NIH-RAID Program
(Rapid Access to Interventional Development)
PAR-09-027

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NIH-RAID Program
Position in Drug Development Pipeline

Basic
- Target ID
- Assay
- Screening

Translational
- Proof of Concept
- Lead Optimization
- Preclinical Studies

Clinical
- Clinical Trials

Access to contract services
- Synthesis
- Formulation
- In vivo PK and Tox studies
- Product development planning

NCI-RAID/NExT
T1D-RAID
NIH-RAID
NIH-RAID Program Overview

• The NIH Roadmap established NIH-RAID Program to make available, on a competitive basis, certain critical resources needed for the development of new therapeutic agents
• Applications are accepted for the development of therapies for all diseases
• Lead by OSC, NINDS & NIAMS, project team
• Began accepting applications in 2005
NIH-RAID Program Overview

• X01 Resource Access Award
• Approved projects are provided access to the expertise and contract resources of NCI and NHLBI programs
• Open to domestic and foreign academic and non-profit institutions, as well as SBIR-eligible businesses, and NIH Intramural programs
• Small molecules, peptides, oligonucleotides, natural products, gene vectors, monoclonal antibodies and recombinant proteins are eligible for development
• IP retained by owner
• R&D costs split 50/50 between Roadmap and ICs (IR dollars must be used for IR applicants)
NIH-RAID Services

• Synthesis in bulk of small molecules
• Synthesis of oligonucleotides
• Chemical synthesis of peptides
• Synthesis of gene vectors
• Scale-up production
• Development of analytical methods
• Isolation and purification of natural products
• Development of suitable formulations
• Manufacture of clinical trial drug supplies
• Pharmacokinetic/ADME studies including bioanalytical method development*
• Range-finding initial toxicology*
• IND-directed toxicology*
• Product development planning and advice in IND preparation
Entry Points

• Lead Molecule Identified
  – NIH-RAID X01 does not provide lead optimization or animal efficacy services

• Lead Molecule Not Identified
  – Scale-up Synthesis
  – Preliminary PK/Tox
Application & Approval Process

- Three receipts per year
- CSR review
- Preliminary cost estimate from NCI/NHLBI
- Investigator seminar, if warranted
- Preparation of tasks, timeline, milestones, and costs by NCI
- Funding decisions by co-sponsoring ICs
- Applications do not go to council
- Total time from submission to approval: 10+ months
- Independent Product Development Plans available
Project Management

• NIH-RAID Material Transfer Agreements
• IC transfers funds to the NCI or NHLBI
  – 50% RM and 50% IC funds
• Projects typically last two years
  – Sequential development
  – Spreads costs across fiscal years, out years committed
• Monthly progress meetings and reports
• ICs in control at decision points
  – Go/no go
  – Insufficient funds
Results to Date

• 105 total applications submitted since 2005
  – 17 approved
  – 29% approval rate

• Status of approved projects
  – 7 completed projects
  – 5 successful INDs (not all projects lead to IND)
  – 10 active, 2 additional INDs expected by Q1 2010

• 10 ICs have co-funded a project
Examples of Approved Projects

- Development of HGF Mimetic (Refanalin) for Hepatic Fibrosis
- Inhibitors of Glutaminase 2 as Therapeutic Agents for Neuro-Oncological Diseases and Celiac Sprue
- Safety Pharmacology Studies for an IND for Beta Thalassemia
- Metastin Administration in Humans: Preclinical Toxicology Studies
- Redox Encrypted Therapeutics for Treatment of Friedreich’s Ataxia
- Advanced Studies with 5HMF – Potent Antisickling Agent
- Preclinical Development of CDD-0102 Treatment of Alzheimer’s disease
- cGMP Synthesis of Selective Kappa Opioid Receptor Antagonist JDTic
Efficacy Administrative Supplements

- Jan/Feb 2010 receipt date
- Any research project (Rs, Ps, Us) with a year of funding remaining
- In vitro or in vivo efficacy
- $2M Budget
  - 100% Roadmap funds
  - 50K direct costs
  - up to 25 awards made
Future Funding

- X01 Applications accepted through Sept 2011
- Approved projects completed through 2013
- Efficacy Supplements accepted in FY10 and FY11
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