Patient-Focused Drug Development and Cancer Trials

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Partners in Progress- 2017

OCE Patient-Focused Drug Development (PFDD) Program

The Oncology Center of Excellence PFDD program fosters collaboration between FDA Centers and external stakeholders involved in patient outcomes research in cancer populations.

- Engage with patients and advocacy groups,
- Research the measurement of the patient experience,
- Develop science-based recommendations for regulatory policy.

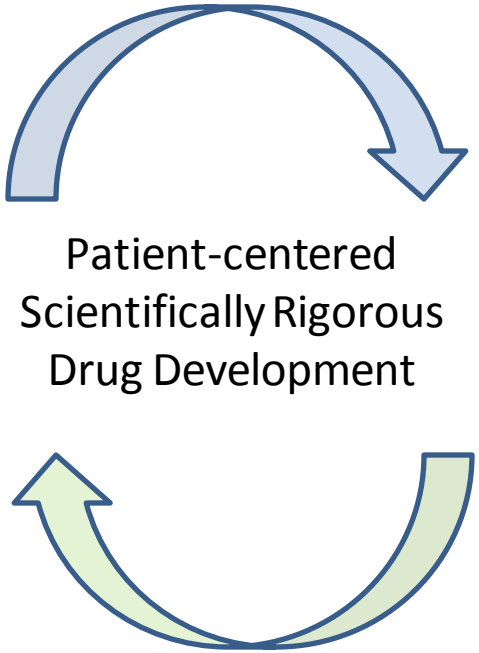
The overarching goal is to identify rigorous methods to assess the patient experience that will complement existing survival and tumor information to better inform a cancer therapy's effect on the patient.

https://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/OCE/ucm544143.htm

Patient Engagement as a Dialogue

Patients

- Experts in how they experience their disease
- Identify what matters most to patients
- Identify areas to make clinical trials more patient-friendly

