GAIN THERAPEUTICS

Corporate Presentation

January 2026

NASDAQ: GANX

Forward-Looking Statements

Certain statements set forth in this presentation are forward-looking and reflect the Company's plans, beliefs, expectations and current views with respect to, among other things, future events and financial performance (collectively referred to herein as "forward-looking statements"). Forward-looking statements can be identified by the fact that they do not relate strictly to historical or current facts and are often characterized by the use of words such as "believe," "can," "could," "potential," "plan," "predict," "goals," "seek," "should," "may," "may have," "would," "estimate," "continue," "anticipate," "intend," "expect" or by discussions of strategy, plans or intentions. Such forward-looking statements involve known and unknown risks, uncertainties, assumptions and other important factors that could cause our actual results, performance or achievements or industry results to differ materially from historical results or any future results, performance or achievements expressed, suggested or implied by such forward-looking statements.

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Industry Information Market data and industry information used throughout this presentation are based on management's knowledge of the industry and the good faith estimates of management. We also relied, to the extent available, upon management's review of independent industry surveys and publications and other publicly available information prepared by a number of third-party sources. All of the market data and industry information used in this presentation involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. Although we believe that these sources are reliable, we cannot guarantee the accuracy or completeness of this information, and we have not independently verified this information. While we believe the estimated market position, market opportunity and market size information included in this presentation are generally reliable, such information, which is derived in part from management's estimates and beliefs, is inherently uncertain and imprecise. No representations or warranties are made by the Company or any of its affiliates as to the accuracy of any such statements or projections. Projections, assumptions and estimates of our future performance and the future performance of the industry in which we operate are necessarily subject to a high degree of uncertainty and risk due to a variety of factors, including those described above. These and other factors could cause results to differ materially from those expressed in our estimates and beliefs and in the estimates prepared by independent parties.

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
- **Phase 1b trial in GBA1 and idiopathic PD patients ongoing; study extension has commenced**
- **Initial results from Phase 1b suggest GT-02287 has a disease-slowing effect**

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

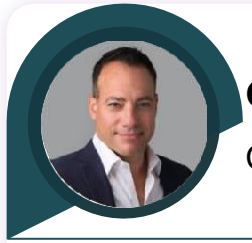
Strong intellectual property estate

- 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

- IND Submission **– 2H 2025**
- GT-02287 Phase 1b study extension analysis – **2H 2026**
- Commencement of Phase 2 in people with Parkinson's disease – **2H 2026**

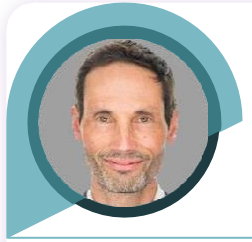
Leadership: Extensive Biotech And Pharma Experience



