

Innovative Molecular Analysis Technology Development for Cancer Research and Clinical Care (SBIR-IMAT, PAR-13-327)

Presented by

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Today's Presentation



- **Overview of the NIH SBIR Program**
- **The NCI SBIR Development Center:
Special Initiatives at NCI**
- **Overview of the IMAT Program**
- **The SBIR-IMAT Funding Opportunity
Announcement**

Congressionally-Mandated Programs



**Set Aside
(FY13)**

➤ **Small Business Innovation Research (SBIR)**

Set-aside program for small business concerns to engage in Federal R&D with the potential for commercialization

Federal agencies with an extramural R&D budget > \$100M

2.7%

➤ **Small Business Technology Transfer (STTR)**

Set-aside program to facilitate cooperative R&D between small business concerns and U.S. research institutions with the potential for commercialization

Federal agencies with an extramural R&D budget > \$1B

0.35%

**~\$700M annually at NIH
~\$110M annually at NCI**

- Provides seed funding for innovative technology development
- **Not a Loan**
 - ∴ No repayment is required
 - ∴ Doesn't impact stock or shares in any way (i.e., non-dilutive)
- Intellectual property rights retained by the small business
- Provides recognition, verification, and visibility
- Helps provide leverage in attracting additional funding or support (e.g., venture capital, strategic partner)

SBIR & STTR: Three-Phase Program



- Proof-of-Concept study
- \$150,000 over 6 months (SBIR) or 1 year (STTR)

- Commercialization stage
- Use of non-SBIR/STTR funds

**Fast-Track Application
Combined Phase I & II**

**Phase III
COMMERCIALIZATION**

- Research & Development
- Commercialization plan required
- \$1 million over 2 years

* Hard caps on award sizes: \$225,000 for Phase I; \$1.5 million for Phase II

Note: Actual funding levels may vary by topic.

SBIR Eligibility Requirements

New Rules starting 1/28/13



- Applicant is a Small Business Concern (SBC)
 - Organized for-profit U.S. business
 - 500 or fewer employees, including affiliates
 - PI's primary employment (>50%) must be with the SBC at time of award & for duration of project
 - **> 50% U.S.- owned by individuals** and independently operated*
- OR**
- > 50% owned and controlled by other business concern/s that is/are
> 50% owned and controlled by one or more individuals*
- OR**
- > 50% owned by multiple **venture capital** operating companies, hedge funds, private equity firms, or any combination of these *

*Formerly $\geq 51\%$; *New rule starting 1/28/13, NIH SBIR only

NCI's primary resource for enabling commercialization of high impact technologies that can benefit patients, such as:

- Small Molecules and Biologics
- Cancer Diagnostics
- Cancer Imaging
- Electronic Health & Education Tools

A \$110M Program at the NCI

New Model: SBIR Development Center

- 10-member management team exclusively focused on the administration of NCI's SBIR/STTR portfolio
- Center staffed by program directors with industry experience and a broad range of scientific expertise
- Center collaborates with staff from across other NCI divisions to integrate the small business initiatives with the NCI's scientific priorities

NCI SBIR Development Center Program Staff



Michael Weingarten, MA (*Director*)

Previous

- **NASA** – Program Manager, NASA Technology Commercialization Program



Greg Evans, PhD (*Lead Program Director*)

Previous

- **NHLBI/NIH** – Program Director, Translational and Multicenter Clinical Research in Hemoglobinopathies
- **NHGRI/NIH** – Senior Staff Fellow



Patti Weber, DrPH (*Program Director*)

Previous

- **International Heart Institute of Montana** – Tissue Engineering and Surgical Research
- **Ribi ImmunoChem Research, Inc.** – Team Leader, Cardiovascular Pharmacology



Deepa Narayanan, MS (*Program Director*)

Previous

- **Naviscan PET Systems, Inc.**, Director, Clinical Data Management (Oncology Imaging & Clinical Trials)
- **Fox Chase Cancer Center**, Scientific Associate (Molecular Imaging Lab)



