

**TRANSFORMING THE TREATMENT OF
PARKINSON'S DISEASE**

Corporate Presentation

August 2024

Forward-Looking Statements

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 our 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.

These statements are based on the Company's historical performance and on its current plans, estimates and projections in light of information currently available to the Company, and therefore, you should not place undue reliance on them. The inclusion of forward-looking information should not be regarded as a representation by the Company or any other person that the future plans, estimates or expectations contemplated by us will be achieved. Forward-looking statements made in this presentation speak only as of the date of this presentation, and the Company undertakes no obligation to update them in light of new information or future events, except as required by law.

You should carefully consider the above factors, as well as the factors discussed elsewhere in this presentation and our public filings, before deciding to invest in our common stock. The factors identified above should not be construed as an exhaustive list of factors that could affect the Company's future results, and should be read in conjunction with the other cautionary statements that are included in this presentation and our public filings. New risks and uncertainties arise from time to time, and it is impossible for the Company to predict those events or how they may affect the Company. If any of these trends, risks or uncertainties actually occurs or continues, the Company's business, revenue and financial results could be harmed, the trading prices of its securities could decline and you could lose all or part of your investment. All forward-looking statements attributable to the Company or persons acting on its behalf are expressly qualified in their entirety by this cautionary statement.

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Industry Information

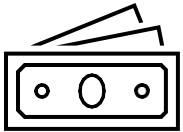
Market data and industry information used throughout this presentation are based on management's knowledge of the industry and the good faith estimates of management. We also relied, to the extent available, upon management's review of independent industry surveys and publications and other publicly 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



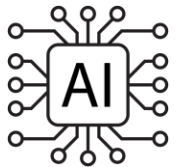
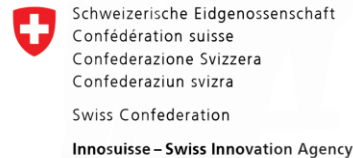
Lead Product GT-02287 Advancing into GBA1 Parkinson's Disease Patients

- Restoring motor function and improving cognition – potential to slow or stop PD progression
- Safety and tolerability has been established in healthy volunteers with further PK analysis forthcoming
- Translation of GT-02287's impact on important biomarkers of PD from animal models to PD patients is the focus of clinical development over the next 12 months



GANX Early Support for GT-02287

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



Our Proprietary Magellan™ AI Platform Discovers Novel First-in-Class Therapeutics

- Capable of efficiently screening library compounds in the trillions
- Select or provide constructs of molecules that can potentially target previously undruggable proteins
- We estimate our AI approach can generate leads for optimization in a little as three months



Experienced Leadership Team and Board

- 42 drug approvals
- \$45B+ in strategic transactions

Experienced Leadership

EXTENSIVE BIOTECH AND PHARMA EXPERIENCE (>\$15B TRANSACTIONS)



Khalid Islam, PhD
Founder and Executive
Chairman



Gene Mack, MBA
Interim – CEO
Chief Financial Officer



Jonas Hannestad, MD, PhD
Chief Medical Officer



Joanne Taylor, PhD
SVP Research



Terezio Ignoni, PharmD
SVP Technical Operations



Strong Board of Directors

(>\$30B IN TRANSACTIONS)



Khalid Islam, PhD
Founder and
Executive Chairman



Claude Nicaise, MD
Independent Member



Eric I. Richman
Independent Member



Gwen Melincoff
Independent Member



Hans Peter Hasler
Independent Member



Dov Goldstein, MD
Independent Member



Jeffrey Riley
Independent Member



Novel Pipeline Generated from Magellan™ Platform

THERAPEUTIC AREA	INDICATION	TARGET	DISCOVERY	RESEARCH	PRECLINICAL	PHASE 1
Neurodegenerative Diseases	Parkinson's Disease	GCase				
	Dementia with Lewy Bodies Alzheimer's Disease	GCase				
Lysosomal Storage Disorders	Gaucher Disease	GCase				
	GM1 Gangliosidosis	GLB1				
	Krabbe Disease	GALC				
Metabolic Diseases	Alpha1-Antitrypsin Deficiency	AAT				
Oncology	Solid Tumors	UNDISCLOSED				
	Solid Tumors	UNDISCLOSED				

