

## **Corporate Presentation**

August 2021

## **Forward-Looking Statements**

This presentation contains forward-looking statements regarding our 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, ZFP-Epi, CAR-Treg and other technologies to develop safe and effective therapies, the potential for us to benefit and earn milestone and royalty payments from our collaborations and the timing of such benefits and payments, our financial resources and expectations, plans and timelines for opening manufacturing facilities, plans and timelines for us and our collaborators to enroll patients in and conduct clinical trials and present clinical data 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 evolving COVID-19 pandemic and its impact 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 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 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; and the uncertainty of our future capital requirements, financial performance and results. 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 year ended December 31, 2020 as supplemented by our Quarterly Report on Form 10-Q ended June 30, 2021. 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.



## Leading Genomic Medicines into the Clinic

We are a genomic medicines company committed to translating ground-breaking science into medicines that transform the lives of patients with serious disease





Robust Set of Genomic Medicines Addressing Rare Disease, CNS, Oncology and Autoimmune Indications





## **2021 Expected Focus**





## Building Value through Biopharmaceutical Partnerships

~\$815M received in cash			~\$7B in potential milestones		Potential royalty payments		
Centr	ral Nervous System		Hemoglobino	pathies/	Blood Disorders		Oncology
U NOVARTIS	Takeda		Pfizer	S	ANOFI 🎝		
<b>\$75M</b> upfront & up to <b>\$720M</b>	\$I3M upfron	\$82	<b>2M</b> upfront p to <b>\$625M</b>		<b>\$20M</b> upfront & up to <b>\$276M</b>		\$ <b>150M</b> upfront + <b>\$50M</b> in equity
Biogen	\$125M upfront + \$225M in equity purcha & up to \$2.37B			& up t			purchase & up to <b>\$3.01B</b>



## **Proprietary programs**

Fabry Disease – ST-920 Renal Transplant – TX200

## Phase 1/2 Study Evaluating ST-920 in Fabry Disease



#### Entry Criteria

Male and female subjects ≥ 18 years of age with classic Fabry disease

On enzyme replacement therapy (ERT) regimen; or ERT-naïve; or ERT-pseudonaïve and has not received ERT treatment in the prior 6 months

#### Primary Objective

Assess safety and tolerability of ST-920

#### Secondary Objectives

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



First 4 patients dosed



Dose escalate to high dose per SMC recommendation



Now screening Cohort 3 patients

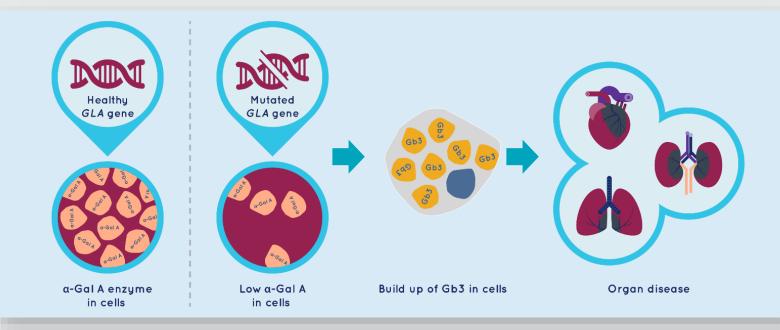
The goal is to provide predictable, durable expression of  $\alpha$ -Gal A at levels which may eliminate the need for enzyme replacement therapy



## ST-920 Offers a Potentially Differentiated Treatment for Fabry Disease

The lack of functioning enzyme results in an excessive accumulation of substrate in the kidney, heart, skin and vessels leading to reduced life expectancy

#### **Fabry Disease**



Our preclinical studies showed strong expression of  $\alpha$ -Gal A which results in a reduction of Gb3 substrate across tissue types



**ST-920** 



One-time IV infusion No preconditioning regimen

May provide continuous, potentially life-long expression of endogenously expressed α-Gal A

**Potential to deliver:** 

Preserved renal function

Reduced cardiac morbidity

Decreased neuropathy



# **Pioneering the CAR-T<sub>REG</sub> Frontier**



## CAR-T<sub>REG</sub>: Pioneering a New Frontier with TX200 for Renal Transplantation





**TX200** Single infusion

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

Therapeutic hypothesis and goals:

Promote immunological tolerance to renal graft

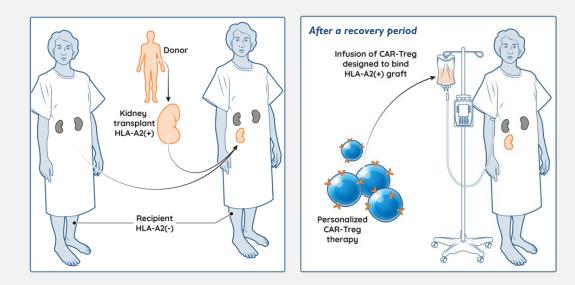
Help preserve graft function and reduce graft loss

Reduce need for systemic immunosuppressive therapy

HLA-A2 Mismatched Renal Transplant

~46,000 renal transplantations expected in 2021 (US + EU)<sup>1</sup>

21-26% of transplanted organs are HLA-A2 mismatched<sup>2</sup>



#### Time from pre-transplant through TX200 administration may be several months



I. IROdat: <u>https://www.irodat.org</u>

## Phase 1/2 Study Evaluating TX200 in Renal Transplantation







First clinical sites initiated

1	وموقوما	

First patient expected to be enrolled by the end of 2021

#### Entry Criteria

Male or female subjects aged 18-70 years, diagnosed with End Stage Renal Disease (ESRD) and waiting for a new kidney from an identified living donor

HLA-A2 mismatch between kidney donor and kidney recipient

#### Primary Objective

Assess safety and tolerability of TX200

#### Secondary Objectives

Assess incidence of acute graft rejection (confirmed by biopsy) and chronic graft rejection

Assess ability of TX200 to reduce need for immunosuppressive therapy up to 84 weeks

**Assess** localization of TX200 cells in the transplanted kidney

**Assess** impact of TX200 on chronic graft-related outcomes

TX200 is designed to help the recipient accept their donated kidney and prevent their immune system from rejecting it, thereby reducing the need for systemic immunosuppressive therapy



TX200 Establishes Potential Foundation for Allogeneic CAR-T<sub>REG</sub> and Major Autoimmune Indications



Allogeneic CAR-T<sub>REGS</sub> may represent a more scalable option for major autoimmune diseases

Autologous CAR-T<sub>REG</sub> clinical research (TX200)

Renal Transplant



#### Allogeneic CAR-T<sub>REG</sub> preclinical research

Renal Transplant Multiple Sclerosis Inflammatory Bowel Disease Potential for allogeneic autoimmune indications

Autoimmune Hepatitis Crohn's Disease Neuromyelitis Optica Rheumatoid Arthritis Systemic Sclerosis Type I Diabetes Mellitus Ulcerative Colitis Vitiligo



## 2021 Clinical Partnered Programs

Pfizer – Hemophilia A – Phase 3 Sanofi – Hemoglobinopathies – Phase 1/2 Giroctocogene Fitelparvovec (SB-525 / PF-07055480) Program Transitioned to Pfizer for Phase 3 Development

## Phase 3 AFFINE Study

Pfizer expects pivotal data readout in 2022; Phase 3 lead in study fully enrolled

Dosed first subject in Oct 2020

Open-label, global, multicenter, single arm study evaluating the efficacy and safety of SB-525 in patients with moderately severe to severe hemophilia A

Primary endpoint is impact on annual bleed rate, or ABR, through 12 months following treatment versus Factor VIII replacement therapy collected in the Phase 3 lead-in study, which will provide a baseline for Phase 3 study participants

Participants will be analyzed throughout the 5-year study period following the single infusion to further assess durability and efficacy

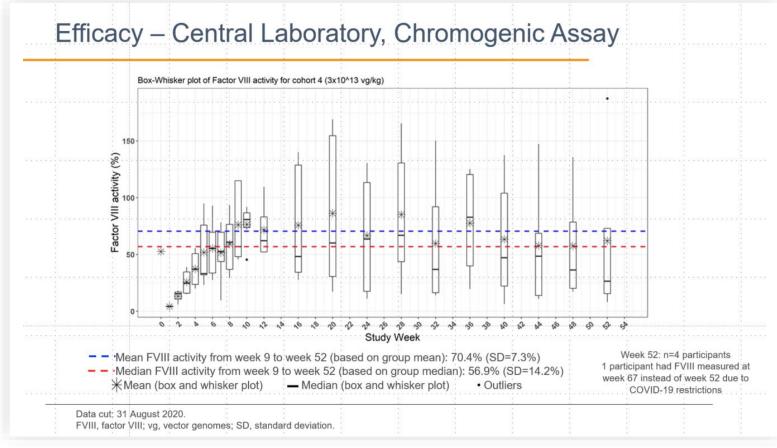








### Phase 1/2 Alta Study of Giroctocogene Fitelparvovec (SB-525 / PF-07055480) Gene Therapy for Hemophilia A - Efficacy, Cohort 4 (3e13 vg/kg)



