Efficacy and safety of cipaglucosidase alfa/miglustat versus alglucosidase alfa in late-onset Pompe disease: A global, double-blind, randomized phase 3 trial (PROPEL)

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Disclosures and Disclaimers

- Dr. Mozaffar has served as a consultant for Amicus Therapeutics, Sanofi Genzyme, Spark Therapeutics and Audentes, and as a speaker for Sanofi Genzyme, and his institution has received grants from Sanofi Genzyme, Valerion, Spark Therapeutics and Audentes for the participation in their clinical trials
- This presentation shares information about Amicus Therapeutics' investigational therapy AT-GAA, which is in development for the treatment of Pompe disease. This investigational therapy is not approved by any regulatory agency at this time
- The study was funded by Amicus Therapeutics, Inc.

Pompe Disease Overview

- Pompe disease (PD) is a rare, autosomal recessive lysosomal disorder caused by pathogenic variants of the GAA gene[1,2]
- Functional deficiency of GAA leads to lysosomal accumulation of glycogen in all tissues, especially skeletal, cardiac, and smooth muscles[1,3]
- The clinical presentation of PD includes 2 phenotypes: infantile-onset (IOPD) and late-onset (LOPD)[3]
- LOPD is primarily characterized by progressive weakness in the limb-girdle and respiratory muscles, leading to motor and respiratory difficulties[2]
  - Respiratory failure is a common cause of mortality in LOPD[3]
- LOPD may involve other organ systems, including the central and peripheral nervous system, bone, vasculature, heart, gastrointestinal and urinary tract[2,4]
- Alglucosidase alfa, a rhGAA, is the only approved treatment that has shown to improve prognosis in patients with IOPD and LOPD[5,6]

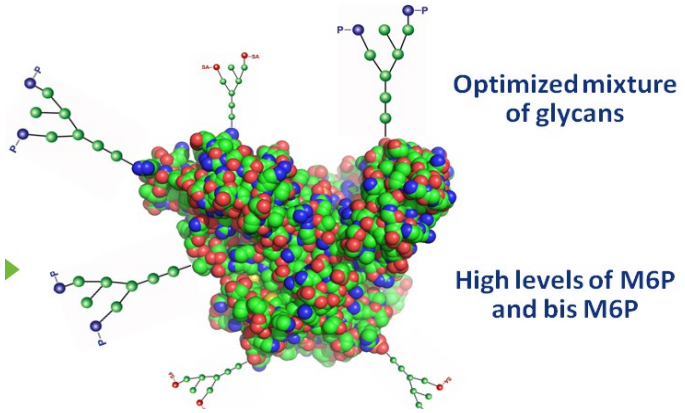
GAA=acid alpha-glucosidase; rhGAA=recombinant human GAA;

References: 1. Hers HG. *Biochem J.* 1963;86(1):11-16. **2.** Kishnani PS, et al. *J Pediatr*. 2004;144(5 Suppl):S35-43. **3.** Kishnani PS, et al. *Genet Med*. 2006;8(5):267-288. **4.** Chan J, et al. *Mol Genet Metab*. 2017;120(3):163-172. **5.** Lumizyme® [prescribing information]. Sanofi Genzyme: Cambridge, MA; 2020. **6.** Do HV, et al. *Ann Transl Med*. 2019;7(13):291.

AT-GAA: Cipaglucosidase alpha/miglustat

- AT-GAA is an investigational, 2-component therapy comprising cipaglucosidase alfa administered in conjunction with miglustat
  - Cipaglucosidase alfa is a novel rhGAA with enhanced glycosylation designed for improved uptake and processing
  - Miglustat is a small molecule that stabilizes cipaglucosidase alfa in blood and enhance delivery of the active enzyme to tissues
- In a murine model of PD, AT-GAA was shown to be superior to alglucosidase alfa in reversing or improving all aspects of disease pathogenesis that were measured—glycogen clearance, lysosomal enlargement, autophagic buildup, muscle fiber size and muscle strength

Cipaglucosidase alfa
(intravenous novel ERT)



Miglustat
(oral enzyme stabilizer)

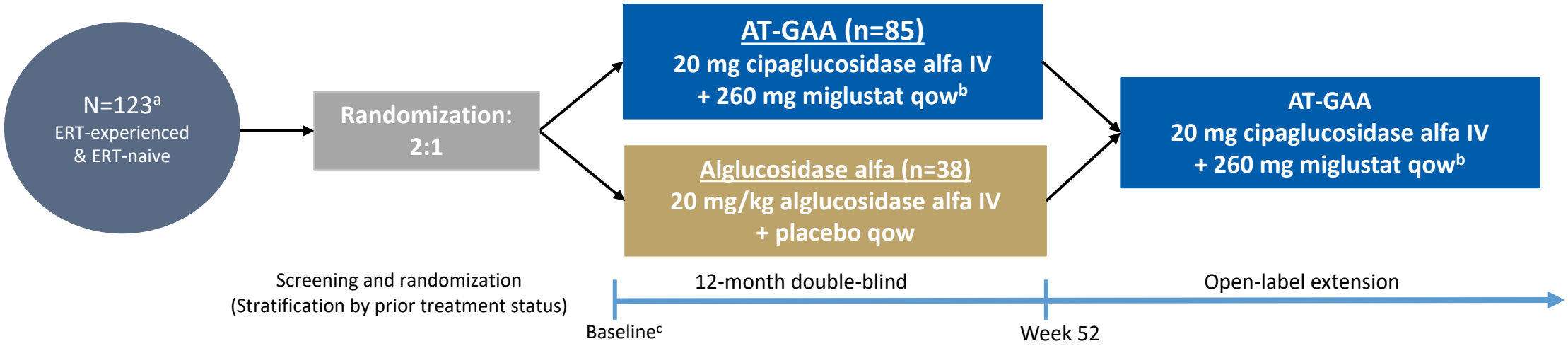
AT-GAA=Amicus Therapeutics GAA; ERT=enzyme replacement therapy; rhGAA=recombinant human acid alpha-glucosidase.

Reference: Xu S, et al. *JCI Insight*. 2019;4(5).

PROPEL Study Design

- A phase 3, randomized, double-blind, active-controlled trial to assess the efficacy and safety of AT-GAA in adult patients with LOPD compared with alglucosidase alfa/placebo (NCT03729362)

Patients were enrolled in 62 sites across 24 countries



Key Enrollment Criteria:

- ≥18 years old, weighing ≥40 kg at screening with confirmed diagnosis of LOPD
- Classified as one of the following with respect to ERT status:
  - ERT experienced, defined as currently receiving standard of care ERT (alglucosidase alfa) for ≥24 months
  - ERT naive, defined as never having received ERT
- 6MWD ≥75 meters and ≤90% of the predicted value for healthy adults at screening
- Sitting FVC ≥30% of the predicted value for healthy adults at screening

[a]2 patients were randomized but not dosed. [b]195 mg for patients weighing 40-<50 kg. [c]Baseline values were measured during screening (up to 30 days before dosing). For 6MWD and FVC: the baseline value is the average of last 2 measurements obtained on or prior to 1st dose date.

6MWD=6-minute walk distance; ERT=enzyme replacement therapy; FVC=forced vital capacity; LOPD=late-onset Pompe disease; qow=every other weeks.

