



saniona™

Improving the lives
of rare disease patients
through scientific
innovation

Forward-Looking Statements

This presentation contains forward-looking statements that provide Saniona's expectations or forecasts of future events such as new product developments, regulatory approvals and financial performance. Such forward-looking statements are subject to risks, uncertainties and may be impacted by inaccurate assumptions. This may cause actual results to differ materially from expectations and it may cause any or all of Saniona's forward-looking statements here or in other publications to be wrong. Factors that may affect future results include currency exchange rate fluctuations, delay or failure of development projects, loss or expiry of patents, production problems, breaches or terminations of contracts, government-mandated or market-driven price decreases, introduction of competing products, exposure to product liability claims and other lawsuits, changes in reimbursement rules, changes of laws and/or regulations or interpretation thereof, and unexpected cost increases. Saniona undertakes no obligation to update forward-looking statements.

Saniona Investment Highlights

Clinical-stage biopharmaceutical company focused on discovering, developing, and commercializing innovative therapies for rare disease patients

Shares listed on Nasdaq Stockholm Small Cap (OMX: SANION). Research team in Copenhagen, Denmark. Corporate office in Boston area.

1 Tesomet: positive data from initial Phase 2 trials in two rare disorders

Hypothalamic obesity (HO)

Phase 2b trial expected to begin H2 2021; top-line data expected in H2 2023

Prader-Willi syndrome (PWS)

Phase 2b trial expected to begin H2 2021; top-line data expected in H1 2023

2 Proprietary ion-channel drug discovery engine driving pipeline

SAN711

For rare neuropathic disorders, Phase 1 data expected in H1 2022

SAN903

For rare inflammatory, fibrotic, and hematological disorders, expected to enter Phase 1 in H2 2022

IONBASE Database

20,000 proprietary ion channel modulators

3 Validation from multiple strategic partnerships

CAD-1883

for movement disorders



Novel target

for schizophrenia



Tesofensine

for obesity



4 Robust financing activity to drive current operating plan

Well-funded into H2 2022

Strong institutional support

RA Capital, Pontifax Venture Capital, New Leaf Venture Partners

Saniona Executive Team



Rami Levin, MBA
President &
Chief Executive Officer



Jason Amello
Chief Financial Officer



Linea Aspesi
Chief Human
Resources Officer



Rudolf Baumgartner, MD
Chief Medical Officer &
Head of Clinical Development



Jørgen Drejer, PhD
Chief Scientific Officer



Wendy Dwyer
Chief Business Officer



Kyle Haraldsen
Chief Technical
Operations Officer



Trista Morrison
Chief Communications
Officer

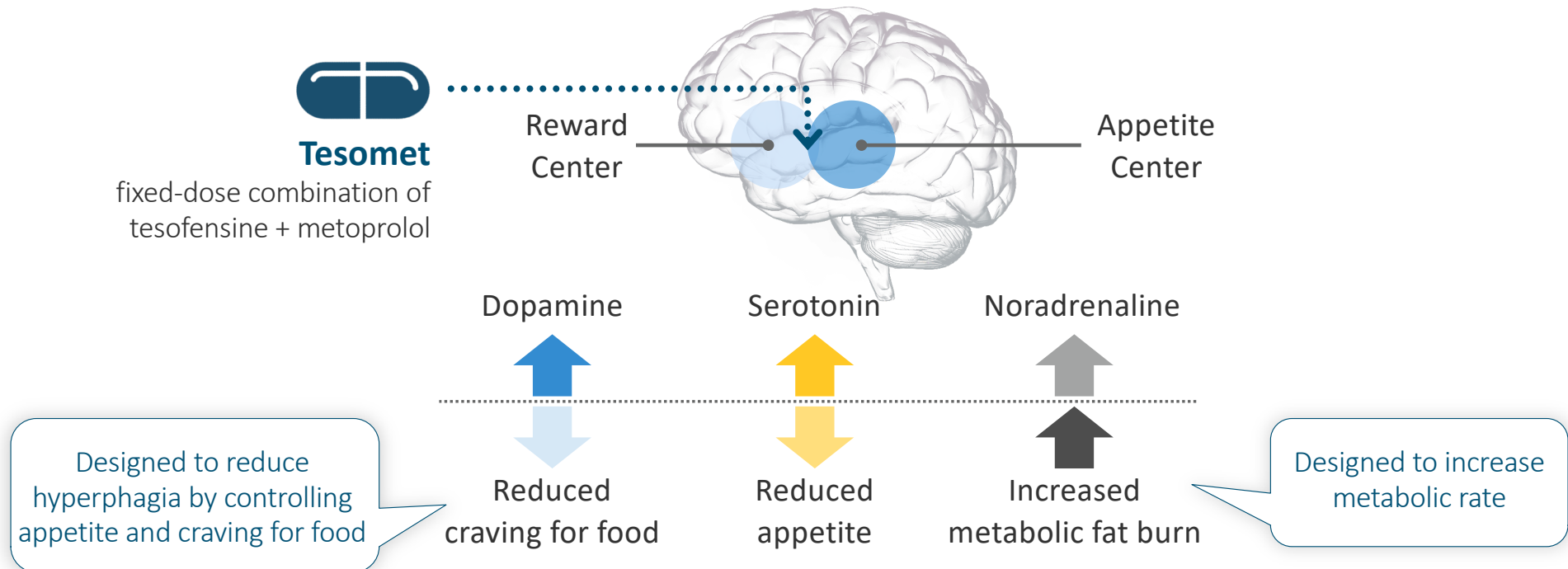


Denelle Waynick
Chief Legal Officer

Tesomet: A Potentially First-in-Class Triple Monoamine Re-Uptake Inhibitor/Beta-1 Blocker