Lead Clinical Program

GT-02287

GBA1 Parkinson's Disease

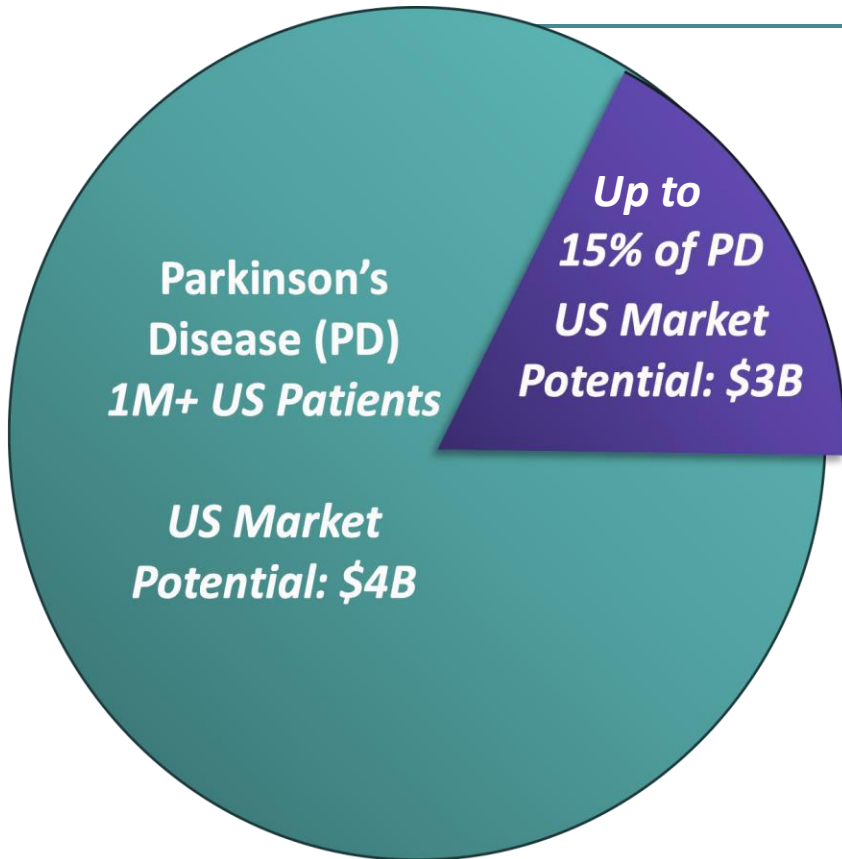
GBA-Parkinson's – A Genetically Defined Subpopulation of Parkinson's Disease

Parkinson's Disease

- Parkinson's disease is the second most common neurodegenerative disease¹
- Current therapies only treat symptoms and do not prevent neuronal cell death

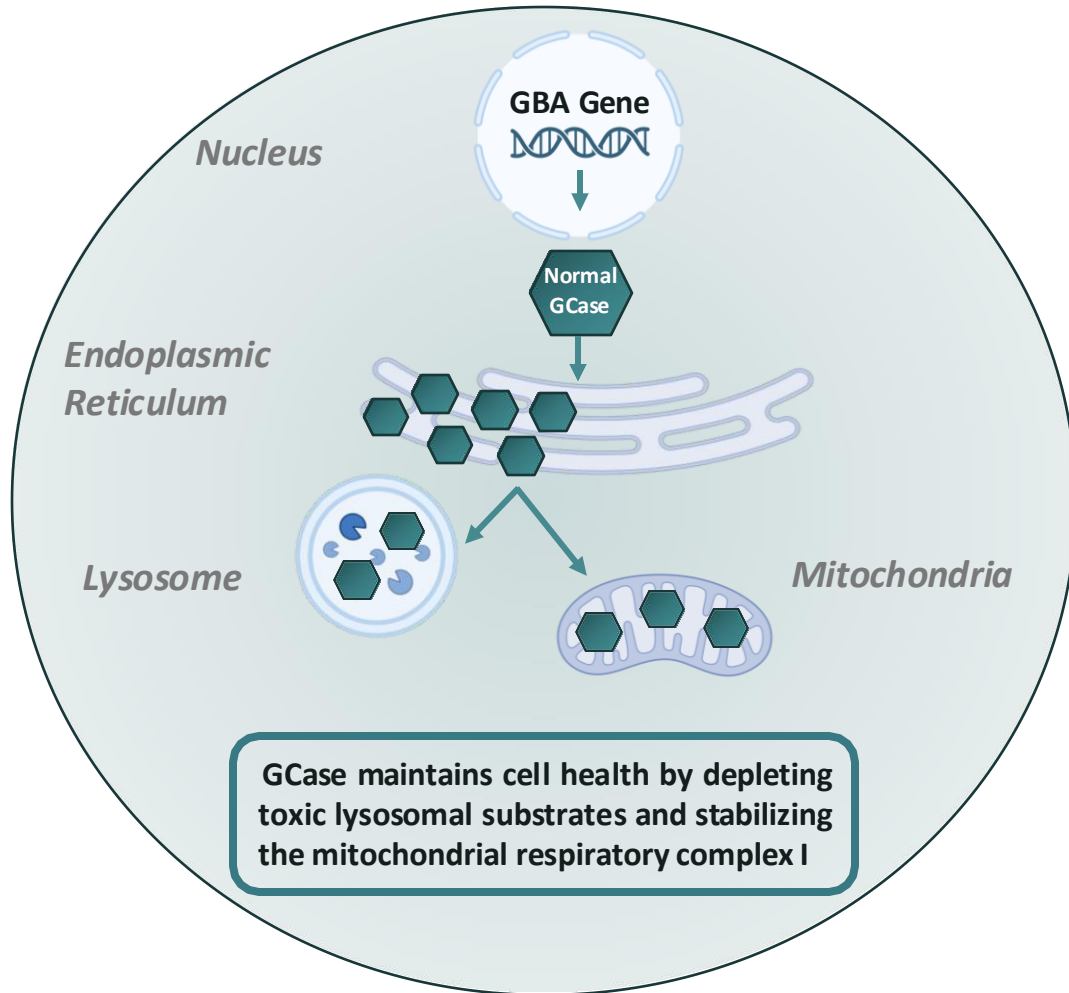
GBA-Parkinson's Disease

- Largest genetic risk factor for development of Parkinson's disease
- GBA mutations cause misfolding of an important enzyme called GCase
- GBA1-Parkinson's patients have more severe form of PD²

