



Delivering the Future of Genomic Medicines

May 14, 2026

Forward-Looking Statements and Legal Disclaimers

This presentation, and accompanying oral commentary, contains forward-looking statements regarding our current expectations. These forward-looking statements include, without limitation, statements relating to: the therapeutic and commercial potential and value of our product candidates and engineered capsids, including the ability of our zinc finger epigenetic regulators to address various neurological diseases and our capsid engineering platform to expand delivery beyond currently available methods; potential STAC-BBB partnerships and its manufacturability at commercial scale; 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, SIFTER and other technologies to develop durable, safe and effective therapies and capsids; the potential for us to benefit and earn development and commercial milestone and royalty payments and additional licensed target fees from our collaborations and the timing of any such benefits and payments; plans for the execution of a Fabry commercialization license agreement; anticipated revenues from existing and new collaborations and the timing thereof; plans and expectations to seek partners or collaborators for certain of our programs; the potential for isaralgagene civaparvec to qualify for the FDA's Accelerated Approval program, including the adequacy of data generated in the Phase 1/2 STAAR study to support any such approval; expectations concerning the availability of additional data to support a potential BLA submission for isaralgagene civaparvec, and the timing of such submission; the potential to accelerate the expected timeline to approval and bring isaralgagene civaparvec to patients sooner than previously expected; the anticipated advancement of isaralgagene civaparvec to registration; the advancement of our preclinical neurology programs, including the potential of ST-503 to enable a pain franchise and plans to initiate patient enrollment and dosing for ST-503; plans regarding our financial resources, including the impact of a potential Fabry commercialization license agreement to provide cash runway through clinical data readouts for lead neurology programs, small fiber neuropathy and prion disease; anticipated plans and timelines for us and our collaborators conducting our ongoing and potential future clinical trials and presenting data from our clinical trials and those of our partners and making regulatory submissions; the anticipated advancement of our product candidates to late-stage development, including potential future registrational 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, the uncertain and costly research and development process, including the risk that preclinical results may not be indicative of any future clinical trials, to the effects of macroeconomic factors or financial challenges, including as a result of ongoing overseas conflicts, tariffs, geopolitical instability, inflation and fluctuations in interest rates 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 unpredictable regulatory approval process for product candidates across multiple regulatory authorities; the potential for Sangamo to cease development of the Hemophilia A program, whether due to its inability to secure options to bring the program forward or otherwise; the manufacturing of products, product candidates and capsids; 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 collaboration for some of our programs, our ability to secure the funding required to advance our preclinical programs in a timely manner or at all; and our lack of capital resources and need for substantial additional funding in the very near term to execute our operating plan and to operate as a going concern, including the risk we will be unable to obtain the funding or partnerships, in particular for our Fabry disease program, or additional collaboration partners necessary to advance our preclinical and clinical programs and to otherwise operate as a going concern in which case at any time we may be required to cease operations entirely, liquidate all or portion of our assets and/or seek protection under applicable bankruptcy laws in the very near term. These risks and uncertainties are described more fully in our Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 2026 and Annual Report on Form 10-K for the fiscal year ended December 31, 2025, each as filed with the Securities and Exchange Commission ("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. All forward-looking statements about Sangamo's future plans and expectations, including Sangamo's financial guidance and development plans for its product candidates, are subject to Sangamo's ability to secure adequate additional funding. 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. There can be no assurance that we and our collaborators will be able to develop commercially viable products.

Sangamo Value Proposition

- ✓ Differentiated genomic medicine platform
- ✓ Focused neurology pipeline
- ✓ Proven clinical execution
- ✓ Clear pipeline expansion opportunities

- **Differentiated genomic medicine platform combines core capabilities required to unlock treatments for devastating neurological diseases**
 - Genome-targeting epigenetic regulation cargo
 - Capsid delivery engine for central nervous system administration
- **Focused neurology pipeline in valuable indications, with best-in-class potential and near-term clinical efficacy data**
 - Chronic neuropathic pain – starting with Small Fiber Neuropathy (SFN)
 - Prion disease
- **Proven clinical execution expertise**
- **Clear neurology pipeline expansion opportunities**
 - SFN program provides pain franchise potential
 - Prion program would unlock STAC-BBB capsid for broader neurology indications
- **Best-in-class Fabry gene therapy with Biologics License Application (BLA) submission in progress**

Why neurology genomic medicines?

- Widespread, debilitating diseases, largely unserved by current approaches
- Many neurology indications are single-gene or gene-associated
- Genomic medicines are well suited to neurology:
 - Targeting diseases at the DNA level reduces therapeutic complexity
 - Gene expression can be fine-tuned to the level needed for proper brain function
 - Potential for durable effect as most brain cells do not divide
- Addressing the issues of widespread brain delivery is critical to creating an effective neurology medicine



Sangamo pairs the epigenetic regulation *and* capsid delivery capabilities needed to create neurology genomic medicines

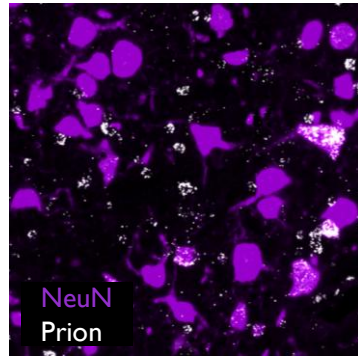
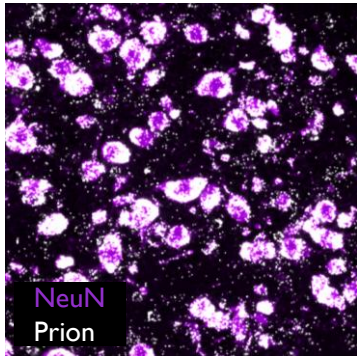
Genome-Targeting Cargo



Capsid Delivery Engine

No Treatment:
Prion RNA

SGMO Prion ZF
Repressor (ZFR)

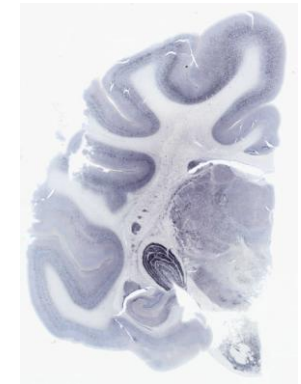
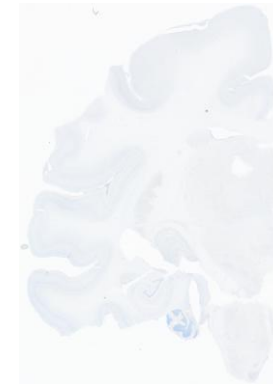


Nonhuman primate (NHP) pons



No Treatment

SGMO STAC-BBB
Intravenous Capsid



NHP

Future of Neurology Genomic Medicines

Differentiated neurology pipeline with franchise potential and industry validation

NEUROLOGY PIPELINE - WHOLLY OWNED						
Indication	Preclinical	Phase 1/2	Pivotal	Partner	Cargo	STAC-BBB
Small Fiber Neuropathy (ST-503)				-	✓	-
Prion Disease (ST-506)				-	✓	✓
Undisclosed neurology target(s)				-	✓	✓

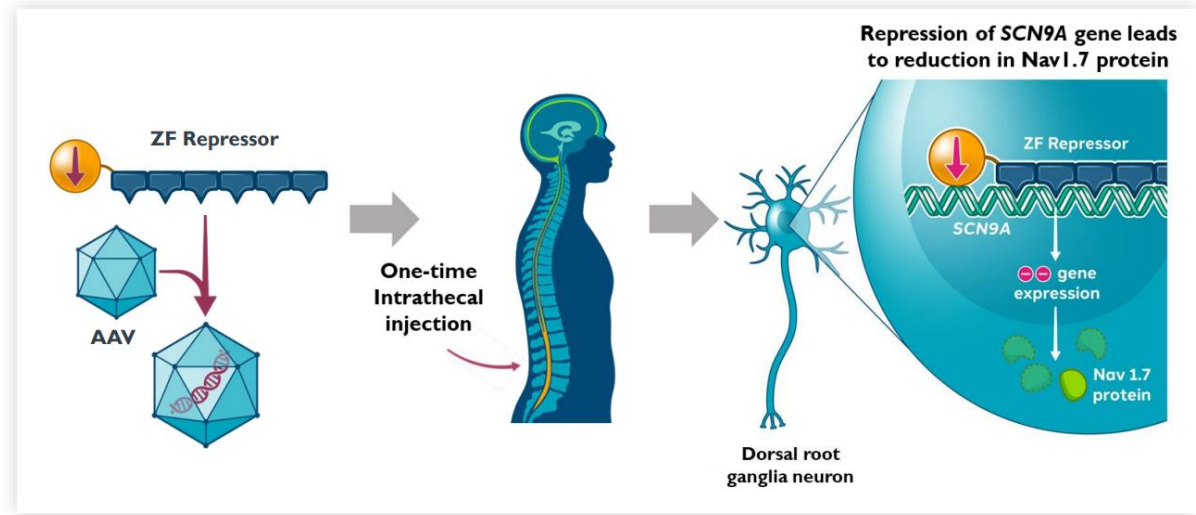
NEUROLOGY PIPELINE - PARTNERED						
Indication	Preclinical	Phase 1/2	Pivotal	Partner	Cargo	STAC-BBB
Tauopathies				 <small>A Member of the Roche Group</small>	✓	✓
Undisclosed neurology target				 <small>A Member of the Roche Group</small>	✓	✓
Undisclosed neurology target					-	✓
Undisclosed CNS target					-	✓
Amyotrophic Lateral Sclerosis (ALS)				 <small>AstraZeneca Plans Disease</small>	✓	-
Huntington's Disease					✓	-

OTHER PROGRAMS						
Indication	Preclinical	Phase 1/2	Pivotal	Partner	Cargo	STAC-BBB
Fabry Disease (Isaralgagene civaparvovec)						✓
Hemophilia A (Giroctogene fitelparvovec)						✓

ST-503 for chronic neuropathic pain



Clinical-stage chronic pain program with **compelling preclinical data**, short timeframe to **expected clinical readout**, and **pain franchise potential**

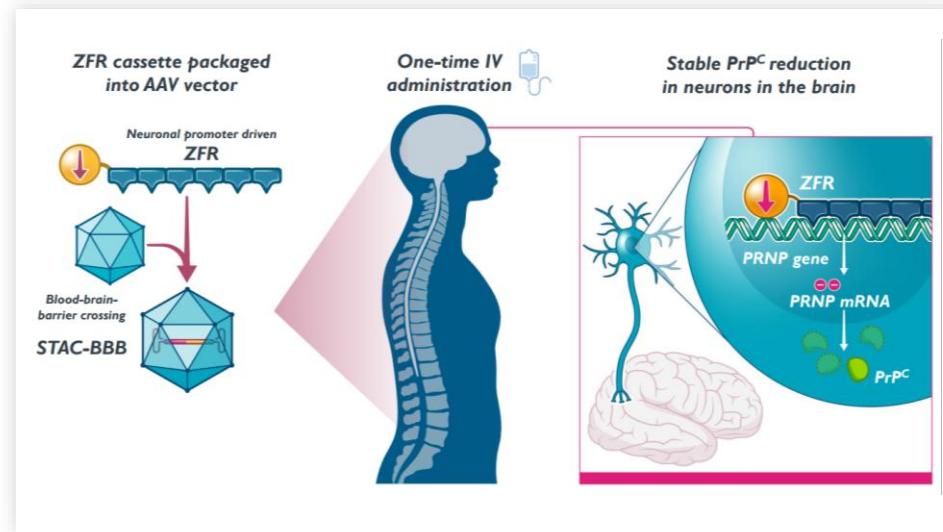


Indication	<ul style="list-style-type: none"> Starting in small fiber neuropathy (SFN), a debilitating chronic neuropathic pain impacting 650,000 people across the US, Europe and Japan
Construct	<ul style="list-style-type: none"> Nav1.7 targeted zinc finger repressor (ZFR), delivered intrathecally via AAV9
Reasons to believe	<ul style="list-style-type: none"> Well proven target with human genetic validation Ability to induce a durable analgesic response in mice, with high levels of Nav1.7 specificity Durable, potent, selective and safe up to 6-months in NHPs
Program status	<ul style="list-style-type: none"> Six clinical sites active in Phase I/2 STAND study Granted Fast Track Designation by U.S. FDA
Value opportunity	<ul style="list-style-type: none"> SFN represents a multi billion-dollar commercial opportunity
Franchise potential	<ul style="list-style-type: none"> Opportunity to broaden to other, high-value potential targets e.g., trigeminal neuralgia or oncology-related chronic pain

ST-506 for prion disease



Compelling preclinical data with short timeframe to **expected clinical readout** in a **deadly disease with no currently approved treatment options**

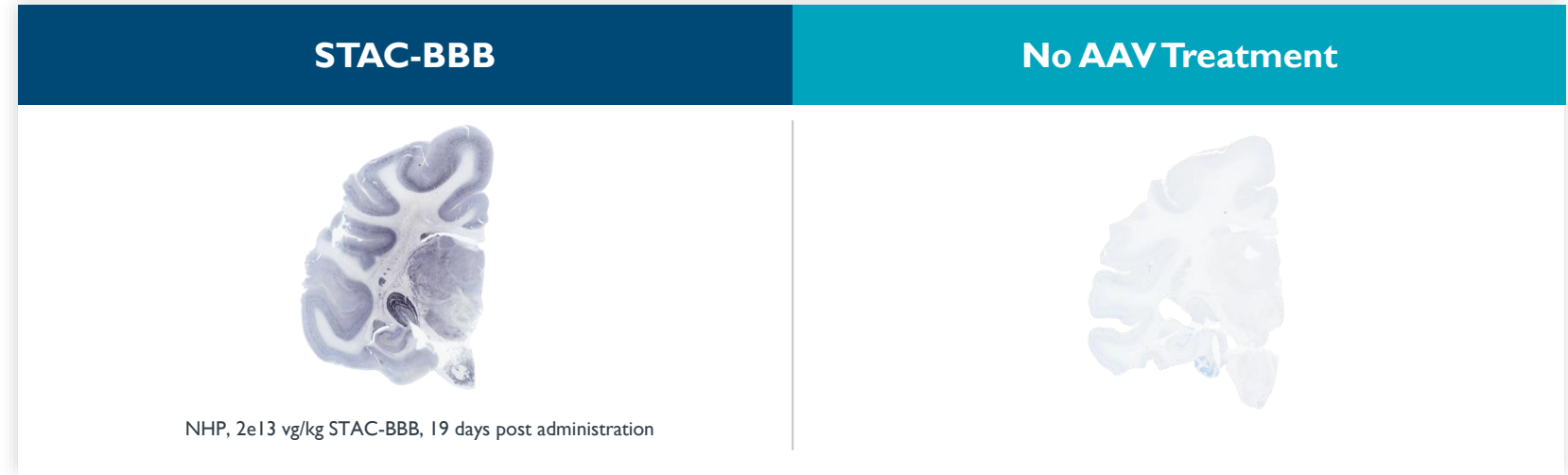


Indication	<ul style="list-style-type: none"> A progressive condition leading to rapid neurodegeneration and death, resulting in 1,500 deaths per year across the US, Europe and Japan
Construct	<ul style="list-style-type: none"> Prion targeted ZFR, delivered intravenously via STAC-BBB neurotropic capsid
Reasons to believe	<ul style="list-style-type: none"> Profound post-symptomatic survival extension in mice, with stable prion repression for at least 17 months (duration of study) Prion repression in NHPs following STAC-BBB administration that matched or exceeded levels seen with mice lifespan extension
Program status	<ul style="list-style-type: none"> Clinical Trial Application (CTA) enabling activities in progress Good Laboratory Practice (GLP) toxicology study is complete, with analysis ongoing
Value opportunity	<ul style="list-style-type: none"> Assuming effect is transformative, ST-506 could generate >\$1 billion in annual sales
Franchise potential	<ul style="list-style-type: none"> First-in-human trial of STAC-BBB capsid, which if successful, could validate broader neurology pipeline

