Amicus Therapeutics®

Amicus 2018 Analyst Day

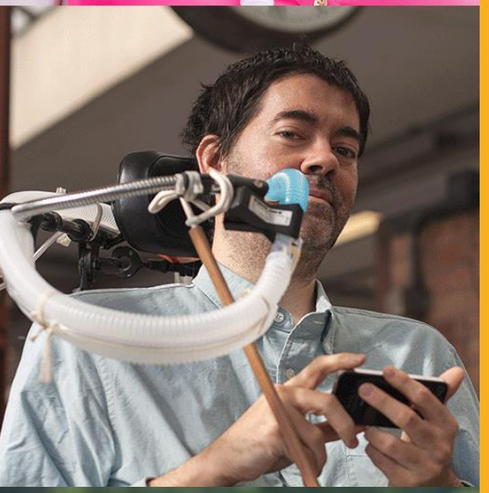
October 11, 2018 | New York, NY

Forward Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the collaboration with the University of Pennsylvania, the recent acquisition of Celenex, preclinical and clinical data, regulatory strategy and the development of potential gene therapy product candidates. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this presentation may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, the benefits of this collaboration may never be realized, 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 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; the potential that we will need additional funding to complete all of our studies and manufacturing and the potential that certain individuals may not continue to support the development of product candidates. . 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, 2017 as well as our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018 filed August 7, 2018 with the Securities and Exchange Commission. 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.

Agenda

8:30 a.m. – 8:35 a.m.	**WELCOME & INTRODUCTIONS**	Sara Pellegrino, *Vice President, Investor Relations and Corporate Communications*
8:35 a.m. – 8:50 a.m.	**VISION, MISSION AND STRATEGY**	John F. Crowley, *Chairman and Chief Executive Officer*
8:50 a.m. – 9:50 a.m.	**AAV GENE THERAPY PLATFORM FOR NEUROLOGIC LSDs**	**Strategic Fit for Amicus Entry into Gene Therapy** John F. Crowley, *Chairman and Chief Executive Officer*
		AAV Platform Overview and Proof of Concept Data Kathrin Meyer, Ph.D., *Principal Investigator, Nationwide Children's Hospital Center for Gene Therapy*
		CLN6 Clinical Summary Jay Barth, M.D., Chief Medical Officer
		Q&A and Break
10:00 a.m. – 10:30 a.m.	**NEW PLATFORMS FOR GENE THERAPY IN RARE METABOLIC DISORDERS**	**Amicus-Penn Collaboration and Perspectives on Gene Therapy Approaches** James M. Wilson, M.D., Ph.D., *Professor of Medicine and Pediatrics at the Perelman School of Medicine*
		Applying Amicus Expertise to Optimize Gene Therapy Hung Do, Ph.D., *Chief Science Officer*
		Q&A
10:30 a.m. – 11:40 a.m.	**AT-GAA – POTENTIAL TO SHIFT TREATMENT PARADIGM FOR POMPE DISEASE**	**AT-GAA Phase 1/2 18-Month Data** Mark Roberts, M.D., *Dept. of Neurology, Salford Royal NHS Foundation Trust*
		Patient Advocacy and Personal Perspectives on Pompe Disease • Jayne Gershkowitz, *Chief Patient Advocate* • George Fox, *Dad and Caregiver to son, Phoenix* • Mike Stanzione, *Courageously living with late-onset Pompe*
		AT-GAA Development Strategy John F. Crowley, *Chairman and Chief Executive Officer*
11:40 a.m. – 12:00 p.m.	**GALAFOLD ORAL PRECISION MEDICINE FOR FABRY DISEASE**	**Global Launch Overview** Bradley Campbell, *President and Chief Operating Officer* Detlef Wolff, *Senior Vice President, Head of International*
12:00 p.m. – 12:10 p.m.	**CLOSING REMARKS**	John F. Crowley, *Chairman and Chief Executive Officer*
12:10 p.m. – 12:30 p.m.	**Q&A SESSION**	



Vision, Mission & Strategy

John F. Crowley

2018 Analyst Day | October 11, 2018 | New York, NY

Amicus Today

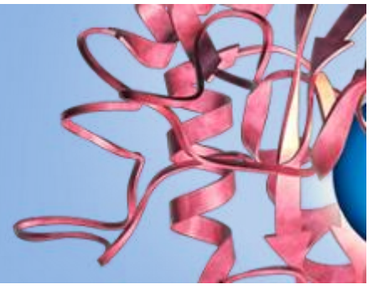
Galafold™ (migalastat)

First Oral Precision Medicine for Fabry Disease

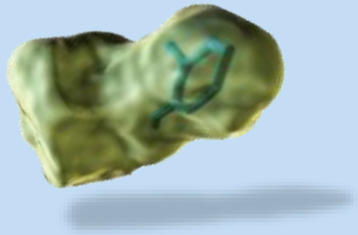
500+ EMPLOYEES globally

PIPELINE
of 15 products for rare metabolic diseases

BIOLOGICS PLATFORM
Protein Engineering & Glycobiology

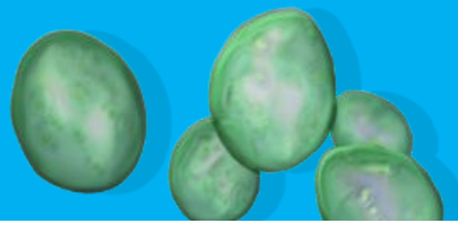


AT-GAA*
Investigational Therapy for Pompe in Phase 3



GLOBAL FOOTPRINT
in 27 countries

Leading Expertise in **Lysosomal Storage Disorders**



approx. **$550M** Cash (6/30/18)

Gene Therapy Platforms

* AT-GAA, also known as ATB200/AT2221

Amicus Therapeutics

Robust Rare Disease 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					
Other Gene Therapy Programs						
CLN6 Batten Disease	NCH					
CLN3 Batten Disease	NCH					
CLN8 Batten Disease	NCH					
Neimann-Pick C	NCH					
Wolman Disease	NCH					
Tay Sachs	NCH					
Multiple Other CNS LSDs	NCH					
CDKL5 Gene Therapy / ERT	PENN					
Other	PENN					

Advancing One of the Most Robust Portfolios in Rare Diseases in All of Biotechnology

2018 Key Strategic Priorities

On Track to Achieve All FIVE Key Strategic 2018 Priorities Outlined in January

1. **Double Galafold (migalastat) revenue to $80-$90M**
2. ✓ **Secure approvals for migalastat in Japan and the U.S.**
3. ✓ **Achieve clinical, manufacturing and regulatory milestones to advance AT-GAA toward global regulatory submissions and approvals**
4. ✓ **Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019**
5. ✓ **Maintain financial strength**

What's New at Amicus Analyst Day

10 Important New Data Points and Updates to Share Today

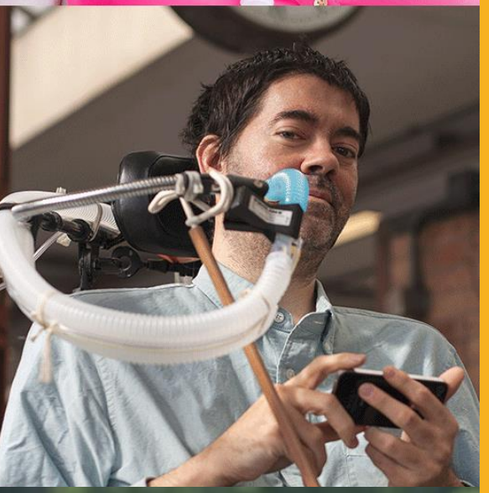
- Clinical safety data for lead CLN6 Batten disease program
- Additional proof of concept for CLN8 Batten Program
- Preliminary Amicus DNA constructs for Pompe Gene Therapy
- Preliminary Amicus DNA constructs for Fabry Gene Therapy
- Amicus/Penn AAV gene therapy approach and strategies
- AT-GAA patient-level data on 6-minute walk test at 18 months
- AT-GAA muscle strength data at 18 months
- Galafold milestone of 500 patients reached in Q3 for International (ex-US)
- Current International market dynamics and trends
- Galafold U.S. adoption trends and key metric 8 weeks into launch

Key Takeaways for Amicus Analyst Day

- **Vision 2023: 5,000+ Patients & $1B+ in Revenue**
- **Galafold: Cornerstone of Success**
  - $500M+ Peak Revenue Potential
  - $1B+ Cumulative Revenue from 2019E-2023E to Drive R&D Engine
- **AT-GAA: The Crown Jewel**
  - Highly differentiated ERT with Potential to Obsolete Current Standard of Care
  - $1B+ Peak Revenue Potential
- **Gene Therapy: Foundation for the Future**
  - Amicus as “Best in Class” Consolidator and Integrator
  - Potential $1B+ in Recurring Peak Revenue from Current Gene Therapy Portfolio
- **World Class, Global Team of “Passionate Entrepreneurs ”**
- **Extraordinary and Intense Patient Focus**

Our Passion for Making a Difference Unites Us

Rare Company Video



Gene Therapy Pipeline in Rare Metabolic Diseases

John F. Crowley
Kathrin Meyer, Ph.D.
Jay Barth, M.D.
Jeff Castelli, Ph.D.

2018 Analyst Day | October 11, 2018 | New York, NY

Amicus Establishes Gene Therapy Portfolio

License Through Nationwide Children's Hospital Combines Successful Amicus Development and Commercial Track Record in LSDs with Ten AAV Gene Therapy Programs for Rare Neurologic LSDs

- Ground Breaking, Clinically Validated Science
- Ten Gene Therapy Programs
- Expertise and Relationships in Gene Therapy
- Compelling Data in Three Lead Programs
- Leading Gene Therapy Portfolio in Neurologic Lysosomal Storage Disorders

"I firmly believe that Amicus is the optimal scientific and clinical partner to move these programs forward and I look forward to actively collaborating with the Amicus team on the development of these important potential therapies."

- Kathrin Meyer, Ph.D., PI at Meyer Lab Nationwide Children's Hospital and Assistant Professor at The Ohio State University

Validated Gene Therapy Platform

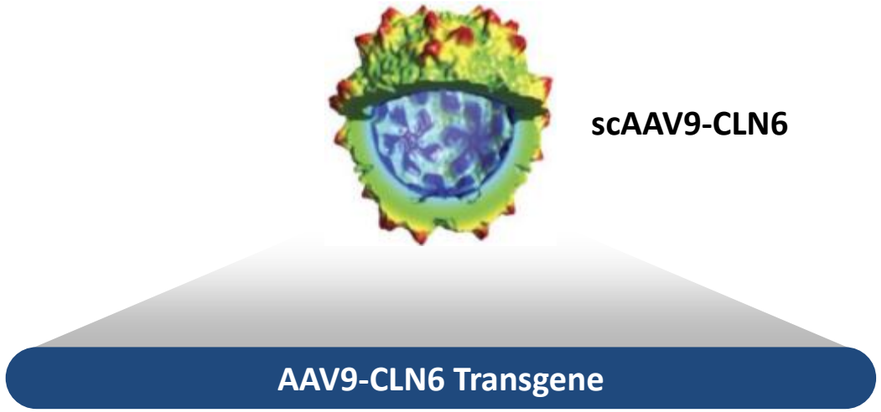
Portfolio is Based on a Validated Gene Therapy Approach Across Multiple CNS Diseases

Clinically validated AAV gene therapy approach

- 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
- Rett Syndrome
- ALS
- CLN6
- CLN3



Foust, Kaspar et al, 2009

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

Amicus Therapeutics

Batten Disease Overview

Batten Disease is a Group of Rare, Fatal, Lysosomal Storage Disorders of the Central Nervous System with High Unmet Need and Limited Treatment Options

Disease Overview

- A group of disorders known as neuronal ceroid lipofuscinoses (NCLs), collectively referred to as Batten disease
- Mutation in one of 13 different CLN genes leads to lysosomal dysfunction
- Signs and symptoms typically begin in early and late childhood
- Most affected children do not survive into adulthood

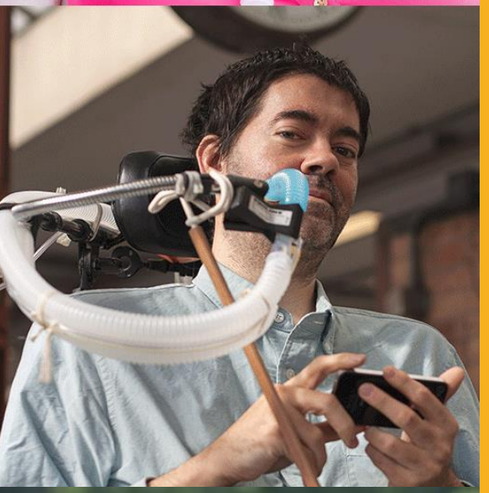
Source: Batten Disease Fact Sheet, NINDS, Publication date June 2018.

Lead Program Status

The CLN6 and CLN3 Program are Clinical Stage; CLN8 has Definitive Preclinical Efficacy Data in a Mouse Model of Disease

	PRECLINICAL MOUSE MODEL DATA						
	Storage Material and Glial Activation	Motor & Cognitive Function	Survival	Safety & Brain Expression in NHP	GMP Clinical Supply	IND Active	Preliminary Clinical Data
CLN6	✓	✓	✓	✓	✓	✓	✓
CLN3	✓	✓	N/A*	✓	✓	✓	Pending
CLN8	✓	✓	✓	Pending	Pending	Pending	Pending

*CLN3 mouse model does not have impaired survival



Preclinical Proof of Concept Data in CLN6 Batten Disease

Kathrin Meyer, Ph.D.

Disclosure Information

I have the following financial relationships to disclose:

- Consultant for Amicus Therapeutics, Inc.

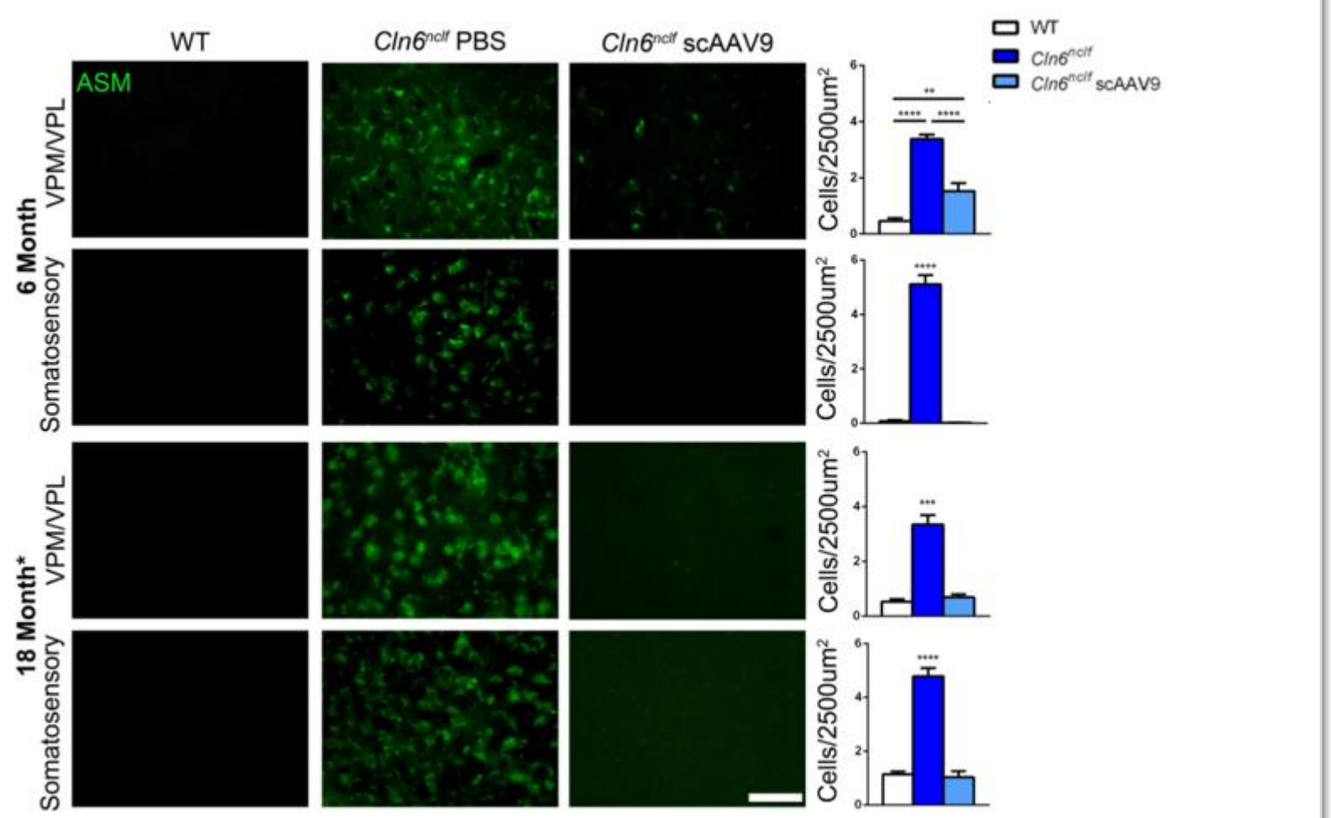
I will discuss the following off-label use and/or investigational use in my presentation:

- Preclinical, proof of concept data from studies for the treatment of patients with Batten disease

CLN6: Preclinical Mouse Data – Autofluorescent Storage Material

Single AAV9-CLN6 Administration Results in Reduction of Autofluorescent Substrate Material Throughout the Brain

Autofluorescent Storage Material Accumulation

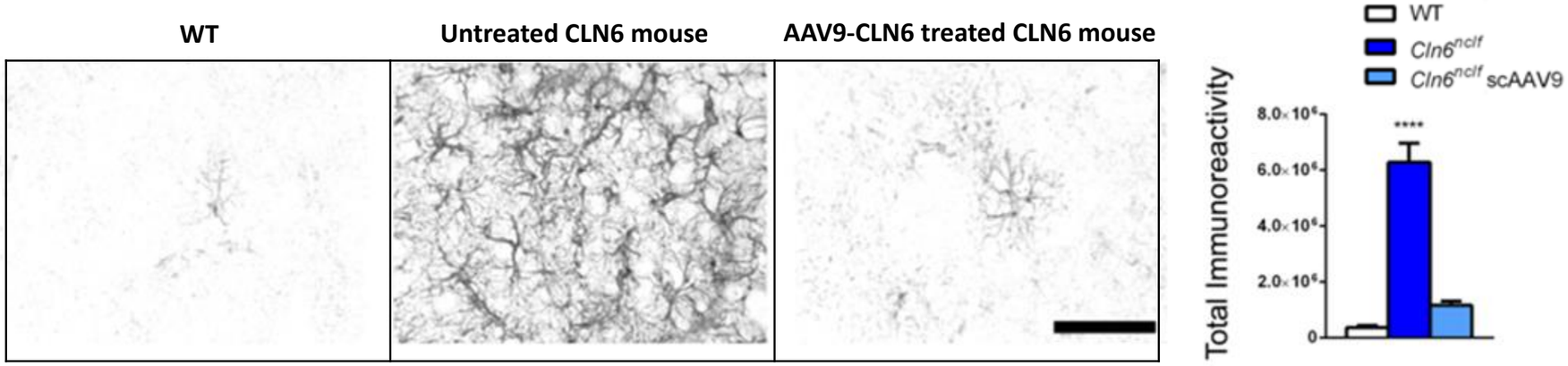


Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

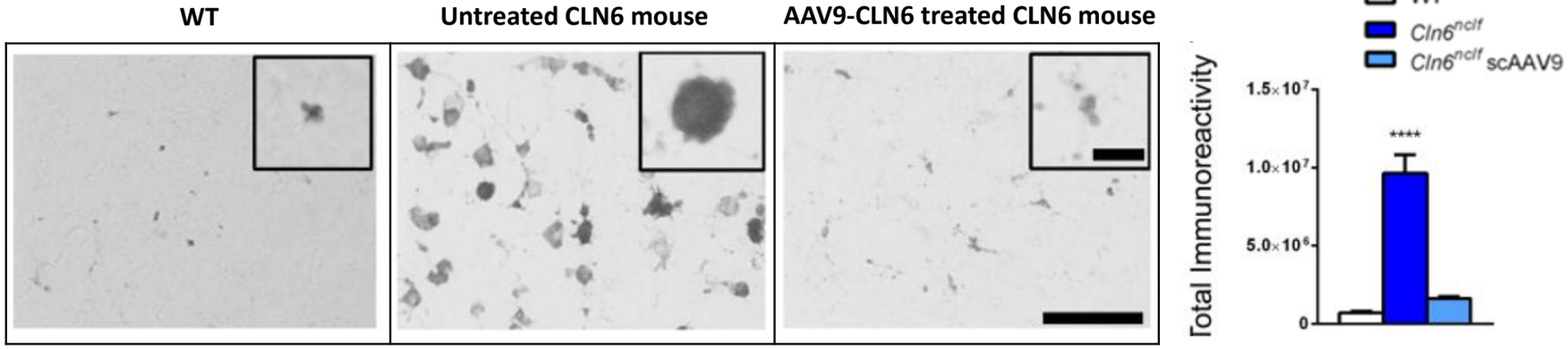
CLN6: Preclinical Mouse Data – Somatosensory Glial Activation

Single AAV9-CLN6 Administration Results in Reduction of Glial Activation

Astrocyte Activation: Month 18



Microglial Activation: Month 18

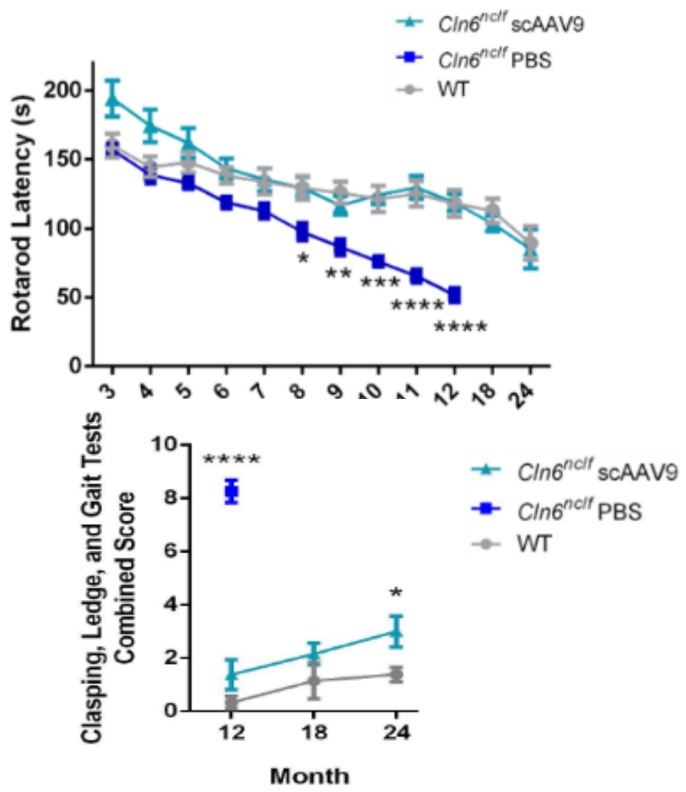


CLN6: Preclinical Mouse Data

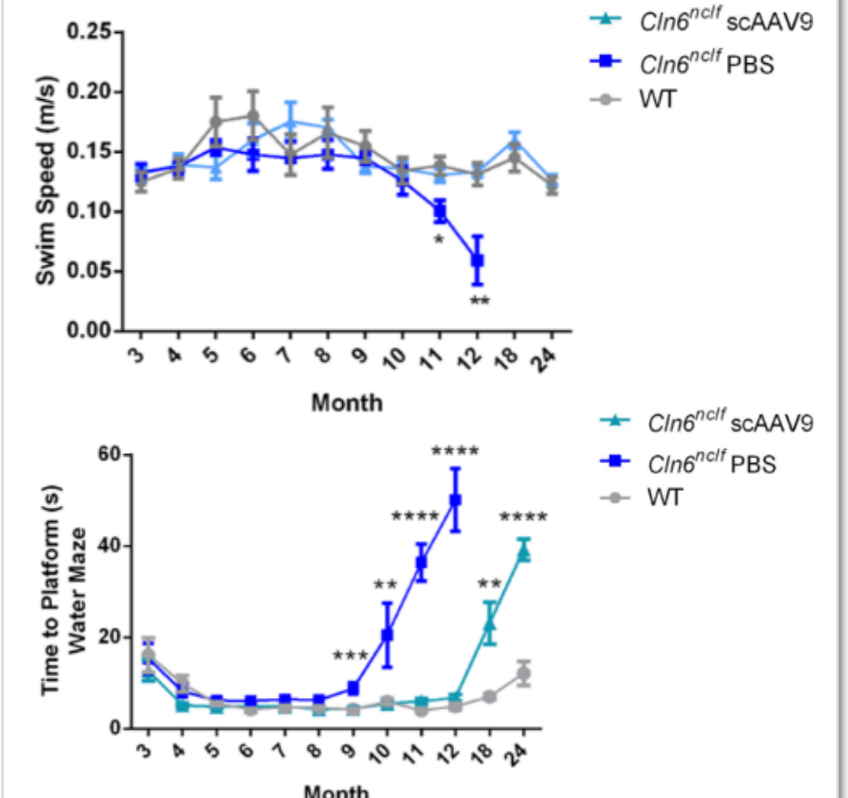
Motor Performance and Cognitive Behavior

