Full Year 2020 Financial Results Conference Call & Webcast

March 1, 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, and revenue goals, including as they are impacted by COVID-19 related disruption, are based on current information. The potential impact on operations and/or revenue 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 or to treatment sites. 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, UK, 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, commercialization 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 corporate financial guidance and financial goals and the attainment of such goals and 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, 2020 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.

<u>Non-GAAP Financial Measures</u>

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.

Rare Disease Day® 2021
Remembering Rossella


CRONACA

MARTEDÌ 30 APRILE 2002

Napoli, la bambina ha due anni ed è in terapia intensiva per una malattia rarissima. La speranza dagli Usa

"Senza farmaci Rossella morirà"

Genitori in sciopero della fame finché non arriverà la cura

DATECI IL FARMACO PER ROSSELLA

DANIELA D'ANTONIO


NAPOLI

LA BUONA SANITÀ

Cresce la fiducia intorno alla bimba affetta da una rara malattia genetica e curata col medicinale giunto dagli Usa

La cura funziona, Rossella sta meglio

Ma i medici invitano alla prudenza: è ancora presto


LA SANITÀ IN CAMPANIA

GIOVEDÌ 12 SETTEMBRE 2002 NAPOLI CRONACA

Il medicinale dagli Stati Uniti, grazie all'interessamento del ministro Sirchia che domani assisterà alla somministrazione

Una visita del ministro Sirchia a Rossella, la bimba ricoverata al Monaldi. Sotto, il medico Carlo Vosa con il farmaco

La madre: "So che potrebbe non essere la soluzione, ma abbiamo il dovere di sperare"

Il medico: "Per noi è non una paziente, ma una figlia che abbiamo visto crescere..."

Rossella: dall'America con amore

A Napoli il farmaco per la bimba del Monaldi. "Aveva diritto a una chance"

IL CASO

Mucca pazza è polemica sui controlli


Rossella, Biagio e Tilde sono i "Crowley" italiani

Rossella Passero ha compiuto 10 anni. A soli 9 mesi, però, pareva che per lei non ci fosse speranza alcuna di varcare l'anno di vita. Ma la fibra è forte. I suoi genitori, Biagio e Tilde, sono persone tenaci. Hanno superato assieme i lunghi ricoveri, e persino uno sciopero della fame e della sete (foto sopra), quando l'unico farmaco che avrebbe potuto salvare la figlia (l'enzima mancante prodotto artificialmente) non veniva distribuito in Italia, perché ancora in fase sperimentale. Oggi, ogni 15 giorni la bimba si reca in ospedale, dove riceve 22 flebo di Myozyme, il medicinale salva-vita: la terapia è assai costosa, circa 800 euro a fiala, ed è riservata a un centinaio di pazienti in Italia (a carico del Sistema sanitario).

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

$483.3M Cash as of 12/31/20

Two Clinical-Stage Gene Therapies

2021 Key Strategic Priorities

1. **Achieve double-digit Galafold growth and revenue $300M to $315M**
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

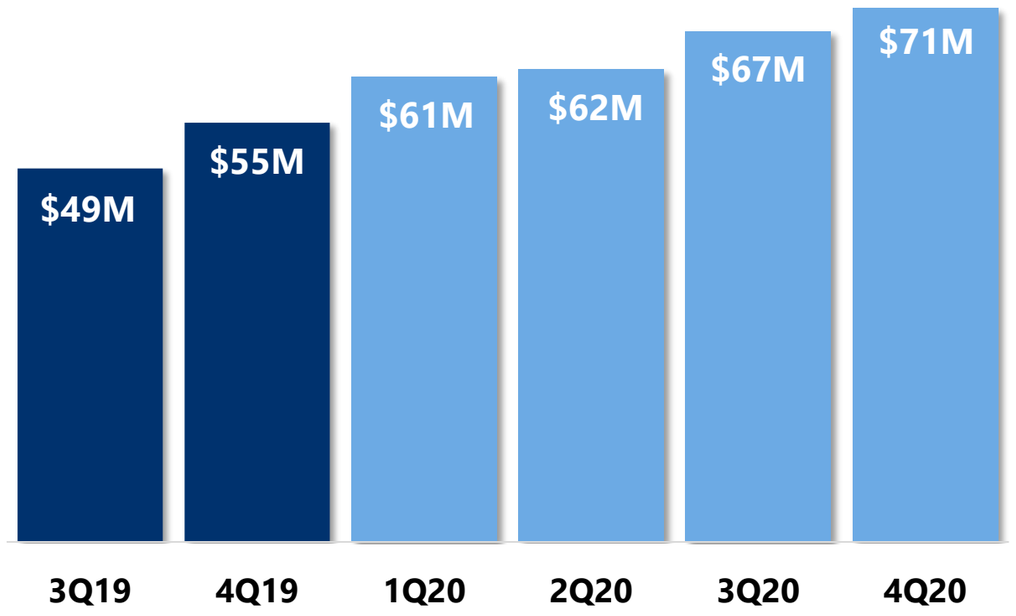
- **$260.9M** FY20 Galafold Revenue
- **$300M-$315M** FY21 Global Galafold Revenue
- **348** Amenable Variants in U.S. Label
- **40+** Countries with Regulatory Approvals: including U.S., EU, Japan, and other Countries
- **Continued Expansion in 2021**
- **1,384** Amenable Mutations Included in the EU 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.

