Getting Clinical Trial Approval and Running a Trial

Transforming Research Discoveries into Therapeutics and Clinical Impact

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Overview

- **Translational Therapy Development Pathway:** From Discovery to Clinical Translation
- **Development Stages:** Exploring Each Phase of the Translational Process
- **UCI Drug Discovery Platforms:** Integrated Infrastructure and Resources
- **Funding & Partnership Models:** Opportunities & Engagement Strategies

Vision & Opportunity: Transforming Academic Discovery

Core Capability

- UCI's integrated research and clinical trial infrastructure could transform discoveries into de-risked therapeutics for high-value markets

Target Markets

- Neurology & Neuropsychiatry ($50B+)
- Oncology ($200B+)
- Inflammation & Immunology ($100B+)
- Metabolic Disorders ($80B+)
- Rare Diseases ($50B+)

Unique Position

- Multi-modal: Small molecules, biologics, devices, diagnostics, cell therapies
- Active research in developmental therapeutics in multiple UCI laboratories, centers, schools and ORUs
- Global collaboration network
- Flexible engagement models

Strategic Opportunity

- Leverage validated targets and directed pipeline to translate therapeutic assets while building a self-sustaining, campus-wide engine delivering new therapies

Market Opportunity: $400B+ Pharmaceutical Market | $180B Medical Device Market | 6% Compound Annual Growth Rate

Breaking the Valley of Death in Drug Development

FDA Development Timeline

Pre-clinical: 3-6 years
Phase I: 1-2 years
Phase II: 2-3 years
Phase III: 3-4 years
NDA Review: 0.5-2 years

Total: 10-15 Years

Key Challenges

Time to Market
10-15 years (drugs)

Average Total Cost
$1-2.6B total
$1-5 M preclinical

Success Rate
<10% lab to market

Patent Life
8-10 years pre-revenue

NIH Translational Framework

Basic Research → Preclinical Research → Clinical Research → Clinical Implementation

The process by which laboratory results develop into new ways to diagnose and treat disease

UCI Translational Drug Development Pathway

1. **Disease Biology & Target Discovery**
   Faculty Labs
2. **Assay Development & Screening**
   Center for Neurotherapeutics Core (2M+ compound library)
3. **Hit to Lead & Lead Optimization**
   Medicinal chemistry (MIC)+ Drug Metabolism and Pharmacokinetics (DMPK/PIKA)
4. **Preclinical Evaluation & Regulatory Support**
   Drug Metabolism and Pharmacokinetics (DMPK/PIKA) + IND Office/Support
5. **Clinical Evaluation & Commercialization**
   UCI Health+ Beall Applied Innovation (BAI

Key Features

Multiple Entry/Exit Points
Partnerships at any development stage

Multi-Modal Applicability
Drugs, devices, diagnostics, combinations

Continuous Learning
Feedback loops between stages

Expert Coordination
PhD project managers managing timelines

FDA Defines…

What is Research?

Research means a **systematic investigation**, including research development, testing, and evaluation, **designed to develop or contribute to generalizable knowledge**.

45 CFR 46.102(l)

What is Clinical Trial/Investigation/Study?

An investigation or a research study in which one or more **human subjects** are prospectively assigned to one or more **interventions** (which may include placebo or other control) to evaluate the effects of the interventions on **biomedical or behavioral health-related outcomes**.

FDA Defines…

What is a Human Subject?