Reference: ClinicalTrials.gov NCT03729362. Available at: https://clinicaltrials.gov/ct2/show/NCT03729362

Efficacy Endpoints

- **Primary endpoint:** Change from baseline to Week 52 in 6-minute walk distance (6MWD) measured in meters.
  - The primary endpoint was tested for superiority of AT-GAA vs alglucosidase alpha, using MMRM and pre-specified nonparametric test in case of violation of normality
- **Key secondary efficacy endpoints** in a pre-specified hierarchical order of importance are:
  - Change from baseline to Week 52 in sitting FVC (% predicted)
  - Change from baseline to Week 52 in the manual muscle test (MMT) score for the lower extremities
  - Change from baseline to Week 52 in the total score for the PROMIS– Physical Function
  - Change from baseline to Week 52 in the total score for the PROMIS – Fatigue
  - Change from baseline to Week 52 in the total score for the GSGC (Gait, Stairs, Gowers' maneuver, Chair)

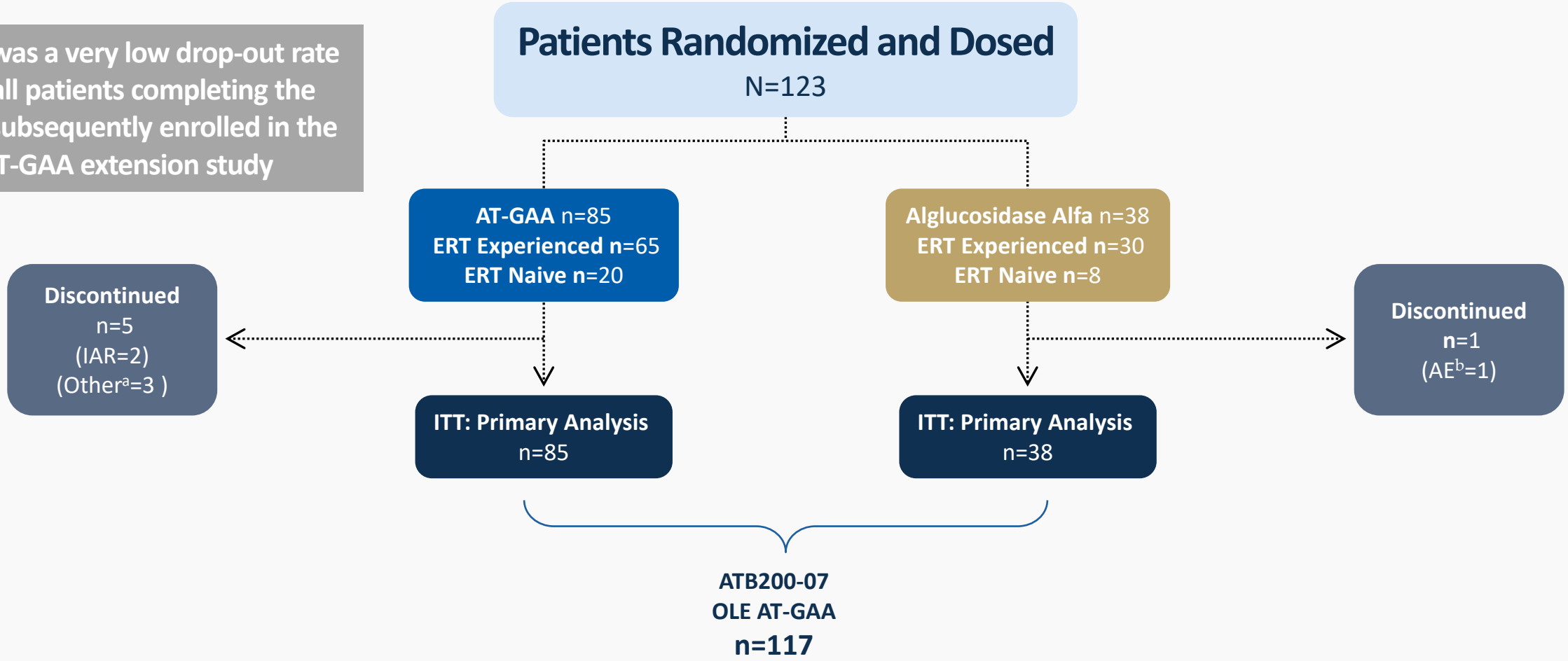
Secondary endpoints were analyzed using ANCOVA model with last observation carried forward (ITT LOCF)

ANCOVA=analysis of covariance; FVC=forced vital capacity; ITT=intention to treat; MMRM= mixed-effect model for repeated measures; PROMIS=Patient-reported Outcomes Measurement Information System.

Results

Patient Disposition

There was a very low drop-out rate and all patients completing the study subsequently enrolled in the AT-GAA extension study



AE=adverse event; ERT=enzyme replacement therapy; IAR=infusion-associated reaction; ITT=intention to treat.

[a]1 Covid pneumonia, 2 withdrew, no longer wanting to travel to sites for infusion all unrelated to study drug. b1 stroke, unrelated to study drug.

Baseline Demographics

Baseline demographics were representative of the population and generally similar in the 2 treatment arms

	AT-GAA n=85	Alglucosidase alfa n=38	Total N=123
Age (years)			
Mean (SD)	47.6 (13.3)	45.1 (13.3)	46.8 (13.3)
Median (Min, Max)	48.0 (19, 74)	46.0 (22, 66)	47.0 (19, 74)
Sex, n (%)			
Male	36 (42.4)	20 (52.6)	56 (45.5)
Female	49 (57.6)	18 (47.4)	67 (54.5)
Previous ERT Duration (years, ERT-experienced only)			
Mean (SD)	7.5 (3.4)	7.1 (3.6)	7.4 (3.4)
Median (Min, Max)	7.6 (2.0, 13.7)	7.1 (2.1, 13.2)	7.4 (2.0, 13.7)
Race, n (%)			
White	74 (87.1)	30 (78.9)	104 (84.6)
Asian	5 (5.8)	5 (13.2)	10 (8.1)
Other	6 (7.1)	3 (7.9)	9 (7.3)
Regions, n (%)			
North/South America	26 (30.6)	15 (39.5)	41 (33.3)
Europe	43 (50.6)	12 (31.6)	55 (44.7)
Asia Pacific	16 (18.8)	11 (28.9)	27 (22.0)

ERT=enzyme replacement therapy; SD=standard deviation.

Baseline Characteristics: 6MWD and FVC

Baseline 6MWD and FVC were representative of the population and generally similar in the 2 treatment arms

Overall population		AT-GAA n=85	Alglucosidase alfa n=37
6MWD, m	Mean (SD)	357.9 (111.8)	351 (121.3)
	Median (Min, Max)	359.5 (79.0, 575.0)	365.5 (112.5, 623.0)
FVC, % predicted	Mean (SD)	70.7 (19.6)	69.7 (21.5)
	Median (Min, Max)	70.0 (30.5, 132.5)	71.0 (31.5, 122.0)
ERT-Experienced		**AT-GAA n=65**	**Alglucosidase alfa n=30**
6MWD, m	Mean (SD)	346.9 (110.2)	334.6 (114.0)
	Median (Min, Max)	352.5 (79.0, 557.5)	343.5 (112.5, 532.3)
FVC, % predicted	Mean (SD)	67.9 (19.1)	67.5 (21.0)
	Median (Min, Max)	68 (30.5, 132.5)	69.0 (31.5,122.0)
ERT-Naïve		**AT-GAA n=20**	**Alglucosidase alfa n=7**
6MWD, m	Mean (SD)	393.6 (112.4)	420.9 (135.7)
	Median (Min, Max)	375.2 (154.0, 575.0)	385.5 (201.0, 623.0)
FVC, % predicted	Mean (SD)	80.2 (18.7)	79.1 (22.6)
	Median (Min, Max)	82.3 (48.0, 111.0)	93.5 (46.5, 98.0)

6MWD and FVC: Overall Population (n=122)

6MWD showed greater improvement with AT-GAA vs alglucosidase alfa but did not reach statistical superiority;
FVC demonstrated a nominally statistically significant & clinically meaningful improvement with AT-GAA vs alglucosidase alfa

6MWD (m)

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

FVC (% predicted)

