Stem Cell Research in Rare Neuroimmune Diseases
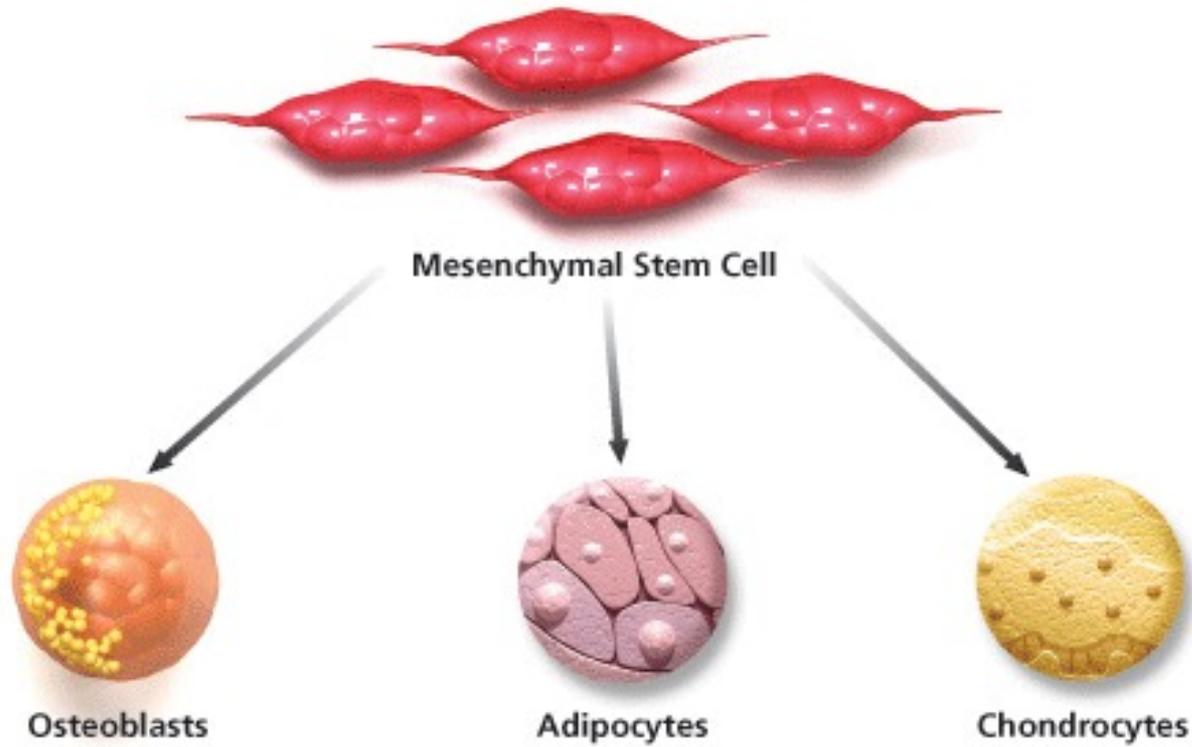
Michael Levy, MD, PhD

