

# Corporate Presentation

May 2020



# Forward-Looking Statements

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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 with Biogen and its other partners 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 March 31, 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 therapy provides tractable, valuable near-term opportunities

Gene-Edited Cell Therapy  
AAV/LV; ZFN



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

Genome Editing  
AAV; ZFN



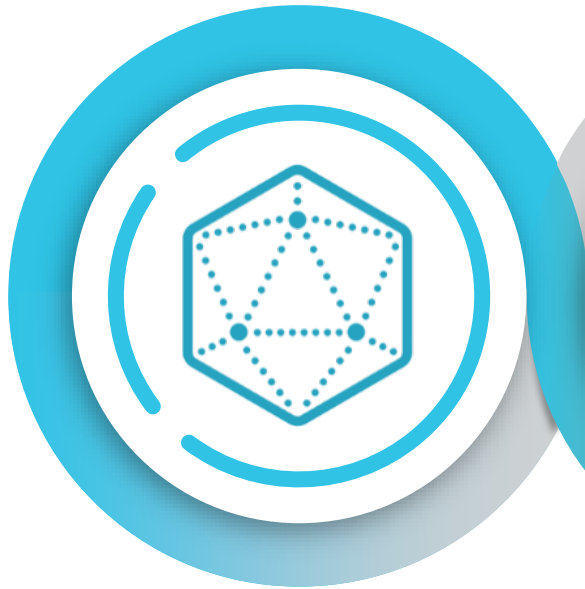
Sustain momentum with *in vivo* genome editing and genome regulation

Genome Regulation  
AAV; ZFP-TF



# 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:  $\alpha$ -synuclein  
C9ORF72-linked ALS/FTLD  
TAK-686: Huntington's disease  
Prion  
Undisclosed targets

# Responsibly advancing the business during COVID-19

- Sangamo has prioritized employee safety and welfare



- R&D activities continue with modified operations and protocols for on-site labs
- Non-lab employees are effectively collaborating together remotely

- In-house AAV manufacturing facility expected to be operational in Brisbane by YE 2020
- Valbonne and Brisbane cell therapy manufacturing expected to be operational by YE 2021
- No delays reported by CDMO partners

# Clinical programs updates and COVID-19 impact



Hemophilia A  
(SB-525 /  
PF-07055480)

PARTNER 

- Pfizer continuing to target first dosing in registrational Phase 3 trial in 2H 2020
- Pfizer and Sangamo seeking opportunity this year to provide next clinical data update from Phase 1/2 ALTA study



Fabry Disease  
(ST-920)



- Several patients successfully screened and enrolled in the Phase 1/2 STAAR study, and screening and enrollment continues
- First patient dosing pending due to COVID-19



Beta  
Thalassemia  
(ST-400)

PARTNER 

- Patient follow-up continues in Phase 1/2 THALES study



Sickle Cell  
Disease  
(BIVV003)

PARTNER 

- Sanofi continues to screen and enroll patients in Phase 1/2 PRECIZN-1 study



Oncology  
(KITE-037)

PARTNER 

- COVID-19 may impact timing of clinical trial initiation planned for 2020




















Solid Organ  
Transplant  
(TX200)









- Phase 1/2 STEADFAST study first patient dosing expected to occur in 2021

# Robust pipeline of genomic medicines

Preclinical				Phase 1/2			Phase 3
 <p>PKU (ST-101)</p> <p><b>Sangamo</b> THERAPEUTICS</p>	 <p>IBD</p> <p><b>Sangamo</b> THERAPEUTICS</p>	 <p>MS</p> <p><b>Sangamo</b> THERAPEUTICS</p>	 <p>Oncology (Undisclosed)</p> <p>PARTNER <b>Kite</b> A GILEAD Company</p>	 <p>Fabry Disease (ST-920)</p> <p><b>Sangamo</b> THERAPEUTICS</p>	 <p>Beta Thalassemia (ST-400)</p> <p>PARTNER <b>SANOFI</b></p>	 <p>Sickle Cell Disease (BIVV003)</p> <p>PARTNER <b>SANOFI</b></p>	 <p>Hemophilia A (SB-525 / PF-07055480)</p> <p>PARTNER <b>Pfizer</b></p>
 <p>Oncology (KITE-037)</p> <p>PARTNER <b>Kite</b> A GILEAD Company</p>	 <p>Solid Organ Transplant (TX200)</p> <p><b>Sangamo</b> THERAPEUTICS</p>	 <p>ALS/FTD</p> <p>PARTNER <b>Pfizer</b></p>	 <p>Huntington's Disease (TAK-686)</p> <p>PARTNER <b>Takeda</b></p>	 <p>MPS II (SB-913)</p> <p><b>Sangamo</b> THERAPEUTICS</p>			
 <p><math>\alpha</math>-Synuclein (ST-502)</p> <p>PARTNER <b>Biogen</b></p>	 <p>Tauopathies (ST-501)</p> <p>PARTNER <b>Biogen</b></p>	 <p>Neurology (Undisclosed)</p> <p>PARTNER <b>Biogen</b></p>	 <p>Prion</p> <p><b>Sangamo</b> THERAPEUTICS</p>				



# Increasing productivity and realizing value through biopharmaceutical partnerships

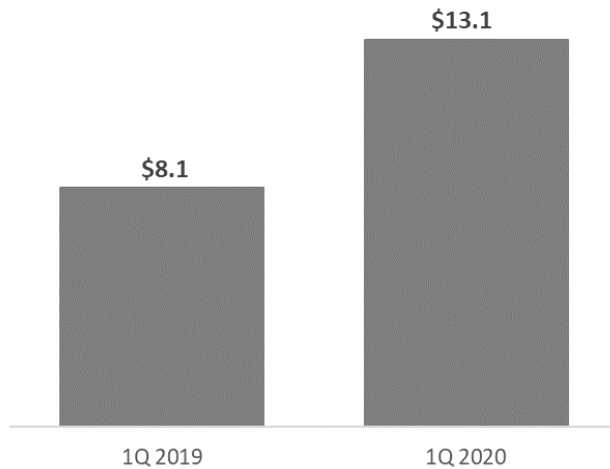
	 <b>Biogen</b>	 <b>GILEAD</b>	 <b>Pfizer</b>	 <b>Pfizer</b>	 <b>SANOFI</b>	 <b>Takeda</b>
<b>Target/ therapeutic area</b>	Neurological including AD, PD	Oncology anti-CD19 CAR-T	C9ORF72 ALS	Hemophilia A	Beta thalassemia, Sickle Cell disease	Huntington's disease
<b>Development phase</b>	Preclinical	Preclinical	Preclinical	Phase 3	Phase 1/2	Preclinical
<b>Technology</b>	Genome regulation	Cell therapy	Genome regulation	Gene therapy	Cell therapy	Genome regulation
<b>Royalties (% on net sales)</b>	High-single to sub-teen double-digit	Single-digit	Mid- to high-single digit	Low teens to 20	Double-digit	Single-digit
<b>Upfront &amp; equity</b>	\$125M payment + \$225M in equity purchase	\$150M payment + \$50M in equity purchase	\$12M	\$70M	\$20M	\$13M
<b>Milestones</b>	Up to \$2.37B (\$925M pre-commercial, and \$1.445B for 1 <sup>st</sup> sale and sales thresholds)	Up to \$3.1B (\$1.3B through 1st sale, and \$1.8B sales thresholds)	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: ~\$700 million  
 Future opportunity: **Royalties** on net product sales, as well as **\$6.34 billion** in potential milestone payments

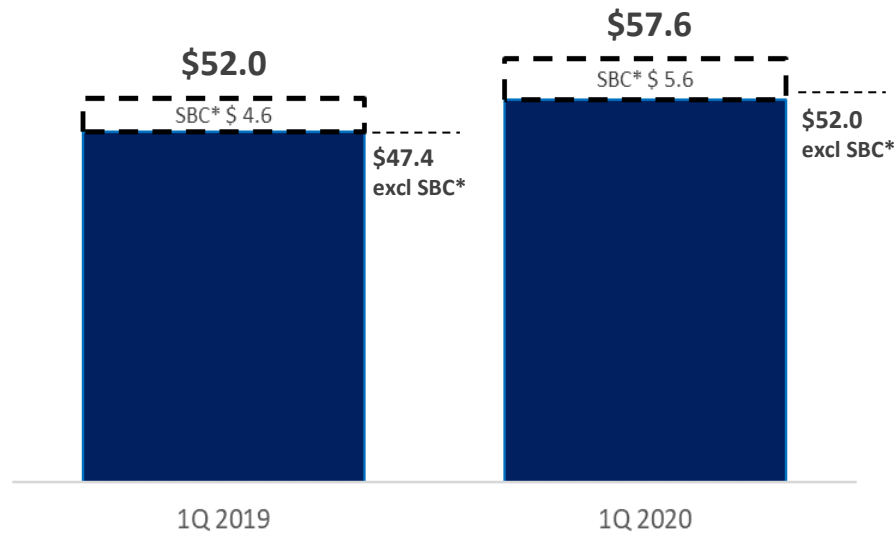
# 1Q 2020 financial results; 2020 guidance reiterated

