Amicus Therapeutics Analyst Day 2019

October 10, 2019

Forward-Looking Statements

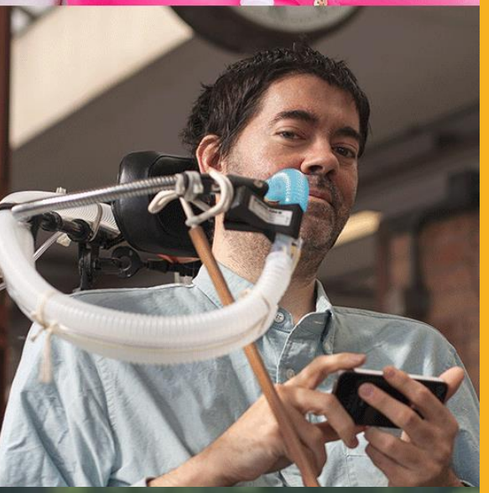
This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, business development plans and the projected revenues, sales, expenses 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 or projections 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 presentation 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. With respect to statements regarding projections of the Company's revenue, sales, expenses and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans and strategies. 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 presentation 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.

Agenda

8:30 a.m. – 8:35 a.m.	WELCOME & INTRODUCTIONS	**Sara Pellegrino,** *Vice President, Investor Relations*
8:35 a.m. – 8:45 a.m.	VISION, MISSION AND STRATEGY	**John F. Crowley,** *Chairman and Chief Executive Officer*
8:45 a.m. – 9:00 a.m.	FINANCIAL AND OPERATIONAL STRATEGY	**Daphne Quimi,** *Chief Financial Officer* **Bradley Campbell,** *President and Chief Operating Officer*
9:00 a.m. – 9:30 a.m.	GALAFOLD: ROADMAP TO $1B IN SALES AND PATIENT PERSPECTIVES	**Bradley Campbell,** *President and Chief Operating Officer* **Simon Jordan,** *SVP, Head of International* **Mike Keavany,** *SVP, Head of US* **Jayne Gershkowitz,** *Chief Patient Advocate* with Alex Dencker and Sabina Kineen
9:30 a.m. – 9:50 a.m.	AT-GAA – POTENTIAL TO SHIFT THE TREATMENT PARADIGM IN POMPE DISEASE	**Jay Barth, M.D.,** *Chief Medical Officer* **John F. Crowley,** *Chairman and Chief Executive Officer*
9:50 a.m. - 10:00 a.m.	Q&A SESSION	
10:00 a.m. - 10:10 a.m.	BREAK	
10:10 a.m. – 10:50 a.m.	NEXT GENERATION GENE THERAPY PLATFORM & RESEARCH PROGRAM	**Hung Do, Ph.D.,** *Chief Science Officer* **Jeff Castelli, Ph.D.,** *Chief Portfolio Officer and Head of Gene Therapy* **Jim Wilson, M.D., Ph.D.,** *Rose H Weiss Professor and Director, Orphan Disease Center, Perelman School of Medicine at the University of Pennsylvania*
10:50 a.m. – 11:00 a.m.	Q&A SESSION	
11:00 a.m. – 11:30 a.m.	BATTEN DISEASE GENE THERAPY PORTFOLIO AND PATIENT PERSPECTIVES	**Jill Weimer, Ph.D.,** *SVP of Discovery Research & Gene Therapy Science* **Jayne Gershkowitz,** *Chief Patient Advocate* with the Kahn Family **Jeff Castelli, Ph.D.,** *Chief Portfolio Officer and Head of Gene Therapy*
11:30 a.m. – 11:50 a.m.	CLOSING REMARKS	**John F. Crowley,** *Chairman and Chief Executive Officer*
11:50 a.m. – 12:00 p.m.	Q&A SESSION	



Vision, Mission & Strategy

John F. Crowley
Chairman and Chief Executive Officer

2019 Analyst Day | October 10, 2019 | New York, NY

A RARE COMPANY

A leading fully-integrated, global rare disease biotechnology company

Galafold™ (migalastat)

First Oral Precision Medicine for Fabry Disease

Gene Therapy PLATFORM

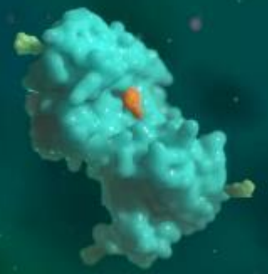
Protein Engineering & Glycobiology

World Class BIOLOGICS Capabilities

EMPLOYEES in 27 Countries

AT-GAA

Phase 3 in Pompe Disease



~$575M Cash as of 6/30/19

Two Clinical-Stage Gene Therapies

GLOBAL COMMERCIAL ORGANIZATION

Robust R&D Engine

Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases

A RARE OPPORTUNITY

A broad and patient focused portfolio to drive value creation

Galafold

$1B+ Opportunity

Pompe ERT

$1B-2B+ Opportunity

Gene Therapy Portfolio

$1B+ Opportunity

Transform the Lives of Thousands of Patients

A RARE PORTFOLIO

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3	REGULATORY	COMMERCIAL
Fabry Franchise						
Galafold® (migalastat) Monotherapy						
Fabry Gene Therapy	PENN					
Pompe Franchise						
AT-GAA (Novel ERT + Chaperone)						
Pompe Gene Therapy	PENN					
Batten Franchise – Gene Therapies						
CLN6 Batten Disease	NCH					
CLN3 Batten Disease	NCH					
CLN8 Batten Disease	NCH					
CLN1 Batten Disease	NCH					
Next Generation Research Programs and CNS Gene Therapies						
CDKL5 Deficiency Disorder GTx / ERT	PENN					
Niemann-Pick Type C (NPC)	NCH / PENN					
Tay-Sachs Disease	NCH					
Other	NCH / PENN					
MPS Franchise						
Mepsevii™ (vestronidase alfa) *(Japan Only)**						
Next Generation MPSIIIA	PENN					
MPSIIIB	PENN					

Amicus has assembled one of the **largest portfolios of gene therapy programs for rare diseases** in the entire industry

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

Amicus Therapeutics

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

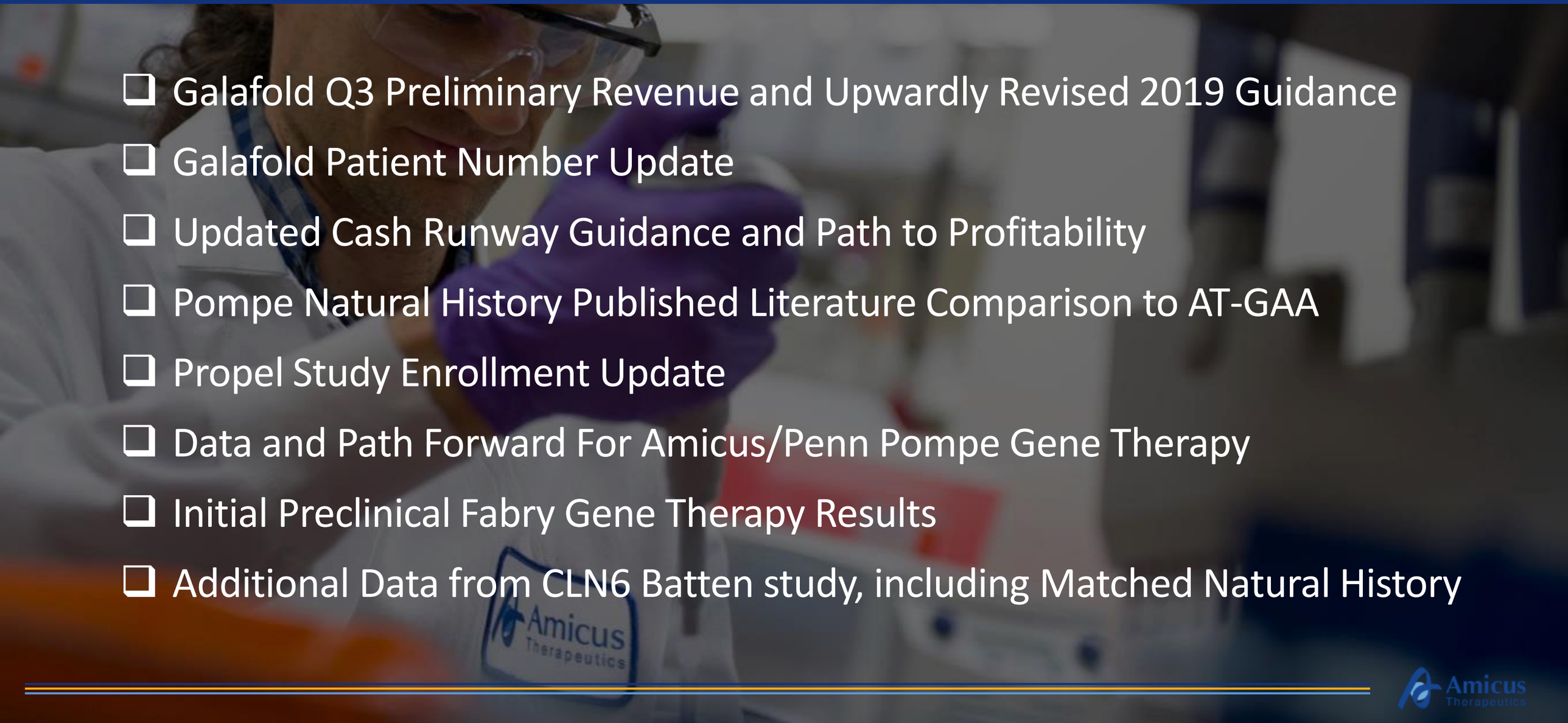






Amicus Therapeutics

What's New at Amicus Analyst Day 2019



- ❑ Galafold Q3 Preliminary Revenue and Upwardly Revised 2019 Guidance
- ❑ Galafold Patient Number Update
- ❑ Updated Cash Runway Guidance and Path to Profitability
- ❑ Pompe Natural History Published Literature Comparison to AT-GAA
- ❑ Propel Study Enrollment Update
- ❑ Data and Path Forward For Amicus/Penn Pompe Gene Therapy
- ❑ Initial Preclinical Fabry Gene Therapy Results
- ❑ Additional Data from CLN6 Batten study, including Matched Natural History

Key Takeaways from Amicus Analyst Day 2019

Today's Analyst Day highlights our recent 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 to 1H2022

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

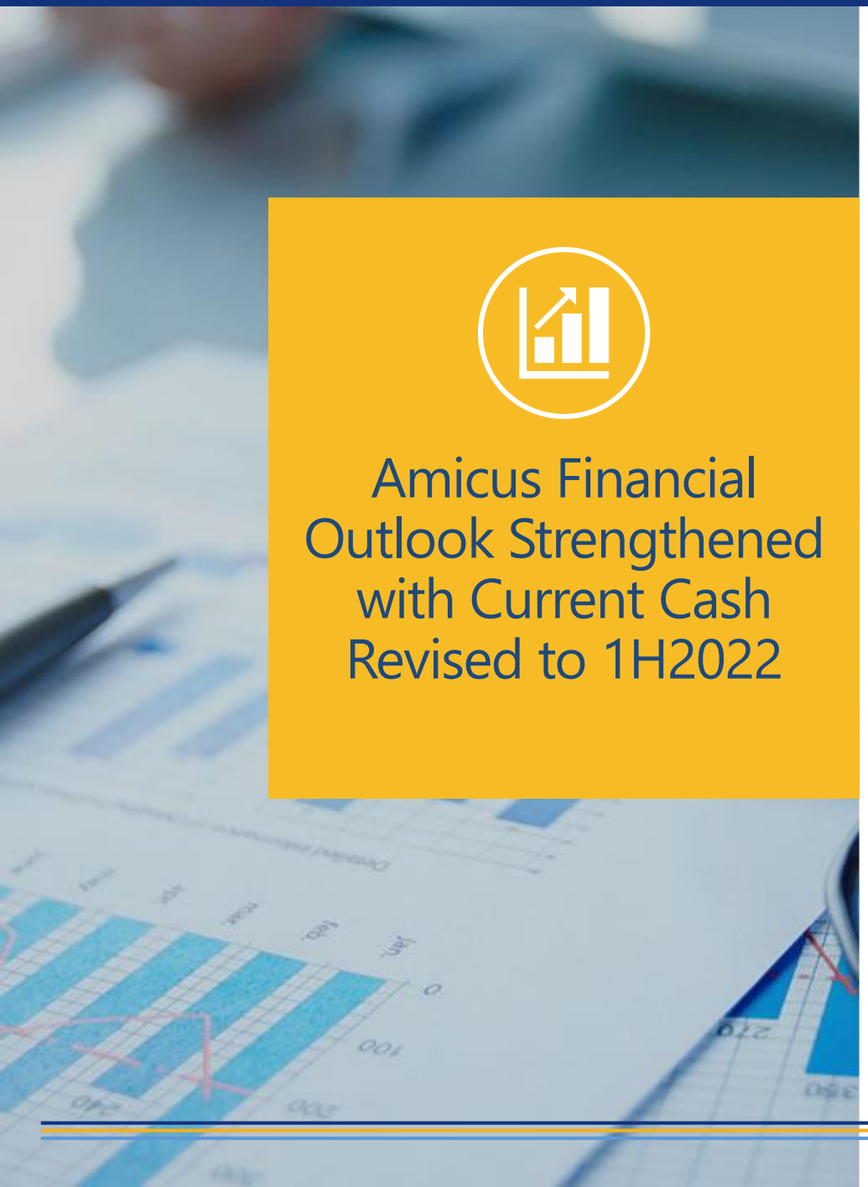
Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

Galafold: Key Takeaways from Amicus Analyst Day 2019

Galafold Continues Strong Launch Performance & Cornerstone of Amicus Success

- Preliminary unaudited 3Q19 Galafold revenue of $48.0M+ exceeds expectations
- Achieved 1,000+ net global Galafold patients by end of Q3
- FY19 Galafold guidance upwardly revised to $170M-$180M range
- On clear path to $500M+ in 2023 revenue and $1B+ peak

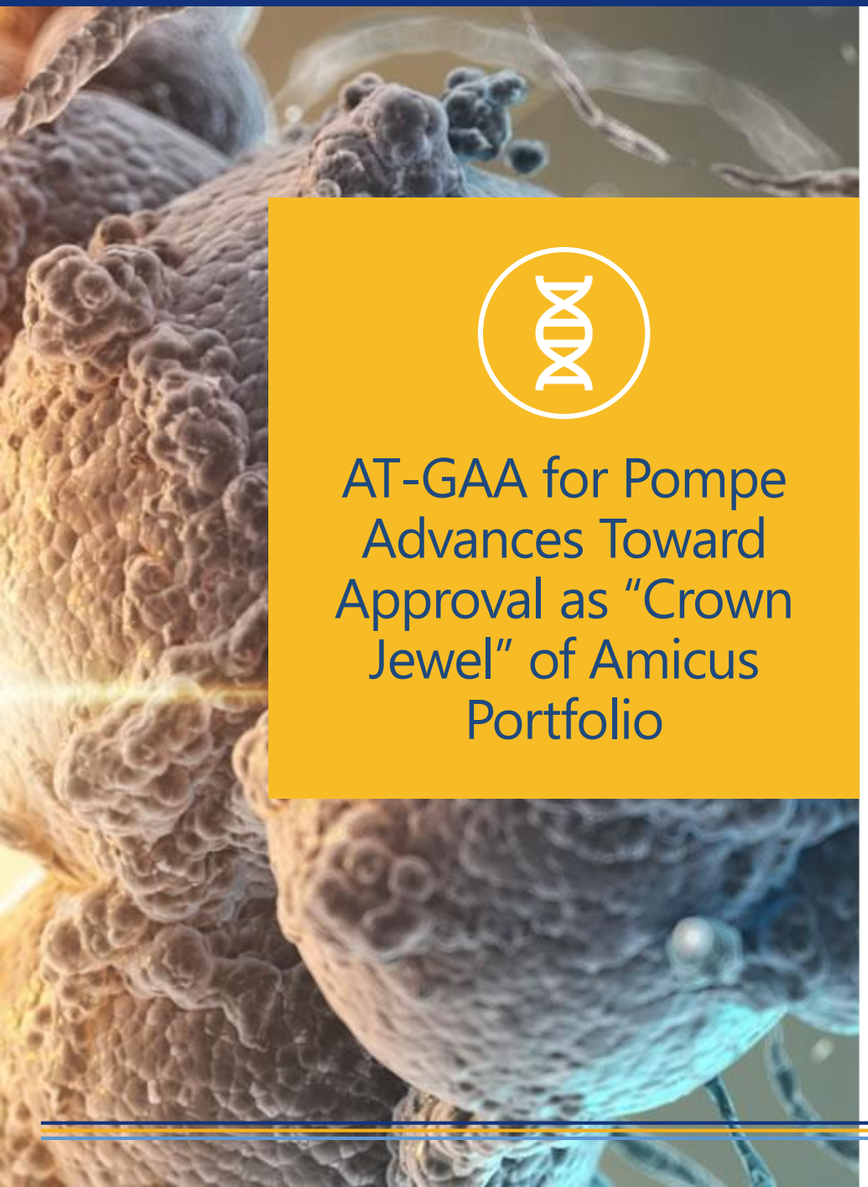
Financial Outlook: Key Takeaways from Amicus Analyst Day 2019



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

AT-GAA: Key Takeaways from Amicus Analyst Day 2019



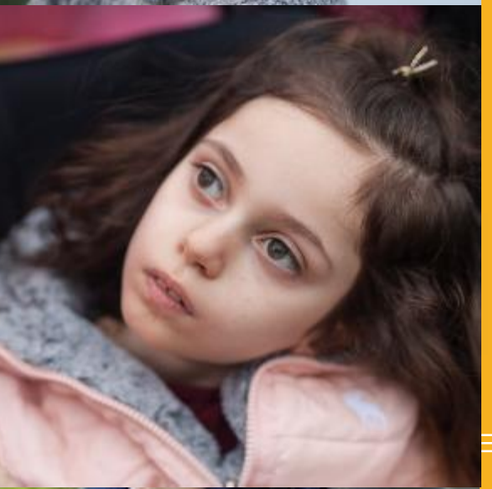
AT-GAA for Pompe Advances Toward Approval as "Crown Jewel" of Amicus Portfolio

- PROPEL pivotal study 80%+ enrolled and expected now to over-enroll (~120 Patients) by YE 2019
- Pediatric study underway
- Manufacturing PPQ runs at WuXi biologics on track to start this month
- New 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: Key Takeaways from Amicus Analyst Day 2019

Portfolio of Gene Therapy Programs and Technologies Provides Foundation for Future

- CLN6 data shows profound impact as compared to natural history now matched for age and baseline. Potential to become first ever approved gene therapy for fatal brain disease in children
- CLN3 additional patients to be dosed and AAV intrathecal platform increasingly gives confidence in CLN3 program (largest cause of childhood neurodegeneration, 5,000+ children)
- Penn Collaboration is R&D engine, with rights to 50+ diseases
- 8 preclinical gene therapies in development and one clinical candidate now generated (Pompe)



Financial & Operational Strategy

Daphne Quimi, ***Chief Financial Officer***
Bradley Campbell, ***President and Chief Operating Officer***

2019 Analyst Day | October 10, 2019 | New York, NY

A RARE COMPANY

Our strategy continues to be "Go it Alone" because we believe it is the best way to deliver our medicines to patients and maximize long term shareholder value

Galafold™ (migalastat)

First Oral Precision Medicine for Fabry Disease

Gene Therapy PLATFORM

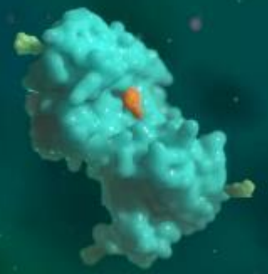
Protein Engineering & Glycobiology

World Class BIOLOGICS Capabilities

EMPLOYEES in 27 Countries

AT-GAA

Phase 3 in Pompe Disease



GLOBAL COMMERCIAL ORGANIZATION

Robust R&D Engine

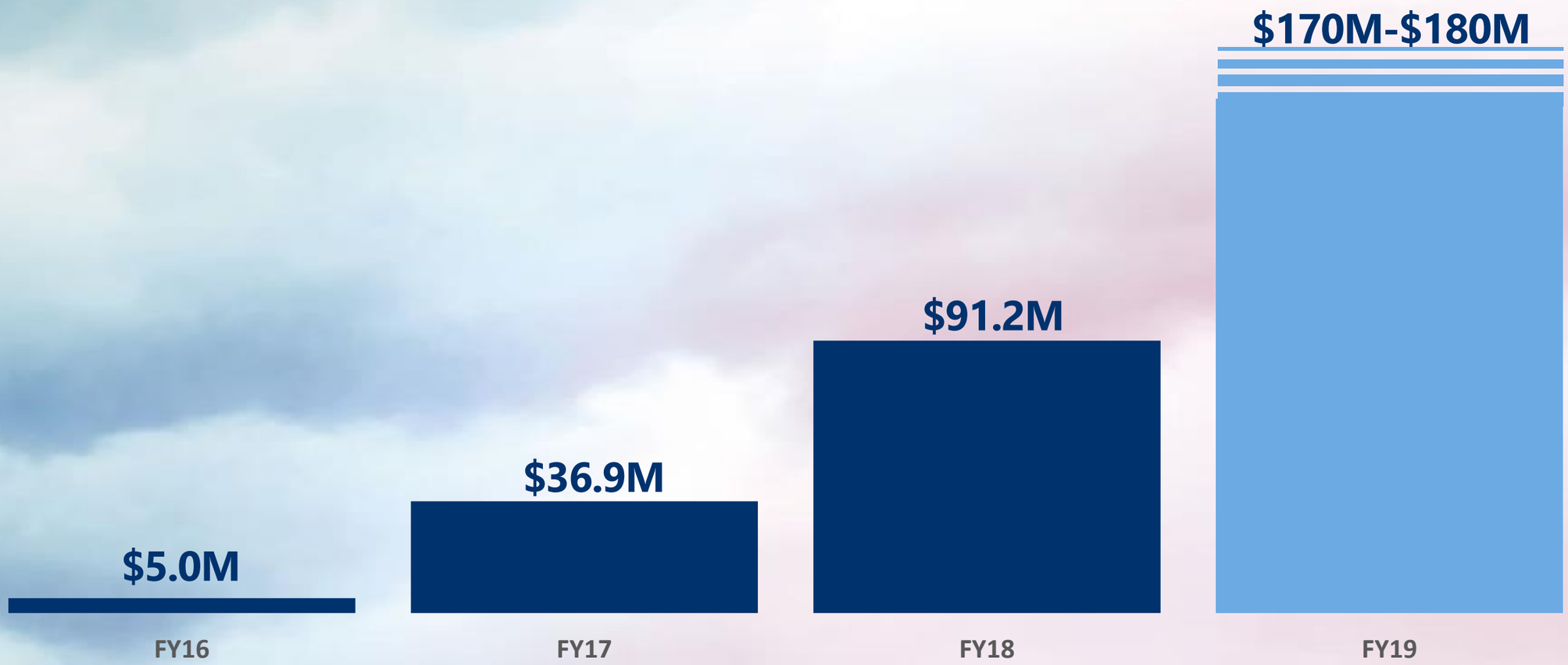
Nearly 50+ Lysosomal Disorders and More Prevalent Rare Diseases

~$575M Cash as of 6/30/19

Two Clinical-Stage Gene Therapies

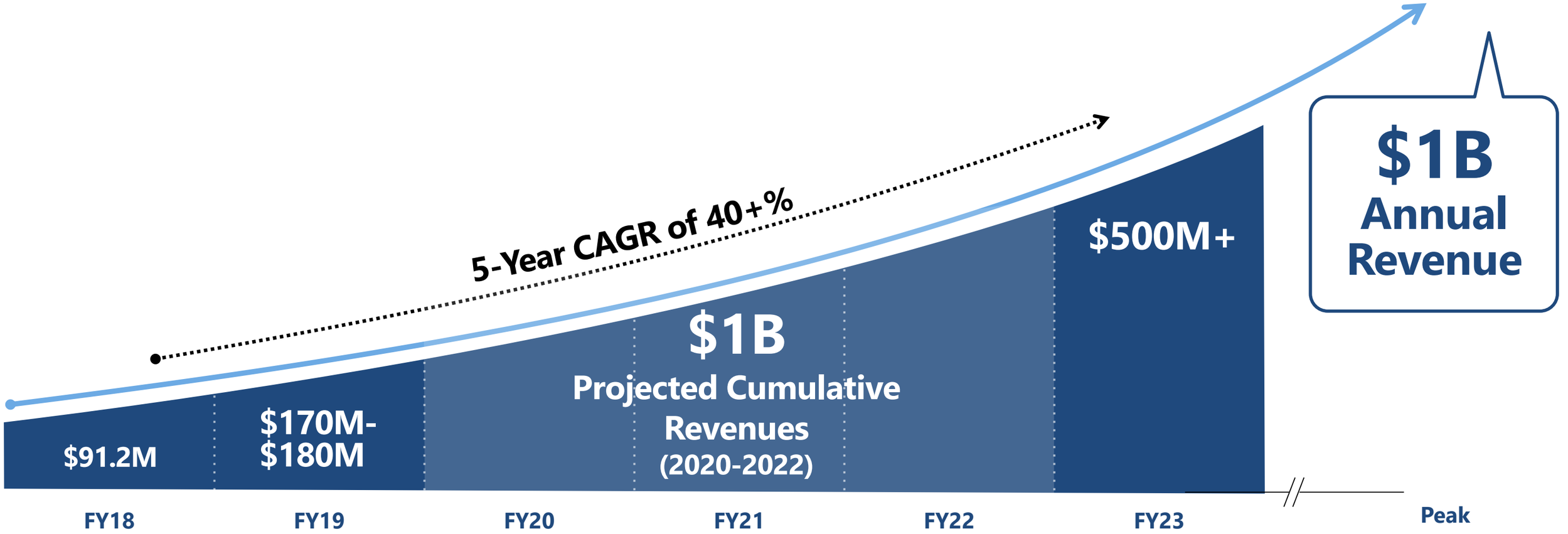
Galafold Success and FY19 Galafold Revenue Guidance

Strong Q3 performance of ~$48M+ (preliminary/unaudited) gives confidence in upwardly revised guidance of $170-$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



Building the Foundation: 2016-2018

Amicus has invested substantially over the past several years to build world class global commercial capabilities and to develop/advance AT-GAA for Pompe

SG&A: Global Commercialization

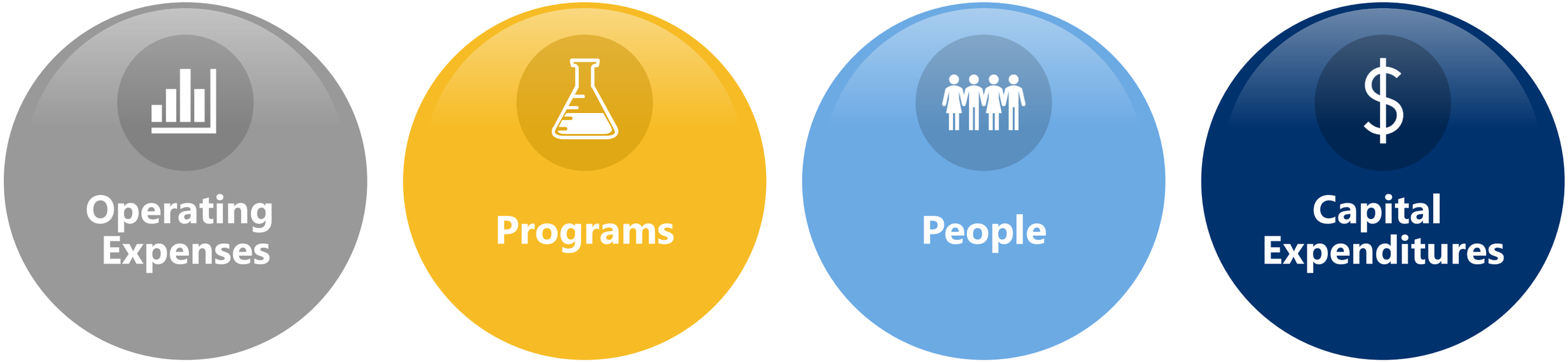
- ☑ Established 200+ person team
- ☑ Strong access and reimbursement expertise
- ☑ Presence in 27 countries, 5 continents
- ☑ Cover most major global metabolic centers
- ☑ Experienced team to support entire Amicus portfolio

