GAIN THERAPEUTICS

Corporate Presentation

July 2025

NASDAQ: GANX

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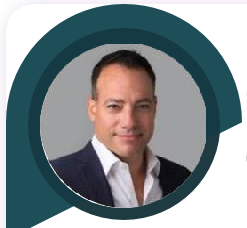
GANX Corporate Highlights

Lead Product GT-02287 Being Evaluated in Parkinson's Disease Patients	• **Allosteric modulator** of glucocerebrosidase enzyme (GCase) • **Disease modifying potential**: altering progression of motor/cognitive decline in GBA1 and iPD • **Safe and well tolerated** in Phase 1 SAD/MAD study and demonstrated target engagement • **Phase 1b trial in GBA1 and idiopathic PD patients ongoing**
Multiple Assets in Discovery and Preclinical Development	• Assets discovered and developed with our **proprietary Magellan AI platform** • Initial disease targets include neurodegenerative diseases, lysosomal storage disorders including Gaucher disease as well as metabolic disease and solid tumors
Strong intellectual property estate	• GT-02287 composition of matter patent application with term through 2038 not including Hatch Waxman extension • Patent applications for 5 NCE families under review
Upcoming Milestones	• GT-02287 Phase 1b full biomarker analysis in CSF and blood – **4Q 2025** • GT-02287 Phase 2 planning (US/EU) - **2H 2025** • IND Submission **– 2H 2025**

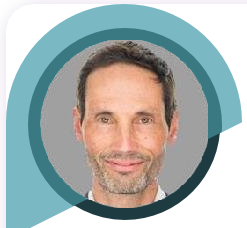
Leadership: Extensive Biotech And Pharma Experience



Gene Mack, MBA

Chief Executive Officer

Imcyse · OncoC4 · biom'up · nicox · ΣELLAS · MERCK Oncolmmune · MIZUHO · HSBC · LAZARD



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Chief Medical Officer

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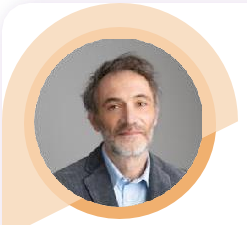


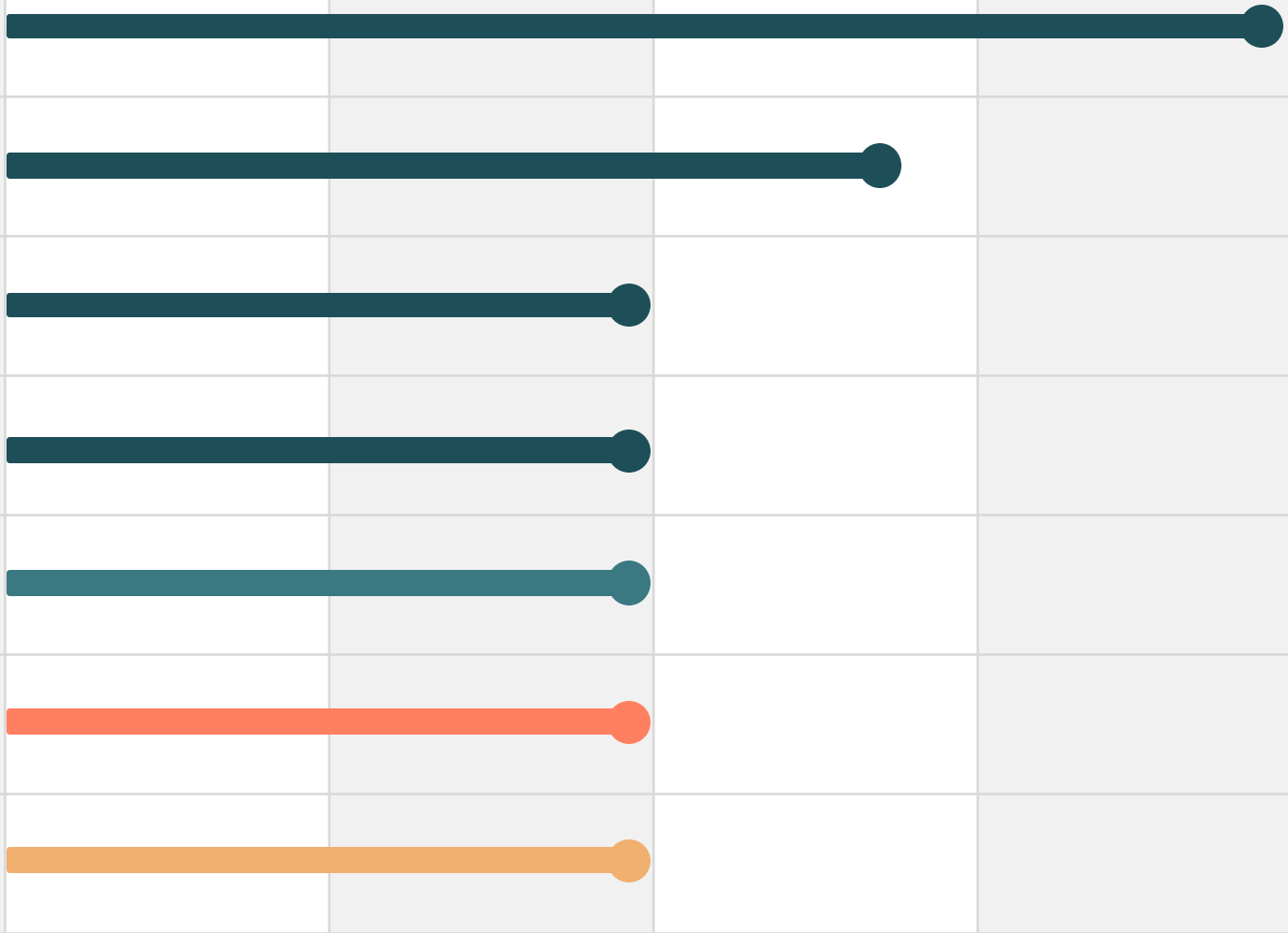
Terenzio Ignoni, PharmD

SVP Technical Operations

Jazz Pharmaceuticals · Vicuron Pharmaceuticals · gentium · Zambon

Gain Therapeutics Pipeline

ASSET	INDICATION	TARGET	DISCOVERY	RESEARCH	PRECLINICAL	PHASE 1
GT-02287	*Parkinson's Disease*	GCase				
	Gaucher's Disease	GCase				
	Dementia with Lewy Bodies	GCase				
	Alzheimer's Disease	GCase				
Multiple Undisclosed	*Lysosomal Storage Disorders*	GALC GLB1				
Undisclosed	*Metabolic Diseases*	AAT				
Multiple Undisclosed	*Oncology: Solid Tumors*	DDR2				