\$ in Millions

## Revenues



## Operating Expenses



2020 non-GAAP  
Opex excluding SBC\*\*

**\$245M-  
\$260M**



Sangamo and Biogen  
Collaboration

Gene regulation  
therapies for  
neurological diseases

# Sangamo and Biogen collaboration

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- Strategically partners Sangamo's Alzheimer's and Parkinson's programs with Biogen's world-class neuroscience expertise
- Biogen's access to Sangamo's gene regulation therapies complements its expanding efforts in gene therapy across diverse neurological diseases
- ZFP-TFs are ideally suited to neurological disorders due to ability to up or down regulate gene expression, targeting disease pathology at its genesis
- Sangamo's balance sheet significantly strengthened by Biogen's investment

# Collaboration scope and responsibilities

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- Exclusive global rights to 3 neurological targets: tau (Alzheimer's), alpha-synuclein (Parkinson's), and one neuromuscular target
- Option for exclusive rights for up to 9 additional targets over 5 years
- Access to Sangamo's zinc finger protein technology (ZFP-TFs and ZFNs) and novel AAV serotypes
- Sangamo to lead early research; Biogen responsible for global development and commercialization
- Sangamo responsible for GMP manufacturing activities for use in initial clinical trial for first 3 products\*, leveraging in-house capacity and capabilities; Biogen responsible for subsequent GMP manufacturing activities



# Collaboration financial summary

**Upfront**

**\$350M**

\$125M upfront payment\*  
\$225M purchase of ~24.4M Sangamo shares  
@ \$9.21/share\*

**Milestones**

**\$2.37B**

\$925M – precommercial activities  
\$1.445B – 1<sup>st</sup> commercial sale and other sales-based milestones

**Royalties**

**Net sales %**

High single to sub-teen double digits

**R&D**

**Funding**

Cost sharing of early research;  
Biogen responsible for all costs thereafter






























\*Sangamo announced the closing of the Biogen collaboration agreement on April 9<sup>th</sup> and has received \$225 million in stock proceeds and \$125 million for the upfront license fee

The background is a solid dark blue color. On the left side, there are two thick, light blue curved lines that sweep from the bottom left towards the top right, partially framing the text.

Applying Sangamo's  
technology to  
neurological diseases

# In vivo genome regulation for neurological diseases

Preclinical				Phase 1/2			Phase 3
 <p>PKU (ST-101)</p> <p>Sangamo</p>	 <p>IBD</p> <p>Sangamo</p>	 <p>MS</p> <p>Sangamo</p>	 <p>Oncology (Undisclosed)</p> <p>PARTNER </p>	 <p>Fabry Disease (ST-920)</p> <p>Sangamo</p>	 <p>Beta Thalassemia (ST-400)</p> <p>PARTNER </p>	 <p>Sickle Cell Disease (BIVV003)</p> <p>PARTNER </p>	 <p>Hemophilia A (SB-525 / PF-07055480)</p> <p>PARTNER </p>
 <p>Oncology (KITE-037)</p> <p>PARTNER </p>	 <p>Solid Organ Transplant (TX200)</p> <p>Sangamo</p>	 <p>ALS/FTD</p> <p>PARTNER </p>	 <p>Huntington's Disease (TAK-686)</p> <p>PARTNER </p>	 <p>MPS II (SB-913)</p> <p>Sangamo</p>			
 <p><math>\alpha</math>-Synuclein (ST-502)</p> <p>PARTNER </p>	 <p>Tauopathies (ST-501)</p> <p>PARTNER </p>	 <p>Neurology (Undisclosed)</p> <p>PARTNER </p>	 <p>Prion</p> <p>Sangamo</p>				



# 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

- Tauopathies
- $\alpha$ -synuclein
- Prion

### 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<sub>REGS</sub> for remyelination and inhibition of neuroinflammation

