39th Annual J.P. Morgan Healthcare Conference

John F. Crowley, Chairman and Chief Executive Officer

January 12, 2021

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 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, 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, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations from the COVID-19 pandemic is inherently unknown and cannot be predicted with confidence and may cause actual results and performance to differ materially from the statements in this release, including without limitation, because of the impact on general political and economic conditions, including as a result of efforts by governmental authorities to mitigate COVID-19, such as travel bans, shelter in place orders and third-party business closures and resource allocations, manufacturing and supply chain disruptions and limitations on patient access to commercial or clinical product. In addition to the impact of the COVID-19 pandemic, 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. Statements regarding corporate financial guidance and financial goals and the attainment of such goals. 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 and the Quarterly Report filed on Form 10-Q for the quarter ended September 30, 2020. 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.

Non-GAAP Financial Measures

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. 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

GLOBAL COMMERCIAL ORGANIZATION

Robust R&D Engine

Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases

Cash Sufficient to Achieve Self-Sustainability

Two Clinical-Stage Gene Therapies

Making A Difference – People and Culture

A commitment to Diversity, Equity and Inclusion

BEGINNING 2021

Strengthen our culture of inclusivity by delivering on our diversity, equity and inclusion programs

- 50% of all hiring slates will include diverse* candidates
- 50% of all Director and above hires should be diverse
- 33% of all other hires should be diverse

2023 AND BEYOND

- Maintain global gender diversity of 50% or greater
- Increase U.S. diversity* to 40%
- Maintain pay parity

**Diversity includes maintaining/increasing gender diversity and increasing representation of all underrepresented races, veterans, disabled, and LGBTQ employees*

2021: Our Passion for Making a Difference Unites Us

Per Ardua Ad Astra







Positioned for Significant Value Growth

Strong Revenue Drivers

- Exceptional Galafold launch continues with $1B+ opportunity
- Anticipate further growth driven by potential AT-GAA launch (2022) - a $1B-$2B+ opportunity

Diverse Gene Therapy Portfolio

- Broad clinical and preclinical pipeline
- Established clinical proof of concept in CLN6 Batten disease
- Gene therapy platform with rights to 50+ lysosomal disorders and 12 additional rare diseases; together a $1B+ opportunity

Financial Strength

- Cash position sufficient to achieve self-sustainability without the need for future dilutive financing
- Non-GAAP Operating Expense to remain flat YoY driven by strong financial discipline

A RARE PORTFOLIO

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMMERCIAL
Fabry Franchise						
Galafold®(migalastat) Monotherapy ODD						
Fabry Gene Therapy	PENN					
Pompe Franchise						
AT-GAA (Novel ERT + Chaperone) ODD BTD						
Pompe Gene Therapy	PENN					
Batten Franchise – Gene Therapies						
CLN6 Batten Disease ODD RPD PRIME	NCH					
CLN3 Batten Disease ODD RPD	NCH					
CLN1 Batten Disease	NCH/PENN					
Next Generation Research Programs and CNS Gene Therapies						
CDKL5 Deficiency Disorder GTx / ERT	PENN					
Angelman Syndrome	PENN					
Others	NCH / PENN					
MPS Franchise						
Mepsevii™ (vestronidase alfa) *(Japan Only)**						
Next Generation MPSIIIA	PENN					
MPSIIIB	PENN					

LEGEND

- **ODD** - Orphan Drug Designation
- **RPD** - Rare Pediatric Disease Designation
- **PRIME** - Priority Medicines Designation
- **BTD** - Breakthrough Therapy Designation

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

Amicus Therapeutics

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** ✓

2021 Key Strategic Priorities

1. **Achieve double-digit Galafold growth and revenue of at least $300M+**
2. **Report data from the AT-GAA Phase 3 PROPEL study and complete BLA and MAA filings for regulatory approvals**
3. **Advance clinical studies, regulatory discussions and scientific data across industry leading gene therapy pipeline**
4. **Further manufacturing capabilities and capacity to build world-class technical operations to support all gene therapy programs**
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, 2020)

Galafold 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 ERT

One of the Most Successful Rare Disease Launches

- **~$261M** 2020 Galafold Revenue*
- **1,384** Amenable Mutations Included in the EU Label
- **$300M+** FY21 Global Galafold Revenue
- **Continued Expansion in 2021**
- **348** Amenable Variants in U.S. Label
- **40+** Countries with Regulatory Approvals

Galafold is indicated for adults with a confirmed diagnosis of Fabry Disease and an amenable 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.

*Preliminary and unaudited

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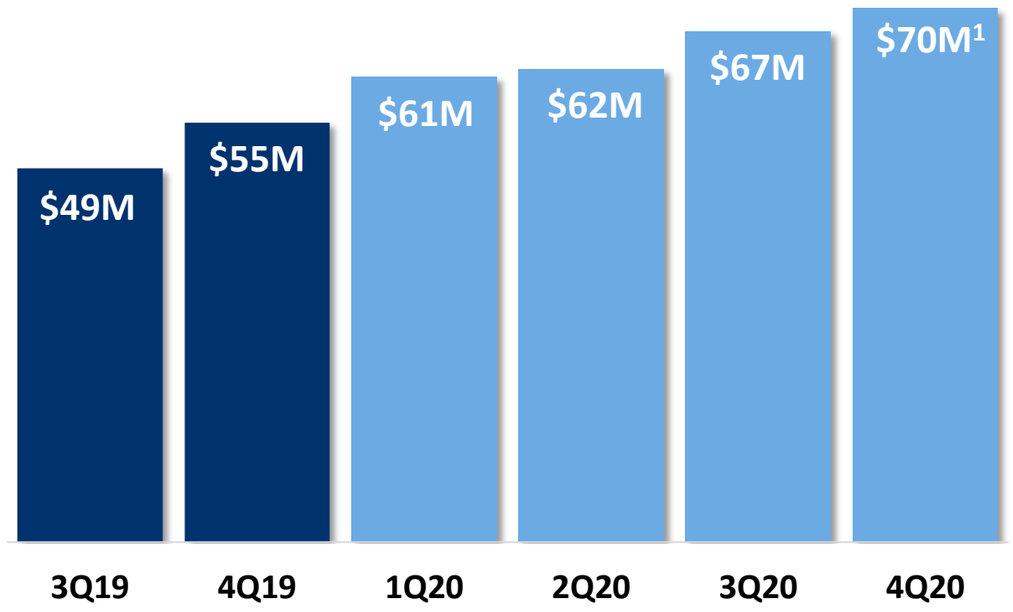
2020 Galafold Success