Amicus Therapeutics

2020 Galafold Success

Growth remains strong with Q4 revenue of $70.9M and FY2020 revenue of $260.9M



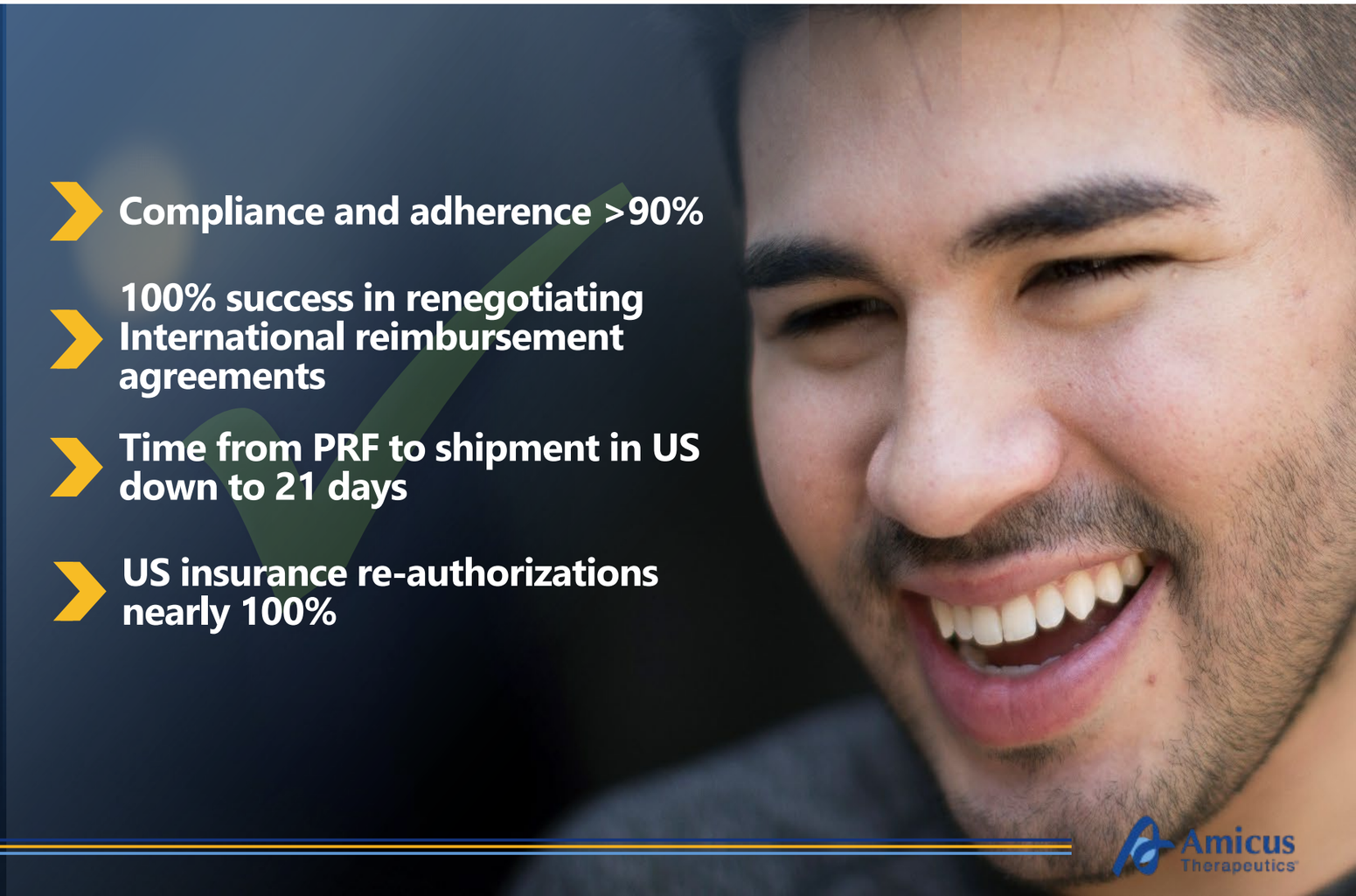
Galafold Global Commercial Momentum

Strong global demand supported by high compliance and adherence rates laying foundation for continued growth anticipated in 2021

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

- Strong patient demand and several new launch countries anticipated in 2021
- Global mix of switch (60%) and previously untreated patients (40%)
- Continued growing prescriber base in the U.S. to nearly 200 physicians
- Continue to support diagnostic initiatives to drive a shorter pathway to diagnosis

