



Pioneering and Delivering the Future of Genomic Medicines

December 2023

Forward-Looking Statements

This presentation contains forward-looking statements regarding our current expectations. These forward-looking statements include, without limitation, statements relating to our focus on epigenetic regulation and delivery engineering, the potential to develop, obtain regulatory approvals for and commercialize durable, safe and effective therapies to treat certain diseases and the timing, availability and costs of such therapies, the potential to use ZF, ZF-TR, CAR-Treg, SIFTER and other technologies to develop durable, safe and effective therapies, the potential for us to benefit and earn milestone and royalty payments from our collaborations and the timing of any such benefits and payments, our cell therapy strategy, including expansion to additional indications, plans and timing regarding our financial resources, including the sufficiency thereof and plans to reduce our operating expenses, the impact of our announced restructuring and Brisbane headquarter shutdown and future potential cost reductions, anticipated plans and timelines for us and our collaborators to enroll patients in and conduct clinical trials, dose and screen patients, present clinical data and make regulatory submissions, the anticipated advancement of our product candidates to late-stage development, including potential future Phase 3 trials, execution of our corporate strategy, our pipeline, the identification of additional targets, and the advancement of preclinical programs to the clinic, key milestones and catalysts, 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. Our 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 effects of the COVID-19 pandemic and the impacts of the pandemic and other macroeconomic factors, including as a result of ongoing overseas conflicts, disruptions in access to bank deposits and lending commitments due to bank failure, on the global business environment, healthcare systems and business and operations of us and our collaborators, including the initiation and operation of clinical trials; the research and development process; the uncertain timing and unpredictable results of clinical trials, including whether preliminary or initial clinical trial data will be representative of final clinical trial data and whether final clinical trial data will validate the safety, efficacy and durability of product candidates; the impacts of clinical trial delays, pauses and holds on clinical trial timelines and commercialization 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 us and our collaborators; the potential for us or our collaborators to breach or terminate collaboration agreements; the potential for us to fail to realize our expected benefits of our collaborations; the uncertainty of our future capital requirements, financial performance and results, our lack of capital resources to fully develop, obtain regulatory approval for and commercialize our product candidates, including our ability to secure the funding required to initiate a potential Phase 3 trial of ST-920 in a timely manner or at all; and our need for substantial additional funding to execute our operating plan and to operate as a going concern. There can be no assurance that we and our collaborators will be able to develop commercially viable products. These risks and uncertainties are described more fully in our Annual Report on Form 10-K for the fiscal year ended December 31, 2022, as supplemented by our Quarterly Report on Form 10-Q for the quarter ended September 30, 2023 filed with the Securities and Exchange Commission, or SEC, and future reports filed with the SEC. Forward-looking statements contained in this presentation speak only as of the date hereof, and we undertake 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.

We are a genomic medicine company dedicated to translating ground-breaking science into medicines that transform the lives of patients and families afflicted with serious neurological disease



Nav1.7 and Prion programs address high unmet needs to treat chronic neuropathic pain and prion disease. IND submission for Nav1.7 expected in 2024.



Zinc finger epigenetic regulators ideally suited for neurology disorders. Advancing both wholly-owned and partnered programs toward the clinic.



Robust AAV capsid discovery platform making strong progress expanding delivery beyond currently available intrathecal delivery capsids, including in the central nervous system.



Demonstrated track record of successful partnerships with up to \$1.9bn in potential future earnouts, including \$220m for Hem A from Pfizer.
Also seeking ways to optimize value from Fabry and CAR-Treg programs.

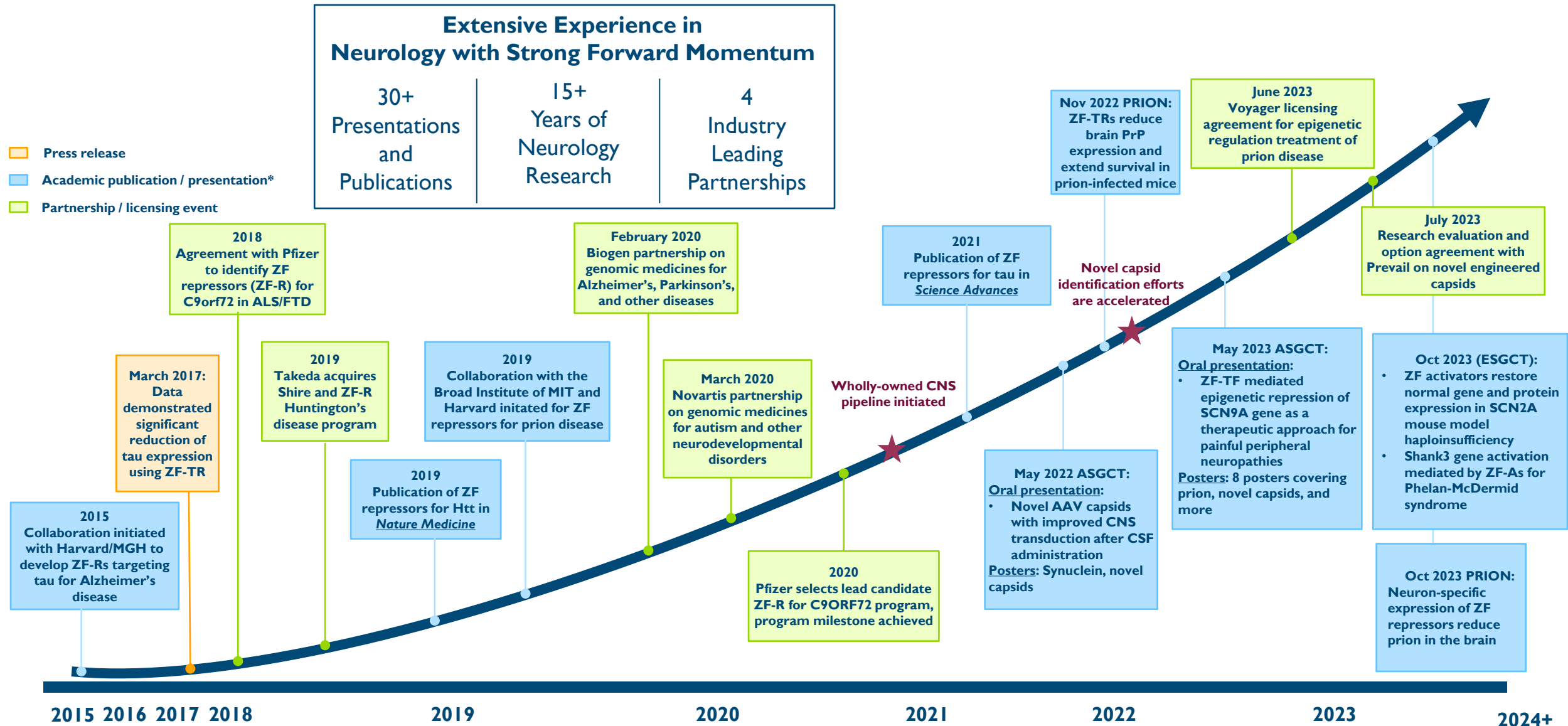
SHARPENED STRATEGIC FOCUS IN NEUROLOGY

OPTIMIZE ASSET VALUE

Company Highlights

- 1** Disciplined focus on advancing epigenetic editing cargo and AAV capsids for high-value gateway neurology diseases like chronic neuropathic pain and prion disease.
- 2** Toolbox of highly-optimized zinc finger epigenetic regulators designed for efficient delivery unlocks broader opportunities.
- 3** Proprietary AAV delivery technology with early data suggesting potentially best-in-class CNS tropism.
- 4** History of high-profile partnerships with Biogen, Novartis, Pfizer (now with Alexion) and Takeda validate payloads for neurological disease and provide access to potential non-dilutive funding.
- 5** Pfizer collaboration in Hemophilia A further substantiates AAV capabilities with late-stage royalty/milestone-bearing opportunity (\$220m potential milestones and 14-20% potential sales royalties).
- 6** Pioneers for 25+ years developing genomic medicines and zinc finger technology, with excellent track record of clinical execution.

Significant momentum building a genomic medicine franchise in neurology



Sangamo is advancing a next-generation neurology genomic medicine company by combining our expertise in epigenetic regulation and delivery engineering

Genome-Targeting Cargo

Epigenetic regulation platform



Capsid Delivery Engine

AAV capsid delivery platform

- Highly potent, specific and optimizable
- Easily packaged into AAVs
- Highly flexible - epigenetic gene repression, activation, and multiplexed approaches
- Led by Nav1.7 program in chronic neuropathic pain
- Prion disease program advancing a potential treatment for devastating and fatal disease

- SIFTER platform is advancing discovery of CNS-tropic AAV delivery capsids
- One-time administration with durable expression
- Engineered capsids for enhanced intrathecal delivery are already identified and being optimized
- Making strong progress identifying IV-delivered capsids to access previously untouched areas of the nervous system, which potentially open significant untapped indications

Neurology Genomic Medicines



High-value gateway indications to unlock broader neurology pipeline

Chronic Neuropathic Pain (Nav1.7)

Est.
>43,000
Patients
in US*

- Significant unmet need and lack of effective therapies
- Preclinical data shows potent and specific repression of Nav1.7 expression at the RNA and protein level, and the reversal of neuropathic pain
- Highly specific zinc finger (ZF) repression, with no repression of other Nav channels in preclinical experiments
- Starting with small fiber neuralgia, with potential to broaden to other neuropathic pain indications
- IND submission expected 2024

Prion Disease

Est.
1,000+
Patients
Per
Year**

- Devastating condition with completely unmet medical need – rapidly progressive and always fatal
- ZF-mediated gene repression potently reduces expression of prion in mice brains and significantly extended survival in a disease mouse model
- Potential for accelerated regulatory pathway
- IND-enabling studies are in progress

Prioritized Pipeline and Delivery Platform

Prioritized Neurology Pipeline				
Indication	Technology	Preclinical	Phase I/2	Pivotal
Chronic Neuropathic Pain (<i>Nav1.7</i>)	ZF Genome Engineering	Data presented at ASGCT 2023		
Prion Disease	ZF Genome Engineering	Data presented at Prion 2023		
Neurology (<i>undisclosed</i>)	ZF Genome Engineering			

Partnered Programs				
Indication	Technology	Preclinical	Phase I/2	Pivotal
Hemophilia A (<i>Giroctogene fitelparvec</i>)	Gene Therapy	Pfizer		
Oncology	Cell Therapy	Kite		
ALS/FTD	ZF Genome Engineering	ALEXION Autizonevri Park Disease		
Huntington's Disease	ZF Genome Engineering	Takeda		

Other Pipeline – Seeking Partnership / Investment				
Indication	Technology	Preclinical	Phase I/2	Pivotal
Fabry Disease	Gene Therapy	Anticipate presenting updated Phase I/2 data in early 2024		
Renal Transplant (<i>TX200; Auto</i>)	Treg Cell Therapy	Four patients dosed in Phase I/2		
Inflammatory Bowel Disease	Treg Cell Therapy	Data presented at ESGCT 2023		
Multiple Sclerosis	Treg Cell Therapy	Data presented at ESGCT 2023		

Multiple biopharma collaborations demonstrate the platform's potential and provided significant economics for Sangamo

Gene Therapy



Genome Engineering



Cell Therapy



Numerous Benefits of Partnerships:

Large Pharma buy-in validates the science

Provides potential non-dilutive capital to advance pipeline

Leverages partner domain expertise

Promotes optimal resource allocation to advance late-stage clinical development

\$817m

cash received from partners to date

Up to \$1.9b

in potential future milestones and exercise fees assuming exercise of all options and targets

Additional potential product royalties



3Q23 Business Update

3Q23 Key Takeaways

Pipeline prioritization and sharpened strategic focus



Announced strategic transformation to become a neurology-focused genomic medicines company. Focusing resources on neurology-optimized proprietary epigenetic regulation programs and novel capsid delivery engine.



