Sangamo THERAPEUTICS

Corporate Presentation

August 5, 2020

Forward-Looking Statements

This presentation contains forward-looking statements regarding Sangamo's current expectations. These forward-looking statements include, without limitation, statements relating to the potential to develop, obtain regulatory approvals for and commercialize safe and effective therapies to treat certain diseases and the timing, availability and costs of such therapies, the potential to use ZFP, ZFP-TF, ZFN, CAR-Treg, iPSC, ESC and other technologies to develop safe and effective therapies, the potential for Sangamo to benefit and earn milestone and royalty payments from its collaborations and the timing of such benefits and payments, Sangamo's financial resources and expectations, the evolving COVID-19 pandemic and the impact of the pandemic on Sangamo's business and operations, plans and timelines for manufacturing product candidates and opening manufacturing facilities, plans and timelines for Sangamo and our collaborators to conduct clinical trials and share clinical data, Sangamo's 2020 financial guidance related to GAAP and non-GAAP total operating expenses and stock-based compensation and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to certain risks and uncertainties that are difficult to predict. Sangamo's actual results may differ materially and adversely from those expressed. Factors that could cause actual results to differ include, without limitation, risks and uncertainties related to the evolving COVID-19 pandemic and its impact on the global business environment, healthcare systems and Sangamo's business and operations; the research and development process; the uncertain timing and unpredictable results of clinical trials, including whether initial clinical trial data will be representative of final clinical trial data and whether final clinical trial data will validate the safety and efficacy of product candidates; the unpredictable regulatory approval process for product candidates across multiple regulatory authorities; the manufacturing of products and product candidates; the commercialization of approved products; the potential for technological developments that obviate technologies used by Sangamo; the potential for collaborators to breach or terminate collaboration agreements; the potential for Sangamo to fail to realize its expected benefits of its collaborations; and the uncertainty of Sangamo's future capital requirements, financial performance and results. There can be no assurance that Sangamo and its collaborators will be able to develop commercially viable products. These risks and uncertainties are described more fully in Sangamo's Quarterly Report on Form 10-Q for the quarter ended June 30, 2020 and Annual Report on Form 10-K. Forward-looking statements contained in this presentation speak only as of the date hereof, and Sangamo undertakes no duty to update such information except as required under applicable law. This presentation concerns investigational product candidates that are under preclinical and/or clinical investigation and which have not yet been approved for marketing by any regulatory agency. They are currently limited to investigational use, and no representations are made as to their safety or efficacy for the purposes for which they are being investigated. Any discussions of safety or efficacy are only in reference to the specific results presented here and may not be indicative of an ultimate finding of safety or efficacy by regulatory agencies.

To supplement Sangamo's financial results and guidance presented in accordance with GAAP, Sangamo presents non-GAAP total operating expenses, which exclude stock-based compensation expense from GAAP total operating expenses. Sangamo believes that this non-GAAP financial measure, when considered together with its financial information prepared in accordance with GAAP, can enhance investors' and analysts' ability to meaningfully compare Sangamo's results from period to period and to its forward-looking guidance, and to identify operating trends in Sangamo's business. Sangamo has excluded stock-based compensation expense because it is a non-cash expense that may vary significantly from period to period as a result of changes not directly or immediately related to the operational performance for the periods presented. This non-GAAP financial measure is in addition to, not a substitute for, or superior to, measures of financial performance prepared in accordance with GAAP. Sangamo encourages investors to carefully consider its results under GAAP, as well as its supplemental non-GAAP financial information, to more fully understand Sangamo's business.

We are committed to translating ground-breaking science into genomic medicines that transform patients' lives

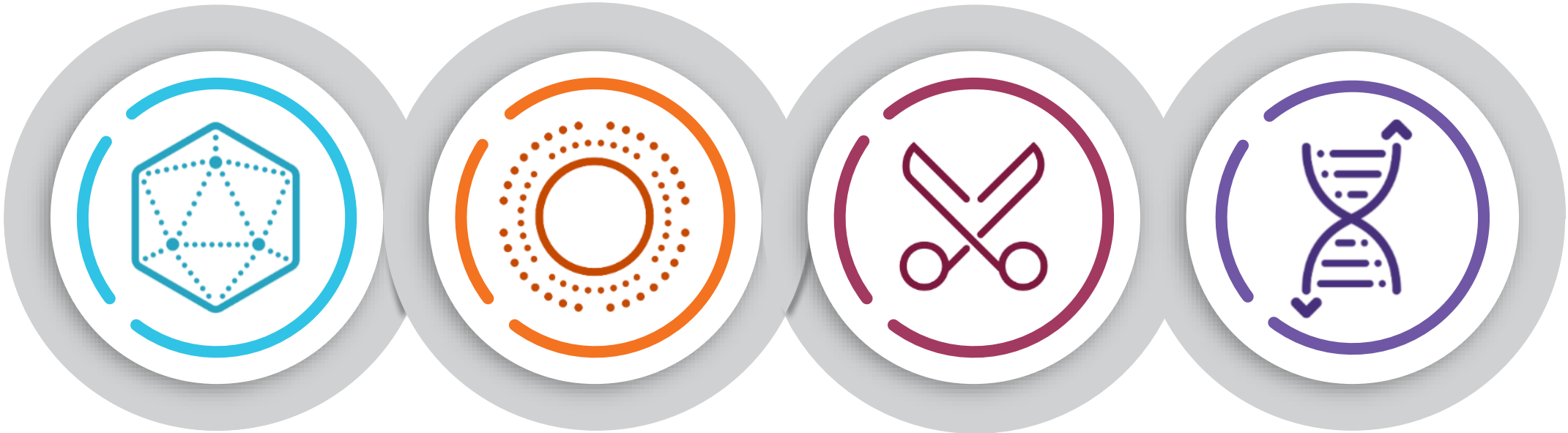
Our proprietary suite of genomic medicine technologies

Gene Therapy
AAV

Gene-Edited Cell Therapy
AAV/LV; ZFN

Genome Editing
AAV; ZFN

Genome Regulation
AAV; ZFP-TF

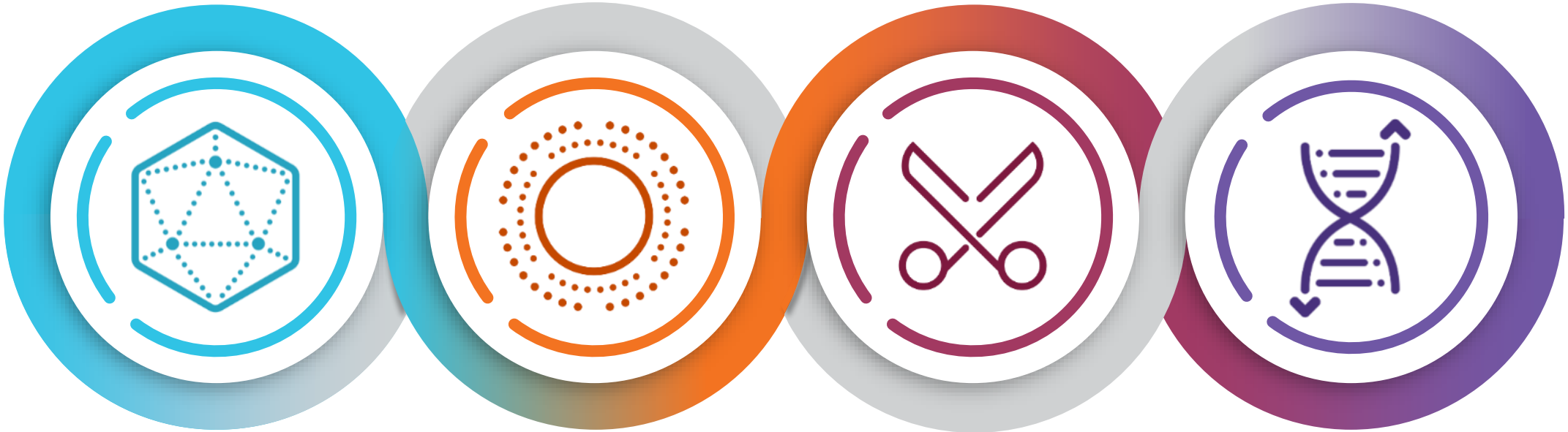


Gene therapy provides tractable, valuable near-term opportunities

Continue to advance *ex vivo* editing to create cell therapies

Sustain momentum with *in vivo* genome editing and genome regulation

Our capabilities allow us to design therapeutic approaches targeting the underlying genetic causes of disease



Gene Therapy
AAV

SB-525 / PF-07055480: Hemophilia A
ST-920: Fabry disease
ST-101: PKU

Gene-Edited Cell Therapy
AAV/LV; ZFN

ST-400: Beta thalassemia
BIVV003: Sickle cell disease
TX200: Solid organ transplant
KITE-037: Allo-CD19 CAR-T
Undisclosed targets

Genome Editing
AAV; ZFN

SB-913: MPS II

Genome Regulation
AAV; ZFP-TF

ST-501: Tauopathies
ST-502: α-synuclein
C9ORF72-linked ALS/FTLD
TAK-686: Huntington's disease
Prion
Neurodevelopmental disorders
Undisclosed targets

Sangamo THERAPEUTICS

Robust pipeline of genomic medicines

Preclinical

- PKU (ST-101) — Sangamo
- IBD — Sangamo
- MS — Sangamo
- Oncology (Undisclosed) — PARTNER Kite
- Oncology (KITE-037) — PARTNER Kite
- Solid Organ Transplant (TX200) — Sangamo
- ALS/FTD — PARTNER Pfizer
- Huntington's Disease (TAK-686) — PARTNER Takeda
- α-Synuclein (ST-502) — PARTNER Biogen
- Tauopathies (ST-501) — PARTNER Biogen
- Neurology (Undisclosed) — PARTNER Biogen
- Prion — Sangamo
- Neuro-developmental disorders — PARTNER NOVARTIS

Phase 1/2

- Fabry Disease (ST-920) — Sangamo
- Beta Thalassemia (ST-400) — PARTNER SANOFI
- Sickle Cell Disease (BIVV003) — PARTNER SANOFI
- MPS II (SB-913) — Sangamo

Phase 3

- Hemophilia A (giroctocogene fitelparvovec) — PARTNER Pfizer

Gene Therapy | *Ex Vivo* Gene-Edited Cell Therapy | *In Vivo* Genome Editing | *In Vivo* Genome Regulation

Sangamo THERAPEUTICS

Novartis collaboration for neurodevelopmental disorders

- Strategically partners Sangamo's proprietary genome regulation technology with Novartis' deep experience pioneering treatments in neurodevelopment.
- Leverages Sangamo's zinc finger protein transcription factors (ZFP-TFs) in an effort to upregulate, or activate, expression of genes that are inadequately expressed with certain neurodevelopmental disorders.
- Aims to develop and commercialize gene regulation therapies to address three neurodevelopmental targets, including genes linked to autism spectrum disorder (ASD) and other neurodevelopmental targets.