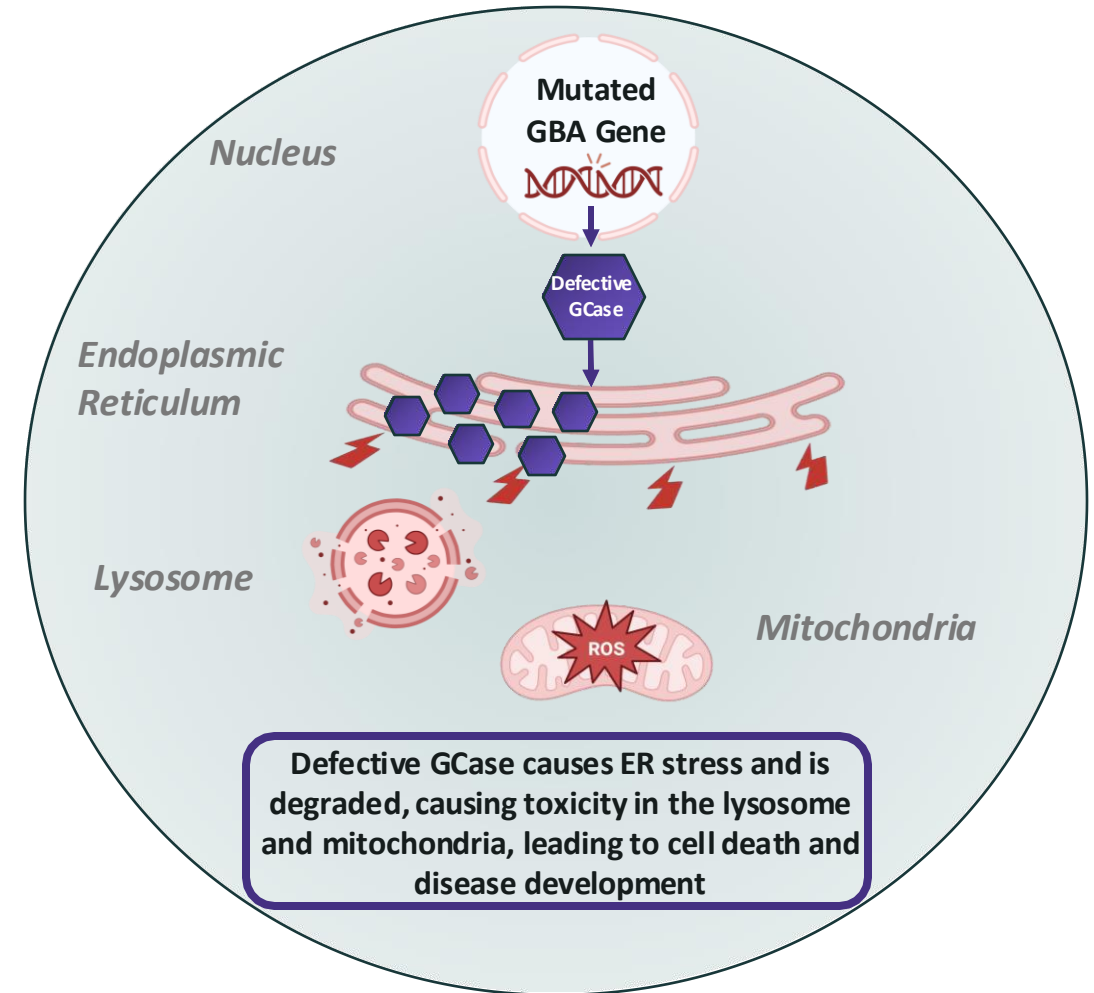


Dysfunctional GCase Triggers Disease Cascade Affecting Multiple Organelle Functions and Leading to Neurodegeneration