R&D: Develop/Advance AT-GAA

- ☑ Conducted multicenter global Phase 1/2 studies
- ☑ Largest pivotal study ever in lysosomal disorders (PROPEL)
- ☑ Advance manufacturing to commercial scale and quality
- ☑ Begin early commercial inventory build

Extension of Cash Runway to Well into 1H2022

Completion of strategic business review and strategy has driven efficiencies and cost savings while advancing all key programs forward



Non-GAAP Operating Expense Guidance

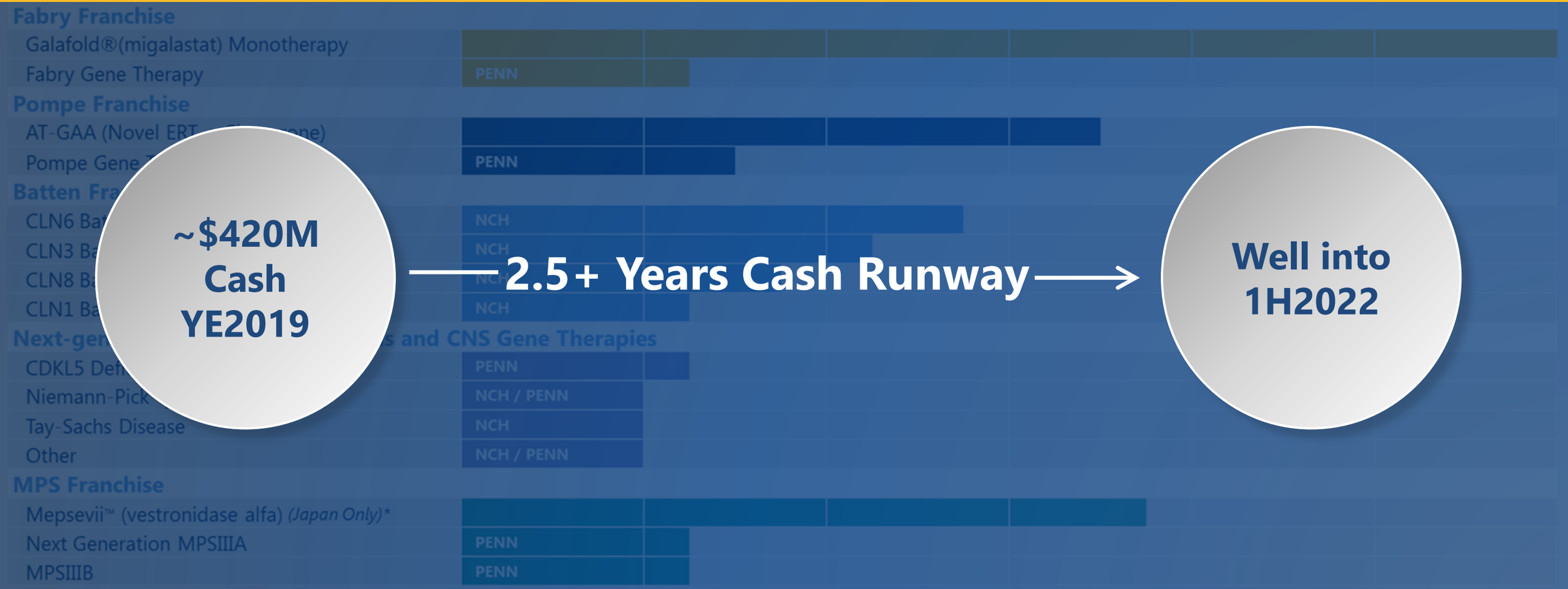
With these major investments in SG&A and R&D poised to yield results, 2019 is peak year for non-GAAP operating expense on path to profitability

- FY19 anticipated non-GAAP operating expense of $410M-$420M
- Strong operating support organization in place to maximize value of future program advancements and products
- Expected non-GAAP operating expense to remain relatively flat in 2020-2022
- Minimal further investment in global commercial infrastructure and team needed to launch AT-GAA

Non-GAAP operating expense excludes share-based compensation expense, changes in fair value of contingent consideration, and depreciation

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

Fully funded through major milestones in portfolio and continued global growth



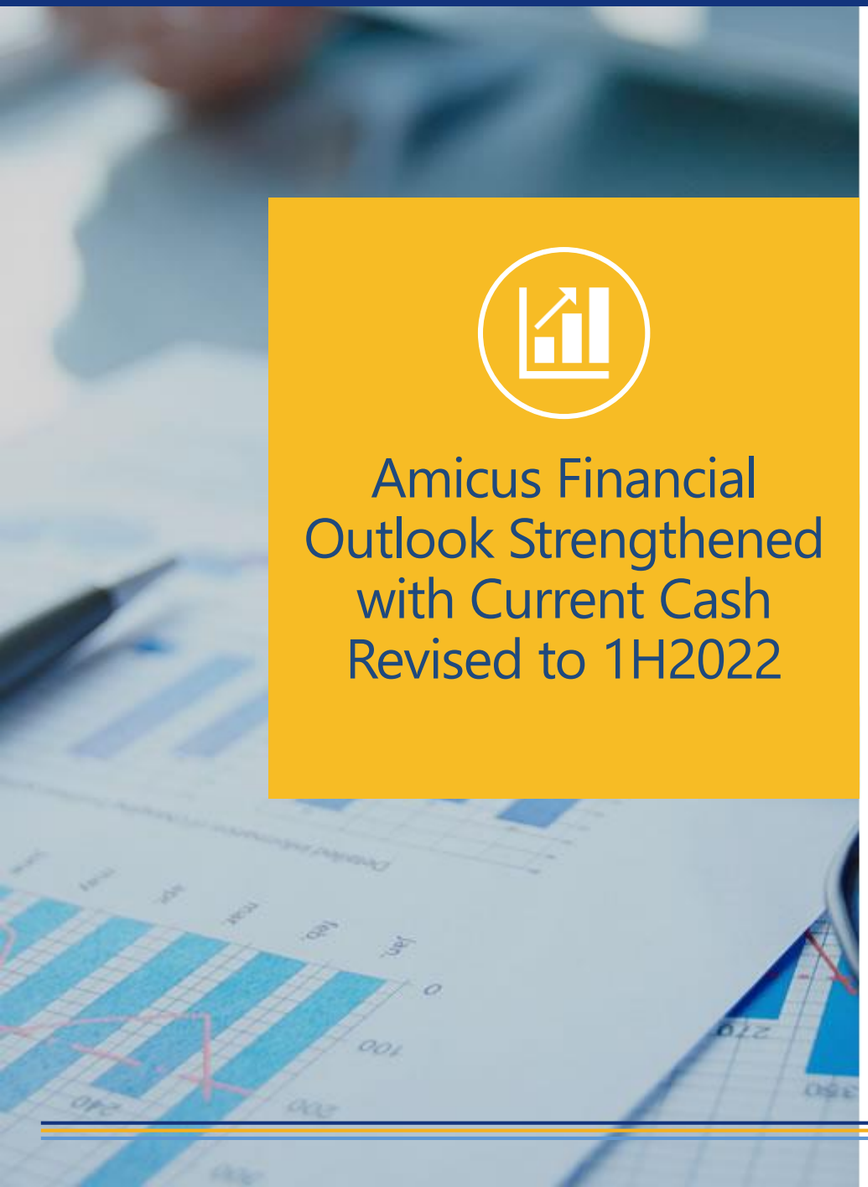
At Major Inflection Point: Path to Profitability

Clear strategy to build our business, advance our portfolio, and achieve profitability with the following key priorities

- Grow Galafold
- AT-GAA to pivotal data, global approvals and launch
- CLN6, CLN3 and Pompe gene therapies into and through the clinic
- 1-2 gene therapy INDs every year starting in 2021
- Discover and develop next generation protein engineering and gene therapy technologies with Penn

Only modest additional capital required in outer years to extend runway into profitability with multiple non-equity sources available as/when needed

Financial Outlook: Key Takeaways from Amicus Analyst Day 2019



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Galafold® (migalastat) Global Launch…

…taking a leadership role in the treatment of Fabry disease

Bradley Campbell, ***President and Chief Operating Officer***

Simon Jordan, ***Senior Vice President and Head of International***

Mike Keavany, ***Senior Vice President and Head of US***

"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

- **$48.0M+** — 3Q19 Galafold Revenue (preliminary unaudited)
- **$170-180M** — FY19 Global Galafold Rev. Guidance
- **27** — Countries with Pricing & Reimbursement
- **348** — Amenable Variants in U.S. Label
- **9** — Regulatory Approvals* : Argentina, Australia, Canada, EU, Israel, Japan, S. Korea, Switzerland , U.S.
- **Geographic Expansion in 2019**

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.

Global Commercial Team

World class global commercial leadership team to drive Galafold's success

- **11** countries with offices, including US, EU5, Japan
- **20** countries with direct presence (Amicus personnel)
- **27** markets with reimbursement

North America

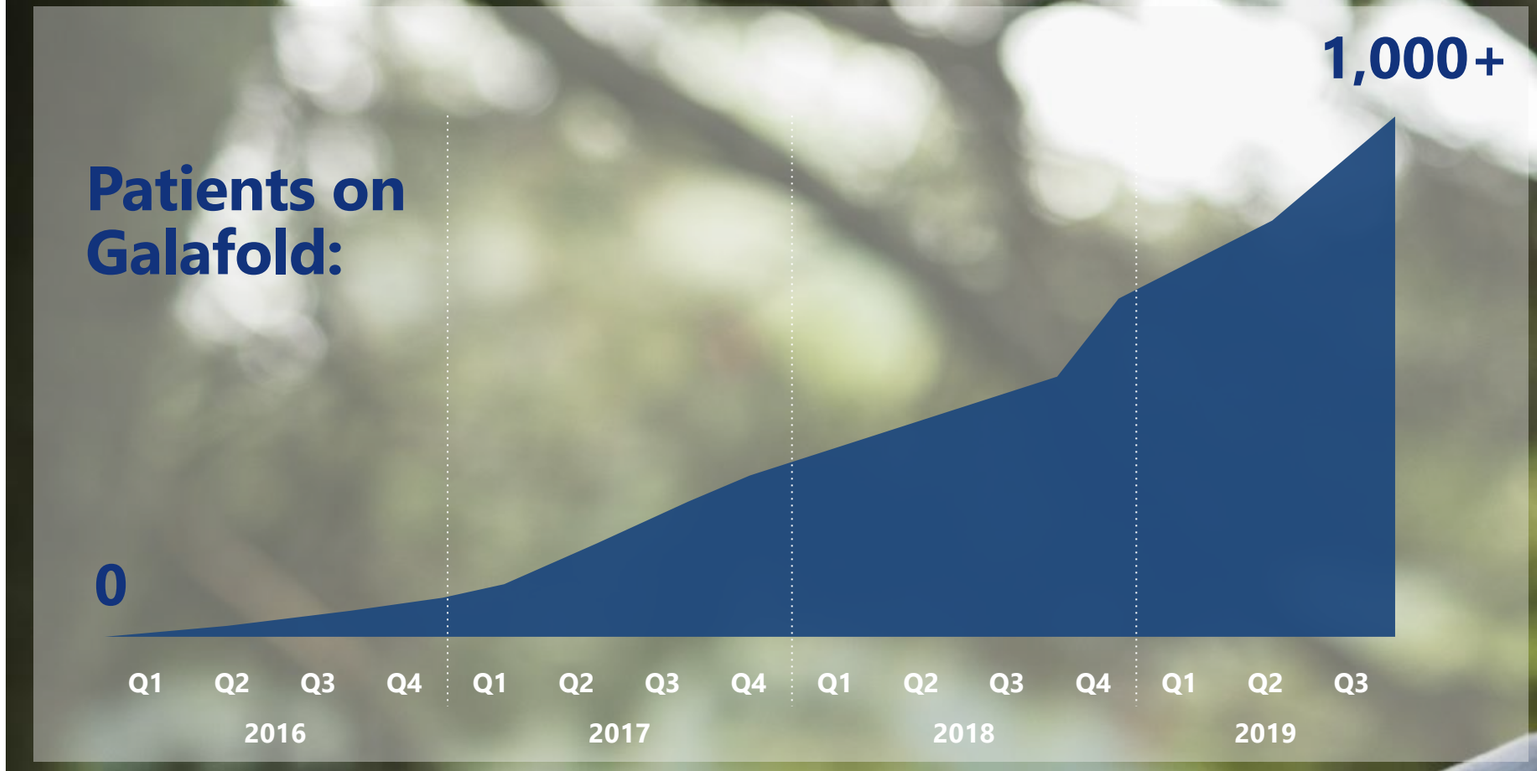
EU

ROW

APAC

Galafold Performance

We have now reached our 2019 goal of 1,000+ net patients on therapy.



Galafold Success and FY19 Galafold Revenue Guidance

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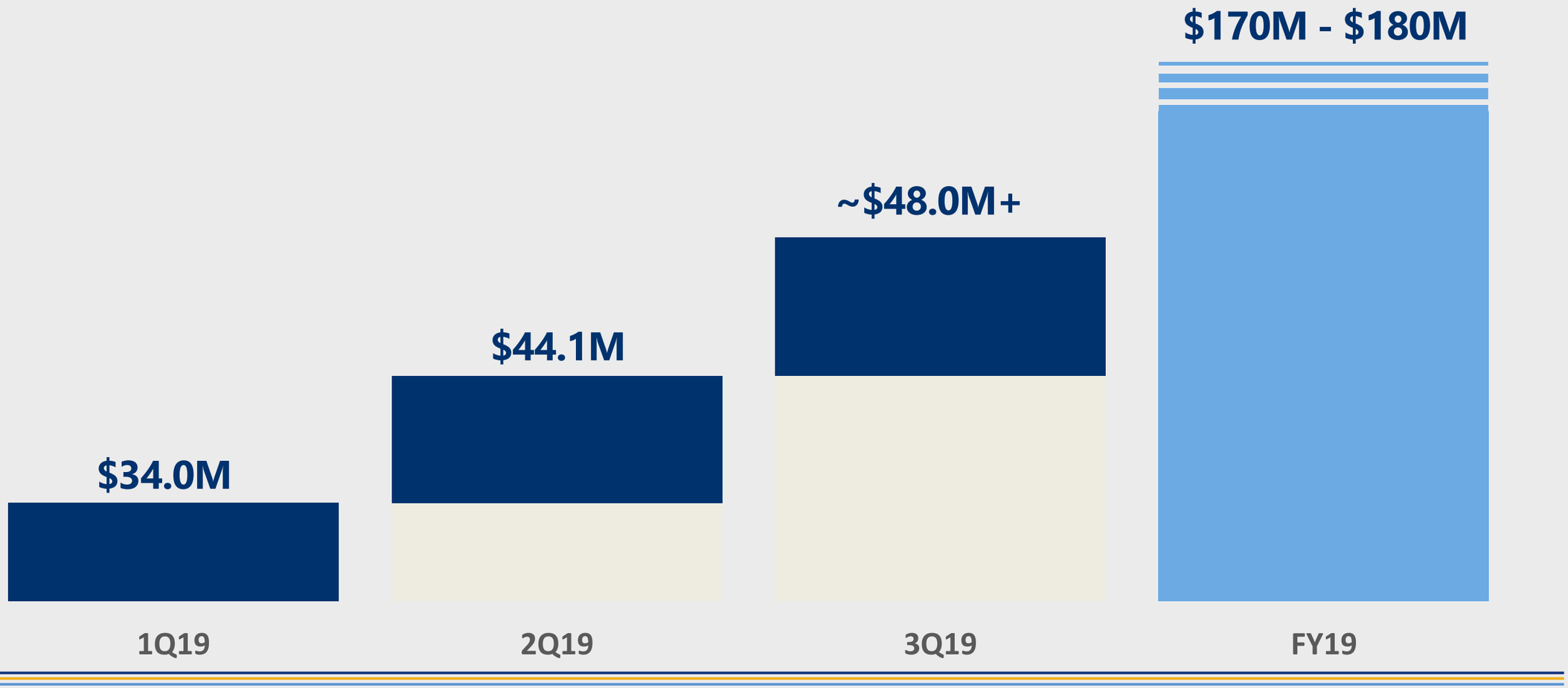
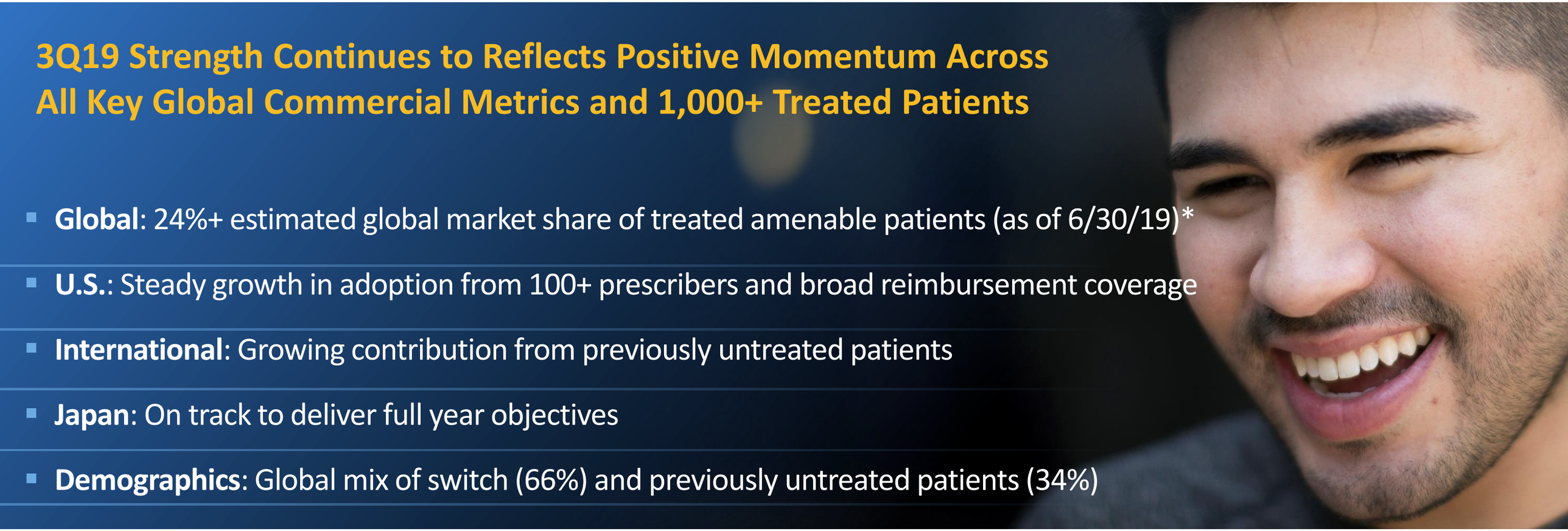


Galafold Global Launch Momentum (as of September 30, 2019)

Global commercial metrics continue to be very strong with >90% compliance and adherence, 24% global market share of treated amenable patients and continued broad market access.

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



- **Global**: 24%+ estimated global market share of treated amenable patients (as of 6/30/19)*
- **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 2Q19 and assumes a 35% amenability rate.

Integrity Leadership

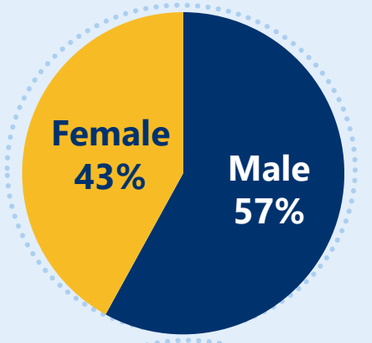
A culture of driving performance with the highest business integrity

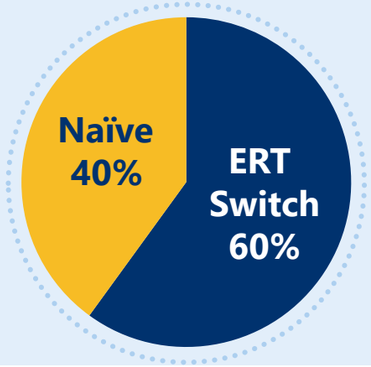
A high integrity mission for patients and shareholders

International Update (as of September 30, 2019)

Galafold international launch continues to show robust uptake across the more mature countries and in newly launched countries with further geographic expansion underway

International Patient Dynamics





Interactions with over 500 Fabry specialists across 26 countries ex-US and 2,500 other healthcare professionals

Over 170 Galafold prescribing centers ex-US

Amicus International Affiliate team members ~77% customer facing

Fabry Connections Global meeting with ~200 Fabry thought leaders, prescribers and healthcare professionals

US Launch Update (as of September 30, 2019)

Galafold U.S. launch continues to outperform on new patient starts, broad prescriber base and strong metrics

Patient Route to Galafold:

Decision to initiate Galafold

MD Completes Patient Referral Form (PRF)
Fax/Email to Amicus Assist

Amicus Case Manager Patient Intake
Preliminary Benefits Investigation (BI) for in-network specialty pharmacy (SP)
Oversee SP process
Track PA requirements/timing

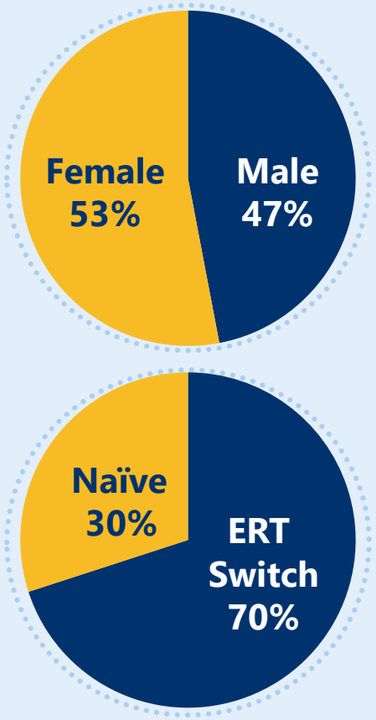
Patient on Therapy
SP obtains prior authorization (PA)
SP ships directly to patient
Coordinated with Case Manager

100+
Unique Prescribers

43 Days
Average Days to 1st Shipment
(24 Days in the last 6 months)

94%
Adherence Rate

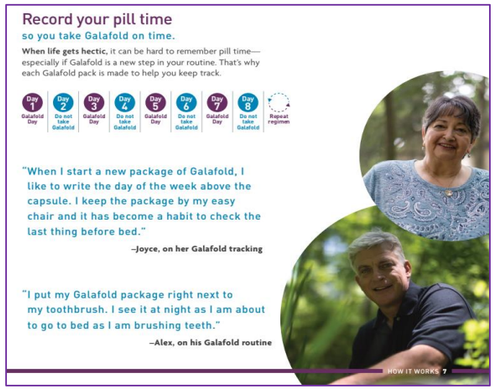
US Patient Dynamics



U.S. Momentum in First Year of Launch

Reaching the Fabry community through patient education initiatives

- Patient meetings across the US with one of 4 patient ambassadors
  - YTD: 9 meetings with 5 additional planned
  - 150 patients/family in attendance
  - Positive interactions and great interest
- Introduction of new patient education materials & digital campaign
- Formal adherence campaign began in Q3



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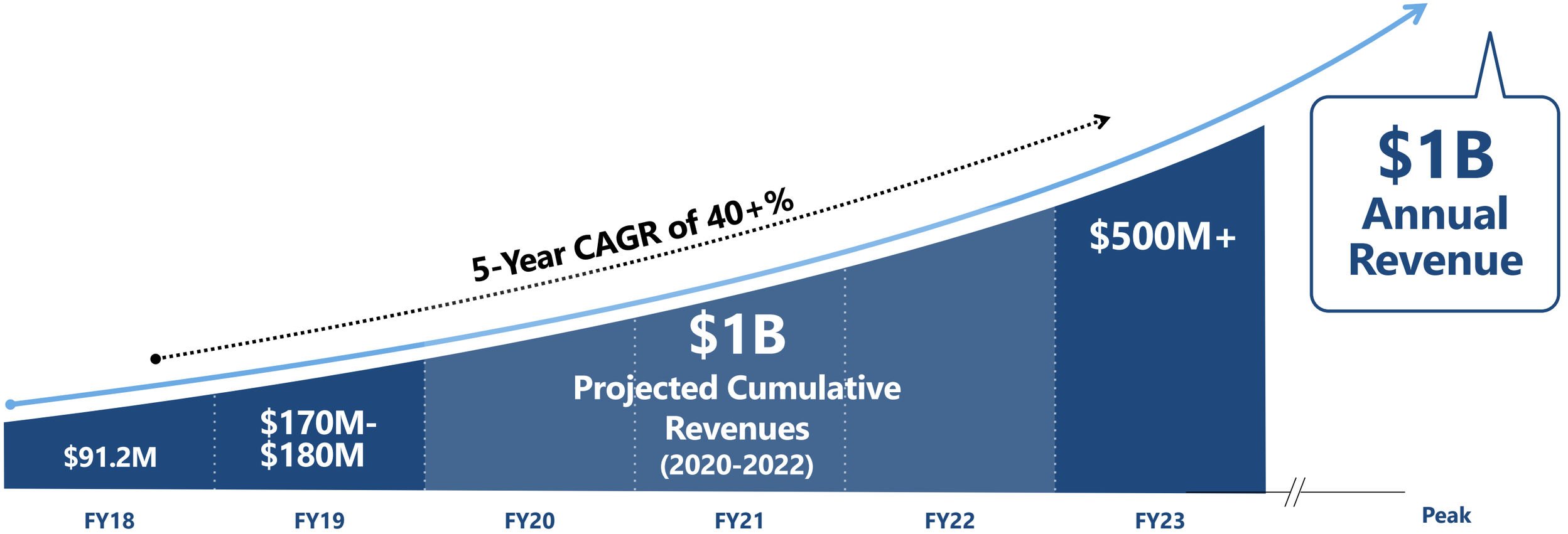
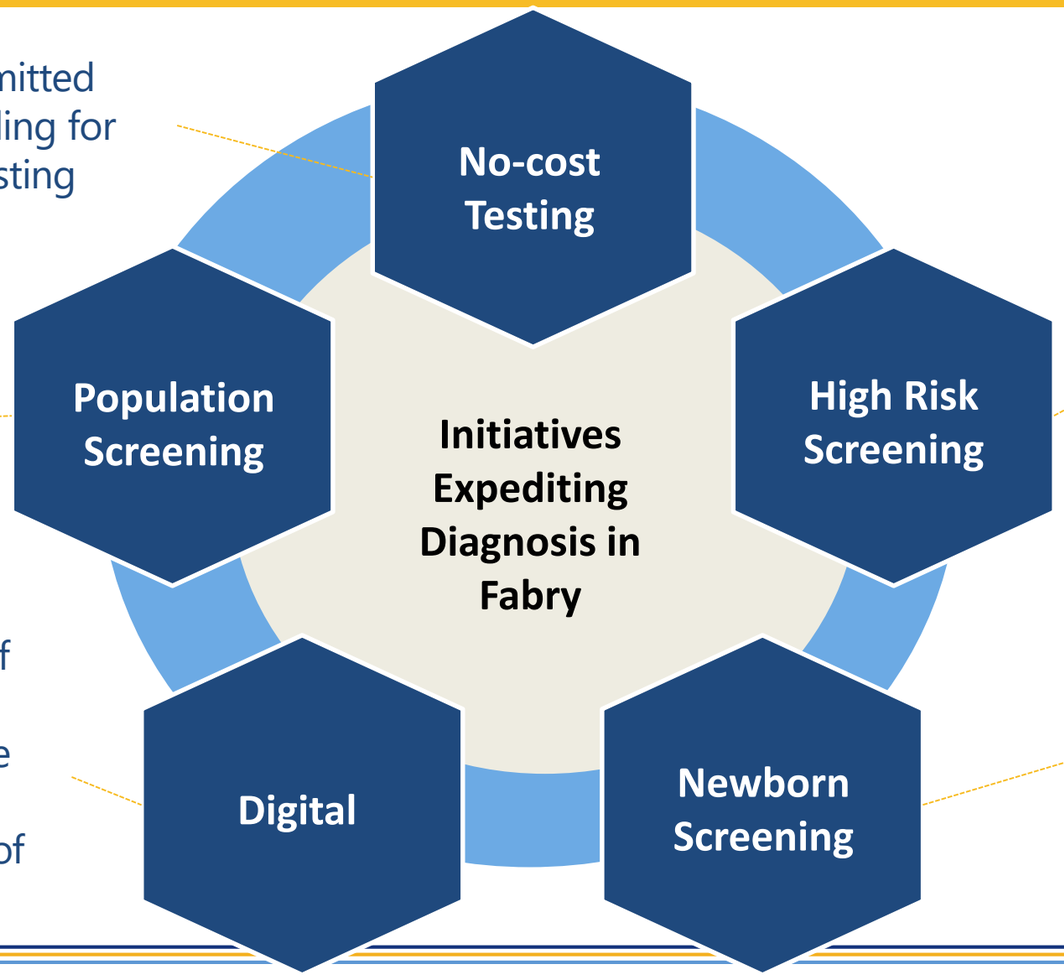


Fabry Disease Diagnostic and Growth Drivers

Fabry disease is both underdiagnosed and misdiagnosed. Expanded screening initiatives have the potential to drive a shorter pathway to correct diagnosis for individuals living with Fabry disease.



