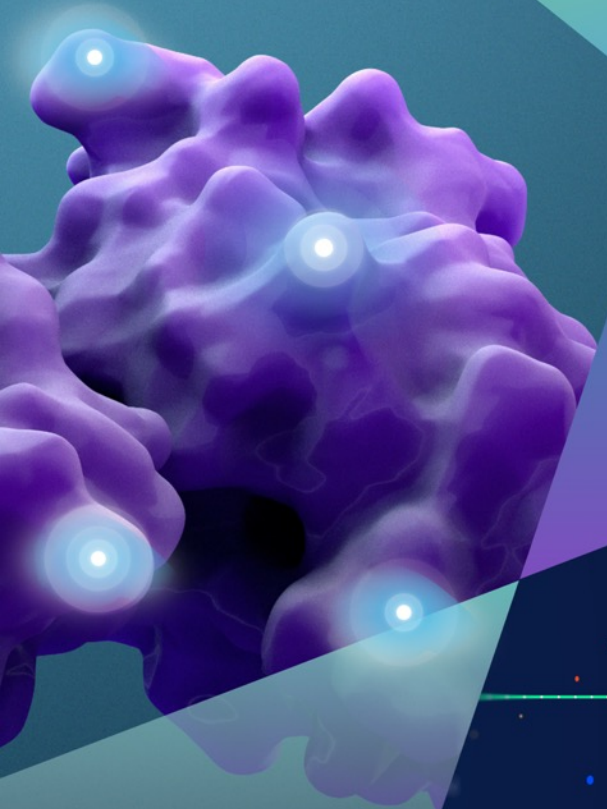
GAIN THERAPEUTICS



Leading the Discovery of Allosteric Binding Sites to Create New Medicines

Corporate Presentation

March 2021

Forward-Looking Statement

Forward-Looking Statements

Certain statements set forth in this presentation contain forward-looking statements that 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 SEE-Tx 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 SEE-Tx platform; the Company's ability to identify, recruit and retain key personnel; the Company's expected use of net proceeds from this offering and the sufficiency of such net proceeds, together with the Company's cash and cash equivalents, to fund its operations; 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.

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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.

Executive Summary

Therapeutic Focus	• Addressing Protein Misfolding by identifying and targeting novel previously undruggable allosteric binding sites • Initial focus on lysosomal storage and CNS diseases -- large & growing markets with unmet medical need
Platform Technology	• Proprietary and patented SEE-Tx platform identifies allosteric (non-catalytic) binding sites in proteins and enzymes that can regulate proper protein folding to restore functional activity
Robust Pipeline	• Demonstrated targeted engagement • Strong intellectual property estate-5 new NCE families patented
Global Partnerships	• Strong relations with academic centers, key patient associations, and development partners • Industrial partnership established with Sumitomo and potential to establish additional partnerships
Team	• Seasoned and experienced management team, board of directors and scientific advisors
Upcoming Milestones	• Near term milestones: several development candidates currently completing animal POC • IND enabling studies starting 2021

Gain Therapeutics' Dual-pronged Approach to Value Generation

Gain leverages its proprietary platform technology to engineer novel therapeutics targeting conditions with significant unmet needs

1 SEE-Tx Discovery Platform

- Proprietary supercomputer-driven drug discovery platform
- Identifies novel allosteric binding sites on misfolded proteins
- Enables targeting of novel allosteric binding sites and generation of proprietary molecules efficiently and quickly

2 Diversified Pipeline of Drug Candidates targeting multi-billion $ markets

- Pipeline candidates translated from SEE-Tx platform to preclinical development
- Primarily targeting lysosomal storage disorders and generic neurodegenerative disorder, including:
  - Morquio B/ GM1 Gangliosidosis
  - Gaucher Disease
  - Parkinson's Disease
- Opportunity to expand into other indications addressing protein misfolding

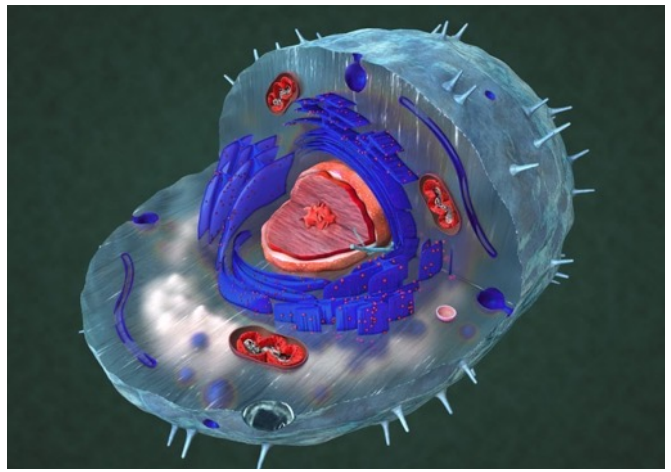
		Discovery		Pre-Clinical		Clinical		
Gene	Disease	*in vitro*	*in vivo*	Lead OP	IND-Enabling	Phase 1	Phase 2	Phase 3
GLB1 gene	Morquio B / GM1 Gangliosidosis							
GBA1 gene	Gaucher Disease							
GBA1 gene	Parkinson's Disease							
IDUA gene	Mucopolysaccharidosis type 1							
GALC gene	Krabbe Disease							

Sumitomo Dainippon Pharma — R&D collaboration with Sumitomo on an undisclosed demyelinating disease target

Key Markets: Broad Platform Applicability

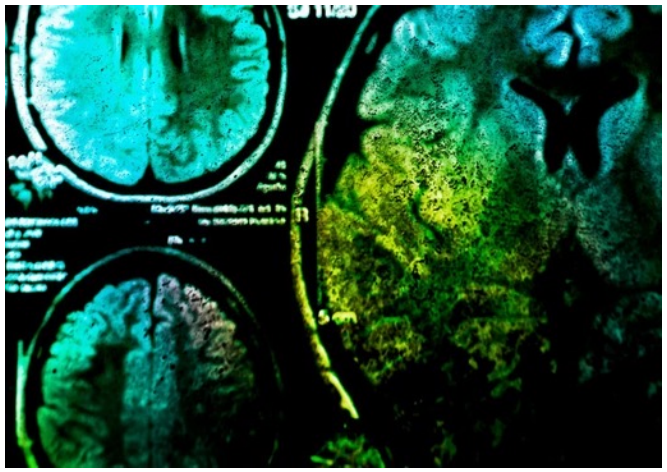
SOC Challenges: Inability to access hard to reach tissues

Lysosomal Storage Disorders



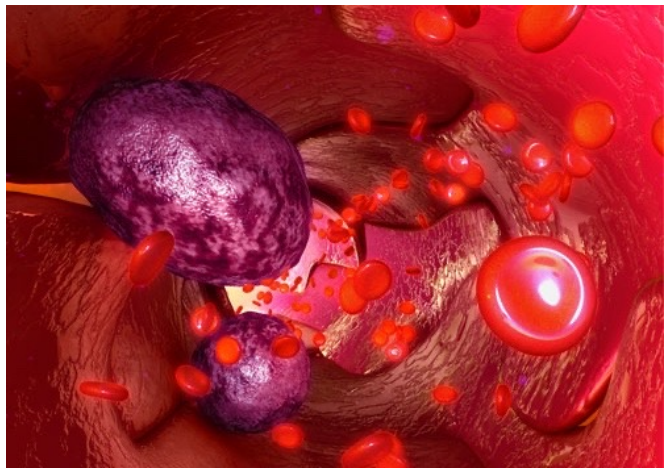
LSDs represent significant markets with high unmet needs, especially in neurological conditions

Neurodegeneration



A multi billion-dollar market with high unmet needs in a growing patient population

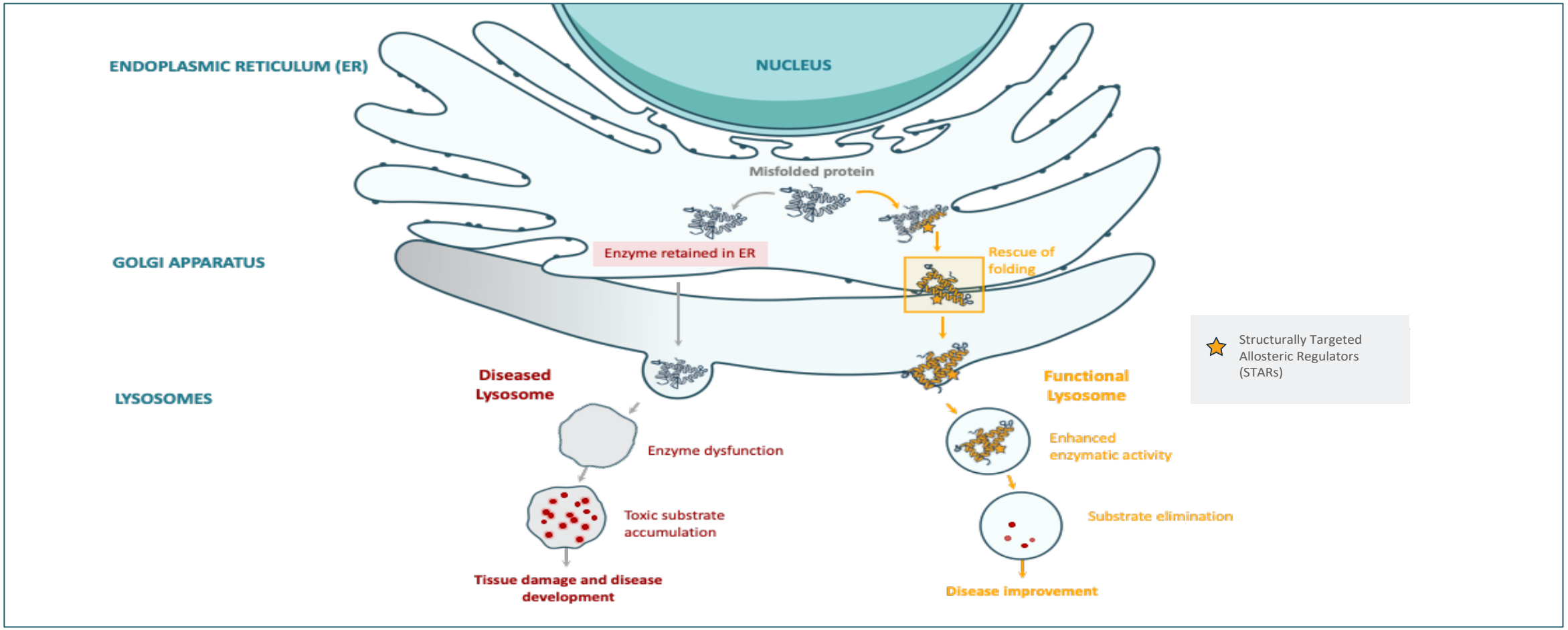
Future Applications



Oncology, Mitochondrial Diseases, Immunology, Metabolic Disorders

Corrected Folding of Enzymes to Restore Functional Activity

Misfolded enzymes cannot properly catalyze their intended substrates, leading to toxic substrate accumulation.