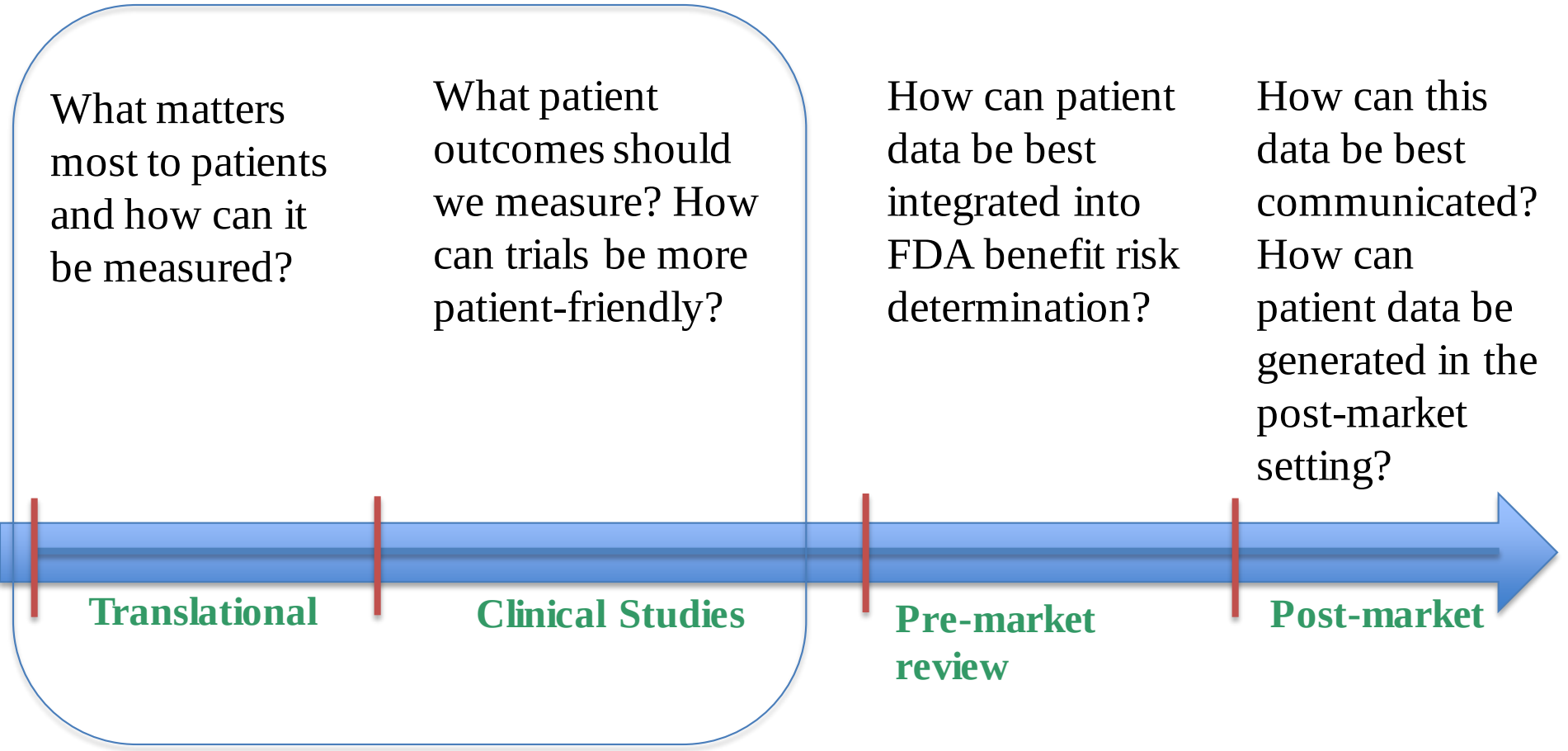


Patient-centered
Scientifically Rigorous
Drug Development

Clinicians/ Trialists/ Health Policy Leaders

- Experts in clinical trial design and conduct
- Medical expertise
- Assess feasibility of trial modifications and outcome measures

Further integrating patient perspective into drug development and decision making



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We are getting there…

How can trials be more patient friendly?

- Exploring broadening eligibility criteria
- Considering patient-friendly language and simplified informed consent
- Investigating the role of pragmatic / practical trials

What matters most to patients?

- FDA Patient Focused Drug Development Meetings- Lung and Breast Cancer
- Core Outcome Sets
- Development of PRO tools- Qualitative Work

Patient would like a cure… but they would also like to know
"How will I feel and function while taking my cancer therapy?"

What outcomes can we measure in clinical trials?

COA Glossary & Abbreviations

Clinical Outcome Assessment (COA)

Assessment of a clinical outcome made through report by a clinician, a patient, a non-clinician observer or through a performance-based assessment

Patient Reported Outcome (PRO)

A measurement based on a report that comes directly from the patient about the status of a patient's health condition without amendment or interpretation of the patient's response by a clinician or anyone else.

Ex. Numeric rating scale of pain intensity

1

Clinician Reported Outcome (ClinRO)

A measurement based on a report that comes from trained health-care professional after observation of a patient's health condition.

Ex. Psoriasis Area and Severity Index

2

Observer Reported Outcome (ObsRO)

A measurement based on a report of observable signs, events or behaviors related to a patient's health condition by someone other than the patient or a health professional.

Ex. Observer-completed log of seizure episodes

3

Performance Outcome (PerfO)

A measurement based on a task(s) performed by a patient according to instructions that is administered by a health care profession.