Healthy Dopaminergic Neuron

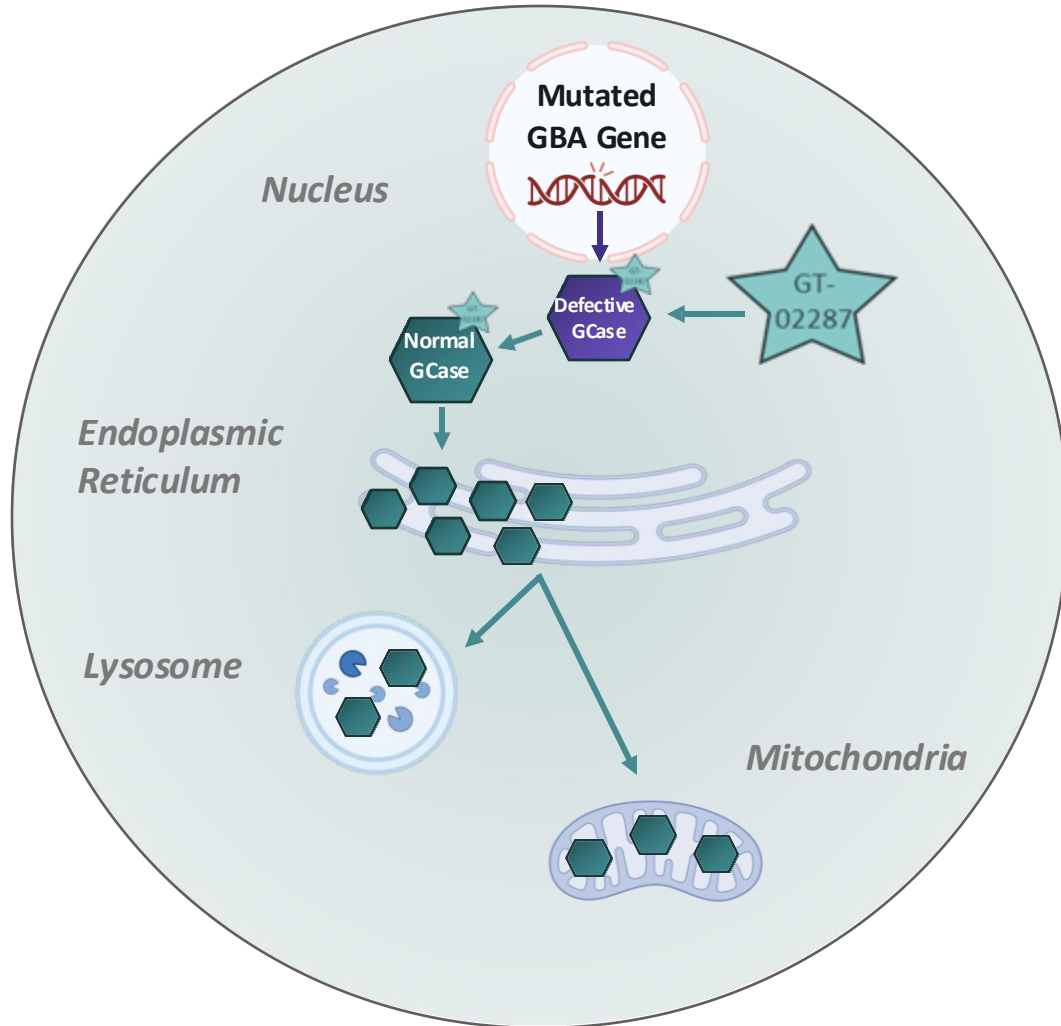


Diseased Dopaminergic Neuron

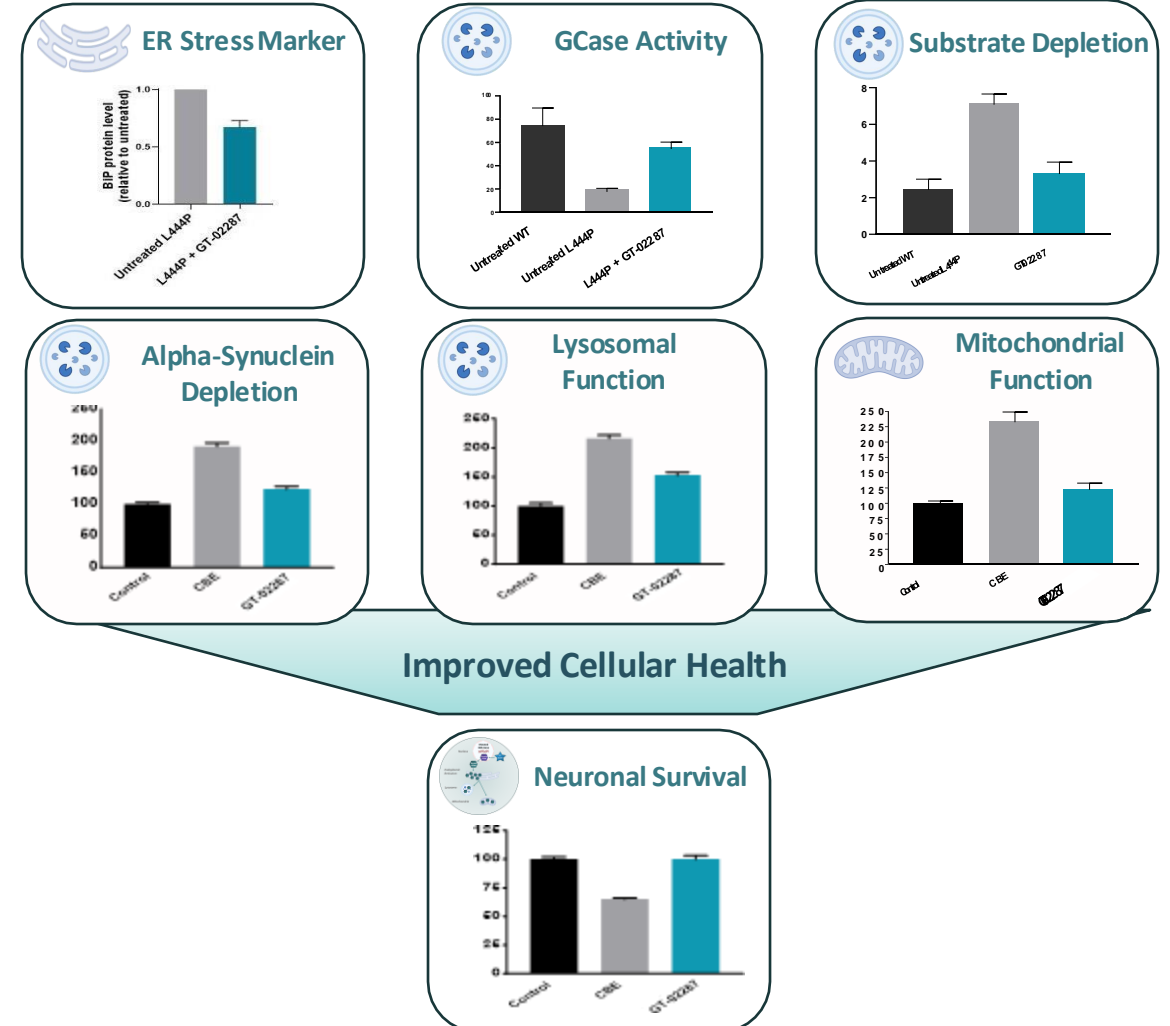


Treatment with GT-02287 restores GCase Function, which improves Disease Cascade and Neuronal Survival

