Amicus Therapeutics®

3Q18 Financial Results & Corporate Highlights

November 5, 2018

Forward Looking Statements

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, manufacturing and supply plans, financing plans, and the projected revenues and cash position for the Company. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular the potential goals, progress, timing, and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in our business, including, without limitation: the potential that results of clinical or preclinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe 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 and cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. In addition, all forward-looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2017 as well as our Quarterly Report on Form 10-Q for the quarter September 30, 2018 to be filed November 6, 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 news release to reflect events or circumstances after the date hereof.

Amicus Today

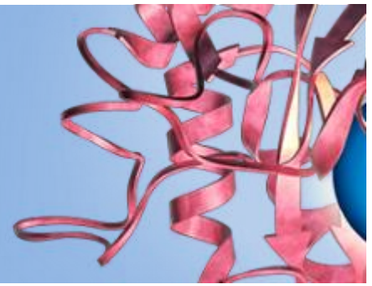
Galafold™ (migalastat)

First Oral Precision Medicine for Fabry Disease

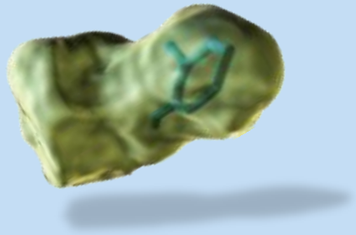
500+ EMPLOYEES globally

PORTFOLIO
of 15 programs for rare metabolic diseases

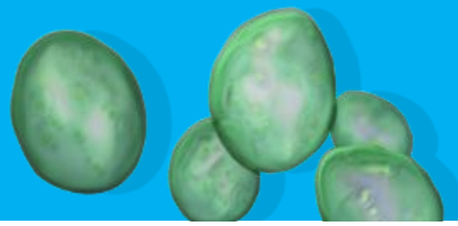
BIOLOGICS PLATFORM
Protein Engineering & Glycobiology



AT-GAA*
Investigational Therapy for Pompe Entering Phase 3



Leading Expertise in Lysosomal Storage Disorders



~$564M Cash (9/30/18)

Gene Therapy Platforms

GLOBAL FOOTPRINT in 27 countries

* AT-GAA, also known as ATB200/AT2221

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Corporate Highlights: 3Q18 and Early 4Q18

- **Well Capitalized to Advance Toward 2023 Vision: 5,000+ Patients & $1B+ in Revenue**
- **Current Cash Position is Sufficient to Fund Operations into at least 2021**
- **Galafold: International Growth and Strong U.S. Launch Momentum**
  - U.S. launch exceeding expectations following August 2018 approval; now reimbursed in 22 countries
  - 3Q18 revenue of $20.6M – on track to meet $80M-90M FY18 guidance range
  - $500M+ peak revenue potential; $1B+ cumulative revenue from 2019E-2023E to drive R&D engine
- **AT-GAA: Positive 18-month Data Presented World Muscle Society (October 2018)**
  - Highly differentiated ERT with potential to be the future standard of care
  - On track to initiate pivotal study by YE18
  - $1B+ peak revenue potential
- **NEW Gene Therapy Portfolio for 14 Rare Metabolic Diseases**
  - Industry leading Batten disease portfolio: Two clinical stage programs (CLN6 and CLN3); One preclinical (CLN8)
  - Preclinical AAV (intrathecal) gene therapy programs for 7 additional neurologic LSDs
  - Next-generation preclinical gene therapies for Fabry, Pompe, CDKL5 and one other indication
  - $1B+ peak revenue potential

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 Type C (NPC)	NCH					
Wolman Disease	NCH					
Tay-Sachs Disease	NCH					
Multiple Other CNS LSDs	NCH					
CDKL5 Deficiency Disorder Gene Therapy / ERT	PENN					
Other	PENN					

