



GAIN THERAPEUTICS

Corporate Presentation
August 2025

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.

These include, but are not limited to, statements about the Company's ability to develop, obtain regulatory approval for and commercialize its product candidates; the timing of future IND submissions, initiation of preclinical studies and clinical trials, and timing of expected clinical results for our product candidates; the Company's success in early preclinical studies, which may not be indicative of results obtained in later studies or clinical trials; the outbreak of the novel strain of coronavirus disease, COVID-19, which could adversely impact our business, including our preclinical studies and any future clinical trials; the potential benefits of our product candidates; the Company's ability to obtain regulatory approval to commercialize our existing or any future product candidates; the Company's ability to identify patients with the diseases treated by our product candidates, and to enroll patients in clinical trials; the success of our efforts to expand our pipeline of product candidates and develop marketable products through the use of our Magellan platform; the Company's expectations regarding collaborations and other agreements with third parties and their potential benefits; the Company's ability to obtain, maintain and protect our intellectual property; the Company's reliance upon intellectual property licensed from third parties, including the license to use the Company's Magellan platform; the Company's ability to identify, recruit and retain key personnel; the Company's financial performance; developments or projections relating to the Company's competitors or industry; the impact of laws and regulations; the Company's expectations regarding the time during which it will be an emerging growth company under the JOBS Act; and other factors and assumptions described in the Company's public filings.

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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 analysis of functional changes and 90-day biomarker activity – **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