Where permitted provide funding for genetic testing

Exploring partnerships with genetic testing companies

Algorithm analysis of claims/EHR data underway in multiple geographies across common symptoms of Fabry

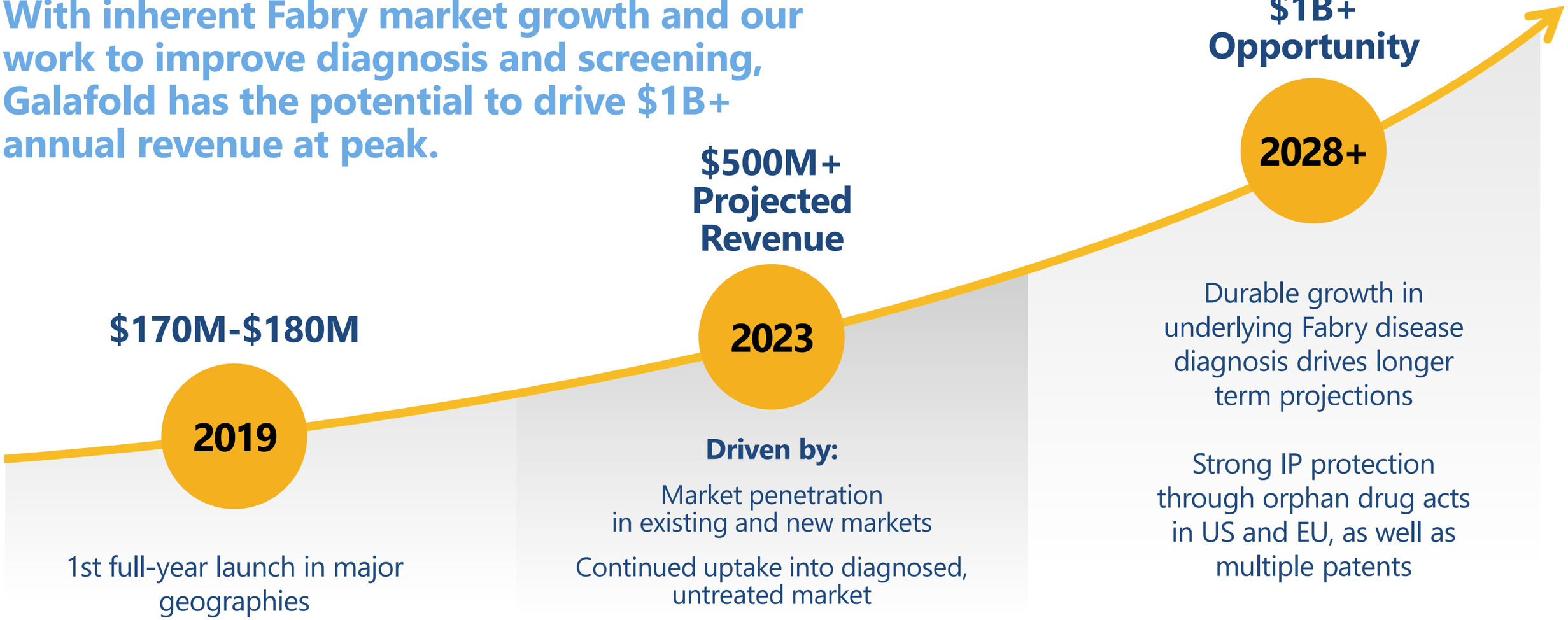
Efforts underway in areas where patients may be misdiagnosed or do not know the cause of their symptoms (e.g. pain clinics)

Amicus held Summit with world experts to discuss strategies to expand new born screening

Amicus Therapeutics

Galafold Opportunity

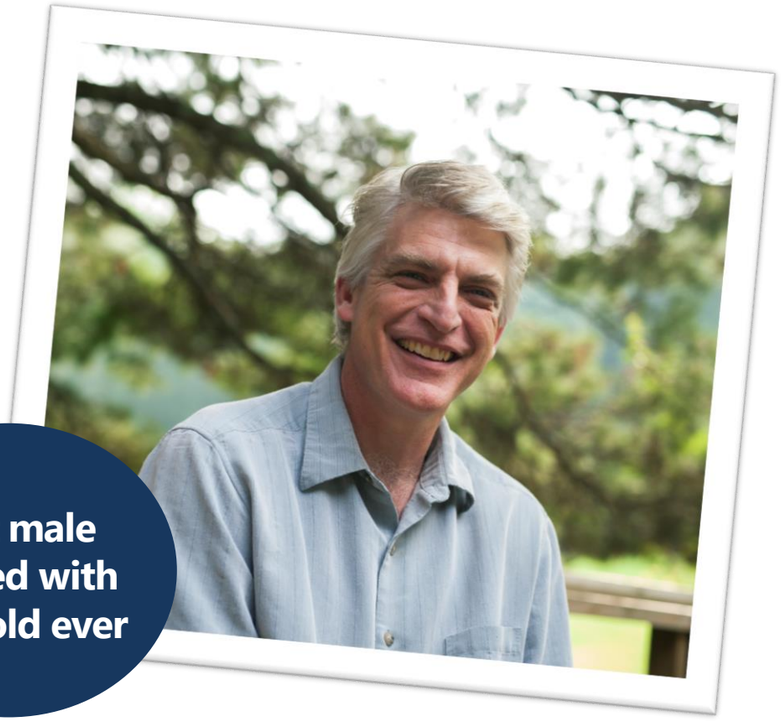
With inherent Fabry market growth and our work to improve diagnosis and screening, Galafold has the potential to drive $1B+ annual revenue at peak.



Fabry Patient Perspectives

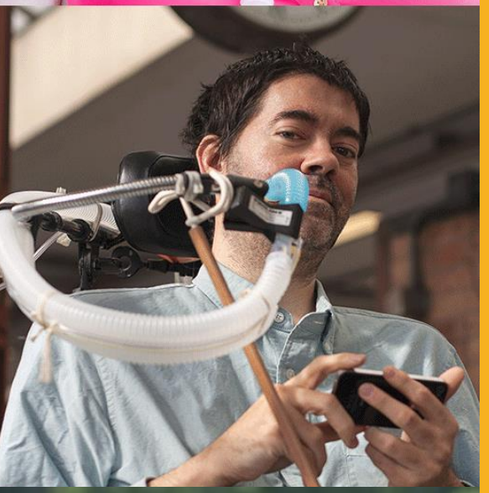
Perspectives of two people living with Fabry disease who also have extended family members living with Fabry

Alex



First male treated with Galafold ever

Sabina

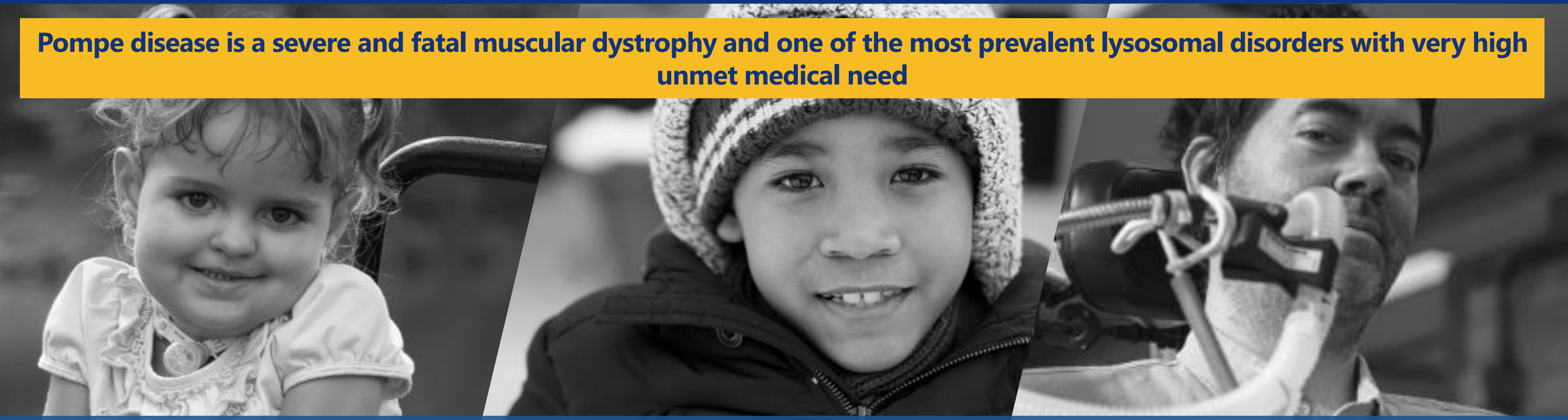


AT-GAA: Next Potential Standard of Care for Pompe Disease

Jay Barth M.D., ***Chief Medical Officer***
John F. Crowley, ***Chairman and Chief Executive Officer***

Pompe Disease Overview

Pompe disease is a severe and fatal muscular dystrophy 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

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

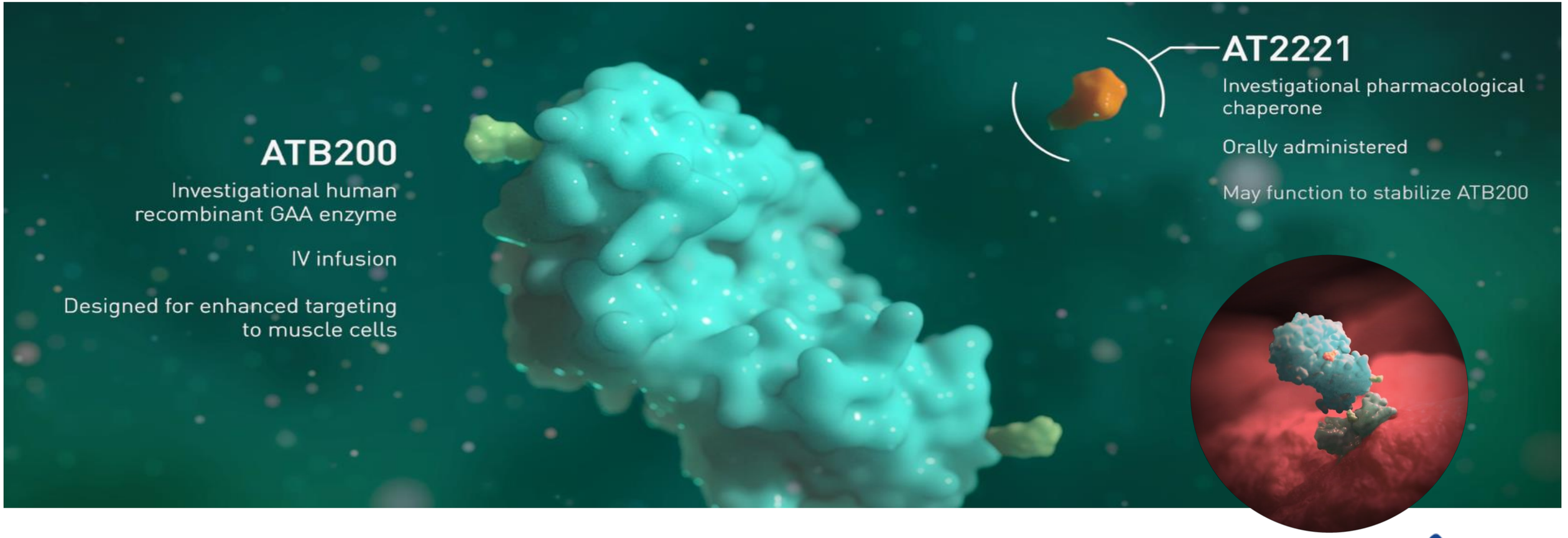
~$900M+ global Pompe ERT sales in FY18[2]

1. National Institute of Neurological Disorders and Stroke (NIH). 2. Sanofi Press Release & 10-K

Amicus Therapeutics

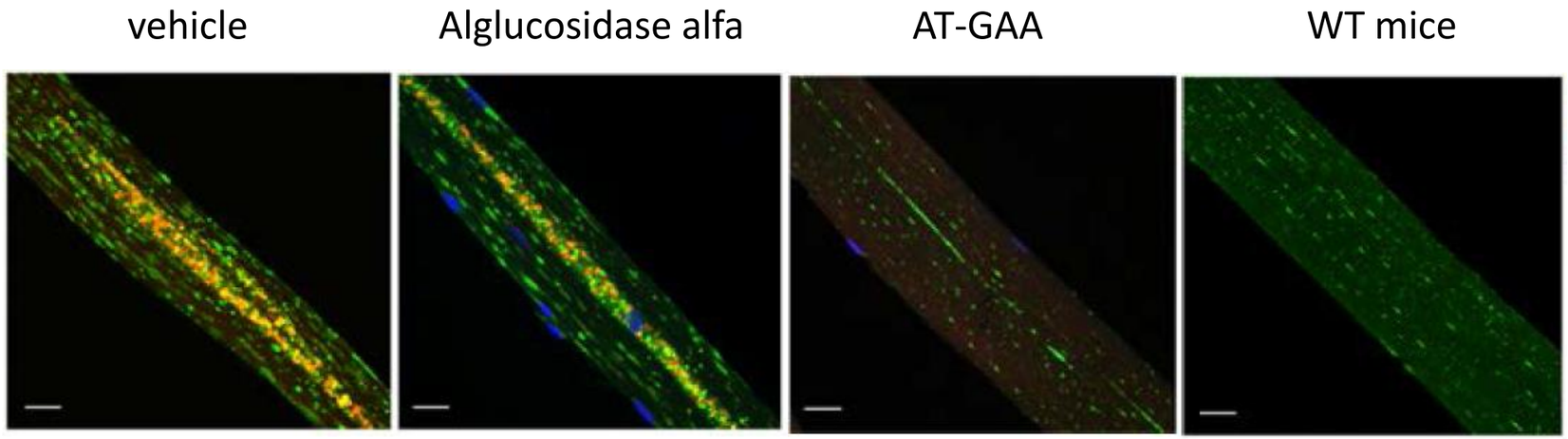
AT-GAA: Foundation in Protein Engineering

Amicus scientists specializing in protein engineering and glycobiology created a uniquely glycosylated and highly phosphorylated ERT (AT-GAA) that significantly enhances targeting to key muscles affected in patients.

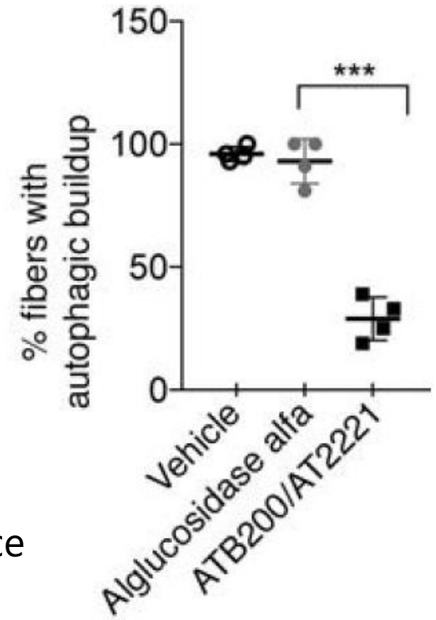


Preclinical Proof-of-Concept

Preclinical data for AT-GAA demonstrate a distinct profile for targeting and uptake into key tissues, substrate reduction, restoring cellular health and muscle integrity



Xu et al. (2019)



- GAA deficiency leads to substantial muscle damage as evidenced by autophagic buildup in *Gaa* KO mice
- Alglucosidase alfa does not reverse damage
- AT-GAA significantly clears autophagic buildup in vast majority of muscle fibers
- First treatment to demonstrate reversal of defective autophagy and muscle damage

- **Following 2 doses of 20mg/kg alglucosidase alfa or ATB200 + AT2221 in Gaa KO mice**

Source: Xu et l. 2019, JCI Insights 4(5)e125358

Breakthrough Therapy Designation

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

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

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

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

Phase 1/2 results showed strong and durable effects in patients out to two years, leading to dramatic improvements in muscle strength and function, as well as significant improvements in key biomarkers of disease

6-Min Walk Test (m)

Cohort	Baseline (n=10)	Change at Month 6 (n=10[a]) Mean (SD)	Change at Month 12 (n=10[a]) Mean (SD)	Change at Month 24 (n=9[a,b]) 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[c])	Change at Month 6 (n=9[c]) Mean (SD)	Change at Month 12 (n=9[c]) Mean (SD)	Change at Month 24 (n=8[b,c]) 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.

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

Amicus Therapeutics

Initial 6-month data in Cohort 4 (ERT-Switch Patients)

Data in additional ERT-switch patients in a decline phase support consistent, sustained and durable effects on functional outcomes and biomarkers after switching to AT-GAA

Cohort 4	Baseline		CFBL to 6M		CFBL to LOCF	
	mean (SD)	n	mean (SD)	n[a]	mean (SD)	n
6MWD	**387.3** (161.3)	6	**+24.3** (60.5)	5	**+19.3** (53.3)	6
% predicted sitting FVC	**65.3** (21.1)	6	**+6.6** (4.2)	5	**+5.2** (6.0)	6
MMT (max 80)	**59.7** (6.0)	6	**+4.0** (2.0)	5	**+3.8** (3.8)	6
Timed up and go	**9.1** (4.2)	6	0.3 (1.6)	5	**+0.6** (1.4)	5[b]
GSGC	**17.2** (5.0)	6	**-2.8** (4.0)	5	**-2.2** (3.9)	6
FSS (max 63)	**42.8** (14.0)	6	**-3.3** (4.6)	5	**-3.0** (7.2)	5[b]

- 6MWT increased in 2/5 patients at Month 6 and 4/6 patients at last available time point (3-15 months of treatment)
- FVC increased in 5/5 patients at Month 6 and 5/6 at last available time point; MIP and MEP both increased
- Last available time point includes 1 subjects at Month 3, 2 subjects at Month 6, 2 subjects at Month 12 and 1 subject at Month 15

Historical data on 6MWT showed an average decline of ~7 meters per year while on standard of care ERT prior to switching to AT-GAA (n=6), with 5/6 patients declining.

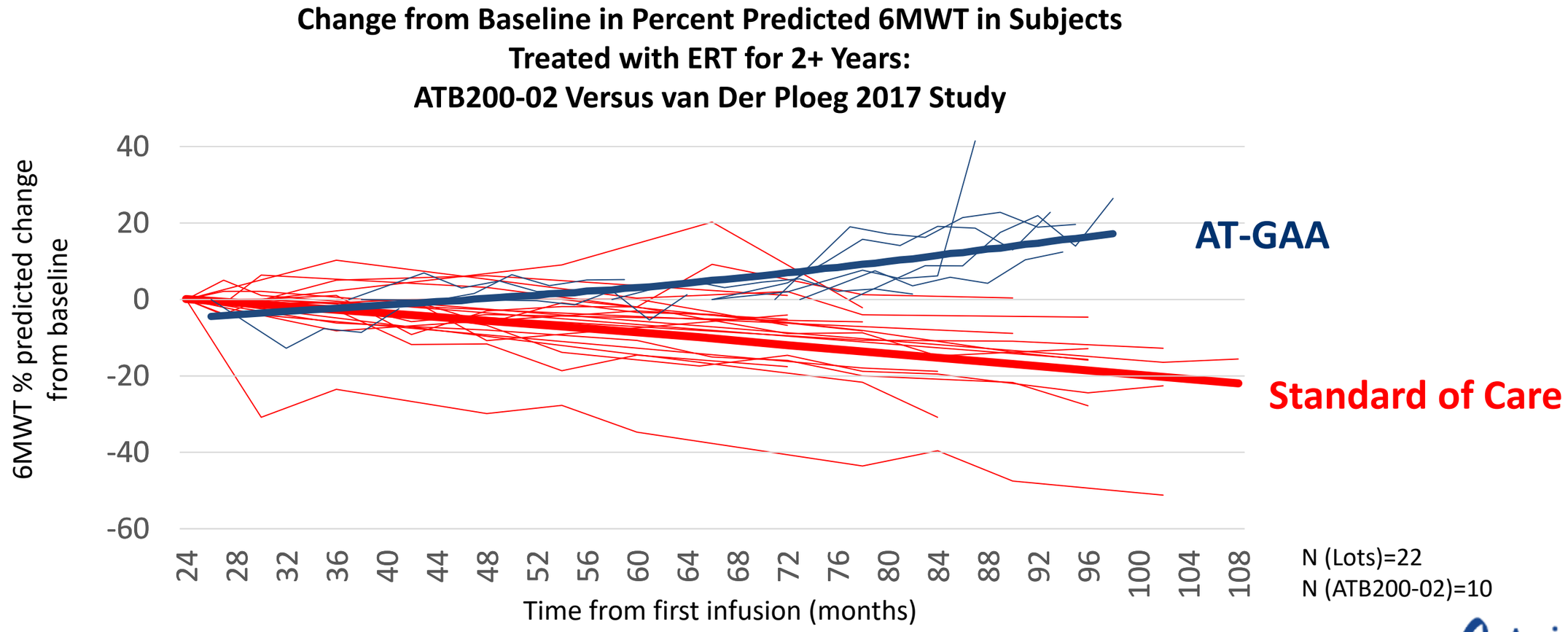
[a] only 5 patients had completed month 6 assessment at time of IA; [b] One patient missing data for Timed up and go and one patient missing data for FSS

Amicus Therapeutics

6MWT Natural History: Phase 1/2 AT-GAA Data vs. Medical Literature

van Der Ploeg 2017

Natural history comparisons show large treatment effect of AT-GAA on 6MWD in Phase 1/2 which supported statistical power of PROPEL pivotal study

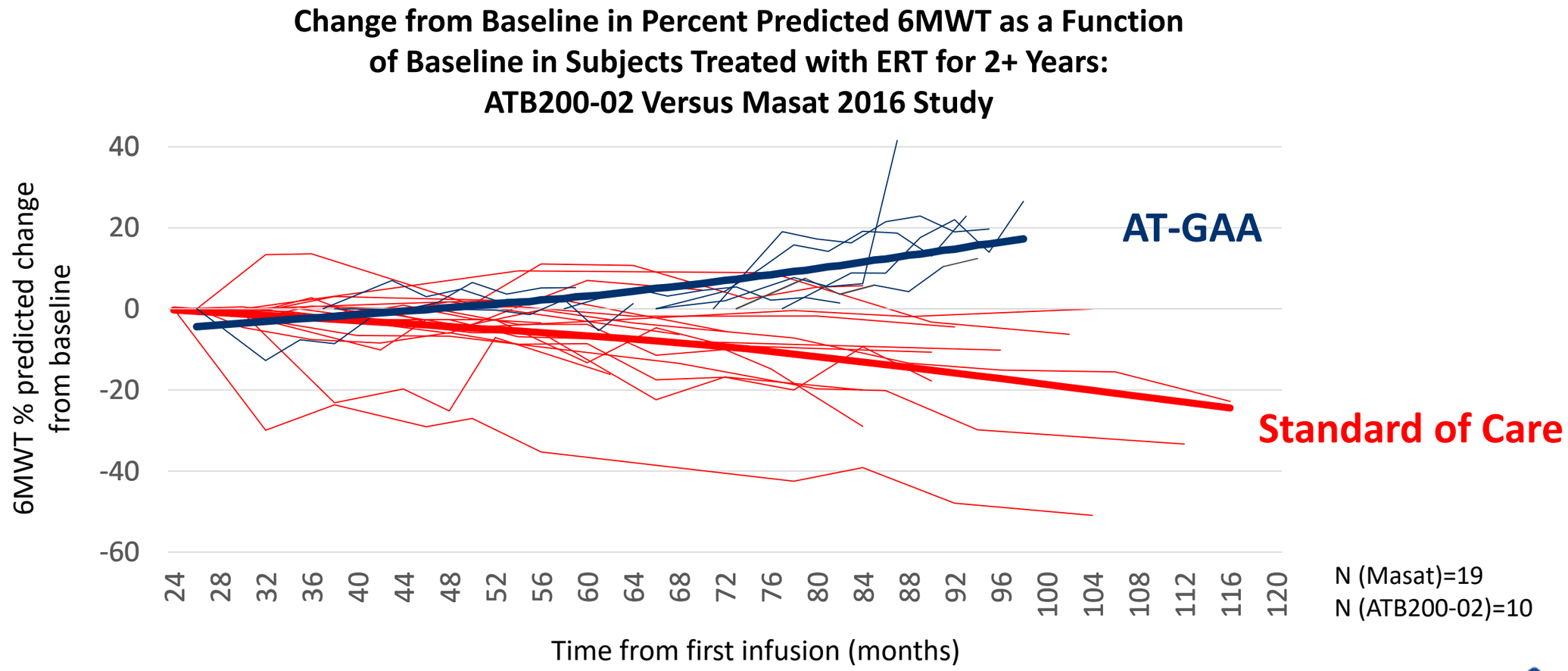


Source: ATB200-02 IA#7; Ans T. van der Ploeg [et] al. Poster presented at the 13th Annual WORLD Symposium™ 2017, February 13–17, 2017, San Diego, CA, USA

6MWT Natural History: Phase 1/2 AT-GAA Data vs. Medical Literature

Masat 2016

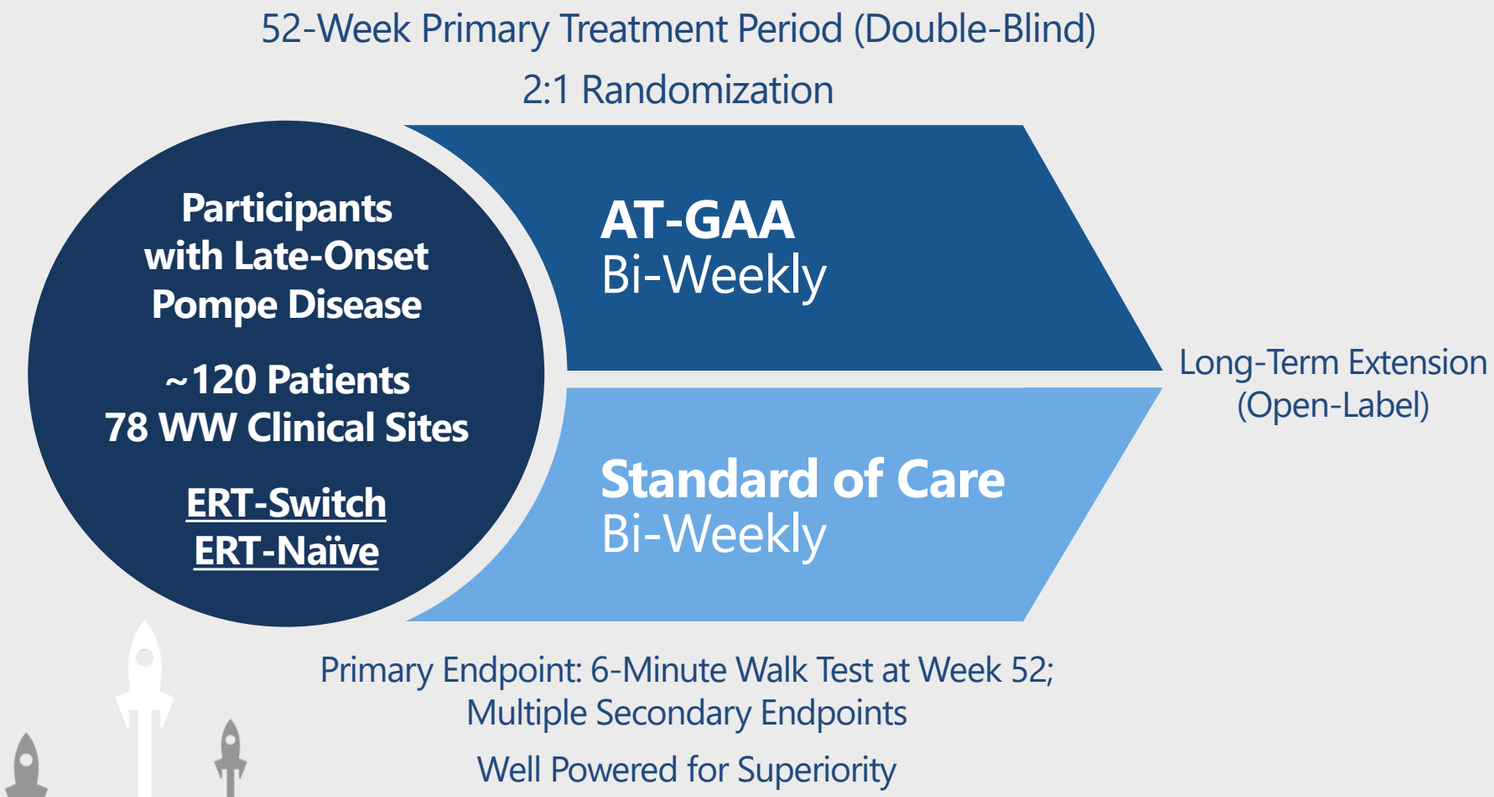
Second natural history data set confirms the large treatment effect of AT-GAA



