



## **Restoring neural networks**

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# ReNetX Bio: Clinical stage company

## Significant Opportunity for Patients and Partners

### Leadership Expertise

Extensive experience in building & exiting biotech investments; Raised \$30M with vetted partners

### Technology/IP

Yale Innovation; extensive issued worldwide patents; new IP filed & actively in process; additional 12 years of regulatory exclusivity with BLA

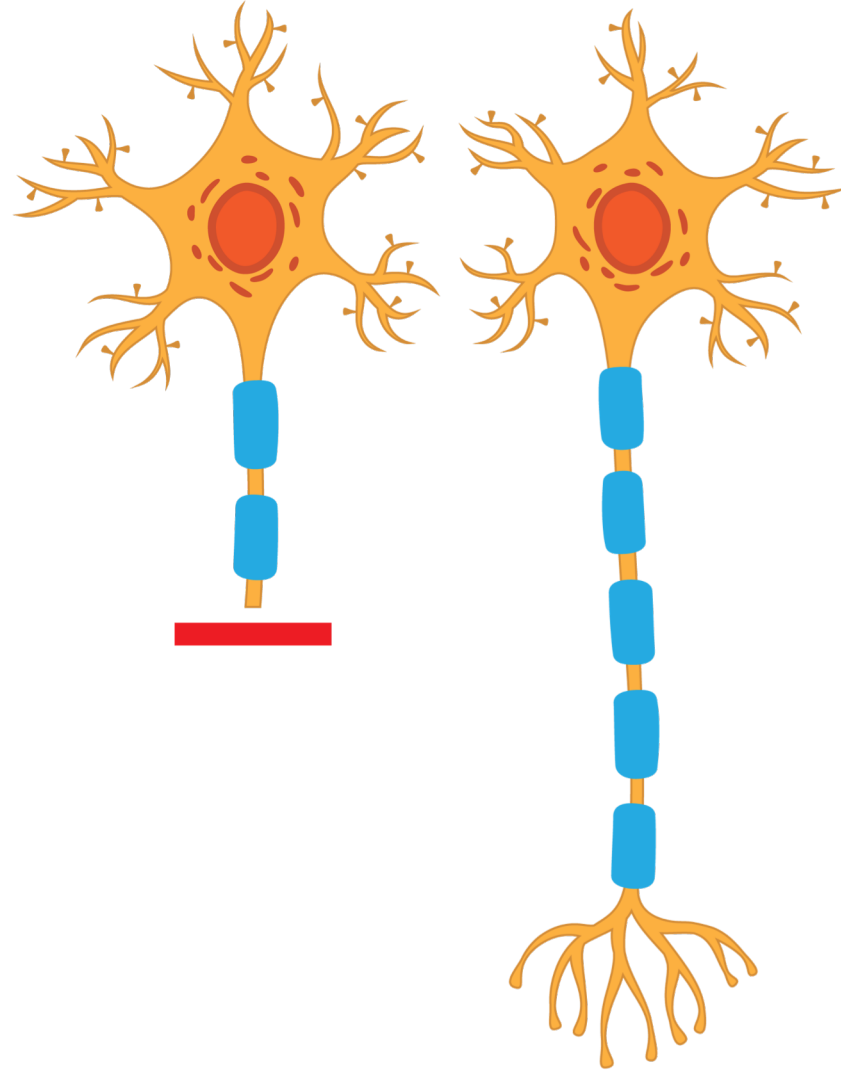
### Unmet Market Need

Pipeline >\$5B indications across neurology & ophthalmology

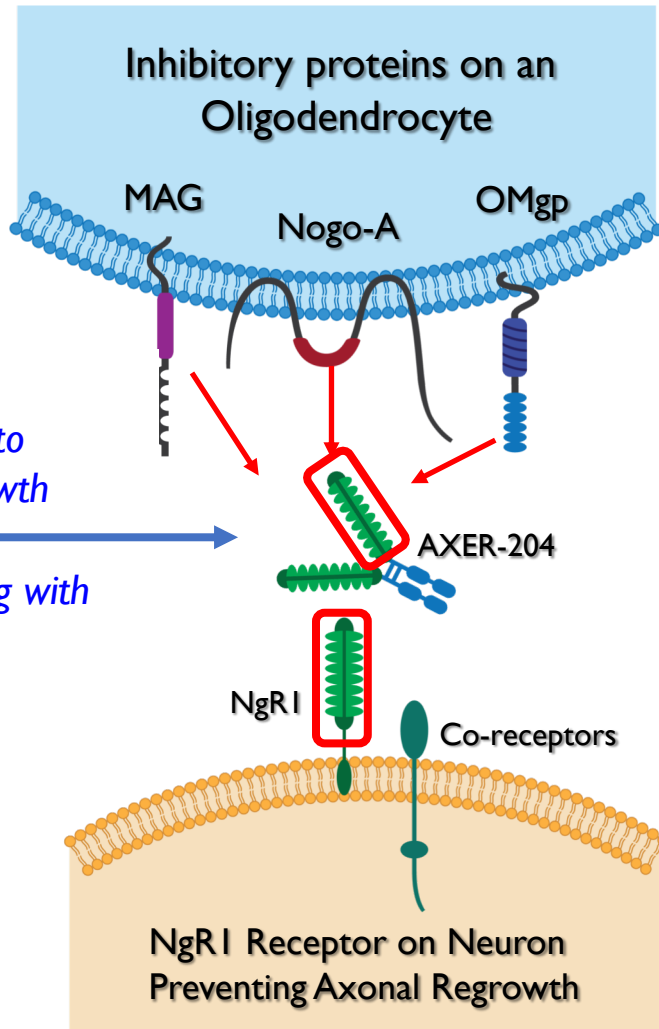
### Significant Return

Support from global biotech; Key Exit Inflection Point with data readout in early 2022

# What is the Problem We're Solving?



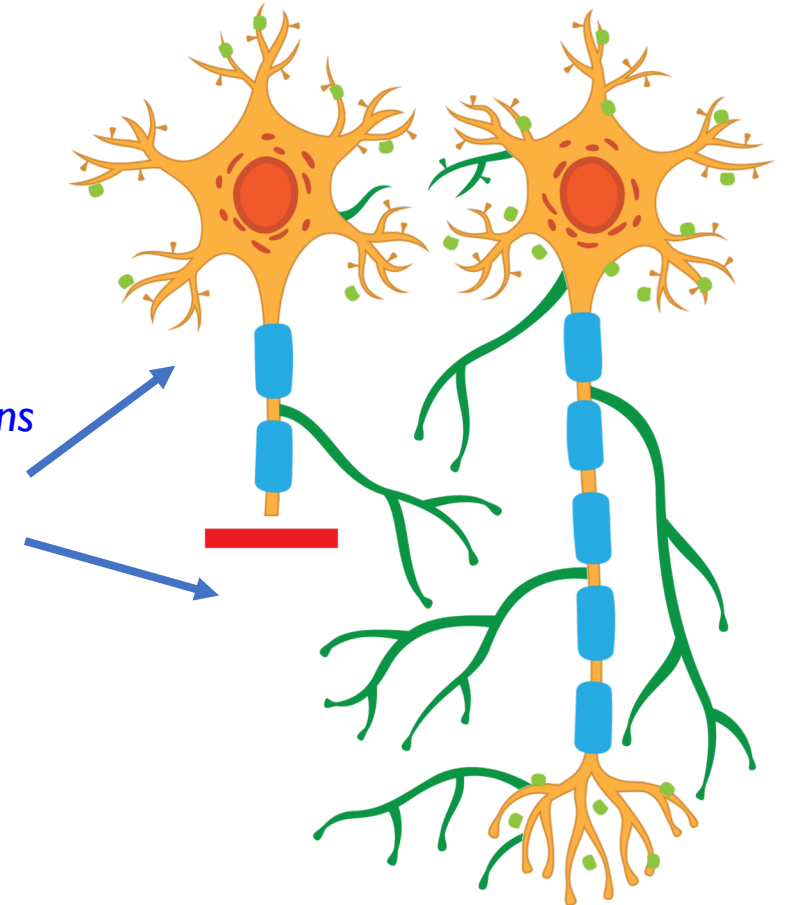
# ReNetX Approach: Block Axonal Growth Inhibitors in the CNS to Promote Neural Repair