Jonas Hannestad, MD, PhD
Chief Medical Officer



Gianluca Fuggetta
Senior Vice President,
Finance



Joanne Taylor, PhD
SVP Research



Terenzio Ignoni, PharmD
SVP Technical
Operations



Gain Therapeutics Pipeline

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



Lead Clinical Program

GT-02287

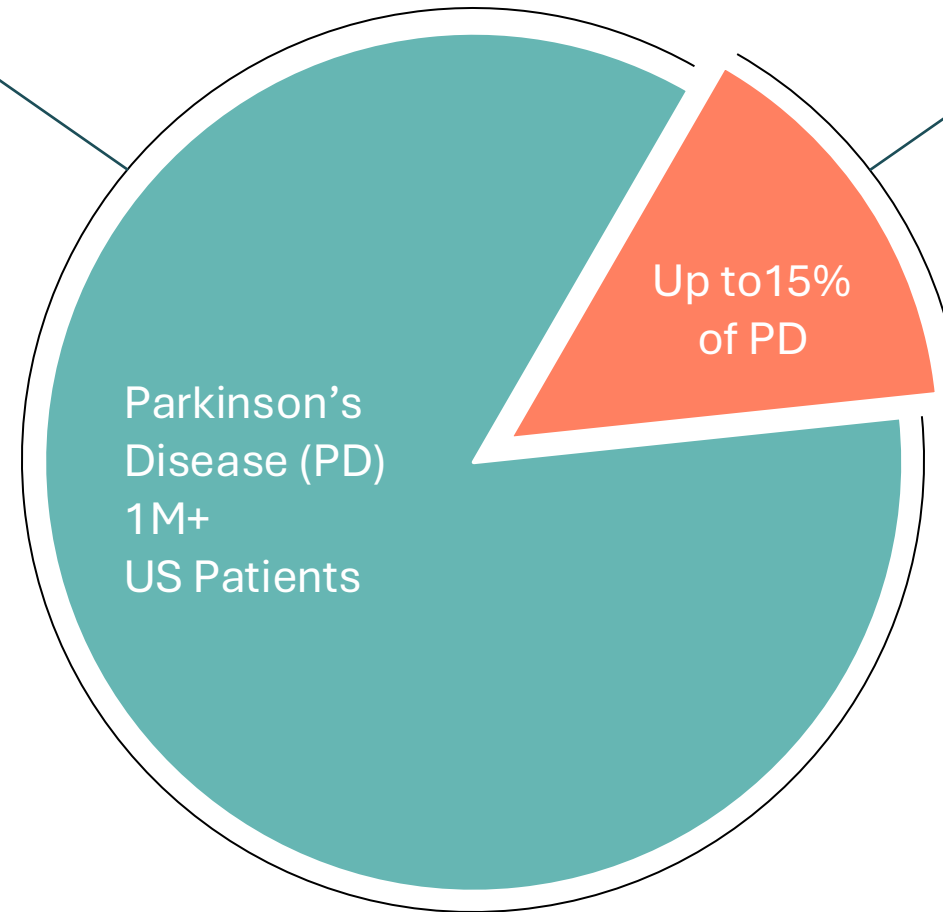
GBA1 Parkinson's Disease

Parkinson's Disease – Market Opportunity

Parkinson's Disease
US Market Potential:
\$4B

Parkinson's disease is the second most common neurodegenerative disease¹

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

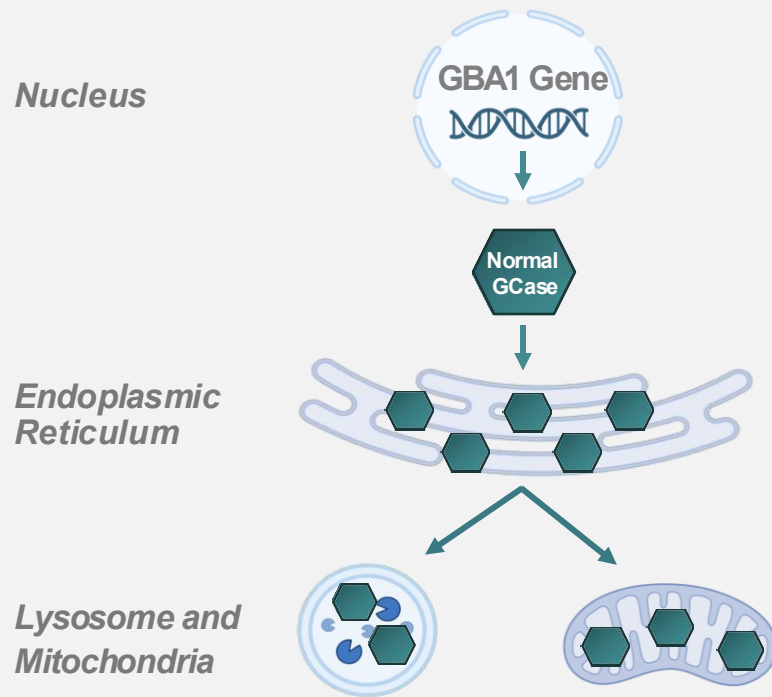
GBA1-PD patients experience earlier disease onset and more severe disease with faster decline in motor and cognition functions.

A therapy for disease progression in this subpopulation is needed

Largest genetic risk factor for development of Parkinson's disease

GCase Plays Integral Role in Organelle and Cellular Health

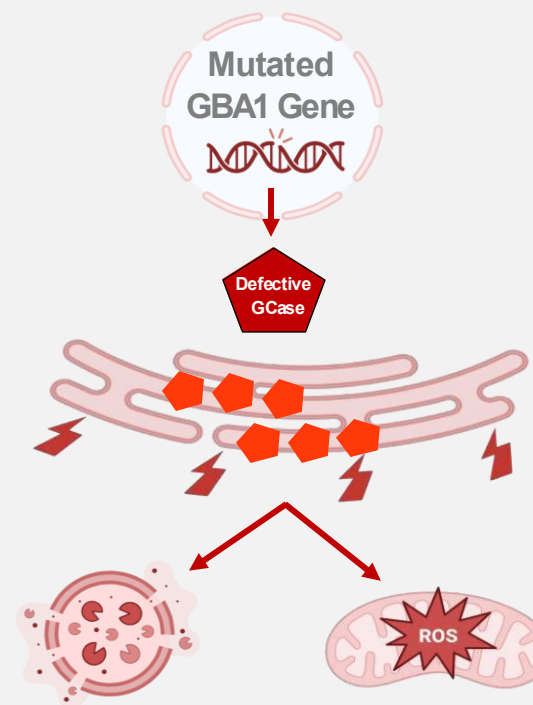
Healthy Dopaminergic Neuron



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

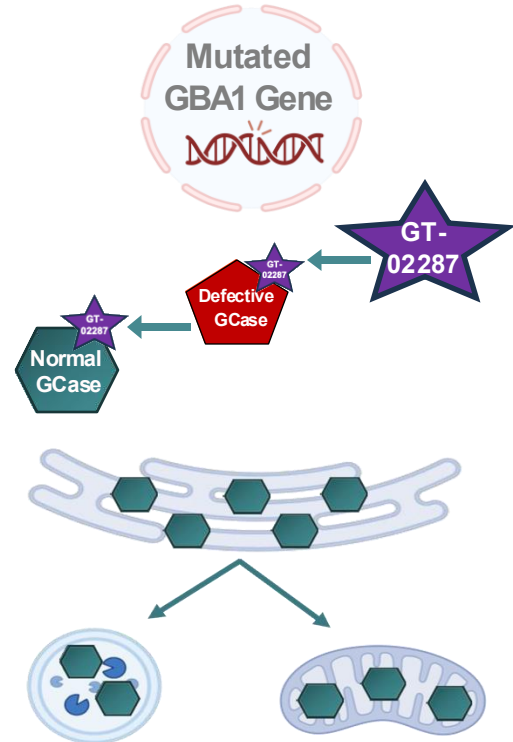
Alpha synuclein aggregation

Dysfunctional GCase Increases PD Risk

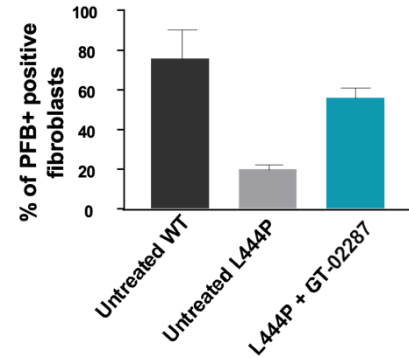
- Dysfunctional GCase triggers disease cascade affecting multiple organelle functions leading to core motor and cognitive dysfunction

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

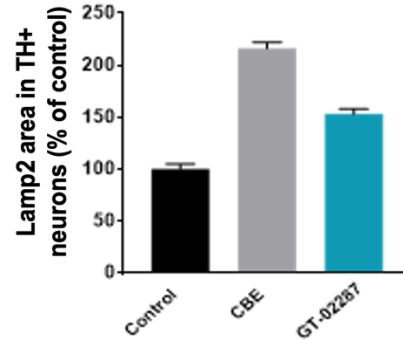
Dopaminergic Neuron with Restored GCase Function