Associate Professor

State of the Field

- Stem cell trials in demyelinating conditions:
  - Spinal cord injury: 13 (US sites: 2)
  - Multiple sclerosis: 13 (US sites: 4)
  - Neuromyelitis optica: 2 (US sites: 2)
- Routes of administration:
  - Direct surgical transplantation
  - Intrathecal injection
  - Intravenous infusion
  - Combinations
- Types of stem cells: Mesenchymal, Schwann, Olfactory ensheathing, fetal neural, induced pluripotent stem cells
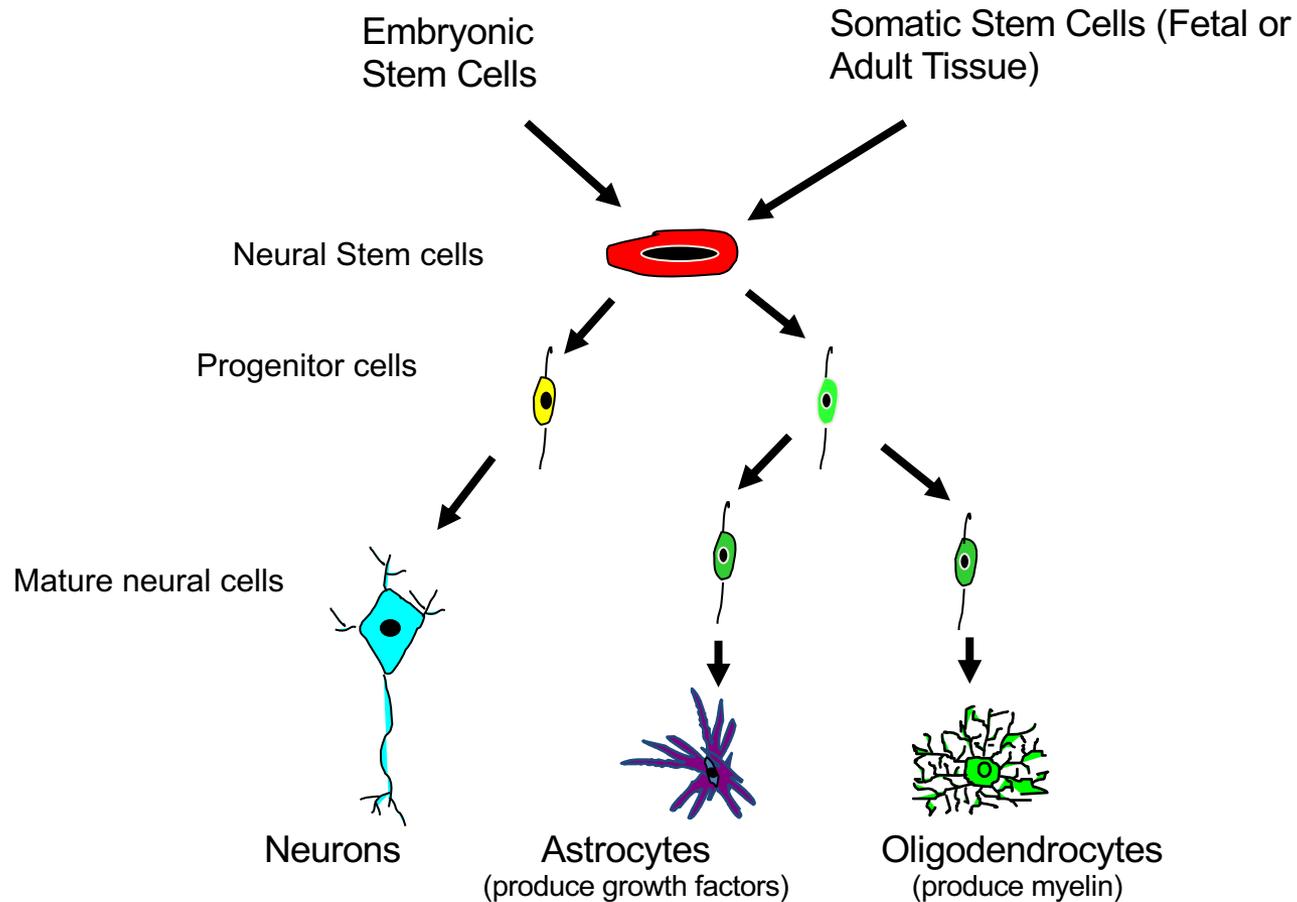
Sources of Stem Cells: Bone Marrow

