

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

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# **State of Orphan Drug Development in the US**

**Since passage of the 1983 Orphan Drug Act**

- $\approx$  1800 treatments designated as orphan products**
- $\approx$  319 orphan products approved by FDA**

# Conventional Paradigm for Drug Discovery & Development

## PRE-CLINICAL RESEARCH

## CLINICAL STUDIES

Academe  
Industry

Industry

NDA REVIEW

Disease Biology  
Targets  
Drug Design  
Screening

Synthesis  
Formulation

PHASE 1

PHASE 2

Academic  
Researchers

PHASE 3

ANIMAL  
TESTING

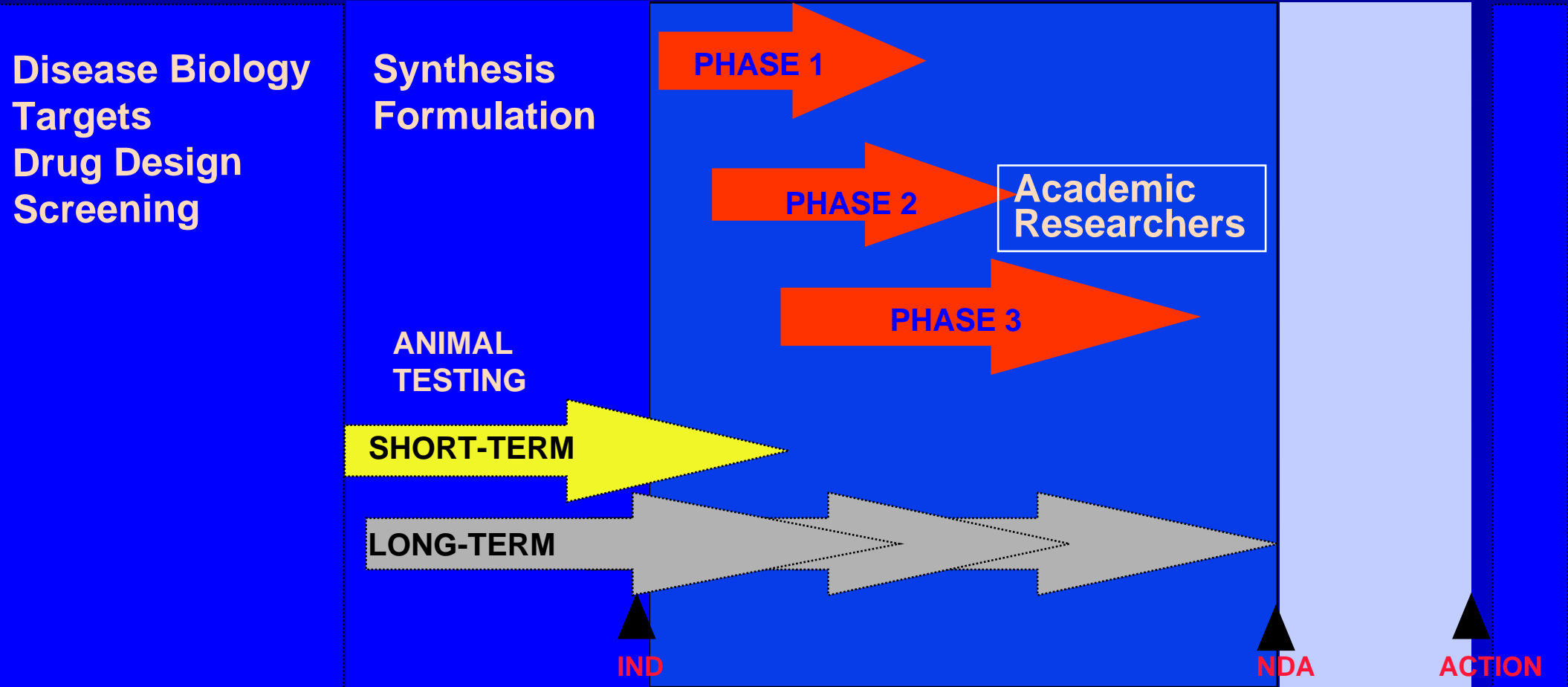
SHORT-TERM

LONG-TERM

IND

NDA

ACTION



# **Industry Participation in Orphan Drug Discovery and Development**

- Large companies generally possess all resources and expertise needed to develop orphan drugs**
- Smaller companies more likely to consider orphan drugs due to market size**
- Smaller companies may have limited expertise in certain areas of drug development**

# **Academic Research Center Participation in Orphan Drug Discovery and Development**

## **Conventional involvement**

- **Basic research on biology of disease**
- **Drug discovery**
- **Rare disease centers**
- **Phase II-III clinical trials**