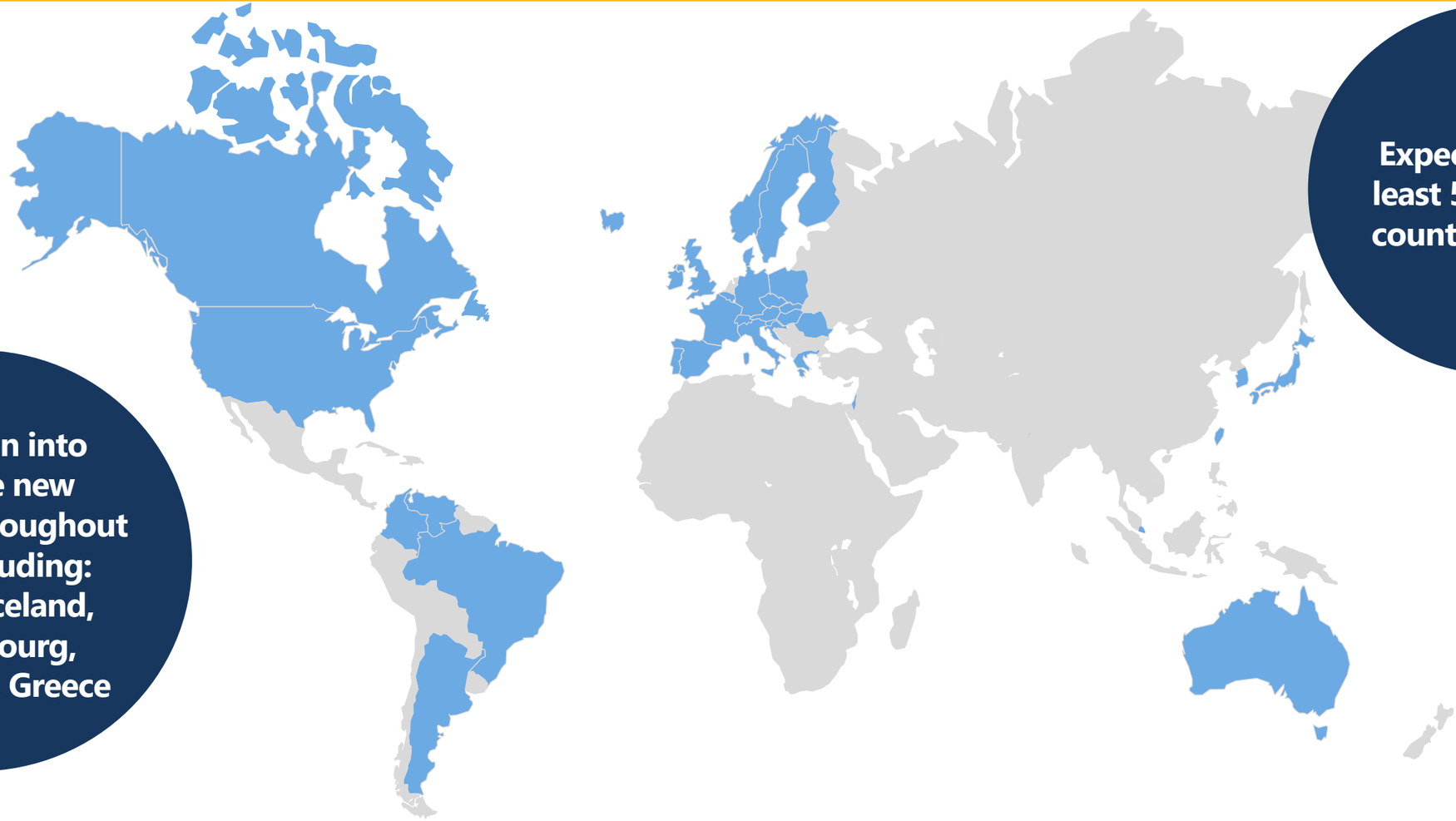
- **Compliance and adherence >90%**
- **100% success in renegotiating International reimbursement agreements**
- **Time from PRF to shipment in US down to 21 days**
- **US insurance re-authorizations nearly 100%**



Amicus Therapeutics

Galafold Geographic Growth

Regulatory approvals in 40+ countries and commercial sales in 30+ of those today



Expect to add at least 5 additional countries in 2021

Expansion into multiple new markets throughout 2020 including: Poland, Iceland, Luxembourg, Argentina, Greece