Intravenous delivery via STAC-BBB



Mediates brain-wide delivery in nonhuman primates and mice, with large pharma endorsement and near-term first-in-human anticipated study



Problem statement	<ul style="list-style-type: none"> Widespread central nervous system (CNS) delivery is challenging with conventional AAVs
Reasons to believe	<ul style="list-style-type: none"> Enabled strong expression of zinc-finger cargo throughout the brain of NHPs, including all key brain regions Industry-leading performance, with 700-fold higher transgene expression than benchmark capsid AAV9 STAC-BBB delivery of zinc finger cargo resulted in widespread repression of target genes, with no observed toxicity
Partner buy-in	<ul style="list-style-type: none"> External validation through partnerships with Genentech, Astellas and Eli Lilly
Program status	<ul style="list-style-type: none"> Advancing CTA-enabling activities for prion program. Next generation capsid discovery progressed.
Franchise potential	<ul style="list-style-type: none"> Potential to unlock a wide range of previously hard-to-treat neurology diseases

Demonstrated industry interest in STAC-BBB could provide significant economics for Sangamo

**STAC-BBB
partnerships**

Genentech
A Member of the Roche Group

Lilly

 **astellas**

\$88m

cash received from
partners to date

Up to \$4.6b

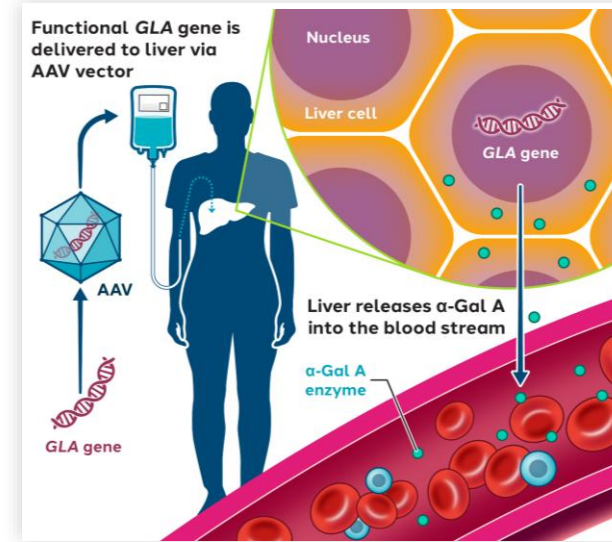
in potential future milestones and exercise fees
assuming exercise of all options and targets

**Additional potential
product royalties**

ST-920 for Fabry disease



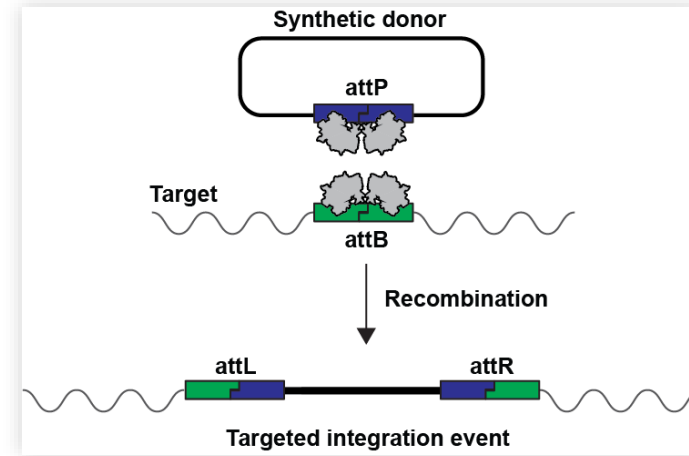
Best-in-class clinical program demonstrating durable, meaningful patient benefits above current standards of care, with **clear regulatory pathway** and **near-term regulatory filing**



Indication	<ul style="list-style-type: none"> A lysosomal storage disorder caused by mutations in the GLA gene, causing serious damage to vital organs, with approximately 18,000 patients diagnosed worldwide
Construct	<ul style="list-style-type: none"> Functional GLA gene delivered intravenously to the liver via AAV2/6 vector
Reasons to believe	<ul style="list-style-type: none"> Clinical data supports potential for ST-920 as one-time, durable treatment for Fabry disease, providing meaningful, multi-organ benefits above current standards of care Positive mean annualized eGFR slope at 52-weeks observed across all 32 dosed patients ST-920 showed a favorable safety and tolerability profile, without preconditioning Clear Accelerated Approval pathway with U.S. FDA, reiterated in October 2025
Program status	<ul style="list-style-type: none"> Rolling submission of BLA in progress – nonclinical and clinical modules submitted BLA submission completion anticipated as early as summer 2026* Commercialization agreement discussions in progress
Value opportunity	<ul style="list-style-type: none"> \$1-2 billion in estimated peak year sales across US and Europe
Discharged risks	<ul style="list-style-type: none"> Compelling clinical data with strong durability (5 years+), FDA alignment on Accelerated Approval pathway and data required for Traditional Approval

Next-generation, large-scale genome editing - Modular Integrase (MINT) platform

A versatile, protein-guided genome editing method designed to integrate large sequences of DNA into the desired chromosomal sites to treat – with a single medicine – patients who have unique mutations in the same gene



Problem statement

- Effectively and precisely integrating large synthetic DNA constructs into desired chromosomal sites is challenging

Reasons to believe

- Successfully reprogrammed the specificity of the serine integrase BxbI to target clinically relevant sites
- Large payload delivery with no inherent size limit
- Ability to target a gene-of-choice at high frequency
- Gene integration at native locus without double-stranded breaks or dependence on any cellular DNA repair machinery

Program status

- Achieved 34% targeted integration at the TRAC locus in K562 cells
- Achieved 44% targeted integration in T cells
- Platform presentation at ASGCT 2026

Franchise potential

- Potential lucrative applications in gene therapies, agriculture and CAR-T oncology

Company Highlights



Developing epigenetic regulation for important gateway neurology diseases like chronic neuropathic pain and prion disease



Proprietary AAV blood-brain barrier penetrant capsid (STAC-BBB) with industry leading CNS tropism in NHPs. Already the subject of license agreements with Genentech, Astellas and Lilly, with potential for additional partnerships.



STAC-BBB potentially unlocks multiple neurology epigenetic programs that could be advanced ourselves or with partners



Novel next-generation modular integrase (MINT) platform allows targeting of a serine recombinase engineered to enable large-scale genome editing



Positive topline readout in registrational STAAR study in Fabry disease. Rolling submission of BLA to FDA in progress.

1Q26 Business Updates



1Q26 Key Takeaways

Rolling submission of BLA to FDA seeking ST-920 approval is in progress under Accelerated Approval pathway

Fabry Disease

- The rolling submission of a BLA to the FDA is in progress, with the preclinical and clinical modules submitted.
- Clear pathway to accelerated approval from the FDA, using mean annualized eGFR slope at 52-weeks across all dosed patients. FDA affirmed two-year eGFR data may serve as confirmatory evidence for traditional approval.
- In February, presented detailed data from the registrational Phase I/2 STAAR study via four platform and poster presentations at the 22nd Annual *WORLD Symposium™* in San Diego, California.
- Sangamo is advancing the CMC module, ahead of completion of the rolling BLA submission, expected as early as the summer of 2026, subject to our ability to secure adequate additional funding, while continuing discussions for a potential Fabry commercialization agreement.

Neurology Pipeline

- Six clinical sites are activated in the Phase I/2 STAND study evaluating ST-506 in chronic neuropathic pain.
- Manuscript published in *Science Translational Medicine* detailing the preclinical safety and pharmacology of ST-503 in human neurons, mice and nonhuman primates.
- CTA-enabling activities are in progress for ST-506 in prion disease.
- Held productive interaction with the UK's MHRA, including alignment on diagnostic testing, analytical validation and nonclinical safety matters. The GLP toxicology study has been completed and analysis is ongoing.
- Presented neurology pipeline advances and developments in modular integrase technology at ASGCT 2026.



Financial Highlights

As of March 31, 2026, we had cash and cash equivalents of \$27.6 million. Based on our current operating plan, including the implementation of potential additional cost reductions, we believe that our cash and cash equivalents as of March 31, 2026, will be sufficient to fund our planned operations into the third quarter of 2026.

Financial metrics

Historical

\$911m

Cash received from partners to date

\$31.7m*

Non-GAAP OpEx – Q1 2026

~\$27.6m

Cash and cash equivalents balance as of 3/31/26

Forward Looking

Up to \$4.8b

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

\$100m – \$120m (2026)**

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



Engineering Versatile Zinc Finger Payloads for Neurology

Sangamo has the tools needed to advance a next-generation neurology genomic medicine company



Potent Zinc Finger Cargo

Level of potency is precisely customizable to the indication being targeted



Versatility and Exquisite Specificity

We believe any gene in the genome is targetable for up- or down-regulation



All Human Derived

Potentially avoids issues with immunogenicity



Small Size. Easily Packaged.

Zinc fingers can be easily packaged into viral vectors



Powerful AAV Delivery Platform

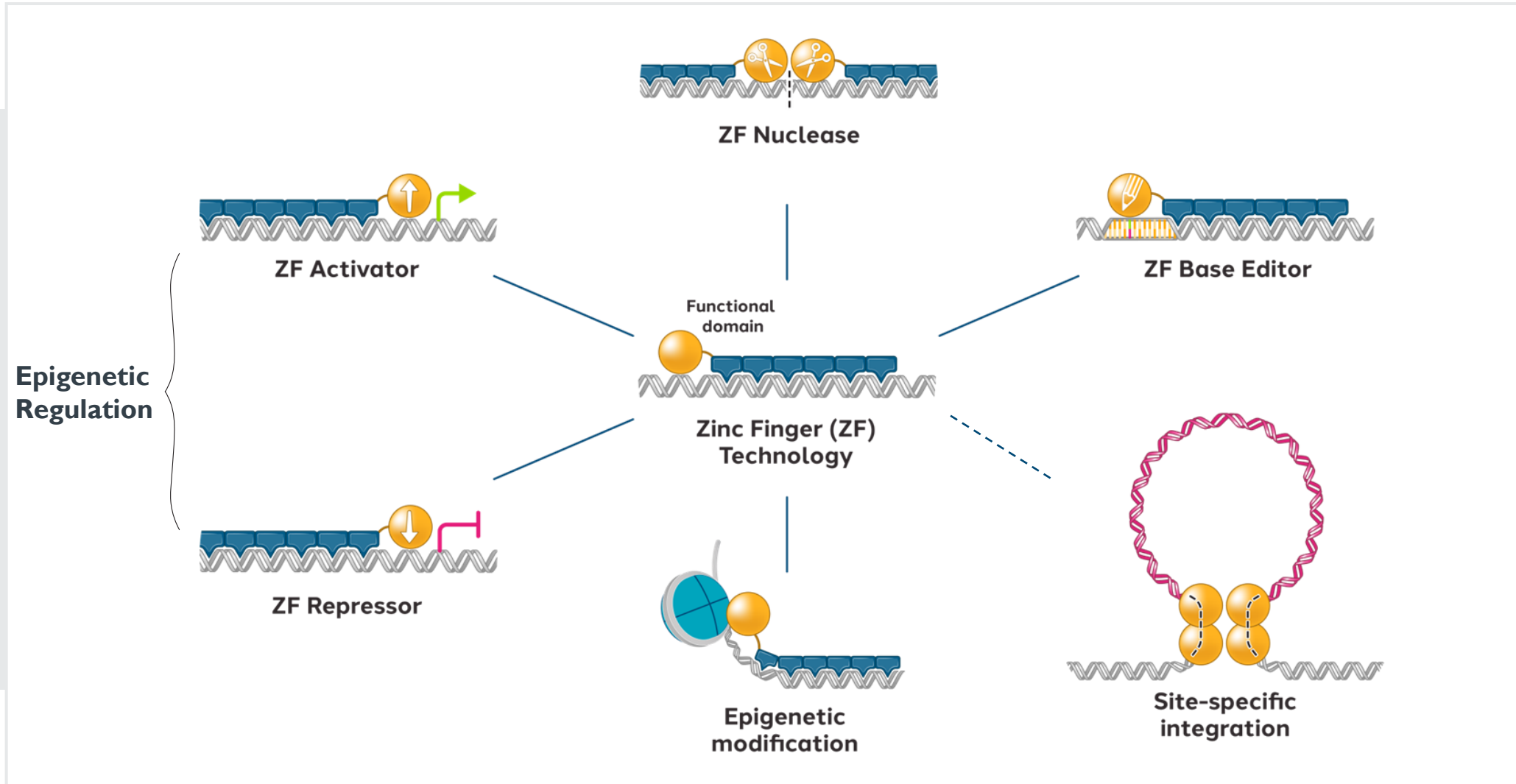
Widespread zinc-finger 'cargo' delivery – via both intravenous AND intrathecal delivery



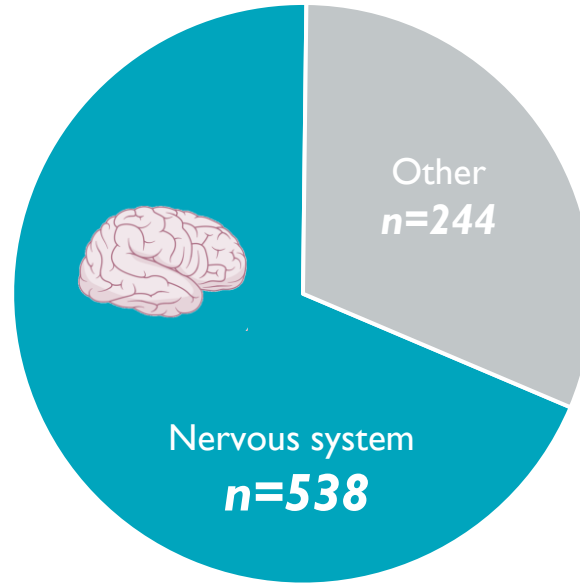
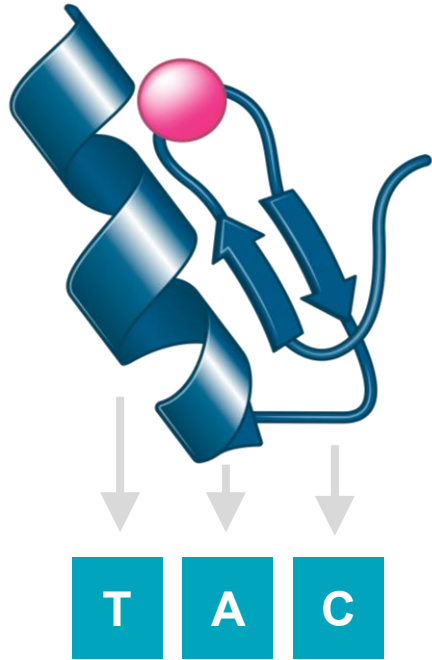
Industry Leading CNS Tropism