Mesenchymal Stem Cell

Osteoblasts   Adipocytes   Chondrocytes
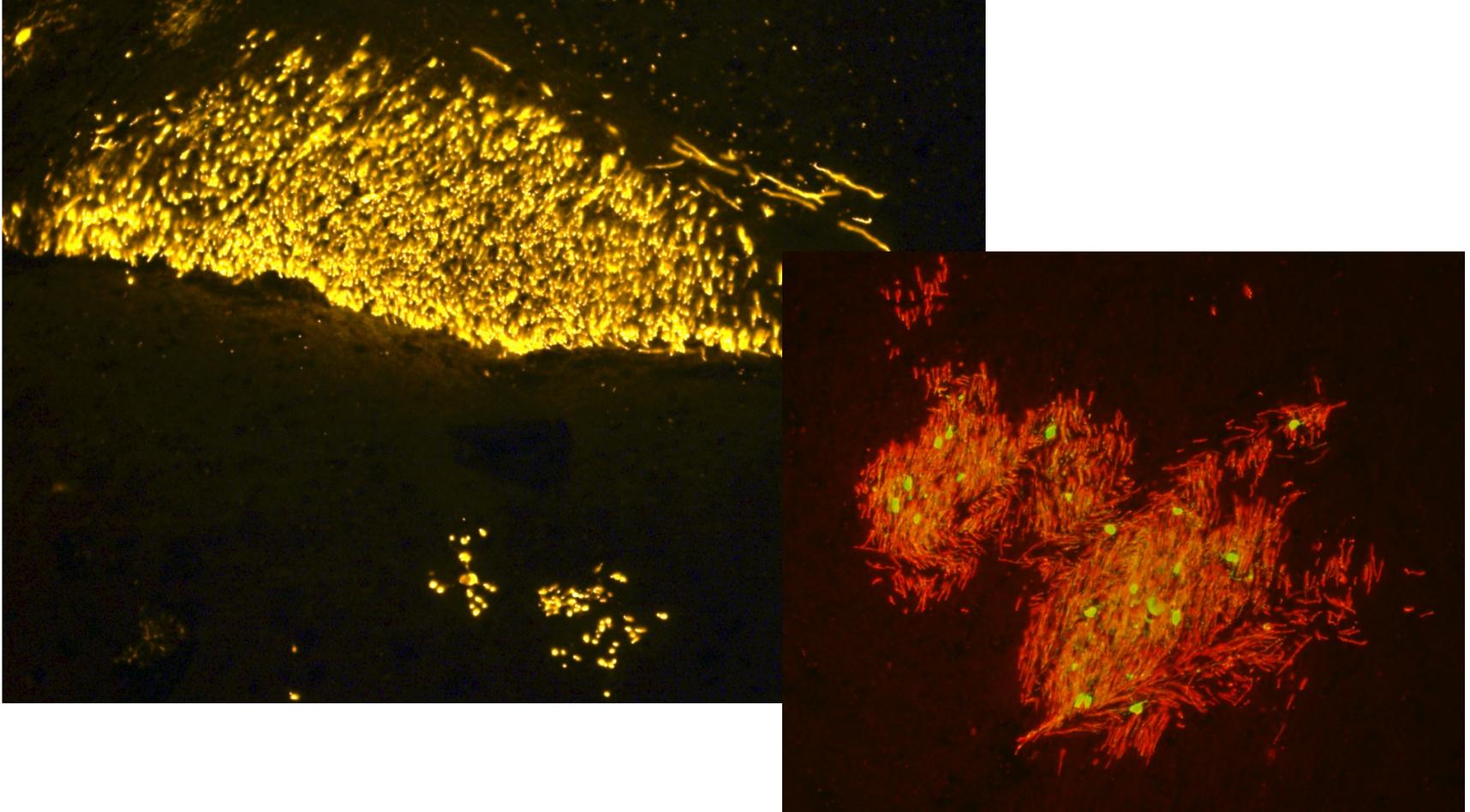
Results of Cleveland Clinic Trial