Single AAV9-CLN6 Administration Improves Motor Performance & Cognitive Behavior Out to Month 24

Motor Performance



Water Maze Analysis

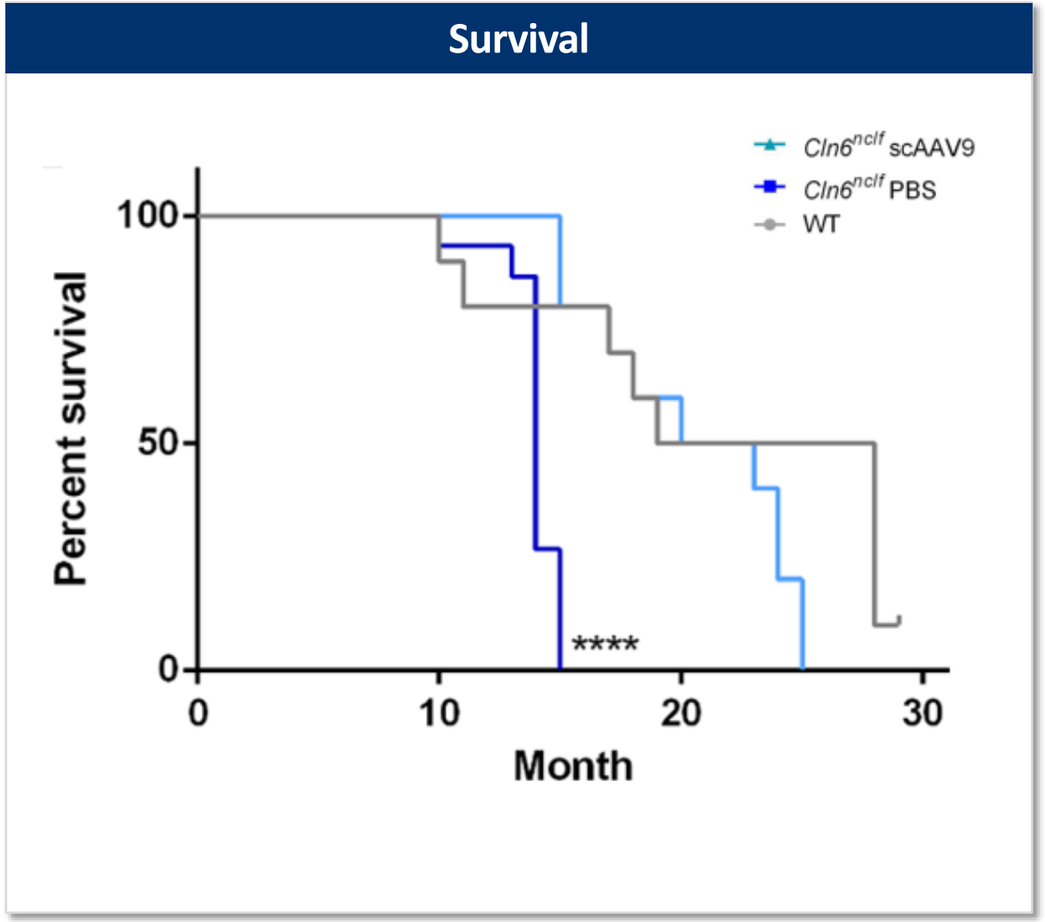


Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy; Data on file

Amicus Therapeutics

CLN6: Preclinical Mouse Data - Survival

Single AAV9-CLN6 Administration Significantly Extends Median Survival



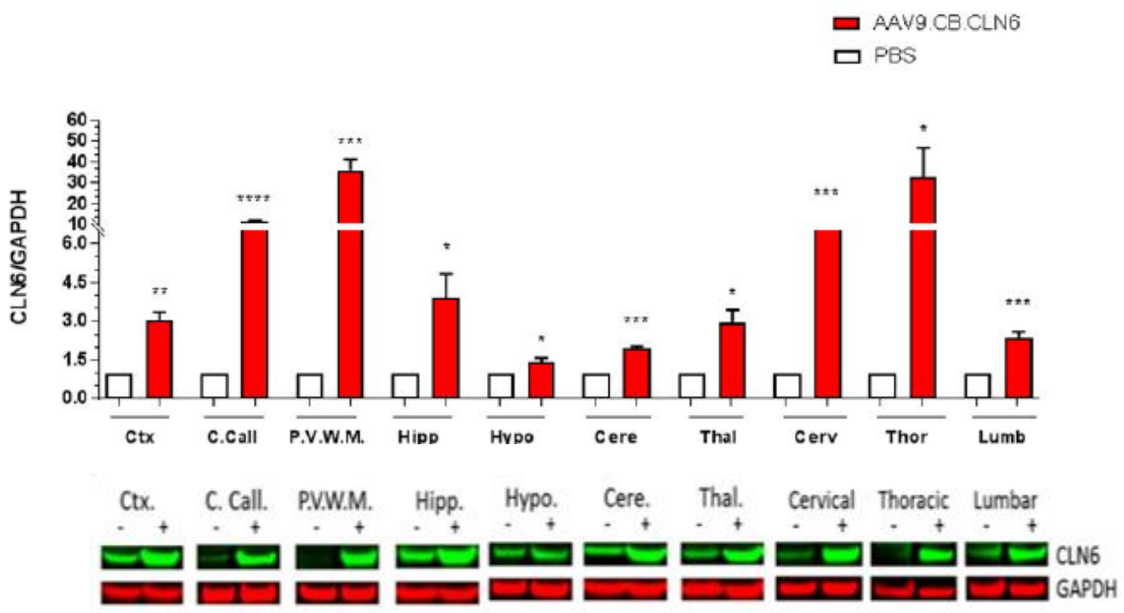
Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

Amicus Therapeutics

CLN6 Expression in NHP Safety Study

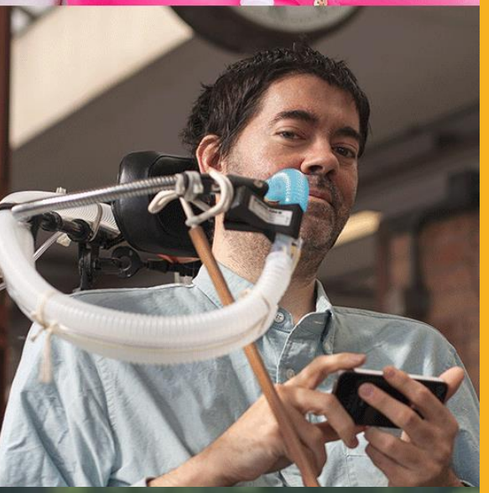
Demonstrated Safety and Meaningful Transduction and CLN6 Expression Throughout the Brain in NHPs

Western Blot on various brain regions of AAV9-CLN6 injected juvenile NHPs



Source: Meyer 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, From mouse to human –Translating intrathecal gene therapy for NCLs; Data on file

Amicus Therapeutics

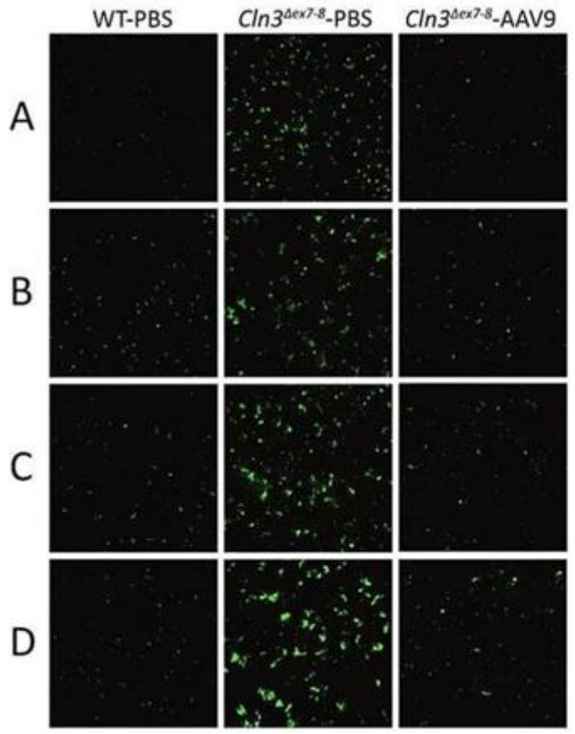


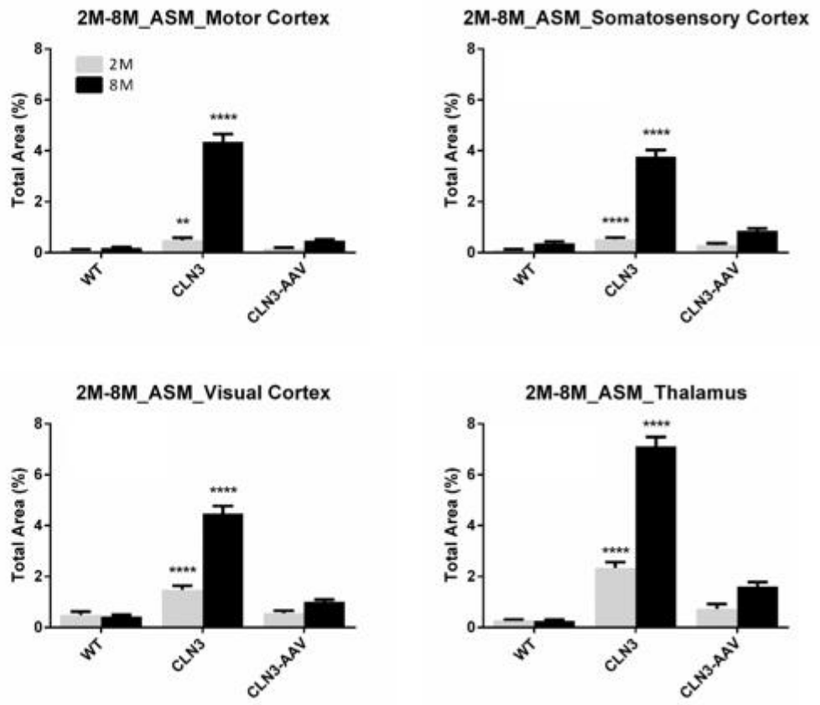
Preclinical Proof of Concept Data in CLN3 Batten Disease

CLN3: Preclinical Mouse Data – Autofluorescent Substrate Material

Single AAV9-CLN3 Administration Results in Reduction of Autofluorescent Substrate Material Throughout the Brain

Autofluorescent Storage Material Accumulation





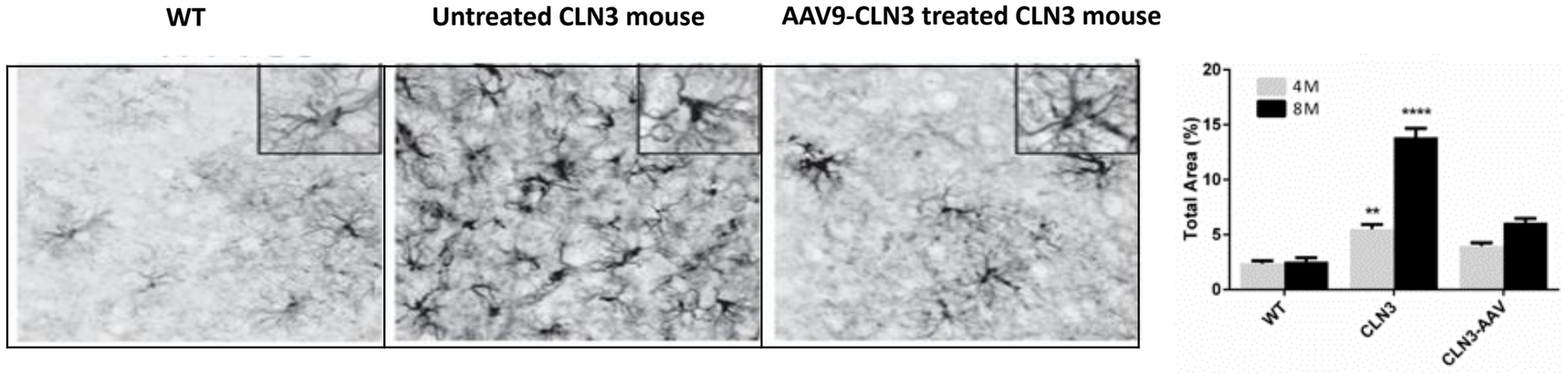
Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

Amicus Therapeutics

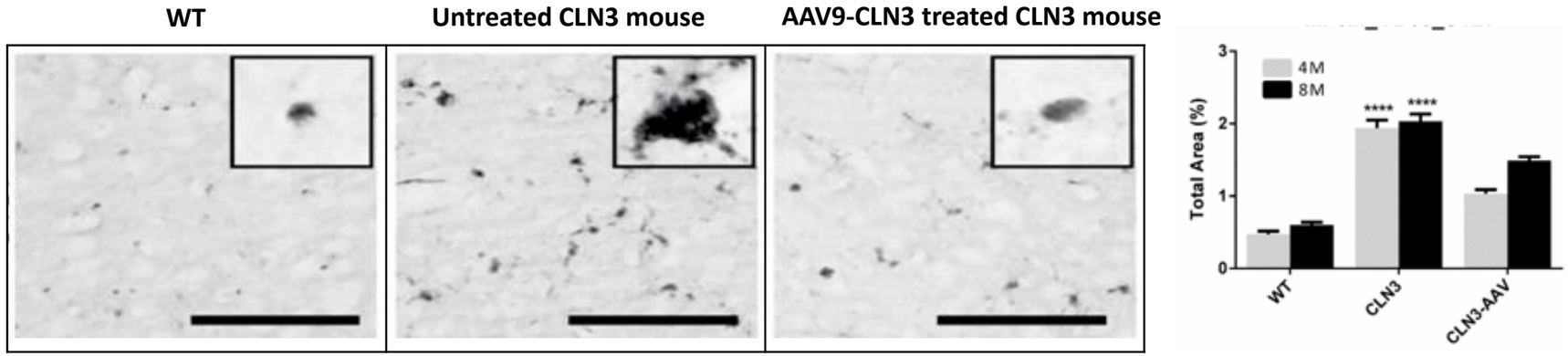
CLN3: Preclinical Mouse Data – Somatosensory Glial Activation

Single AAV9-CLN3 Administration Results in Reduction of Glial Activation

Astrocyte Activation: Month 8



Microglial Activation: Month 8



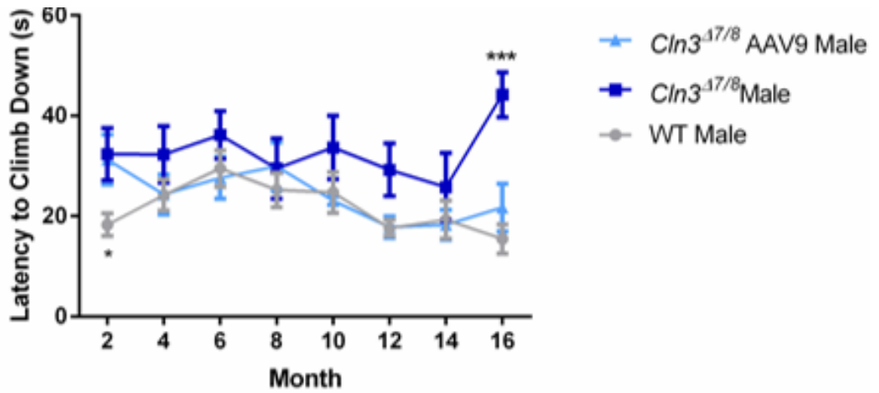
Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

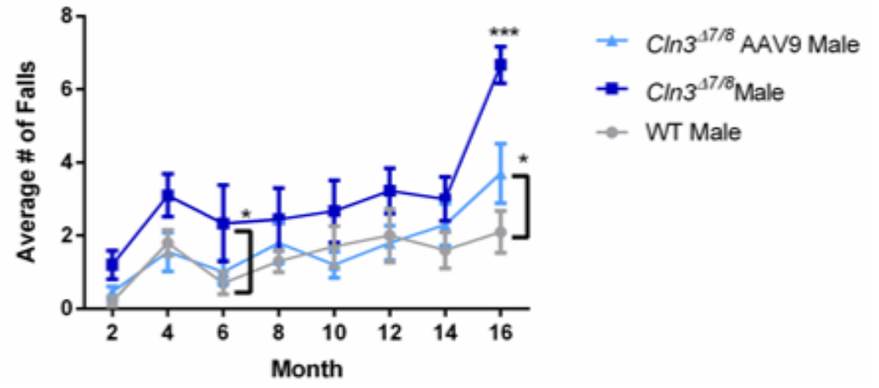
Amicus Therapeutics

CLN3: Preclinical Mouse Data - Motor Performance & Cognitive Behavior

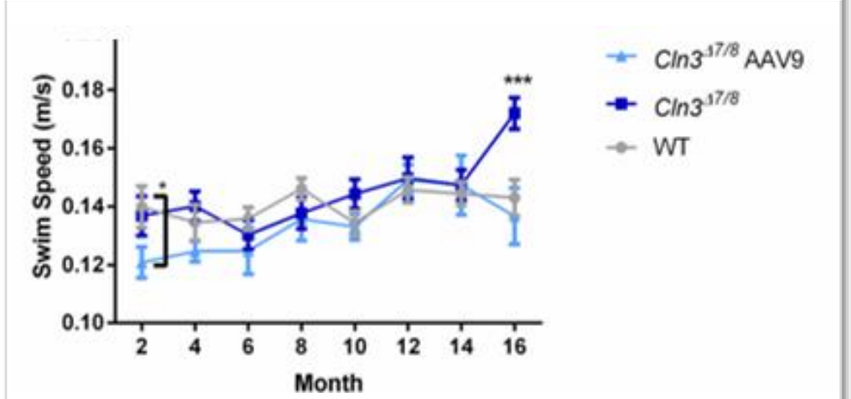
Single AAV9-CLN3 Administration Improves Motor Performance and Cognitive Behavior at Month 16

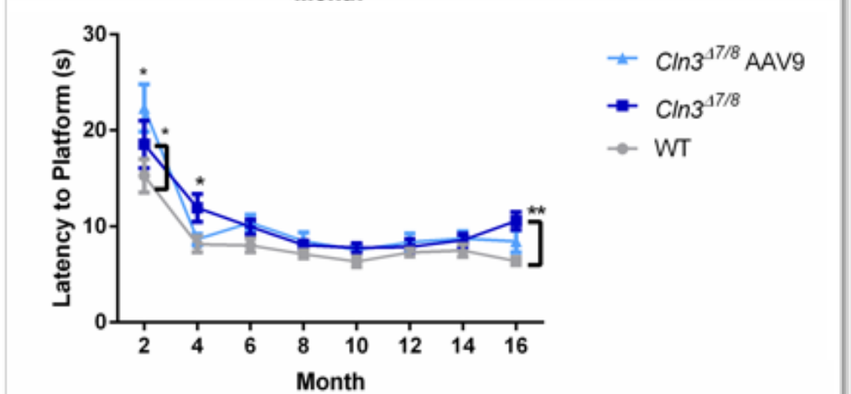
Motor Performance





Water Maze Analysis



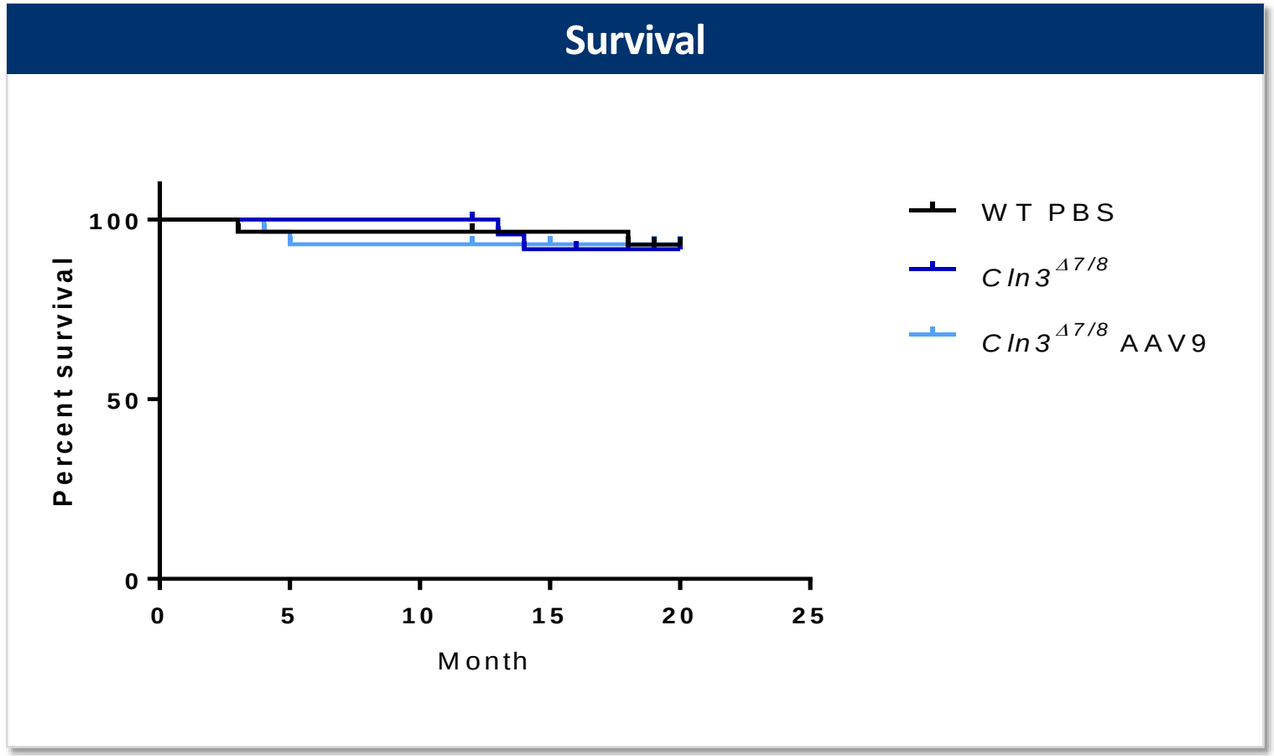


Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

Amicus Therapeutics

CLN3: Preclinical Mouse Data - Survival

Effect of AAV9-CLN3 On Survival Cannot Yet Be Determined Given Minimal Phenotype in Mouse Model



