

# Introduction to Challenges in Translational Research

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MDA<sup>®</sup>

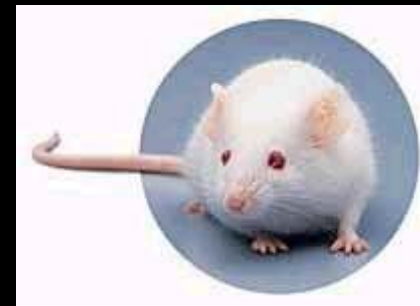
# The Evolution of Research

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**Basic Research:**  
How does that work?



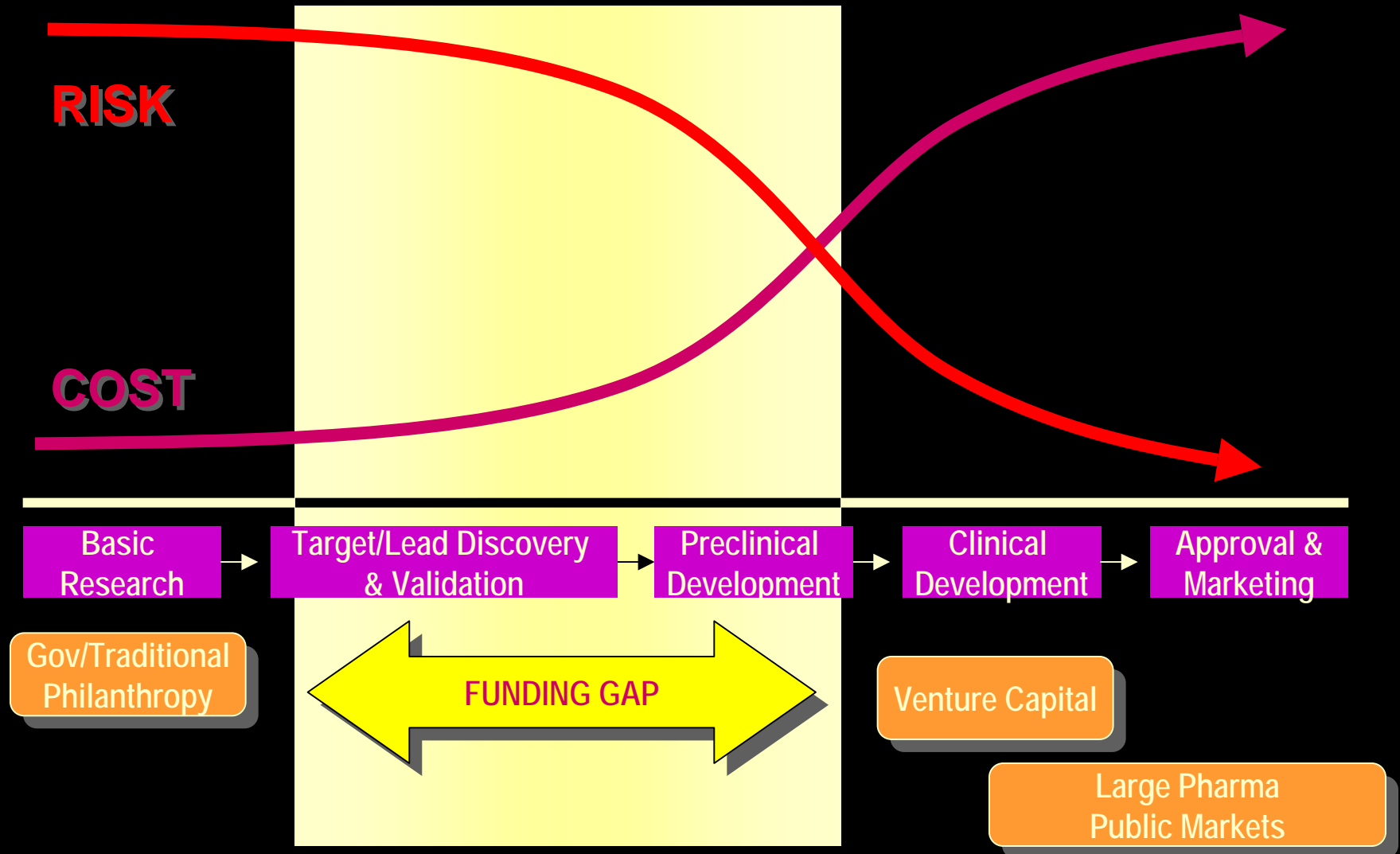
**Medical/Clinical Research:**  
How can we leverage  
what we know to design  
and test therapies?



**Translational Research:**  
Now we've cured mice,  
can we manufacture a drug  
or biological molecule  
that will help humans ?



# Mind the Gap!



# MDA Translational Research Survey

August-September 2003

Target: MDA Advisory Committee members and Ad Hoc Reviewers:  
36 Respondents

53% of respondents combined research and clinical practice

41% of respondents have worked with a for-profit company

## Summary Results:

- Most anticipated translational projects within one year
- Most wanted to retain some input into that research
- Larger laboratories more likely to undertake translational projects without collaborations

# MDA Translational Research Survey

August-September 2003

Survey Cont.

## Needs for Translational Research

- Better **collaborations**
- Development, housing and distribution of **animal models**
- Contract **manufacturers** for biologics, particularly viral vectors
- Better **infrastructure** for conducting clinical trials

# Key Bottlenecks for Academic Labs

MDA Translational Research Advisory Committee Meeting 2004

- Lack of Understanding of Regulatory Approval Process
- Lack of Manufacturers for Some Biologics
- Lack of Efficiency in Clinical Trial Planning
- Lack of Funding for:

**Toxicology**

**-Manufacturing**

**-Quality Assurance**

**-Regulatory Compliance**

**-Infrastructure**

# **Muscular Dystrophy Clinical Research Coalition Meeting June 11-12, 2004**

## **Therapeutics Break-Out Group:**

- **Feed the therapeutic pipeline**
- **Define patient populations: epidemiology, natural history, burden of disease, genotype**
- **Support Infrastructure for centralized diagnostics and outcomes**
- **Expand communications with: FDA, drug companies, gov funding agencies, those affected by the disease**

# MDA Translational Research Program

## **Advisory Committee:**

**Academic scientists, industry and business people, NIH representative**

## **Grant Categories:**

- **Pre- IND Grant**
- **Infrastructure Grant**
- **Corporate Grant (milestone-driven contract)**
- **Clinical research training grant**