U.S. FOOD & DRUG ADMINISTRATION

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

Theresa Mullin, PhD
Associate Director for Strategic Initiatives
FDA Center for Drug Evaluation and Research

NASEM Workshop
May 9, 2018

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

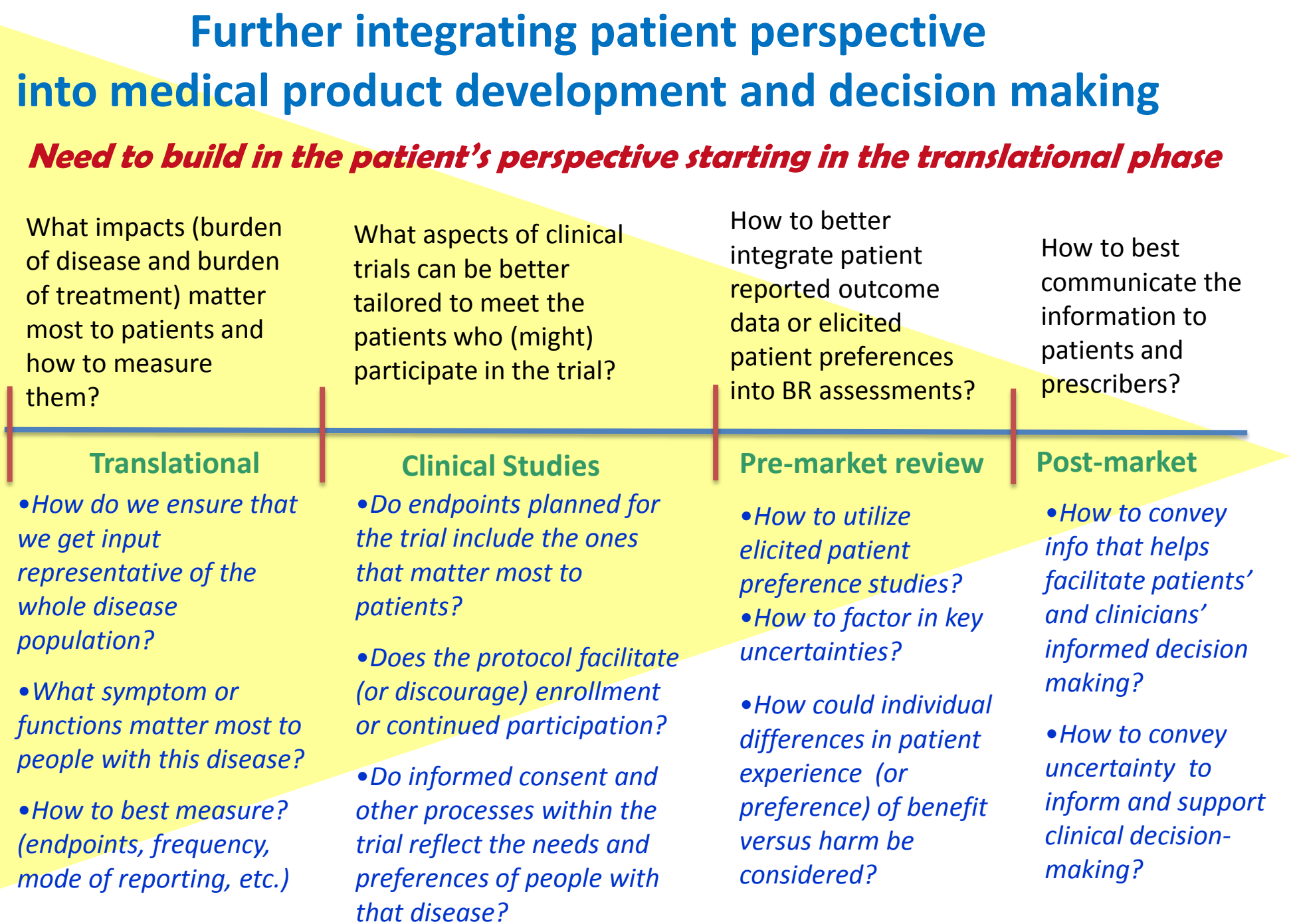
PDUFA V Patient-focused meetings provided such important perspectives for each disease area we addressed

Fiscal Year 2013	Fiscal Year 2014	Fiscal Year 2015	Fiscal Year 2016	Fiscal Year 2017
• Chronic Fatigue Syndrome/ Myalgic Encephalo-myelitis • HIV • Lung Cancer • Narcolepsy	• Sickle Cell Disease • Fibromyalgia • Pulmonary Arterial Hypertension • Inborn Errors of Metabolism • Hemophilia A, B, and other Heritable Bleeding Disorders • Idiopathic Pulmonary Fibrosis	• Female Sexual Dysfunction • Breast Cancer • Chagas Disease • Functional Gastro-intestinal Disorders • Parkinson's Disease and Huntington's Disease • Alpha-1 Antitrypsin Deficiency	• Non-Tuberculous Mycobacterial Lung infections • Psoriasis • Neuropathic pain associated with peripheral neuropathy • Patients who have received an organ transplant	• Sarcopenia • Autism • Alopecia Areata • Hereditary Angioedema