Announced planned shutdown of Brisbane headquarters, restructuring of operations, and US workforce reduction of approximately 40% to reduce operating expenses.

Fabry Disease

- Deferring additional investments in Phase 3 planning until collaboration partner or Phase 3 trial funding is secured.
- Expect to complete dosing of the remaining enrolled Phase 1/2 STAAR study patients in the first half of 2024.

CAR-Tregs

- Received necessary approvals for accelerated dosing protocol for Phase 1/2 STEADFAST study from European regulatory authorities.
- Deferring new investments in the CAR-Treg cell therapy programs until collaboration partner or external investment is secured.



Financial Highlights

- Approximately **\$132 million in cash, cash equivalents, and marketable securities** as of September 30, 2023 which, in combination with cost savings from the restructuring, workforce reduction and other potential cost reductions, we believe will be sufficient to fund planned operations into 3Q 2024.
- Restructuring activities and other potential cost reductions expected to reduce our **non-GAAP operating expenses** by approximately 50%.
- Continue to actively explore ways to raise additional capital.



Q3 Pipeline Progress & Anticipated Milestones

NEUROLOGY EPIGENETIC REGULATION

- ✓ Progressed IND-enabling activities for Nav1.7 program for chronic neuropathic pain.
- ✓ Presented updated preclinical data from [prion disease program](#) at the Prion 2023 Conference.
- ✓ Presented preclinical data showing zinc finger activators in [SCN2A](#) and [Shank3](#) programs at ESGCT 2023.
- IND submission for Nav1.7 expected in 2024.
- Anticipate sharing nonhuman primate data from engineered AAV capsid development efforts in early 2024.

HEMOPHILIA A (PFIZER)

- ✓ Dosing complete in Phase 3 AFFINE trial.
- ✓ Updated Phase 1/2 ALTA data presentation at ASH, December 11, 2023.
- Pivotal data read-out expected in mid-2024.
- BLA and MAA submissions anticipated in second half of 2024.

FABRY DISEASE

- ✓ Dosed an additional three patients in Phase 1/2 STAAR study to achieve a total of 25 patients dosed.
- ✓ All patients dosed to date continue to demonstrate sustained, elevated α -Gal levels for up to 3 years' follow-up.
- ✓ Received U.S. FDA Regenerative Medicine Advanced Therapy (RMAT) Designation.
- Expect to complete dosing of the remaining enrolled Phase 1/2 STAAR study patients in the first half of 2024.
- Anticipate presenting updated Phase 1/2 data at a medical meeting in early 2024.

CARTREG IMMUNE REGULATION

- ✓ Received necessary approvals for accelerated dose escalation protocol for Phase 1/2 STEADFAST study.
- ✓ Dosed first patient in second dose cohort. Manufactured dose for cohort three and four patients.
- ✓ Presented preclinical data on [MOG-CAR-Tregs](#) in multiple sclerosis, and [IL23R](#) in Crohn's disease at ESGCT 2023.
- Expect to dose patient in third cohort in 4Q 2023 and first patient in fourth cohort in January 2024.
- Plan to provide an update on discussions with potential investors in 1Q 2024 and Phase 1/2 clinical data by mid-2024.

Our financial resources are focused on pipeline progression and value creation

Key Q3 Financial Metrics

~\$132m

Cash and Marketable Securities Balance as of 9/30/23

\$817m

Cash Received from Partners to date

Up to \$1.9bn

In potential future milestones and exercise fees, assuming exercise of all options and targets

Up to \$220m

in potential milestone payments from Hemophilia A[†] plus 14-20% in potential sales royalties

Financial Guidance

\$9.4m

Revenues – Q3 2023

\$64.8m*

Non-GAAP OpEx – Q3 2023

\$240 – \$260m (2023)

\$115 – \$135m (2024)

Reiterated Non-GAAP OpEx Guidance excludes certain non-cash charges as noted below**

Our resources are tightly allocated in line with our business priorities



Zinc Finger Genomic Engineering for Neurology

Sangamo's differentiated zinc finger (ZF) genomic engineering platform



Versatile, modular, customizable

Flexible configuration and multiple functionalities



High activity and specificity

Tunable and optimizable DNA:protein interface



High-resolution targeting

Genome-wide coverage, no restrictions

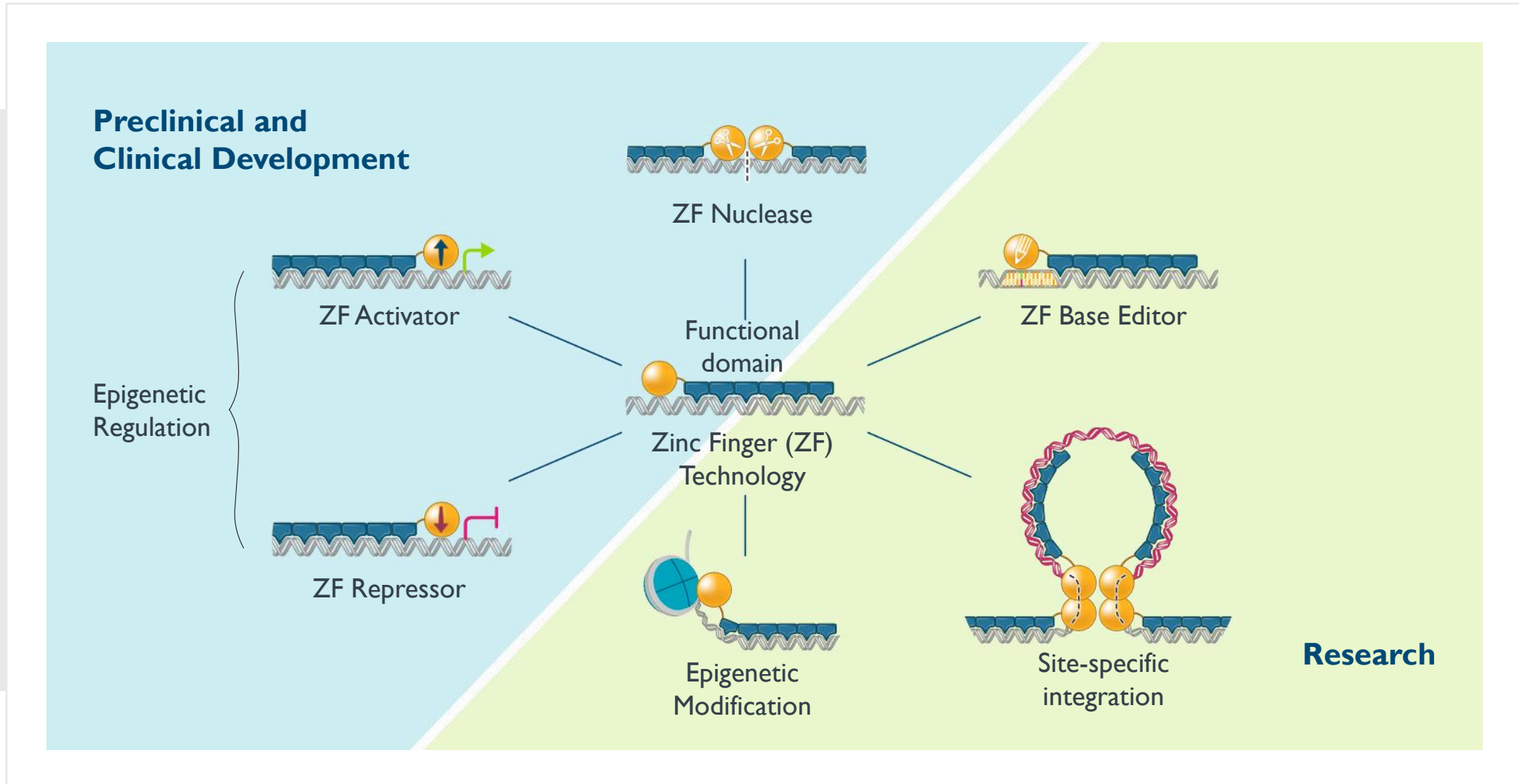


Compact

Improved delivery vector compatibility and genome accessibility



A diverse set of functional domains can be appended to the platform, creating tools with specificity for the needs of each target



— We can repress or activate the expression of target genes without the introduction of mutations, breaks or other permanent changes to the genome