Robust penetration of the blood-brain barrier and widespread brain distribution in NHPs

Sangamo's differentiated genomic engineering platform is flexible, creating specific tools for the needs of each target



Zinc finger epigenetic regulators are the ideal cargo for neurology-focused genomic medicines



	ZFR/ZFA	ASO	CRISPR
Single administration	✓	✗	✓
Human derived	✓	✗	✗
Target any sequence	✓	✗	✗
Cell-type specificity	✓	✗	~
Compact / multiplexing	✓	~	✗
Supplement with cDNA	✓	✗	✗
All RNA / protein forms	✓	~	✓
Allele specific	✓	✗	~

Zinc Fingers are natural proteins that bind DNA with high specificity
















At least 782 human genes encode Zinc Finger Proteins

Zinc fingers are differentiated in key therapeutic features for potentially treating neurologic diseases

Most regulate the epigenetic state of other genes

n=782 C2H2 ZF-containing genes
Sources: Ensembl human genes; GTEx: CNS (>5 TPM)
ASO: antisense oligonucleotide

Sangamo's neurology portfolio provides opportunities for wholly owned program advancement and potential partnering opportunities

<p>WHOLLY OWNED PRIORITY PROGRAMS</p>	<p>Chronic Neuropathic Pain</p> <p>Nav1.7</p> 	<p>Prion Disease</p> <p>PRNP</p> 							
<p>PARTNERED PROGRAMS</p>	<p>ALS</p> <p>C9orf72</p> 	<p>Huntington's Disease</p> <p>HTT</p> 	<p>Tauopathies</p> <p>MAPT</p> 	<p>Undisclosed neurology</p> 	<p>Undisclosed neurology</p> 	<p>Undisclosed CNS</p> 			
<p>CURRENTLY PAUSED CARGO PROGRAMS ENABLED BY STAC-BBB</p>	<p>Phelan-McDermid Syndrome</p> <p>SHANK3</p> 	<p>Dravet Syndrome</p> <p>SCN1A</p> 	<p>Myotonic Dystrophy Type I</p> <p>DMPK</p> 	<p>ALS</p> <p>SOD1</p> 	<p>Charcot Marie Tooth 2A</p> <p>MFN2</p> 	<p>Charcot Marie Tooth 1A</p> <p>PMP22</p> 	<p>Haploinsufficiency Syndrome</p> <p>SCN2A</p> 		

ALS: Amyotrophic Lateral Sclerosis; CMT: Charcot-Marie Tooth

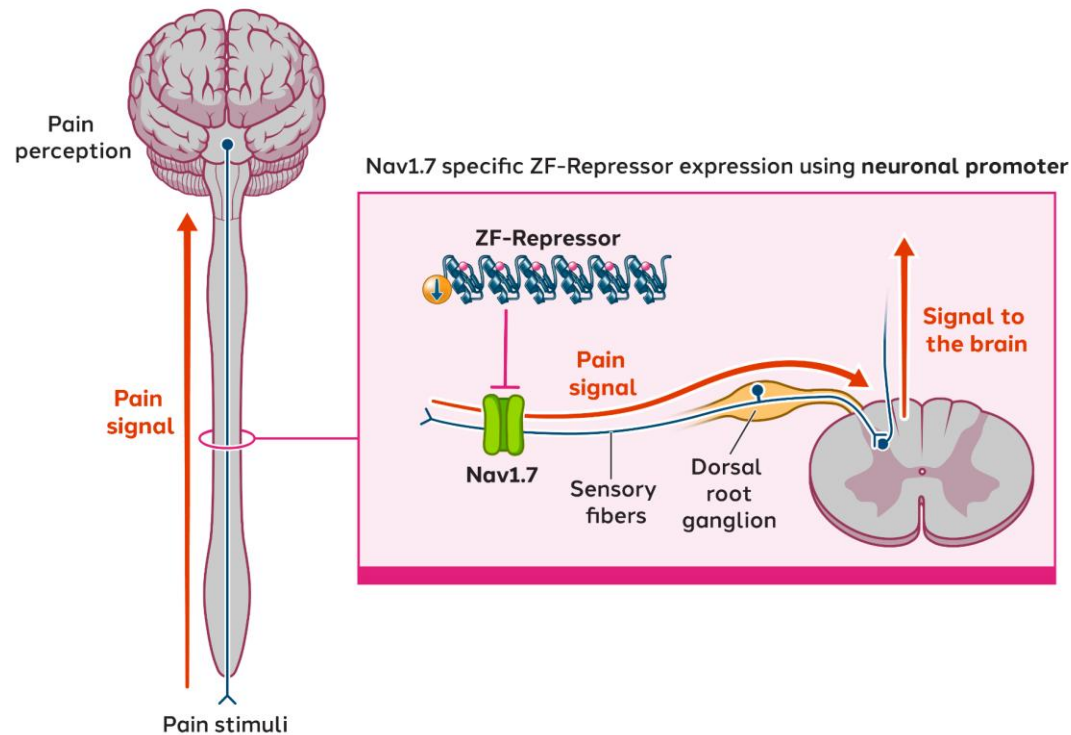
 Cerebrospinal fluid (CSF) capsid  Intravenous (IV) capsid



Epigenetic regulation to address chronic neuropathic pain

Targeting the Nav1.7 pathway at the DNA level, we seek to succeed where others have failed

Clinical-stage chronic pain program with compelling preclinical data, short timeframe to expected clinical readout, and pain franchise potential



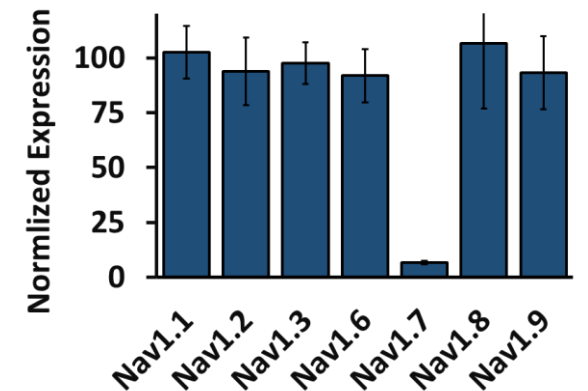
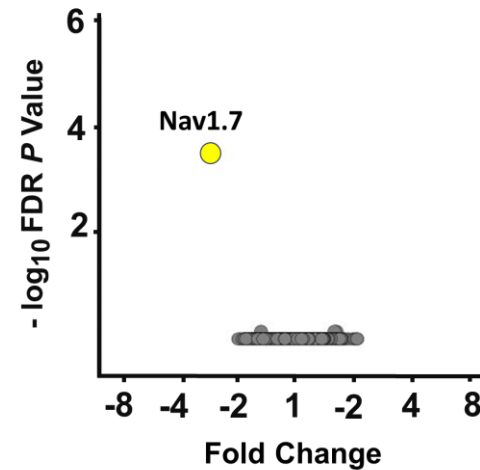
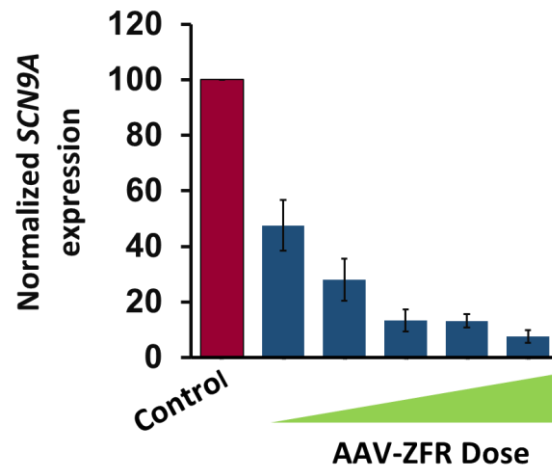
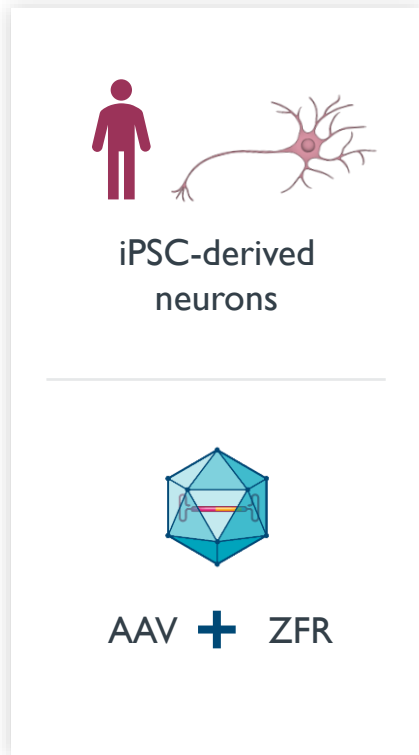
- **Nav1.7** is a voltage gated sodium channel expressed in the Dorsal Root Ganglion (DRG)
- Well proven target with **human genetic validation**
- Blocking Nav1.7 in the DRG is expected to prevent the **transmission of nociceptive pain signals** to the brain
- Reducing pain by inhibiting Nav1.7 is not predicted to be associated with **any neurological side effects**
- Administered **intrathecally via AAV9**, a well-established, well-tolerated capsid
- **Opportunity to broaden** to other, high-value potential targets e.g., trigeminal neuralgia or oncology-related chronic pain

Zinc finger repressors potently reduced Nav1.7 in human neurons with a high level of specificity

Potent and dose-dependent repression of *SCN9A* gene, which encodes Nav1.7

Selective repression of *SCN9A*, out of >20,000 genes tested

Specific repression of Nav1.7 without impacting other sodium channels



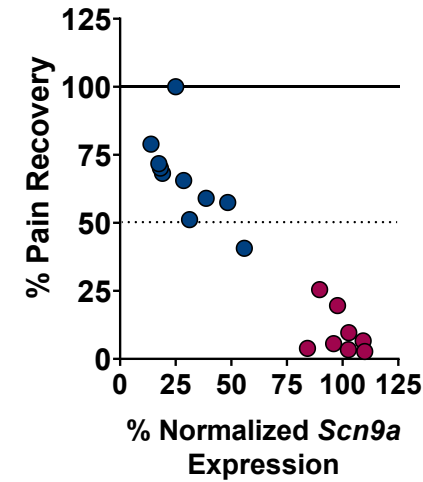
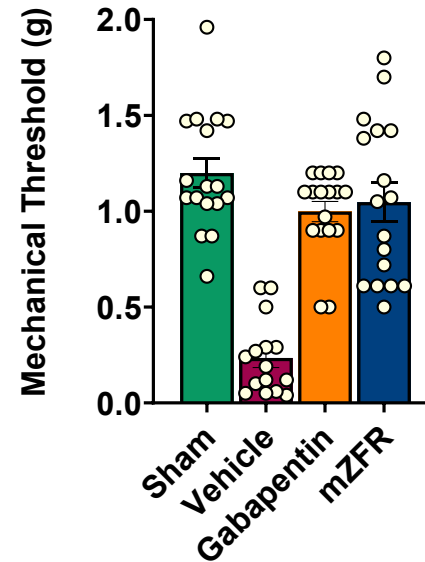
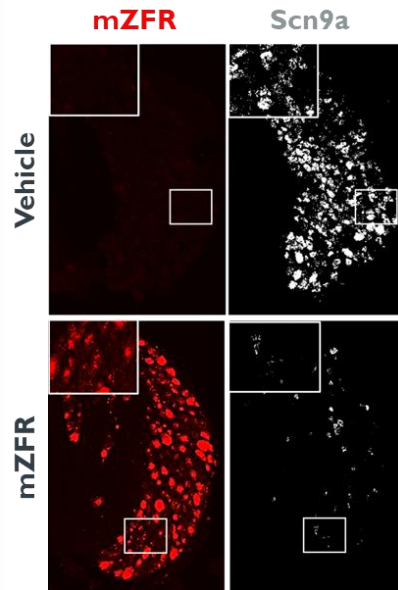
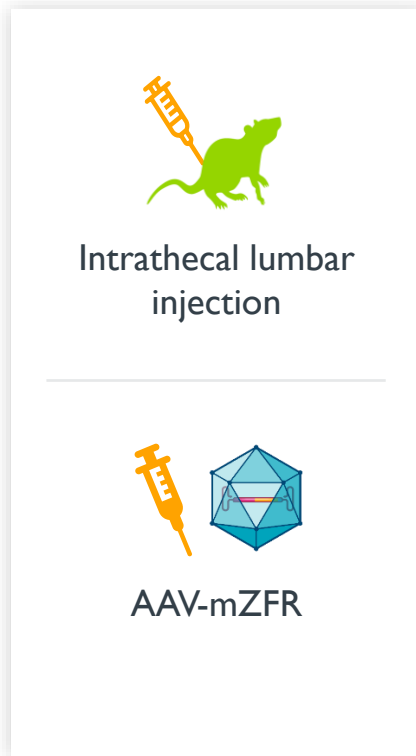
Nav1.7 repressor reversed neuropathic pain in mouse models

Potent *Scn9a* mRNA repression in mouse

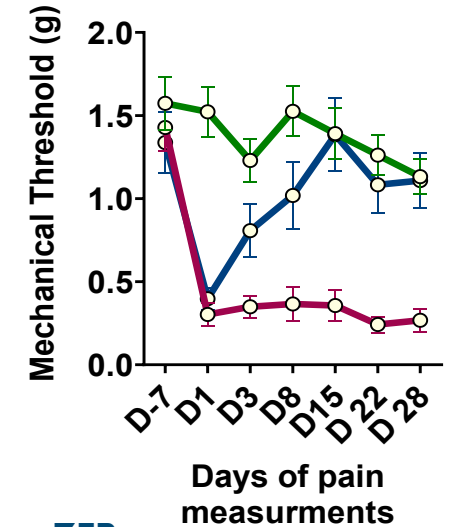
Full restoration of normal sensitivity to mechanical pain