Growth remains strong with Q4 revenue of $70M and FY 2020 revenue of $261M[1]



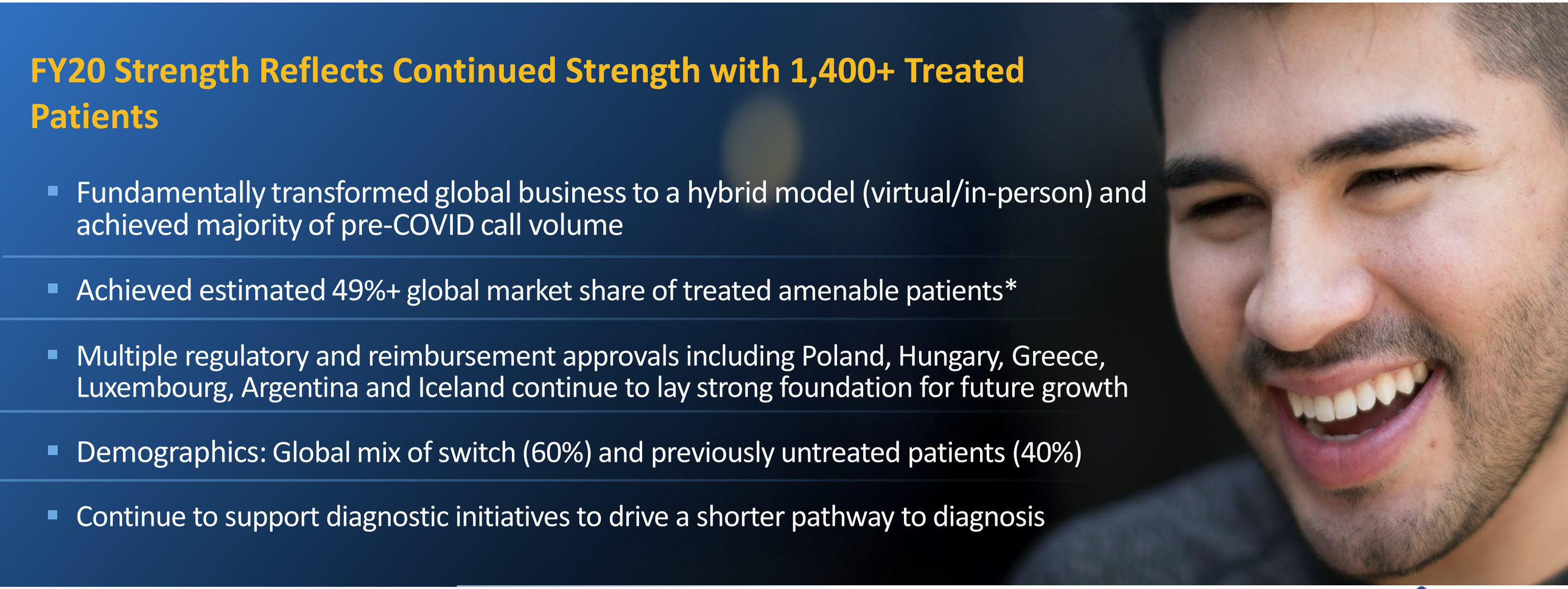
[1]Preliminary and unaudited

Galafold Global Commercial Momentum (as of December 31, 2020)

2020 exceeded revenue goals even with COVID related disruptions to healthcare systems

FY20 Strength Reflects Continued Strength with 1,400+ Treated Patients

- Fundamentally transformed global business to a hybrid model (virtual/in-person) and achieved majority of pre-COVID call volume
- Achieved estimated 49%+ global market share of treated amenable patients*
- Multiple regulatory and reimbursement approvals including Poland, Hungary, Greece, Luxembourg, Argentina and Iceland continue to lay strong foundation for future growth
- Demographics: Global mix of switch (60%) and previously untreated patients (40%)
- Continue to support diagnostic initiatives to drive a shorter pathway to diagnosis



*Market share based on reported global Fabry sales for the calendar year ending 3Q20 and assumes a 35% amenability rate

Amicus Therapeutics

Adapting to a New Way of Delivery

Amicus was able to fully transform its commercial model to adapt to a new environment and achieve a substantial majority of pre-COVID touchpoints

Conducted HCP research to understand their needs and best channels to utilize

Retrained team members on Fabry disease to ensure that virtual calls were impactful

Global work to bring external programs and initiatives under one digital umbrella

All affiliates are trained and fully utilizing digital channels for external communication