# New Paradigm for Drug Discovery & Development

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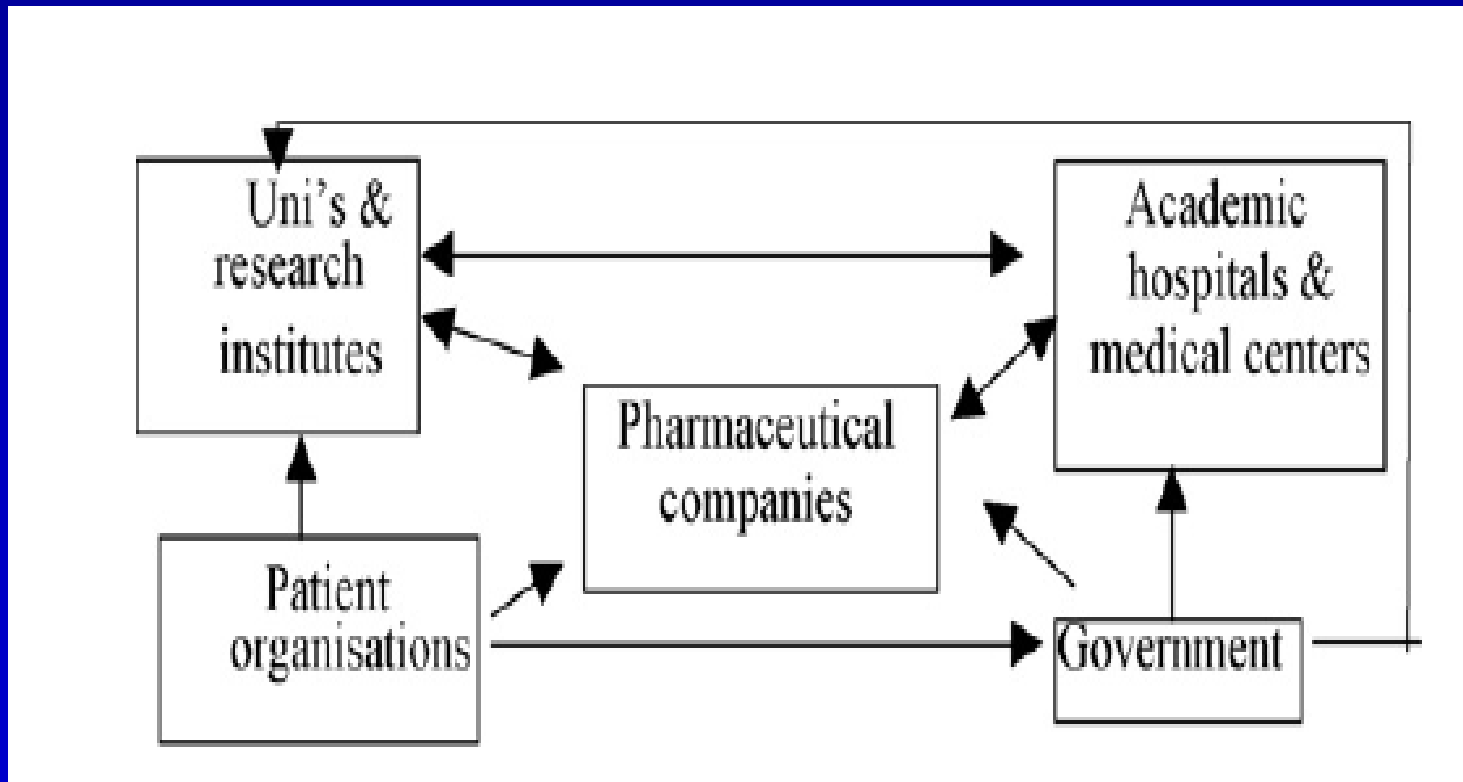
NDA

ACTION

# **New Paradigm for Discovery and Development of Orphan Drugs**

- Drug Discovery**
- Drug Synthesis**
- Formulation**
- Pharmacometrics**
- Bioanalytical Support**
- Clinical Trials: Phase I-III**
- Orphan Product Regulatory Support**

# Sectors Involved in Orphan Drug Development





# Summary

- **Advances in basic biomedical research and drug discovery will accelerate opportunities to develop new therapies for rare diseases**
- **Many of these advances will originate in academic research centers**
- **Academic research centers are now expanding capabilities in drug development**
- **Opportunities for partnerships between academe and the industry will grow substantially and have the potential to increase the number of marketed orphan drugs.**