The Patient Perspective: Expedited Pathways to Market

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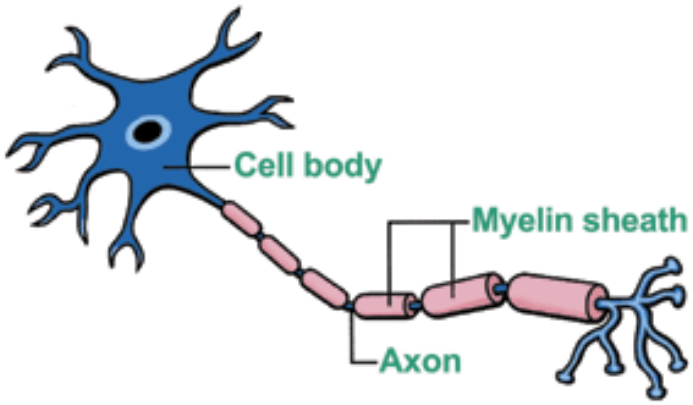
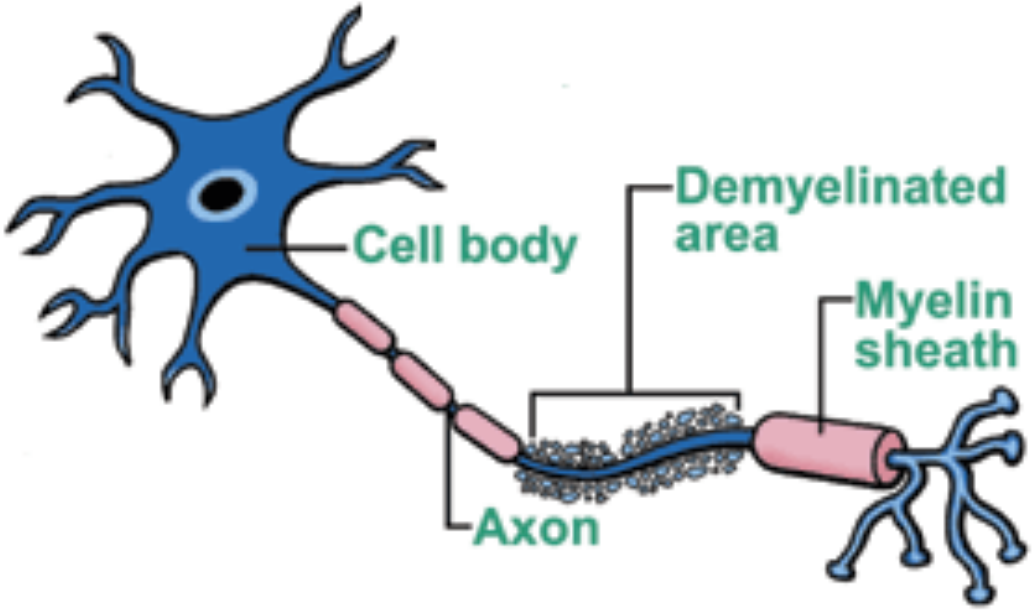
What We'll Talk About

- MS overview
- Development of MS Therapies
- Risk Tolerance for Treatment
- Potential Policies to Expedite Availability of Therapies

MS Interrupts the Flow of Information Between the Brain and the Body



Functional nerve cell



Nerve cell with MS damage

Common Symptoms of MS

- Sensory problems
- Vision problems
- Vertigo & dizziness
- Spasticity
- Fatigue
- Tremor
- Gait & balance problems
- Cognitive problems
- Sexual dysfunction
- Speech problems
- Weakness & paralysis
- Bladder & bowel problems
- Pain

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Spectrum of Functioning Varies

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Current Treatments for MS

- 12 disease-modifying agents, including injected, infused and orals, to reduce frequency & severity of attacks and slow disease activity and damage – largely for relapsing forms
- Symptomatic treatments (walking speed, fatigue, spasticity, bladder and bowel symptoms, pain, emotional changes, etc.)

There is no therapy that can stop or treat MS progression

Approval for Neurological Conditions Often Take Longer

Tufts 2014 Impact Study:

Average clinical development time for approved-CNS drugs was 12.8 months (18%) longer than non-CNS drugs.

	CNS Drugs	Non-CNS Drugs
Approval Success Rate	***6.2%***	***13.3%***
Avg. Approval Time	19.3 months	14.7 months
Priority Review Rating	***~1 out of 6 drugs***	***~1 out of 2 drugs***

Accelerated Approval Pathway could help shorten this timeframe

Tufts Center for the Study of Drug Development . Impact Study. Volume 16, Number 6 http://csdd.tufts.edu/files/uploads/Summary__NovDecImpactIR.pdf

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Despite Risks, Patients Have Stated Access Is a Priority

Anecdotal statements from patients:

- "When will the next therapy be available for me?"
- "I want choice to make decisions about my treatment and risk tolerance with my physician"

Case study

Tysabri: Taken off the market four months after approval. FDA later put it back on the market after a scientific assessment and a better understanding of MS patients' risk tolerance.

PFDD Could Help Inform FDA About Breadth of Accelerated Approvals

- Different diseases have differing perspectives on risk.
- Expanding upon FDA's current Patient-Focused Drug Development initiative could help inform FDA's review process.
  - Next step: Provide guidance on how to systematically solicit incorporate patient perspectives into drug review.