Oncology Center of Excellence
Patient-Focused Drug Development Update

Paul G Kluetz, MD
Associate Director of Patient Outcomes
Oncology Center of Excellence
U.S. Food and Drug Administration

Partners in Progress- 2018

Patient-Focused Drug Development (PFDD) Program

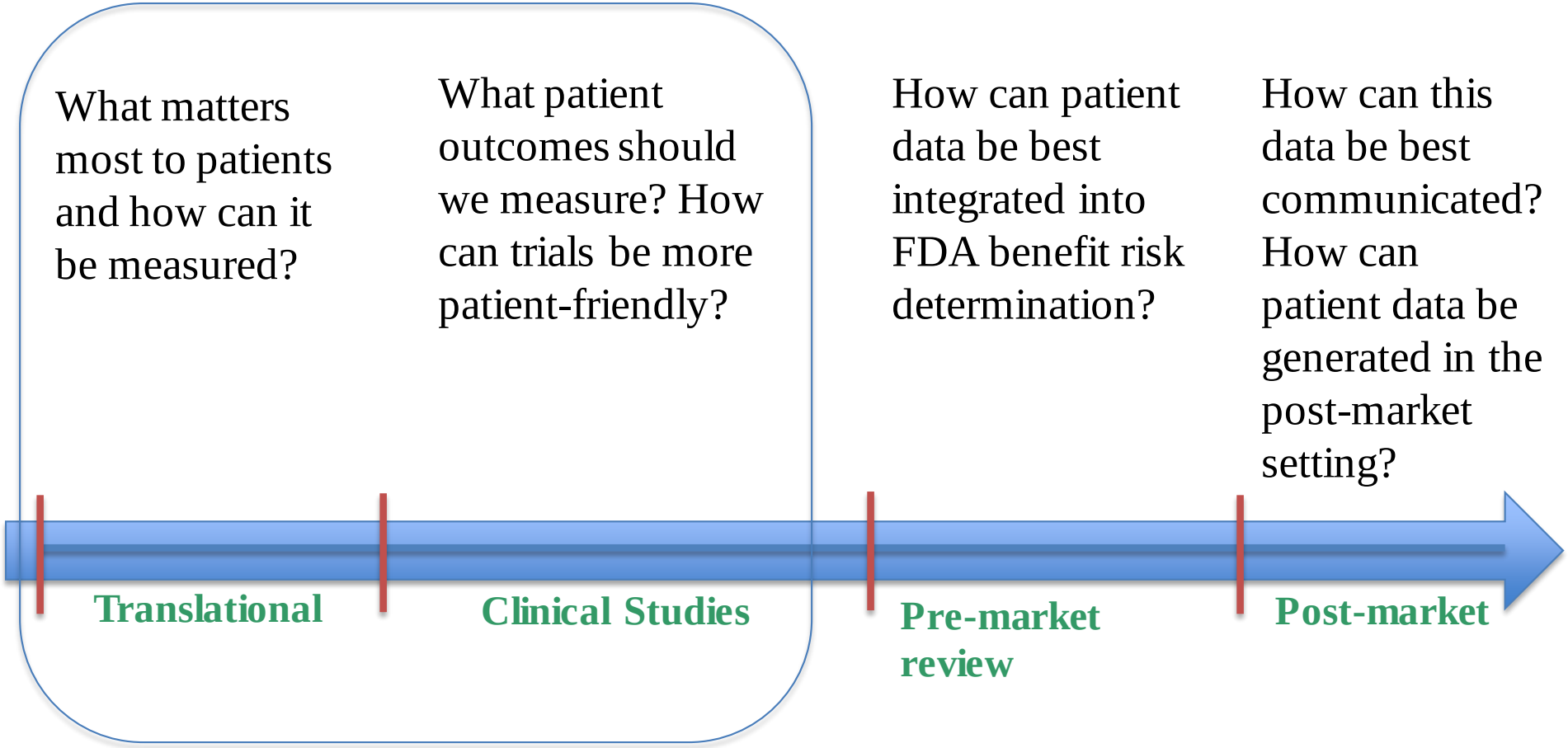
Fosters collaboration between FDA Centers and external stakeholders involved in patient outcomes research in cancer populations.

- 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

Further integrating patient perspective into drug development and decision making



Adapted slide by permission from Theresa Mullin, Office of Strategic Programs, FDA

Efforts ongoing across the Oncology Center of Excellence

How can trials be more patient friendly?