Well positioned for continued success in 2021

Outlook for 2021

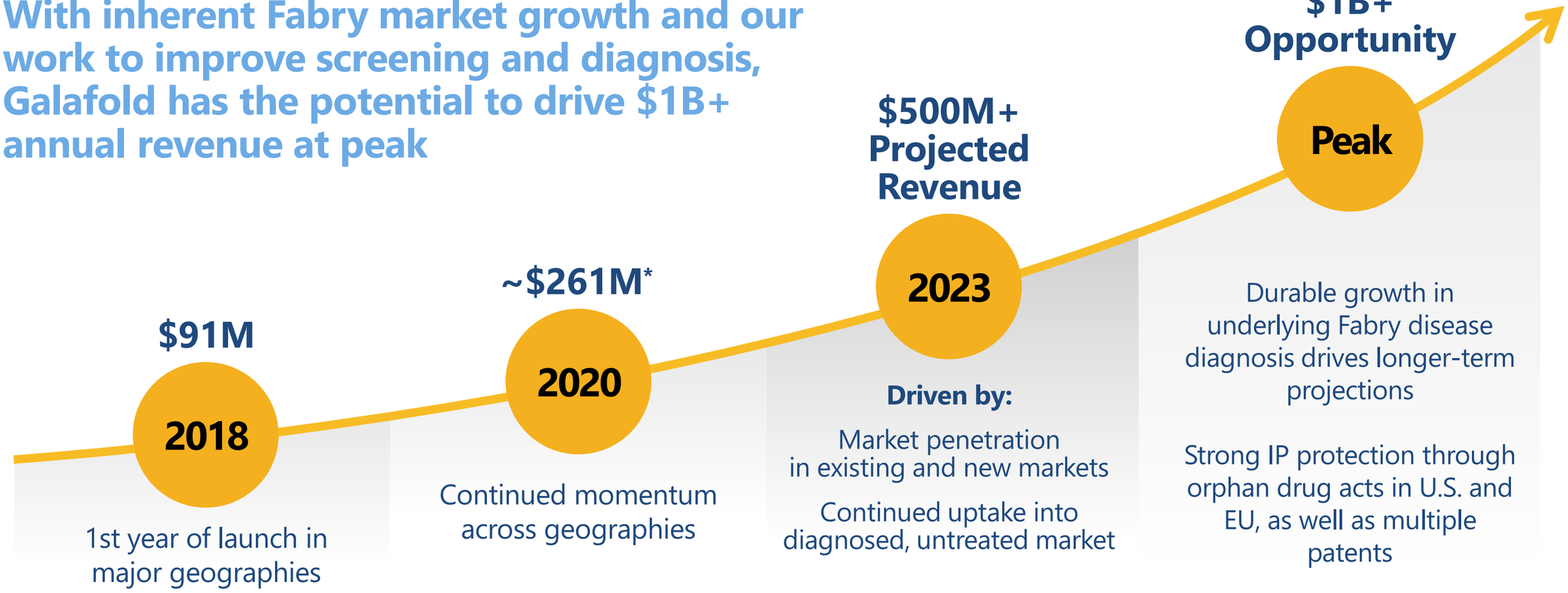
Continued double-digit Galafold revenue growth to at least $300 million in 2021

Galafold Continues Strong Launch Performance & Cornerstone of Amicus Success

- 2020 execution lays a solid foundation and global demand remains strong with continued growth anticipated in 2021 and beyond
- New Galafold patient additions slowed in Q4 due to COVID reemergence and resulting in increased lag time between patient identification and treatment initiation
- In 2021, project double-digit revenue growth with new patient starts to be at least consistent with 2020
- Expect higher patient adds and revenue growth in the second half of 2021 as COVID impact eases
- Continue to see greater than 90% compliance and adherence rates globally

Galafold Opportunity

With inherent Fabry market growth and our work to improve screening and diagnosis, Galafold has the potential to drive $1B+ annual revenue at peak



*Preliminary and unaudited

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Galafold Long-Term Opportunity

With inherent Fabry market growth and our work to improve diagnosis and screening of this underdiagnosed and misdiagnosed disorder, Galafold has the potential to drive $1B+ annual revenue at peak

~15,000+
Total Diagnosed Fabry Patients[1]

~9,000+
Total Treated Fabry Patients

~3,000+ T_x

Treated Amenable Patients

Patients on a Fabry therapy with a genetic mutation that is amenable to Galafold treatment

~6,000+ D_x

Diagnosed Untreated Patients[2]

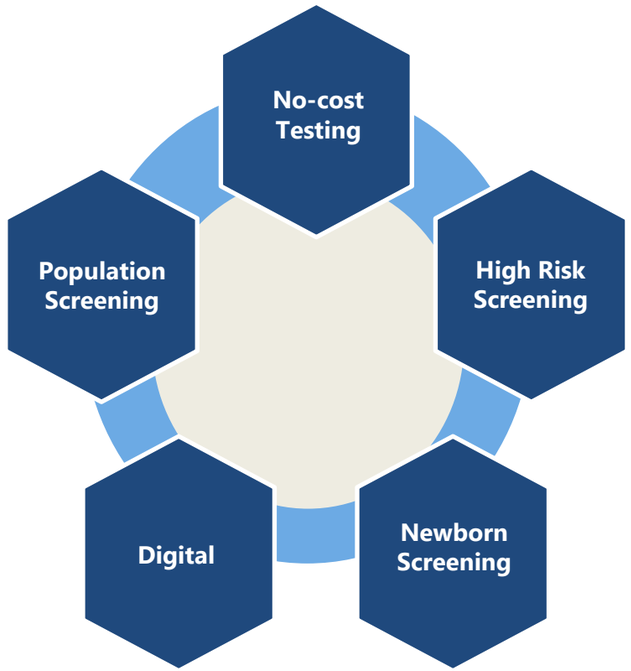
Patients with a Fabry diagnosis who are not currently on any treatment

~1,400+ R_x

Galafold Patients on Therapy

Patients who are currently on Galafold as a treatment for their Fabry disease

Diagnostic Initiatives



1. Management estimates excluding China, India and other non-commercial countries. 2. Company estimates 35% up to 50% of diagnosed untreated patients have an amenable variant