~40% repression of *SCN9A* is sufficient to induce more than 50% pain recovery

Efficacy is observed as early as Day 3 post-treatment



mZFR
Vehicle



mZFR
Vehicle
Sham

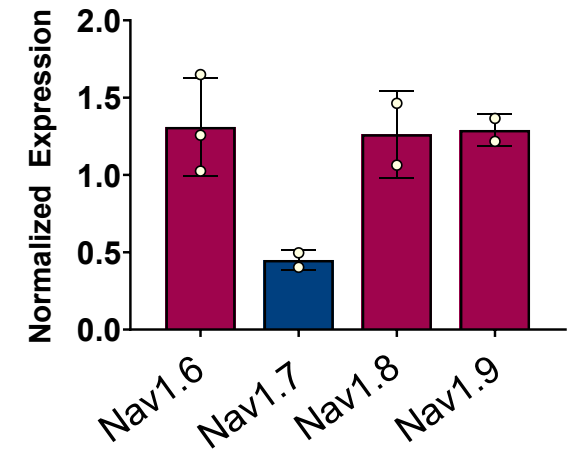
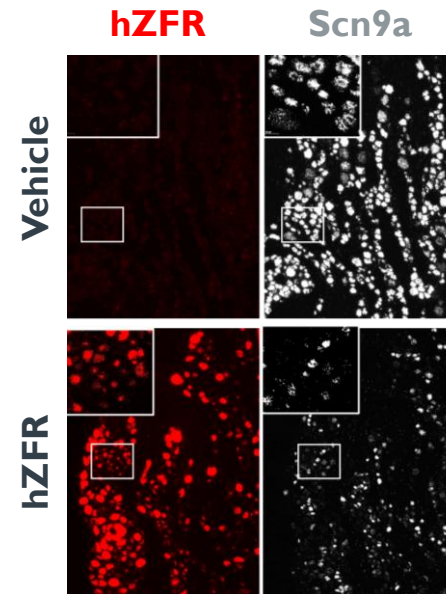
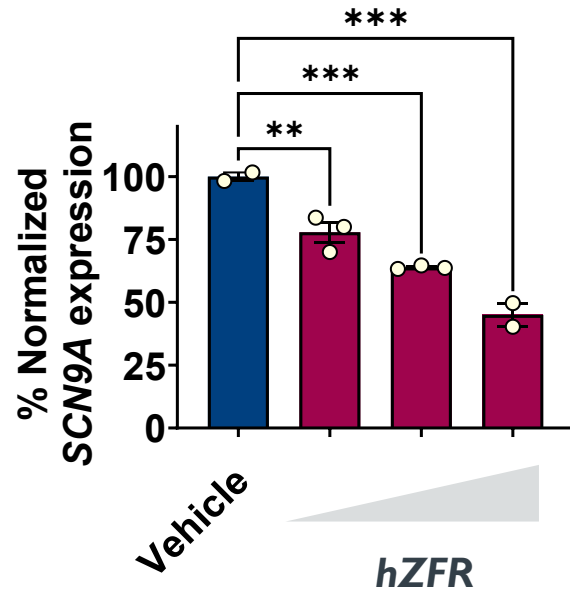
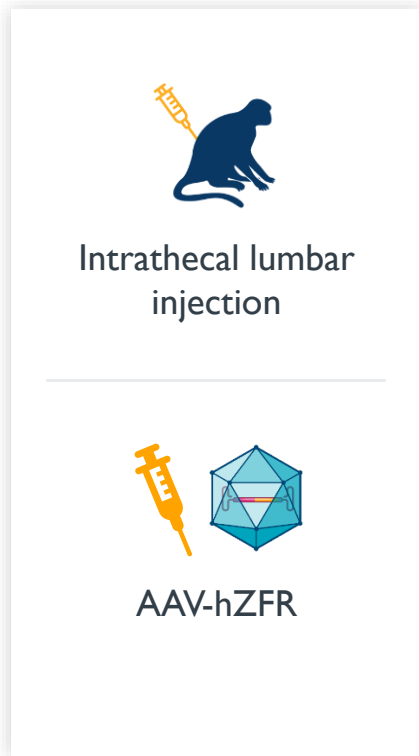
mZFR: mouse ZFR

Potent and selective repression of *SCN9A* observed in NHPs, with no signs of toxicity or adverse clinical pathology

Potent and dose-dependent repression of *SCN9A* gene

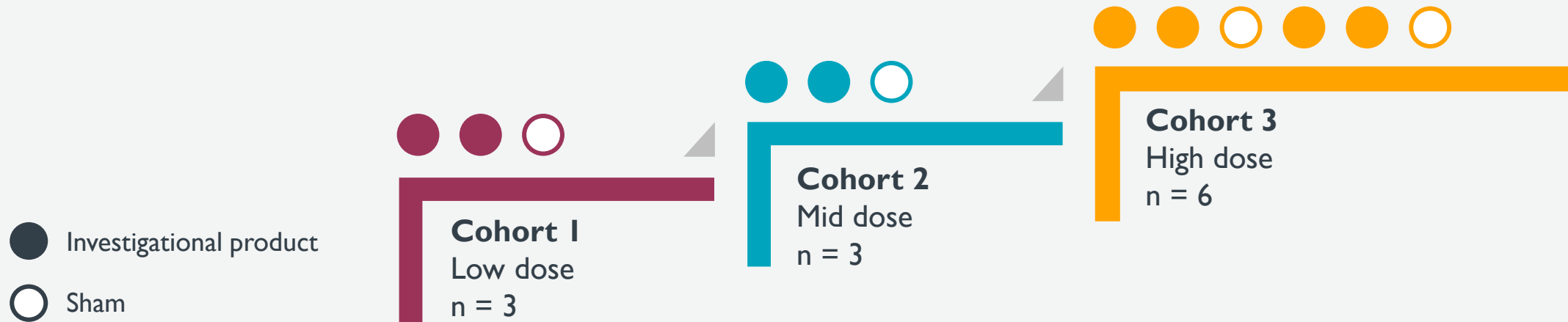
Selective repression of *SCN9A*

Specific repression of Nav1.7 without impacting other sodium channels



hZFR: human ZFR

First six clinical sites have been activated in Phase 1/2 STAND study



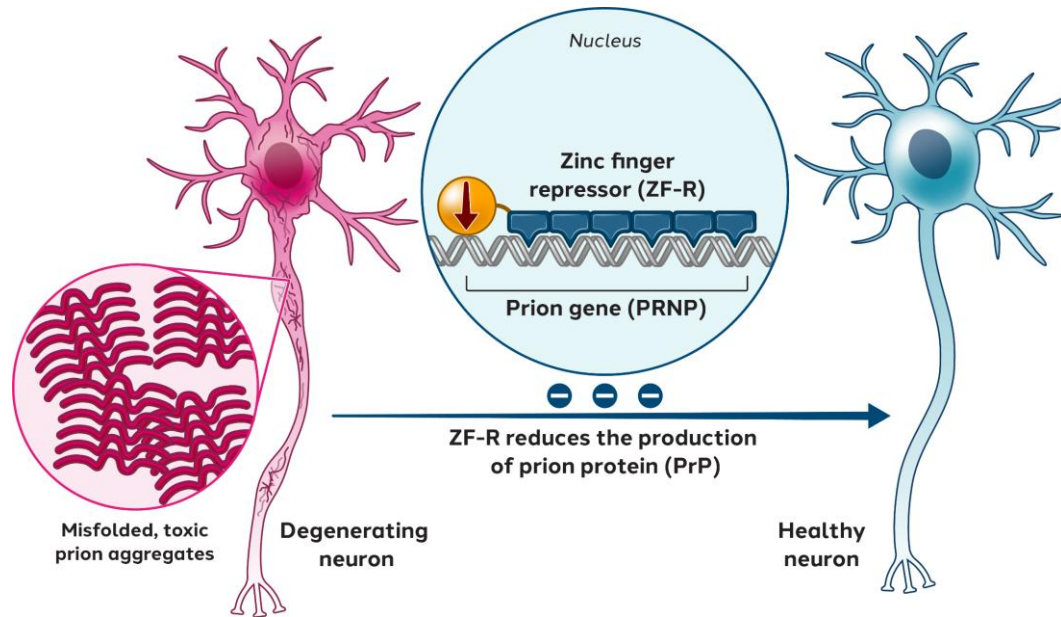
- FDA **clearance of IND** received to assess **ST-503 in SFN patients** (which impacts 650,000 people across the US, Europe and Japan)
- Preparing for **double-blind, randomized, sham-controlled dose escalation** study to determine safety, tolerability and efficacy of a single dose **intrathecal ST-503** gene therapy
- Dose escalation protocol with a **2:1 randomization** of investigational product to sham
- **Six clinical sites active** in Phase 1/2 STAND study
- Received **FDA Fast Track Designation** in December 2025



Epigenetic regulation to address prion disease, leveraging STAC-BBB

Prion disease is rapidly progressive and always fatal

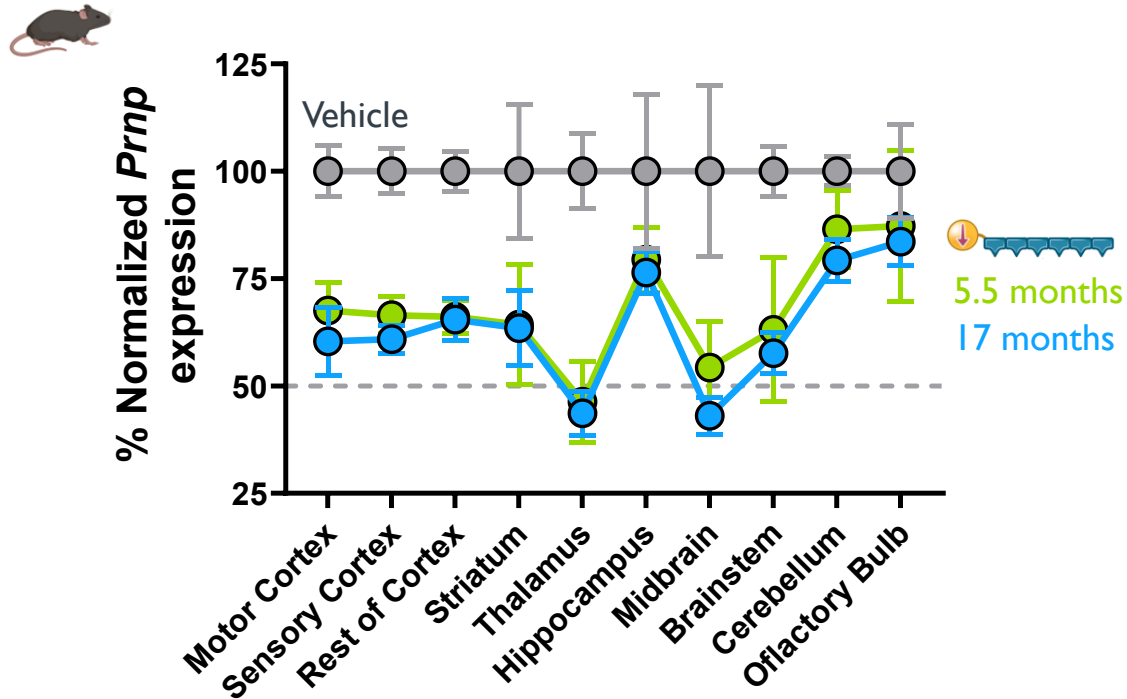
Compelling preclinical data with short timeframe to expected clinical readout in a deadly disease with no currently approved treatment options



- Progressive condition, with **no disease modifying therapy**
- Caused by the misfolding of the prion protein (PrP) into toxic species, **leading to neurodegeneration and death**
- At least **1,500 new cases** each year in **U.S., Europe and Japan**
- **Sporadic, inherited and acquired** forms
- **Well-defined** patient population
- Repression of prion expression in the brain **should slow or halt disease progression and neurodegeneration**
- Assuming effect is transformative, **ST-506 could generate >\$1 billion in annual sales**
- **First-in-human** trial of **STAC-BBB** capsid, which if successful, could validate broader wholly owned and partnered programs

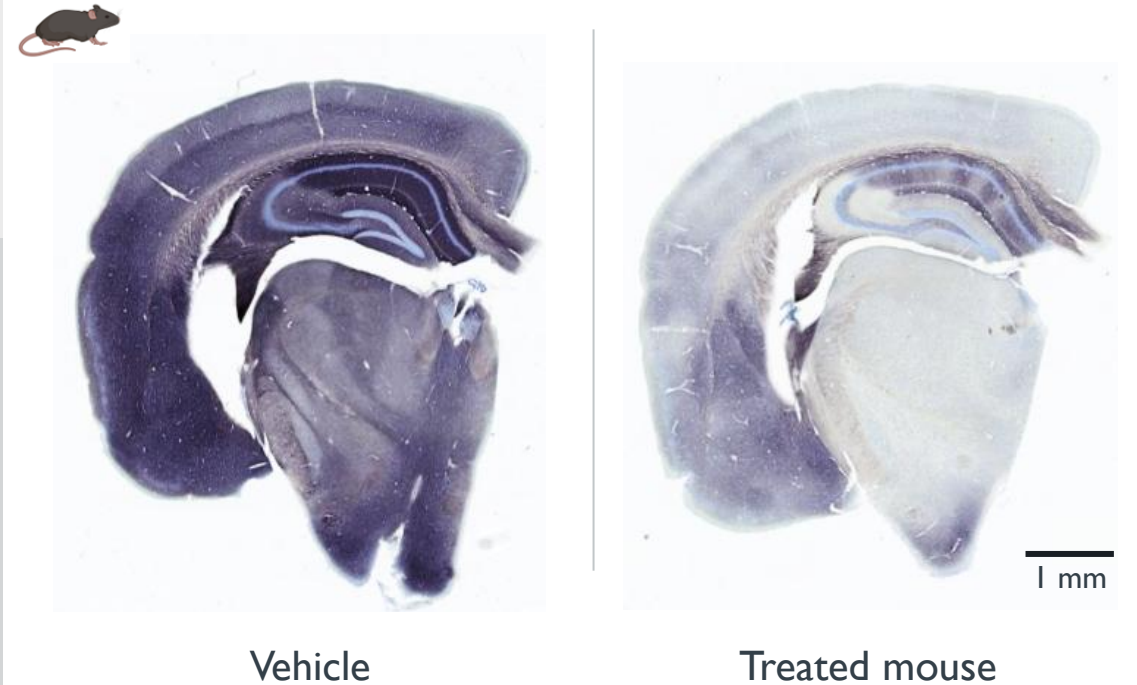
Persistent ZFR activity up to 17-months suggests the potential for lifelong prion suppression in neurons

Sustained brainwide *Prnp* mRNA repression



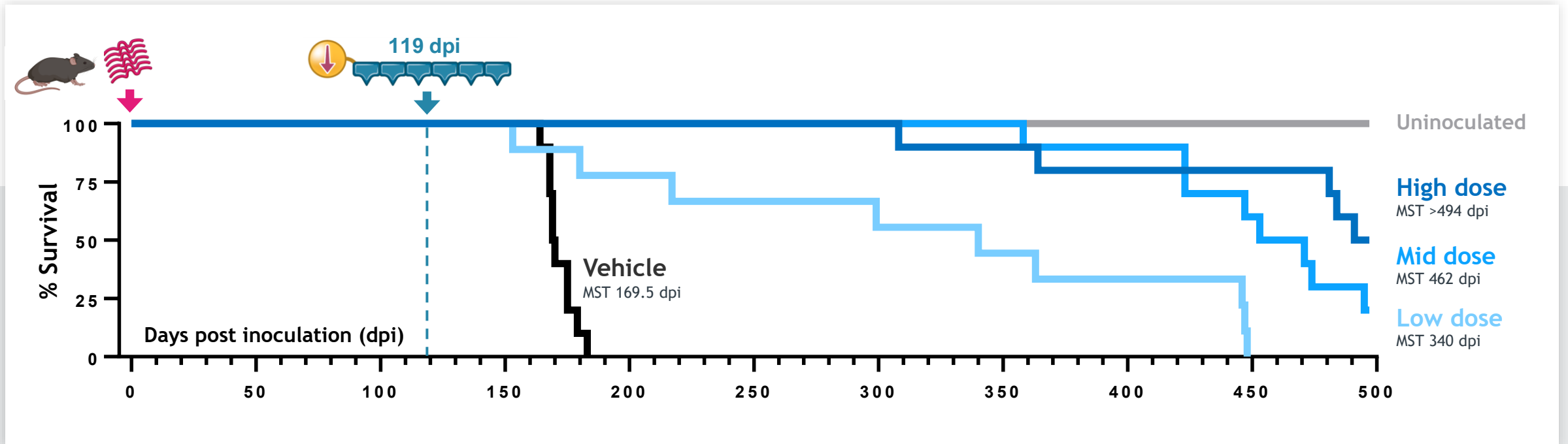
3E+13 vg/kg dose shown.