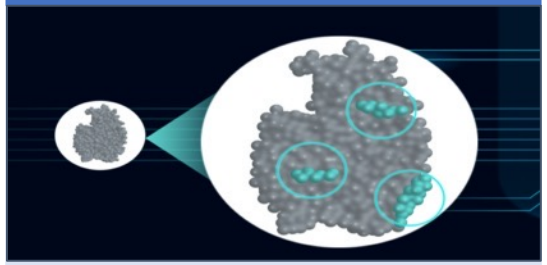
Binding allosteric sites has the potential to provide superior regulation of misfolded enzymes implicated in diseases.

SEE-Tx™ Discovery Platform

DIFFERENTIATED and PATENTED highly-specific, efficient and cost-effective drug discovery approach

DEFECTIVE PROTEIN — Caused by genetic mutation, ageing, and disease. Results in loss of protein function and toxic substrate buildup.

IDENTIFY



Using the 3D structure of proteins and supercomputing technology, SEE-Tx **identifies** novel druggable binding hotspots

SCREEN & SELECT

Utilizing proprietary supercomputer driven **screening** methodology, SEE-Tx filters up to 10 million compounds to **select** a pool of candidates that may bind to the novel druggable hotspots

OPTIMIZE

Gain **optimizes** this pool of small molecules to identify and develop proprietary STARs that stabilize misfolded proteins and to restore their biological activity

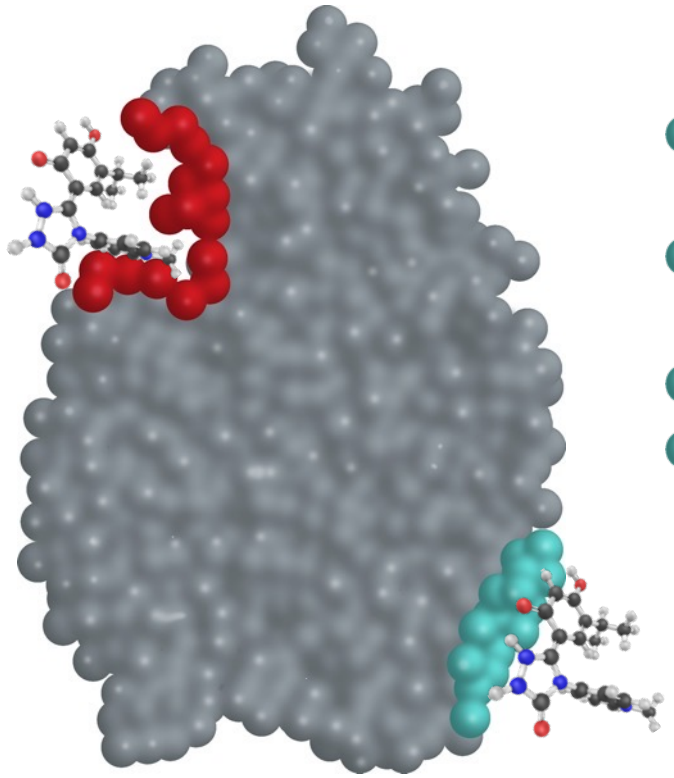
DEVELOPMENT CANDIDATE — Stabilizes protein enzyme, restores enzyme function, reduces substrate buildup, crosses the blood brain barrier, and penetrates other hard to reach tissues such as bone and cartilage.

Gain has patented methodology & algorithm (WIPO/PCT WO 2013/092922 A2).

Advantages of Drugging Allosteric Binding Sites

Active Binding Site

- Site where enzyme binds substrate to catalyze reaction
- Traditional drugs bind to active sites
- Competes with active substrate - decreases efficacy
- May lead to selectivity issues



Allosteric Binding Site

- Non-competitive with the natural substrate
- High specificity for the targeted enzyme
- Superior drug-like properties
- Offer a broader therapeutic window

Active binding site

Allosteric binding site

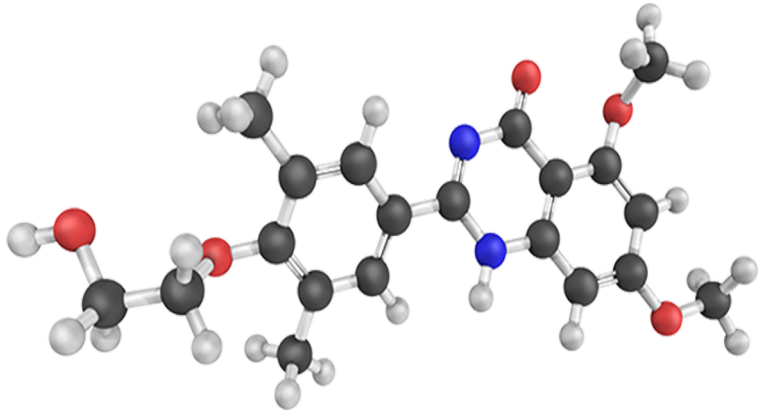
Binding allosteric sites has the potential to provide superior regulation of misfolded enzymes implicated in diseases.

SEE-Tx™ Platform Produces Novel STARs

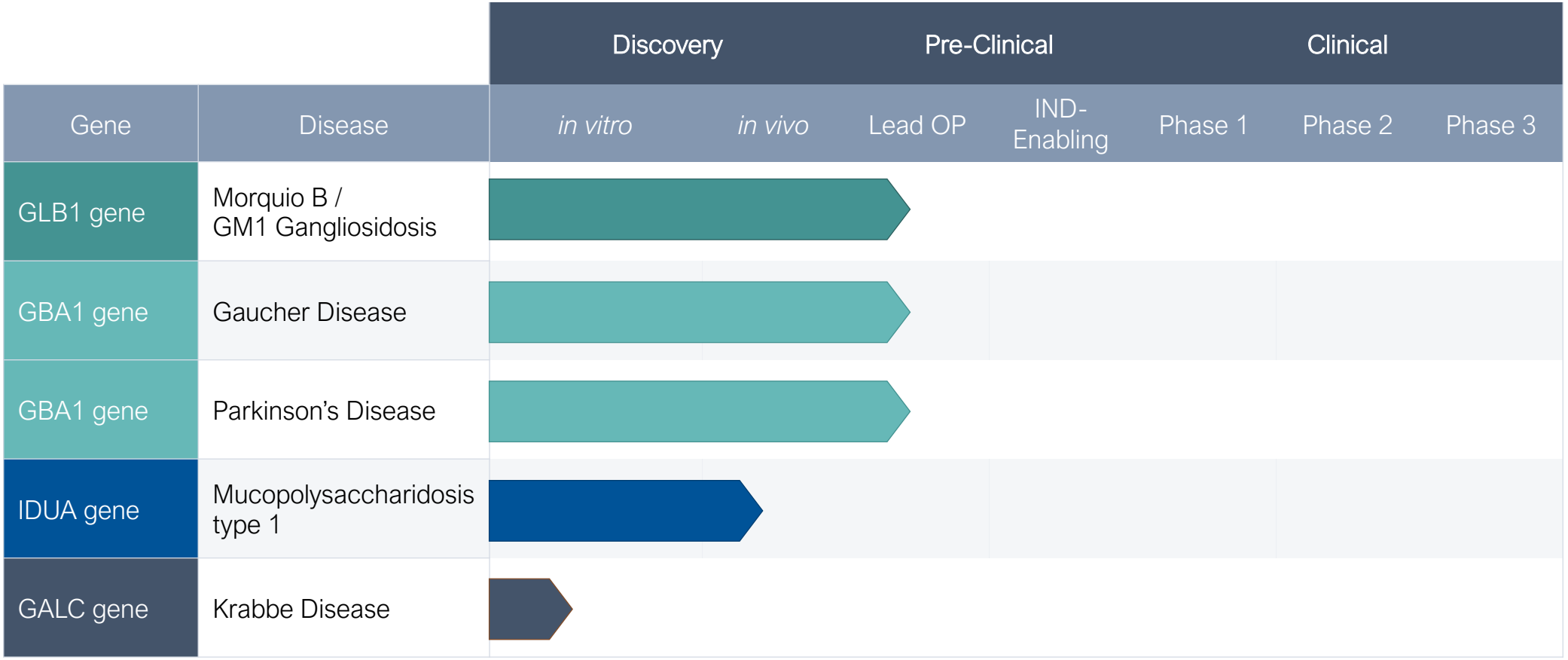
Criteria for Selection of STARs

1. Previously unidentified allosteric site
2. Stabilizes misfolded protein with potential to restore biological activity
3. Crosses the blood brain barrier after oral administration
4. Reduces toxic substrate
5. Highly specific

- STARs: Novel small molecule compounds selected to bind to allosteric sites and restore protein function, cross blood-brain barrier and access bone, cartilage and other hard to reach tissues
- Easily scalable small molecules with potential oral bioavailability
- Improved pharmacokinetic (PK) profile with better safety profile and therapeutic window
- No interaction with the target enzyme's active site and ability to stimulate wild-type enzyme activity



Diversified Pipeline Targeting Multi-Billion Dollar Markets



Sumitomo Dainippon Pharma

R&D collaboration with Sumitomo on an undisclosed demyelinating disease target

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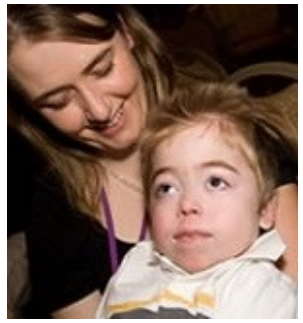
The Programs

GM1 Gangliosidosis/Morquio B

Beta-galactosidase (GLB1)

GLB1-related Disorders: GM1 and Morquio B

Deficiency of acid β -galactosidase causes **GM1 gangliosidosis** and **Morquio disease type B** (MPS IVB)



GM1 Gangliosidosis

- Indidence worldwide (1:100,000-200,000 newborns).[1]
- β-galactosidase is responsible for breaking down GM1-ganglioside, abundance of ganglioside results in neurodegeneration and severe neurological conditions.
- Symptoms include developmental regression, skeletal abnormalities, loss of vision, and seizures.