*Nogo-A, MAG, OMgp bind to NgR1 preventing axonal growth*

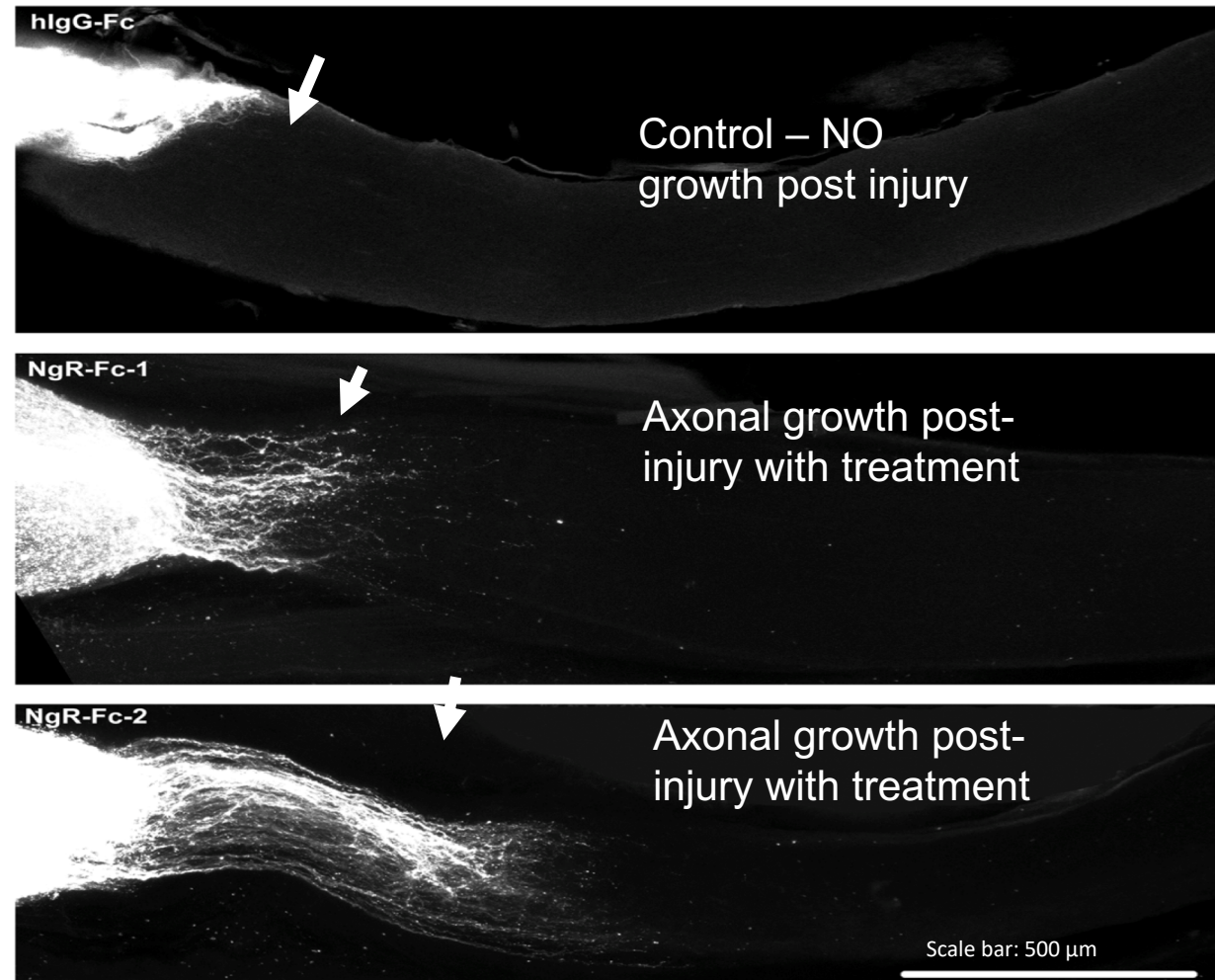
*AXER-204 prevents binding with broader targets than single antibodies.*

