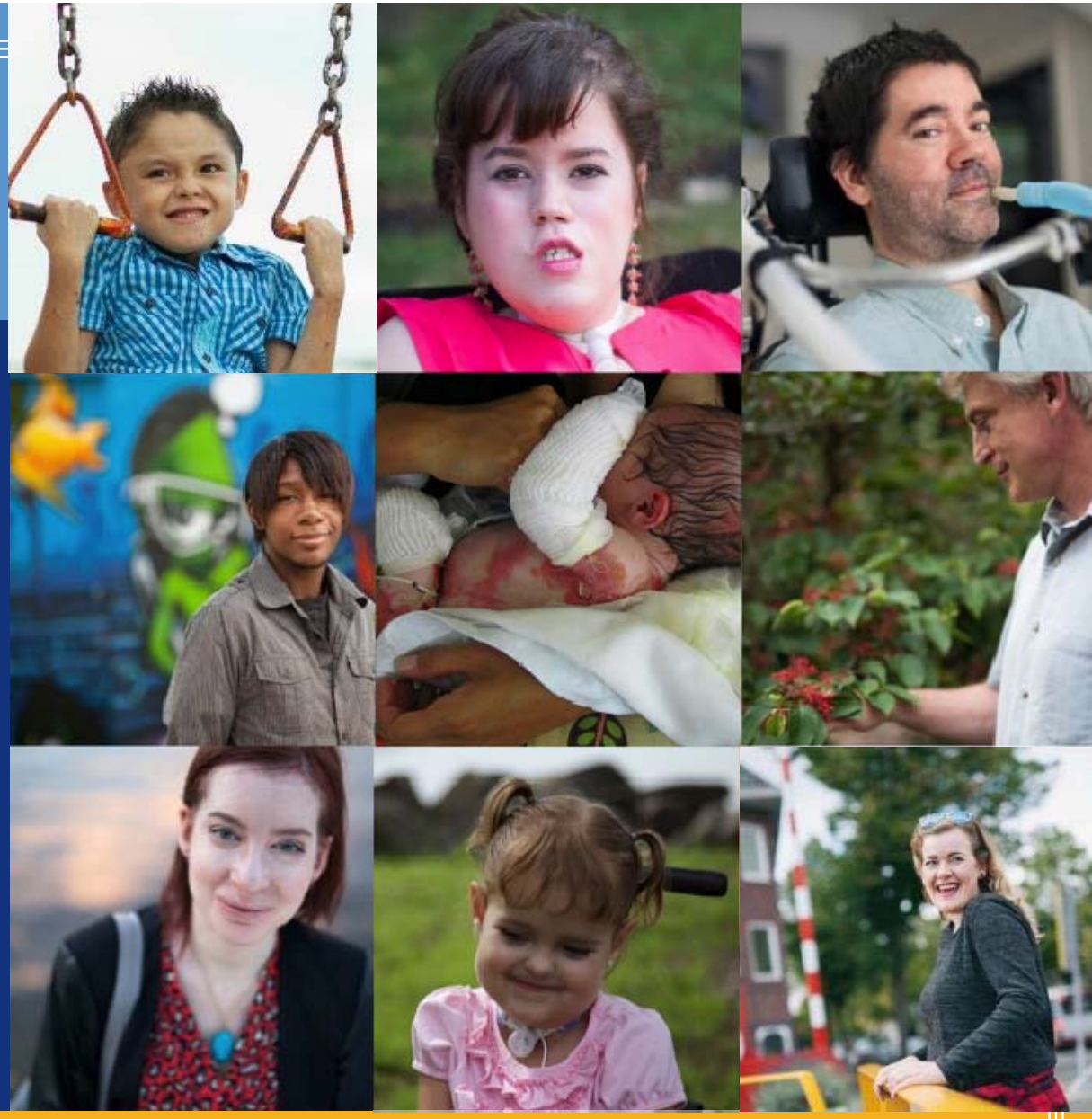
Amicus Therapeutics

Positive Pompe Phase 1/2 Functional Data in Initial Patients

Conference Call & Webcast



May 15, 2017

Safe Harbor

This presentation contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to encouraging preliminary data from a global Phase 1/2 study to investigate ATB200/AT2221 for the treatment of Pompe and the potential implications on these data for the future advancement and development of ATB200/AT2221. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "confidence," "encouraged," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. The forward looking statements included in this presentation are based on management's current expectations and beliefs which are subject to a number of risks, uncertainties and factors, including that the preliminary data based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Company will not be able to demonstrate the safety and efficacy of ATB200/AT2221, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize ATB200/AT2221. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2016 and Quarterly Report on 10-Q for the Quarter ended March 31, 2017. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