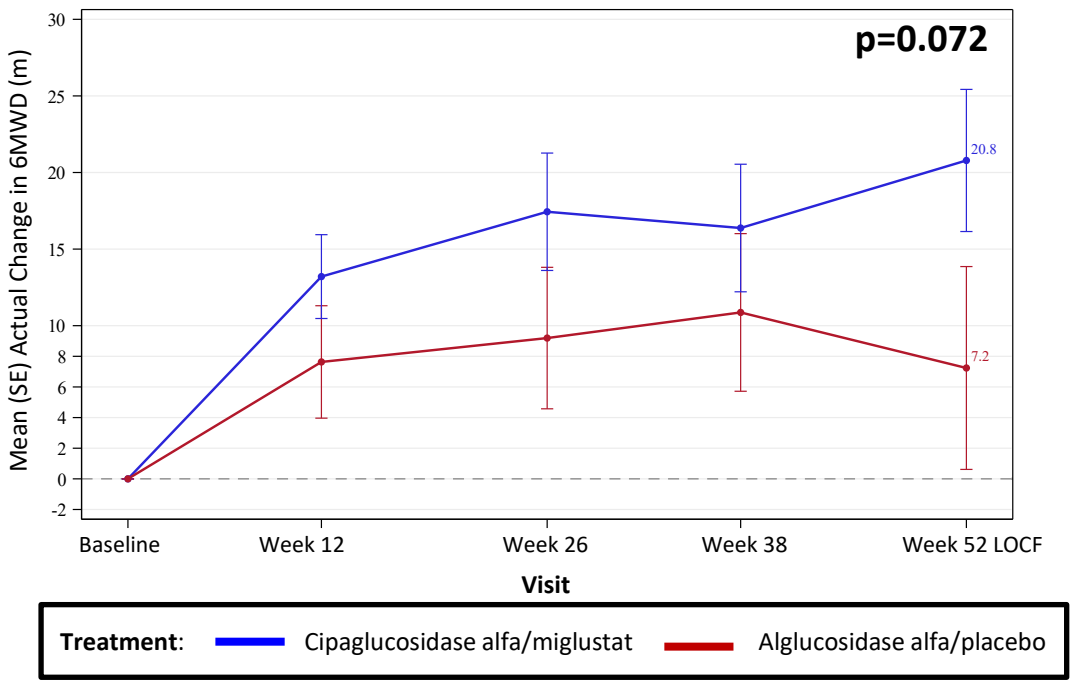
Treatment	Baseline	CFBL at Week 52	Difference	*P* Value
AT-GAA (n=85)	70.7 (19.6)	-0.9 (0.7)	+3.0 (1.2)	***P*=0.023**
Alglucosidase alfa (n=37)	69.7 (21.5)	-4.0 (0.8)		

6MWD=6-minute walk distance; ANCOVA=analysis of covariance; CFBL=change from baseline; FVC=forced vital capacity; LOCF=last observation carried forward; MMRM= mixed-effect model for repeated measures; SD=standard deviation; SE=standard error.
Baseline is mean (SD); CFBL is mean LOCF (SE); *P* values are nominal 2-sided. 6MWD data were not normally distributed and 6MWD *P* value is for non-parametric ANCOVA; 6MWD parametric MMRM *P*=0.097. FVC data were normally distributed and *P* values are from ANCOVA.
Results exclude 1 clinically implausible patient who used an investigational anabolic steroid ostarine (selective androgen receptor modulator) just prior to study start.

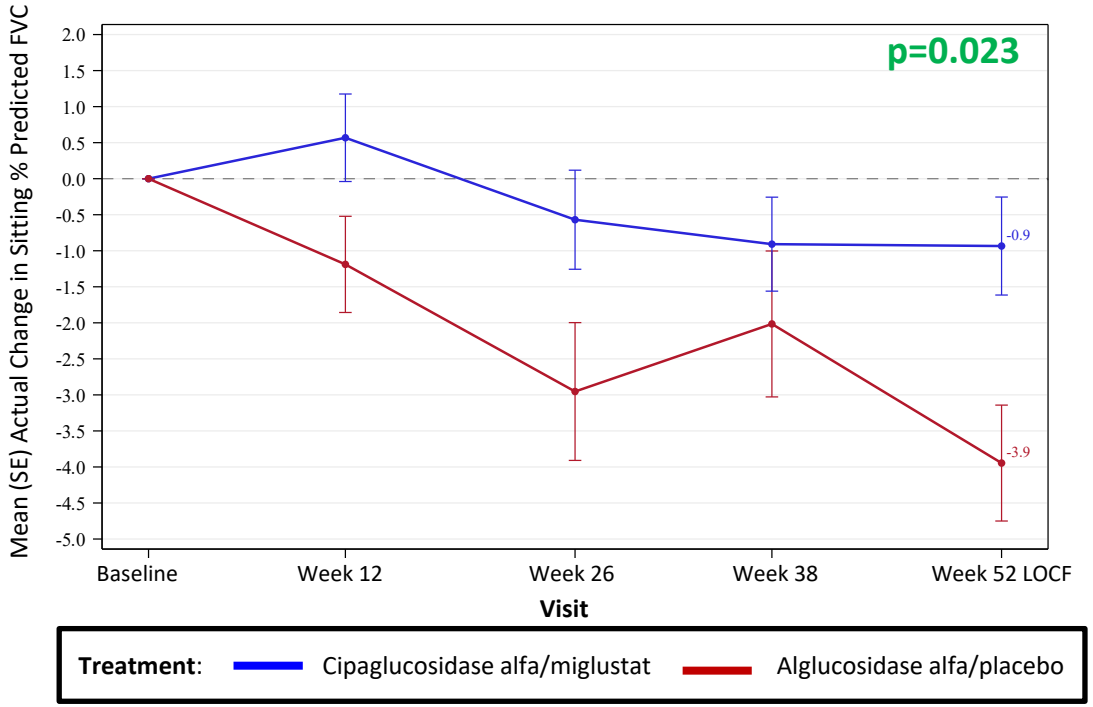
6MWD and FVC Over Time: Overall Population (n=122)

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

6MWD (m): Change from baseline (n=85, n=37)



FVC (% predicted): Change from baseline (n=85, n=37)



6MWD=6-minute walk distance; ANCOVA=analysis of covariance; CFBL=change from baseline; ERT=enzyme-replacement therapy; FVC=forced vital capacity; LOCF=last observation carried forward; MMRM= mixed-effect model for repeated measures; SD=standard deviation; SE=standard error. Baseline is Mean (SD); CFBL is Mean (SE); *P* values are nominal 2-sided; FVC data normally distributed and *P* values are from ANCOVA. 6MWD data not normally distributed and 6MWD *P* value is for non-parametric ANCOVA; 6MWD parametric MMRM *P*=0.097. Results exclude one clinically implausible patient who used an investigational anabolic steroid ostarine (selective androgen receptor modulator) just prior to study start.

6MWD and FVC: ERT-Experienced Population (n=95)

In the ERT-experienced population, 6MWD and FVC demonstrated a nominally statistically significant and clinically meaningful improvement with AT-GAA vs alglucosidase alfa

6MWD (m)

Treatment	Baseline	CFBL at Week 52	Difference	*P* Value
AT-GAA (n=65)	346.9 (110.2)	+16.9 (5.0)	+16.9 (8.8)	***P*=0.046**
Alglucosidase alfa (n=30)	334.6 (114.0)	0.0 (7.2)		

FVC (% predicted)

Treatment	Baseline	CFBL at Week 52	Difference	*P* Value
AT-GAA (n=65)	67.9 (19.1)	+0.1 (0.7)	+4.1 (1.2)	***P*=0.006**
Alglucosidase alfa (n=30)	67.5 (21.0)	-4.0 (0.9)		

