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MEDICINE

Presented by: Michael Levy, MD, PhD

Research in TM

Johns Hopkins Neurology TM Symposium 2013

Outline: 20 minutes

- Acute transverse myelitis:
  1. Cinryze
- Chronic transverse myelitis:
  2. Stem cell research
  3. Ampyra trial

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Acute Transverse Myelitis: Complement Cascade

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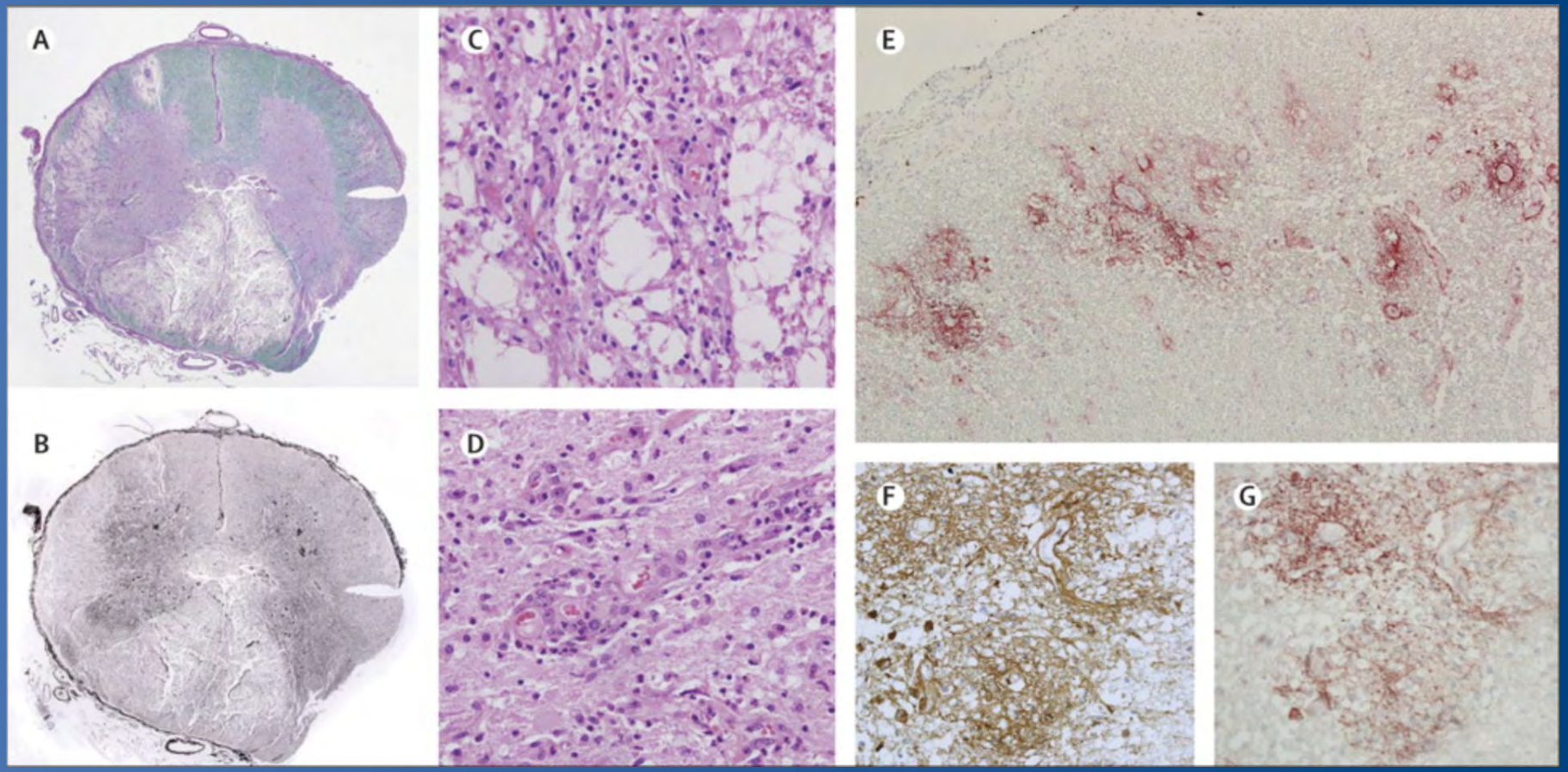