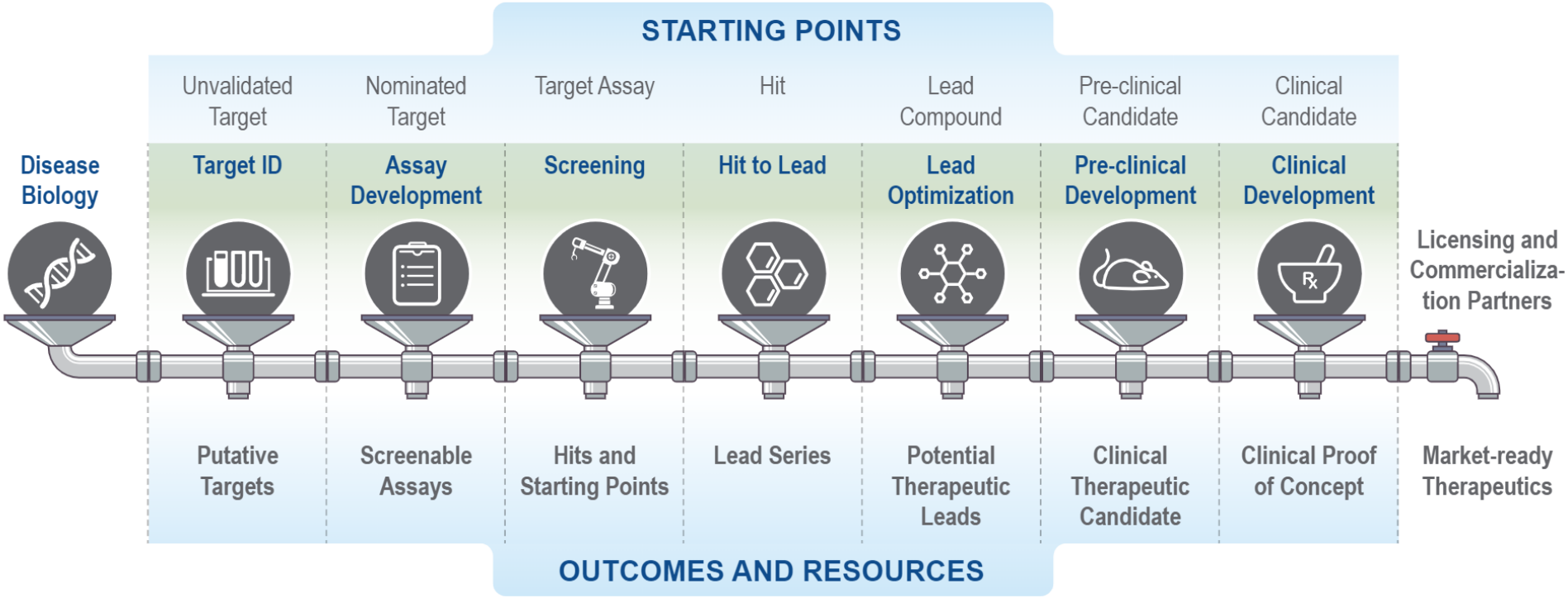
A living individual about whom an investigator (whether professional or student) is conducting research:

- Obtains information or biospecimens through intervention or interaction with the individual, and uses, studies, or analyzes the information or biospecimens; or
- Obtains, uses, studies, analyzes, or generates identifiable private information or identifiable biospecimens.

45 CFR 46.102(e)

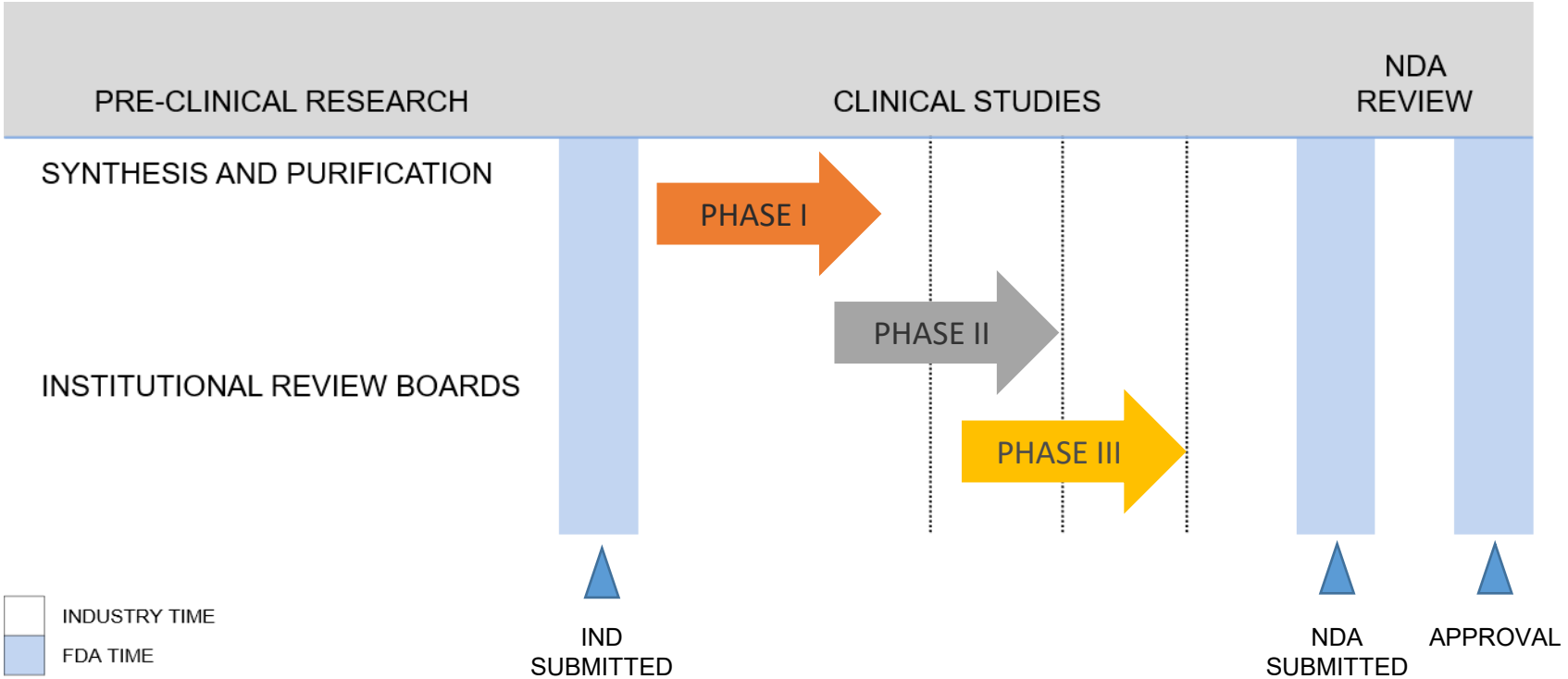
What is an Intervention?