Amicus Therapeutics

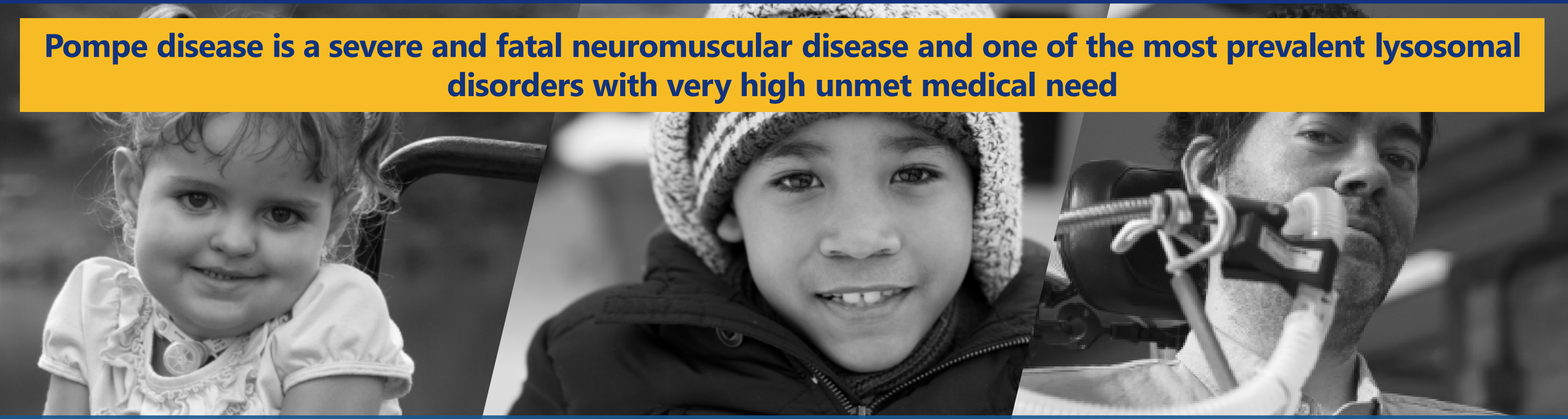
AT-GAA: Next Potential Standard of Care for Pompe Disease

“We encourage and embrace constant innovation”

- Amicus Belief Statement

Pompe Disease Overview

Pompe disease is a severe and fatal neuromuscular disease and one of the most prevalent lysosomal disorders with very high unmet medical need



5,000 – 10,000+ patients diagnosed WW[1]; newborn screening suggests underdiagnosis

Age of onset ranges from infancy to adulthood

Patients on current standard of care decline after ~2 years

Respiratory and cardiac failure are leading causes of morbidity and mortality

Deficiency of GAA leading to glycogen accumulation and cellular dysfunction

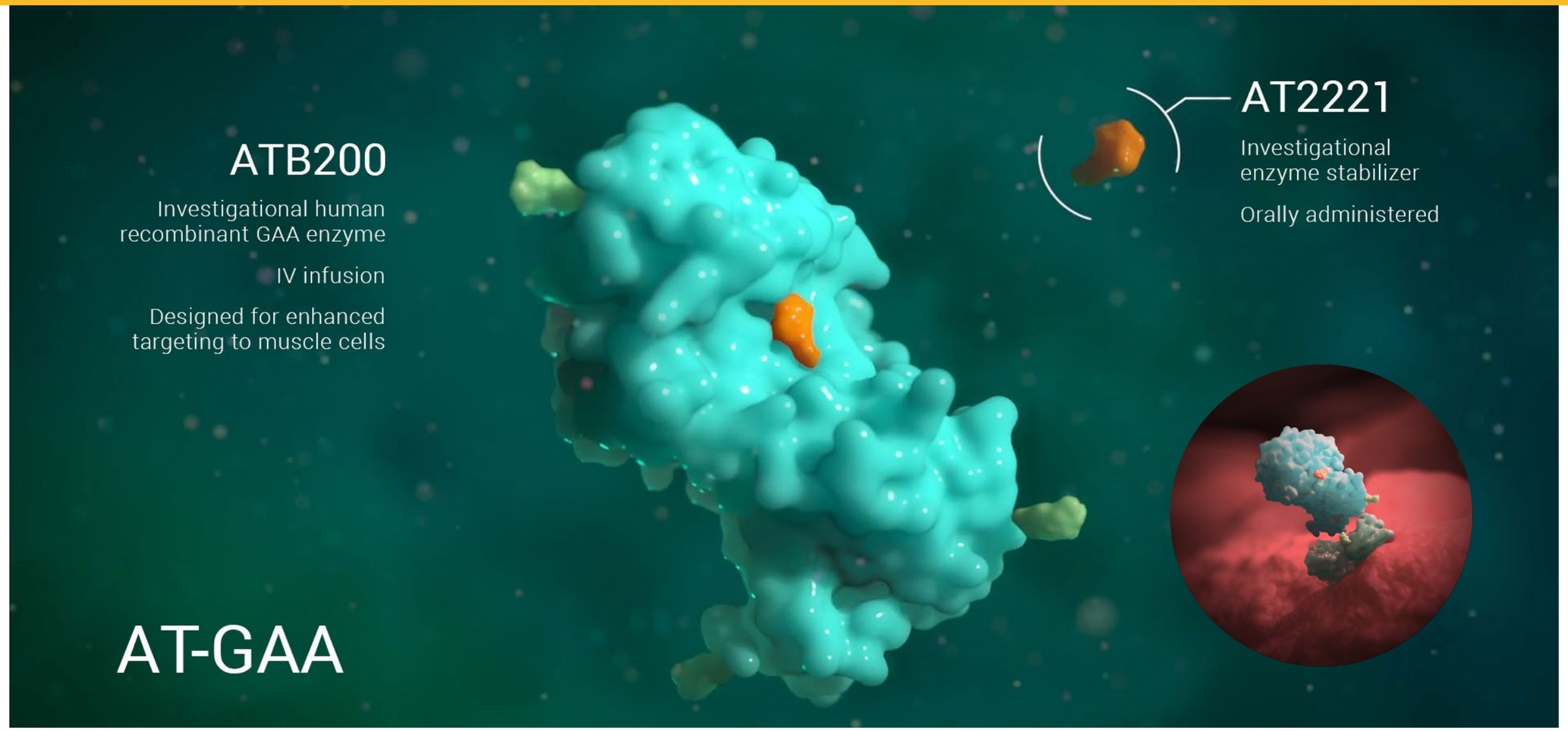
Symptoms include muscle weakness, respiratory failure and cardiomyopathy