Jennifer Shieh, PhD (*Program Director*) *Previous*

- **NIH** – AAAS Science & Technology Policy Fellow
- **National Academy of Sciences** – Christine Mirzayan Science and Technology Policy Fellow
- **Syapse, Inc.** – Biology Associate



Andrew J. Kurtz, PhD (*Lead Program Director*)

Previous

- **NIH** – AAAS Science & Technology Policy Fellow
- **Cedra Corporation** – Research Associate, Bio-Analytical Assays and Pharmacokinetics Analysis



Jian Lou, PhD (*Program Director*)

Previous

- **Johnson & Johnson** – Research Scientist, Target Validation & Biomarker Development
- **Lumicyte, Inc.** – Director, Molecular Biology Systems Analysis



Todd Haim, PhD (*Program Director*)

Previous

- **National Academy of Sciences** – Christine Mirzayan Science and Technology Policy Fellow
- **Pfizer Research Laboratories** – Postdoctoral Fellow, Cardiac Pathogenesis & Metabolic Disorders



Amir Rahbar, PhD, MBA (*Program Director*)

Previous

- **NCI** – Program Manager, Center for Strategic Scientific Initiatives
- **Bioinformatics, LLC** – Senior Science Market Analyst
- **Naval Research Laboratory** – Research Scientist



Ming Zhao, PhD (*Program Director*)

Previous

- **NCI** – Program Director, Center to Reduce Cancer Health Disparities
- **GE Global Research** – Senior Scientist
- **Pfizer** – Scientist

Development Center staff are responsible for:

- Conducting regular outreach events to help recruit more focused, commercially-minded SBIR applicants
- Coaching applicants on developing stronger applications
- Providing oversight and active management of projects
- Mentoring and guiding companies throughout the award period
- Facilitating matchmaking with potential third-party investors and strategic partners

NCI SBIR Phase IIB Bridge Award



- Provides up to \$1M per year for up to 3 years to extend promising projects
- Open to any NIH-funded Phase II awardees
- Accelerates commercialization by incentivizing partnerships with third-party investors & strategic partners earlier in the development process
- Competitive preference and funding priority to applicants that can raise substantial third-party funds (i.e., $\geq 1:1$ match)

NCI SBIR Investor Forum



Exclusive opportunity for some of the most promising NCI-funded companies to showcase their technologies

<http://sbir.cancer.gov/investorforum/>

National Cancer Institute

2012 NCI SBIR
**Investor
Forum**

APRIL 18, 2012
Agilent Technologies,
Aristotle Room
5301 Stevens Creek Blvd
Santa Clara, CA 95051

FNIH
Foundation for the
National Institutes of Health

PRESCIENCE
INTERNATIONAL

The poster features a photograph of four diverse individuals (three women and one man) smiling and looking at a magnifying glass held by one of the women. The background of the photo is a blue molecular or network structure. The text is arranged in a clean, professional layout with a mix of blue, orange, and black colors.

- In 2012, 18 top SBIR-funded companies presented
- Over 200 life science investors & leaders
- 150+ one-on-one meetings
- **2010 Investor Forum: 8 out of 14 presenting companies closed deals valued at over \$230M**

Bringing together NCI SBIR/STTR awardees to move funded technologies from bench to bedside

<http://sbir.cancer.gov/FRACWorkshop>

- May 7, 2013 at NCI Shady Grove
- Speakers from FDA, CMS, USPTO, and White House OSTP
- Panels on other sources of federal funding, resources & collaborative programs at NIH, and unique life science investment organizations
- One-on-one meetings with program directors and speakers