Sustained brain wide PrP knockdown at 17 months



1E+14 vg/kg dose shown.

Zinc finger repressors extended survival in a mouse model of aggressive prion disease, even when administered post-symptomatically



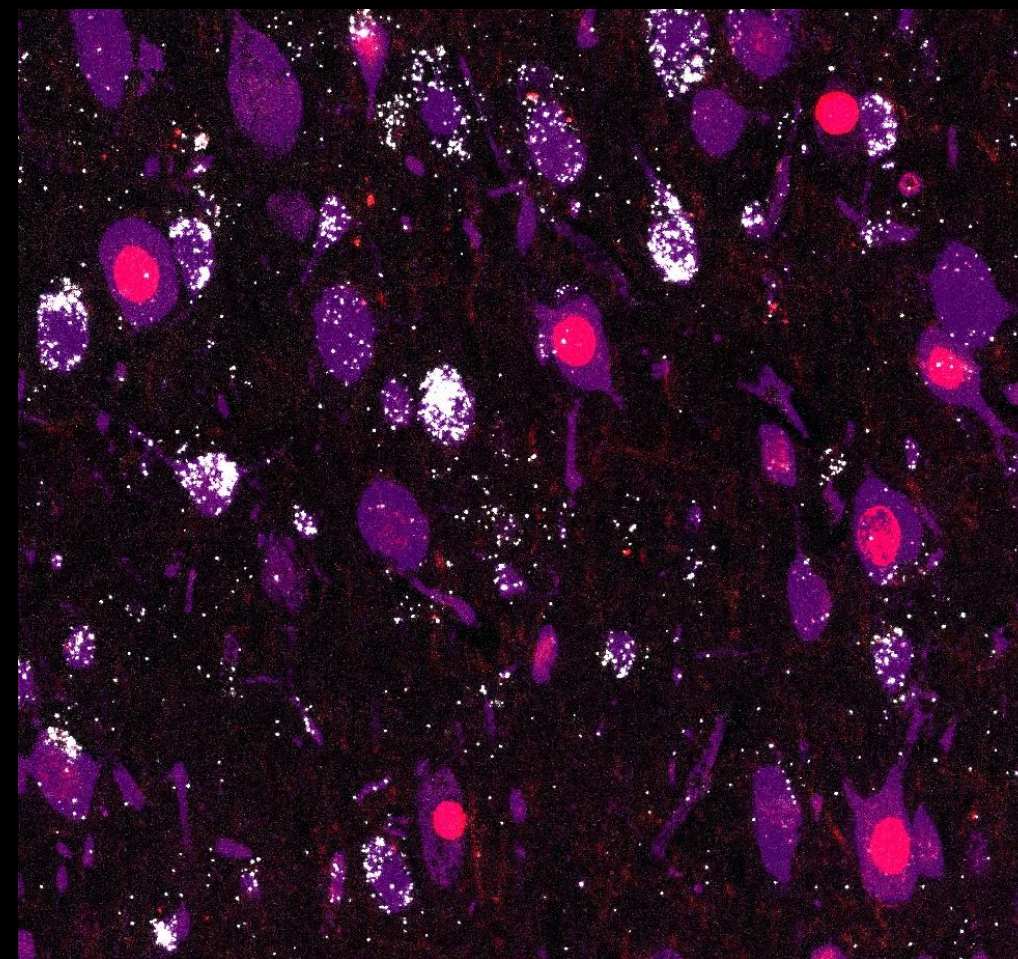
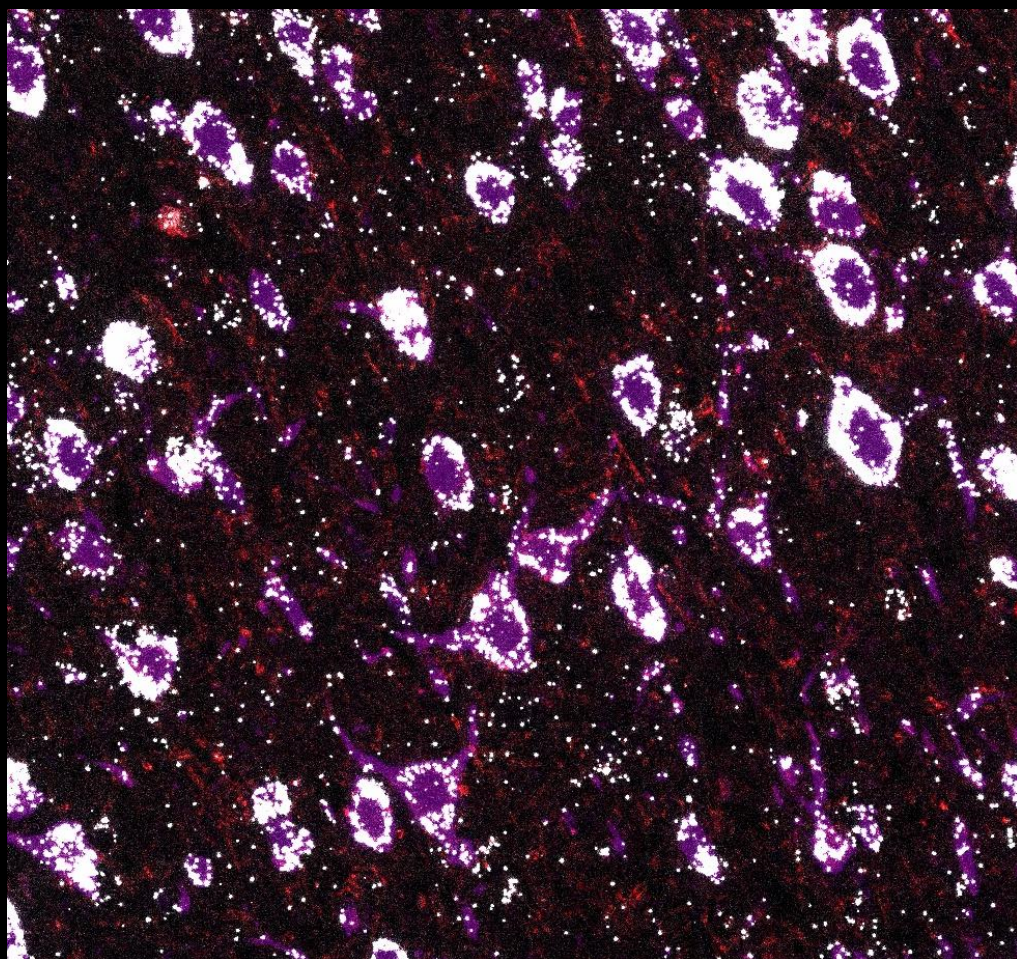
STAC-BBB mediated ZFR expression and Prion repression in the NHP brain

ZFR+ cells (GFP)
Neurons (NeuN)
Prion mRNA

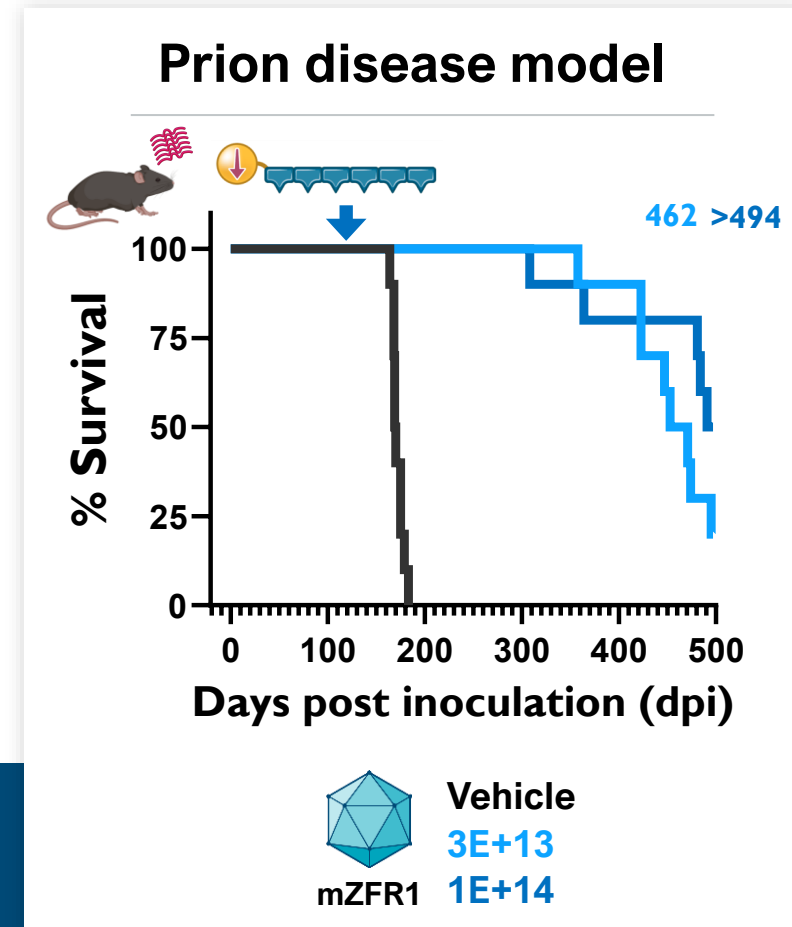
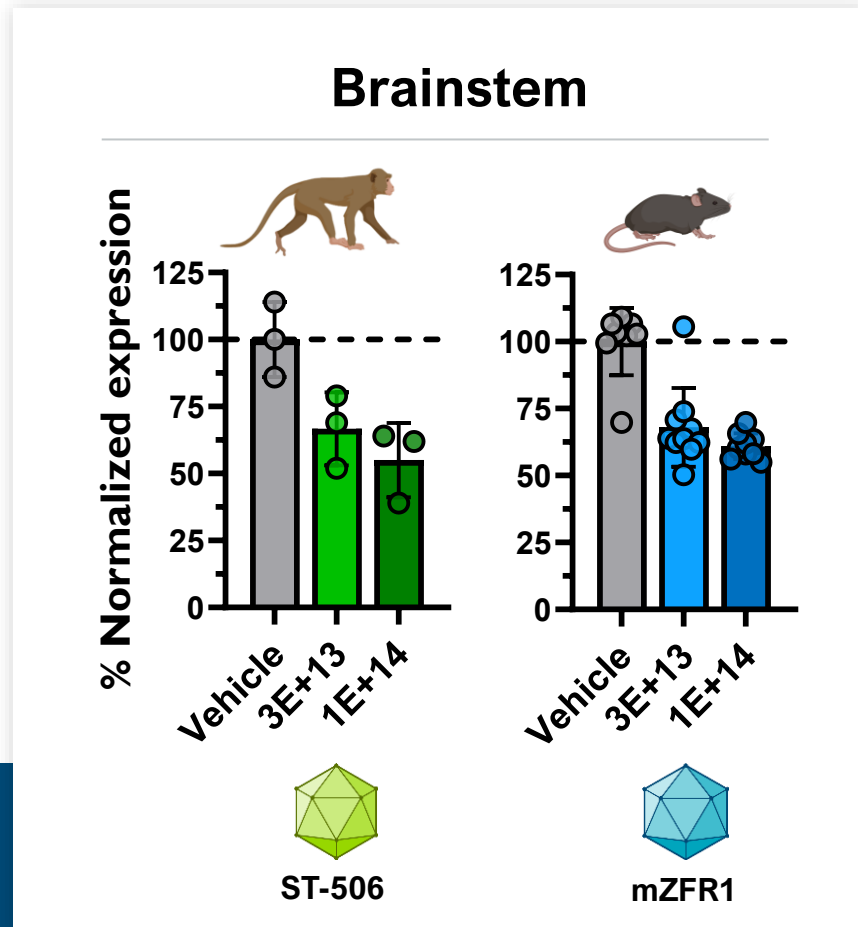
Vehicle Control

Motor cortex

STAC-BBB



ST-506 mediated prion repression in NHPs that matched or exceeded levels associated with profound survival extension in mice



ST-506 was safe at both dose levels in NHPs, with no adverse safety findings in any tissue

Phase 1/2 CTA-enabling activities and clinical study preparations are ongoing

Item	Category criteria	Score
Bowel function	At least one episode of incontinence in last 7 days	0
	Continent for last 7 days	1
Bladder function	Always incontinent or catheterized	0
	Continent or occasional accidents	1
Toilet use	Fully dependent	0
	Needs some help	1
	Independent	2
Bathing	Fully dependent or needs some help	0
	Independent	1
Feeding	Unable or NG/PEG/RIG fed (takes nothing by mouth)	0
	Needs help but can swallow (even if unsafe)	1
	Independent	2
Transfers and mobility	Bedbound, unable to sit	0
	Can sit, but cannot mobilize or transfer without help (from person or walking aid)	1
	Can transfer or mobilize independently or both	2
Stairs	Unable	0
	Needs help	1
	Independent	2
Best verbal response	Mute	0
	Incomprehensible sounds	1
	Single words	2
	Sentences, but difficulty in finding words, uses incorrect words or is often disoriented/confused	3
	Normal conversation	4
Memory and orientation to surroundings	Shows no awareness of surroundings or any evidence of memory	0
	Evidence of retaining some highly learned material (e.g. recognizing familiar people) or awareness of surroundings but no evidence of acquiring new material	1
	Able to retain some new information but memory consistently impaired	2
	Memory normal or some impairment off and on	3
Judgement and problem solving	Unable to show any judgement or problem-solving	0
	Able to show some judgement or problem-solving, even if this is severely impaired	1
Use of tools	Unable to use any tools or objects	0
	Able to use some tools or objects, with help if necessary	1

NG = nasogastric; PEG = percutaneous endoscopic gastrostomy; RIG = radiologically inserted gastrostomy.

