National Cancer Institute

Advanced Technology Partnerships Initiative

Program Narrative

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U.S. DEPARTMENT
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Executive Summary:

The number of candidate drug targets and products aimed at prediction, prevention, diagnosis, and treatment of cancer has increased dramatically during the past decade. This is a direct outgrowth of increased support for biomedical research and advances across the entire research spectrum. The sequencing of the human genome alone has brought science to the threshold of unparalleled opportunity. However, the promise of these basic research discoveries will not be realized without a translational research enterprise that can rapidly and cost-effectively transition them into products for cancer patients.

The National Cancer Institute's (NCI) Advanced Technology Partnerships Initiative (ATPI) will address this need through development of partnerships involving advanced technologies and translational science in collaboration with other government and nongovernmental research and development organizations. Procedures will be in place to ensure protection of intellectual property and appropriate management of potential conflicts of interest.

NCI will draw upon its wide array of cutting-edge technologies at its national laboratory in Frederick, Md. NCI-Frederick is a Federally Funded Research and Development Center (FFRDC), a government-owned, contractor-operated national laboratory, one of only 38 in the nation designed to accomplish special long-term research or development needs that cannot be met as effectively by existing in-house or contractor resources. NCI-Frederick is currently home to many state-of-the-art and cost-effective advanced technology programs for the rapid translation of basic discoveries into interventions for cancer patients. NCI will build on this capability to capitalize on existing expertise and to avoid redundancy of infrastructure.

Strategic Objective: The Basis for Measuring Progress Against Cancer

NCI's mission is to reduce the burden of cancer. The report, "*National Cancer Institute Response to Congressional Report Language on Strategic Plan Implementation (Response*)," notes that to achieve this strategic objective, NCI must integrate all of its activities across a seamless research continuum. This will require partnerships and the ability to transition new research discoveries to the clinic in order to preempt cancer and to expedite the delivery of effective interventions to cancer patients.

This strategic goal is not unique to NCI. In fact, the objective of delivering high-quality, safe, and effective medical products to patients faster and more cost efficiently is embodied in the Department of Health and Human Services' (HHS) *Priorities for America's Health Care,* as well as the National Institutes of Health's (NIH) *Roadmap for Medical Research.*

The basis for measuring how well NCI is meeting its strategic objective is its ability to deliver effective interventions to cancer patients.

Barriers to Achieving NCI's Strategic Objective:

Several independent studies have noted that barriers exist to the translation of new discoveries into effective clinical products. In 2006, the Government Accountability Office (GAO) cited two major barriers:

- the inability of drug developers to effectively harness new technologies
- and the shortage of researchers with expertise in translating basic discoveries into new drugs.

In its November 2006 report to Congress entitled *New Drug Development: Science, Business, Regulatory, and Intellectual Property Issues Cited as Hampering Drug Development Efforts,* the GAO said the number of new molecular entities submitted to FDA for approval from 1993 to 2004 increased by only 7 percent despite an increase in private-sector R&D expenditures of 147 percent. The report cites an FDA study acknowledging, "There is an urgent need to improve the drug development process and to enhance collaboration among the Government, Industry, and Academia."

NCI addressed these issues by convening a roundtable of cancer research community leaders representing cancer centers, industry, academia, philanthropic and advocacy groups, and the financial markets to examine, among other things, the key barriers that stand in the way of accelerating progress against cancer and strategies for speeding the development and delivery of new diagnostics, preventives, and treatments for cancer. The roundtable recommended four approaches for eliminating major barriers to translational research:

- Building cross-disciplinary collaborations and teams
- Bridging the gap between late discovery and early development of diagnostics and therapeutics
- Developing data standards
- Developing cross-cutting technology platforms

In February 2006, the NCI sponsored the first of two Translational Research Working Group Roundtables for advice on how to create and sustain a 21st century translational research model using multidisciplinary collaborations with coordinated, complex infrastructures and streamlined, efficient procedures. The working group is now focusing on early translation involving partnerships and collaborations, intervention development, and phase I and II clinical trials.