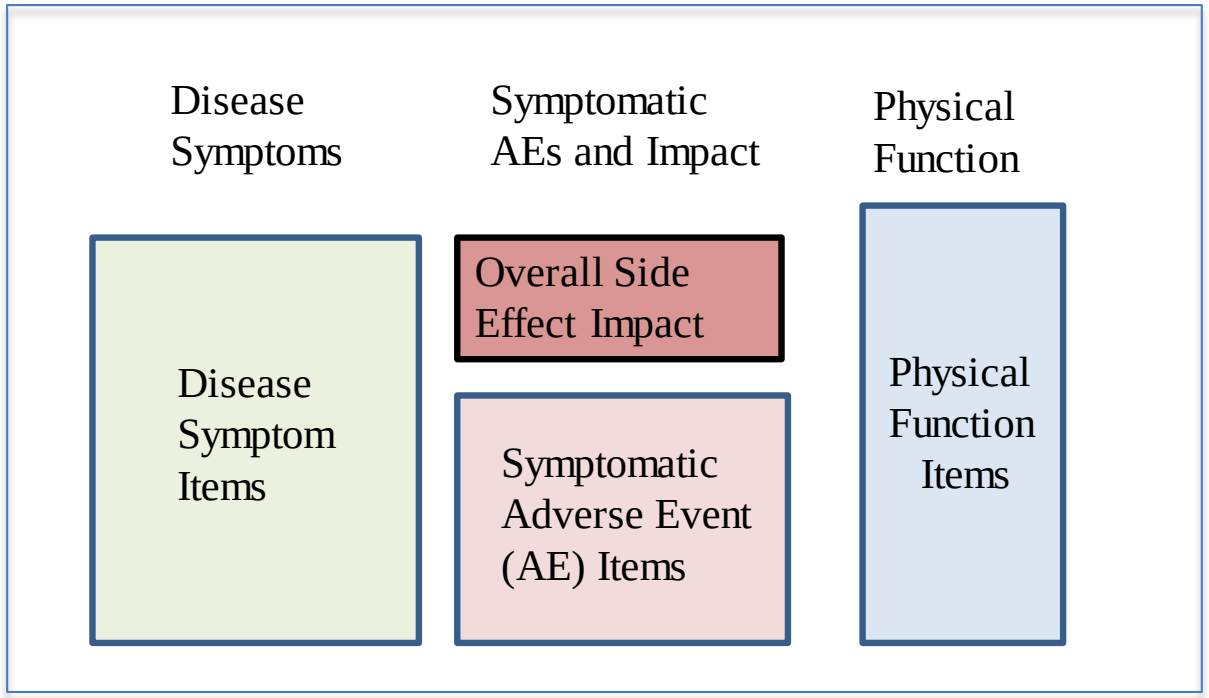
- Exploring broadening eligibility criteria
- Judicious use of placebo-controlled trial designs
- Considering patient-friendly language and simplified informed consent
- Investigating the role of pragmatic / practical trials

What matters to patients?

- FDA Patient Focused Drug Development Meetings- Lung and Breast Cancer

How will patients feel and function while taking cancer therapy?

Symptom and Functional Impacts can Form a Core Set of Important Clinical Outcomes

