PIONEERING GENE THERAPIES FOR ORPHAN CNS DISEASES
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Corporate Highlights

**01 Pioneering Gene Therapy in Orphan CNS Diseases**

Positioned to successfully become the first player to develop and commercialize safe and efficacious gene therapy products for CNS diseases in the US and EU

**02 Three Gene Therapy Assets**

MPS IIIA / Sanfilippo Syndrome
LYS-SAF302 advancing to Pivotal Trials

GM1 Gangliosidosis
LYS-GM101 currently in pre-clinical studies

Fragile X
An asset in proof-of-concept studies

**03 Global Offices**

Located in Cambridge, MA, USA and Paris, France

**04 Experienced Management Team**

Karen Aiach,
Co-Founder, CEO and parent of an MPS IIIA (Sanfilippo A) child

Oliver Danos,
Co-Founder and world-leading gene therapy expert, currently CSO at RegenXbio Inc.
Pipeline: Focus in Orphan CNS Diseases Initiating Pivotal Study in MPS IIIA in 2018

<table>
<thead>
<tr>
<th>INDICATION</th>
<th>PROGRAM</th>
<th>VECTOR</th>
<th>ENZYME</th>
<th>POC</th>
<th>PRE-CLINICAL</th>
<th>PHASE I/II*</th>
<th>PIVOTAL</th>
<th>COMMENTS</th>
</tr>
</thead>
<tbody>
<tr>
<td>Sanfilippo A (MPS IIIA)</td>
<td>LYS-SA302</td>
<td>AAVrh10</td>
<td>N-sulfoglycosamine sulphohydrolase</td>
<td></td>
<td></td>
<td></td>
<td>Non clinical regulatory studies</td>
<td>PIVOTAL TRIAL TO BEGIN BY H2 2018 (FPI**)</td>
</tr>
<tr>
<td>GM1 Gangliosidosis</td>
<td>LYS-GM101</td>
<td>AAVrh10</td>
<td>Beta-galactosidase-1</td>
<td></td>
<td></td>
<td></td>
<td>Phase I/II trial to begin by 2019 (FPI**)</td>
<td>Phase I/II trial to begin by 2019 (FPI**)</td>
</tr>
<tr>
<td>Fragile X syndrome (FXS)</td>
<td>LYS-XXX</td>
<td>AAVrh10 or AAV PHP.B</td>
<td>5'-truncated Diacylglycerol Kinase Kappa (Dgkk)</td>
<td></td>
<td></td>
<td></td>
<td>Proof of concept</td>
<td>Proof of concept</td>
</tr>
</tbody>
</table>

* MPS IIIA Phase I/II: LYS-SA301, first generation program ** FPI: « First Patient In »: First Patient Enrolled
**STRATEGY**

Adding to the pipeline in capitalizing on the rapid deployment of its technology and know-how in new therapeutic indications

<table>
<thead>
<tr>
<th></th>
<th>IP (Freedom To Operate)</th>
<th>Orphan Drug Designation (FDA)</th>
<th>Rare Pediatric Disease Designation (FDA)</th>
<th>Orphan Drug Designation (EMA)</th>
</tr>
</thead>
<tbody>
<tr>
<td>LYS-SAF302</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>LYS-GM101</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
</tbody>
</table>
**MPS IIIA**

**INHERITANCE**
Autosomal recessive lysosomal storage disease

**POPULATION**
Incidence: 0.5-1.2 / 100 000 live births (1)
Point prevalence worldwide: 3 000 (2)

**BURDEN OF DISEASE**
Extremely deteriorating quality of life, devastating for patients and families (3)
Massive social/economic costs

**TREATMENT**
No approved or curative treatments

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**Lysosomal Storage Disease**
- Gene Defect
- Enzyme Defect
- Accumulation of substrates
- Cellular Alteration
- Tissue Deterioration
- Function Losses

**MPS IIIA**
- Chromosome 17
- Heparan Sulfamidase
- Heparan Sulfate
- Neurons
- Brain
- Cognitive decline, severally handicapped, short life expectancy