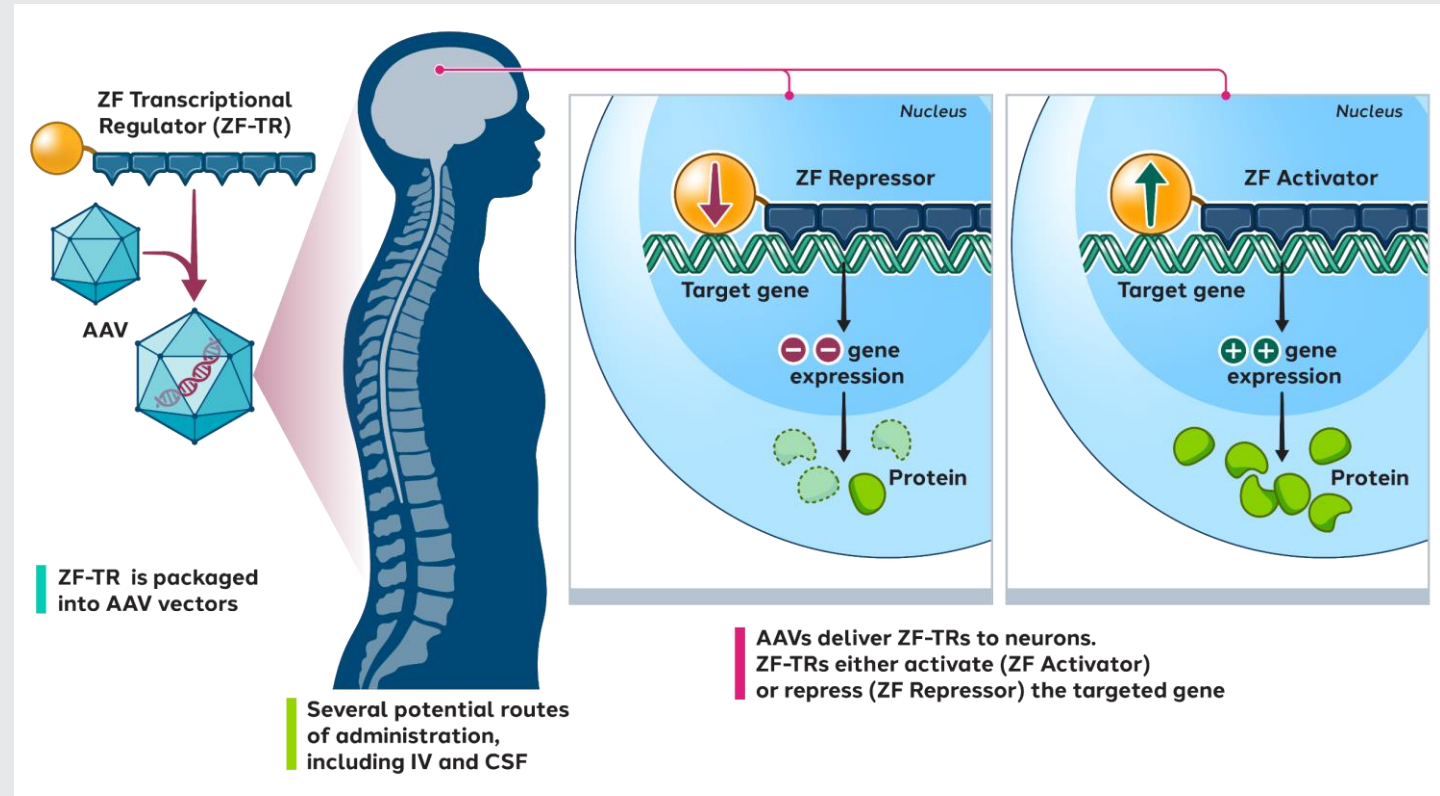
ZF transcriptional regulators can be designed to:

ZF repressors

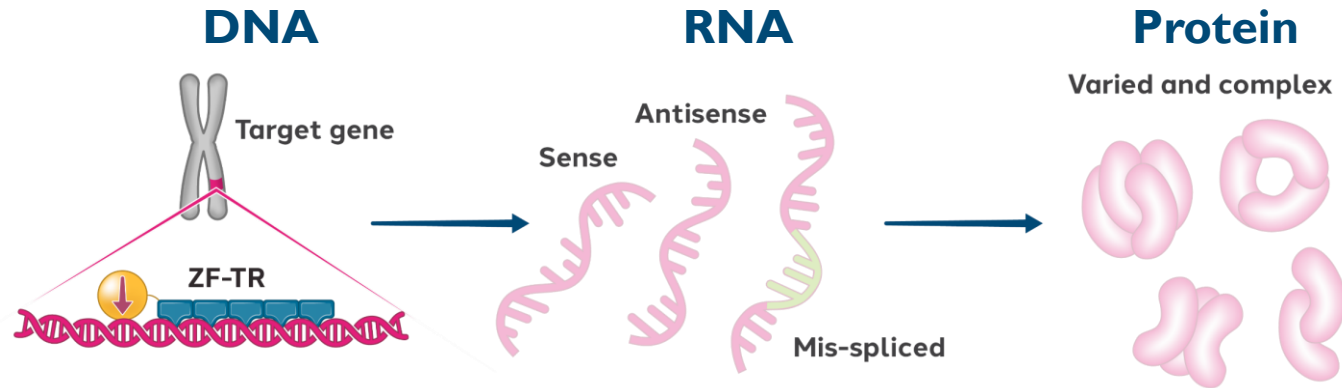
- Reduce the expression of a pathogenic gene
- Selectively repress expression of a mutant allele while allowing for the expression of the healthy allele

ZF activators

- Activate the expression of genes that are inadequately expressed



Transcriptional regulators target the genomic source of diseased mRNA and protein for maximal efficacy



Gateway Neurology Indications

NEUROPATHIC PAIN

Nav1.7

Sangamo
THERAPEUTICS

PRION DISEASE

Prion

Sangamo
THERAPEUTICS

NEUROLOGY
(paused pending capsid)

Tau, Synuclein,
Shank3, Scn2a, DMPK,
multiple undisclosed

Sangamo
THERAPEUTICS

ALS

C9orf72

ALEXION

HUNTINGTON'S

Huntington

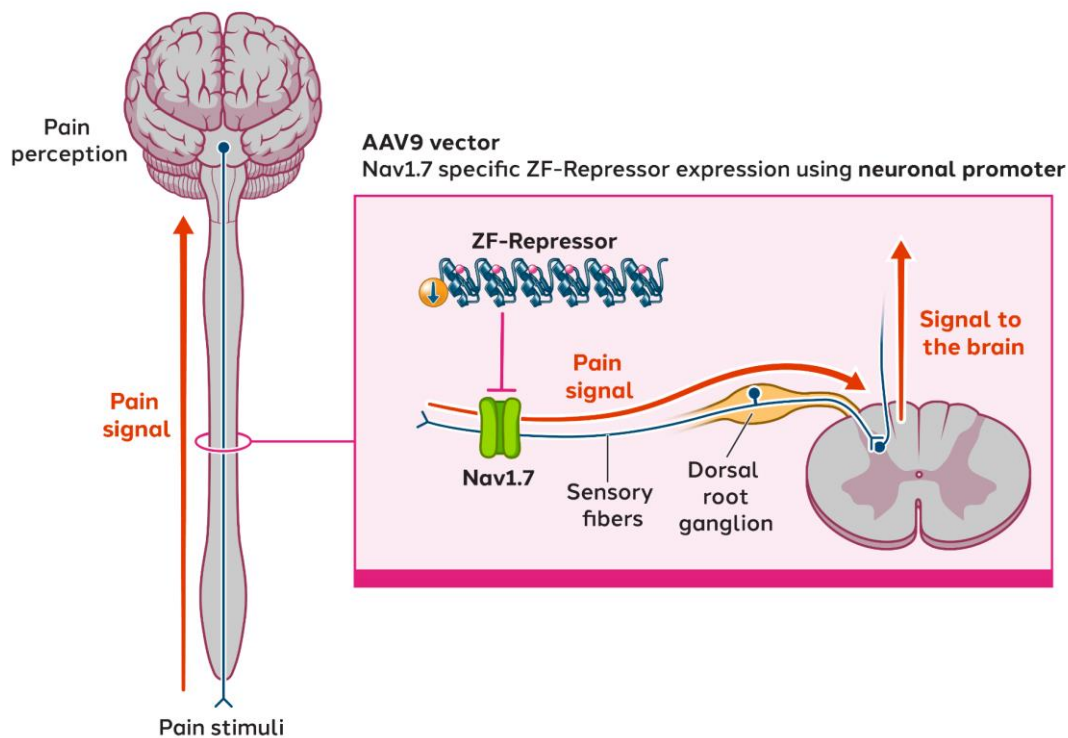
Takeda



Initial Focus on Two High Value Neurology Indications: Nav1.7 & Prion Disease

Nav1.7 specific Zinc Finger repression is ideally suited to treat neuropathic pain

Given the high unmet need and lack of effective treatments, there is an urgent need to develop novel therapeutics for the treatment of chronic neuropathic pain

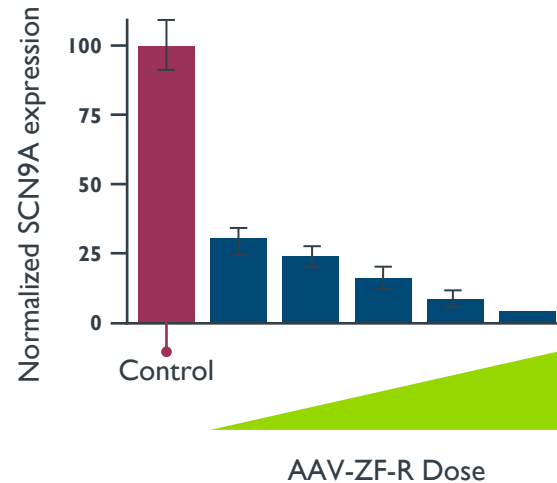


- Nav1.7 is a voltage gated sodium channel expressed in the Dorsal Root Ganglion (DRG)
- Alterations in Nav1.7 activity **directly regulate pain levels** in several genetic disorders
- Blocking Nav1.7 in the DRG is expected to prevent the **transmission of nociceptive pain signals** to the brain
- This allows us to target multiple **neuropathic pain indications**, regardless of the cause of the pain
- Reducing pain by inhibiting Nav1.7 is not predicted to be associated with **any neurological side effects**

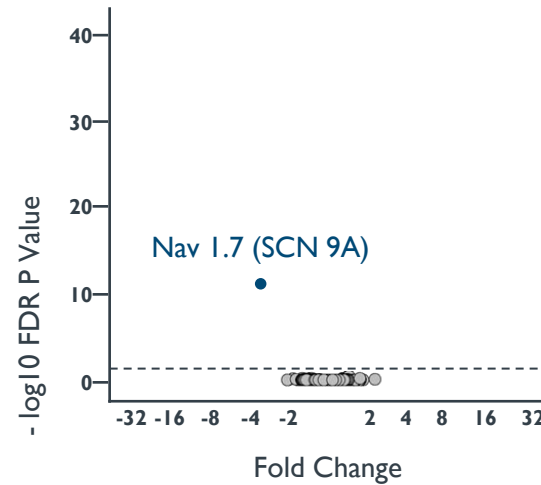
Zinc finger repressors potently reduce Nav1.7 in human neurons with exquisite specificity



