Advancing the Science of Patient Input in Medical Product R&D: Towards a Research Agenda

Session 1: Understanding Patient Experience with Disease or Medical Condition

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Listening to Patients and Caregivers

Case Study:
Sharing a sampling of perspectives shared at Duchenne Patient Focused Compass Meeting in March 2018 convened by Parent Project Muscular Dystrophy
PDUFA V Patient-focused meetings provided such important perspectives for each disease area we addressed

<table>
<thead>
<tr>
<th>Fiscal Year 2013</th>
<th>Fiscal Year 2014</th>
<th>Fiscal Year 2015</th>
<th>Fiscal Year 2016</th>
<th>Fiscal Year 2017</th>
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<tbody>
<tr>
<td>• Chronic Fatigue Syndrome/Myalgic Encephalo-myelitis</td>
<td>• Sickle Cell Disease</td>
<td>• Female Sexual Dysfunction</td>
<td>• Non-Tuberculous Mycobacterial Lung infections</td>
<td>• Sarcopenia</td>
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<td>• HIV</td>
<td>• Fibromyalgia</td>
<td>• Breast Cancer</td>
<td>• Autism</td>
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<td>• Lung Cancer</td>
<td>• Pulmonary Arterial Hypertension</td>
<td>• Chagas Disease</td>
<td>• Alopecia Areata</td>
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<td>• Narcolepsy</td>
<td>• Inborn Errors of Metabolism</td>
<td>• Functional Gastrointestinal Disorders</td>
<td>• Neuropathic pain associated with peripheral neuropathy</td>
<td>• Hereditary Angioedema</td>
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<td></td>
<td>• Hemophilia A, B, and other Heritable Bleeding Disorders</td>
<td>• Parkinson’s Disease and Huntington’s Disease</td>
<td>• Patients who have received an organ transplant</td>
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<td></td>
<td>• Idiopathic Pulmonary Fibrosis</td>
<td>• Alpha-1 Antitrypsin Deficiency</td>
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Further integrating patient perspective into medical product development and decision making

Need to build in the patient’s perspective starting in the translational phase

Translational
- How do we ensure that we get input representative of the whole disease population?
- What symptom or functions matter most to people with this disease?
- How to best measure? (endpoints, frequency, mode of reporting, etc.)

Clinical Studies
- Do endpoints planned for the trial include the ones that matter most to patients?
- Does the protocol facilitate (or discourage) enrollment or continued participation?
- Do informed consent and other processes within the trial reflect the needs and preferences of people with that disease?

Pre-market review
- How to better integrate patient reported outcome data or elicited patient preferences into BR assessments?

Post-market
- How to best communicate the information to patients and prescribers?

• How to convey info that helps facilitate patients’ and clinicians’ informed decision making?
• How to convey uncertainty to inform and support clinical decision-making?
Some Key Topics to be Addressed in the PDUFA VI Guidance

1. Collecting comprehensive patient community input on burden of disease and current therapy
   – How to engage with patients to collect meaningful patient input?
   – What methodological considerations to address?

2. Development of holistic set of impacts (e.g., burden of disease and burden of treatment) most important to patients
   – How to develop a set of impacts of the disease and treatment?
   – How to identify impacts that are most important to patients?

3. Identifying and developing good measures for the identified set of impacts that can then be used in clinical trials.
   – How to best measure impacts (e.g., endpoints, frequency..) in a meaningful way?
   – How to identify measure(s) that matter most to patients?

4. Incorporating measures (COAs) into endpoints considered significantly robust for regulatory decision making
   – Topics including technologies to support collection through analysis of the data
When would the methods addressed in these guidances be applicable?

**Guidance 1**  Glossary of Terminology

**Guidance 1**  

**Guidance 2**  

**Guidance 3**  

**Guidance 4**

Understanding Patient Experience—Sampling of Questions of Interest to FDA

• What disease impacts matter most to patients?
  – How does that vary by socio-demographic factors? By subgroup group of patients (e.g., a pediatric subpopulation, geriatric subpopulation, subpopulation with major co-morbidities), by culture? Severity of disease? Other life circumstances?

• How do attitudes toward or tolerance of potential drug risks or therapy side effects (“preference” considerations) vary by patient subgroup?
  – By subgroup group of patients (e.g., a pediatric subpopulation, geriatric subpopulation, subpopulation with major co-morbidities), by culture? Severity of disease? Other life circumstances?

• How well do the most commonly studied endpoints in clinical trials for a given disease area align with outcomes or aspects of disease that matter most to patients? How does that vary by subgroup?
• Are currently conducted clinical trials in a given disease area excluding patients who want to be enrolled? *If so, why and how might it be addressed?*

• Are currently or commonly used clinical trial protocols intolerable or otherwise unworkable for some patients who are otherwise eligible to participate?  
  – *Why? What might be done to address that?*

• What measures can be taken to increase the likelihood of patient enrollment in a study and increase the likelihood of participant retention in a study in a given disease area?  
  – *Are there further suggested considerations by patient subgroup?*

• What if any challenges do patients face in trying to adhere to their prescribed drug regimen?  
  – *How does this vary by patient subgroup? What might be considered to address this?*