2019 Financial Results Conference Call & Webcast

March 2, 2020

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 presentation 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, 2019 to be filed today. 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.

In addition to financial information prepared in accordance with U.S. GAAP, this presentation 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. We typically exclude certain GAAP items that management does not believe affect our basic operations and that do not meet the GAAP definition of unusual or non-recurring items. Other companies may define these measures in different ways. Full reconciliations of GAAP results to the comparable non-GAAP measures for the reported periods appear in the financial tables section of this presentation. When we provide our expectation for non-GAAP operating expenses on a forward-looking basis, a reconciliation of the differences between the non-GAAP expectation and the corresponding GAAP measure generally is not available without unreasonable effort due to potentially high variability, complexity and low visibility as to the items that would be excluded from the GAAP measure in the relevant future period, such as unusual gains or losses. The variability of the excluded items may have a significant, and potentially unpredictable, impact on our future GAAP results.

A RARE COMPANY

A leading fully-integrated, global rare disease biotechnology company

Galafold® (migalastat)

First Oral Precision Medicine for Fabry Disease

Gene Therapy PLATFORM

Protein Engineering & Glycobiology

World Class BIOLOGICS Capabilities

EMPLOYEES in 27 Countries

AT-GAA

Phase 3 in Pompe Disease

$450M+ Cash as of 12/31/19

Two Clinical-Stage Gene Therapies

GLOBAL COMMERCIAL ORGANIZATION

Robust R&D Engine

Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases

Key Takeaways

Recent successes across our science, clinical, regulatory and commercial efforts position us for the future

Galafold Continues Strong Launch Performance & Cornerstone of Amicus Success

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

Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

Strong Financial Outlook with Current Cash Well into 2022

2020 Key Strategic Priorities

1. **Achieve global product revenue for Galafold of $250M-$260M**
2. **Complete Pompe Phase 3 PROPEL study, enroll pediatric studies and advance manufacturing to support 2021 BLA and MAA**
3. **Advance clinical development, manufacturing and regulatory discussions for CLN6 and CLN3 Batten programs**
4. **Progress Pompe gene therapy towards IND and disclose up to two additional IND candidates**
5. **Maintain strong financial position**

Galafold® (migalastat) Global Launch…

…taking a leadership role in the treatment of Fabry disease

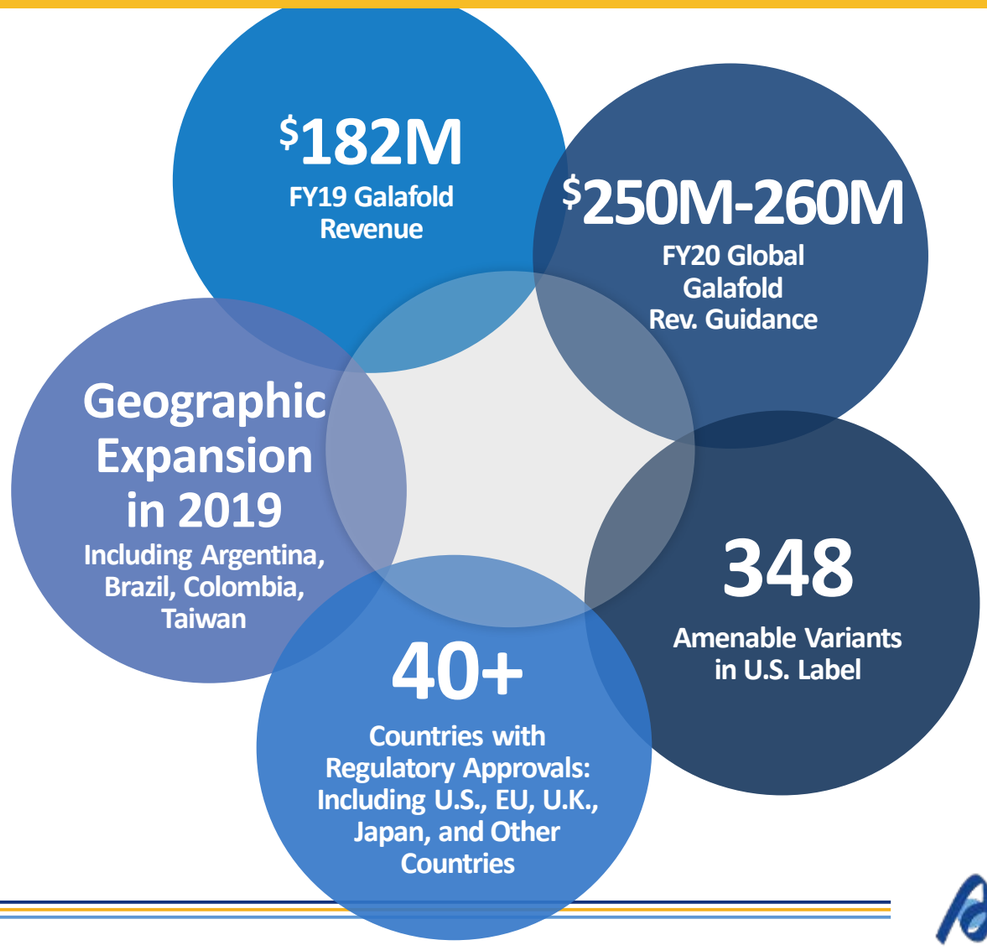
“We push ideas as far and as fast as possible”