## Updated Alta Study Results Presented by Pfizer at ASH 2020 on Dec. 7, 2020



## Durable FVIII expression through one year

Steady state FVIII activity achieved by Week 9

#### FVIII activity from Week 9 to 52:

- Median = 56.9%
- Mean = 70.4%

FVIII activity from Week 9 to longest available follow up (Week 82 for patient 7):

- Median = 50.2%
- Mean = 63%

No bleeds in the first year; one treated target joint bleed was reported during the 2nd year following vector infusion Phase 1/2 Alta Study of Giroctocogene Fitelparvovec (SB-525 / PF-07055480) Gene Therapy for Hemophilia A - Safety

#### Treatment-Related Adverse Events

MedDRA Preferred Term	Cohort 2 2e12 vg/kg (n=2)		Cohort 4 3e13 vg/kg (n=5)		All Participants (N=11)	
	Subjects, n (%)	No. of Events	Subjects, n (%)	No. of Events	Subjects, n (%)	No. of Events
Any treatment-related event	2 (100.0)	5	4 (80.0)	21	6 (54.4)	26
ALT increased <sup>a</sup>	2 (100.0)	3	3 (60.0)	10	5 (45.5)	13
Pyrexia			3 (60.0)	3	3 (27.3)	3
AST increased	1 (50.0)	2	2 (40.0)	3	3 (27.3)	5
Tachycardia			2 (40.0)	2	2 (18.2)	2
Fatigue			1 (20.0)	1	1 (9.1)	1
Hypotension			1 (20.0)	1	1 (9.1)	1
Myalgia			1 (20.0)	1	1 (9.1)	1

No treatment-related AEs for participants in cohorts 1 and 3

<sup>a</sup>One participant had an ALT increase per central lab results that the Investigator has not reported increase as an adverse event. Data cut: 31 August 2020. AE, adverse event; ALT, alanine transaminase; AST, aspartate aminotransferase; vg, vector genomes.

#### Updated Alta Study Results Presented by Pfizer at ASH 2020 on December 7, 2020

Giroctocogene fitelparvovec was generally well tolerated

Treatment-related SAEs occurred in 1 participant in cohort 4 (grade 3 hypotension and grade 2 fever ≈6 hours after completion of the vector infusion); resolved ≈12 hours post infusion

4 of 5 cohort 4 participants required corticosteroid treatment for ALT/AST elevation; all resolved with intervention

As of 52 weeks, no corticosteroid use has been required



## Hemoglobinopathies Collaboration with Sanofi

#### Phase I/2 PRECIZN-I Study in sickle cell disease

Sanofi continuing to screen/enroll subjects into the Phase I/2 PRECIZN-I clinical trial evaluating SAR445136 (formerly BIVV003), ex vivo gene-edited cell therapy product candidate for sickle cell disease

- FDA granted Fast Track designation
- EMA granted Orphan Designation based in part on early data
- Sanofi expects to present initial data at a medical meeting in 2021

#### Phase I/2 Thales Study

Five subjects dosed in Sangamo's Thales study evaluating ST-400 for transfusion dependent beta thalassemia

- No additional subjects to be enrolled until data from PRECIZN-1 and Thales have been collected and analyzed
- Follow-up data expected in 2021 from the Thales study