Lead Clinical Program

GT-02287

GBA1 Parkinson's Disease

Parkinson's Disease – Market Opportunity

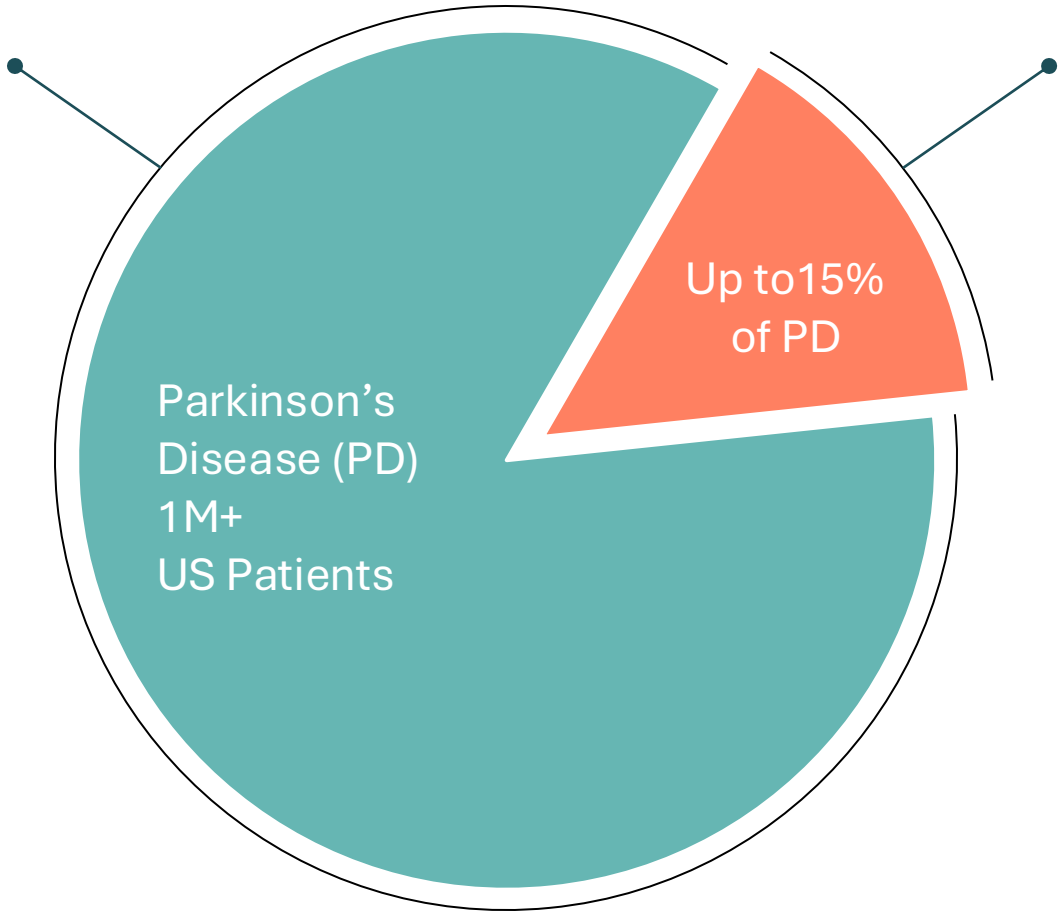
Parkinson's Disease

US Market Potential:

$4B

Parkinson's disease is the second most common neurodegenerative disease[1]

But current therapies only treat symptoms and do not prevent disease progression



GBA1-Parkinson's Disease

US Market Potential:

$3B

Genetically defined subpopulation of Parkinson's disease

GBA1 mutations cause misfolding of an important enzyme called Gcase

GBA1-PD patients experience earlier disease onset and more severe disease with faster decline in motor and cognition functions.

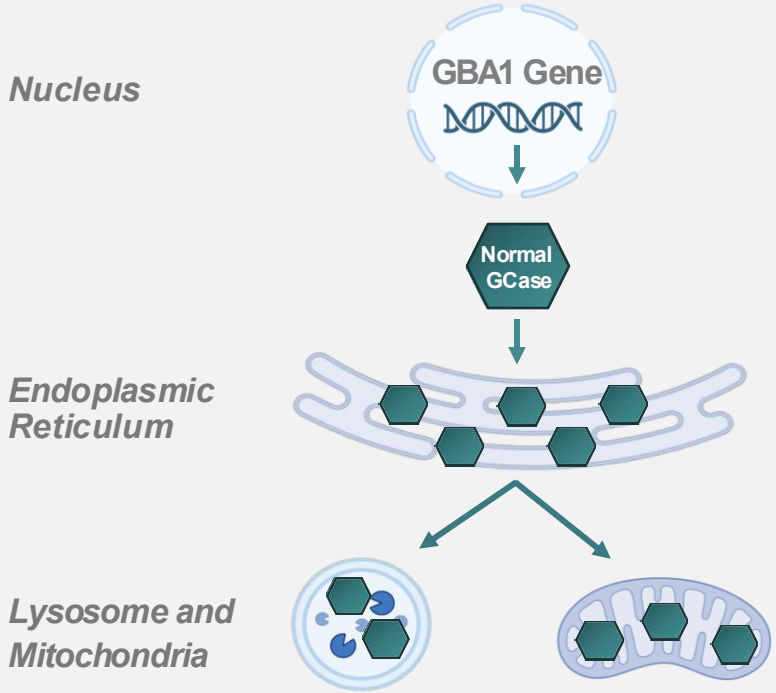
A therapy for disease progression in this subpopulation is needed

Largest genetic risk factor for development of Parkinson's disease

1."Parkinson's Disease: Challenges, Progress, and Promise." National Institute of Neurological Disorders and Stroke, U.S. Department of Health and Human Services
2. Smith L and Schapira AHV. GBA Variants and Parkinson Disease: Mechanisms and Treatments. Cells. 2022 Apr 8;11(8):1261. doi: 10.3390/cells11081261

GCase Plays Integral Role in Organelle and Cellular Health

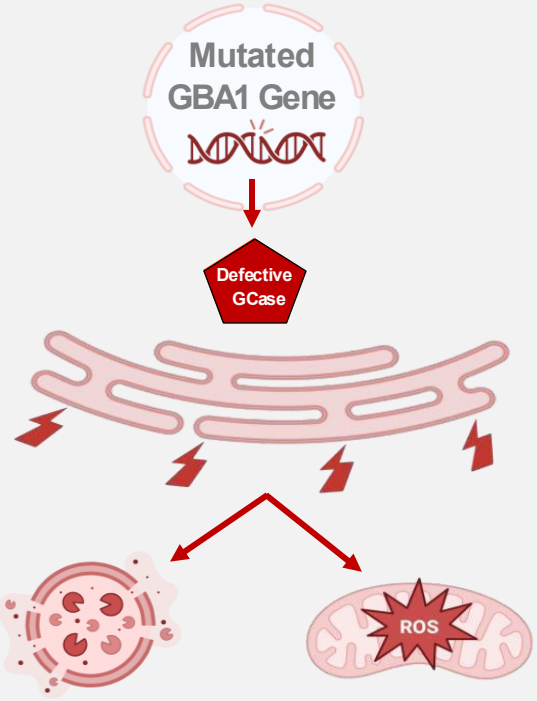
Healthy Dopaminergic Neuron



GCase Maintains Cell Health

- Depletes toxic lysosomal substrates
- Stabilizes mitochondrial respiratory complex I

