

Developing Orphan Drug Products

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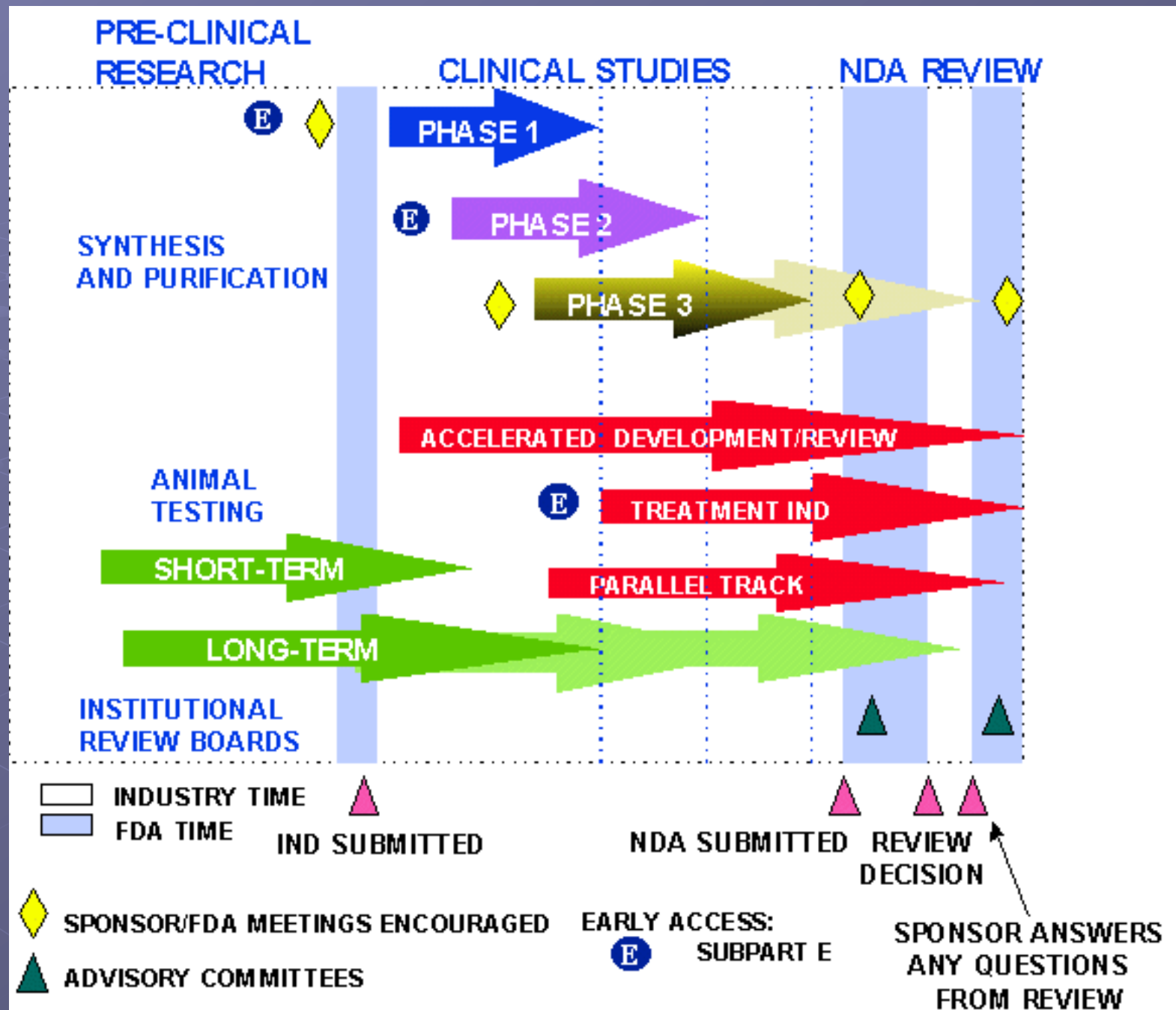
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Drug Development Process

- **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**



PHASE 1

20-100 Patients

Testing mainly
for safety

PHASE 2

Up to several
hundred patients

Testing for some
short-term safety
but mainly for
effectiveness.

PHASE 3

Several hundred to
several thousand
patients

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