MRC Prion Disease Rating Scale

- **CTA-enabling activities in progress** with GLP Toxicology study complete and analysis ongoing

- Clinical study expected to be a **Bayesian Optimal Interval (BOIN) design** to assess safety and efficacy

- Study expected to use the **MRC prion disease rating scale** to assess efficacy of the ZFR and **compare to matched historic controls**

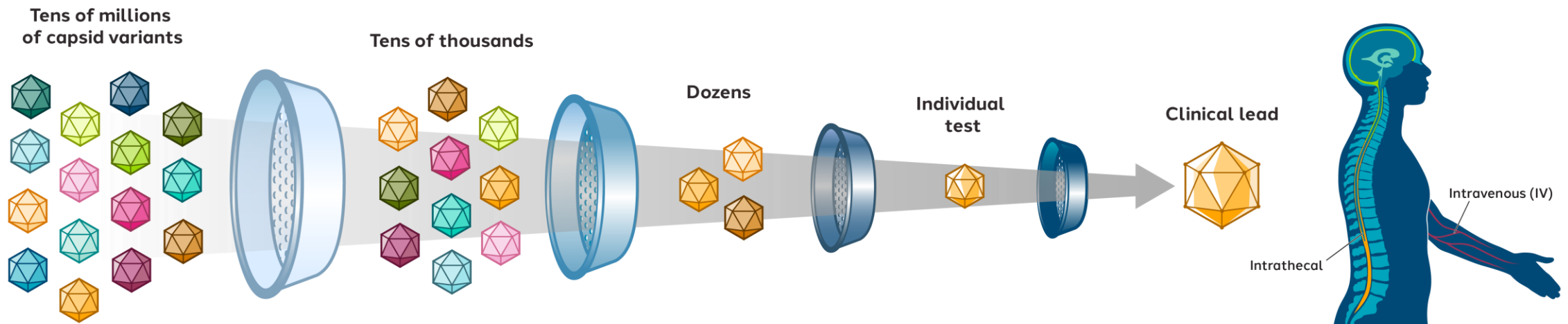
- **Aim is to delay progression of disease**, offering potential for meaningful extension of survival



Achieving Widespread Central Nervous System Delivery for Optimal Therapeutic Benefit

Widespread CNS delivery is challenging with conventional AAVs. Our SIFTER platform is designed to enable the selection of neurotropic AAV capsids to potentially advance our innovative preclinical programs to the clinic.

SIFTER Platform AAV Capsid Engineering



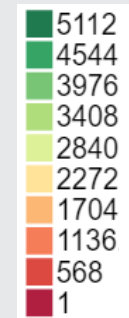
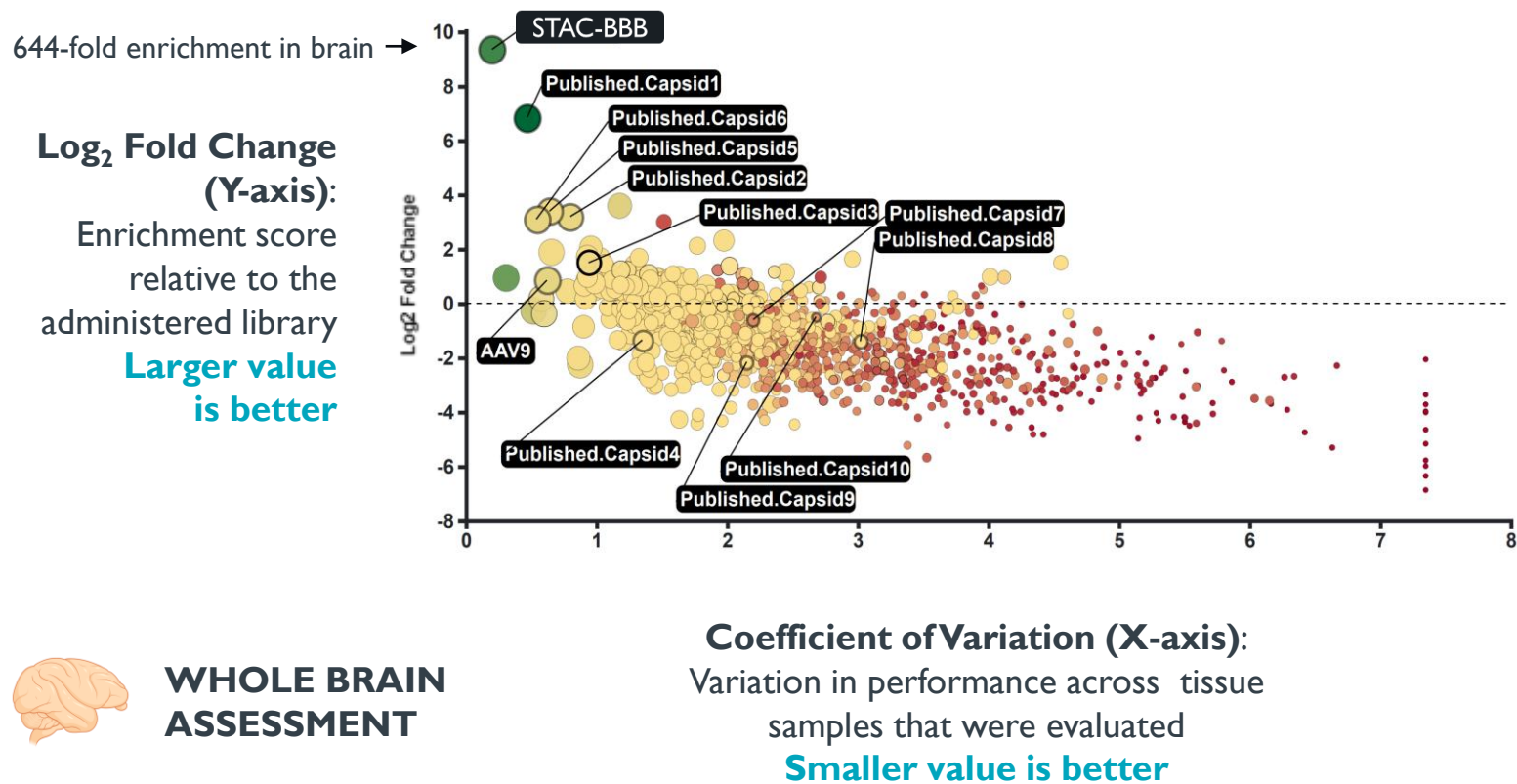
SIFTER: Selecting In vivo For Transduction and Expression of RNA

STAC-BBB findings exceeded expectations for a successful blood-brain barrier penetrant capsid

- ✔ STAC-BBB achieved robust penetration of the blood-brain barrier and widespread distribution throughout the brain in NHPs
- ✔ Industry-leading performance: 700-fold better enrichment than the benchmark AAV9
- ✔ Appears to primarily target neurons regardless of promoter
- ✔ Results are consistent across individual animals and groups
- ✔ Enabled robust expression of zinc-finger cargo throughout the brain, including all key brain regions
- ✔ Vector genomes are enriched in the CNS and appear de-targeted from the DRG and the liver
- ✔ We believe STAC-BBB is manufacturable at scale

In vivo library evaluation in cynomolgus macaques identified STAC-BBB as the top performing BBB-penetrant capsid, with additional enhancements in progress

Capsid-mediated expression of cargo in neurons



Unique Molecular Identifier count (Color):
Informs number of unique AAV transduction events
Darker green is better

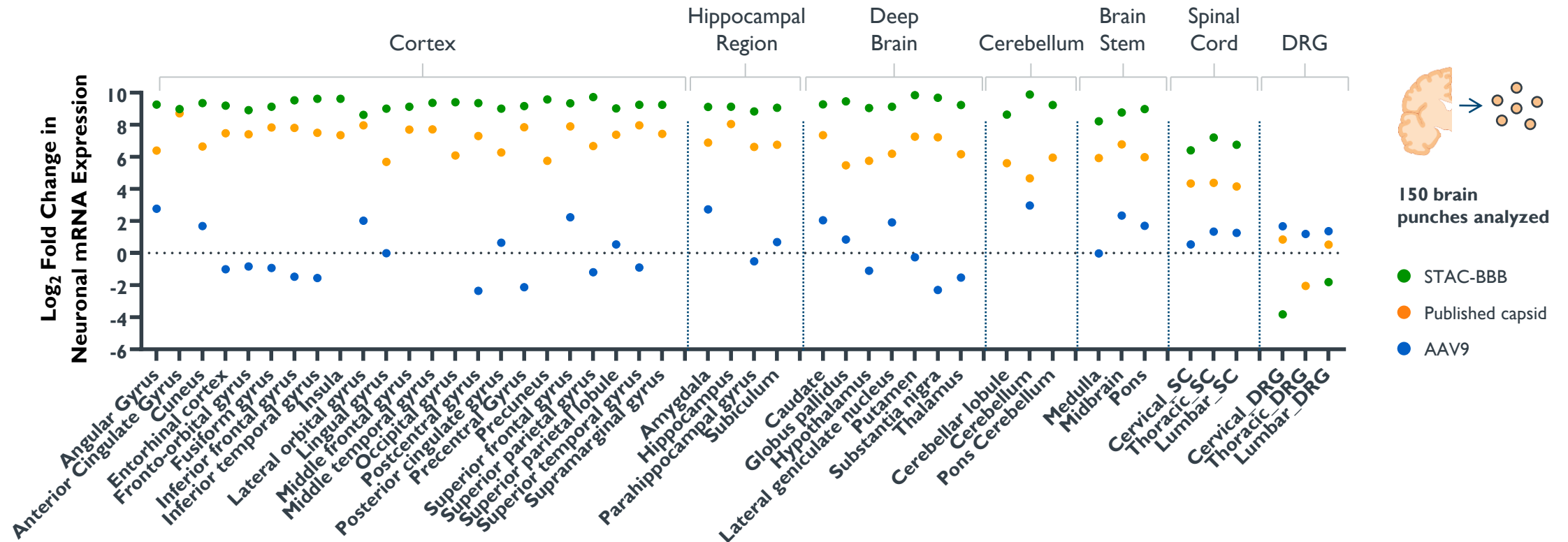


Fraction of replicates found (Bubble size):
Informs consistency of replicate recovery
Larger circle is better

Neuronal RNA expression (3-week study, hSyn1)

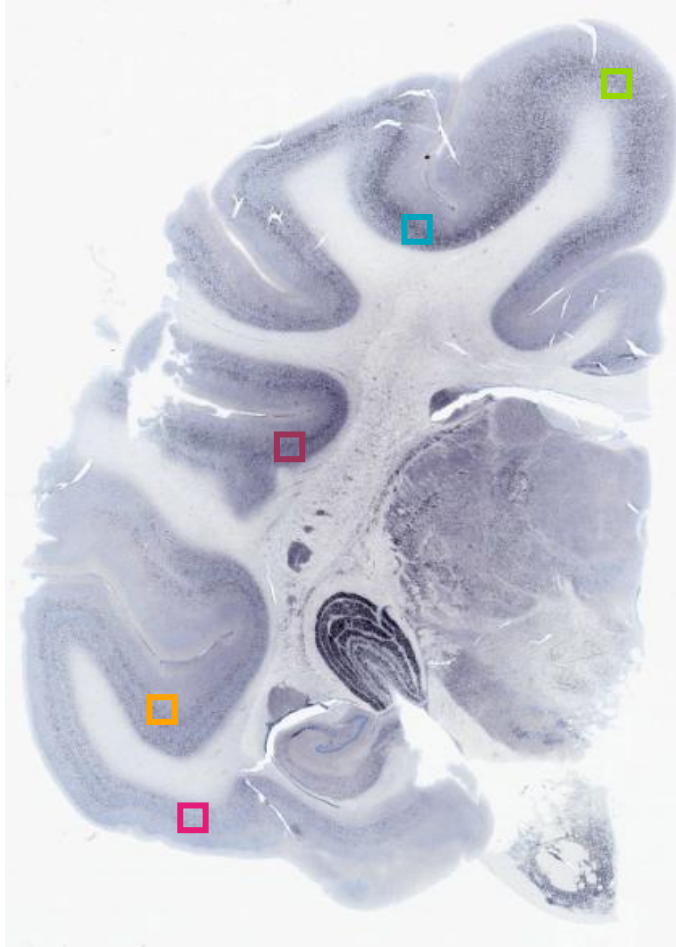
STAC-BBB was enriched in neuronal RNA expression in all CNS regions

Capsid-mediated expression of cargo in neurons

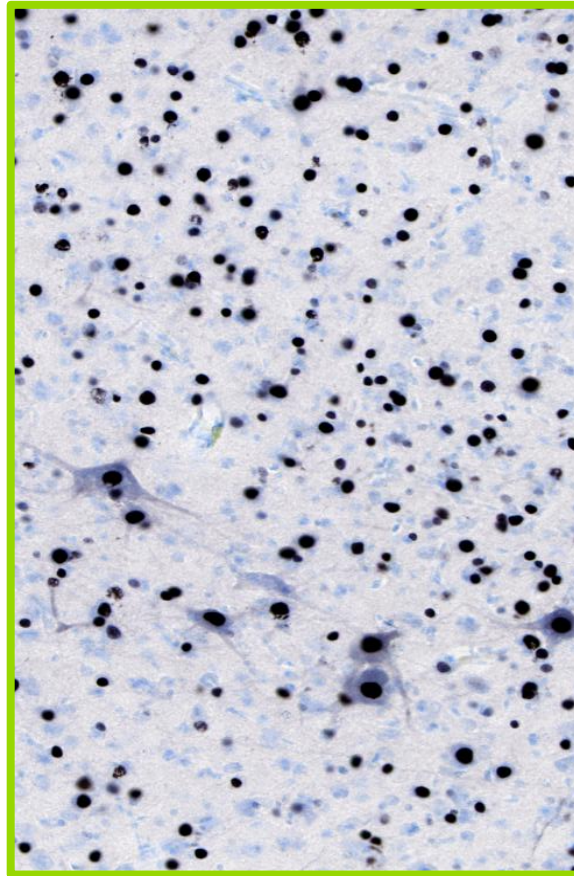


Neuronal RNA expression (3-week study, hSyn I)

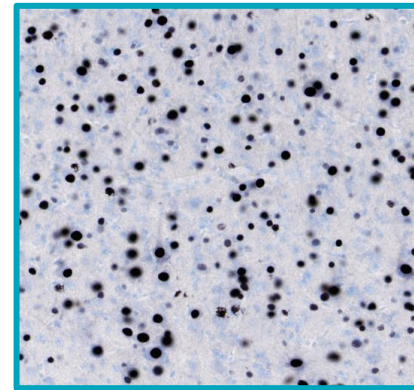
STAC-BBB showed widespread neuronal transduction across all cortical regions



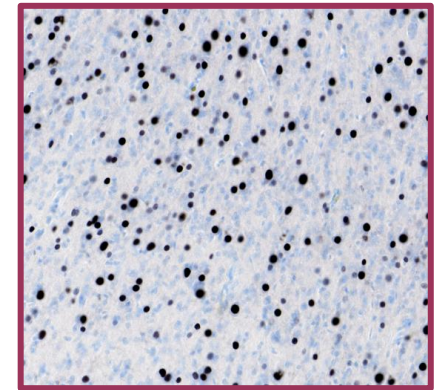
Precentral Gyrus (Motor Cortex)