- Amicus Belief Statement

Galafold Snapshot (as of December 31, 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

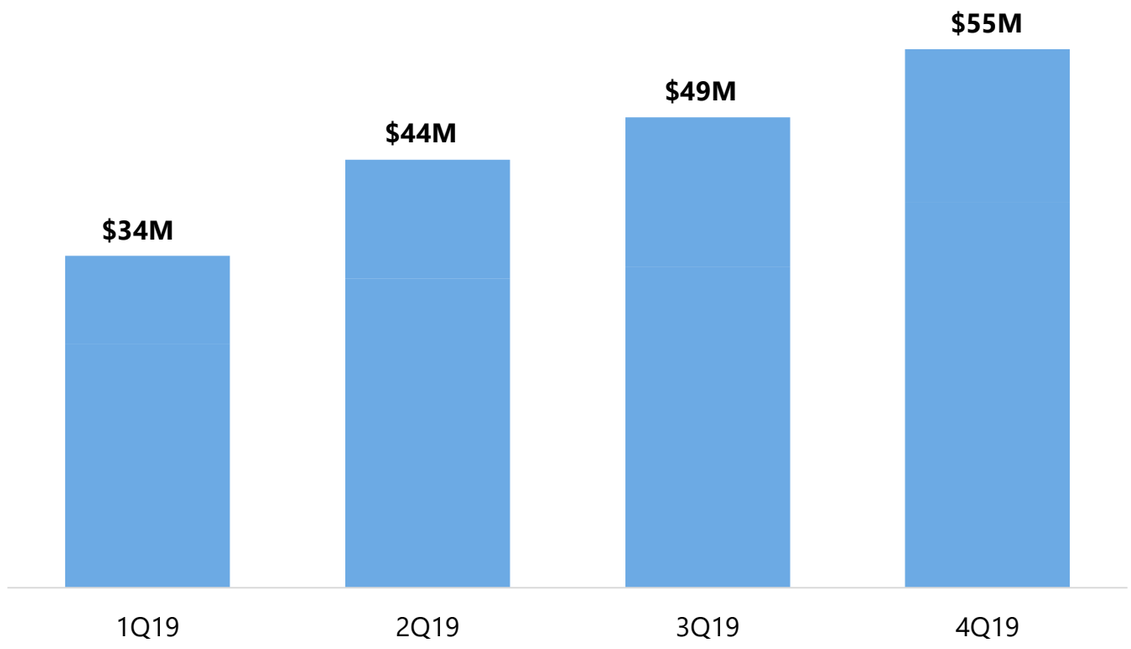
One of the Most Successful Rare Disease Launches