Outlook for 2021

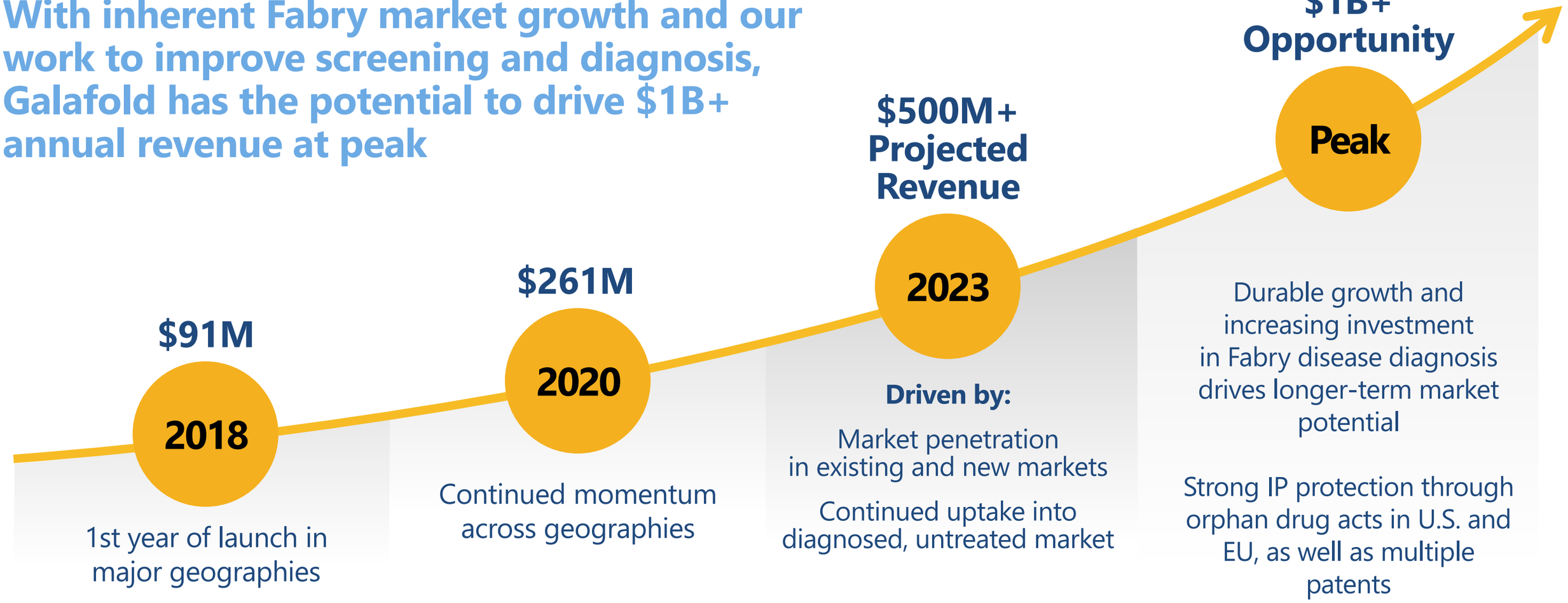
Continued double-digit Galafold revenue growth to $300M-$315M in 2021

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 net new patient starts expected to be greater than in 2020
- Expect higher patient adds and revenue growth in the second half of 2021 as COVID impact eases
- Continue to see >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



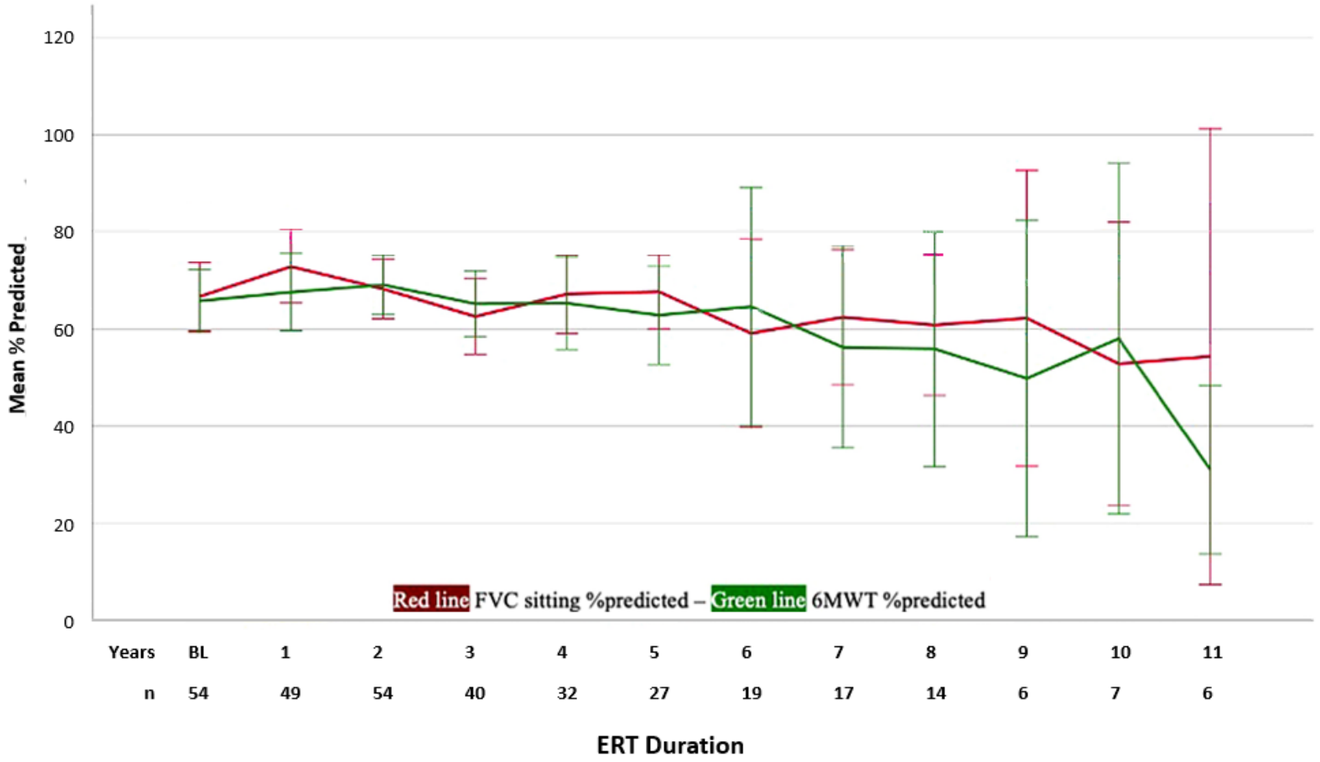
AT-GAA: Next Potential Standard of Care for Pompe Disease