3

Further integrating patient perspective into medical product development and decision making

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



Some Key Topics to be Addressed in the PDUFA VI Guidance

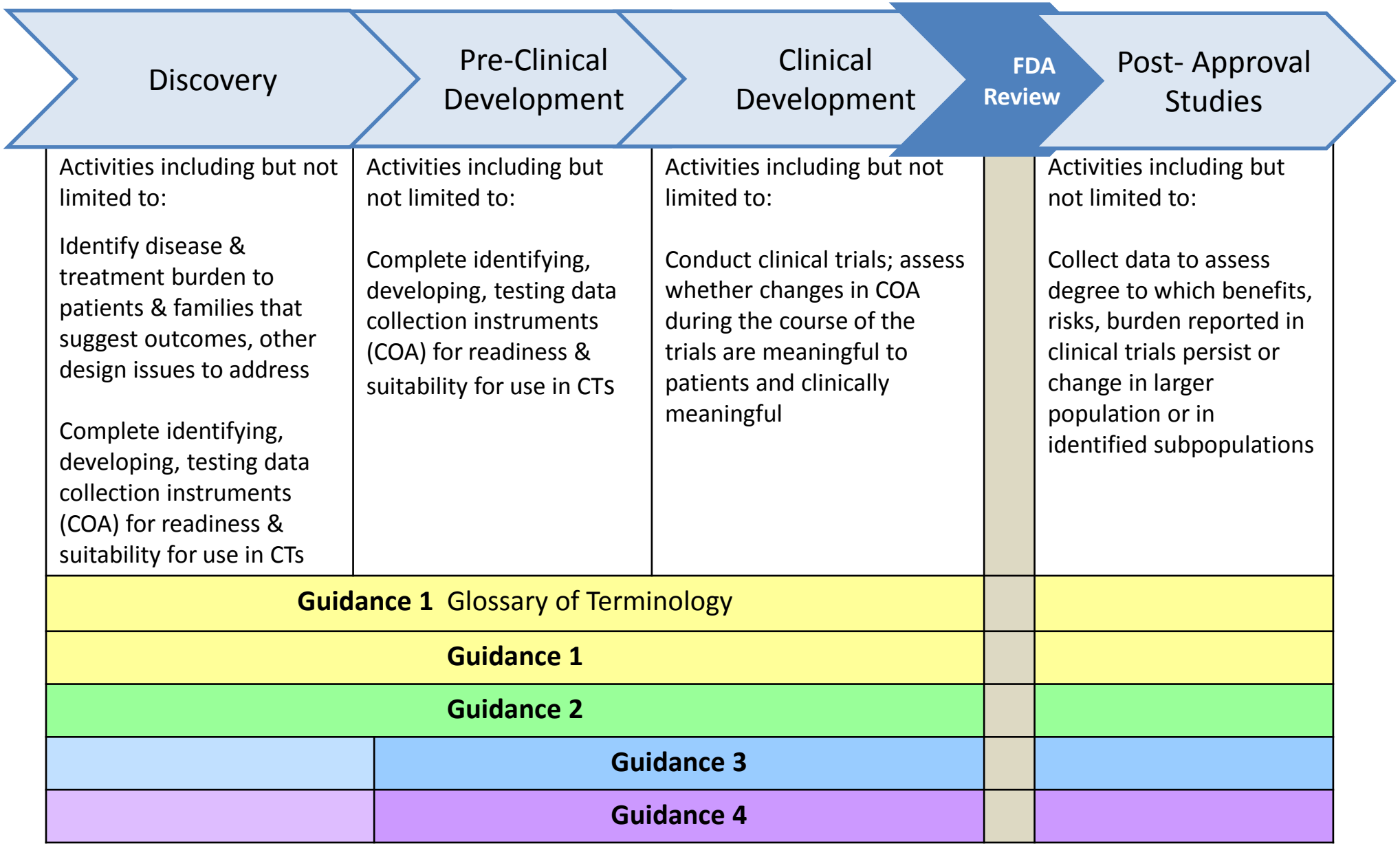
1. Collecting comprehensive patient community input on burden of disease and current therapy
   - How to engage with patients to collect meaningful patient input?
   - What methodological considerations to address ?

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?



https://www.fda.gov/downloads/forindustry/userfees/prescriptiondruguserfee/ucm563618.pdf

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?*


FDA
U.S. FOOD & DRUG
ADMINISTRATION