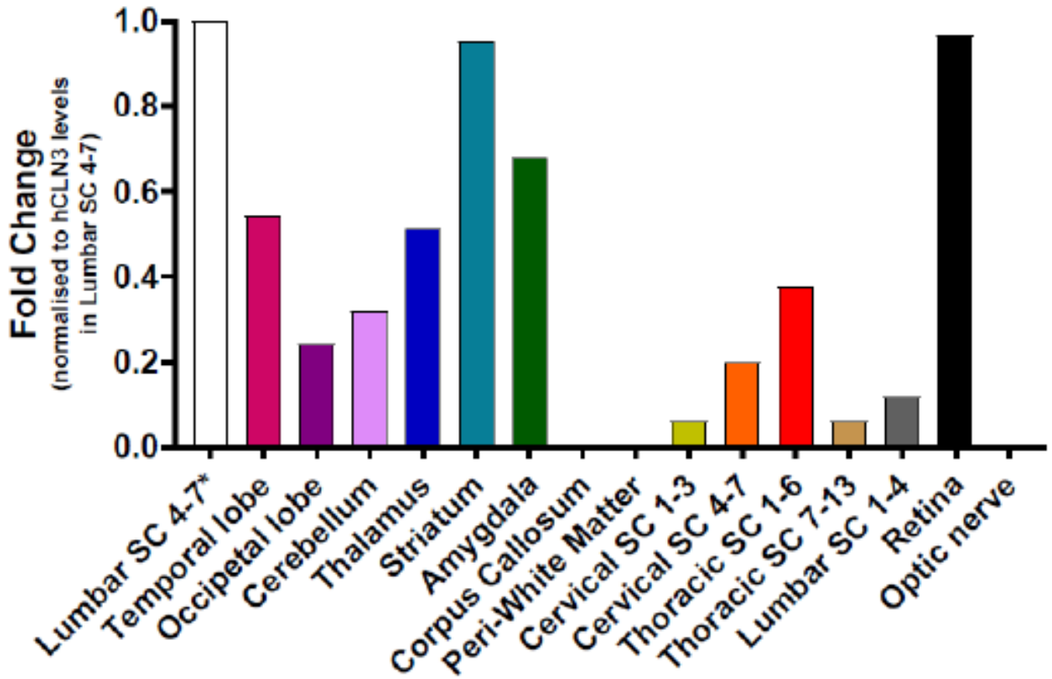
Source: Likhite 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, IND-enabling Preclinical Studies for Batten Disease Gene Therapy

Amicus Therapeutics

CLN3 Expression in NHP Safety Study

Demonstrated Safety and Meaningful Transduction and Expression Throughout the Brain in NHPs

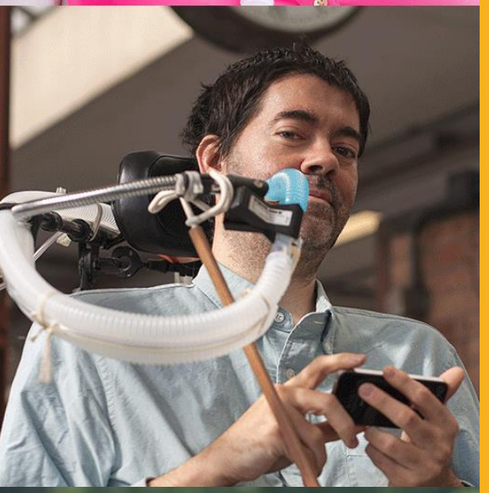
Reverse transcriptase qPCR on various brain regions of AAV9-CLN3 injected juvenile NHPs



Note: CLN3 Western blot -data were not assessable

Source: Meyer 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, From mouse to human –Translating intrathecal gene therapy for NCLs

Amicus Therapeutics

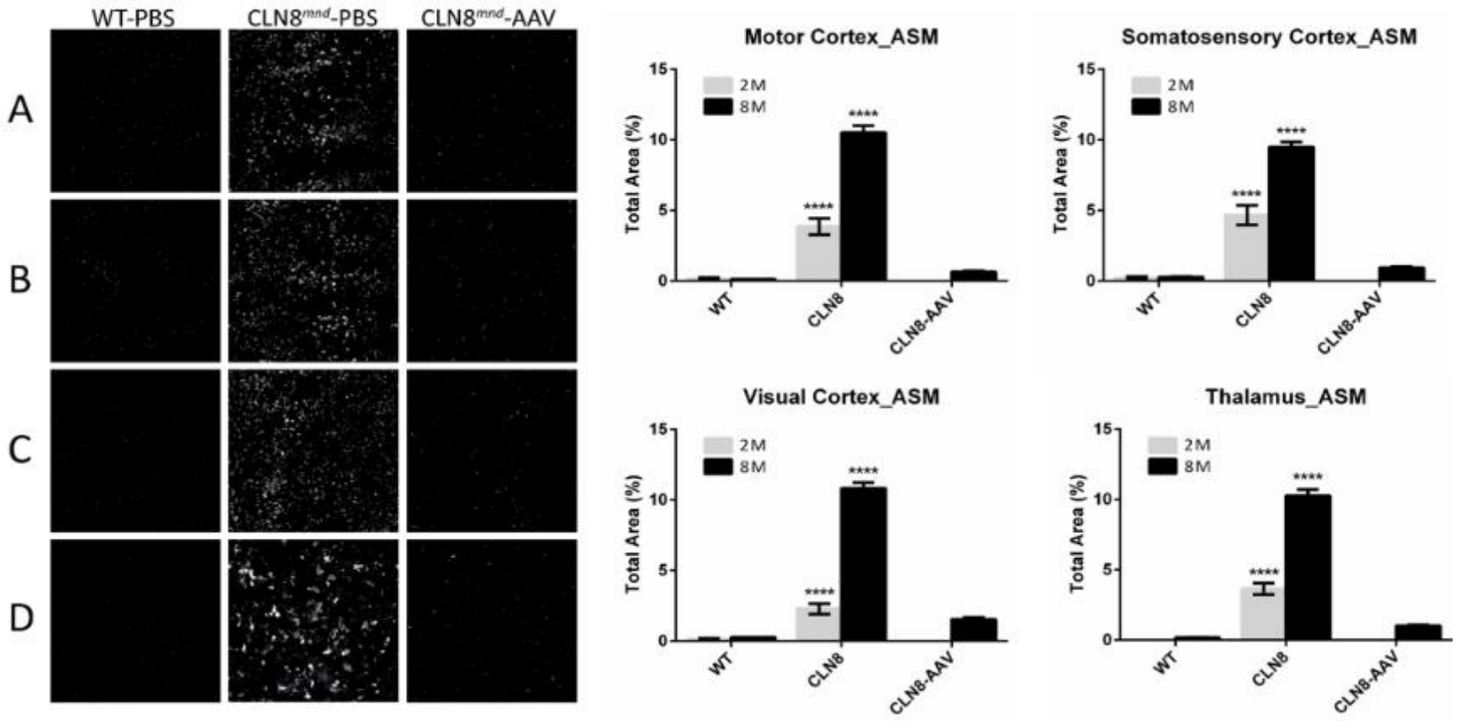


Preclinical Proof of Concept Data in CLN8 Batten Disease

CLN8: Preclinical Mouse Data – Autofluorscent Storage Material

Single AAV9-CLN8 Administration Results in Reduction of Autofluorescent Substrate Material Throughout the Brain

Autofluorescent Storage Material Accumulation



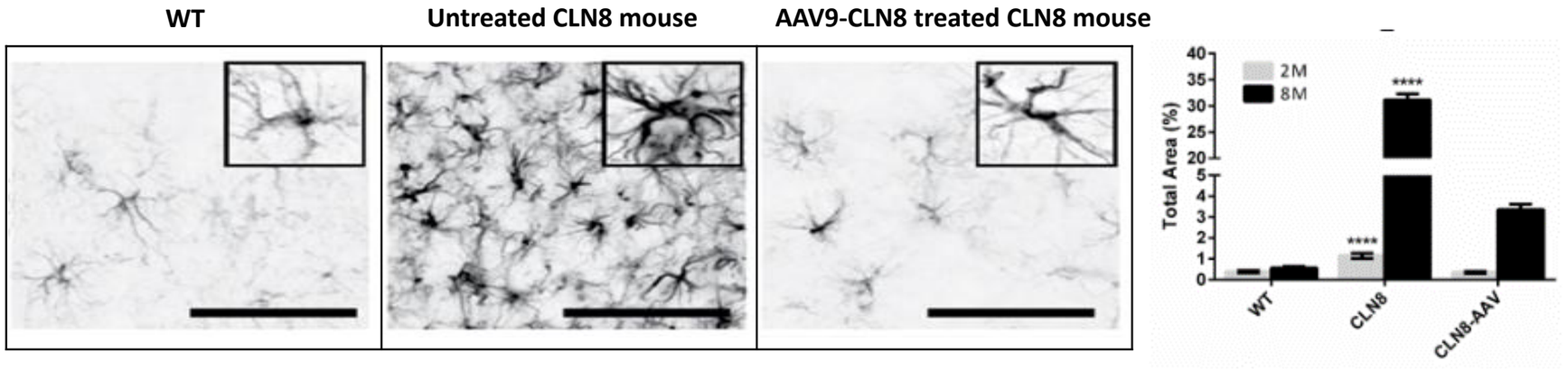
Source: Johnson 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, Testing the safety and efficacy of CLN8 gene therapy in the Cln8mnd mouse model

Amicus Therapeutics

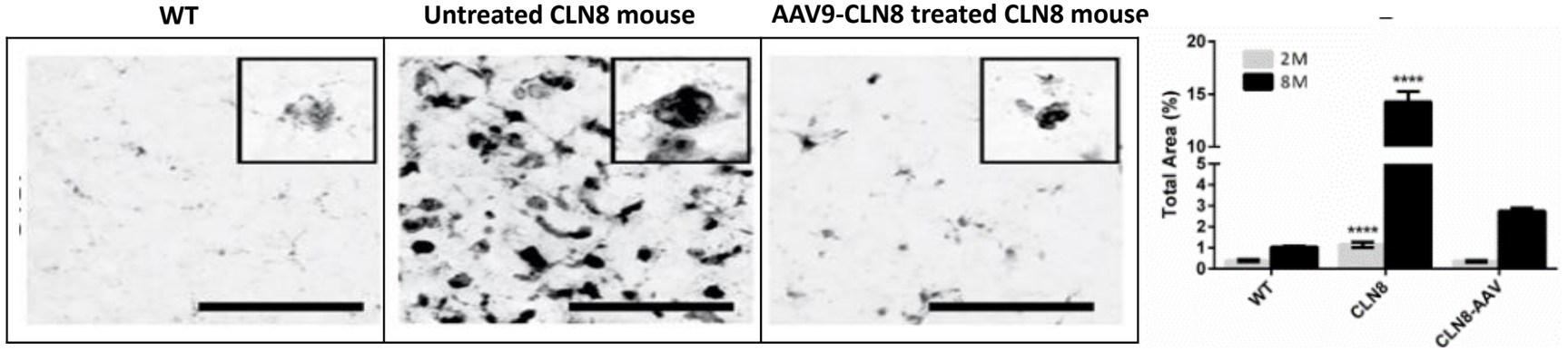
CLN8: Preclinical Mouse Data - Somatosensory Glial Activation

Single AAV9-CLN8 Administration Results in Reduction of Glial Activation

Astrocyte Activation: Month 8



Microglial Activation: Month 8



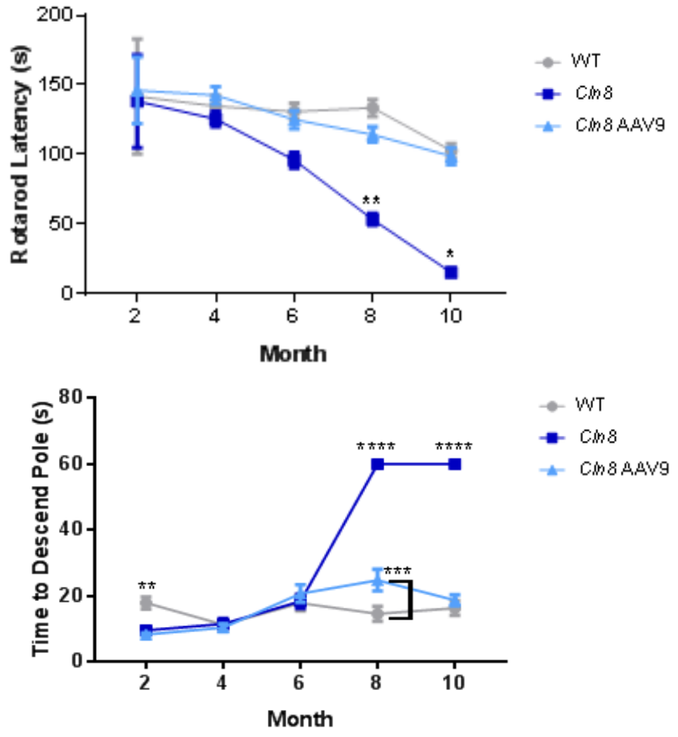
Source: Johnson 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, Testing the safety and efficacy of CLN8 gene therapy in the Cln8^{mnd} mouse model

Amicus Therapeutics

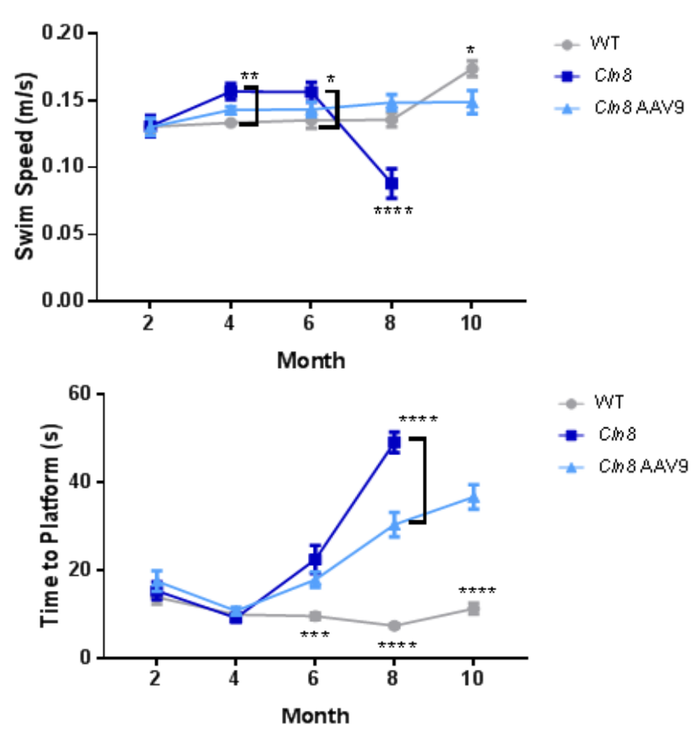
CLN8: Preclinical Mouse Data - Motor Performance and Cognitive Behavior

Single AAV9-CLN8 Administration Improves Motor Performance & Cognitive Behavior Out to Month 10

Motor Performance



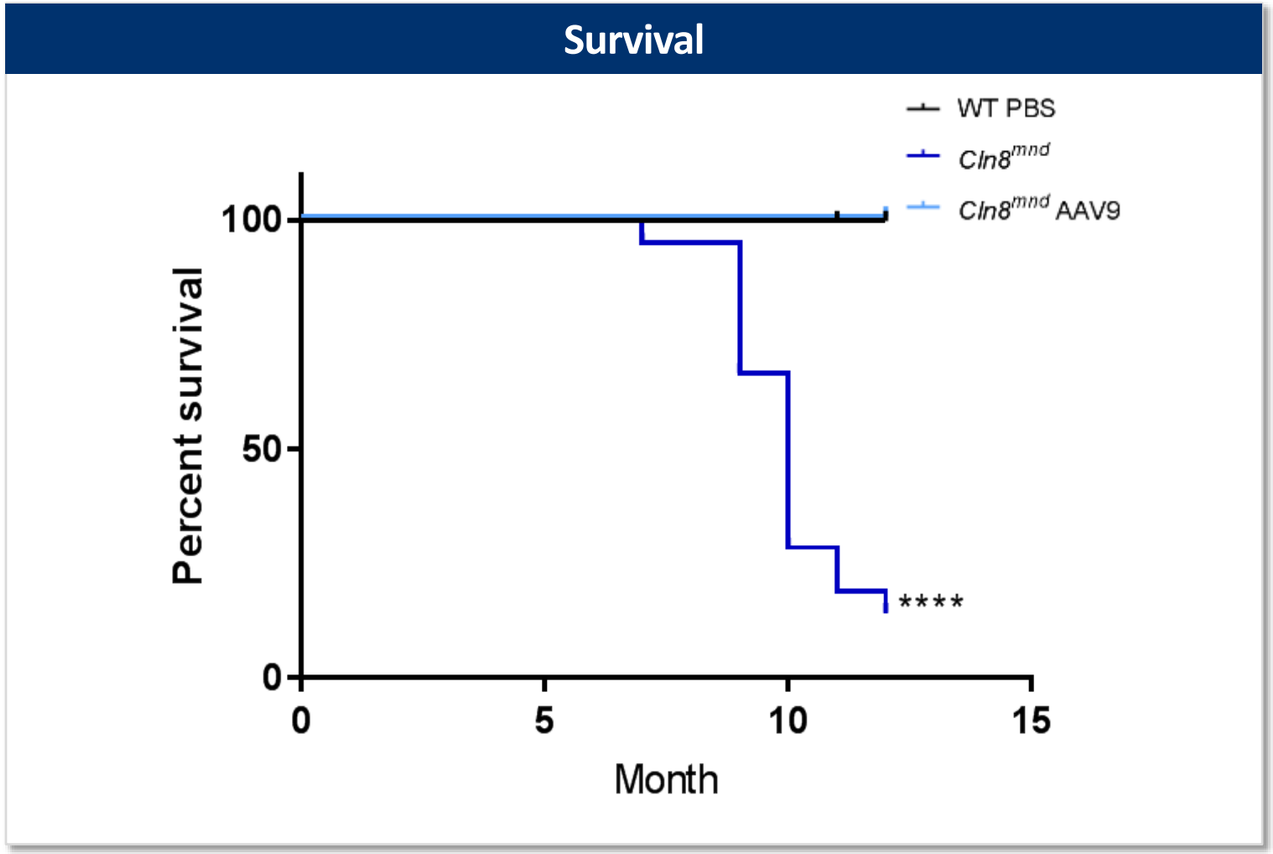
Water Maze Analysis



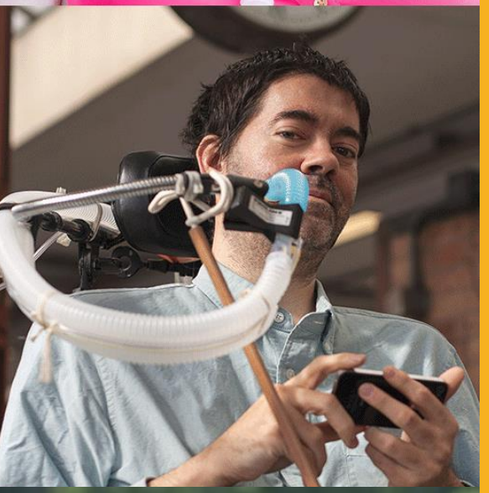
Source: Johnson 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, Testing the safety and efficacy of CLN8 gene therapy in the Cln8^{mnd} mouse model

CLN8: Preclinical Mouse Data - Survival

Single AAV9-CLN8 Administration Significantly Extends Median Survival



Source: Johnson 2018, 16th International Conference on Neuronal Ceroid Lipofuscinoses, Testing the safety and efficacy of CLN8 gene therapy in the Cln8^{mnd} mouse model



CLN6 Clinical Summary

Jay Barth, M.D.

CLN6: Clinical Data Summary

Encouraging Safety and Efficacy Data from an Ongoing Single-arm Phase 1/2 study

- Single-arm study with all patients receiving gene therapy
  - Single intrathecal administration
- Ten patients currently treated; additional patients in screening
- Generally well-tolerated
- Encouraging preliminary efficacy data
- Additional data to be presented in 2019

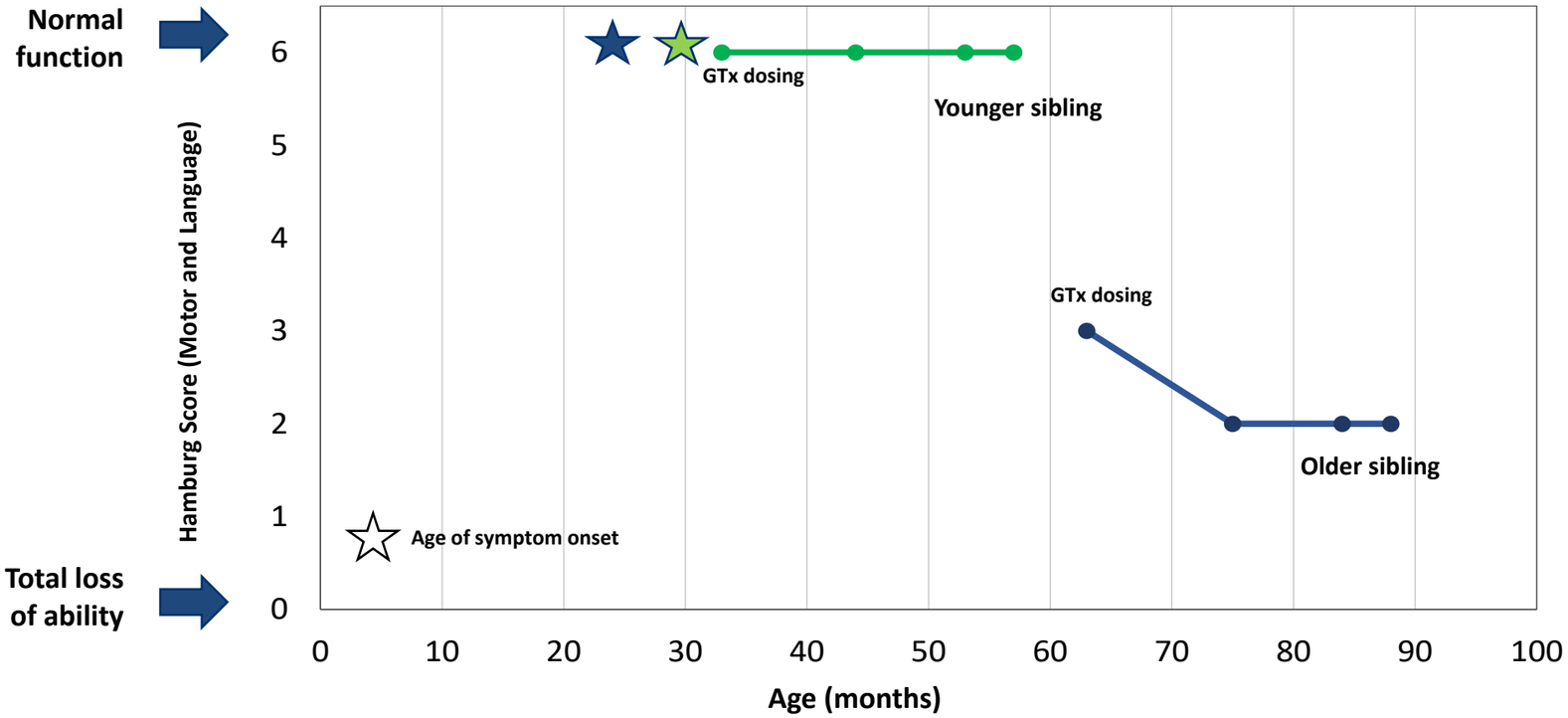
CLN6: Clinical Study Safety Summary Interim Data

Data from an Ongoing Single-arm Phase 1/2 Study Indicate Single AAV9-CLN6 Administration is Generally Well Tolerated

- Ten patients currently treated with single intrathecal administration
  - Average follow-up duration: 12 months (range 1-24 months)
- Adverse events (n=94 events reported)
  - Majority of adverse events (AEs) were mild and unrelated to treatment
  - Five Grade 3 (severe) AEs (defined as medically significant) reported in 4 patients
  - No Grade 4 (life-threatening) or Grade 5 (death) AEs reported to date
- T-cell response and antibody elevations not associated clinical manifestations
  - No changes in treatment required
- Data Safety Monitoring Board (DSMB) has permitted study to proceed and enroll additional patients

Efficacy Data: Matched Sibling Case Report

Encouraging Interim Efficacy Data in First Two Patients Treated with Gene Therapy with Two Years of Follow-up



- Two siblings (same genotype) treated with gene therapy at ages 2.8 and 5.3 years, respectively
- Two years post treatment, Hamburg motor and language scores indicate no disease progression in the younger sibling
- Disease progression in older sibling has shown evidence of stabilization