Nav1.7 (SCN9A) expression



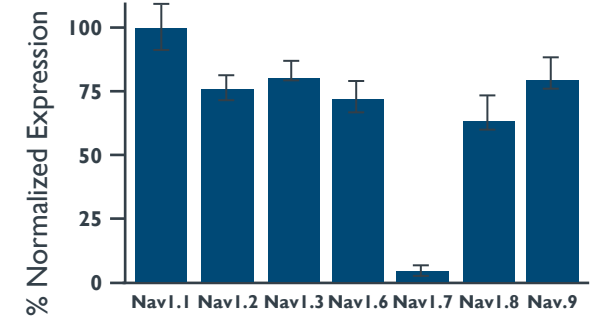
Global genomic analysis



Differential expression of 20,000 genes was evaluated

- Genes down-regulated
- Genes up-regulated
- SCN9A

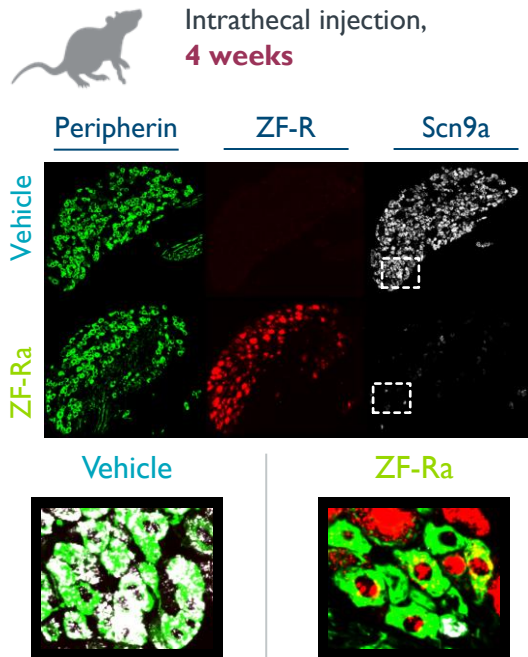
ZF repressors (ZF-Rs) specifically repress human Nav1.7 without impacting other sodium channels



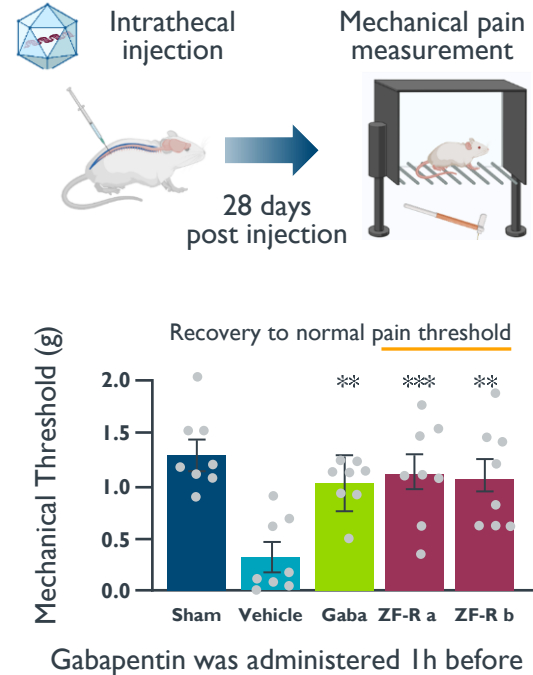
Nav1.7 repressors reverse neuropathic pain in preclinical models

Results support IND-enabling GLP Toxicology studies which are in progress

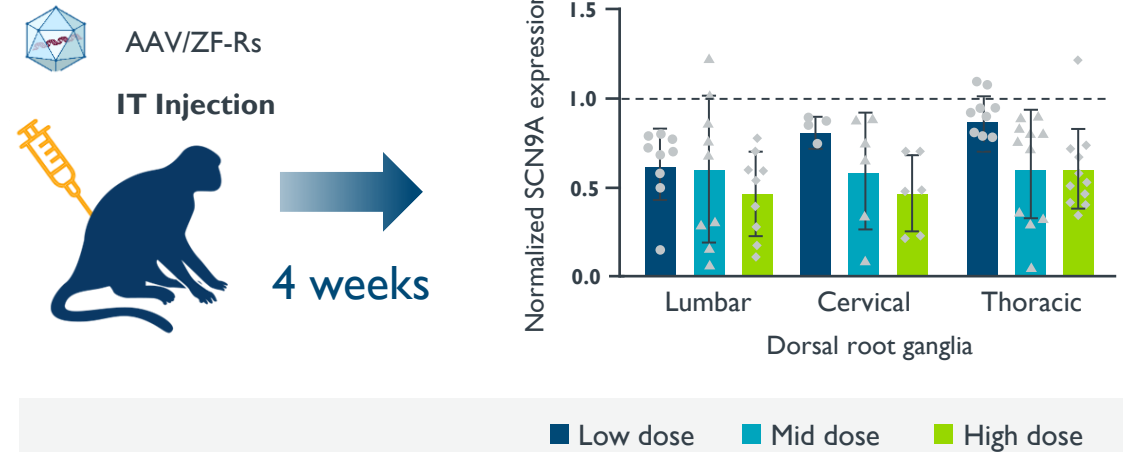
Potent Scn9a mRNA repression in mouse DRG neurons



Full restoration of healthy sensitivity to mechanical pain



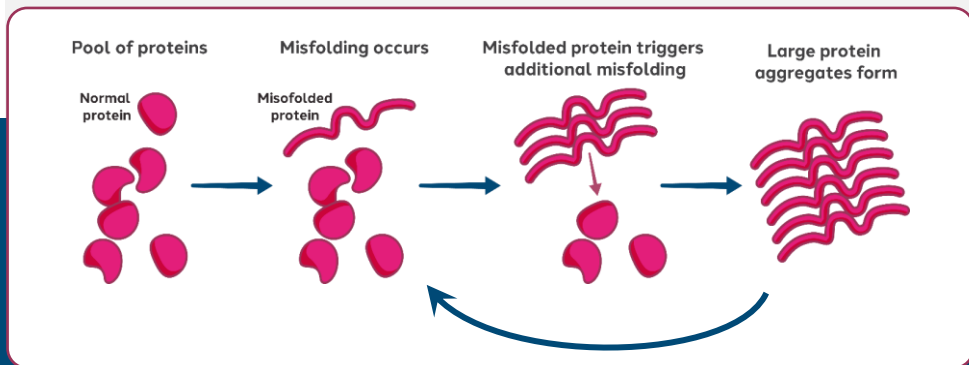
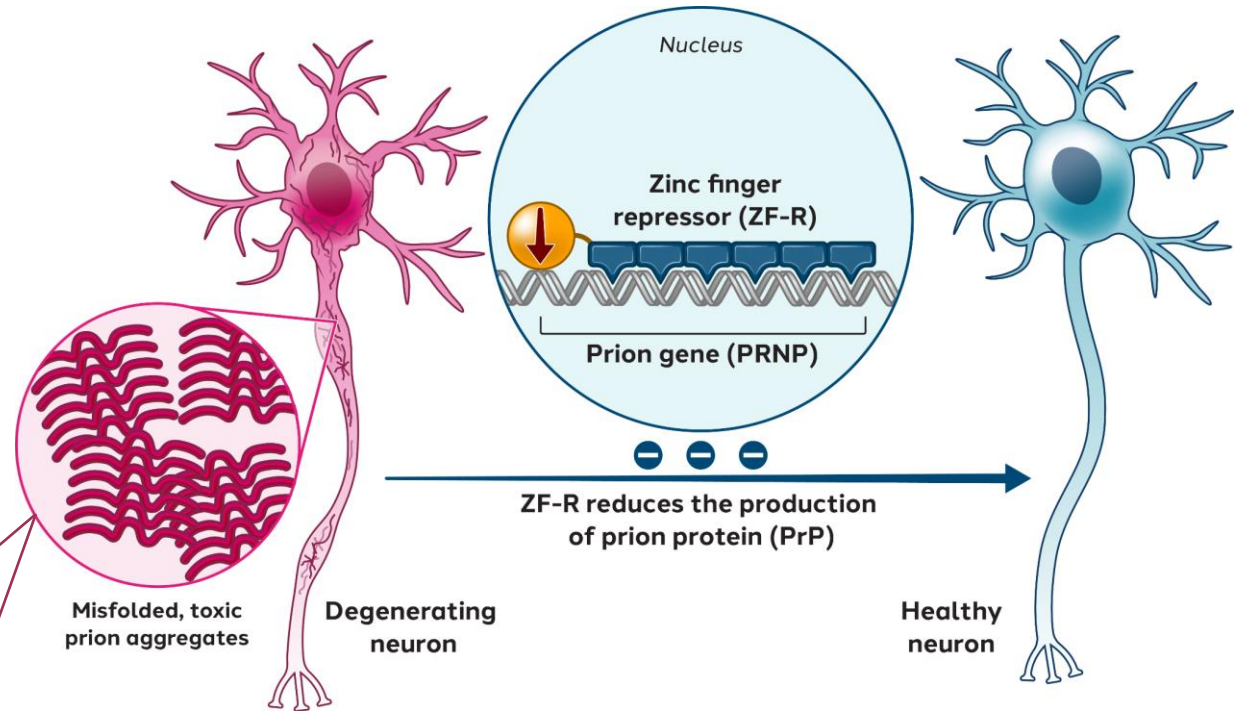
ZF-Rs were well tolerated in nonhuman primates



- ZF-Rs were well tolerated across a 100x dose range
- No clinical signs of toxicity
- No adverse macroscopic or microscopic findings

Zinc finger-mediated gene repression is also ideally suited to the treatment of prion disease

- Progressive, with no disease modifying therapy
- Sporadic, inherited and acquired forms
- Spectrum of symptoms can include cognitive, psychiatric, and motor deficits
- Excellent fit for a ZF repression approach
 - Prion knockout animals do not get disease
 - Prion reduction can delay or prevent disease
 - Neuronal PrP reduction prevents disease



Repression of prion expression in the brain may slow or halt disease progression and neurodegeneration

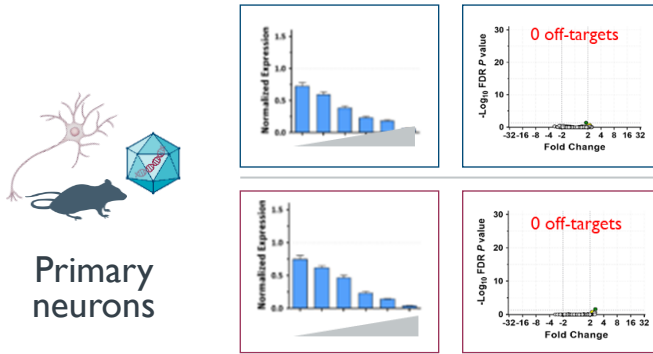
Maddox et al, 2020
Mead et al, 2022

Bueler et al., 1993
Fischer et al., 1996

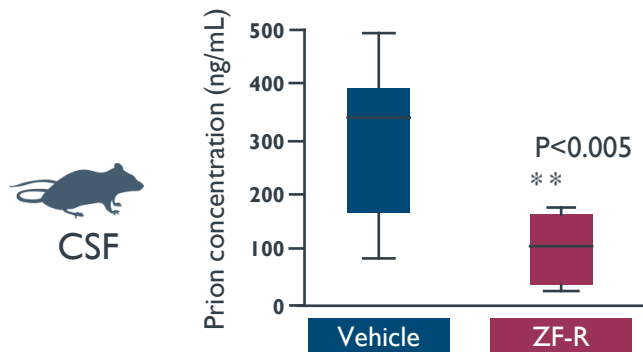
Mallucci et al., 2003
Safar et al., 2005

Zinc finger repressors extend survival in a mouse model of aggressive prion disease

