

Thursday, October 31, 2024 | 1:25 – 2:05 PM

A Sustainable Organization and Brighter Future for the Nano-rare

PRESENTER

Sarah Glass, PhD
Chief Operating Officer, n-Lorem Foundation



Nano-rare
Patient
Colloquium
2024

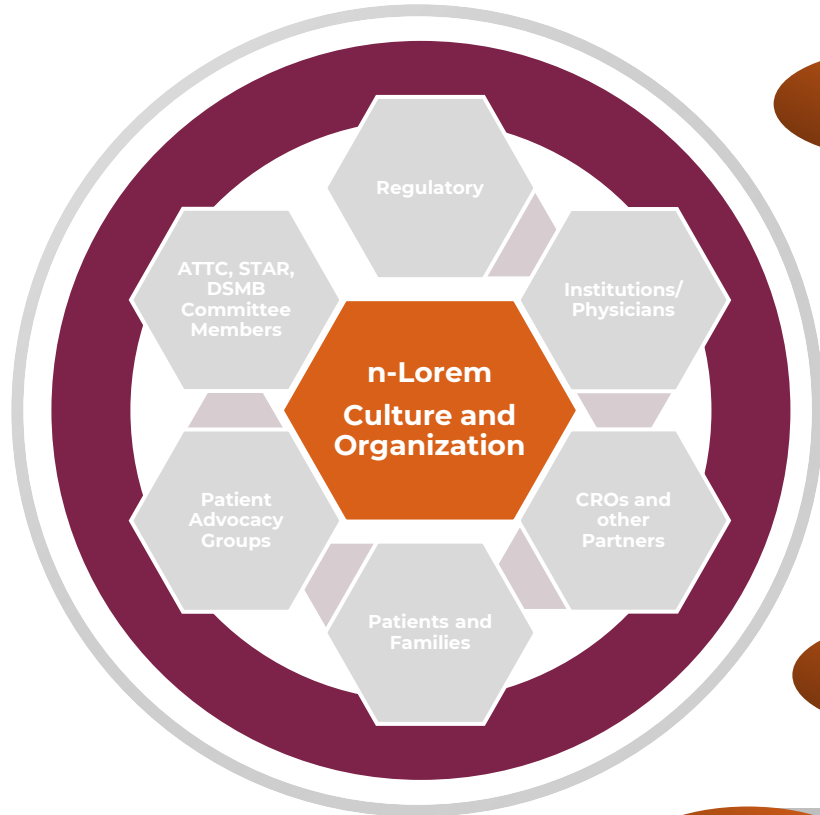
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n-Lorem is Here to Stay

- n-Lorem is a well-established, **fully operational** entity that **effectively integrates** all necessary functions and operates at **maximum efficiency**
- Significant **hurdles have been overcome** to establish the systems and processes required to achieve the efficiencies that led to the 21 INDs in a remarkable period of time
- Key **challenges with access and funding** continue to provide opportunities and requirements to evolve the landscape for the nano-rare