Morquio B

- Prevalence worldwide (1:250,000-1,000,000).[2]
- It is caused by genetic mutation of GLB1 gene that eliminates activity of β-galactosidase leading to accumulation of keratan sulphate in organs and tissues.
- Symptoms include loss of nerve function, abnormal bone and spine development, and hearing loss.

Life-long chronic treatment targeting over 7000 patients

Peak sales: > $1 billion

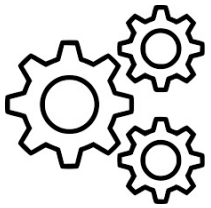
Currently, no effective medical treatment for GLB1-related disorders.
Symptomatic treatment for some neurologic sequelae but does not alter the clinical course.

GAIN THERAPEUTICS

[1]Lysogene
[2]Elsevier doi:10.1016/j.ymgmr.2018.06.006

GM1/Morquio B Program Summary: Lead Candidate GT-00513

Small (400 Da) molecule with good drug-like properties



Mechanism of Action

- Dose-dependent binding of β-gal
- Non-competitive activity
- Support proper folding and protein maturation

in vitro Target Engagement

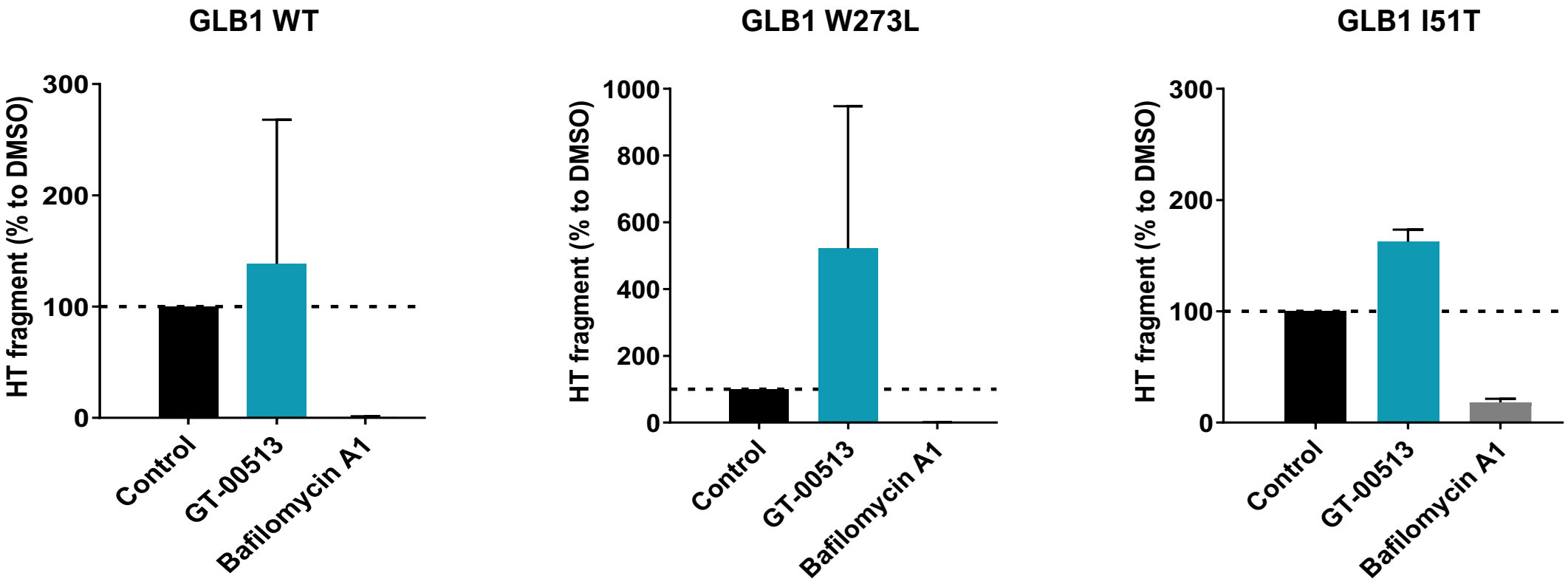
- Enzyme stabilization and enzymatic enhancement in fibroblasts
- Clearance of toxic substrate in fibroblasts

Drug Properties

- High oral bioavailability
- Penetrates well into Brain (CNS) and bone (skeletal)
- Half-life suitable for once daily use
- Well-tolerated in multiple dose (sub-chronic) toxicity studies

Intellectual Property (IP)	PCT application published July 2018 National phases 2019

GT-00513: Increases the Delivery of β-Gal to the Lysosome



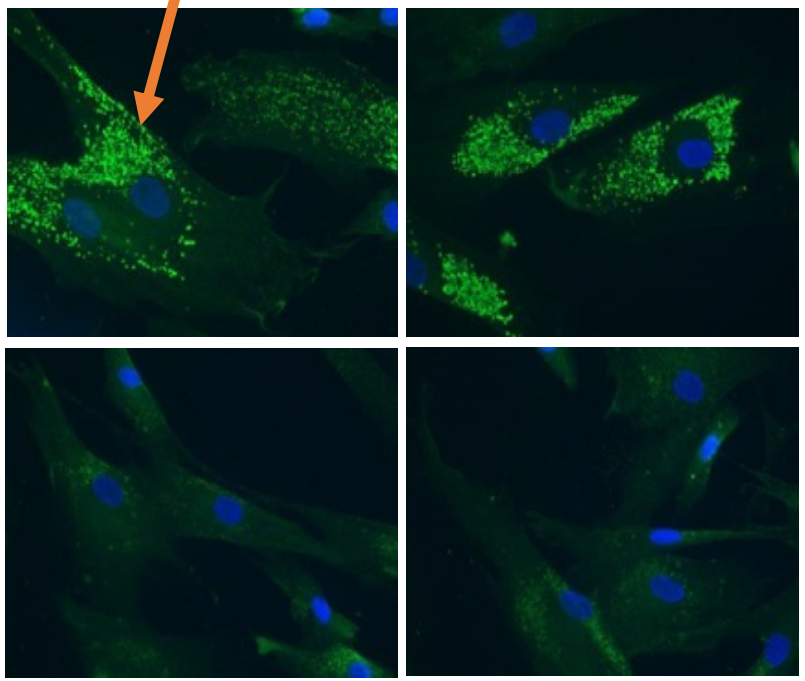
HEK293 were transfected with either GLB1 WT, GLB1 I51T, GLB1 R201C and GLB1 W273L with HaloTag. 30 hours later, compounds (25 µM) and a fluorescent Halo ligand that covalently binds to a pocket in the tag were added for 17 hours. Once the protein reaches the lysosome, the tag is cleaved off. The 31 KDa tag is resistant to lysosomal hydrolases and it can be detected as a fluorescent fragment that corresponds to the protein that reached the lysosomes. Full length protein and lysosomal fragment are measured by western blot.

GT-00513: Reduces GM1 Ganglioside Accumulation

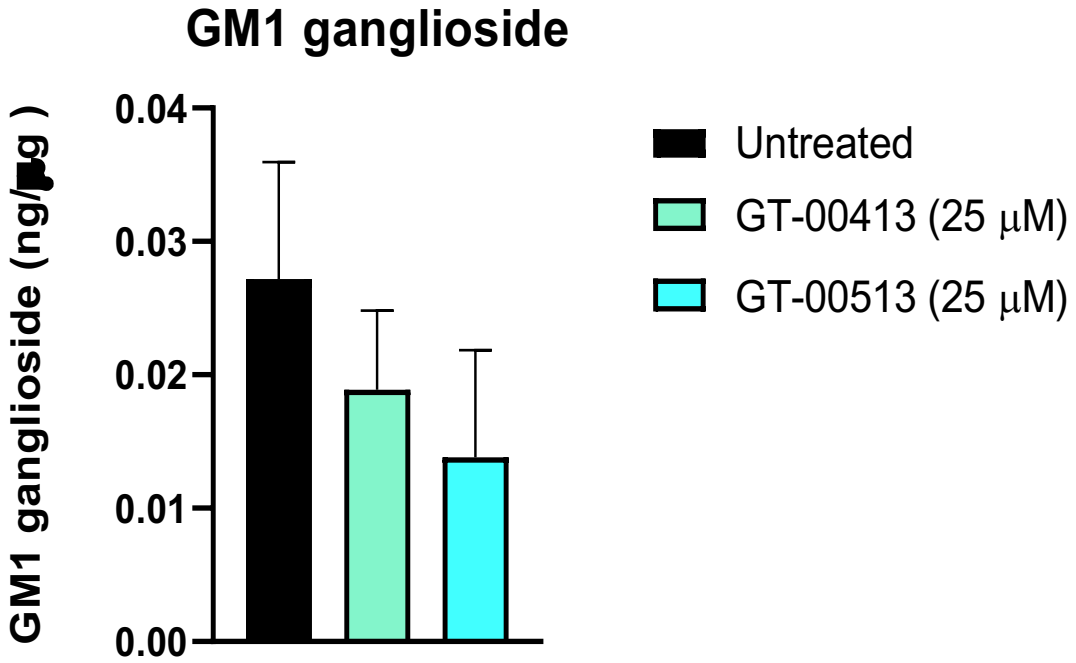
GM1 ganglioside

GLB1 fibroblasts accumulate GM1 ganglioside

STAR^{S} are effective in reducing substrate accumulation



GM1 gangliosidosis canine fibroblasts (p.R60H/p.R60H; equivalent to R59H mutation in humans) were loaded with GM1 ganglioside for 2 days followed by culture in the presence of GT-00413 at 25 µM for 4 subsequent days. The cells were fixed, permeabilized and stained to detect GM1 ganglioside. Nuclei were counterstained with DAPI.

