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
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
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

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