Source: ATB200-02 IA#7; Masat et al 2016; Nature Scientific Reports | 6:36182

PROPEL (ATB200-03) Study Design

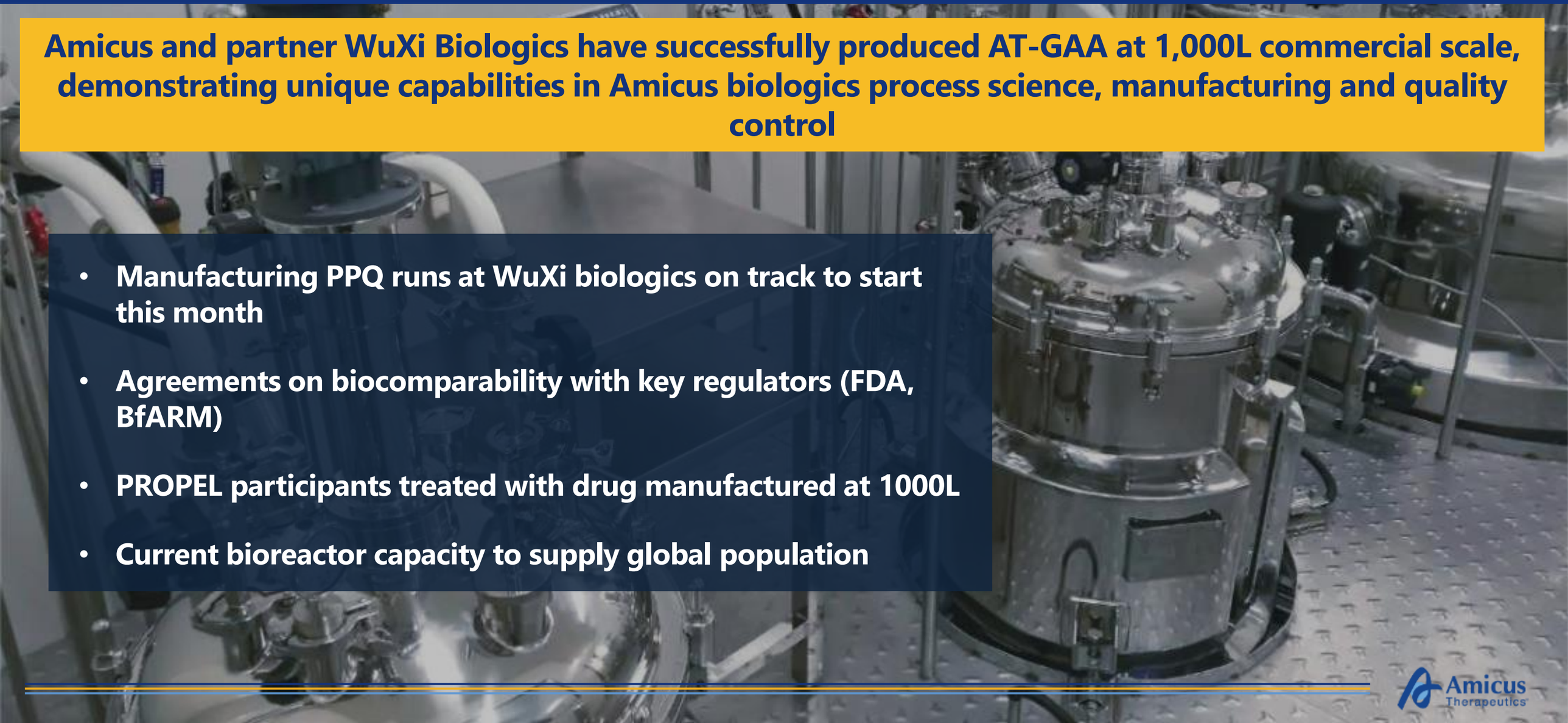
Phase 3 enrollment is expected to complete in 2019 with data in 1H2021. The study is highly powered for success and supports a broad label, with FDA and EMA agreement on study design and primary endpoint (6MWT).



PROPEL Pivotal Study 80%+ Enrolled and Expected Now to Over-enroll By YE2019 with data in 1H2021

Pompe Biologics Manufacturing

Amicus and partner WuXi Biologics have successfully produced AT-GAA at 1,000L commercial scale, demonstrating unique capabilities in Amicus biologics process science, manufacturing and quality control

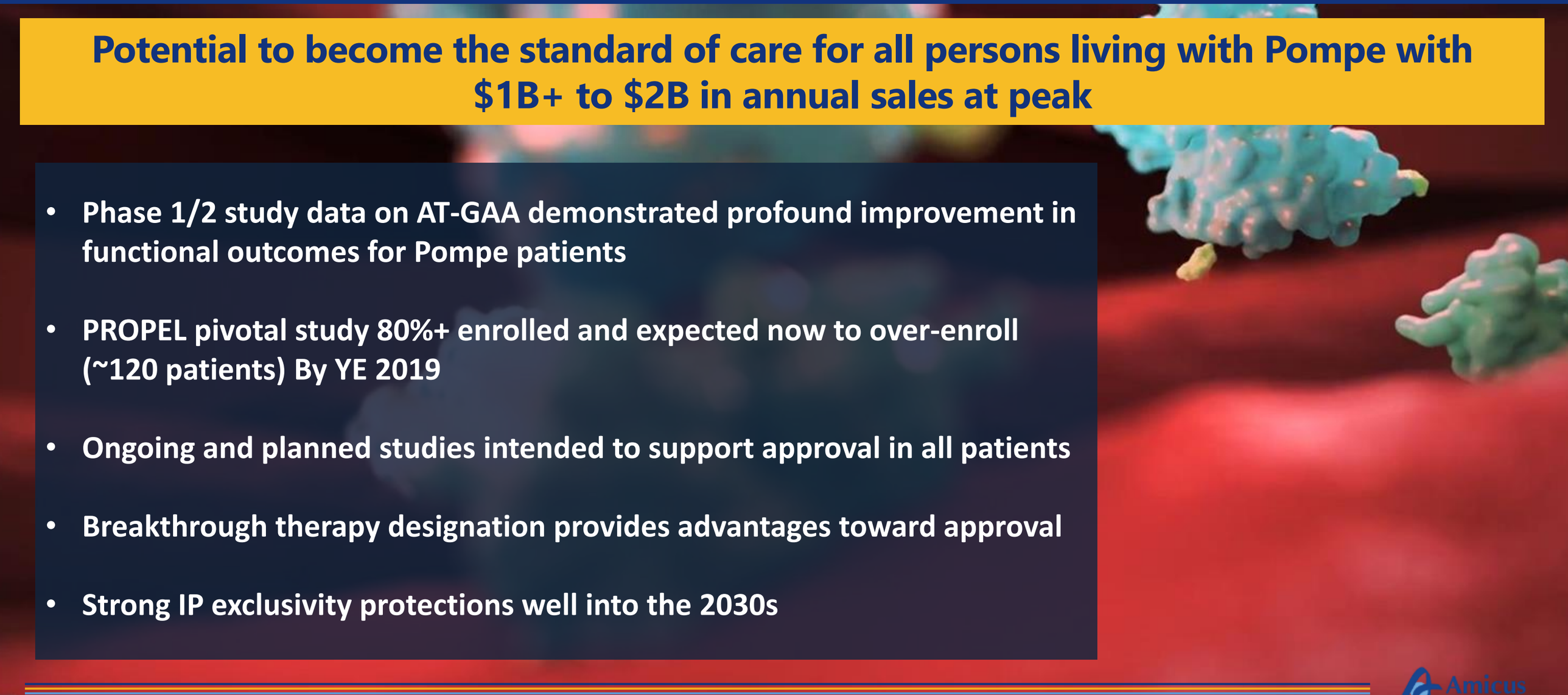


- **Manufacturing PPQ runs at WuXi biologics on track to start this month**
- **Agreements on biocomparability with key regulators (FDA, BfARM)**
- **PROPEL participants treated with drug manufactured at 1000L**
- **Current bioreactor capacity to supply global population**

AT-GAA Treatment Opportunity

Potential to become the standard of care for all persons living with Pompe with $1B+ to $2B in annual sales at peak

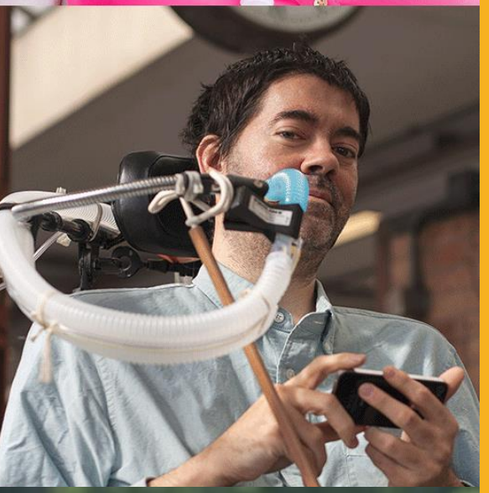
- Phase 1/2 study data on AT-GAA demonstrated profound improvement in functional outcomes for Pompe patients
- PROPEL pivotal study 80%+ enrolled and expected now to over-enroll (~120 patients) By YE 2019
- Ongoing and planned studies intended to support approval in all patients
- Breakthrough therapy designation provides advantages toward approval
- Strong IP exclusivity protections well into the 2030s



Pompe Disease Overview

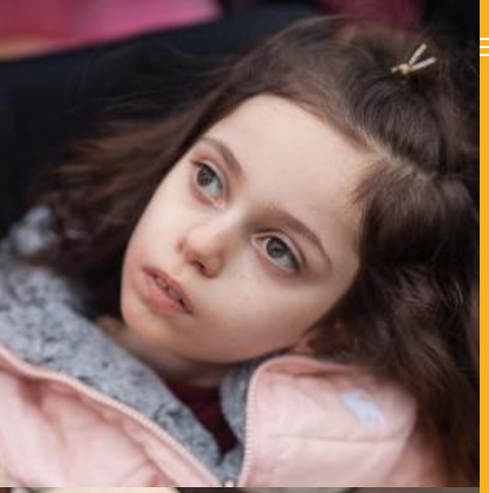
A perspective of Pompe disease demonstrates the unmet medical need

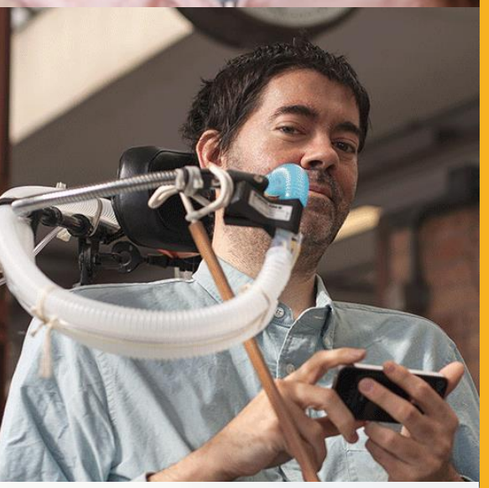
Video:

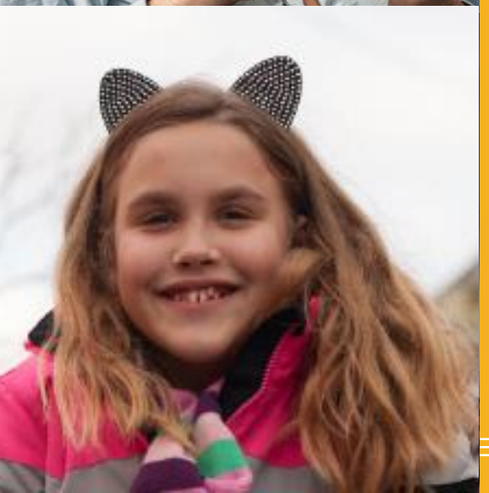


Q&A Session

2019 Analyst Day | October 10, 2019 | New York, NY







Next Generation Gene Therapy Platform

Hung Do, Ph.D.

2019 Analyst Day | October 10, 2019 | New York, NY

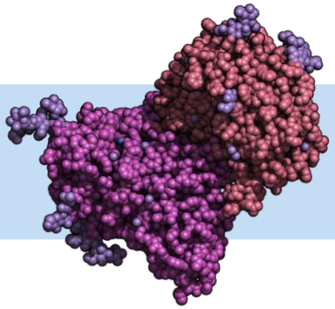
A Natural Evolution: Chaperones to Optimized ERT to Gene Therapy

Amicus' shift towards gene therapy is built upon years of experience in developing genetic medicines designed to deliver deficient proteins to target cells and organelles

Pharmacological Chaperones

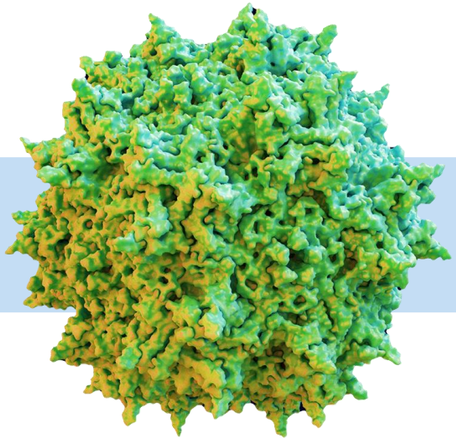
Stabilize
"naturally produced" enzymes

Next-Generation ERTs



Stabilize and target
"externally produced" enzymes

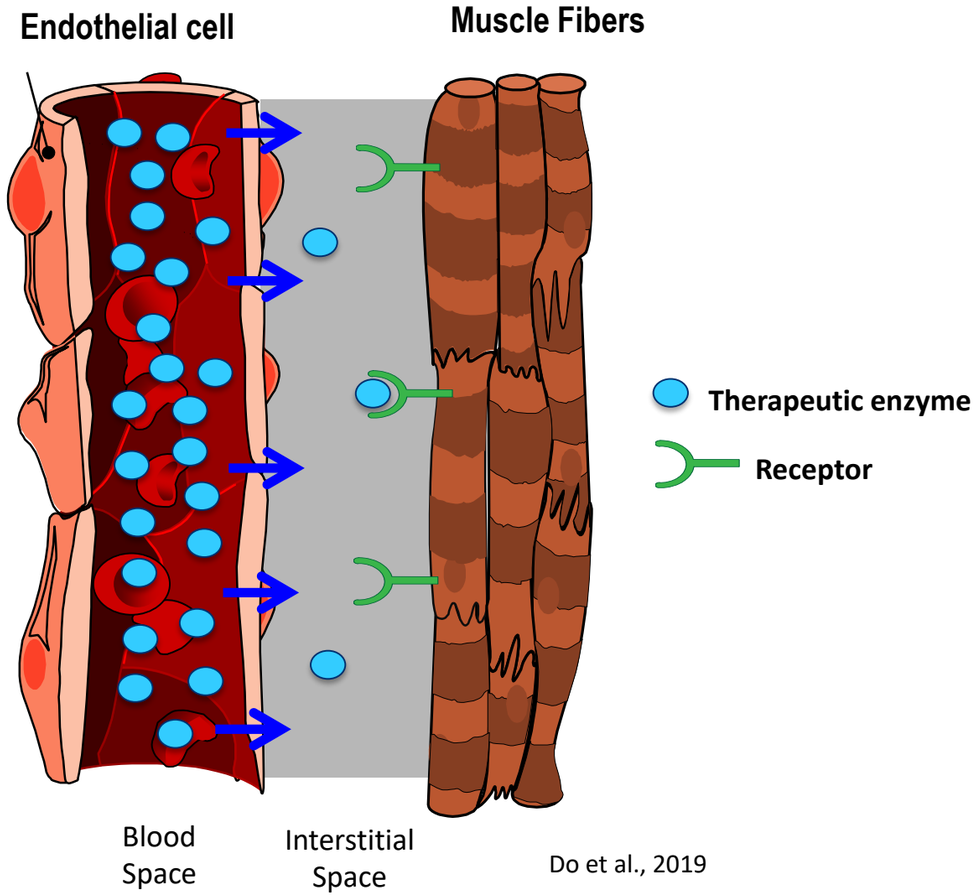
Gene Therapies



Stabilize and target
"internally produced" enzymes

Challenges of Protein Delivery Today

Therapeutic enzymes have similar challenges whether delivered exogenously by ERT or via gene therapy



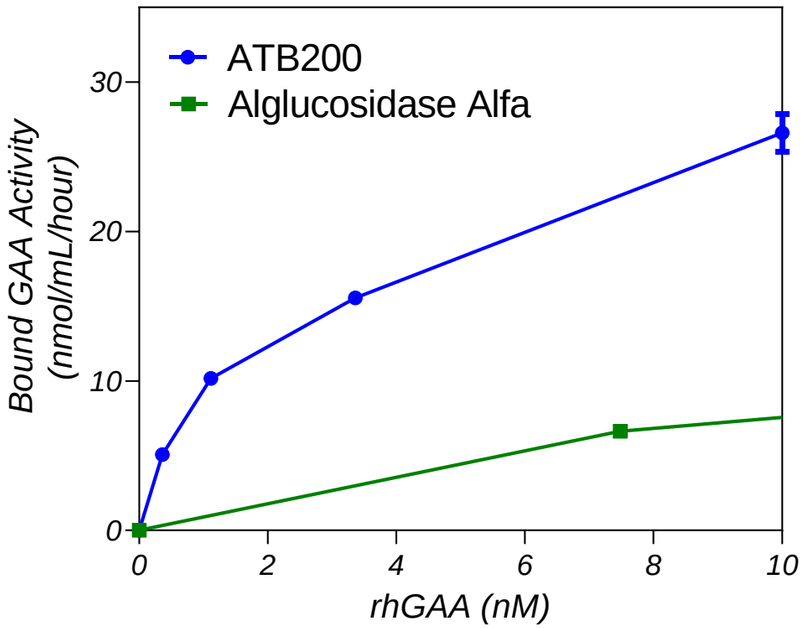
Do et al., 2019

- Biodistribution of therapeutic proteins from circulation to intended cells is poor
- These challenges necessitate highly efficient uptake and trafficking mechanisms
- Only therapeutic enzymes with optimal characteristics for cellular uptake can be efficiently internalized in target cells at low enzyme levels

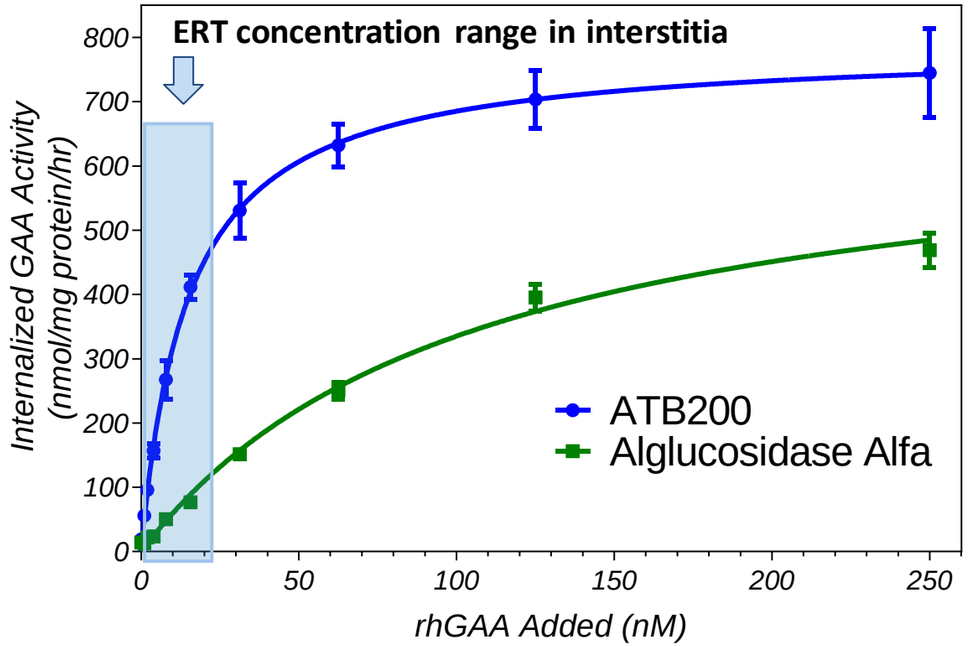
Importance of Cell Surface Receptor Binding

Therapeutic enzymes must have high affinity for cell surface receptors to enable efficient cellular uptake

Binding Affinity for M6P Receptor: Alglucosidase alfa vs ATB200



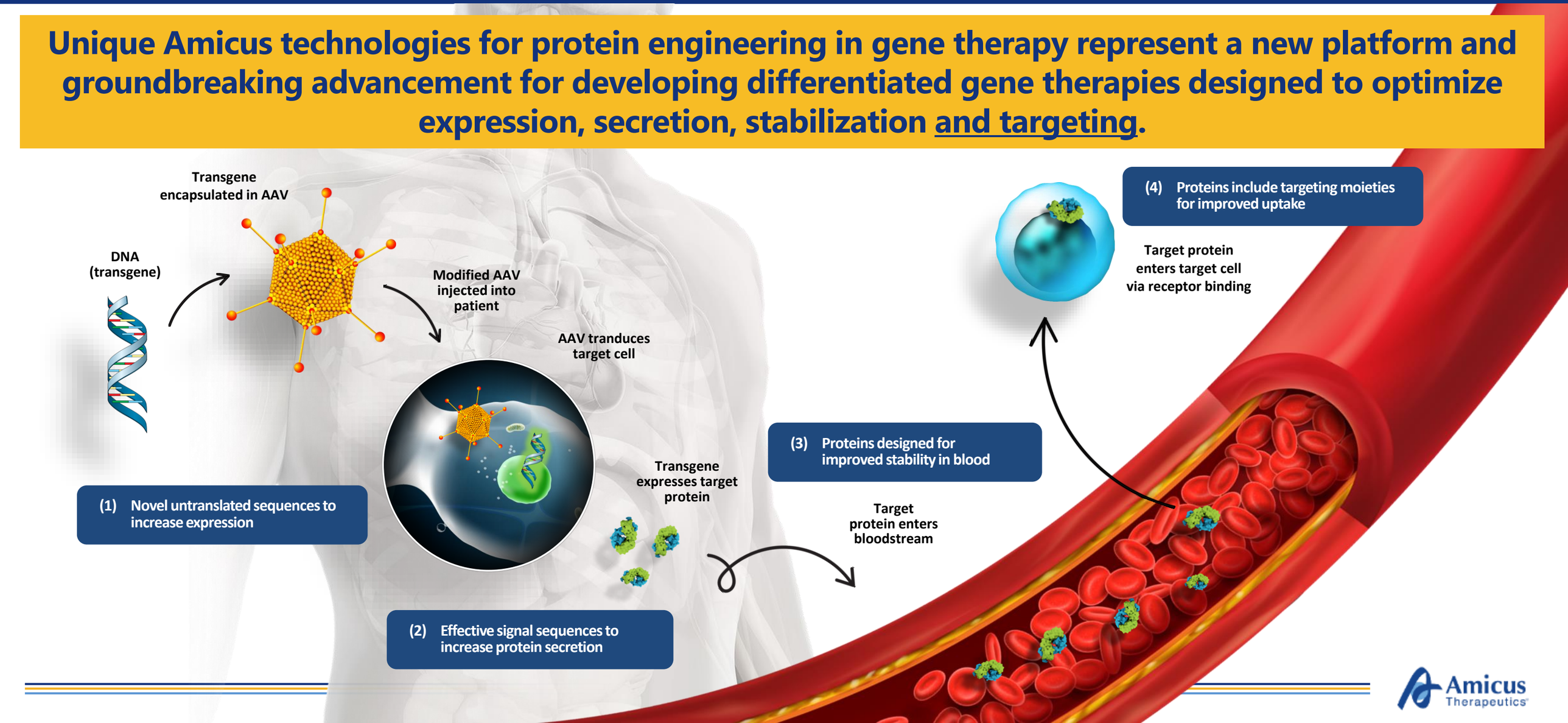
rhGAA Uptake in Skeletal Muscle Myoblasts: Alglucosidase alfa vs ATB200



Source: Data on file

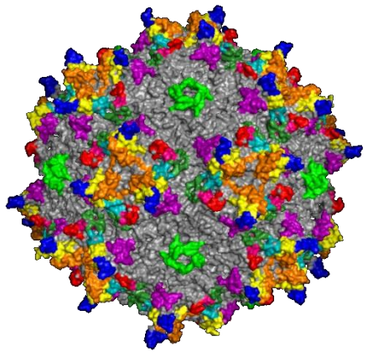
Amicus Approach: Engineered Transgenes for Optimal Cross-Correction

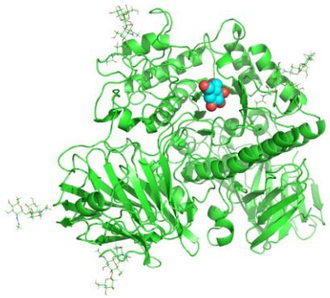
Unique Amicus technologies for protein engineering in gene therapy represent a new platform and groundbreaking advancement for developing differentiated gene therapies designed to optimize expression, secretion, stabilization and targeting.