Advancing one of the **most robust rare disease portfolios** in biotechnology

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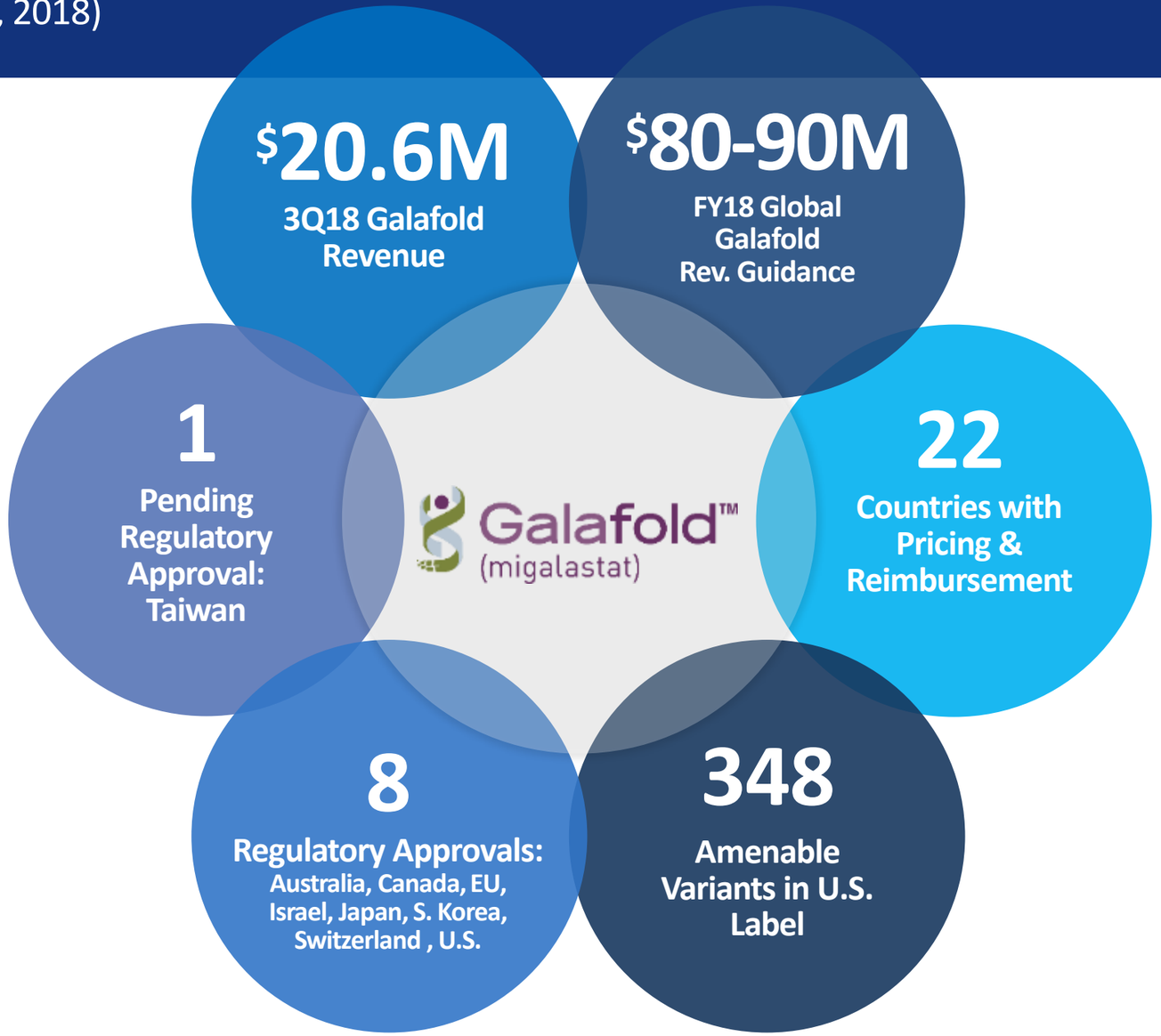
Galafold® (Migalastat) Precision Medicine for Fabry Disease