Building a Sustainable Organization and Ecosystem



1

Expand solutions for **treatment administration**

2

Decrease **time between diagnosis and n-Lorem**

3

Access to long-term **affordable** care

4

Bridge to treatment for **n of few**

5

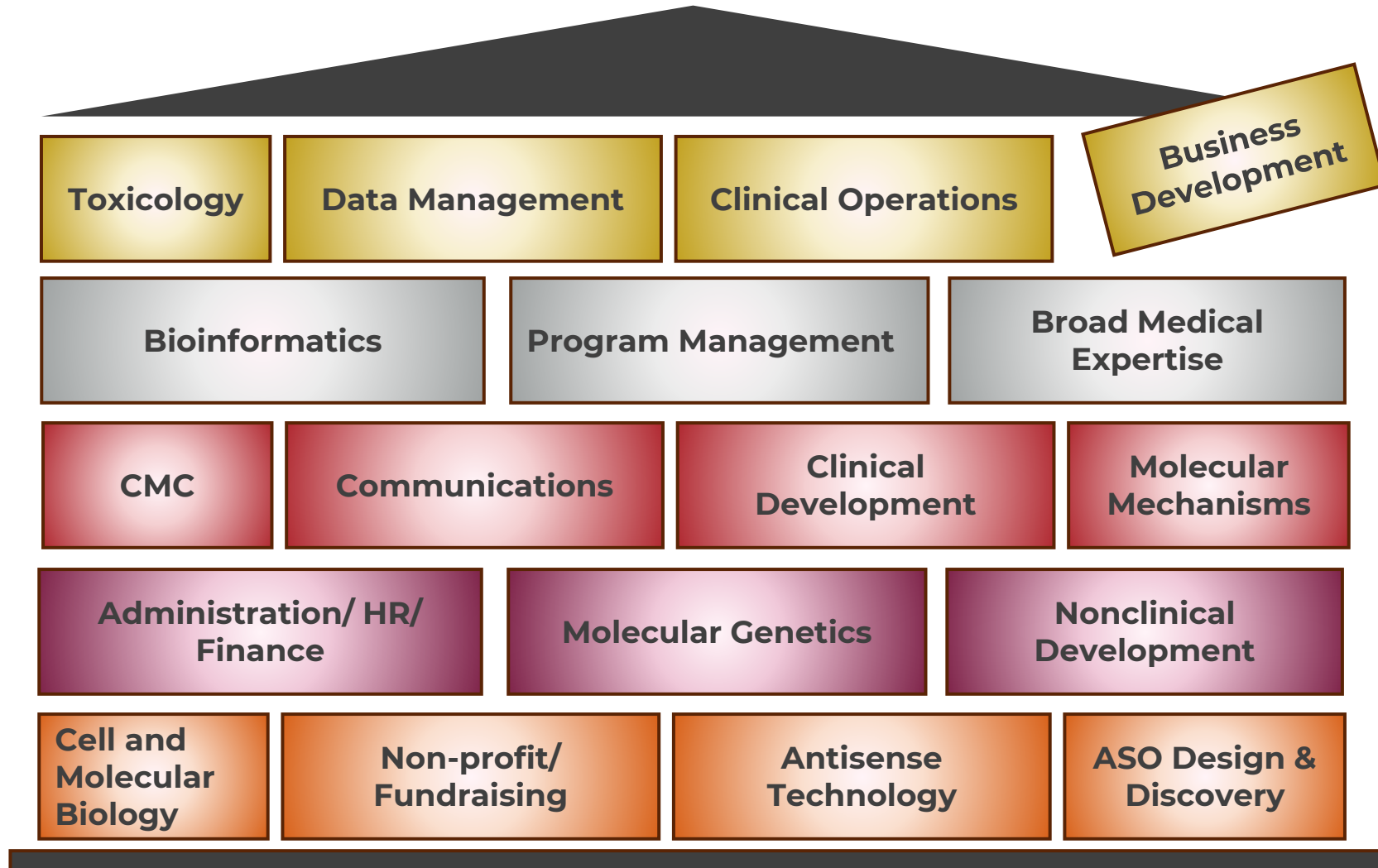
Expand **beyond the US**

6

Dedicated Initiatives for **unique patient subgroups**

Building the Foundation for a Lasting Organization

Hand-picked Talent with Expertise Integrated Across
all Core Functions



Cultivating a Powerhouse Team: Smart, Strong, Mission-driven

- Focus on the mission
- Grow and strengthen culture
 - Core values
 - Social activities
 - General support
- Develop team members
 - Education
 - Career advancement
- Expand internal capacity in accordance with demand and funding



Breakfast Club
June 04, 2024

Stanley Crooke's
Leadership Lessons
45 - 50

LESSONS IN ANTISENSE 2

HOW ARE ASOs MADE AND WHY

A central graphic element consisting of several overlapping rectangular panels. The top panel is white with the text 'Breakfast Club' and 'June 04, 2024'. To the right is a circular logo with a heart and a hand. Below this is a dark orange panel with the text 'LESSONS IN ANTISENSE 2'. To the left is a white panel with the text 'Stanley Crooke's Leadership Lessons 45 - 50'. At the bottom right is another dark orange panel with the text 'HOW ARE ASOs MADE AND WHY'.