Dopaminergic Neuron with Restored GCase Function



Confirmatory Data Generated with GT-02287



Animal PoC Studies – GT-02287 Improves Effects of GCase Dysfunction

CBE PD Model



- CBE is an inhibitor of the GCase enzyme
- Administration of CBE models the effects of dysfunctional GCase seen in GBA-Parkinson's

Results*

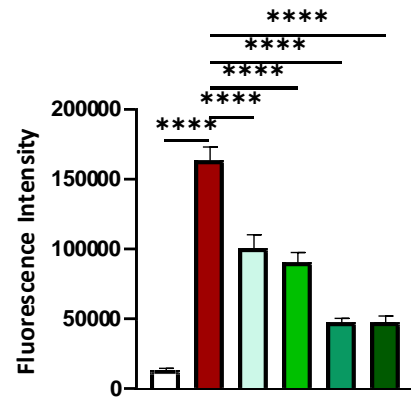
GT-02287 shows effects on neuronal health

- Depleted toxic substrate α -synuclein
- Reduced neuroinflammation (Iba-1)
- Increased neuron survival (NfL)

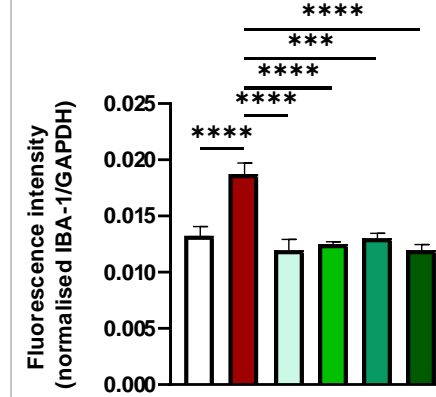
GT-02287 shows effects on motor function

- Increased fine motor skills and coordination (wire hang and beam walk)

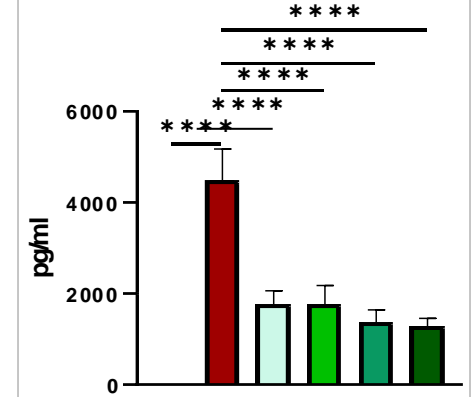
Aggregated α -syn (SN)



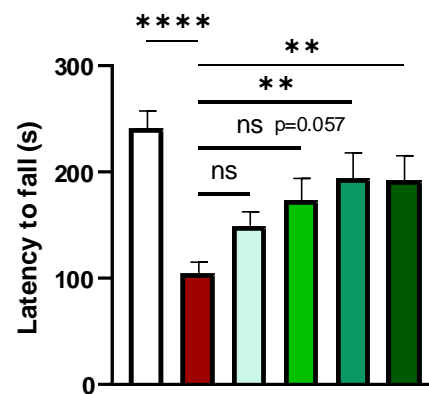
Iba-1 (SN)



