

Clinical Breakout session

Co-chairs

Mark N. Melkerson

David Feigal, M.D.

- Rapporteurs

- Daya Ranamukhaarachchi, Ph.D.

- Viju Bhadkamkar

- Presentation Assistance

- Daniel C. Sullivan, M.D.

Project:

**Define appropriate tools to describe
biodistribution and activity throughout
body and locality of target over time**

Issue to be Solved

- **Issue Categories:**
 - Dosing, scaling, maximum dose, and PK distribution
 - Identification of existing methods and Development of new methods to monitor the biodistribution over time
 - In order to facilitate qualification of imaging as biomarker of biodistribution, re-evaluate safety and efficacy criteria for Group 1 imaging agents (e.g., generate data to allow consideration of aligning CDER efficacy criteria with CDRH criteria for in vitro diagnostics)
 - Impact and ultimate fate of nanoparticles

Project Description

- Identify and Develop new and existing quantitative tools for monitoring biodistribution over time for various types of nanomaterials

Expected Results (applicability)

- **Output**
 - **Biodistribution tools and methods to:**
 - Enhance in vivo diagnostics
 - Enhance in vivo evaluation of nanomaterials
 - Enhance and understand toxicity issues
 - Enhance our ability to understand targeting
 - Increase the number of acceptable biomarkers for evaluation of biodistribution over time
 - Identify the limitations, if any, of existing biomarkers for evaluation of biodistribution over time
- **How the outputs will address the issue**
 - Tools and methods will provide in vivo spatial localization of nanomaterials
 - Tools and methods will aid in evaluation of safety and efficacy of nanotechnology products

Critical Study Design Elements and Data Output Elements

- **Design**
 - Coordination with existing government, private and academics involved in imaging and diagnostics, etc.
 - Utilizing existing government and academic programs to address common issues (e.g., NIH probe development)
 - Ability to articulate safety and effectiveness of nanotechnology products and spatial localization of nanomaterials
- **Data Outputs**
 - biodistribution data with cell, tissue, organ and body over time
 - Dosing, scaling, maximum dose, and PK distribution data
 - Impact and ultimate fate of nanomaterials
 - Metabolism data
 - Clearance data

Timeline/ Level of Effort

- **Timeline** (roughly: initiation to completion)
 - 1-3 years – Identification and modification of existing tools and methods for evaluating biodistribution over time
 - 3-5 years – New tools and methods for evaluating biodistribution over time
- **Level of effort**
 - Identification and Modification – Moderate Cost/Effort
 - New tools and methods – High Cost/Effort