

Workshop Structure and Outputs

Flow for the workshop

- Panels – to get us thinking
- Breakouts to formulate projects
- Plenary to
 - Clarify
 - Cross-cut
 - Prioritize

Outputs

- Power Point presentations of projects to discuss concepts during the workshop
 - By co-chairs as developed in breakouts
- Text descriptions of top projects as the main product at the end of Wednesday
 - By rapporteurs
- Publication of priority list of top project descriptions
 - By ANH and FDA after the meeting

FDA Panel

- One speaker from each of the three centers that address medical product authorizations
 - Center for Drug Evaluation and Research
 - Center for Biologics Evaluation and Research
 - Center for Devices and Radiological Health
- Perspectives on products using nanoscale materials
 - What are we seeing
 - What data needs – and any “challenges” we see

“Case study” Panel

- 4 perspectives on what has happened and what might lie ahead in getting FDA authorization when nanoscale materials are used
 - General imaging perspective for nanoparticle biodistribution
 - A product candidate in clinical trial
 - Established product
 - Multiple platforms and therapies in pre-IND to clinical trial phases

Help us make this work

- Clear, doable projects that are fleshed out enough to make a pitch for funding
- Something that you really want done so that it will solve a problem that is in front of you

Example project:

**Biodistribution modeling for
particle size**

Issue to be Solved

- **Issue Category:** Uncertainty reduction
- Uncertainty regarding generalized effects of size characteristics (hard particle, hydrodynamic size, aspect ratio, etc) on biodistribution forces more data development for each new nanoscale drug/device than would be necessary if we had a greater baseline understanding of biological interactions of nanoscale materials.

Project Description

- Develop data sets across size characteristics.
- Develop predictive models of size variation with respect to distribution in the body within a set of characteristics.

Expected Results (applicability)

- **Output**

- Freely available knowledge in data sets and validated models for standard materials in size ranges relevant to their use in medical products

- **How the outputs will address the issue**

- *Manufacturers*: Use knowledge to choose safer alternatives (or alternatives that target safely)
- *FDA*: Use knowledge in quantitative, model-based, learn-confirm cycles to improve review response and lessen need for testing and characterization data

Cost effectiveness

- **How widely useful are the outputs?**
 - Data sets and models would likely be limited to a class of materials (dendrimers, gold spheres, etc).
 - Could also generate leads and development of product areas (so potentially building larger user needs).
- **What benefit value of the project?**
 - Difficult to estimate. Depends on the class of materials chosen, stimulus toward future market development, future market potential, and the baseline data needs.

Critical Study Design Elements and Data Output Elements

- **Design**

- Standard animal models to relate to NCE data and modeling
- IV administration
- Radiolabel? – other methods for biodistribution evaluation?

- **Output**

- Broad spectrum of size measurements carried through to open literature access associated with biodistribution data
- Quantitative models

Timeline/ Level of Effort

- **Timeline** (roughly: initiation to completion)
 - ~1 year for testing – including time to select materials and design tests of general applicability
 - Model development would take another year
- **Level of effort** (low, medium, high)
 - Low – per material evaluated and “model element” developed
 - Each is potentially a “one post-doc and one year” type effort
 - Combining and linking efforts across model elements is medium