Sangame





## Innovation in Neurological Disease

## Sangamo ZFP Technology: Potential to Access Hundreds of Genomic Targets in CNS

Sangame

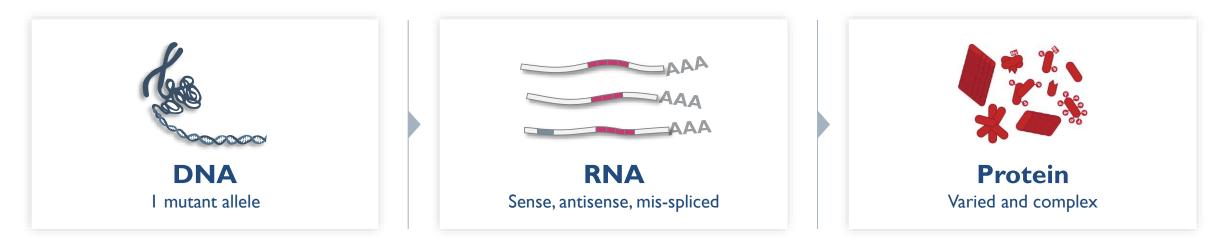
ZFP-TF genome regula	Example target		
Pan-Allele	ZFP-TFs for single gene repression and activation	<ul> <li>Tauopathies</li> <li>α-Synuclein</li> <li>Autism Spectrum Disorder</li> </ul>	
Allele-Selective	ZFPs target disease allele repeats selectively	<ul><li>Huntington's Disease</li><li>C9ORF72-linked ALS</li></ul>	
Epigenetic Editing	<b>ZFP-Epi to demethylate</b> select sites	<ul><li> Rett Syndrome</li><li> Fragile X</li></ul>	
ZFN genome editing		Example target	
Inflammation	<b>CAR-T<sub>REGS</sub> for remyelination and inhibition of neuroinflammation</b>	<ul><li>Multiple Sclerosis</li><li>ALS</li></ul>	
Mitochondrial	<b>ZFNs for selective clearance of mutant mitochondrial genomes</b>	<ul><li>Cerebellar Ataxia</li><li>Leigh Syndrome</li></ul>	



## ZFP-TFs Target Upstream at the Source of Mutant Protein Isoforms and Complexes

Sangame







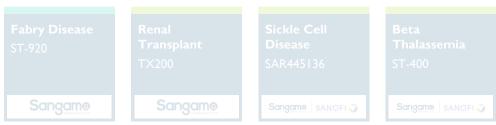
Hill et al., 2003 Jucker & Walker 2013 Irwin et al., 2015 Waldvogel et al., 2014

### Robust Pipeline of Genomic Medicines in Neurological Diseases

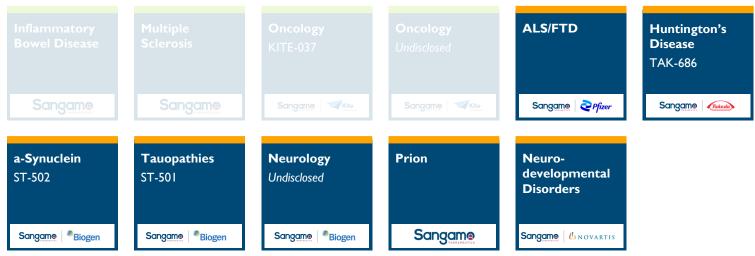
#### Phase 3



#### Phase 1/2



#### Preclinical





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## Key Points in Sangamo's 2021 Story