- 24 patients with MS
- Intravenous infusion
- No safety issues
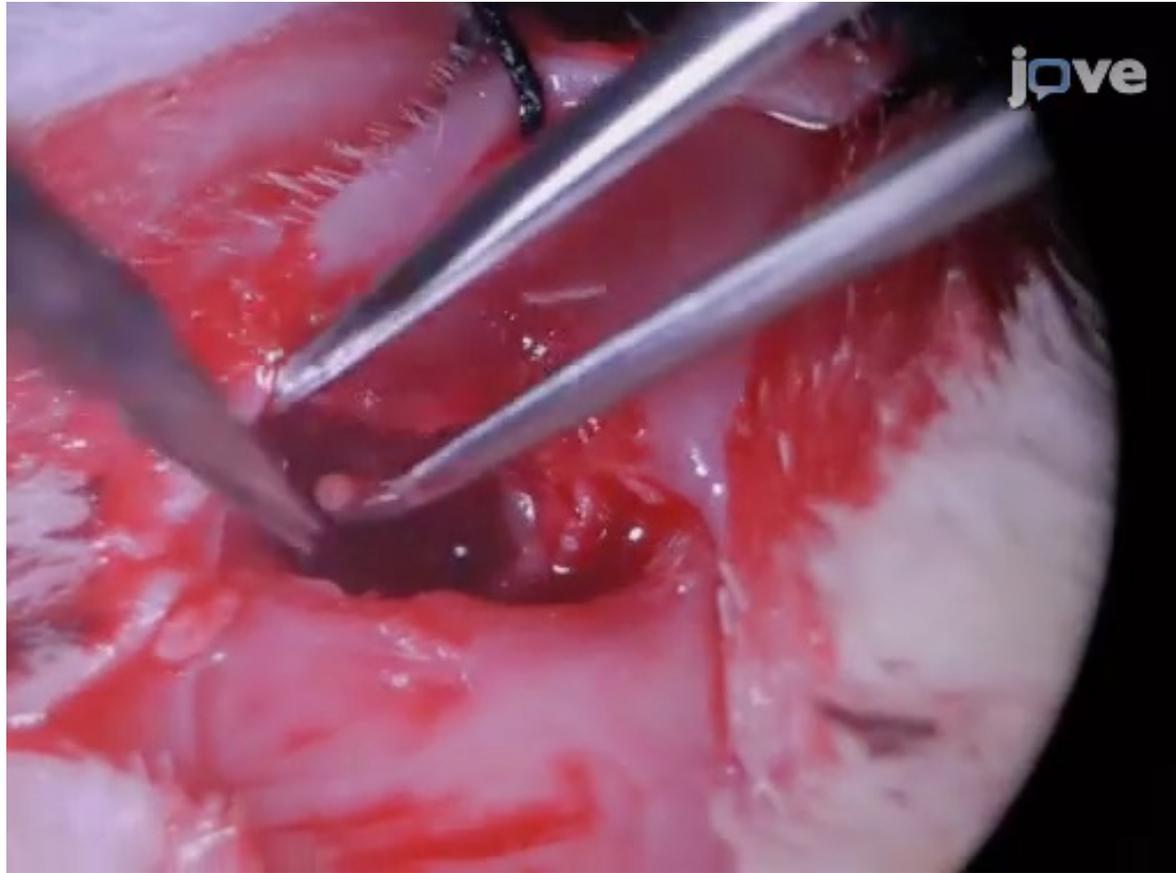- 6 months later: no clear benefit