Source: Data on file

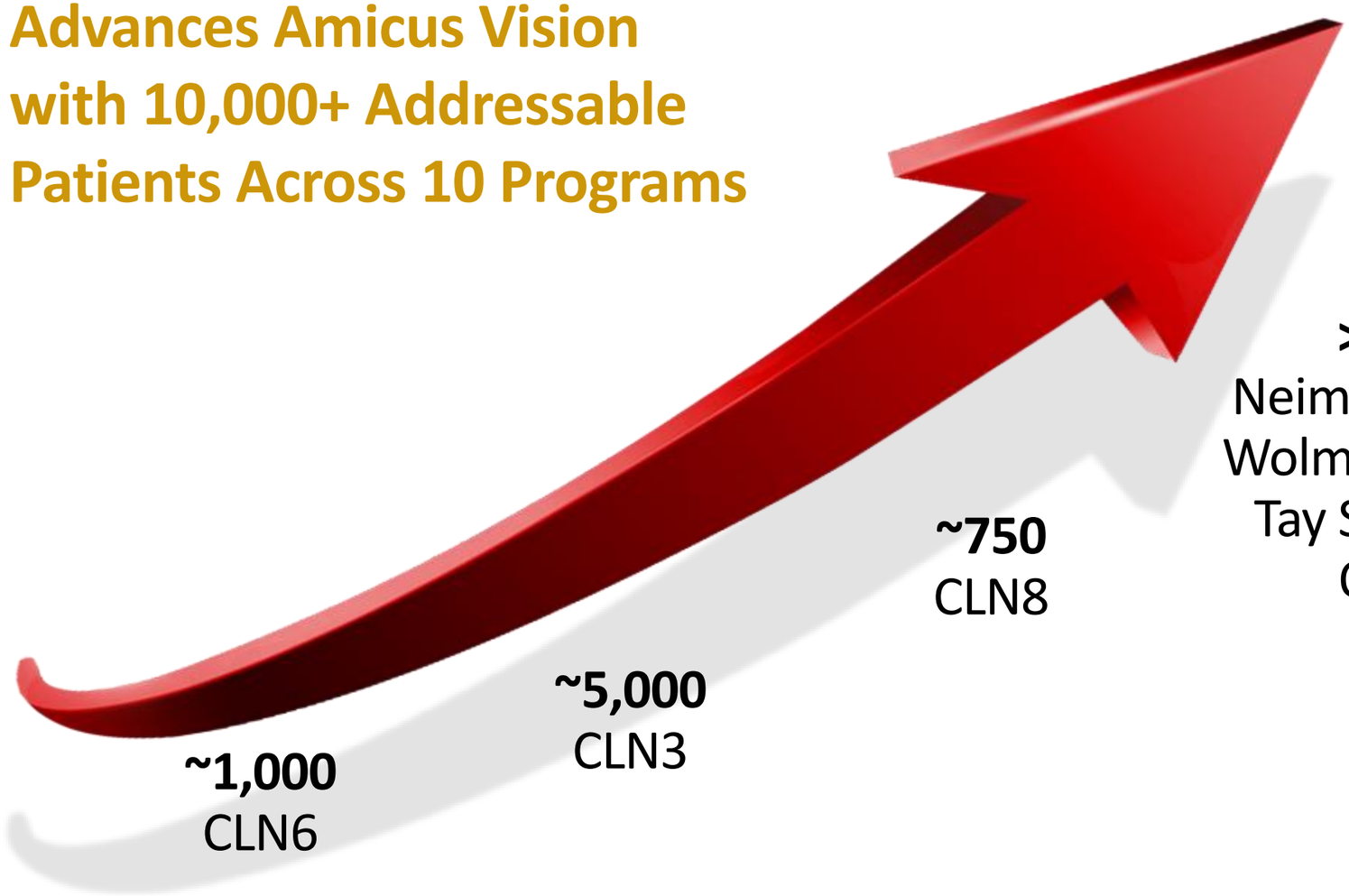
Upcoming Batten Disease Program Milestones

Anticipating Multiple Program Milestones throughout 2018 & 2019

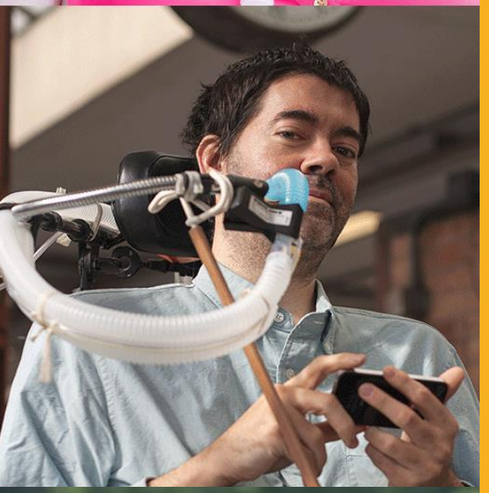
- First Patient in CLN3 Phase 1/2 Study
- Complete Enrollment in CLN6 Phase 1/2 Study
- Preliminary Phase 1/2 Data in CLN6
- Complete Enrollment in Initial Cohort in CLN3 Phase 1/2 Study

Addressable Patient Populations*

Advances Amicus Vision with 10,000+ Addressable Patients Across 10 Programs



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



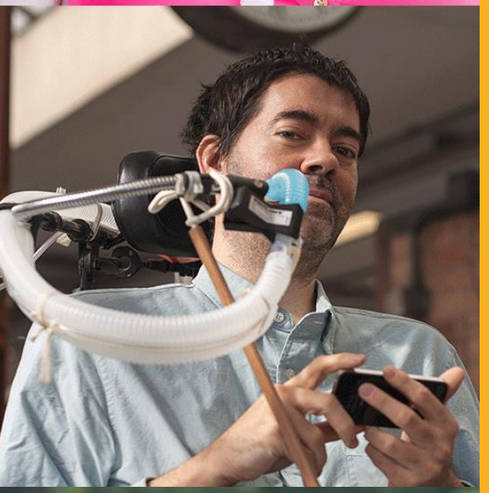
Q&A Session

John F. Crowley

Kathryn Meyer, Ph.D.

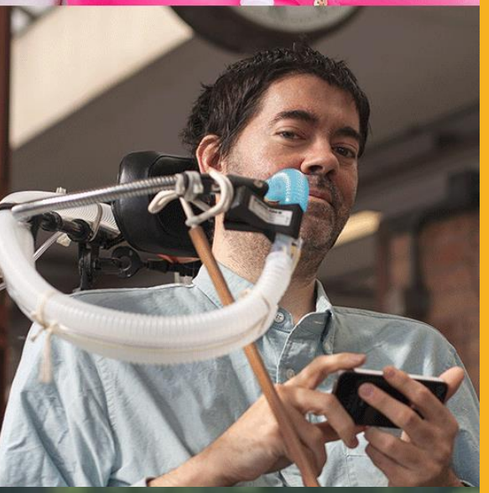
Jay Barth, M.D.

Jeff Castelli , Ph.D.



Break

2018 Analyst Day | October 11, 2018 | New York, NY



Next Generation Gene Therapy Programs

Jeff Castelli, Ph.D.

Jim Wilson, M.D., Ph.D.

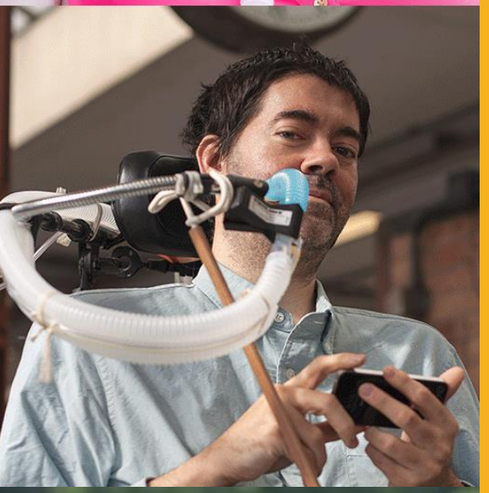
Hung Do, Ph.D.

2018 Analyst Day | October 11, 2018 | New York, NY

Collaboration Overview

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

- Dr. James Wilson and Gene Therapy Program (GTP) at Penn: Renowned center of excellence
  - >20 years of gene therapy experience
  - Proven platform with numerous clinical programs across multiple disease indications
  - Leader in next generation AAV technologies
- Strong synergy with Amicus protein engineering capabilities
  - Leverages Amicus expertise in optimizing protein expression, secretion, stabilization and targeting
- Strategic fit with Amicus R&D, commercial and manufacturing capabilities
- Worldwide rights to 4 LSD programs



The Gene Therapy Program at University of Pennsylvania

Jim Wilson, M.D., Ph.D.

2018 Analyst Day | October 11, 2018 | New York, NY

The **Orphan Disease Center** will develop **transformative** therapies using **platform** technologies that can be deployed across multiple rare diseases. We will emphasize disorders with substantial **unmet need** independent of their incidence and will strive to assure **access** to patients of all populations.

- Areas of emphasis for accelerating therapeutic development
- Establish and execute research agenda
- Partner with patient advocacy groups and create alliances
- Engage in biopharmaceutical partnerships
- Early regulatory engagement
- Priority areas:
  - Lysosomal Storage Diseases
  - Infantile Epilepsies
  - Liver Metabolic Diseases
  - Neurodegenerative Diseases

Symbiotic Relationship of Gene Therapy Program and Orphan Disease Center

GENE THERAPY PROGRAM

Gene therapy development & research

Gene therapy for orphan diseases

Accelerating rare disease research & clinical development*

the orphan disease center

* therapeutic approach agnostic

Overview of GTP Vector Operations at Penn

Numerous Vector Operations Become Available to Amicus as the Relationship Continues to Develop

Clinical Vector Services

- Vector Manufacturing in support of pharm/tox IND-enabling studies
- Development of scalable upstream & downstream manufacturing processes
- Transfer of process technologies to CMOs
- Establishment of GMP manufacturing capabilities at GTP

Analytics

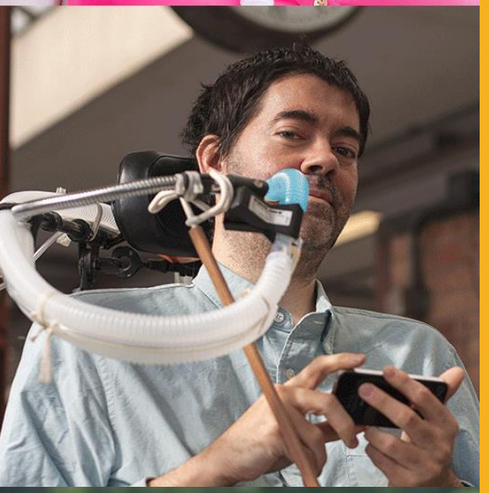
- GLP Quality Control of GMP and GMP process-comparable vectors
- Quality control of research vectors
- Development of assays for vector characterization and release
- Transfer of assay technologies to CMO/CROs

Vector Design & Construction

- Design, construction & optimization of vector constructs
- Production & outsourcing of plasmid DNA source materials
- Structure & sequence analysis of plasmid DNA
- Next generation sequencing (NGS) of plasmid source materials

Preclinical Vector Services

- Production of research vectors for basic and preclinical studies
- Distribution of AAV 1.0 and 2.0 research vectors worldwide (eventually 3.0)
- Management of material transfer agreements (MTAs) for outgoing vector materials
- IBC registration of recombinant DNA



Next Generation Gene Therapy Programs

Hung Do, Ph.D.

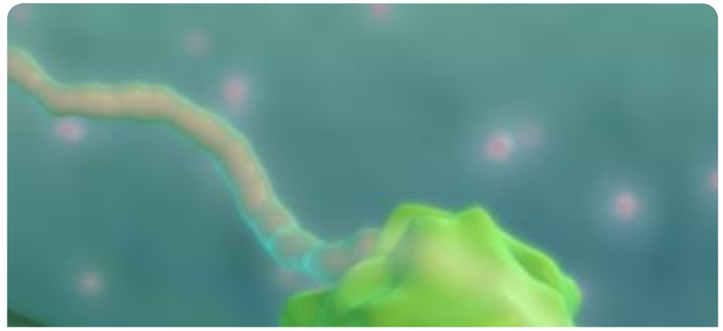
2018 Analyst Day | October 11, 2018 | New York, NY

Applying Amicus Protein Engineering Expertise and Technologies to Gene Therapy

Enabling Greater Protein Expression and Delivery at Lower Gene Therapy Doses

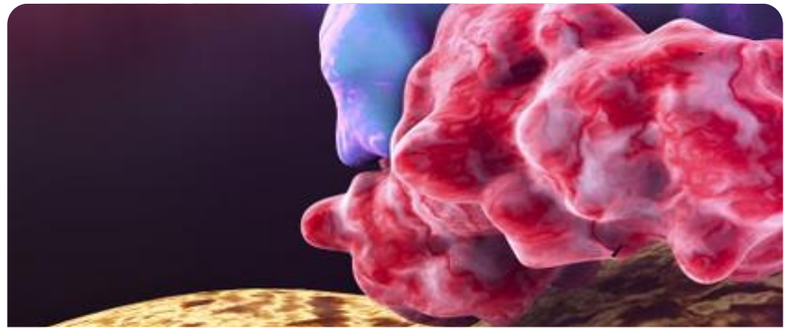
Increased Protein Expression

Novel untranslated sequences to avoid inhibition of initiation and drive efficient protein synthesis



Increased Protein Secretion

Effective signal sequences to increase protein expression & secretion



Improved Protein Targeting and Stabilization

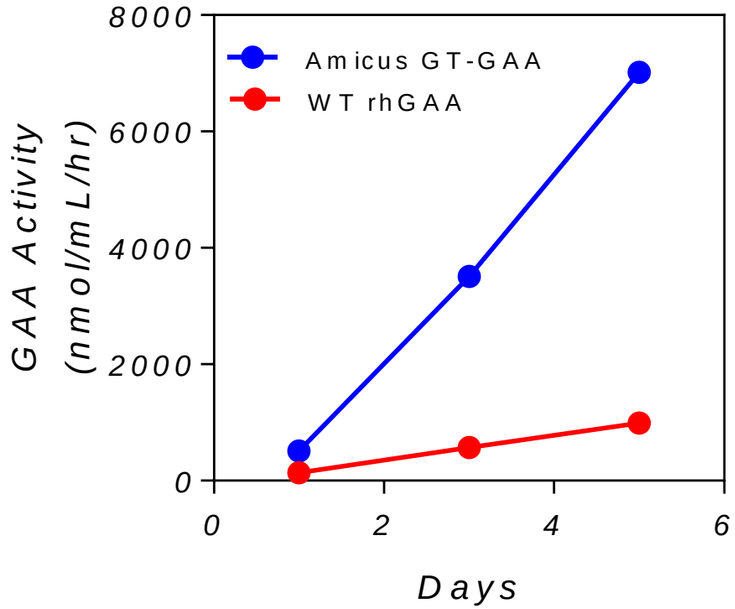
Targeting moieties

Protein design

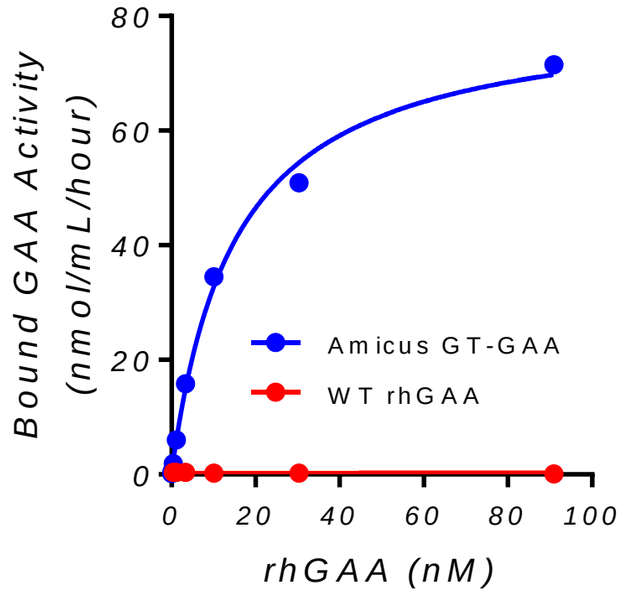
Early Proof of Principle for Optimized Pompe Gene Therapy

Amicus DNA Constructs Enable Highly Expressed GAA and Vastly Improved Cellular Uptake

Secreted GAA in Media



GAA Binding to Intended Receptor

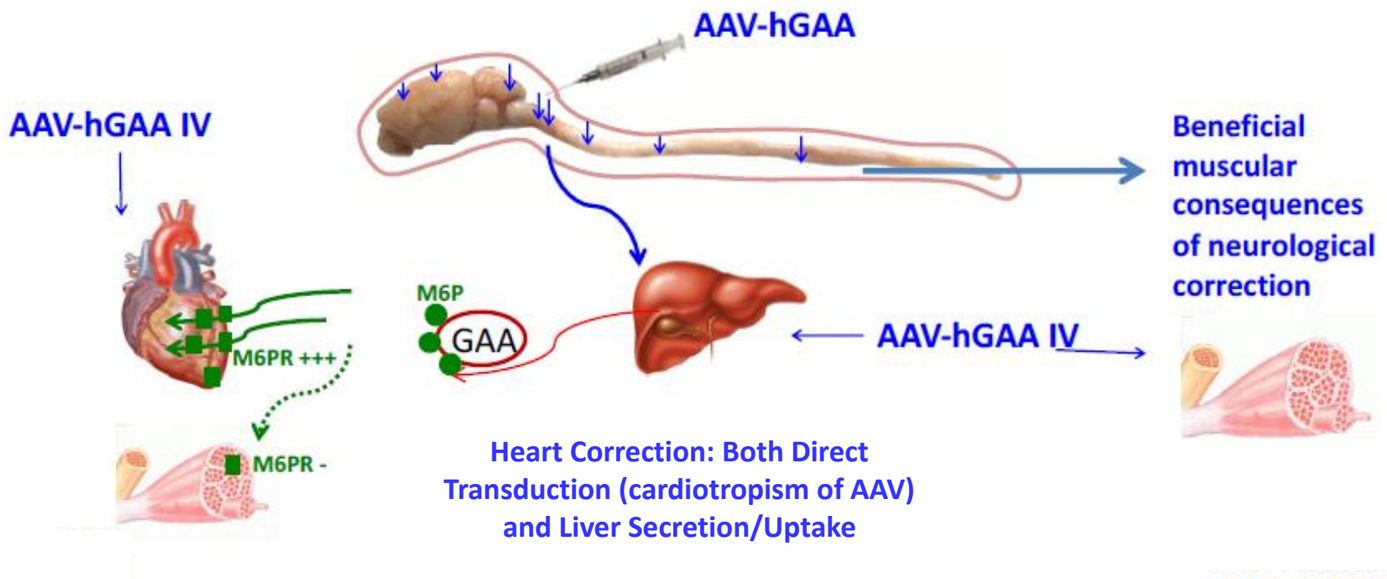


Pompe Disease: AAV Gene Therapy Approach

An Optimized Enzyme Delivered to Key Tissues May Correct both Central Nervous System and Musculoskeletal Aspects of Pompe to Address All Aspects of Disease

Aim: **Globally** Target and Correct the CNS, Heart, Muscles by AAV-hGAA Gene Therapy

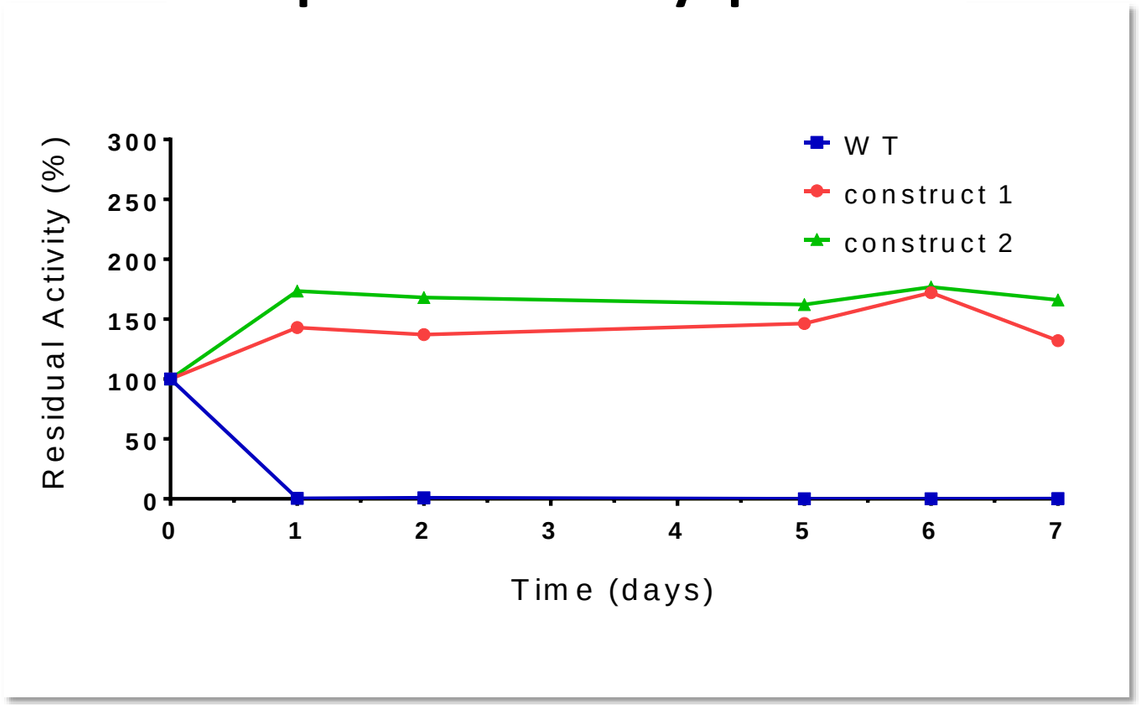
- Intravenous and/or intrathecal injection
- AAV: Neuronal + glial tropism, cardiac tropism, liver tropism



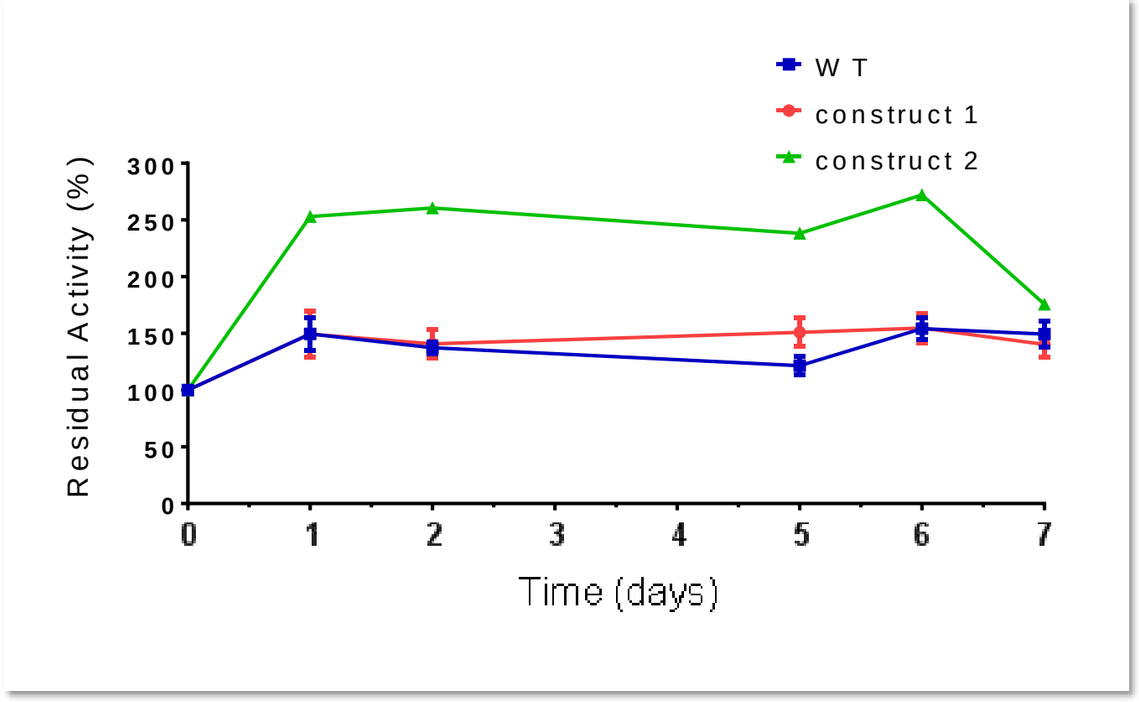
Early Proof of Principle for Optimized Fabry Gene Therapy