Gene Mack, MBA

Chief Executive Officer

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



Jonas Hannestad, MD, PhD

Chief Medical Officer

TRANQUIS THERAPEUTICS · ucb · CAPACITY BIO THE MITOPHAGY COMPANY · ALKAHEST · DENALI

Gianluca Fuggetta

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pwc

Joanne Taylor, PhD

SVP Research

Prescient · Eisai · ETH zürich

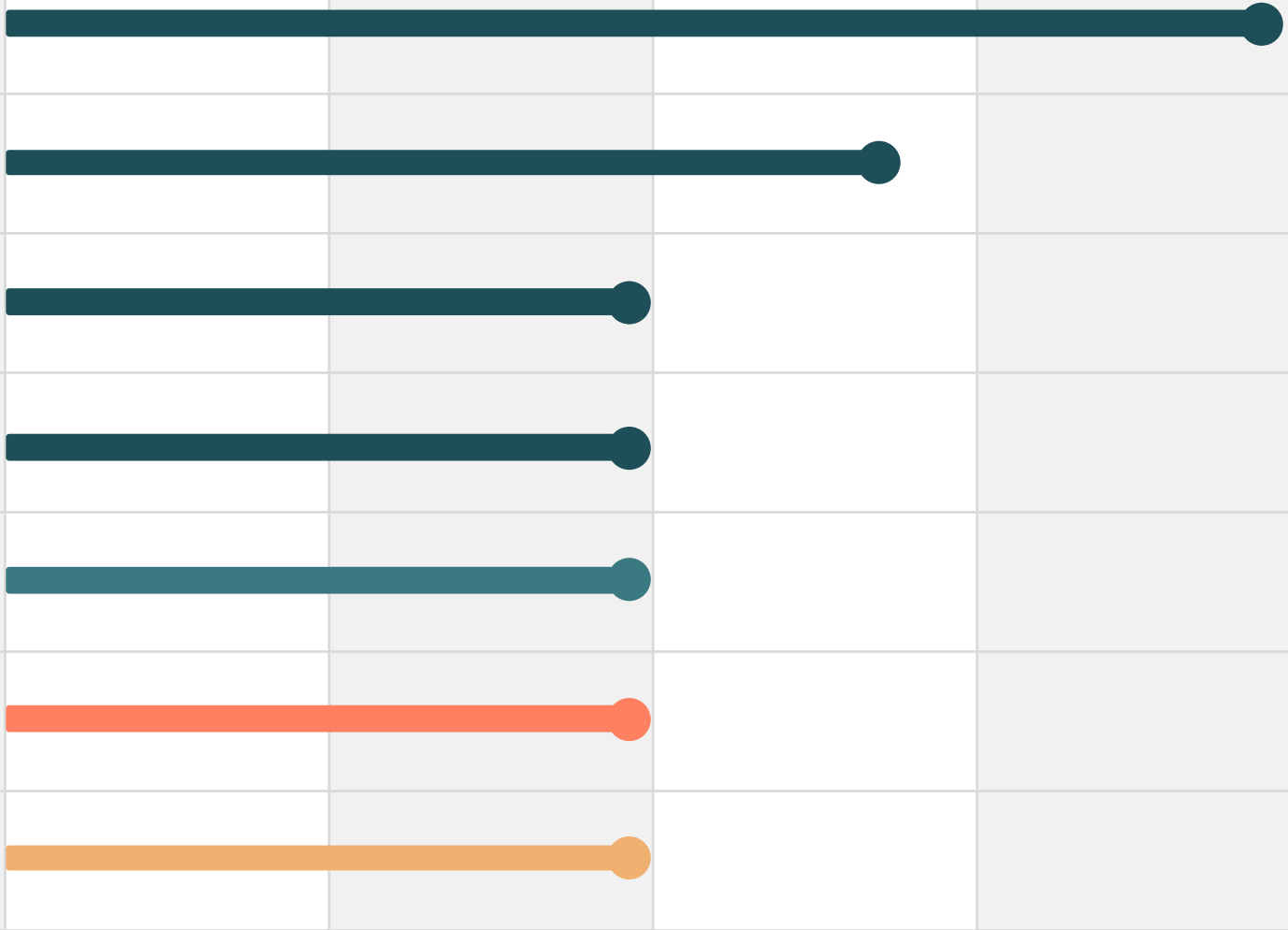
Terenzio Ignoni, PharmD

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

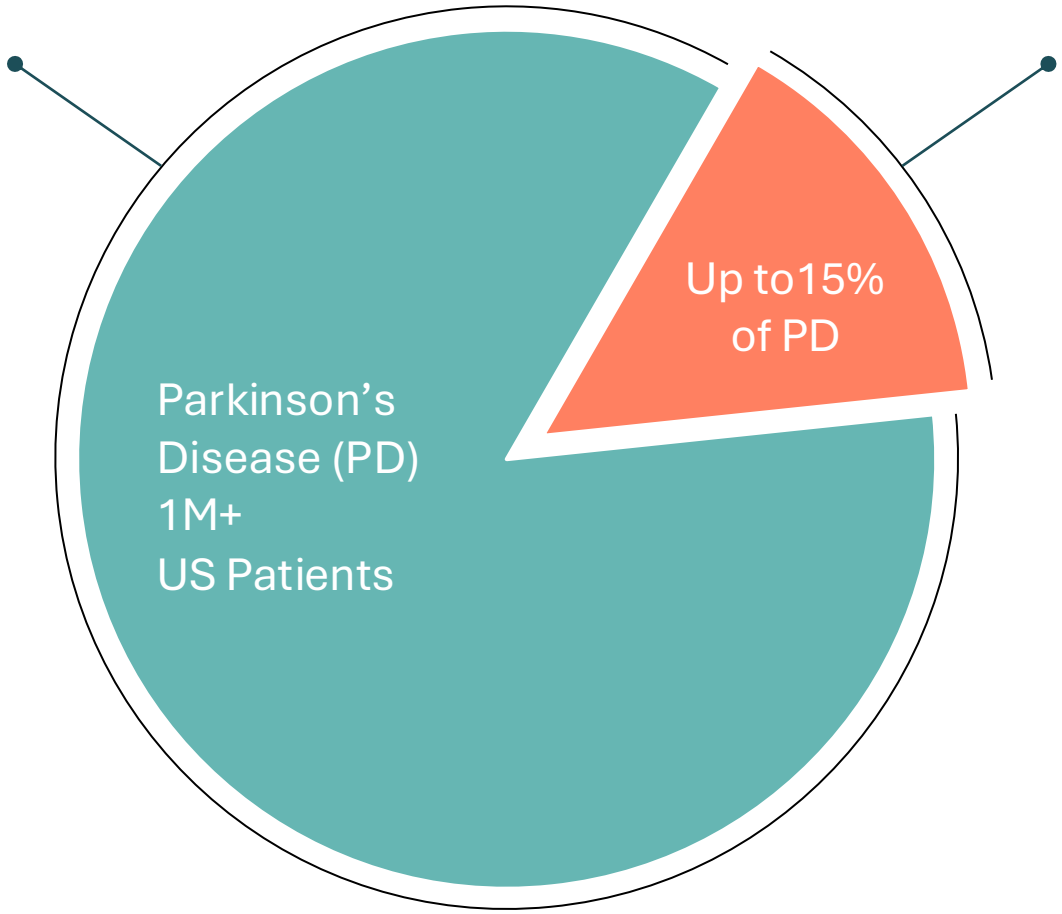
Parkinson’s Disease

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.

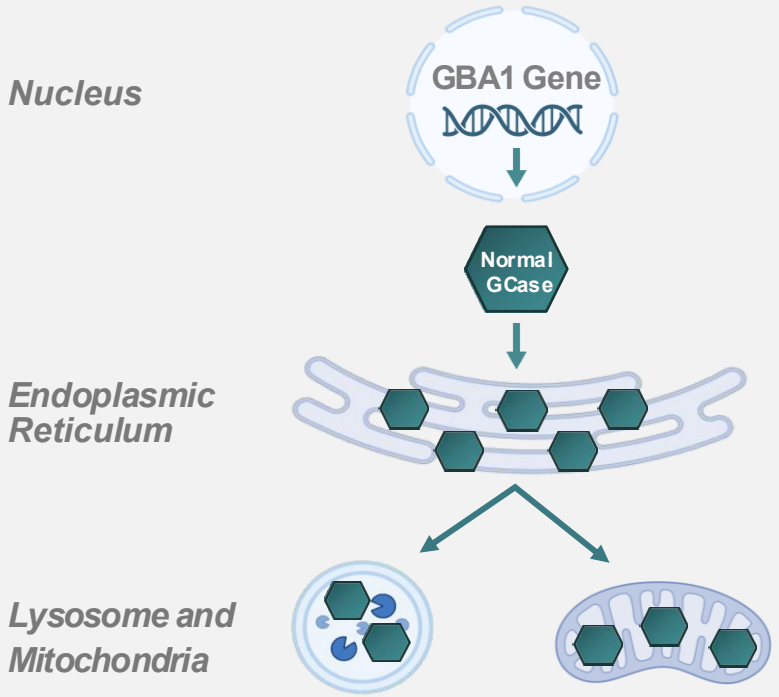
A therapy for disease progression in this subpopulation is needed