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Notes: (1) Heron (2011); Poorthuis (1999)  
(2) Average life expectancy: 15 years; (3) Grant (2012)
MPS IIIA: Gene Defect Results in Devastating Neurological Symptoms

Affected children progressively develop profound mental retardation with minimal somatic manifestations (Valstar et al, 2008)

Clinical evolution of MPS IIIA

Note: Graph adapted from UMN natural history study (Shapiro et al)
## MPS IIA: Current Competitive Landscape

<table>
<thead>
<tr>
<th>Approach</th>
<th>Route of administration</th>
<th>Companies</th>
<th>Status of Trials</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Gene Therapy</strong></td>
<td>Intracranial</td>
<td>Lysogene</td>
<td>Moving to Pivotal Trials in 2018</td>
</tr>
<tr>
<td>Intracerebroventricular</td>
<td></td>
<td>Esteve</td>
<td>Phase I/II planned to start in 2018</td>
</tr>
<tr>
<td>Intravenous</td>
<td></td>
<td>Abeona</td>
<td>Currently in Phase I/II – 11 pts enrolled</td>
</tr>
<tr>
<td>Ex vivo lentiviral</td>
<td></td>
<td>Orchard</td>
<td>Expected to start Phase I/II end of 2018</td>
</tr>
<tr>
<td><strong>Enzyme Replacement Therapy</strong></td>
<td>Intrathecal</td>
<td>Shire</td>
<td>Trial suspended</td>
</tr>
<tr>
<td>Intrathecal</td>
<td></td>
<td>Sobi, Armagen</td>
<td>Sobi: estimated start date July 2018</td>
</tr>
<tr>
<td>Intravenous</td>
<td></td>
<td></td>
<td>Armagen: still exploratory</td>
</tr>
<tr>
<td><strong>Substrate reduction Therapy</strong></td>
<td>Oral genistein</td>
<td>Investigator initiated study</td>
<td>Publication expected end of 2017 – delayed sine die</td>
</tr>
</tbody>
</table>
MPS IIIA Our Approach - Targeting CNS Directly

Uses a well-known, safe neurosurgical procedure to deliver the enzyme where it is most needed – in the CNS

- Circumvents the blood brain barrier
- Broad distribution throughout the brain
- Enzyme sustainably produced
- Experience in ~50 children with rare genetic diseases: MLD, MPS IIIA, MPS IIIB, Canavan, CLN2

N-sulfoglucosamine sulfohydrolase (SGSH) is a secreted enzyme

- Affects cells distant from transduced cells via cross correction as successfully shown in IND enabling studies
## MPS IIIA LYS-SAF301 (First-Generation): Phase 1-2 Study (1)

<table>
<thead>
<tr>
<th>PATIENT COHORT</th>
<th>DRUG DOSING</th>
</tr>
</thead>
<tbody>
<tr>
<td>Four children with severe classical form of MPS IIIA, diagnosed between 6 to 37 months; treated between 2y8m to 6y9m.</td>
<td>LYS-SAF301 delivered simultaneously via 6 tiny burr holes total of $7.2 \times 10^{11} \text{vg}$ ($0.6 \times 10^{11} \text{vg}$) at $0.5 \mu l/min$ directly into the white matter at two different depths. Complete scarring within 15 days.</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>SAFETY</th>
<th>EFFICACY</th>
</tr>
</thead>
<tbody>
<tr>
<td>5 years post-surgery data showed no treatment-related adverse events</td>
<td>One-year reported an improvement on behavior, hyperactivity, sleep disturbances in 3 out of 4 patients during the first year, and cognitive improvement for the youngest patient. All patients are alive at 6 years post surgery.</td>
</tr>
</tbody>
</table>
MPS IIIA LYS-SAF302 (Second Generation): An optimized vector

<table>
<thead>
<tr>
<th>Components</th>
<th>LYS-SAF301 (1st generation)</th>
<th>LYS-SAF302 (2nd generation)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Vector</td>
<td>AAVrh.10</td>
<td>AAVrh.10</td>
</tr>
<tr>
<td>Promoter</td>
<td>mPGK</td>
<td>CAG</td>
</tr>
<tr>
<td>Transgenes</td>
<td>SGSH + SUMF-1</td>
<td>SGSH</td>
</tr>
</tbody>
</table>

Improved construct provides increased enzyme activity in MPS IIIA mouse brain

**Not drawn to scale**
**Primary objective**

Assess the efficacy of direct to CNS delivery of LYS-SAF302 in improving or stabilizing the neurodevelopmental status of severe MPS IIIA patients after 24 months, compared with the natural history. Powered to demonstrate disease stabilization using Development Quotient as PEP\(^{(1)}\).