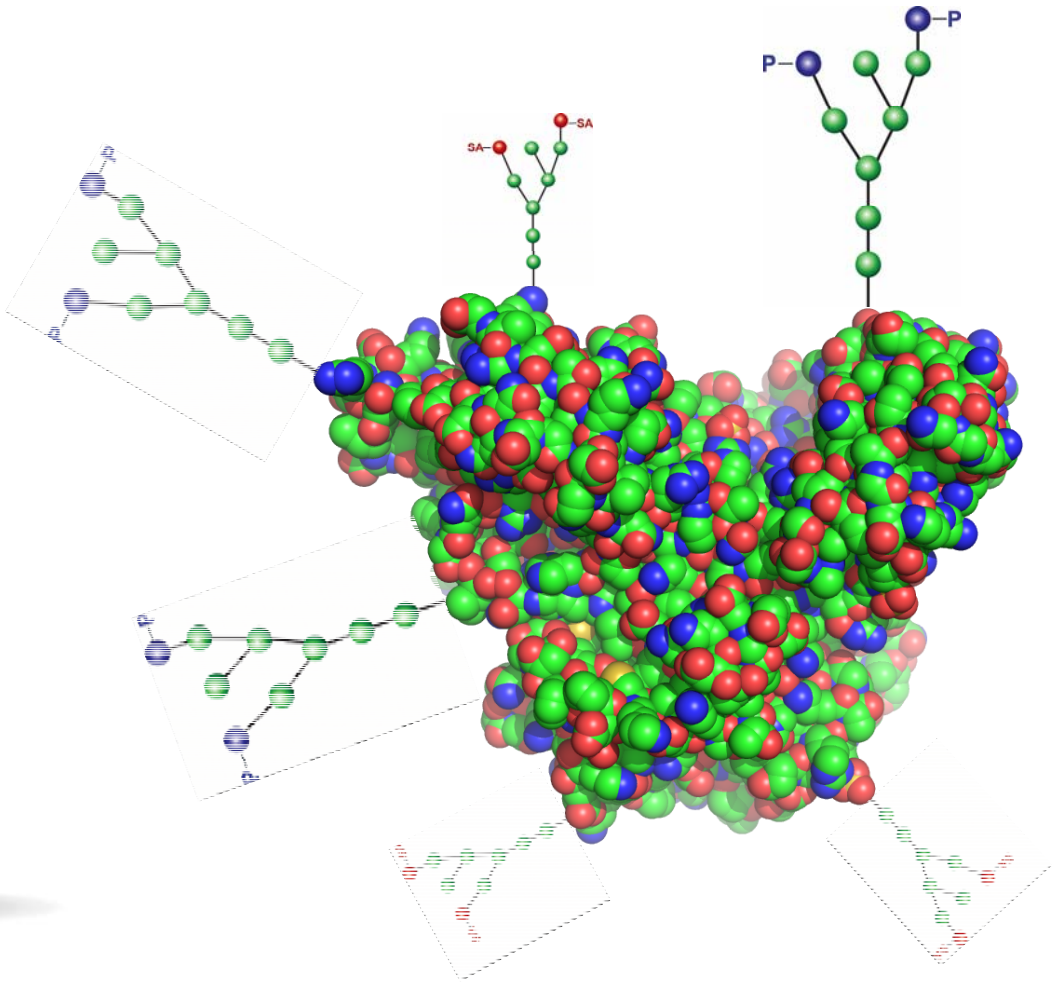
ATB200 + Chaperone: A Highly Differentiated Approach

Novel Pompe Treatment Paradigm with Three Key Differentiators

ATB200 (Novel ERT)