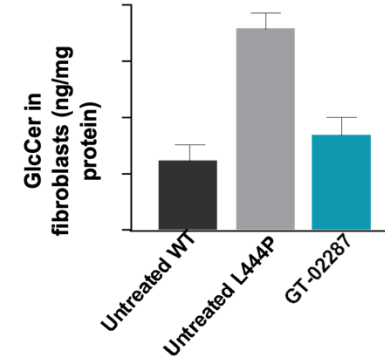
GCase Activity Restoration



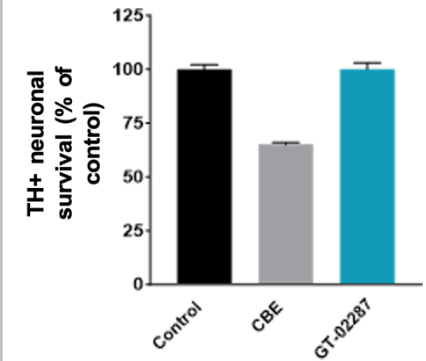
Restored Lysosomal Function



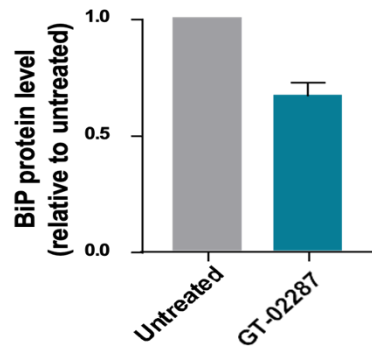
Toxic Substrate Depletion



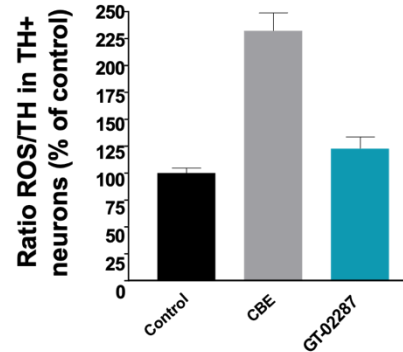
Neuronal Survival



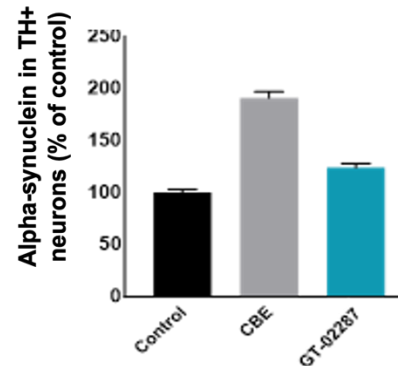
Reduced ER Stress



Restored Mitochondrial Function



Alpha-Synuclein Depletion



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

GBA1- PD Model (CBE+PFFs)

α -Syn PFFs

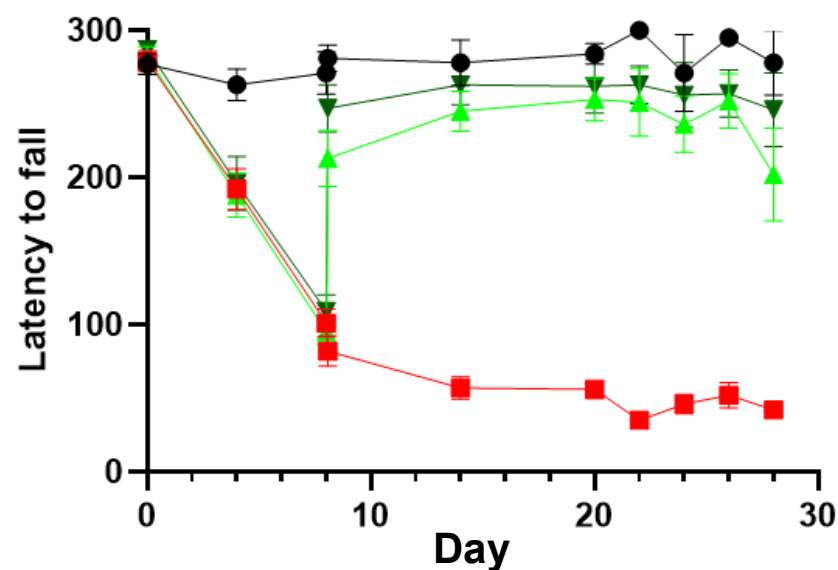


Low Level CBE

No drug

GT-02287

Washout



Idiopathic PD Model (PFFs only)