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

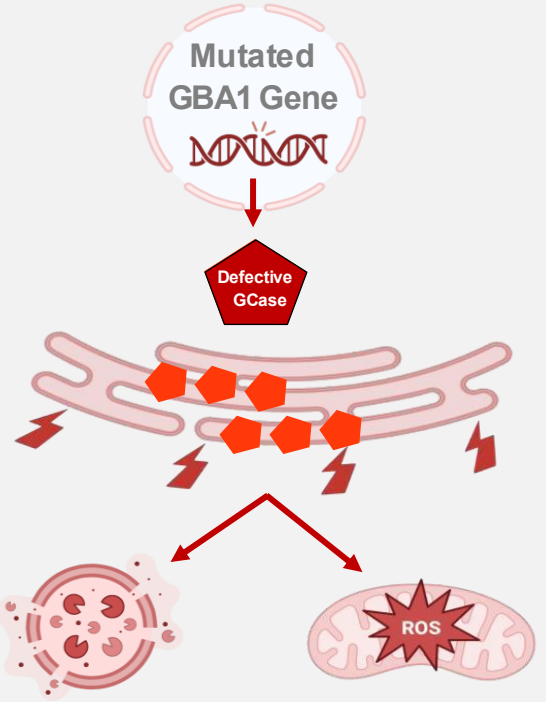
Healthy Dopaminergic Neuron



GCase Maintains Cell Health

- Depletes toxic lysosomal substrates
- Stabilizes mitochondrial respiratory complex I

Diseased Dopaminergic Neuron



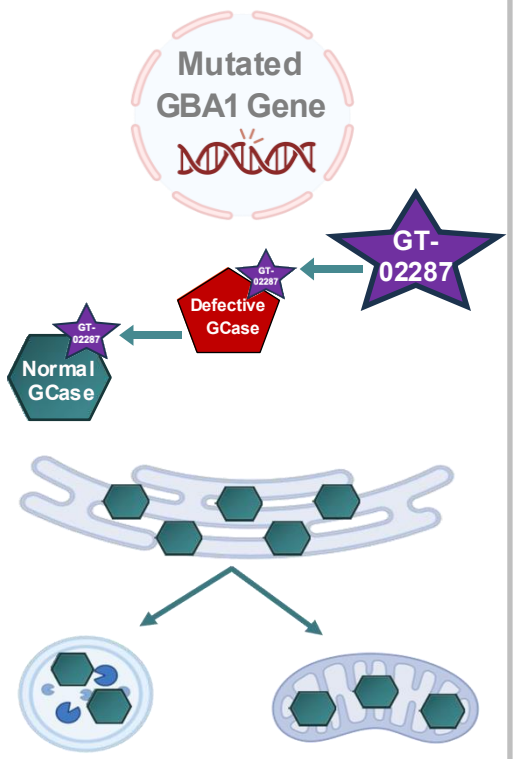
1. GCase protein misfolding
2. Increased ER stress and ER-associated protein degradation
3. Lysosomal and mitochondrial dysfunction
   Accumulation of toxic lysosomal lipid substrates resulting from reduced GCase activity
   Alpha synuclein aggregation

Dysfunctional GCase Increases PD Risk

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

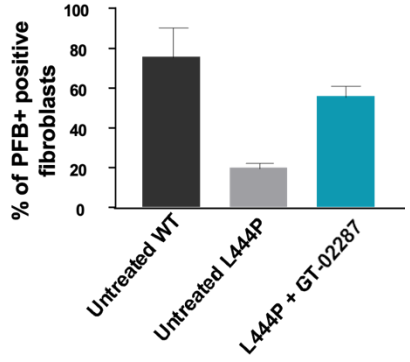
Allosteric modulator GT-02287 restores GCase function, which improves disease cascade and neuronal survival

