



'Innovative RNA-based Therapeutics

acting at

the cause of the disease'

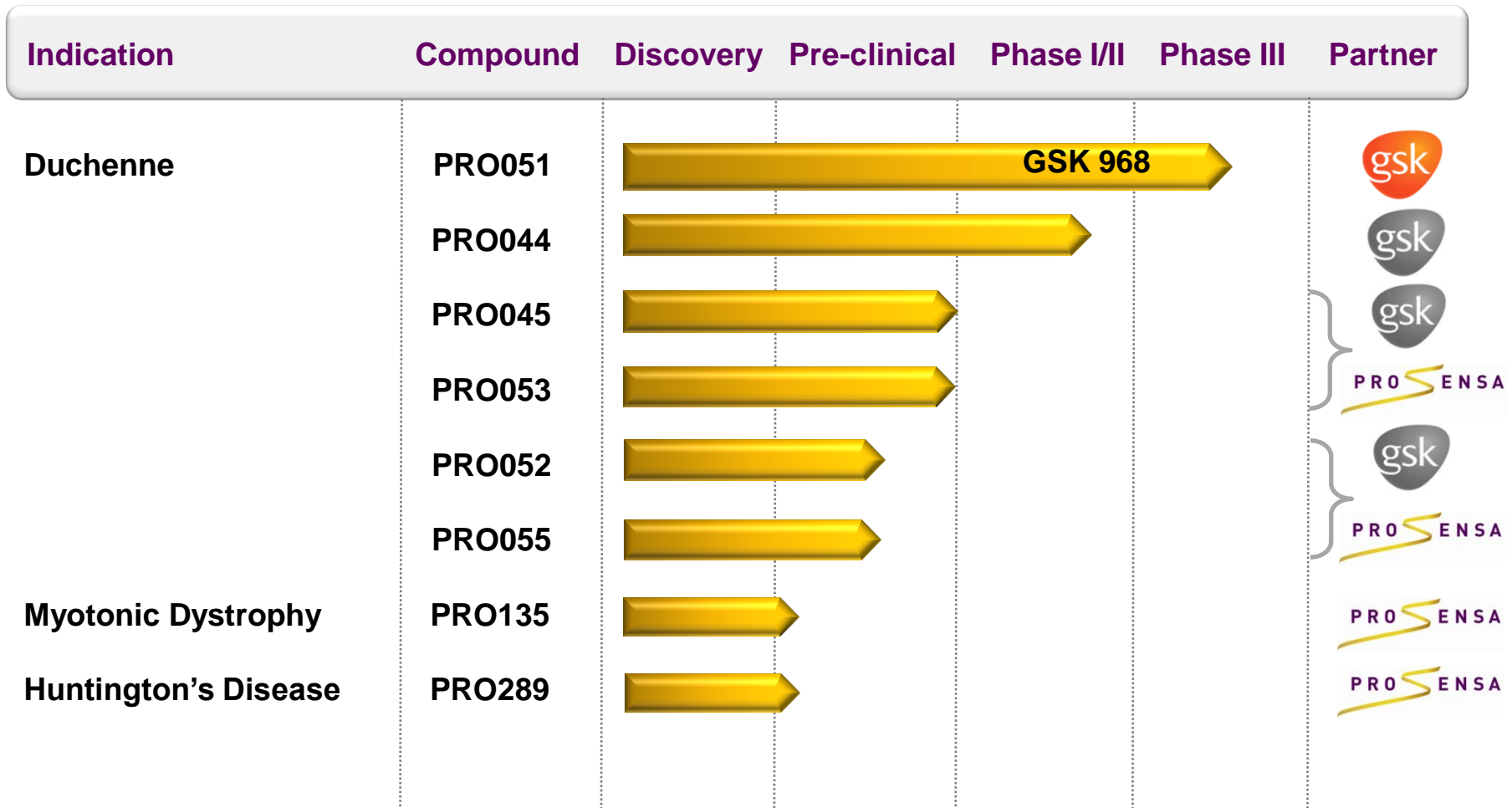
Expectations of Clinical Trials

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Action Duchenne Conference – 4th and 5th November 2011

*To develop innovative,
RNA-based therapeutics
to fill unmet medical needs
for patients with genetic
diseases*