Diseased Dopaminergic Neuron



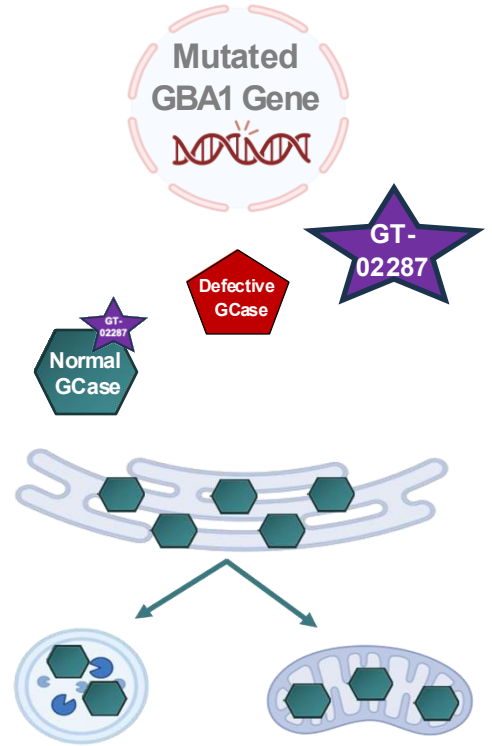
1. GCase protein misfolding
2. Increased ER stress and ER-associated protein degradation
3. Lysosomal and mitochondrial dysfunction
   Accumulation of toxic lysosomal lipid substrates resulting from reduced GCase activity
   Alpha synuclein aggregation

Dysfunctional GCase Increases PD Risk

- Dysfunctional GCase triggers disease cascade affecting multiple organelle functions leading to core motor and cognitive dysfunction

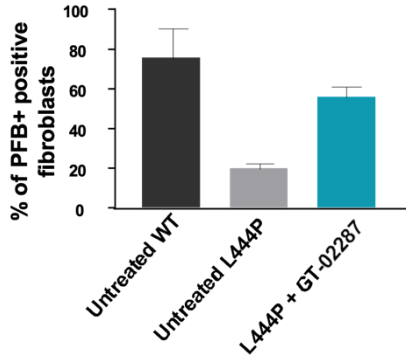
Allosteric modulator GT-02287 restores GCase function, which improves disease cascade and neuronal survival