Amicus DNA Constructs Enable Highly Stable and Active α-Gal A Enzymes

Alpha-Gal Activity: pH 7.4



Alpha-Gal Activity: pH 4.6

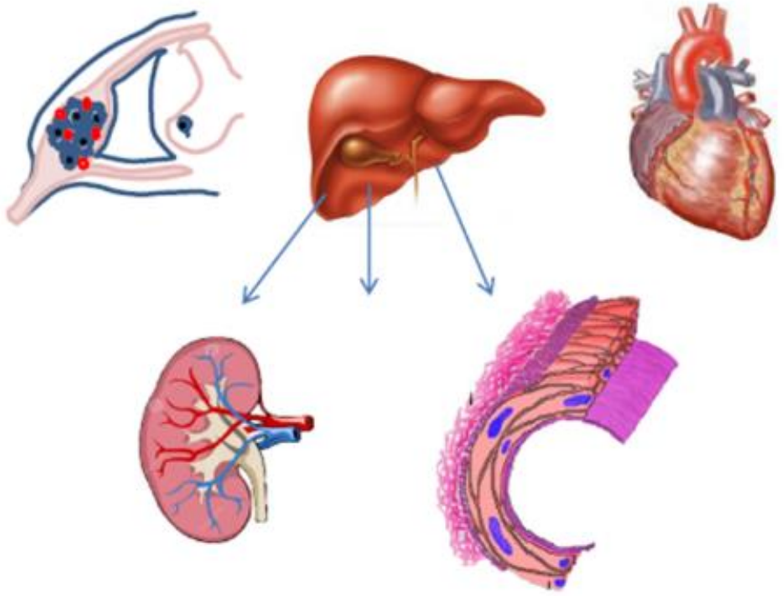


Fabry Disease: AAV Gene Therapy Approach

Goal is to Develop AAV Gene Therapies with Higher Transduction in Heart, Peripheral Nervous System and Liver with More Stable Enzyme and Better Uptake to Target Tissues

Direct AAV robust transduction: in situ correction

Cross-correction from liver secretion

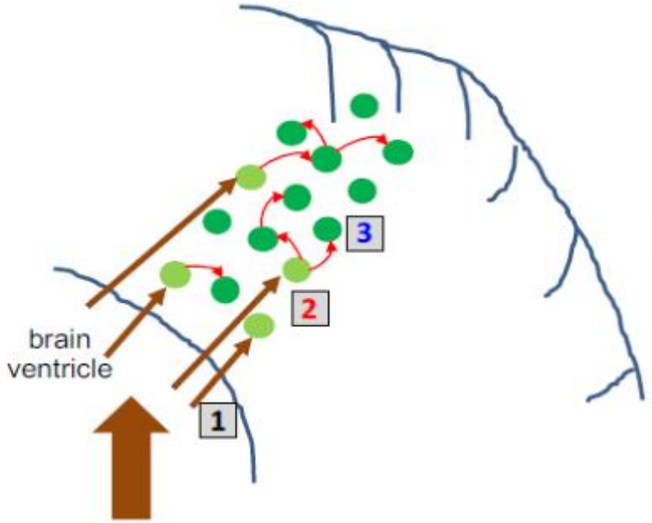


Liver targeted cross-correction: constant, high, steady enzyme levels
Heart and DRG tropism: direct in situ correction

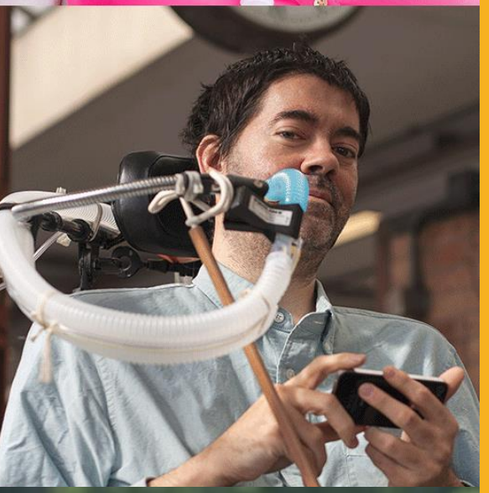
CDKL5 Deficiency Disorder (CDD) AAV Gene Therapy

Utilizing an Amicus Cell Penetrating Peptide for Delivery of CDKL5 in Target Neuronal Cells

Goal: ***Develop a clinical candidate for CDKL5 gene therapy with enhanced efficacy through CDKL5 secretion and uptake by neighboring neurons.***



Therapeutic Benefit
Increased expression of CDKL5 in the brain



Q&A Session

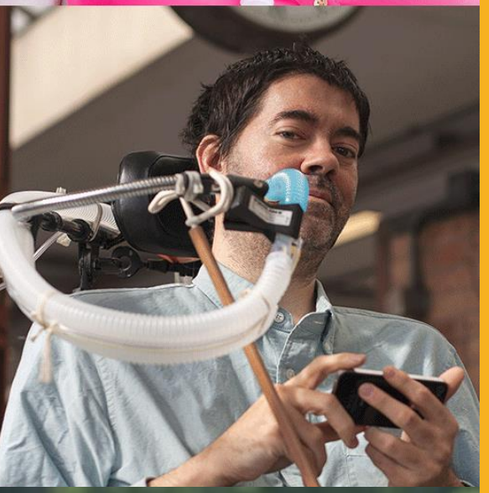
John F. Crowley

Hung Do, Ph.D.

Jeff Castelli, Ph.D.

Jim Wilson, M.D., Ph.D.

2018 Analyst Day | October 11, 2018 | New York, NY



First-in-human Study of ATB200/AT2221 in Patients With Pompe Disease: 18 Month Safety and Efficacy Data From the ATB200-02 Trial

Mark Roberts, M.D.

Benedikt Schoser,[1] Drago Bratkovic,[2] Barry J. Byrne,[3] Paula Clemens,[4] Tarekegn Geberhiwot,[5] Ozlem Goker-Alpan,[6] Priya Kishnani,[7] Xue Ming,[8] Tahseen Mozaffar,[9] Peter Schwenkreis,[10] Kumaraswamy Sivakumar,[11] Ans T. van der Ploeg,[12] Jacquelyn Wright,[13] Swati Sathe,[13] Sheela Sitaraman,[13] Hjalmar Lagast,[13] Jay A. Barth,[13] Mark Roberts[14]

[1]Klinikum der Universität München-Neurologische Klinik, Friedrich-Baur-Institut, Munich, Germany; [2]PARC Research Clinic, Royal Adelaide Hospital, Adelaide, SA, Australia; [3]University of Florida, Gainesville, FL, USA; [4]University of Pittsburgh and Department of Veterans Affairs Medical Center, Pittsburgh, PA, USA; [5]University Hospital Birmingham NHS Foundation Trust, Queen Elizabeth Medical Center, Birmingham, UK; [6]O&O Alpan LLC, Fairfax, VA, USA; [7]Duke University Medical Center, Durham, NC, USA; [8]Rutgers New Jersey Medical School, Newark, NJ, USA; [9]University of California, Irvine, Orange, CA, USA; [10]Neurologische Klinik und Poliklinik des Berufsgenossenschaftlichen, Universitätklinikum Bergmannsheil, Bochum, Germany; [11]Neuromuscular Research Center, Phoenix, AZ, USA; [12]Erasmus MC University Medical Center, Rotterdam, The Netherlands; [13]Amicus Therapeutics, Inc., Cranbury, NJ, USA ; [14]Salford Royal NHS Foundation Trust, Salford, UK;

2018 Analyst Day | October 11, 2018 | New York, NY

Disclosure Information

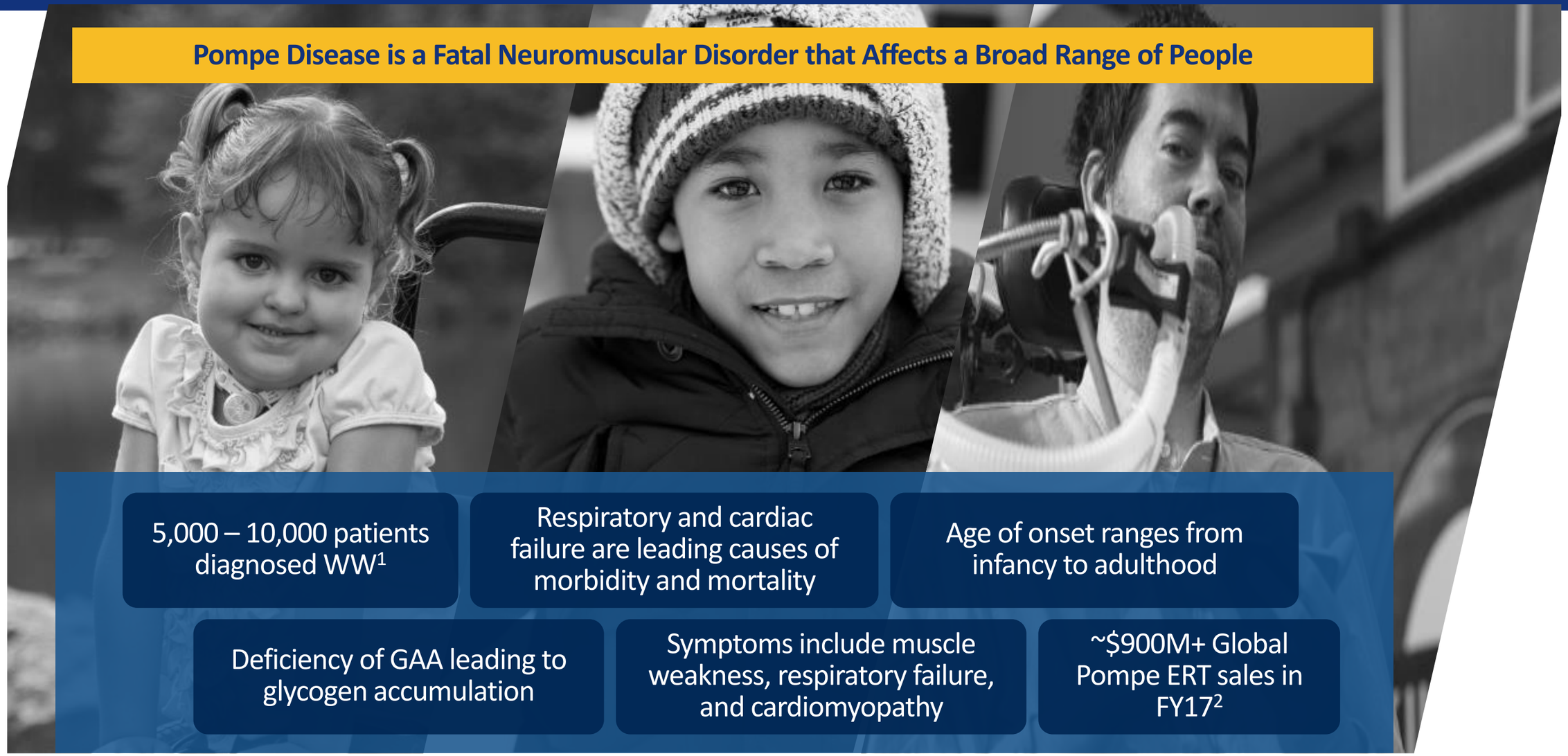
I have the following financial relationships to disclose:

- Consultant for Amicus Therapeutics, Inc.
- Consultant and member of speaker bureau for Audentes, Biomarin, and Sanofi

I will discuss the following off-label use and/or investigational use in my presentation:

- Data from a phase 1/2 trial of ATB200/AT2221 for the treatment of patients with Pompe disease
- ATB200/AT2221 is an investigational therapy that has not been approved for commercial use

Pompe Disease Overview



Pompe Disease is a Fatal Neuromuscular Disorder that Affects a Broad Range of People

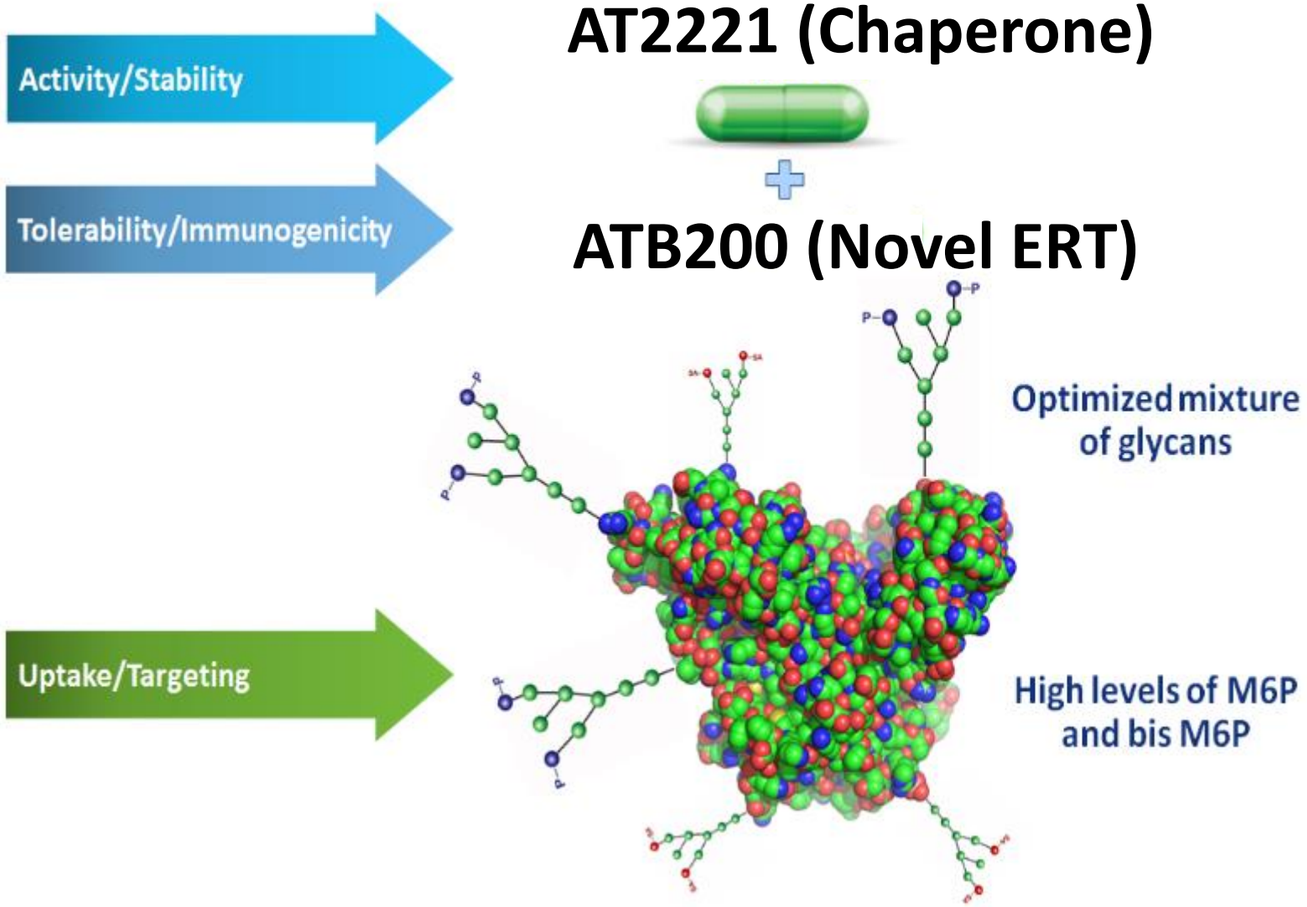
- 5,000 – 10,000 patients diagnosed WW[1]
- Respiratory and cardiac failure are leading causes of morbidity and mortality
- Age of onset ranges from infancy to adulthood
- Deficiency of GAA leading to glycogen accumulation
- Symptoms include muscle weakness, respiratory failure, and cardiomyopathy
- ~$900M+ Global Pompe ERT sales in FY17[2]

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

Amicus Therapeutics

ATB200 Co-administration With AT2221

- AT2221: orally administered investigational PC prior to infusion of ATB200
  - Shown to stabilize ERT in blood and maintain catalytic activity to enhance delivery of active enzyme to lysosomes[1,2]
- ATB200: investigational next-generation ERT
  - Designed with optimized glycosylation and high levels of mannose 6-phosphate residues for better uptake to target tissues

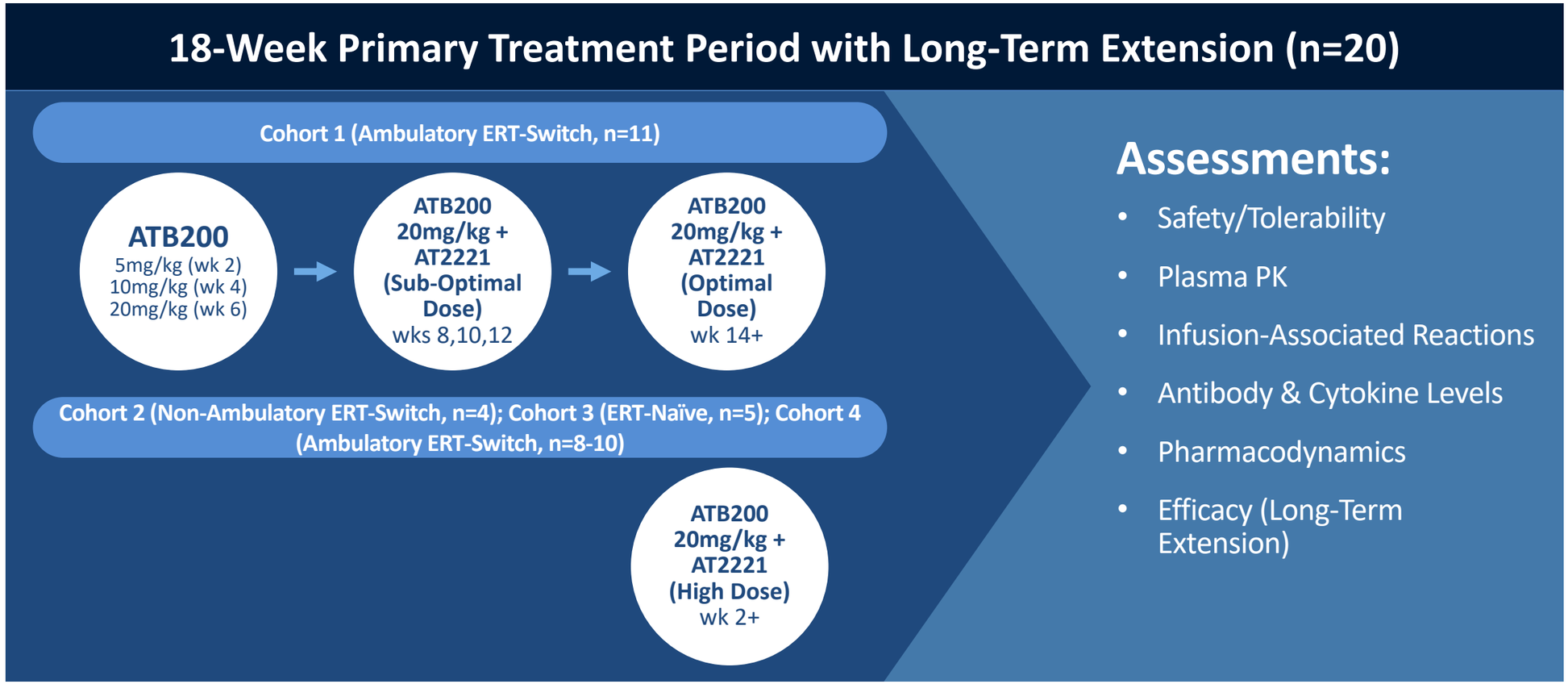


M6P=mannose-6-phosphate; PC=pharmacologic chaperone.

1. Gotschall R et al. *Mol Genet Metab*. 2015;114(2):S49. Abstract 94. 2. Khanna R et al. Presented at: the 12th Annual WORLD*Symposium*™; February 29-March 4, 2016; San Diego, CA, USA

ATB200-02 Study Design (NCT02675465)

Phase 1/2 Clinical Study to Evaluate Safety, Tolerability, Pharmacokinetics (PK), and Pharmacodynamics (PD) of ATB200 + Chaperone (AT-GAA) at 16 Sites in 5 Countries



Cohort 1: Ambulatory ERT-Switch 2-6 yrs. on SOC, n=11; Cohort 4: Ambulatory ERT-Switch >7 yrs. On SOC , Planned n=8-10

Baseline Characteristics (N=20)

Patients Enrolled Across Three Cohorts are Representative of the Overall Late-Onset Pompe Population, with Significant Impairment at Baseline

	Cohort 1 ERT-Switch (N=11[#])	Cohort 2 ERT-Switch Non-ambulatory (N=4)	Cohort 3 ERT-Naïve (N=5)
Age, years, mean (min, max)	49.4 (28, 66)	36.0 (18, 56)	49.4 (24, 65)
Sex, M:F	9:2	3:1	1:4
Time on alglucosidase alfa, years, mean (SD)	4.8 (1.42)[a]	8.9 (3.8)	-
6MWT, meters, mean (SD)	392.0 (93.4)	NA	399.5 (83.5)
FVC Upright, % predicted, mean (SD)	52.3 (13.2)	NA	53.4 (20.3)

6MWT=6-Minute Walk Test; FVC=forced vital capacity; LOPD=late-onset Pompe disease; NA=not applicable; SD=standard deviation.
[a]Cohort 1 patients were required to have been on alglucosidase alfa for 2-6 years at baseline. # One Cohort 1 patient discontinued after 18 weeks due to burden of travel

6-Minute Walk Test

6MWT Improved for Both ERT-switch Ambulatory and ERT-naive Patients at Month 6 with Continued Benefit Observed out to Month 18

All results are mean (SD), meter	Baseline	Change From Baseline		
		Month 6	Month 12	Month 18
Cohort 1 ERT-Switch Ambulatory	n=10	n=10	n=10	n=9[a]
	397.2 (96.8)	**+23.9** (52.2)	**+42.2** (46.5)	**+51.7** (45.9)
Cohort 3 ERT-Naive	n=5	n=5	n=5	n=5
	399.5 (83.5)	**+41.8** (29.4)	**+63.1** (29.1)	**+49.0** (28.3)

- 6MWT increased in 7/10, 9/10, and 9/9 ERT-switch patients at Months 6, 12, and 18, respectively
- 6MWT increased in 5/5, 5/5, and 5/5 ERT-naive patients at Months 6, 12, and 18, respectively
- Timed motor function tests were consistent with 6MWT

6MWT=6-minute walk test; ERT=enzyme replacement therapy; SD=standard deviation. [a]Data for one patient is pending (visit had not occurred at time of interim data cut).