## Executing on Clinical-stage Pipeline

#### Phase 3 2021 Clinical Program



#### Phase 1/2 2021 Clinical Programs







Investing in Manufacturing to Provide Greater Capacity, Flexibility and Control

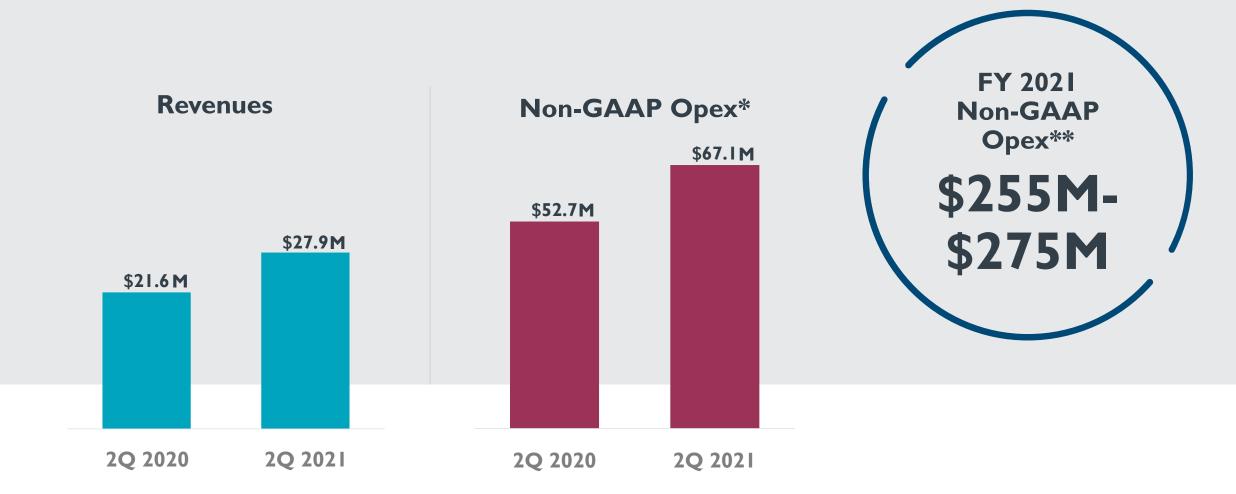
## **Operational Capabilities**

In-house Phase I/2 cGMP facilities – increased control and flexibility of our processes, quality, supply and timeline

- Brisbane, USA
  - Gene therapy (operational as of end of 2020)
  - Cell therapy (projected 2021)
- Valbonne, France
  - Cell therapy (projected 2021)

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

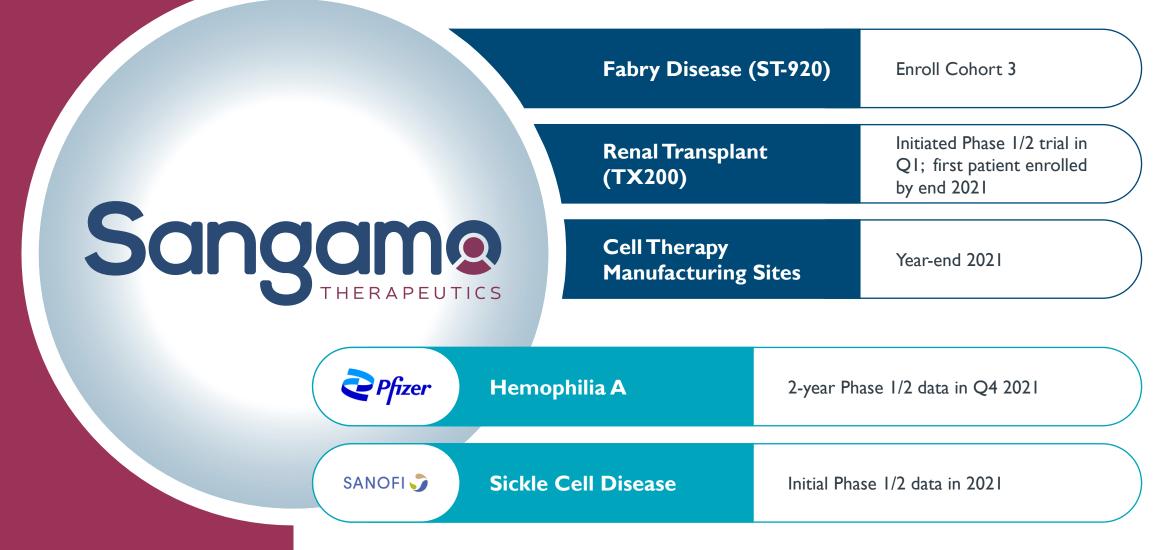
## 2Q 2021 Financial Results; 2021 Guidance



\* GAAP total operating expenses were \$76.6 million for 2Q 2021, compared to \$59.4 million for 2Q 2020 and included stock-based compensation expense ("SBC") of \$9.5 million and \$6.7 million, respectively \*\* On a GAAP basis we expect our 2021 operating expenses to be in the range of \$285 - \$305 million including anticipated SBC of approximately \$30 million



## Anticipated 2021 Catalysts



## Key Takeaways



Broad portfolio with prioritized candidates in both rare and large indications



Technology validating biopharma partners



Strong balance sheet, cash of approximately \$579M (as of Jun 30, 2021)



In-house cGMP facility provides manufacturing capacity, flexibility and control of our processes



Genomic medicine company executing on a clinical stage pipeline