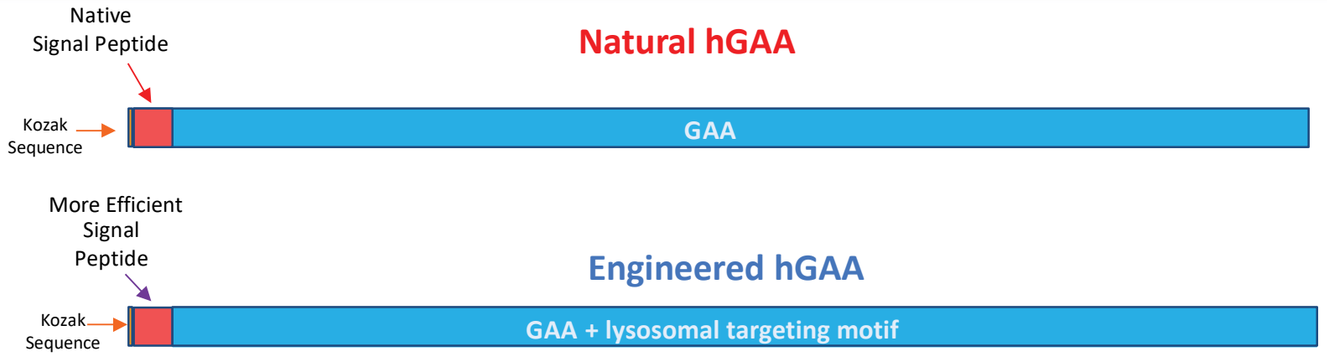
Pompe AAV Gene Therapy Initial High-Dose Preclinical POC Study

- AAV Transgene:
  - Natural-hGAA (AAV.hGAA nat)
  - Engineered-hGAA (AAV.hGAA eng)
- Dose\Route:
  - **5e11 gc/mouse (~2.5e13 gc/kg)**
  - Tail Vein IV
- Animal Model:
  - Pompe Model *Gaa* -/- B6:129-GAAtm1Rabn/J (aka 6neo)
  - Wild-type *Gaa* +/+ (Pompe model litter-mates)
- Gender:
  - Male
  - Female
- Age: 4-6 weeks at AAV dosing

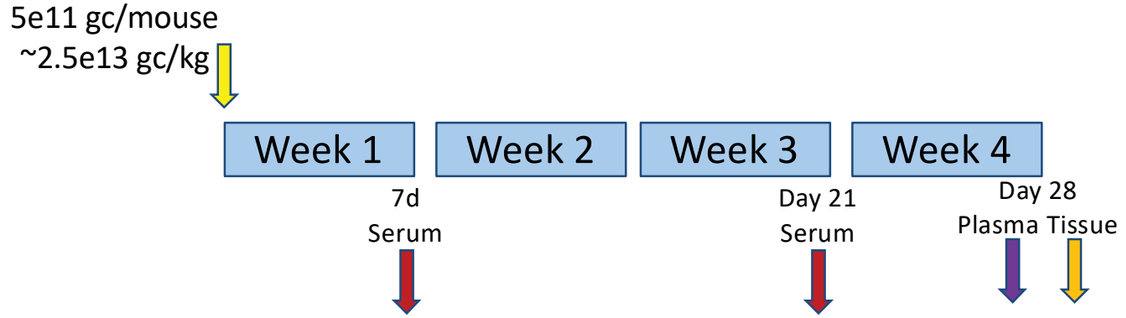




AAV Expressing Trans-Gene Co nstructs



Study Design: 4 Week Animal Study

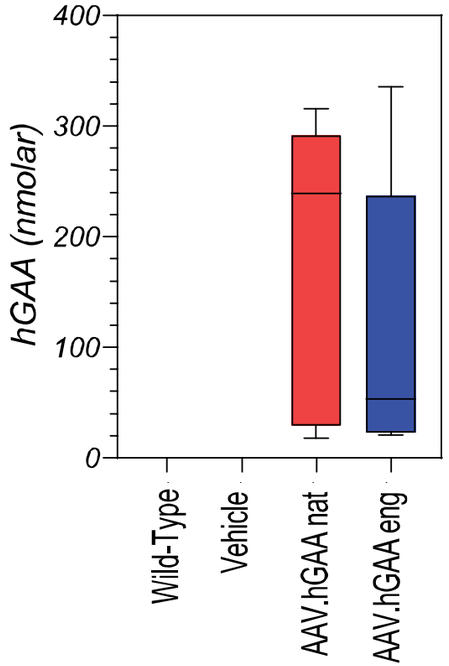


Source: Molecular Therapy Vol 27 No 4S1 April 2019, Abstract 518

Initial Preclinical Pompe Gene Therapy Results: Plasma

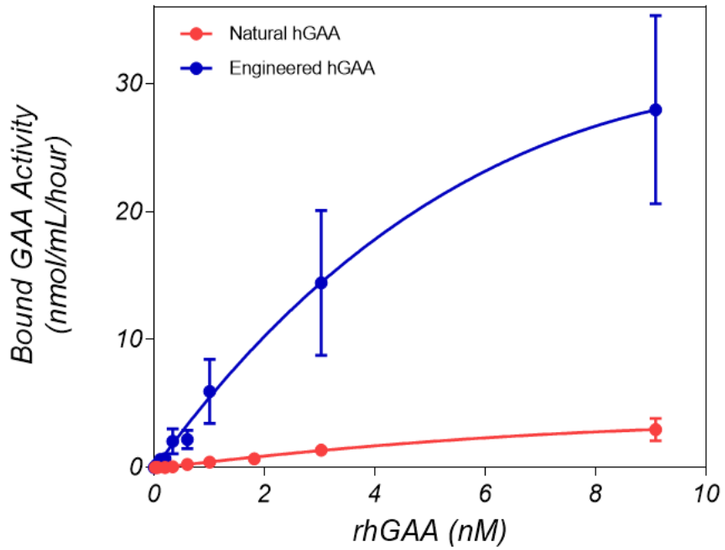
AAV with the Amicus engineered hGAA transgene was expressed at similar levels in plasma and had significantly enhanced binding for cell uptake receptors compared to the unmodified natural GAA transgene

Plasma Expression



- **High levels of engineered and natural hGAA were measured in plasma at day 28**

Receptor Binding



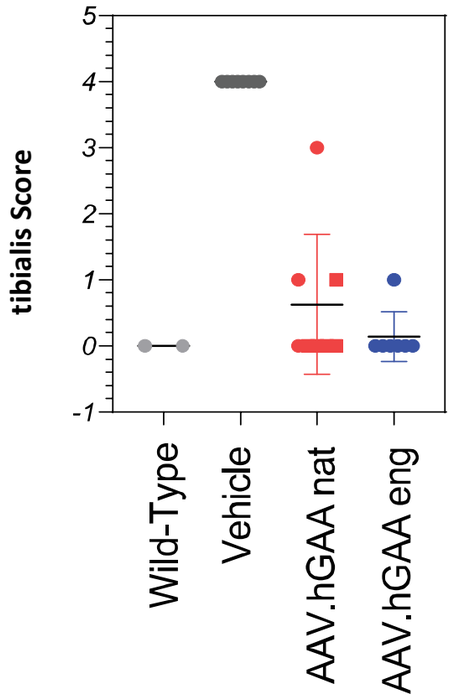
- **Only engineered hGAA was able to efficiently bind the intended cell uptake receptor**

Source: Molecular Therapy Vol 27 No 4S1 April 2019, Abstract 518

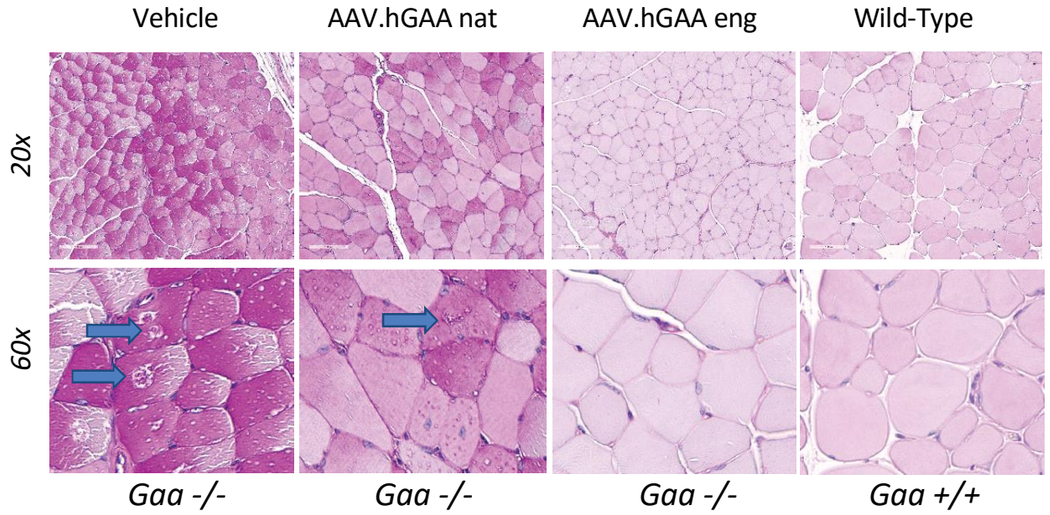
Initial Preclinical Pompe Gene Therapy Results: Muscle

AAV with the Amicus engineered hGAA transgene had a more uniform and complete impact on cell pathology and glycogen reduction in muscle compared to the unmodified natural GAA transgene

Tibialis: Histopath



Tibialis: Glycogen PAS



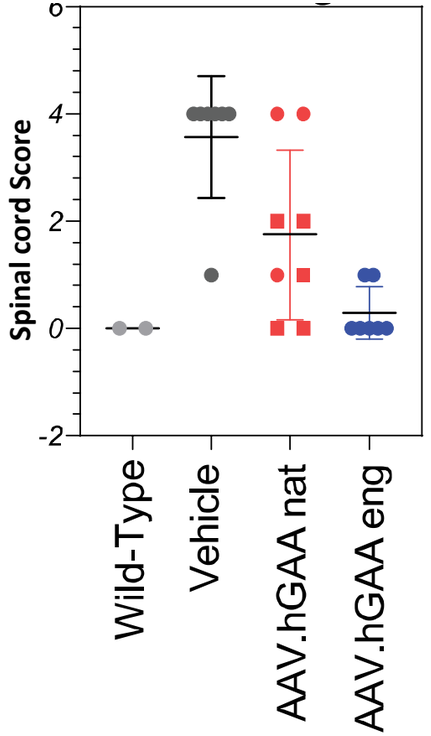
- **Similar results observed in other muscle groups**

Source: Molecular Therapy Vol 27 No 4S1 April 2019, Abstract 518

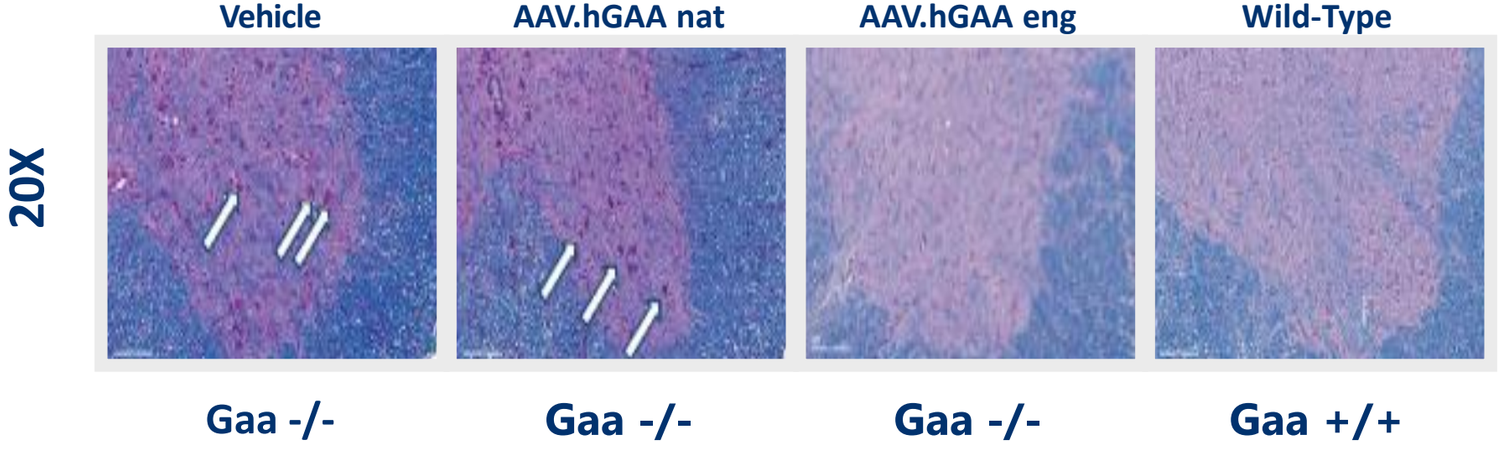
Initial Preclinical Pompe Gene Therapy Results: CNS

Only the AAV with the Amicus engineered hGAA transgene was able to significantly impact cell pathology and glycogen reduction in the CNS

Spinal Cord: Histopath



Spinal Cord: Glycogen PAS



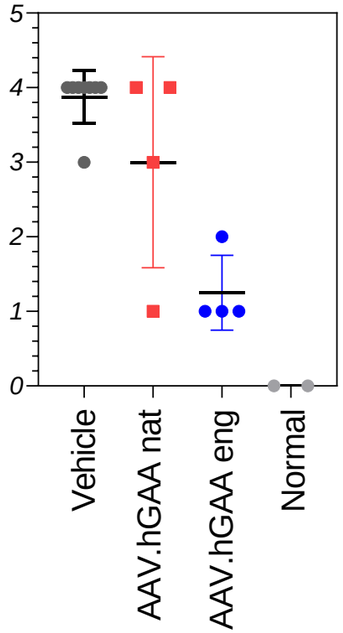
- Similar results observed in brain

Source: Molecular Therapy Vol 27 No 4S1 April 2019, Abstract 518

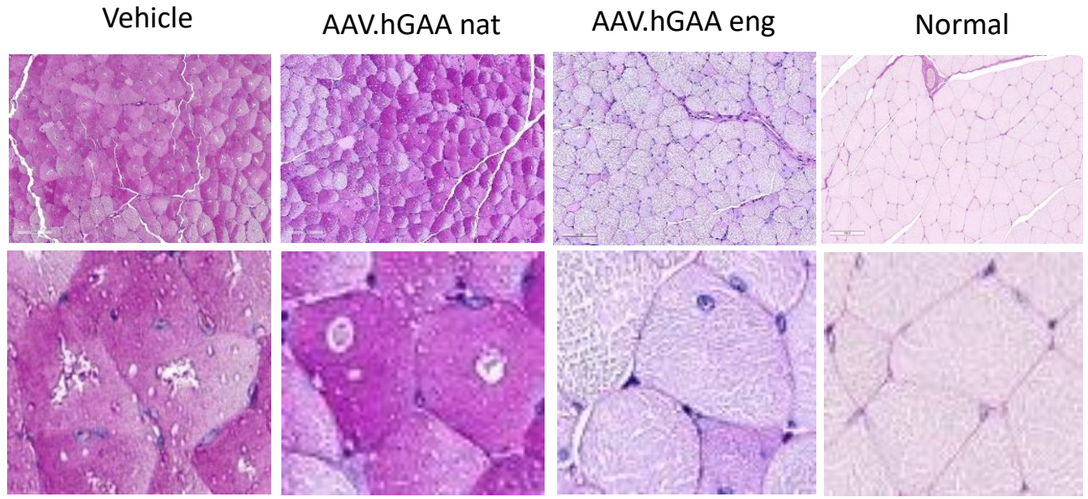
New Pompe Gene Therapy Low Dose Preclinical Data

Results from the low dose (2.5e12 gc/kg) study of engineered AAV-hGAA also showed improved cell pathology and glycogen reduction of the engineered construct versus natural GAA

Quadriceps: Histopath Scoring



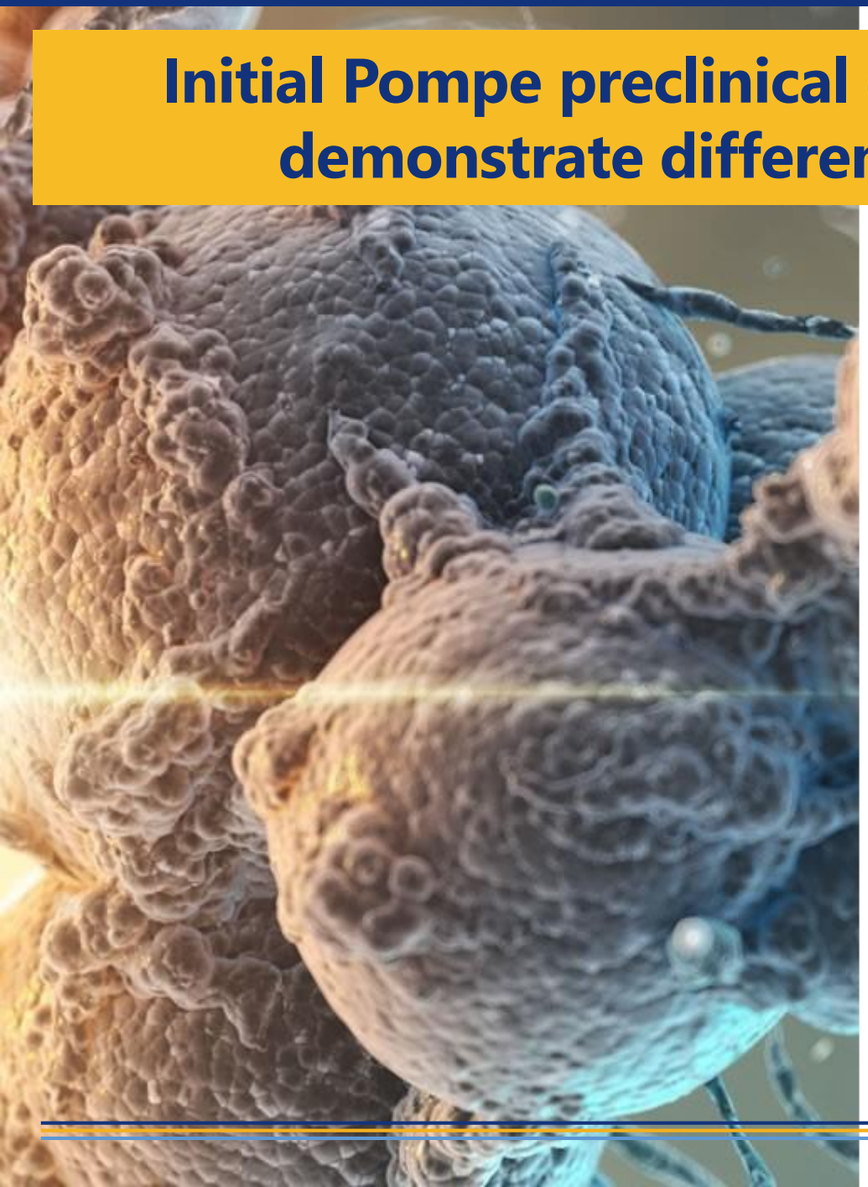
Quadriceps: Glycogen PAS



- **Similar design as high dose preclinical study**

Source: Data on file

Pompe Gene Therapy Summary and Next Steps



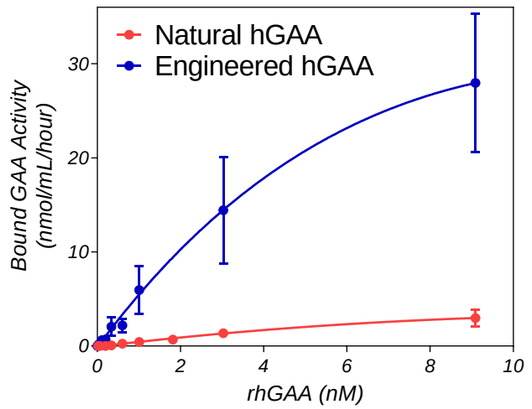
Initial Pompe preclinical gene therapy data at ASGCT and NEW preclinical data demonstrate differentiated profile and clear pathway toward the clinic

- Clinical candidate selected
- Toxicology batch manufacturing and GMP tech transfer to Paragon underway
- IND enabling toxicology studies to begin shortly
- Potential to enter clinic in 1H2021

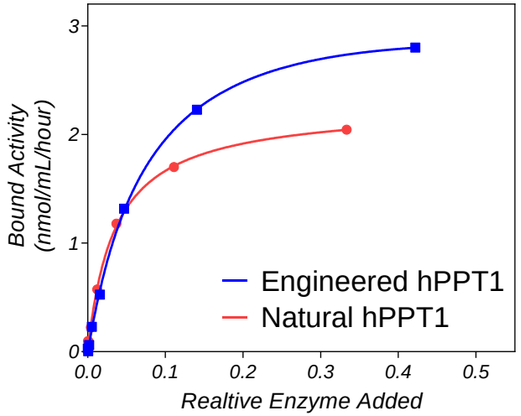
Protein Engineering Platform Has Potential To Be Broadly Applicable to Gene Therapies For Majority of LSDs

Amicus has repeatedly validated the protein engineering platform approach in multiple indications to design transgenes with improved cellular uptake

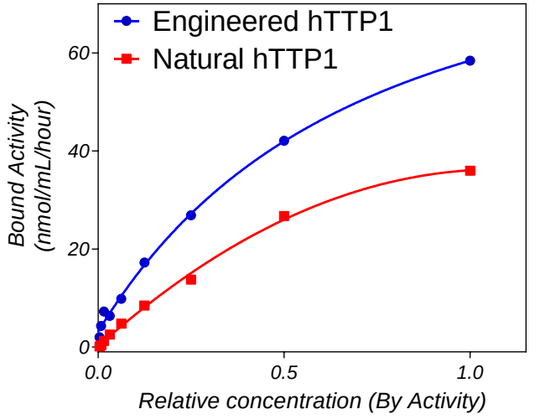
Pompe - GAA



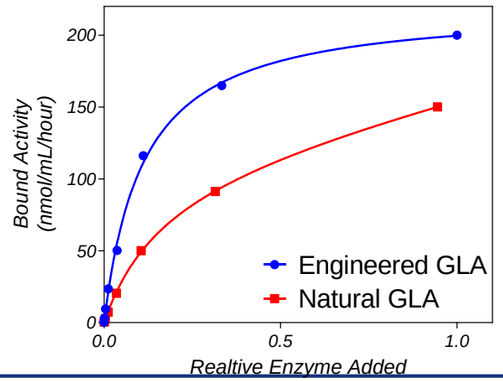
CLN1 – PPT1



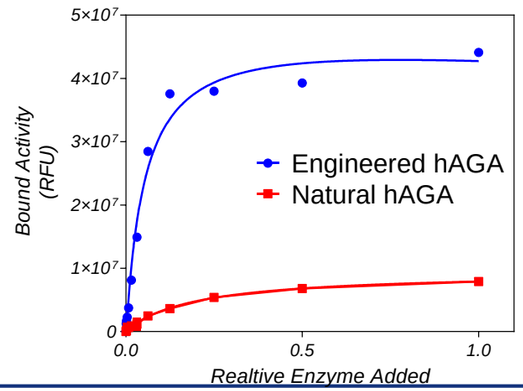
CLN2 – TTP1



Fabry – GLA



AGU – AGA



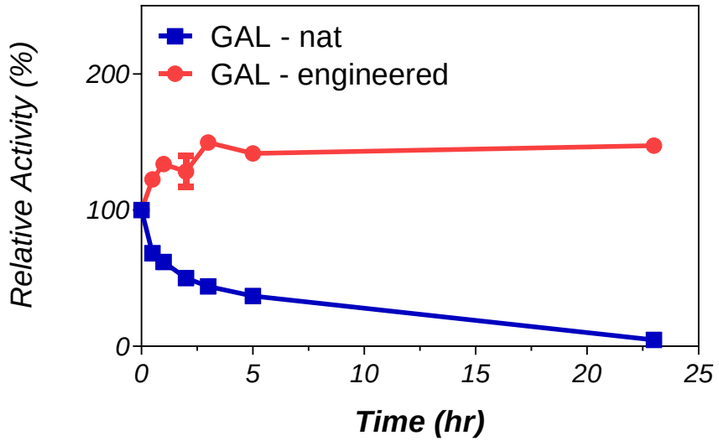
Source: Data on file

Initial Preclinical Fabry Gene Therapy Results

Engineered GLA transgene increased enzyme activity in kidney >7 fold higher than wildtype and lowered GL-3 substrate levels comparable to wildtype

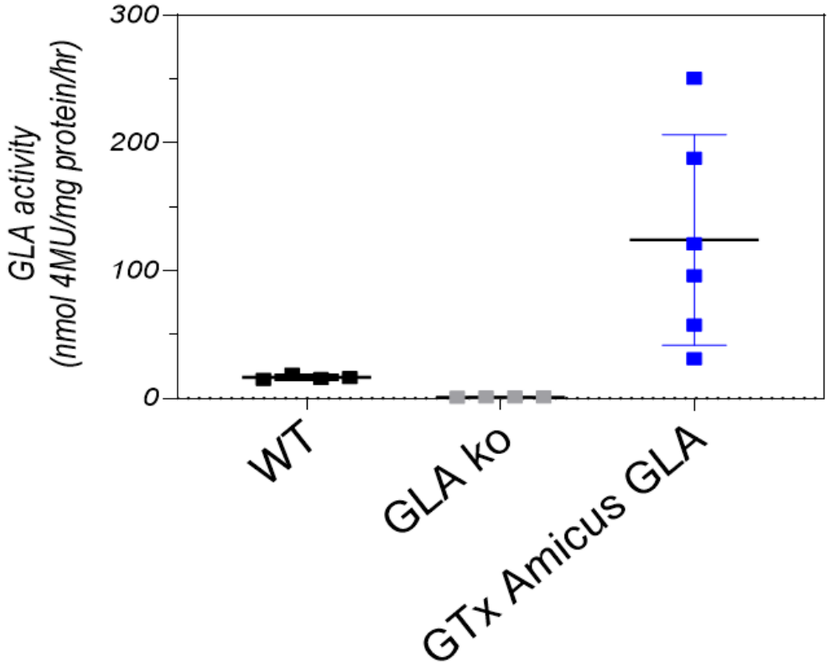
Stabilized Transgene Product

GLA activity



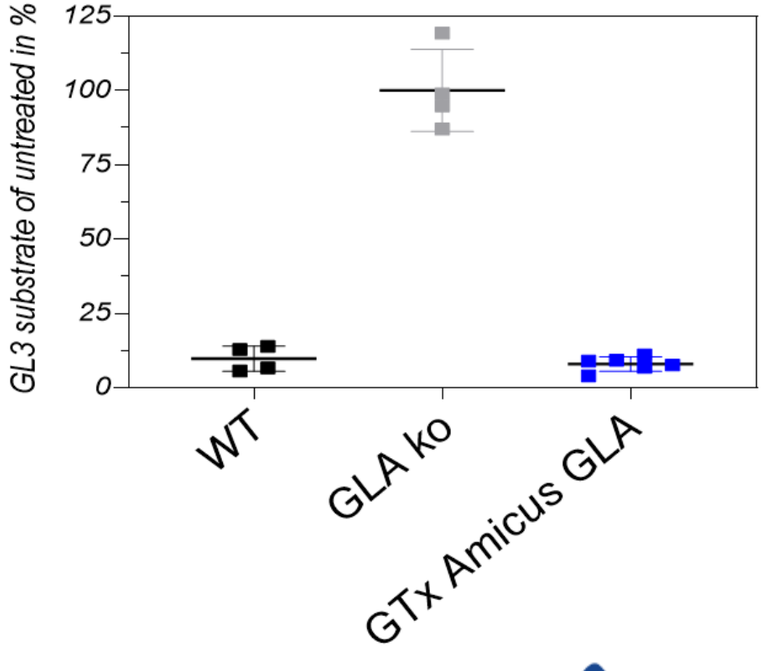
Increased Enzyme Activity

Kidney: GLA Enzyme Activity



Robust Substrate Clearance

Kidney: GL3 Substrate Levels



Source: Data on file

GTx Manufacturing Strategy for Initial Penn Programs at Catalent

Catalent to leverage Penn's AAV manufacturing expertise and Amicus experience in complex biologics manufacturing and quality control as a competitive advantage.