**Study design**

Single arm with Natural History Study as comparator. Open label allowing for intermediary analysis.

**Endpoints**

Primary endpoints: neurocognitive and motor development (Development Quotient)
- Ratio DQ\(_{24}\)/DQ\(_{0}\) under treatment divided by expected DQ\(_{24}/DQ\(_{0}\) (calculated from natural history)
- Interim analysis at 12 months post-surgery
  - If robust data at 12 months, initiate filing preparation

Secondary endpoints: Behavior, sleep, QOL\(^{(2)}\), MRI\(^{(3)}\), Biomarkers (PBMC, CSF, urine)

**Sample size and sites**

N=20 patients
- US: up to 4 sites
- EU: up to 4 sites

**Target population**

Homogenous population with DQ ≥50% at baseline
Predictable disease progression 12 to 24 months

**First patient enrolled**

By H2-2018

**Study duration**

24 months (efficacy analysis)
Study extension of additional 4 years

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Notes:  
\(^{(1)}\): DQ = Development Age/Calendar age;  
\(^{(2)}\): QOL = Quality Of Life;  
\(^{(3)}\): MRI = Magnetic Resonance Imaging
Unique Natural History Study
Pooling U.S.\(^{(1)}\) and European\(^{(2)}\)
Data Sets will serve as control arm for pivotal trial

**Evolution of DA and DQ in Natural History Studies using same assessment tools**

European + Brazil NHS (n=23) and US NHS (n=19) including 18 children with DQ>50% at baseline

Variability between the children in early stages of disease

Linear DQ decline by calendar age (cross-sectional analysis)

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**Notes:** (1) Shapiro, et al., A Prospective Natural History Study of Mucopolysaccharidosis Type IIIA. The Journal of Pediatrics (2016). (2) Unpublished data
Primary Endpoint agreed with FDA and EMA: Cognition
Expected Change From Baseline in DQ in Treated Group Compared To NHS

Primary Endpoint
Ratio DQ12-24/DQ0 under treatment divided by expected DQ12-24/DQ0 (NH)

If DA stable in treated group:
> At M12: expected ratio = 1.62
> At M24: expected ratio = 1.79
# Pricing and Early Access Strategy

## Value drivers for a new MPS IIIA treatment

<table>
<thead>
<tr>
<th>Importance</th>
<th>Cognitive function</th>
<th>Behavior</th>
<th>Safety</th>
</tr>
</thead>
<tbody>
<tr>
<td>High</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Moderate</td>
<td>Family or patient reported QoL</td>
<td>Sleep disturbance</td>
<td></td>
</tr>
<tr>
<td>Moderate-to-low</td>
<td>Level of CSF HS</td>
<td>Liver volume</td>
<td>Spleen volume</td>
</tr>
<tr>
<td>Low</td>
<td>Ease of use</td>
<td>Mode of administration</td>
<td></td>
</tr>
</tbody>
</table>

## Strategy Validated by Payer Survey to 26 External Experts in Europe and US

**Improvement in neurocognitive / behavioral endpoints is a crucial issue for payers**
- LYS-SAF302 positioned as first therapy for MPS IIIA that improves patients’ neurocognitive development and behavior.

**Pricing**
- If all new cell/gene therapies launch by offering a conditional price or pay-for-performance type of model, Lysogene may consider the same.
- Price potential of LYS-SAF302 validated in payer survey. Likely scenario will be annual price of chronic benchmark with multiplying factor.