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NOVARTIS

Novartis collaboration scope and responsibilities

- Over a three-year period, Novartis has exclusive rights to ZFP-TFs targeted to three undisclosed genes associated with neurodevelopmental disorders, including ASD and intellectual disability.
- Novartis has the option to license Sangamo's proprietary AAVs.
- Sangamo is responsible for certain research and associated manufacturing activities, all of which will be funded by Novartis.
- Novartis will assume responsibility for additional research activities, IND-enabling studies, clinical development, related regulatory interactions, manufacturing, and global commercialization.

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NOVARTIS

Novartis collaboration financial summary

Upfront	$75M	Upfront license payment
Milestones	$720M	$420M – development milestones $300M – commercial milestones
Royalties	Net sales %	High single to sub-teen double digits
R&D	Funding	Novartis will fund certain research and manufacturing activities by Sangamo, and be responsible for further research and commercialization activities

Sangamo THERAPEUTICS | NOVARTIS

Increasing productivity and realizing value through biopharmaceutical partnerships

	Biogen	GILEAD	NOVARTIS	Pfizer	Pfizer	SANOFI	Takeda
Target/ therapeutic area	Neurological including AD, PD	Oncology anti-CD19 CAR-T	Neurodevelopmental disorders	C9ORF72 ALS	Hemophilia A	Beta thalassemia, Sickle Cell disease	Huntington's disease
Development phase	Preclinical	Preclinical	Preclinical	Preclinical	Phase 3	Phase 1/2	Preclinical
Technology	Genome regulation	Cell therapy	Genome regulation	Genome regulation	Gene therapy	Cell therapy	Genome regulation
Royalties (% on net sales)	High-single to sub-teen double-digit	Single-digit	High-single to sub-teen double-digit	Mid- to high-single digit	Low teens to 20	Double-digit	Single-digit
Upfront & equity	$125M payment + $225M in equity purchase	$150M payment + $50M in equity purchase	$75M payment	$12M	$70M	$20M	$13M
Milestones	Up to $2.37B ($925M pre-commercial)	Up to $3.01B ($1.3B through 1st sale)	Up to $720M ($420M in development and $300M in commercial)	Up to $150M preclinical and commercial	Up to $475M ($300M for SB-525 and $175M other)	Up to $276M for both programs	-

Cash received through license fees, milestones, and equity: **~$780 million**
Future opportunity: **Royalties** on net product sales, as well as ~**$7 billion** in potential milestone payments

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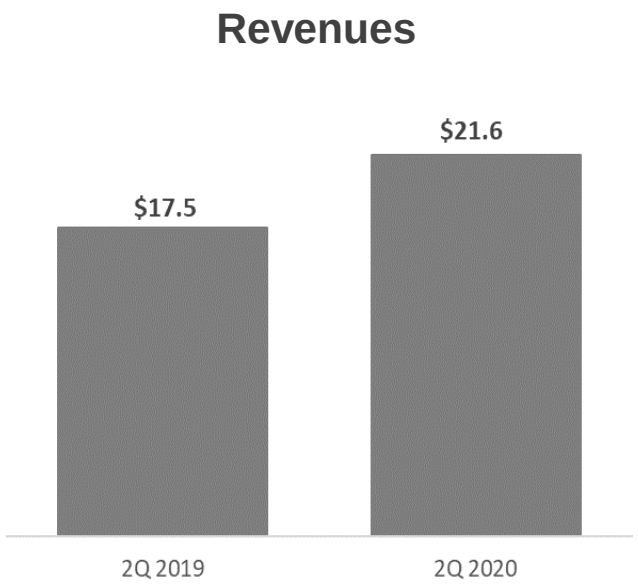
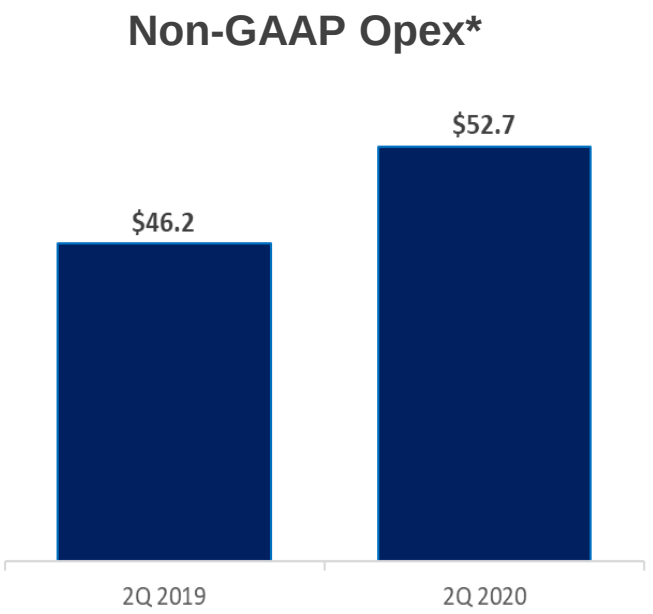
2Q 2020 Financial Results

2020 guidance revisions driven by COVID-19 and its impact on clinical programs

$ in Millions





2020 Non-GAAP Opex**

$210M-$225M

* GAAP total operating expenses were $59.4 million for 2Q 2020, compared to $51.1 million for 2Q 2019 and included stock-based compensation expense (“SBC”) of $6.7 million and $4.9 million respectively

** On a GAAP basis we expect our 2020 operating expense to be in the range of $235 - $250 million including anticipated SBC of $25 million

Sangamo THERAPEUTICS

Clinical and preclinical pipeline

Gene Therapy
Cell Therapy
Genome Editing

Gene therapy in 2020: Building on hemophilia A data

Preclinical

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Phase 3

- Hemophilia A (giroctocogene fitelparvovec) — PARTNER Pfizer

Gene Therapy | *Ex Vivo* Gene-Edited Cell Therapy | *In Vivo* Genome Editing | *In Vivo* Genome Regulation

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Updated Follow-up of the High-Dose Cohort in the Alta Study, a Phase 1/2 Study of giroctocogene fitelparvovec (SB-525) Gene Therapy in Adults With Severe Hemophilia A

Thomas J. Harrington, MD[1]; Barbara A. Konkle, MD[2]; Kimo Stine, MD[3]; Nathan Visweshwar, MD[4]; Andrew D. Leavitt, MD[5]; Adam Giermasz, MD, PhD[6]; Steven Arkin, MD[7]; Annie Fang, MD, PhD[8]; Li-Jung Tseng, MBA, PhD[8]; Gregory Di Russo, MD[7]; Bettina M.Cockroft, MD, MBA[9]; Adrian Woolfson, MD, PhD[9]; Jeremy Rupon, MD, PhD[10]; Didier Rouy, MD, PhD[9]

[1]University of Miami Miller School of Medicine, Miami, FL, USA; [2]Bloodworks Northwest and the University of Washington, Seattle, WA, USA; [3]UAMS at Arkansas Children's Hospital, Little Rock, AR, USA; [4]University of South Florida, Tampa, FL, USA; [5]University of California, San Francisco, CA; [6]University of California Davis, Sacramento, CA, USA; [7]Pfizer Inc, Cambridge, MA, USA; [8]Pfizer Inc, New York, NY, USA; [9]Sangamo Therapeutics, Brisbane, CA, USA; [10]Pfizer Inc, Collegeville, PA, USA

Presented at the World Federation of Hemophilia (WFH) Virtual Summit, June 14–19, 2020

Safety Summary: Treatment-Related Adverse Events

MedDRA Preferred Term	Cohort 4 3e13 vg/kg (N=5)	
	Subjects, n (%)	No. of Events
Any treatment-related event	5 (100.0)	42
Alanine aminotransferase increased*	3 (60.0)	9
Pyrexia	4 (80.0)	4
Aspartate aminotransferase increased	1 (20.0)	2
Tachycardia	2 (40.0)	2
Fatigue	1 (20.0)	1
Hypotension	1 (20.0)	1
Myalgia	1 (20.0)	1

*Subject 113001 had an ALT increase as per central lab results, but Investigator has not reported increase as an Adverse Event

ALT Elevations: Cohort 4 ($3x10^{13}$ vg/kg)

- 4 of 5 subjects in cohort 4 had an ALT elevation

Subject ID Number	Time of First ALT Elevation (Week)	Maximum ALT Value, U/L (Grade)	Steroids, >60mg (Weeks)	Steroids, Taper (Weeks)	FVIII levels (Chromo, IU/dL) at Start of Steroids	FVIII Levels (Chromo, IU/dl) at End of Taper	Time of Second ALT Elevation (Week)	Weeks of Steroids After Second Elevation
7	4.5	91 (gr 1)	3	11	94.8	108.2	48#	16#
8	12	66 (gr 1)	1	16	83.1	112.6	N/A	N/A
10	5.5	63 (gr 1)	N/A*	6	46.4	57.1	20	9
11	8	192 (gr 2)	1.5	4	80.2	27.7	16	18

