NCI SBIR “HIGH-RISK HIGH-REWARD” INNOVATIVE CONCEPT AWARD

RFP Concept Review
Presented to BSA

Presented by
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May 11, 2020
THREE-PHASE PROGRAM

PHASE I
- Proof-of-Concept
- Up to $400K over 6 to 12 months

PHASE II
- Research & Development
- Commercialization plan required
- Up to $2M over 2 years

PHASE III
- Technology validation & clinical translation
- Follow-on funding for SBIR Phase II awardees from any federal agencies
- Expectation that applicants will secure substantial third party investor funds
- $4M over 3 years

DIRECT TO PHASE II

FAST-TRACK
(PH I & II)

NCI SBIR PHASE IIB BRIDGE AWARD
Crossing the Valley of Death

- Commercialization stage
- Use of non-SBIR/STTR funds
SBIR/STTR vs. ACADEMIC GRANTS

**ACADEMIC GRANT**

- **Expertise/Team** (Discovery Research)
- **Approach** (Appropriate for discovery)
- **Product** (optional)
- **Significance** (Knowledge increase)
- **Innovation** (What could be/theoretically possible)
- **Environment**

**SBIR/STTR GRANT**

- **Expertise/Team**
  - Phase I: Research and Development
  - Phase II: Product Development
- **Approach** (Product Development)
- **Science** (MANDATORY)
- **Innovation** (Competitive Advantage)
- **Significance** (Changing a paradigm)
- **Commercialization**

**SCIENCE**

**PRODUCT**
NCAB WORKING GROUP REPORT ON THE NATIONAL CANCER INSTITUTE SMALL BUSINESS INNOVATION RESEARCH PROGRAM

February 2019

Elizabeth M. Jaffee, M.D.
Deputy Director
The Sidney Kimmel Comprehensive Cancer Center
The Dana and Albert “Cubby” Broccoli Professor of Oncology
Co-Director, Skip Viragh Center for Pancreas Cancer
Johns Hopkins University

Mel Billingsley, Ph.D.
President & CEO
Life Science Greenhouse of Central Pennsylvania
Professor of Pharmacology
Pennsylvania State University
Milton S Hershey College of Medicine
PRIORITY GOALS FOR NCI SBIR/STTR

❖ Implement SBIR “Concept grant”

❖ Use supplements to advance companies to value-creating milestones

❖ Develop FDA regulatory assistance program

❖ Develop postdoctoral training program in entrepreneurship and tech transfer

❖ Continue and enhance metrics collection

❖ Promote diversity

❖ Reduce time-to-award for SBIR contracts

❖ Increase Phase I award size
WORKING GROUP RECOMMENDATION

Launch SBIR Concept Award

- Support high-risk/high-reward technologies in targeted areas
- De-risk projects
- Short applications
- Preliminary data are not required.
- Make awards rapidly (within six months).
- Leverage I-Corps at NIH Program
- Followed by another Phase I, Direct to Phase II or Fast-Track
Encourage small businesses to develop:

- **high risk/high impact** technologies
- Disruptive innovation
- Pre-SBIR/Phase “0”
- Product-focused projects
- Focus areas
  - pediatric or rare cancers.
**FOCUS ON INNOVATION**

<table>
<thead>
<tr>
<th>What is innovative?</th>
<th>What is NOT innovative?</th>
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<tbody>
<tr>
<td>• Development of ground-breaking new products, technologies or tools</td>
<td>• Therapeutics targeting known pathways with FDA-approved agents</td>
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<tr>
<td>• Disruptive innovation rather than incremental innovation</td>
<td>• Technologies in clinical stage or already far down the development pathway</td>
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<tr>
<td>• Transformative technologies with the potential to change clinical care</td>
<td>• Continuation of already funded SBIR/STTR projects</td>
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CONCEPT AWARD: NCI SBIR CONTRACTS

Purpose
Support small businesses developing highly innovative and transformative technologies that have the potential to create new scientific paradigms, establish entirely new and improved clinical approaches to significantly improve cancer research, prevention, detection and care for pediatric or rare cancers.

Important Points
- Short Application
- Faster Turn around (<6 months)
- No preliminary data required
- Applicants encouraged to go through I-Corps at NIH
- Followed by another Phase I, Direct to Phase II or Fast-Track

FOA
- RFP- 3 year pilot
- Phase I only, clinical trials not allowed
- Only one receipt date per year in pilot period
- Estimated awards per year/per round: 5-10
- Estimated cost per year: $1.5M - $3M

Budget
- $300K total costs
- 1 year award

Eligibility
- Must be a small business
- At least 66% of the work must be done by the small business
PILOT FOCUS AREA: PEDIATRIC CANCERS

- Only 12 were new molecular entities intended to treat cancer seen primarily in children in last 25 years.
- Private investment in pediatric cancer is low.

### NCI SBIR Awards focused on pediatric cancer (2010-2018)

<table>
<thead>
<tr>
<th>Type</th>
<th>Number of Competing Awards</th>
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<tbody>
<tr>
<td>Can be used for pediatric cancer</td>
<td>26</td>
</tr>
<tr>
<td>Specifically for pediatric cancer</td>
<td>20</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td><strong>46</strong></td>
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PILOT FOCUS AREA: RARE CANCERS

Number of Competing Awards

• Rare cancer defined as: [www.rare-cancer.org/info/raw-adult-list.php](http://www.rare-cancer.org/info/raw-adult-list.php)
### Examples of Projects/Activities

#### Therapeutics
- New mechanism of action
- New targets

#### Devices
- Innovative drug delivery tools

#### Diagnostics
- AI Driven prognostics/diagnostics tool
WHY SBIR CONTRACT MECHANISM?

• Application
  • Shorter proposal (up to 15-20 pages max with up to 3 pages for research strategy)
  • Modify proposal (application) components
  • 1-2 page **Letter of Intent** to be reviewed for responsiveness by NCI PDs

• Review
  • Focus on Innovation/Special Review Criteria and Panel
  • Ability to modify review criteria weightage unlike the omnibus grant mechanism
  • Assess scientific rationale given the preliminary data
  • **NCI DEA Special Review Panel** with mix of academic industry venture and biotech

• Milestone-driven
  • Quarterly reporting
  • Payment based on achieving milestones
February 2020: Present to SPL
May 2020: Present to BSA
June 2020: RFP Opens
July 2020: LOI Due
August 2020: Invitation to Apply
October 2020: Proposal Due Date
November 2020: Review
March 2021: Award
THE END
EXAMPLE : REFLEXION MEDICAL

See & Treat Mechanism

Treat multiple tumors or metastasis in one treatment session

NCI SBIR funded them at early concept stage with no preliminary data.

Right now raised over $100M, will be in clinic in the next 2 months

Excellent team

Biologically Guided Radiation Therapy - Combine PET Imaging and LINAC
Examples of Activities & Deliverables

**Budget:** Phase I $300,000 for up to 9-12 months

**Activities & Deliverables could include:**

- Identify and define the clinical need that the product or technology will address.
- Obtain feasibility data/ proof of concept data that the proposed product or technology can solve a significant unmet need in pediatric/rare cancers.
  - Therapeutics: Validation of a novel target; identification and development of a lead compound; *in vitro* and/or *in vivo* efficacy studies
  - Medical Devices: Evaluation and validation of clinical need; development of a prototype or minimal viable product; phantom and/or *in vivo* safety and/or efficacy studies
  - Diagnostics: Biomarker discovery and validation; assay development and optimization; define assay performance and analytic validation
- Identify next steps and develop a product development plan (to be pursued under a future SBIR Phase I or Phase II award)