

Activities at Academic Research Centers: Identifying Present Activities and Future Opportunities

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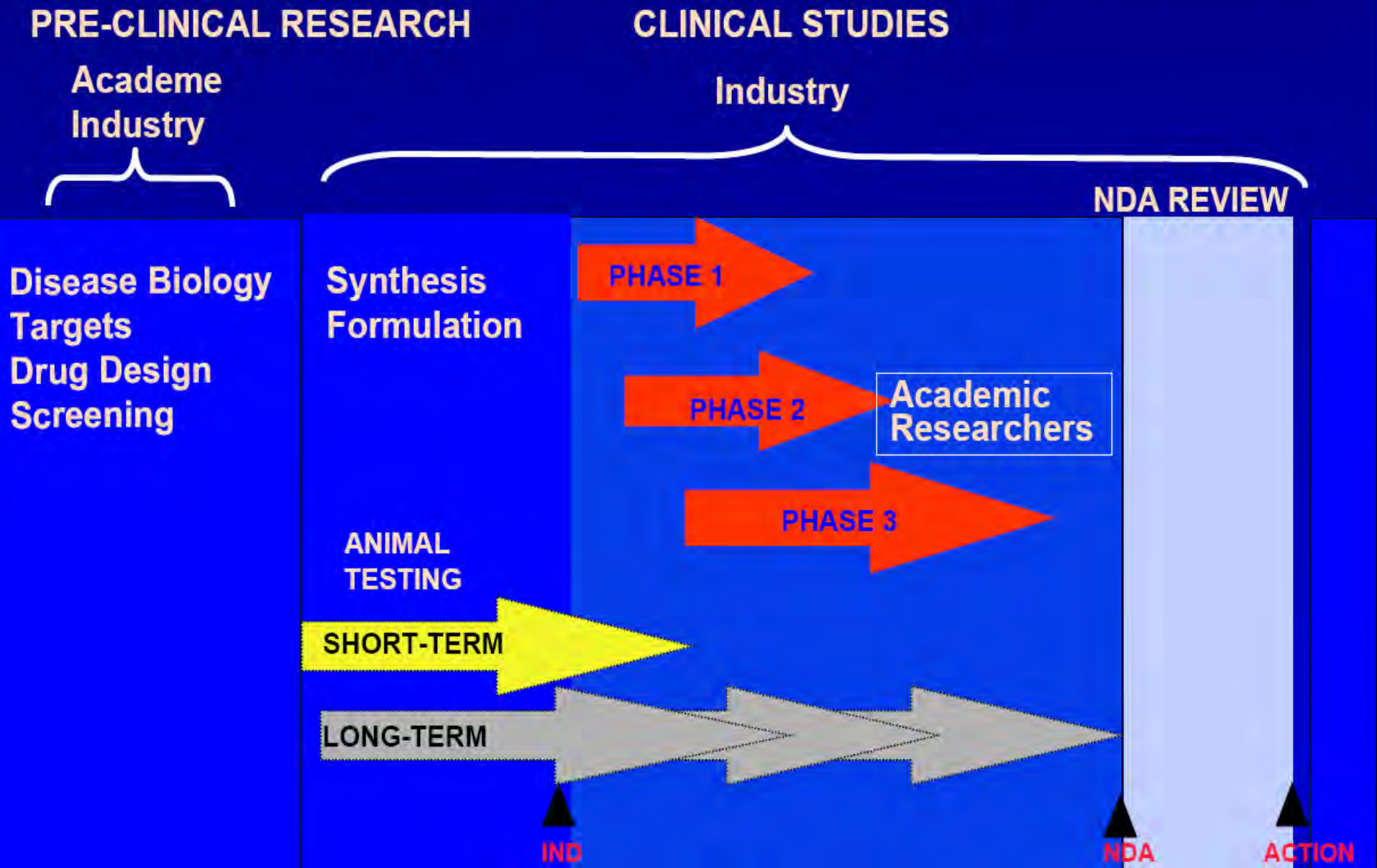
Winter in Minnesota

Wish I was
in Rome!

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Historical Paradigm of Drug Research in Academic Institutions