Dopaminergic Neuron with Restored GCase Function

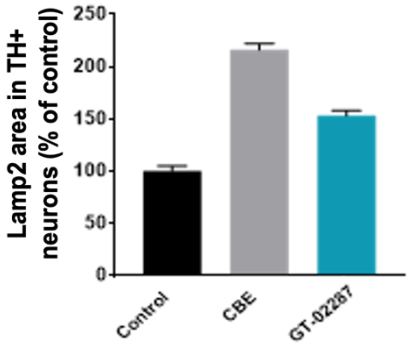


- Corrects protein misfolding
- Restores enzymatic activity

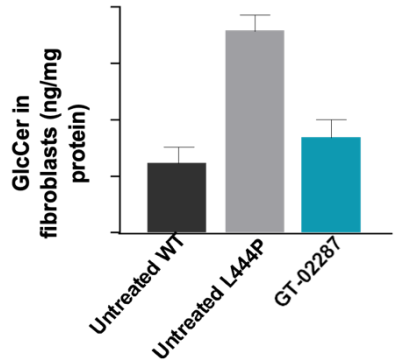
GCase Activity Restoration



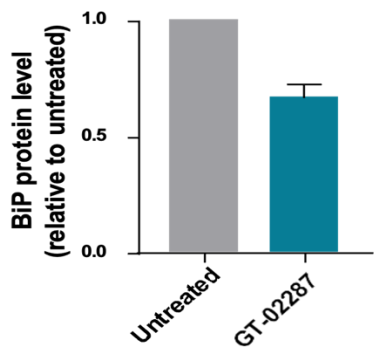
Restored Lysosomal Function



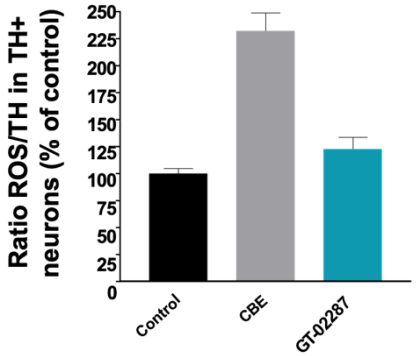
Toxic Substrate Depletion



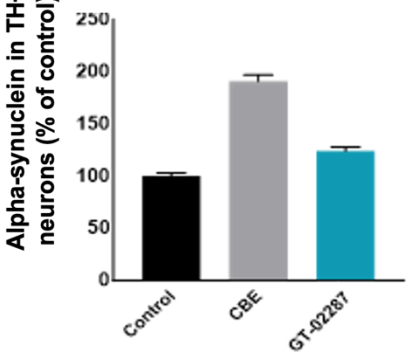
Reduced ER Stress



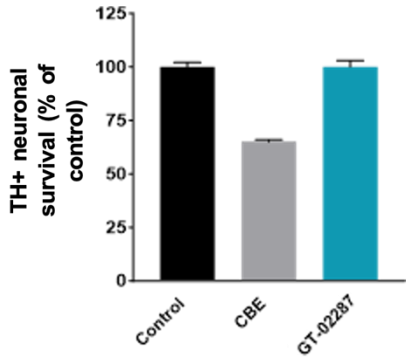
Restored Mitochondrial Function



Alpha-Synuclein Depletion