CHART
CHAPERONE-ADVANCED REPLACEMENT THERAPY

Chaperone addition



Optimized mixture of glycans

High levels of M6P and bis M6P

*Artist rendering, not actual product image

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Phase 1/2 ATB200-02 Study Design

Phase 1/2 Clinical Study to Evaluate Safety, Tolerability, Pharmacokinetics (PK), and Pharmacodynamics (PD) of ATB200 + Chaperone (ATB200/AT2221)

18-Week Primary Treatment Period with Long-Term Extension (n=20)

Cohort 1 (Ambulatory ERT-Switch, n=11)

ATB200
5mg/kg (wk 2)
10mg/kg (wk 4)
20mg/kg (wk 6)

→ **ATB200 20mg/kg + AT2221 (Low Dose)** wks 8,10,12

→ **ATB200 20mg/kg + AT2221 (High Dose)** wk 14+

Cohort 2 (Non-Ambulatory ERT-Switch, n=4) & Cohort 3 (ERT-Naïve, n=5)

ATB200 20mg/kg + AT2221 (High Dose) wk 2+

Assessments:

- Safety/Tolerability
- Plasma PK
- Infusion-Associated Reactions
- Antibody & Cytokine Levels
- Pharmacodynamics
- Efficacy (Long-Term Extension)

Baseline Characteristics of Patients in Phase 1/2 ATB200-02 Study (n=20)

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

Baseline Characteristics (N=20)	Cohort 1: Ambulatory ERT-Switch* (N=11)	Cohort 2: Non-Ambulatory ERT-Switch (N=4)	Cohort 3: ERT-Naïve (N=5)
Time on Lumizyme® – mean years (SD)	4.77 (1.42)*	8.9 (3.8)	N/A
Age – mean years (range)	49.4 (28, 66)	36.0 (18, 56)	49.4 (24, 65)
Sex M:F	9:2	3:1	1:4
6MWT – mean meters (SD)	392.0 (93.4)	N/A	399.5 (83.5)
FVC Upright – mean % predicted (SD)	52.3 (13.2)	N/A	53.4 (20.3)

*Cohort 1 patients required to have been on Lumizyme for 2-6 years at baseline

Safety Summary (n=20)*

Safety Data for ATB200/AT2221 Show AEs Have Been Generally Mild and Transient with No Infusion-Associated Reactions After 200+ Total Infusions Across All Cohorts

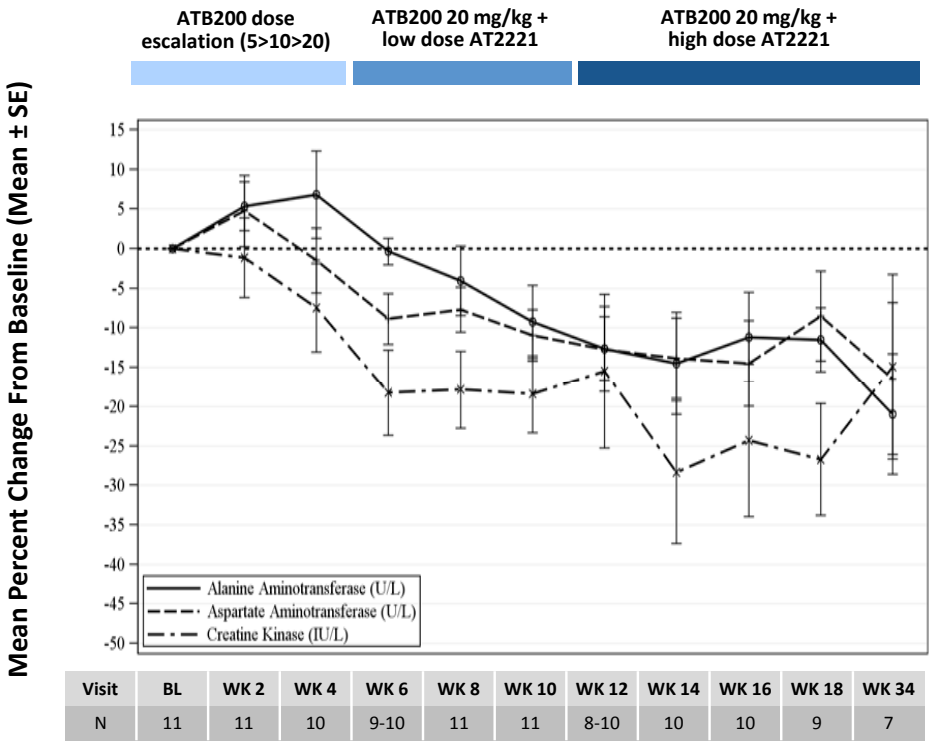
- AEs were generally mild and transient
- No infusion-associated reactions reported after 200+ total infusions across all patients
- Longest duration of treatment is 48 weeks