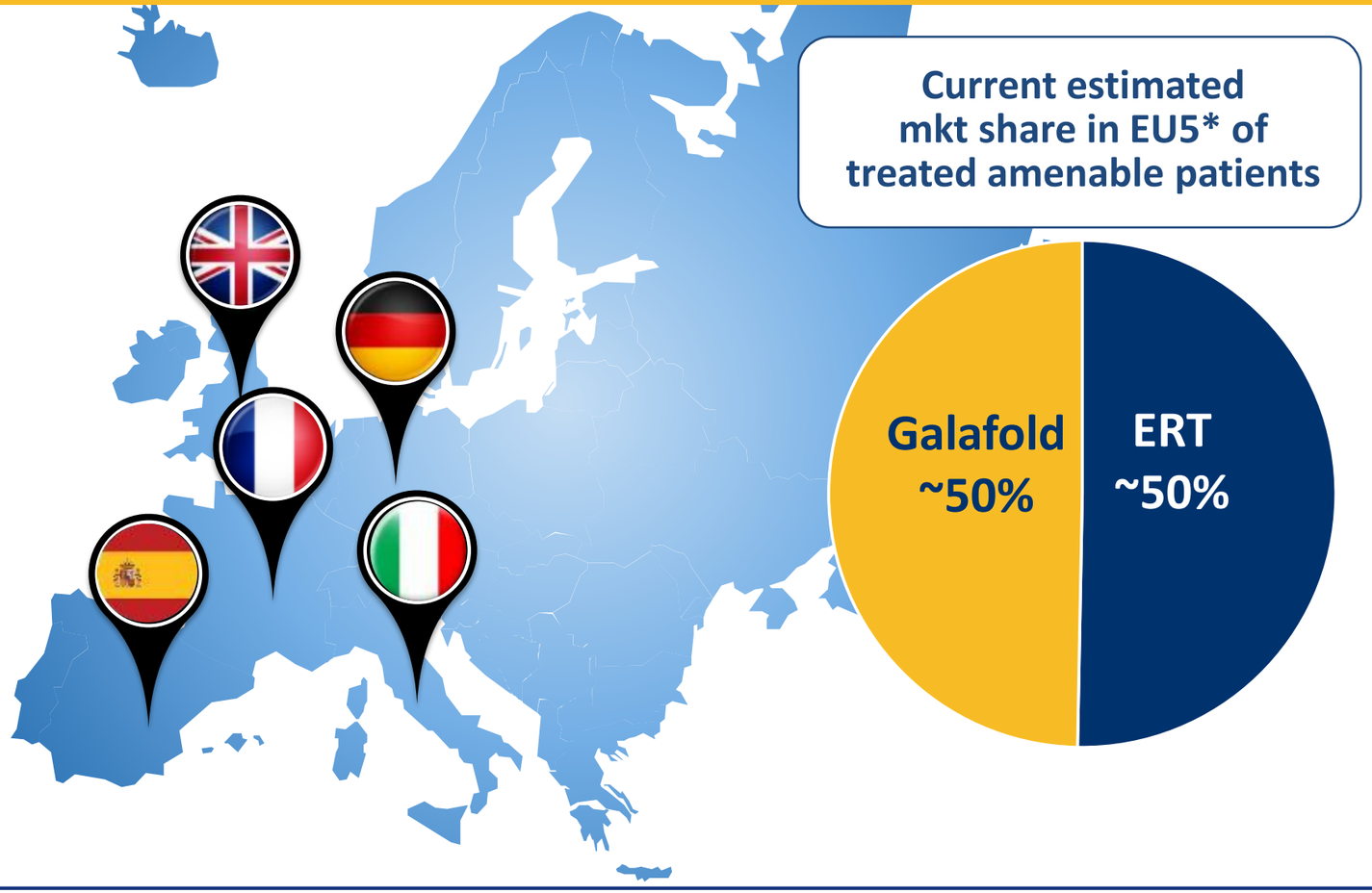
Galafold Snapshot (as of November 5, 2018)

FIRST Oral Precision Medicine for Fabry Disease Patients with Amenable Variants



International Update (as of October 31, 2018)

Continuing to Execute on Our Strategy with High Compliance and Adherence Among 500+ International Patients on Galafold



Current estimated mkt share in EU5* of treated amenable patients

MARKET DYNAMICS

- Continued strong uptake and growth in ERT-switch patients; increasing number of previously 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
- Continued high interest from physician community
- 145 HCPs attended inaugural Amicus *Fabry Connections* meeting in Madrid, Spain