~$1B+ global Pompe ERT sales[2]

1. National Institute of Neurological Disorders and Stroke (NIH). 2. Based on calendar year ending September 30, 2019. Exchange rate as of 1/6/19. Source: Sanofi Press Releases

AT-GAA: Foundation in Protein Engineering

Amicus scientists created a uniquely glycosylated and highly phosphorylated ERT (AT-GAA) that significantly enhances targeting to key muscles affected



Pompe Patient Experience in Phase 1/2 Clinical Study (ATB200-02)

Highly differentiated mechanism of action demonstrated compelling Phase 1/2 results showing strong and durable effects in patients out to two years

6-Min Walk Test (m)

Cohort	Baseline (n=10)	Change at Month 6 (n=10[1]) Mean (SD)	Change at Month 12 (n=10[1]) Mean (SD)	Change at Month 24 (n=9[1,2]) Mean (SD)
Cohort 1 ERT-Switch Ambulatory	397.2 (96.8)	+23.9 (52.2)	+42.2 (46.5)	+36.4 (60.5)
Cohort	**Baseline (n=5)**	**Change at Month 6 (n=5) Mean (SD)**	**Change at Month 12 (n=5) Mean (SD)**	**Change at Month 24 (n=5) Mean (SD)**
Cohort 3 ERT-Naïve	399.5 (83.5)	+41.8 (29.4)	+63.1 (29.1)	+60.7 (36.5)

FVC (% Predicted)

Cohort	Baseline (n=9[3])	Change at Month 6 (n=9[3]) Mean (SD)	Change at Month 12 (n=9[3]) Mean (SD)	Change at Month 24 (n=8[2,3]) Mean (SD)
Cohort 1 ERT-Switch Ambulatory*	52.6 (14.7)	-1.2 (4.0)	-3.0 (6.0)	+0.9 (4.9)
Cohort	**Baseline (n=5)**	**Change at Month 6 (n=5) Mean (SD)**	**Change at Month 12 (n=5) Mean (SD)**	**Change at Month 24 (n=5) Mean (SD)**
Cohort 3 ERT-Naïve	53.4 (20.3)	+4.4 (5.6)	+4.6 (8.8)	+6.8 (6.8)

Data from interim analysis 8.

[1] One patient in Cohort 1 discontinued after 18 weeks due to burden of travel; baseline value is not shown for this patient. [2] One patient in Cohort 1 discontinued from study before Month 24. [3] Baseline FVC not available for 1 patient in Cohort 1

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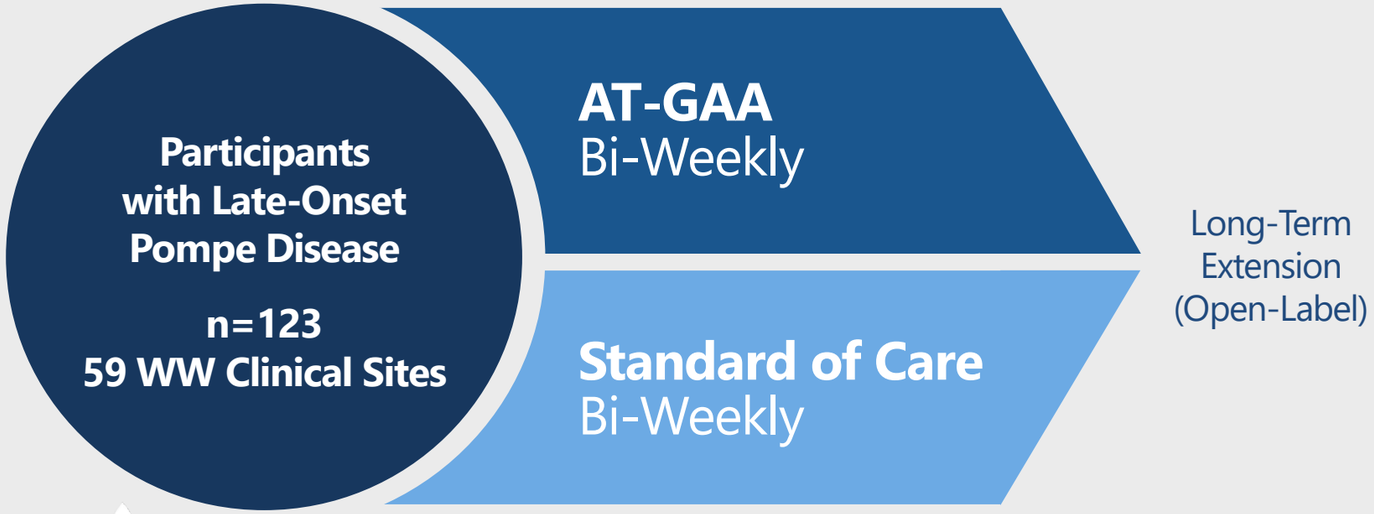
PROPEL (ATB200-03) Study Design

Phase 3 exceeded enrollment and last patient, last visit complete with data expected in 1Q2021 – Highly powered for success and supports a broad label

52-Week Primary Treatment Period (Double-Blind)

2:1 Randomization



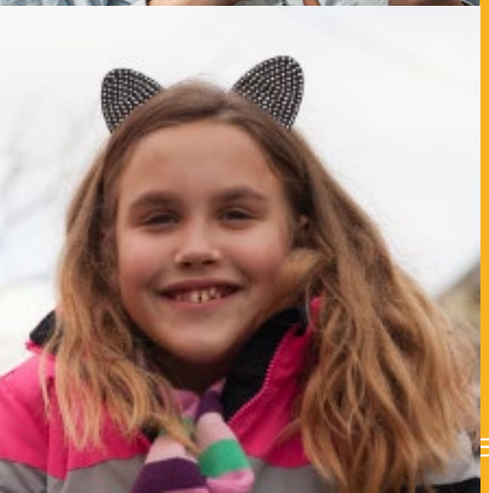
Primary Endpoint: 6-Minute Walk Test at Week 52; Multiple Secondary Endpoints