The diagnostic or therapeutic device, biologic, and/or drug under investigation in a clinical trial that is believed to have an effect on outcomes of interest in a study.



SOURCE: https://www.niddk.nih.gov/research-funding/research-programs/translational-research-therapeutic-discovery-development

New Intervention Development Process



SOURCE (MODIFIED): https://www.fda.gov/drugs/cder-small-business-industry-assistance-sbia/new-drug-development-and-review-process

Drug Discovery: From Disease Biology to Clinical Candidate

Four stages of preclinical development before a clinical trial

1. **Disease Biology & Target Identification**

   Validate disease mechanism, identify a druggable target, establish biomarkers.

2. **Assay Development & Screening**

   Build robust screens (biochemical, cell-based); screen UCI / external compound libraries; confirm hits.

3. **Hit to Lead**

   Confirm hit activity, develop structure-activity relationships, optimize potency and selectivity.

4. **Lead Optimization**

   DMPK profiling, in vitro / in vivo safety, formulation, select clinical candidate, prepare IND-enabling studies.

Preclinical Evaluation: In Vivo and In Vitro Studies

Comprehensive preclinical testing to validate efficacy, safety, and pharmacokinetic properties in animal models

Pharmacology Studies

- Mechanism of action validation
- Dose-response relationship studies
- Target engagement confirmation
- Efficacy in disease models

Toxicology Assessment

- Acute and subacute toxicity studies
- Organ system evaluation
- Dose-limiting toxicities identification
- Margin of safety assessment

DMPK Evaluation

- Pharmacokinetic parameters in animals
- Bioavailability and exposure assessment
- Metabolite identification and safety
- Species comparison and scaling

Efficacy Validation

- Proof-of-concept in disease models
- Biomarker validation
- Dose optimization
- Ready for clinical translation

Regulatory Support & Pre-IND: Preparing for Clinical Development

Preparation of regulatory documentation and FDA engagement to enable IND submission and clinical trials

IND Documentation

- Chemistry, Manufacturing, Controls (CMC)
- Pharmacology and toxicology data
- Clinical protocol and safety monitoring
- Investigator's Brochure (IB) preparation

FDA Interactions

- Pre-IND meeting with FDA
- Regulatory guidance and feedback
- Address regulatory concerns
- Finalize IND application package

Regulatory Strategy

- Define clinical development pathway
- Establish regulatory milestones
- Plan for expedited programs (if eligible)
- Develop post-approval strategy

Readiness Assessment

- Complete IND-enabling studies
- Finalize manufacturing process
- Prepare clinical trial sites
- Ready for IND submission

Clinical Translation & Commercialization: From Trials to Market

Transition to clinical development through IND submission, clinical trials, and partnerships for commercialization

IND Submission

- Complete IND application package
- FDA submission and review
- Address FDA feedback
- Obtain IND clearance for studies

Clinical Trial Design

- Phase I: Safety and dosage
- Phase II: Efficacy and side effects
- Phase III: Confirmation and monitoring
- Protocol development and IRB approval

Partnership Development

- UCI Health clinical trial infrastructure
- Beall Applied Innovation partnerships
- Industry licensing opportunities
- New company formation potential

Commercialization

- Market analysis and positioning
- NDA/BLA preparation and submission
- FDA approval and market launch
- Post-approval monitoring and support

FDA Defines…

Who is the Investigator?