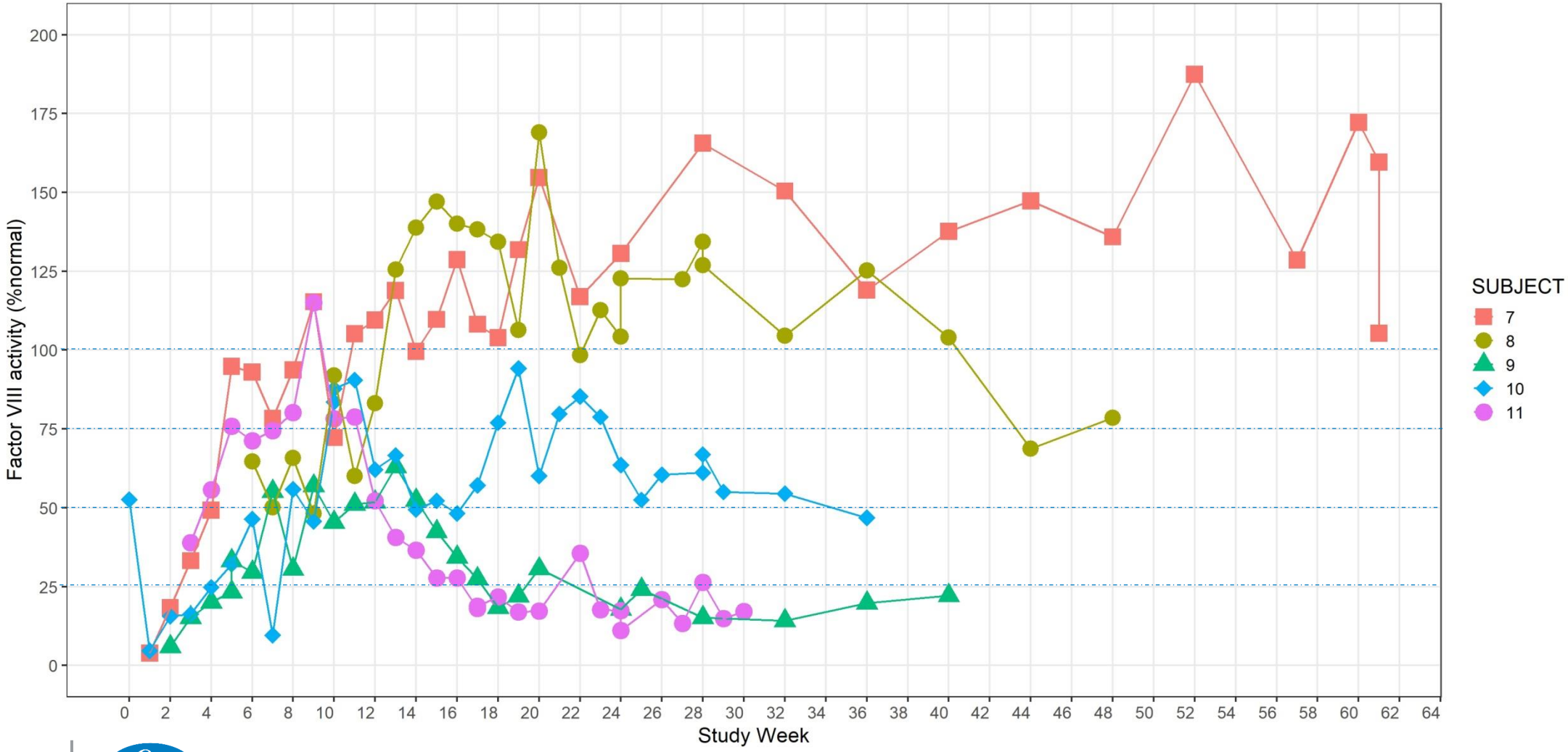
*: Patient started at 60mg.
#: Patient had an additional isolated elevation of ALT at week 28 that was treated with corticosteroids for 1 week and discontinued. Treatment was ongoing at the time of data cut.

Sangamo THERAPEUTICS | Pfizer

Data cut March 2020

Efficacy: Cohort 4 ($3x10^{13}$ vg/kg)

FVIII Activity as measured at Central Laboratory with Chromogenic Assay



Data cut March 2020

giroctocogene fitelparvovec (SB-525 / PF-07055480) program transitioned to Pfizer for Phase 3 development

- Pfizer continues to target dosing of the first patient in the pivotal Phase 3 in 2H 2020
  - Objective: To evaluate the clinical efficacy and safety of a single IV infusion of PF-07055480 in eligible patients who have completed at least 6 months in prior lead-in study
- Enrollment in Pfizer's Phase 3 lead-in study commenced in October and is ongoing
  - Objective: To establish ≥ 6 months of prospective efficacy data of current FVIII prophylaxis replacement therapy in the usual care setting of hemophilia A subjects, who are negative for nAb to SB-525 capsid (AAV6), prior to the Phase 3 gene therapy study

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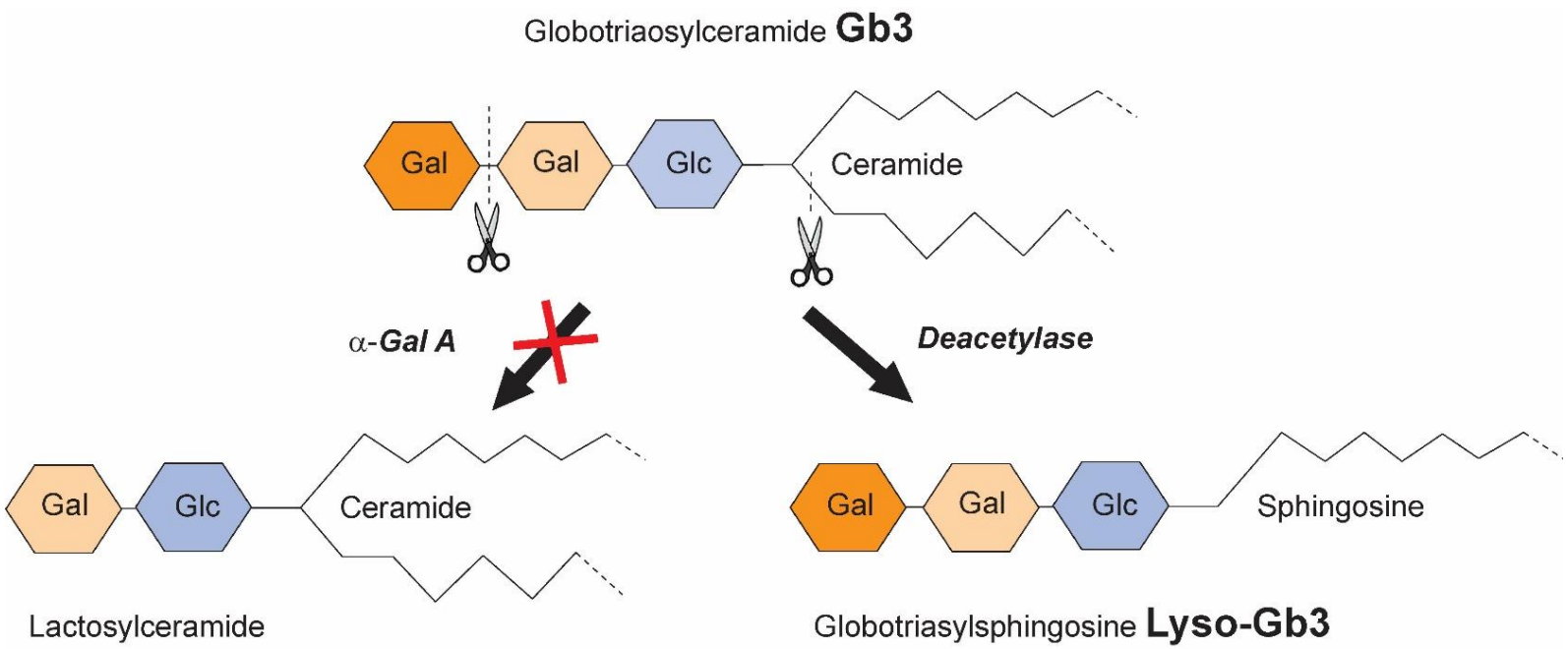
Pfizer

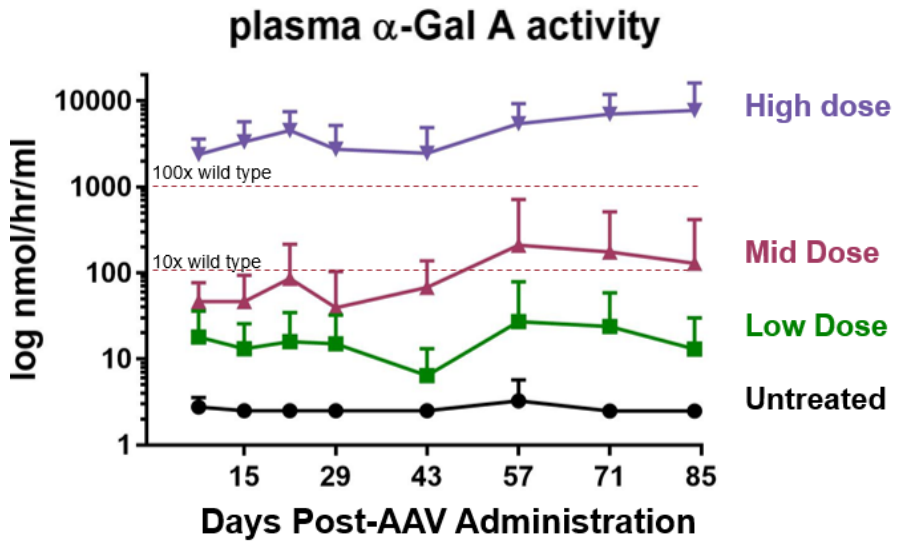
Fabry disease: A lysosomal storage disorder

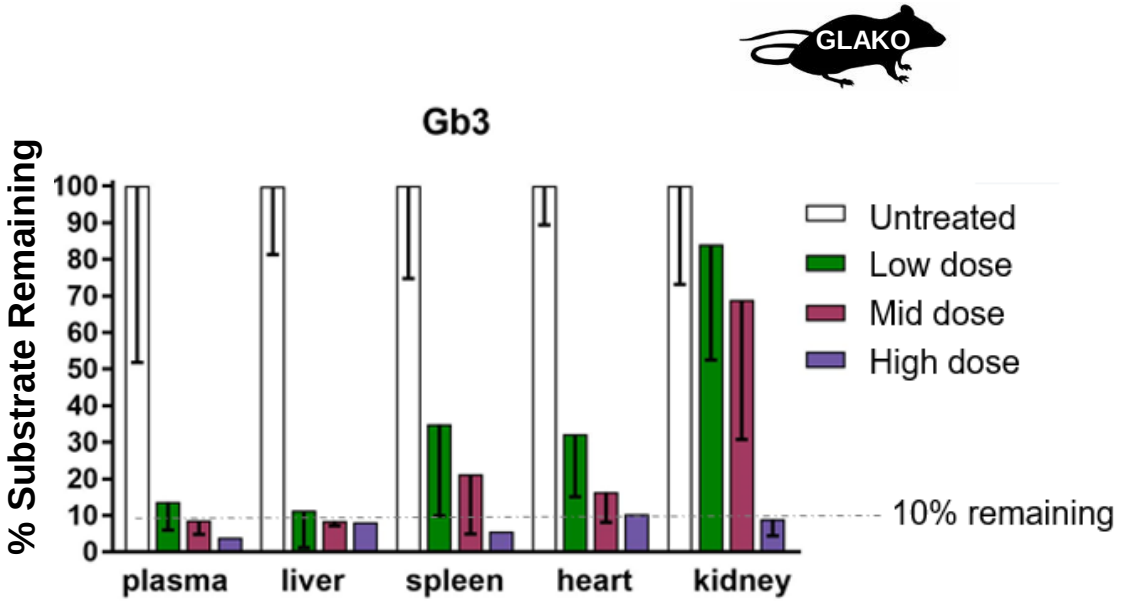


- Fabry disease is an **X-linked** monogenic disease caused by mutations in *GLA* gene encoding the enzyme alpha-galactosidase A **(α-Gal A**)
- **α-Gal A** plays a role in degradation of glycolipids in the lysosome
- The lack of functioning enzyme results in the accumulation of **Gb3** and its soluble form **lyso-Gb3**.