*Reported through interim data analysis (maximum 48 weeks)

Cohort 1: Biomarkers up to Week 34 (N=11)*

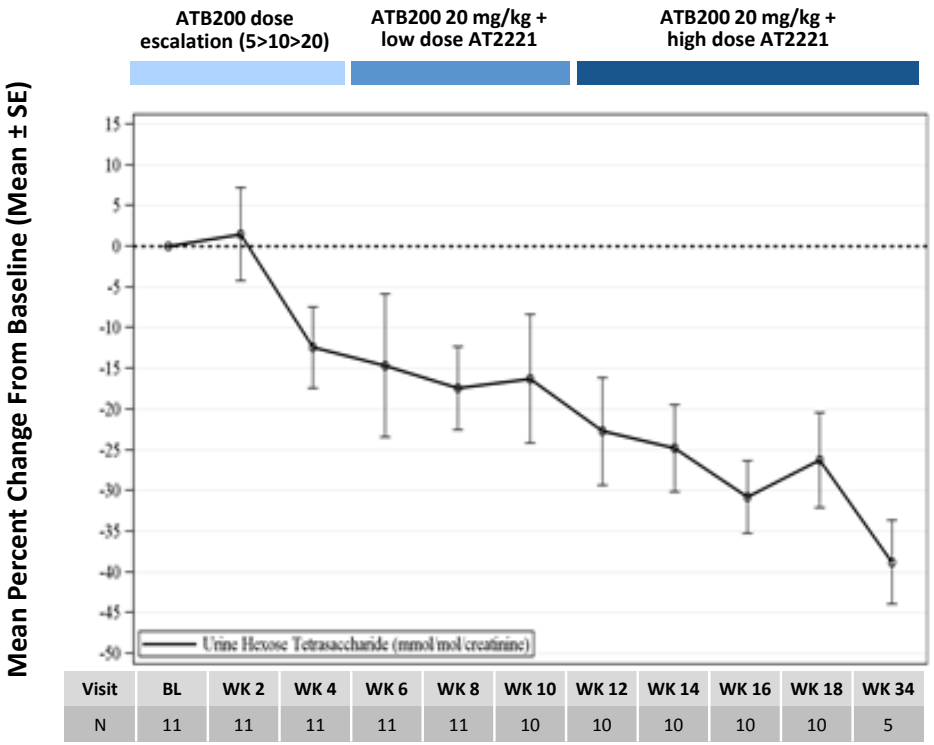
After Switching from SOC to ATB200/A2221 Patients Demonstrated an Improvement in Biomarkers of Muscle Damage (CK, ALT, AST) and Biomarker of Disease Substrate (Hex4) for up to 34 Weeks

Percent Change from Baseline for CK, ALT, AST



Visit	BL	WK 2	WK 4	WK 6	WK 8	WK 10	WK 12	WK 14	WK 16	WK 18	WK 34
N	11	11	10	9-10	11	11	8-10	10	10	9	7

Percent Change from Baseline for Hex 4



Visit	BL	WK 2	WK 4	WK 6	WK 8	WK 10	WK 12	WK 14	WK 16	WK 18	WK 34
N	11	11	11	11	11	10	10	10	10	10	5

*Reported through interim data analysis (maximum 34 weeks); Missing values either unable to be analyzed or not yet analyzed