- **PROPEL pivotal study over-enrolled with data expected in 1Q2021**
- **Study includes ERT-switch and ERT-naïve patients**
- **FDA and EMA agreed upon primary endpoint of 6MWD, an integrated measure of disease progression that evaluates both cardiopulmonary and musculoskeletal systems**

AT-GAA: Key Takeaways

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

- PROPEL study last patient, last visit complete with data expected 1Q2021
- Breakthrough Therapy Designation and Promising Innovative Medicine designation highlight unmet need in Pompe disease
- Rolling BLA submission initiated with the U.S. FDA and on track for submission in 1H2021; EU MAA filing expected 2H2021
- Expanded Access Program for infantile-onset Pompe patients underway
- Process performance qualification (PPQ) runs with our partners at WuXi have been successfully completed for the drug substance and drug product
- Peak revenue potential of $1B-$2B, with exclusivity well into 2030s



Amicus Gene Therapy Pipeline

“We have a duty to obsolete our own technologies”

- Amicus Belief Statement

A World Leading Gene Therapy Company

1
Gene Therapy Center of Excellence

Discovering, developing and applying Amicus protein engineering experience to next-generation gene therapies

3
Industry Leading Collaborations

With key experts in the field at University of Pennsylvania, Nationwide Children's Hospital and Sanford Research

9
Active Clinical and Preclinical Programs

Across devastating neurologic and lysosomal disorders, including human proof of concept data in CLN6 Batten

50+
Rare Disease Indications

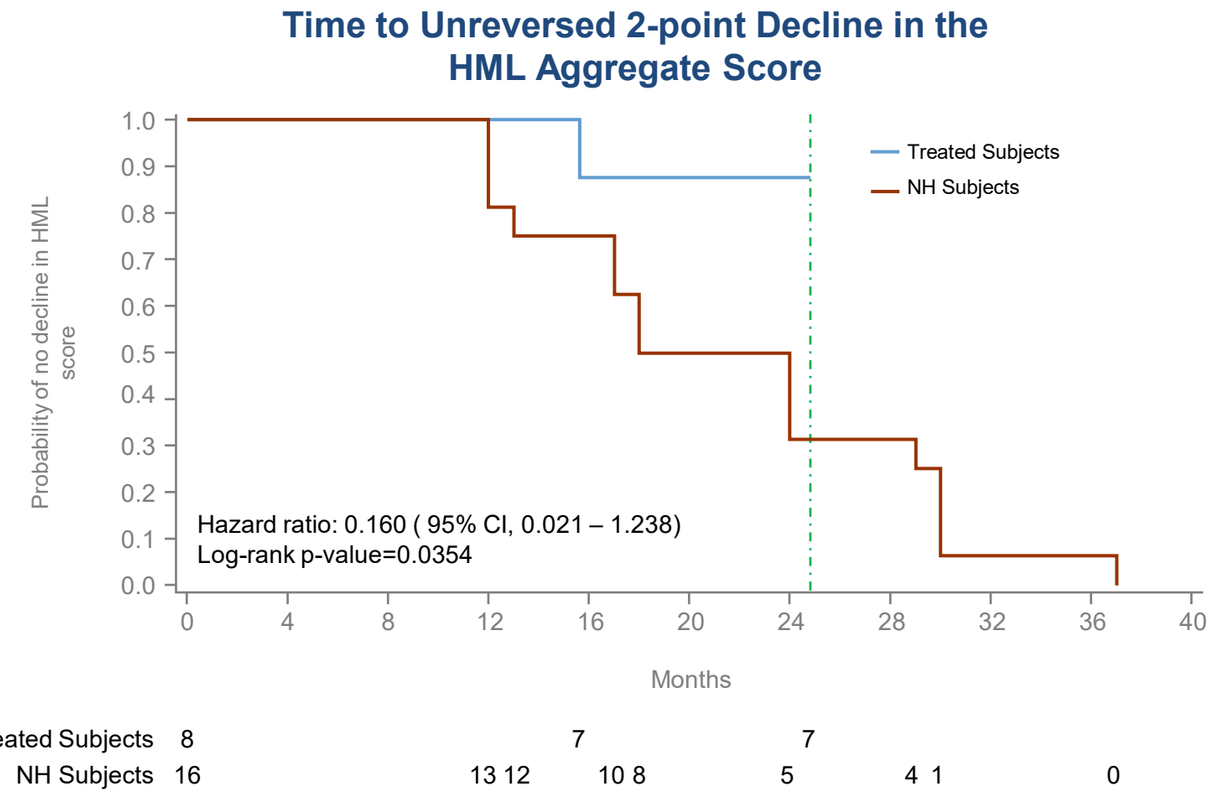
Rights to most lysosomal disorders and 12 larger rare diseases

70+
Dedicated Scientists

Driving innovative science

CLN6 Batten Disease Gene Therapy

Encouraging interim data show a meaningful effect in slowing disease progression at 24 months in devastating early childhood disease that is 100% fatal



M+L, motor and language. NH, natural history.
[a]The efficacy analysis included all patients with 24-month HML data (8 of 13 treated patients in the study). [b]24-month HML data are available for 16 of 17 patients in the natural history cohort derived from a retrospective CLN6 natural history study conducted by Emily de los Reyes, MD (ClinicalTrials.gov Identifier: NCT03285425). Data cutoff March 13, 2020.