6-Minute Walk Test Patient-Level Data – Cohort 1 ERT-Switch (n=10)

6MWT Improved for ERT-Switch Patients at Months 6, 9 and 12 With Continued Benefit Observed Out to Month 18

6-Minute Walk Test (m)

ID	Baseline	Change From Baseline		
		Month 6	Month 12	Month 18
1052	544	+51	+112	+76
1252	379	+125	+103	+147
1251	339	+21	+73	+92
1751	332	+8	+45	+29
1201	456	-5	+41	+29
1451	500	+55	+33	+24
1051	220	+29	+30	+15
1053	410	+38	+22	+50
1701	464	-4	+13	+3
1601	328	-78	-50	N/A
Mean (SD)	**397.2** (96.8)	**+23.9** (52.2)	**+42.2** (46.5)	**+51.7** (45.9)

- 6MWT increased in 7/10, 9/10, and 9/9 ERT-switch patients at Months 6, 12, and 18, respectively

N/A = data not available (patient has not reached 18 month time point)

Amicus Therapeutics

6-Minute Walk Test Patient Level Data – Cohort 3 ERT-Naïve (n=5)

All Five ERT-Naive Patients Showed Increases in 6MWT Distance Out to Month 18

6-Minute Walk Test (m)

ID	Baseline	Change From Baseline		
		Month 6	Month 12	Month 18
3551	480	+41	+95	+82
3552	384	+62	+79	+74
3051	460	+79	+78	+43
3554	406	+14	+33	+33
3553	267	+13	+31	+14
Mean (SD)	399.5 (83.5)	+41.8 (29.4)	+63.1 (29.1)	+49.0 (28.3)

6MWT increased in 5/5, 5/5, and 5/5 ERT-naive patients at Months 6, 12, and 18, respectively

N/A = data not available (patients have not reached 18 month time point)

Amicus Therapeutics

Timed Motor Function Tests

Improvement in Other Motor Function Tests was Generally Consistent for Both ERT-switch and ERT-naive Patients over 18 months

	Test	Baseline, mean (SD)	Change From Baseline, mean (SD)		
			Month 6	Month 12	Month 18
Cohort 1 ERT-Switch Ambulatory		n=10	n=10	n=10	n=9
	Timed Up and Go, sec	**10.5** (6.6)	**−1.8** (3.5)	**-1.5** (2.8)	**-2.4** (3.3)
	GSGC Score	**12.6** (4.8)	**+0.1** (3.9)	**-0.3** (4.1)	**-0.7** (3.2)
Cohort 3 ERT-Naive		n=5	n=5	n=5	n=5
	Timed Up and Go, sec	**9.4** (2.3)	**−1.0** (1.1)	**-0.8** (2.5)	**+1.9*** (7.6)
	GSGC Score	**12.2** (3.6)	**−1.8** (3.8)	**-0.3** (1.9)	**-1.2** (2.1)

**one subject had difficulty with this assessment at month 18 (fall/hernia week prior to assessment) and the median CFBL was -1.5 seconds*

GSGC=Gait, Stairs, Gowers, Chair. GSGC is an observer-rated combined score of 4 motor function assessments: Gait (10-meter walk), 4-Stair Climb, Gowers (Stand From Floor), and Rising From Chair. Each test is rated on a scale of 1 (normal) to 7 (cannot perform, max score 6 for rising from chair). Total scores range from 4 to 27.

Manual Muscle Strength Testing: Cohorts 1, 2 and 3

Increases Were Observed in Manual Muscle Strengths in All Patients Out to Month 18.

	Body Area	Baseline		Change From Baseline					
				Month 6		Month 12		Month 18	
		mean (SD)	n	mean (SD)	n	mean (SD)	n	mean (SD)	n
ERT-switch Ambulatory	**Total Body** Max score 80	**66.4** (8.1)	10	**+2.5** (3.2)	9	**+3.3** (3.4)	9	**+4.5** (3.2)	9
ERT-switch Non-Ambulatory	**Upper Body** Max score 40	**13.3** (12.2)	3[b]	**+4.5** (0.7)	2[bc]	**+2.7** (2.3)	3[b]	**+4.3** (3.5)	3[b]
ERT-Naive	**Total Body** Max score 80	**66.9** (3.7)	5	**+0.3** (2.8)	5	**+1.1** (3.1)	5	**+2.0** (2.9)	4[d]

ERT=enzyme replacement therapy; SD=standard deviation. [a]Measured via the Medical Research Criteria (MRC) scale; [b]Baseline data missing for 1 patient; [c]One patient did not complete Month 6 assessment; [d]Manual muscle testing not completed for one patient; [e]Measured via hand-held dynamometer.

Quantitative Muscle Strength Testing: Cohorts 1, 2 and 3

Increases were Observed in Quantitative Muscle Strength (Dynamometry) in All Cohorts Out to Month 18

All results are mean (SD), lbs	Baseline		Change From Baseline					
			Month 6		Month 12		Month 18	
	mean (SD)	n	mean (SD)	n	mean (SD)	n	mean (SD)	n
Cohort 1 ERT-Switch Ambulatory	33.0 (11.5)	10	**-0.7** (7.0)	10	**+ 0.7** (7.0)	10	**+1.3** (8.6)	9
Cohort 2 ERT-Switch Nonambulatory	6.5(7.0)	4	**+1.6** (4.9)	4	**+3.3** (4.0)	4	**+3.6** (2.8)	3*
Cohort 3 ERT-Naive	21.5(6.5)	5	**+0.9** (2.5)	5	**-0.1** (4.1)	5	**+1.8** (8.6)	5

** QMT not performed for one patient at M18*

Amicus Therapeutics

Sitting Forced Vital Capacity (FVC, % Predicted)

FVC Increased In ERT-Naïve Patients and was Generally Stable in ERT-Switch Patients

	Baseline, mean (SD)	Change From Baseline, mean (SD)		
		Month 6	Month 12	Month 18
Cohort 1 ERT-Switch Ambulatory	n=9[a]	n=9[a]	n=9[a]	n=8[a,b]
	52.6 (14.7)	**−1.3** (4.1)	**−3.3** (6.1)	**−3.7** (7.0)
Cohort 3 ERT-Naive	n=5	n=5	n=5	n=5
	53.4 (20.3)	**+4.2** (5.6)	**+4.4** (8.6)	**+5.0** (2.9)

ERT=enzyme replacement therapy; SD=standard deviation.
[a]Baseline FVC not available for 1 patient in Cohort 1; [b]FVC for one patient in Cohort 1 pending (visit had not occurred at time of interim data cut). .

Other Pulmonary Function Tests: MIP and MEP

MIP was stable and MEP increased in ERT-switch patients;
MIP and MEP increased in ERT-naive patients

	Assessment	Baseline, mean (SD)	Change From Baseline, mean (SD)		
			Month 6	Month 12	Month 18
Cohort 1 ERT-Switch Ambulatory		n=10	n=10	n=10	n=9[a]
	MIP	**35.7** (11.0)	**+0.3** (4.6)	**0.0** (3.2)	**−2.8** (4.4)
	MEP	**72.6** (32.6)	**+16.1** (42.1)	**+28.6** (44.0)	**+30.2** (43.0)
Cohort 3 ERT-Naive		n=5	n=5	n=5	n=5
	MIP	**32.6** (18.5)	**+11.0** (5.0)	**+5.2** (12.2)	**+6.2** (11.5)
	MEP	**60.6** (8.3)	**−0.4** (12.4)	**+8.6** (16.3)	**+9.8** (19.6)

ERT=enzyme replacement therapy; MEP=maximal expiratory pressure; MIP=maximal inspiratory pressure; SD=standard deviation.
MIP and MEP measured in centimeters of water.
[a]Data for one patient in Cohort 1 pending (visit had not occurred at time of interim data cut).

Fatigue Severity Scale (FSS)

All Cohorts were Significantly Impacted by Fatigue at Baseline and Demonstrated a Mean Improvement in Fatigue

	Baseline, mean (SD)	Change From Baseline, mean (SD)		
		Month 6	Month 12	Month 18
Cohort 1 ERT-Switch Ambulatory	n=10	n=10	n=10	n=9
	53.5 (7.7)	**−8.0** (10.7)	**−8.0** (6.5)	**−3.8** (12.2)
Cohort 2 ERT-Switch Nonambulatory	n=4	n=4	n=4	n=3
	42.3 (14.6)	**+2.3** (8.7)	**−12.5** (10.0)	**−13.3** (2.1)
Cohort 3 ERT Naive	n=5	n=5	n=5	n=5
	39.2 (12.7)	**−5.2** (11.7)	**−7.2** (7.5)	**−2.0** (7.5)

ERT=enzyme replacement therapy; SD=standard deviation.

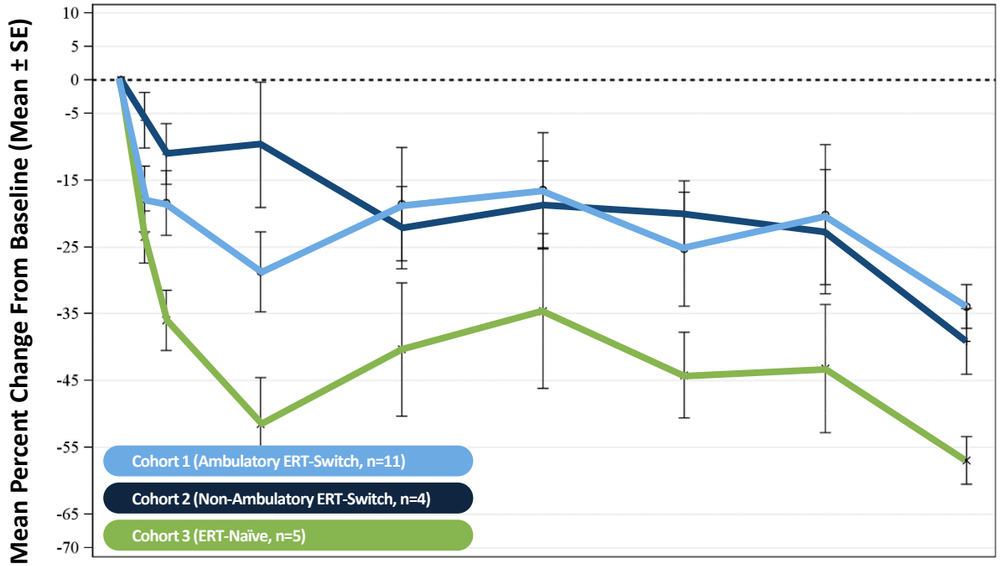
1. Grace J et al. *Parkinsonism Relat Disord*. 2007;13(7):442-445.

FSS consists of 9 questions, each scored on a scale from 1 to 7. Total scores range from 9 to 63, with higher values representing higher levels of fatigue due to the disease condition. The normative value in the healthy population is ~21.[1]

CK and Hex4 Biomarkers

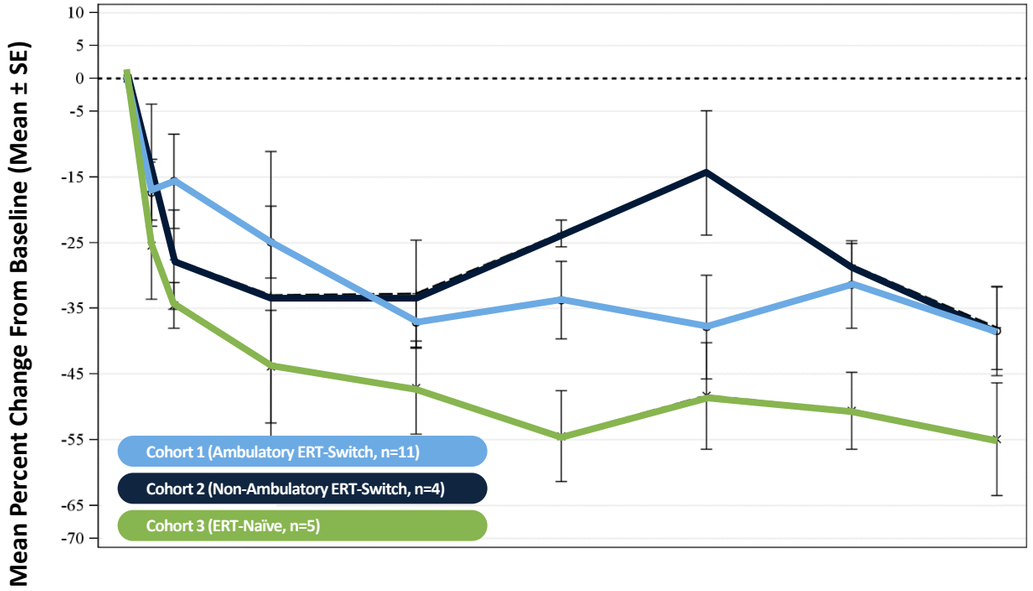
All Cohorts Demonstrated Persistent Improvement in Biomarkers of Muscle Damage (CK) and Disease Substrate (Hex4) For Up To 18 Months

Percent Change from Baseline for CK



Visit	BL	WK 2	WK 4	M3	M6	M9	M12	M15	M18
N	11,4,5	11,4,5	10,4,4	8,3,5	10,4,5	10,4,5	8,1,2	9,4,3	8,3,3

Percent Change from Baseline for Hex 4



Visit	BL	WK 2	WK 4	M3	M6	M9	M12	M15	M18
N	11,4,5	11,4,5	11,4,5	11,4,5	10,4,5	9,4,5	7,1,2	10,4,5	9,2,3

CK=creatine kinase; Hex4=urine hexose tetrasaccharide.
Missing values either unable to be analyzed or not yet analyzed.

Safety Summary

Safety data (N=20) for AT-GAA Show that AEs Have Been Generally Mild and Transient with Very Low Rates of IARs (<1%) after 890+ Total Infusions Across All Cohorts

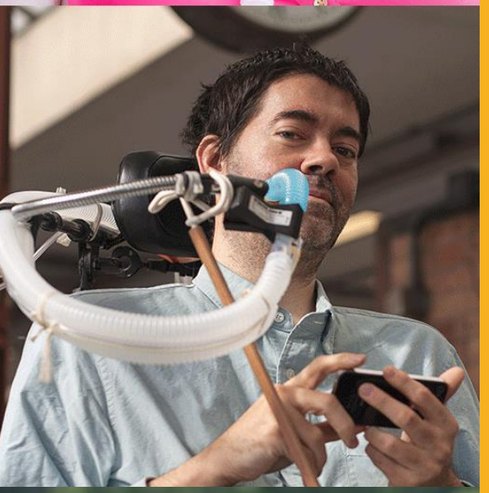
- AEs were generally mild and transient
  - The most common treatment-emergent AEs[a] by decreasing frequencies were nasopharyngitis (10/20); fall (9/20); abdominal pain[b] and diarrhea (8/20); upper respiratory tract infection (7/20); arthralgia, nausea, fatigue, pain in extremities, and myalgia (6/20); and headache, tremor, oropharyngeal pain, and muscle spasms (5/20)
- For SAEs, 5 events occurred in 4 patients (severity: 3 moderate, 2 mild) and were unrelated to treatment. SAEs did not lead to treatment interruption or study discontinuation.
- 7 incidents of IARs in 5 patients in 890+ infusions, which were controlled by standard medication or premedication
  - 1 IAR event each in 3 ambulatory ERT-switch patients
  - 1 IAR event in a non-ambulatory ERT-switch patient
  - 3 IAR events in a ERT-naive patient
- Longest duration of treatment is 28+ months

AE, adverse events; ERT=enzyme replacement therapy; IAR, infusion-associated reaction; SAE=serious adverse event.
[a]Number of patients experiencing the AE; [b]Includes upper and lower abdominal pain.

Conclusions at 18 Months of Treatment

- 6MWT showed continued benefit in ERT-naïve and ERT-switch patients
- Timed motor function tests were generally consistent with 6MWT results in both ambulatory cohorts
- Muscle strength increased in all cohorts, including nonambulatory ERT-switch patients
- Pulmonary function
  - FVC, MIP, and MEP generally increased in ERT-naive patients
  - FVC, MIP, and MEP were generally stable in ERT-switch patients
- Fatigue Severity Scale
  - Improvement in fatigue score was observed in all cohorts
- Biomarkers and Safety
  - CK and Hex4 levels decreased in all cohorts
  - AT-GAA (ATB200/AT2221) was generally well tolerated

6MWT=6-minute walk test; CK=creatine kinase; ERT=enzyme replacement therapy; FVC=forced vital capacity; Hex4=urine hexose tetrasaccharide; MEP=maximal expiratory pressure; MIP=maximal inspiratory pressure.



Amicus Patient Advocacy & Patient Perspectives

Jayne Gershkowitz, Chief Patient Advocate

George Fox, Pompe Caregiver to son Phoenix

Mike Stanzione, Living with Pompe

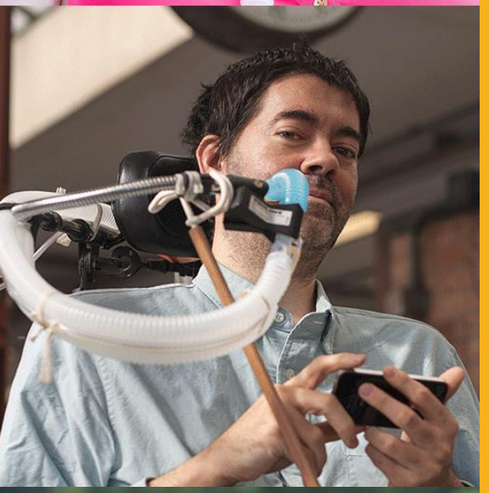
2018 Analyst Day | October 11, 2018 | New York, NY

Amicus Therapeutics is committed to improving the lives of patients and families affected by rare and orphan diseases.

- Belief Statement

What Drives Our Dedication

We are driven by the journeys, experiences, involvement, relationships and outcomes of individuals and families living with rare disease.



Personal Perspectives on Pompe

George Fox, Dad and Caregiver to son, Phoenix

2018 Analyst Day | October 11, 2018 | New York, NY

A Pompe Caregiver's Perspective: George Fox

George Fox's son Phoenix

A Pompe Caregiver's Perspective: George Fox

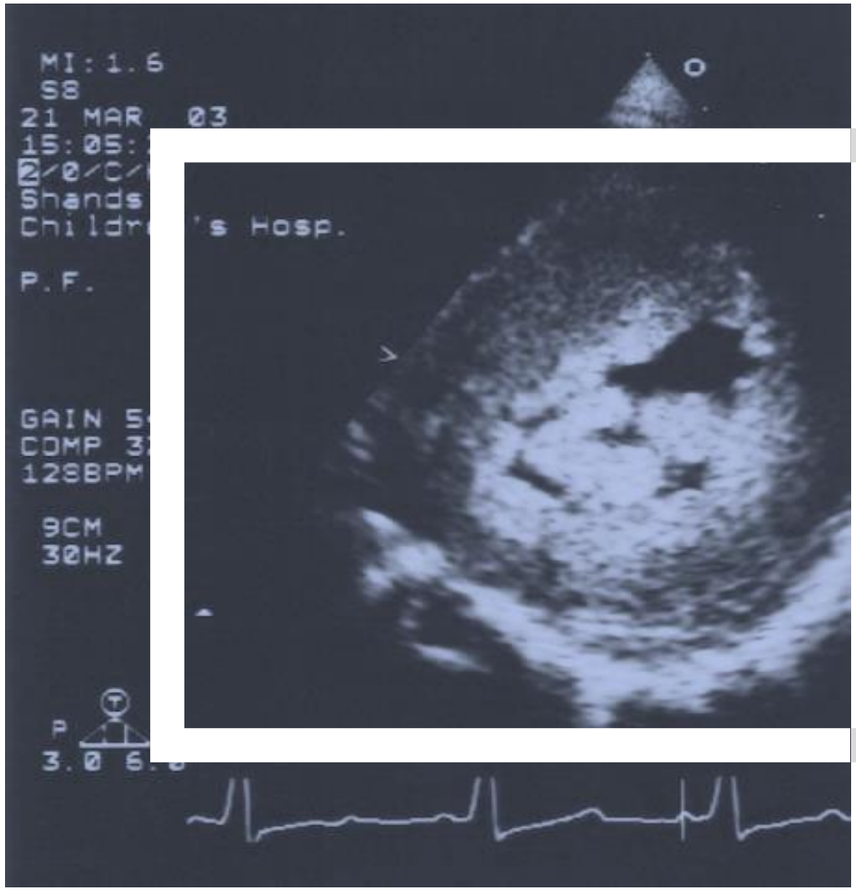
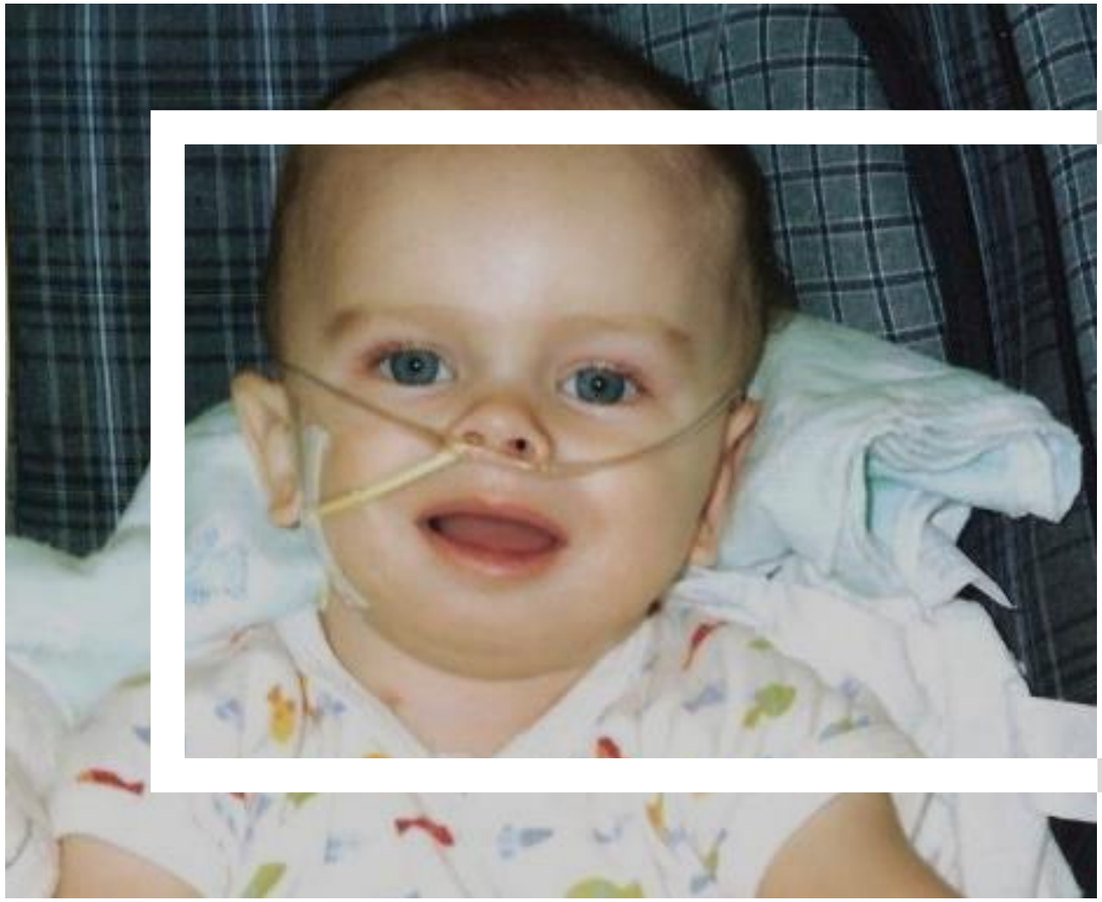


Image of Phoenix' enlarged heart in 2003 at 8 months old, a result of his Pompe disease.