Target indications:

Hypothalamic obesity (HO) and Prader-Willi syndrome (PWS), both rare diseases





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Tesomet in Hypothalamic Obesity (HO)



Jesse, living with hypothalamic obesity

Hypothalamic Obesity, an Acquired Rare Disease

HO

PATIENT POPULATION	10,000-25,000 in the U.S 16,000-40,000 in Europe ^{1,2,3}
CAUSE	Most commonly caused by damage to the hypothalamus sustained during the removal of a craniopharyngioma
DISEASE CHARACTERISTICS	Rapid, excessive and intractable weight gain post hypothalamic injury Hyperphagia (uncontrollable hunger) Memory impairment, attention deficit, lethargy and impulse control issues
CURRENT TREATMENT	No FDA approved therapies



Allie, living with HO, before and after surgery to remove her craniopharyngioma

¹ Bunin *et al.* The descriptive epidemiology of craniopharyngioma. *J Neurosurg*, **89** 547-551 (1998). doi:10.3171/jns.1998.89.4.0547

² Zacharia *et al.* Incidence, treatment and survival of patients with craniopharyngioma in the surveillance, epidemiology and end results program. *Neuro-Oncology*, **14** 1070-1078. (2012). doi:10.1093/neuonc/nos142

³ NIH GARD: <https://rarediseases.info.nih.gov/diseases/6463/hypothalamic-obesity>

Initial Phase 2 Trial in HO Achieved Primary and Several Secondary Endpoints

6.28%

Reduction in body weight vs. placebo

Statistically significant ($p=0.0169$) in 24-week double-blind period. Reduction maintained (5.96%) in 24-week open-label extension.

Over

61%

Patients with $\geq 5\%$ body weight loss

Statistically significant ($p=0.0461$) in 24-week double-blind period. Data indicate Tesomet showed potential to meet FDA Guidance for weight management.

49%

Lowering in HbA1c

The two Type 2 diabetic patients with HO receiving Tesomet showed 48.80% reduction in HbA1c at 24 weeks, versus no change in normoglycemic patients with HO.

Tesomet was generally well tolerated

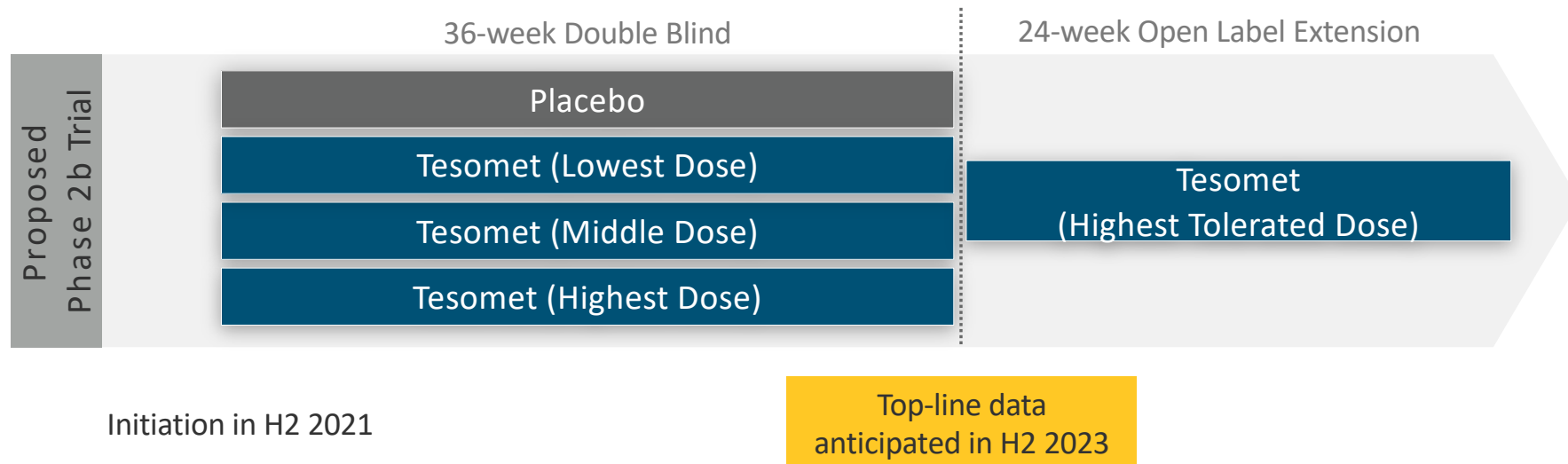
The majority of adverse events were mild or moderate in severity. There were no significant differences in heart rate or blood pressure between treatment groups.

Proposed Phase 2b Clinical Trial in Hypothalamic Obesity (HO)

HO

PROPOSED TRIAL OVERVIEW

- Double-blind, randomized, placebo-controlled, multi-center trial
- Subjects (n≈110) will be randomized 1:1:1:1
- Sites planned in USA and outside the USA
- Primary endpoint will be change in body weight





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Tesomet in
Prader-Willi Syndrome
(PWS)



John, living with Prader-Willi syndrome

Prader-Willi Syndrome, a Debilitating, Rare Genetic Disorder

PWS

PATIENT POPULATION	11,000-34,000 in the U.S 17,000-50,000 in Europe ^{1,2}
CAUSE	Absent or defective paternally expressed genes at Chromosome 15 (q11.2-q13)
DISEASE CHARACTERISTICS	Hyperphagia (uncontrollable hunger) Abnormal growth + body composition, low muscle tone Social, emotional and cognitive deficits Obesity-related comorbidities Significant burden on families and caregivers
CURRENT TREATMENTS	No FDA approved therapies for the treatment of hyperphagia



John, Erik and Erin, who live in a home for adults with PWS

¹ Manzardo et al. Survival trends from the Prader–Willi Syndrome Association (USA) 40-year mortality survey, Genet Med 20, 24–30 (2018) doi:10.1038/gim.2017.92

² National organization of Rare Diseases: <https://rarediseases.org/rare-diseases/prader-willi-syndrome/>

Initial Phase 2 Trial in PWS Showed Reductions in Body Weight, Hyperphagia

5.4%

Reduction in body weight (adults)

Clinically meaningful per FDA benchmarks for weight loss trials (data from adult patients in 12-week double-blind study).

