Developing Orphan Drug Products

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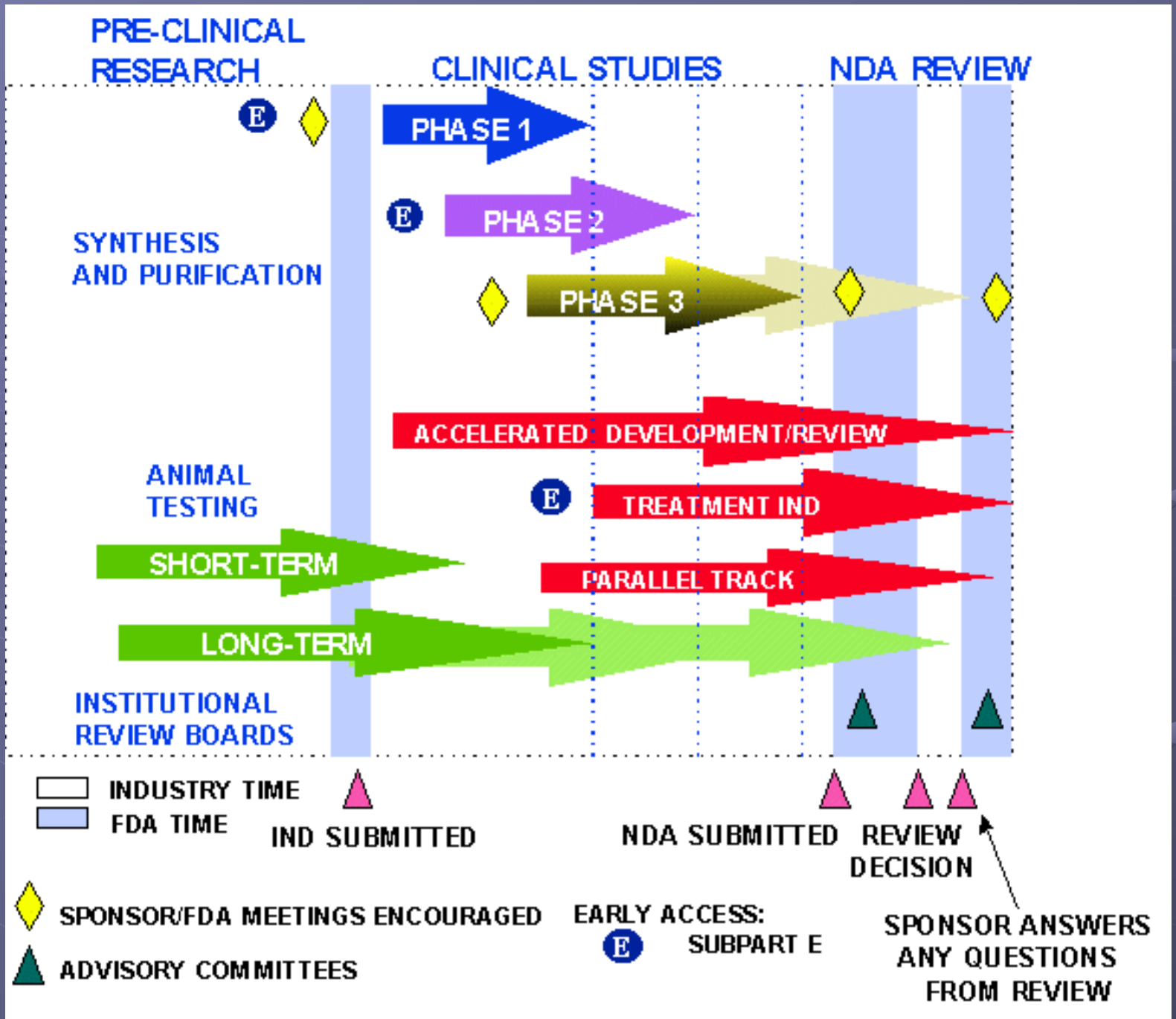
2nd International Conference on Rare Diseases & Orphan Drugs
Carlos III Health Institute
Madrid, Spain
October 25, 2006

Drug Development Process

- Discovery
- Preclinical Development (Animal Testing)
- Clinical Development (Human Testing)
  - Phase 1, Phase 2, Phase 3
- New Drug Application/Approval
- Post-marketing trials

Steps to Drug Approval

- Submission of IND
- Title 21, Code of Federal Regulations, Part 312
- Evaluation of toxicology data
- Evaluation of chemistry, manufacturing & controls (CMC)
- Clinical trials, phase 1, 2, and 3
- NDA


PRE-CLINICAL RESEARCH
CLINICAL STUDIES
NDA REVIEW
E
PHASE 1
E
PHASE 2
SYNTHESIS AND PURIFICATION
PHASE 3
ACCELERATED DEVELOPMENT/REVIEW
ANIMAL TESTING
E
TREATMENT IND
SHORT-TERM
PARALLEL TRACK
LONG-TERM
INSTITUTIONAL REVIEW BOARDS
INDUSTRY TIME
FDA TIME
IND SUBMITTED
NDA SUBMITTED
REVIEW DECISION
SPONSOR/FDA MEETINGS ENCOURAGED
ADVISORY COMMITTEES
EARLY ACCESS:
E
SUBPART E
SPONSOR ANSWERS ANY QUESTIONS FROM REVIEW

PHASE 1	PHASE 2	PHASE 3
20-100 Patients	Up to several hundred patients	Several hundred to several thousand patients
Testing mainly for safety	Testing for some short-term safety but mainly for effectiveness.	Safety, dosage, effectiveness

A New Drug Application (NDA) contains the following:

- Non-clinical studies
- Clinical studies
- CMC information
- Proposed labeling
- Additional information

OOPD/Review Division Interaction

Orphan Designation | **NDA**

OOPD | **FDA** | **Review divisions**

Assessments Follow-up ↔ **Category Experts** ↔ **Scientific Advice**

Interaction with Interested Parties ↔ **Rare Disease Experts** ↔ **Review of Application**

Result: **Orphan Designation**

Result: **Marketing Approval**

OOPD Web Site

http://www.fda.gov/orphan

Your Link to:

- Overview of the FDA Office of Orphan Products Development
- Guidelines for designation application
- List of designated and approved orphan products
- Grant application information
- List of ongoing orphan grant studies

Telephone contact: 301-827-3666 or 1-800-300-7469

Meet the OOPD Staff