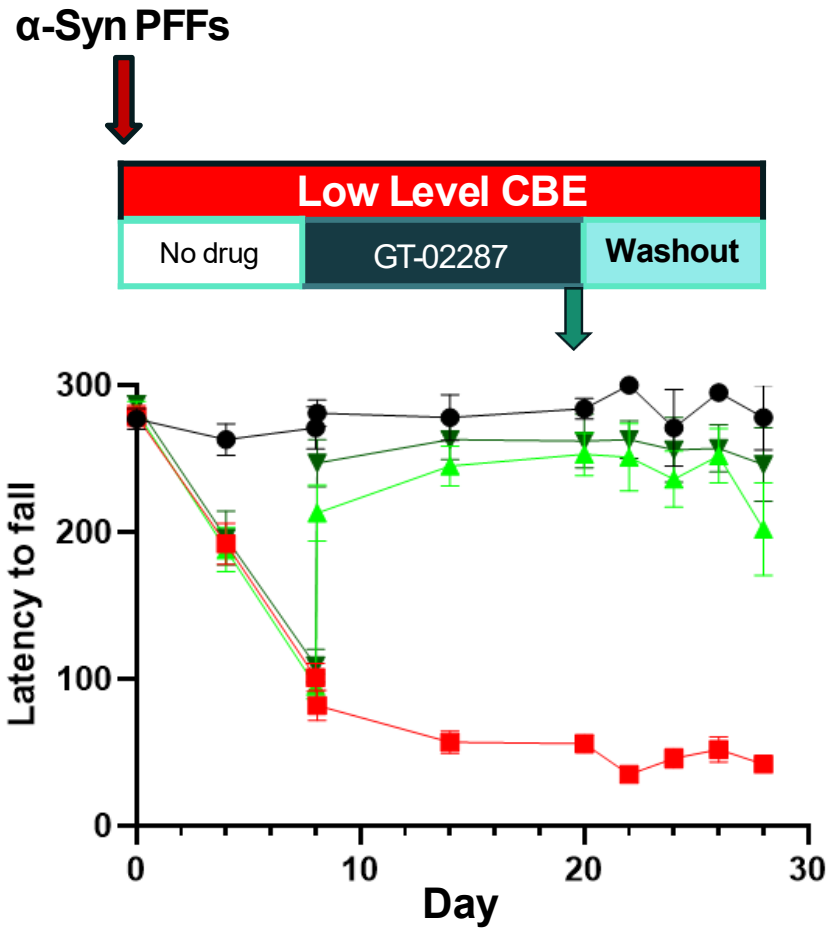


Neuronal Survival

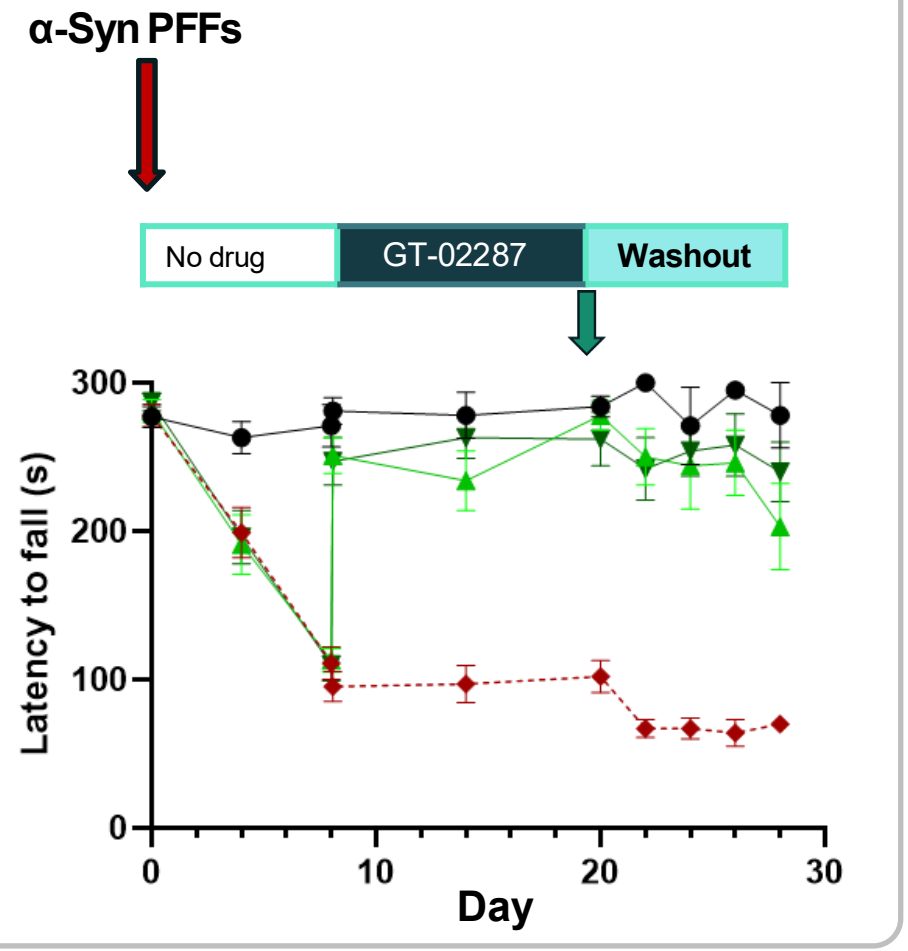


GT-02287 displays a rescue and disease-modifying effect in animal models of GBA1 and iPD

GBA1- PD Model (CBE+PFFs)



Idiopathic PD Model (PFFs only)

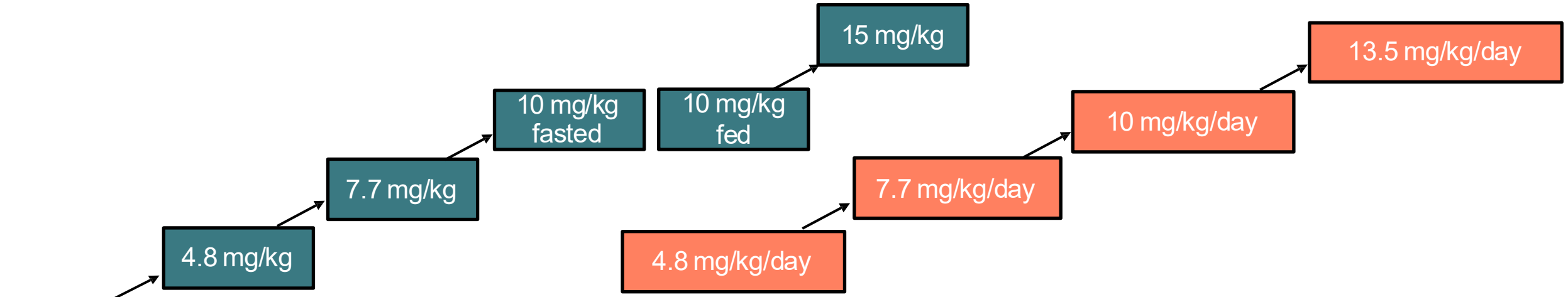


Mouse Wire Hang Rescue & Washout

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

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

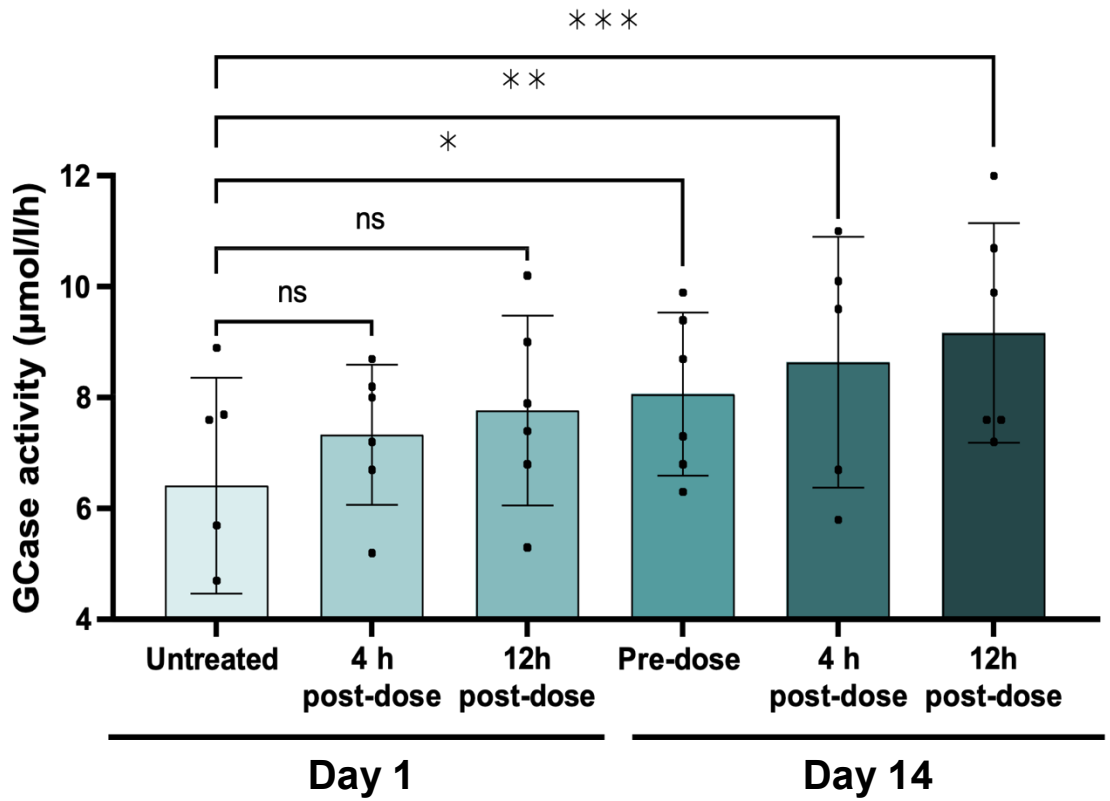
GT-02287 Demonstrates GCase Target Engagement In Healthy Volunteers