"We encourage and embrace constant innovation"

- Amicus Belief Statement

Unmet Need in Pompe Disease Today

Natural history studies and publications in Pompe disease continue to highlight the unmet need and the continued decline on key measures of disease



There is an initial positive effect on the most important outcome measures, however, a more limited long-term benefit of stabilization of the clinical course under ERT for many patients over a long period. Though, according to our data, this long-term therapeutic efficacy is weakest for the lung capacity and consecutively the need of additional ventilatory support over time. As respiratory insufficiency is the most frequent cause of death in Pompe disease, it is important to further improve this organ function in particular. The fast approval of novel therapeutic options is a great unmet need for Pompe patients."

Modified from Goodschmidt et. al., 2021, *STIG study: real-world data of long-term outcomes of adults with Pompe disease under enzyme replacement therapy with alglucosidase alfa*

Amicus Therapeutics

PROPEL Topline Results:
Overall Population (n=122)

PROPEL

6MWD showed greater improvement with AT-GAA versus alglucosidase alfa but did not demonstrate statistical superiority; FVC demonstrated clinically significant improvement with AT-GAA over alglucosidase alfa

6MWD (m)

Treatment	Baseline	CFBL at Week 52	Difference	P-Value
AT-GAA (n=85)	357.9 (111.8)	+20.8 (4.6)	+13.6 (8.3)	p=0.072
Alglucosidase alfa (n=37)	351.0 (121.3)	+7.2 (6.6)		

FVC (% predicted)

Treatment	Baseline	CFBL at Week 52	Difference	P-Value
AT-GAA (n=85)	70.7 (19.6)	-0.9 (0.7)	+3.0 (1.2)	**p=0.023**
Alglucosidase alfa (n=37)	69.7 (21.5)	-4.0 (0.8)		

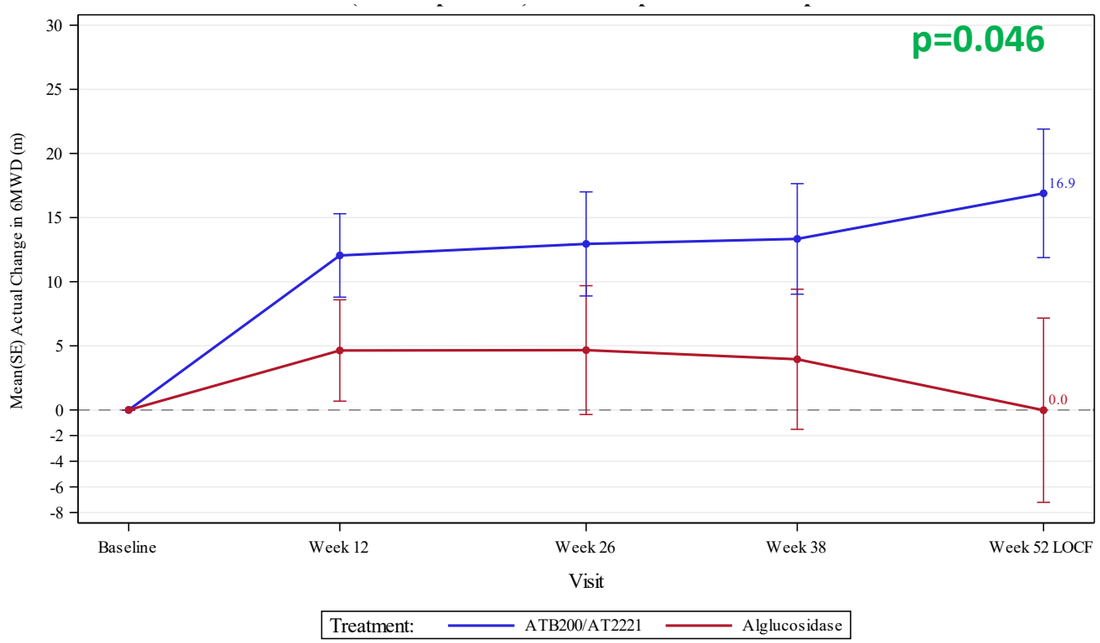
NOTES: Baseline is Mean (STDEV); CFBL is Mean LOCF (SE); P-values are nominal 2-sided; FVC data normally distributed and p–values are from ANCOVA.
Results exclude one clinically implausible patient who used an investigational anabolic steroid ostarine (selective androgen receptor modulator) just prior to study start.
6MWD data not normally distributed and 6MWD p-value is for non-parametric ANCOVA; 6MWD parametric MMRM p-value was p=0.097.