Regenerative Stem Cells

Embryonic
Stem Cells

Somatic Stem Cells (Fetal or
Adult Tissue)

Neural Stem cells

Progenitor cells

Mature neural cells

Neurons

Astrocytes
(produce growth factors)

Oligodendrocytes
(produce myelin)

Can Neural Stem cells remyelinate?

Pre-Clinical Animal Models: Optic Nerve
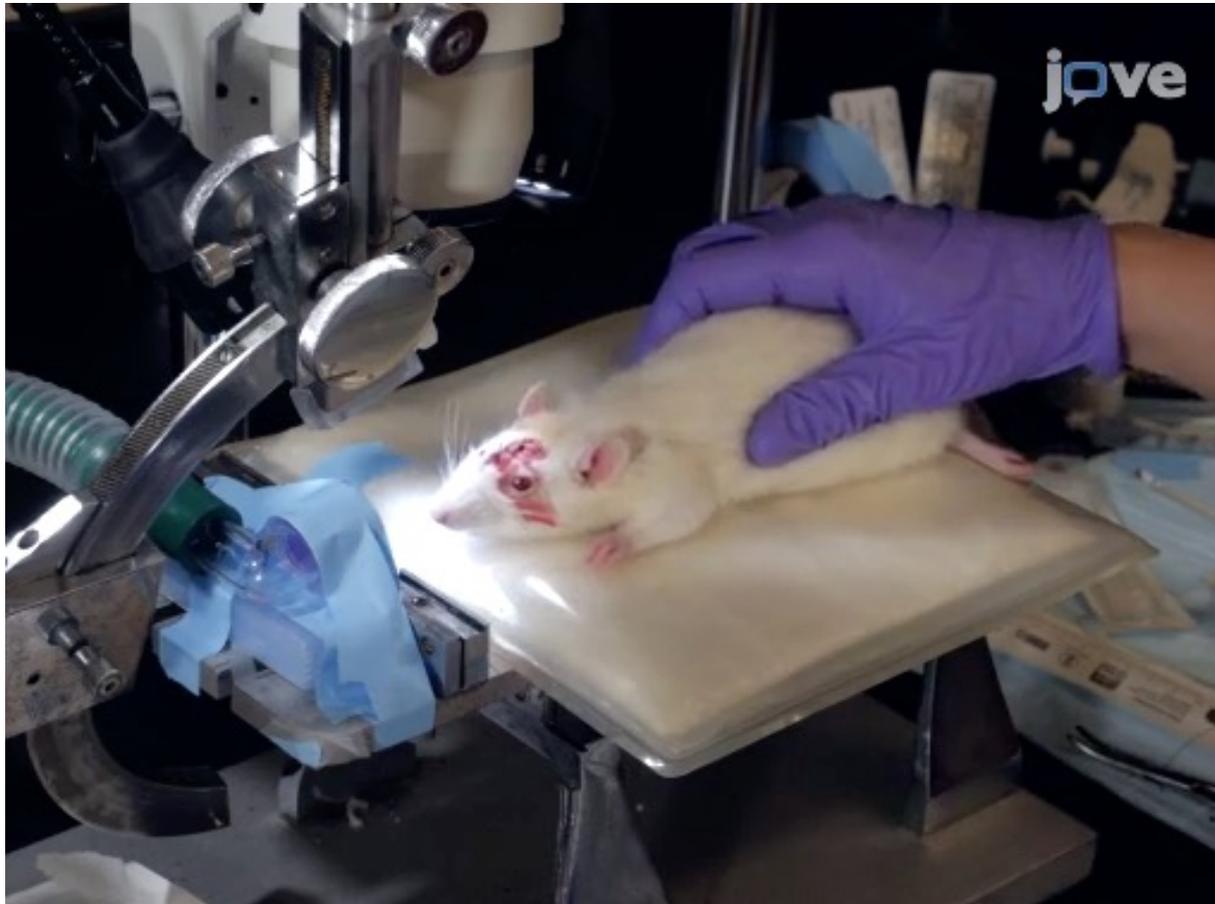
Pre-Clinical Animal Models:
Optic Nerve
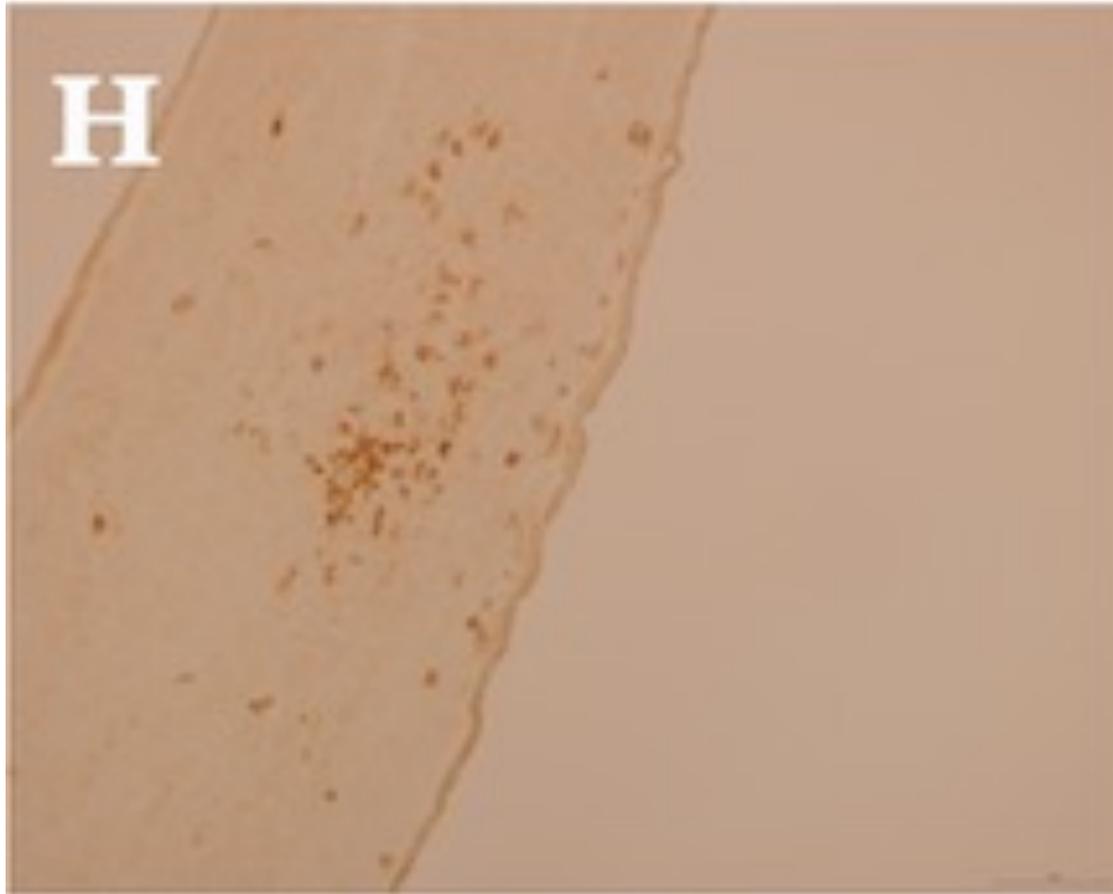
Pre-Clinical Animal Models: Optic Nerve