Potent and specific ZF-Rs



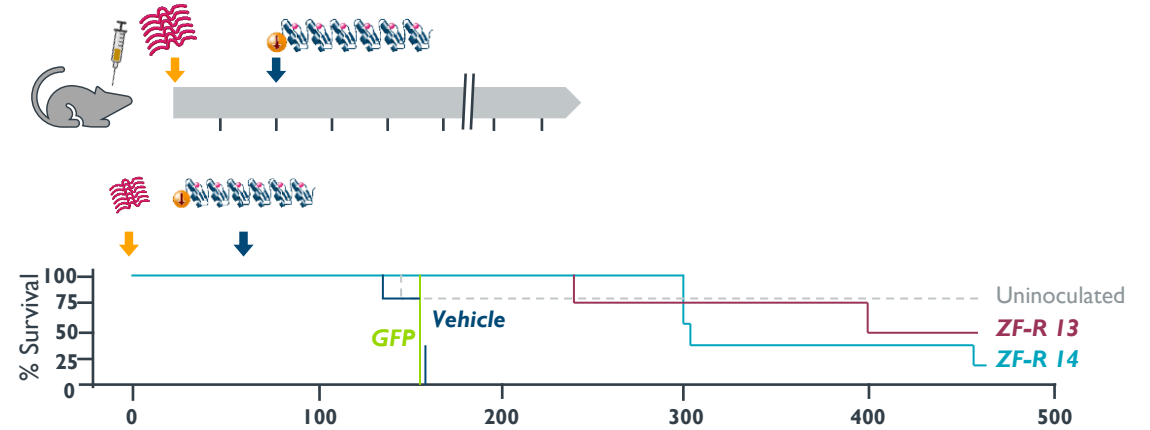
Reduction of CSF biomarker



Remarkable in vivo efficacy

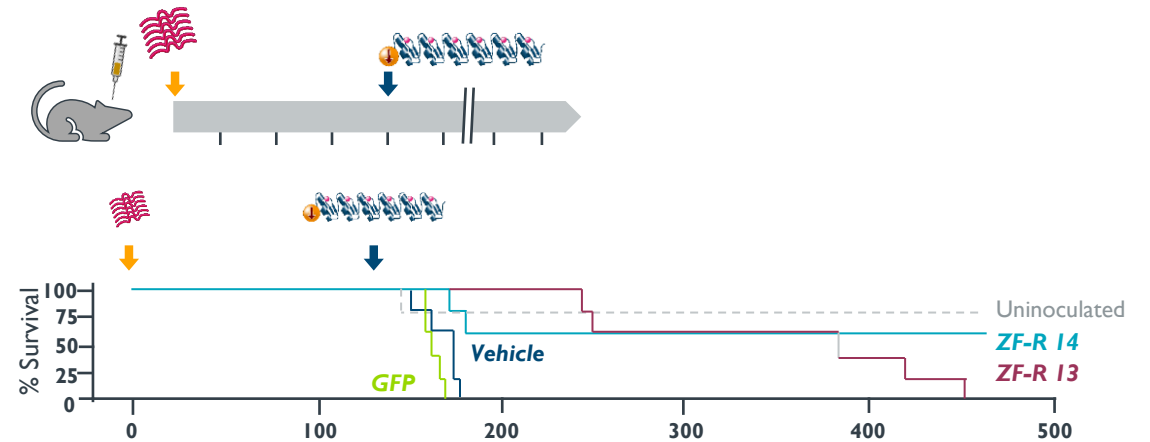
ZF-R
@ 60dpi

ZF-R
60 dpi



ZF-R
@ 122dpi

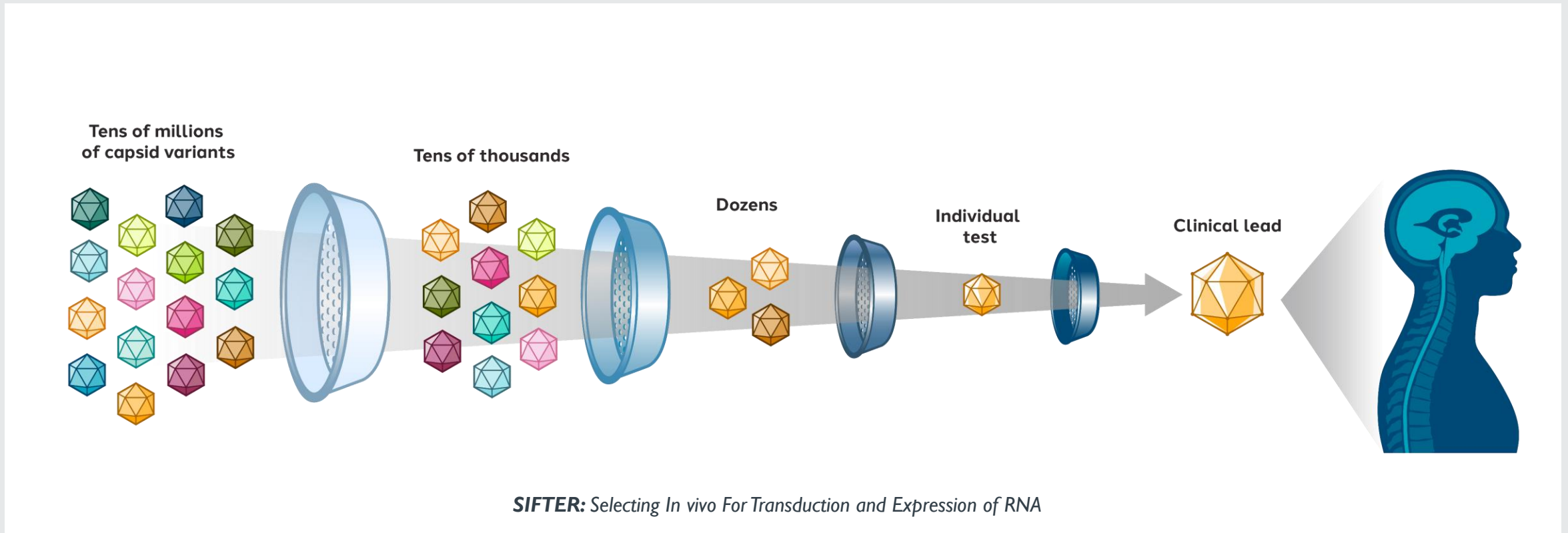
ZF-R
122 dpi





Capsid Delivery Engineering Capabilities

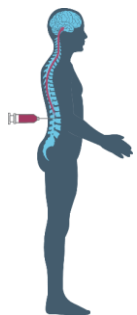
Our SIFTER platform enables selection of CNS-tropic AAV capsids to advance our innovative preclinical programs to the clinic



The Sangamo SIFTER platform is delivering high value neurotropic AAV capsids, with intravenous delivery data expected Q1 2024

Engineered capsids for cerebrospinal fluid delivery

Lead capsids characterized in non-human primates



10-100x higher neuronal transgene expression compared to AAV9



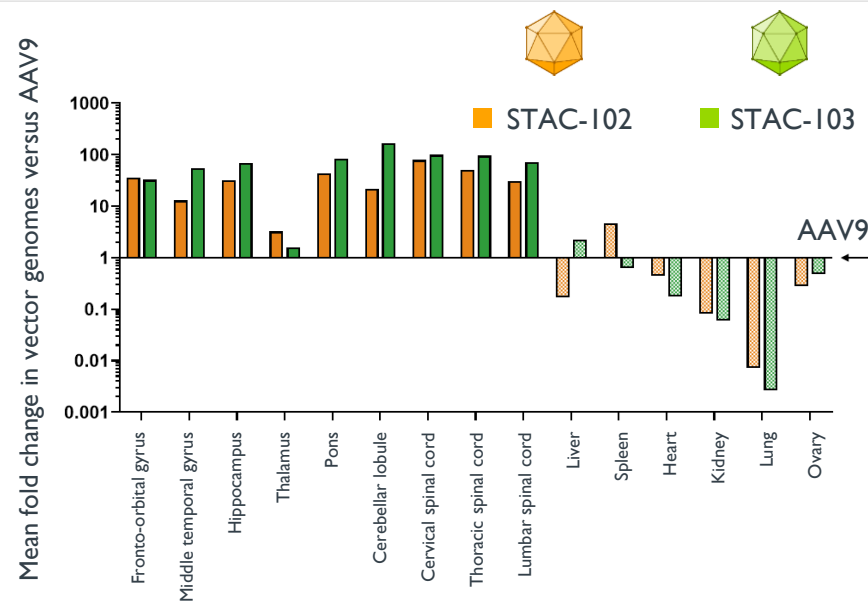
Assessed manufacturing scale-up and critical quality attributes



10-100x higher CNS vector genome delivery than AAV9 and decreased peripheral distribution



Demonstrated on-target pharmacology with minimal safety signal



Engineered capsids for intravenous delivery

Lead capsid assessments progressing well



Multiple screening campaigns are in progress utilizing a diverse panel of AAV libraries

Bioinformatic analysis of data has identified capsids with high CNS enrichment relative to AAV9

STAC = Sangamo Therapeutics AAV Capsid

Individual capsid assessment in non-human primates ongoing. Read-out expected Q1 2024.

Exciting Indications Could be Unlocked with a Neurotropic Capsid

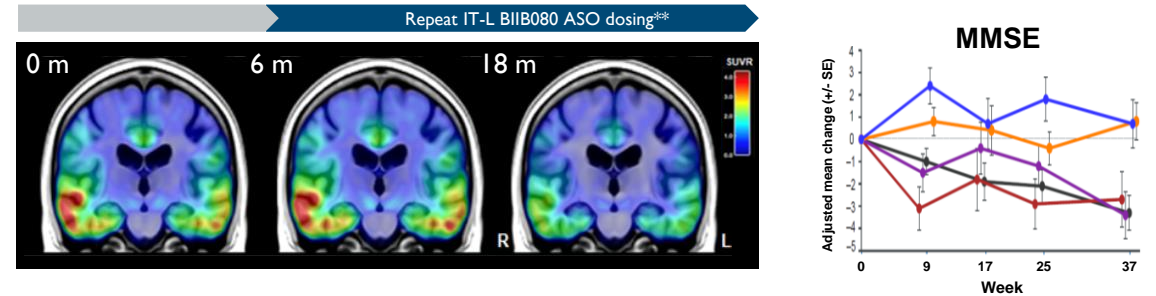


Many neurodegenerative diseases are driven by tau pathology. Lowering expression of *MAPT* may slow or halt neurodegeneration in humans.

