



**Nanomedicines**  
ALLIANCE

# 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?

**AMGEN**<sup>®</sup>

**BIND**  
THERAPEUTICS

**CERULEAN**   
Pharma Inc.

**CYTImmune**  
SCIENCES INC

*Lilly*

**LIQUIDIA**  
TECHNOLOGIES

 **MERCK**  
SERONO

 **NanoCarrier**<sup>®</sup>  
LEADING-EDGE NANOTECHNOLOGY

 **NanoViricides**  
Incorporated

**Pfizer**

# 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.

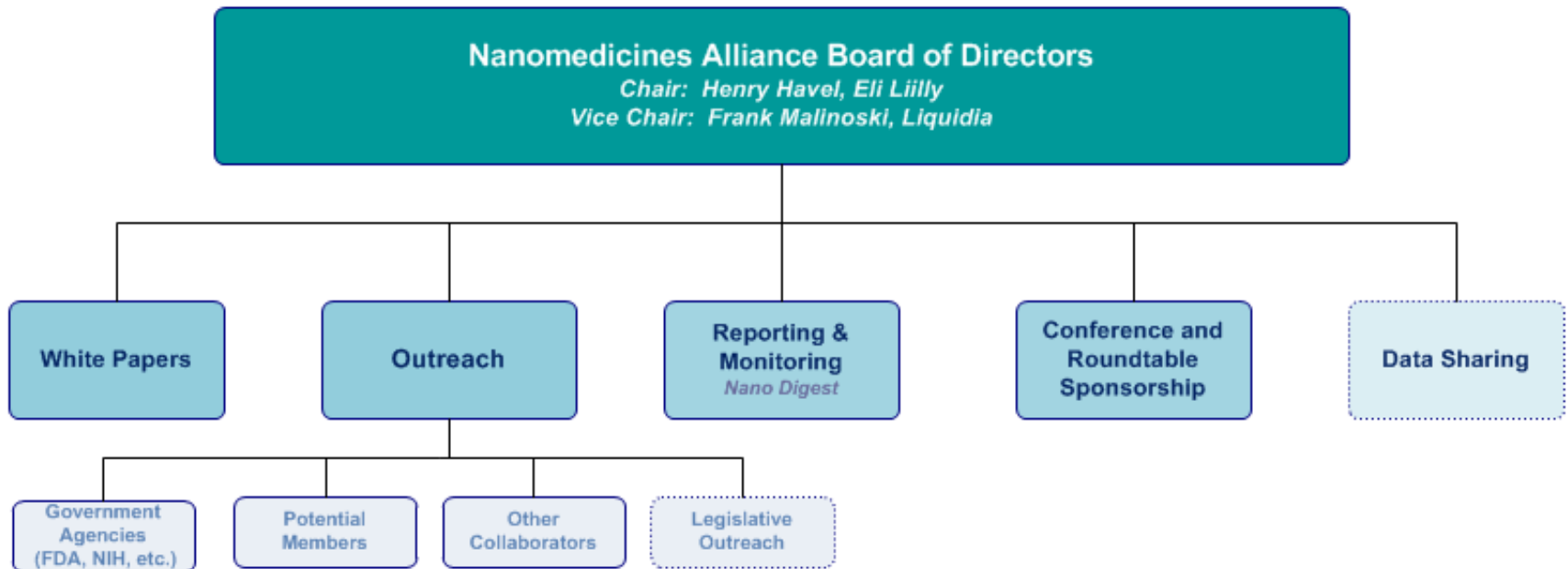
# Scope

- ▶ 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





# 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

- ▶ Publishing Publically Available Monthly NanoMed Digest <http://nanomedicines-alliance.org/publications.html>
  - 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

# 2013 NanoMedicine Symposium

- ▶ 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

Aspect	Conventional nanoparticles	Nanomedicines
Design goal	Engineering/tech performance	Medical benefit
Physicochemical properties	Generally uniform, homogeneous; relatively insoluble	Generally heterogeneous; soluble components
Human exposure	Unintended	Intended
Risk assessment	Hazard identification; appropriate controls	Safety characterized in development; aim for favorable benefit:risk
Regulatory authority oversight (US)	OSHA, EPA	FDA

# 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



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# Thank You

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