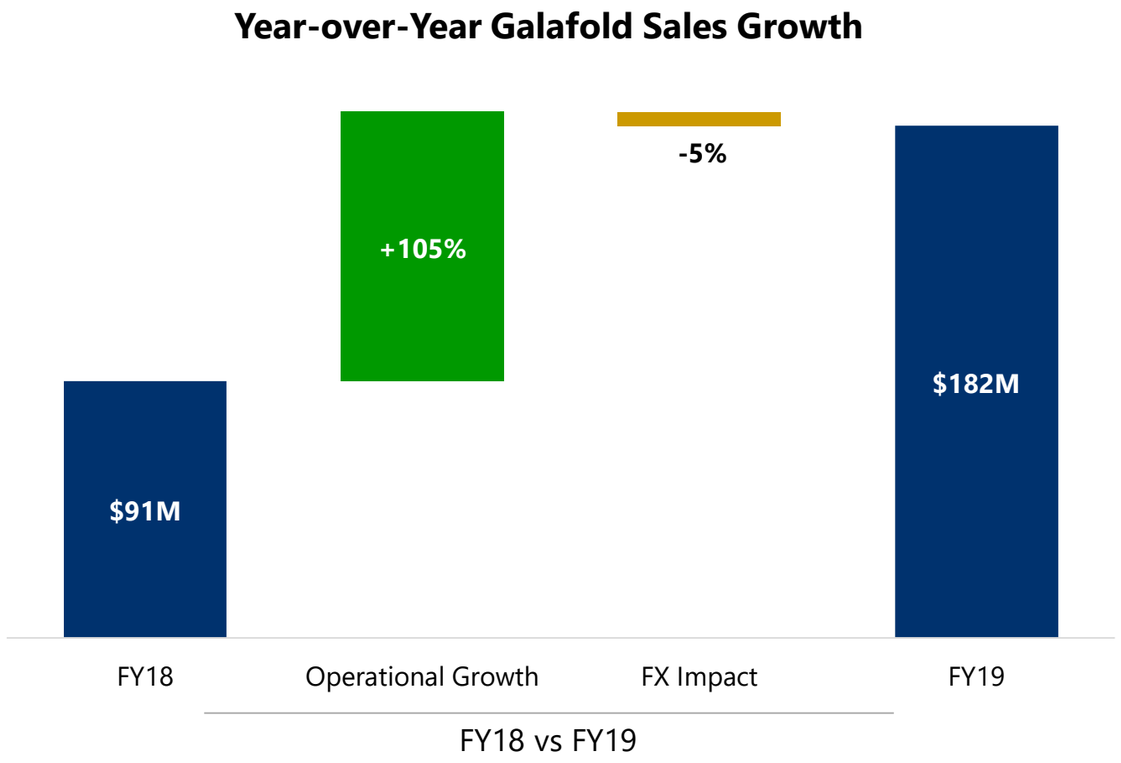


Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable mutation/variant. The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia. For additional information about Galafold, including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

2019 Galafold Success

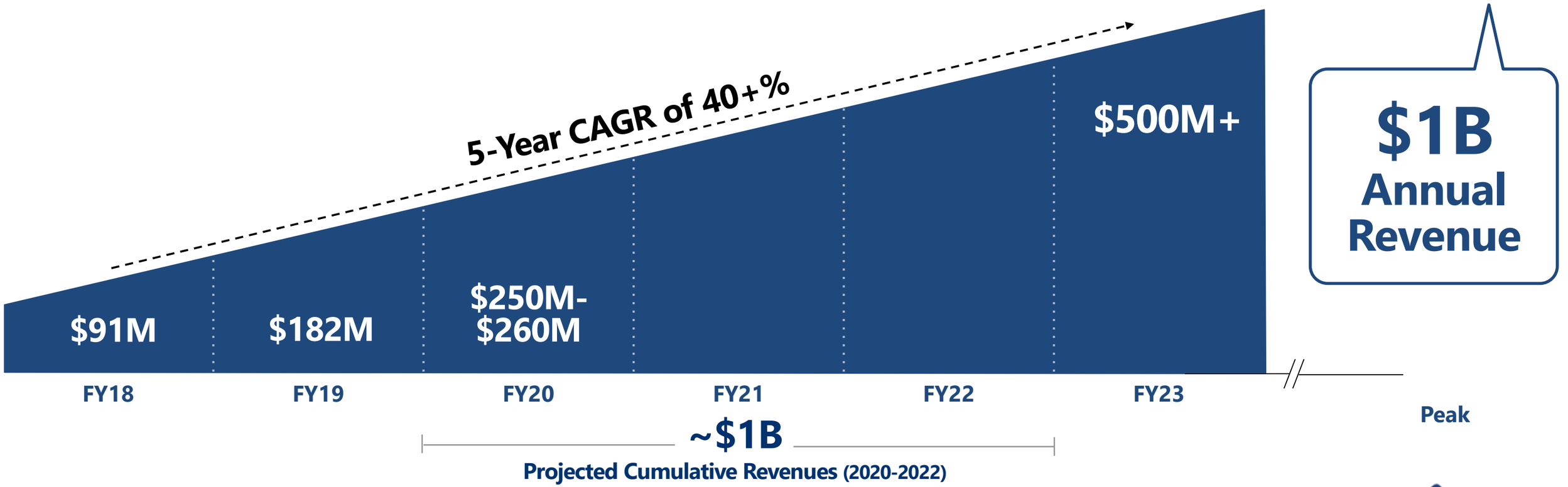
Strong full-year revenue performance of $182M, exceeding guidance of $170M-$180M