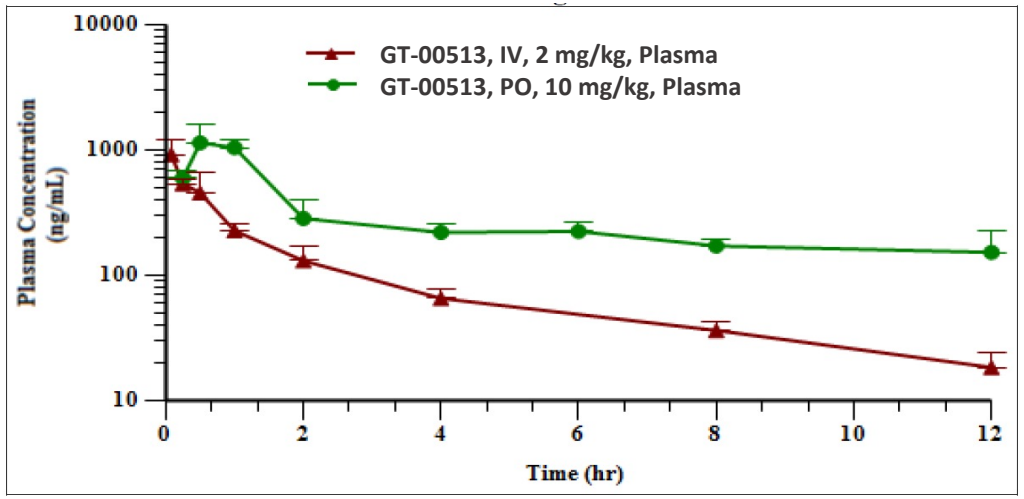


p.R60H/p.R60H canine fibroblasts (Coriell GM11473) were treated with 25 µM of GT-00513 in triplicates. Cells were harvested at day 4 and samples were analysed for GM1 ganglioside quantification using MS/MS. Data is expressed as mean ± SD (*n*=1, preliminary results).

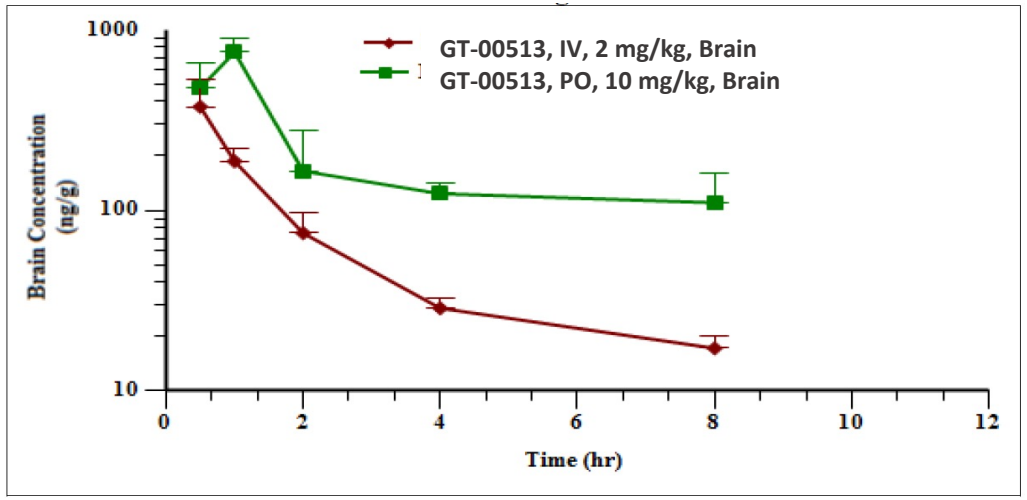
GT-00513: Demonstrates High Oral Bioavailability

	Route	Dose (mg/kg)	Test System	Tmax (hr)	aCo/Cmax (ng/mL)	AUClast (hr*ng/mL)	AUCinf (hr*ng/mL)	T1/2 (hr)	CL (mL/min/kg)	Vss (L/kg)	% Fb
GT-00513	IV	2	male C57BL/6 mice	-	1181.29	1195.16	1314.68	4.39	25.35	6.12	-
GT-00513	PO	10	male C57BL/6 mice	0.5	1140.21	4548.73	4711.51				**76**

Plasma exposure



Brain exposure

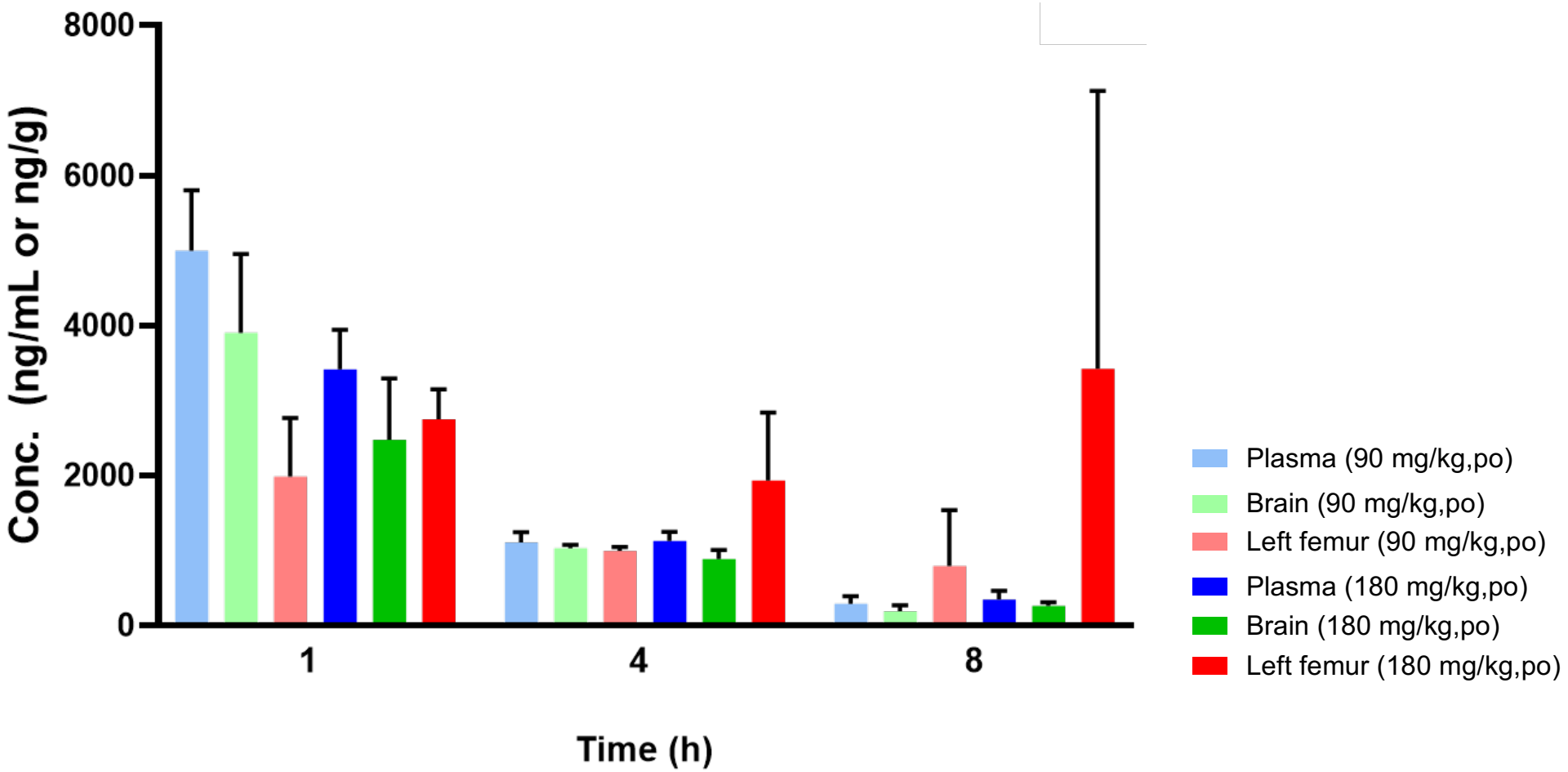


Brain-to-plasma ratio ranged 0.42-0.72

Plasma pharmacokinetics of GT-00513.HCl following a single intravenous and oral administration to male C57BL/6 mice. n=3 and data are expressed as mean ± SD.

GT-00513.HCl salt oral bioavailability, F = 76%.

GT-00513: Penetrates well into both Brain and Bone

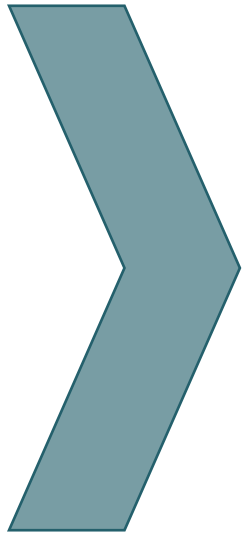


Plasma, brain and left femur concentration-time data of GT-0513 in male C57BL/6 mice on day 7

GM1/Morquio B Program Status and Next Steps

Lead Candidate GT-0513

- Demonstration of mechanism of action indicative of specific binding and bypassing protein quality control of protein misfolding
- Demonstration of *in vitro of* target engagement as measured by β-galactosidase enhancement and toxic GM1 gangliosides depletion
- Orally bioavailable, brain and bone penetrant



Upcoming milestones

- In vivo POCs (Morquio B + GM1) (2021)
- CD Characterization/Synthesis scale-up (2021)
- IND/CTA-enabling studies (2021-2022)
- Phase 1/2: SAD/MAD (safety/PK) in pediatric population (2023-2024)

GAIN THERAPEUTICS

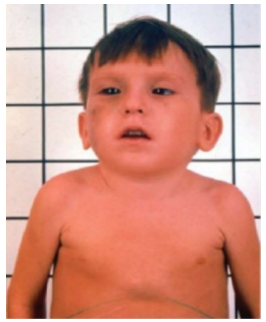
The Programs

Gaucher/Parkinson's Disease

Beta-glucosidase (GBA)

GBA1 Related Disease: Gaucher and Parkinson's Disease

Mutations in the GBA1 gene are responsible for build-up of glucosylceramide in Gaucher disease and is associated with α-synuclein accumulation in Parkinson's.



Gaucher Disease

- There are approximately 1,000-2,000 patients with Neuronopathic (Type 2 and 3) Gaucher's Disease in the US and Europe. (1:100,000 newborns).[1]
- Mutations in the GBA gene cause deficiency in glucocerebrosidase enzyme, resulting in glucosylceramide build up in the liver, spleen, bone marrow and nervous system
- Characterized by severe neurological symptoms such as abnormal eye movements, seizures, and brain damage.

Life-long chronic treatment targeting over 7000 patients

Peak sales: > $900 million

Parkinson's Disease

- Parkinson's Disease affects 10 million people worldwide. Approximately 500,000 with Parkinson's have a GBA1 mutation. (5% of all Parkinson's Patients) [2]
- GBA mutations result in early onset, faster progression, and various non-motor symptoms due to accumulation of α-synuclein.
- Symptoms include uncontrollable shaking/tremors, slowed movement, and stiffness in limbs.