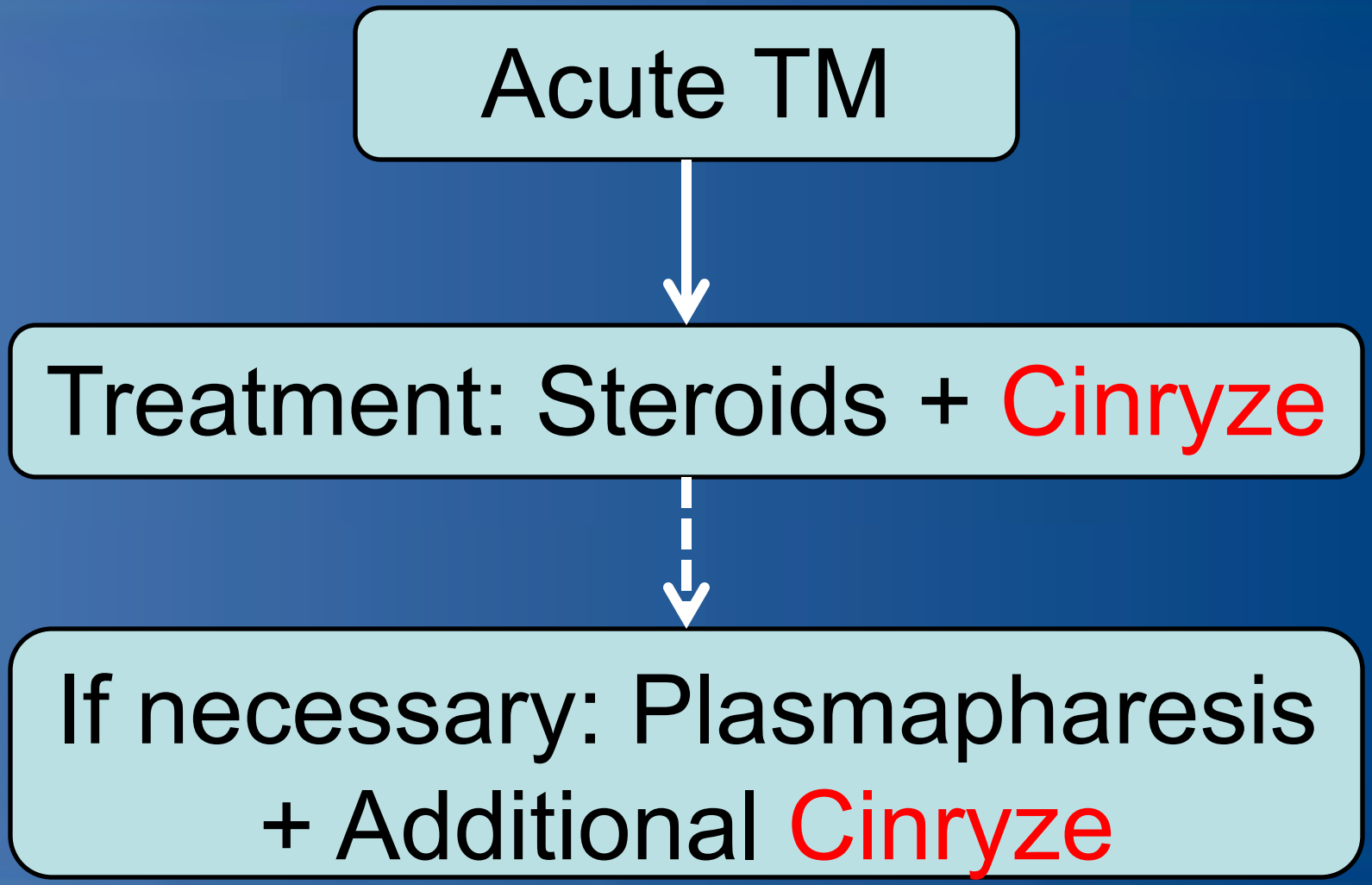
Complement Video

Acute Transverse Myelitis: Pathology



Acute Transverse Myelitis: Cinryze

Acute Transverse Myelitis: Cinryze Trial



Acute Transverse Myelitis: Future applications

- Designed for **ACUTE** myelitis. Once inflammation has resolved, not useful.
- In addition to standard of care.
- Can be used again for relapses.
- Excellent safety profile.