PROPEL Topline Results
ERT Experienced Population (n=95)

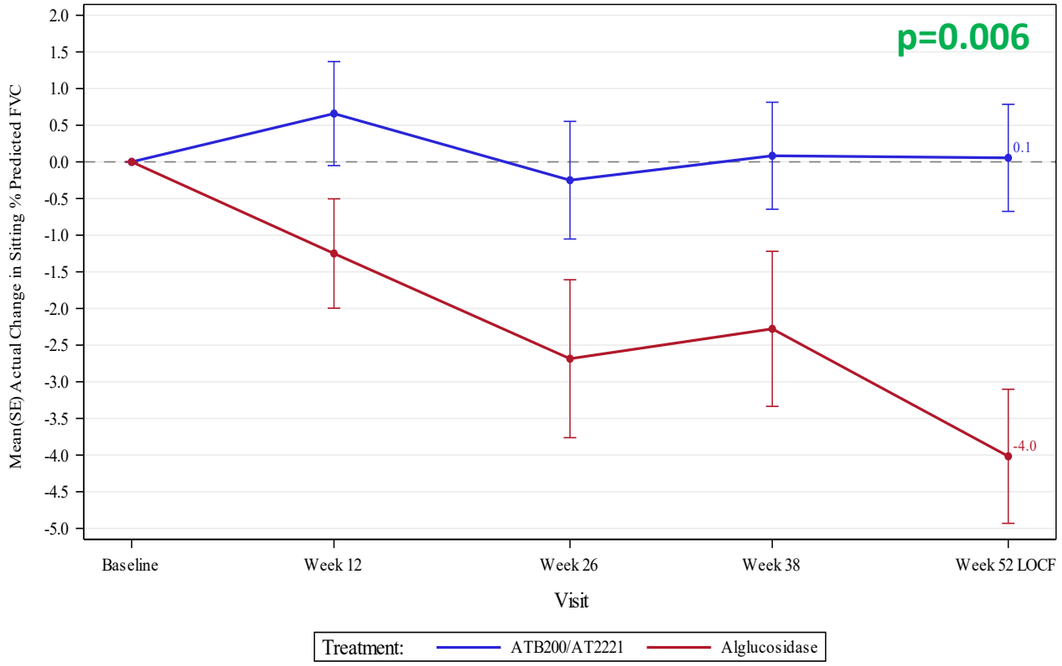
PROPEL

ERT experienced patients treated with AT-GAA demonstrated improvements over time in 6MWD and stabilization over time in FVC versus alglucosidase alfa

6MWD (m): Change from baseline (n=65, n=30)



FVC (% predicted): Change from baseline (n=65, n=30)



NOTE: Baseline is Mean (STDEV); CFBL is Mean (SE); P-values are nominal 2-sided; FVC data normally distributed and p–values are from ANCOVA
6MWD data not normally distributed and 6MWD p-value is for non-parametric ANCOVA; 6MWD parametric MMRM p-value was p=0.078

Primary, Key Secondary and Biomarker Endpoint Heat Map
All Patients & ERT Experienced Patients

Endpoints across motor function, pulmonary function, muscle strength, PROs and biomarkers favored AT-GAA over alglucosidase alfa in both the overall and ERT experienced populations

Overall Population

	Alglucosidase alfa	AT-GAA
Motor Function		6MWD
		GSGC*
Pulmonary Function		FVC*
Muscle Strength		Lower MMT
PROs		PROMIS-Physical
		PROMIS-Fatigue
Biomarker		Hex4*
		CK*

ERT Experienced Population

	Alglucosidase alfa	AT-GAA
Motor Function		6MWD*
		GSGC*
Pulmonary Function		FVC*
Muscle Strength		Lower MMT
PROs		PROMIS-Physical
		PROMIS-Fatigue
Biomarker		Hex4*
		CK*