Disease modifying treatment targeting over 2.4m patients

Peak sales: > $5 billion

Currently, no effective medical treatment for GBA1-related disorders.

Symptomatic treatment for some neurologic sequelae but does not alter the clinical course.

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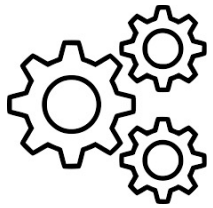
[1]Lysogene
[2]Exon Publications

GBA Program: Lead Candidates GT-02287 and GT-02329

GT02287 and GT02329 are small molecules of 350-450 Da with good drug-like properties



Mechanism of Action

- Dose-dependent and non-competitive binding to GCase
- Stabilization of target enzyme, support proper folding and protein maturation

in vitro Target Engagement

- Enzymatic enhancement in patient cells in the sub-micromolar range
- Enzymatic enhancement in dopaminergic neurons
- Neuroprotection in a cellular model
- Clearance of phosphorylated and aggregated synuclein in neuronal cell models
- Clearance of glucosylceramide and glucosylsphingosine in cells

in vivo Target Engagement

- In vivo GCase enzyme enhancement
- Synuclein depletion, neuroprotection etc.
- In vivo locomotor improvement in rotenone animal model

Drug Properties

- Oral bioavailability
- Brain penetration
- Well tolerated in acute and sub-chronic toxicity studies
- Non cytotoxic & non mutagenic

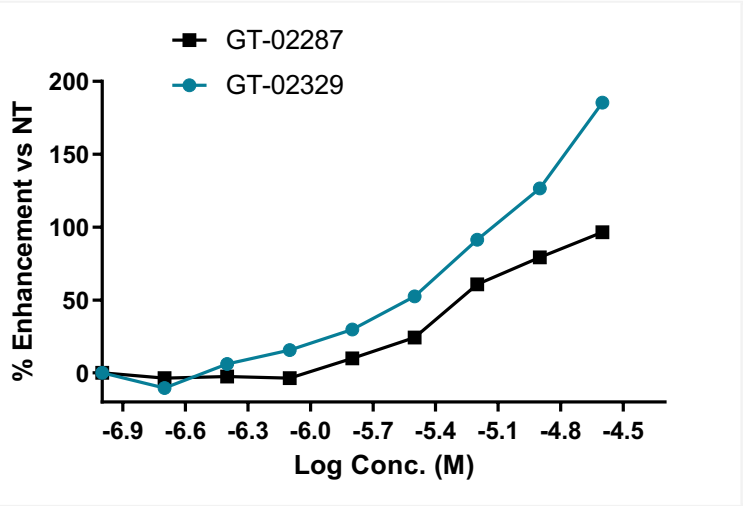
Intellectual Property (IP)

PCT application published July 2018
National phases 2019, Add. PCT files Nov. 2021

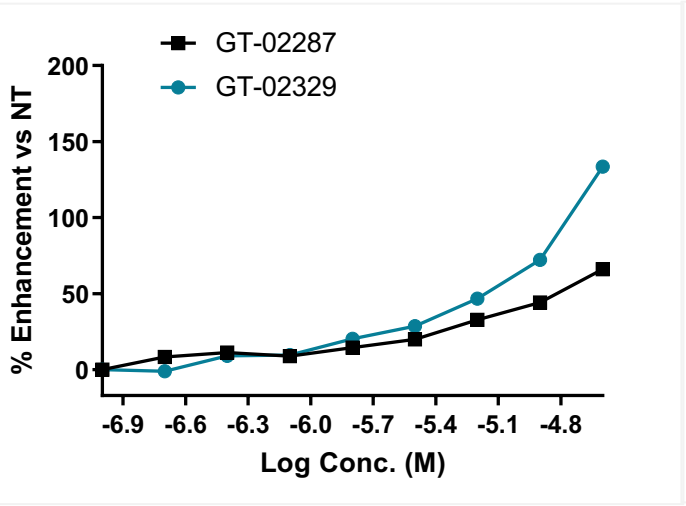
STARs Enhance GCase at Sub-Micromolar Concentrations

GT compounds enhance GCase activity in a dose-dependent manner

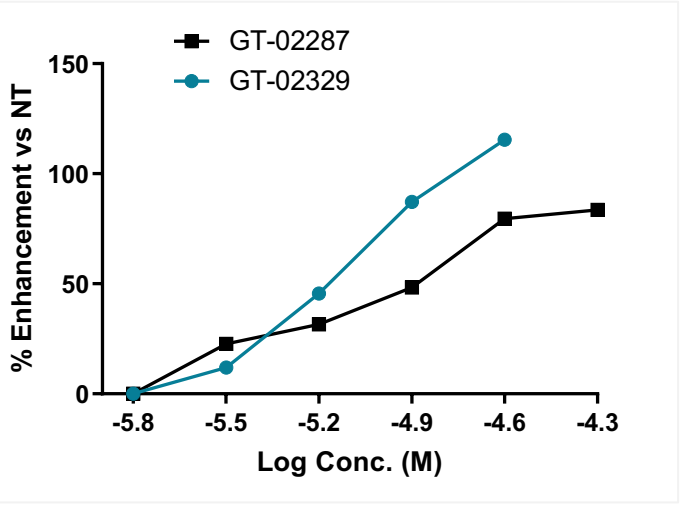
p.L444P/p.L444P fibroblasts (Gaucher type II)



WT fibroblasts



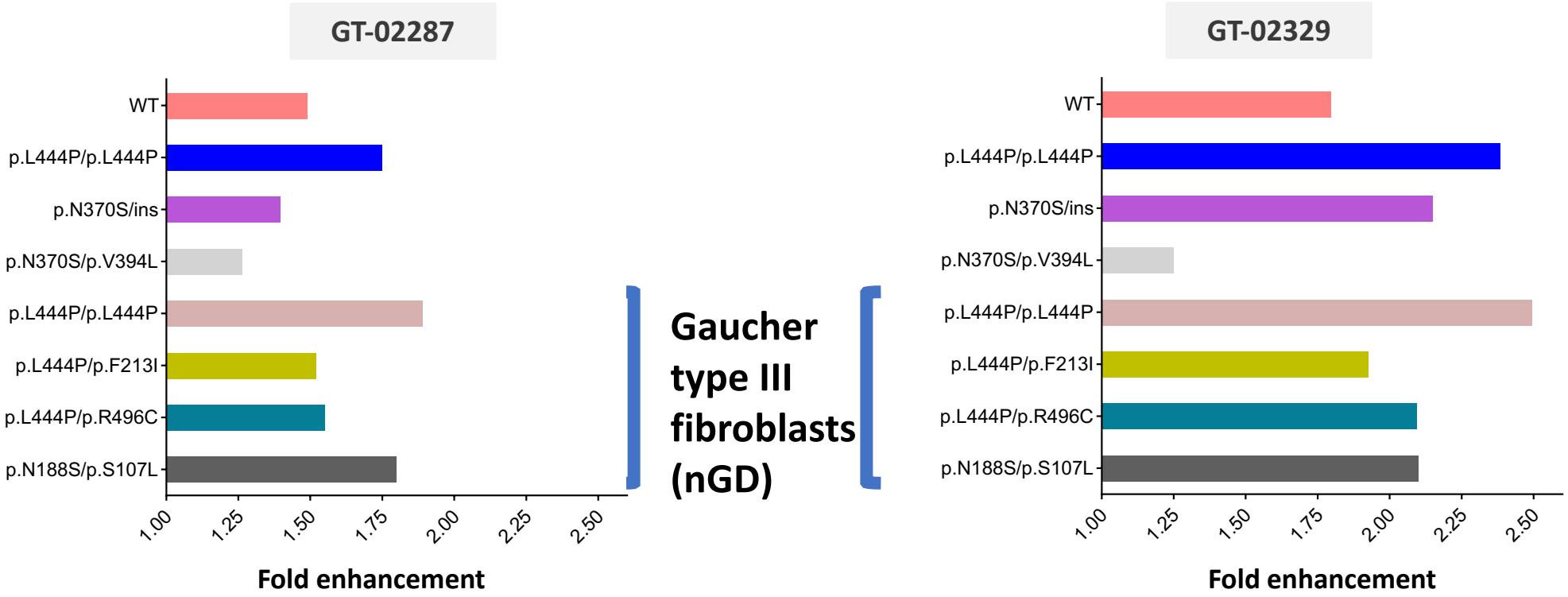
p.N370S/ins fibroblasts (Gaucher type I)



Gaucher-patient fibroblasts were treated with compounds at different concentrations (0.2 – 25 µM) in sextuplicate. After a 4-day treatment, GCase activity was assessed using the 4-MU-β-D-glucopyranoside substrate. Fold increase compared with non-treated cells was calculated.

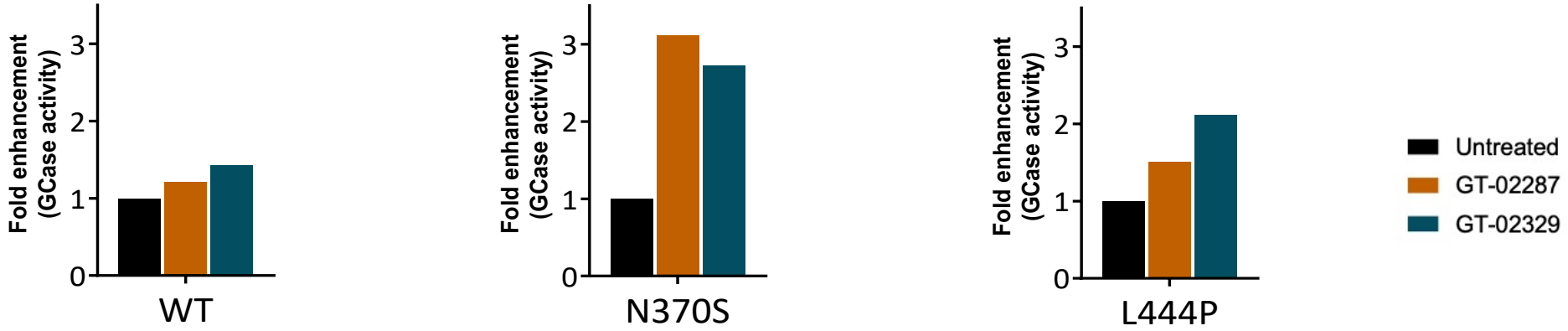
STARs Enhance Enzyme Activity in Patient Fibroblasts