A person responsible for the conduct of the clinical trial at the trial site. If a trial is conducted by a team of individuals at a trial site, the investigator is the responsible leader of the team.

Who is the Sponsor?

An individual, company, institution, or organization that takes responsibility for the initiation, management, and/or financing of a clinical trial.

FDA Defines…

What is a Protocol?

A document that describes the objectives(s), design, methodology, statistical considerations, and organization of a trial. The protocol usually also gives the background and rationale for the trial, but these could be provided in other protocol referenced documents. A protocol amendment is a written description of a change(s) to or a formal change of a protocol.

FDA Defines…

What is Randomization?

The process of assigning trial subjects to investigational treatment or control groups (may use a comparator) using an element of chance to determine the assignments to reduce bias.

What are Randomized Controlled Trials?

A study in which randomization is used to assign patients to treatments.

The purpose of the randomized controlled trial is to:

- to guard against any use of judgment or systematic arrangements leading to one treatment getting preferential assignment; i.e., to avoid bias
- to provide a basis for the standard methods of statistical analysis such as significance tests.

Regulatory Agencies Overseeing Clinical Research

DHHS – Department of Health and human Services

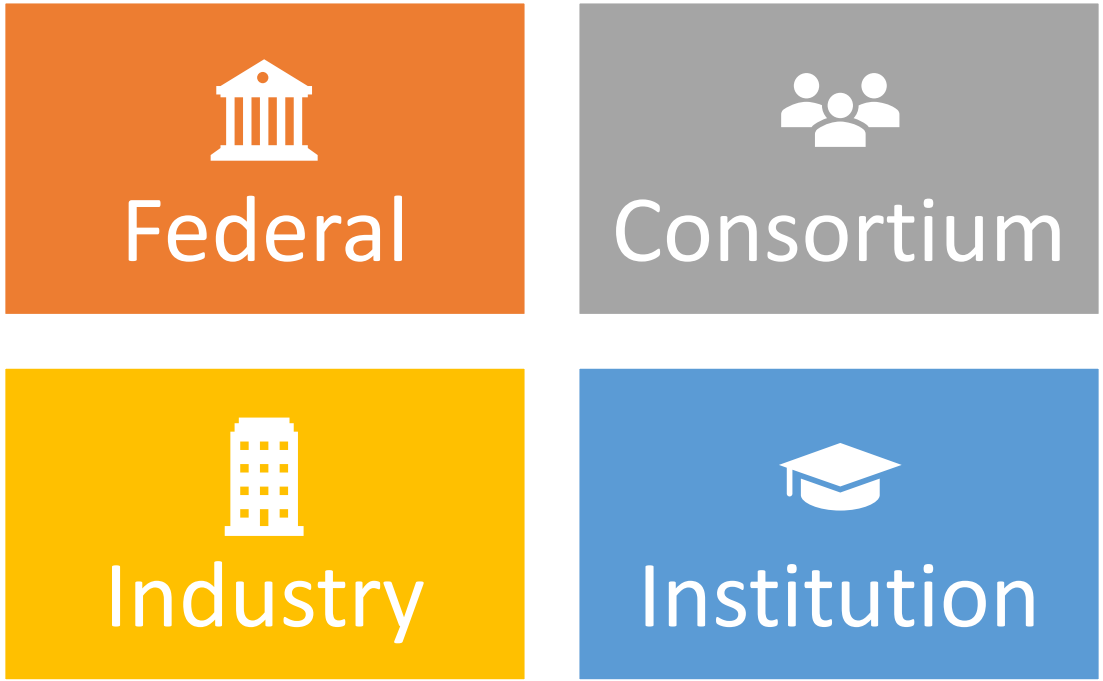
- Office of Human Research Protections (OHRP)
- Office of Research Integrity (ORI)

US-FDA – Food and Drug Authority

- Office of Regulatory Affairs
- Office of the Commissioner, Office of Clinical Policy and Programs, Office of Clinical Policy, Office of Good Clinical Practice
- Center for Drug Evaluation and Research
- Center for Devices and Radiological Health
- Center for Biologics Evaluation and Research

NIH – National Institute of Health

Clinical Trial Sponsor Types



UCI Discovery Platforms

Integrated Infrastructure & Resources

UCI Discovery Platforms

Discovery & Development

CNT - Center for Neurotherapeutics
Specialized neurological drug discovery platform
~2M compound library, 6 active programs

