Date Prepared: February 05, 2010

REQUEST FOR EC/BSA CONCEPT APPROVAL
REQUESTS FOR APPLICATIONS (RFAs)/CONTRACTS (RFPs)

Title: Commercial Application and Use of Emerging Molecular Analysis Technologies (SBIR R43/R44)

PA ___ RFA X Coop. Ag. ___ Activity Code (e.g. R01) R43/R44 RFP___

New X Reissue__

Division:

SBIR Development Center,
Immediate Office of the Director

Program for Innovative Molecular Analysis Technologies,
Office of Biorepositories and Biospecimen Research

Program Director: _____
Deepa Narayanan SBIR Program Director

Division Director: _____
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Length of Award (Yrs.): 1-3

Anticipated Award Date: December 2010

Source of Funds: RPG ___ Control ___ Centers____

Other Res:_____ Construct _____ NRSA _____

SBIR Set-aside: X

RFAs (Set Aside):

Amount of Set Aside in 01 Yr.: $2 M

One Time Issuance (Y/N): N

Est. Number of Awards : 5-7

Est. Cost for Project Period: $4 M

SBIR RFA for Commercial Application and Use of Emerging Molecular Analysis Technologies

Executive Summary

The NCI Small Business Innovation Research (SBIR) Development Center, in partnership with the NCI Program for Innovative Molecular Analysis Technologies (IMAT), proposes the issuance of an SBIR RFA for the Commercial Application and Use of Emerging Molecular Analysis Technologies. The overall goal of this award is to support the further development of prototypes for emerging analytical technologies with a focus on commercialization-relevant milestones. The proposed funding opportunity would supplement the goals of related initiatives from the IMAT program, and would be open to any investigator interested in commercializing their technology through a small business concern.

The SBIR Program represents a key translational vehicle for NCI to fund innovative technology development by the private sector in a range of areas critical to cancer research including new therapeutic agents, diagnostics, and imaging technologies. Per legislation from Congress, 2.8% of the NCI budget ($108M) is set aside annually to fund technology projects under this program. The SBIR Program requires a special skill set in that its success hinges on its ability move technologies from the lab to the marketplace and ultimately out to cancer patients. In order to meet the needs of this program, Dr. Niederhuber consolidated management of all NCI SBIR awards into a single office called the "SBIR Development Center." This Center is now staffed by scientists with wide-ranging technical expertise and extensive industry experience, including hands-on management of technology development for the purpose of commercializing a new product or service. The NCI SBIR Development Center recommends setting aside $2 M **(from the overall SBIR program set-aside)** to fund 5-7 new awards under this RFA in FY11.

The IMAT program is a comprehensive trans-divisional grants program focused on cancer technology development and is coordinated across NCI's divisions by the Office of Biorepositories and Biospecimen Research in the Office of the Director, NCI. As a program that covers a very broad scope of topics, applicants identify the unmet need in cancer research and medicine, and propose an innovative technological approach for addressing that need. IMAT's current R21 and R33 funding opportunities cover the "pre-technology transfer" segments of the pipeline, allowing investigators to obtain the necessary technical data to demonstrate the inception and validation of an innovative cancer technology. Since this program is solely focused on the scientific development of an innovative technology – regardless of whether its end-goal is transfer to the commercial sector or to further hypothesis-based research – it is beyond the scope of IMAT's current funding opportunities to support investigators seeking to translate their prototypes to commercially-relevant products and services. The proposed SBIR funding opportunity would address this need and thus would be critical in supporting the development of early-stage emerging technologies towards commercial dissemination, such as those that have been developed and validated with NCI funding through IMAT.

Proposed here is the establishment of an SBIR funding opportunity to further support the development of an emerging molecular or cellular analytical technology suitable for the detection, isolation, or characterization of cancer-related characteristics towards commercialization through a small business concern (SBC). The investigators applying to this opportunity should be in pursuit of proof-of-concept data in context of the commercial end-use of their technology and will be strongly encouraged to have preliminary validation data reflecting that their initial prototype is technically feasible. The proposed FOA would allow investigators to obtain commercially-relevant feasibility data that would be necessary to validate their prototype for market entry by, for example, meeting milestones demonstrating the ability to scale-up.

