Type 1 Diabetes-Rapid Access to Intervention Development

A collaborative effort between NIDDK and NCI

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NIDDK and NCI

- NCI has a long history of providing drug development resources to their research community through their RAID program
- T1D RAID program set up in 2003 following their procedures. It is a NIDDK-NCI collaboration.
- All actual development work is done through NCI's contracts and managed through NCI staff.
- Subsequently, Roadmap initiated NIH RAID which supports development, but not manufacturing of biologics

RAID programs

- NCI RAID—original program, IC has staff with preclinical drug development expertise
- T1D RAID—initiated in 2003, specifically supported by special congressional appropriation for T1D
- NIH RAID—initiated in 2004/5 as part of roadmap

T1D-RAID

- Is a mechanism for investigators to obtain access to pre-clinical development resources for their potential new therapeutic for type 1 diabetes and its complications

- Is NOT a grant process—no dollars are available for investigators

Eligibility

- Academic Institutions
- Non-profit research institutions
- Biotechnology and pharmaceutical companies
- U.S. and non-U.S. entities

What Type of Resources

- Conduct of pharmacology studies with a pre-determined assay
- Acquisition of bulk substance (GMP and not)
- Scale-up production from lab-scale
- Development of suitable formulations
- Development of analytical methods for bulk substances
- Stability assurance of dosage forms
- Range finding initial and IND-directed toxicology
- Planning of clinical trials
- Regulatory Affairs and IND Filing Advice

Requests

- Submission dates are April 1 and Nov 1

  (letters of intent encouraged 1 month in advance, flexibility in submission dates)

- Components of the Request
  - 15 pages
  - Hypothesis to be tested
  - Novelty
  - Specific request for services
  - Criteria for successful outcome
  - R&D plan
    - Synthesis route
    - Desired specifications for product, i.e. concentration, purity, etc

External Review

- Panel of external experts from academia and industry

- Review Criteria
  - Strength of hypothesis
  - Novelty
  - R & D plan

Internal Review

- Needed to manage available resources

- Criteria
  - NIH programmatic issues
  - Portfolio diversification
  - Projected cost of each aspect of the highly scored projects

T1D-RAID Program—Progress to Date

1. First requests received in Nov 2003
2. 13 cycles of receipt and review so far
3. Two main areas of focus:
   - Immunologic products for prevention and treatment of type 1 diabetes
   - Treatments for diabetic complications

Immunologic products for prevention and treatment of type 1 diabetes

- 15 total requests (5 of those were re-submissions
- 4 recommended with high priority by review
- 3 supported (one with insufficient IP access)
  - 1 in clinical trials
  - 1 product's IP acquired by a company
  - 1 undergoing final manufacturing and toxicology studies

Treatments for diabetic complications

1. 15 total requests (7 of those were resubmissions)
2. 3 recommended with high priority by review
3. 2 supported (one refused to sign NIDDK required agreements)
   - 1 for diabetic neuropathy terminated early due to inability to reproduce efficacy results
   - 1 for diabetic nephropathy is just starting
4. 6 projects referred to NIDDK supported contracts to collect more efficacy data in animal models of diabetes complications (one of those just recently selected for full support)

Summary

- T1D RAID provides hard to find resources to support the needed development steps to be able to get regulatory approval to conduct proof of concept clinical trials
- T1D RAID provides review and, when needed, prompt referral to resources for collection of additional efficacy data
- T1D RAID is available as long as special congressional appropriation for type 1 diabetes continues