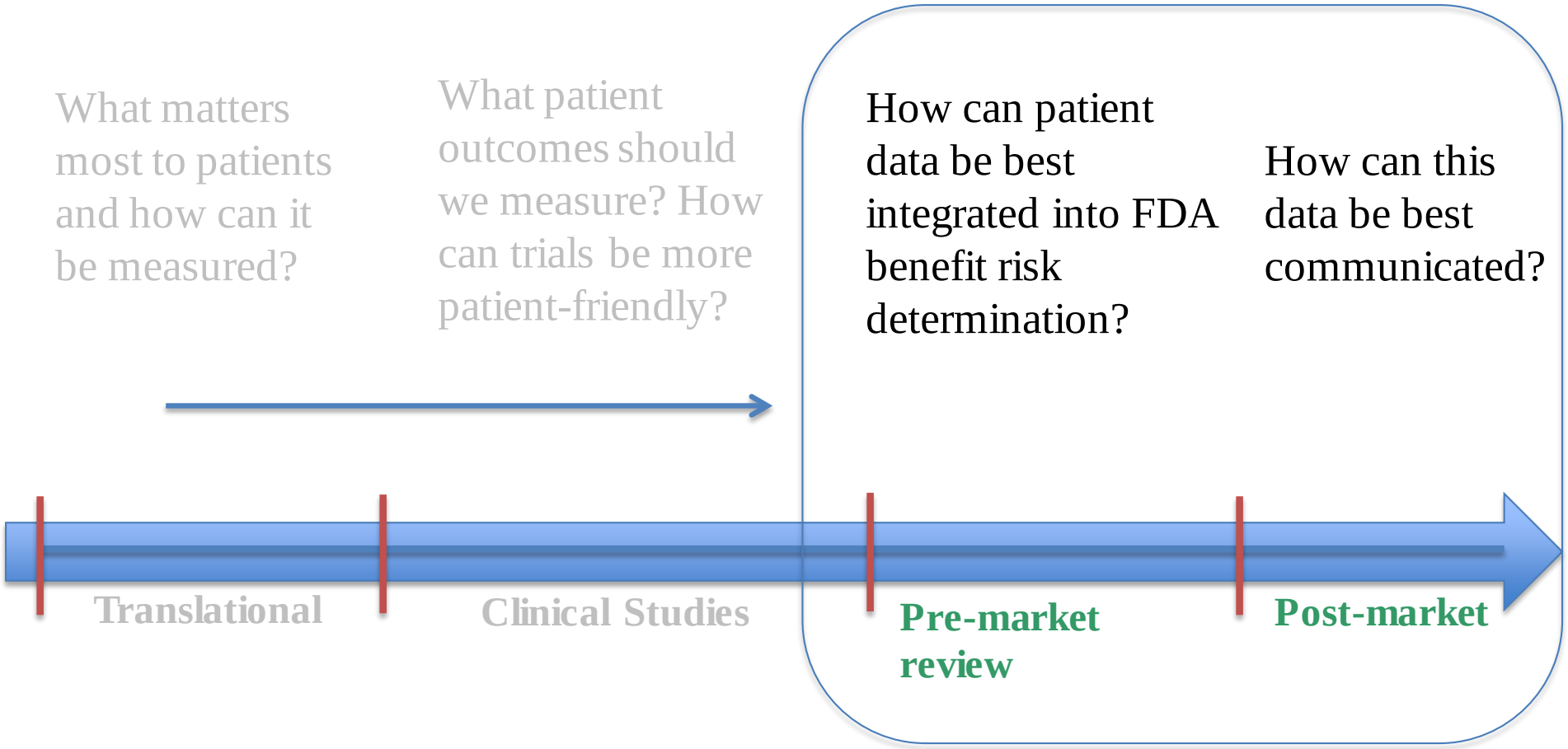


Standard Outcomes

Standard Objectives

Standard Analyses

Further integrating patient perspective into drug development and decision making



Adapted slide by permission from Theresa Mullin, Office of Strategic Programs, FDA

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 Real World Data

- How to take advantage of real world data?
- How to analyze and interpret PRO measures from clinical practice?

We are making progress…

- Collaboration
- Science
- Progress

JAMA | Special Communication

Guidelines for Inclusion of Patient-Reported Outcomes in Clinical Trial Protocols
The SPIRIT-PRO Extension

Melanie Calvert, PhD; Derek Kyte, PhD; Rebecca Mercieca-Bebber, PhD; Anita Slade, PhD; An-Wen Chan, MD, DPhil; Madeleine T. King, PhD; and the SPIRIT-PRO Group

STANDARD PROTOCOL ELEMENTS

- International Delphi Process
- International consensus based PRO-specific protocol guidance

Calvert M, Kyte D, Mercieca-Bebber et al. *JAMA*. (2018) 319(5):483-494.

Analysing data from patient-reported outcome and quality of life endpoints for cancer clinical trials: a start in setting international standards

Andrew Bottomley, Madeline Pe, Jeff Sloan, Ethan Basch, Franck Bonnetain, Melanie Calvert, Alicyn Campbell, Charles Cleeland, Kim Cocks, Laurence Collette, Amylou C Dueck, Nancy Devlin, Hans-Henning Flechtner, Carolyn Gotay, Eva Greimel, Ingolf Griebsch, Mogens Groenvold, Jean-Francois Hamel, Madeleine King, Paul G Kluetz, Michael Koller, Daniel C Malone, Francesca Martinelli, Sandra A Mitchell, Carol M Moinpour, Jammbe Musoro, Daniel O'Connor, Kathy Oliver, Elisabeth Piault-Louis, Martine Piccart, Francisco L Pimentel, Chantal Quinten, Jaap C Reijneveld, Christoph Schürmann, Ashley Wilder Smith, Katherine M Soltys, Martin J B Taphoorn, Galina Velikova, and Corneel Coens, for the Setting International Standards in Analyzing Patient-Reported Outcomes and Quality of Life Endpoints Data (SISAQOL) consortium

STANDARD ANALYSIS METHODS

- PRO research objectives
- Procedures for Missing Data
- Statistical methods suitable for the research objective

Bottomley A, Pe M, Sloan J, et al. *Lancet Oncol* (2016). E510-e514.