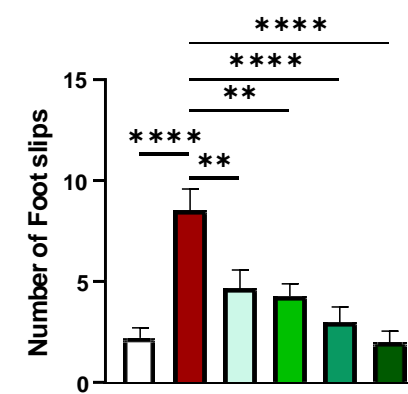
NfL



Wire Hang



Beam Walk



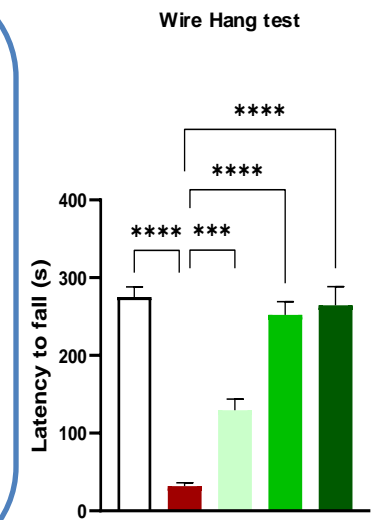
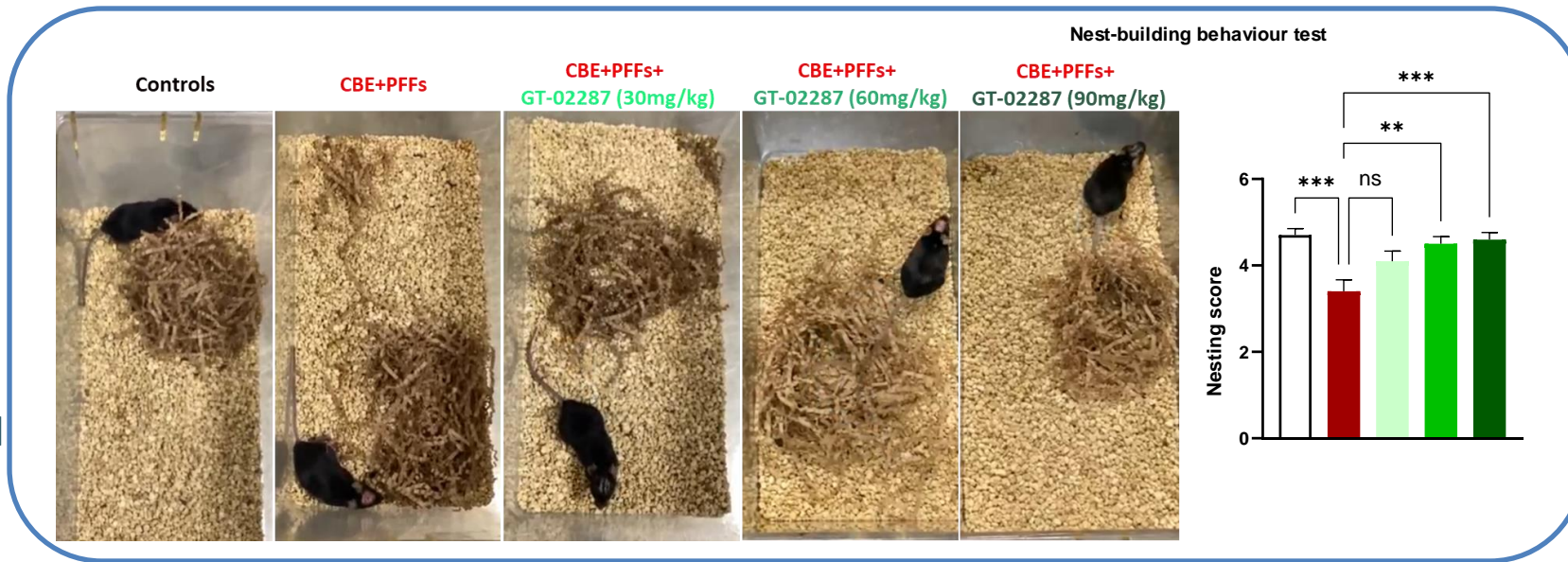
- Vehicle Controls
- CBE (100 mg/kg)
- +GT-02287 (30mg/kg)
- +GT-02287 (60mg/kg)
- +GT-02287 (90mg/kg)
- +GT-02287 (120mg/kg)

Design: 8-10 weeks of age male C57BL/6 mice received daily CBE i.p. injections and oral q.d. drug treatment for 14 days. Wire hang test and beam walk test are performed at day 12. Sacrifice on day 14.

Data is shown as Mean \pm S.E.M. One-way ANOVA followed by Dunnett's Multiple Comparison Test. ****P < 0.0001.

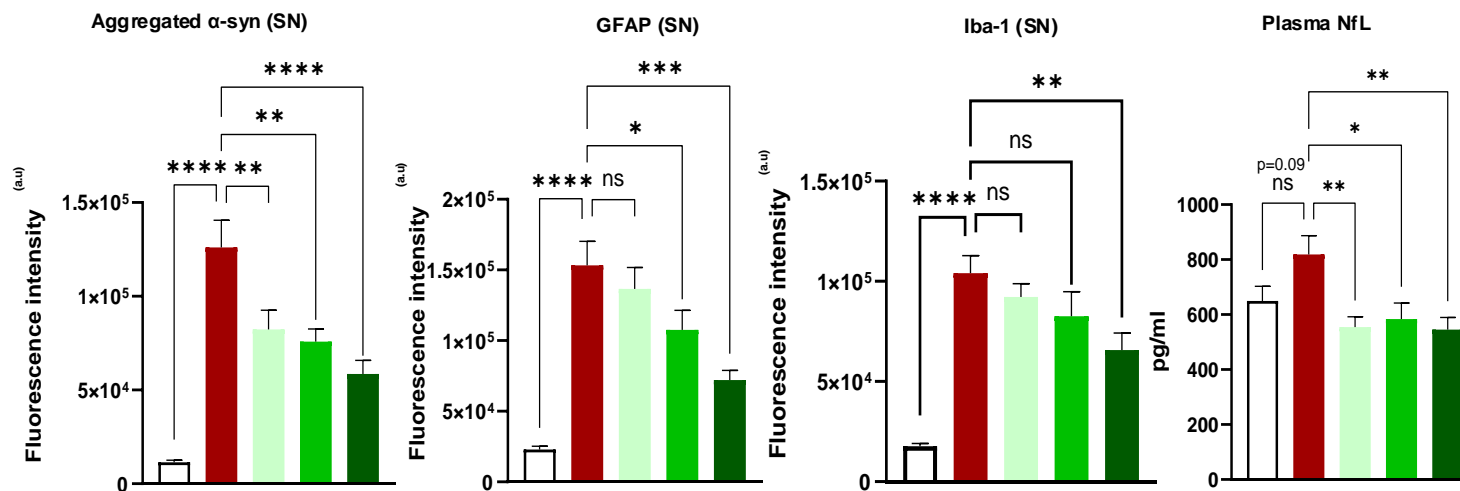
GT-02287 improves activities of daily living and cognitive performance in animal model of GBA1 Parkinson's disease