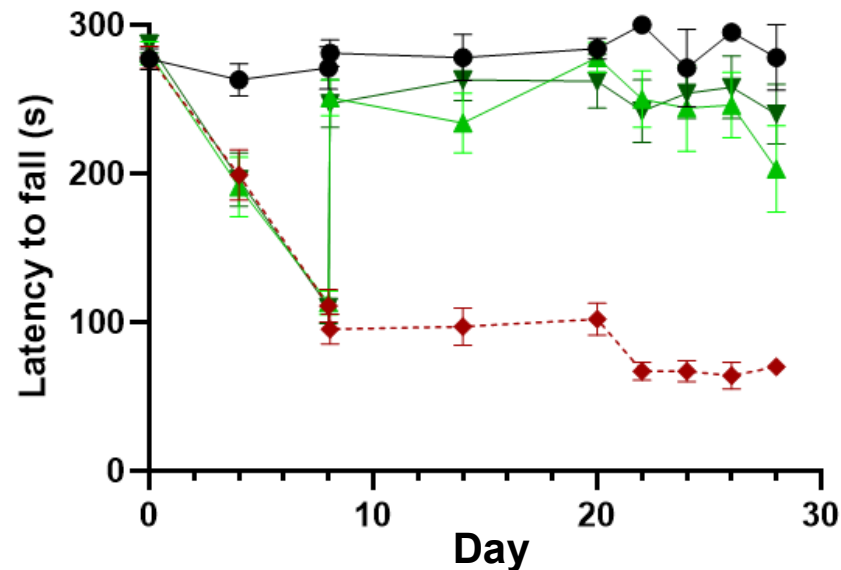
α -Syn PFFs



No drug

GT-02287

Washout

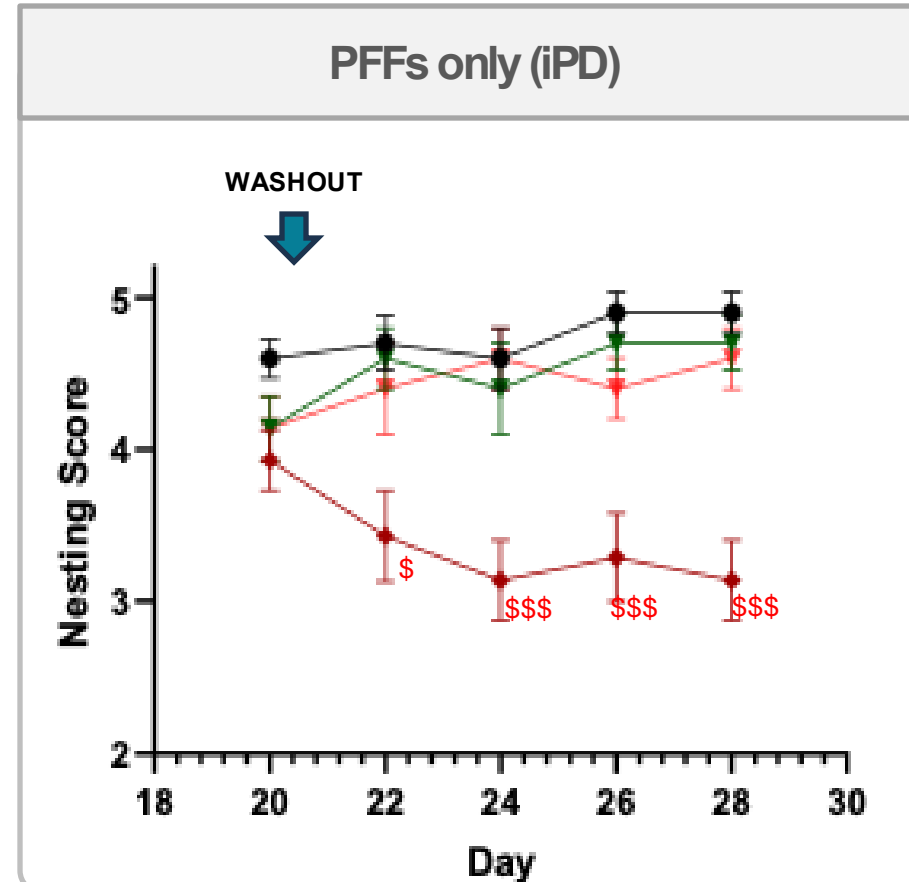
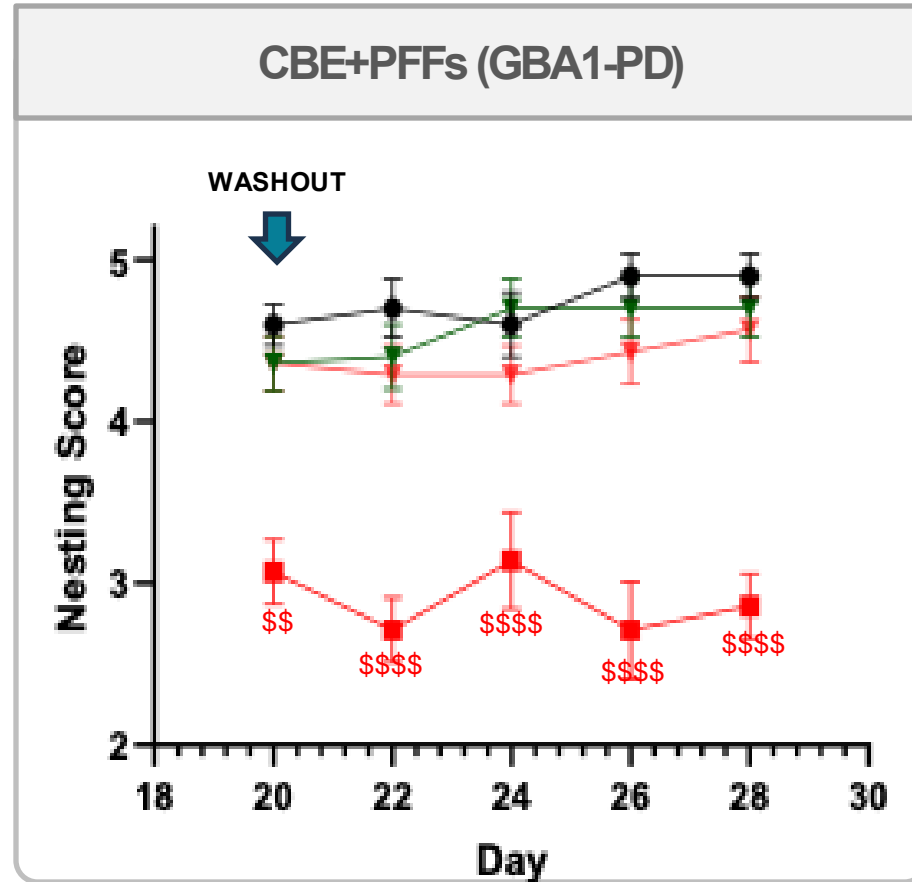


