

SOMETHING BETTER THAN HOPE

Right now.

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Every Moment Counts. **Don't Stop** Now.

Clinical Trials in Regenerative Medicine – Benefits of a Consortium



- o What could a cell or gene therapy approach offer that is different from traditional/past approaches?
- o Are there approaches in other disease areas that the ND community should look to model?

Neuro Degeneration Therapeutics Development

Disease Biology

- Genomics
- Organ in a dish



Mechanism of Action

- Complex
- Monogenic
- Targeted



Regulatory Development

- Surrogate Endpoints
- Novel Endpoints



Clinical Trial Design

- Real World Evidence
- Patient Journey
- Disease Natural History

