Nanomedicine: Risk Assessment and Management

Perspectives from the Nanomedicines Alliance

January 2014
Why Develop Nanomedicines?

- Improve targeting and effectiveness
- Reduce exposure and toxicity
- Reduce environmental burden
- Diagnose pathological conditions at an earlier stage
What is the Nanomedicines Alliance?
What is the Nanomedicines Alliance?

Unique industry group that focuses on the scientific, regulatory and policy needs of nanomedicines.
Alliance Vision & Mission

**Vision**
- To catalyze the use of nanomedicines for patient benefit.

**Mission**
- To promote and facilitate the scientific advancement, regulatory approval, safe use, and public appreciation of nanotechnology-based medicines world-wide for the diagnosis, treatment and prevention of disease.
Scope

- The Alliance covers nanomaterials and nanotechnologies involved in
  - discovery
  - research
  - development
  - manufacturing
  - marketing
  - disposal

- of pharmaceuticals, biologics and medical devices, including imaging agents and diagnostics
What the Alliance Does:

- Provides a forum for pharmaceutical, biotechnology and medical device companies
  - Create and maintain a compilation of relevant guidelines and standards by region (US, Canada, EU, Japan, etc.) with monthly updates.
  - Monitor and report on external nanomedicines and nanotechnology conferences.

- Defines priority issues of science, regulation, and policy

- Works with government partners and other stakeholders to realize the potential of nanomaterials and nanotechnology for medical applications
  - Provides practical, science-driven information on nanotechnology as it is used in the development and manufacturing of nanotechnology-based medicines and medical devices
Organizational Structure

Nanomedicines Alliance Board of Directors
Chair: Henry Havel, Eli Lilly
Vice Chair: Frank Malinoski, Liquidia

- White Papers
  - Government Agencies (FDA, NIH, etc.)
- Outreach
- Reporting & Monitoring
  - Nano Digest
- Conference and Roundtable Sponsorship
- Data Sharing
  - Legislative Outreach
  - Potential Members
  - Other Collaborators
Past/Ongoing Alliance Activities

- Working with government agencies:
  - Food & Drug Administration
  - National Center for Toxicological Research (NCTR)
  - National Cancer Institute Alliance for Nanotechnology in Cancer
  - Nanotechnology Characterization Laboratory (NCL)
  - NCI–NIH Office of Cancer Nanotechnology Research
  - National Nanotechnology Initiative

- Working with more than 80 non-member companies to raise awareness of nanomedicine issues

- Monitoring and reporting on legislative developments related to nanomedicine; identifying key legislators
Past/Ongoing Alliance Activities

  - Regulatory and legislative developments worldwide
  - Scientific reviews and publications
  - Conferences of interest

- Advise Agencies on Draft Guidances
  - “Considering Whether an FDA Regulated Product Involves the Application of Nanotechnology.”
  - “Protecting the Nanotechnology Workforce: NIOSH Nanotechnology Research and Guidance Strategic Plan 2013-2016”
  - “Consideration of FDA Regulated Products That May Contain Nanoscale Materials.”
  - Commented the draft 2014 National Nanotechnology Initiative (NNI) Strategic Plan
Title: “Nanomedicines: Charting a Roadmap to Commercialization”

Focus: Collaboration with NCI & FDA to discuss industry perspectives, needs, and challenges in nanomedicines
- Designing Nanomedicines
- Preclinical Pharmacology
- Toxicology/Absorption, Distribution, Metabolism and Excretion (ADME)
- Chemistry, Manufacturing and Controls (CMC)
- Clinical Studies

Meeting Proceedings to be published:
- AAPS Journal: Journal of the American Association of Pharmaceutical Scientists
- Science Translational Medicine
# Nanomedicines
A different mindset from “conventional” nanoparticles

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<th>Aspect</th>
<th>Conventional nanoparticles</th>
<th>Nanomedicines</th>
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<tr>
<td>Design goal</td>
<td>Engineering/tech performance</td>
<td>Medical benefit</td>
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<tr>
<td>Physicochemical properties</td>
<td>Generally uniform, homogeneous; relatively insoluble</td>
<td>Generally heterogeneous; soluble components</td>
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<tr>
<td>Human exposure</td>
<td>Unintended</td>
<td>Intended</td>
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<tr>
<td>Risk assessment</td>
<td>Hazard identification; appropriate controls</td>
<td>Safety characterized in development; aim for favorable benefit:risk</td>
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<td>Regulatory authority oversight (US)</td>
<td>OSHA, EPA</td>
<td>FDA</td>
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Key Points from Alliance’s Comments on FDA’s Notices

- FDA’s current principles of Pharmaceutical Development, Quality Risk Management and Pharmaceutical Quality Systems as well as the principles of Quality by Design (QbD) provides a valuable framework for evaluating nanomedicines
  - May serve as a model for other segments of nanotechnology industry

- Unique features of nanomedicines will be product specific, and will be considered with currently established processes applied to all new medicines

- For particle characterization [standards], techniques and standards exist
Conclusions from Alliance’s Comments on FDA’s Notices

- The current U.S. regulatory framework is sufficiently comprehensive to accommodate nanomedicinal products
- This framework allows for additional specific considerations on a case-by-case basis
- Forthcoming advances may stimulate development of new tools and approaches in the future
- The Nanomedicines Alliance will continue to embrace and pragmatically apply ongoing and emerging advances in bionanotechnology
Summary of Nanomedicines & Nanomedicines Alliance

- Nanomedicines are intended for human use & offer significant benefit to healthcare

- As with all human use products, benefits are balanced against risks associated with the product

- Nanomedicines are unique in nanotechnology as we operate under established regulations for human use products

- The Nanomedicines Alliance works to ensure establish appropriate technical and policy standards as well as education about nanomedicines
Thank You

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