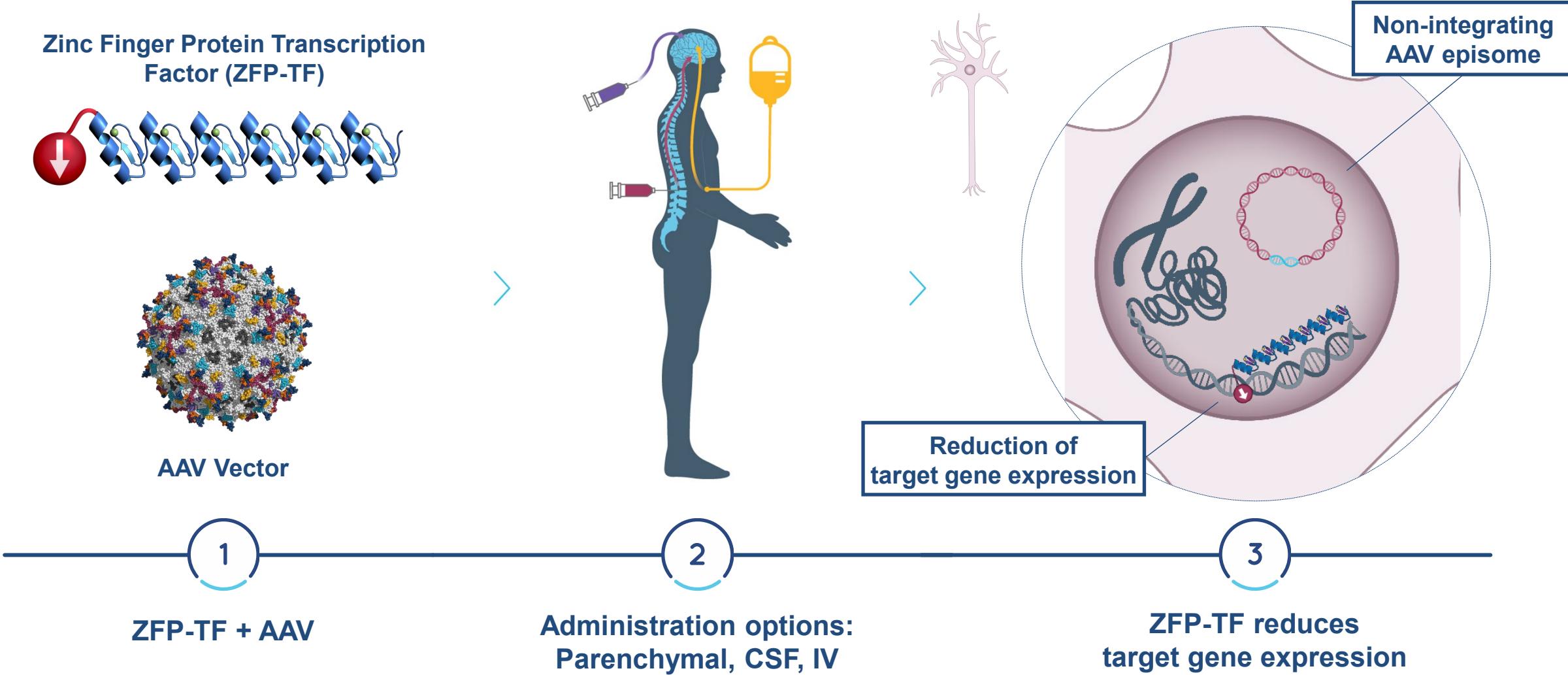
- Multiple Sclerosis
- ALS

### Mitochondrial

ZFNs for selective clearance of mutant mitochondrial genomes

- Cerebellar Ataxia
- Leigh Syndrome

# Zinc finger protein transcription factors (ZFP-TFs)

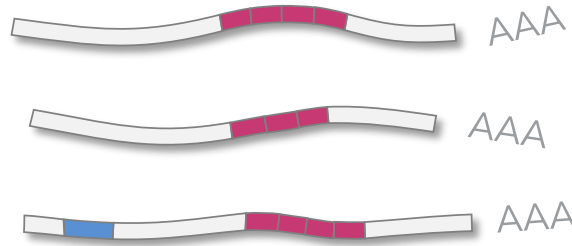


# ZFP-TFs target upstream at the source of mutant protein isoforms and complexes



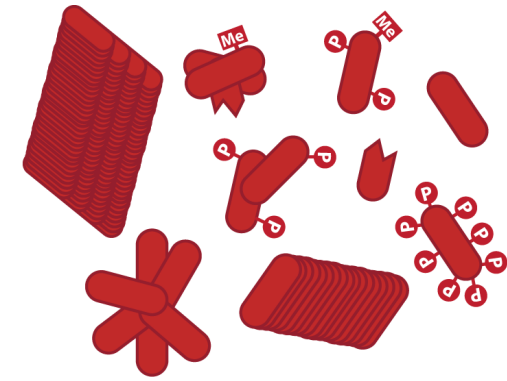
**DNA**

1 mutant allele



**RNA**

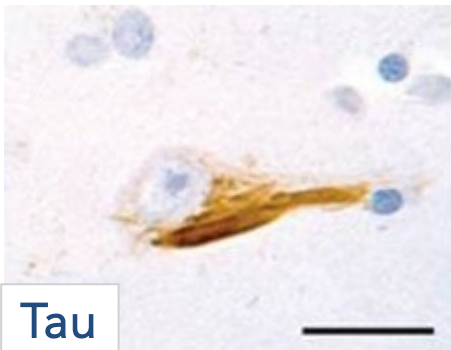
Sense, antisense, mis-spliced



**Protein**

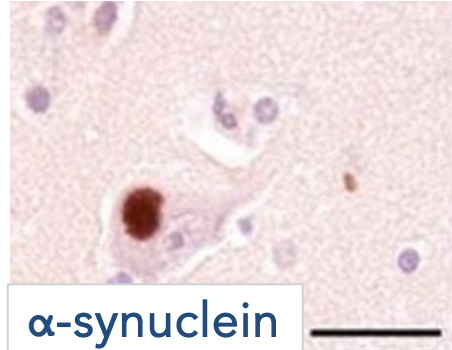
Varied and complex

**ALZHEIMER'S DISEASE**



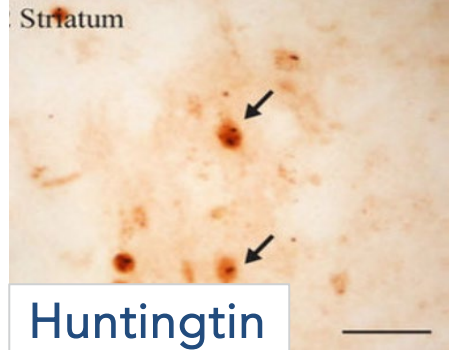
Tau

**PARKINSON'S DISEASE**



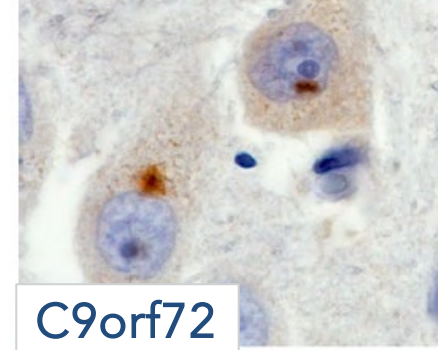
$\alpha$ -synuclein

**HUNTINGTON'S DISEASE**



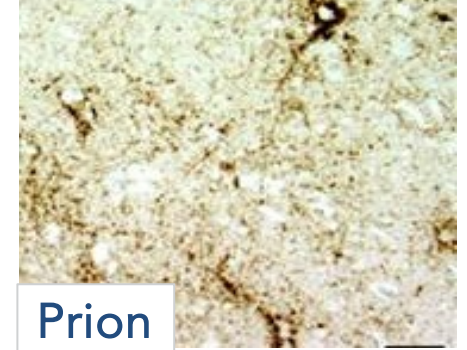
Huntingtin

**ALS**



C9orf72

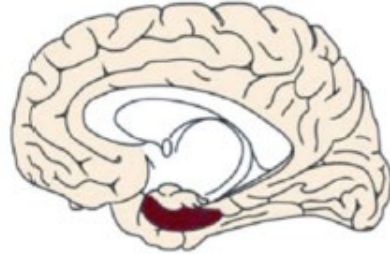
**PRION DISEASE**



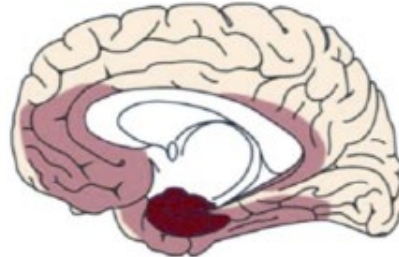
Prion

# Tau accumulation tracks closely with AD progression

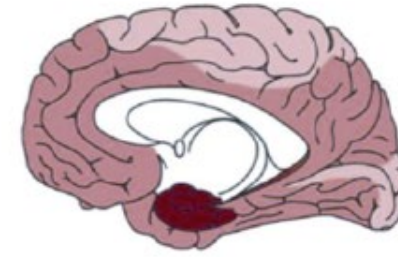
## A. Braak stages (post mortem)



Transentorhinal (I/II)

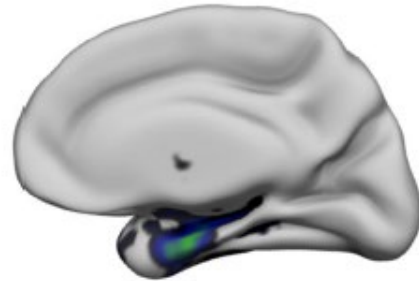


Limbic (III/IV)

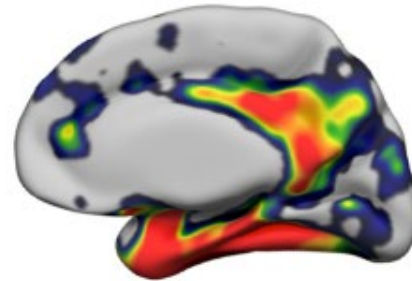


Neocortical (V/VI)

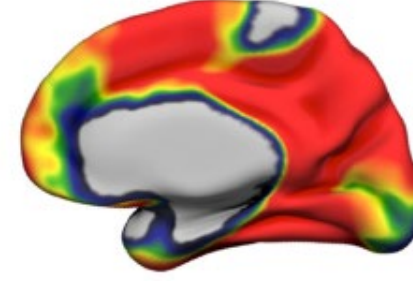
## B. Tau tracer uptake (PET)



Stage<sub>I/II</sub> > Stage<sub>0</sub>



Stage<sub>III/IV</sub> > Stage<sub>I/II</sub>



Stage<sub>V/VI</sub> > Stage<sub>III/IV</sub>

**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



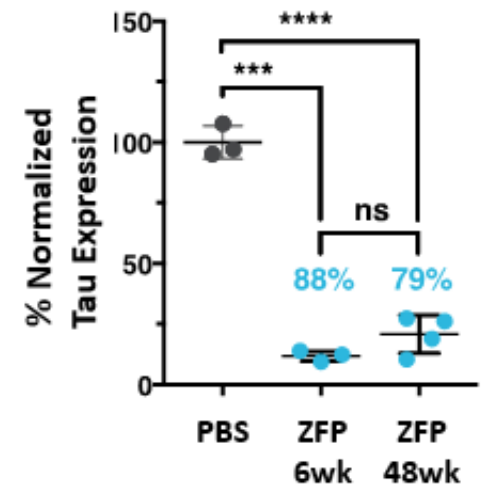
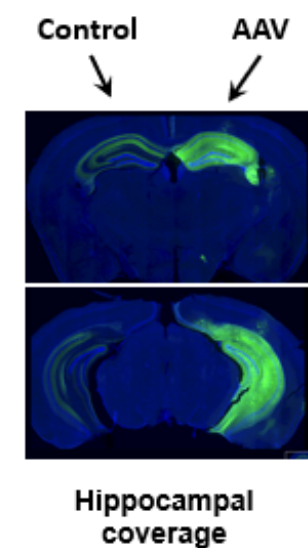
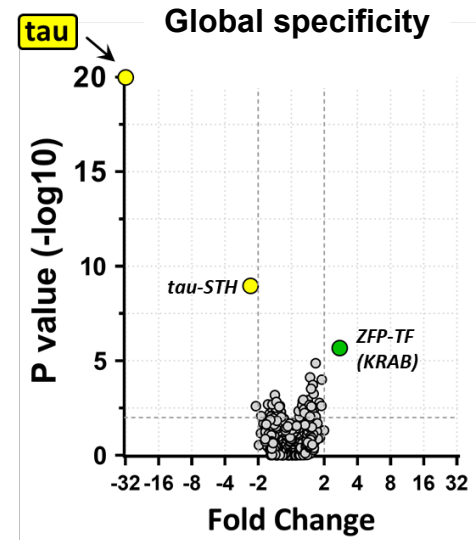
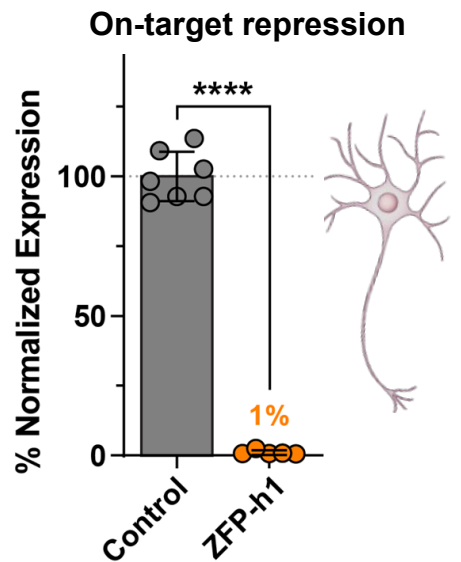
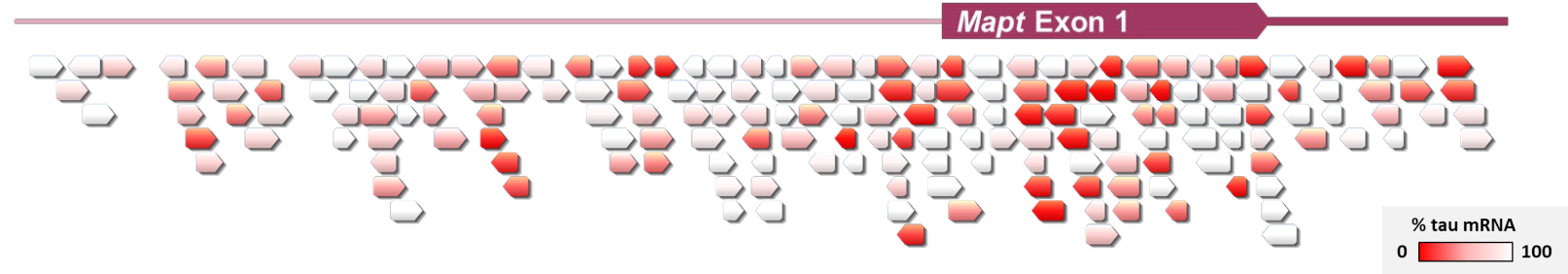
# Potent, long-lasting, specific repression of tau by ZFP-TFs

