

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

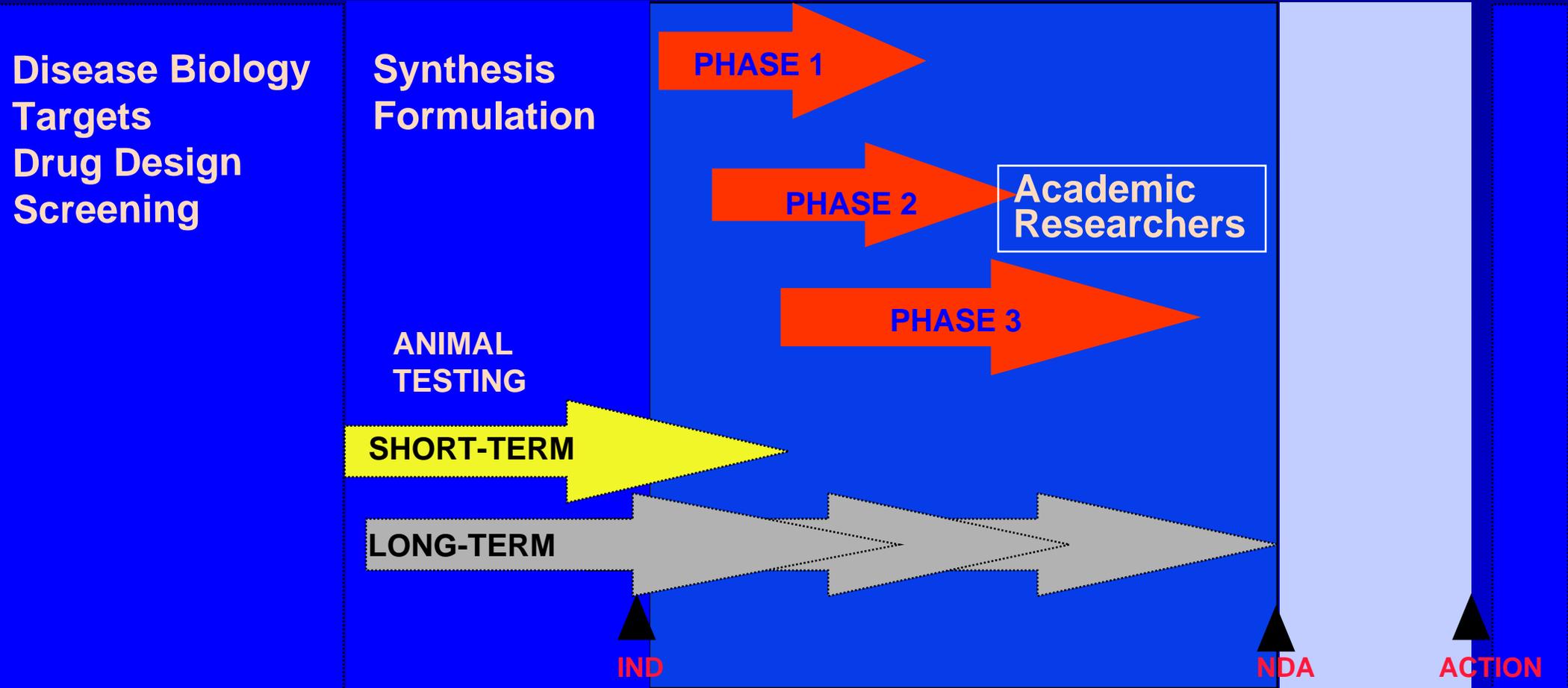
SHORT-TERM

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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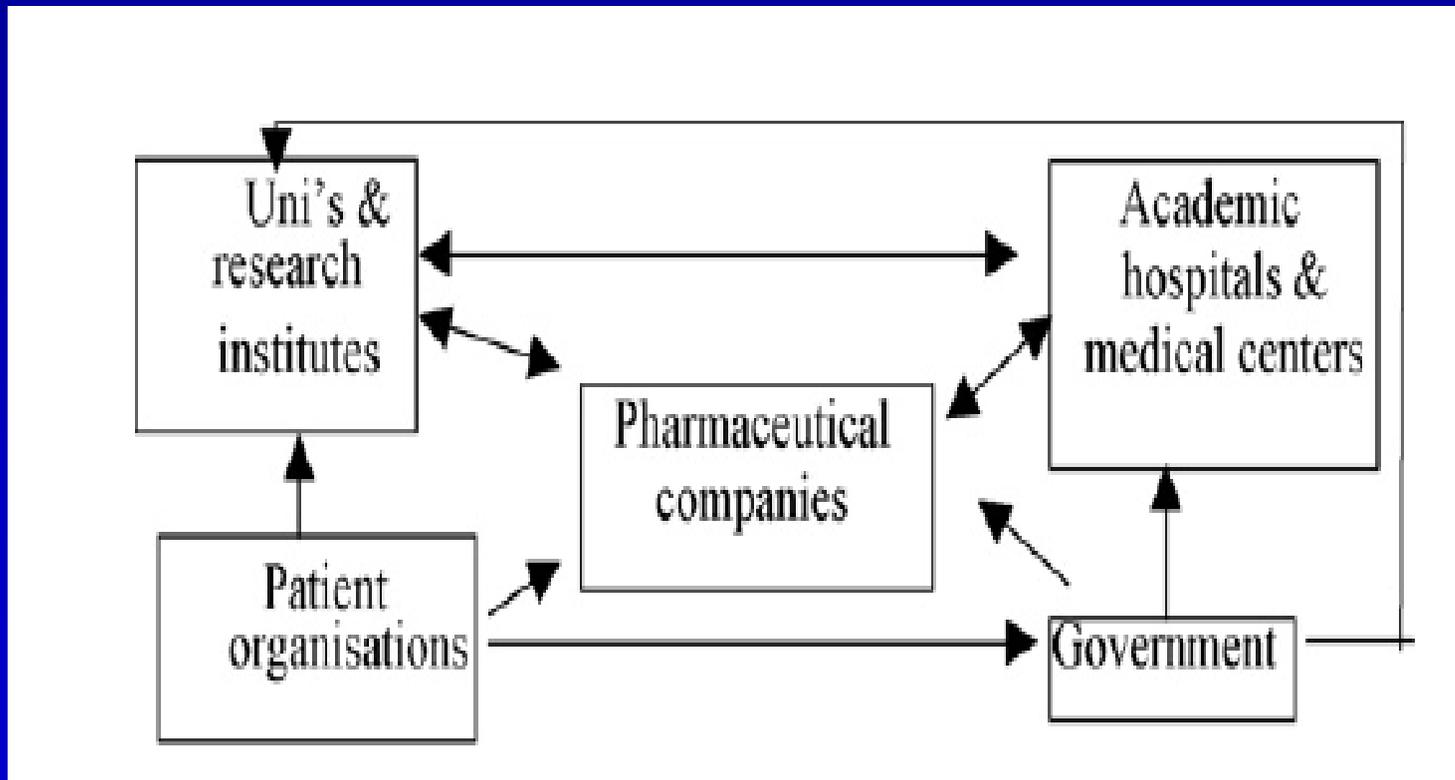
NDA

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.**