The Innovative Molecular Analysis Technologies (IMAT) program

Tony Dickherber, Director

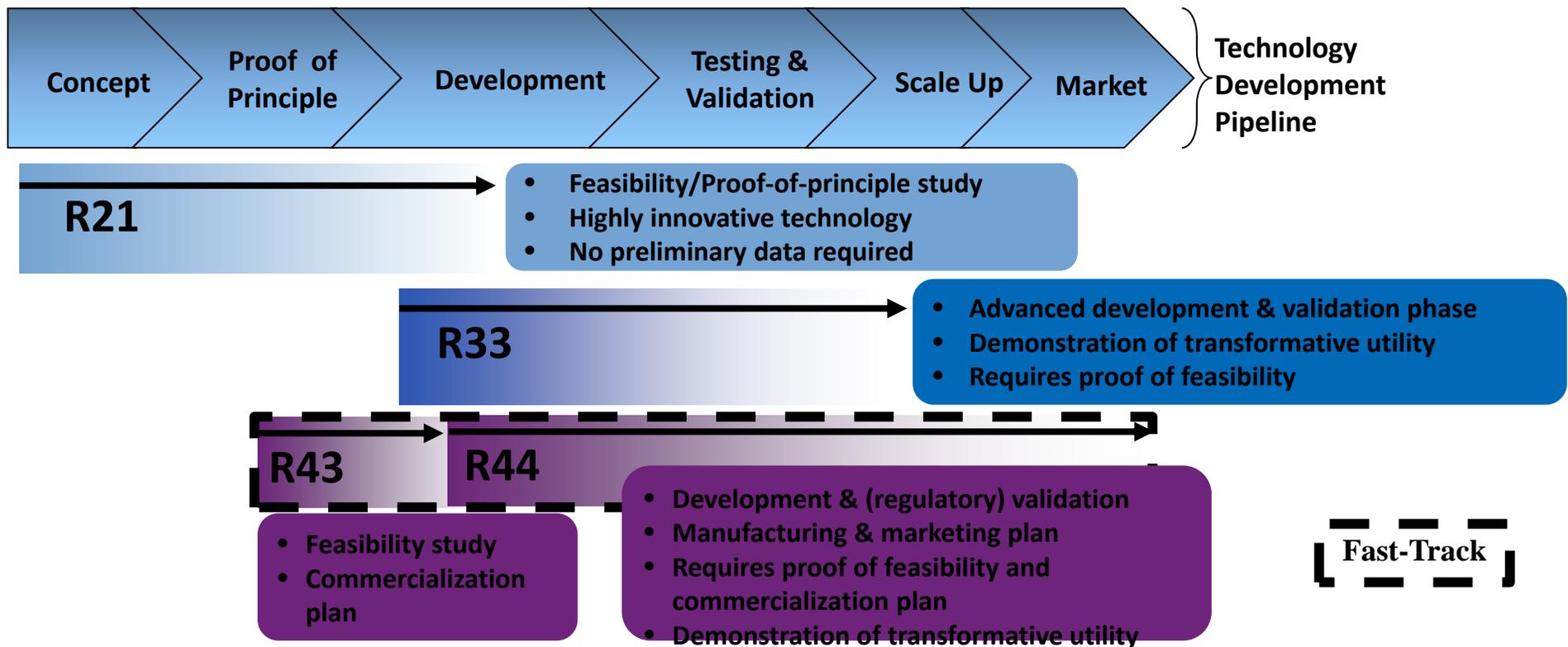


Innovative Molecular Analysis Technologies (IMAT) Program



Program Mission:

To support the development, maturation, and dissemination of novel and potentially transformative next-generation technologies through an approach of balanced but targeted innovation in support of clinical, laboratory, or epidemiological research on cancer.



Sample of Technologies Supported



Older

- ICAT by Applied Biosystems [2001]
- MudPIT, licensed by the Scripps Research Institute [2001]
- DREAMS by Pacific Northwest National Laboratories [2001]
- Rolling Circle Amplification, available from Amersham Biosciences (now GE Healthcare), [2002]
- Affymetrix GeneChip[®] CustomSeq[®] arrays [2002]
- MicroSol-IEF, available from Invitrogen as Zoom-IEF Fractionator [2002]
- Illumina Bead technology (BeadChip, Beadstation, and Sentrix BeadArray) [2004]
- Quantum Dots, purchased by Invitrogen [2005]
- MELT[®] & RNALater[®] by Ambion [2005 and 2008, respectively]