**Early Access**
- Lysogene will pursue funded Early Access Program (EAP) opportunities in the EU-4. If unable to obtain funding, may focus energies on pursuing non-funded EAPs in the EU-5 and US.
MPS IIIA Manufacturing Strategy

- A 15-Year Track Record
- 950m2 Production suits in Belgium
- Commercial facility ready in 2019
- Successfully audited by Belgium authorities, customers and FDA

LYS-SAF302: Master service agreement in place with Henogen (subsidiary of the Group Novasep) to cover preclinical and clinical batches with LYS-SAF302

Tech transfer, process development and process characterization

Engineering runs for GLP TOX studies completed

GMP manufacturing (Phase II/III):
- Drug Substance completed
- Fill & Finish ongoing
MPS IIIA LYS-SAF302: Key Development Milestones

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<tbody>
<tr>
<td>Today</td>
<td></td>
<td></td>
<td></td>
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</tr>
</tbody>
</table>

**Clinical Registration Program**

- June 2016: First patient in
- Q2 2017: Last patient in
- Q1 2018: 1 year Natural History data

**Natural History Study**

- Q4 2017: IND enabling studies report
- H2 2018: Clinical trial start

**Pivotal Trial**

- H1 2020: Last patient in. Primary efficacy analysis 6 months for first patients

**Extension (+4yrs)**

- H1 2021: Efficacy analysis 12 months for all patients
- H2 2021: MAA**, EMA and FDA filling

**Early access program**


18
LYS-GM101 Gangliosidosis
**GM1 Gangliosidosis**

### INHERITANCE
Lysosomal disease caused by mutations in the GLB1 gene, which encodes the β-galactosidase enzyme.

### SYMPTOMS
Four clinical subtypes based on age of symptom onset and disease severity: early infantile, late infantile, juvenile and adult. Severe neurodegeneration, seizures, coarse facial features. 100% lethal.

### POPULATION
Incidence: 1/200 000 to 1/100 000 live births. Prevalence: 2 000

### BURDEN OF DISEASE
Extremely deteriorating quality of life, devastating for patients, and families. Massive social/economic costs.

### TREATMENT
No approved or curative treatments.

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**LYSOSOMAL STORAGE DISEASE**

- Gene Defect
- Enzyme Defect
- Accumulation of substrates
- Cellular Alteration
- Tissue Deterioration
- Function Losses

**GM1 GANGLIOSIDOSIS**

- Chromosome 3
- β-galactosidase
- GM1, galactosyloligosaccharides, Keratan sulfate
- Neurons
- CNS
- Cognitive and movement decline, short life expectancy

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# GM1 GANGLIOSIDOSIS: Current Competitive Landscape

<table>
<thead>
<tr>
<th>Approach</th>
<th>Route of administration</th>
<th>Companies</th>
<th>Status of Trials</th>
</tr>
</thead>
<tbody>
<tr>
<td>Gene Therapy</td>
<td>Direct to CNS</td>
<td>Lysogene</td>
<td>Currently in pre-clinical trials</td>
</tr>
<tr>
<td></td>
<td>Intravenous</td>
<td>Umass - Investigator initiated study</td>
<td>Currently in pre-clinical trials</td>
</tr>
<tr>
<td>Enzyme Replacement Therapy</td>
<td>Intravenous</td>
<td>Biostrategies LC</td>
<td>Proof of Concept</td>
</tr>
<tr>
<td>Chaperone</td>
<td>Targeting β-galactosidase</td>
<td>Dorphan</td>
<td>Proof of Concept</td>
</tr>
<tr>
<td>Substrate Reduction Therapy</td>
<td>Oral miglustat &amp; ketogenic diet</td>
<td>University of Minnesota – Investigator initiated study</td>
<td>Phase IV</td>
</tr>
</tbody>
</table>
GM1 GANGLIOSIDOSIS LYS-GM101: Direct to CNS Approach with AAV Gene Therapy

Direct to brain injections of an AAV prototype in GM1 cat model

6-7 month-old GM1 cat

5 year-old AAV-treated GM1 cat

Source: Douglas R. Martin lab, Scott-Ritchey Center at Auburn University School of Veterinary Medicine
### Study design