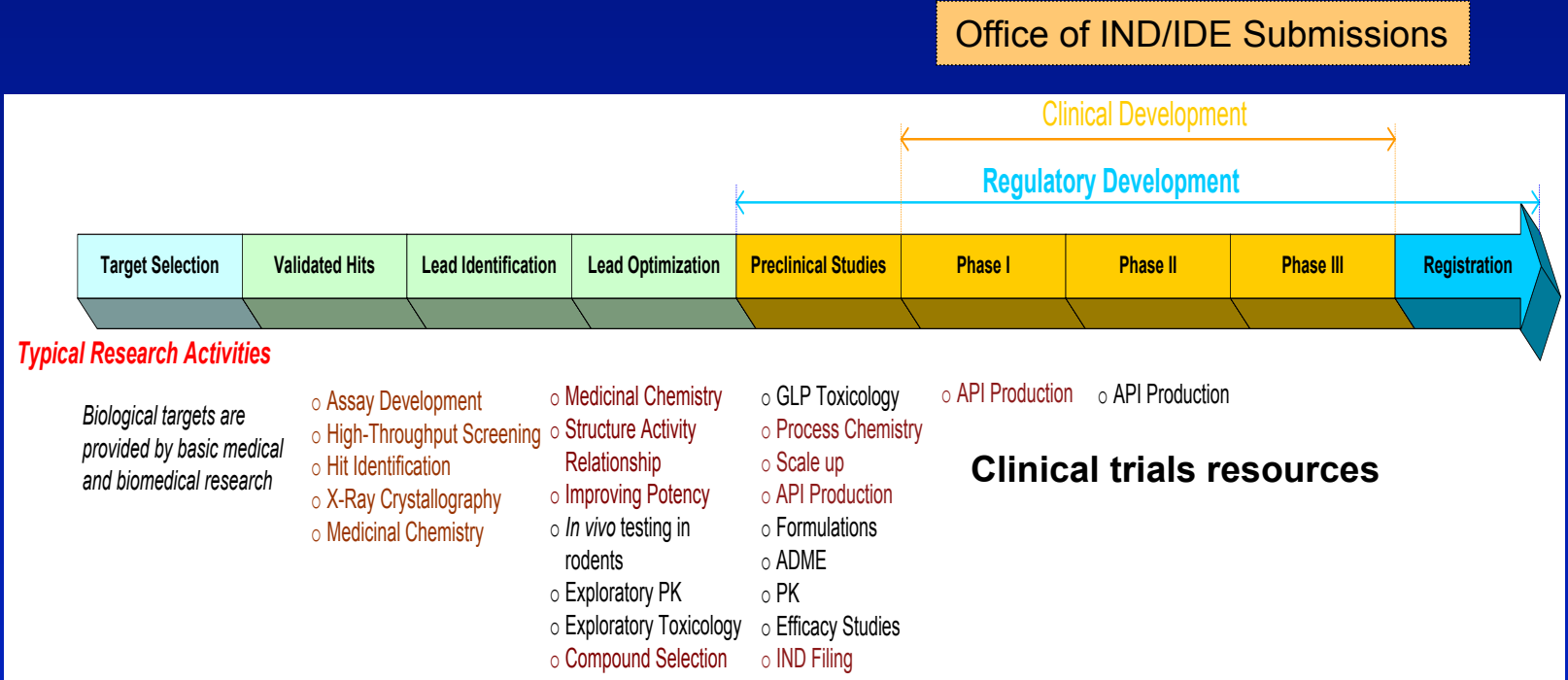


Evolving Role of Academic Institutions in Drug Discovery and Development

“The times they are a changing”*

- Increasing interest by universities in commercializing discoveries**
- Greater industry interest in acquiring drugs in later-stage development**

An Example of Emerging Academic Models of Drug Discovery and Development: University of Minnesota



Institute for Therapeutics Discovery and Development

Clinical and Translational Science Institute

Molecular and Cellular Therapeutics

Center for Forecasting Drug Response

Role of Academic Institutions in Orphan Drug Development

As of late 2008 there were 1956 designations for orphan products:

- **112 belong to individuals, academic centers (laboratories, institutes), or universities***

Emerging Models of Orphan Drug Development in Academic Institutions

- **Disease-disease programs e.g.**
 - **Rare Disease Clinical Network Programs**
- **Drug-centric programs e.g.**
 - **Center for Orphan Drug Research, University of Minnesota**

*** Source: FDA orphan product website**

University of Minnesota Orphan Drug Discovery and Development Initiatives

Institute for Drug Discovery and Development

Center for Forecasting Drug Response

Center for Orphan Drug Research (CODR)

Mission

The mission of the Center for Orphan Drug Research is to improve the care of individuals suffering from rare diseases through research on new drug therapies; education of health professionals and health profession students; and contributions to the discussion and formulation of public policy relating to rare diseases and orphan drugs.

Clinical and Translational Science Institute

Collaborations with internal and external organizations involved in orphan drug research (industry, academe, government)

CODR Drug Discovery and Development Projects

Discovery

- New approaches to Overcoming Bacterial Resistance to Vancomycin
- Discovery of Therapeutics for Spinocerebellar Ataxia Type 1

Pre-clinical Development

- Development of Intravenous Baclofen for Management of Intrathecal Baclofen Withdrawal

Phase I

- Intranasal Diazepam for Seizure Emergencies

Phase II

- Topiramate for Neuroprotection and Seizure Control in Neonates

Phase IV

- N-acetylcysteine as Adjunctive Therapy for Late-stage Adrenoleukodystrophy

Opportunities and Challenges: Conclusions

- Expanding research resources will enhance universities' capabilities to conduct drug discovery and development.
 - This will especially benefit orphan drugs

But

- Academic technology transfer offices must better understand the orphan drug market.
- Faculty & their institutions must learn drug development processes and regulations.
- Funding agencies must fund drug development projects differently than R01s.

Converging opportunities for partnerships among academe, industry, government, and patient advocacy groups will grow substantially and lead to an increase in marketed orphan drugs.

Summer in Minnesota



**Discovery Research Program on Orphan Diseases
An Indo- US Bilateral Workshop**

**April 26-29, 2009
University of Minnesota**

**Sponsored by:
The Indo- US Science and Technology Forum, New Delhi, India**

**Organized by:
`Center for Orphan Drug Research
University of Minnesota
and
Indian Institute of Chemical Technology, Hyderabad**

**For information about the workshop, contact Ramaiah Muthyala:
muthv003@umn.edu**

For registration please contact Amy Olson: olson017@umn.edu

This workshop will foster the exchange and dissemination of information and will provide opportunities to promote bilateral scientific and technological cooperation in the area of rare/neglected diseases. This conference will serve as a forum to address current scientific research in India and the U.S. pertaining to global development of drugs and biologics for rare diseases. Experts across academia and regulatory agencies will convene to present drug discovery, global clinical research and scientific working groups.

Session topics

Metabolic diseases
Cancer
Lysosomal storage diseases
Neurological diseases
Orphan product development, NIH, FDA
Rare diseases, public health, economics

Keynote Speaker:

Dr. Victoria Hale
Founder, Institute of OneWorld Health