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





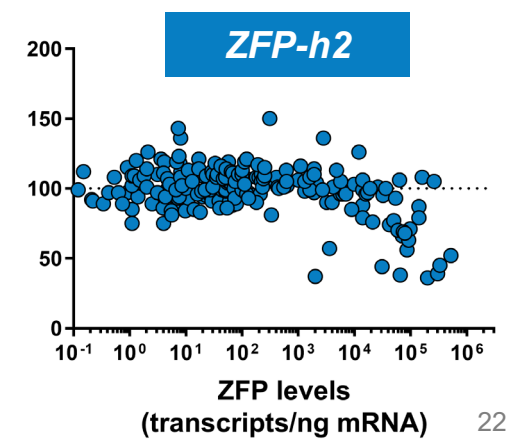
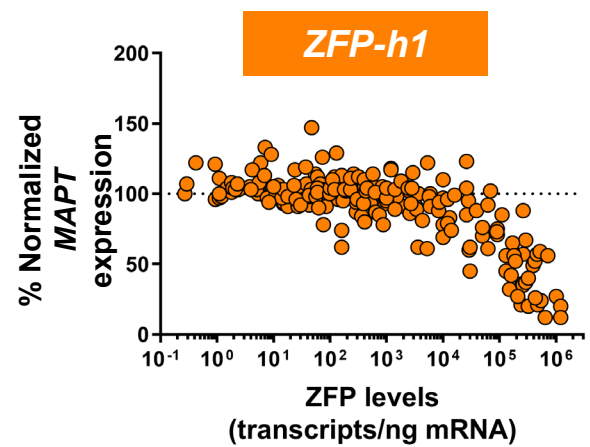
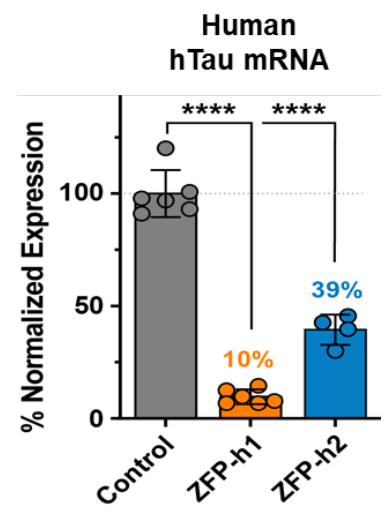
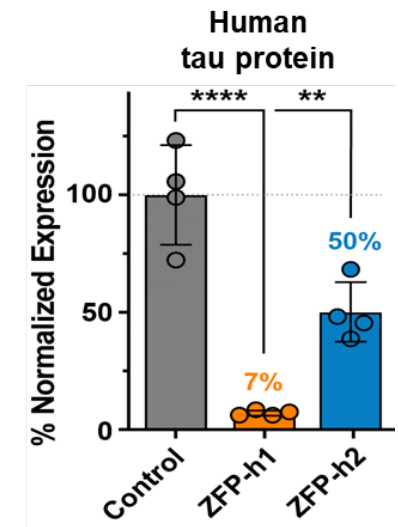
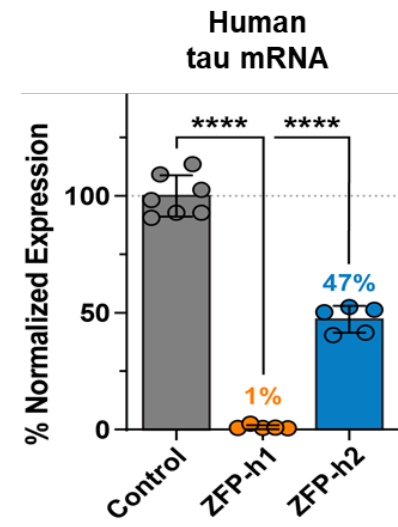
# Tuning gene expression with ZFP-TFs to target disease pathology