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

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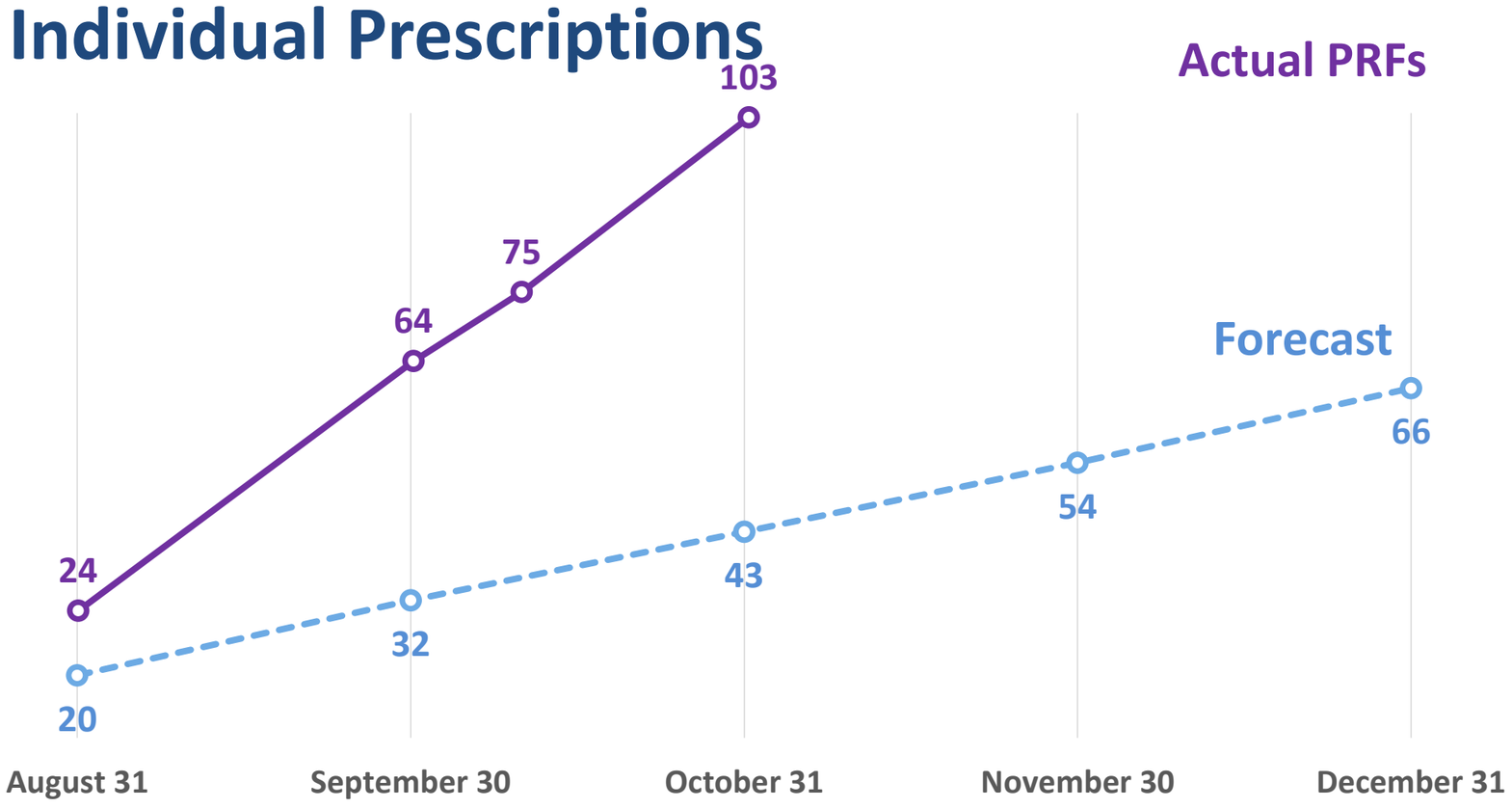
Key U.S. Launch Metric – Individual Prescriptions (Patient Referral Forms)