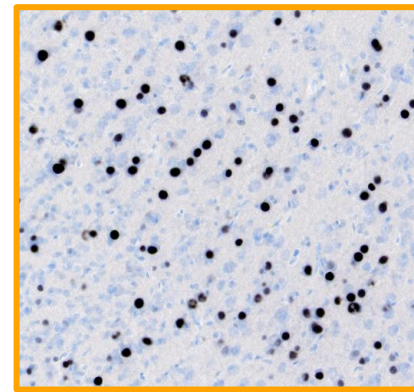
Postcentral Gyrus



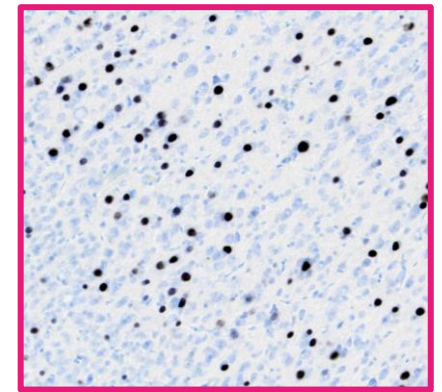
Superior Temporal Gyrus



Middle Temporal Gyrus

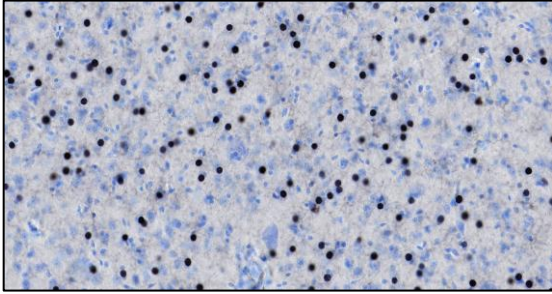


Inferior Temporal Gyrus

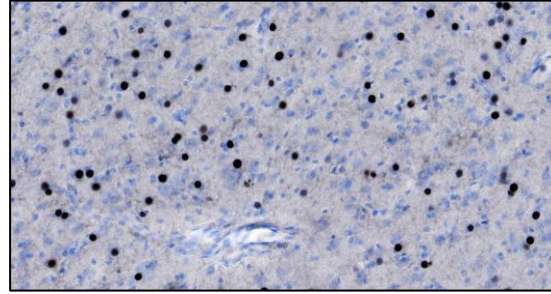


STAC-BBB mediated widespread brain transduction

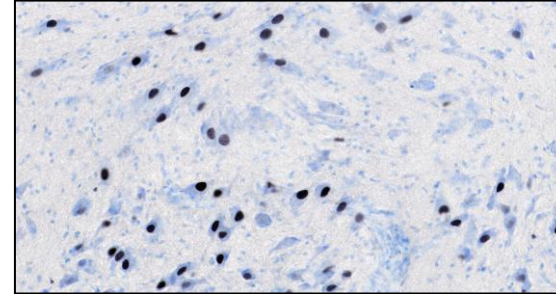
Putamen



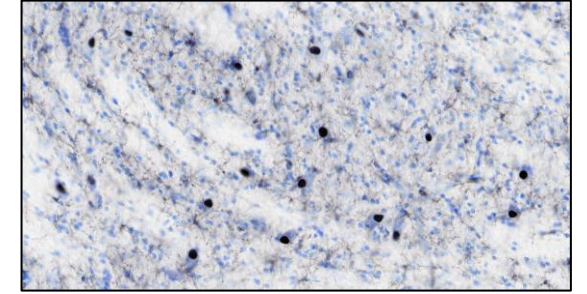
Caudate



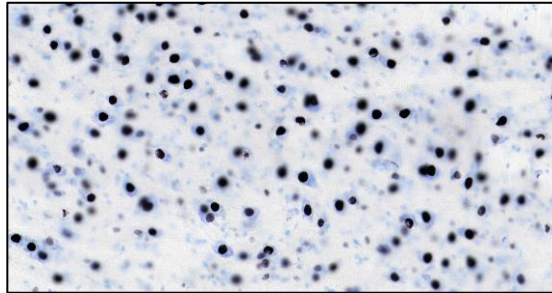
Substantia nigra



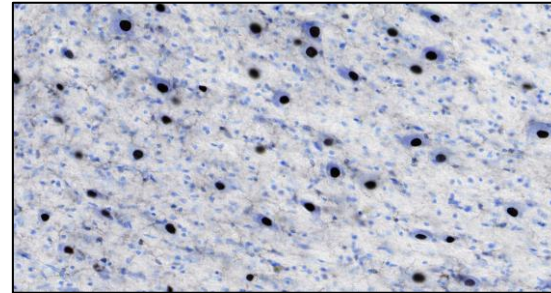
Globus pallidus



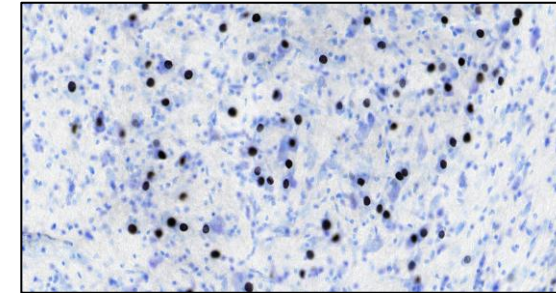
Pons



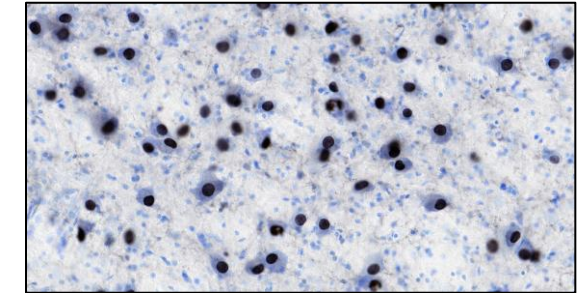
Dentate nucleus



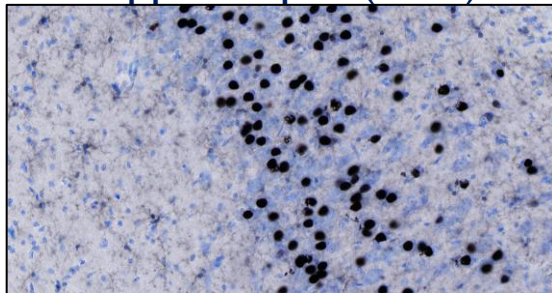
Cuneate nucleus



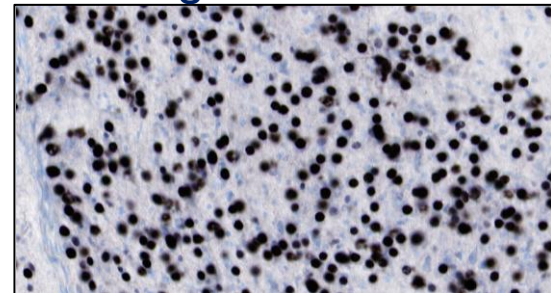
Thalamus



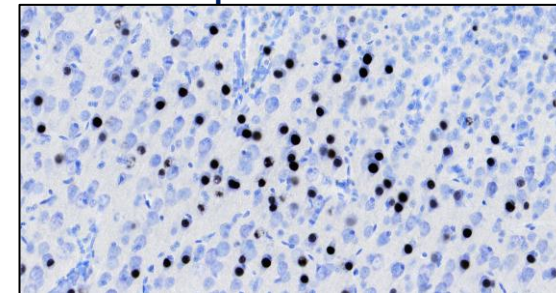
Hippocampus (CA2)



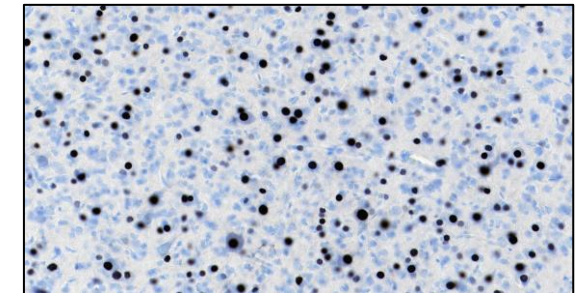
Lateral geniculate nucleus



Temporal cortex



Motor cortex



Neurons were widely transduced in regions integral to disease pathology

Putamen



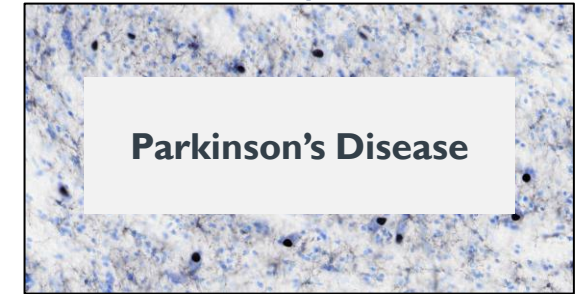
Caudate



Substantia nigra



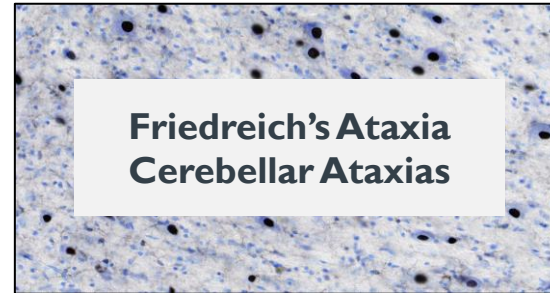
Globus pallidus



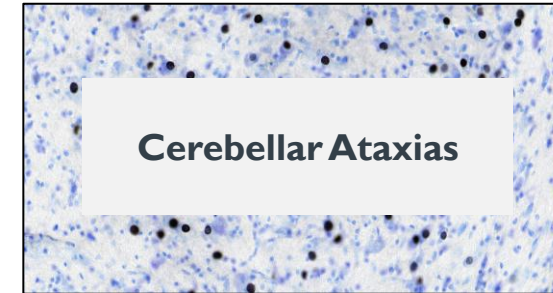
Pons



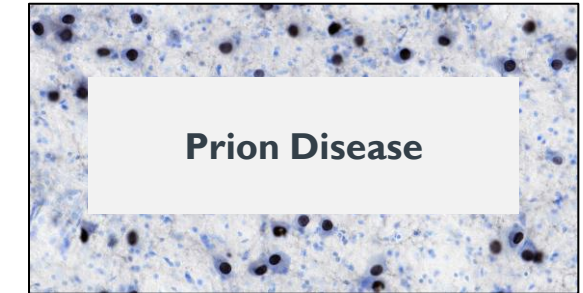
Dentate nucleus



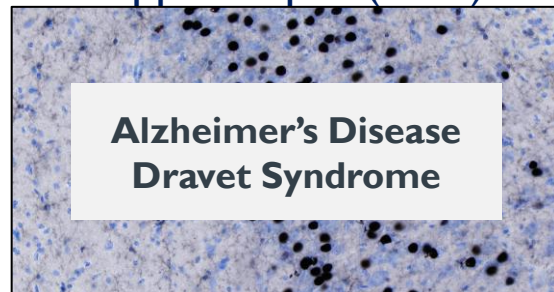
Cuneate nucleus



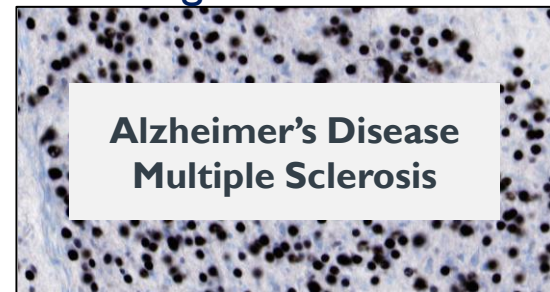
Thalamus



Hippocampus (CA2)



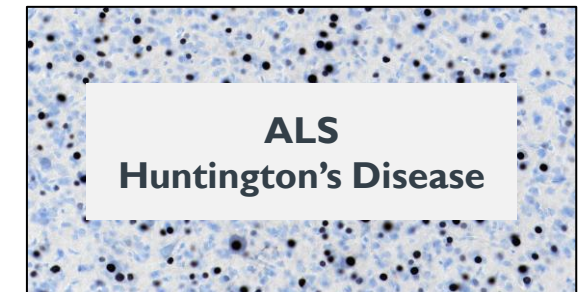
Lateral geniculate nucleus



Temporal cortex




Motor cortex



We believe STAC-BBB is manufacturable at scale

- Capsid manufacturability is critical to create a successful potential commercial drug product for patients
- We believe STAC-BBB is:
 - Manufacturable at commercial scale using standard cell culture and purification processes
 - Soluble using known excipients
 - Can be characterized using available analytics
- We have successfully manufactured up to 50-liter scale, and further scale up to 500-liter is in progress



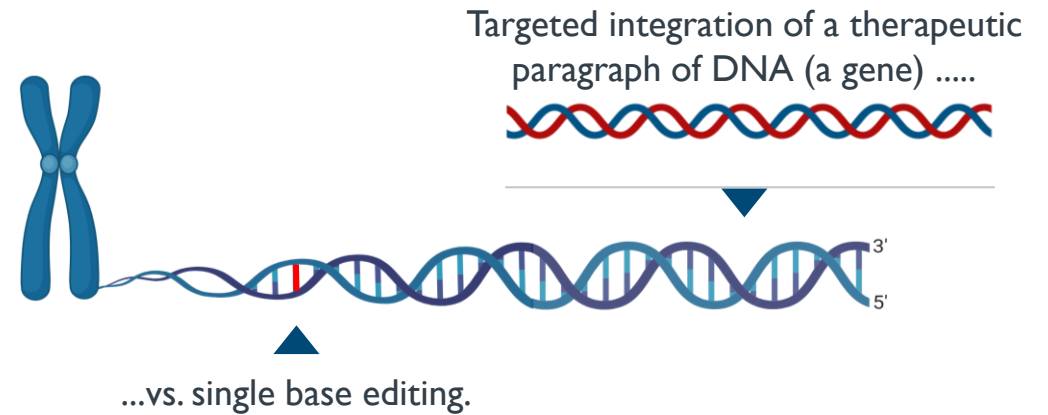


Advancing Next-Generation Genome Engineering

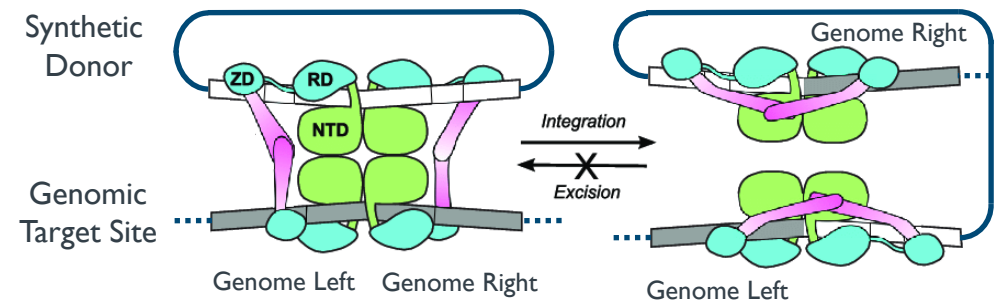
What is an integrase and why is it important?