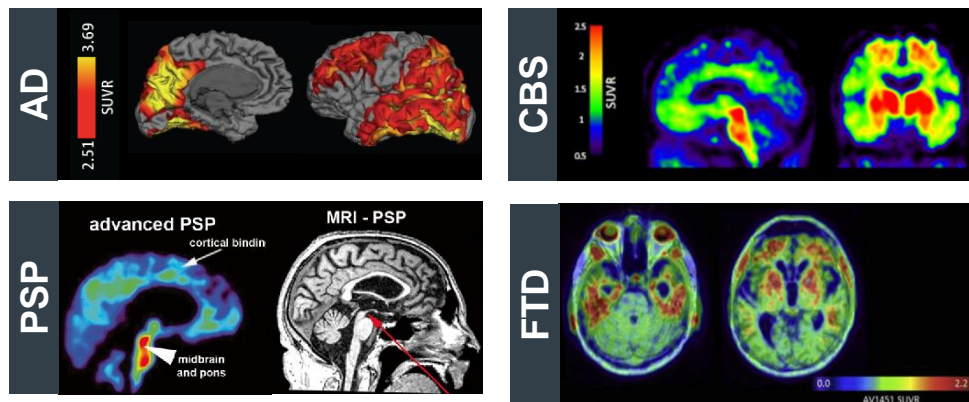
Tauopathy disorders represent a **huge unmet need with millions affected** spanning over a dozen distinct indications, including:

- AD – Alzheimer’s disease
- PSP – Progressive supranuclear palsy
- FTD – Frontotemporal dementia
- CTE – Chronic traumatic encephalopathy
- CBS – Corticobasal syndrome
- LBD – Lewy body disease (+ alpha synuclein)

Lowering *MAPT* expression **can reverse established tau pathology** and potentially halt AD progression in humans*



Different tau forms in different brain regions cause different tauopathies



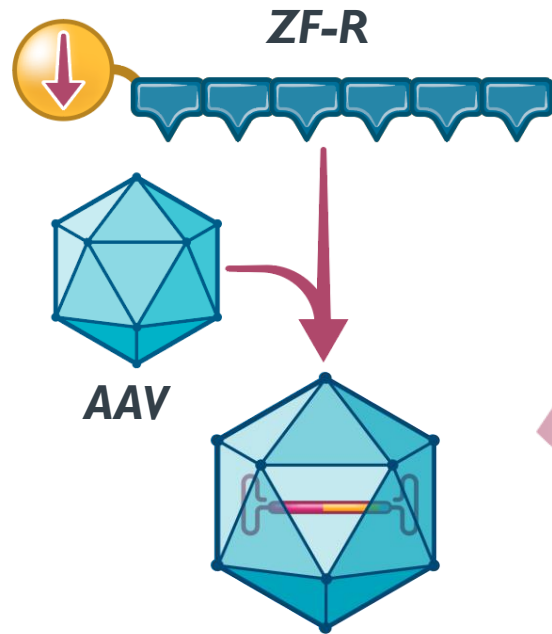
Sangamo’s approach is differentiated in several important ways

- All tau forms targeted at the source, inside neurons
- One-time, IV administration
- All brain regions = all tauopathy indications
- Cell-type specificity, restricted to CNS cell types
- Rapid effect, 100% potency at single-cell level

	ZF-R	ASO
Icon		
All tau forms targeted at the source, inside neurons	✓	✓
One-time, IV administration	✓	✗
All brain regions = all tauopathy indications	✓	✗
Cell-type specificity, restricted to CNS cell types	✓	✗
Rapid effect, 100% potency at single-cell level	✓	✗

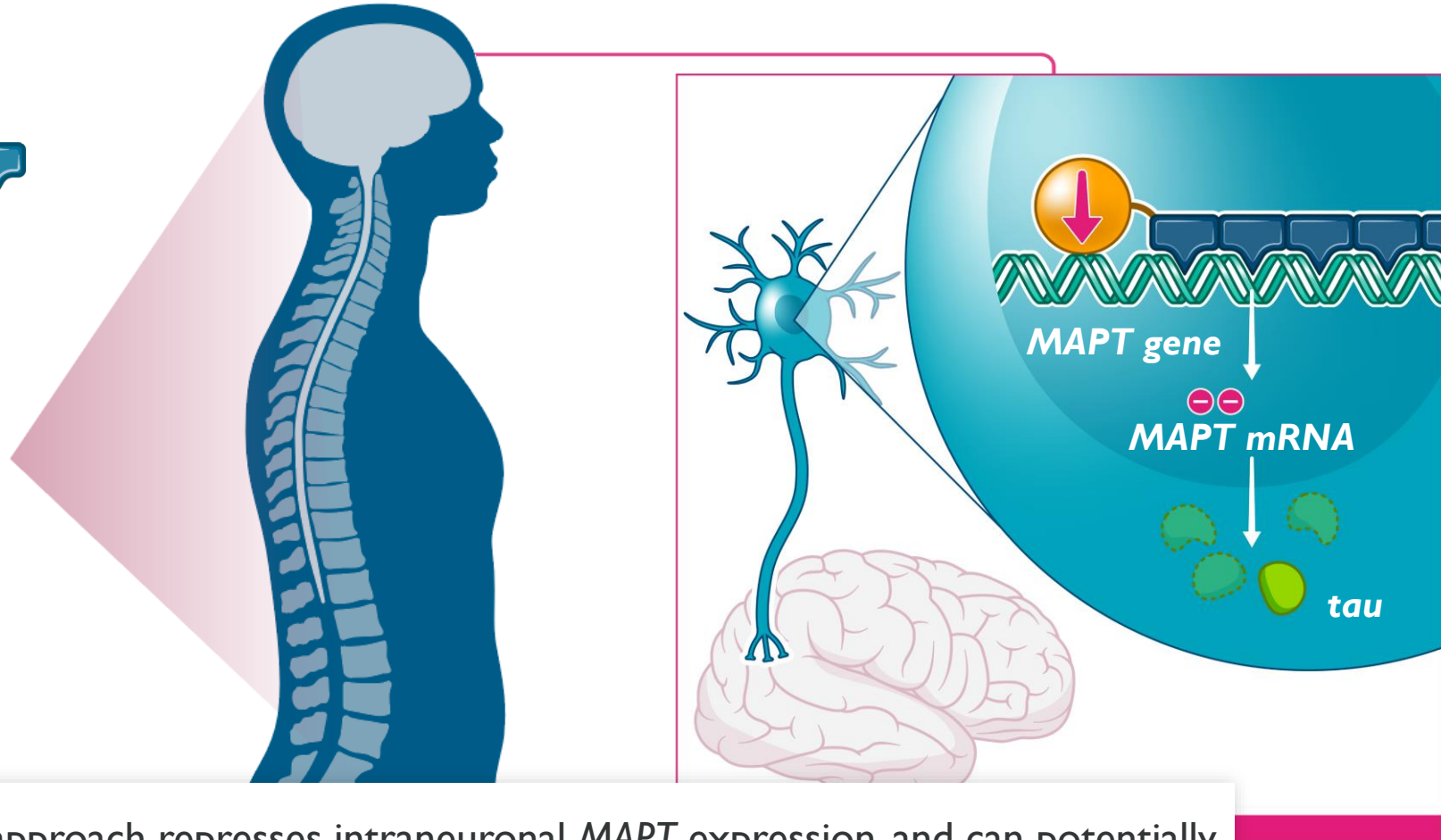
Zinc finger-mediated epigenetic repression of *MAPT* for Tauopathies

ZF repressor cassette
packaged into AAV vector



One-time IV
administration 

Stable tau reduction
in neurons the brain



Our ZF-R approach represses intraneuronal *MAPT* expression, and can potentially prevent and reverse tau pathology and neurodegeneration in tauopathy models

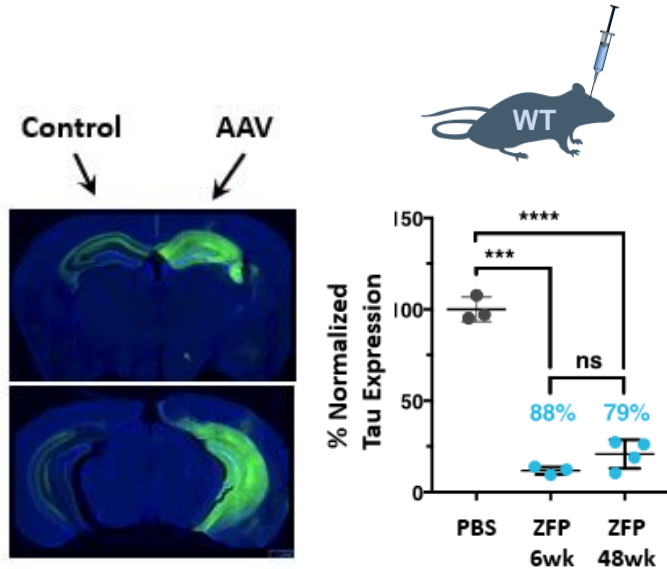
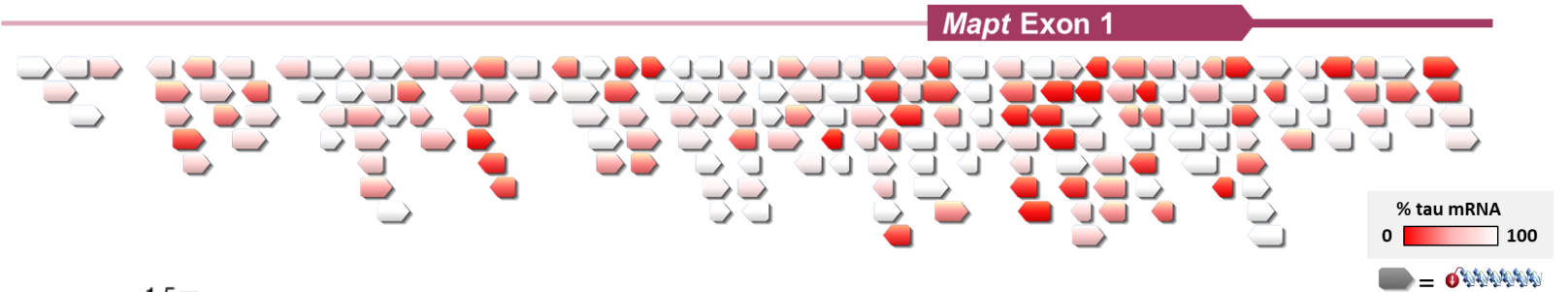
Potent, long-lasting, specific repression of mouse *MAPT*

SCIENCE ADVANCES | RESEARCH ARTICLE

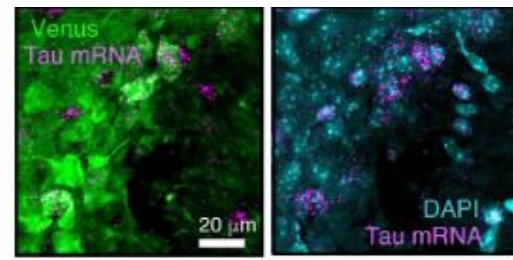
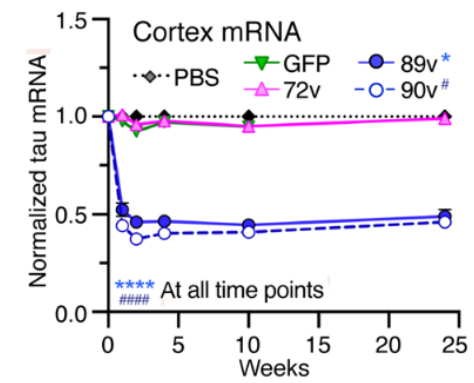
NEUROSCIENCE