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



AT-GAA: Next Potential Standard of Care for Pompe Disease

“We encourage and embrace constant innovation”

- Amicus Belief Statement

U.S. FDA Granted BTD to AT-GAA in Late-Onset Pompe Disease (LOPD)

AT-GAA is the first ever second-generation product for <u>any</u> lysosomal disorder to earn FDA Breakthrough Therapy Designation (BTD)

Plans to apply for and initiate a rolling BLA submission for AT-GAA in LOPD in 2020

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

- Improvements in 6-minute walk distance
- Comparison to natural history of treated patients

BTD Features

- Intensive guidance on an efficient drug development program
- Organizational commitment involving senior agency staff
- All Fast Track program features including rolling submission

BTD Criteria

- Intended to treat a serious or life-threatening disease or condition
- Preliminary clinical evidence indicates drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints

AT-GAA: Key Takeaways

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

- PROPEL pivotal study exceeded enrollment with data expected 1H2021
- Breakthrough Therapy Designation and the Promising Innovative Medicine designation highlight unmet need in Pompe disease today
- Plan to submit and initiate rolling submission of Biologics License Application in 2020
- Manufacturing PPQ runs at WuXi biologics on track
- Peak revenue potential of $1B-$2B, with exclusivity well into 2030s

Financial Summary

“We are business led and science driven”

- Amicus Belief Statement

2019 Select Financial Results

2019 revenue of $182M from global Galafold sales

(in thousands, except per share data)	Dec. 31, 2019	Dec. 31, 2018
Product Revenue	$182,237	$91,245
Cost of Goods Sold	21,963	14,404
R&D Expense*	286,378	270,902
SG&A Expense	169,861	127,200
Changes in Fair Value of Contingent Consideration	3,297	3,300
Depreciation and Amortization	4,775	4,216
Loss from Operations	(304,037)	(328,777)
Income Tax (Expense) Benefit	(478)	94
Net Loss	(356,388)	(348,995)
Net Loss Per Share	(1.48)	(1.88)

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

Cash Runway Now to Well into 2022 (~2+ years)

Fully funded through major milestones in portfolio and continued global growth

$450M+ Cash YE2019

~2+ Years Cash Runway

Well into 2022

1Exclusive license from Ultragenyx for Japanese rights to Mepsevii®; investigator-sponsored trial in Japan underway