-8.1 Points

Reduction in hyperphagia (adults)

Statistically significant ($p=0.0058$) reduction (in adults in 12-week double-blind study).

Reduction in hyperphagia and body weight (adolescents)

Dose-dependent improvements in hyperphagia and body weight (in adolescents in 2nd open-label extension)

Tesomet was generally well tolerated

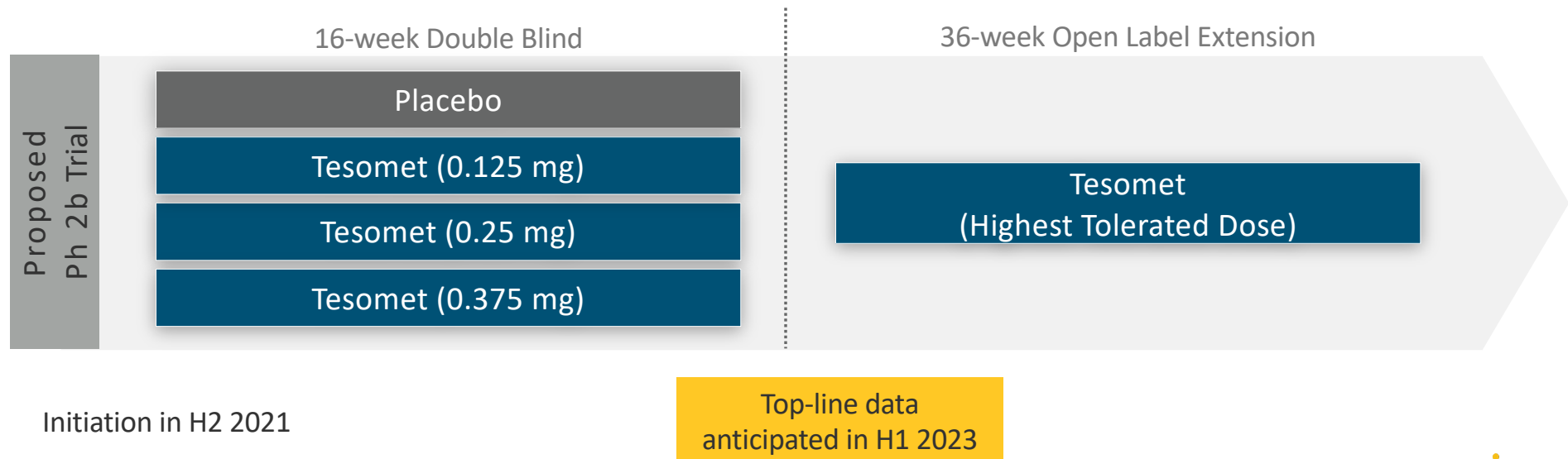
The majority of adverse events were mild or moderate in severity. There were no significant findings in heart rate, blood pressure, ECG or safety laboratory parameters.

Proposed Phase 2b Clinical Trial in Prader-Willi Syndrome (PWS)

PWS

PROPOSED CLINICAL TRIAL OVERVIEW

- Double-blind, randomized, placebo-controlled, multi-center clinical trial
- Subjects will be randomized 1:1:1:1 (n≈120 subjects)
- Sites planned in USA, Canada, EU, UK, Australia, New Zealand, Brazil
- Primary endpoint will be change in hyperphagia scores, as measured by the HQ-CT questionnaire





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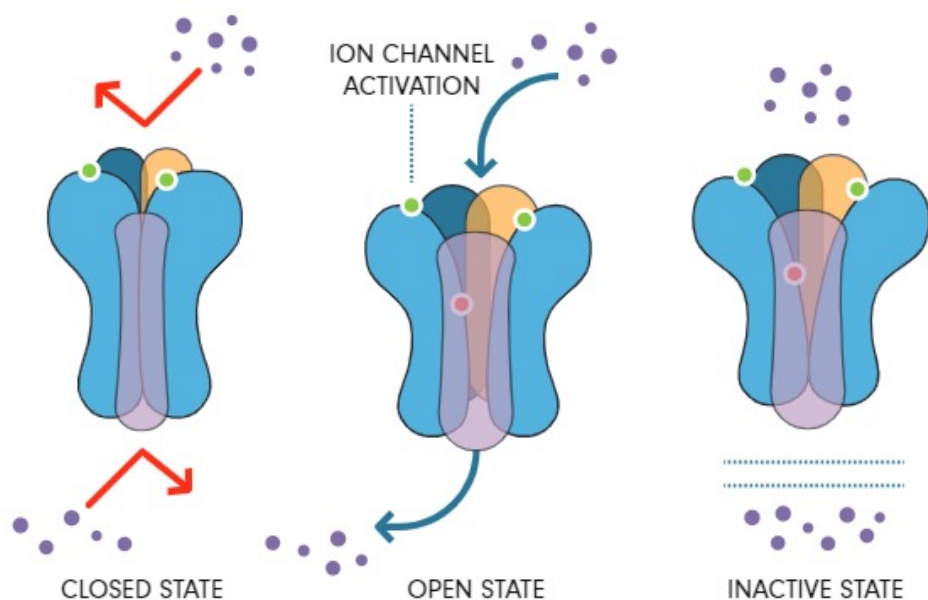
Saniona's Ion Channel Drug Discovery Engine



Saniona Ion Channel Drug Discovery Engine

Ion Channels = scientifically validated yet significantly untapped

Ion channel modulating drugs = \$11.1B industry, yet only 20% of ion channels commercially available as therapeutics.