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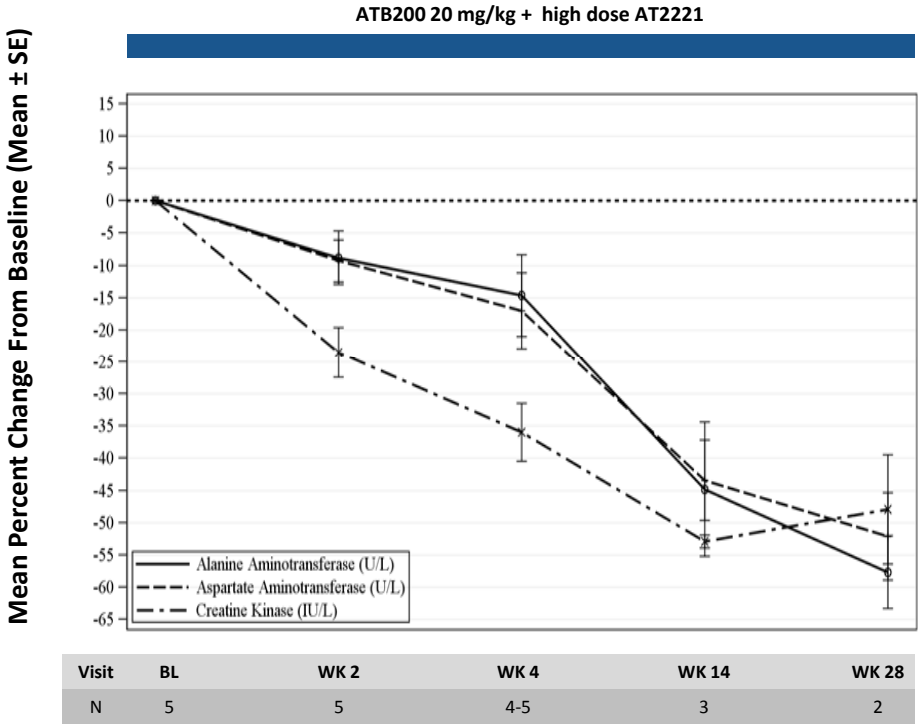
Cohort 3: Biomarkers up to Week 14 (N=5)*

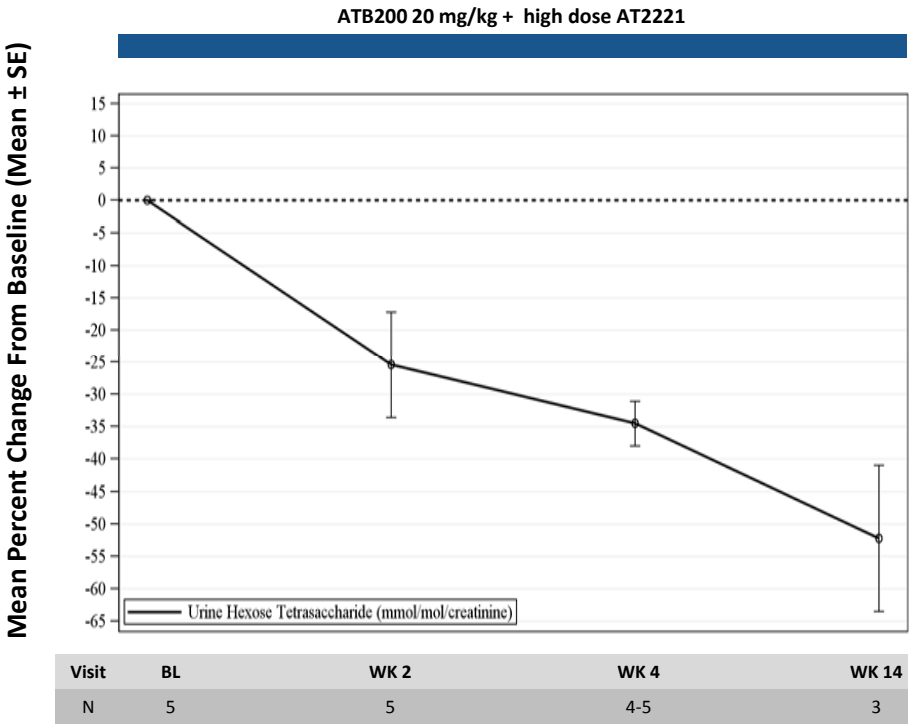
Naïve Patients Treated with ATB200/AT2221 Demonstrated Robust Reduction in Biomarkers of Muscle Damage (CK, ALT, AST) and Biomarker of Disease Substrate (Hex4) for up to 14-28 Weeks