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*

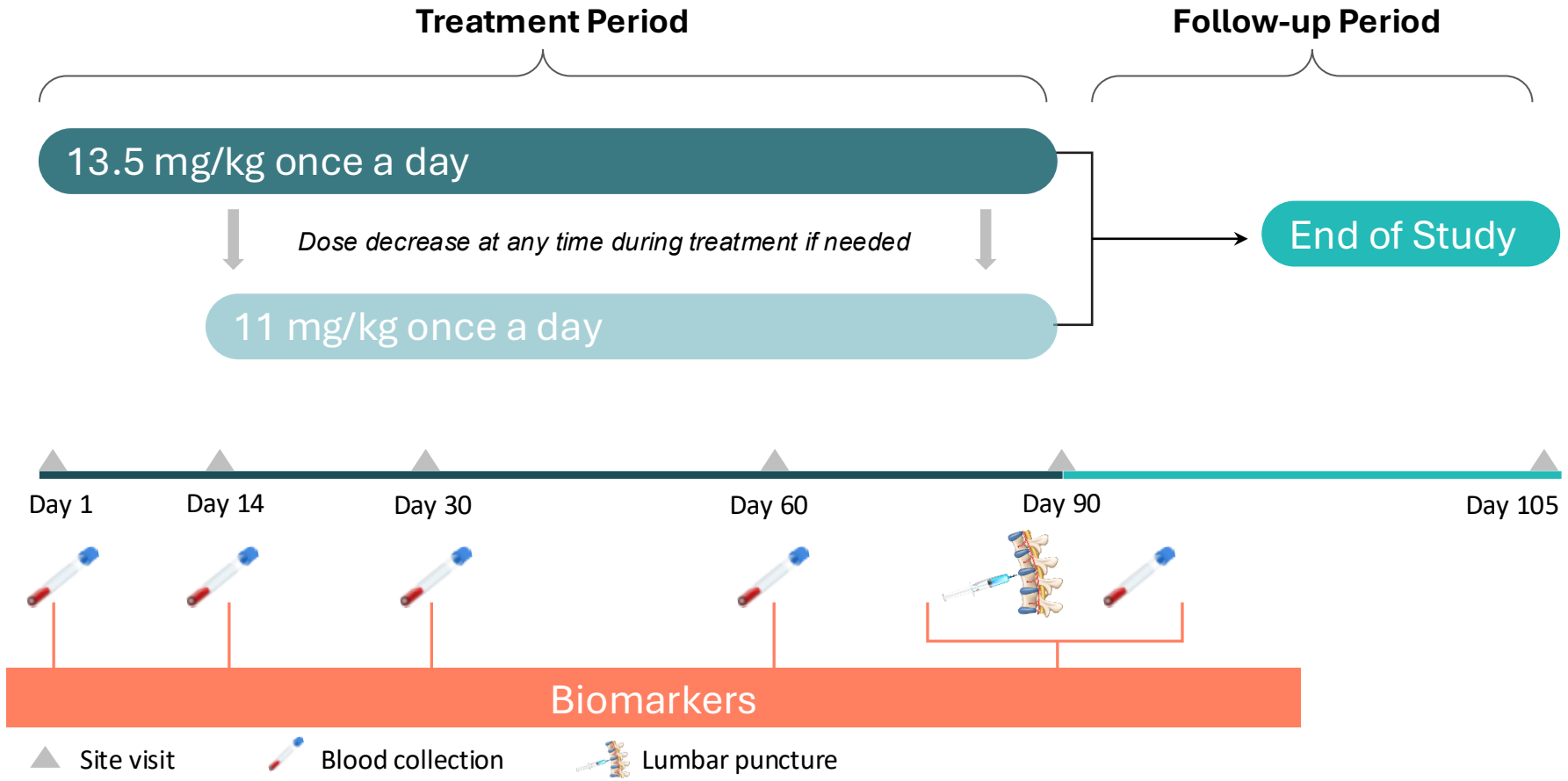
GT-02287 Demonstrates CNS Exposure Comparable to that Observed in Rodents

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

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



Study Extension

Protocol amendment approved by Australian regulatory authorities

More than half of participants from Part 1 have agreed to continue for another 9 months in Part 2

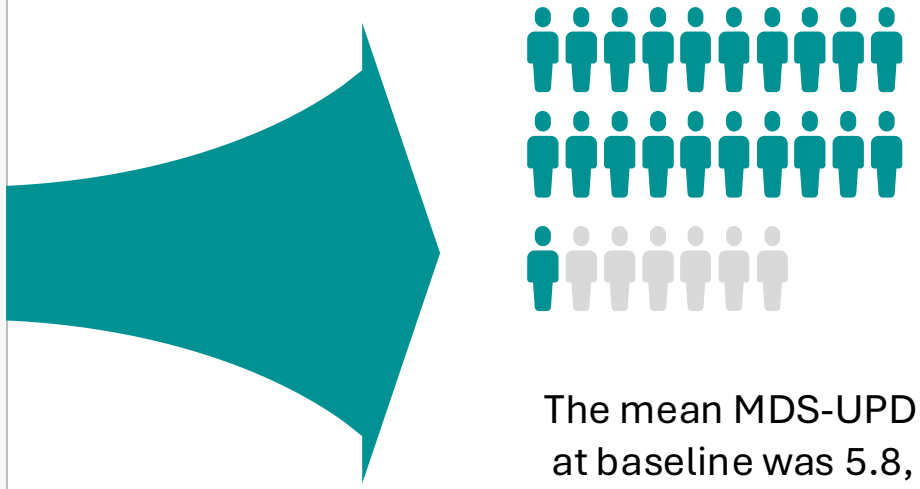
9-month treatment duration

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	
	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
Exploratory	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.