Outline: Next up…

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Chronic Transverse Myelitis: Stem Cells

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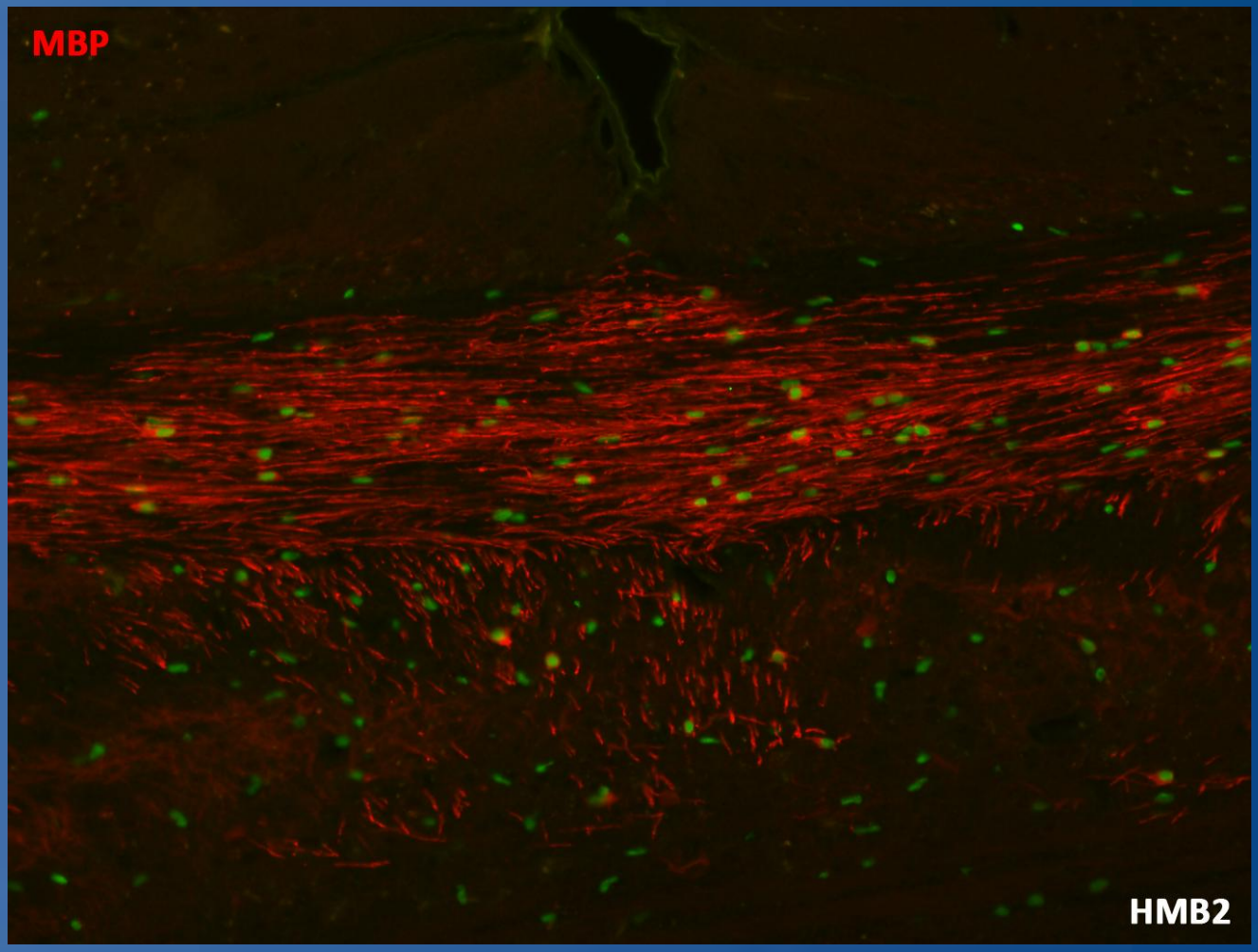
Stem Cells: Introduction