Catalent BIOLOGICS

PARAGON BIOSERVICES

Technologies and capabilities related to Penn collaboration are being transferred to and developed at Catalent

Initial focus on Amicus Pompe AAV gene therapy program

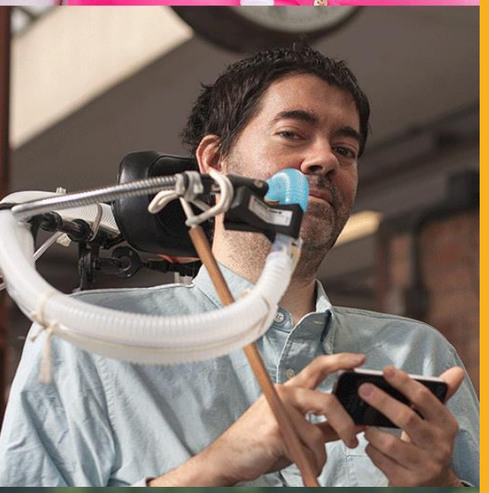
Penn

Penn is supporting technology transfer of existing manufacturing process to Catalent

aldevron

Long-term supply agreements established for research and GMP quality plasmids

Amicus Therapeutics GENE THERAPY



Next Generation Research Program

Jeff Castelli, Ph.D., ***Chief Portfolio Officer and Head of Gene Therapy***

Jim Wilson, M.D., Ph.D., ***Rose H Weiss Professor and Director, Orphan Disease Center, Perelman School of Medicine at the University of Pennsylvania***

2019 Analyst Day | October 10, 2019 | New York, NY

Amicus and Penn Gene Therapy Collaboration

Combines Amicus Expertise in Protein Engineering with Penn's AAV Vector Technology, Manufacturing and Immunology Capabilities to Improve Safety and Efficacy and Speed Development

Penn

GENE THERAPY PROGRAM

Amicus Therapeutics®

Worldwide Rights to Penn's Next Generation Gene Therapy Technologies for the Majority of Lysosomal Disorders

Current Collaboration Includes Pompe, Fabry, CDD, Niemann-Pick Type C, Next Generation MPS IIIA, and MPS IIIB

Partnership Encompasses 12 Additional Rare Diseases, including Rett Syndrome, Angelman Syndrome, Myotonic Dystrophy and Select Other Muscular Dystrophies

Amicus to Invest $10M / Year for 5 Years for Research to Improve Safety, Efficacy and Manufacturability of Next Generation Vectors with Option to Extend

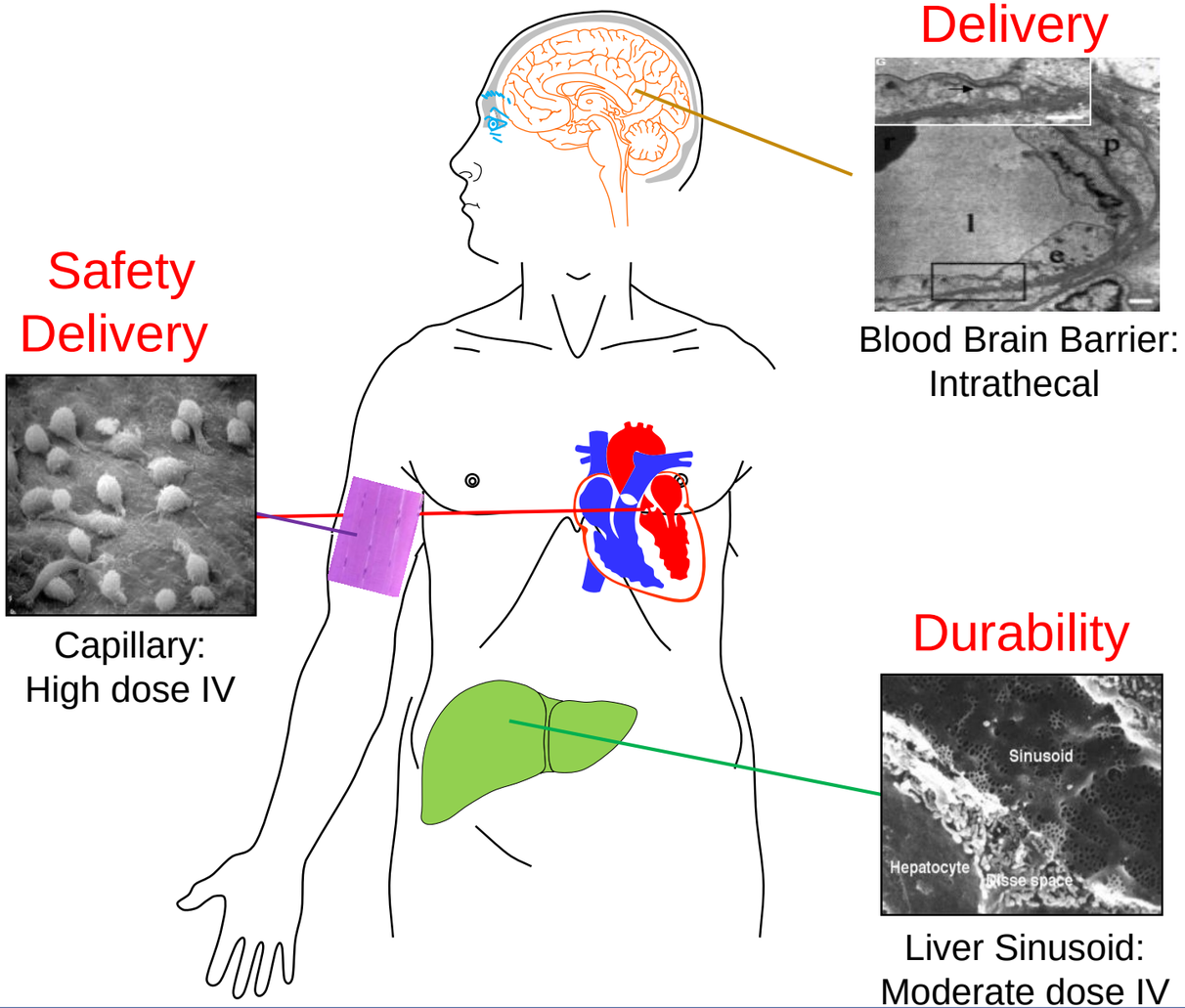
Discovery Program to Improve in vivo AAV Gene Therapy

James M. Wilson, MD, PhD
Rose H. Weiss Professor and Director, Orphan Disease Center
Professor of Medicine and Pediatrics
Director, Gene Therapy Program
Perelman School of Medicine at the University of Pennsylvania

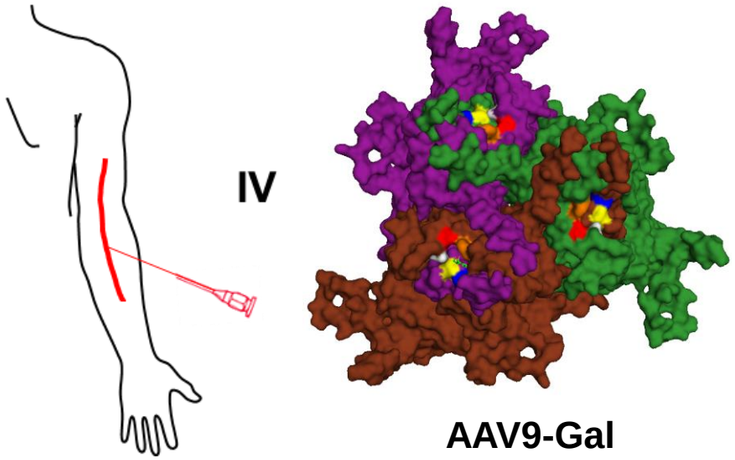
October 10, 2019

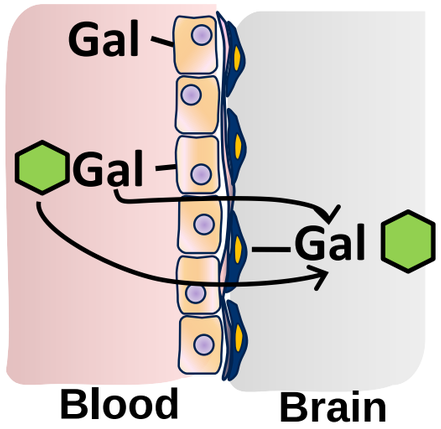
Passage Bio and Scout Bio: stock, grant and consulting

Strategies to Improve Gene Therapy for Targets Relevant to Amicus/Penn Programs: *It's not all about the capsid*

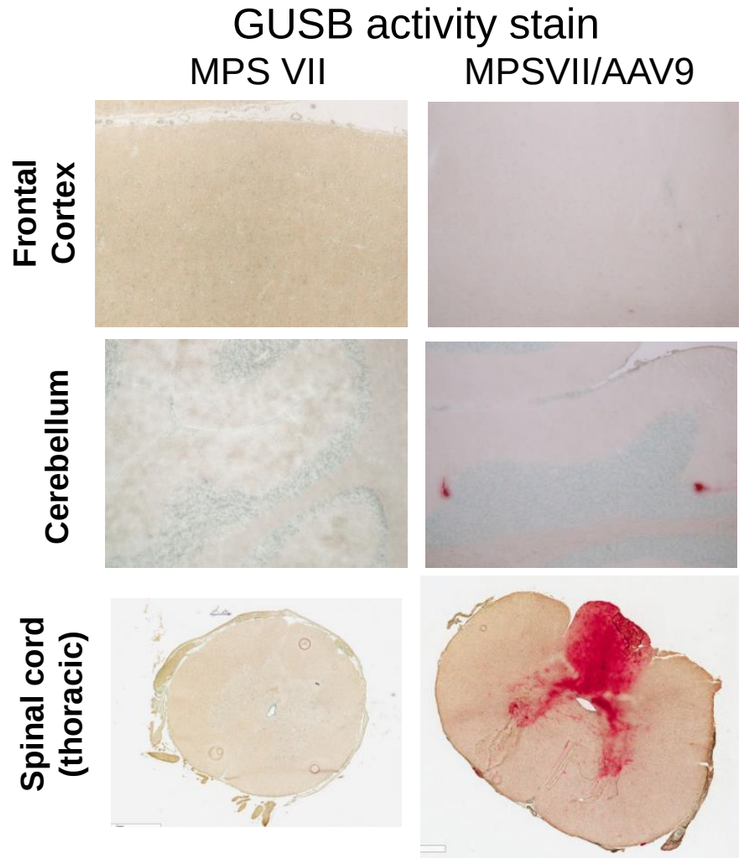


High Dose IV AAV9 Targets Motor Neurons of Spinal Cord but Not Brain in MPSVII Dogs





J Clin Invest (2011)

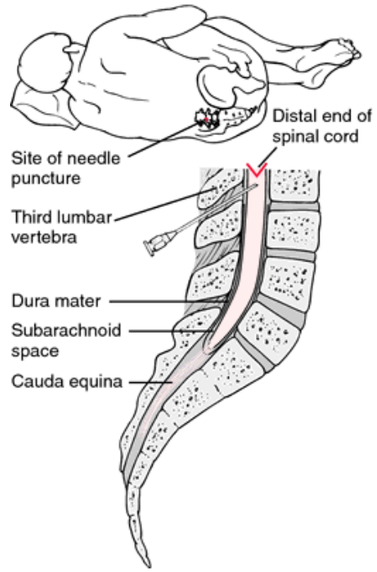


Histochemical stain for GUSB following high dose IV AAV9
In canine model of MPSVII

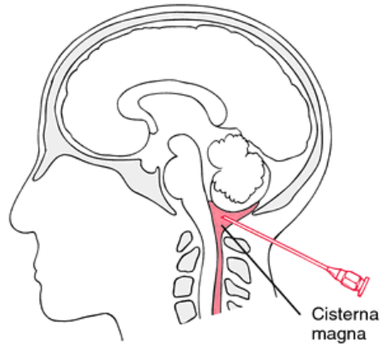
Mol Ther. 24:206 (2016)

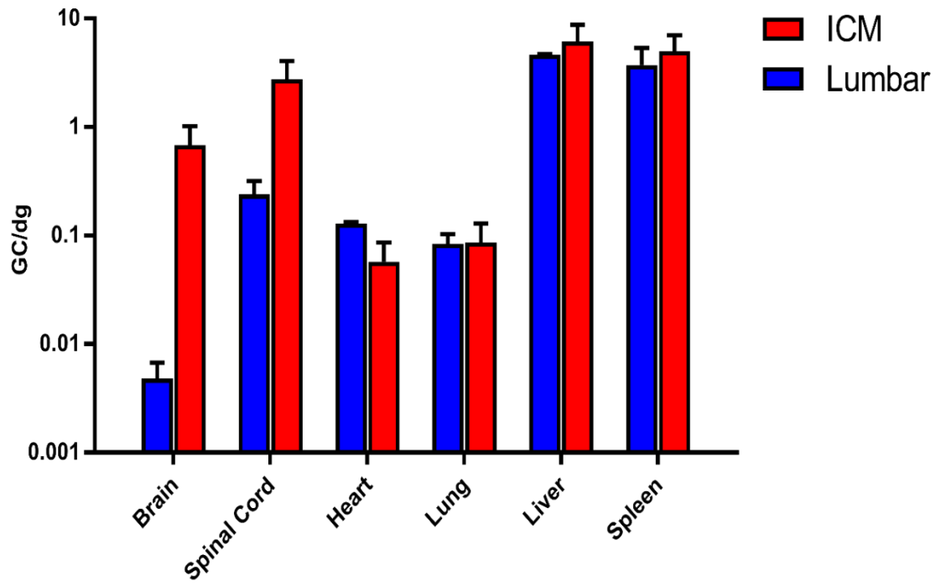
Broader Distribution of AAV9 Gene Transfer in CNS Following Injection into the Cisterna Magna (ICM)

Lumbar Puncture



Cisterna Magna

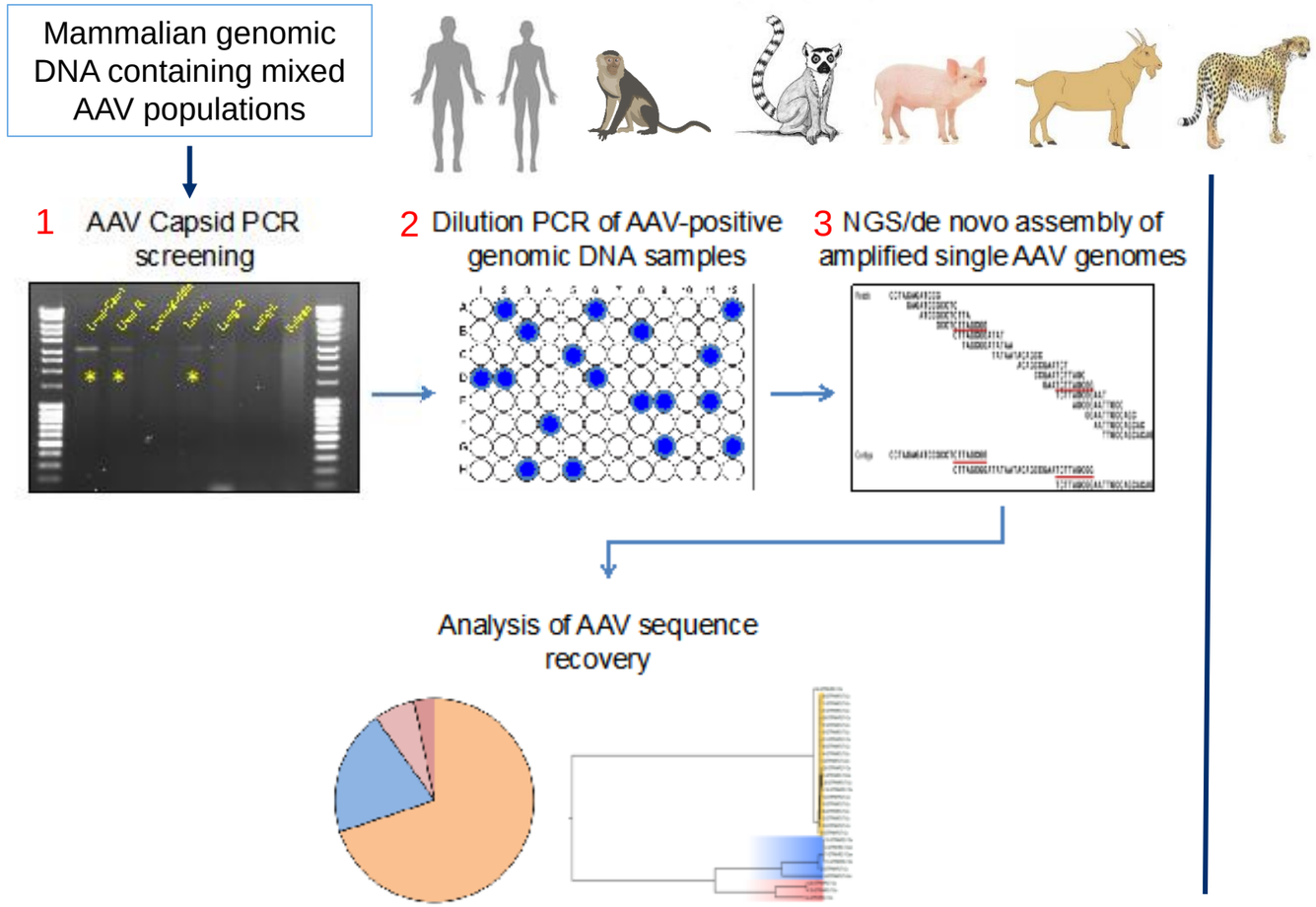




Adult cynomologus macaques were injected with 1 to 2xE11 GC/gm brain in 1ml via LP or ICM. Animals were necropsied 14 days later and brain tissue analyzed for vector GC per diploid genome. This is close to the maximal dose that can be delivered.

Mol Ther. Methods and Clinical Development 1:1 (2014)

Methods for AAV Natural Isolate Detection and Sequencing: Single Genome Amplification and Bioprospecting Techniques

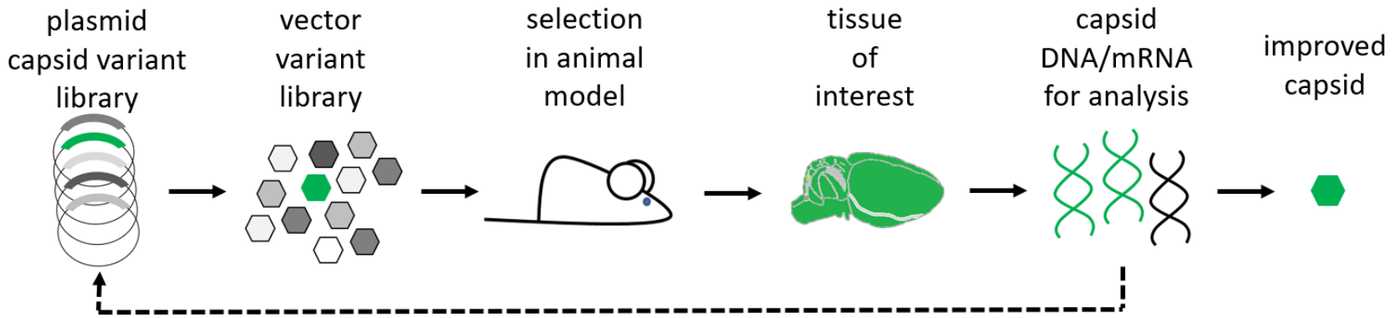


Primate and exotic species

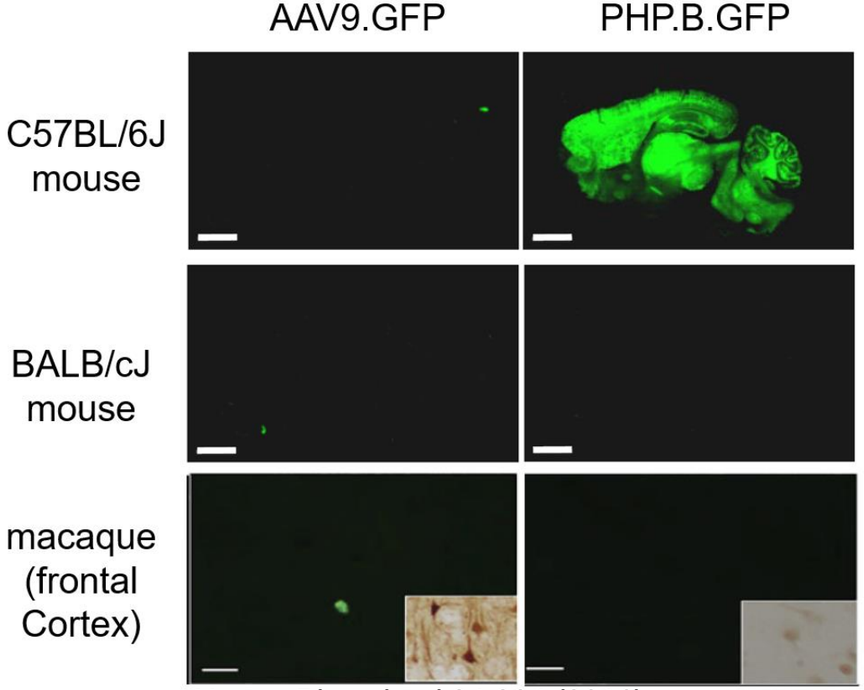
Other methods for Bioprospecting for novel AAV:

- Expansive high-throughput PCR using clade-specific and unbiased primer approaches
- Metagenomic dataset fishing for AAV-like sequences
  - Shotgun sequencing datasets from human and other mammals
- Database mining for AAV reads

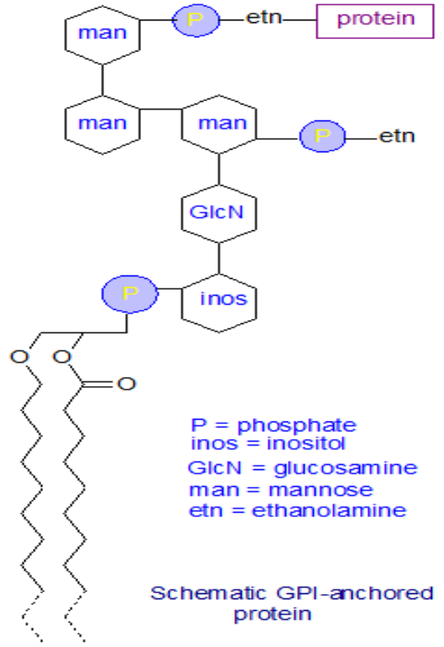
Directed Evolution for Improved Brain Transduction from IV Delivery



- AAV9 insert library
- directed evolution in C57BL/6 mice
- yielded PHP.B: AAV9 + 7aa insert (TLAVPFK)
- ~50-fold improvement in brain transduction



Nature Biotechnol 34:204 (2016)
Mol Ther. 26:664 (2018)



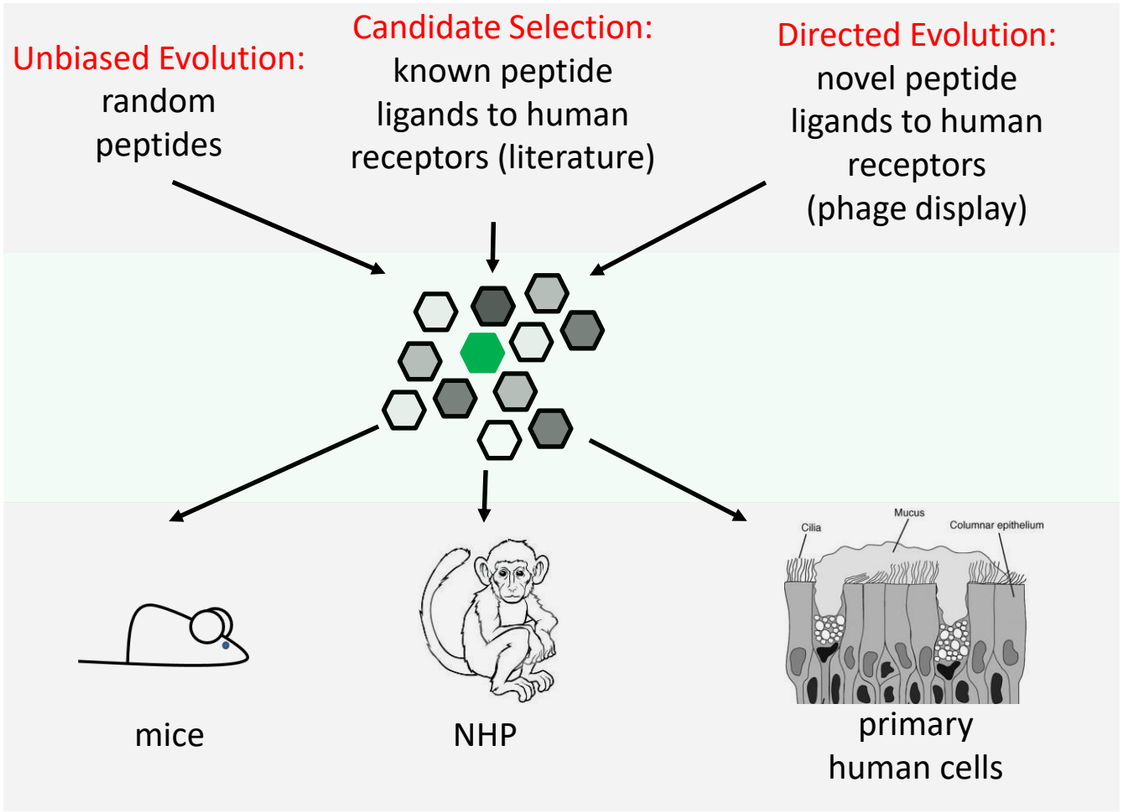
Mol Ther. 27:912 (2019)

- BBB permeability mapped to Ly6a
- GPI-anchored protein (Sca I)
- Located on brain endothelial cells
- Binds specifically to PHP.B

Penn University of Pennsylvania

Amicus Therapeutics

Work Streams for Identifying Novel Capsids with Improved CNS Delivery

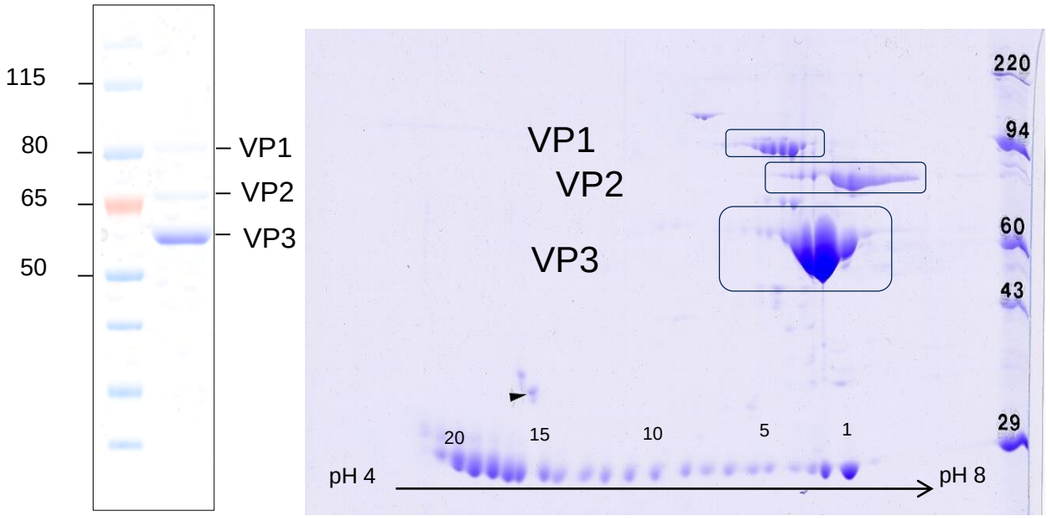


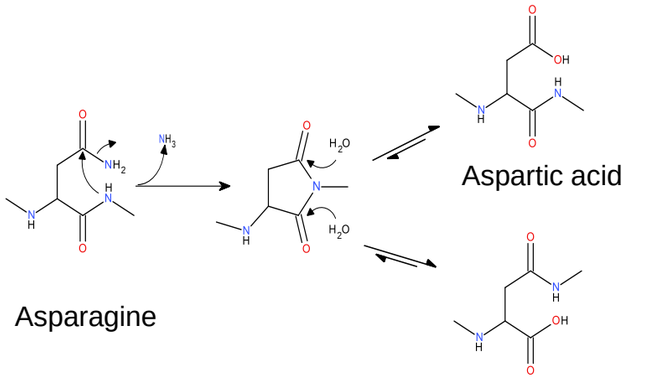
sources of capsid diversity

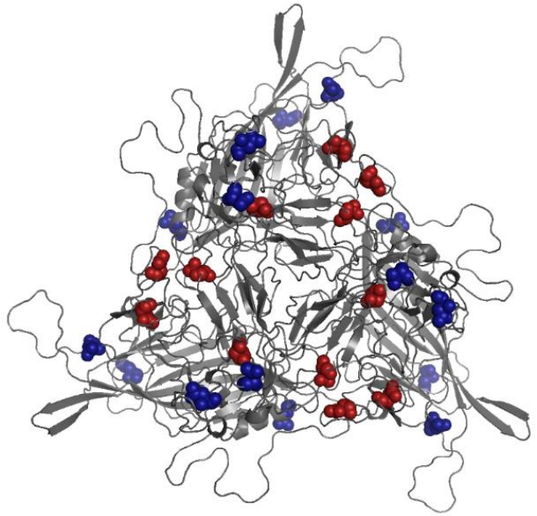
vector libraries

selection system

Manufacturing Process Improvements Can Improve Vector Potency: the Case for AAV Deamidation