Newer

- Microfluidic Genetic Analysis platform, licensed by both Lockheed Martin and MicroLab Diagnostics [2008]
- Raindance[®] RDT-1000 (oil nanodroplet technology) [2009]
- COLD-PCR, licensed by TransGenomic [2010]
- NanoTrap Biomarker Discovery Platform, licensed by Shimadzu Scientific [2010]
- IUVO[™] cell isolation platform from Bellbrook Labs, exclusively licensed by ThermoFisher [2012]
- CellASIC ONIX microfluidic perfusion system, acquired by EMD-Millipore [2012]
- DCP-Bio1 cysteine oxidation probes from Kerafast, Inc [2013]
- INLIGHT from Cambridge Isotope Laboratories [2013]

- **Broad solicitation for highly innovative technology development to support the molecular and/or cellular analysis with significant potential for transformative impact in cancer research.**
 - No prescription on the field or type of innovation sought.
 - Emphasis on early stage and paradigm-shifting capacities (*i.e.* high-risk, high-impact)
- **Focus on technology development (NO biological hypothesis-driven research)**
- **Milestone-based applications that *quantitatively* assess the performance capacities of the technology (such as specificity, sensitivity, and speed) and characterize the improvement over state-of-the-art**

IMAT-SBIR Success: Illumina BeadChip & BeadArray

Mark Chee, Ph.D., *Illumina, Inc*

- **IMAT Awards:**
 - *Gene Expression Analysis of Randomly Ordered DNA Arrays (1999-R43)*
 - *Random Arrays for Gene Expression Profiling (2001-R44)*
 - *Parallel Array Processor (1999-R43, 2002-R44)*
- **Impact:** Ultra-high-throughput Illumina bead platform allows researchers to simultaneously assay over 100,000 points for gene expression, alternative splice detection, and protein expression

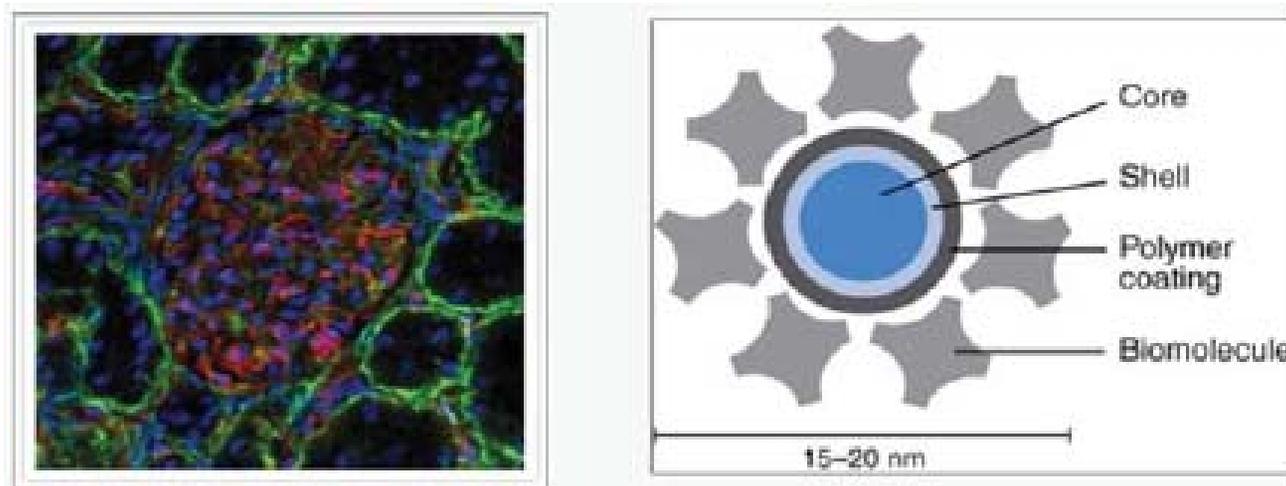


IMAT-SBIR Success : Quantum Dots



Robert Daniels, Ph.D., *Quantum Dot Corp (Invitrogen)*

- **IMAT Award:** Sensitive Multiplexed Analysis of Breast Cancer Markers (2000-R44)
- **Impact:** Quantum dots are photostable labels that emit extremely bright light in a range of colors enabling researchers to monitor complex interactions within living cells or in situ on tissue microarrays