Persistent repression of tau in the brain using engineered zinc finger protein transcription factors

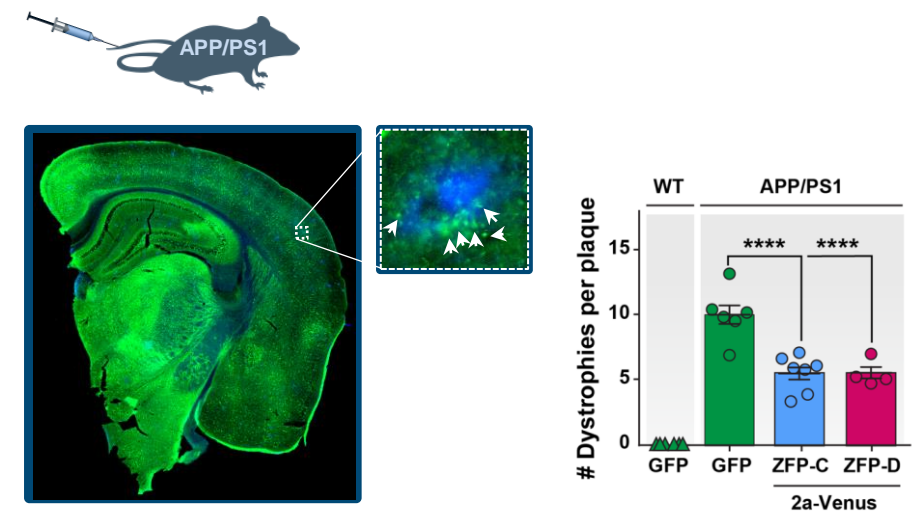
Susanne Wegmann^{1,2*}, Sarah L. DeVos^{1*}, Bryan Zeitler^{3,4*}, Kimberly Marlen³, Rachel E. Bennett¹, Marta Perez-Rando¹, Danny MacKenzie¹, Qi Yu³, Caitlin Commins¹, Riley N. Bannon^{1†}, Blanca T. Corjuc¹, Allison Chase¹, Lisa Diez², Hoang-Oanh B. Nguyen³, Sarah Hinkley³, Lei Zhang³, Alicia Goodwin³, Annemarie Ledebner³, Stephen Lam³, Irina Ankoudinova³, Hung Tran³, Nicholas Scarlott³, Rainier Amora³, Richard Surosky³, Jeffrey C. Miller³, Ashley B. Robbins¹, Edward J. Rebar^{3,6}, Fyodor D. Urnov³, Michael C. Holmes³, Amy M. Pooler³, Brigit Riley³, H. Steve Zhang^{3†}, Bradley T. Hyman^{1†}



Sustained repression out to 11+ months in the mouse hippocampus



Rapid, potent repression of mouse tau at the bulk and single-cell level

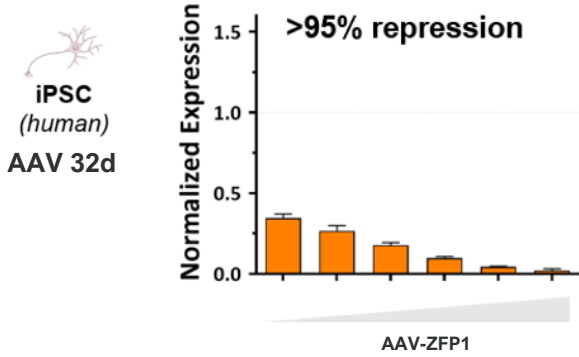


50% reduction of dystrophic neurites in APP/PS1 mice following IV delivery

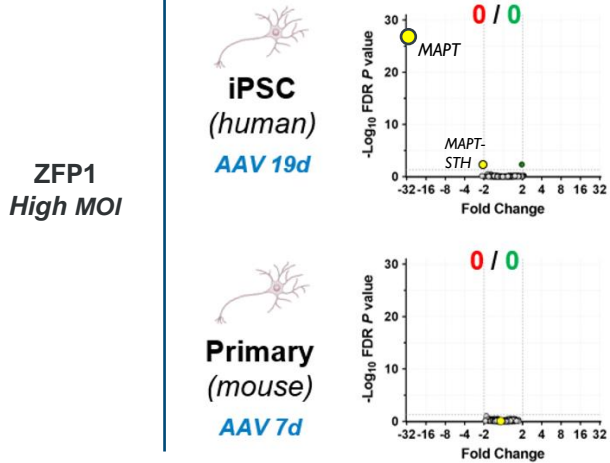
Potent, highly specific repression of human *MAPT* in vitro and in vivo

In vitro: activity and specificity

Potent human *MAPT* repression



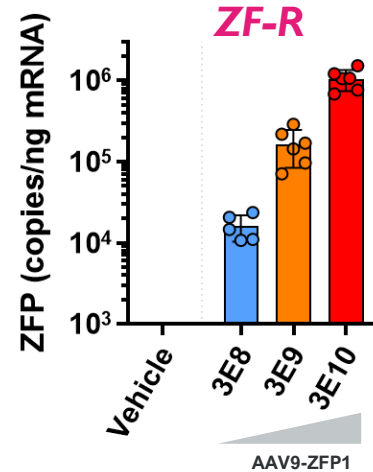
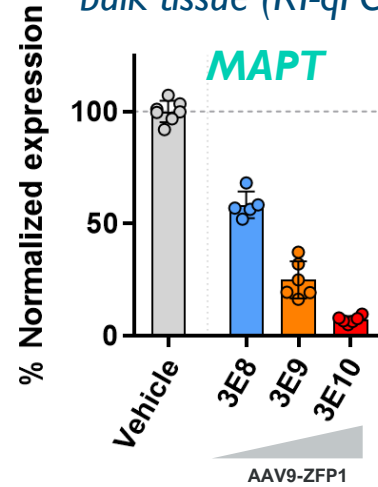
No off-target genes



Mouse brain tissue



Potent human tau repression in bulk tissue (RT-qPCR)

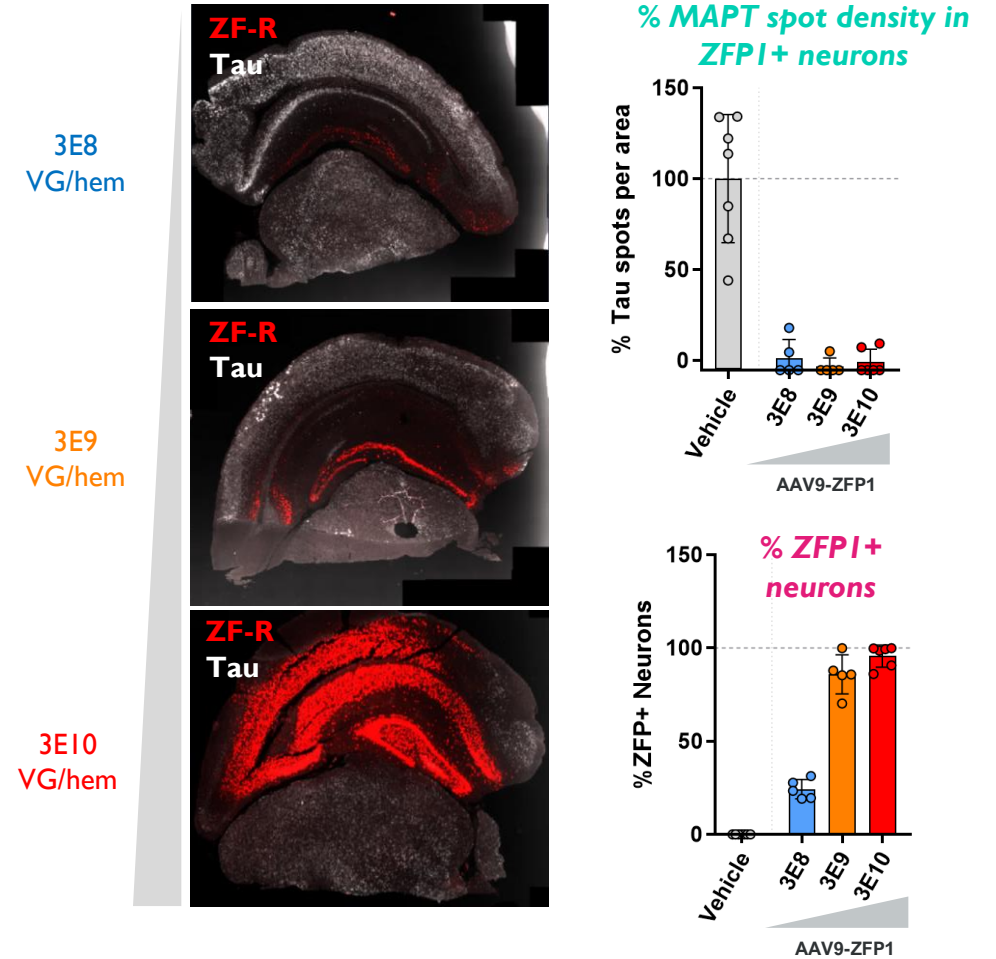


MAPT levels normalized to ATP5B/EIF4A2

Single-cell quantitation in mouse brain



Potent human tau repression in neurons (RNAscope)