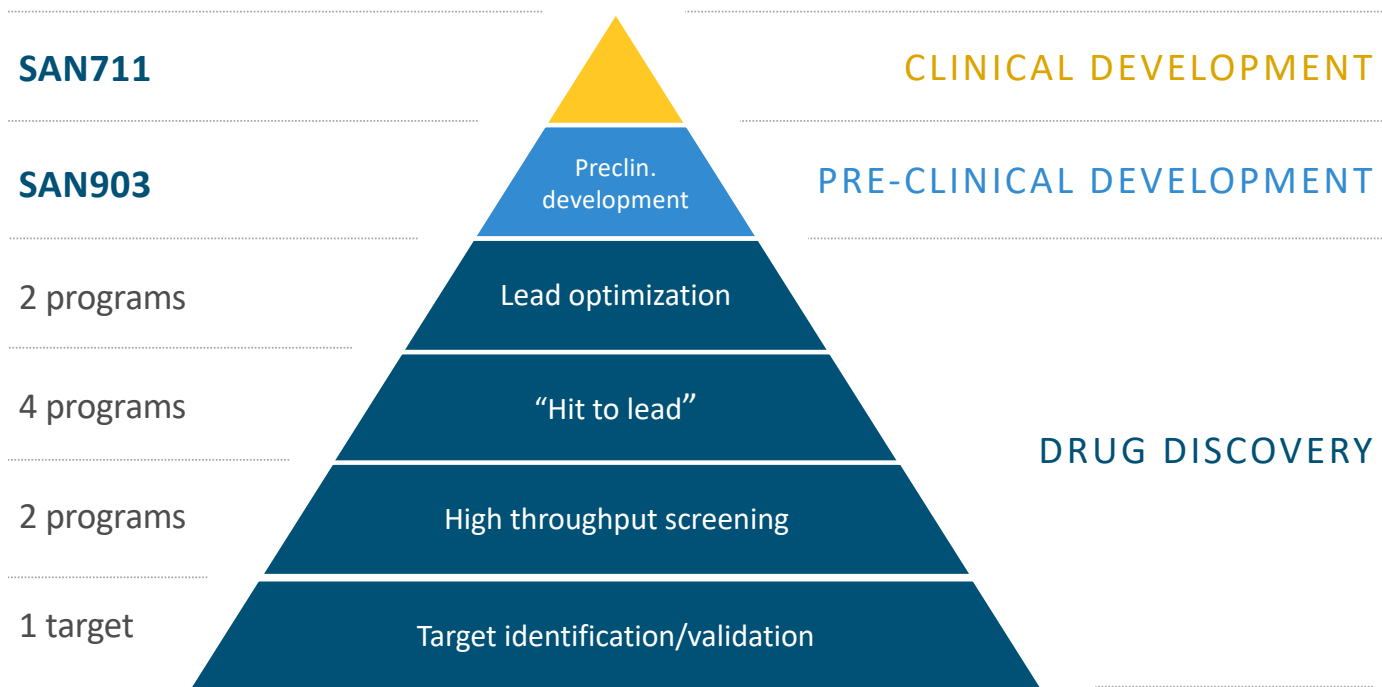


Saniona's foundation for success:

- Library of 20,000+ **proprietary** compounds generated over 20+ years
- Integrated **proprietary** IONBASE™ database with accumulated chemical-biological data for 130,000+ chemical entities
- Program has **generated SAN711 and SAN903**; multiple additional programs in discovery stage
- Positioned to advance multiple **new drug candidates**

Saniona Drug Discovery Engine Generates Continual Pipeline

Saniona expects the ion channel drug discovery engine to deliver a continual stream of new drug candidates





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SAN711 for neuropathic
disorders