1 Expand Solutions for Treatment Administration

- Increase number of patients treated at **existing treatment sites** by driving efficiencies through **repetition**
- Consider **CROs / clinical trial sites**
- Leverage existing NIH-funded **site networks** if possible
- Collaborate with academic or other professional societies to drive additional solutions **to fund clinical treatment** when funding is the sole barrier
- **Foster relationships** with advocacy groups to engage Centers of Excellence
- Maintain **consistent engagement** with academic professional and umbrella groups such as NTC and NORD to ensure broad-reaching solutions

2 Decrease Time Between Diagnosis and n-Lorem

- Build relationships with **diagnosing clinical genetics organizations/ institutions** across the US and world
- Establish collaborations with **biobanks or other databases** of diagnosed individuals that have ability to re-engage participants
- Discuss potential to insert opportunity for n-Lorem ASO treatment into **commercial genetic testing** results/ diagnoses based on mutation prevalence and function

3 Access to Long-term Affordable Care

- Engaging with organizations **focused on policies** related to rare disease treatments
- Payer environment
 - Support physician exploration of **Medicare/ Medicaid coverage** by state
 - Approach **private payers** for coverage
 - Differentiate **'research'** from **'clinical treatment'** after a sufficient time on treatment to show clinical benefit
- Collaborate broadly and share n-Lorem experience with broader **rare disease community** to drive progress

4

Bridge to Treatment for n of few

- **Several scenarios** cause n of 1 to become n of few:
 - Increase in diagnosing/ sequencing between patient acceptance and treatment
 - Non-allele selective approach for n of 1 mutation
 - Allele-selective approach targeting (more) common SNPs
- n-Lorem strategy:
 - To treat **several patients** as allowed by the FDA and supported by n-Lorem's model to generate clinical data
 - Consider submission of several patients with **similar phenotypes** in single IND
- Clinical data exemplifying pristine safety and clinical benefit in a single patient provides a **powerful tool** to bring treatment to n of few populations
- Collaborate with potential partners and FDA to **enable access to additional patients beyond what n-Lorem can help**

5 Expand Beyond the US

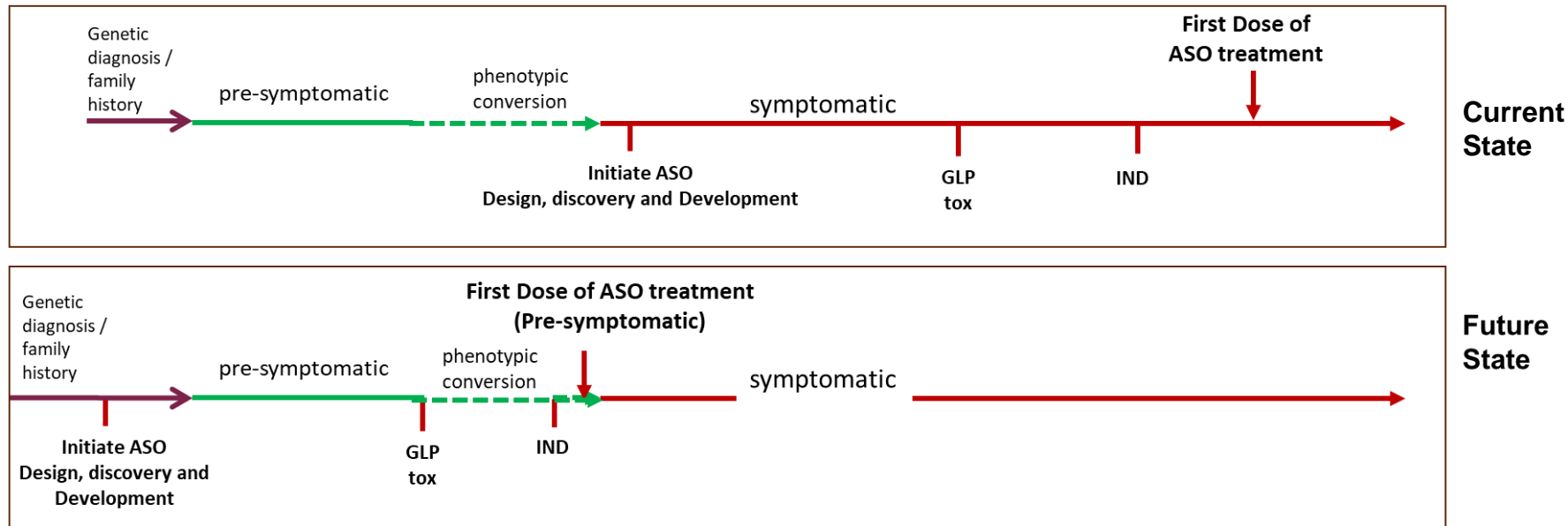
- Leverage ASOs with approved US Research INDs
- Initiate the path for funding and regulatory requirements by treating a single patient
- Requirement to maintain consistency in clinical protocol and data collection to protect ongoing US patient treatment
- Several partnering discussions ongoing with the objective of establishing a funding, regulatory, and operational infrastructure to enable treatment with existing n-Lorem ASOs in other countries