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*

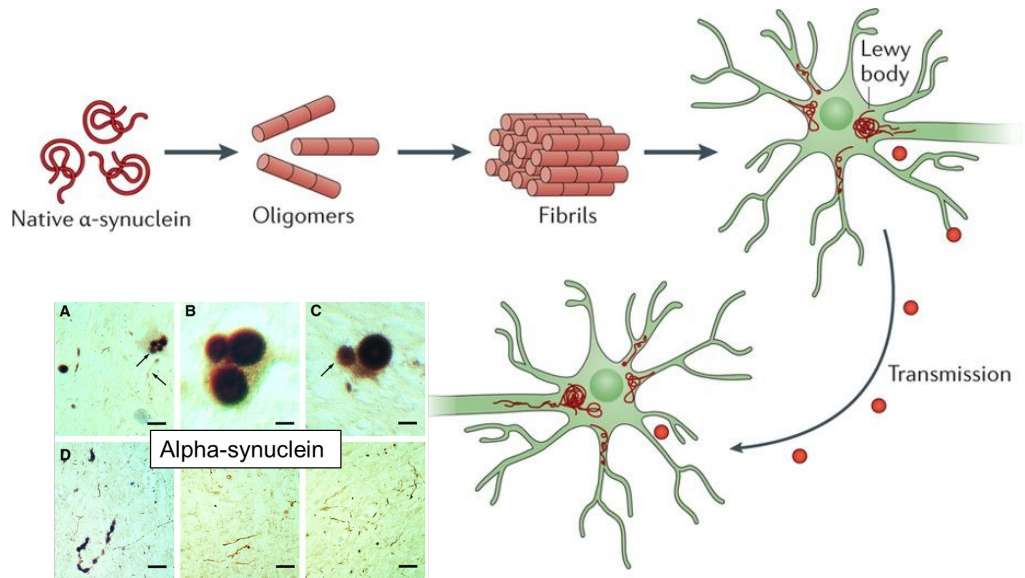


# Reducing gene expression with ZFP-TFs to target disease pathology

Pan-Allele

## ST-502 – PARKINSON'S DISEASE

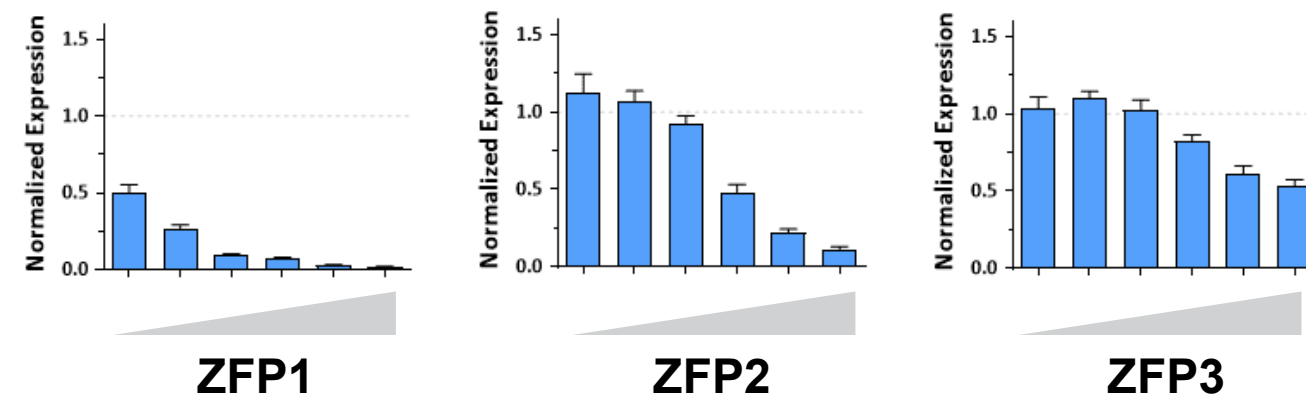
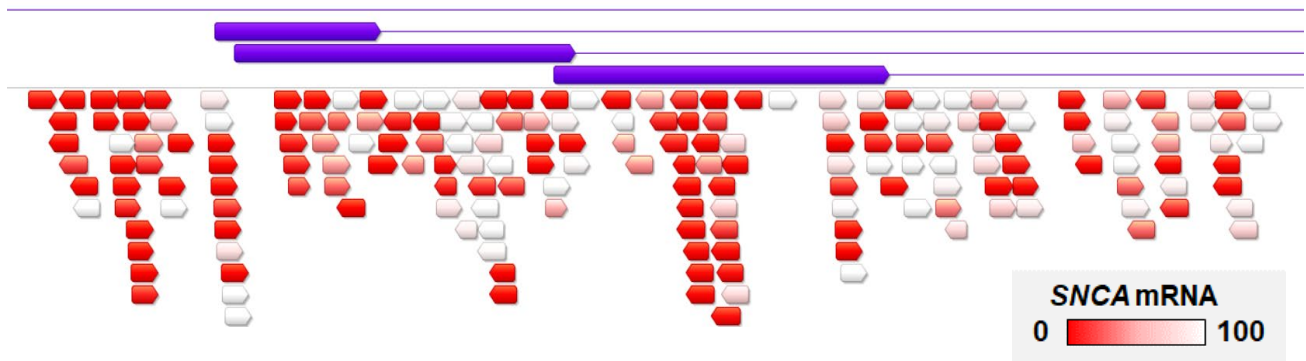
$\alpha$ -synuclein pathology tracks with disease progression in PD



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  $\geq 50\%$**

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






















# Clinical and preclinical pipeline

Gene Therapy  
Cell Therapy  
Genome Editing











# Gene therapy in 2020: Building on hemophilia A data

## Preclinical

 <p>PKU (ST-101)</p> 	 <p>IBD</p> 	 <p>MS</p> 	 <p>Oncology (Undisclosed)</p> <p>PARTNER </p>
 <p>Oncology (KITE-037)</p> <p>PARTNER </p>	 <p>Solid Organ Transplant (TX200)</p> 	 <p>ALS/FTD</p> <p>PARTNER </p>	 <p>Huntington's Disease (TAK-686)</p> <p>PARTNER </p>
 <p><math>\alpha</math>-Synuclein (ST-502)</p> <p>PARTNER </p>	 <p>Tauopathies (ST-501)</p> <p>PARTNER </p>	 <p>Neurology (Undisclosed)</p> <p>PARTNER </p>	 <p>Prion</p> 

## Phase 1/2

 <p>Fabry Disease (ST-920)</p> 	 <p>Beta Thalassemia (ST-400)</p> <p>PARTNER </p>	 <p>Sickle Cell Disease (BIVV003)</p> <p>PARTNER </p>
 <p>MPS II (SB-913)</p> 		

## Phase 3

 <p>Hemophilia A (SB-525 / PF-07055480)</p> <p>PARTNER </p>
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Presented at  
the 2019 American  
Society of Hematology  
Annual Meeting;  
Orlando, Florida;  
December 7, 2019

# Updated Follow-up of the Alta Study, a Phase 1/2, Open Label, Adaptive, Dose-Ranging Study to Assess the Safety and Tolerability of SB-525 Gene Therapy in Adult Subjects With Hemophilia A

*Barbara A. Konkle,<sup>1</sup> Kimo Stine,<sup>2</sup> Nathan Visweshwar,<sup>3</sup> Thomas Harrington,<sup>4</sup> Andrew D. Leavitt,<sup>5</sup> Adam Giermasz,<sup>6</sup> Steven Arkin,<sup>7</sup> Gregory Di Russo,<sup>7</sup> Ashley Snyder,<sup>8</sup> Adrian Woolfson,<sup>8</sup> and Didier Rouy<sup>8</sup>*

*<sup>1</sup>Bloodworks Northwest and the University of Washington, Seattle, WA; <sup>2</sup>Arkansas Children's Hospital, Little Rock, AR; <sup>3</sup>Department of Internal Medicine, Division of Hematology and Medical Oncology, University of South Florida, Tampa, FL; <sup>4</sup>University of Miami Miller School of Medicine, Miami, FL; <sup>5</sup>Department of Laboratory Medicine and Department of Medicine, University of California, San Francisco, CA; <sup>6</sup>University of California, Davis, CA; <sup>7</sup>Pfizer Inc., Cambridge, MA.; <sup>8</sup>Sangamo Therapeutics, Brisbane, CA*

# SB-525 (PF-07055480) program transitioned to Pfizer for Phase 3 development

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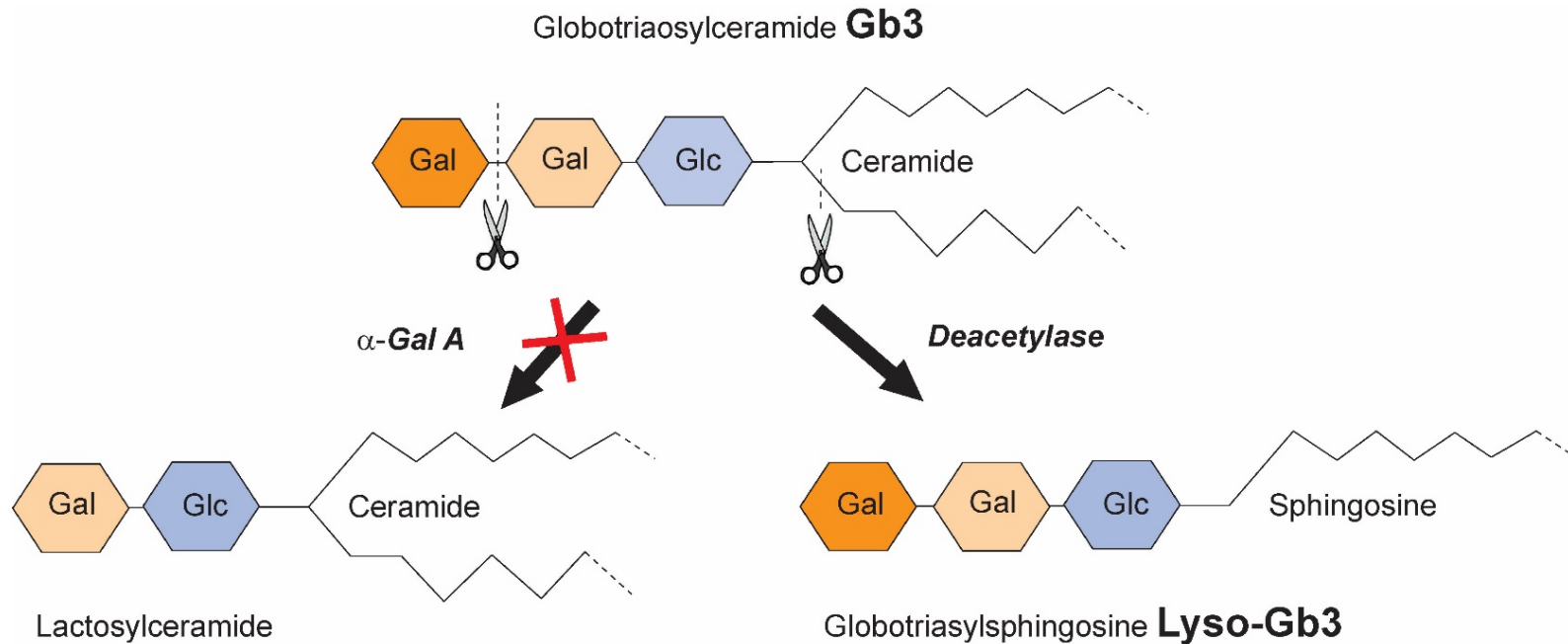
- Pfizer continues to target dosing the first patient in 2H 2020
  - Objective: To evaluate the clinical efficacy and safety of a single IV infusion of PF-07055480 in eligible patients who have completed at least 6 months in prior lead-in study
- Enrollment in Pfizer's Phase 3 lead-in study commenced in October and is ongoing
  - Objective: To establish  $\geq 6$  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