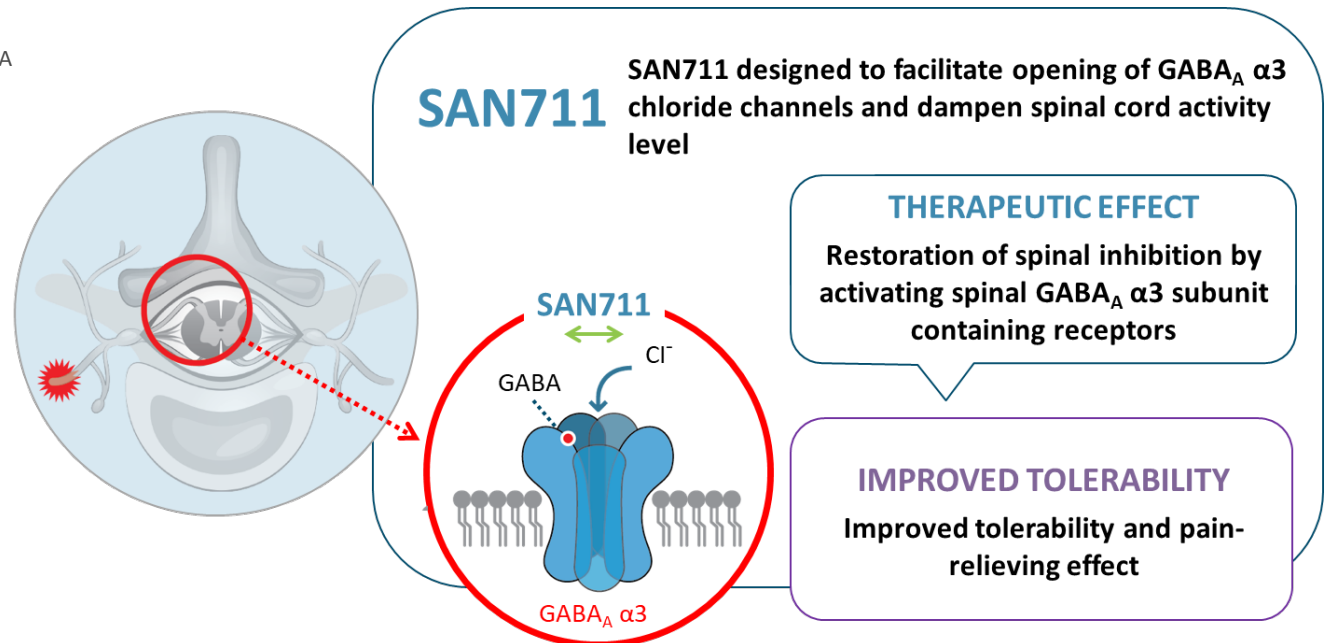


SAN711: Potentially First-in-Class Positive Allosteric Modulator of GABA_A α3 Receptors

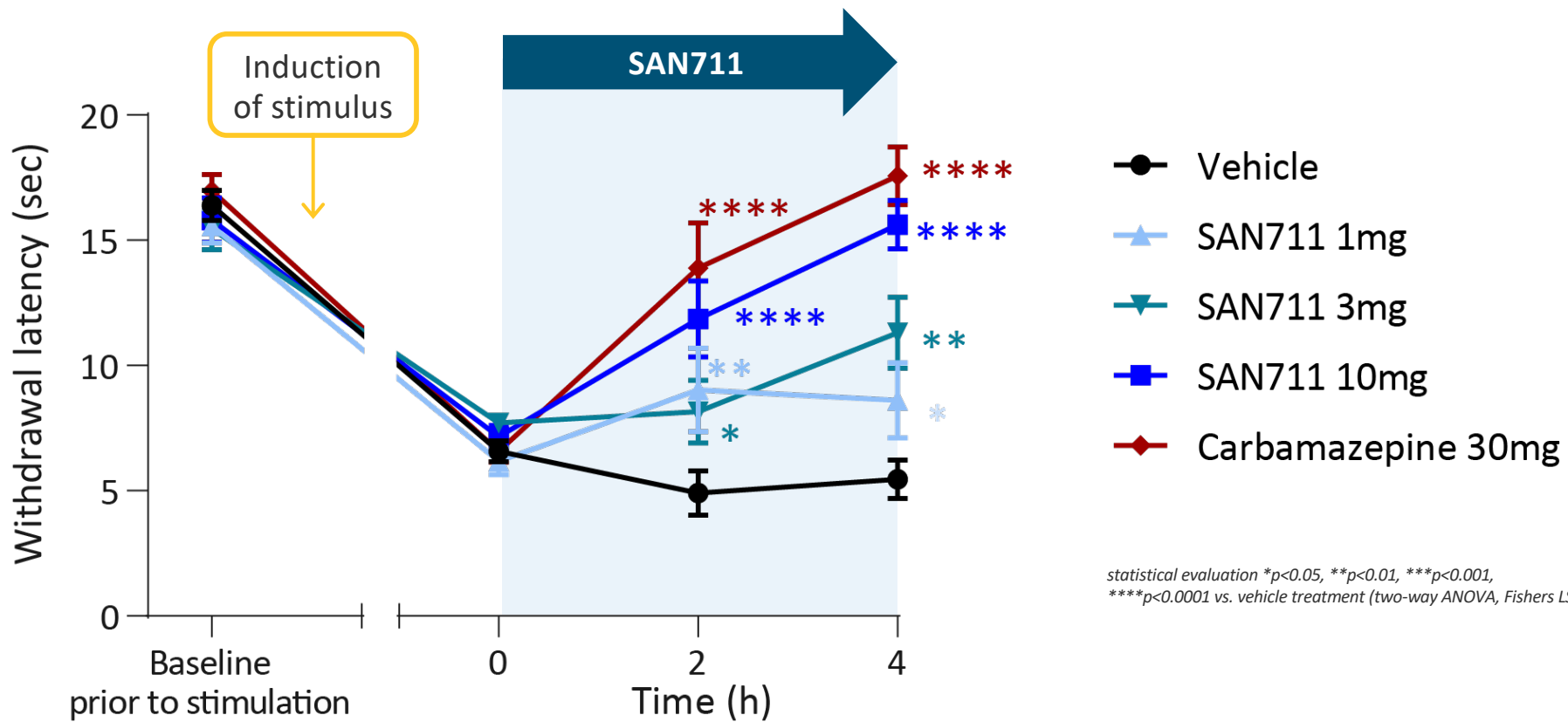
- **Target indication:** rare neuropathic disorders
- **Status:** Phase 1 trial in healthy volunteers initiated in June 2021; top-line data expected in first half of 2022

SAN711 is a highly selective modulator of GABA_A α3 in the spinal cord:

- Designed to **restore dysfunctional spinal inhibition** and **prevent abnormal pain signaling** to the brain
- **Does not impact GABA_A α1 and α5**, the receptors shown to **drive negative side effects off benzodiazepines** (e.g., sedation, motoric instability, abuse liability, and memory impairing effects)
- **No tolerance development** following chronic treatment in preclinical models