Real World Data from Real World Patients

Science.
Collaboration.
Progress.

KAISER PERMANENTE®

FDA U.S. FOOD & DRUG ADMINISTRATION
ONCOLOGY CENTER OF EXCELLENCE

PanPROE: Pancreatic Cancer Patient-Reported Outcomes using the Electronic Medical Record

Electronically capture PRO physical function in pancreatic cancer patients who undergo treatment and follow up for their illness.

Can we better understand a patient's function along their pancreatic cancer journey in real-world practice?

FDA Public Workshop: 2018 Clinical Outcome Assessments in Cancer Clinical Trials

- June 22, 2018 FDA Campus
- Cosponsored with American Society of Clinical Oncology (ASCO)
- Discussed PRO measurement tools

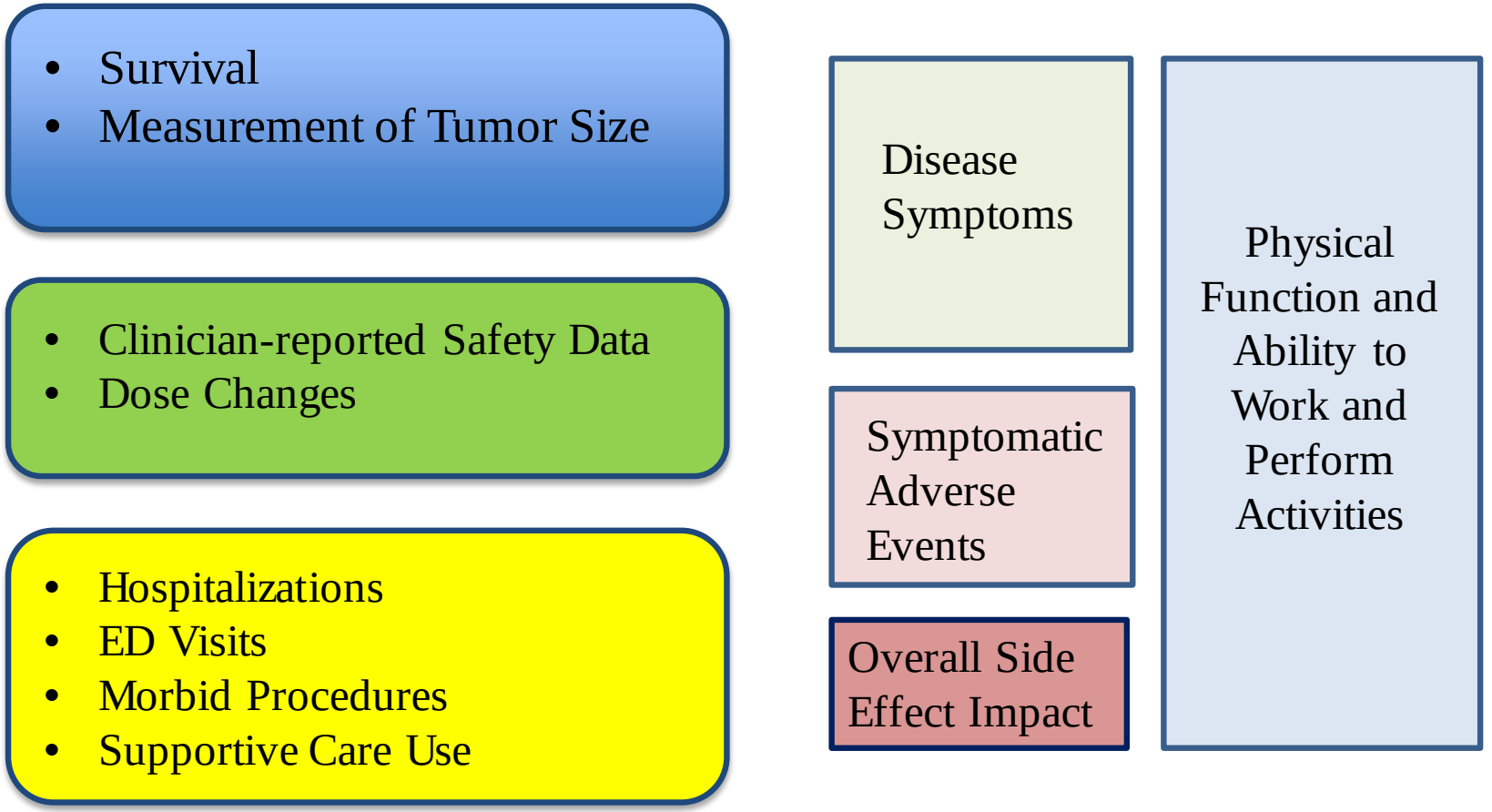
https://www.fda.gov/NewsEvents/MeetingsConferencesWorkshops/ucm602540.htm