MIC - Molecular Innovation Center
Medicinal chemistry infrastructure and expertise
Lead optimization and structure-activity relationship studies

DMPK/PIKA Facility
Advanced analytical chemistry and pharmacokinetics
Triple-quadrupole MS facility

Tech Transfer - Beall Applied Innovation (BAI)
Licensing expertise
Commercialization support

Beall Applied Innovation (BAI)

IP Portfolio Management
Patents, Strategy, Freedom to operate

Market Assessment
Commercial viability, Competitive landscape

Partnership Development
Industry connections, Licensing, Deals

Technology Transfer
Startup incubation, Commercialization

Translational Research: Core Process

Preclinical Therapeutics
Drug candidate development and mechanism validation

Clinical Development and Strategic Market Analysis
Identification of strategic first indications and prospecting future licensing partners

Quantitative Imaging
Advanced imaging biomarkers and analysis

Laboratory & Biomarker Analysis
Molecular and clinical biomarker identification

IND/IDE Regulatory Unit
Specialized regulatory pathway management and FDA coordination

Phase 1 Clinical Trial Unit
First in human clinical trials, PK/PD, early effectiveness signals

Supporting Infrastructure
Academic and industry partnerships to accelerate translation and de-risk development programs

Customized Engagement for Every Development Stage

Three Service Tiers

Consulting Only

Lead Candidate:
- Target validation
- Disease ID
- Mechanism studies
- Market analysis

Features:
- Shared IP development
- Co-funding model
- Joint teams
- Milestone-based

Collaborative Partnership

First in Human:
- Protocol design
- Regulatory strategy
- Site selection
- Biomarker strategy

Features:
- Co-development
- Shared trial costs
- Joint regulatory
- Split commercial rights

Full Service

Established Candidate:
- PI identification
- Trial optimization
- Data analysis
- Publication strategy

Features:
- Complete IND package
- All preclinical work
- Data mining
- cGMP (planned)

Flexibility to match partner needs and risk tolerance

Integrated Biomarker Development Platform

Discovery & Development

- Genomic profiling (WGS, RNA-seq, single-cell)
- Proteomic analysis (mass spec, immunoassays)
- Metabolomic profiling
- Circulating tumor DNA/RNA
- Imaging biomarkers

Validation & Clinical Implementation

- CLIA certification pathway
- Companion diagnostic development
- Clinical trial integration
- Regulatory submission support
- AI/ML predictive modeling

Applications Across Development

- Patient stratification
- Efficacy monitoring
- Safety biomarkers
- Dose optimization
- Resistance mechanisms

Strategic Partnerships

- UCI Genomics High-Throughput Facility
- Clinical Laboratory
- Pathology Core
- Bioinformatics team

UCI Health Clinical Trial Capabilities

Phase I Unit

12-bed dedicated unit
24/7 monitoring capability
PK/PD sampling
Dose escalation expertise
Safety run-in design

Phase II/III Capabilities

Active trials annually:

Patient catchment: 30,000+

Diverse population:

- Specialty clinics in all target areas
- Satellite sites for expanded access

Unique Strengths

Core: Patient identification, protocol optimization
Imaging Core: Advanced quantitative imaging, novel endpoints
Biospecimen Banking: 100,000+ sample repository
Regulatory: 95% FDA inspection pass rate
Network: NCTN, NCCN member

Differentiators

Cell therapy capabilities
Natural products expertise
Device/drug combination trials
Real-world evidence generation

Intellectual Property & Funding Strategy

Standard Income Split

35% Net Licensing Income to Inventors

15% School of Medicine (SOM) Development

50% UCI General Fund

Payment Schedule

Annual accounting and payment with SOM development costs reimbursed first

IP Funding Flow

1. Project Screening & Assessment
2. Memorandum of Understanding (MOU)
3. Invention Disclosure & Patent Filing
4. Patent Protection & Prosecution
5. Licensing Negotiations & Agreements
6. Revenue Distribution & Payment

Patent Cost Timing & Strategy

US Provisional Patent

Cost:

$4,000

Timeline:

12 months

Entry point for patent protection with minimal investment

PCT Coverage

Cost:

$15–25K

Timeline:

30 months

International Patent Cooperation Treaty filing for global reach

National Entry

Cost:

$100K+

Timeline:

Ongoing

Full global protection across multiple jurisdictions

Strategy

Delay patent filing until before first enabling publication. Align publication timing with IND (Investigational New Drug) submission to maximize patent value and regulatory advantage while maintaining competitive positioning.

Questions?