



3RD ANNUAL

Nano-rare Patient Colloquium 2025

Closing Remarks

Stanley T. Crooke, M.D., Ph.D.
Founder, Chairman of the Board and CEO

October 20, 2025

Thank You!

Clinical Investigators

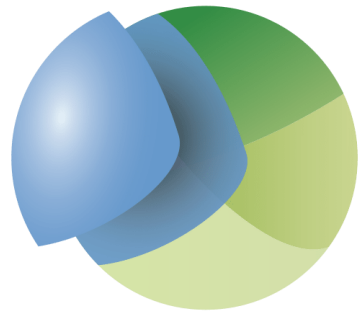




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Biogen



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Thank you

Speakers and Panelists



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Patients and Families



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Thank you

Audience



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Privilege



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All Diseases Are Unfair But Nano-rare Diseases Seem The Most Unfair

The Response to Disease is Primal and Universal

- Deep visceral fear
- Reaction to the unfairness of disease
 - Primal and universal
 - Anger
- But good people know that anger and bitterness simply make things worse
 - Adjust dreams and expectations
 - Find the positives and focus on them
 - Focus outward – How can I help others?



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**We experience humans at their best – as
they express their most noble selves**



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INVICTUS



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**Night can come and it can be as black as
the pit
We can't control that
But, we can control how we respond**

The Privileges of n-Lorem

- We experience humans at their best – as their most noble selves
- Then, to address the problems of our patients, we practice cutting edge science
- We experience the power of **HOPE**
- We experience the impact of effective treatment on **our patients and families**



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A Single Skill That is Unique to Humans

The ability to dream collectively

Collective Dreams

- Reshaped our planet
- Reshaped our understanding of the universe
- Reshaped our understanding of how we function and why we fail





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COLLECTIVE DREAMS Happen the same
way every time

**An individual sees the world as it is
and as he/she thinks it should be –
that is a dream**



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But collective dreamers have more than a dream – they also have a plausible plan to realize the dream

And the dream, the plan, and the dreamer are galvanic enough to recruit others to work to realize the dream



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**COLLECTIVE DREAMS don't just happen,
they are willed into existence
by hard work
And perseverance through disappointments,
failures and skepticism**



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**We share a COLLECTIVE DREAM
We are working toward a future in
which all nano-rare patients are
rapidly diagnosed and have access to
effective treatments**



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**Our Dream Is, as it should be focused on
the future
But, our actions are here and NOW and
directly focused on helping patients in
need TODAY**

Together We have Done the Impossible: We have Shown that We **Can...**

- Establish a non-profit model that delivers care equitably to nano-rare patients
- Start a new ASO discovery program for each patient and deliver the first dose in 18-24 months
- Treat nano-rare patients for life for \$1.2 Million -not hundreds of millions
- Reduce the cost per patient to <\$1 million for life
- Industrialize the process and scale to meet the demand
- Generate high quality data with the modified cross-over clinical trial design we established

Together We have Done the Impossible: We have Shown that We **Can...**

- Create a knowledgeable community of and for the nano-rare patient and family
- Draw attention to the needs of nano-rare patients and families

Together We have Done the Impossible: We have Shown that We **Can...**

- Treat patients with very advanced diseases and show meaningful benefit
- Effectively treat retinal diseases, kidney disease, liver disease and a wide range of CNS diseases
- Reduce neuropathic pain with ASOs
- Treat autonomic disorders with ASOs
- Many developmental delays can be recovered with ASO treatment
- Long-term benefit can be achieved, even in patients with advanced diseases
- With in-depth knowledge of ASO technology, industrialized systems and caution, ASOs can be given safely to nano-rare patients



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We Can Hope for More and Dream Bigger



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**We have not done this alone – we’ve had
the support of
The FDA,
Numerous individual donors,
Numerous patient focused donors,
CRO partners,
CMO partners,
Commercial partners,**



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**And the motivation of the privilege of
serving our patients and families.
We THANK YOU for the opportunity
to serve.**



We Need Everyone's **HELP**



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