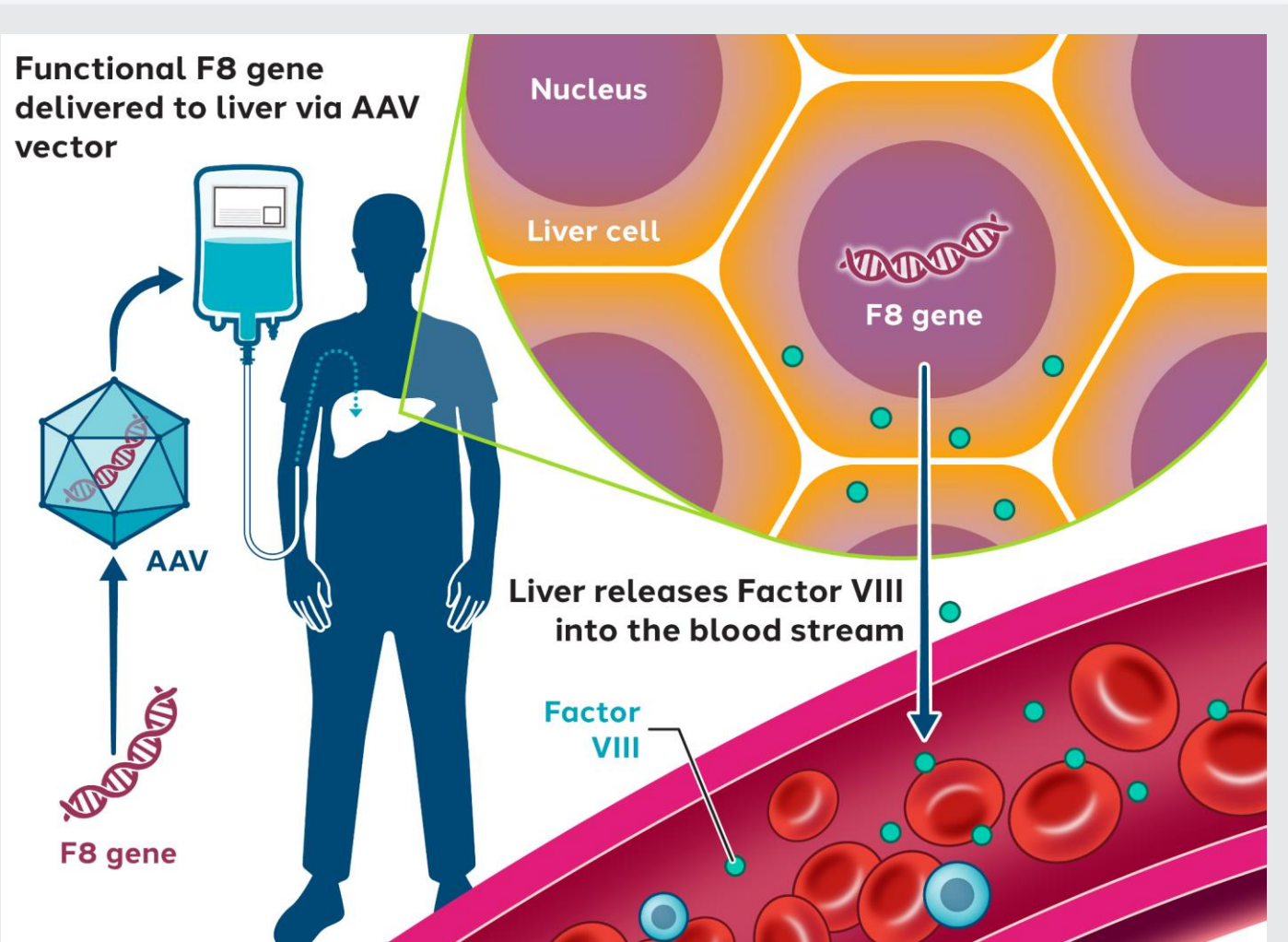
N=11,379 average cells / mouse

Optimizing Value of Clinical Programs



Hemophilia A: giroctocogene fitelparvovec (Pfizer)

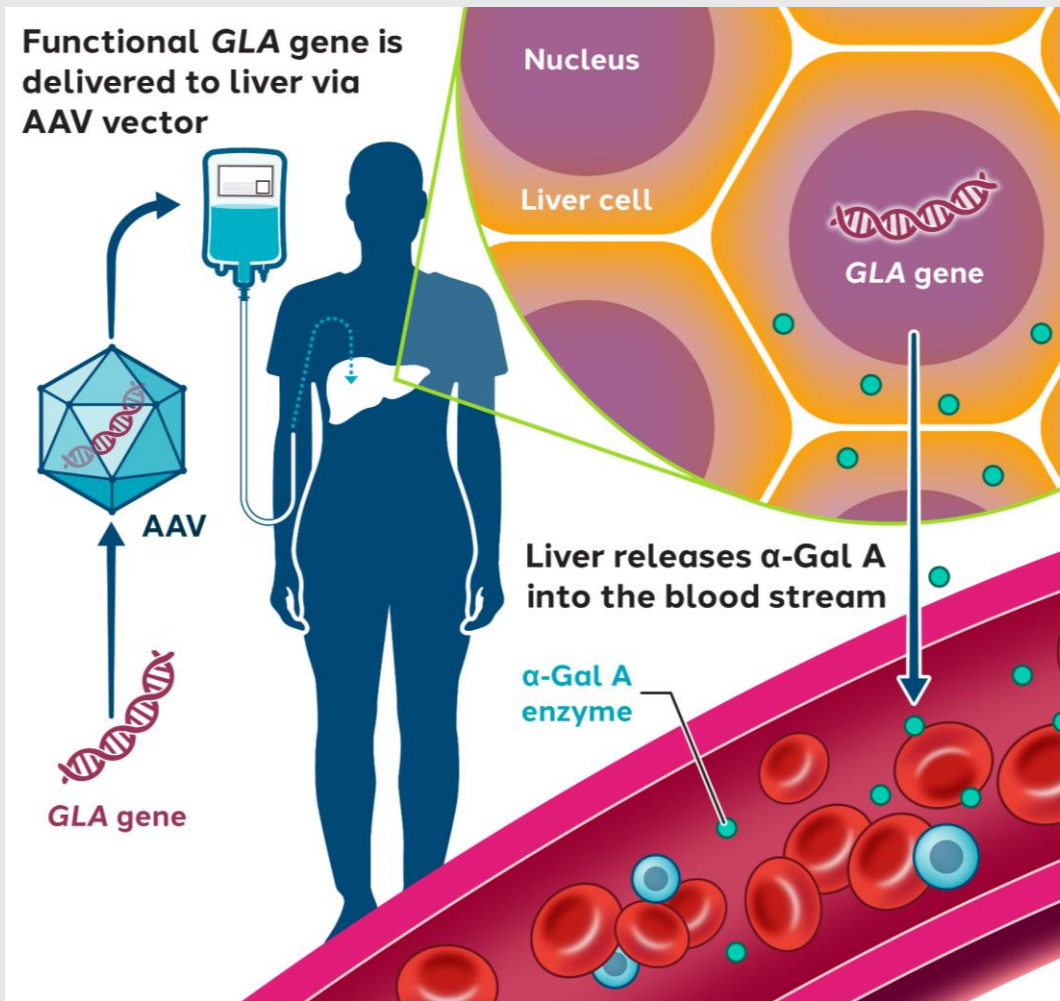
Progressing toward pivotal readout for Phase 3 AFFINE trial



- Program transitioned to Pfizer for Phase 3 development.
- Dosing in Phase 3 AFFINE trial is complete.
- Pivotal readout expected mid-2024.
- BLA and MAA submissions anticipated 2H 2024.
- Potential to generate up to **\$220 million in remaining milestone payments***, and **14-20% royalties on future product sales if approved****

Fabry Disease: isargalgagene civaparvovec (ST-920)

Seeking collaboration partner or Phase 3 financing



- Dosed 25 patients in Ph I/2 STAAR study in Fabry disease.
- All patients dosed to date continue to demonstrate sustained, elevated α -Gal levels for up to 3 years-follow up.
- Received U.S. FDA RMAT Designation.
- Expect to complete dosing of remaining enrolled Ph I/2 patients in 1H24.
- Deferring additional investments in Phase 3 planning until collaboration partner or Phase 3 trial funding is secured.

Sustained, elevated expression of **α -Galactosidase A** (α -Gal A) activity in all patients, with 12 patients having ≥ 1 year follow-up, including longest treated patient with **3 years follow-up**.

All 11 participants who were withdrawn from enzyme replacement therapy (ERT) **remain off ERT, for up to 24 months**.

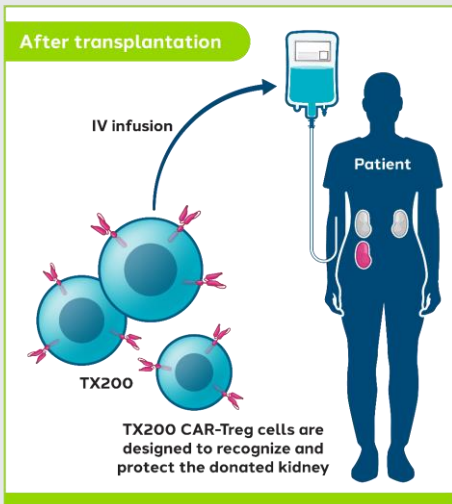
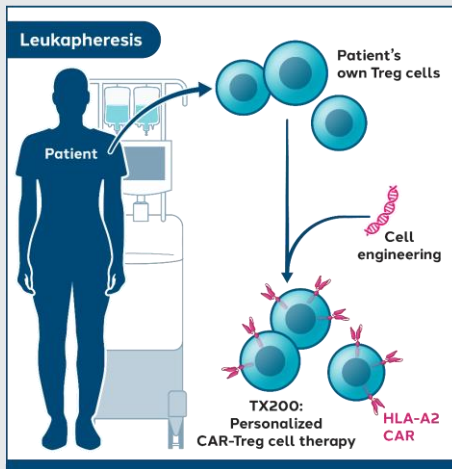
Clearance or stabilization of **renal Gb3 inclusions** along with reductions in **urine podocyte loss** suggests a favorable impact on progression of Fabry nephropathy and tissue absorption.

Patients on ST-920 continue to report **improvements in their quality of life**, even over and above the benefits they were experiencing on ERT.

No requirement for prophylactic corticosteroids or other immune modulating agents.

CAR-Treg cell therapy

Seeking collaboration partner or direct investment



- Four patients dosed in Phase I/2 STEADFAST study of TX200 for the prevention of immune mediated rejection in HLA A2 mismatched kidney transplantation.
- Received necessary approvals for accelerated dose escalation protocol from European regulatory authorities. New, fourth cohort dose will be 18-fold higher than first cohort.
- Expect to dose first patient in fourth cohort in January, 18 months earlier than planned under the original protocol.
- Deferring new investments in the CAR-Treg cell therapy programs until collaboration partner or external investment is secured.

TX200 program establishes manufacturing and Treg engineering experience for potential future expansion into major autoimmune indications.

Promising pre-clinical data presented at ESGCT 2023 showing:

- The potential of autologous MOG-CAR-Tregs to provide a long-lasting treatment option for **multiple sclerosis**.
- Updated animal model data demonstrating the promise of IL23R-CAR-Tregs in the potential treatment of **Crohn's disease**.



[Preclinical data from ESGCT 2023](#)

— We are a genomic medicine company dedicated to translating ground-breaking science into medicines that transform the lives of patients and families afflicted with serious neurological disease

Reasons to Believe

- 1 Disciplined focus** on advancing therapeutics to revolutionize the treatment landscape for devastating neurological disorders.
- 2** Combination of potent epigenetic regulators with potentially transformative AAV delivery – **Cargo + Capsid** – unlocks large-scale neurology indications.
- 3 Pioneers** for 25+ years developing genomic medicines and zinc finger technology, with excellent track record of **clinical execution**.