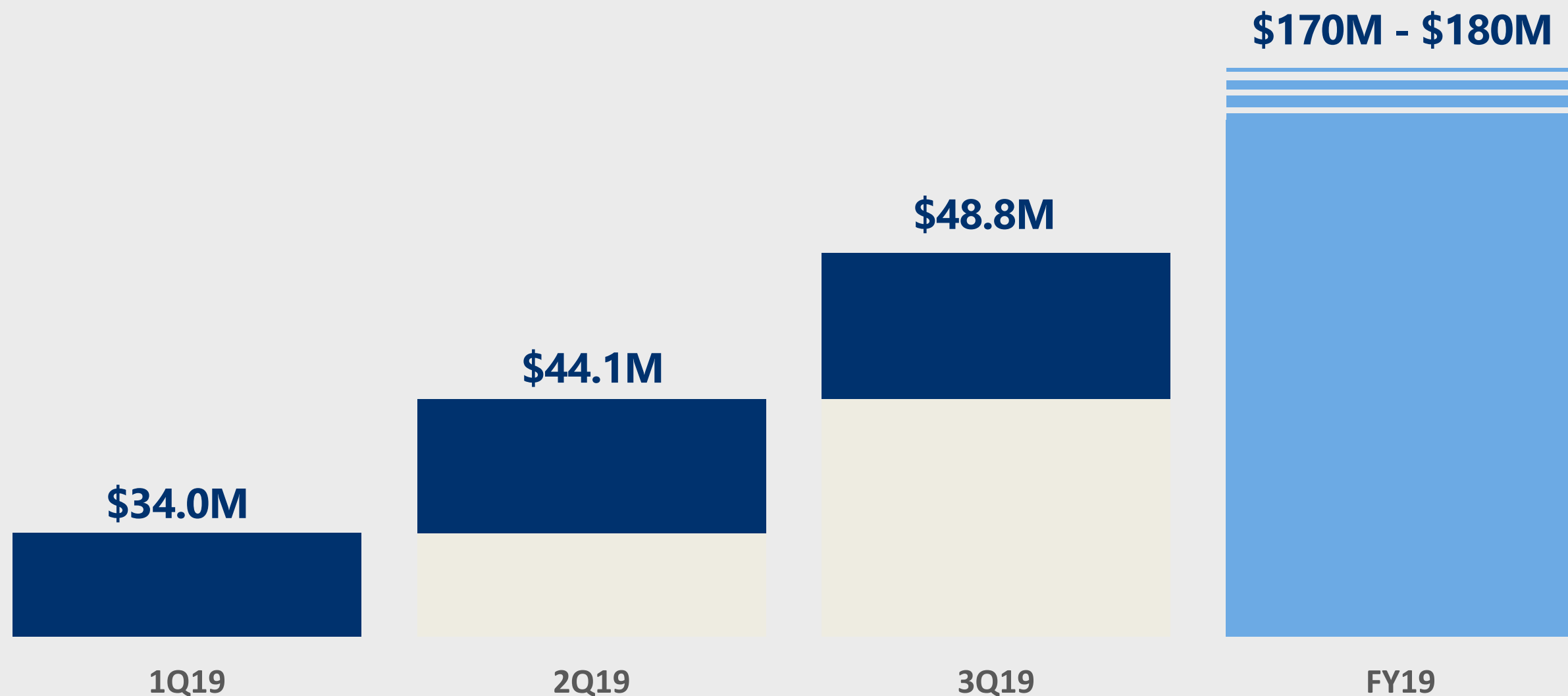


\*Market share based on reported global Fabry sales for the calendar year ending 3Q19 and assumes a 35% amenability rate.



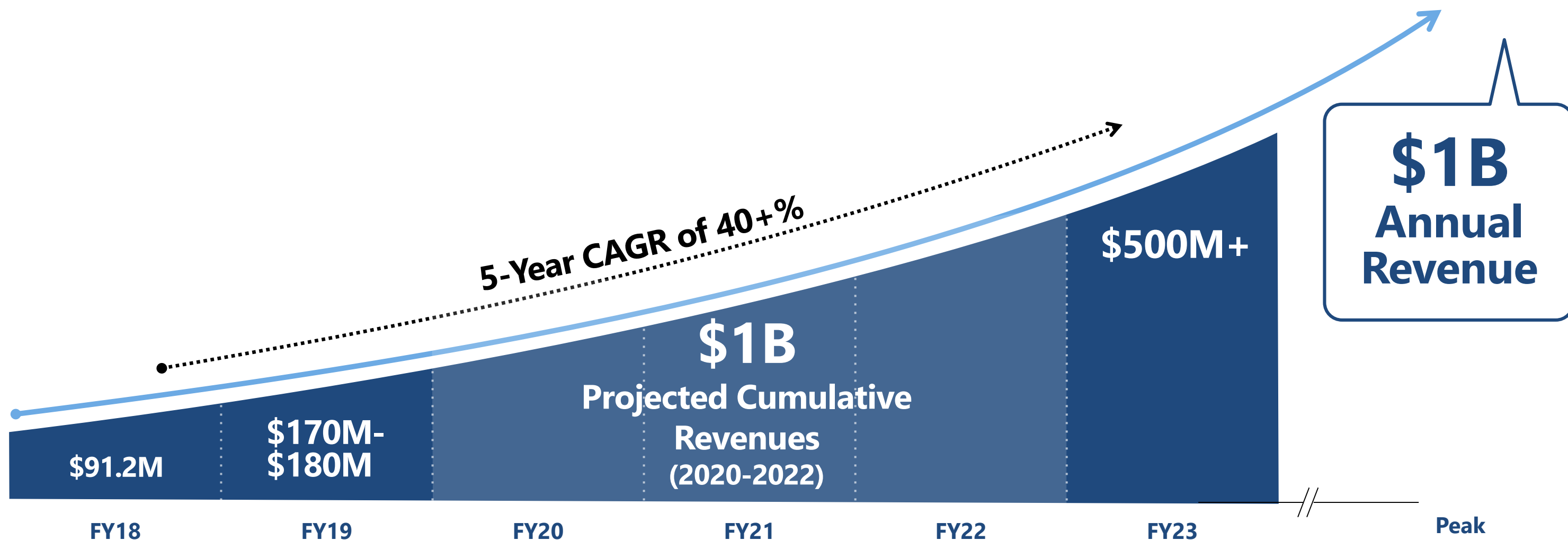
# Galafold Success and FY19 Galafold Revenue Guidance

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