Silence ALS Initiative

- Partnership between n-Lorem and the Eleanor and Lou Gehrig ALS Center, Columbia, **PI Dr. Neil Shneider**, and many treating physicians
 - Enables seamless collaboration with other leading ALS Treatment Centers throughout the US and globally
- Leverages the **existing knowledge base** regarding ALS genetics and regulatory/ clinical experience surrounding FUS ASO
- Tackles **operational challenges** related to aggressive nature of the disease and clinical manifestations
- Accelerated advancement enabled by **directed funding** (NIH Urgent, Target ALS)

Silence ALS Initiative : Enable Patient Treatment Earlier

- Treat ALS patients as close to phenotypic or symptomatic conversion as possible with an optimized/individualized ASO (ideally prior to symptom onset)
- 32 ALS patients with ASO programs in n-Lorem portfolio
- 5 INDs approved for ALS patients
- 4 Patients treated
- Pristine safety and tolerability profile, early indicators of clinical benefit
- Creating knowledge, experience and efficiencies that inform our approach for the next ALS patient and across diseases



Significant Progress has been Made ... and We Need Your Help



1 **Expand Solutions** for treatment administration

2 **Decrease time between diagnosis and n-Lorem**

3 **Access to long-term affordable care**

4 **Bridge to treatment for n of few**






































5 **Expand beyond the US**

6 **Dedicated initiatives for unique patient subgroups**

Conclusions

- n-Lorem organization has been built with a **powerhouse team** with highly integrated functions
- **Significant progress** has been made beyond n-Lorem for the nano-rare, including expanding treatment locations, operational efficiencies for specific disease groups, paving a path for n of few
- n-Lorem is **nearing patient treatment** in several countries **outside of the US** with existing ASOs
- Finding strategic solutions for challenges including **earlier diagnosis** to enable earlier ASO discovery and **access to treatments** is of the **utmost priority** and involves the **broader rare disease community**
- **Partnerships and engagement with community** are critical to progress all

Support From Leaders Across All Areas of Drug Discovery, Development and Manufacturing *More than 30 Partners Support Nano-rare Patients*

Biotech/Pharma Companies	Genomic Sequencing	Pre-clinical Toxicology CROs	Manufacturing/ Drug Supply	Foundations Grant Organizations	Other
      	     <div data-bbox="491 842 848 1078" style="background-color: #4a2020; color: white; padding: 10px; border-radius: 15px; text-align: center;"> <p>Access to Appropriately Characterized Patients and Investigators</p> </div>  <p>Other personalized medical centers</p>	     <div data-bbox="861 928 1217 1028" style="background-color: #800000; color: white; padding: 5px; border-radius: 10px; text-align: center;"> <p>Disease Focused</p> </div> <p>ASXL3 FSHD2 MAPK8IP3 Silence ALS</p>	      <div data-bbox="1230 1156 1587 1256" style="background-color: #808080; color: white; padding: 5px; border-radius: 10px; text-align: center;"> <p>Clinical Management</p> </div> 	<p>THE CONRAD PREBYS FOUNDATION</p>   <p>Wolverine Foundation Anonymous Donor URGenT NIH Grant</p>  <div data-bbox="1600 1013 1956 1113" style="background-color: #808000; color: white; padding: 5px; border-radius: 10px; text-align: center;"> <p>Sterile Fill Product</p> </div>  	    <div data-bbox="1969 785 2326 828" style="background-color: #003366; color: white; padding: 2px; border-radius: 5px; text-align: center;"> <p>J. WOOD CAPITAL ADVISORS</p> </div>  <div data-bbox="1969 956 2326 1056" style="background-color: #e67e22; color: white; padding: 5px; border-radius: 10px; text-align: center;"> <p>Data Partners</p> </div>  

Thank You

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