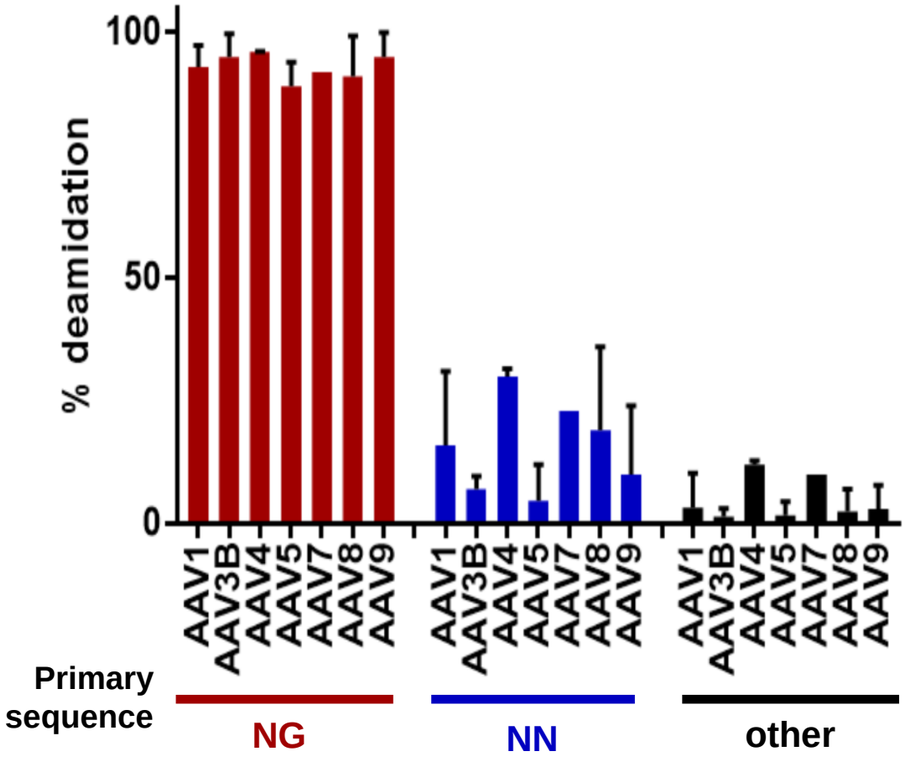


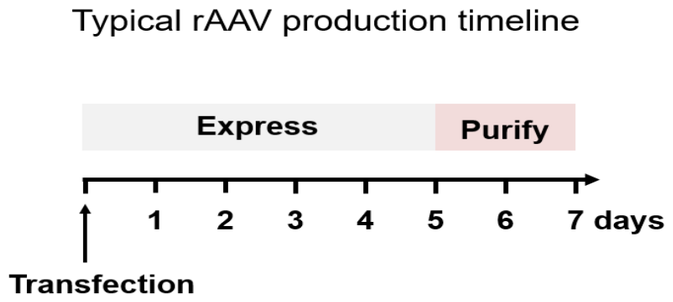


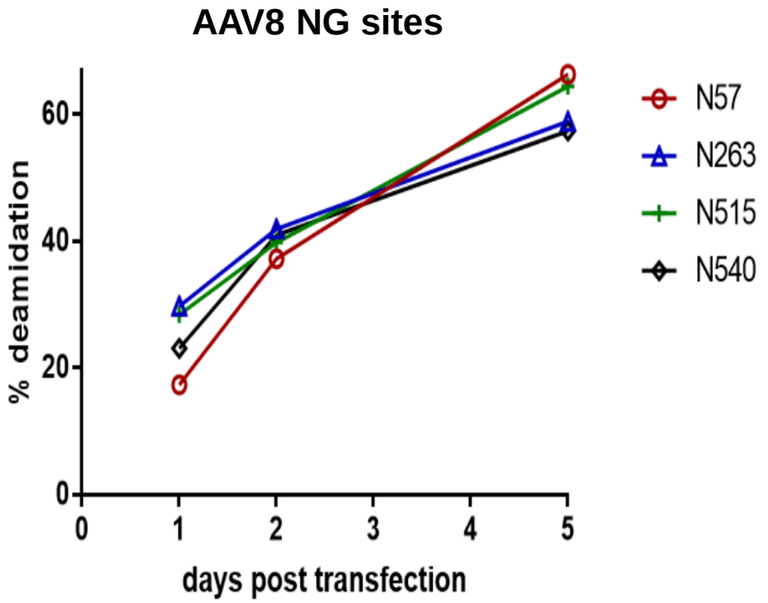


Molecular Therapy (2018)

Deamidation Occurs Across All Serotypes During Production of Vector

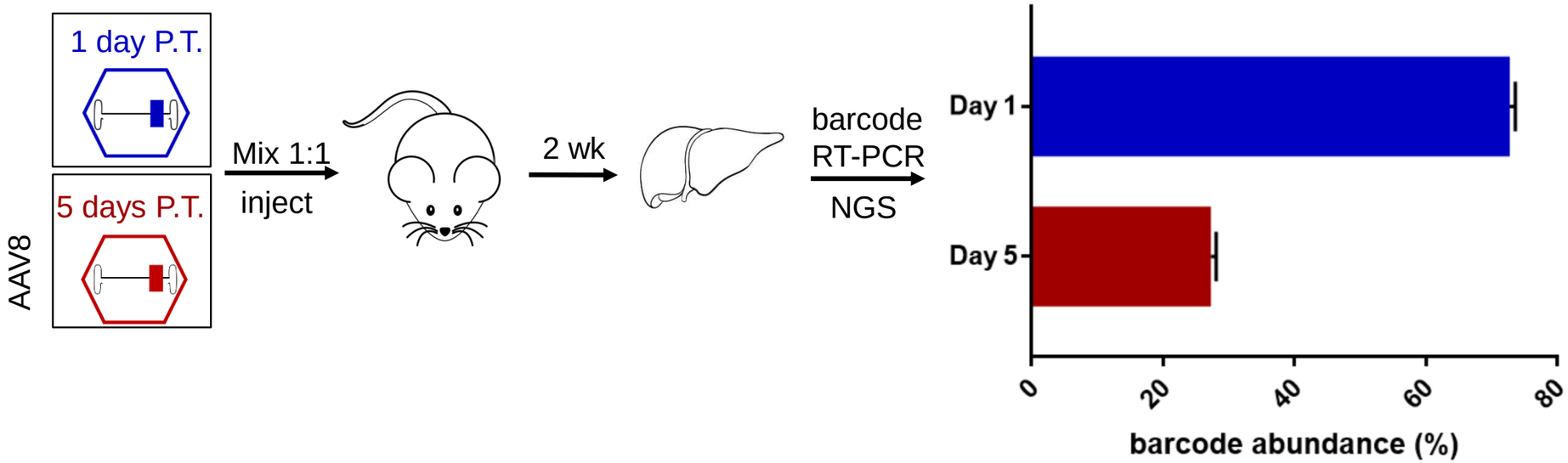






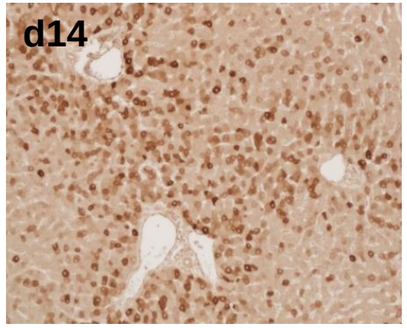
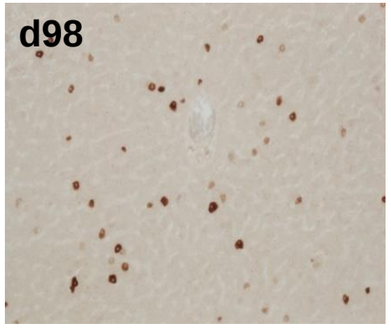
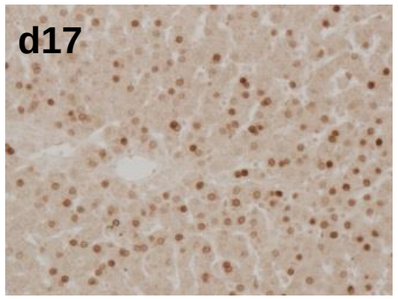
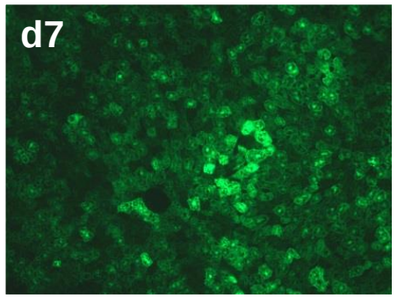
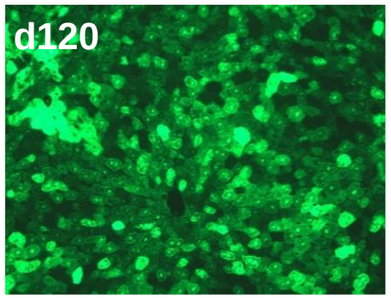
Mol Ther. 26:2848 (2018)

Rapid Deamidation of AAV8 Is Correlated with Potency Loss *in vivo*



AAV8 vectors harvested at day 1 or day 2 were produced using 2 sets of bar codes for each capsid harvest. Day 1 harvest had 20% NG deamidation while Day 2 harvest had 90% NG deamidation. Vectors were mixed and injected 1:1 IV into C57BL/6 mice. Liver harvested 2 weeks later were subjected to NGS to evaluate the abundance barcoded mRNAs which was shown to be approximately 3.5-fold higher for the Day 1 harvest indicating increased potency.

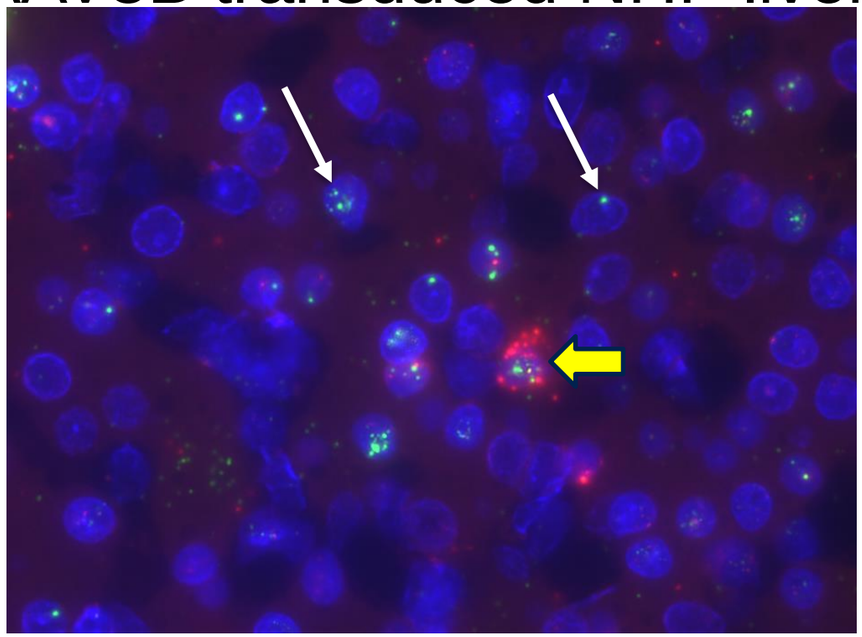
AAV-Mediated Transgene Expression Is Not Stable in Primate Liver

	Early phase	Late phase	LFTs
NHP self protein			**WNL**
NHP non-mammalian protein			**Elevated**
Mouse non-mammalian protein			**WNL**

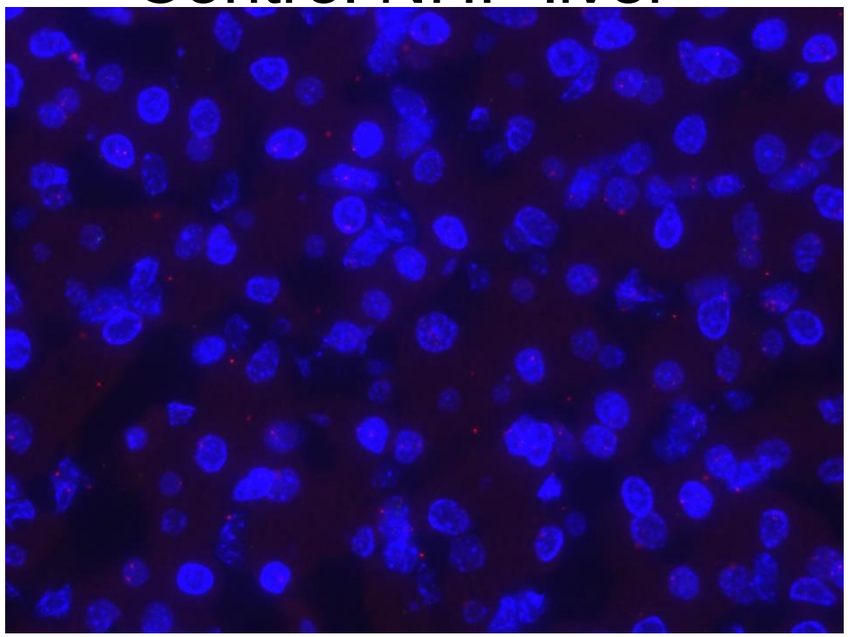
Unpublished Data

Extensive Vector DNA with Limited Expression in Nonhuman Primate Liver

AAV3B transduced NHP liver

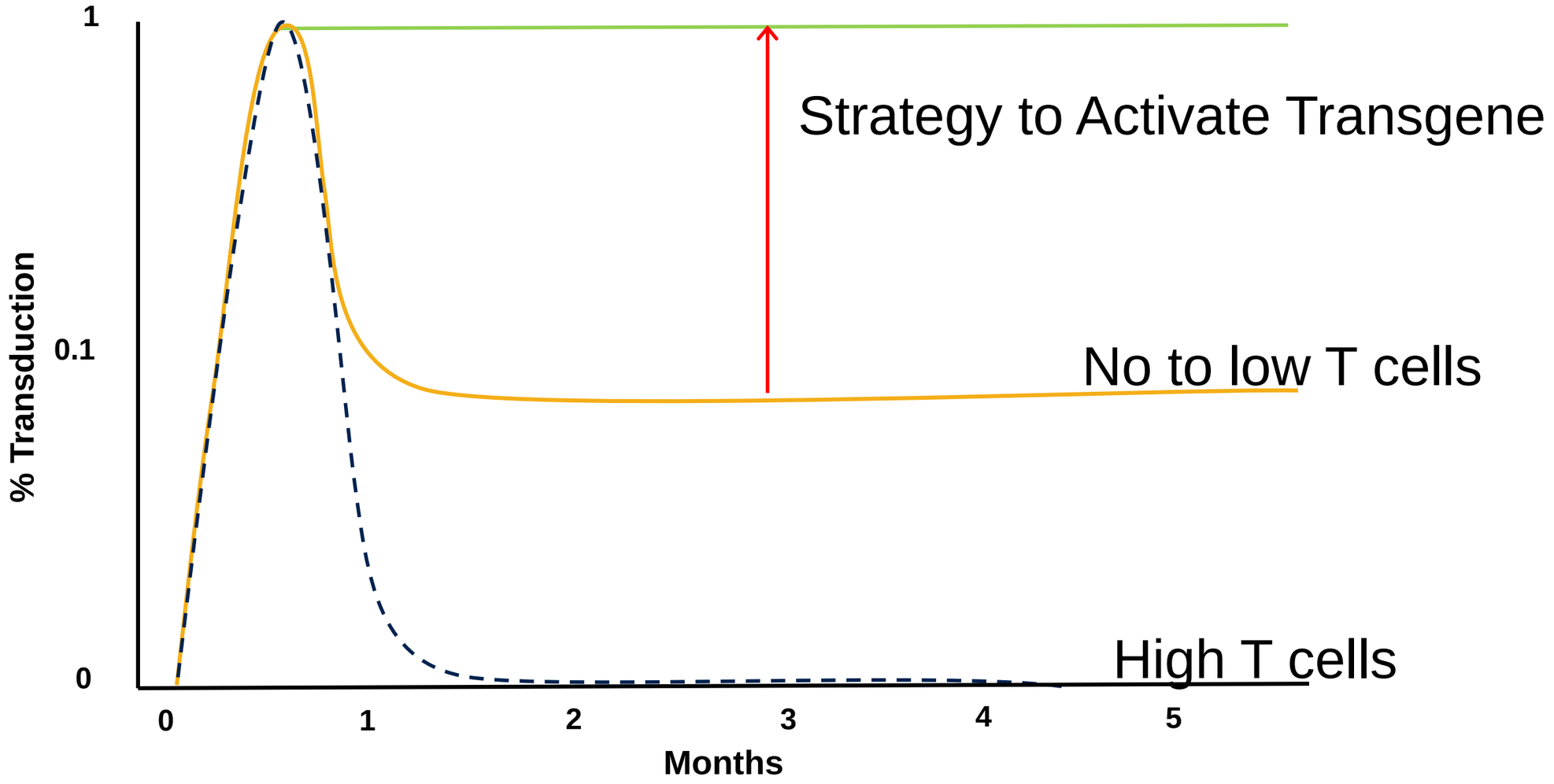


Control NHP liver



Rhesus macaques were injected with AAV3B vector at 3E12 GC/kg and tissue was harvested at week 35 for *in situ* hybridization analysis. Left panel: tissues were analyzed with probes to the mRNA (red) and to DNA (green) that are complementary to the transgene. White arrows indicate punctate signals in nuclei that are specific to DNA while yellow arrowhead points out a cell with extensive cytoplasmic mRNA staining. Right panel: similar in situ Hybridization from liver tissue of an animal that received AAV3B vector at same dose but expressing a different Transgene with a notable absence of staining.

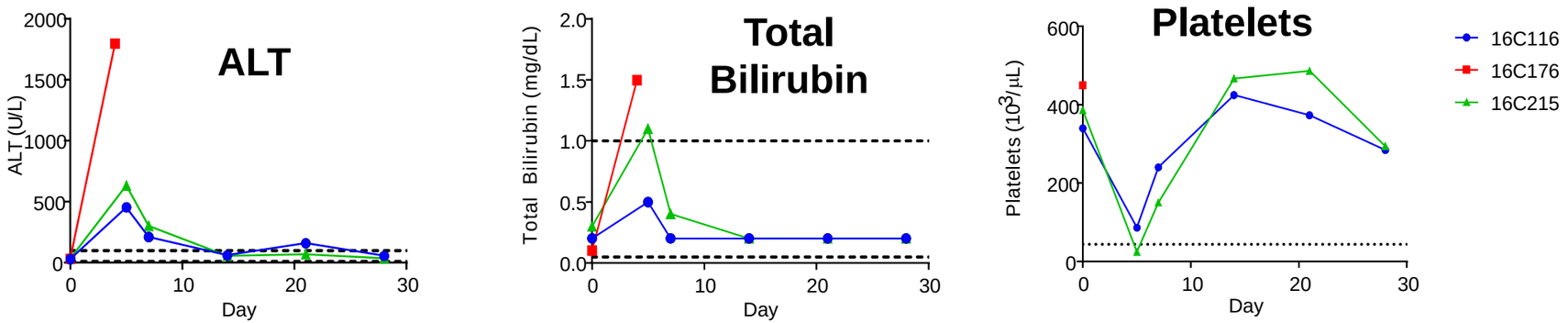
Model for Efficiency and Durability of AAV Liver Gene Therapy



Penn
University of Pennsylvania

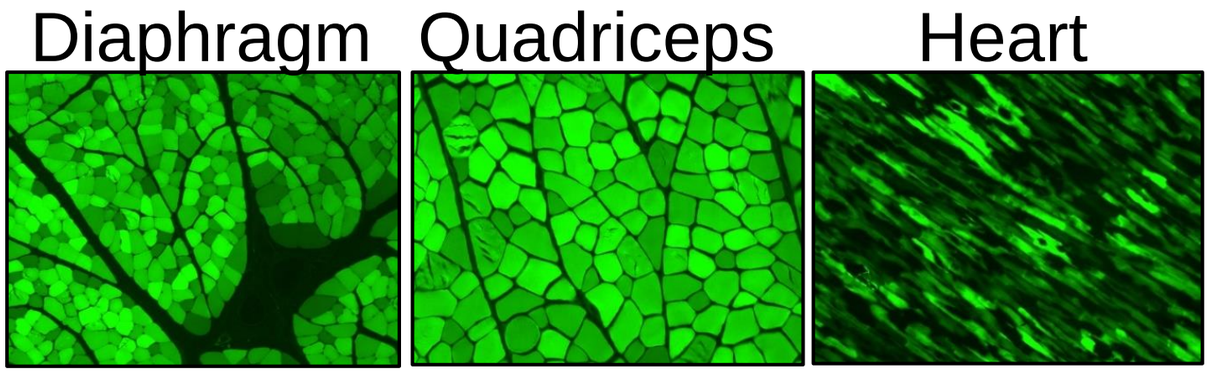
Amicus Therapeutics

Gene Transfer and Toxicity of Systemic High Dose AAV9 in Rhesus Macaques



Rhesus macaques 12-14 months of age dosed with IV AAVhu68.CB7.hSMN at 2xE14 GC/kg. 16C176 was euthanized on Day 4. Dotted lines indicate laboratory reference range.

Human Gene Ther. 29:285 (2018)

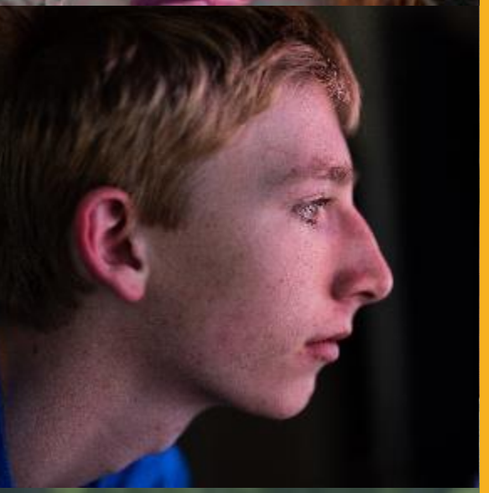


Rhesus macaques were dosed with AAV9.CB7.GFP at 7.5E13 GC/kg and necropsied at 21 days for analysis of GFP expression.

Mol Ther. 26:664 (2018)

Acute Systemic Toxicity to High Dose IV AAV: Mechanisms, Factors and Remedies

- Data
  - Occurs in NHPs and humans
  - Occurs at doses >7.5e13 GC/kg
  - Non-linear relationship between dose and gene transfer/acute toxicity
  - Prodrome is low platelets and high transaminases
- Mechanism(s) – unknown
  - Systemic Inflammation – e.g., CAR-T and Adenovirus
  - Activation of Complement
  - Injury to endothelial cells
  - Direct affect on platelets
- Factors
  - Capsid
  - Residuals
  - Method of purification
  - Age of recipient
  - Co-morbid conditions
- Remedies
  - Decrease dose; improved delivery
  - Modify biodistribution
  - Pharmacology to prevent inciting event or dampen host responses



Batten Disease Gene Therapy Franchise

Jill Weimer, ***SVP of Discovery Research & Gene Therapy Science***

Jeff Castelli, Ph.D., ***Chief Portfolio Officer and Head of Gene Therapy***

2019 Analyst Day | October 10, 2019 | New York, NY

Validated Gene Therapy Platform for CNS

The Amicus Batten programs leverage AAV technologies and platforms utilized in the neuromuscular space at Nationwide Children's Hospital/Sanford and have robust preclinical and now clinical proof of concept

Clinically validated AAV gene therapy approach at NCH and Sanford

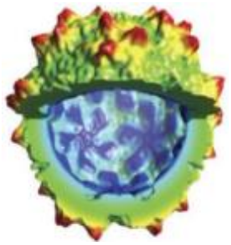
- Nationwide Children's Hospital Center for Gene Therapy (NCH)
- Intrathecal delivery with robust expression throughout CNS

Preclinical safety and efficacy studies replicated across multiple diseases at NCH

- SMA, CLN6, CLN3, CLN8

Amicus applying platform to multiple types of Batten disease and other Neurologic LSDs

- Two clinical programs in CLN6 and CLN3 Batten disease show initial safety in 15 patients; promising efficacy results in first 8 patients in CLN6
- Active preclinical programs in CLN8 and CLN1 Batten disease with other neurologic LSDs in earlier preclinical development



NATIONWIDE CHILDREN'S

SANFORD RESEARCH

Foust, Kaspar et al, 2009

Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

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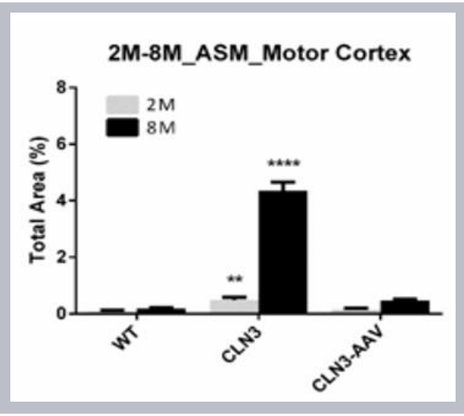
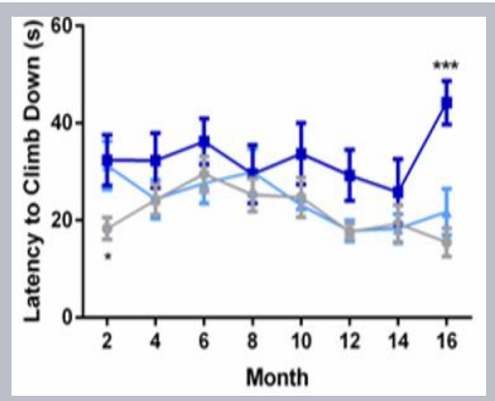
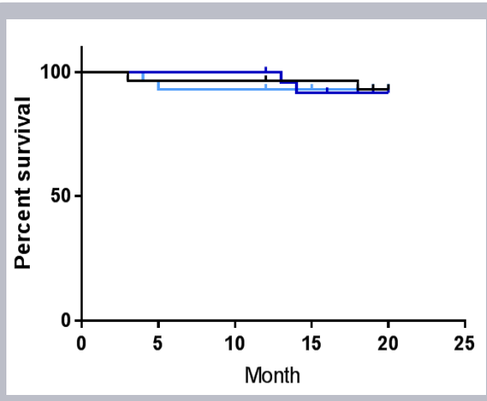
CLN3 Batten Disease: Preclinical and Clinical Summary

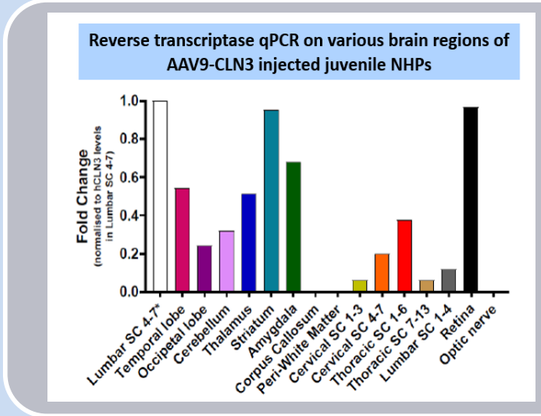
Amicus' second clinical stage gene therapy in CLN3 Batten disease has successfully completed dosing in three children in Cohort 1 (low dose) with dosing of additional Cohort 2 (high dose) patients in 2H 2019

Preclinical Data in KO Mice









Reduction of storage material in mouse model	Improvement of motor function and cognitive behavior in mouse model	Comparable survival in mouse model	Widespread gene expression in brain of NHPs

Source: Data on file

The Kahn Family – Life with CLN3 Batten Disease

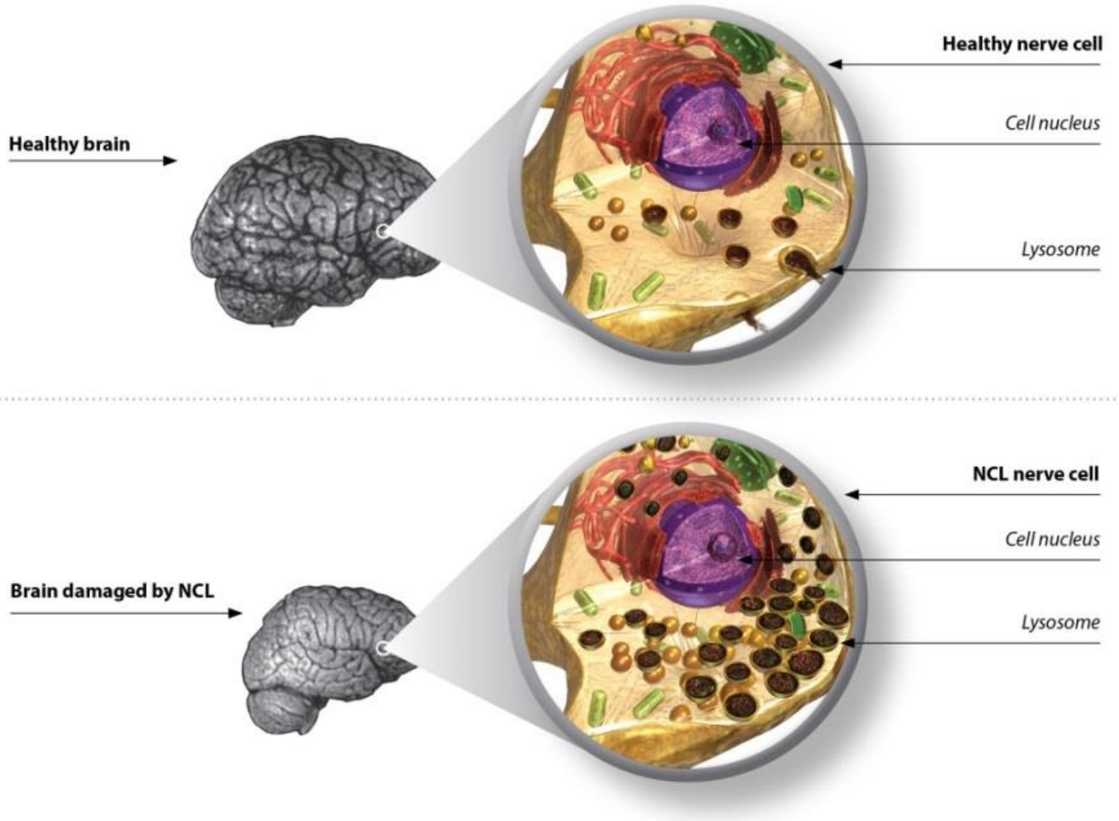
The Kahn Family – Life with CLN3 Batten Disease

Video:

CLN6 Batten Disease Overview

Batten disease is a devastating early childhood disease that is 100% fatal in children. CLN6 is a neurologic disease that rapidly robs children of their ability to walk, speak, think, see, and often ends in death during childhood.