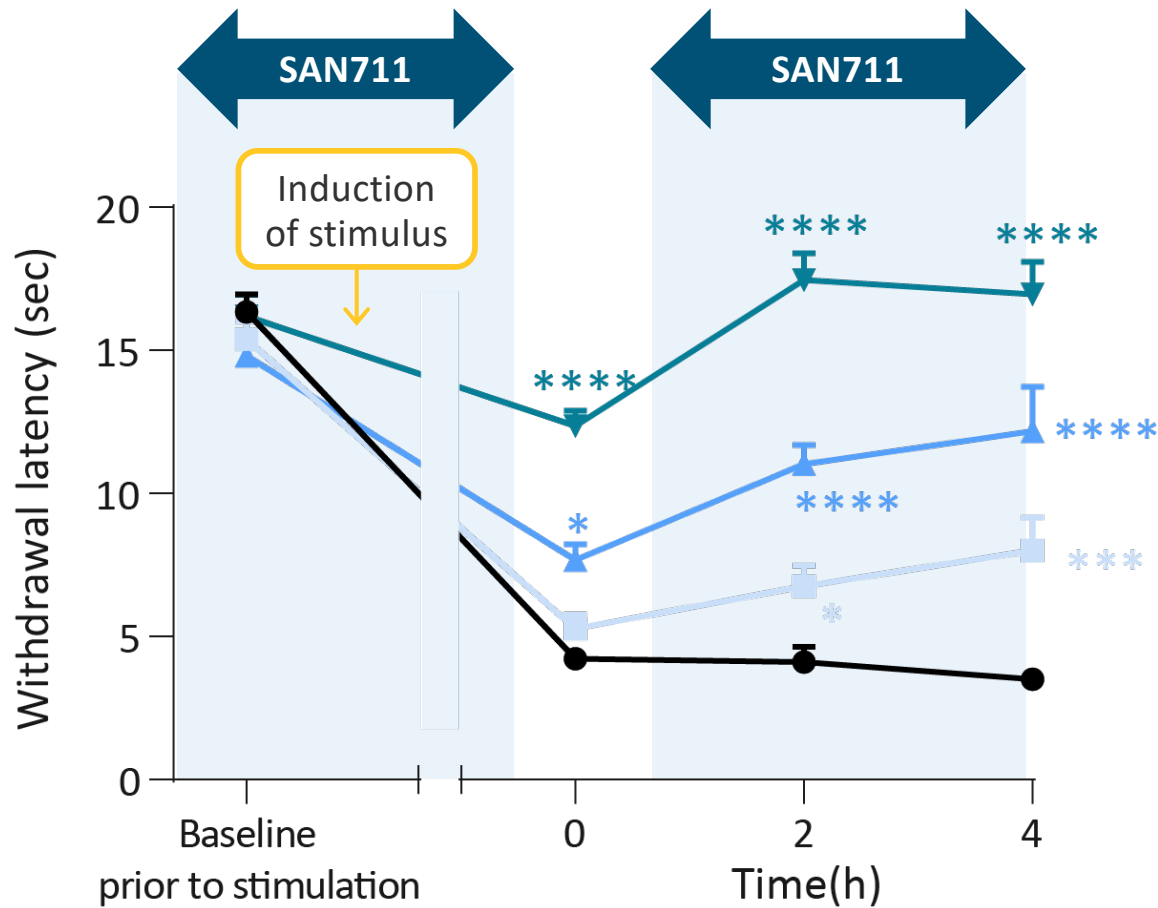


SAN711 reduces Pain (trigeminal neuralgia model): Acute Treatment



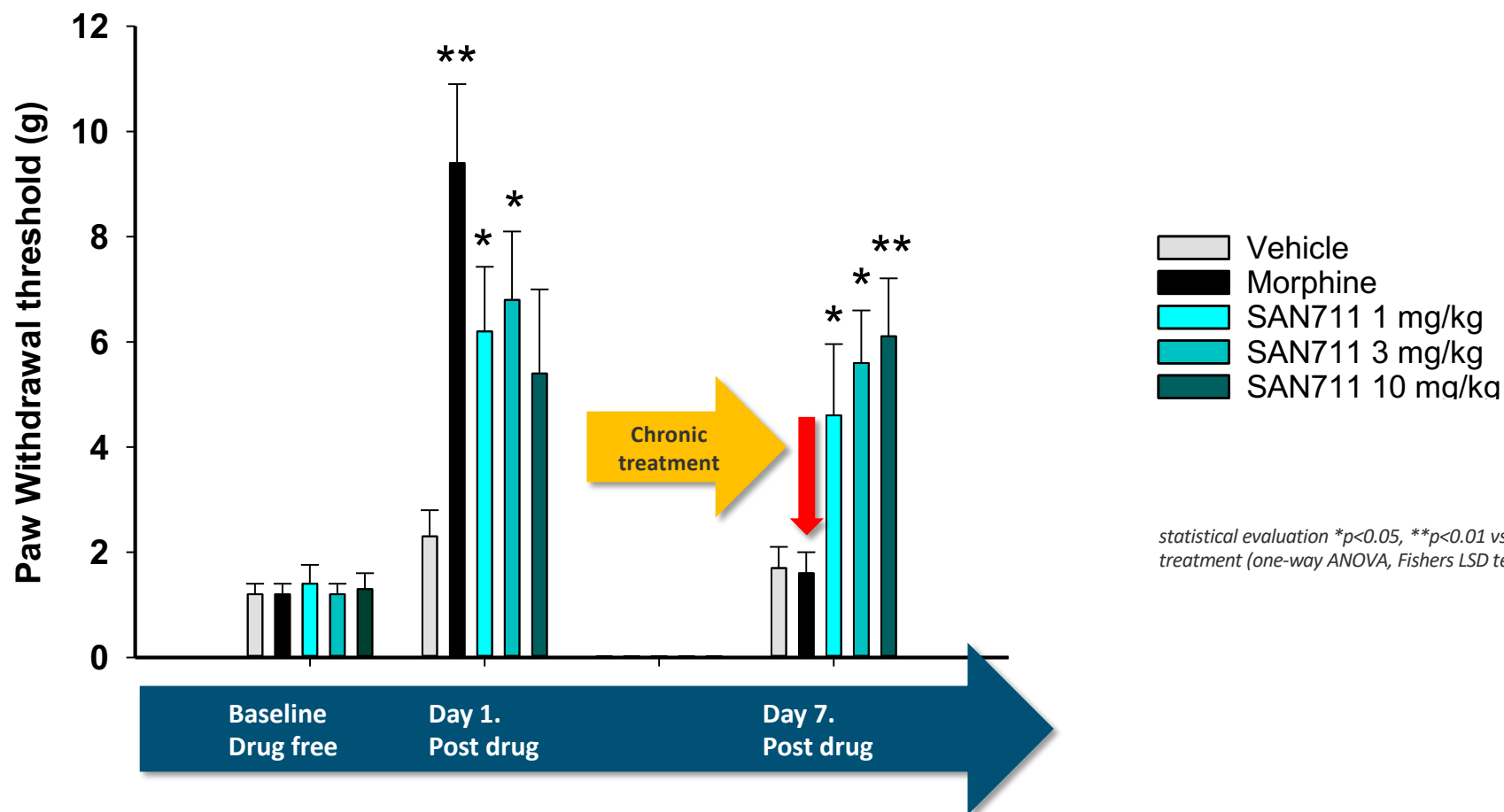
statistical evaluation * $p < 0.05$, ** $p < 0.01$, *** $p < 0.001$,
 **** $p < 0.0001$ vs. vehicle treatment (two-way ANOVA, Fishers LSD test)

SAN711 Prevents Pain (trigeminal neuralgia model): Chronic Treatment



statistical evaluation * $p < 0.05$, ** $p < 0.01$, *** $p < 0.001$,
**** $p < 0.0001$ vs. vehicle treatment (two-way ANOVA, Fishers LSD test)

SAN711 Maintains Efficacy after Repeated Dosing – in Contrast to Morphine



statistical evaluation * $p < 0.05$, ** $p < 0.01$ vs. vehicle treatment (one-way ANOVA, Fishers LSD test)

SAN711 Phase 1 clinical trial initiated in June 2021

- SAN711 represents a novel, first-in-class approach
- Selectively enhances the effects of GABA on $\alpha 3$ containing receptors
- Ideal for rare diseases associated with neuropathic pain or itch