Chronic Transverse Myelitis: Mouse Model



Chronic Transverse Myelitis: Stem Cells Preclinical Studies

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1. Potential to produce myelin
2. Migration – where do the stem cells go?
3. Survival
4. **Restoration of Function**

Chronic Transverse Myelitis: Stem Cells Human Study

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- Human trial of TM
- NIH Clinical Center (Bethesda, MD)
- Collaboration: JHH, NIH, NeuralStem
- 6 patients
  - 3 ASIA A
  - 3 ASIA C
- Direct surgical transplantation

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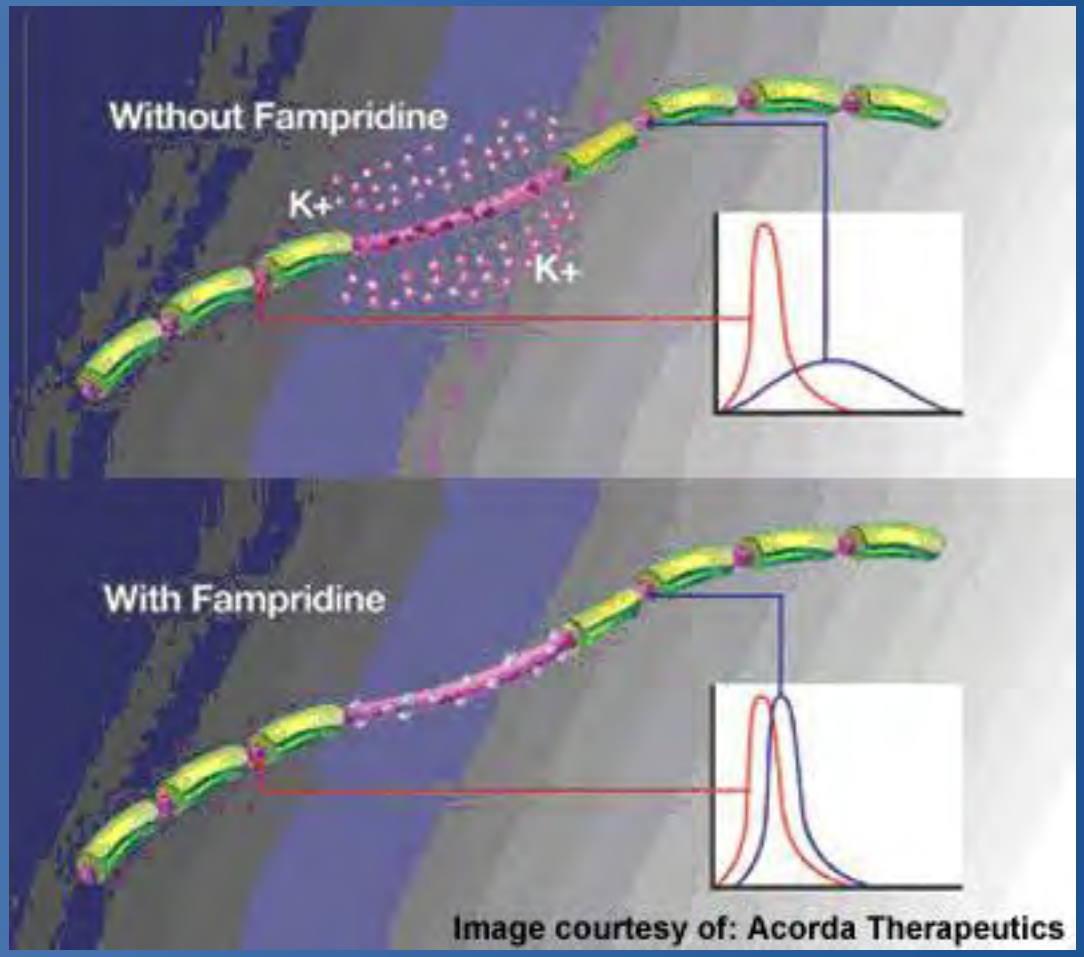
Just one lesion