- Mutation in CLN6 gene leads to lysosomal dysfunction
- Usually presents at 2-3 years of age after typical childhood development
- Rapidly robs children of their ability to walk, speak, think, and see
- No approved therapy and urgent need for treatment
- Early intervention is critical
- Estimated population is ~1,000 globally



Source: Batten Disease Fact Sheet, NINDS, Publication date June 2018.

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Hamburg Motor and Language Scale

Following symptom onset, natural history indicates rapid degradation of motor and language ability, on the Hamburg scale, with each point decline representing significant impairment

Hamburg Motor & Language Scale

Motor Function	Language Function
3 Normal	3 Normal
2 Clumsy, falls	2 Abnormal
1 Non-walking	1 Minimal
0 Immobile	0 Unintelligible or no vocalization

In each domain, the rating is structured so that a score of:

3 = normal condition

2 = slight or just noticeable abnormality

1 = severe abnormality

0 = complete loss of function

Source: Wyrwich et al. 2018. Journal of Inborn Errors of Metabolism and Screening

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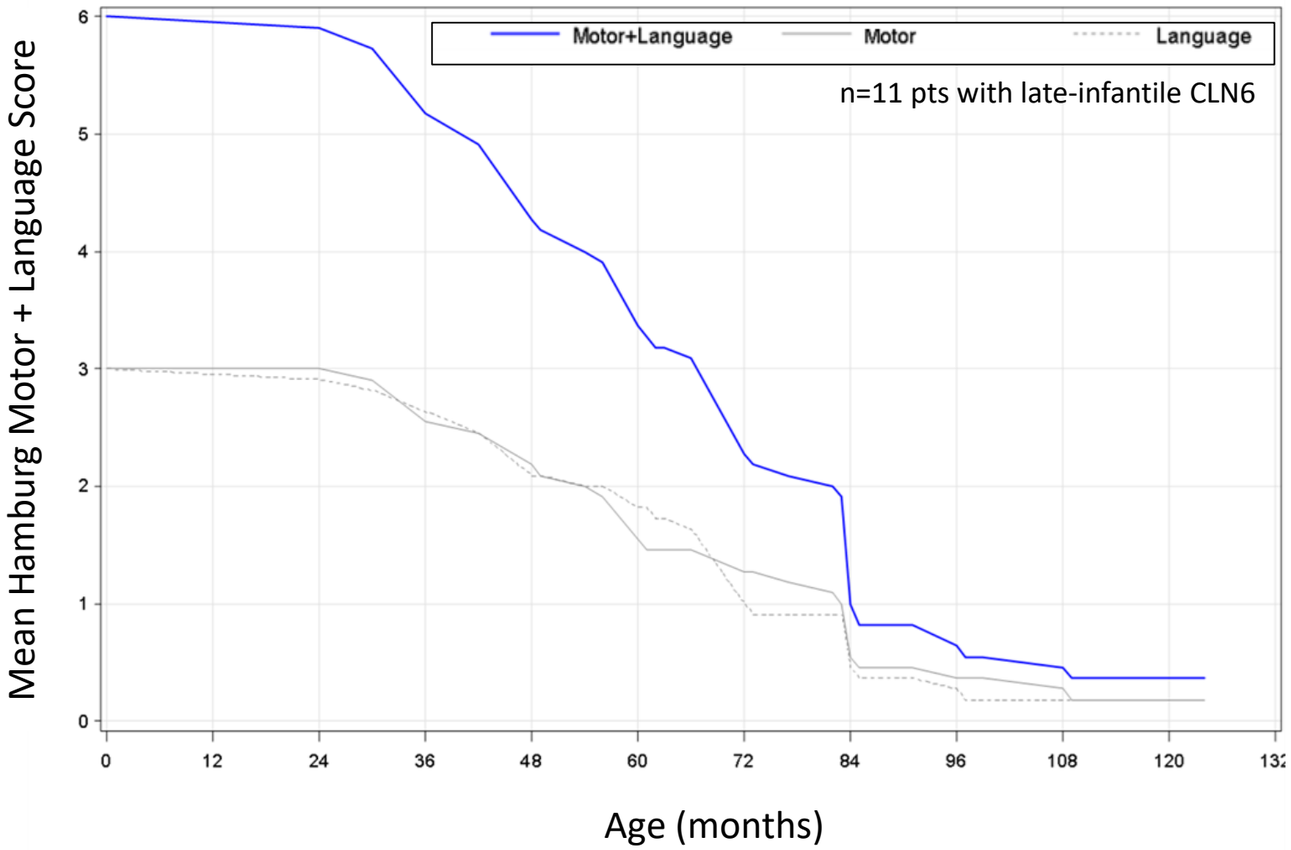
CLN6 Natural History

CLN6 natural history shows a progressive decline of approximately one point per year in the Hamburg score from age two onwards with similar decline in motor and language



Source: Data on file: Ongoing Natural History study conducted by Nationwide Children's Hospital and Dr. Emily de los Reyes

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Clinical Study Design

Safety and efficacy of a single administration of intrathecally delivered AAV-CLN6 gene therapy evaluated for a number of key parameters including Hamburg Motor + Language score

Key Eligibility Criteria

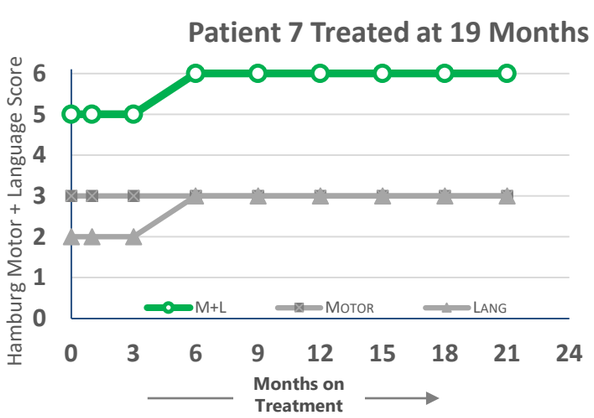
- Diagnosis of CLN6 determined by genotyping
- Hamburg motor and language score ≥3
- Age ≥1 year

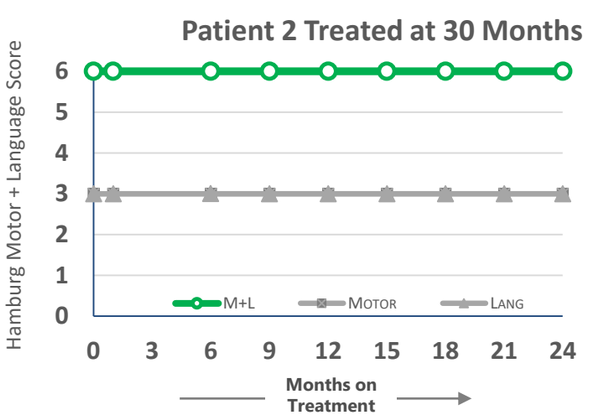
Efficacy Evaluations

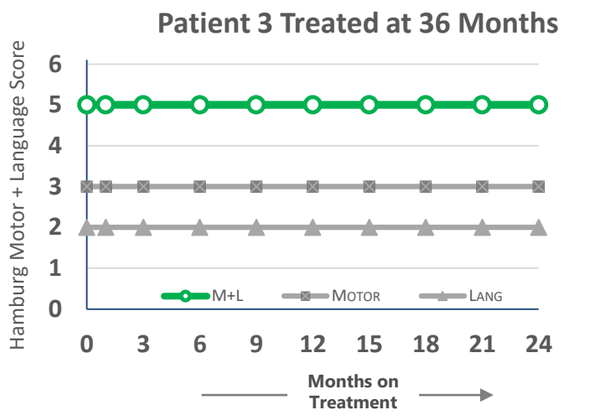
- Hamburg scale
- Additional measures include: UBDRS, Cognitive and Language Ability, Vision, QOL, Ophthalmologic Assessments, Brain MRI

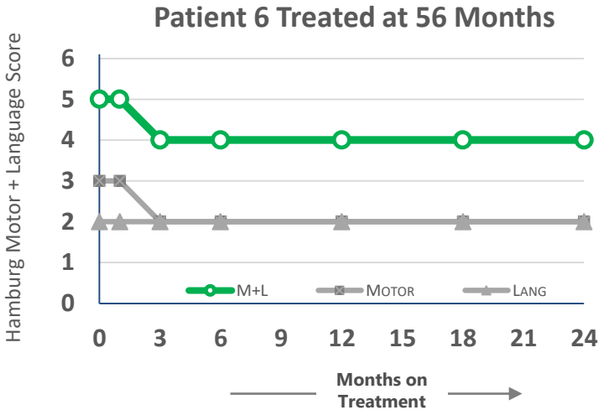
Clinical Efficacy: Combined and Individual Hamburg Scores (n=8)

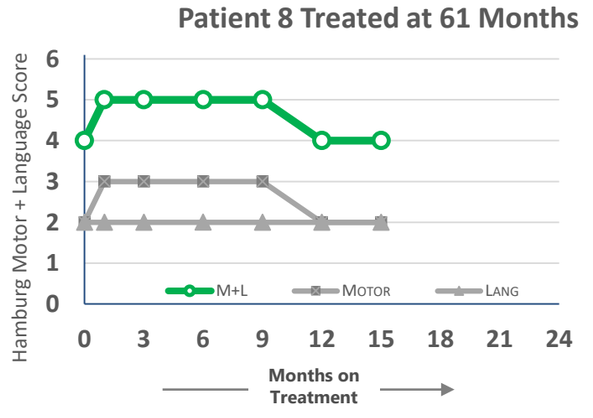
CLN6 gene therapy halts or substantially slows progression of disease with a positive impact on motor and language function in 7 out of 8 patients

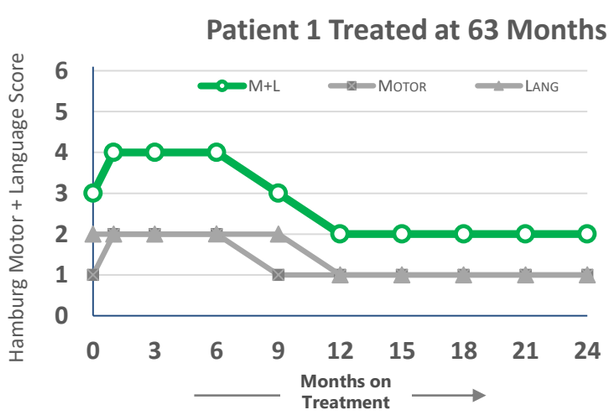


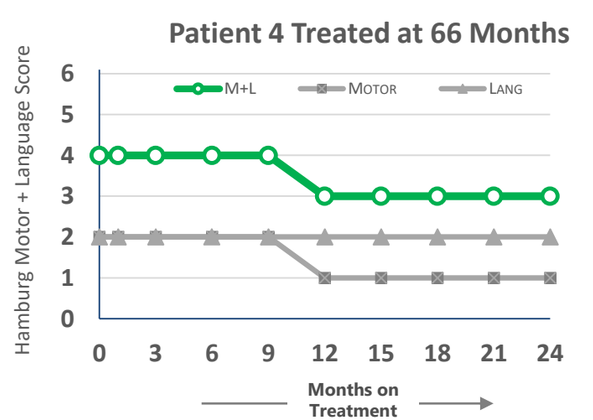


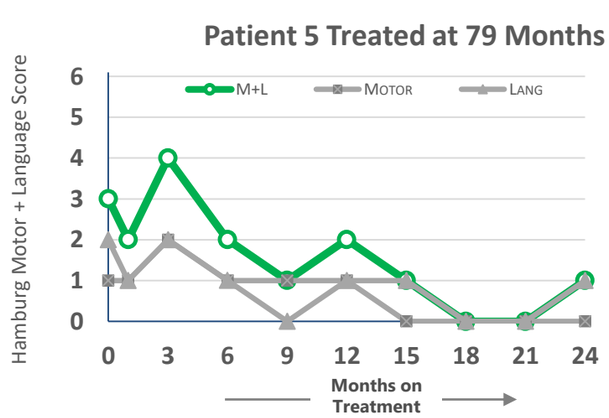












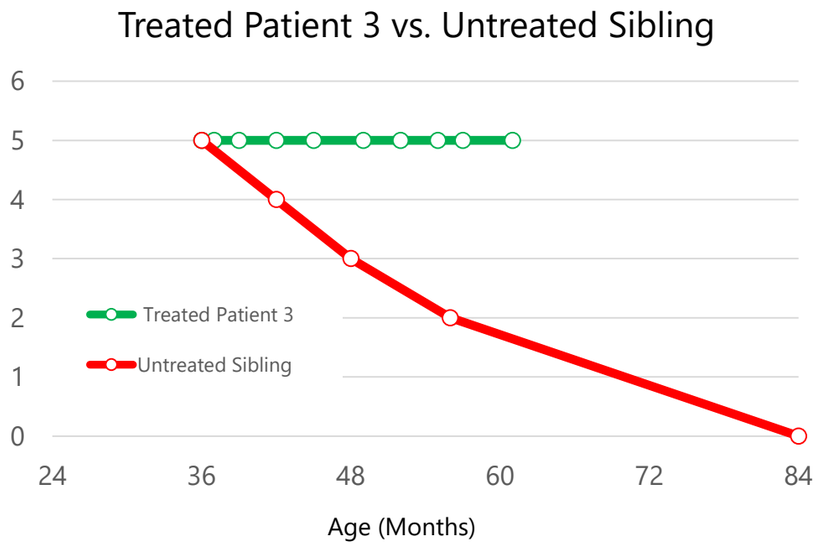
Separate Motor and Language Scores are Consistent with the Respective Combined Score.

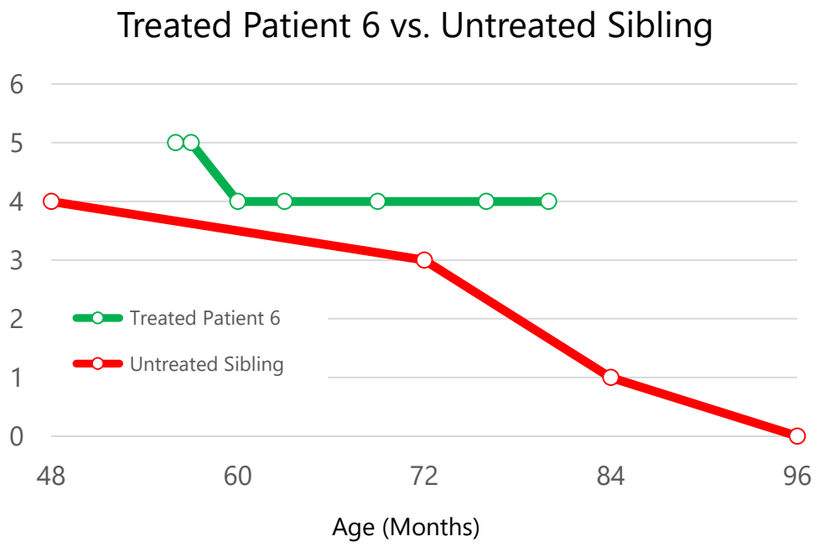
Source: Data on file

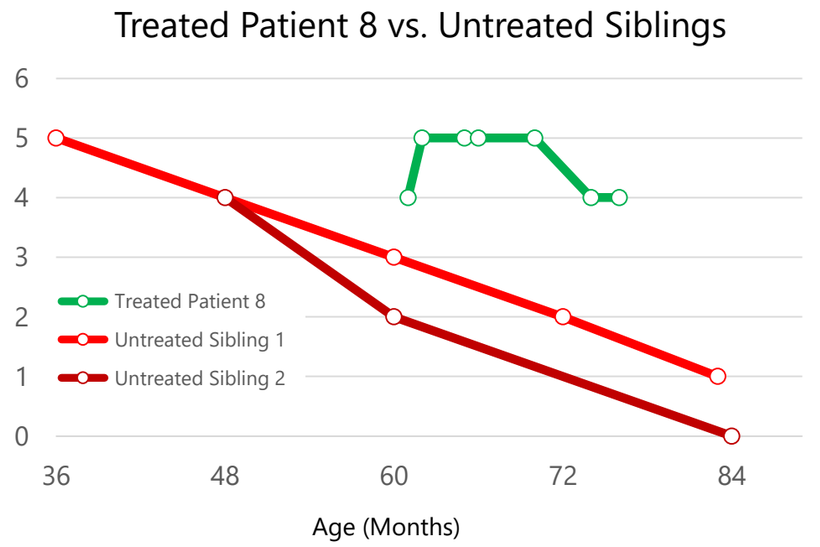
CLN6 Clinical Efficacy Data: Sibling Comparisons (Natural History)

Treated patients demonstrated stabilization relative to untreated siblings in the natural history data set who experienced substantial declines in motor and language ability

Treated AAV-CLN6 Patients vs Natural History Sibling with CLN6 (Hamburg Score: Motor + Language over time)



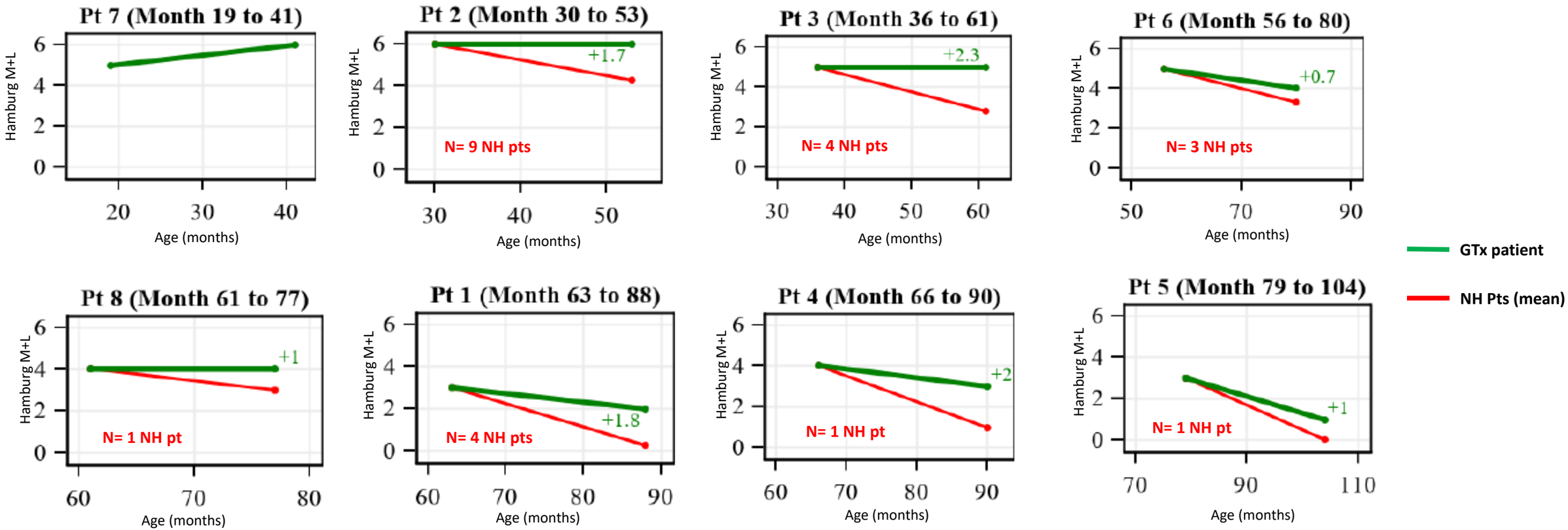




Source: Data on file

CLN6 Clinical Efficacy Data: Natural History Matched Comparisons

New analysis of treated patients demonstrate improvement compared to natural history patients matched for age and baseline Hamburg M+L score*



*Matched for age and exact baseline Hamburg score. No current match (for age and exact M+L score at baseline) for youngest patient (pt. 7)

Source: Data on file

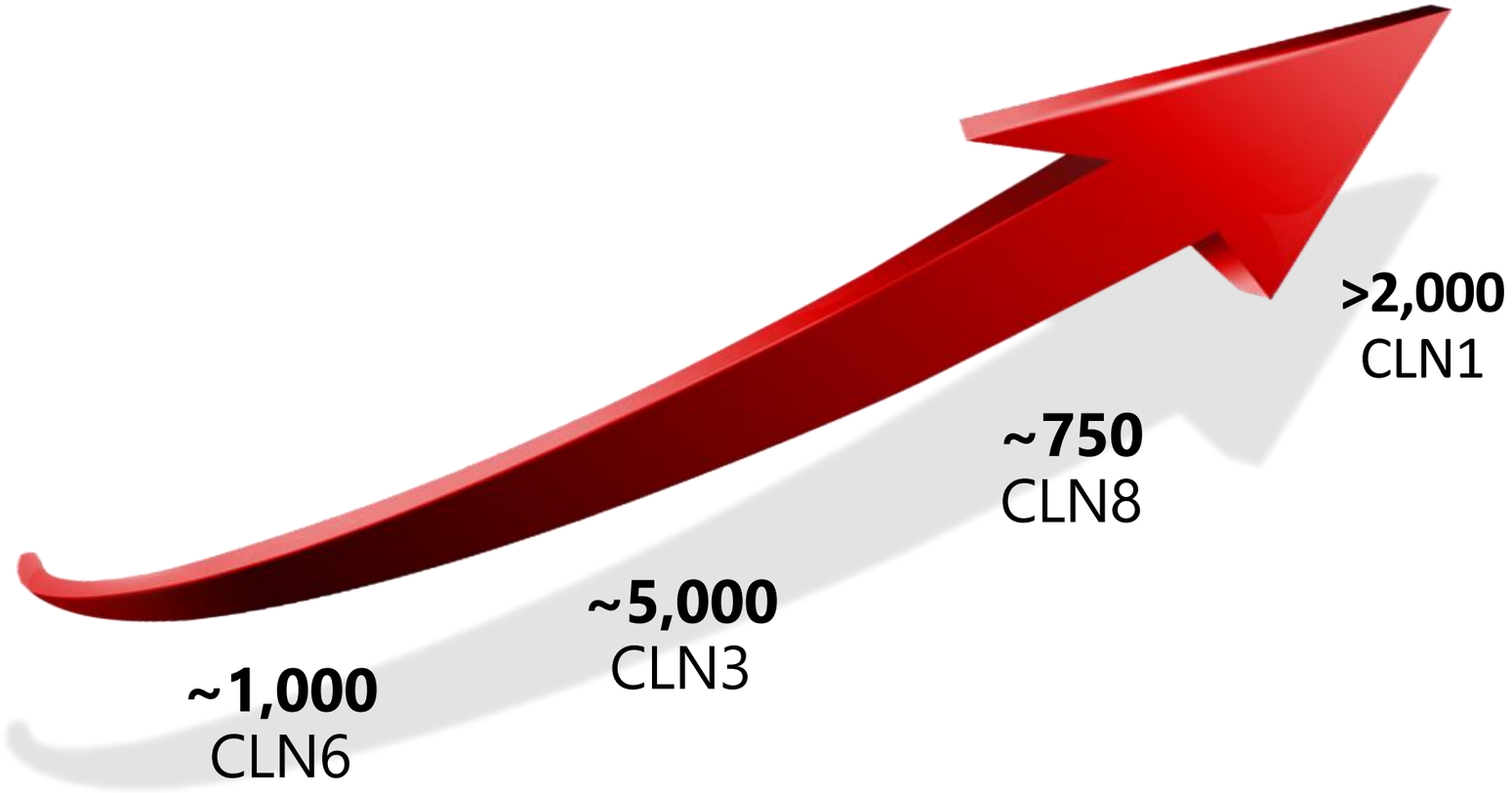
5 Key Takeaways for AAV-CLN6 Gene Therapy

Interim safety and efficacy data demonstrate the potential for AAV-CLN6 gene therapy to stabilize progression of a devastating disease

- **Meaningful impact on motor and language function** in children with a fatal neurologic disease that destroys brain function
- **Evidence of disease stabilization** in seven out of the eight children following AAV-CLN6 gene transfer
- **Natural history cohort shows progressive loss of language and motor function** in untreated patients
- **Sibling comparisons (in-study and natural history) and matched natural history comparisons provide further support** for AAV-CLN6 gene therapy and early intervention
- **Favorable safety profile** with intrathecal administration of AAV in all study participants

Batten Disease Franchise

CLN6 results validate the broad potential of the intrathecal AAV platform to build a valuable and significant franchise to save thousands of children suffering from multiple types of Batten diseases with potential for $1B+ in recurring peak revenue



*Estimated addressable U.S., EU, Japan, and other major, reimbursable markets based on published incidence and prevalence

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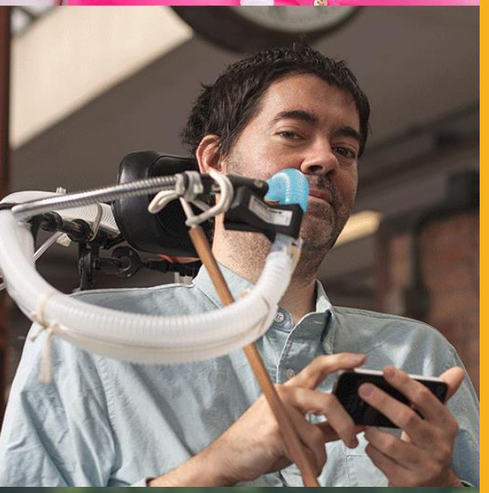
CLN6 Disease Overview

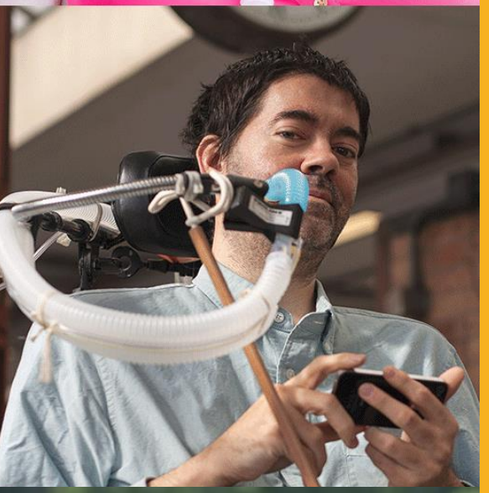
The perspectives of people living with CLN6 demonstrate the urgent need for treatment

Video:



Q&A

2019 Analyst Day | October 10, 2019 | New York, NY



Closing Remarks

John F. Crowley

Chairman and Chief Executive Officer

2019 Analyst Day | October 10, 2019 | New York, NY

Thank You

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