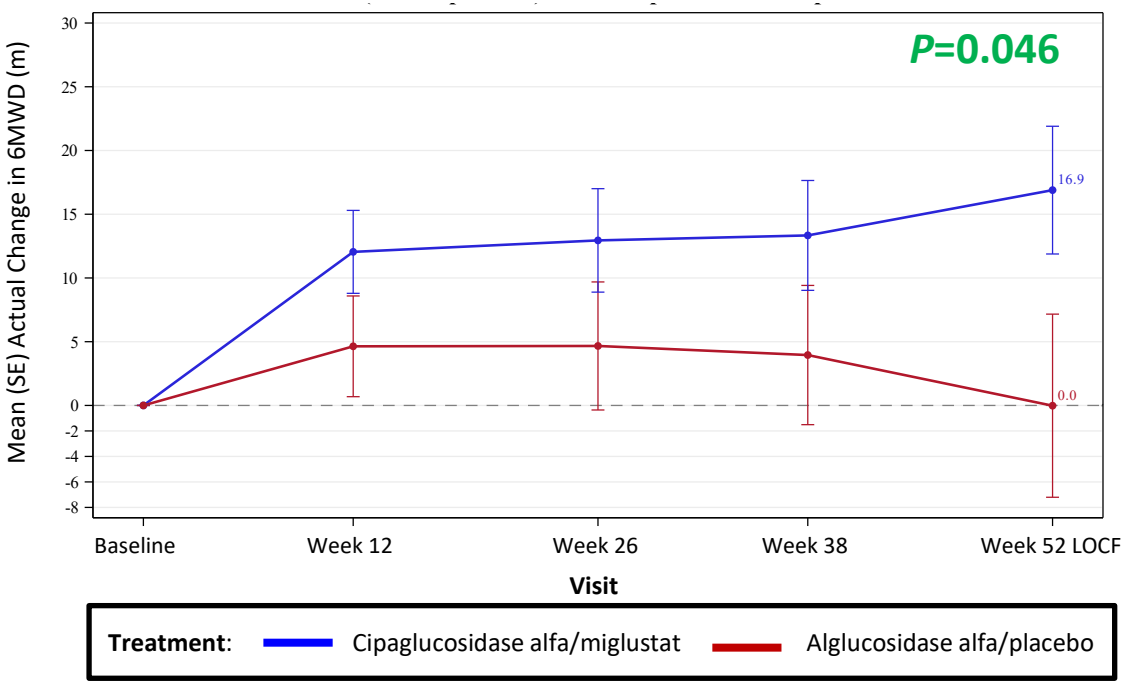
6MWD=6-minute walk distance; ANCOVA=analysis of covariance; CFBL=change from baseline; ERT=enzyme-replacement therapy; FVC=forced vital capacity; MMRM= mixed-effect model for repeated measures; SD=standard deviation; SE=standard error.

Baseline is mean (SD); CFBL is mean LOCF (SE); *P* values are nominal 2-sided. 6MWD data were not normally distributed and 6MWD *P* value is for non-parametric ANCOVA; 6MWD parametric MMRM *P*=0.078. FVC data were normally distributed and *P* values are from ANCOVA.

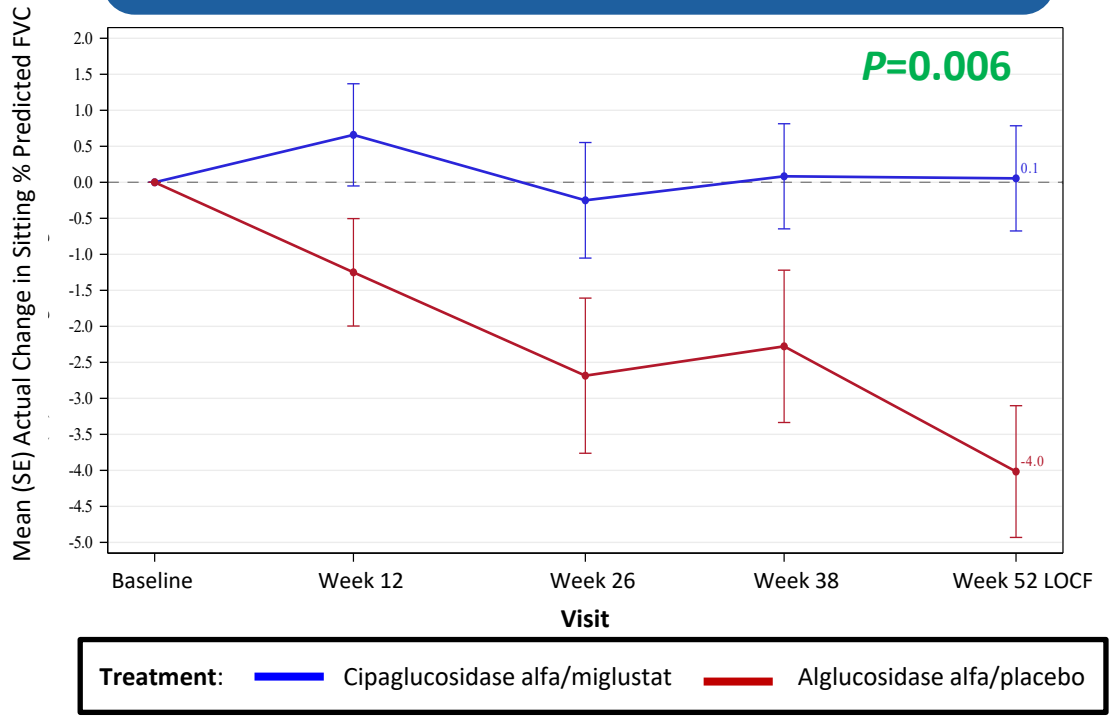
6MWD and FVC Over Time: ERT-Experienced Population (n=95)

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

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



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



6MWD=6-minute walk distance; ANCOVA=analysis of covariance; CFBL=change from baseline; ERT=enzyme-replacement therapy; FVC=forced vital capacity; LOCF=last observation carried forward; MMRM= mixed-effect model for repeated measures; SD=standard deviation; SE=standard error.

P values are nominal 2-sided. 6MWD data not normally distributed and 6MWD *P* value is for non-parametric ANCOVA; 6MWD parametric MMRM *P*=0.078.

FVC data normally distributed and *P* values are from ANCOVA.

6MWD and FVC: ERT-Naive Population (n=27)

In the smaller ERT-naive population, variability was greater and 6MWD and FVC both numerically favored alglucosidase alfa

6MWD (m)

Treatment	Baseline	CFBL at Week 52	Difference	*P* Value
AT-GAA (n=20)	393.6 (112.4)	+33.4 (10.9)	-4.9 (19.7)	*P*=0.60
Alglucosidase alfa (n=7)	420.9 (135.7)	+38.3 (11.1)		

FVC (% predicted)

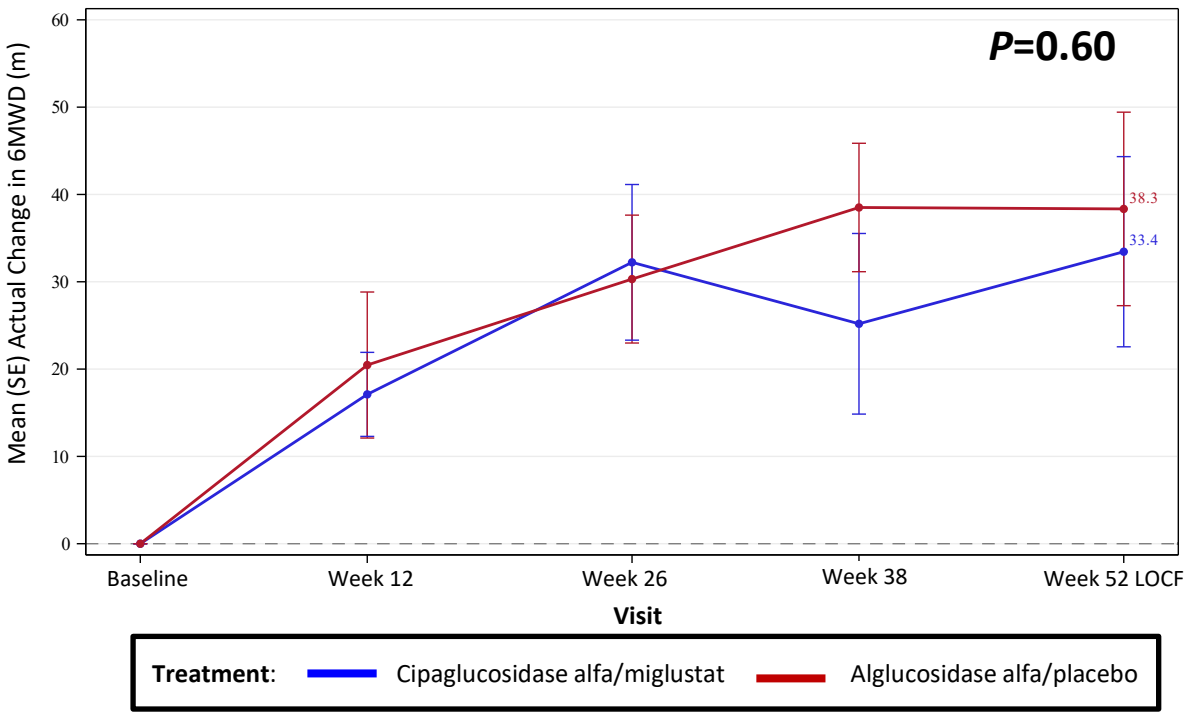
Treatment	Baseline	CFBL at Week 52	Difference	*P* Value
AT-GAA (n=20)	80.2 (18.7)	-4.1 (1.5)	-0.5 (2.7)	*P*=0.57
Alglucosidase alfa (n=7)	79.1 (22.6)	-3.6 (1.8)		