- Devoid of typical adverse effects (sedation, motoric instability, cognitive impairment, abuse liability and physical dependence)
- Strong preclinical proof of concept in in vivo models for neuropathic pain.

*Acute and chronic efficacy is maintained;
without tolerance development*

Phase 1 clinical trial
initiated in June 2021
Data expected early
2022

SAN711 Phase 1 Clinical Trial Design

Objectives:

1. Determine the tolerability (of SAN711) and the maximum tolerated dose (MTD)
2. Measure binding to target receptors

Subject Population:

~80 healthy volunteers

Intervention:

SAN711 vs Placebo

Single Ascending Dose (SAD) ~56 Patients

Multiple cohorts receiving increasing doses

Positron Emission Tomography (PET) Up to 8 Subjects

Analysis of cohorts
determined from SAD

Multiple Ascending Dose (MAD) ~24 Patients

Multiple cohorts receiving increasing doses



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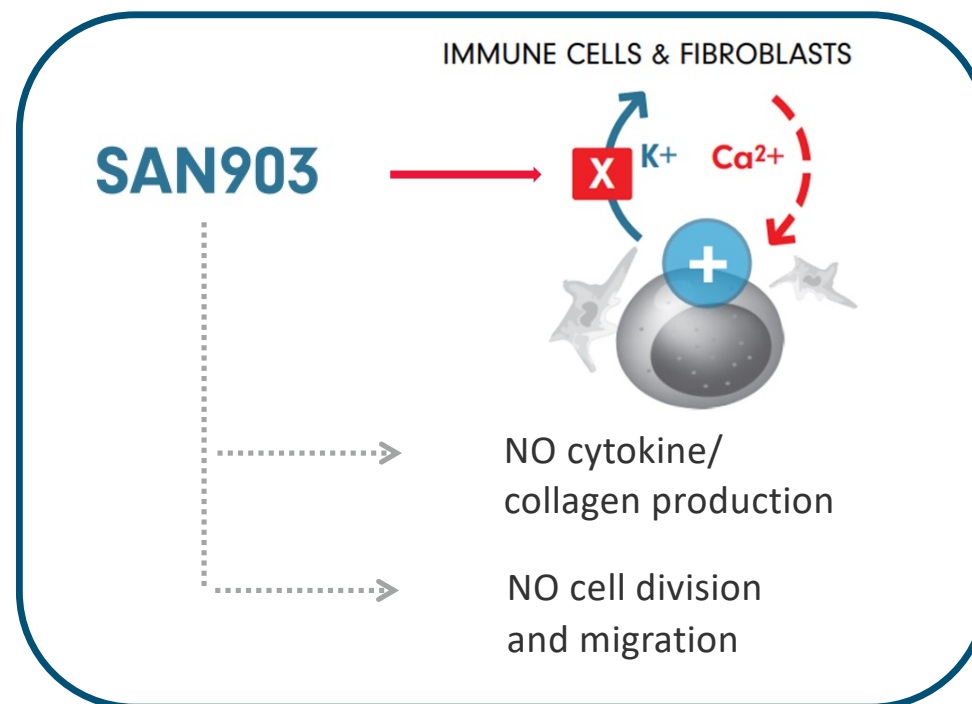
SAN903
for rare inflammatory
and fibrotic disorders