ATPI: An Approach for Achieving NCI's Strategic Objective

The ATPI seeks to accelerate the delivery of effective products to cancer patients. This will be achieved by utilizing advanced technology platforms and collaborative research agreements to identify the most promising candidates and then move them efficiently through the translational continuum.

NCI-Frederick has a proven track record in managing research and development agreements with other organizations, both inside and outside of the government. This expertise combined with a broad array of advanced technologies will support ATPI objectives.

The FFRDC contractor is required by Federal Acquisition Regulation to be an autonomous organization that operates in the public interest with full disclosure of its activities to the government. The FFRDC also enjoys access to government and supplier data, employees, and facilities beyond that which is found in other contractual relationships. The FFRDC environment will provide a wealth of resources to accelerate the development of new, more effective ways to diagnose, treat, and ultimately prevent, cancer.

Congress has rightfully expressed concerns about ensuring that interactions among the government and other research and development organizations avoid conflicts of interest. The ATPI is well-suited to provide an environment in which these interactions between the government and other organizations may occur transparently, thus mitigating conflict-of-interest concerns.

The use of the FFRDC is also consistent with the NCI FY 08 Bypass Budget (Budget), known formally as *The Nation's Investment in Cancer Research.* The FY 08 Budget lists five key forces as critical to the realization of NCI's strategic objective. One of these five key forces, "Leveraging Resources and Knowledge – *where collaborative efforts and entities meet,*" acknowledges that research and development partnerships are changing the way NCI works to reduce the burden of cancer. It goes on to state that NCI's FFRDC

comprises an effective resource of world-class biomedical research and technology capabilities that can support a collaborative approach to the translation of basic research results into new prototype cancer diagnostics and therapies for patients.

NCI-Frederick technologies to which the Budget refers include translational research programs in:

- Genomics
- Proteomics
- Imaging
- Nanotechnology
- Mouse Models
- High-throughput screening
- Biospecimen repositories
- High-Performance Computing and Bioinformatics
- Biopharmaceutical Development
- cGMP manufacturing
- Associated Quality Assurance Capabilities
- Regulatory affairs programs that can support development of Investigational New Drug (IND) applications

Benefits of the ATPI to the Public Health

The ATPI is designed to reduce the time and cost associated with the translation of candidate drug products into effective interventions that would be available to cancer patients more rapidly and at lower cost.

The ATPI will take advantage of technology platforms that help identify the candidates most likely to be efficacious and have low toxicity when they enter clinical trials. This will enable the partners to allocate their finite resources to high-probability candidates and to move them more rapidly through development and clinical testing.

For example: the development of mouse models that mimic human disease would be valuable in determining whether a drug or biologic candidate is likely to be successful in human clinical trials; development of standardized biomarkers would facilitate evaluating a products effectiveness in treating a disease; and standardized genomic profiling techniques would enable clinicians to prescribe drugs and biologics that are most likely to be effective against an individual patient’s specific disease.

The ATPI will also enable studies that partners may otherwise be unable to accomplish. An example is the conduct of phase I, II, or III clinical trials using multiple-combination drugs from multiple organizations. This type of clinical trial can be difficult to undertake for companies that are normally competitors; it may, however, be possible by partnering with other organizations in such a way that does not compromise proprietary rights.

The ATPI will offer:

- access to a diverse and integrated portfolio of cost-effective, state-of-the-art advanced technology platforms, thus avoiding the cost to establish and sustain a redundant technology infrastructure
- access to translational and clinical drug development expertise that is distinct from the drug discovery paradigm
- expanded access to cGMP development and manufacturing capabilities and regulatory affairs support

The NIH has a number of partnerships, including the NIH Biomarkers Consortium, which began in 2006 to identify and validate biomarkers for disease. The ATPI shares features and benefits of the biomarkers consortium, but will address a more diverse set of challenges.