6MWD=6-minute walk distance; ANCOVA=analysis of covariance; CFBL=change from baseline; ERT=enzyme-replacement therapy; FVC=forced vital capacity; LOCF=last observation carried forward; MMRM= mixed-effect model for repeated measures; SD=standard deviation; SE=standard error.
Baseline is mean (SD); CFBL is Mean LOCF (SE); *P* values are nominal 2-sided; FVC data normally distributed and *P* values are from ANCOVA.
Results exclude 1 clinically implausible patient who used an investigational anabolic steroid ostarine (selective androgen receptor modulator) just prior to study start.
6MWD data not normally distributed and *P* value is for Wilcoxon test; 6MWD parametric MMRM *P*=0.75.

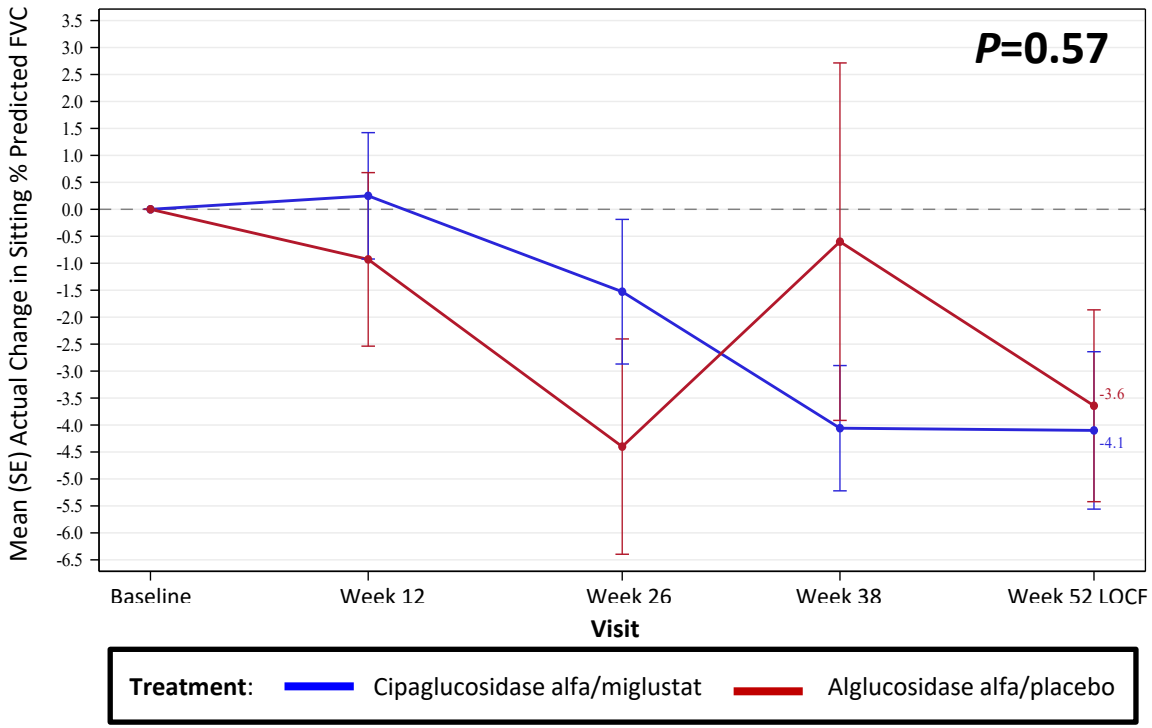
6MWD and FVC Over Time: ERT-Naive Population (n=27)

ERT naive patients treated with AT-GAA and alglucosidase alfa had similar improvements over time in 6MWD and both declined over time in FVC versus alglucosidase alfa

6MWD (m): Change from baseline (n=20, n=7)



FVC (% predicted): Change from baseline (n=20, n=7)



6MWD=6-minute walk distance; ANCOVA=analysis of covariance; CFBL=change from baseline; ERT=enzyme-replacement therapy; FVC=forced vital capacity; LOCF=last observation carried forward; MMRM= mixed-effect model for repeated measures; SD=standard deviation; SE=standard error. Baseline is Mean (SD); CFBL is Mean (SE); *P* values are nominal 2-sided; FVC data normally distributed and *P* values are from ANCOVA. 6MWD data not normally distributed and 6MWD *P* value is for Wilcoxon Text; 6MWD parametric MMRM *P*=0.75. Results exclude one clinically implausible patient who used an investigational anabolic steroid ostarine (selective androgen receptor modulator) just prior to study start.

Key Secondary Endpoints and Biomarkers

Baseline characteristics: Key Secondary Endpoints and Biomarkers

Parameters, mean (SD)	AT-GAA		Alglucosidase alfa	
	Overall n=85	ERT-Experienced n=65	Overall n=37	ERT-Experienced n=30
MMT lower extremities score	28.0 (5.8)[a]	26.4 (5.1)[b]	27.7 (6.2)[c]	26.1 (5.8)[d]
PROMIS-Physical Function	66.9 (12.3)[a]	64.4 (11.4)[b]	68.0 (13.1)	66.9 (12.3)
PROMIS- Fatigue	22.3 (8.3)	22.0 (7.9)	21.1 (6.1)	20.4 (5.4)
GSGC total score	14.5 (5.2)[e]	15.6 (4.1)[f]	14.5 (4.7)[g]	15.5 (4.4)[h]