Stem Cells in Optic Nerve

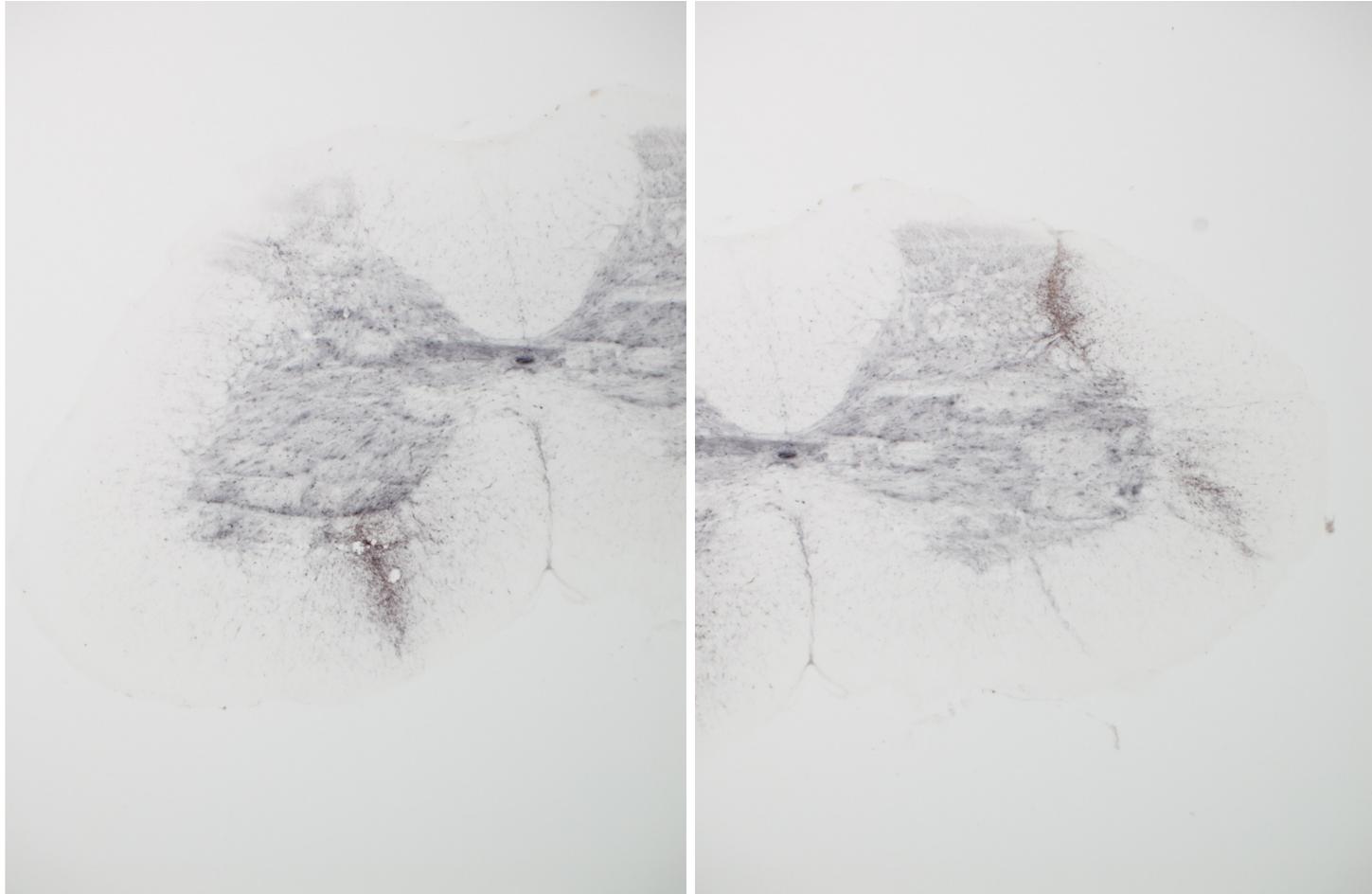# Stem Cells Injections in Spinal Cord

Stem cells in Rat Spinal Cord

Nutrition

Michael Levy, MD, PhD

Associate Professor

Diet Survey/Questionnaire

The DHQIII Demo is designed to showcase the look and feel of the survey to respondents. You can use the DHQIII Demo to complete the survey and generate the Respondent Nutrition Report; however, please note that data is not saved and any loss of connectivity or navigation away from the webpage will result in the loss of data and will require you to begin the survey again. If you wish to access data or obtain the Respondent Nutrition Report after leaving the DHQ III Demo website, we advise you to setup a study on the DHQIII Researcher Website.

Nutrition Survey

Resize font: ➕ | ➖

Please complete the survey below.

Thank you!

Thank you for taking part in this survey. We hope it will shed light on any connections between diet, disease activity and quality of life. This survey is in two parts. Here, we ask questions about you, your disease and your quality of life. When you complete this section, we will email you a link to the diet portion of the survey.

Consent Form

Have you read and signed the consent form on www.nmo.mgh.harvard.edu?	○ Yes
* must provide value	○ No

reset

You must sign the consent form in order to take this survey.

Demographics

First name	[ ]
Email address * must provide value	[ ]
Age * must provide value	[ ]
Gender * must provide value	[▾]

Hypotheses vs. Results

Hypothesis #1: Elevated body mass index (kg/m²) correlates with poor outcomes.