103 Individual Prescriptions (10/31/18) Significantly Exceeds Internal Forecast and Provides Strong Foundation for 2019

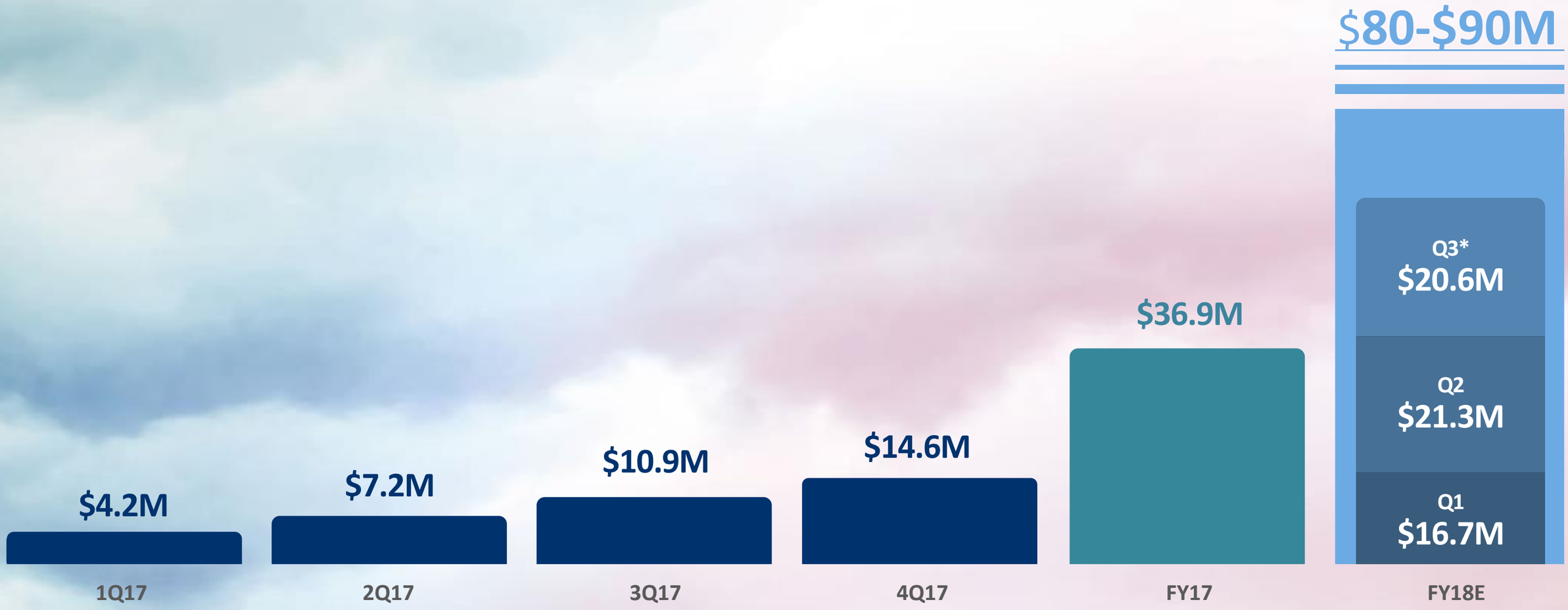


Market Dynamics

- Strong patient and physician demand
- High conversion of study patients
- Growing prescriber base of 40+ physicians
- Patient demographics in line with launch strategy
- ~60 day average PRF to shipment limits FY18 impact
- Solid foundation for 2019

Galafold Success and FY18 Galafold Revenue Guidance

On Track to Achieve Higher End of FY2018 Revenue Guidance of $80-$90M



*QoQ revenue reflects new ordering patterns

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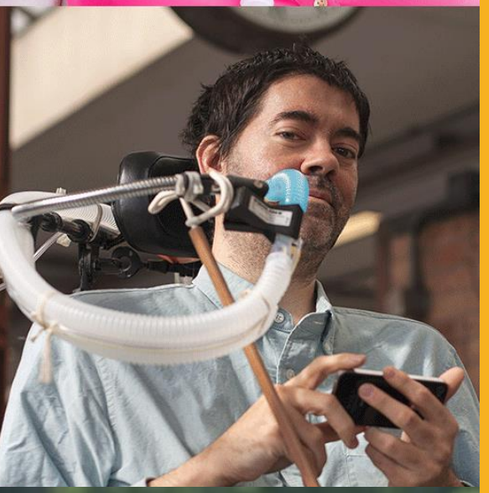

Financial Summary

3Q18 Select Financial Results

3Q18 Revenue of $20.6M Primarily from International Galafold Sales

(in thousands, except per share data)	Sept. 30, 2018	Sept. 30, 2017
Product revenue	20,596	10,874
Cost of goods sold	4,310	1,790
R&D expense	138,227*	40,641
SG&A expense	31,867	21,647
Changes in fair value of contingent consideration	1,300	(244,250)
Loss on impairment of assets	-	465,427
Loss from operations	(156,181)	(275,232)
Income tax benefit	51	164,683
Net loss	(159,163)	(111,666)
Net loss per share	(0.84)	(0.69)