FDA Public Workshop: 2019 Clinical Outcome Assessments in Cancer Clinical Trials

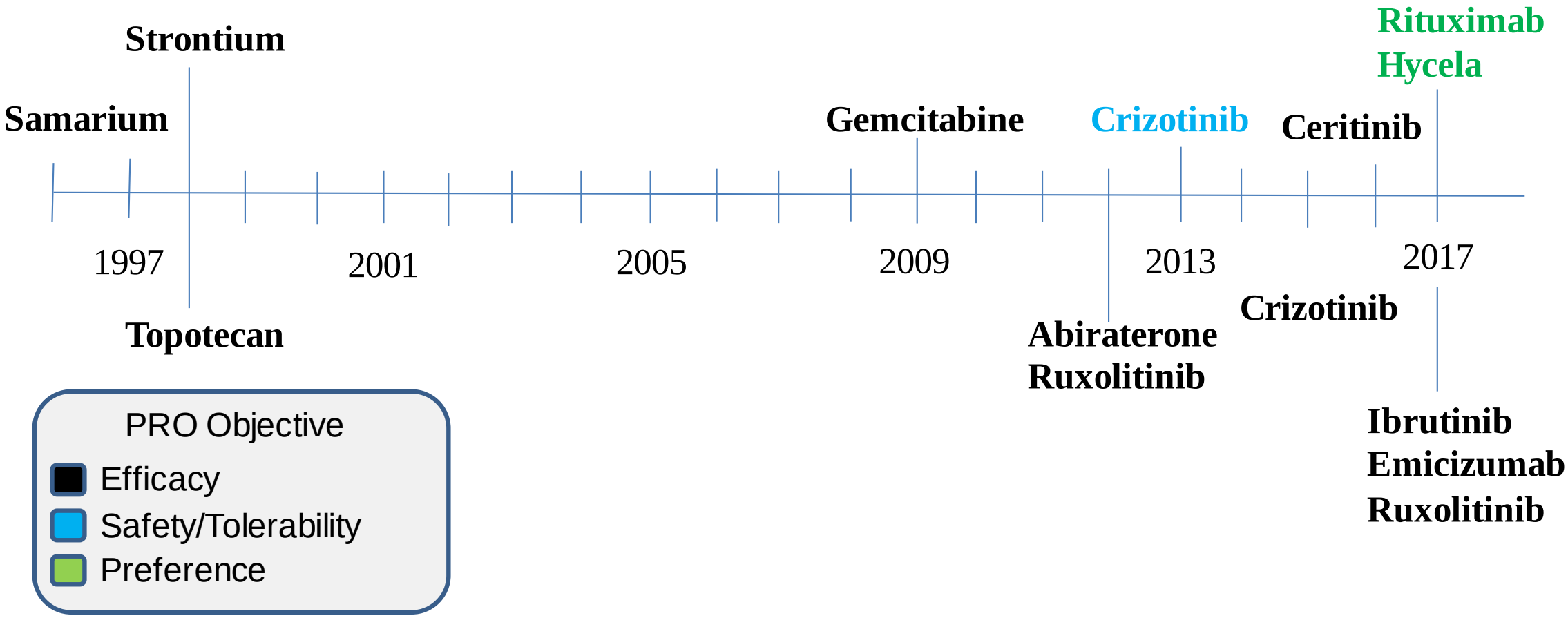
- July 12, 2019 FDA Campus
- Cosponsored with American Society of Clinical Oncology (ASCO)

- How can we best measure and communicate a patient's **physical function** while undergoing a cancer therapy?

Patient Experience data to Complement our Existing Information about a Drug



Communicating Patient Experience Data:
Patient-reported symptom and function data in FDA labels



In Closing…

- We continue to look for ways to make clinical trials more patient friendly
- Work is progressing to develop standard ways to measure, interpret and communicate symptoms and function
- Engaging with patients and the international drug development community remains a priority


FDA
U.S. FOOD & DRUG
ADMINISTRATION