Dopaminergic Neuron with Restored GCase Function

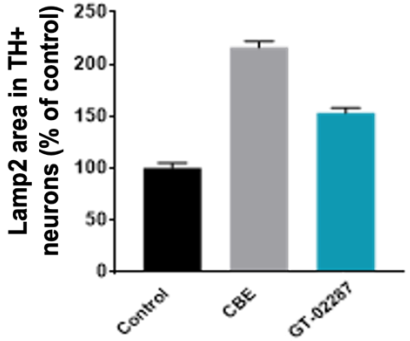


- Corrects protein misfolding
- Restores enzymatic activity

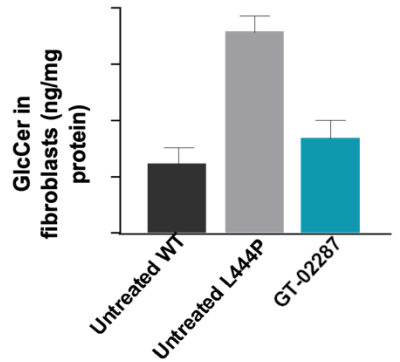
GCase Activity Restoration



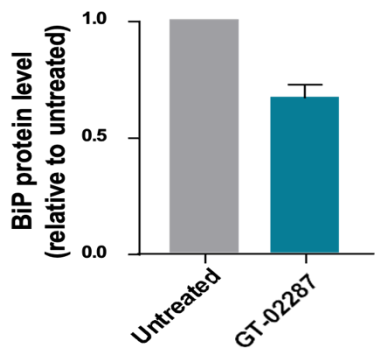
Restored Lysosomal Function



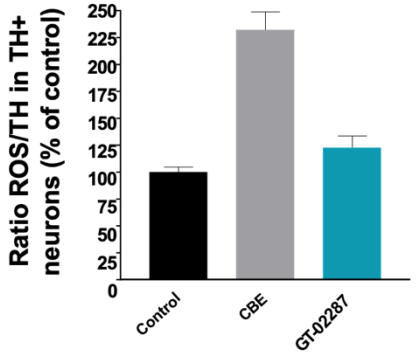
Toxic Substrate Depletion



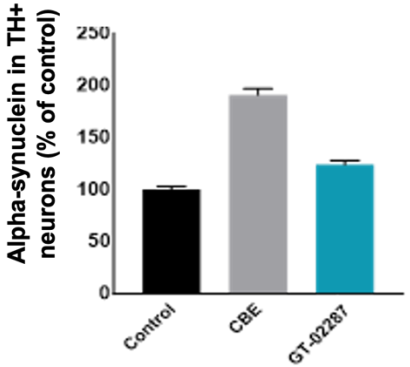
Reduced ER Stress



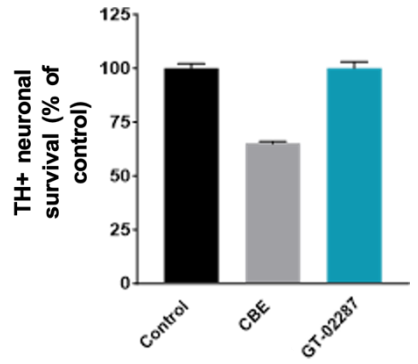
Restored Mitochondrial Function



Alpha-Synuclein Depletion

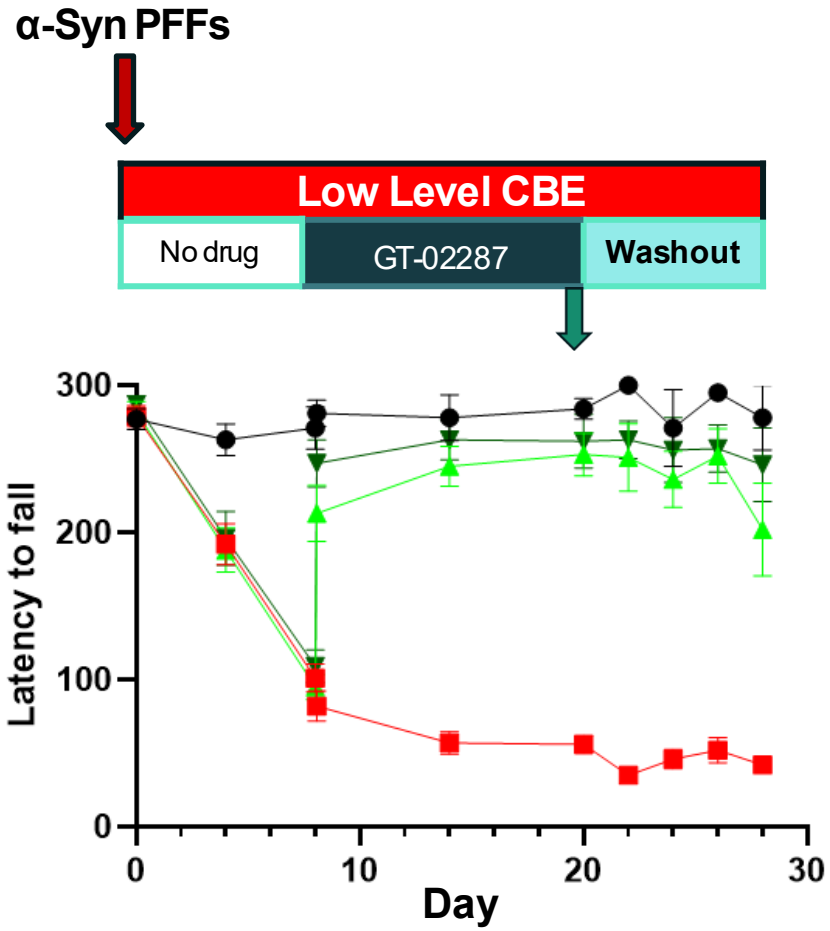


Neuronal Survival

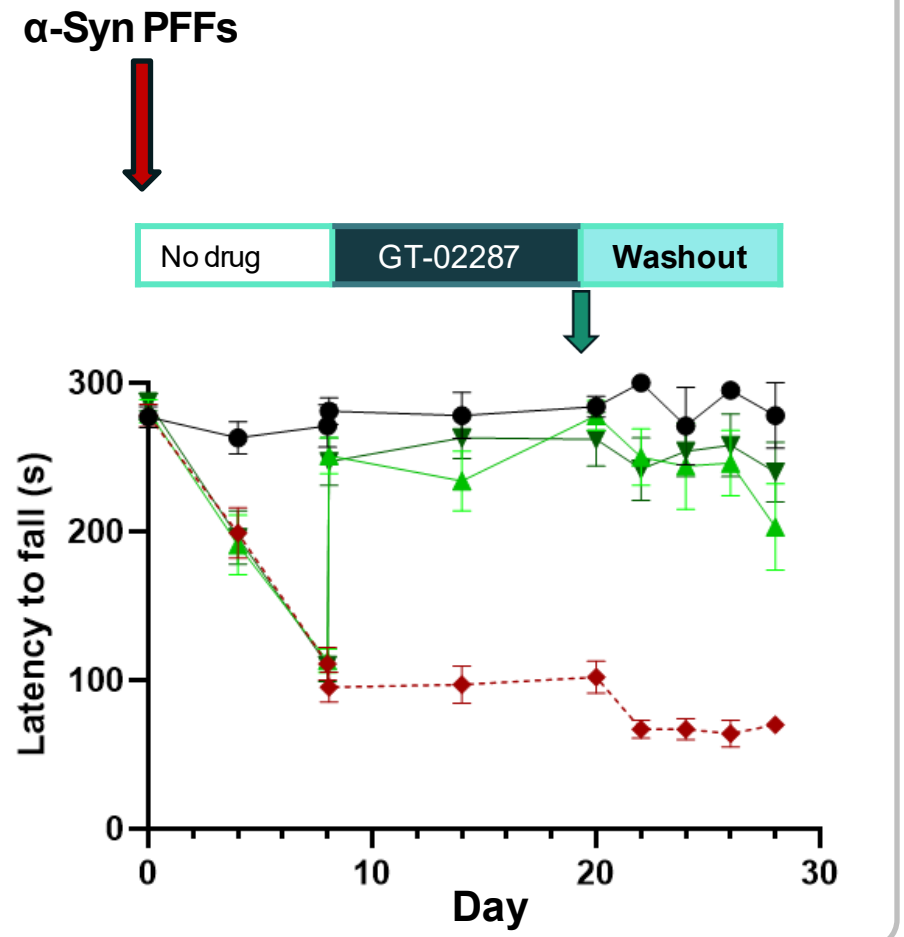


GT-02287 displays a rescue and disease-modifying effect in animal models of GBA1 and iPD

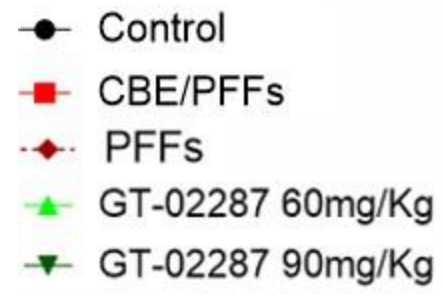
GBA1- PD Model (CBE+PFFs)



Idiopathic PD Model (PFFs only)