- Nest building is a non-invasive test to study activities of daily living and cognitive performance in rodent models
- Reduction in plasma levels of NfL, aggregated alpha-synuclein, GFAP, and Iba-1



GT-02287 improves nest-building performance

GT-02287 improves motor performance



GT-02287 has Best-in-Class Profile for GBA1-Parkinson's Disease of known GCase-targeting candidates

	Effect on Disease Cascade	GAIN THERAPEUTICS GT-02287
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	✓

GT-02287 restores GCase function and demonstrates effect on entire disease cascade

GT-02287 has potential to be true disease-modifying therapy for Parkinson's and other CNS diseases

Single Ascending Dose

- Completed in March - 40 subjects treated
- Generally well tolerated, no SAEs
- Exposures in humans reached levels observed in preclinical efficacy studies

Multiple Ascending Dose

- Completed in July - 32 subjects
- Generally well tolerated, no SAEs
- Achieved therapeutic plasma levels with oral dosing

Upcoming Anticipated Milestones and Potential Value Inflection Points

2H 2024

- **Start of Phase 1b POC clinical trial in GBA1-Parkinson's disease**
 - 3-month treatment, open label
 - Safety and tolerability in patients
 - Biomarkers of GCase disease cascade and PD disease
- **Type B FDA Meeting**
- **Potential target engagement readout (GCase Activity)**
- **International Congress of Parkinson's Disease and Movements Disorders**
 - September 27-October 1, Philadelphia
- **Neuroscience 2024**
 - October 5-9, Chicago

1H 2025

- **Potential clinical proof of concept based on biomarkers of GBA1-Parkinson's disease** (interim data from Phase 1b clinical trial)
- **Pre-IND Meeting with FDA in preparation of Phase 2 Clinical Trial**

GT-02287
Differentiated Profile
within Competitive Landscape

Clinical Stage Programs targeting GBA1 and GCase Disease Cascade

Competitive landscape is uncluttered

GCCase-Targeting Small Molecules

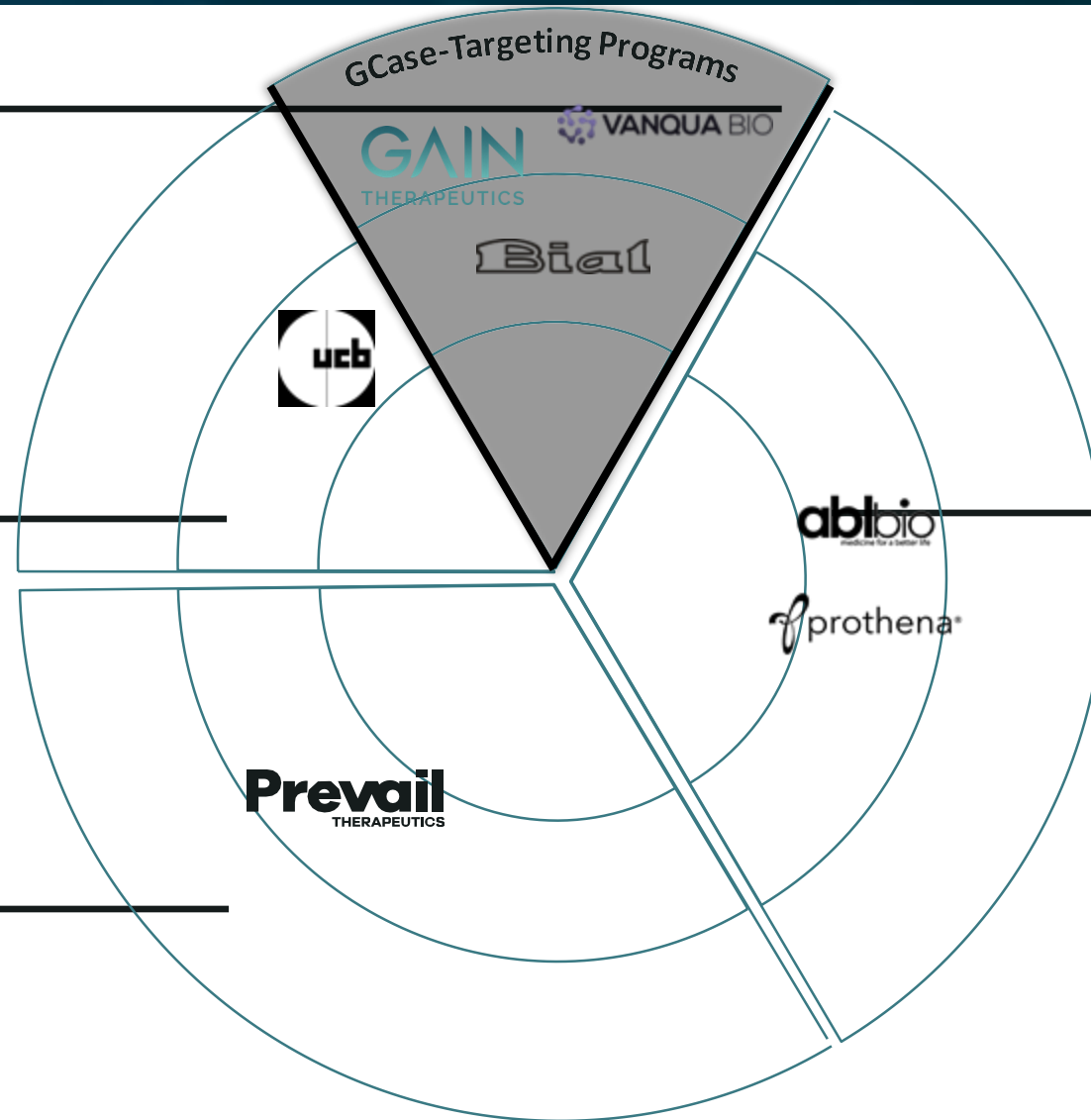
- ★ Gain Therapeutics, Inc.
GT-02287 allosteric GCase chaperone
- ★ Vanqua Bio (Private)
Allosteric GCase activator
- ★ Bial (formerly LTI - acquired for \$130M)
Allosteric GCase activator