SAN903: Potentially First-in-Class Inhibitor of Calcium-Activated Potassium Ion Channel

- **Target indications:** rare inflammatory, fibrotic, and hematological disorders
- **Status:** Phase 1 clinical trial in participants expected to begin in the second half of 2022

- **K_{Ca}3.1** is important for activation of immune cells, fibroblasts, and red blood cells
- **SAN903** specifically inhibited K_{Ca}3.1 potassium channels, leading to reduced calcium influx
- **SAN903** inhibited inflammation and fibrosis
 - Effectively dampened cell division and migration
 - Reduced cytokine and collagen production



K_{Ca}3.1 is Involved in Many Fibrotic, Inflammatory and Blood Diseases

K_{Ca}3.1



Immune Cells

the cell type that protects us from infections, including T cells

K_{Ca}3.1 plays a pathological role in **overactive immune responses** in chronic inflammatory diseases

Fibroblasts

the cell type that maintains the connective tissue in our body

K_{Ca}3.1 plays a pathological role in **excessive connective tissue production** in chronic fibrotic diseases

Erythrocytes

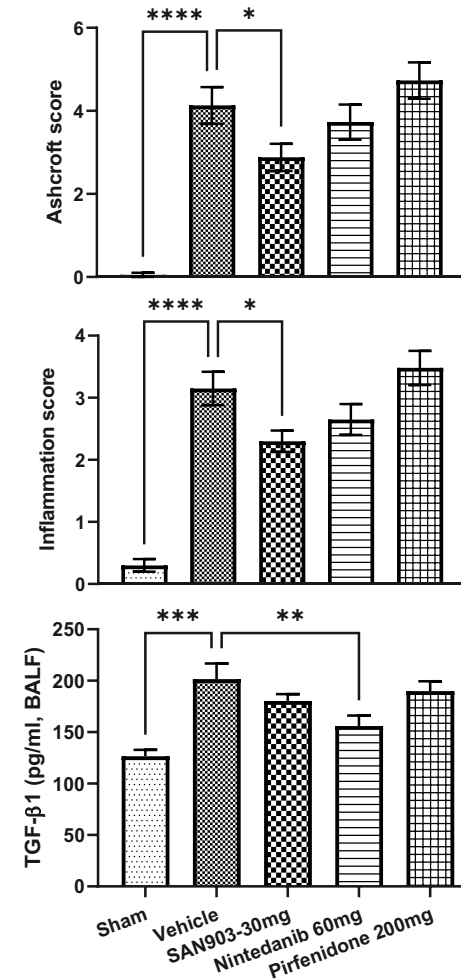
the cell type that carries oxygen in our blood

K_{Ca}3.1 plays a pathological role in **erythrocyte destruction** in certain inherited blood diseases

SAN903 Reduces Lung Inflammation, Fibrosis (Idiopathic Pulmonary Fibrosis model)



- SAN903 attenuated lung fibrosis and inflammation
- SAN903 outperformed nintedanib and pirfenidone on reducing inflammation and fibrosis
- In contrast to nintedanib, the effect of SAN903 appeared independent of TGF- β 1 inhibition
- Overall, the therapeutic effect of SAN903 seemed superior to IPF standard-of-care medicines



SAN903 Expected to Advance into Clinic H2 2022

- SAN903 specifically inhibits $K_{Ca}3.1$ potassium channels
- Demonstrated efficacy in multiple in vitro and in vivo models of inflammation, fibrosis and blood disorders
- Outperformed two marketed products in an in vivo model of idiopathic pulmonary fibrosis; potential first-in-class AND best-in-class profile

First-in-human program
(Phase 1, healthy adults)
expected to start H2
2022







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Key Milestones



Key 2021 – 2023 Milestones

 Orphan Drug Designation (ODD) for Tesomet in Prader-Willi Syndrome (PWS)	Q1 2021
 Resolution with FDA on regulatory path for Tesomet in hypothalamic obesity (HO)	Q1 2021
 Initiated Phase 1 clinical trial of SAN711 in healthy volunteers	H1 2021
 Orphan Drug Designation (ODD) for Tesomet in hypothalamic obesity (HO)	Q3 2021
Initiate Phase 2b clinical trial of Tesomet in hypothalamic obesity (HO)	H2 2021
Initiate Phase 2b clinical trial of Tesomet in Prader-Willi Syndrome (PWS)	H2 2021
Top line data from Phase 1 clinical trial of SAN711 in healthy volunteers	H1 2022
Initiate Phase 1 clinical trial of SAN903	H2 2022
Top line data from Phase 2b clinical trial of Tesomet in Prader-Willi Syndrome (PWS)	H1 2023
Top line data from Phase 2b clinical trial of Tesomet in hypothalamic obesity (HO)	H2 2023

Building Tomorrow's Rare Disease Company

Tesomet is only the beginning...



Tesomet for HO and PWS

SAN711 for rare neuropathic disorders

SAN903 for rare inflammatory disorders

Library of 20,000 ion channel modifiers

Tesomet, SAN711, SAN903 LCM

Business Development



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Thank You