The Patient Perspective: Expedited Pathways to Market

January 20, 2015
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

Diagram showing a functional nerve cell and a 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
Spectrum of Functioning Varies
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.

<table>
<thead>
<tr>
<th></th>
<th>CNS Drugs</th>
<th>Non-CNS Drugs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Approval Success Rate</td>
<td>6.2%</td>
<td>13.3%</td>
</tr>
<tr>
<td>Avg. Approval Time</td>
<td>19.3 months</td>
<td>14.7 months</td>
</tr>
<tr>
<td>Priority Review Rating</td>
<td>~1 out of 6 drugs</td>
<td>~1 out of 2 drugs</td>
</tr>
</tbody>
</table>

Accelerated Approval Pathway could help shorten this timeframe

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.