Mouse Wire Hang
Rescue & Washout

- Control
- CBE/PFFs
- ◆ PFFs
- ▲ GT-02287 60mg/Kg
- ▼ GT-02287 90mg/Kg

Nesting Score Unaffected by Drug Washout

Evidence of disease modifying effect on complex behaviors

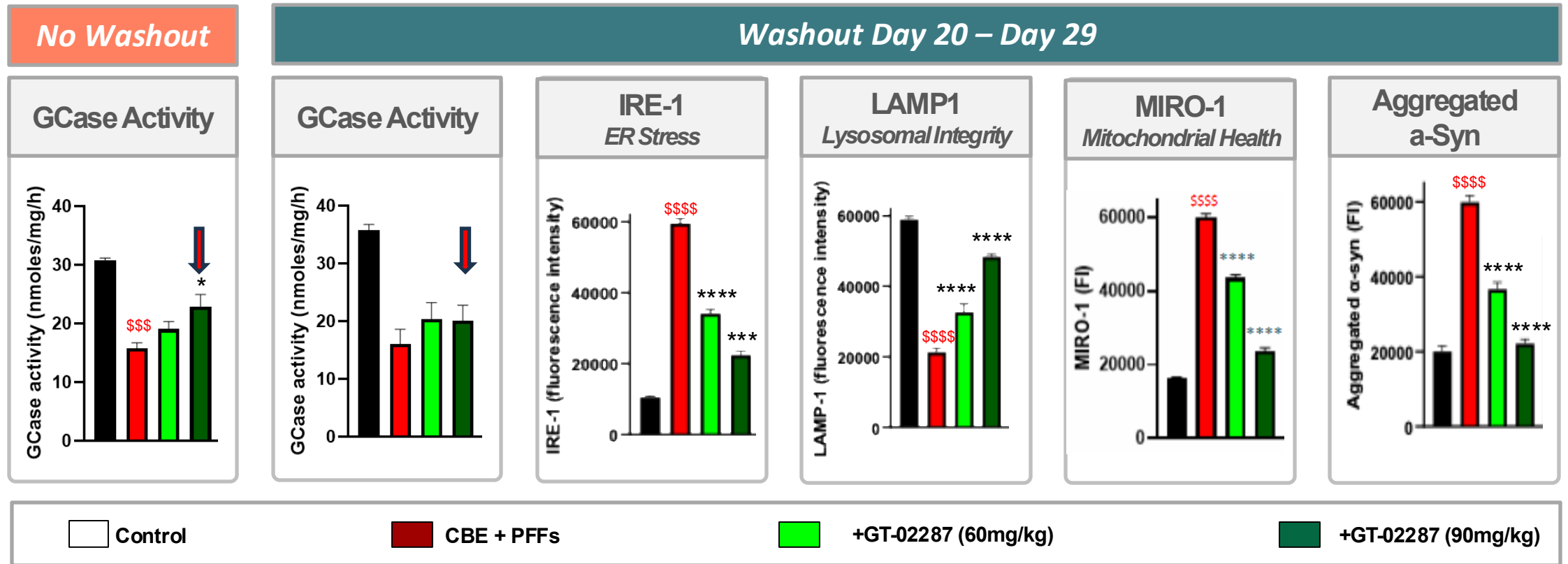


Mouse Nesting Building Washout

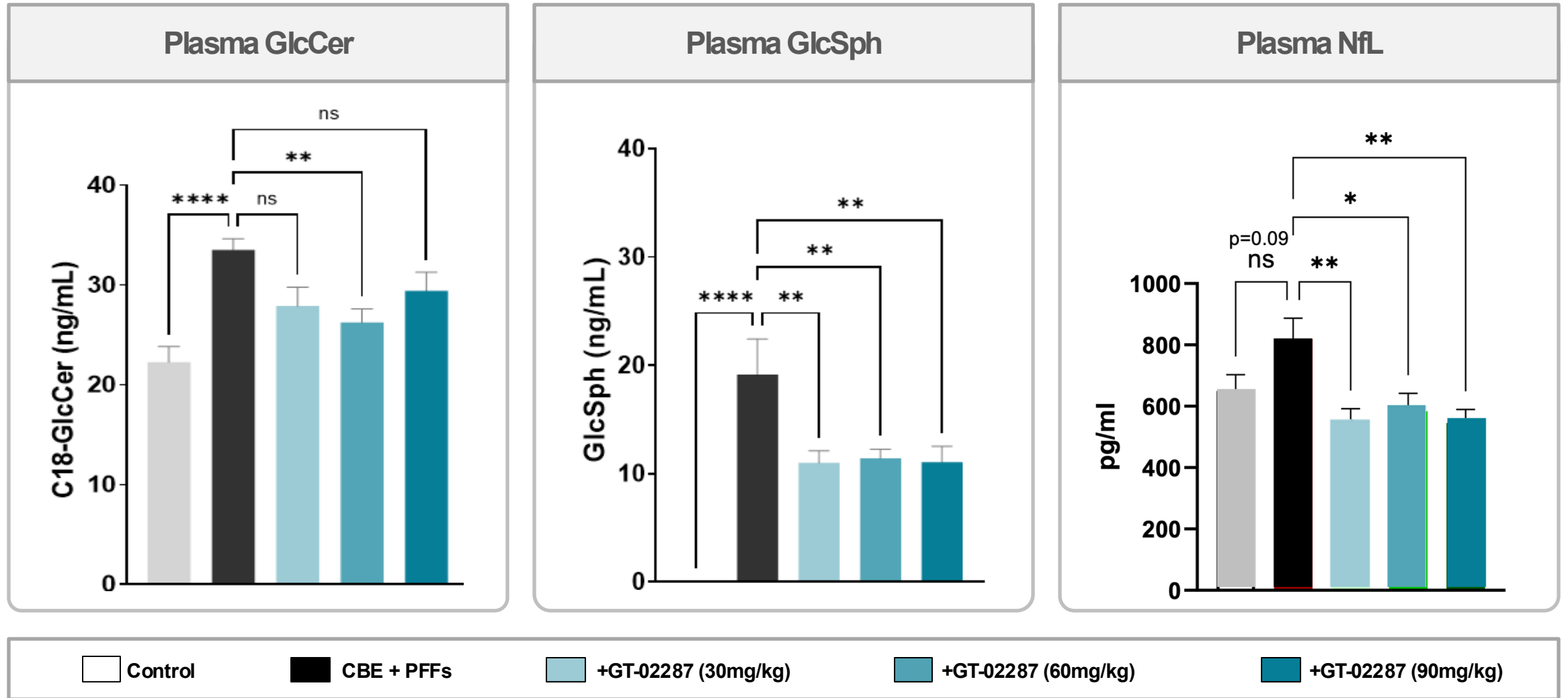
- Control
- CBE+PFFs
- ◆ PFFs
- ▼ GT-02287 90mg/Kg
- ▼ GT-02287 90mg/Kg + washout

Brain biomarker changes maintained following GT-02287 washout

