

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



Fiscal Year 2013	Fiscal Year 2014	Fiscal Year 2015	Fiscal Year 2016	Fiscal Year 2017
<ul style="list-style-type: none"> • Chronic Fatigue Syndrome/ Myalgic Encephalo-myelitis • HIV • Lung Cancer • Narcolepsy 	<ul style="list-style-type: none"> • Sickle Cell Disease • Fibromyalgia • Pulmonary Arterial Hypertension • Inborn Errors of Metabolism • Hemophilia A, B, and other Heritable Bleeding Disorders • Idiopathic Pulmonary Fibrosis 	<ul style="list-style-type: none"> • Female Sexual Dysfunction • Breast Cancer • Chagas Disease • Functional Gastro-intestinal Disorders • Parkinson’s Disease and Huntington’s Disease • Alpha-1 Antitrypsin Deficiency 	<ul style="list-style-type: none"> • Non-Tuberculous Mycobacterial Lung infections • Psoriasis • Neuropathic pain associated with peripheral neuropathy • Patients who have received an organ transplant 	<ul style="list-style-type: none"> • Sarcopenia • Autism • Alopecia Areata • Hereditary Angioedema

Further integrating patient perspective into medical product development and decision making

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

What impacts (burden of disease and burden of treatment) matter most to patients and how to measure them?

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.)*

What aspects of clinical trials can be better tailored to meet the patients who (might) participate in the trial?

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?*

How to better integrate patient reported outcome data or elicited patient preferences into BR assessments?

Pre-market review

- *How to utilize elicited patient preference studies?*
- *How to factor in key uncertainties?*
- *How could individual differences in patient experience (or preference) of benefit versus harm be considered?*

How to best communicate the information to patients and prescribers?

Post-market

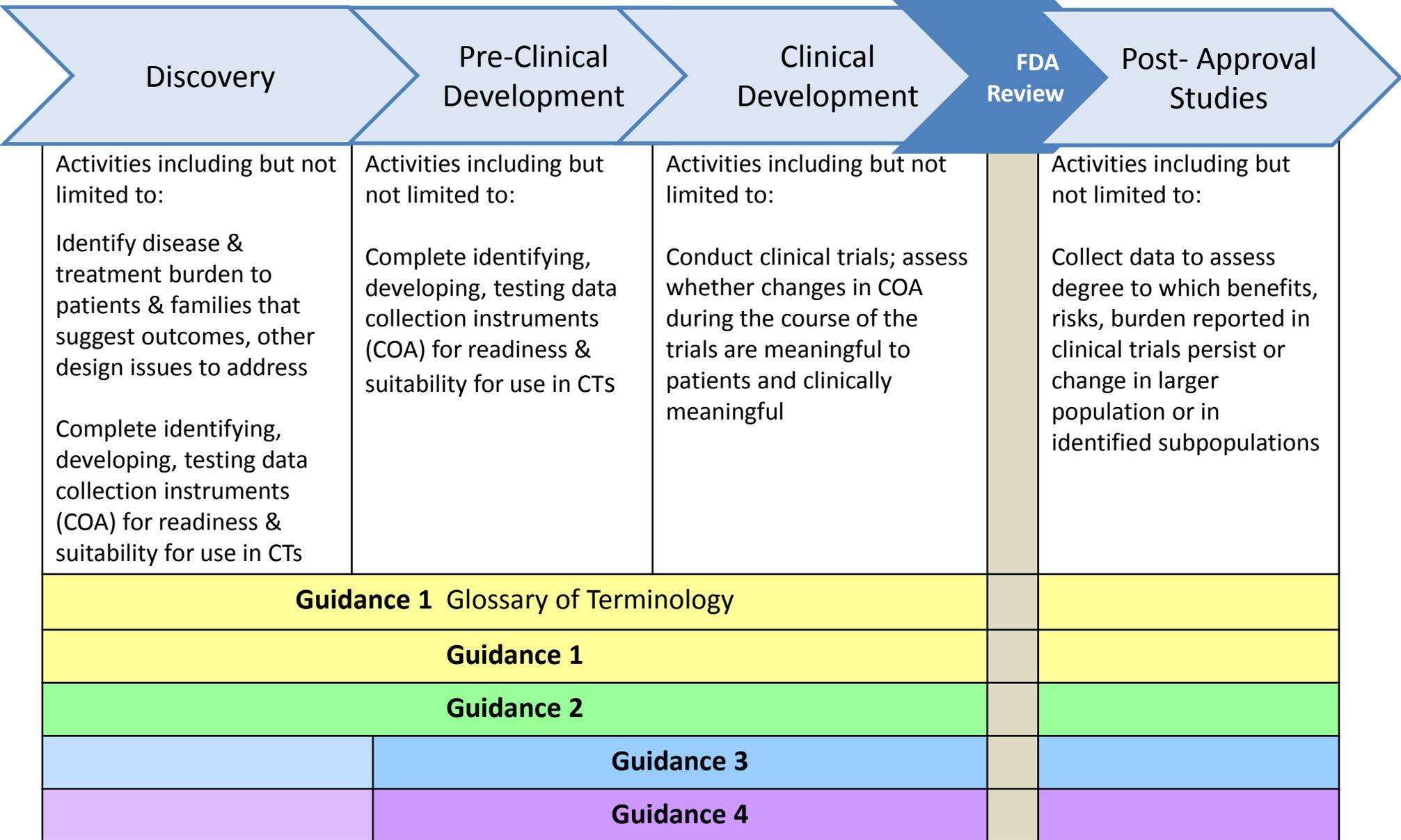
- *How to convey info that helps facilitate patients' and clinicians' informed decision making?*
- *How to convey uncertainty to inform and support clinical decision-making?*

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?



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?

Understanding Patient Experience—Sampling of Questions of Interest to FDA (cont.)



- 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?*



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