Percent Change from Baseline for CK, ALT, AST



Visit	BL	WK 2	WK 4	WK 14	WK 28
N	5	5	4-5	3	2

Percent Change from Baseline for Hex 4



Visit	BL	WK 2	WK 4	WK 14
N	5	5	4-5	3

*Reported through interim data analysis (maximum 28 weeks); Missing values either unable to be analyzed or not yet analyzed

6-Minute Walk Test (6MWT) Summary at Month 6 (n=9)

6MWT Distance Improved for Both ERT-Naïve Patients (Mean +52 Meters) and ERT-Switch Patients (Mean +38 Meters) at Month 6

6-Minute Walk Test (m): Month 6

Cohort	Baseline Mean (SD)	Change at Month 6 Mean (SD)
Cohort 3 ERT Naïve (n=2)	432 (68)	+52 (15)
Cohort 1 ERT Switch (n=7)	383 (103)	+38 (43)

6MWT Increased in 2/2 ERT-Naïve Patients and 6/7 ERT-Switch Patients

Other Motor Function Tests at Month 6 (n=9)

Other Motor Function Tests Show Improvements for Both ERT-Naïve and ERT-Switch Patients, Consistent With 6MWT

Other Motor Function Tests: Month 6

Patients	Timepoint	4 Stair Climb Mean (SD) (sec)	Timed Up and Go Mean (SD) (sec)	10M walk Mean (SD) (sec)
Cohort 3: ERT Naïve (n=2)	Baseline	3.9 (0.6)	8.9 (0.9)	6.9 (0.8)
	Change at Month 6	-0.3 (0.0)	-1.4 (0.4)	-0.5 (0.2)
Cohort 1: ERT Switch (n=7)	Baseline	4.4 (3.1)	11.0 (7.7)	7.5 (3.5)
	Change at Month 6	-1.1 (1.3)	-1.9 (2.8)	-0.04 (1.6)

Cohort 2 Muscle Strength Testing at Month 6 (n=1)

Substantial Improvement Observed in Shoulder and Elbow Strength in First Non-Ambulatory ERT-Switch Patient with Available Data at Month 6

Quantitative Muscle Testing (QMT) - Dynamometer

Assessment	Elbow Flex		Elbow Extension		Shoulder Adduction		Shoulder Abduction	
	Right	Left	Right	Left	Right	Left	Right	Left
Baseline	1.0	0.9	1.2	1.1	0.8	0.5	1.3	0.9
Month 6	4.1	3.3	3.5	3.2	2.8	0.0	3.3	3.6
CFBL	**+3.1**	**+2.4**	**+2.3**	**+2.1**	**+2.0**	**-0.5**	**+2.0**	**+2.7**

Scoring

Measurement of force production in pounds as measured by dynamometer

Manual Muscle Testing (MMT)*

Assessment	Elbow Flex		Elbow Extension		Shoulder Adduction	
	Right	Left	Right	Left	Right	Left
Baseline	2	2	2	2	2	2
Month 6	4	3	4	3	2	2
CFBL	**+2**	**+1**	**+2**	**+1**	**0**	**0**

Scoring

1. Visible muscle movement, but no movement at the joint
2. Movement at the joint, but not against gravity
3. Movement against gravity, but not against added resistance
4. Movement against resistance, but less than normal
5. Normal strength

*R/L shoulder abduction by MMT not assessed at M6

Forced Vital Capacity (FVC) Summary at Month 6 (n=8)*

FVC Results Show Improvement in ERT-Naïve Patients (Mean +3.0%) and Stability in ERT-Switch Patients (Mean +0.3%) at Month 6

FVC (% Predicted): Month 6

Cohort	Baseline Mean (SD)	Absolute Change at Month 6 Mean (SD)
Cohort 3 ERT Naïve (n=2)	51 (27)	+3 (0)
Cohort 1 ERT Switch (n=6)*	51 (17)	+0.3 (3)