TM patients have only
1 lesion to overcome

Keep the signal from dissipating



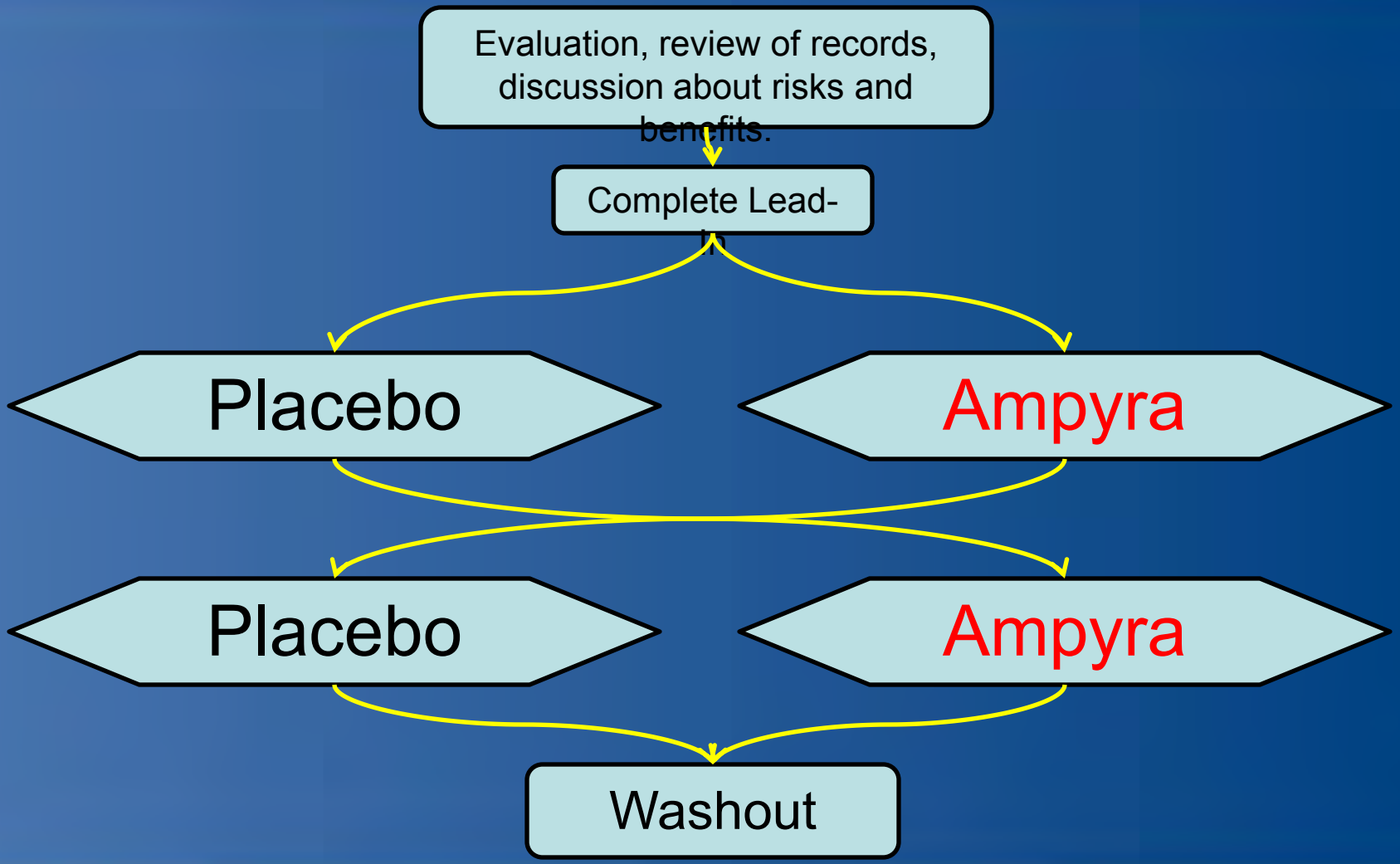
Potassium ions dissipate the signal.

Ampyra: block the potassium channels

Helps MS patients walk

Ampyra patient videos

Ampyra in TM trial design



Ampyra in TM: recruiting

- Currently, 6 enrolled. Seeking 19 additional patients.
- Requires Hopkins visit every 2 weeks.
- Total trial period = 22 weeks.

Future of TM Research

- Excellent “proof of concept” model
- Remyelinating medication: rIgM22, stimulators of own stem cells
- Stem cell transplantation