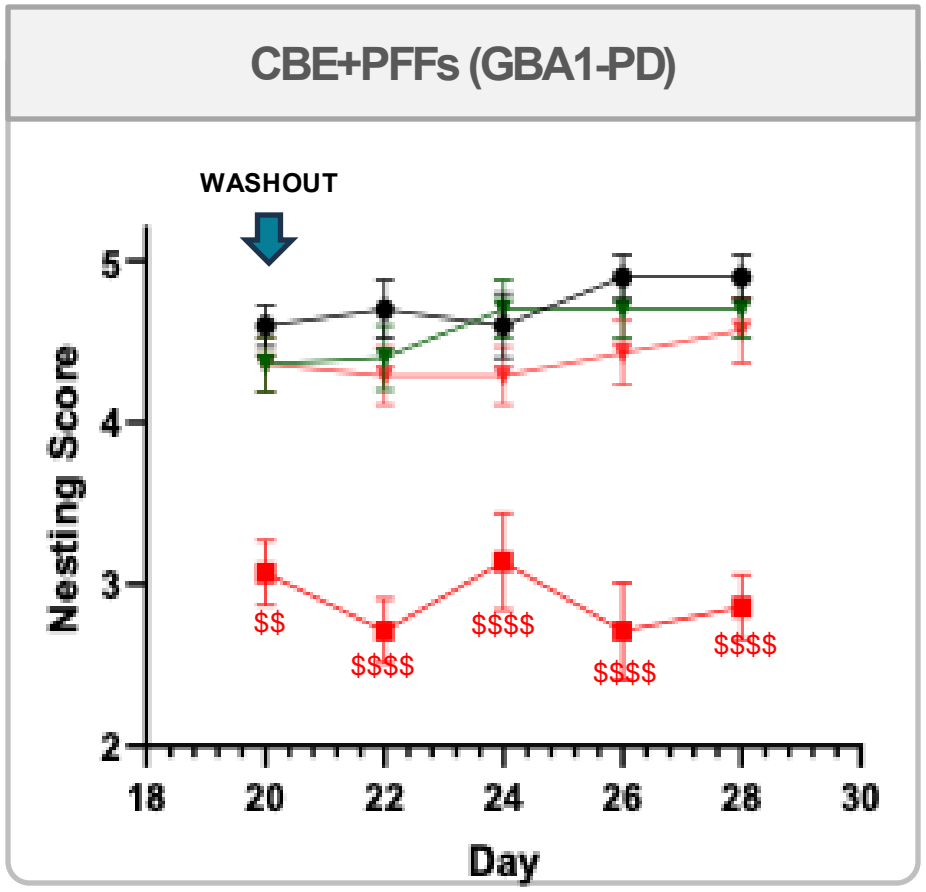
Mouse Wire Hang Rescue & Washout

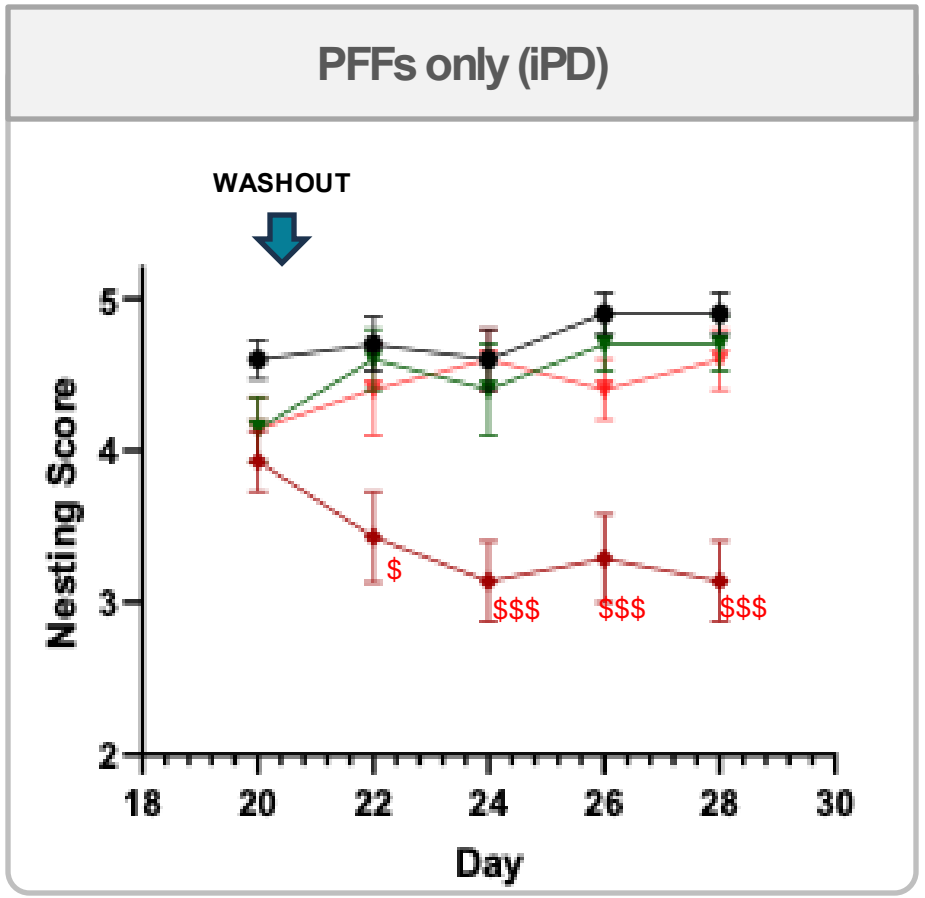


Data is shown as Mean ± S.E.M.(n=14 till day 20, then n=7)

Nesting Score Unaffected by Drug Washout

Evidence of disease modifying effect on complex behaviors





Mouse Nesting Building Washout

- Control
- CBE+PFFs
- PFFs
- GT-02287 90mg/Kg
- GT-02287 90mg/Kg + washout

Data is shown as Mean ± S.E.M.(n= 7). $Significant difference as compared to sham control group
One-way ANOVA followed by Dunnett's Multiple Comparison Test. $P < 0.05, $$P < 0.01 , $$$P < 0.001 & $$$$P < 0.0001.

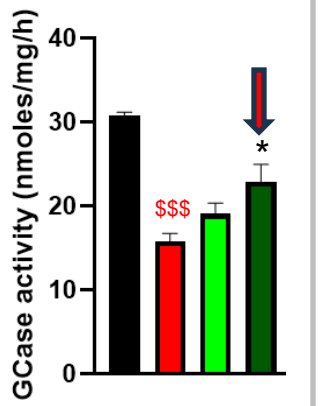
Brain biomarker changes maintained following GT-02287 washout