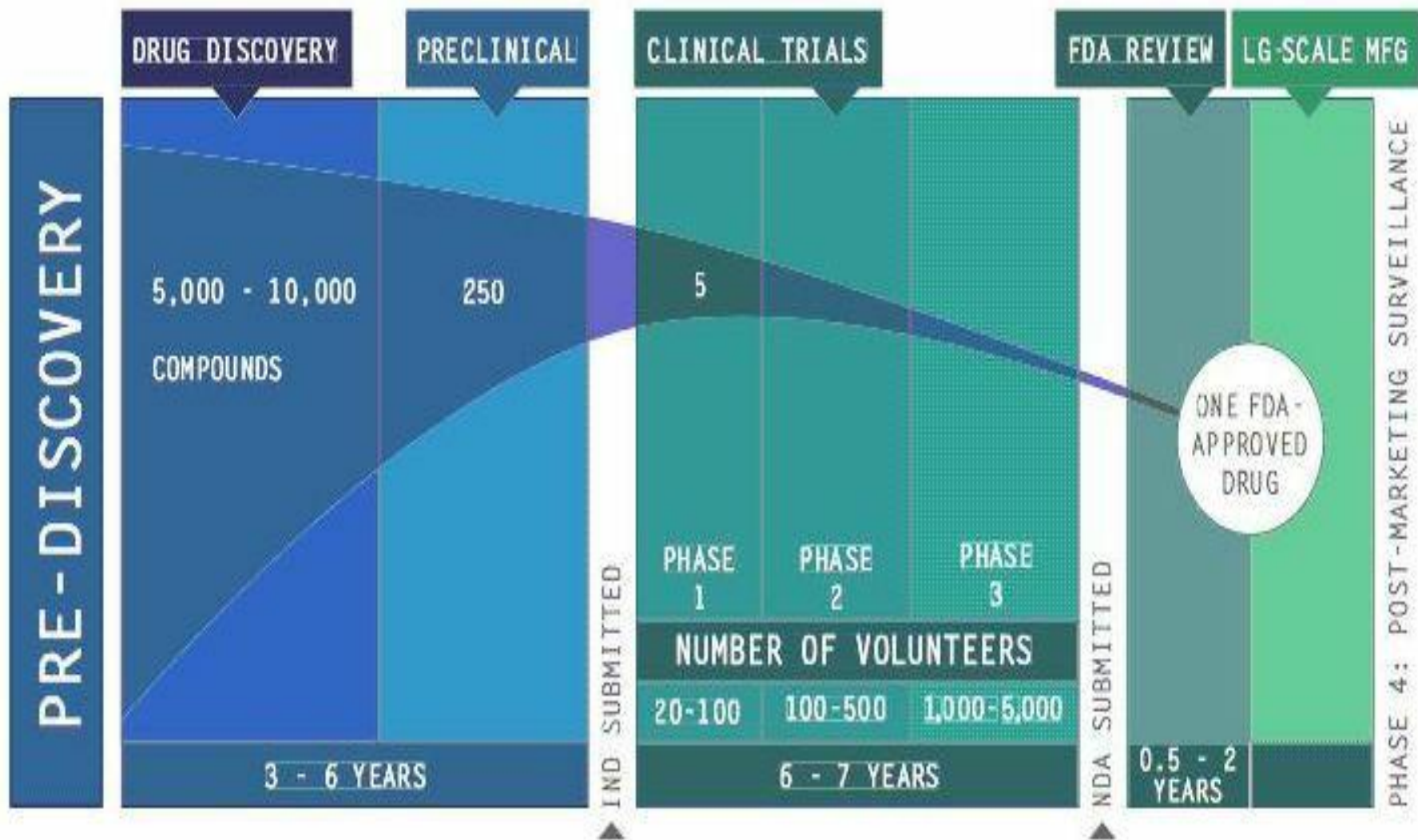


- What is a clinical trial
- The Orphan World
- The Challenges we face
- Participating in a Clinical Trial
- Expectations

What is a Clinical Trial?