ST-920 promising preclinical data

- ✓ US FDA and EMA orphan drug designation granted
- ✓ AAV produced using clinical scale manufacturing methods





Sangamo's gene therapy demonstrated strong expression of α-Gal A and Gb3 substrate reduction across tissue types in GLAKO murine model

Phase 1/2 study evaluating ST-920

Primary Objective

- Assess safety & tolerability of ST-920

Secondary Objectives

- Assess the pharmacodynamics of α-Gal A and the presence of its substrates in plasma over time
- Assess impact of ST-920 on ERT administration required for subjects on ERT
- Assess impact of ST-920 on renal function
- Evaluate AAV2/6 vector DNA shedding over time

Target Population

- Male subjects ≥ 18 years of age with classic Fabry disease
- On ERT regimen; or ERT-naïve; or ERT-pseudo-naïve and has not received ERT treatment in the prior 6 months

The goal is to abrogate the need for ERT with a recombinant AAV2/6 vector encoding cDNA for human α-Gal A, resulting in long-term expression of α-Gal A

ST-920 offers a potentially differentiated treatment for Fabry

- In a single IV infusion, ST-920 may provide continuous, potentially life-long expression of endogenously expressed α-Gal A
- No preconditioning regimen
- Potential to deliver efficacy with preserved renal function and reduced cardiac morbidity
- FDA draft guidance may considerably shorten time to approval and allow ST-920 to be among the first Fabry disease gene therapies on the market

Ex vivo gene-edited cell therapy in 2020

Gene Therapy | *Ex Vivo* Gene-Edited Cell Therapy | *In Vivo* Genome Editing | *In Vivo* Genome Regulation

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Presented at the 2019 American Society of Hematology Annual Meeting; Orlando, Florida; December 9, 2019

Preliminary Results of a Phase 1/2 Clinical Study of Zinc Finger Nuclease-Mediated Editing of *BCL11A* in Autologous Hematopoietic Stem Cells for Transfusion-Dependent β-Thalassemia

Angela R. Smith, MD, MS[1]; Gary J. Schiller, MD[2]; Gregory M Vercellotti, MD[3]; Janet L. Kwiatkowski, MD, MSCE[4]; Lakshmanan Krishnamurti, MD[5]; Erica B. Esrick, MD[6]; David A. Williams, MD[7]; Weston P. Miller, MD[8]; Adrian Woolfson, MD, PhD[8] and Mark C. Walters, MD[9]

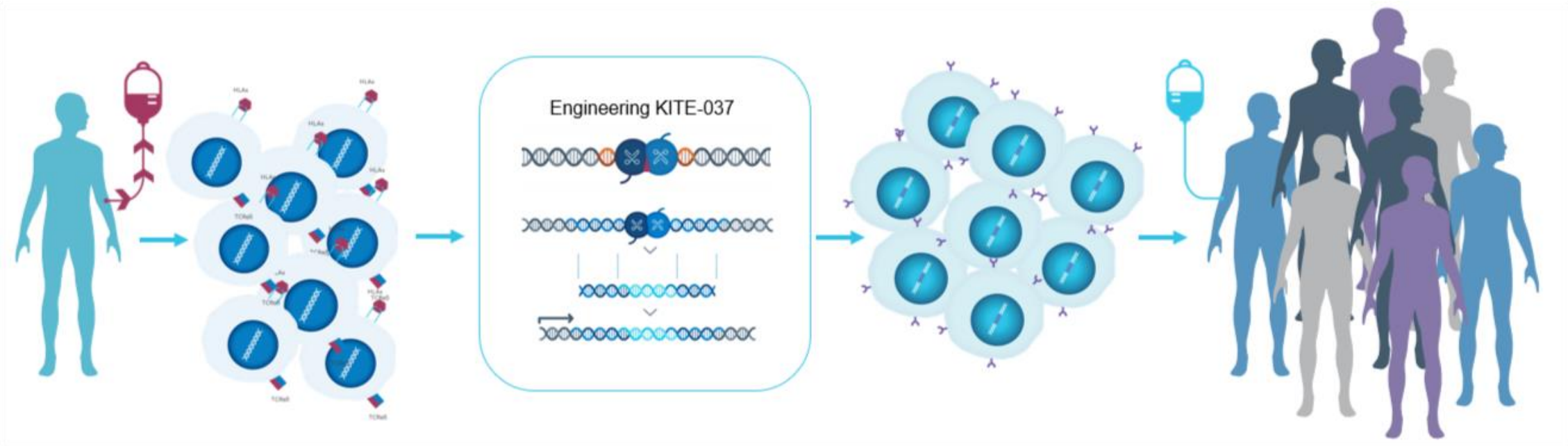
[1]Pediatric Blood and Marrow Transplantation, University of Minnesota, Minneapolis, MN; [2]Division of Hematology and Oncology, Department of Medicine, David Geffen School of Medicine at University of California Los Angeles, Los Angeles, CA; [3]Division of Hematology, Oncology and Transplantation, Department of Medicine, University of Minnesota Medical School, Minneapolis, MN; [4]The Children's Hospital of Philadelphia, Philadelphia, PA; [5]Aflac Cancer and Blood Disorders Center, Department of Pediatrics, Children's Healthcare of Atlanta, Emory University, Atlanta, GA; [6]Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Harvard Medical School, Boston, MA; [7]Boston Children's Hospital, Harvard Medical School, Boston, MA; [8]Sangamo Therapeutics, Brisbane, CA; [9]USCF Benioff Children's Hospital Oakland, Oakland, CA

Hemoglobinopathies collaboration with Sanofi: BIVV003 for Sickle Cell Disease and ST-400 for Beta Thalassemia

- Sanofi continuing to screen/enroll subjects into the PRECIZN-1 clinical trial evaluating BIVV003 for sickle cell disease
- Five subjects dosed in Sangamo's Thales study evaluating ST-400 for transfusion dependent beta thalassemia
  - No additional beta thalassemia subjects to be treated until data from PRECIZN-1 and Thales have been collected and analyzed
- Sangamo and Sanofi will look to present BIVV003 and ST-400 data once sufficient number of patients have been enrolled and follow-up has accumulated

Sangamo THERAPEUTICS + SANOFI

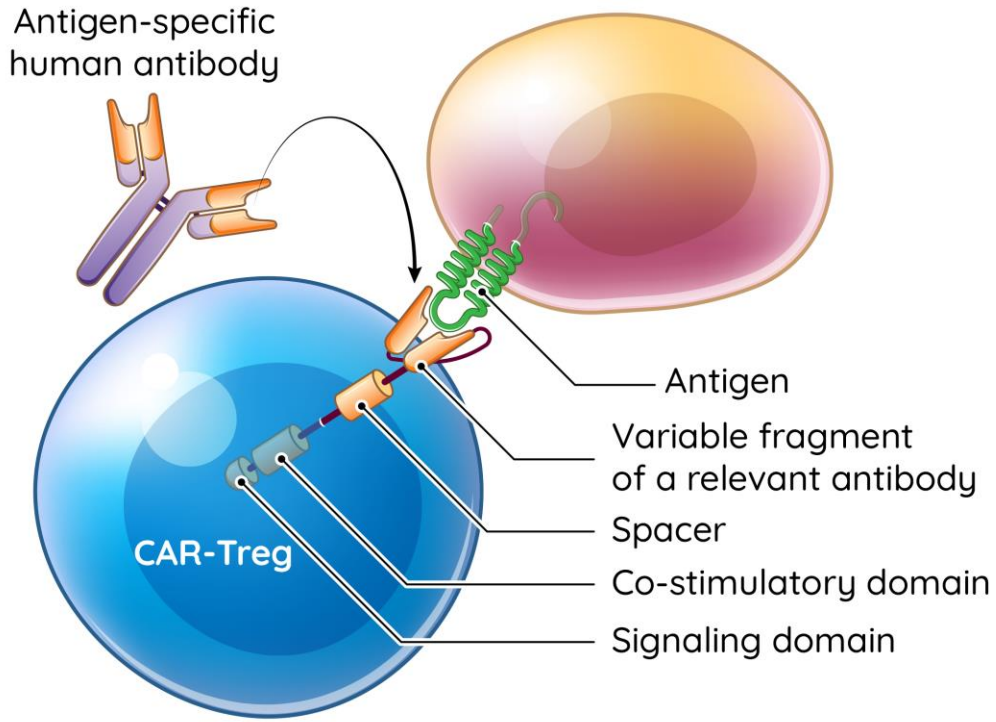
KITE-037, the first product candidate of the collaboration with Kite, a Gilead Company



- First product candidate: KITE-037, an allogeneic anti-CD19 CAR-T
- KITE-037 clinical trial planned for 2020 potentially delayed due to COVID-19

Harnessing T_{REG} Function with CAR-T_{REG} Therapy

CAR-T_{REGS} may overcome limitations of polyclonal T_{REG} Therapy



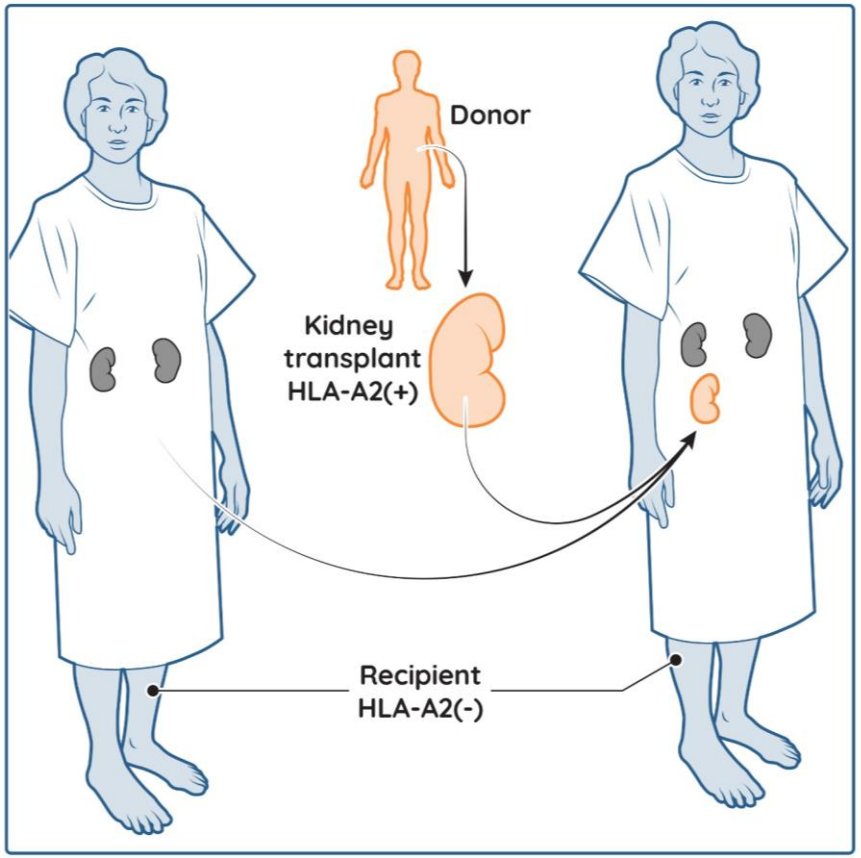
- *Ex vivo* engineered and expanded
- Tissue targeted
- Antigen activated & expanded
- Multiple mechanisms of immune regulation

