Session 4: Summary of the Day- Seeing the Forest Through the Trees

Moderators:

- Ethan Basch, M.D. – University of North Carolina
- Stacy Gray, M.D., A.M. – City of Hope

July 12th, 2019 FDA-ASCO Public Workshop:
Clinical Outcome Assessments in Cancer Clinical Trials

Workshop Sessions 1-3

- Session 1
  - Examples of ePRO in multiple healthcare systems
  - Importance of clearly defining research objectives to inform regulatory and policy decisions
- Session 2
  - Physical function as a core clinical outcome that could be used for regulatory approval
  - Introduction of the estimand framework as a way to rigorously structure PRO studies
- Session 3
  - Scenarios using the estimand framework to assess 2 physical function endpoints: comparative benefit and on treatment maintenance

PRO work going forward

- Where we are
  - Multiple core outcomes have been identified for oncology
  - Increasing adoption of ePRO across health systems
  - Estimand framework to enable standardized approach to PRO research
- Key barriers
  - Changing the culture of clinical trial design and execution
  - Financing PRO and workflow modifications
  - Developing platforms and processes to keep providers and patients engaged
  - Incorporating PROs into drug development and real-world evidence generation
- Next steps…

FDA Panelists

Theresa Mullin, PhD - Associate Director of Strategic Initiatives, CDER

Core Clinical Outcomes across Therapeutic Areas

Paul Kluetz, MD – Deputy Director, Oncology Center of Excellence

Core Clinical Outcomes in Oncology – Symptoms and Physical Function

Amy Abernethy, MD/PhD – Principal Deputy Commissioner

Physical function data in the trial and clinical care setting

FDA U.S. FOOD & DRUG ADMINISTRATION

ONCOLOGY CENTER OF EXCELLENCE

PROJECT FACILITATE

Assisting healthcare providers with requests for access to investigational oncology products

DO YOU NEED HELP SUBMITTING A SINGLE PATIENT IND EXPANDED ACCESS (EA) REQUEST (ALSO KNOWN AS COMPASSIONATE USE) FOR A PATIENT WITH CANCER?

...FDA's Oncology Center of Excellence (OCE) can help:

- Locate IRB resources
- Find an EA contact for a drug/biotech company
- Complete Form FDA 3926

9:00 AM - 5:00 PM Eastern Time (M-F)

Phone: (240) 402-0004

Email: OncProjectFacilitate@fda.hhs.gov

www.fda.gov/oce

Patients: Talk to your healthcare provider to discuss whether expanded access is an appropriate option.