Data from CBE+PFF Model support GT-02287's disease modifying effect

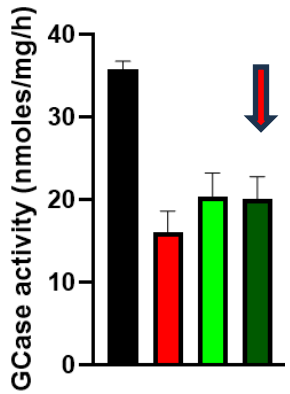
No Washout

Washout Day 20 – Day 29

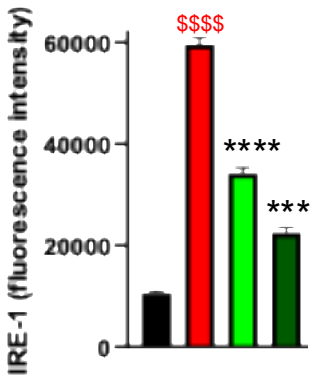
GCase Activity



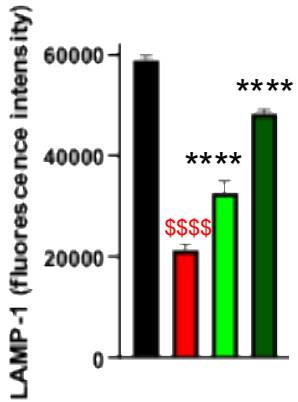
GCase Activity



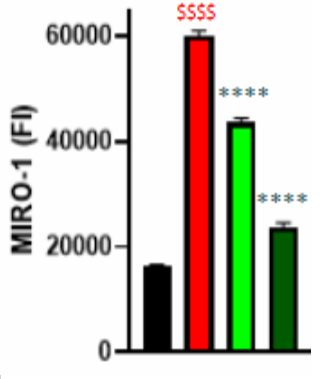
IRE-1
ER Stress



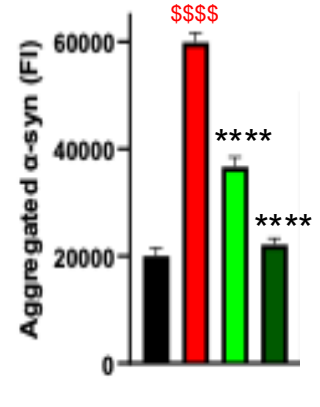
LAMP1
Lysosomal Integrity



MIRO-1
Mitochondrial Health



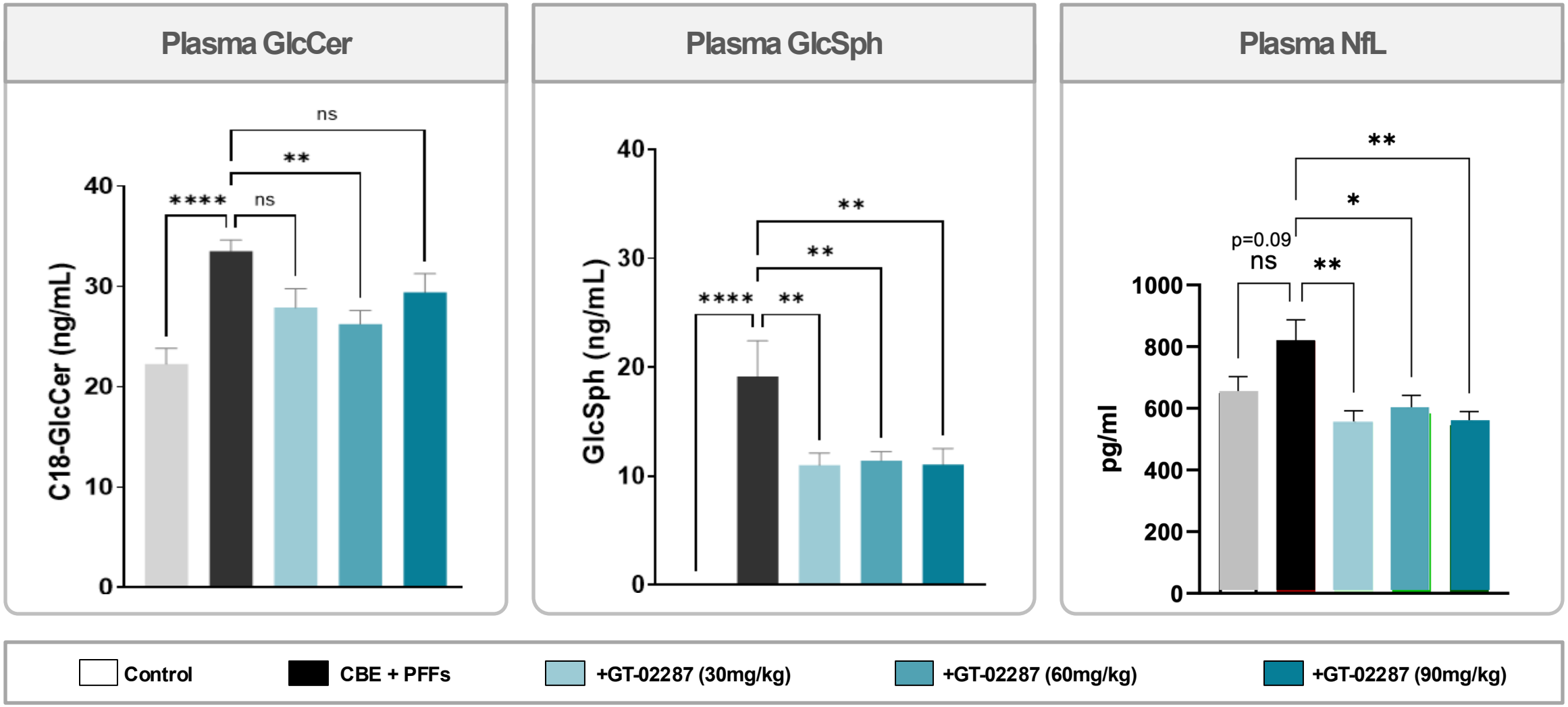
Aggregated a-Syn



Control | CBE + PFFs | +GT-02287 (60mg/kg) | +GT-02287 (90mg/kg)

Data is shown as Mean ± S.E.M. $Significant difference as compared to sham control group * Significant difference as compared to α-synuclein PFF+CBE. One-way ANOVA followed by Dunnett's Multiple Comparison Test. $/*P < 0.05, $$/**P < 0.01 , $$$/***P < 0.001 & $$$$/****P < 0.0001.

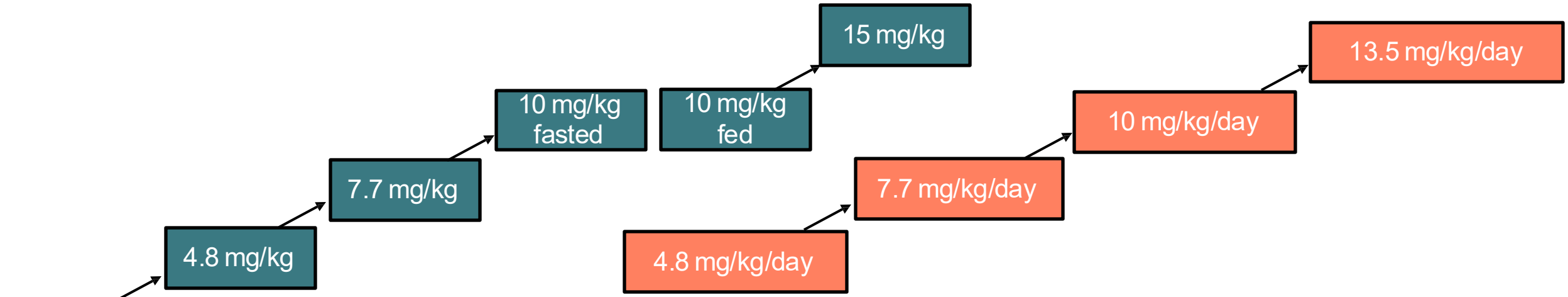
Translational plasma-based biomarkers of GT-02287 rescue effect



Data shown as Mean ± S.E.M. (n=10). Significant difference as compared to PFFs or PFFs + CBE.
One-way ANOVA followed by Dunnett's Multiple Comparison test. ns (non significant). * < P 0.05, ** < P 0.01, **** < 0.0001.

Single- and Multiple-ascending Dose First-in-human Phase 1 Study

Participants	SAD/MAD Endpoints	MAD Cohort 4
• Healthy men and women ages 18-65 • 8 subjects per cohort • 2 placebo; 6 active	• Treatment-emergent adverse events • Clinical labs, vital signs, ECGs, C-SSRS • Plasma pharmacokinetics	• CSF drug levels • GCase activity in dry blood spots



5 SAD cohorts
Food effect at 10 mg/kg

4 MAD cohorts
Daily dosing for 14 days

GT-02287 was Generally Well-Tolerated, No Serious Adverse Events Observed

TEAE	SAD GT-02287 N=30	SAD Placebo N=10	MAD GT-02287 N=25	MAD Placebo N=8	All GT-02287 N=55	All Placebo N=18
Any	17 (56.7%)	4 (40.0%)	16 (64.0%)	4 (50.0%)	33 (60%)	8 (44%)
Related to Study Drug	10 (33.3%)	1 (10%)	11 (44.0%)	0	21 (38%)	1 (6%)
CTCAE Grade 1 (Mild)	15 (50.0%)	4 (40.0%)	16 (64.0%)	4 (50.0%)	31 (56%)	8 (44%)
CTCAE Grade 2 (Moderate)	4 (13.3%)	1 (10.0%)	4 (16.0%)	2 (25.0%)	8 (15%)	3 (17%)
CTCAE Grade 3 (Severe)	0	0	0	0	0	0
Serious	0	0	0	0	0	0
Leading to Discontinuation	0	0	0	0	0	0

Adverse Event Profile

Most common TEAEs in MAD
• Nausea 32%
• Abdominal Pain 8%
• Diarrhea 8%
• Headache 8%
Nausea
• >90% of events were mild
• >90% of events were <3h in duration
• Incidence increased with dose level
• Incidence decreased with continued dosing