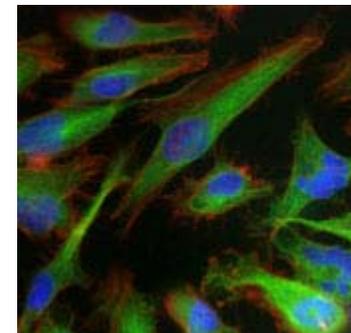
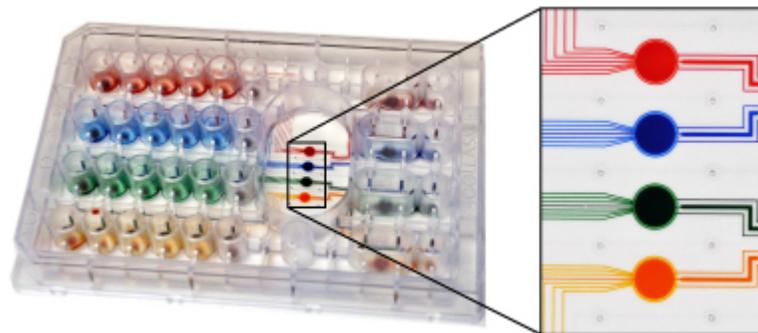


IMAT-SBIR Success: CellASIC



Philip Lee, Ph.D., CellASIC Corp (EMD-Millipore)

- **IMAT Award:**
 - Microfluidic System for Automated Cell Toxicity Screening (2006 – R43)
 - Microfluidic Liver Array for Long Term In Vitro Hepatocyte Culture and Screening (2008 R44)
- **Impact:** Microfluidic cell culture instrumentation with various customizable plates for advanced cell biology studies. Performs long-term tracking (days to weeks) of live cells with precise microenvironment control.



Innovative Molecular Analysis
Technology Development for Cancer
Research and Clinical Care
(SBIR-IMAT, PAR-13-327)



The SBIR DC and IMAT programs share the goals of stimulating the development of innovative molecular analysis technologies

The scope of the R21/R33 IMAT PAR is limited to early-stage development

- Does not support commercial validation activities.
- Primarily academic in focus

SBIR/IMAT PAR seeks to catalyze technology development and commercial validation of these technologies:

- Provide a cohesive program that is aligned with the goals of the IMAT R21/R33 programs
- Emphasis on commercialization
- Focus on high risk/high reward projects

Purpose of Program Announcement



Innovative technology development for the **academic community**

Innovative technology development for the **small business community**

IMAT

R21/R33

University Spinouts – Pipeline opportunity

SBIR DC

R43/R44

Technology development in basic science and beyond

- Side-by-side companion to the IMAT program
- Outlet for small businesses to develop innovative technologies using the R43/R44 mechanisms
- Academic institutions can continue to be funded through the standard IMAT R21/R33 mechanisms.

IMAT like projects with a focus on commercialization

IMAT/SBIR Pipeline for Technology Development Support



Separate Application Process

R43/Phase I

Mechanism:

Exploratory/pilot phase for proof-of-concept

Requirements:

- Relevance to cancer
- Quantitative milestones
- Truly novel tool/capability
- Improvement over state-of-the-art
- Commercial feasibility

R44/Phase II

Mechanism:

Developmental/validation phase plus initial commercialization efforts

Requirements:

- Advanced development and scaling of technology appropriate for cancer researchers or clinicians
- Validation for clinical and research with significant potential impact for the field
- Evidence of technical feasibility completed

Fast-track Award (Phase I+II)

Technology Dissemination via:

- Publication
- Licensing
- Commercialization

Technology Tools for Researchers:

- **New** – molecular and cellular analysis capabilities unavailable through other approaches/technologies
- **Better** – higher resolution, more detailed analysis, improved specificity/ selectivity/ sensitivity, etc.
- **Faster** – faster processing, massively multiplexed
- **Cheaper** – simpler or more robust design, field-ready

→ Application points of entry

Scientific/Technical Scope of this FOA



- **This FOA will support researchers at SBCs, who wish to develop and validate their innovative technologies in the context of commercial use. These technologies could have been invented, discovered, and/or initially developed with support from any funding source including, but not limited to, the NCI IMAT Program, or may be entirely new invention applications.**
- **Technologies proposed for this FOA are expected to exhibit a high degree of innovation with transformative potential, or otherwise demonstrate clear advantages over currently available technologies as is required for applications to the IMAT Program.**
- **Prospective applicants are advised to visit the IMAT website for more information about the program.**
 - <http://innovation.cancer.gov/>

Definition of “Technologies”



- **Novel techniques, materials, instrumentation, and devices that offer significant improvements in terms of novel types of cancer-relevant analyses, and/or greater resolution, specificity, and/or throughput relative to the currently available methods/tools.**
- **Highly-innovative platforms for sample preparation and/or processing, and for improved downstream analysis are also within the scope of this FOA.**
- **The proposed technology and application must correspond to an important unmet need relevant to cancer research and/or clinical aspects.**
- **These technologies (for both Phase I and Phase II applications) must have a strong potential for commercial success**

Technology areas of interest



- **Technologies capable of deciphering basic mechanisms underlying cancer initiation and progression;**
- **Technologies that enable substantially-improved early cancer detection and/or cancer risk assessment;**
- **Technologies capable of distinguishing, assessing, and/or monitoring cancer stage, and progression;**
- **Technologies to facilitate/accelerate the processes of drug discovery or development of generic approaches to improve drug delivery;**
- **Technologies that can facilitate and/or enhance molecular analyses in cancer epidemiology (e.g., by allowing for rigorous and/or expeditious collection of various relevant types of data);**
- **Technologies for sample preparation and/or processing for improved downstream analysis;**
- **Technologies that offer a novel means for assessing general analyte quality to determine sample fitness-for-purpose for a known analytical platform; and**
- **Technologies or tools that may help overcome various barriers in research on the incidence, prevalence, mortality, and burden of cancer among members of underserved populations.**

Technologies that are generally not appropriate for this FOA



- **Projects describing milestones that do not indicate advanced capabilities or offer progress towards commercialization;**
- **Projects proposing software/informatics solutions, database development, data mining, statistical tools, and computational/mathematical modeling (including those applicable to drug and/or patient responses);**
- **Projects in which the main thrust of effort is on exploring biological or clinical hypotheses (i.e., traditional hypothesis-driven projects) rather than on technology development;**
- **Projects proposing whole-body or *in vivo* imaging methods, or specific contrast agents; and**
- **Projects centered on development of specific drugs or therapies.**

Phase I projects



- **Preliminary data are strongly encouraged but not required.**
 - If preliminary data are not available, Phase I projects must be based on rigorous scientific rationale.
- **Phase I projects are expected to prove technical feasibility of the technology (and possibly generate a prototype, if appropriate) in a degree that is sufficient to support the use of the proposed technology in a cancer relevant application.**
- **Project goals must be supplemented with specific key technical and commercially-relevant milestones**
- **Quantitative milestones are required**

Phase I projects



- **Innovative, Cancer-relevant Technology**
- **Substantial Improvement and/or New Capabilities**
- **Transformative Potential**
- **Commercial Potential**
- **Quantitative Milestones**

Phase II projects



- **The Phase I results should have already demonstrated technical feasibility of the invention.**
- **Phase II projects are expected to concentrate on further technology development and improvements, to address intellectual property protection (including work towards filing patent application if not done already) and preparation for regulatory steps, as applicable, that might be needed for a commercial application of the technology.**
- **Project goals must be supplemented with specific key technical and commercially-relevant milestones**
- **Quantitative milestones are required**