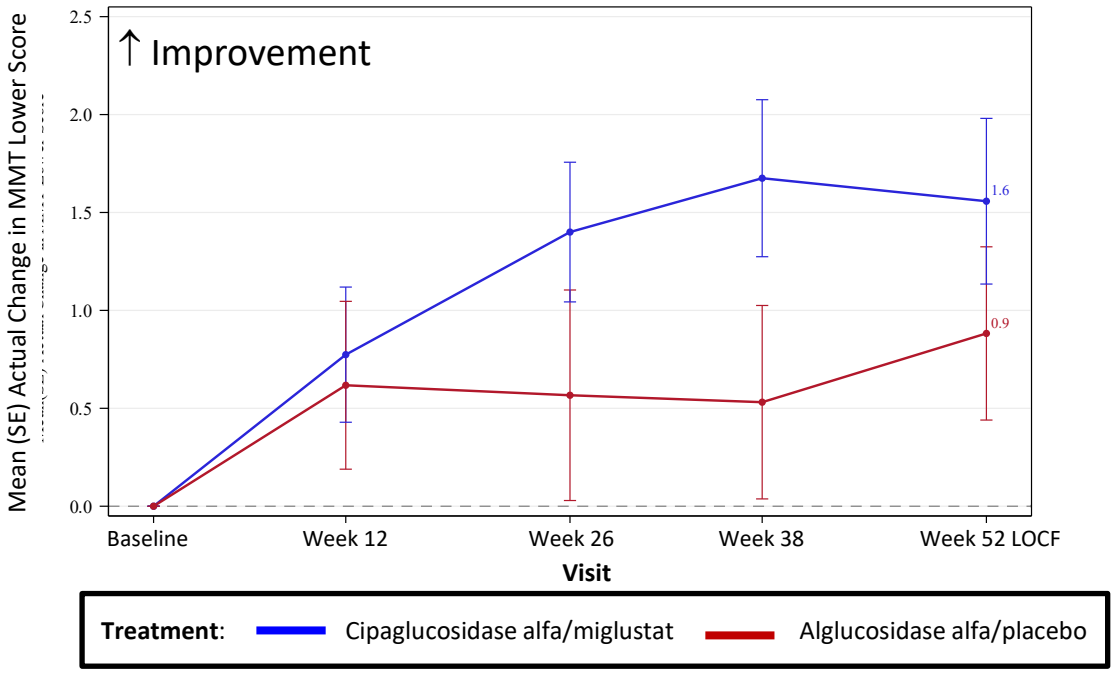
GSGC=Gait, Stairs, Gowers, Chair.
Results exclude 1 clinically implausible patient who used an investigational anabolic steroid ostarine (selective androgen receptor modulator) just prior to study start.
[a]n=84; [b]n=64; [c]n=34; [d]n=27; [e]n=74; [f]n=55; [g]n=32; [h]n=25.

Key Secondary: Lower Extremities MMT

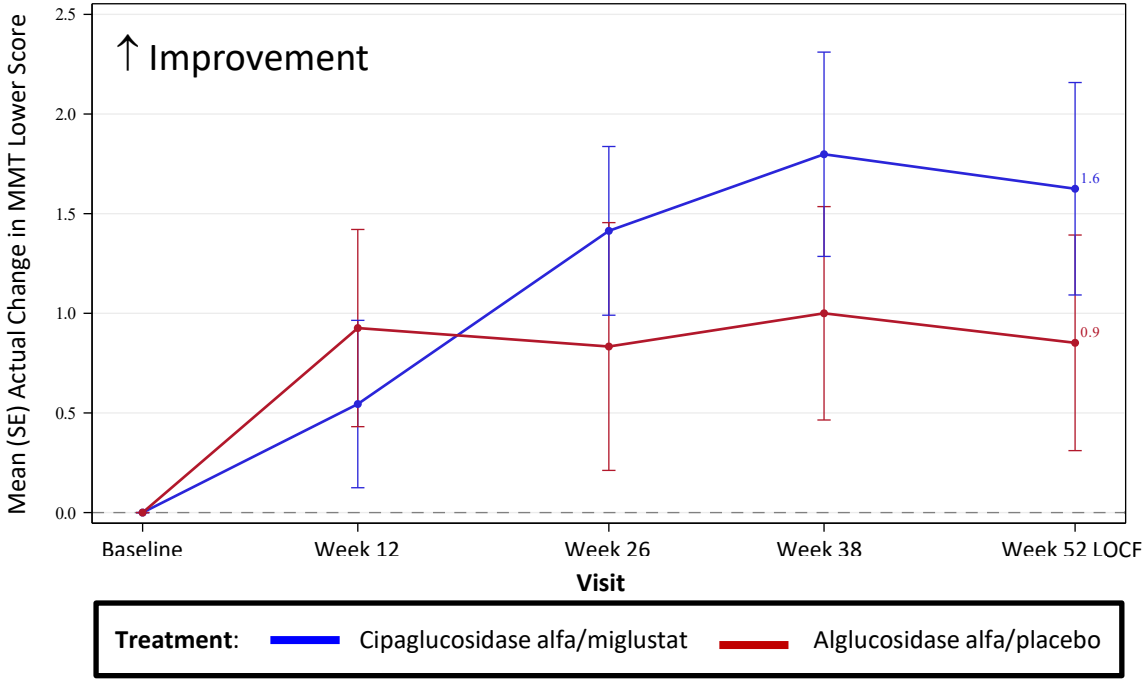
All Patients and ERT-Experienced Patients

In the overall population and ERT-experienced population, lower extremities MMT numerically favored AT-GAA

Overall Population



ERT-Experienced Population

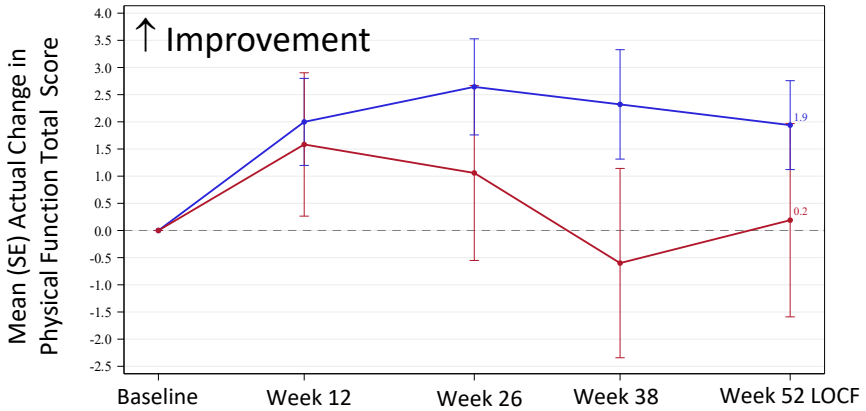


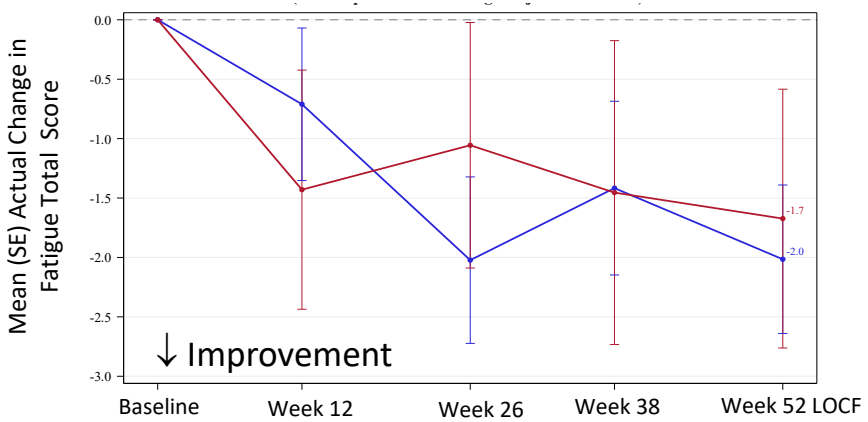
ERT=enzyme-replacement therapy; LOCF=last observation carried forward; MMT=manual muscle test; SE, standard error.
MMT measured via the Medical Research Criteria scale.

