Meeting with CDER Nanotechnology Risk Assessment Working Group

October 16, 2013

Nanomedicines Alliance Attendees

- **Dr. Henry Havel, Ph.D. – Chairman**
  Senior Research Fellow, Eli Lilly and Company
- **Dr. Frank Malinoski, M.D., Ph.D. – Vice Chairman**
  Chief Medical Officer, Liquidia Technologies
- **Dr. Lawrence Tamarkin, Ph.D. – Board member**
  President and CEO, CytImmune Sciences Inc.
- **Marc Wolfgang – Board member**
  VP Pharmaceutical Sciences and Manufacturing, Cerulean Pharma Inc.
- **Dr. Maggie Liu, Ph.D. – Secretariat**
  Science Advisor, Drinker Biddle & Reath, LLP

Meeting Agenda

- Introductions
- Overview of the Nanomedicines Alliance
  - Key objectives
- Overview of CDER Nanotechnology Risk Assessment Working Group
  - Key objectives
- Discuss ways NMA may be of help to CDER's Working Group
- Discuss potential collaborations
  - or other types of beneficial interactions
- Q&A

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?

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.

What the Alliance Does:

- Provides a forum for pharmaceutical, biotechnology and medical device companies
- 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 government agencies and other stakeholders with practical, science-driven information on nanotechnology as it is used in the development and manufacturing of nanotechnology-based medicines and medical devices.

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
- 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

- Publishing Monthly NanoMed Digest
  http://nanomedicines-alliance.org/publications.html
  - Regulatory and legislative developments worldwide
  - Scientific reviews and publications
  - Conferences of interest
- Comment on FDA 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."

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

- 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

2013 NanoMedicine Symposium

- Title: *"Nanomedicines: Charting a Roadmap to Commercialization"*
- Focus: Collaboration with NCI & FDA to discuss industry perspectives, needs, and challenges in nanomedicines
- Over 70 attendees representing government, academia and pharmaceutical industry
- Meeting Proceedings to be published:
  - *AAPS Journal* : Journal of the American Association of Pharmaceutical Scientists
  - *Science Translational Medicine*

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 the establishment of appropriate technical and policy standards as well as education about nanomedicines

Questions for CDER Nanotech Working Group

1. What are FDA's thoughts on Global Harmonization of regulatory guidance for Nanomedicines (e.g. with EMA, ICH)?
2. Can CDER comment on if and how it is working with other domestic agencies [e.g. NIH, NNI, NIST] in the area of nanomedicine drug development and regulation?
3. How is Nanotechnology Risk Assessment integrated into other areas of the FDA, e.g. medical devices and biologics? How does the CDER working group interact with the other divisions?
4. Does CDER still feel that current regulations and guidance are sufficient for registration of nanomedicines?
   - If CDER feels that additional nanomedicine-related regulations/guidance might be useful, what are the issues that CDER views as needing to be addressed?
5. Has CDER identified any trends or generalizations regarding nanomedicines from the database analysis referenced in the July 2013 AAPS Journal article?
   - Are there any general areas in which CDER feels Industry is doing particularly well?
   - Are there any general areas which CDER feels Industry could be doing better?
6. Does CDER have any advice for companies preparing regulatory submissions (INDs, NDAs, …) for nanomedicines?
7. What have been the Working Groups biggest challenges in meeting nanotechnology/nanomedicines objectives?
8. How can the Nanomedicines Alliance best partner with CDER to advance the development of safe and effective nanomedicines?

Contact Information

- For more information on the Nanomedicines Alliance, see http://www.nanomedicines-alliance.org/

- Any questions? Contact the Alliance Secretariat at Alexis.Robertson@dbr.com or 202-230-5653