1. Background

In 1998, the National Cancer Institute (NCI) recognized the importance of innovative approaches for novel cancer technologies and created the program for Innovative Molecular Analysis Technologies

(IMAT) as an investment in the potential of innovation to revolutionize its mission for reducing the burden associated with cancer. The emphasis of IMAT is on supporting high-risk and high-payoff innovations, which if successful, would transform cancer research and medicine. The intent of the program is to catalyze the development of a technology that at the time of its inception may have little to no data demonstrating its technical feasibility. IMAT is a trans-divisional program, allowing individual investigators to access the resources at the NCI and direct the application of their technologies in the areas of cancer biology, prevention, therapy and detection, cancer control and epidemiology, or as tools used for the reduction of cancer-associated disparities.

Recognizing that most innovative ideas cannot be anticipated, IMAT's philosophy is to allow the individual investigator to identify the unmet need in cancer research and medicine, and define how the use of their proposed technology will address that need. To jumpstart and advance innovation, IMAT utilizes the NIH R21 (standard and modified) and R33 funding mechanisms to support the inception, development, and validation of an innovative or emerging cancer technology to the prototype stage. IMAT divides technology development into two phases. Innovative technologies are platforms that have the potential to revolutionize the current state-of-the-science and have not yet been fabricated, and emerging technologies are platform technologies that have not yet demonstrated feasibility in an intended use or application. Successful investigators who have completed the goals outlined in their terminal R33 IMAT funding opportunity, "Application and Use of Transformative Emerging Technologies in Cancer Research" (RFA-CA-09-007 for FY09, RFA-CA-10-003 for FY10), should have developed a prototype that has data supporting technical feasibility toward a specific application.

By focusing on the technical development of a prototype, IMAT support allows the investigator to obtain pre-tech transfer feasibility data regardless of whether the end-goal is widespread dissemination via licensing/intellectual property protection or through publication of the methodology to advance hypothesis-based research. For those investigators interested in the commercial dissemination of their IMAT-supported technology, some already represent a small business' interests and others have obtained enough confidence in their technology to consider the inception of a small business. IMAT has a history of investigators who have disseminated their technologies to the commercial market, such as Raindance Technologies and Affymetrix Chips, as well as those who have used their technologies to successfully compete for larger grants in the pursuit of hypothesis-based research. An examination of IMAT's active R21/R33 grants in October, 2009 (n=117) has shown that 5% of the R21's and 3% of all funded R33's are from commercial interests (small, medium, and large enterprises), with an unknown number of academic researchers who have yet to decide on the commercial feasibility of their technology. As shown in Section 3, 15% of all applications (n=125) submitted to IMAT's September 2009 receipt date were affiliated with a commercial interest (i.e., a non-academic, for-profit organization).

Except for the NIH Omnibus Solicitation for SBIR Grants, there is a dearth of available funding opportunities from the NCI to support the commercial development of broad-based, investigator-initiated molecular analysis platforms. IMAT's current scope is limited to the development of a technology to point of validating a prototype, rather than the support of commercial validation activities, such as scale-up and multi-site evaluation. Thus, NCI's current landscape for supporting the translation of molecular analysis technologies has a significant funding gap for those who have developed an emerging technology through IMAT's pipeline, but have not yet mitigated sufficient technical risk to attract downstream financial support from the private sector.

The RFA proposed here would target those technologies whose development has already been supported, or would have been supported, with NCI divisional funding through the IMAT program. This partnership between the NCI IMAT program and the NCI SBIR Development Center would provide an additional level of support that is broad in scope for the further development of IMAT-related technologies, with a focus on preparation for entry to the commercial market. By allowing

technology innovators to obtain market-relevant feasibility data, this opportunity would reduce the risk inherent in their technologies and thus accelerate its dissemination and widespread access to the research and clinical communities via the commercial market. Importantly, IMAT continues to receive competitively scored applications that are responsive to its R33 solicitation but appear more appropriate for SBIR funding; therefore, this proposed funding opportunity would free up a portion of the current IMAT R21/R33 set-aside, so that it can continue to support the development of innovative technologies at its earliest stages. Conceivably, all of the projects that are responsive to IMAT's R21/R33 solicitations, when mature, could feed directly into the SBIR pipeline, helping to fulfill the NCI SBIR program's mission of supporting the development of a broad array of commercial products for the detection, diagnosis, treatment, and prevention of disease.