Key Secondary: PROMIS Physical Function and Fatigue

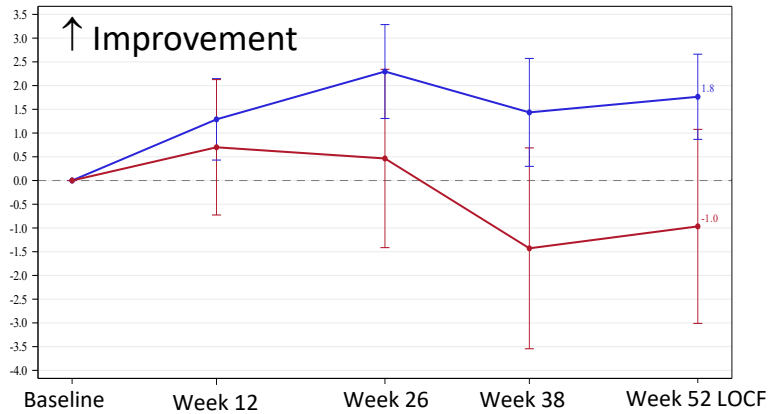
All Patients and ERT-Experienced Patients

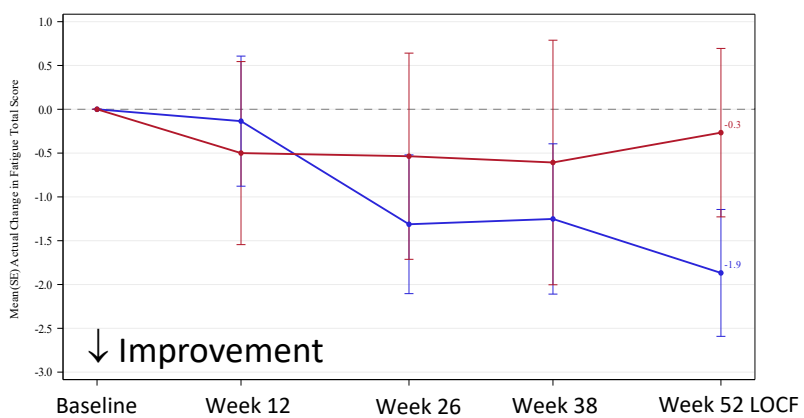
Overall Population





ERT-Experienced Population





In the overall population and ERT experienced population, PROMIS physical function numerically favored AT-GAA

In the overall population and ERT-experienced population, PROMIS fatigue numerically favored AT-GAA

Treatment: Cipaglucosidase alfa/miglustat — Alglucosidase alfa/placebo

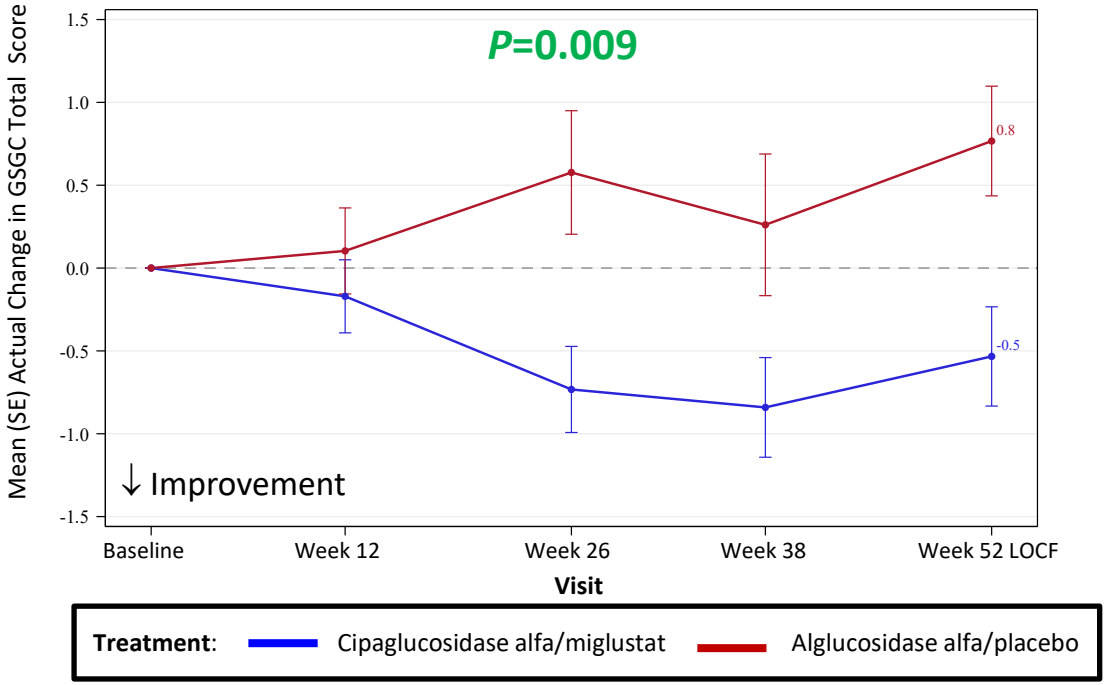
ERT=enzyme-replacement therapy; LOCF=last observation carried forward; PROMIS=Patient-Reported Outcomes Measurement Information System; SE, standard error.
PROMIS – Physical Function Short Form 20a (v2.0) comprises 20 questions scored on a scale from 1 to 5: 1=unable to do; 5=without any difficulty; minimum score 20, maximum score 100.
PROMIS – Fatigue Short Form 8a comprises 8 questions scored on a scale from 1 to 5: 1=not at all; 5=very much; minimum score 8, maximum score 40.

Key Secondary: GSGC (Gait, Stairs, Gowers, Chair)

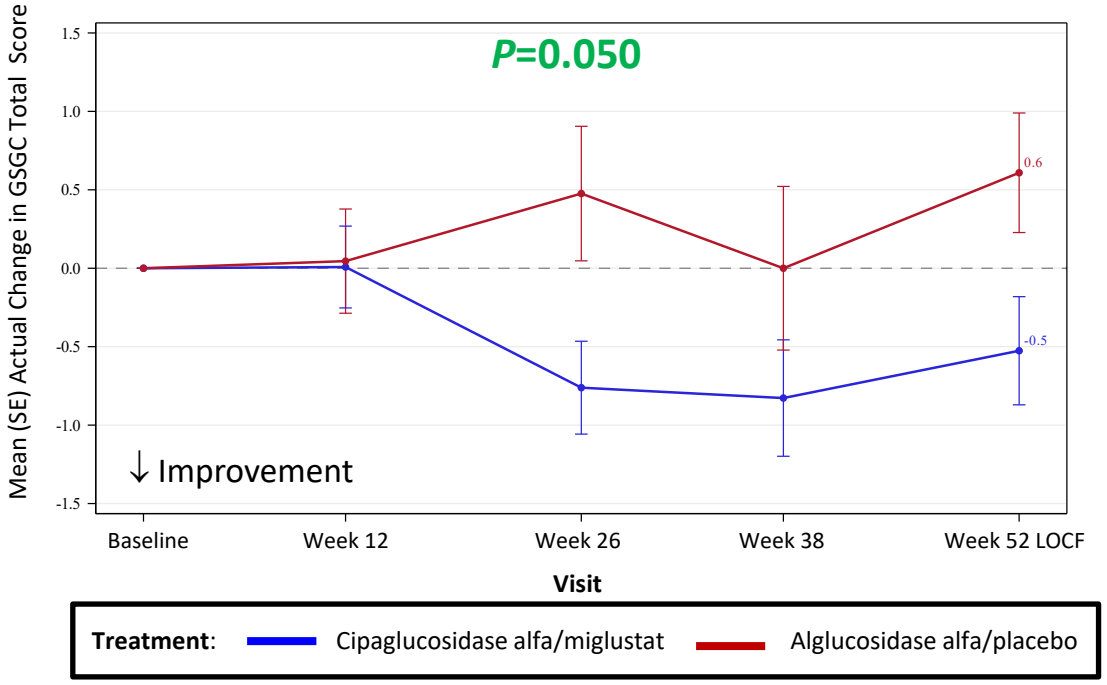
All Patients and ERT-Experienced Patients

In the overall population and ERT-experienced population, clinically and nominally statistically significant improvement was observed in GSGC total score with AT-GAA compared with alglucosidase alfa/placebo