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



Translational plasma-based biomarkers of GT-02287 rescue effect



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

Participants

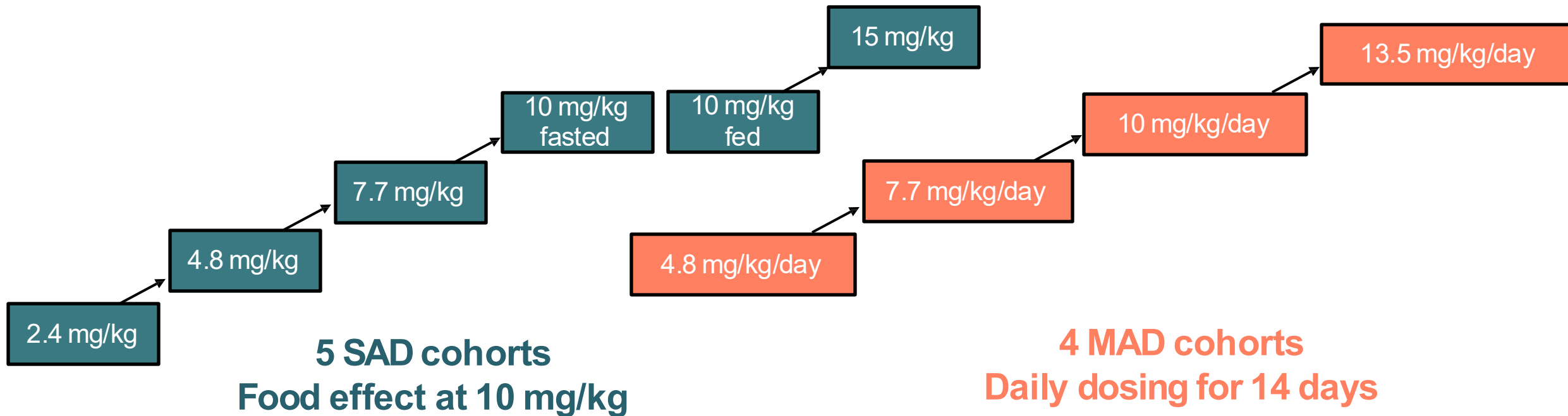
- Healthy men and women ages 18-65
- 8 subjects per cohort
- 2 placebo; 6 active

SAD/MAD Endpoints

- Treatment-emergent adverse events
- Clinical labs, vital signs, ECGs, C-SSRS
- Plasma pharmacokinetics