2. Purpose of RFA

The advance of personalized cancer medicine is dependent on the detection of specific molecular signatures indicative of a patient's state of health. This advance is limited by the types of technologies that are available to the researcher and clinician, with an increasing need for novel analytical tools that have greater resolution, specificity, and/or throughput. The purpose of this funding opportunity announcement (FOA) is to support the continuation of technology development projects in these areas that have produced a validated prototype but still lack data supporting its ability to be commercialized, such as those supported by an IMAT R33 in the area of "Application and Use of Emerging Technologies in Cancer Research" solicitation (RFA-CA-09-007).

Investigators applying for this opportunity would be interested in the dissemination of their technology to the commercial market through a small business concern. Since these technologies have already demonstrated technical validity, this FOA would further their development by supporting the pursuit of commercially-relevant milestones. NCI support is thus intended to maximize the return on the Institute's initial investment in these projects by accelerating the commercial dissemination of these novel biomedical technologies.

Eligibility

Applicants responding to this proposed FOA will be encouraged to already have a novel cancer technology that has been technically validated for use in a particular application, but need to further develop their technology beyond the prototype stage. These technologies could have been developed with support from any funding source, such as the NCI IMAT program. These prototypes should have demonstrated enough technical feasibility to be transferred for licensing/intellectual property protection, or could be used in support of hypothesis based research. This proposed opportunity would allow them to accelerate the development of these technologies by obtaining commercial metrics of feasibility, so that the technologies would be more attractive to private investors.

Applicants that have completed a Phase I application through another SBIR/STTR program announcement are eligible to submit either a combined Phase I or II application for the Fast Track application or a Phase II application under this RFA.

Technical Scope

This funding opportunity will cover a broad range of topics relevant to the IMAT program, where the complexity and scope of the molecular analysis technologies being proposed cannot be anticipated and are appropriate for NCI's strategic interests. As this FOA is intended to encourage commercial development of prototype analysis technologies, achieving key commercially-relevant milestones will be strongly emphasized.

3. SBIR Development Center

The NCI SBIR Development Center was established in 2008 with the goal of consolidating the management of all SBIR and STTR awards into a centralized office. The Center was established to provide a strategic focus to the program and to ensure that NCI had the right expertise in house to manage the SBIR program. The SBIR DC is staffed by scientists with wide-ranging technical expertise and extensive industry experience, including hands-on management of technology development for the purpose of commercializing a new product or service. As part of this effort, Center staff work closely with small business awardees to assist them with commercialization strategies, as well as in developing plans for leveraging their Federal SBIR/STTR funding to negotiate strategic partnerships with industry that will be necessary to reach their commercialization goals. Through the SBIR Development Center, it is possible to support small businesses that are in the area of molecular analysis technologies to go beyond the prototype phase to commercial product development phase. These businesses are also able to take advantage of the various services provided by the NCI SBIR Development Center to accelerate the commercialization of their technologies such as providing Bridge funding after SBIR Phase II and tapping into the Development Center's network with investors and strategic partners.

Awards under this RFA will be managed by the SBIR Development Center. The recommended budget of $2M for the first year will be from the overall SBIR set-aside.

The partnership between IMAT and the SBIR Development Center

This FOA will be a close partnership between IMAT program staff and the SBIR Development Center. Assistance will be provided in drafting the FOA, publicizing the FOA to all IMAT investigators, inviting SBIR investigators to participate in the annual IMAT PI meeting, referring IMAT applications that are appropriate for this targeted FOA, and in ensuring a seamless transition for applicants that are transitioning from IMAT to SBIR. The proposed FOA would reflect different responsiveness criteria from IMAT's current FOAs, with substantial emphasis on pursuing commercialization-relevant metrics (see Appendix 1). Pending approval, the SBIR Development Center will work in close collaboration with both IMAT program staff, as well as staff from the NCI DEA, to develop all sections of the FOA, including commercially-focused review criteria.