GT compounds enhance GCase activity in wild-type and patient-derived Gaucher fibroblasts

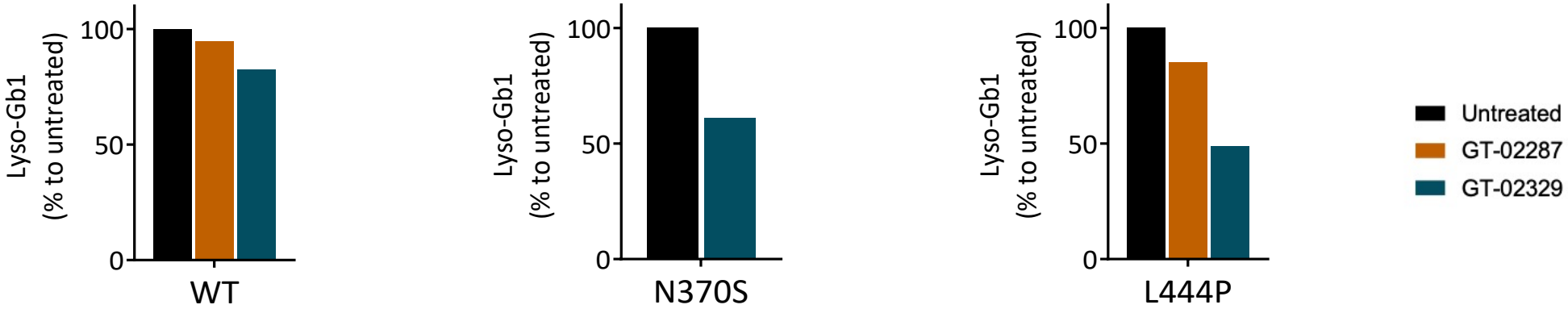


Gaucher patient-fibroblasts, WT fibroblasts were treated with GT compounds, isofagomine at 12.5 µM. After 4-day treatment, GCase activity was assessed using 4-MU-β-D-glucopyranoside substrate. The assay reaction is started by the addition of 28 µL of 5 mM of 4-MU-beta-D-glucopyranoside in 0.1 M acetate buffer (pH 4) to each well. Plates are incubated at 37ºC for 1h and the reaction is stopped by the addition of 200 µL of glycine buffer (pH 10.7) to each well. Liberated 4-methylumbelliferone is measured (excitation 340 nm, emission 460 nm). Fold increase compared with non-treated cells was calculated.

In a Neuronal Cellular Model STAR^{s} increase GCase activity of wild-type as well as GCase carrying the most prevalent mutations (top) and Reduce Glucosylsphingosine Levels (bottom)

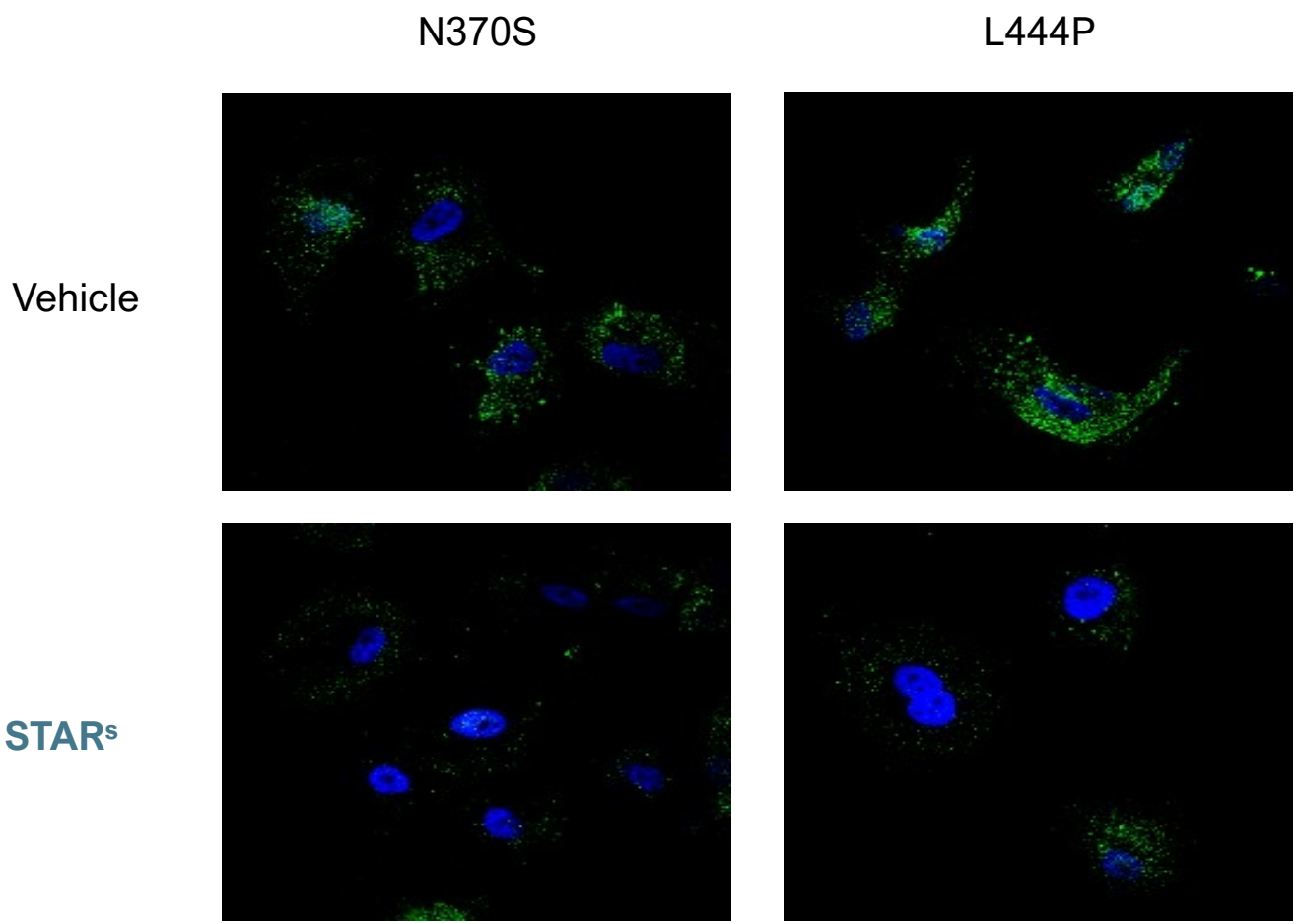


Dopaminergic neurons BE(2)-M17 carrying either wild-type or mutant N370S, L444P or D409V GBA1 mutations were treated for 4 days with 25 µM of GT-compounds. GCase activity was measured with 4-methylumbelliferyl-β-D-glucopyranoside. For the 10 days incubation experiment, media exchange with compound is performed after 3 days, then after 4 days and finally cells are harvested after 3 additional days.



Dopaminergic neurons BE(2)-M17 carrying either wild-type or mutant N370S, L444P or D409V GBA1 mutations were treated for 10 days with 25 µM of GT-02287 or GT-02329 compounds. Media exchange with compound was performed after 3 days, then after 4 days and finally cells are harvested after 3 additional days. GlcSph (Lyso-Gb1) levels were quantified by LC-MS/MS.

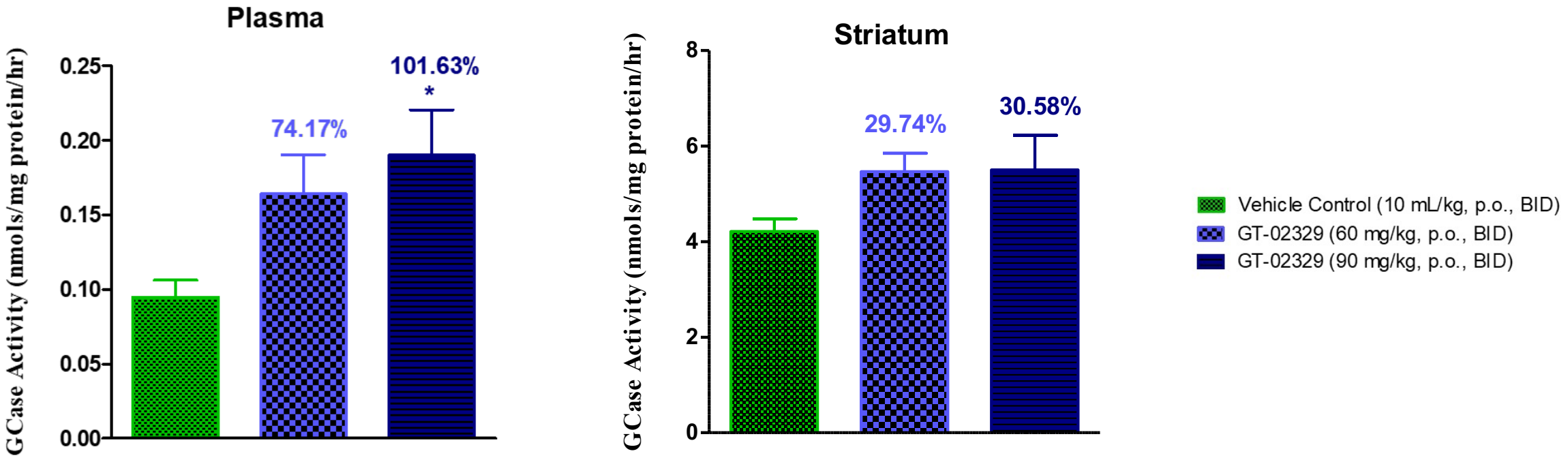
STAR^{s} Decrease α-Synuclein Levels in a Neuronal Cell Model



Hoechst Phosphorylated-α-synuclein (S129)

Decrease in syn levels. Phosphorylated–synuclein (S129) detected by immunofluorescence in 3 differentiated neuronal cell lines (WT, N370S and L444P) treated for 15 days with 25 µM of the selected GT-1 chaperone. Between 30 and 40 individual cells for each condition were quantified (mean intensity in cytosol area). Results are presented as mean ± S.E.M. value.