Drug Development Timeline



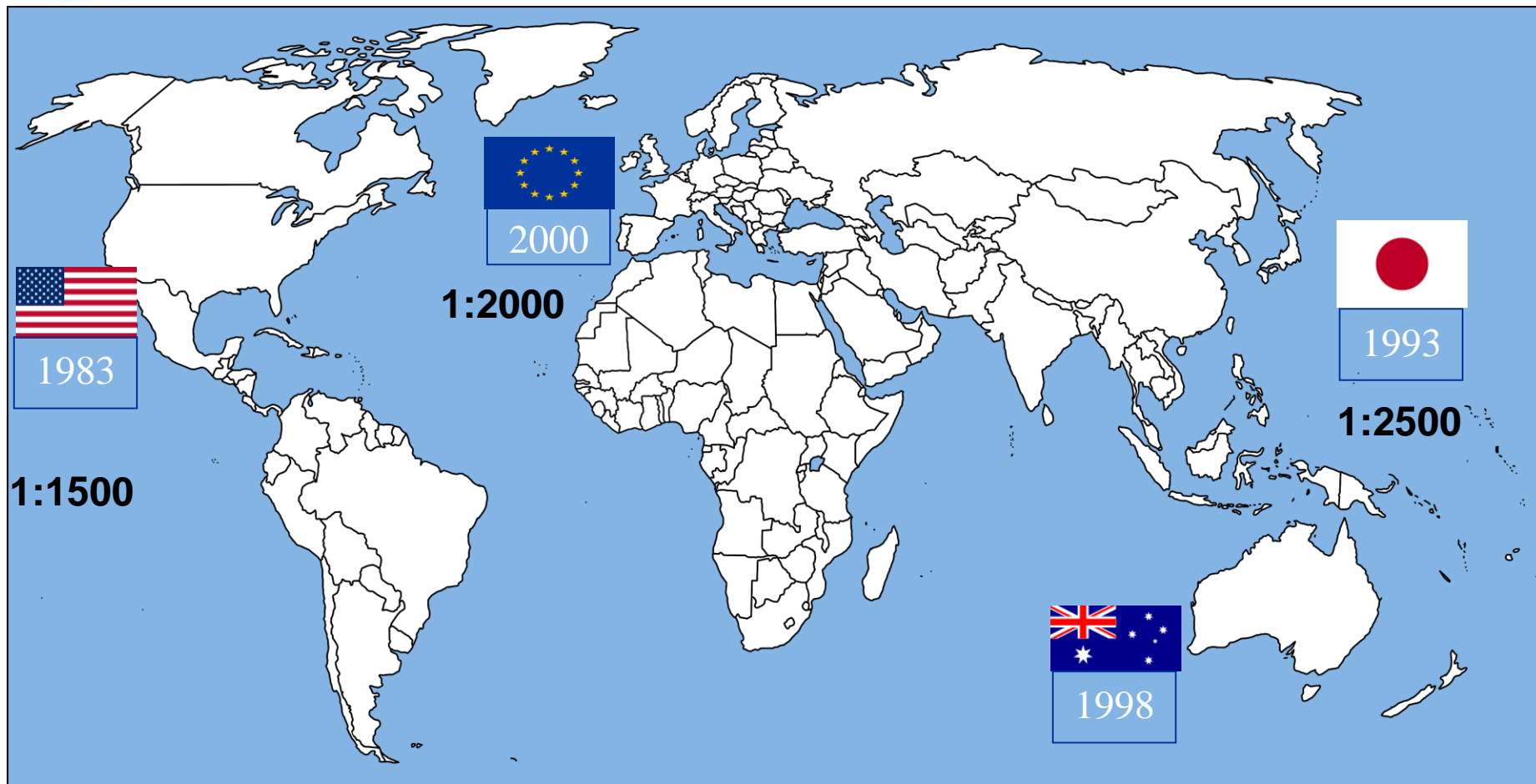
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- The Rarity
- Science and animal models
- Manufacturing specialist
- Functional outcome parameters
- Regulatory pathway for approval
- Reaching the market



- **Orphan drugs are drugs intended for the diagnosis, prevention or treatment of rare diseases**
- **Orphan drug legislation for severe, life-threatening or chronic disabling diseases that strongly reduce the quality of life**
- **Most rare diseases affect 1 in 100,000 people or less.**
- **Under usual circumstances it would cost more to develop an orphan drug, than would be gained in sales**



To encourage research and drug development for diseases in smaller patient populations.

Rare, Rarer, Rarest...