Phase II projects



- **Validation of cancer-relevant technology**
- **Substantial improvement and/or new capabilities**
- **Transformative potential**
- **Quantitative milestones**
- **Commercialization plan**

Scored Review Criteria



- **Significance***
- **Investigator(s)**
- **Innovation***
- **Approach**
- **Environment**

Commercialization Plan



- **All applications must also describe the commercialization strategy for marketing the proposed technology as a product, process, service, or combination thereof.**
- **Commercialization plans should discuss the clear need and window of opportunity within a specific market, highlight the competitive edge over existing products or services, and outline the key steps that will be taken over the period of support towards achieving commercial success.**

Quantitative Milestones



- **Milestones should be well described, quantitative, and scientifically justified.**
- **Discuss the milestones as a means of judging the success of the phase I project as well as providing proof-of-principle for justifying further developmental effort (e.g., under a future phase II project).**
- **Where appropriate, milestones should include the relevant statistical context for the targeted parameter.**
- **Listing all milestones in a single location is helpful for review of the application.**

Examples of Quantitative Milestones



- **Detection of one cancer cell in 10^8 normal blood cells;**
- **Detection of a target analyte at a concentration of 1 fmol/mL in serum;**
- **Demonstrate that the measured analyte is highly correlated (Pearson correlation coefficient $r > 0.95$) for a given human serum sample when analyzed on different days. Include mean, standard deviation, and relative standard deviation for repeatability targets superior to next best approach;**
- **Detection of one mutated gene in the presence of 1,000,000 wild-type copies;**
- **Demonstration that the technology gives the same result in 95 out of 100 assays; and/or**
- **Demonstration that the technology can be N-fold faster (or N-fold more sensitive, or less expensive, etc.) than the current "gold standard" technology**

- **CSR review with a Special Emphasis Panel**
- **Access to information about NCI-available resources**
- **FDA interaction**
- **Webinar information sessions**
 - Potential topic areas will potentially include:
 - Regulatory Activities
 - Technology Transfer
 - Business Development
 - In-licensing/Out-licensing
 - Commercialization Strategies
 - Strategic Partner Development
 - Fundraising

- **Standard applications as directed by SF424**
 - Possible exception of “direct to R44” allowance, in which R43 is not required for eligibility for R44 submission
- **Phase I (R43) – TC up to \$225k/2-yrs**
 - Feasibility study
 - Commercial feasibility
 - Quantitative milestones
- **Phase II (R44) – TC up to \$1.5M/3-yrs**
 - Development & (regulatory) validation study
 - Manufacturing & marketing plan
 - Requires proof of feasibility and commercialization plan
 - Demonstration of transformative utility

Application Information



Funding Instrument	R43, R44, & fast-track grants
Application Types Allowed	New Resubmission
Award Budget	<u>Guidelines</u> <u>R43</u> : Total funding support (direct costs, indirect costs, fee) normally may not exceed \$150,000 for Phase I awards <u>R44</u> : Total funding support (direct costs, indirect costs, fee) normally may not exceed \$1,000,000 for Phase II awards <u>Fast-track</u> : Combination phase I and II application submitted simultaneously in accord with SF424 rules. <u>Application budgets must reflect actual needs of the proposed project</u>
Award Project Period	According to statutory guidelines, award periods normally may not exceed 6 months for Phase I and 2 years for Phase II.
Application Due Date(s)	Nov 4, 2013; May 28, 2014, by 5:00 PM local time of applicant organization.
Earliest Start Date(s)	July 2014

SBIR-IMAT Information

<http://sbir.cancer.gov/funding/technology/>

SBIR-IMAT PAR-13-327 Full Funding Announcement

<http://grants1.nih.gov/grants/guide/pa-files/PAR-13-327.html>

NCI SBIR Development Center

<http://sbir.cancer.gov>

NCI's Innovative Molecular Analysis Technologies (IMAT) program

<http://innovation.cancer.gov/>

Send questions using the “Chat” function

For project-specific or other questions, please contact us directly at :

- Amir Rahbar, NCI SBIR Development Center
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