Sangamo THERAPEUTICS

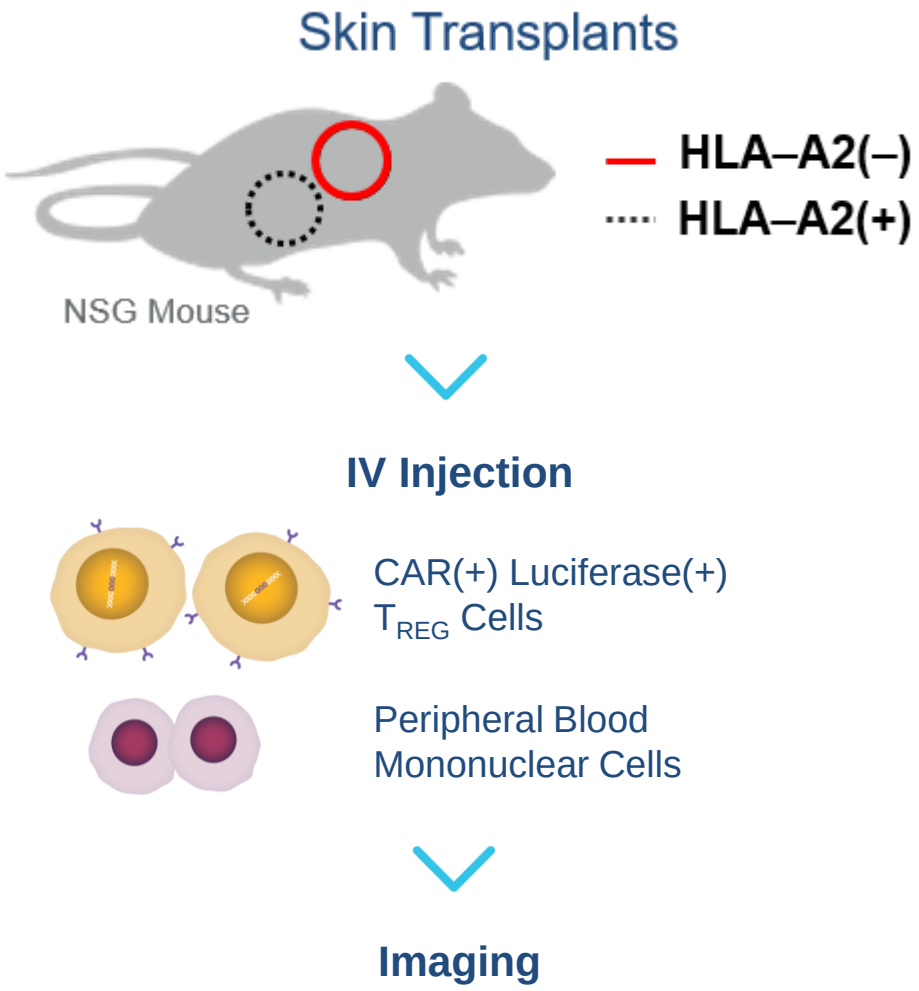
Sangamo is pioneering this new frontier with TX200 for solid organ transplantation

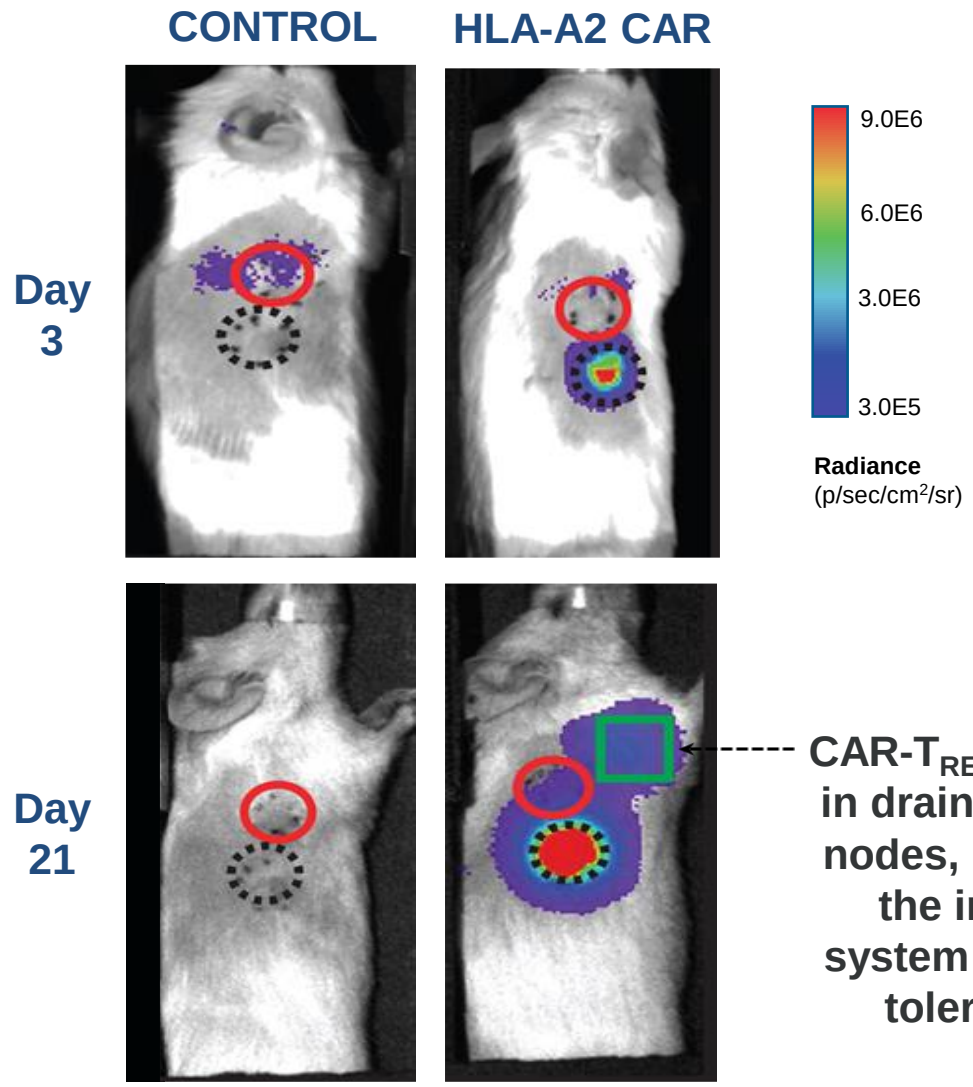
Autologous HLA-A2 specific CAR-T_{REG} cell therapy

- Initial target indication: Prevention of immune mediated rejection in living donor renal transplantation
  - The STEADFAST Study will evaluate the safety and mechanism of action of TX200 in renal transplant recipients
  - 80,000 renal transplantations per year (US and EU)
  - 20-25% of transplanted organs are HLA-A2 mismatched
- Therapeutic hypothesis and goals
  - Regulate the immune system in a targeted manner
  - Promote immunological tolerance to the renal transplant
  - Help preserve graft function and reduce graft loss



HLA-A2 CAR-T_{REGS} achieve precise and durable targeting of skin graft in a mouse model





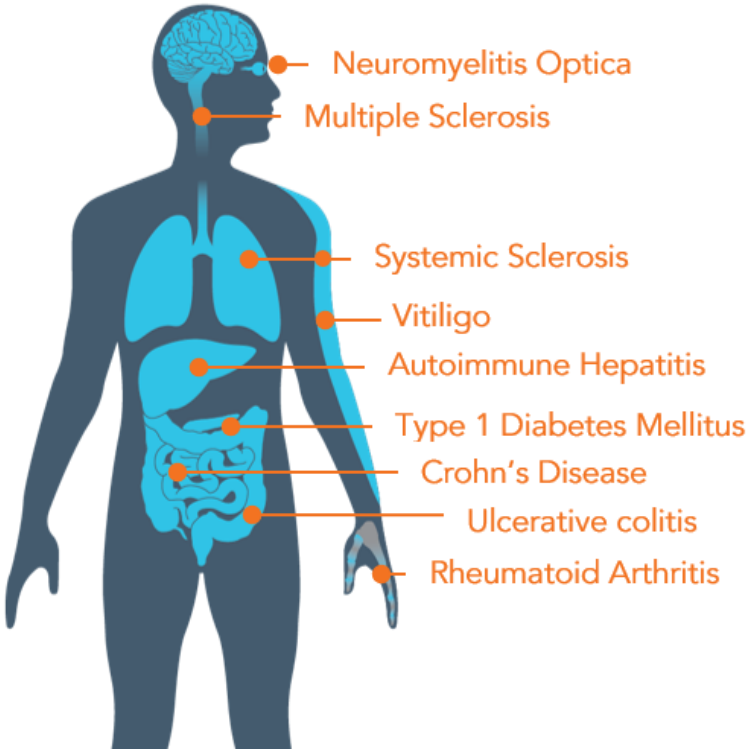
CAR-T_{REGS} present in draining lymph nodes, educating the immune system to induce tolerization

Dawson NA, Lamarche C et al. JCI Insight 2019;4(6):e123672.