- **Primary endpoint:** motor function
- **Secondary endpoint:** speech ability
- Natural history data, potentially serving as external control, already documented and published by Pr. Cynthia Tifft (MD, PhD, NIH\(^{(1)}\)/NHGRI\(^{(2)}\))

### Sample size

- Initial 3 patients / dose x 2 doses
- Additional 12 patients efficacy study

### Target population

- GM1 patients

### First patient enrolled

- 2019

### Study duration

- 14 months dose escalation
- 12 months + 12- 24 months follow-up

### Sites

- USA and Europe
GM1 GANGLIOSIDOSIS Manufacturing Strategy

**Extensive track record of viral vector process/analytical development, and GMP manufacturing**
- Over 28 products and 47 cGMP Lots
- AAV, lentivirus, HSV, adenovirus vectors

**AAV Serotypes**
- AAV1, AAV2, AAV2 engineered, AAV5, AAV6, AAV8, AAV9 & AAVrh10

**Beyond Phase 3: commercial manufacturing facility in place, Cambridge, MA**

**Vision**
To be the best in class cell and gene therapy CDMO
We are Manufacturing Personalized™

**Mission**
To enable our clients to improve patient’s health by providing process development, clinical, and commercial manufacturing services

**Production Systems**
- Transient transfection (Adherent and suspension)
- Baculovirus-based
- HSV-based

**2015-2016 FL Manufacturing Performance Metrics**
- GMP Drug Substance (11 50-200L scale and 16 400L scale GMP bioreactor runs) : Success rate 93% (25/27)
- GMP aseptic Filling : Success rate 100% (19/19)

**LYS-GM101: Strategic manufacturing agreement with Brammer Bio to produce GM1 Gangliosidosis gene therapy product**
Tech transfer completed  |  Process development ongoing  |  MCB manufactured  |  Scale-up and Engineering runs on going
# GM1 GANGLIOSIDOSIS LYS-GM101: Key Development Milestones

<table>
<thead>
<tr>
<th>Year</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>2016</td>
<td>Q2 2016 - Q1 2017 ODD**(1) - RPDD**(2) obtained</td>
</tr>
<tr>
<td>2017</td>
<td>H1 2017 Pre-IND</td>
</tr>
<tr>
<td>2018</td>
<td>H1 2018 IND submission</td>
</tr>
<tr>
<td>2019</td>
<td>H1 2019 HTA/EMA SA</td>
</tr>
<tr>
<td>2020</td>
<td>H2 2021 HTA/EMA SA</td>
</tr>
<tr>
<td>2021</td>
<td>H1 2021 Adaptive clinical study – Confirmatory phase (n=12)</td>
</tr>
<tr>
<td>2022</td>
<td>H1 2024 BLA - MAA**, EMA and FDA filling</td>
</tr>
<tr>
<td>2023</td>
<td>HTA/EMA SA</td>
</tr>
<tr>
<td>2024</td>
<td>BLA - MAA**, EMA and FDA filing</td>
</tr>
</tbody>
</table>

Fragile X Syndrome (FXS)

**INHERITANCE**
Deficiency of the Fragile X mental retardation protein (FMRP) due to hypermethylation of a CGG trinucleotide repeat in the FMR1 gene localized on the X chromosome.

**SYMPTOMS**
Most common inherited cause of intellectual disability and autism spectrum disorder.

**POPULATION**
1/4000 males and 1/4 000-6 000 females worldwide (1). Around 10 000 patients in France, 71 000 patients in the USA.

**BURDEN OF DISEASE**
Extremely deteriorating quality of life, devastating for patients and families. Massive social and healthcare costs.

**TREATMENT**
No approved curative/disease modifying treatment.

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**Fragile X syndrome**
- X chromosome
- FMR1 gene silencing
- Dysregulation of neuron mRNAs
- Excessive production of neuronal proteins
- Neuronal hyperexcitability, spinal dysmorphogenesis
- Intellectual impairment and abnormal behavior (stereotypies, social anxiety, seizure susceptibility, hyperactivity)

**Partnership**

Co-conception scheme with an option to Lysogene, allowing to develop, manufacture, commercialize or sublicense the product as long as IP exists.