Note: * Nominal P-value <0.05; based on LOCF means

Post Hoc Non-Inferiority Analyses

Post hoc non-inferiority analyses of 6MWD and FVC are highly statistically significant

Parameter	Non-Inferiority Margin	1-sided p-value
6MWD	15 meters	0.0004
	10 meters	0.0026
	5 meters	0.0127
	3 meters	0.0224
% predicted FVC	1.1%	0.0008

NOTE1: 6MWD results are based on MMRM analysis using observed cases and FV results are based on ANCOVA using LOCF.
NOTE2: As Lumizyme did not show statistical significance versus placebo for 6MWD, the NI margins were not statistically justified.
Results exclude one clinically implausible patient who used an investigational anabolic steroid ostarine (selective androgen receptor modulator) just prior to study start.

AT-GAA: Key Takeaways

AT-GAA for Pompe Advances Toward Approval

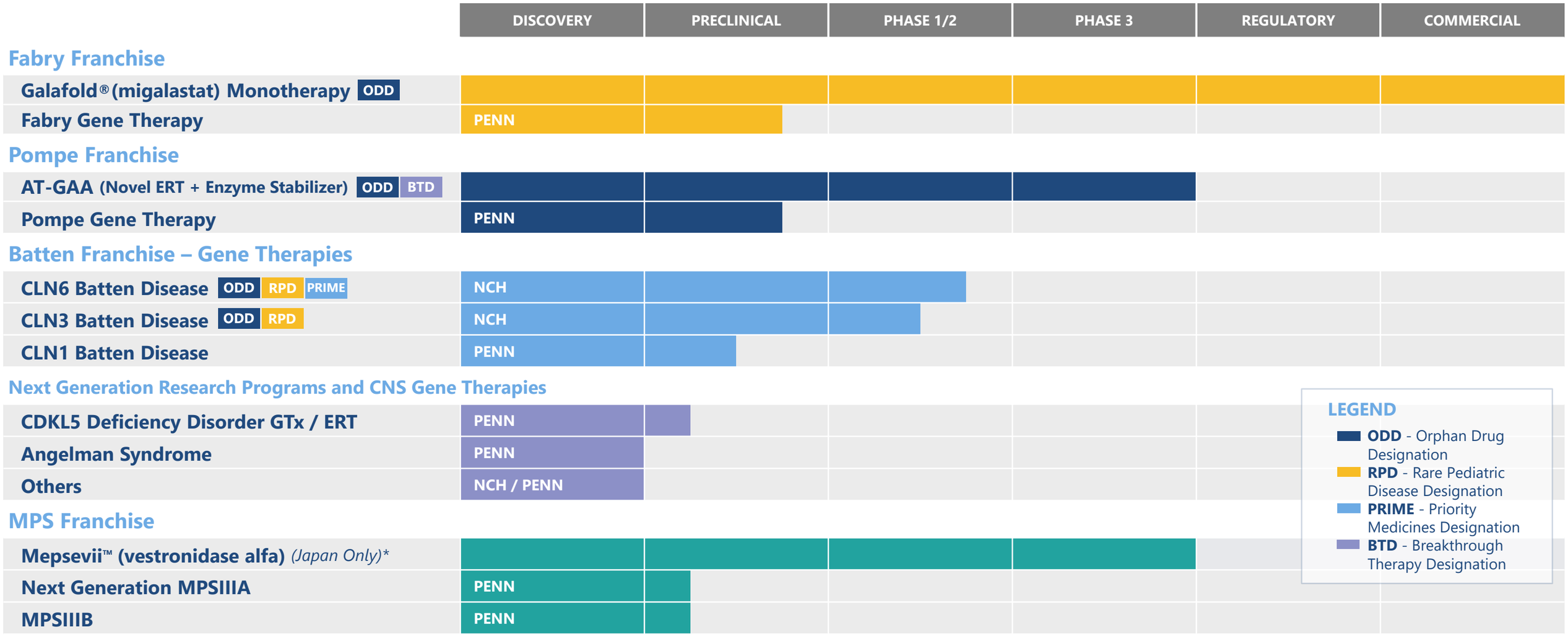
- Rolling BLA submission expected to complete in Q2
- Other key regulatory submissions for approval throughout 2021 including MAA in Europe
- Potential for early approval under EAMS framework with Priority Innovative Medicines Designation in UK
- 150+ patients worldwide now being treated with AT-GAA including adults, adolescents and infants
- Pediatric study for Pompe patients aged 12 to <18 with late-onset Pompe disease ongoing
- Clinical study for Pompe patients with infantile onset disease expected to begin this year
- Expanded access program for Pompe infantile patients and adult-onset patients open and has enrolled multiple patients with Pompe. Further expanded access for all Pompe patients being considered