Hypotheses vs. Results

Hypothesis #1: Elevated body mass index (kg/m²) correlates with poor outcomes.

Results: Correct. BMI correlated to best overall general health.

Hypotheses vs. Results

Hypothesis #2: High fiber diet and vitamin D improves quality of life.

Hypotheses vs. Results

Hypothesis #2: High fiber diet and vitamin D improves quality of life.

Results: Only in AQP4 Ab NMOSD.

That's interesting!

Dairy intake – the more dairy, the more pain.

Future Nutrition Studies

- Elimination Studies
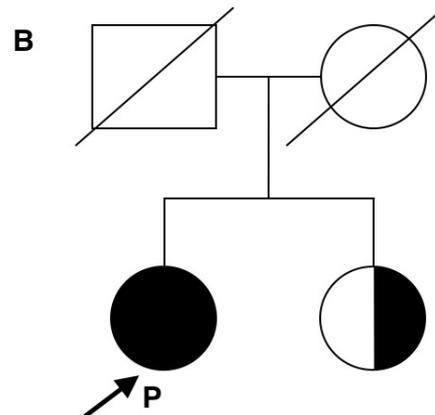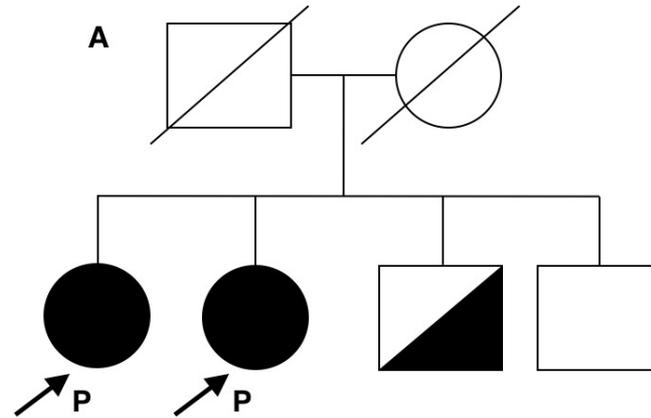 - Dairy
  - Gluten
  - Fat
  - Sugar
  - Salt
  - ...

Genetics of Familial TM

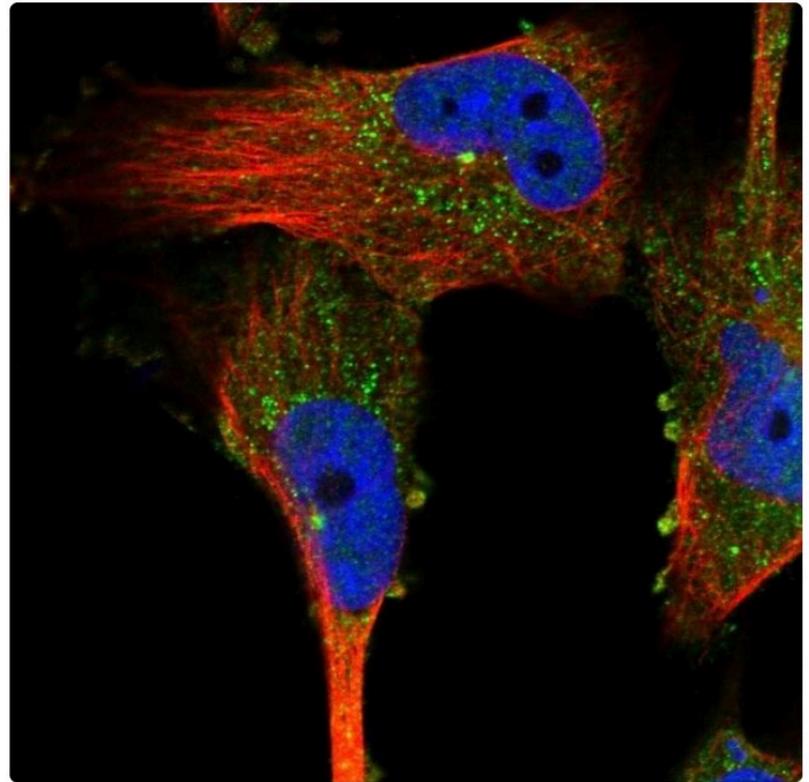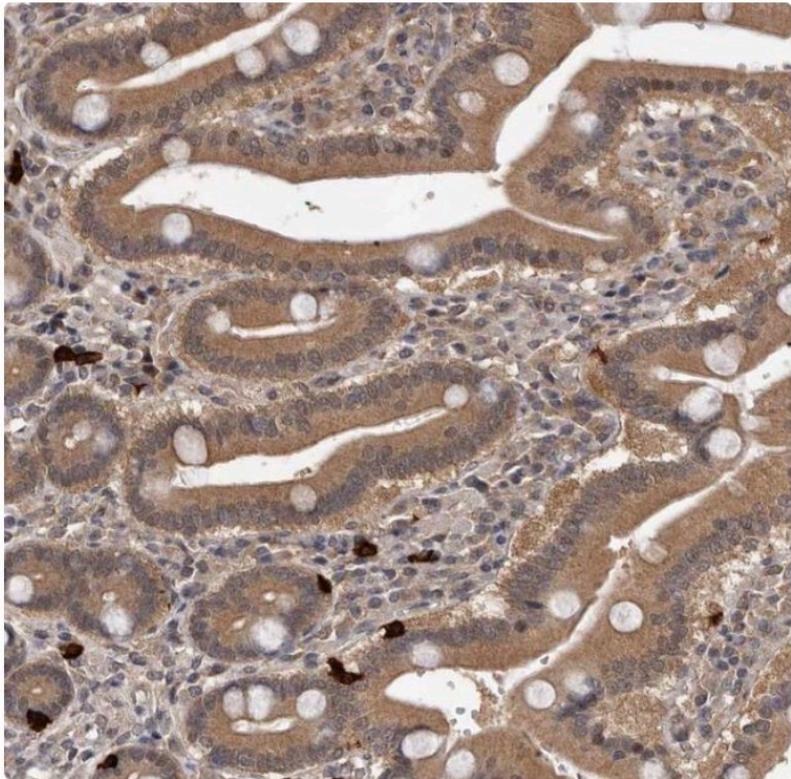**Michael Levy, MD, PhD**