Financial Outlook: Key Takeaways

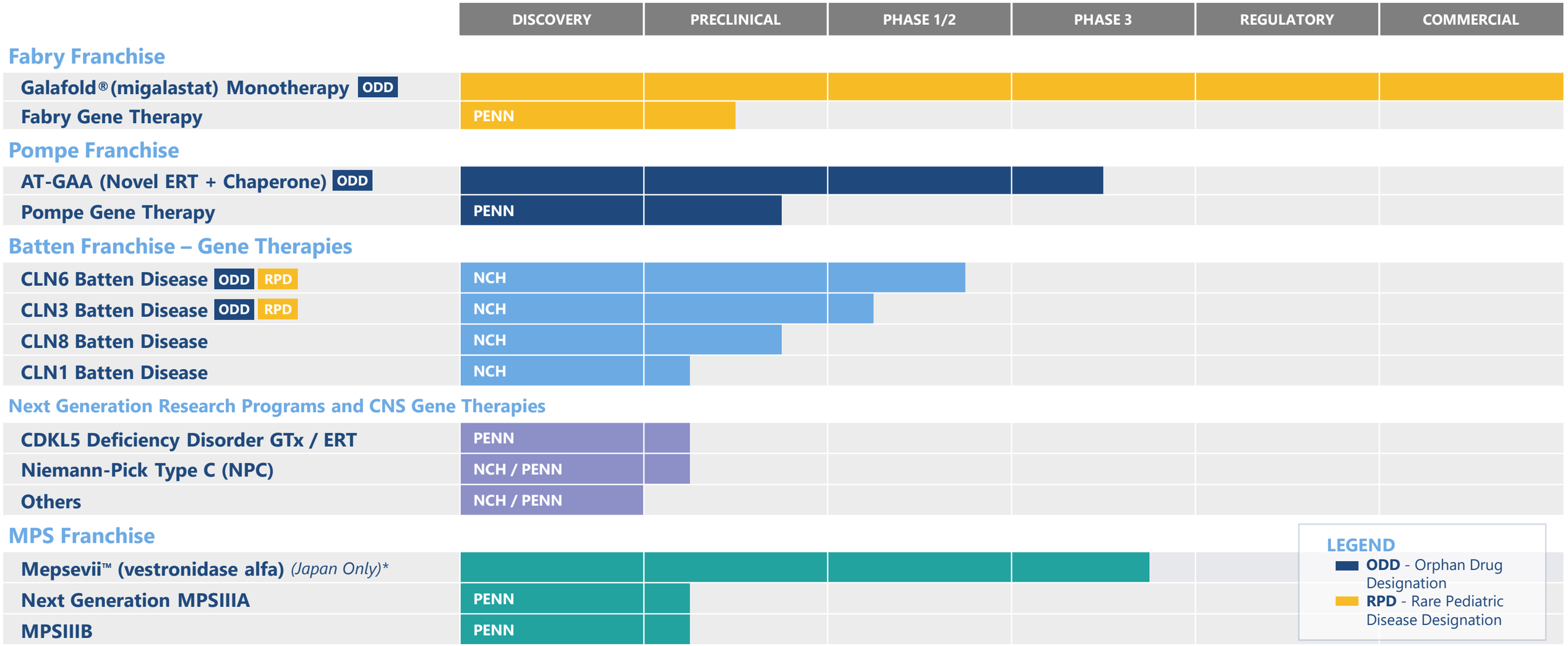
- Company fully funded through major milestones in portfolio and continued global growth
- Cumulative Galafold projected revenue of $1B+ in 2020-2022 offsets significant majority of company spend/investments
- Extended cash flow runway through OpEx savings, CapEx phasing, program prioritization and increased Galafold revenue projections
- No material business development planned or needed in next several years
- Only modest additional capital required in the outer years to extend runway into profitability with multiple non-equity sources available as/when needed

Next Generation Gene Therapy Platform

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement

A RARE PORTFOLIO



*Exclusive license from Ultragenyx for Japanese rights to Mepsevii™, investigator-sponsored trial in Japan underway

Amicus Therapeutics

Combines Amicus and Penn Expertise Across Lysosomal and Rare Diseases

An R&D platform with rights to 50+ diseases, including 8 active preclinical programs

Amicus Therapeutics®

Protein Engineering & Glycobiology Expertise

Clinical and Regulatory Expertise

Global Commercial Infrastructure

Next-Generation Gene Therapy Platform

Team of 200+ scientists bringing expertise and experience in:

Vectors, Tropisms, Capsids

Safety

Dosing, Immunology

Manufacturing, Scalability

Penn
University of Pennsylvania

Driving 1-2 new INDs every year starting in 2020

Gene Therapy: Updates & Key Takeaways

Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

- CLN6 Phase 1/2 interim data shows profound impact with potential to become first ever approved gene therapy for fatal brain disease in children
- Plan to report initial data for patients enrolled in CLN3 Phase 1/2 study in 2H'20
- Orphan drug designations granted in U.S. and EU for intrathecal AAV gene therapies for CLN6 and CLN3 Batten disease.
- Pompe gene therapy moving 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

Thank You

"Our passion for making a difference unites us"

-Amicus Belief Statement

Amicus Therapeutics®

Appendix

Amicus Therapeutics®

Non-GAAP Reconciliation

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

	Years Ended December 31,		
	2019	**2018**	**2017**
Total operating expenses - as reported GAAP	$ 464,311	$ 405,618	$ 472,679
Research and development:			
Share-based compensation	17,575	11,740	10,328
Research and development asset acquisition expense	—	100,000	—
Selling, general and administrative:			
Share-based compensation	26,855	17,520	12,773
Loss on impairment of assets	—	—	465,427
Changes in fair value of contingent consideration payable	3,297	3,300	(234,322)
Depreciation and amortization	4,775	4,216	3,593
Total operating expense adjustments to reported GAAP	52,502	136,776	257,799
Total operating expenses - as adjusted	$ 411,809	$ 268,842	$ 214,880