*AXER-204 promotes new neural connections through axonal sprouting, axonal regeneration, and synaptogenesis*



# Neuroprotection & Neurorestoration in Ophthalmology

## Optic Nerve Axonal Growth



# The ReNetX Difference

## Insults to the Nervous System

Inflammation →

Intraocular pressure →

Trauma →

Metabolic/Toxins →

**AXON  
LOSS**

## Diseases

Multiple Sclerosis

Neurodegenerative  
Diseases

Optic Nerve Damage  
(Glaucoma/Optic Neuritis, etc)

Traumatic injuries

## **ReNetX Next Generation Therapy**

- ✓ Changing the underlying environment in the CNS promoting regrowth.
- ✓ Synergistic with existing therapies.
- ✓ Strong safety profile – no off-target effects

# Extensive Independent Preclinical Validation of Mechanism

Glaucoma models & optic nerve injury	Rat	RGC protection ↑ Axonal growth	<b>Yale, Hong Kong</b>	Wang et al, IOVS 2015 56:1357-1366 & Fu et al, IOVS 2011 52: 8374-8380.
Sub-chronic SCI, hemisection	Monkey	↑ Forelimb use ↑ Motor function ↑ Axonal growth	<b>Yale</b>	Wang et al, Brain 2020 143:112-121.
Chronic SCI, Contusion	Rat	↑ Functional Recovery ↑ %Weight bearing ↑ Axonal growth	<b>Yale</b>	Wang et al, J Neurosci. 2013 33(10):3431-3441.
Sub-acute SCI & Acute SCI, Contusion	Rat	↑ Axonal growth	<b>Yale</b>	Wang et al, J Neurosci. 2013 33(10):3431-3441.
Sub-acute SCI, Contusion	Rat	↑ Axonal growth	<b>Yale</b>	Wang et al, J Neurosci. 2013 33(10):3431-3441.
Acute SCI, Contusion	Rat	↑ Axonal growth	<b>Yale</b>	Wang et al, J Neurosci. 2013 33(10):3431-3441.
Acute SCI, Trauma	Rat	↑ Axonal growth	<b>Univ. of Toronto</b>	Guo et al, Cell Transplant. 2012 Jan 10. epub ahead of print
Dorsal root crush	Rat	↑ Forelimb use ↑ Synaptic function ↑ Axonal growth	<b>Tufts University</b>	Harvey et al, J Neurosci. 2009 13;29(19):6285-95.
Dorsal column crush	Rat	↑ Axonal regeneration	<b>Yale</b>	Wang et al, Exp Neurol. 2012 237(1):55-69.
Middle cerebral artery occlusion	Rat	↑ Forepaw use ↑ Rotorod performance ↑ Axonal growth	<b>Yale</b>	Lee et al, J Neurosci. 2004 24(27):6209-17.

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**Biotech Companies Seek to Restore Functions to Spinal-Cord Injury Patients**

PRO VC INDUSTRY NEWS

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Biotech companies are racing to develop therapies that can restore functions to spinal-cord injury patients. The race is on to develop therapies that can restore functions to spinal-cord injury patients. The race is on to develop therapies that can restore functions to spinal-cord injury patients.

Biotech companies are racing to develop therapies that can restore functions to spinal-cord injury patients. The race is on to develop therapies that can restore functions to spinal-cord injury patients. The race is on to develop therapies that can restore functions to spinal-cord injury patients.