FVC increased in 2/2 ERT-Naïve patients and 3/6 ERT-Switch patients

*FVC results not available for 1 subject at month 6

Other Pulmonary Function Tests at Month 6 (n=8-9)*

MIP increased and MEP decreased in ERT-naïve patients,
MIP and MEP both increased in ERT-switch patients

Other Pulmonary Function Tests: Month 6

Patients	Timepoint	MIP Mean (SD)	MEP Mean (SD)
Cohort 3: ERT Naïve (n=2)	Baseline	45.5 (27.6)	57.5 (9.2)
	Change at Month 6	+8.5 (3.5)	-4.5 (17.7)
Cohort 1: ERT Switch (n=6-7)*	Baseline	35.4 (11.3)	69.5 (21.2)
	Change at Month 6	+1.0 (5.2)	+15.5 (25.4)

*MEP results not available for 1 patient at month 6

Functional Data Summary (n=10)

- Muscle function at Month 6
  - Muscle function improved in 9/10 patients
  - Mean 6MWT distance improved in both naïve (+52 Meters) and ERT-switch (+38 Meters) patients (8 out of 9)
  - Other motor function tests in ambulatory patients consistent with 6MWT
  - First non-ambulatory patient showed significant improvements in muscle strength tests
- Pulmonary function at Month 6
  - FVC increased in ERT-naïve patients (mean +3.0%) and was stable in ERT-switch patients (mean +0.3%)
  - MIP and MEP generally consistent with FVC

Pompe Phase 1/2 Study ATB200-02 Data Cascade

On Track to Report Full Data Set in 3Q17

Pompe Milestones in 2017

- **Preliminary 18-Week Data at WORLD*Symposium***
- **Additional 18-Week & Initial Extension Data in 2Q**
- **Additional 18-Week & Extension Data in 3Q**
- **Full Data Set at Scientific Congress**
- **Meeting with U.S. and EU regulators**

18-WEEK DATA

- Safety / tolerability
- Pharmacokinetics (PK)
- Biomarkers
- Immunogenicity

EXTENSION DATA

- Motor/pulmonary function

Thank you

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Appendix

Pompe Disease Overview

Pompe Disease is Heterogeneous Across a Broad Spectrum of Patients

Deficiency of GAA leading to glycogen accumulation

Respiratory and cardiac failure are leading causes of morbidity and mortality

Age of onset ranges from infancy to adulthood

5,000 – 10,000 patients diagnosed WW[1]