TX200: Gateway to major autoimmune indications

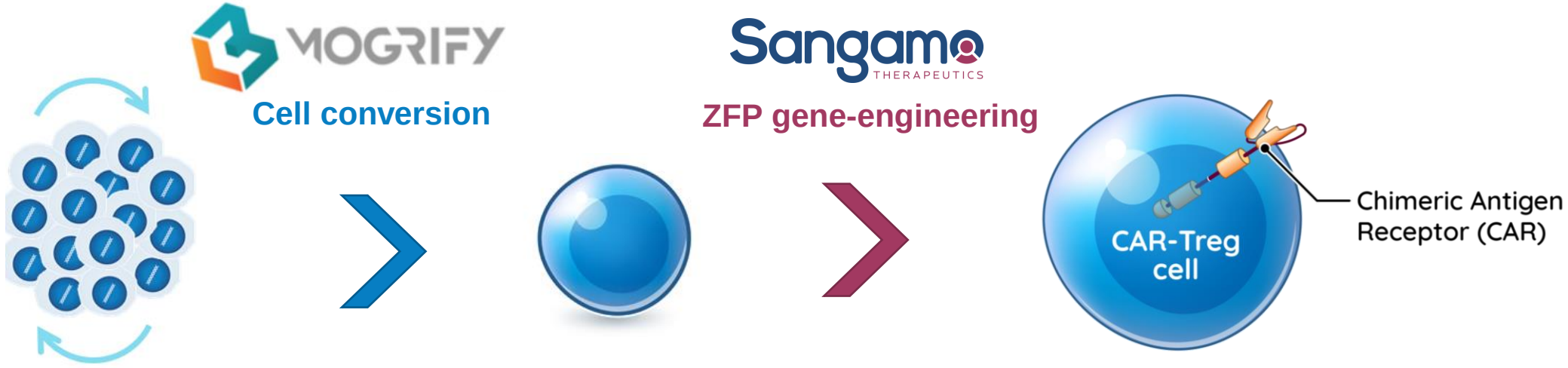
Key outcomes from TX200 CAR-T_{REG} program

- Data on safety and proof of concept
- Answers critical questions on CAR-T_{REG} pharmacology and biology in patients
- Establishes CAR-T_{REG} cell therapy process development know-how and manufacturing infrastructure
- Gateway to multiple autoimmune indications with large patient populations and high unmet need



Sangamo licenses global rights to Mogrify's cell conversion technology to develop allogeneic cell therapies

Mogrify's technology enables the transformation of any human cell type into any other human cell type



Induced pluripotent stem cell (IPSC)

Regulatory T cell (T_{REG})

Renewable cell source allogeneic CAR-T_{REG}

- Collaboration expected to accelerate the development of scalable and accessible CAR-T_{REG} cell therapies for the treatment of inflammatory and autoimmune diseases
- Complements ongoing cell therapy pipeline of CAR-T_{REG} programs

Applying Sangamo's technology to neurological diseases

Genome Regulation

In vivo genome regulation for neurological diseases

Preclinical					Phase 1/2		Phase 3
PKU (ST-101)	IBD	MS	Oncology (Undisclosed)	Oncology (KITE-037)	Fabry Disease (ST-920)	Beta Thalassemia (ST-400)	Hemophilia A (giroctocogene fitelparvovec)
Sangamo	Sangamo	Sangamo	PARTNER Kite	PARTNER Kite	Sangamo	PARTNER SANOFI	PARTNER Pfizer
Solid Organ Transplant (TX200)	ALS/FTD	Huntington's Disease (TAK-686)	α-Synuclein (ST-502)	Tauopathies (ST-501)	Sickle Cell Disease (BIVV003)	MPS II (SB-913)	
Sangamo	PARTNER Pfizer	PARTNER Takeda	PARTNER Biogen	PARTNER Biogen	PARTNER SANOFI	Sangamo	
Neurology (Undisclosed)	Prion	Neuro-developmental disorders					
PARTNER Biogen	Sangamo	PARTNER NOVARTIS					

Gene Therapy | *Ex Vivo* Gene-Edited Cell Therapy | *In Vivo* Genome Editing | *In Vivo* Genome Regulation

Sangamo ZFP technology: Multiple approaches to access hundreds of genomic targets in CNS

ZFP-TF genome regulation		Example targets
Pan-Allele	ZFP-TFs for single gene repression and activation	• Tauopathies • α-synuclein • Autism Spectrum Disorder
Allele-Selective	ZFPs target disease allele repeats selectively	• Huntington's Disease • C9ORF72-linked ALS
Epigenetic editing	ZFP-Epi to demethylate select sites	• Rett Syndrome • Fragile X

ZFN genome editing		Example targets
Inflammation	CAR-T_{REGS} for remyelination and inhibition of neuroinflammation	• Multiple Sclerosis • ALS
Mitochondrial	ZFNs for selective clearance of mutant mitochondrial genomes	• Cerebellar Ataxia • Leigh Syndrome

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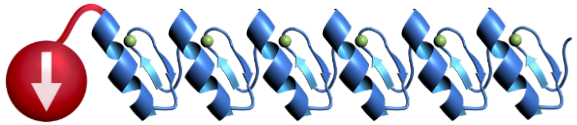
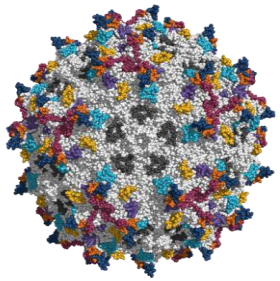
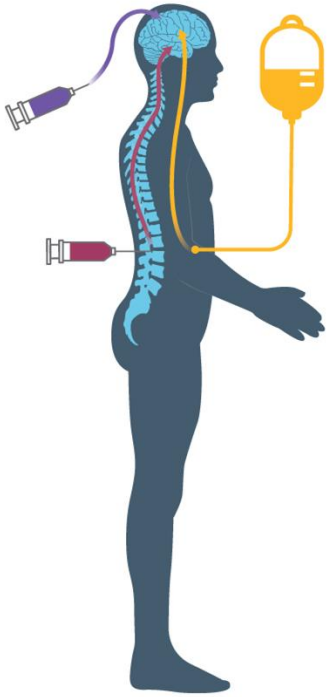
Zinc finger protein transcription factors (ZFP-TFs)

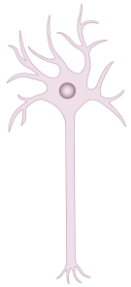
Zinc Finger Protein Transcription Factor (ZFP-TF)

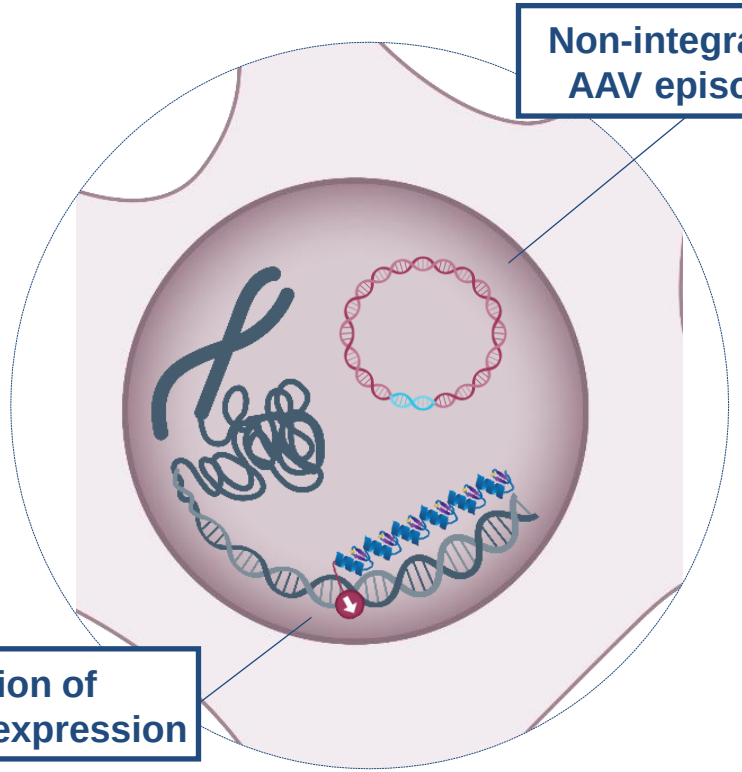




AAV Vector







1

ZFP-TF + AAV

2

Administration options: Parenchymal, CSF, IV

3

ZFP-TF reduces target gene expression

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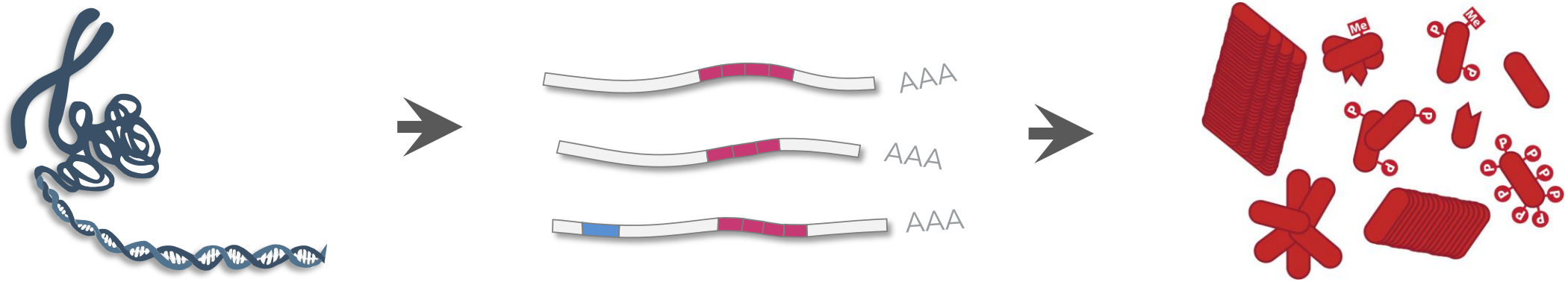
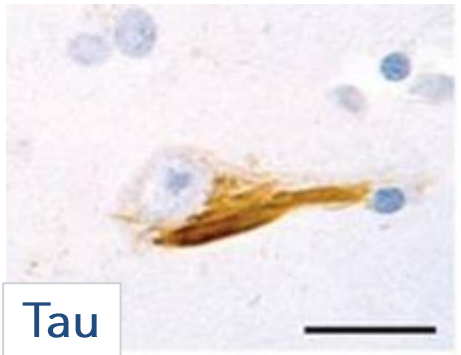
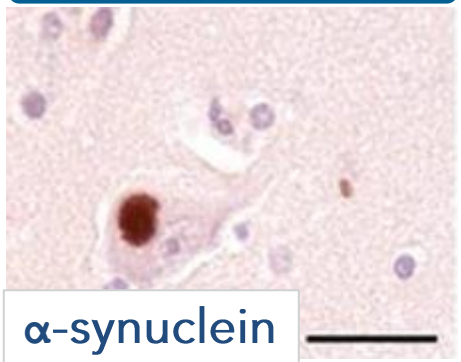
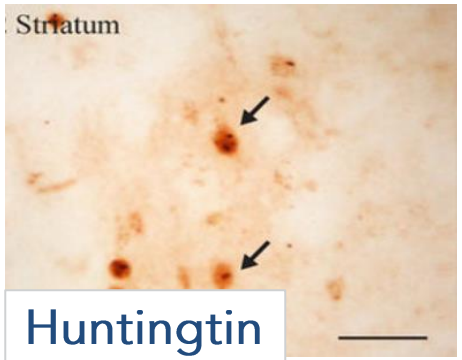
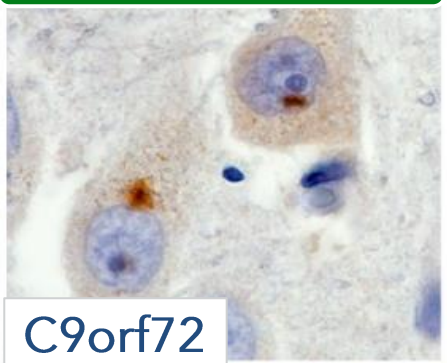
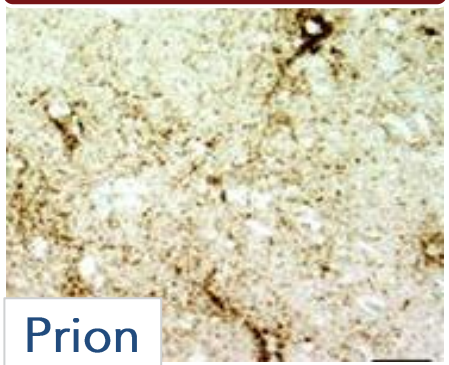
ZFP-TFs target upstream at the source of mutant protein isoforms and complexes