- No upfront cost
- POC development costs financed by SATT
- For the duration of the project, Lysogene will bring its expertise
- Exclusive option will expire at the end of the 18-month duration of the project

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**Project proposal and development of innovative projects**

Dr. Hervé Moine
Group leader in the translational medicine and neurogenetic department
Shareholders As Of December 2017

The main 4 shareholders hold more than 75% of the capital structure as of December 2017

- Sofinnova Partners: 29.41%
- BPI France: 22.15%
- NOVO AS: 17.45%
- KGA: 10.37%
- Others: 20.62%
Experienced Management Team: Based In Cambridge, MA And Paris, France

A team with an average experience of 20 years

Karen Aiach
Chief Executive Officer

Philippe Mendels-Flandre
Chief Operating Officer

Ralph Lauffer, PhD
Chief Scientific Officer
Paris, France
(as of May 2nd)

25 years of industry experience in drug discovery, translational research and drug development

Formerly at

Mark Plavsic, PhD
Chief Technical Officer
Cambridge, MA – USA

30 years in process development and manufacturing in USA, Europe and Australia

Formerly at

genzyme

Sean O’Bryan
VP Regulatory Affairs & Quality Assurance
Cambridge, MA – USA

20 years regulatory expertise across gene therapy, biologics and small molecules

Formerly at

bluebirdbio

Sophie Olivier, MD
Chief Medical Officer
Paris, France

25 years experience in pharmaceutical and healthcare industry

Formerly at

Samantha Parker, MBA
Chief Patient Access Officer
Paris, France

17 years orphan space EGRD/IRDIRC board member

Formerly at

Philippe Mendels-Flandre
Chief Operating Officer

16 years experience across healthcare industry

Formerly at

Karen Aiach
Chief Executive Officer

A team with an average experience of 20 years
Why Invest In Lysogene?

01 PIONEERING GENE THERAPY
A pioneering gene therapy technology suited to fatal neurological diseases, applied directly to CNS, to maximize therapeutic benefits

02 SKILLED MANAGEMENT
A highly skilled management team: renowned expertise and know-how on the whole value chain of drug development in rare diseases

03 GLOBAL OUTREACH
A global outreach with EU and US presence and coverage

04 CLINICAL DEVELOPMENT
First candidate product LYS-SAF302 for treating MPS IIIA is in advanced-stage clinical development

05 CLINICAL TRIAL
Second product candidate LYS-GM101 to treat GM1 Gangliosidosis: planned clinical trial in 2019

06 INNOVATIVE THERAPEUTIC
Potential to replicate innovative therapeutic approach and target other rare, fatal CNS diseases and thus expand product portfolio: GM1 as first example and Fragile X as second one
Lysogene Clinical Advisory Board

**ANUPAM CHAKRAPANI, MD**
Great Ormond Street Hospital for Children NHS (GOSH),
London, UK

**RONALD G. CRYSTAL, MD**
Weill Cornell Medical College
New-York City, USA

**ROBERTO GIUGLIANI, PHD, MSC, MD**
University of Rio Grande do Sul
Porto Alegre, Brazil

**BÉNÉDICTE HÉRON, MD**
Armand-Trousseau Hospital, APHP
Paris, France

**MICHEL ZERAH, MD**
Necker Enfants Malades
Paris, France

**NICOLE M. MUSCHOL, MD**
University Medical Center
Hamburg-Eppendorf, Germany

**ANUPAM CHAKRAPANI, MD**
Great Ormond Street Hospital for Children NHS (GOSH),
London, UK

**RONALD G. CRYSTAL, MD**
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University of Rio Grande do Sul
Porto Alegre, Brazil