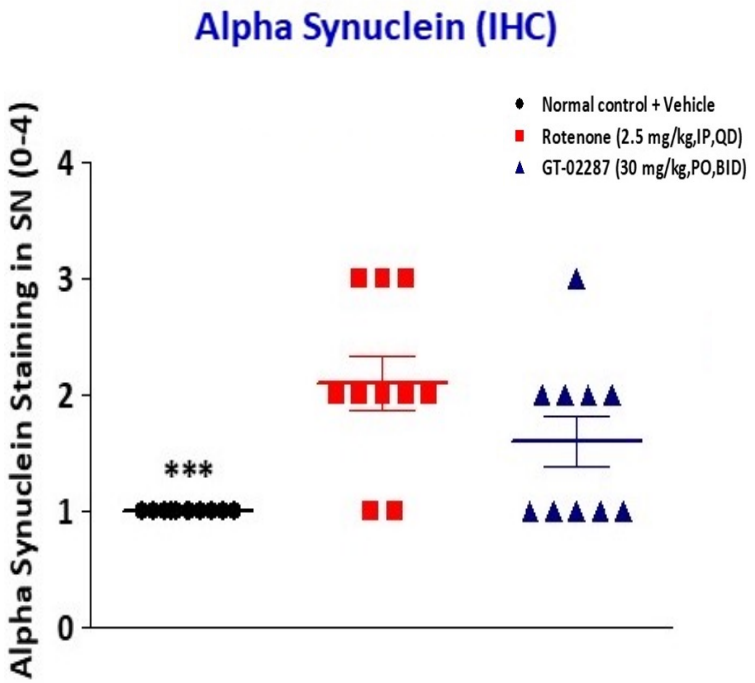
STARs Enhance WT GCase Activity in Plasma and Striatum



Statistical analysis was performed using GraphPad Prism software version 5.0. and used One way ANOVA followed by Dunnett's post-hoc test; All the values are expressed as Mean ± SEM. *P<0.05 Vs Vehicle control GT-02329 was administered orally, twice a day, for 12 days. Samples were collected 1 hr after the last administration. N=10 per group

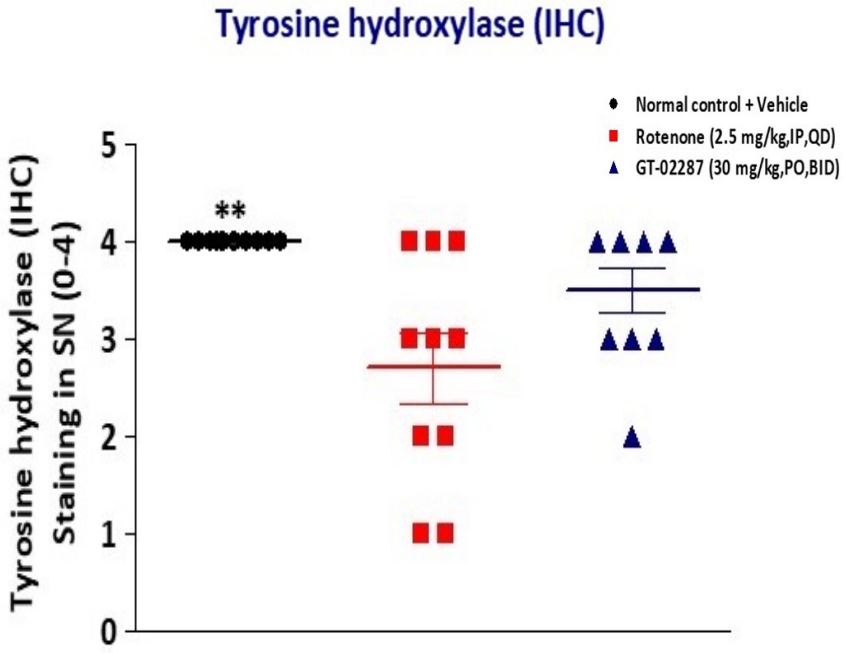
STARs Show Depletion of alpha-Synuclein, Increase in Tyrosine Hydroxylase and Improvement in Locomotor Activity and Markers in a Rotenone Rat Model

alpha-Synuclein Depletion



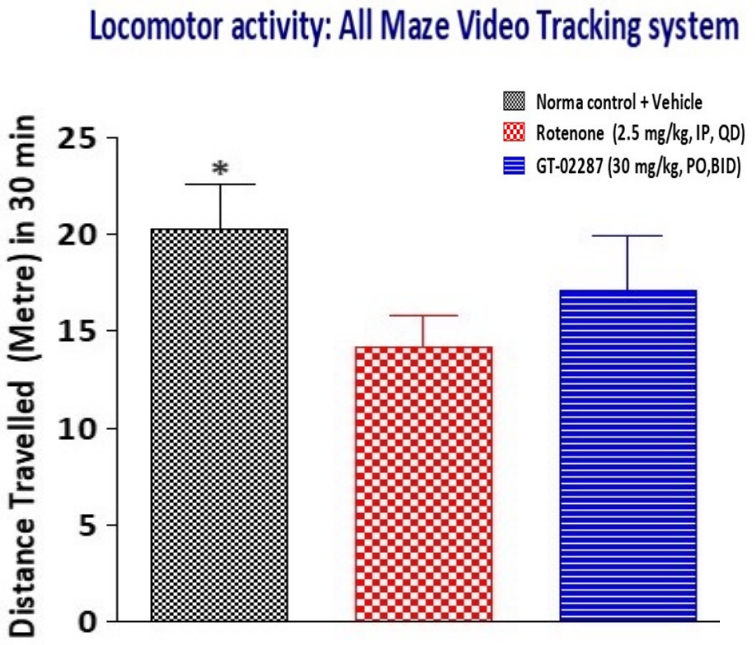
Data is shown as Mean ± S.E.M.(n=10), Ove-way ANOVA followed by Dunnett's Multiple Comparison test, *Significant difference as compared to Rotenone ***P<0.0001, Vs Vehicle control. GT-02287 was administered orally, twice a day, for 7 days.

Dopaminergic Neurons Activity Improvement



Data is shown as Mean ± S.E.M.(n=10), Ove-way ANOVA followed by Dunnett's Multiple Comparison test, *Significant difference as compared to Rotenone ***P<0.0001, Vs Vehicle control. GT-02287 was administered orally, twice a day, for 7 days

Improvement in Locomotor Activity



Data is shown as Mean ± S.E.M.(n=10). Unpaired T test. Significant difference as compared to Rotenone control *P < 0.05 P<0.05 Vs Vehicle control. GT-02287 was administered orally, twice a day, for 7 days.

Gaucher and Parkinson's Disease Program Summary

Lead Candidates

- Demonstration of mechanism of action indicative of specific binding and bypassing protein quality control of protein misfolding
- Demonstration of *in vitro of* target engagement as measured by enzyme enhancement and toxic substrates depletion
- Demonstration of *in* vivo target engagement as measured of GCase enhancement, toxic α-synuclein, glucosylceramide and glucosylsphingosine depletion, increase od dopaminergic neurons viability marker, decrease of locomotor deficits
- Orally bioavailable, brain penetrant

Upcoming milestones

- Gaucher and Parkinson animal models POC (2021)
- CD Characterization/Synthesis scale-up (2021)
- IND/CTA-enabling studies (2021-2022)
- GD: Phase 1/2: SAD/MAD (safety/PK) in pediatric population (2023-2024)
- PD: Phase 1: SAD/MAD (safety/PK) 60 healthy volunteers (2022 -2023)

GAIN THERAPEUTICS

Program to bring forward

Mucopolysaccharidosis Type I (MPS I)

alpha-L-iduronidase (IDUA)

IDUA Related Disorders: Mucopolysaccharidosis Type I (MPS I)

Mucopolysaccharidosis type I (MPS I) is an autosomal recessive inherited disorder of metabolism where large sugar molecules glycosaminoglycans (GAGs) cannot be adequately degraded

Mucopolysaccharidosis Type I

- Annually, there are approximately 1,400 patients with MPS I worldwide (1:100,000).[1]
- Mutations in the IDUA gene cause deficiency in the enzyme alpha-L-iduronidase, resulting in GAG build up within cells and lysosomes
- Symptoms include enlarged tonsils and/or adenoids, coarse facial features, abnormal curvature of the spine enlargement of the liver and spleen, compression of the spinal cord, and progressive neurological decline
- Disease progression poses a significant burden to patients and majority of progressive symptoms occur in hard-to-treat tissues such as bone and cartilage – areas Gain's STAR molecules can penetrate.

Current treatments are enzyme replacement therapy and stem cell transplant.
Significant unmet medical are related to hard-to-treat tissues such bone and cartilage

[1]US National Library of Medicine

MPS-I Program Summary

Status

Animal PoC

Druggable allosteric site identified

Test compound displays:

- Stabilization and prevention of enzyme denaturation in physiological conditions
- Enzymatic enhancement of ERT
- In vivo stabilization of recombinant IDUA (laronidase) including prolonged exposure in bone tissue and cartilage. Allows laronidase (Biomarin/Sanofi's drug) to reach good exposure levels in bone tissue and cartilage (makes it effective in those tissues)

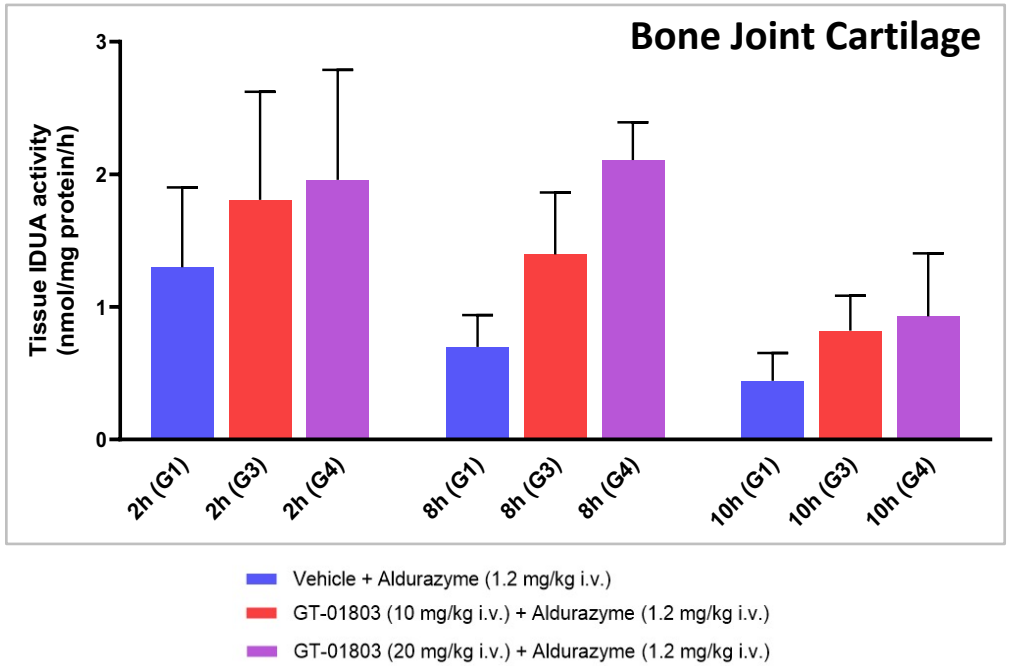
Intellectual Property (IP)