DNA
1 mutant allele

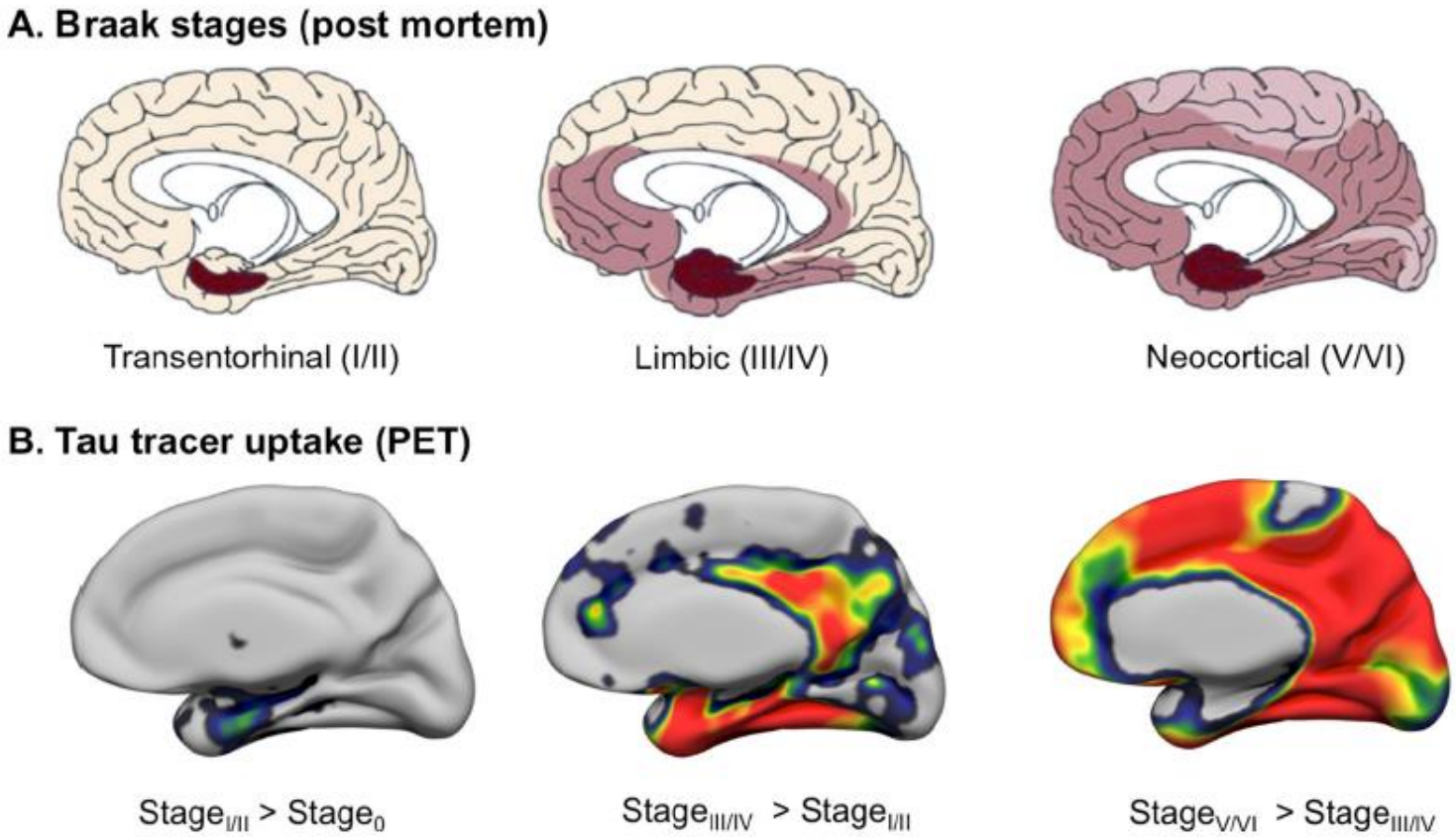
RNA
Sense, antisense, mis-spliced

Protein
Varied and complex

ALZHEIMER'S DISEASE	PARKINSON'S DISEASE	HUNTINGTON'S DISEASE	ALS	PRION DISEASE
				
Tau	α-synuclein	Huntingtin	C9orf72	Prion

Hill et al., 2003
Irwin et al., 2015
Jucker & Walker 2013
Waldvogel et al., 2014

Tau accumulation tracks closely with AD progression



Tau pathology is associated with several other diseases, including PSP, FTD, CTE and CBS

Progressive supranuclear palsy, Frontotemporal dementia, Chronic traumatic encephalopathy, Corticobasal syndrome

Figure: Schöll et al., 2018. Mol Cell Neurosci

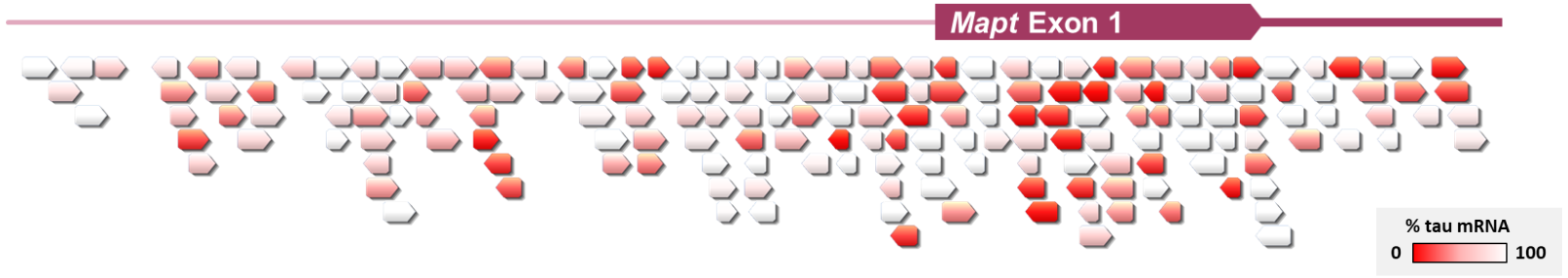
Potent, long-lasting, specific repression of tau by ZFP-TFs

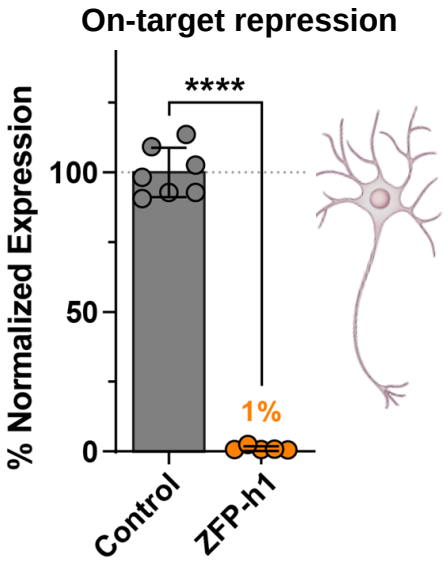
Pan-Allele

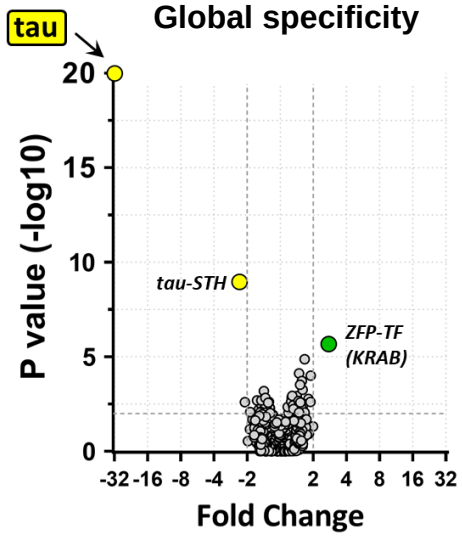
ST-501 – ALZHEIMER'S DISEASE AND OTHER TAUOPATHIES

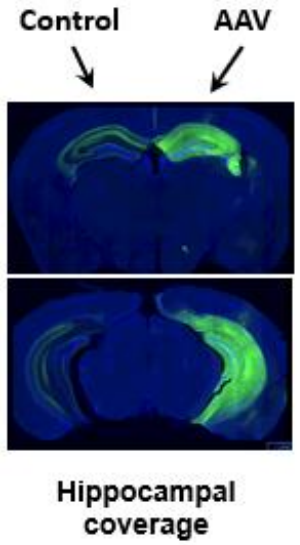
Abnormal tau is toxic to brain cells and leads to neurodegeneration in Alzheimer's disease

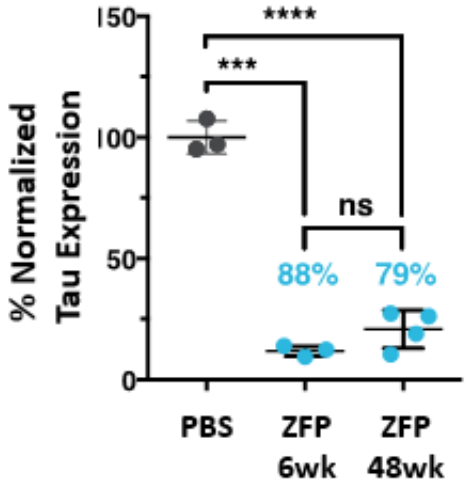
Tau reduction is a therapeutic strategy for targeting these diseases











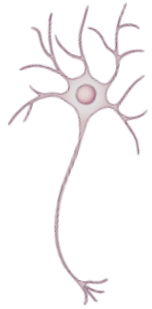
Tuning gene expression with ZFP-TFs to target disease pathology