Phase 1b Initial Data: Demographics and Baseline Characteristics

- 27 individuals were screened and 21 enrolled from March through September 2025; enrollment in Part 1 is now complete
- The 21 participants include 3 women and 18 men, 2 treatment-naïve, 2 on DBS, and 18 on levodopa +/- dopamine agonists and other PD drugs
- Mean age was 63.5 years (range 42-83), mean disease duration was 3.0 years (range 0.5-7.0), and the mean H&Y score was 1.6 (range 1-2.5)
- Genetic data are currently available in 15 participants: 2 have severe GBA1 variants and 1 has a mild GBA1 variant



The mean MDS-UPDRS score at baseline was 5.8, 7.4, and 24.7 for Part I, II, and III, respectively

Phase 1b Initial Data: Safety and Tolerability

Adverse Events

18 participants have experienced 93 treatment emergent adverse events (TEAEs) as of 03 Sep 2025

The most common TEAEs were headache (n=6 participants), lab abnormalities (n=6), diarrhea (n=6), fatigue (n=4), and nausea (n=3)

85% of TEAEs were mild, 11% were moderate, and 5% were severe; there have been no treatment-emergent SAEs

Discontinuations

One participant discontinued from the study after 24 days due to panic attacks, nausea, and headaches

Dosing reduction

One participant reduced the dose due to headaches

Two participants reduced the dose due to lab abnormalities (see below)

Dosing interruptions

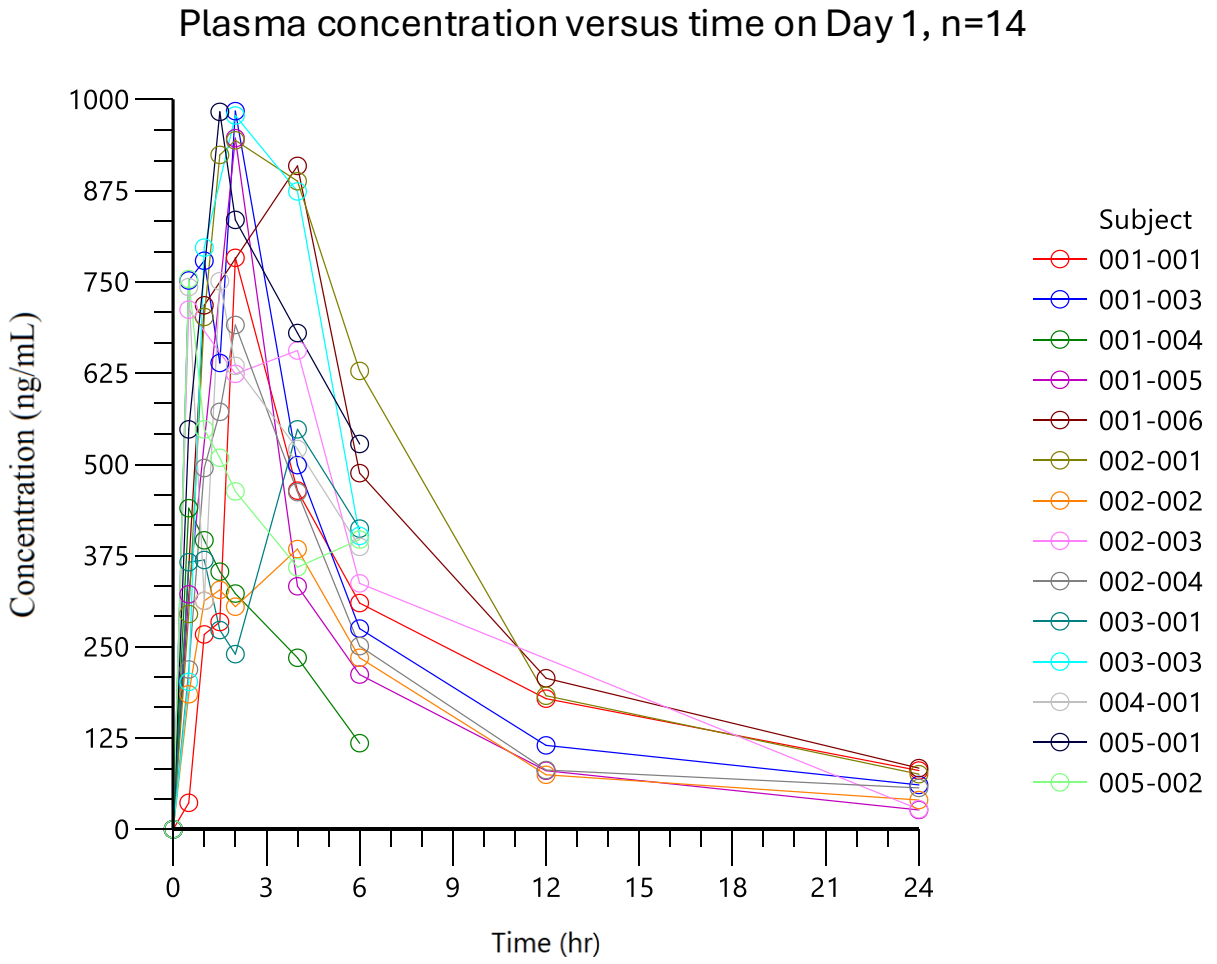
One participant interrupted dosing for 7 days due to constipation