Overall Population



ERT-Experienced Population

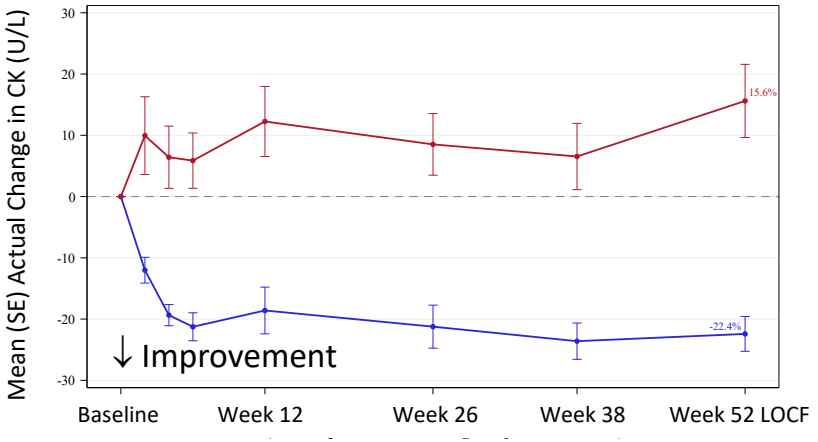


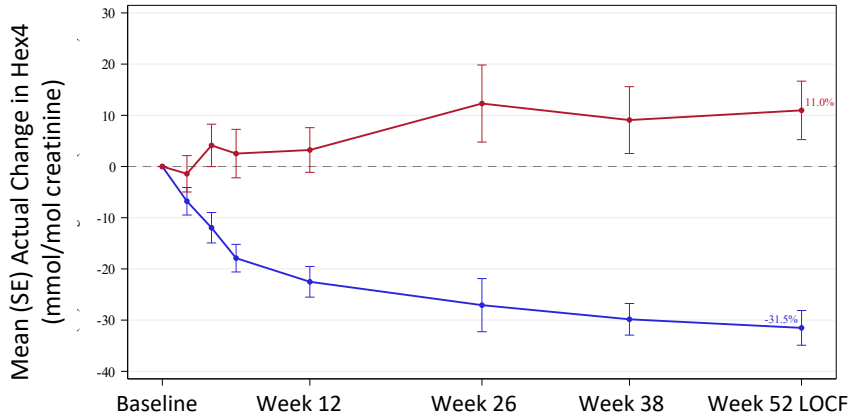
ERT=enzyme-replacement therapy; LOCF=last observation carried forward; SE, standard error.
GSGC total score is the sum of 4 tests and ranges from a minimum of 4 points (normal performance) to a maximum of 27 points (worst score).

Biomarker: Creatine Kinase (CK) and Urinary Hex4

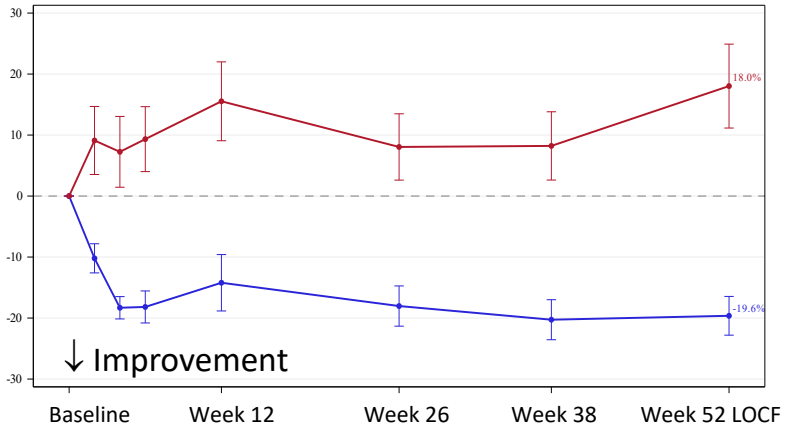
All Patients and ERT-Experienced Patients

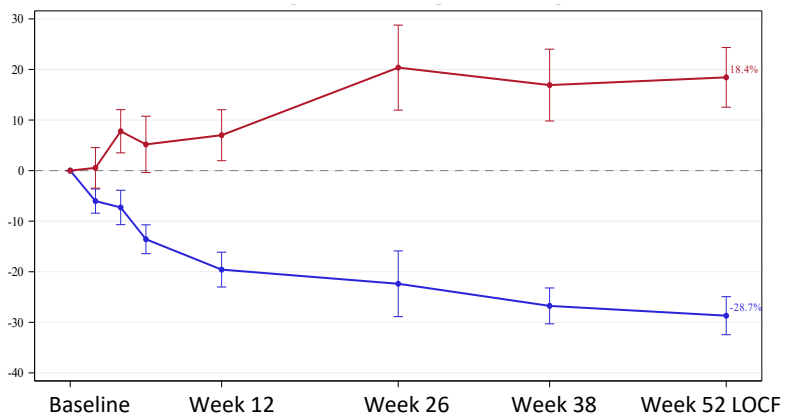
Overall Population





ERT-Experienced Population





In the overall and ERT-experienced populations, reductions in CK were greater with AT-GAA (nominal *P*-value <0.05)

In the overall and ERT-experienced populations, reductions in Hex4 were greater with AT-GAA (nominal *P*-value <0.05)

Treatment: Cipaglucosidase alfa/miglustat — Alglucosidase alfa/placebo

CK=creatine kinase; ERT=enzyme-replacement therapy; Hex4= hexose tetrasaccharide; LOCF=last observation carried forward; SE, standard error.

Primary, Key Secondary and Biomarker Endpoint Heat Map
Overall & ERT-Experienced Populations

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

Overall Population

	Alglucosidase alfa	AT-GAA
Motor Function		6MWD
		%Predicted 6MWD
		GSGC*
		10-meter walk*
		4-stair climb*
	Gowers	
		Rising from Chair
Pulmonary Function		FVC*
		MIP
		MEP
Muscle Strength		Lower MMT
		Upper MMT
		Total MMT
PROs		PROMIS-Physical
		PROMIS-Fatigue
Biomarkers		Hex4*
		CK*

ERT-Experienced

	Alglucosidase alfa	AT-GAA
Motor Function		6MWD*
		%Predicted 6MWD*
		GSGC*
		10-meter walk*
		4-stair climb
	Gowers	
		Rising from Chair
Pulmonary Function		FVC*
		MIP
		MEP
Muscle Strength		Lower MMT
		Upper MMT
		Total MMT
PROs		PROMIS-Physical
		PROMIS-Fatigue
Biomarkers		Hex4*
		CK*

6MWD=6-minute walk distance; CK=creatine kinase; FVC=forced vital capacity; GSGC=Gait, Stairs, Gowers, Chair; Hex4= hexose tetrasaccharide 4; LOCF=last observation carried forward; MEP=maximum expiratory pressure; MIP=maximal inspiratory pressure; MMT=manual muscle test; PRO=patient-reported outcome; PROMIS=Patient-reported Outcomes Measurement Information System.
Based on LOCF means; *Nominal P-value <0.05

Safety Summary

Safety profile was similar for AT-GAA and alglucosidase alfa

	AT-GAA n=85	Alglucosidase Alfa n=38
TEAEs	81 (95.3%)	37 (97.4%)
TEAEs Potentially Related to Treatment	26 (30.6%)	14 (36.8%)
Serious TEAEs	8 (9.4%)	1 (2.6%)
Serious TEAEs Potentially Related to Treatment	1 (1.2%)	0
TEAEs Leading to Study Withdrawal	2 (2.4%)	1 (2.6%)
TEAEs Leading to Death	0	0
IARs	21 (24.7%)	10 (26.3%)

- **TEAEs leading to withdrawal in the AT-GAA arm were 2 IARs, 1 of which was a serious AE**
- **TEAE leading to withdrawal in the alglucosidase arm was due to stroke (unrelated)**
- **Overall safety profile of AT-GAA is similar to alglucosidase alfa**

AE=adverse event; IAR=infusion-associated reaction; TEAE=treatment-emergent adverse event.

Conclusions

- In the overall study population of ERT-naive and ERT-experienced patients, AT-GAA showed clinically meaningful improvements on motor and respiratory functions and biomarkers, compared with alglucosidase alfa
- Among the ERT-experienced patients (mean ERT duration of 7.4 years), those randomized to AT-GAA showed clinically meaningful improvements on motor and respiratory functions and biomarkers, compared with patients randomized to alglucosidase alfa
- Of the 17 efficacy and biomarker endpoints assessed, 16 favored AT-GAA compared with alglucosidase alfa in both the overall study population and ERT-experienced patients
- AT-GAA demonstrated a similar safety profile to that of alglucosidase alfa

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