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# Executive Leadership

**CEO**



Erika R. Smith, MBA

**President &  
CSO**



George Maynard, PhD

**CMO**



Gilbert Block, MD, PhD

**VP Process  
Science &  
Manufacturing**



Craig Hackett, PhD



Yale



# Advisory Board Leadership

**Dr. Stephen Strittmatter, MD, PhD**  
Founder, Chief Scientific & Medical Advisor

Recognized key opinion leader in neuro-regeneration & neuro-degeneration from Yale University

**Dr. Daniel Lammertse, MD**, Medical Director (ret.); Craig Hospital

30+ years managing a leading rehabilitation center & supporting all key SCI trials

**Dr. Andrew Blight, PhD, CSO** (ret.)  
Acorda (NASDAQ: ACOR)

Career leadership in neurology-based innovation with extensive experience in SCI

**Dr. Jeffrey Goldberg, MD, PhD**,  
Professor & Chair of Ophthalmology,  
Byers Eye Institute; Stanford University

Key opinion leader in glaucoma innovation & biomarker strategies

**Dr. Jim Tsai, MD, MBA**, President, New York Eye & Ear Infirmary of Mt. Sinai

Delafield-Rodgers Professor & System Chair, Dept. of Ophthalmology; Icahn School of Medicine at Mount Sinai

**Dr. Kevin Malobisky, PhD**, Chief Regulatory & Strategic Operations Officer, Tavanta

Previously led the Achillion team to FDA breakthrough therapy & \$1B acquisition to Alexion



**The 2017 Blavatnik Award winners flanked by  
Erika Smith, Director of the Innovation Fund  
& Yale President Peter Salovey**

# The RESET Clinical Trial

*Objectives: To evaluate the safety, pharmacokinetics & efficacy of AXER-204 in participants with chronic cervical SCI having significant but incomplete impairment of hand & arm function*

## **Part 1: Single Ascending Dose**

Four cohorts

Each successive cohort receives a higher single dose

**Part I Complete**



Data and Safety Monitoring Board Review

## **Part 2: Repeat Dose**

Dose and frequency of dosing based on data from Part I

**Data Readout in Early 2022**

The Trial will test:

- Safety & tolerability
- Hand & arm function changes
- Changes in ability to perform daily living activities
- Pharmacokinetics
- Antidrug antibodies
- Potential biomarkers

## **Results so far:**

- **Strong safety profile**
- **Trending efficacy**
- **Biomarkers of target engagement and axonal growth**

<https://clinicaltrials.gov/ct2/show/NCT03989440>

# Extensive Pipeline Opportunity

## NgR1 Platform

### NEUROSCIENCE

Indication	Research	Preclinical	IND/Phase 1	Phase 2	Phase 3
SPINAL CORD INJURY AXER-204	<b>FDA Fast Track: <a href="https://clinicaltrials.gov/ct2/show/NCT03989440">https://clinicaltrials.gov/ct2/show/NCT03989440</a></b>				
STROKE	██████████	██████████	██████████	██████████	██████████
MULTIPLE SCLEROSIS	██████████	██████████	██████████	██████████	██████████
OTHER CNS AXONAL INJURIES & DISEASES	██████████	██████████	██████████	██████████	██████████

### OPHTHALMOLOGY

Indication	Research	Preclinical	IND/Phase 1	Phase 2	Phase 3
GLAUCOMA	██████████	██████████	██████████	██████████	██████████
OPTIC NEURITIS	██████████	██████████	██████████	██████████	██████████
OTHER DISORDERS WITH OPTIC NERVE INJURY	██████████	██████████	██████████	██████████	██████████

# ReNetX Bio: Clinical stage company

## Significant Opportunity for Patients and Partners

Leadership Expertise

Extensive experience in building & leading clinical stage companies; Raised \$30M with vetted partners

Technology/IP

Year 1 data expected early 2022; New clinical trials, new IP filed and 5 years of regulatory exclusivity with BLA

Unmet Market Need

5 FDA indications across neurology & ophthalmology

Significant Return

Support from global biotech; Key Exit Inflection Point with data readout in early 2022

Top line data expected early 2022  
Let's talk about partnerships & funding  
esmith@renetx.com