α-Syn-Targeting Small Molecules

- ◆ UCB
Small molecule targeting a-synuclein
partnered with Novartis

Gene Therapy

- ★ Prevail Therapeutics (acquired by Lilly for \$1B)
AAV gene therapy for PD-GBA and nGD



PHASE 1

PHASE 2


PHASE 3

α-Syn-Targeting Antibodies

- ◆ ABL Bio, Inc.
Anti-Synuclein/IGF1R monoclonal antibody
partnered with Sanofi
- ◆ Prothena Corporation
Small molecule targeting a-synuclein
partnered with Roche

- ★ GBA/GCase Targeted Approaches
- ◆ Alpha-Synuclein Targeted Approaches

GT-02287 has Favorable Profile over other known MOAs and Modalities

Modality	GCCase-Targeting Small Molecules 	α -Syn-Targeting Small Molecules	α -Syn-Targeting Antibodies	Gene Therapy
Upstream Effect	✓	✗	✗	✓
Brain Penetration	✓	✓	✗	✗
Convenience of Administration	✓	✓	✗	✗
Safety	✓	✓	✓	✗
Commercial (COGS/Pricing)	✓	✓	✓	✗







GT-02287 has Best-in-Class Profile for GBA1-Parkinson's Disease of known GCase-targeting candidates

	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	✓	⊗	⊗

Large Pharma is interested in GBA1-related Programs

Recent acquisitions at >10x of Gain's market cap

	Effect on Disease Cascade	GAIN THERAPEUTICS GT-02287	
GCase Mechanism of Action	Increases Lysosomal GCase Activity	✓	<div style="border: 1px solid black; border-radius: 15px; padding: 10px; text-align: center;"> <p>\$1.2B+ in big pharma acquisitions of preclinical GBA programs in the last year</p> </div>
	Reduces ER Stress	✓	
	Reduces Toxic Lipid Substrates	✓	
	Reduces Aggregated α -Synuclein	✓	
	Improves Lysosomal Function	✓	  <p>\$610M in upfront/milestones</p>
	Improves Mitochondrial Function	✓	  <p>\$655M in upfront/milestones</p>
	Reduces Neuroinflammation	✓	
Disease- Modifying Effect	Provides Neuroprotection	✓	<div style="border: 1px solid black; border-radius: 15px; padding: 10px; text-align: center;"> <p>Gain is the only small molecule GBA approach to show disease modifying effects</p> </div>
	Increases Dopamine Levels	✓	
	Restores Motor Function	✓	
	Improves Cognitive Function	✓	

Large Market Potential for GT-02287 Across Several Neurodegenerative Diseases

Indication	Rationale	Number of Patients (US)	Total Addressable Market (US)
GBA1-Parkinson's Disease	<ul style="list-style-type: none"> Patients have dysfunctional GCase due to heterozygous GBA mutation 	150,000	\$1.5B
Idiopathic Parkinson's Disease	<ul style="list-style-type: none"> GT-02287 has shown effects in preclinical WT models of PD 	1,000,000	\$10B
Gaucher Disease	<ul style="list-style-type: none"> Patients dysfunctional GCase due to homozygous GBA mutation 	6,000	\$1.2B
Dementia with Lewy Bodies	<ul style="list-style-type: none"> GT-02287 depletes aggregated alpha-synuclein which forms Lewy bodies 	1,000,000	\$10B
Alzheimer's Disease	<ul style="list-style-type: none"> GT-02287 has shown positive effects in preclinical models of Alzheimer's disease 	5,800,000	\$58B

Company Background



CORPORATE BACKGROUND

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

FINANCIAL AND STOCK DATA

IPO (Nasdaq: GANX)

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

CASH POSITION

- \$16.9 million at June 30, 2024

CAPITAL STRUCTURE

- 25.1 million shares outstanding
- No debt*

*Except a 9-year interest-free Swiss government loan
~\$0.5 million outstanding at September 30, 2023

ANALYST COVERAGE

BTIG:

Tom Shrader, PhD, CFA: Buy

Oppenheimer & Co.

Jay Olson, CFA: Outperform

H.C. Wainwright

Ram Selvaraju, PhD: Buy

Chardan

Keay Nakae, CFA: Buy

The Maxim Group

Jason McCarthy, PhD: Buy

INTELLECTUAL PROPERTY

Strong intellectual property estate

- Patent applications for 5 NCE families
- GT-02287 composition of matter patent application with term through 2038 (to be extended under Hatch Waxman Act)

Company Contacts

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Apaar Jammu: Manager, Investor Relations and Public Relations

ajammu@gaintherapeutics.com