*Inclusive of upfront payment of $100 million for the Celenex asset acquisition

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Financial Summary & Guidance

Strong Balance Sheet with $564M Cash at 9/30/18 - Cash Runway into at Least 2021

FINANCIAL POSITION	September 30, 2018
Cash	$564M
Debt	$319M
Cash Runway[1]	Into at least 2021
CAPITALIZATION	
Shares Outstanding[2]	189,254,341
FINANCIAL GUIDANCE	
FY18 Net Cash Spend Guidance	$190M-$210M
Galafold Revenue Guidance	$80-$90M

[1]Based on existing operating plan. [2]Includes shares from the February 2018 equity offering

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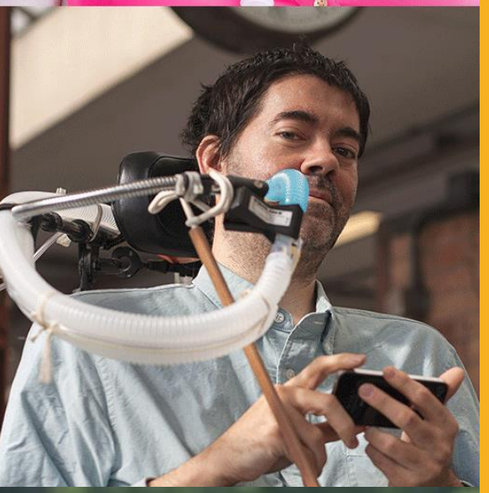


AT-GAA Novel ERT for Pompe Disease

AT-GAA 18-Month Clinical Data Summary (ATB200-02 Study)

Consistent and Durable Responses Across Key Measures of Safety, Functional Outcomes and Biomarkers in both ERT-Switch and ERT-Naïve Pompe Patients out to Month 18

- 6-minute walk test (6MWT) showed continued benefit in ERT-naïve and ERT-switch patients
- Timed motor function tests generally consistent with 6MWT results in both ambulatory cohorts
- Muscle strength increased in all cohorts, including nonambulatory ERT-switch patients
- Pulmonary function
  - Forced vital capacity (FVC), maximal inspiratory pressure (MIP), and maximal expiratory pressure (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
  - Creatine kinase (CK) and urine hexose tetrasaccharide (Hex4) levels decreased in all cohorts
  - AT-GAA (ATB200/AT2221) was generally well tolerated
  - Adverse Events Generally Mild and Transient
- Very low rates of IARs (<1%) after 890+ total infusions across all cohorts

Key Activities in 2018

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

Year-to-Date Progress

CLINICAL

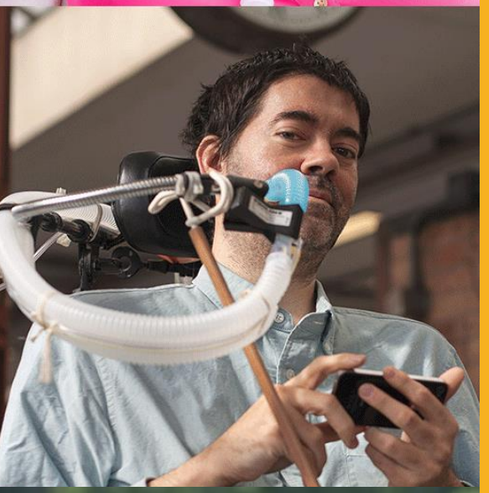
- ☑ Addt'l. Phase 1/2 ATB200-02 extension data presented at WORLD*Symposium*
- ☑ Addt'l. patients in Phase 1/2 ATB200-02 clinical study
- ☑ Initiation of retrospective natural history of ERT-treated patients
- ☑ 18-month data from ATB200-02 clinical study (4Q18)
- ☐ Initiation of larger registration-directed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

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



Gene Therapy Pipeline

Leading Gene Therapy Portfolio in Lysosomal Storage Disorders

License Through Nationwide Children's Hospital and Collaboration with Penn
Combine with Successful Amicus Development and Commercial Track Record in LSDs