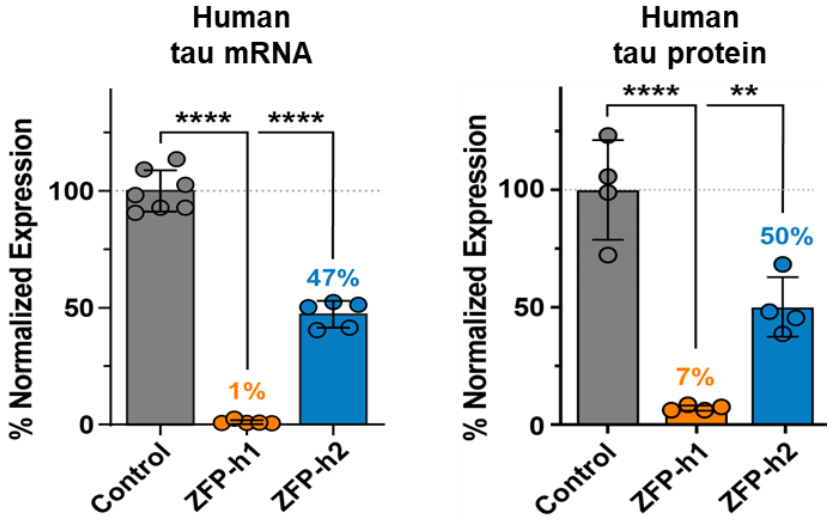
Pan-Allele

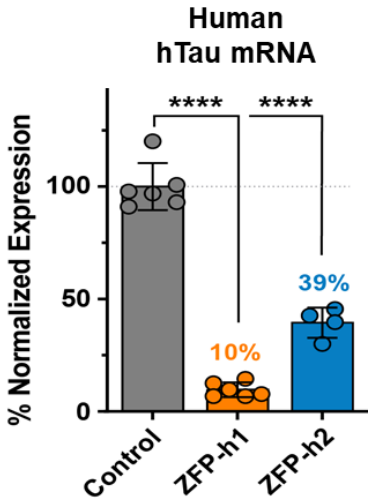
ST-501 – ALZHEIMER'S DISEASE AND OTHER TAUOPATHIES

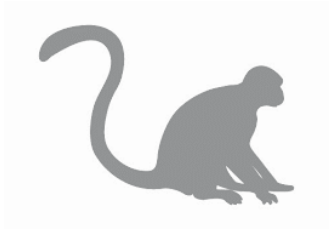
ZFP-TFs reduce expression of tau in a highly specific, tunable manner

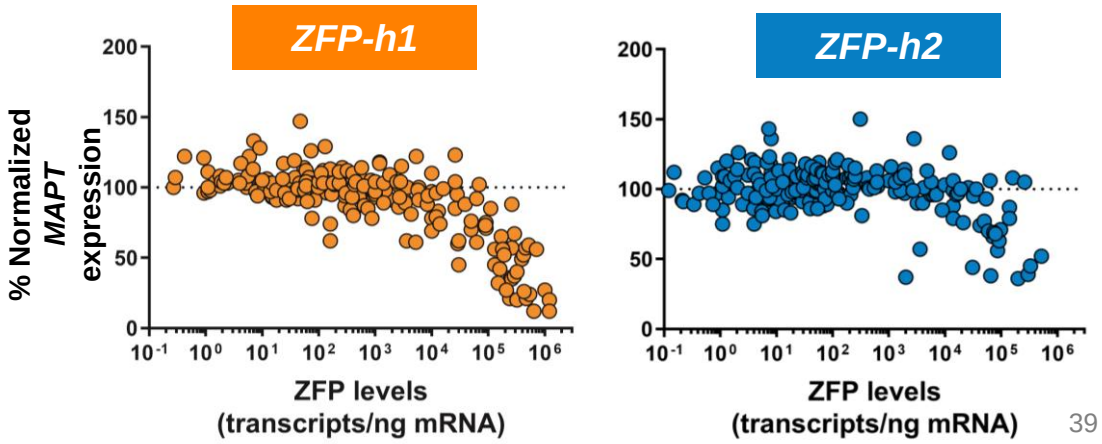
ZFP-TFs therefore represent a *novel therapeutic strategy for treating tauopathies*











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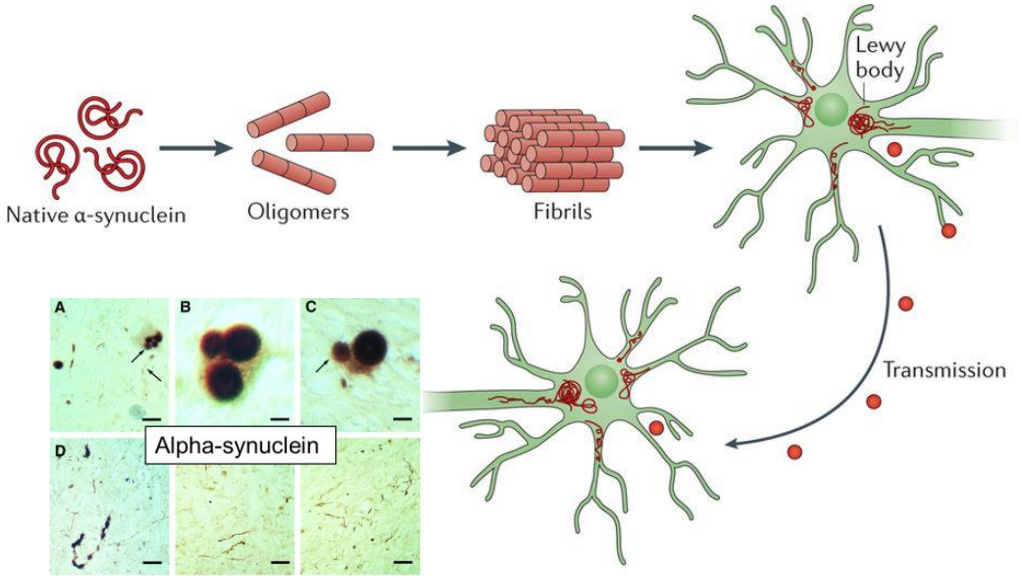
Reducing gene expression with ZFP-TFs to target disease pathology

Pan-Allele

ST-502 – PARKINSON'S DISEASE

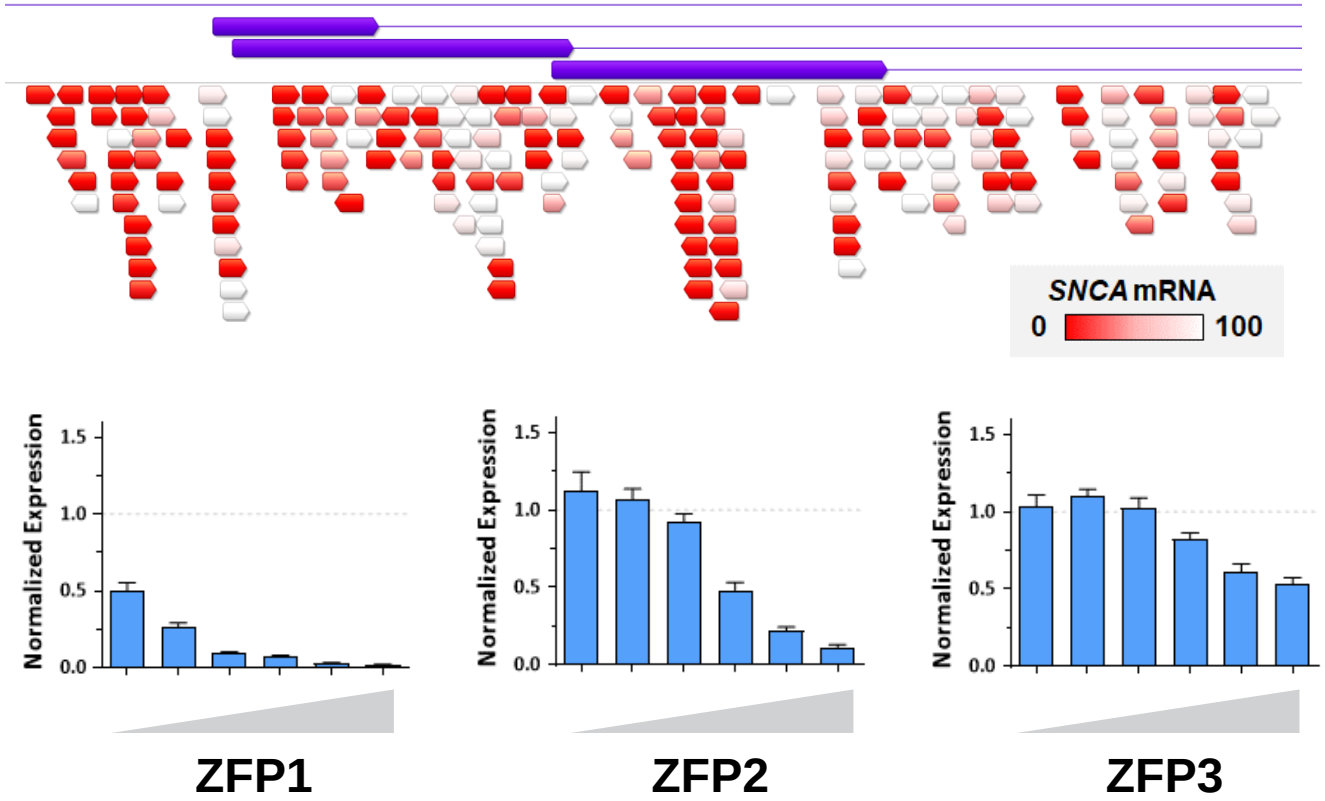
α-synuclein pathology tracks with disease progression in PD



Alpha-synuclein fibrils identified as major components of Lewy bodies and Lewy neurites (Goedert and Spillantini, 1998)

Nature Reviews | Drug Discovery

Kingwell 2017



55% of ZFP-TFs reduced total *SNCA* by ≥ 50%

3 / 10 / 30 / 100 / 300 / 1000
ng ZFP mRNA

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In-house cGMP facility and dedicated external manufacturing capacity

Ensuring control of quality, cost, IP and timelines

In-house Phase 1/2 cGMP Facilities

Brisbane, USA (late 2020/early 2021)
- Cell therapy
- Gene therapy

Valbonne, France (late 2021)
- Cell therapy

CDMO Thermo Fisher – dedicated access to AAV capacity up to 2000-L bioreactor scale

- Leveraging Thermo Fisher AAV manufacturing know-how
- Enables seamless transition from early to late-stage development
- Provides late-stage clinical and large-scale commercial grade supply

ThermoFisher SCIENTIFIC

Conclusions

Key takeaways

- Genomic medicine company building value with gene therapy, *ex vivo* gene-edited cell therapy, *in vivo* genome editing and genome regulation technology
- Precise, efficient and specific genomic medicine technology (ZFPs) backed by a robust patent estate
- Broad portfolio of rare and large indications across inherited metabolic diseases, immunology, CNS, hematology and oncology
- In-house cGMP facility and dedicated CDMO capacity provide manufacturing scale for clinical and commercial supply
- Strong balance sheet, six validating biopharma partnerships (Biogen, Gilead, Novartis, Pfizer, Sanofi, Takeda)


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