Symposium
Best Practices in Clinical Study Design for Rare Diseases

April 29-30, 2013
Jack Morton Auditorium
Washington, DC

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Research in Pediatric Developmental Pharmacology Centers

Children's National Medical Center

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Clinical and Translational Science Institute at Children's National

Symposium
Best Practices in Clinical Study Design for Rare Diseases

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How we got here….

* Interested parties
  - From academia, government, industry, advocates
* Planning group worked on various formats
* Engagement of NIH, FDA, and other stakeholders
* CTSI-CN agreed to serve as catalyst
  - Partnership with Developmental Pharmacology Center(s)
* Industry engagement, but no commercial support

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Objectives

* "Working meeting" format
* Dialogue that creates opportunities for action
* Identification of key issues
* Define real potential solutions
* Articulate outcomes

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Meeting Agenda

Morning Day 1

Rare Diseases Landscape and Overview

Challenges and Solutions in Drug Development for Rare Diseases

The Regulatory Pathway for Rare Diseases: Lessons Learned from Previous Examples of Clinical Study Designs for Small Populations

Regulatory Experience from Clinical Studies in Enzyme Deficiency Disease

Potential Path for Regulatory Process in Rare Disease Space

A Patient Advocate's View of Drug Development in Rare Diseases

Clinical and Genetic Characterization of Duchenne Muscular Dystrophy Patients

Clinical and Genetic Characterization of Cystic Fibrosis Patients

Meeting Agenda

Afternoon Day 1

Roundtable 1: Statistical Challenges in Clinical Study Design in Rare Diseases

Roundtable 2: Drug Development Tools in Rare Diseases

Morning Day2

Roundtable 3: Considerations for Drug Development Programs in Rare Diseases

Roundtable 4: The Economics of Rare Diseases Drug Development

Afternoon Day 2

Rare Diseases Drug Development Infrastructure

Summary, Outcomes, Next Steps

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Setting the Stage…

* Pediatric and Rare/Orphan Disease Drug Development have a lot in common
* Decades of relevant legislation
  - Orphan Drug Act 1983
  - More than 400 drugs approved (7,000 diseases)
* "Carrots and Sticks"

1. A New Era in Drug Development

A "good" time for Rare and Orphan Diseases Research

* **Significant advances in biomedical science is producing real opportunities**
* **Legislative and regulatory advances that facilitate pediatric and rare diseases drug development**
* **NIH, FDA and the global community are addressing rare and orphan diseases issues**
* **Increased impact of patients advocacy POV**

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1. A New Era in Drug Development

* **Vanguard drug development successes**
* **Increasing interest and investment by industry and others**
* **The pipeline is filling up…..**

Knowing is not enough; we must apply. Willing is not enough; we must do.

Johann Wolfgang von Goethe

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2. Paradigm Shift

* There is still a lot of work but the time is right for a paradigm shift.
* Discovery – Understanding how things work
  - New knowledge
  - Solutions in search of needs
* Development – Assessing solutions for unmet needs
  - Start with the end in mind
  - Actionable knowledge

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2. Paradigm Shift

* **BIO/NCATS priorities**
  - **Predictive Biomarkers**
  - **Predictive pre-clinical efficacy and toxicity testing methods**
  - **Improve patient recruitment, clinical trial design, and IRB process**
  - **Leadership role in working with FDA to develop and validate novel approaches to drug development**
* **Other Issues**
  * **Legal and other process challenges**
  * **Team Development – What does the "draft look like?"**

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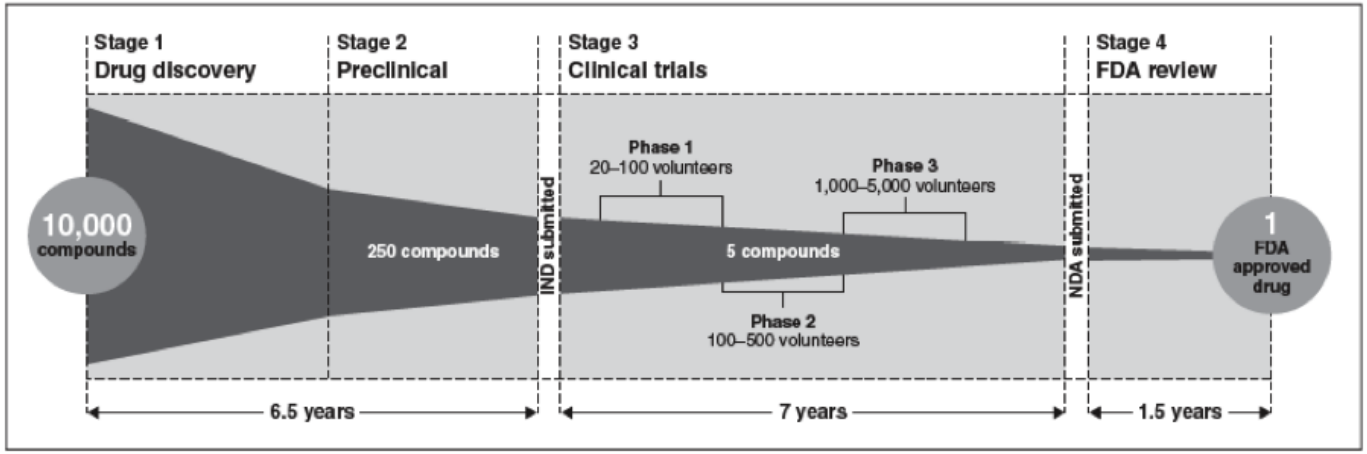
3. Uncertainty/Risk Management

* Scientific/Technical
* Human
* Professional
* Personal
* Financial
* Societal
* Others



Source: Pharmaceutical Research and Manufacturers of America.

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Critical Issues

* New approaches to partnerships and collaboration
* New models for clinical and translational research
* Processes, infrastructure and standardization
* Understanding and dealing with risk
* Creating sustainability
* Urgency

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Critical Issues in Development

* A defined unmet medical need
* A sense of urgency/priority to fill the need
* An available candidate solution
* A feasible plan for evaluation and regulatory path
* A rational business plan for making the product available
* A capable business partner

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