**BÉNÉDICTE HÉRON, MD**
Armand-Trousseau Hospital, APHP
Paris, France

**MICHEL ZERAH, MD**
Necker Enfants Malades
Paris, France

**RAYMOND Y. WANG, MD**
Children's Hospital of Orange Country
California, USA

**CHESTER B. WHITLEY, PHD, MD**
University of Minnesota School of Medicine
Minnesota, USA

**NICOLE M. MUSCHOL, MD**
University Medical Center
Hamburg-Eppendorf, Germany

**ANUPAM CHAKRAPANI, MD**
Great Ormond Street Hospital for Children NHS (GOSH),
London, UK

**RONALD G. CRYSTAL, MD**
Weill Cornell Medical College
New-York City, USA

**ROBERTO GIUGLIANI, PHD, MSC, MD**
University of Rio Grande do Sul
Porto Alegre, Brazil

**BÉNÉDICTE HÉRON, MD**
Armand-Trousseau Hospital, APHP
Paris, France

**MICHEL ZERAH, MD**
Necker Enfants Malades
Paris, France
Lysogene Scientific Advisory Board

NATHALIE CARTIER, MD, PHD
INSERM Neurogenerative disorders – CNS Genetics
Paris, France

THOMAS VOIT, MD, PHD
Biomedical Research Centre at Great Ormond Street Hospital (GOSH)
London, United Kingdom

RAY BARTUS, PHD
RTBio Consultants
California, USA

NICOLAS FERRY, PHD
Inserm,
France

Frits Wijburg, MD
Academic Medical Center Genetic Metabolic Disorders
Amsterdam, The Netherlands

Manfred Westphal, MD
University Clinic Hamburg
Eppendorf, Germany

Miguel Esteves, PHD
University of Massachusetts
Massachusetts, USA
Natural History Study (NHS): A Collaborative Approach

<table>
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<tr>
<th><strong>Objectives</strong></th>
<th>Strengthen published data on natural disease course</th>
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<tbody>
<tr>
<td></td>
<td>Will serve as control group for pivotal study</td>
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<td><strong>International expert recommendations</strong></td>
<td>Lysogene organized clinical and neuropsychologists expert groups to define meaningful endpoints and assessment tools.</td>
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<td><strong>Target population</strong></td>
<td>Up to and including 9 years of age</td>
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<td>With a focus on younger patients &lt; 5 years to compare to pivotal trial patients</td>
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<td>Data on older patients for long-term comparison of treated patients</td>
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<tr>
<td><strong>Sample Size &amp; Patient enrollment</strong></td>
<td>23 patients recruited.</td>
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<tr>
<td></td>
<td>13 patients have completed one year data</td>
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<tr>
<td><strong>Study duration</strong></td>
<td>Up to 24 months</td>
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<tr>
<td><strong>Sites</strong></td>
<td>France, Germany, Netherlands, UK and Brazil</td>
</tr>
<tr>
<td><strong>Type of quantitative data</strong></td>
<td>Bayley’s scale (3rd edition), Vineland extended interview, Actiwatch®</td>
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Coronal caudal (A), coronal rostral (B) and horizontal (C) MRI images following the injection of 500μl in two sites per hemisphere showing gadolinium spread along the rostro-caudal axis.
MPS IIA Rationale for Direct to CNS Dosing

Biodistribution Study in Dog

Gadolinium distribution

Coronal caudal (A), coronal rostral (B) and horizontal (C) MRI images following the injection of 500μl in two sites per hemisphere showing gadolinium spread along the rostro-caudal axis.
Distribution In Large Animals Supports Route Of Administration

SGSH activity in Non Human Primate 6-w following injections

**NHP #763E**
R: 2x 50µl SAF302 (3,6E11gc)
L: 2x 50µl SAF302 (3,6E11gc)

**NHP #169I**
R: 2x 50µl SAF302 (3,6E11gc)
L: 2x 50µl SAF302 (3,6E11gc)
PLANNED SURGICAL PROCEDURE TO ACHIEVE CLINICALLY RELEVANT ENZYME ACTIVITY IN THE BRAIN

3 injections/hemisphere planned in clinical trial

Injection sites

>20% enzyme activity increase over PBS injected

Gadolinium distribution

>100% enzyme activity increase over PBS injected

Adult Dog
65 cm³

IIIA child 6 year old
1003 cm³