**Sangamo**  
THERAPEUTICS

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**Pfizer**

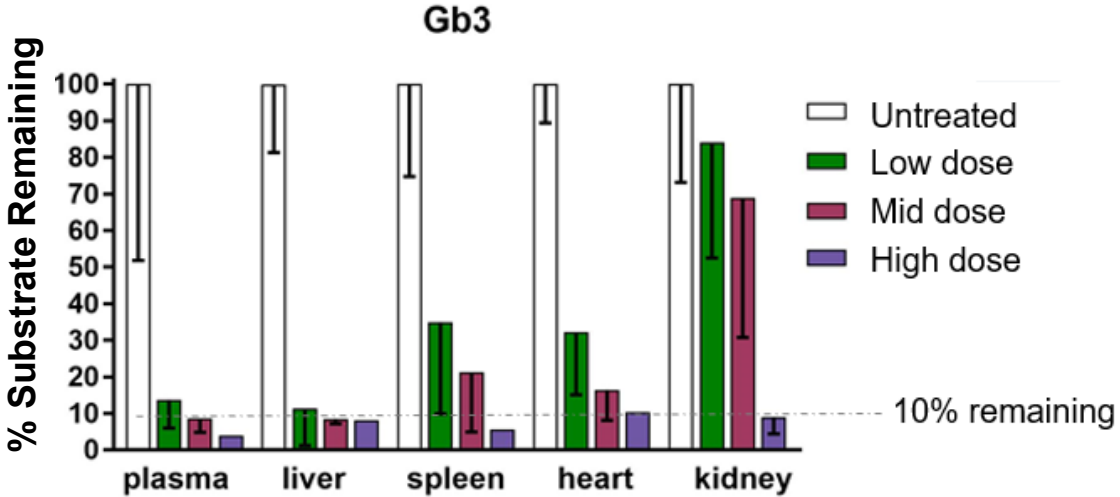
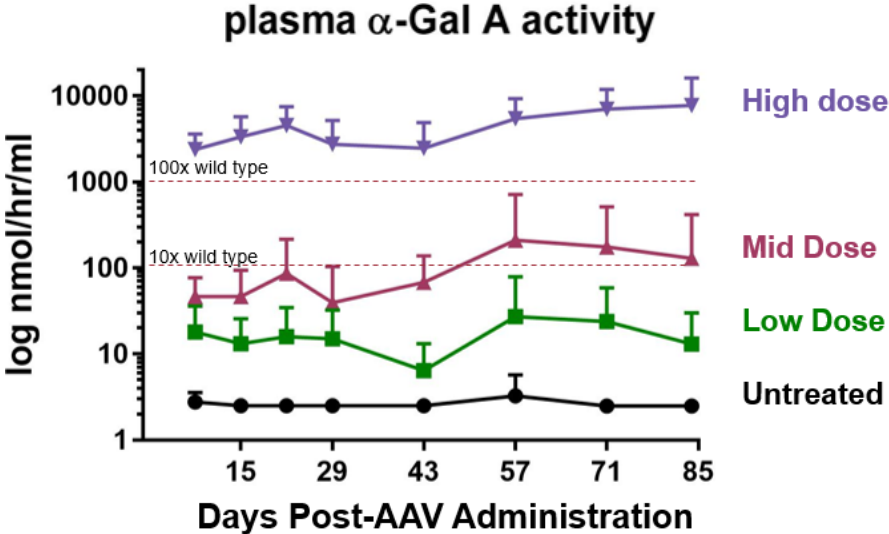
# Fabry disease: A lysosomal storage disorder



- Fabry disease is an **X-linked** monogenic disease caused by mutations in GLA gene encoding the enzyme alpha-galactosidase A ( **$\alpha$ -Gal A**)
- **$\alpha$ -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; UK approval granted for CTA
- ✓ AAV produced using clinical scale manufacturing methods



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

## Primary Objective

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- Assess safety & tolerability of ST-920

## Secondary Objectives

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- Assess the pharmacodynamics of  $\alpha$ -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

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- Male subjects  $\geq$  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  $\alpha$ -Gal A, resulting in long-term expression of  $\alpha$ -Gal A

# ST-920 offers a potentially differentiated treatment for Fabry








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- In a single IV infusion, ST-920 may provide continuous, potentially life-long expression of endogenously expressed  $\alpha$ -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

## Preclinical

 <p>PKU (ST-101)</p> <p>Sangamo</p>	 <p>IBD</p> <p>Sangamo</p>	 <p>MS</p> <p>Sangamo</p>	 <p>Oncology (Undisclosed)</p> <p>PARTNER </p>
 <p>Oncology (KITE-037)</p> <p>PARTNER </p>	 <p>Solid Organ Transplant (TX200)</p> <p>Sangamo</p>	 <p>ALS/FTD</p> <p>PARTNER </p>	 <p>Huntington's Disease (TAK-686)</p> <p>PARTNER </p>
 <p><math>\alpha</math>-Synuclein (ST-502)</p> <p>PARTNER </p>	 <p>Tauopathies (ST-501)</p> <p>PARTNER </p>	 <p>Neurology (Undisclosed)</p> <p>PARTNER </p>	 <p>Prion</p> <p>Sangamo</p>

## Phase 1/2

 <p>Fabry Disease (ST-920)</p> <p>Sangamo</p>	 <p>Beta Thalassemia (ST-400)</p> <p>PARTNER </p>	 <p>Sickle Cell Disease (BIVV003)</p> <p>PARTNER </p>
 <p>MPS II (SB-913)</p> <p>Sangamo</p>		

## Phase 3

 <p>Hemophilia A (SB-525 / PF-07055480)</p> <p>PARTNER </p>
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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 $\beta$ -Thalassemia**

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

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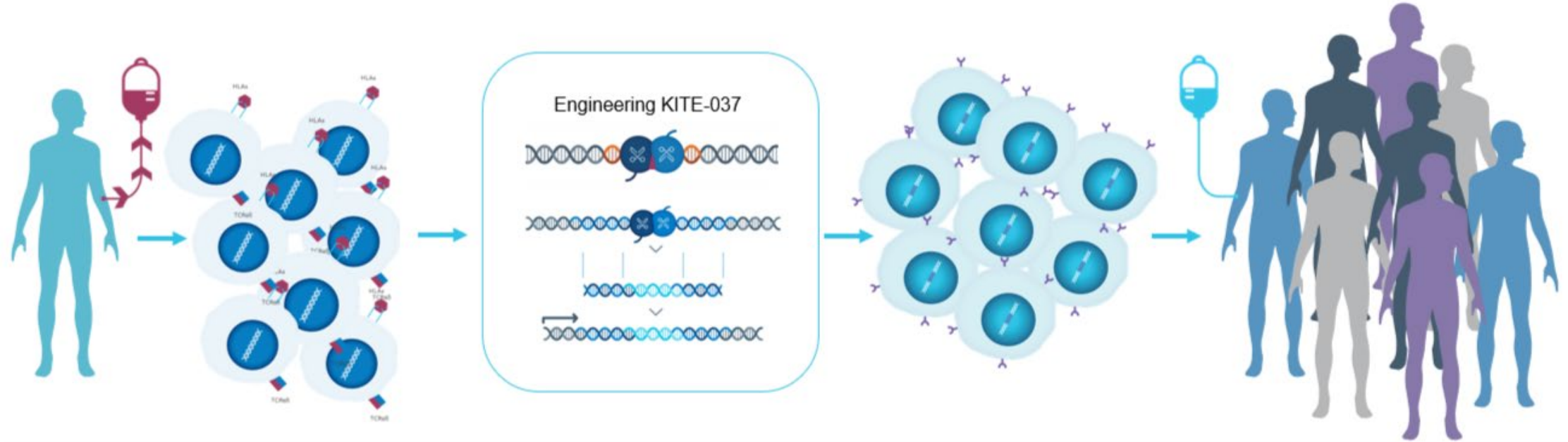
# Hemoglobinopathies collaboration with Sanofi: BIVV003 for Sickle Cell Disease and ST-400 for Beta Thalassemia



- Sanofi continuing to screen/enroll subjects into the PRECIZN-1 clinical trial evaluating BIVV003 for sickle cell disease
  - Sangamo earned a \$7.5 million milestone payment with first subject dosed in 4Q 2019
- 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