One participant had dosing withheld for 30 days due to transient increases in ALT, ALP, and GGT; upon reinitiation of dosing at a lower dose, liver enzymes normalized and remained within normal limits thereafter

One participant had dosing withheld for 4 days due to a transient increase in lipase; upon reinitiation of dosing at a lower dose, lipase levels had normalized and remained within normal limits thereafter

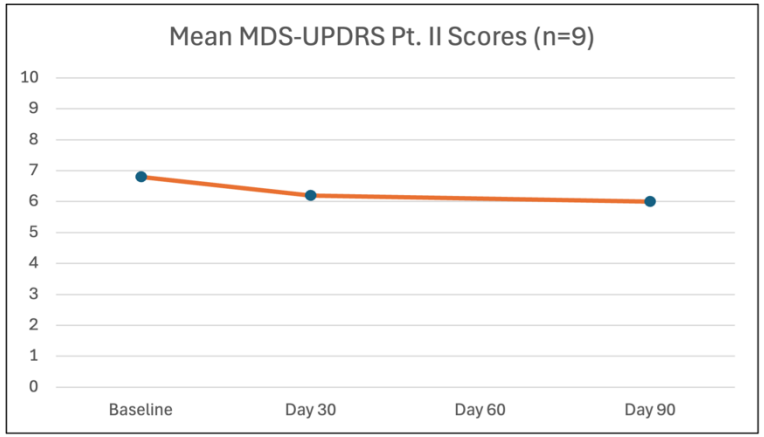
Phase 1b Initial Data: Plasma PK

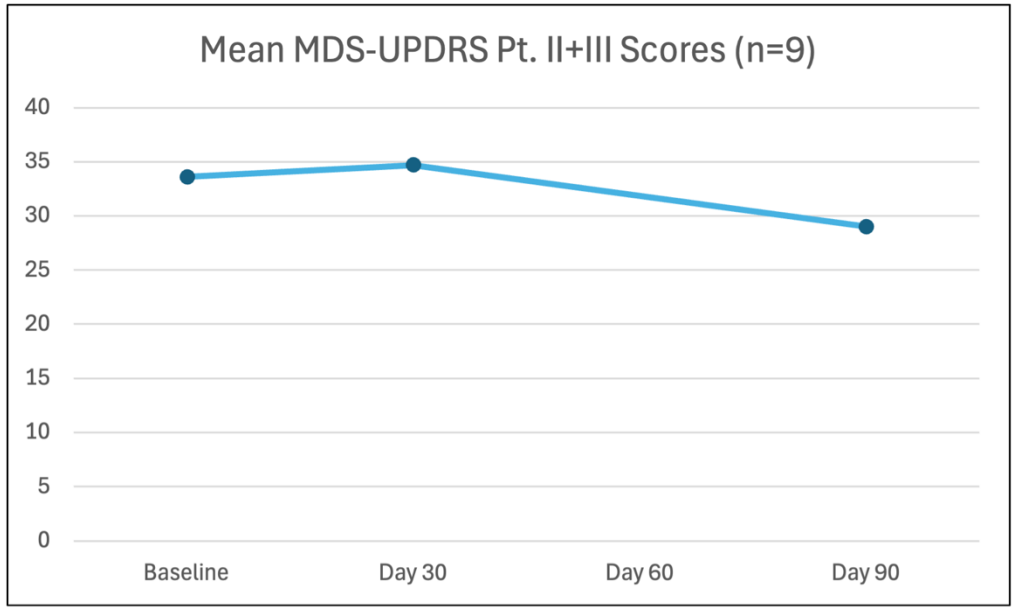
Plasma exposures were within the projected therapeutic range and comparable to exposures observed in healthy volunteers in Phase 1

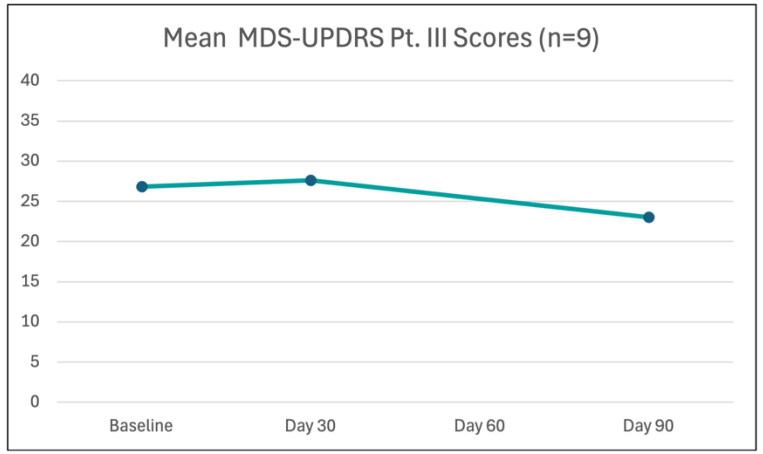


Phase 1b Initial Data: MDS-UPDRS Changes

- No mean improvement by Day 30, but mean improvement by Day 90
- This indicates that there is no acute, dopaminergic, symptomatic effect, but that a slower effect may occur
- Reduction of 1-2 points on UPDRS Part II and 4-8 points on Part III is clinically meaningful
- Continued dosing required to potentially increase clinical benefit further







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
- 25 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 Selvaraju, Ph.D.*
Maxim	*Jason McCarthy, Ph.D.*
ROTH	*Boobalan Pachaiyappan, Ph.D.*

Financial and Stock Data

IPO (NASDAQ: GANX)

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

CAPITAL STRUCTURE

- 36.0 million shares outstanding
- No debt*

CASH POSITION

- $8.8 million as of September 30, 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
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Swiss Confederation
Innosuisse – Swiss Innovation Agency