4. Current Portfolio Analysis

The following table lists a snapshot of all applications submitted to the IMAT program's September 2009 receipt date for all of its R21/R33 funding opportunities. The table lists the applications by theme, funding mechanism, and the type of institution the investigator represents (academic/non-profit vs. commercial interest).

	Institution Type	Applications Received in Sept 2009
Innovative Technology Development for Cancer Research (R21 – CA-09-008)	Academic/Non-profit	60
	Commercial	9
Application and Use of Emerging Technologies for Cancer Research (R21 – CA-09-006)	Academic/Non-profit	25
	Commercial	1
Application and Use of Emerging Technologies for Cancer Research (R33 – CA-09-007)	Academic/Non-profit	10
	Commercial	4
Application and Use of Emerging Technologies for the Biospecimen Sciences (R21 – CA-09-004)	Academic/Non-profit	10
	Commercial	3
Application and Use of Emerging Technologies for the Biospecimen Sciences (R33 – CA-09-005)	Academic/Non-profit	1
	Commercial	2
	Total	**125**

5. **Budget:**
Based on historical trends, the SBIR Development Center estimates to fund 5-7 awards in FY2011 which includes both Phase I and Phase II awards. The total costs estimated for applications are approximately $150,000 for a Phase I application and $ 750,000 for Phase II application. Therefore, the SBIR Development Center recommends setting aside $2M in FY11 from the overall NCI –SBIR set aside to fund these awards. The total budget estimated for the entire project duration is $4M.

6. **Justification for use of RFA Mechanism**

It is recommended that NCI issue an RFA to solicit the commercial development of emerging cancer technologies, using a set-aside of funds as part of the overall SBIR set-aside. The investigator, who will define the scope and complexity of their technology, will initiate the application in response to this FOA. It is also recommended that special review panels be convened by NCI's DEA to conduct a rigorous evaluation of applications similar to that for the IMAT program, and this review process should include a range of professionals covering both business and technical expertise. An RFA provides the best mechanism to accommodate both of these requirements.

Review Considerations

Due to the strong emphasis on the commercialization and technology aspects of these applications, the proposed FOA would be similar to IMAT's existing FOA's, except that the milestones proposed by the applicant would be more heavily weighted towards commercial feasibility. The SBIR Development

Center and IMAT Program Staff will draft the technical scope and the review criteria will be enhanced in collaboration with the Division of Extramural Activities (DEA). The DEA will conduct the review session.

7. Justification for Use of Cooperative Agreement

Not Applicable

APPENDIX 1

Evaluation Criteria

The SBIR Development Center will work in close collaboration with IMAT program staff to evaluate the initial cohort of SBIR awardees under this FOA based on three different metrics: Innovation, Commercialization and Regulatory. The outcome of this review will be used to make a recommendation to the NCI Executive Committee regarding the continuation of this FOA in future years.

A. Innovation Metrics

The following innovation metrics will be used to evaluate projects under this FOA.

- Patents (Pending and approved)
- Copyrights
- Trademarks
- Formal Invention Disclosures
- Publications in peer reviewed journals (forthcoming or in print)
- Conference Presentation
- Commercial grade prototype development
- Preliminary Data demonstrating metrics of optimized sensitivity and specificity
- Completion of grant milestones

B. Commercialization Metrics

The following commercialization metrics will be used to evaluate projects under this FOA.

- Number of products yielding sales (includes licenses)
- Dollar volume of cumulative sales
- Number of license agreements
- Company sold or merged
- Acquisition of outside capital to continue product development

C. Regulatory Metrics

The following regulatory metrics will be used to evaluate projects under this FOA.

- Number of 510(K) or PMA Approvals for Marketing
- Investigational Device Exemptions (IDE)
- Number of successful CLIA Certifications