Ex. 6-Minute Walk Test

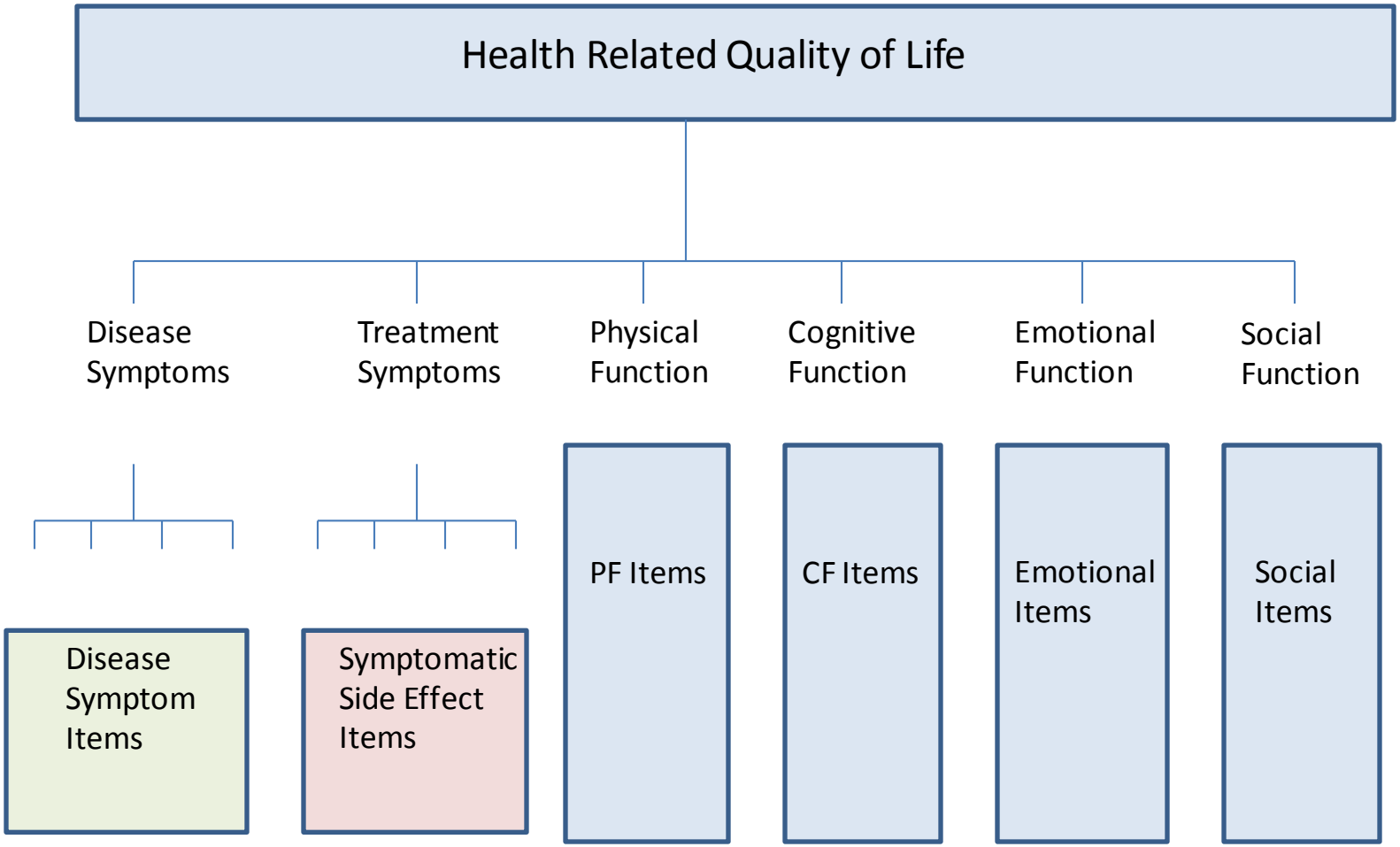
4

Slide courtesy of Nikunj Patel, FDA COA Staff

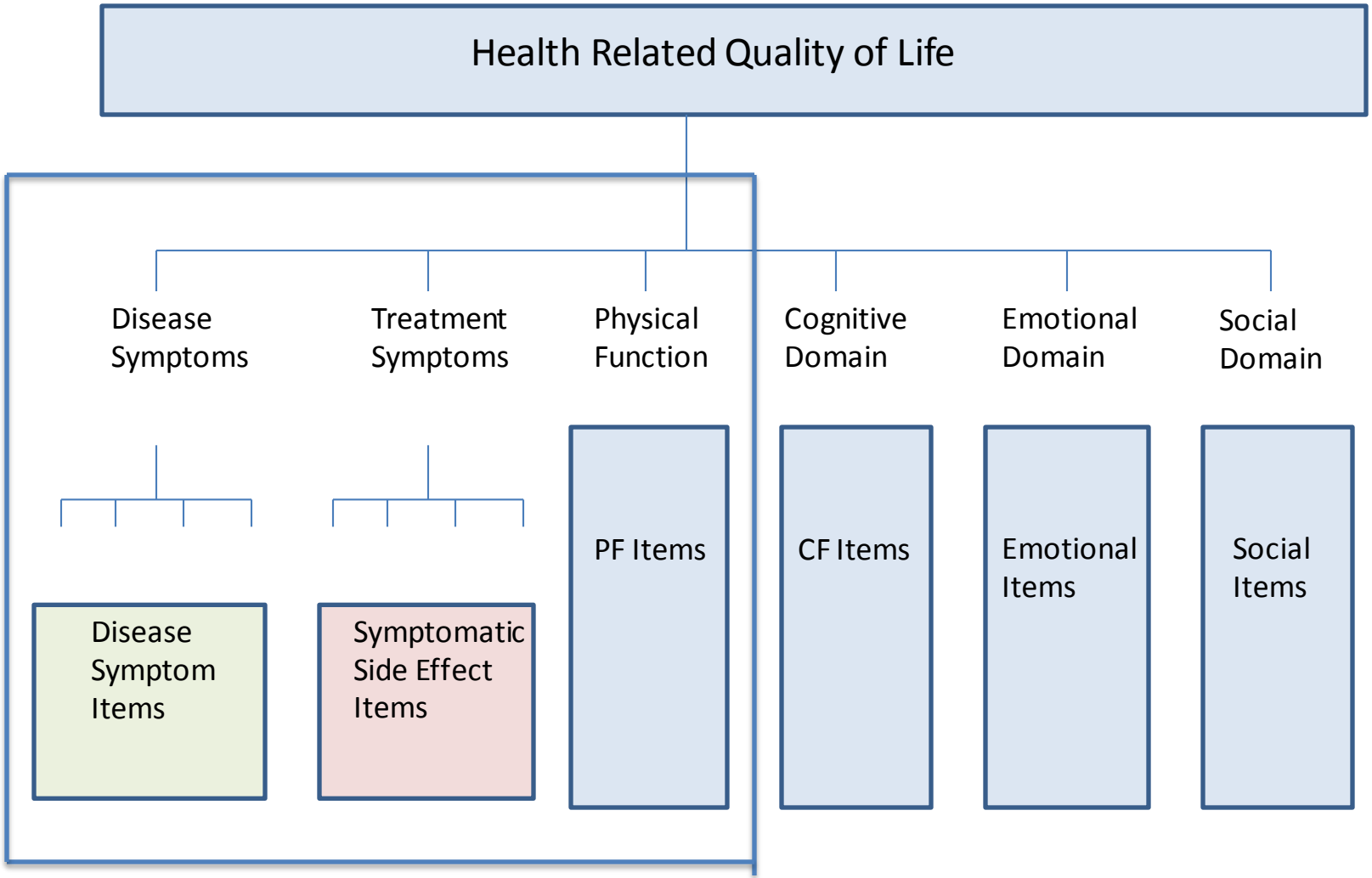
Traditional ("Regular") FDA Approval

- Regular approval requires
  - Substantial evidence of Safety and Efficacy based on prolongation of life, **a better life** or an established surrogate for either of the above
- How can we measure a "better life"?

PRO to measure symptoms and function-> Informing Health Related Quality of Life



Symptoms (and to some degree level of physical function) can be quite different from trial to trial- Need flexibility to adapt to different trial contexts



What PRO Questions Should be Asked? What is the Goal?

- Comprehensive evaluation of the patient experience most affected by the therapy
- Maximize the relevance of individual questions
- Minimize the overall burden and duplication[1]

[1] "Focusing on core patient-reported outcomes in cancer clinical trials. *Clin Can Res* 22.7 (2016): 1553-1558.

Further integrating patient perspective into drug development and decision making

What matters most to patients and how can it be measured?

What patient outcomes should we measure? How can trials be more patient-friendly?

How can patient data be best integrated into FDA benefit risk determination?

How can this data be best communicated?

Translational

Clinical Studies

Pre-market review

Post-market

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Areas of Active Investigation…

Pre-market review

Incorporating patient data in FDA risk:benefit

- How to analyze and interpret PRO and COA data?
- How to analyze and interpret wearable device data?
- How to incorporate patient preference data?
- How to communicate results in a meaningful way

Post-market

Exploring structured and unstructured RWD

- How to take advantage of Real world data (RWD)?
- How to analyze and interpret PRO measures from clinical practice?

In Closing…

- FDA supports advancing clinical outcome data!
  - The science of how to best achieve this is underway…
- FDA has dedicated efforts across Centers (including the new Oncology Center of Excellence) to advance patient focused drug development science
- There are many patients and advocacy groups-
  - Resources will need to be taken into account


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