- Ground-Breaking, Clinically Validated Science
- 14 Gene Therapy Programs
- Expertise and Relationships in Gene Therapy
- Compelling Data in Three Lead Batten Disease Programs; Earlier-Stage Fabry and Pompe Programs
- Leading Gene Therapy Portfolio in Lysosomal Storage Disorders

Amicus Gene Therapy Portfolio

	DISCOVERY	PRECLINICAL	PHASE 1/2	PHASE 3
CLN6 Batten Disease	NCH			
CLN3 Batten Disease	NCH			
CLN8 Batten Disease	NCH			
Fabry Gene Therapy	PENN			
Pompe Gene Therapy	PENN			
Neimann-Pick C	NCH			
Wolman Disease	NCH			
Tay-Sachs	NCH			
Multiple Other CNS LSDs	NCH			
CDKL5 Gene Therapy / ERT	PENN			
Other	PENN			

Platform Proof-of-Concept for Lead Batten Disease Programs

CLN6 and CLN3 Programs are Clinical Stage; CLN8 has Definitive Preclinical Efficacy Data in a Mouse Model of Disease – All Following Single AAV Intrathecal Administration

	PRECLINICAL MOUSE MODEL DATA						
	Storage Material & 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

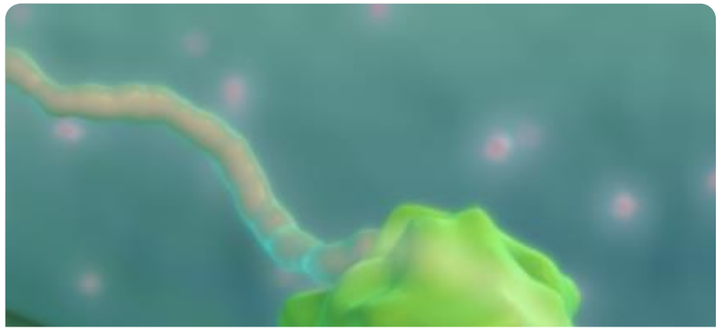
*CLN3 mouse model does not have impaired survival

Amicus Protein Engineering Expertise & Technologies for Gene Therapy

Collaboration with Penn to Enable Greater Protein Expression and Delivery at Lower Gene Therapy Doses for Fabry, Pompe, CDKL5 Deficiency Disorder and 1 Additional Indication

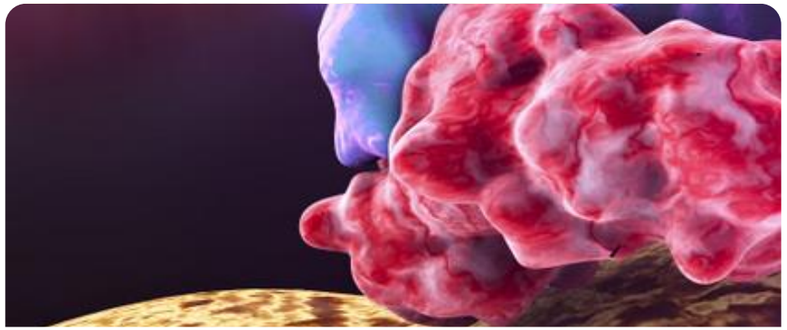
Increased Protein Expression

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



Increased Protein Secretion

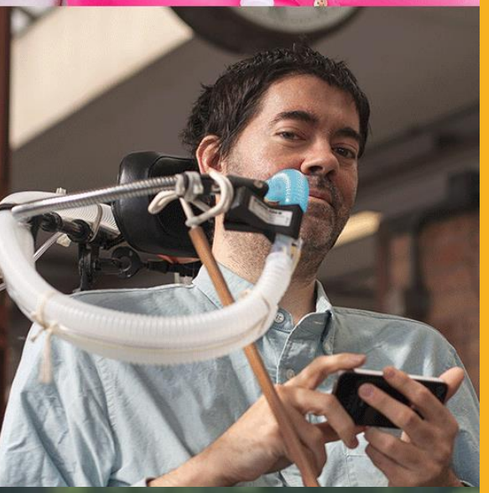
Effective signal sequences to increase protein expression & secretion



Improved Protein Targeting and Stabilization

Targeting moieties

Protein design



Closing Remarks

John F. Crowley

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**

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

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Thank You

Amicus Therapeutics®