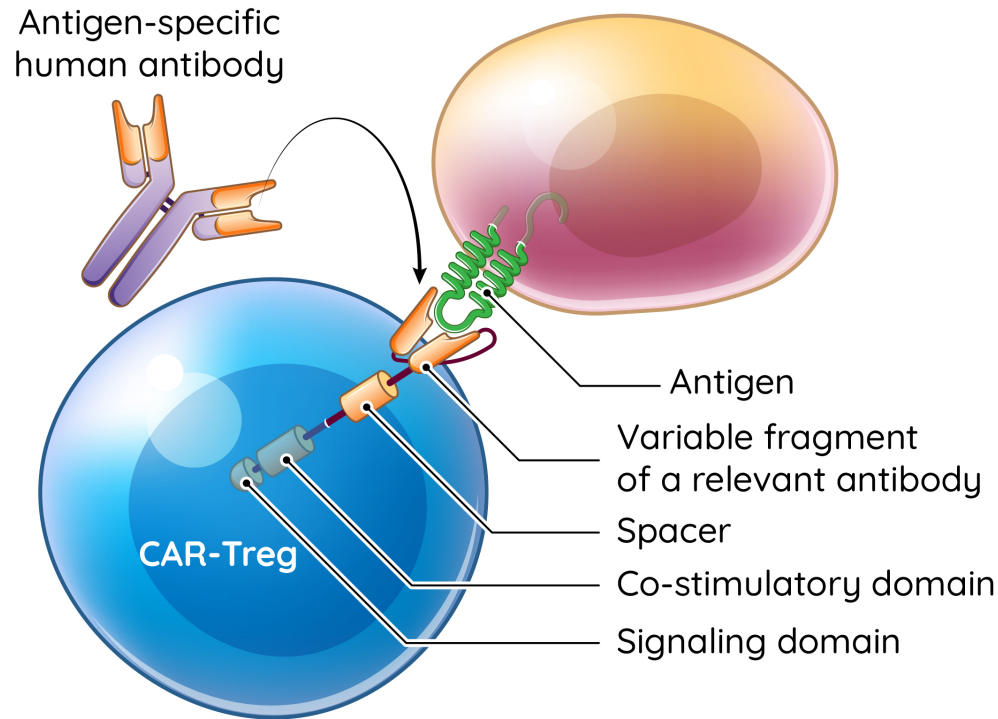
# KITE-037, the first product candidate of the collaboration with Kite, a Gilead Company



- 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<sub>REG</sub> Function with CAR-T<sub>REG</sub> Therapy

## CAR-T<sub>REGS</sub> may overcome limitations of polyclonal T<sub>REG</sub> Therapy



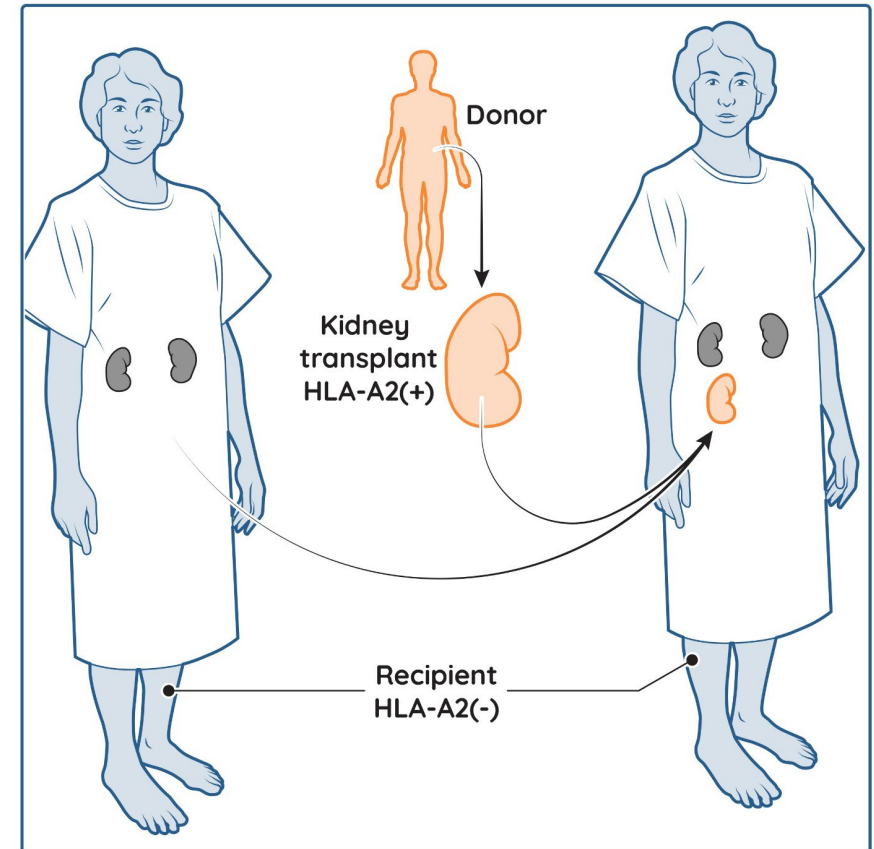
- *Ex vivo* engineered and expanded
- Tissue targeted
- Antigen activated & expanded
- Multiple mechanisms of immune regulation

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

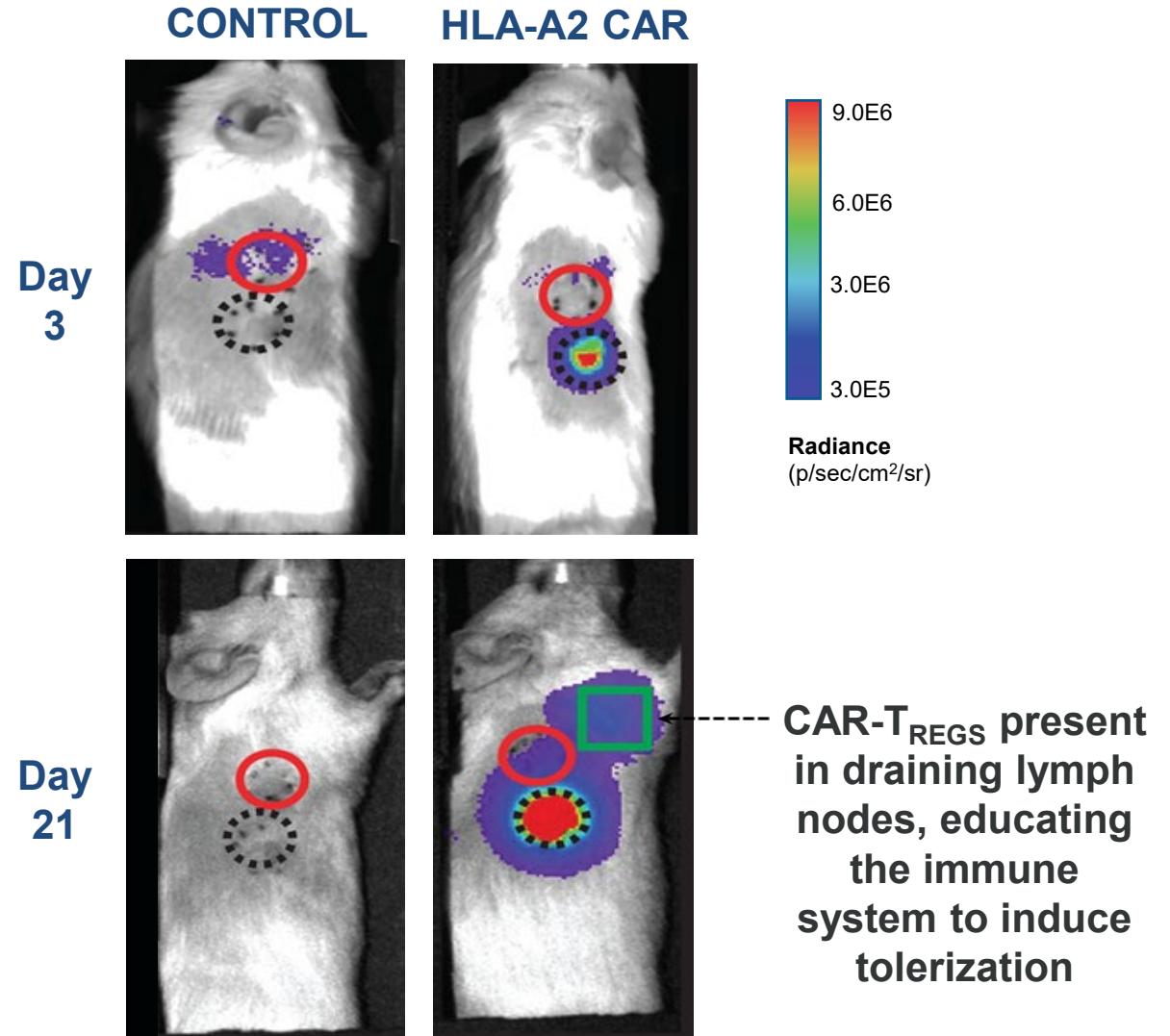
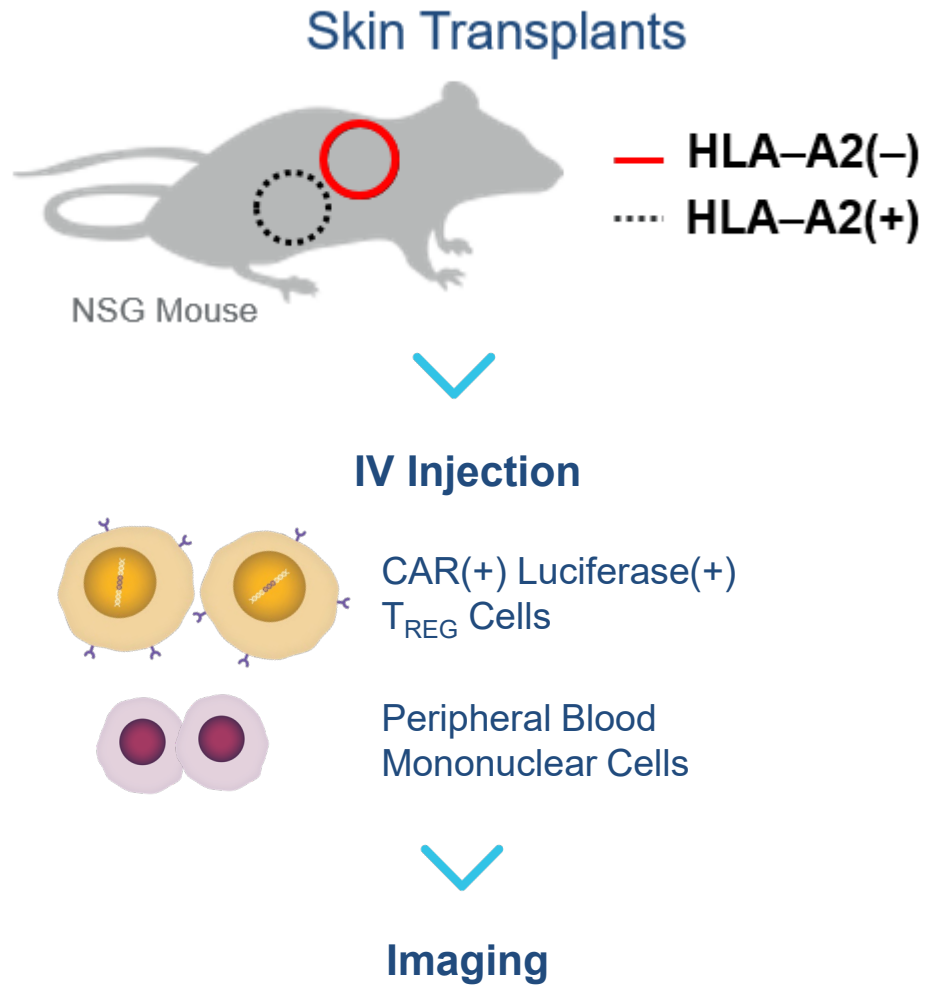