Associate Professor

Disclosures: Dr. Levy receives research support from: National Institutes of Health and has previously received research support from Genzyme, Alexion, Alnylam, and Shire. He also received personal compensation for consultation with Alexion, Acorda, Genentech, Viela Bio, Quest Diagnostics and Genzyme and he serves on the scientific advisory boards for Alexion, Viela Bio, Genentech and Quest Diagnostics.
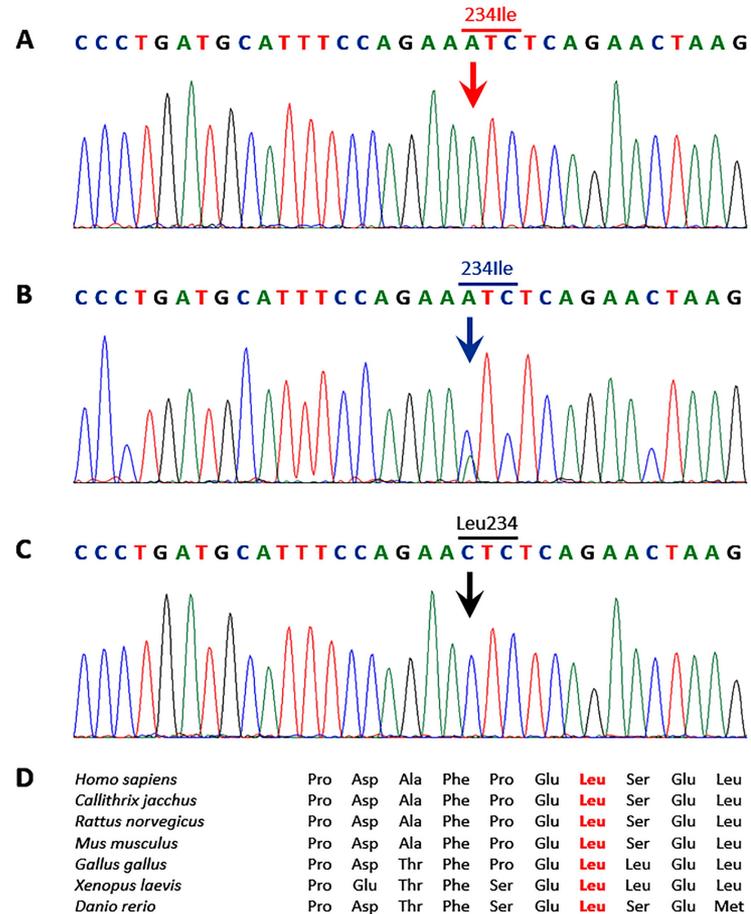
Family pedigrees

VPS37A: Vacuolar Protein Sorting 37A

From http://www.thermofisher.com

Gene Identified: VPS37A

Modeling these mutations...