MAD Cohort 4

- CSF drug levels
- GCase activity in dry blood spots



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

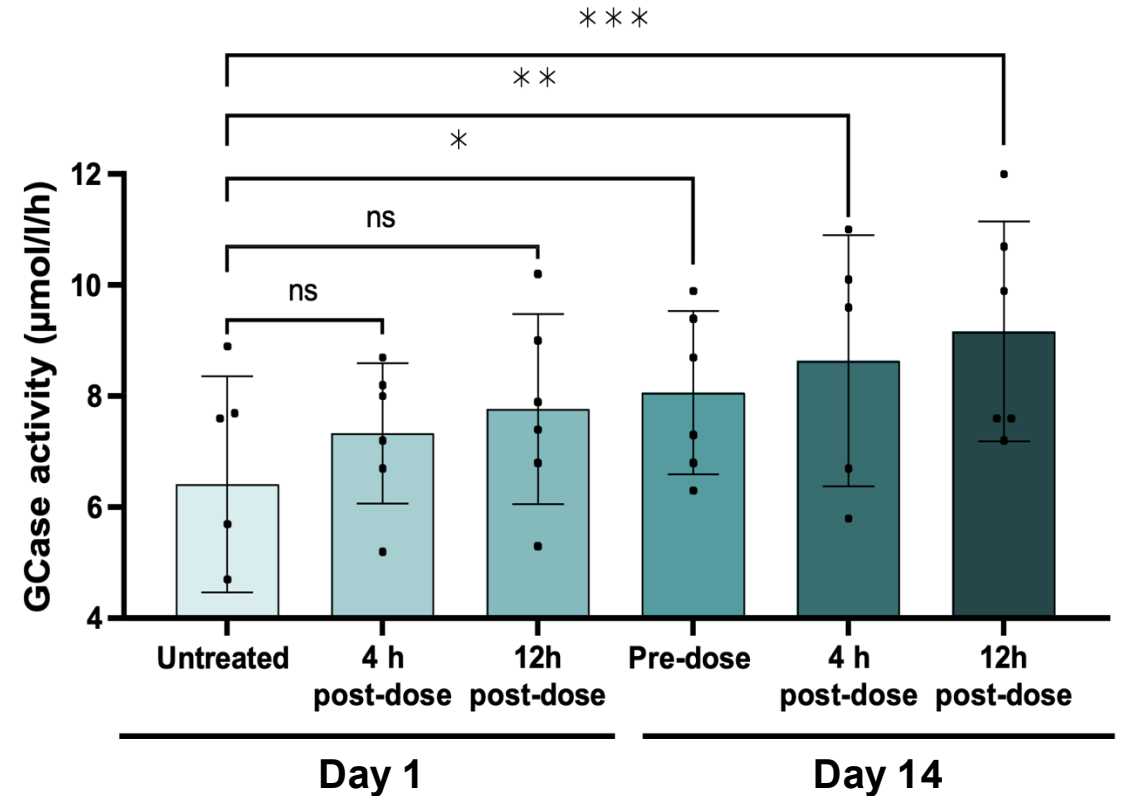
GT-02287 Demonstrates GCase Target Engagement In Healthy Volunteers

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)

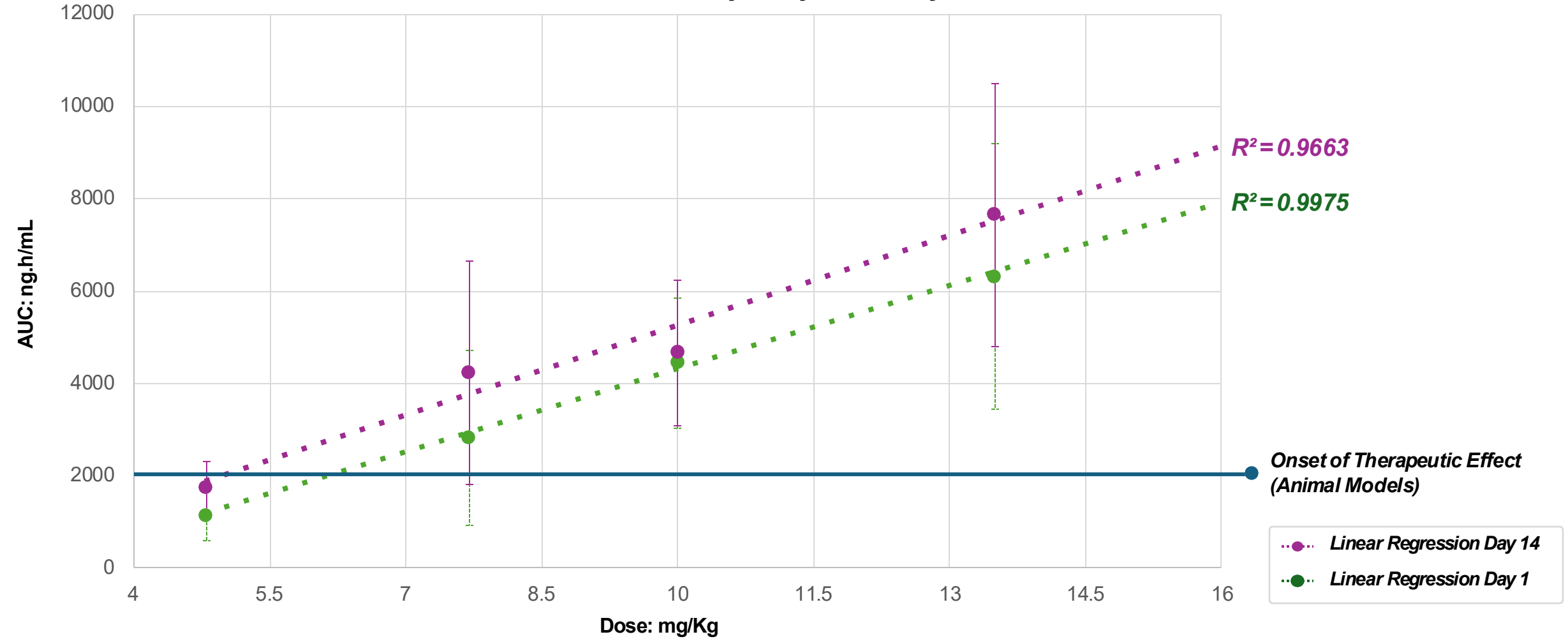
**53% increase in GCase activity
observed by Day 14 ($p < 0.001$)**