## Autologous HLA-A2 specific CAR-T<sub>REG</sub> cell therapy

- 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<sub>REGS</sub> achieve precise and durable targeting of skin graft in a mouse model

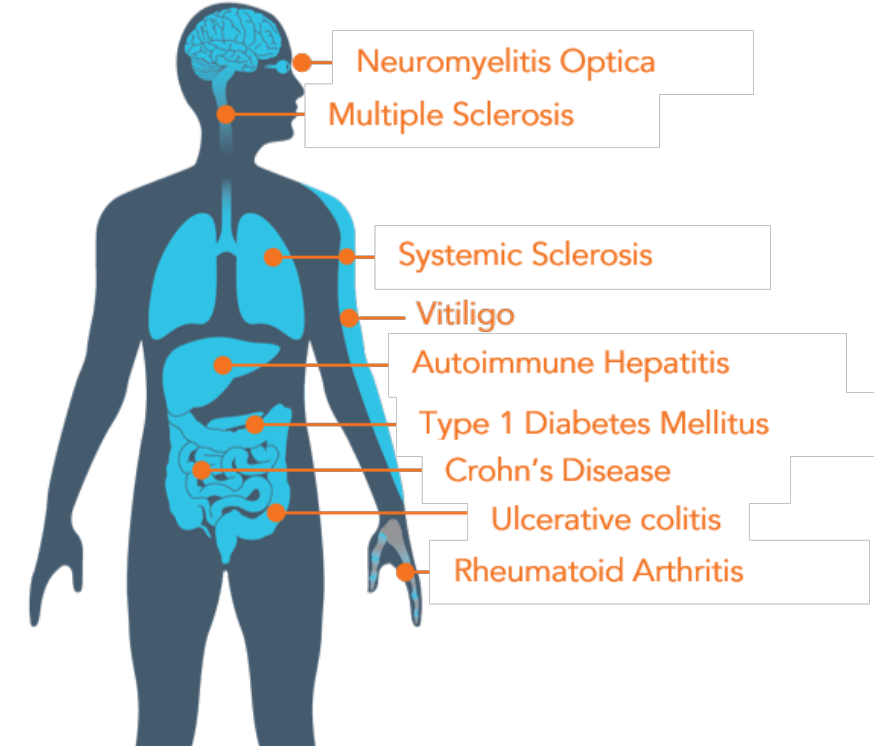


# TX200: Gateway to major autoimmune indications



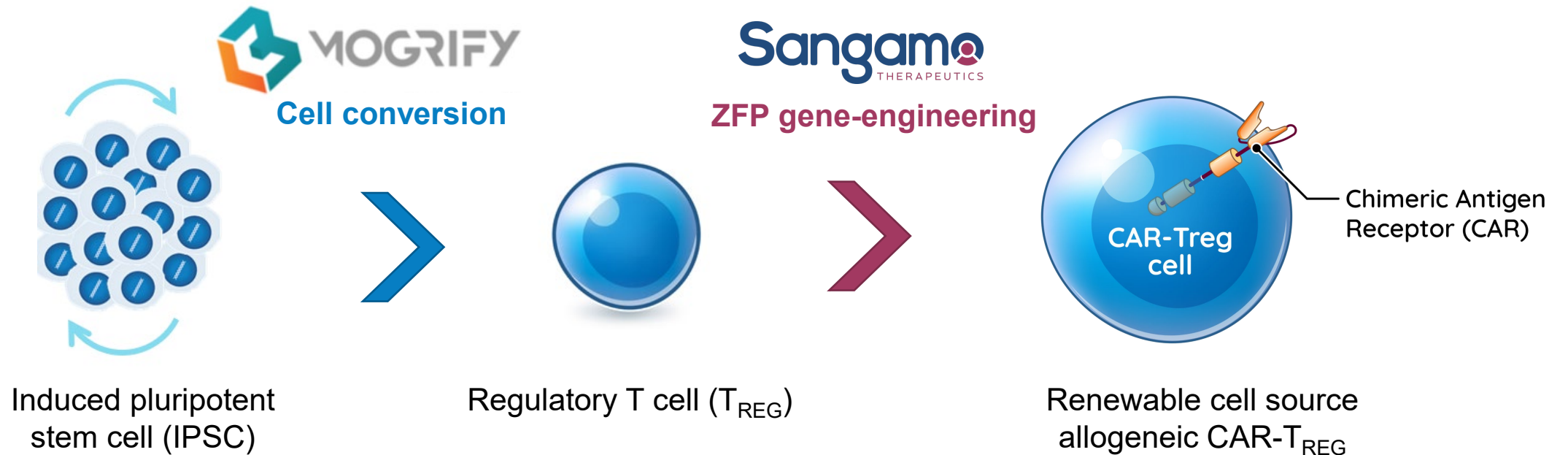
## Key outcomes from TX200 CAR-T<sub>REG</sub> program

- Data on safety and proof of concept
- Answers critical questions on CAR-T<sub>REG</sub> pharmacology and biology in patients
- Establishes CAR-T<sub>REG</sub> 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



- Collaboration expected to accelerate the development of scalable and accessible CAR-T<sub>REG</sub> cell therapies for the treatment of inflammatory and autoimmune diseases
- Complements ongoing cell therapy pipeline of CAR-T<sub>REG</sub> programs



# In-house cGMP facility and dedicated external manufacturing capacity

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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**  
S C I E N T I F I C

# Conclusions

A decorative graphic consisting of two thick, light-colored curved lines. One line is positioned higher and further to the right, curving downwards and to the left. The second line is positioned lower and further to the left, curving upwards and to the right. The lines appear to be part of a larger circular or arc-like structure.

# Key takeaways

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- 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, five validating biopharma partnerships (Biogen, Gilead, Pfizer, Sanofi, Takeda)