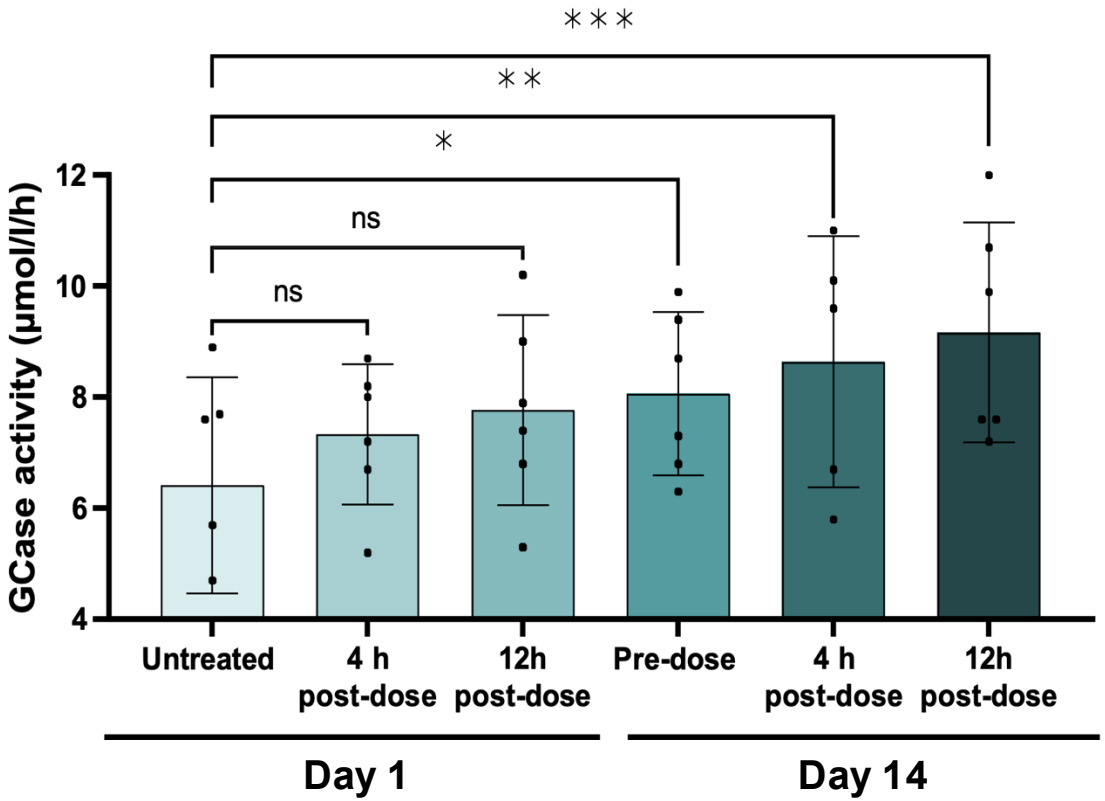
GT-02287 Demonstrates GCase Target Engagement In Healthy Volunteers

Healthy Volunteer Results

- GCase activity in dry blood spots was measured in MAD Cohort 4
- In GT-02287 subjects, 5 out of 6 had increased GCase activity
- In placebo subjects, no increase was observed (+4% change from baseline)

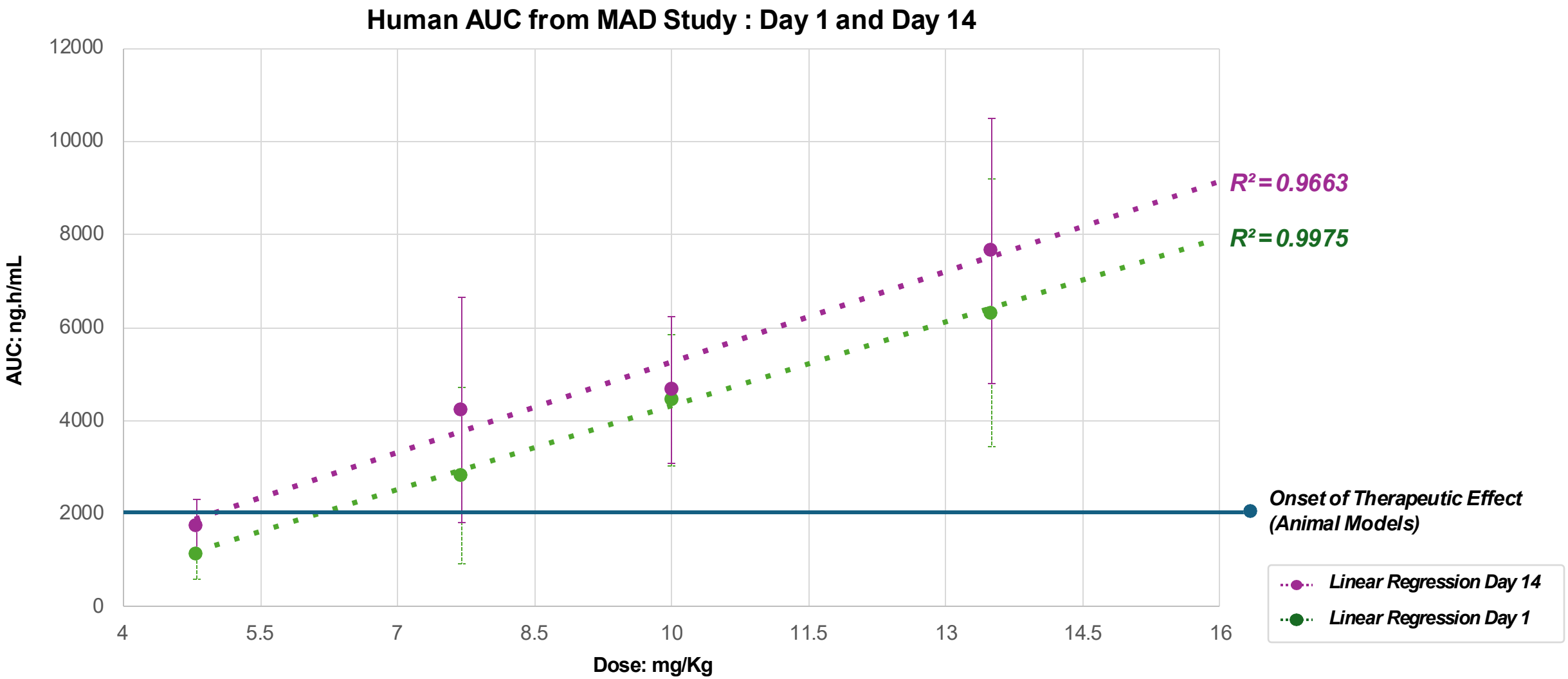
53% increase in GCase activity observed by Day 14 (p<0.001)

GCase Activity in Dried Blood Spots (DBS)



One-way, paired, repeated measures ANOVA
**p <0.05; **p <0.01; ***p <0.001*

Therapeutic Range: Phase 1 PK Data in Healthy Volunteers



GT-02287 Demonstrates CNS Exposure Comparable to that Observed in Rodents

Species	Mean CSF level (ng/mL)	Total brain level (ng/mL)	Mean plasma Cmax (ng/mL) mean Day 14	Timepoint	Dose (mg/kg)
Human (MAD4)	3.1 (1.7-4.9)	Not sampled	850	Day 13	13.5 PO
Mouse	4	6592	2414	15 min	10 IV
Rat	3	2441	680	1 hour	30 PO

- CSF levels in Humans comparable to those observed at efficacious dose levels in rodents
- CSF levels are low in all species due to low aqueous solubility and high protein binding
- Observed total brain levels in rodents are 2-8 times higher than total plasma levels