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

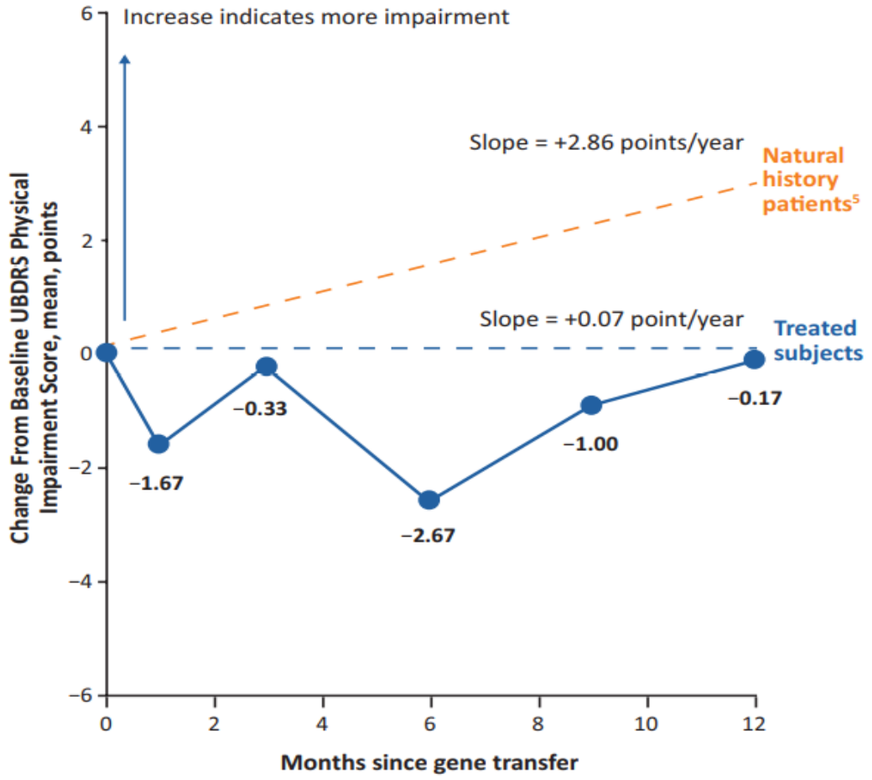
Amicus Therapeutics

CLN3 Batten Disease Gene Therapy
AT-GTX-502

Early clinical data suggest early signs of disease stabilization compared to natural history; plan to submit IND for next clinical study in 2H2021

- Batten disease is a group of disorders known as neuronal ceroid lipofuscinoses (NCLs)
- Mutation in one of 13 different CLN genes leads to neuronal lysosomal dysfunction
- CLN3 Batten disease is one of the most common neurodegenerative disorders affecting children leading to blindness, motor impairment, learning difficulties, epilepsy and, ultimately, premature death
- UBDRS-physical is the key efficacy endpoint for this study in CLN3 Batten assessing gross and fine motor function, vision and speech

Low-Dose Cohort (n=3) Compared with Patients in the Natural History Study (n=82)



UBDRS=Unified Batten Disease Rating Scale. [a] Scheduled month 12 in-person UBDRS assessment was impacted by the COVID-19 pandemic.

Amicus Therapeutics

Financial Summary

“We are business led and science driven”

- Amicus Belief Statement

2020 Select Financial Results

2020 revenue of $260.9M from global Galafold sales

(in thousands, *except per share data)*	**Dec. 31, 2020**	**Dec. 31, 2019**
Product Revenue	$260,886	$182,237
Cost of Goods Sold	31,044	21,963
R&D Expense	308,443	286,378
SG&A Expense	156,407	169,861
Changes in Fair Value of Contingent Consideration	3,144	3,297
Depreciation and Amortization	8,846	4,775
Loss from Operations	(246,998)	(304,037)
Loss on Extinguishment of Debt	(7,276)	-
Income Tax (Expense) Benefit	(2,598)	(478)
Net Loss	(276,852)	(356,388)
Net Loss Per Share	(1.07)	(1.48)

Financial Outlook: Key Takeaways

- Galafold revenue in 2020 was $260.9 million, exceeding the Company's guidance
- 2020 Non-GAAP Operating Expenses of $415.7M in line with guidance of $410 million to $420 million
- 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

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

Reconciliation

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

	December 31		
	2020	**2019**	**2018**
Total operating expenses - as reported GAAP	**$ 476,840**	**$ 464,311**	**$ 405,618**
Research and development:			
Share-based compensation	20,817	17,575	11,740
Asset acquisition related expenses for in-process R&D	-	-	100,000
Selling, general and administrative:			
Share-based compensation	28,334	26,855	17,520
Changes in fair value of contingent consideration payable	3,144	3,297	3,300
Depreciation and amortization	8,846	4,775	4,216
Total operating expense adjustments to reported GAAP	61,141	52,502	136,776
Total operating expenses - as adjusted	**$ 415,699**	**$ 411,809**	**$ 268,842**