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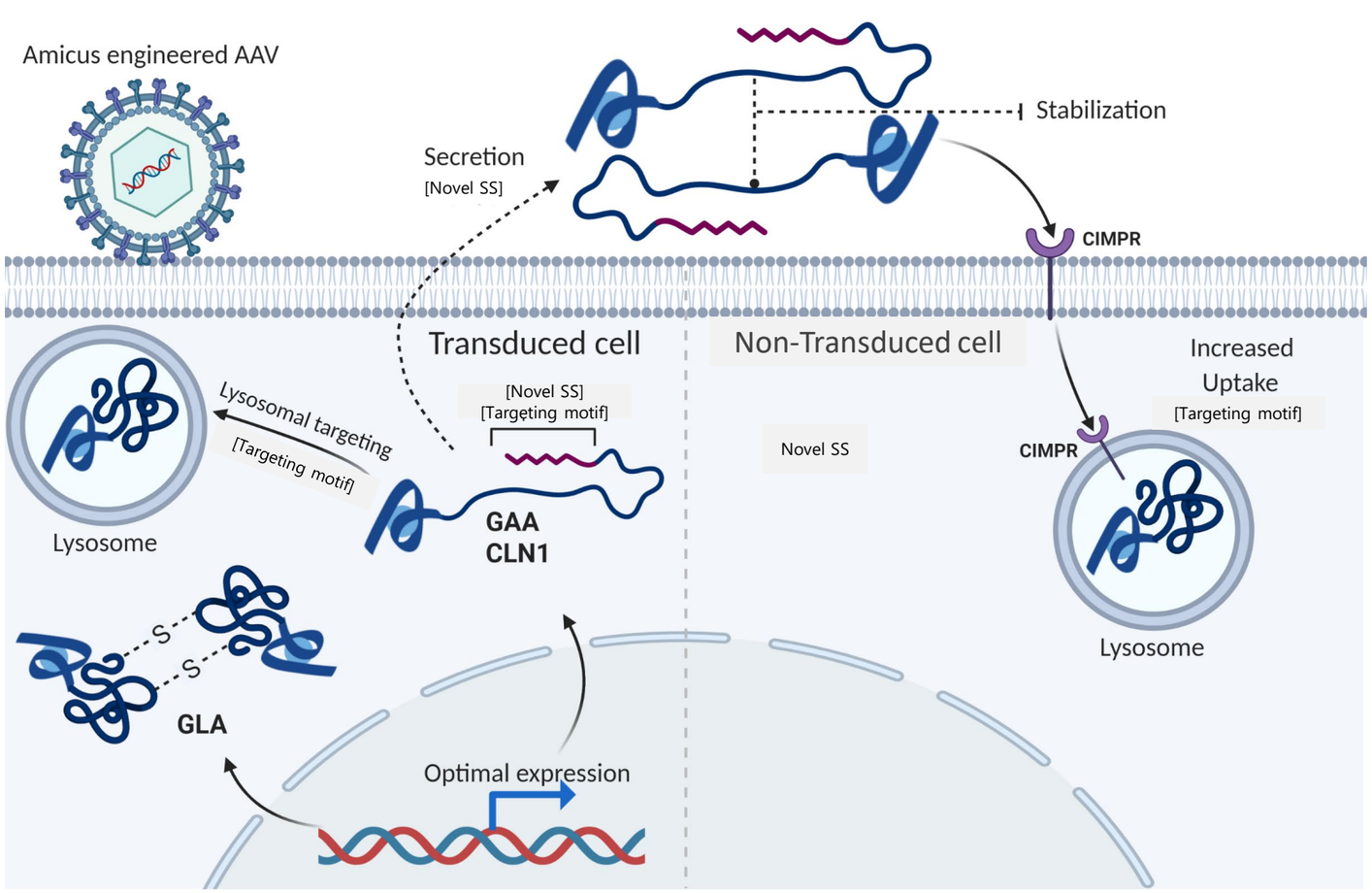
Amicus Protein Engineering Technologies for Lysosomal Targeting

Preclinical POC established for multiple engineered lysosomal targets for optimal expression, secretion, stability and/or cell targeting



- Proof of concept established for Pompe (GAA), Fabry (GLA), CLN1 (PPT1)
- Enhances targeting of therapeutic proteins to lysosome and uptake/cross-correction of neighboring non-transduced cells

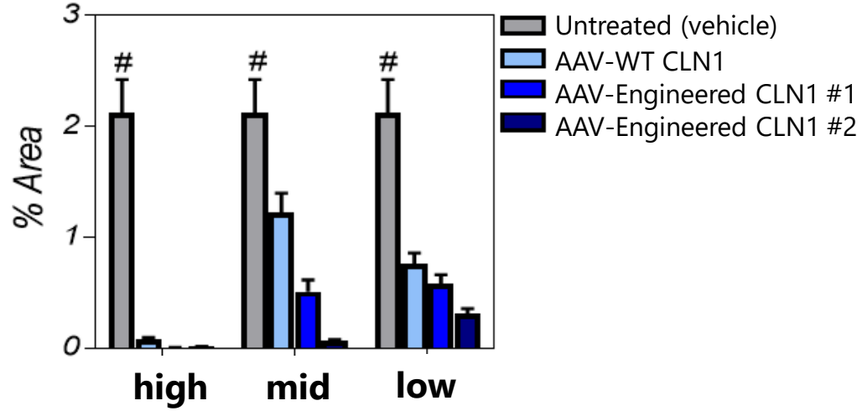
Gene Therapy Pipeline Update: CLN1 Batten Disease

Initial preclinical proof of concept data demonstrate that Amicus-engineered constructs prevent accumulation of substrate material at lower doses versus wild type

CLN1 Batten Disease Overview

- Infantile-onset form of Batten with high unmet need
- Disease onset between 1-3 years of age with rapid loss of motor function, language and vision with mortality before age 10
- Estimated prevalence of 1,000-2,000 children in addressable commercial markets
- Strong strategic fit with Amicus Batten franchise

CLN1 KO Mouse: Thalamus Accumulated Substrate Material (ASM)



- Provides preclinical proof of concept for improved potency with Amicus-engineered transgene
- Additional proof of concept studies planned for 2021 to support IND candidate selection