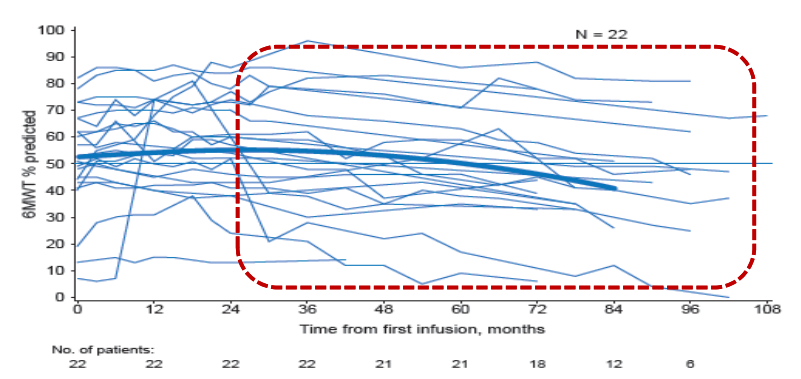
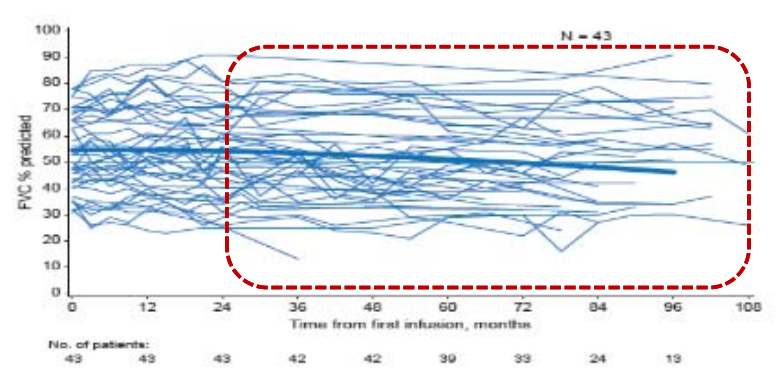
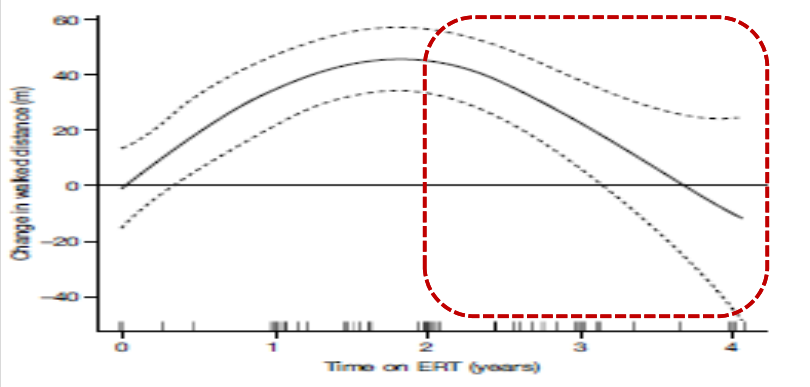
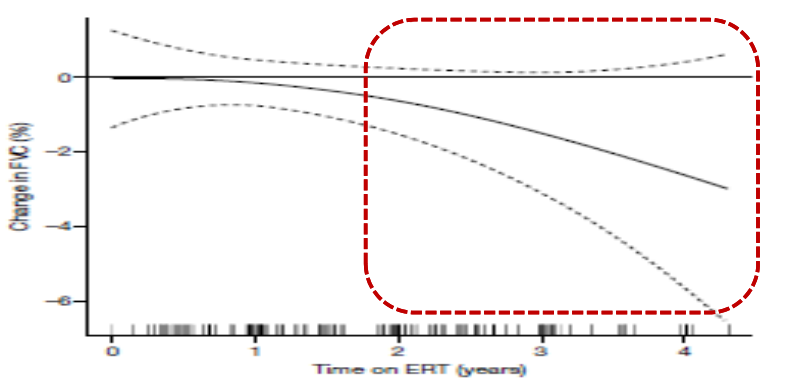
Symptoms include muscle weakness, respiratory failure, and cardiomyopathy

~$800M+ Global Pompe ERT sales in FY15[2]

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

Long-Term Motor and Pulmonary Function on Lumizyme Treatment

Patients Expected to Stabilize or Experience Progressive Decline in Motor and Pulmonary Function After 2-6 Years on Lumizyme Treatment

	Six Minute Walk Test (m)	FVC (%)
LOTS Study Extension (van der Ploeg et al. 2017)		
UK Health Technology Assessment* (Wyatt et al. 2012)		

*Data show the age-adjusted association between time on ERT and 6MWT/FVC

Summary of Motor (6MWT) and Pulmonary (FVC) Function with Standard of Care and Investigational ERTs

General Improvement in ERT-Naïve Population During Initial Treatment on All ERTs – Data on Development Stage ERTs in Switch Population has Been Inconsistent

Patients	Treatment	Study (Duration, n)	CFBL 6MWT (m)	CFBL FVC (%)
Untreated	Placebo	LOTS (72 wks, n=30)	-3	-2.2
ERT-Naïve	Lumizyme	LOTS (78 wks, n=60)	+25	+1.2
	Neo-GAA	Neo-GAA Phase 2 (24 wks, n=3)	+24	+6.2
	BMN701	BMN701 Phase 1/2 (24 wks, n=16)	+22	+1.2
ERT-Switch	Neo-GAA	Neo-GAA Phase 2 (24 wks, n=6)	-6	+1.4
	BMN701	BMN701 Phase 2 (24 wks, n=18)	+26	-3.7

Sources: van der Ploeg, *et al.* NEJM (2010); WORLD*Symposium* 2016 Poster and Sanofi press release March 2016; BioMarin press release March 2013; BioMarin J.P. Morgan 2016 presentation