A Pompe Caregiver's Perspective: George Fox

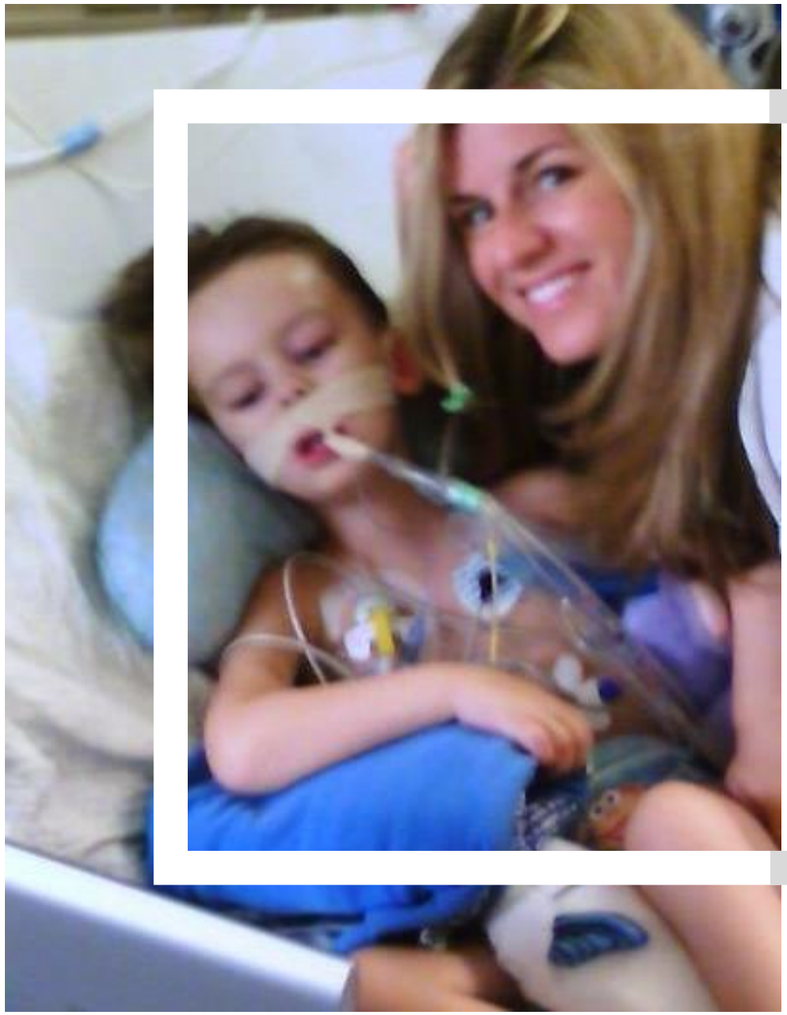


Phoenix at diagnosis.

A Pompe Caregiver's Perspective: George Fox

Phoenix swimming;
Standing in the water
for the first time

A Pompe Caregiver's Perspective: George Fox



Phoenix develops pneumonia and goes on ventilator at 3 years old

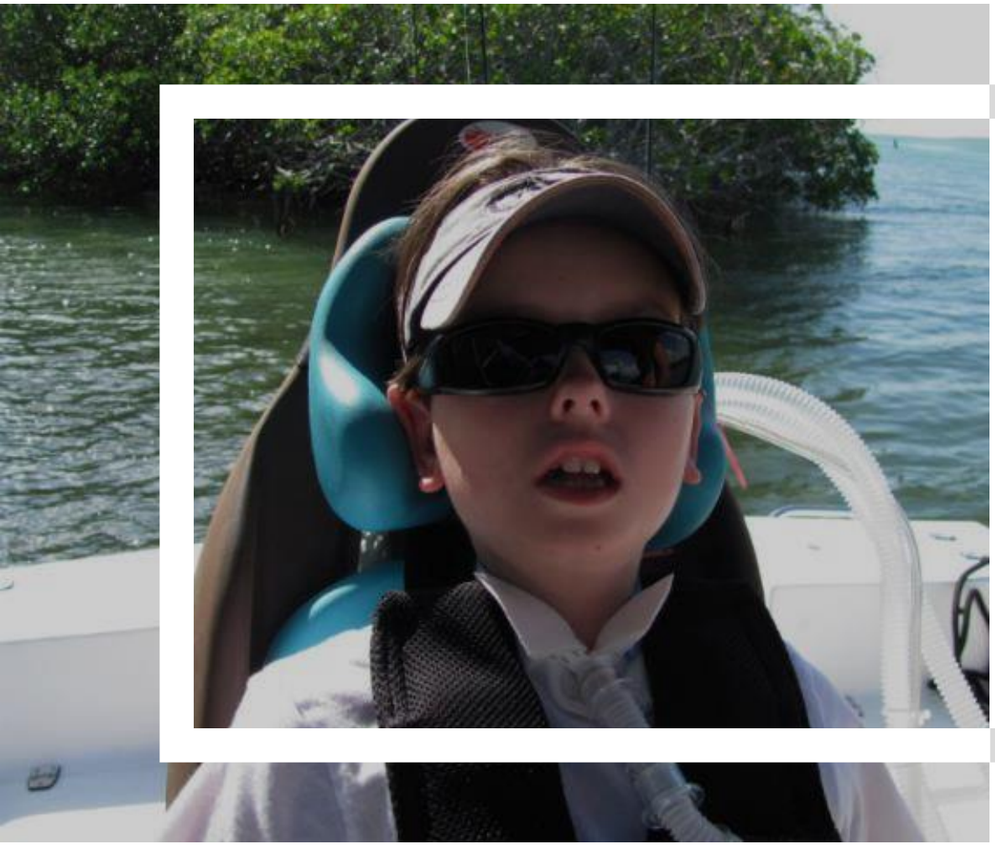
A Pompe Caregiver's Perspective: George Fox

Phoenix enjoying the zoo with Dad.

A Pompe Caregiver's Perspective: George Fox

Phoenix swimming at age 10 years old

A Pompe Caregiver's Perspective: George Fox



Phoenix fishing.

A Pompe Caregiver's Perspective: George Fox

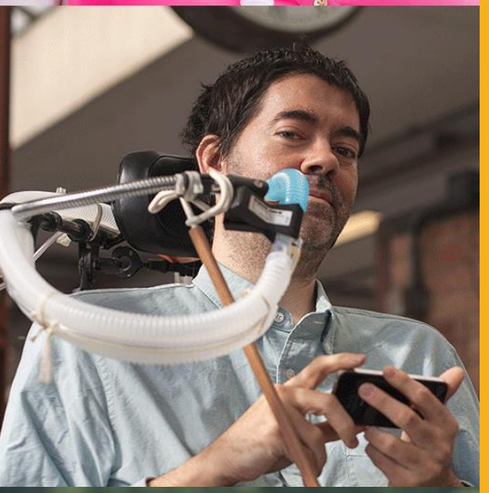
Phoenix greeting dolphins.

A Pompe Caregiver's Perspective: George Fox

Fox Family at Jerry Lewis MDA Telethon. Phoenix Fox Foundation raises money for research or clinical care for folks with neuromuscular disease

A Pompe Caregiver's Perspective: George Fox

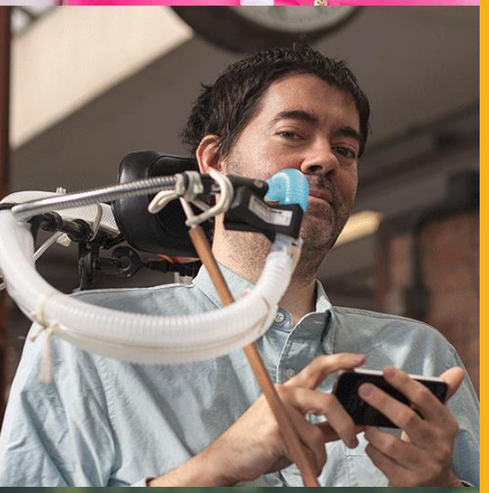
Fox family out for a run;
Phoenix at 16 years old



Personal Perspectives on Pompe

Mike Stanzione, Living with Pompe

2018 Analyst Day | October 11, 2018 | New York, NY



Potential to Shift Treatment Paradigm for Pompe Disease

John F. Crowley

2018 Analyst Day | October 11, 2018 | New York, NY

Key Activities in 2018

Significant Progress toward Clinical, Regulatory, and GMP Manufacturing Activities in 2018

Year-to-Date Progress

CLINICAL

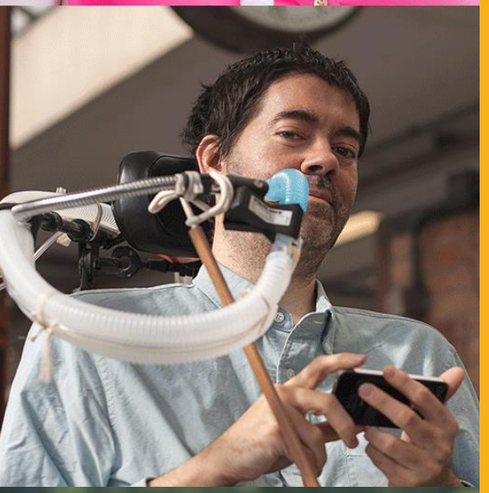
- ☑ Addt'l. Phase 1/2 ATB200-02 extension data presented at WORLD*Symposium*
- ☑ Addt'l. patients in Phase 1/2 ATB200-02 clinical study
- ☑ Initiation of retrospective natural history of ERT-treated patients
- ☑ Prospective data collection on current ERT-treated patients
- ☑ 18-month data from ATB200-02 clinical study (4Q18)
- ☐ Initiation of larger registration-directed study
- ☐ Completion of a retrospective natural history study (2H18)

REGULATORY

- ☑ EMA: Received Scientific Advice Working Party Guidance
- ☑ U.S. FDA type C meeting and U.S. update

MANUFACTURING

- ☑ Final FDA agreement on comparability between 1,000L and 250L GMP scale
- ☑ German regulatory authorities (BfArM) agreement on strategy to demonstrate comparability between 1,000L and 250L GMP scale
- ☑ Release for clinic of 1,000L GMP commercial scale material
- ☐ Announce plan for long-term commercial manufacturing

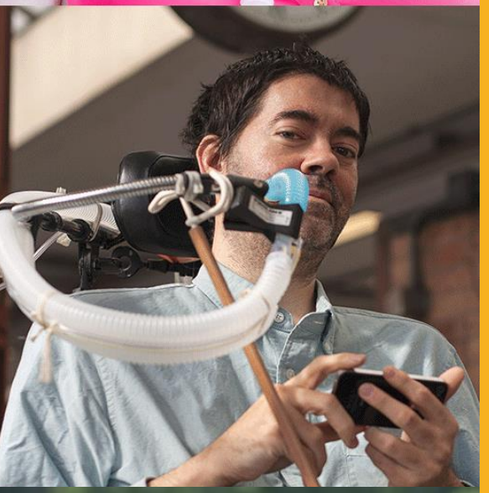


Q&A Session

John F. Crowley

Jayne Gershkowitz

Mark Roberts, M.D.



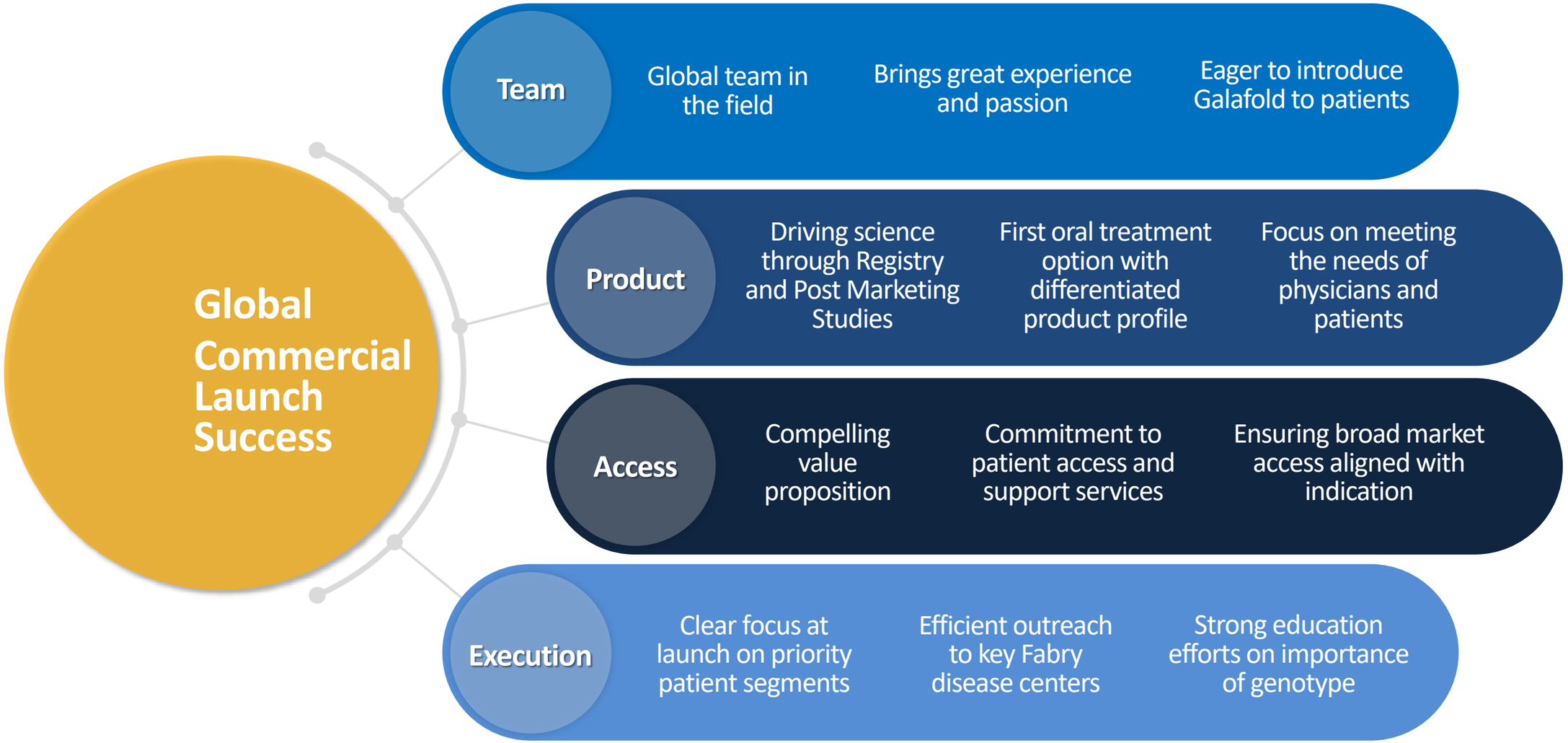
Galafold for Fabry Disease

Detlef Wolff

Bradley Campbell

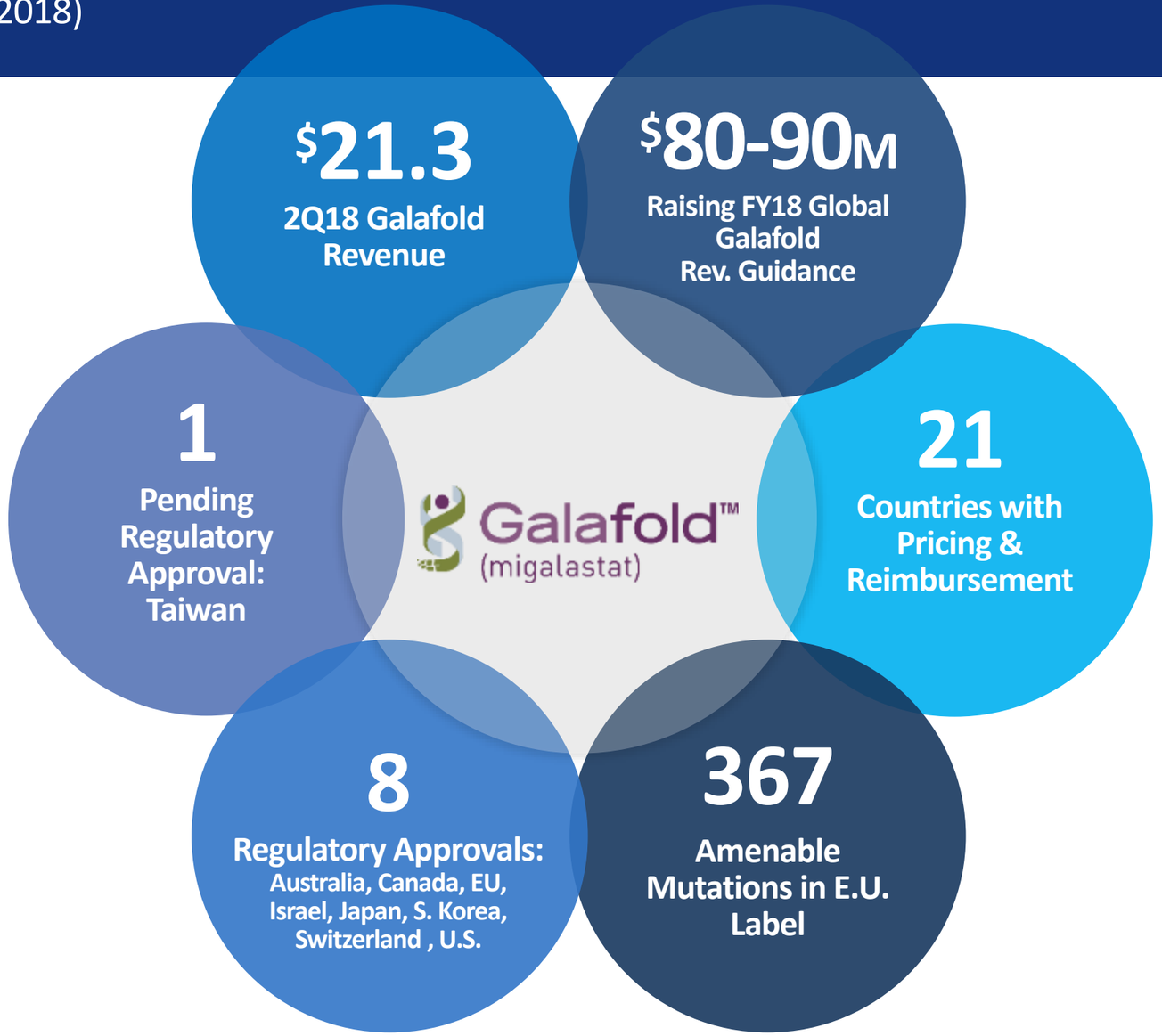
2018 Analyst Day | October 11, 2018 | New York, NY

Leveraging Our Operations Excellence



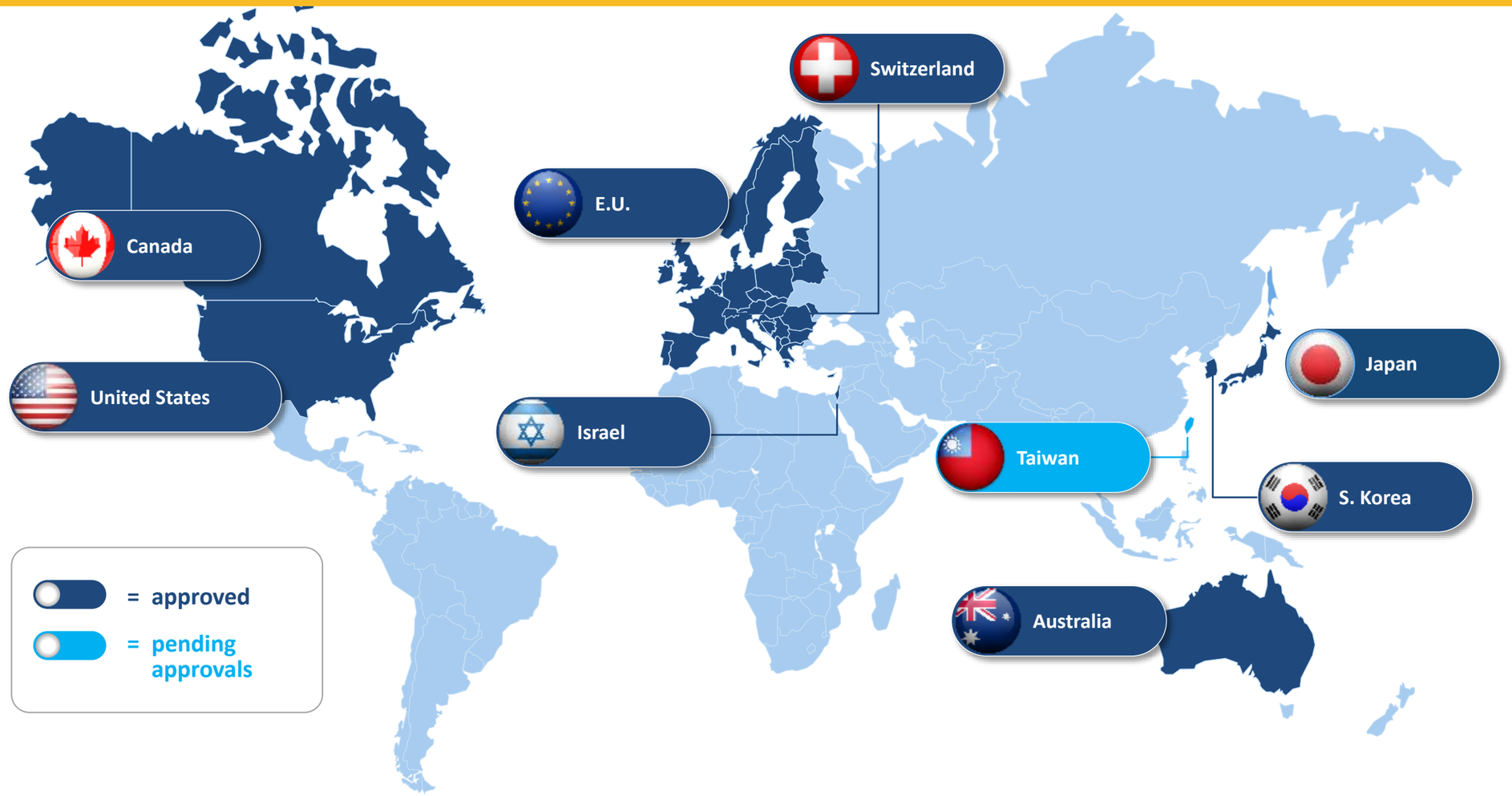
Galafold Snapshot (as of October 11, 2018)

FIRST Oral Precision Medicine for Fabry Disease Patients with Amenable Variants



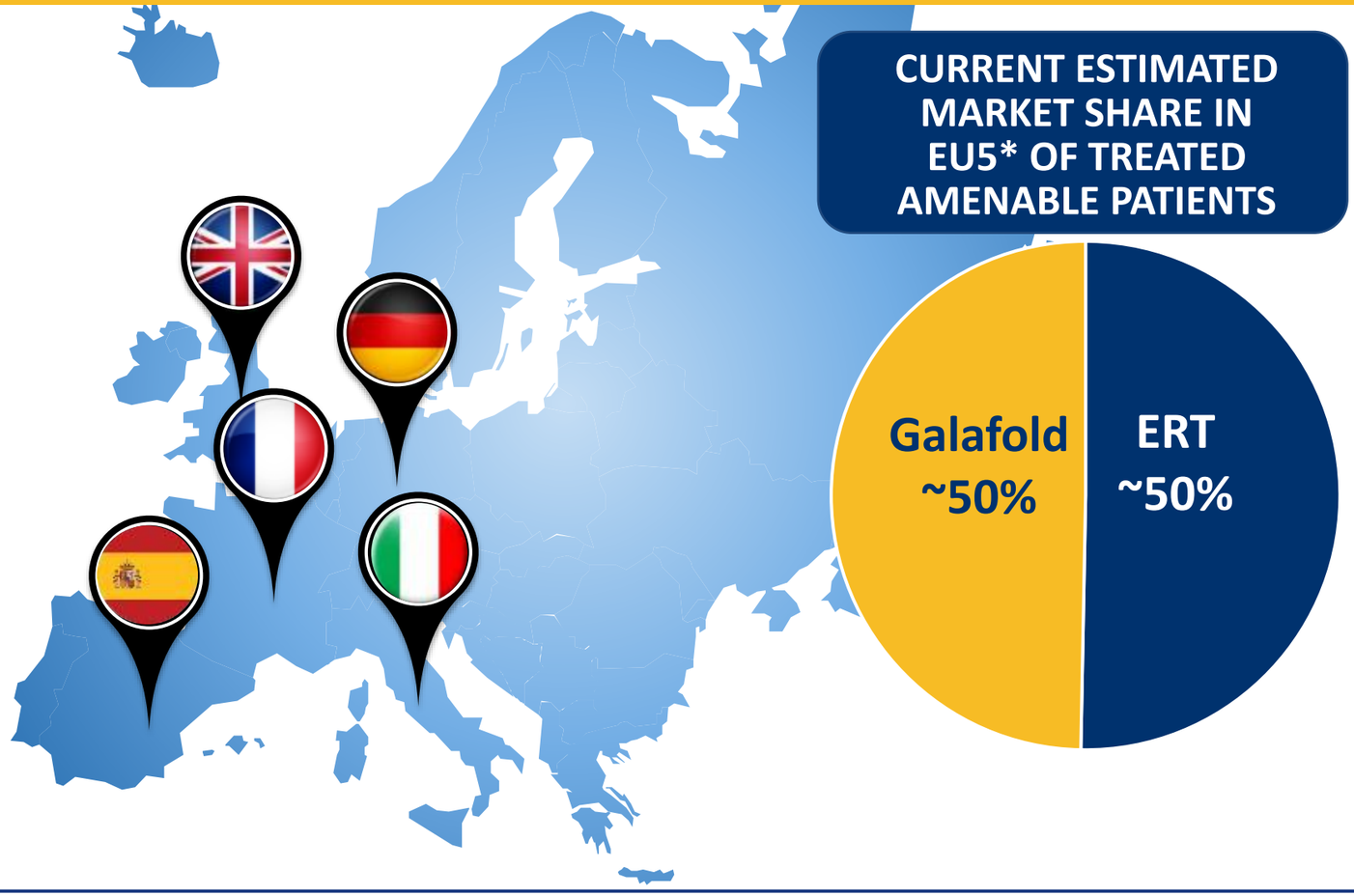
Galafold Snapshot (as of October 11, 2018)

Launched in Majority of Target Geographies with Continued Expansion into LatAm and SE Asia



International Update (as of October 11, 2018)

Continuing to Execute on Our Strategy with High Compliance and Adherence Among 500+ patients on Galafold (Ex-U.S.)