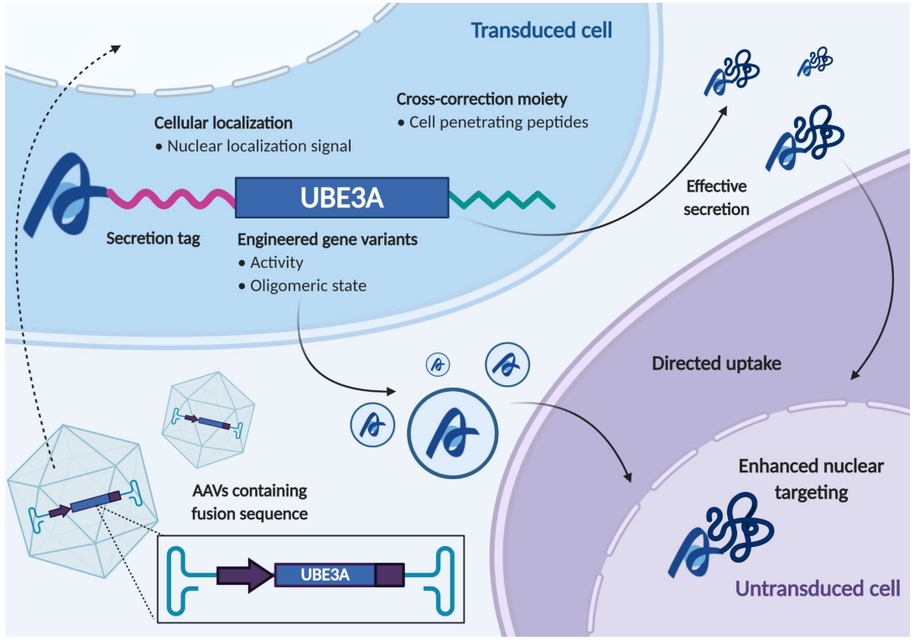
Gene Therapy Pipeline Update: Angelman Syndrome

Amicus plans to initiate a program to leverage our protein engineering to construct an optimized AAV gene therapy approach in Angelman Syndrome

Angelman Syndrome Overview

- Severe neurodevelopmental disorder resulting in severe cognitive, motor and language impairment and seizures
- Caused by mutations in ubiquitin-protein ligase E3A (UBE3A) in neurons
- Disease onset in childhood with survival into late adulthood
- Estimated 30,000+ patients in addressable commercial markets
- No current disease modifying treatment
- Significant opportunity for a one-time AAV gene therapy to restore UbE3A activity leveraging Amicus protein engineering

Amicus Engineering Approach: Angelman



Gene Therapy Manufacturing

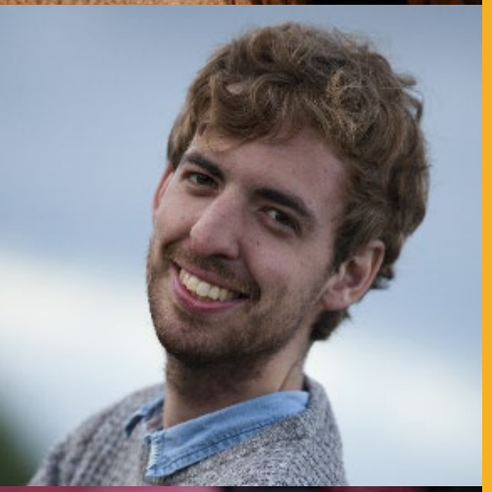
Amicus will build, staff and operate its own Gene Therapy Manufacturing Center of Excellence to become one of the leading, global gene therapy manufacturers

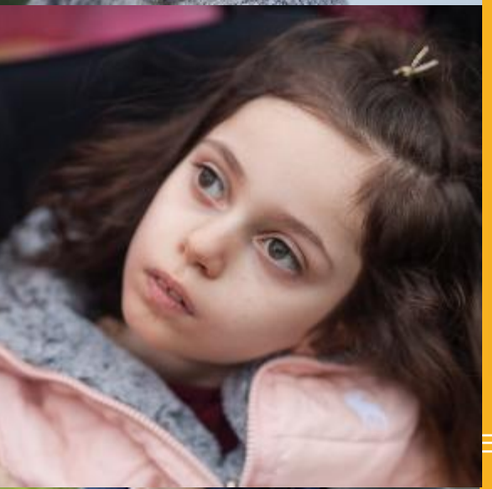
- Manufacturing and process science capabilities and capacity in gene therapy will be crucial for Amicus success
- Work underway towards Amicus first clinical manufacturing and process development facility
- Experience in complex biologics manufacturing and quality control provide critical expertise

Gene Therapy: Updates & Key Takeaways

Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

- CLN6 Phase 1/2 interim data have shown positive impact with potential to become first approved gene therapy for fatal brain disease in children
- Initial data from the CLN3 Batten disease Phase 1/2 study in 1Q21
- Manufacturing on track to initiate next clinical studies in CLN6 and CLN3 using material from planned commercial process
- Preclinical POC in CLN1 demonstrates ability to continue leveraging protein engineering capabilities towards new targets
- Progressing manufacturing and IND-enabling work for Fabry and Pompe gene therapy programs
- Additional data and potential IND candidate disclosed across multiple preclinical programs this year
- Continued foundational gene therapy discovery and research activities across 50+ diseases





Financial Summary

“We are business led and science driven”

- Amicus Belief Statement

Financial Outlook: Key Takeaways

- Galafold revenue in 2020 was ~$261[1] million, exceeding the Company's guidance
- Non-GAAP operating expense guidance for 2021 is expected to remain flat at $410 million to $420 million
  - Driven by disciplined expense management and continued investment in the global Galafold launch, AT-GAA clinical studies and advancing our gene therapy pipeline
- Current cash position is sufficient to achieve self-sustainability without the need for future dilutive financing

[1]Preliminary and unaudited

Key Takeaways

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

Galafold Continues Strong Launch Performance and 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

Cash Position Sufficient to Achieve Self-Sustainability without the Need for Future Dilutive Financing

Thank You

Amicus Therapeutics®