Orphan drug Europe 1 : 2,000

DMD 1 : 3,500

Ultra-orphan 1 : 50,000

Exon 51 skip 1 : 200,000

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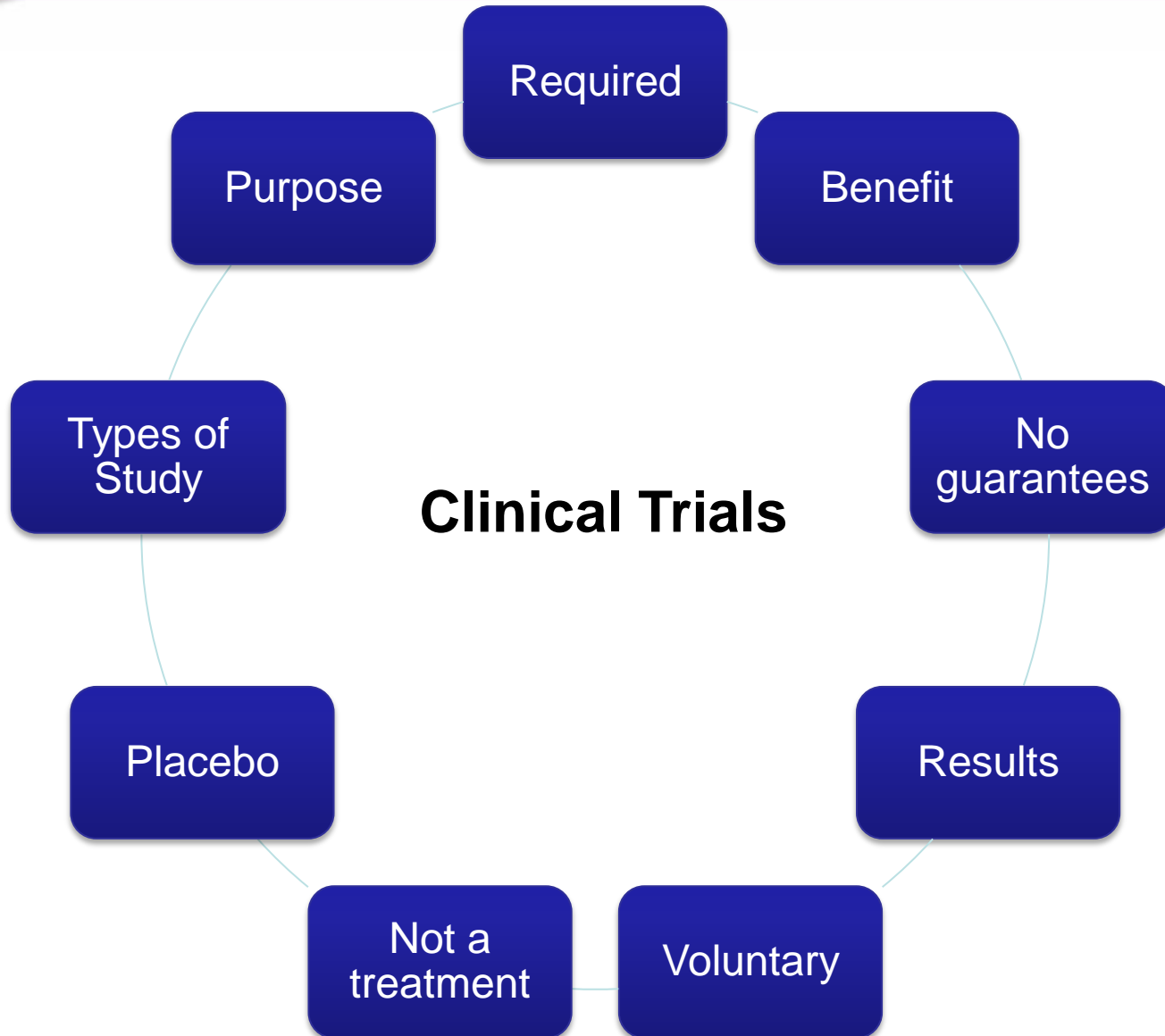
- **Wall Street Journal 12th Oct 11 - National Organisation of Rare Disease (NORD) review of FDA approach to orphan drugs**
- 2 of every 3 orphan drugs approved (135 drugs since 1983) show FDA's flexibility in its review.
- More limited data than usual accepted for approval
- More customised approval process of orphan drugs
- "FDA has demonstrated that it recognizes the importance of therapies for persons with rare disorders".
- Recommended flexibility documented in formal FDA policy

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Participating in a Clinical Trial – The Potential Benefits

- Standard of care
- Access to drugs before they are widely available
- Helping to progress research in DMD – without clinical trials no drug would be approved
- DMD114118



- Consent/Assent
- Screening visit
- Randomised
- Study visits/procedures
- Early Withdrawal visit
- End of Study
- Extension/follow on study



Benefits?

Previous trials?

Purpose?

Adverse events?

Questions to ask

What else?

Active vs Placebo?

Who knows?

Further treatment?

Results?

How many doses?

Route?

Length of study?

Questions to ask

Financial Costs?

How many visits?

Impact on family life?

What do I have to do?

- The production of high-quality knowledge on DMD and development of effective and safe treatments is our overall aim
- Clinical trials are not straightforward
- Regulatory agencies have shown flexibility
- Clinical Trials are not a treatment/cure
- Clinical Trials is the road to our objective

PROSENSA

RNA modulation to fight Duchenne Muscular Dystrophy

How **PROSENSA** can make a difference:

- RNA modulating therapeutics
- Correction of gene expression in diseases with a large unmet medical need
- Focus on neuromuscular disorders

“Why are my muscles
not so strong?
I really hope they find
something soon!”

Prosensa

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