CURRENT ESTIMATED MARKET SHARE IN EU5* OF TREATED AMENABLE PATIENTS

MARKET DYNAMICS

- Continued strong uptake in ERT-switch patients
- Significant growth opportunity with diagnosed untreated patients
- Very high rates of adherence and compliance (>90%)
- Balanced mix of males and females, classic and late-onset patients
- Oral ROA allows for new ordering patterns and seasonality
- Continued strong interest from physician community
- ~150 HCPs attending upcoming Amicus Fabry Meeting in Madrid

*Market share assumptions based on estimated number of treated amenable patients in EU5 as of October 2018

Amicus Therapeutics

Japan Launch Update

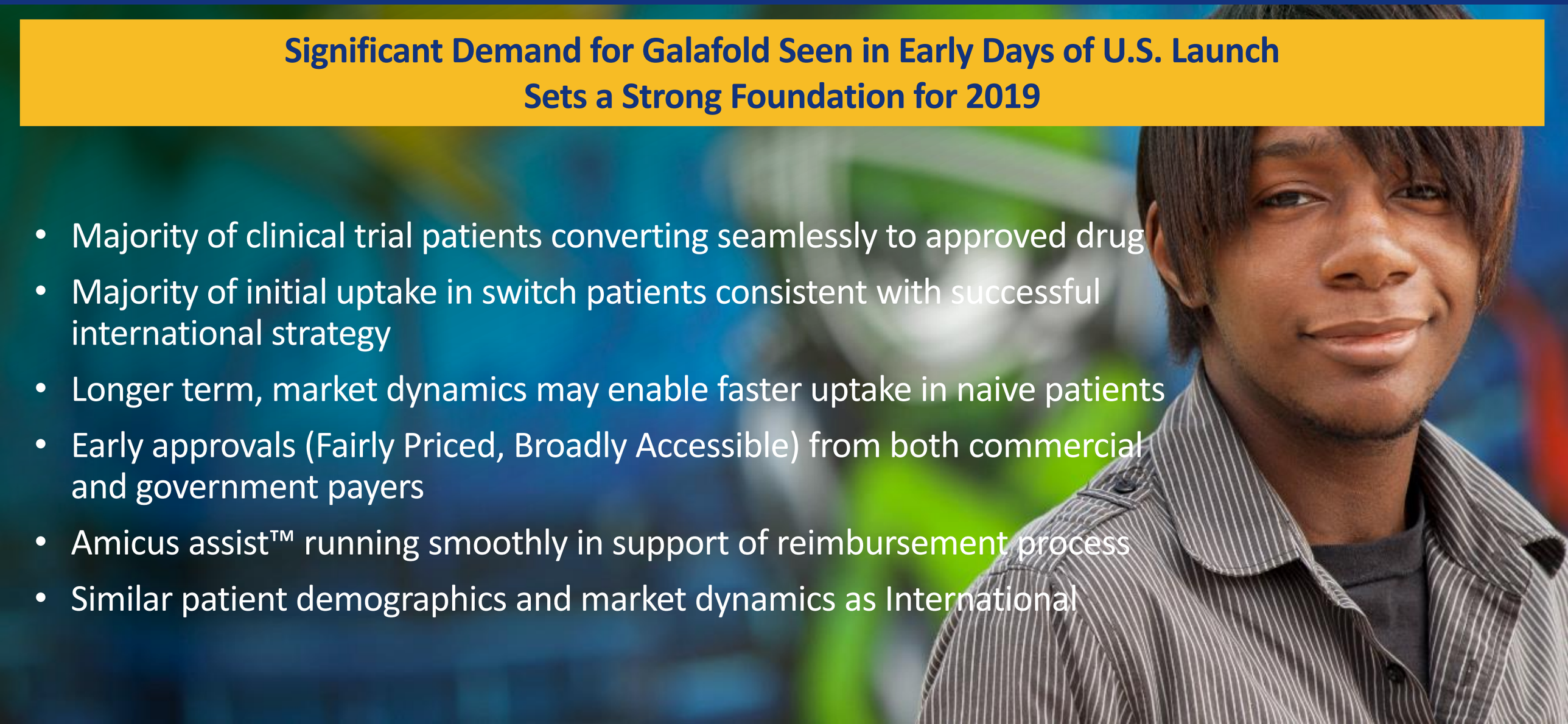
Japan Launch on Track and Setting Foundation for Significant Growth in 2019

- First commercial patients commenced treatment in late 2Q18
- Double digit number of patients now on Galafold
- ~900 patients diagnosed (>750 treated with an ERT)*
- No ERT home infusion currently available
- Broad interest from a wide set of KOLs / Treatment Centers

*Clinical & commercial, all figures approximate

U.S. Progress in First 8 Weeks of Launch

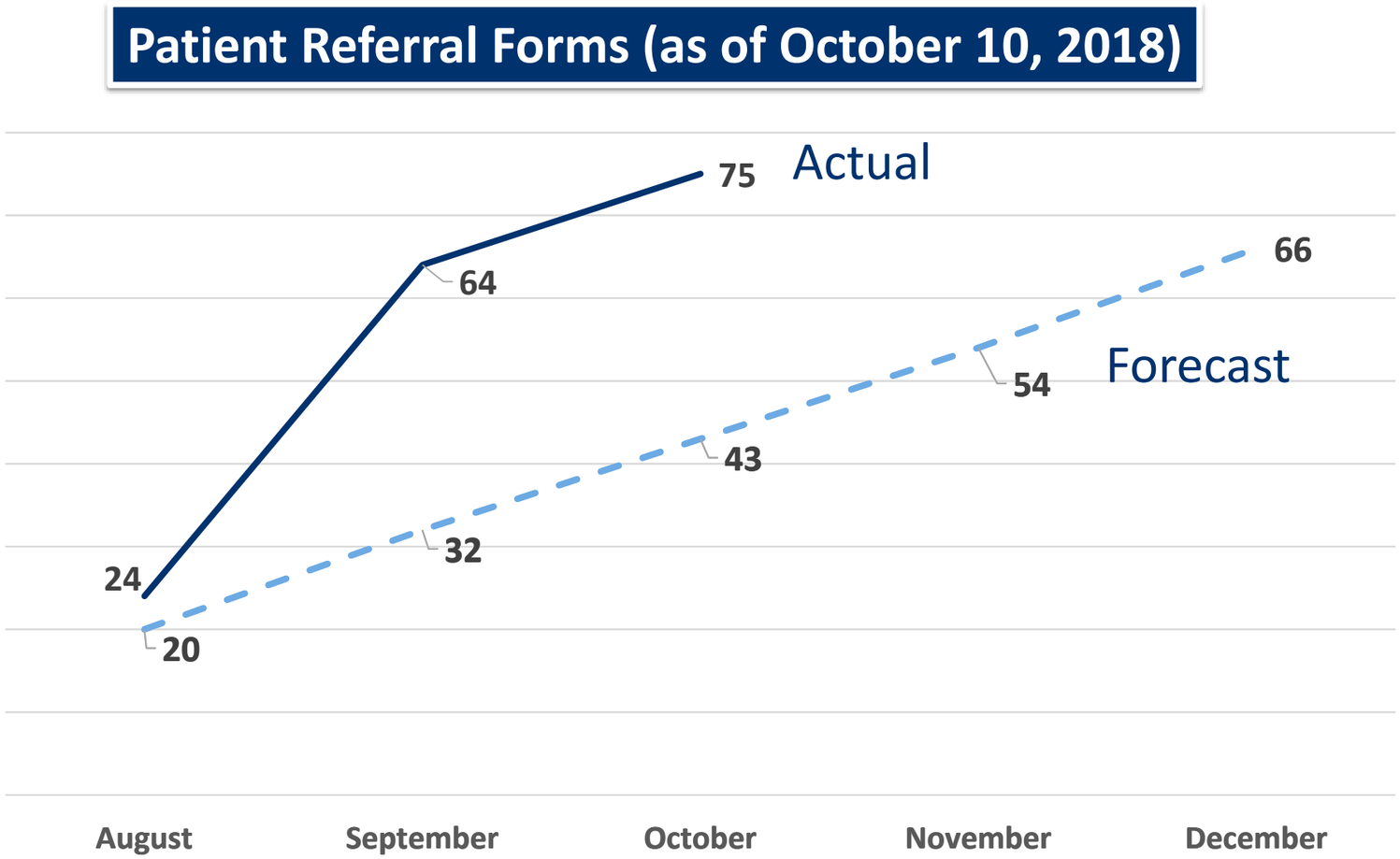
Significant Demand for Galafold Seen in Early Days of U.S. Launch Sets a Strong Foundation for 2019



- Majority of clinical trial patients converting seamlessly to approved drug
- Majority of initial uptake in switch patients consistent with successful international strategy
- Longer term, market dynamics may enable faster uptake in naive patients
- Early approvals (Fairly Priced, Broadly Accessible) from both commercial and government payers
- Amicus assist™ running smoothly in support of reimbursement process
- Similar patient demographics and market dynamics as International

Key U.S. Launch Metric

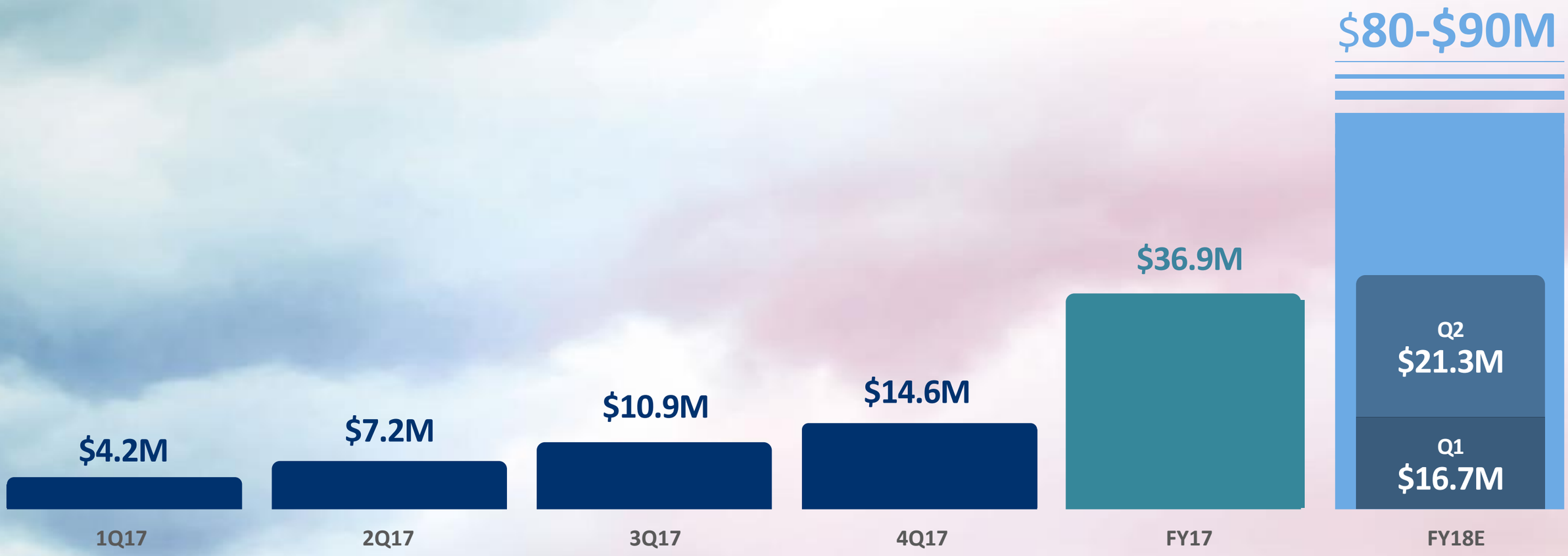
Patient Referral Forms (PRFs) Significantly Exceed Initial Full-Year Forecast 8 Weeks into Launch



- PRF rate reflects very strong demand
- Initial ~60 day average PRF to shipment time limits FY18 revenue impact
- Solid foundation for 2019

Galafold Success and FY18 Galafold Revenue Guidance

International Launch Achieved Significant Growth in 2018 and Sets Foundation for 2019



Total Amenable Patient Population ("TAPP")

Estimate based on 35% - 50% amenability

$1B+ in 2019E-2023E in Cumulative Revenue Supporting R&D Investment

2017

EU & ROW Only

TAPP: 2,000-3,000

2018

Today

WORLDWIDE*
(U.S. & Japan Added)

TAPP: 3,800-5,500

2028

Peak Potential

WORLDWIDE

Diagnosis continues at current rate

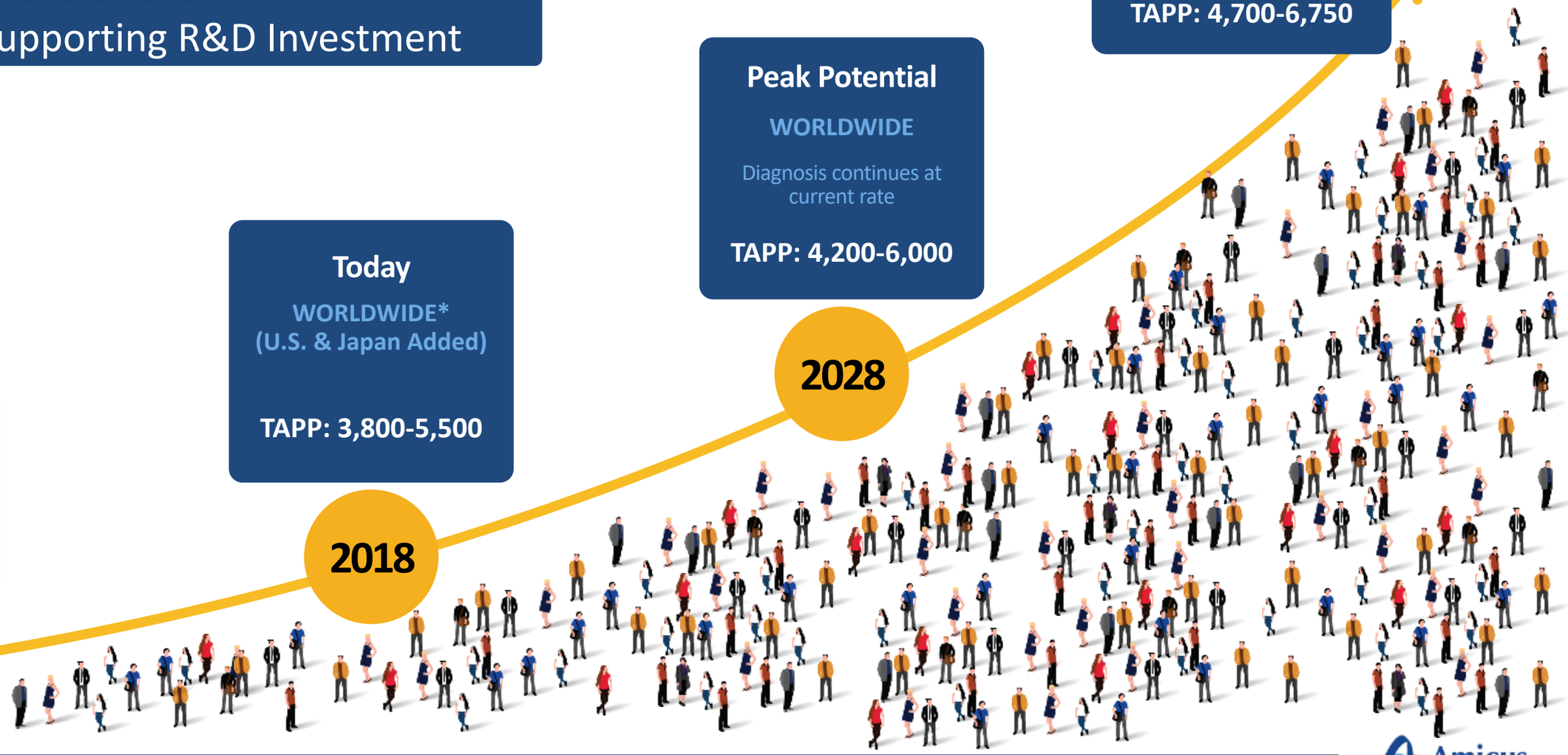
TAPP: 4,200-6,000

Upside Potential

WORLDWIDE

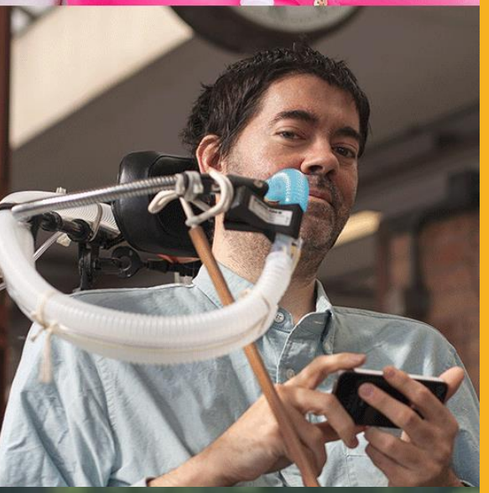
Diagnosis grows due to newborn screening in U.S. & Japan

TAPP: 4,700-6,750



*WORLDWIDE includes total amenable patient population in all Fabry ERT commercial markets today Estimated effect of newborn screening on adult diagnostic rate.

Amicus Therapeutics

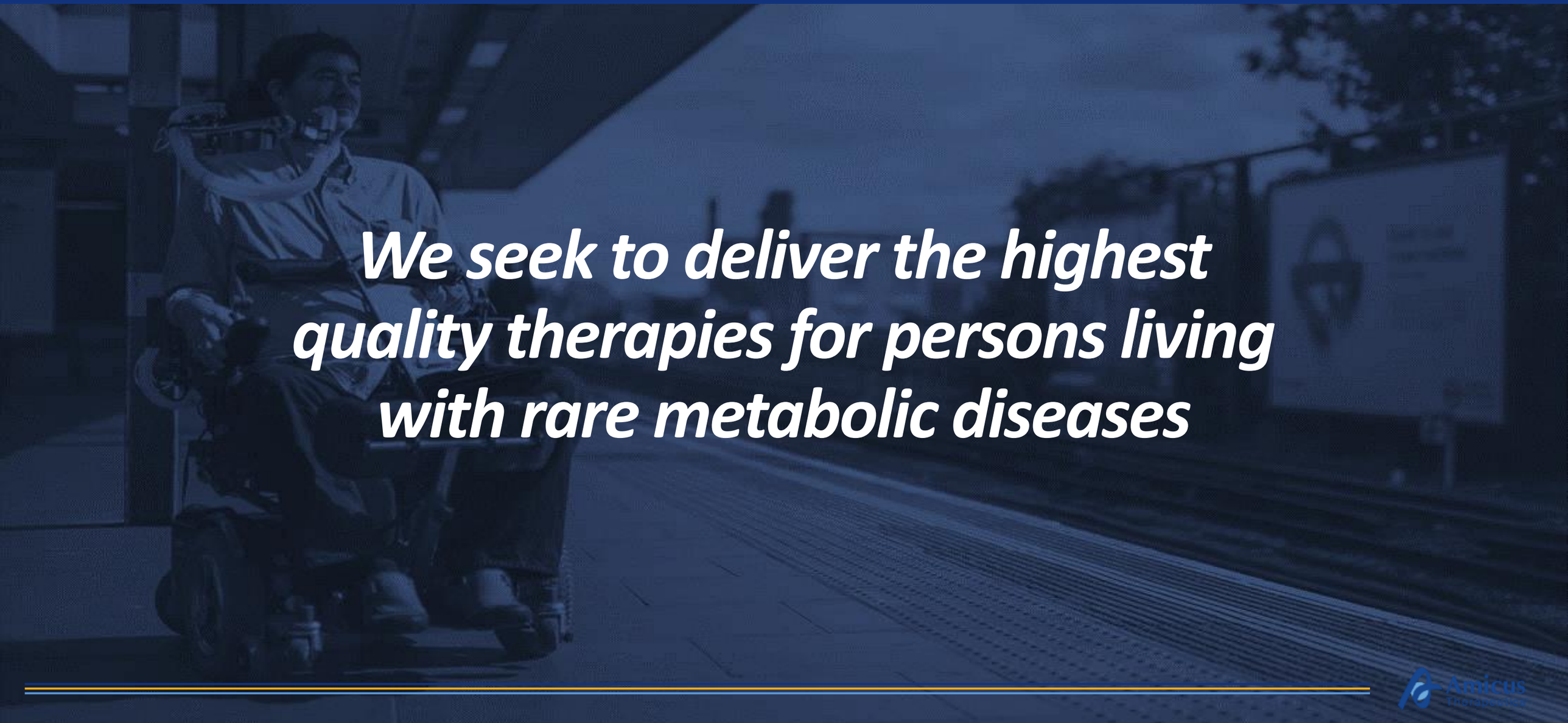


Closing Remarks

John F. Crowley

2018 Analyst Day | October 11, 2018 | New York, NY

Amicus Mission



Amicus Vision: Delivering for Patients and Shareholders

To build a top-tier, fully integrated, global biotechnology company whose medicines treat 5,000+ patients with $1B+ in worldwide sales revenue by 2023

>350 Patients* | $36.9M Global Sales

5,000 Patients* | $1B Global Sales

YE17 → 2023

*Clinical & commercial, all figures approximate

Amicus Therapeutics

2018 Key Strategic Priorities

On Track to Achieve All FIVE Key Strategic 2018 Priorities Outlined in January

1. **Double Galafold (migalastat) revenue to $80-$90M**
2. ✓ **Secure approvals for migalastat in Japan and the U.S.**
3. ✓ **Achieve clinical, manufacturing and regulatory milestones to advance AT-GAA toward global regulatory submissions and approvals**
4. ✓ **Develop and expand preclinical pipeline to ensure at least one new clinical program in 2019**
5. ✓ **Maintain financial strength**

Integrity Leadership

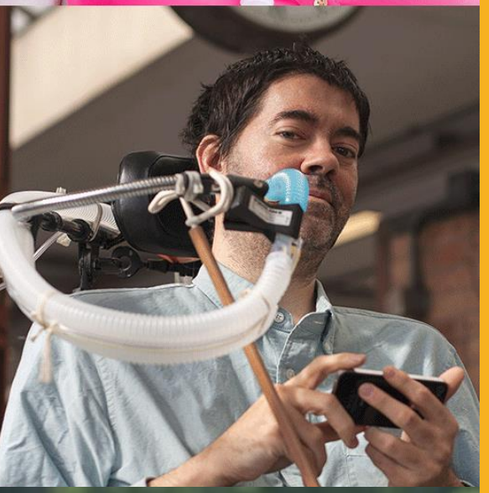
To achieve our mission for patients and shareholders, we will adhere to the highest levels of business ethics and compliance.

Persistence

"Now this is not the end. It is not even the beginning of the end. But it is, perhaps, the end of the beginning."

-Winston Churchill

Video on Persistence



Q&A Session

2018 Analyst Day | October 11, 2018 | New York, NY

Thank You

Amicus Therapeutics®