- PCT Patent filed (Feb. 2021)

Next Steps

Industry Collaboration

- *in vitro* efficacy study (2021)



Academic and Development Partners

Research and Development Grants

Patient Associations and Advocacy Groups

Academic Partnerships with Centers of Research, Translational Science & Clinical Excellence

Patent Estate

Patent	Status
Technology Platform Patent:	• WO2013092922 (Published June 2013) • National Phases granted
GLB1 Program:	• WO2018122746A1 (Published July 2018) • National Phases filed
GBA1 Program:	• WO2018122775 (Published July 2018) • National Phases filed • PCT application filed (to be published May 2021)
GALC Program:	• PCT application filed (to be published May 2021)
IDUA Program:	• Provisional patent (Filed February 2020) • PCT application to be filed (February 2021)

Use of Proceeds and Upcoming Milestones

2021

MorquioB / GM1 Gangliosidosis: Animal POCs – completion (Q3), IND-enabling studies – initiation (Q4)

Gaucher / Parkinson's Disease: Animal POC – completion (Q3), IND-enabling studies – initiation (Q4)

Krabbe: Lead Optimization & Characterization

2022

Morquio B / GM1 Gangliosidosis: IND filing

Gaucher / Parkinson's Disease: IND filing

Krabbe: Animal Proof of Concept

2023

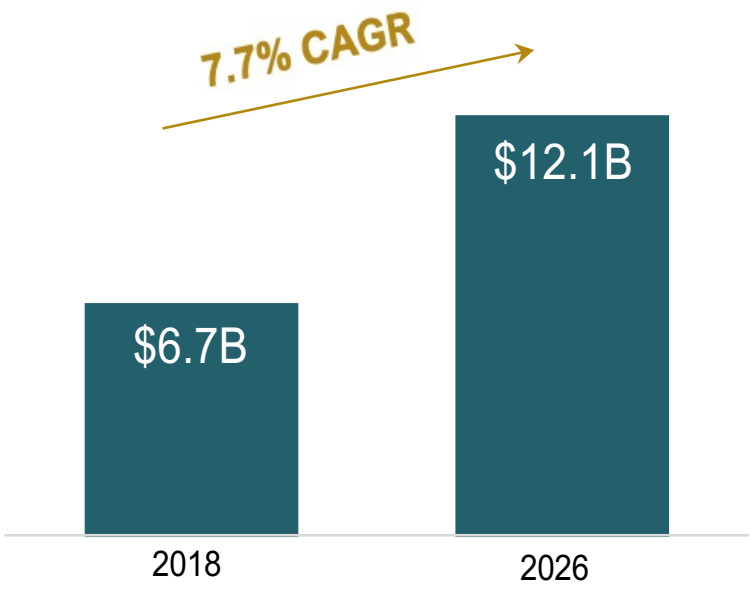
Morquio B / GM1 Gangliosidosis: Start of clinical studies

Gaucher / Parkinson's Disease: Start of clinical studies

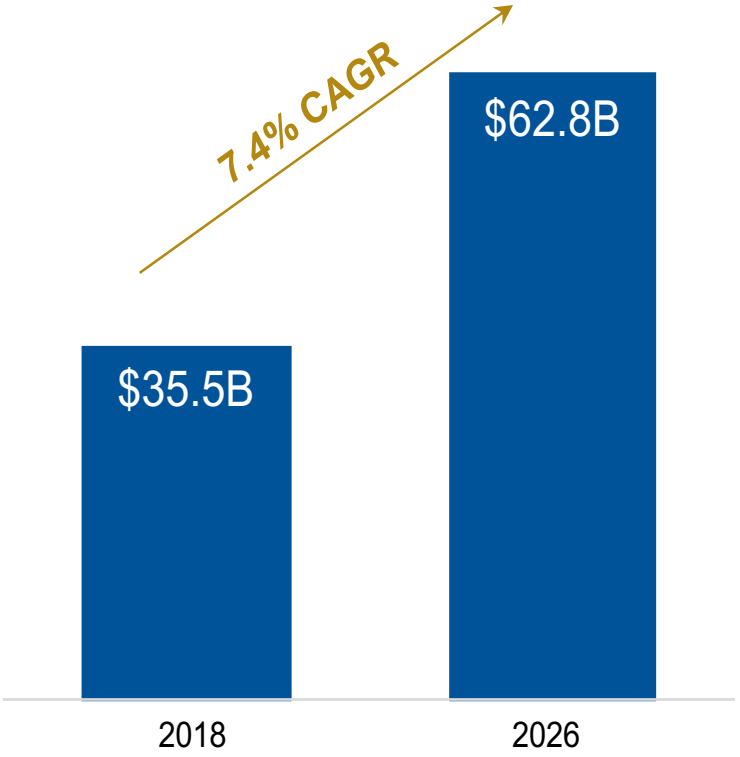
Krabbe: IND filing

Diversified Pipeline Targeting Multi-Billion Dollar Markets

Global ERT Sales**



Global Neurodegenerative Diseases Drug Market†



Enzyme Replacement Therapy (ERT)*

Takeda
SANOFI GENZYME
BIOMARIN
ultragenyx pharmaceutical

Gene Therapy, Chaperones, & Others*

Abeona THERAPEUTICS
Amicus Therapeutics
armagen
DENALI
azafaros
LTI
REGENXBIO
HotSpot Therapeutics
Prevail THERAPEUTICS
lysogene
ESCAPE BIO
Sangamo THERAPEUTICS
Orchard therapeutics
PassageBio
ORPHAZYME

* Data provided by S&P CapIQ and Company Websites as of October 22, 2020
** Grandview Market Research Report, Published Oct, 2019. "Enzyme Replacement Therapy Market Size, Share & Trends Analysis By Enzyme (Pancreatic Enzymes, Agalsidase Beta), By Therapeutic Condition (Gaucher, Fabry, Pompe, MPS), By Route of Administration, By End Use, And Segment Forecasts, 2019 – 2026"
† www.fortunebusinessinsights.com/industry-reports/neurodegenerative-diseases-drugs-market-100661; 2026 is estimated

Financing History and Stakeholders

Series A Institutional Investors and Collaborators:

Originally established in Switzerland in 2017 as Gain Therapeutics, SA with 16 employees in Lugano, Switzerland, Barcelona, Spain – HQ in Bethesda, Maryland

Cash & Cash Equivalents of $7.5m as of December 31st, 2020

Awarded funding support from The Michael J. Fox Foundation for Parkinson's Research and The Silverstein Foundation for Parkinson's with GBA

Completed Series B Financing of $10m in July 2020

Recently announced research collaboration with Sumitomo Dainippon Pharma Co

Strong Board of Directors

(>$30B in transactions)

Khalid Islam, PhD
Founder and Chairman

Immunomedics
minoryx therapeutics
ARPIDA
KAROLINSKA DEVELOPMENT
OxThera
Gentium
sanofi aventis
MOLMED
FENNEC PHARMA

Eric I. Richman
CEO and Member

MedImmune
BRACE PHARMA CAPITAL
PharmAthene
HealthCare VENTURES
ADMA BIOLOGICS

Gwen Melincoff
Independent Member

VERGE GENOMICS
PROTALIX Biotherapeutics
BTG
AGENT CAPITAL
Shire

Hans Peter Hassler
Independent Member

HBM Healthcare Investments
Biogen
MINERVA NEUROSCIENCES
SHIELD THERAPEUTICS PLC
Wyeth

Dov Goldstein, MD
Independent Member

indapta
LOXO ONCOLOGY
Vicuron Pharmaceuticals
AISLING CAPITAL
ADMA BIOLOGICS

Jeffrey Riley
Independent Member

Pfizer
Quest Diagnostics
SB SmithKline Beecham
Synthetic BIOLOGICS

Claude Nicaise, MD
Independent Member

ALEXION
Ovid THERAPEUTICS
SAREPTA THERAPEUTICS
Bristol Myers Squibb

Experienced Management Team

Extensive biotech and pharma experience (>$3B transactions)

Eric I. Richman
CEO

MedImmune
HealthCare VENTURES
BRACE PHARMA CAPITAL
PharmAthene

Manolo Bellotto, PhD
President & General Manager

DKSH
ESMO
HELSINN
NOVARTIS
VIFOR PHARMA

Salvatore Calabrese
CFO

Gentium
Jazz Pharmaceuticals
MOLMED
Novus Pharmaceuticals
pwc

Xavier Barril, PhD
CSO

ICREA
Universitat de Barcelona
Vernalis THERAPEUTICS

Roberto Maj, PharmD
Head of Development

AMAL Therapeutics
: inositec
Newron
Pharmacia &Upjohn
Telormedix

Ana Maria Garcia Collazo, PhD
Head of Research

cnio stop cancer
karo pharma
minoryx therapeutics

Diverse Group of Scientific Advisors

Joanne Taylor, PhD
Advisor

Eisai

Prescient

Samuel Broder, MD
Advisor

NIH NATIONAL CANCER INSTITUTE

CELERA
an Applera Corporation Business

intrexon

Michel Vellard, PhD
Advisor

ultragenyx pharmaceutical

BIOMARIN

Lorenzo Leoni, PhD
Advisor

ELION ONCOLOGY

Oculox Technologies

salmedix

Telormedix

TiVENTURE

Executive Summary

Therapeutic Focus	• Addressing Protein Misfolding by identifying and targeting novel previously undruggable allosteric binding sites • Initial focus on lysosomal storage and CNS diseases -- large & growing markets with unmet medical need
Platform Technology	• Proprietary and patented SEE-Tx platform identifies allosteric (non-catalytic) binding sites in proteins and enzymes that can regulate proper protein folding to restore functional activity
Robust Pipeline	• Demonstrated targeted engagement • Strong intellectual property estate-5 new NCE families patented
Global Partnerships	• Strong relations with academic centers, key patient associations, and development partners • Industrial partnership established with Sumitomo and potential to establish additional partnerships
Team	• Seasoned and experienced management team, board of directors and scientific advisors
Upcoming Milestones	• Near term milestones: several development candidates currently completing animal POC • IND enabling studies starting 2021