Targeted integration enables large-scale genome editing

- ✓ Capable of delivering large payloads - 10 kb+
- ✓ No copying required - low error rate
- ✓ Self sufficient - no dependence on cell DNA repair machinery
- ✓ No DNA breaks - reduced translocation risk



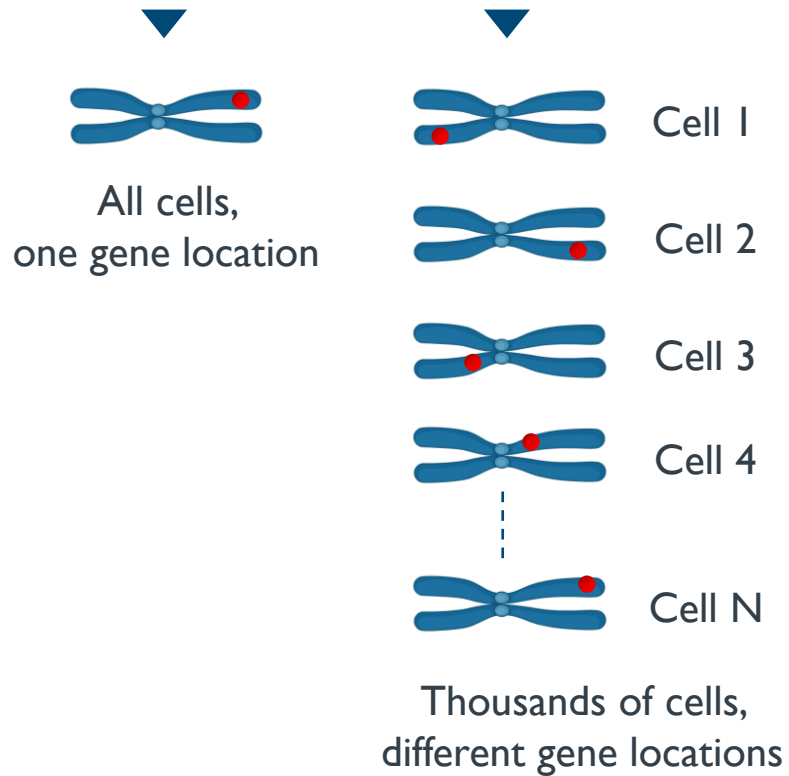
BxbI Integration Mechanism



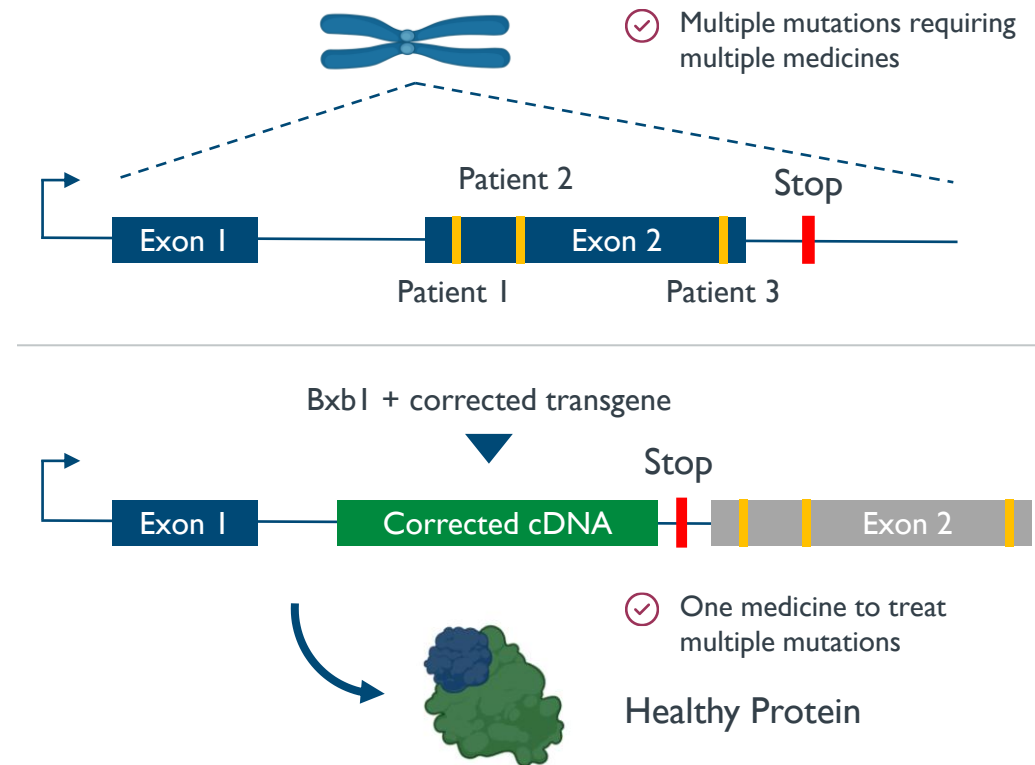
Adapted from Gupta et al., NAR (2017)
doi: 10.1093/nar/gkx474

Targeted integration improves existing therapies, and enables new therapies

Targeted vs. Random Integration



One medicine vs. multiple variants for each mutation



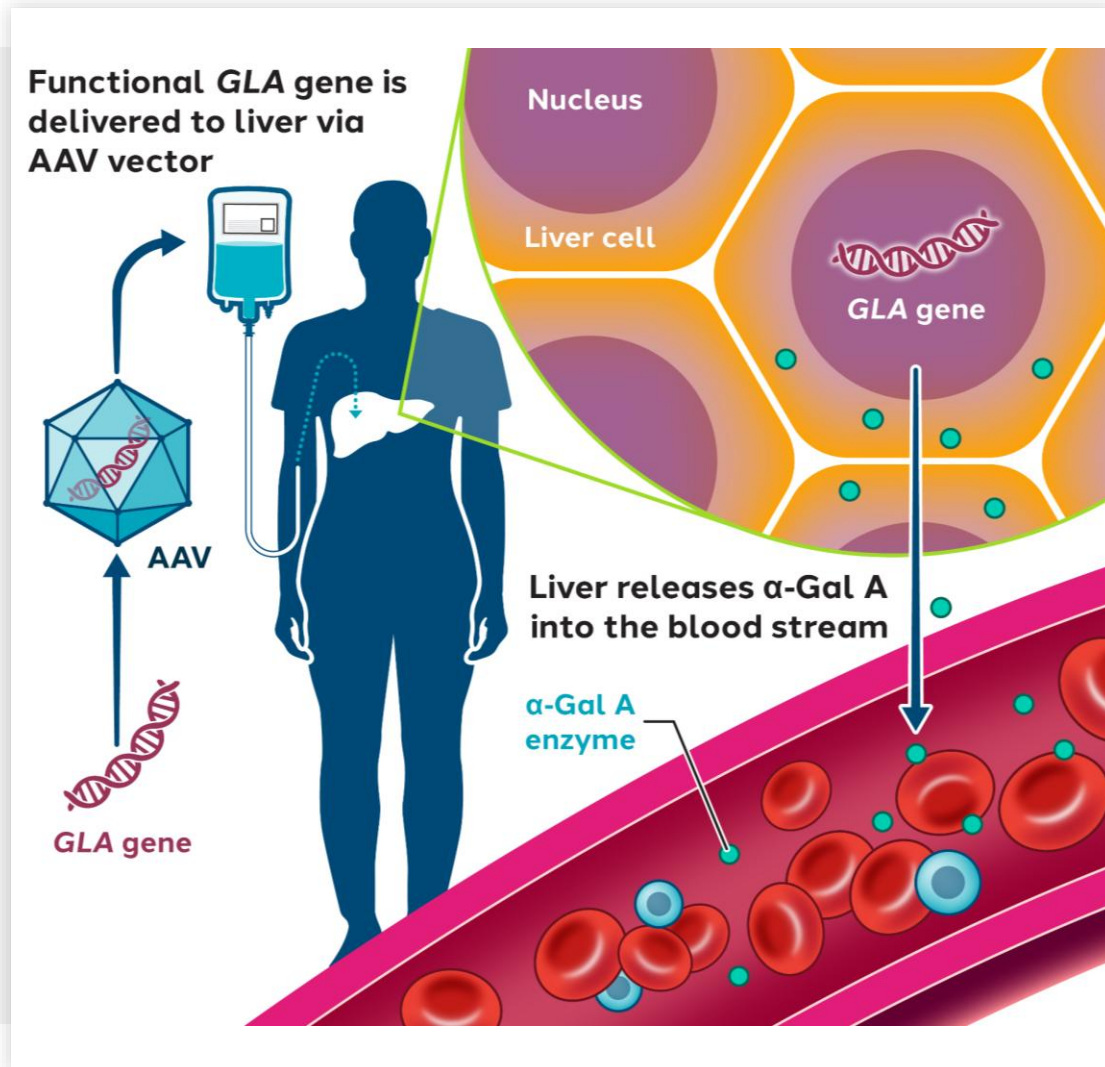
Images by Biorender

Optimizing Value of Clinical Programs



Fabry Disease: Isaralgagene Civaparvovec

Rolling submission of BLA to FDA in progress



> Best-in-class Fabry gene therapy program

- All 32 Phase I/2 STAAR study patients rolled into long-term follow-up

> Compelling clinical data

- Potential to be a one-time, durable treatment to provide meaningful, multi-organ, clinical benefits above current standards of care
- Positive mean annualized eGFR slope observed at 52-weeks
- Stabilization in cardiac function
- Key secondary endpoints also positive
- Favorable safety and tolerability profile, without preconditioning

> FDA alignment on Accelerated Approval pathway

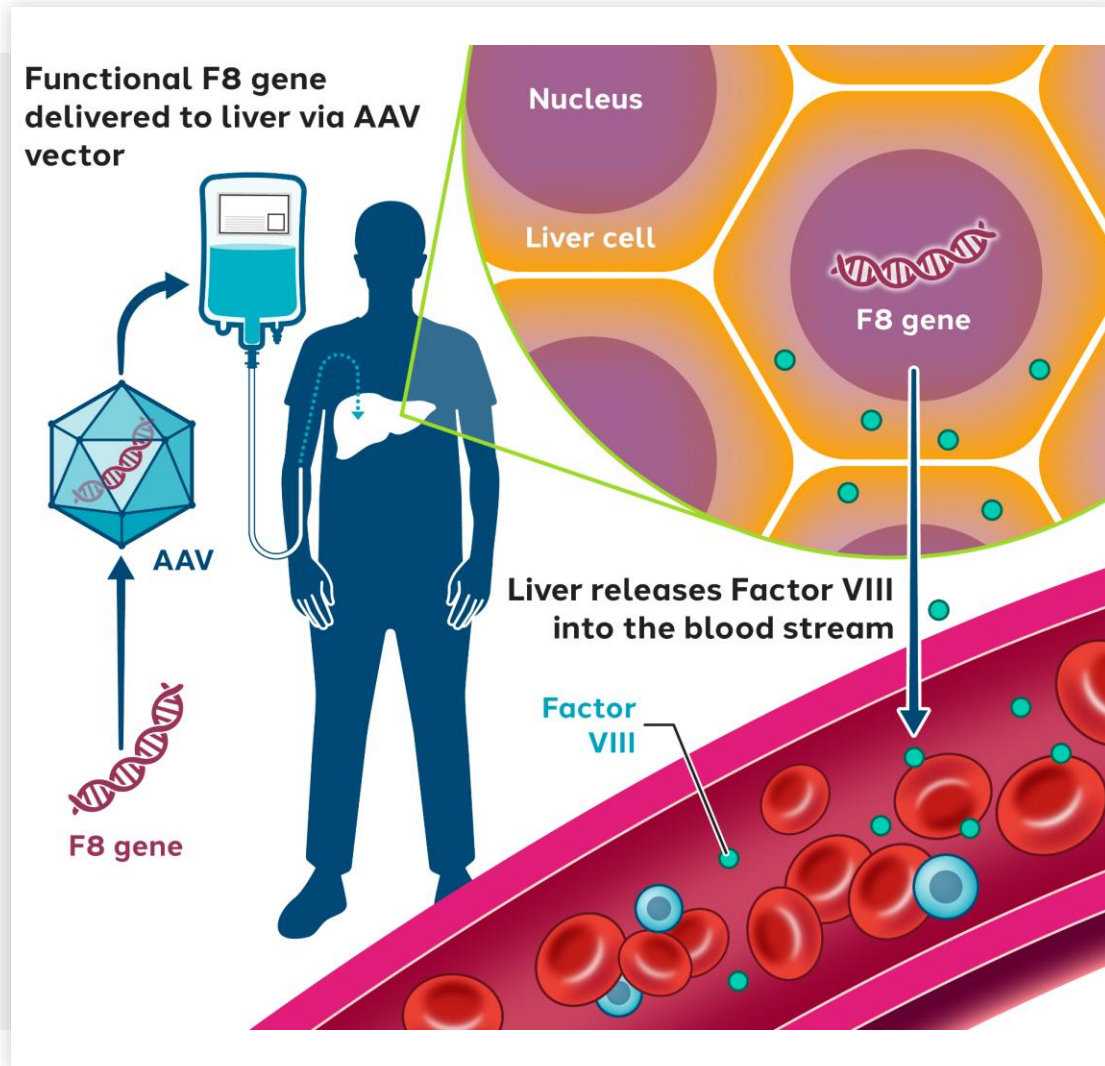
- In October 2025, FDA reiterated its 2024 agreement to use eGFR slope as an endpoint to support Accelerated Approval pathway
- Rolling submission of BLA in progress; expected completion as early as the summer of 2026, subject to adequate additional funding

> ROW regulatory alignment ongoing

- Discussions with EMA on regulatory pathway ongoing
- Has EMA PRIME eligibility and UK MHRA ILAP status

Hemophilia A: Giroctocogene fitelparvovec

Compelling readout for Phase 3 AFFINE trial



- Pfizer reported positive topline results from the Phase 3 AFFINE trial in July 2024, which met primary and key secondary endpoints
- Phase 3 data presented at ASH Annual Meeting and Exposition in December 2024 via platform and poster presentations
- We are in business development negotiations with a potential collaboration partner to commercialize the Hemophilia A program

Sangamo Value Proposition

- ✓ Differentiated genomic medicine platform
- ✓ Focused neurology pipeline
- ✓ Proven clinical execution
- ✓ Clear pipeline expansion opportunities

- **Differentiated genomic medicine platform combines core capabilities required to unlock treatments for devastating neurological diseases**
 - Genome-targeting epigenetic regulation cargo
 - Capsid delivery engine for central nervous system administration
- **Focused neurology pipeline in valuable indications, with best-in-class potential and near-term clinical efficacy data**
 - Chronic neuropathic pain – starting with Small Fiber Neuropathy (SFN)
 - Prion disease
- **Proven clinical execution expertise**
- **Clear neurology pipeline expansion opportunities**
 - SFN program provides pain franchise potential
 - Prion program would unlock STAC-BBB capsid for broader neurology indications
- **Best-in-class Fabry gene therapy with Biologics License Application (BLA) submission in progress**