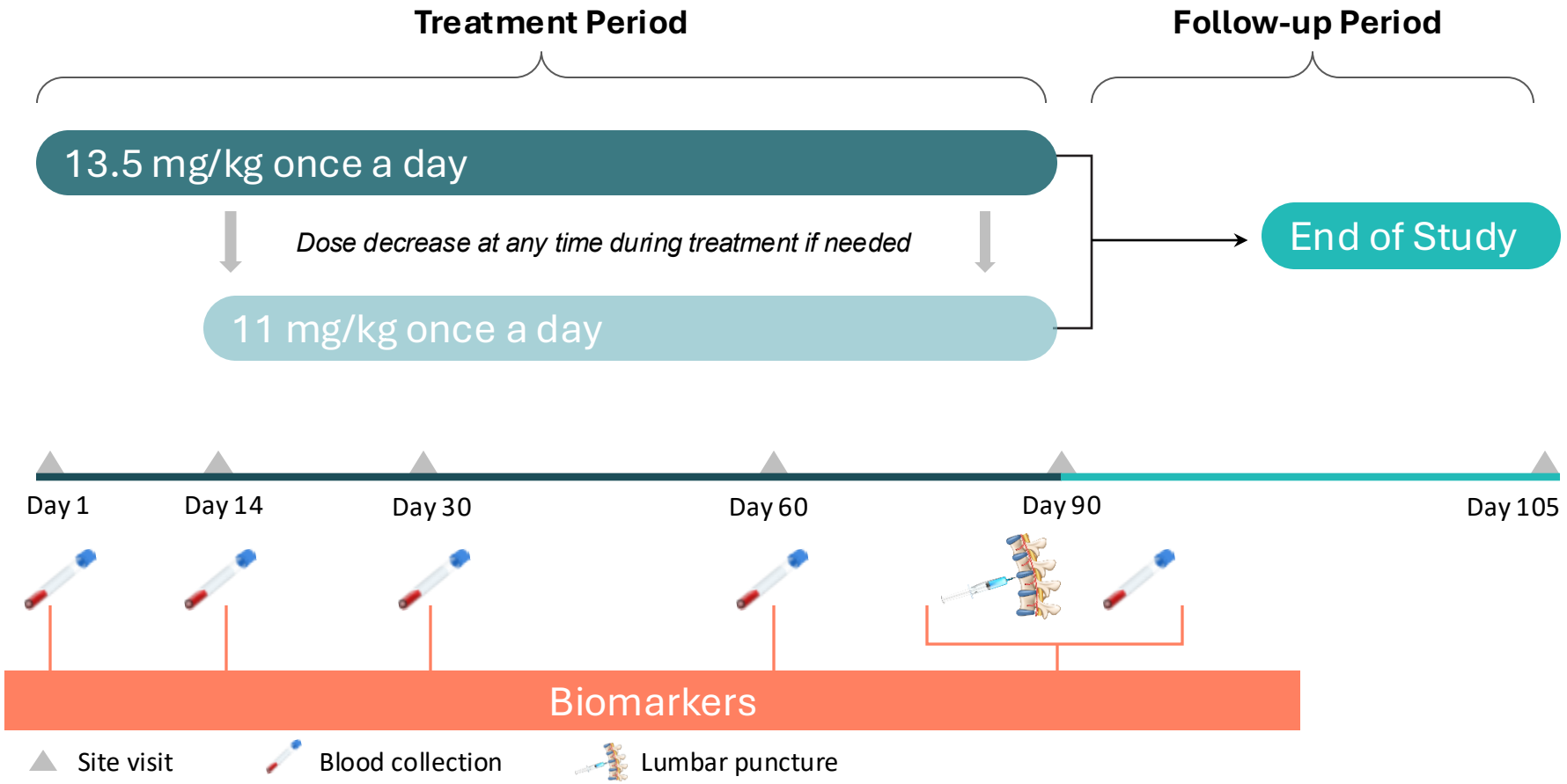
Upcoming Milestones

Q4 2025	Phase 1b full biomarker analysis in CSF and blood
2H 2025	Start of Phase 2 planning and regulatory filings in US/EU

Design of Phase 1b Trial in Parkinson's Disease Patients

An Open-label Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of GT-02287 in Participants with Parkinson's Disease With or Without a Pathogenic GBA1 Mutation

- Open-Label, single-arm, multicenter study
- 16 patients enrolled as of June 30,2025
- 90-day treatment duration
- 7 sites in Australia with potential to expand to other geographies



Phase 1b Study Objectives

	Study Objectives	Endpoints
Primary	To evaluate the safety and tolerability of GT-02287	
Secondary	To characterize the single-dose and steady state plasma PK profile of GT-02287	
Exploratory	To assess levels of GT-02287 in CSF after at least 12 weeks of daily administration in participants with PD	Concentration of GT-02287 in CSF at 4 hours post-dose after at least 12 weeks of daily administration of GT-02287
	Pharmacodynamic response to GT-02287 via biomarkers analysis of plasma, whole blood, blood cells, and CSF samples	• Gcase activity • Sphingolipid levels • Lysosomal and mitochondrial markers • Inflammatory markers
	To explore the effect of GT-02287 on scores from selected clinical scales and questionnaires over a 90-day treatment	Movement Disorder Society Unified Parkinson's Disease Rating Scale **(MDS-UPDRS, OFF state)** and other standard functional scales including MoCA, ADL, etc.

GT-02287 has Best-in-Class Profile for GBA1-Parkinson's Disease

	Effect on Disease Cascade	GAIN THERAPEUTICS GT-02287	Bial BIA 28-6156	VANQUA BIO VQ-101
GCase Mechanism of Action	Increases Lysosomal GCase Activity	✓	?	✓
	Reduces ER Stress	✓	?	?
	Reduces Toxic Lipid Substrates	✓	✓ ✗	✓
	Reduces Aggregated α-Synuclein	✓	?	✓
	Improves Lysosomal Function	✓	✓	✓
	Improves Mitochondrial Function	✓	?	?
	Reduces Neuroinflammation	✓	?	?
Disease-Modifying Effect	Provides Neuroprotection	✓	?	?
	Increases Dopamine Levels	✓	?	?
	Restores Motor Function	✓	?	?
	Improves Cognitive Function	✓	?	?

Company Background

Corporate Background

- Established in 2017
- 27 employees in three locations: HQ in Bethesda, Maryland, Lugano, Switzerland, Barcelona, Spain
- Founder and Executive Chairman: Dr. Khalid Islam

Analyst Coverage	
BTIG	*Tom Shrader, Ph.D., CFA*
Oppenheimer & Co	*Jay Olson, CFA*
H.C. Wainwright	*Ram Selveraju, Ph.D.*
Chardan	*Kaey Nakae, CFA*
Maxim	*Jason McCarthy, Ph.D.*
ROTH	*Boobalan Pachaiyappan, Ph.D.*
Scotiabank	*Louise Chen, MBA*

Financial and Stock Data

IPO (NASDAQ: GANX)

- March 2021
- Led by BTIG and Oppenheimer & Co.

CAPITAL STRUCTURE

- 28.7 million shares outstanding
- No debt*

CASH POSITION

- $9.1 million as of March 31, 2025

GRANT SUPPORT

- Michael J. Fox Foundation for Parkinson's Research
- The Silverstein Foundation for Parkinson's with GBA
- Innosuisse (Swiss Innovation Agency)

THE MICHAEL J. FOX FOUNDATION FOR PARKINSON'S RESEARCH

The Silverstein Foundation for Parkinson's with GBA

Schweizerische Eidgenossenschaft
Confédération suisse
Confederazione Svizzera
Confederaziun svizra
Swiss Confederation
Innosuisse – Swiss Innovation Agency