GCase Activity in Dried Blood Spots (DBS)



Therapeutic Range: Phase 1 PK Data in Healthy Volunteers

Human AUC from MAD Study : Day 1 and Day 14



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 analysis of functional changes and 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

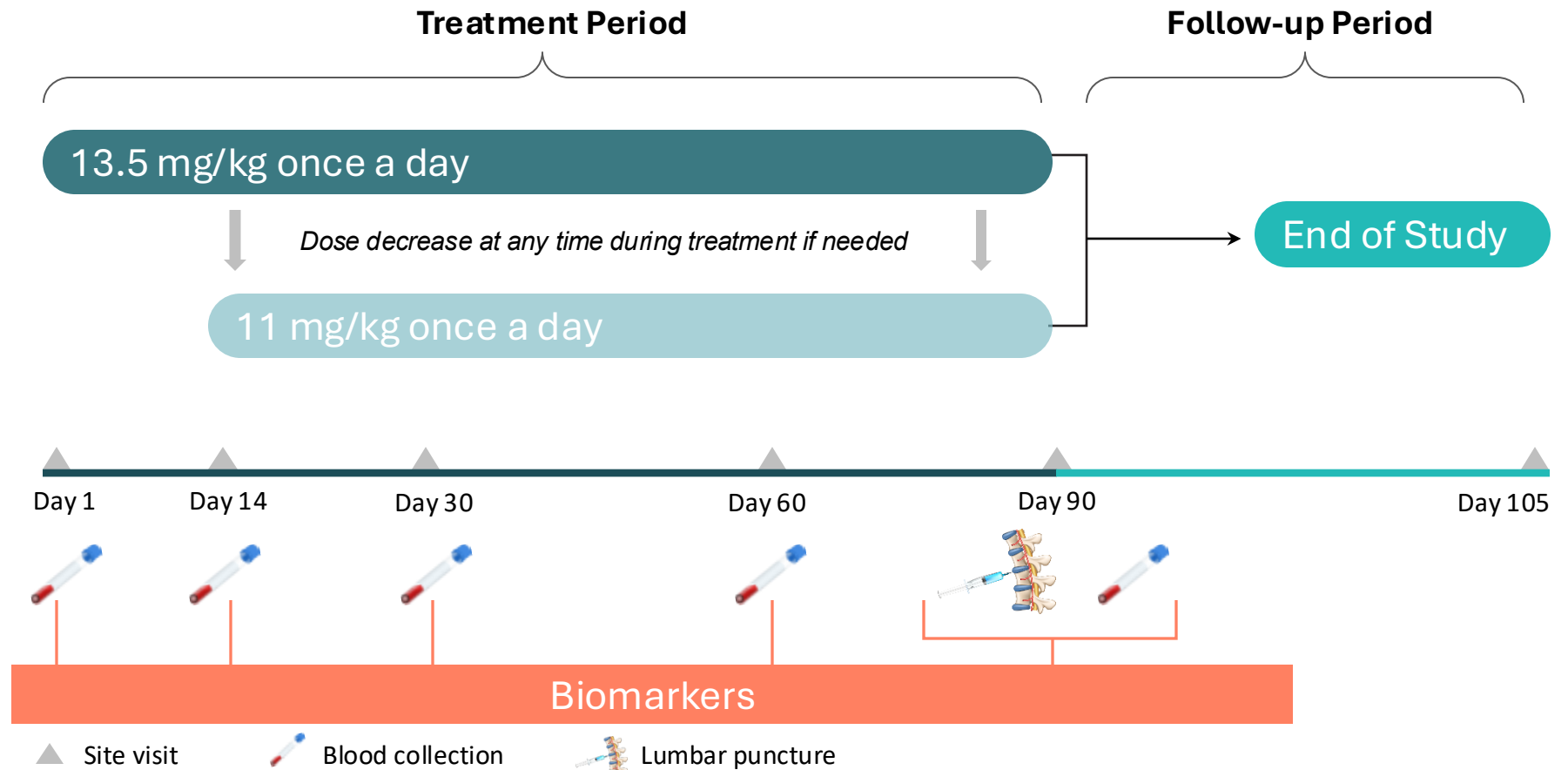
An Open-label Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of GT-02287 in Participants with Parkinson's Disease With or Without a Pathogenic GBA1 Mutation

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	
	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.

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

	Effect on Disease Cascade	 GT-02287	 BIA 28-6156	 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	<i>Tom Shrader, Ph.D., CFA</i>
Oppenheimer & Co	<i>Jay Olson, CFA</i>
H.C. Wainwright	<i>Ram Selveraju, Ph.D.</i>
Chardan	<i>Kaey Nakae, CFA</i>
Maxim	<i>Jason McCarthy, Ph.D.</i>
ROTH	<i>Boobalan Pachaiyappan, Ph.D.</i>
Scotiabank	<i>Louise Chen, MBA</i>

Financial and Stock Data

IPO (NASDAQ: GANX)

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

CAPITAL STRUCTURE

- 30.8 million shares outstanding
- No debt*

CASH POSITION

- \$6.7 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)

