Frederick National Laboratory for Cancer Research

Presentation to the Board of Scientific Advisors

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Nov. 5, 2012
Frederick National Laboratory

Presentation Outline

• Our Identity and Mission

• Exemplifying the impact of Frederick National Laboratory programs

• NCI-Frederick Advisory Committee guidance for the future of Frederick National Laboratory
Overview of Frederick National Laboratory for Cancer Research

• FNLCR is the Federally Funded Research and Development Center
  • Established in 1972
  • Only FFRDC dedicated to biomedical research
• Proudly operated by SAIC-Frederick, Inc. on behalf of the National Cancer Institute
• Main campus on 70 acres at Ft. Detrick, MD
  • Co-located with intramural NCI researchers and other NCI activities
  • Additional FNLCR scientists at Bethesda and Rockville sites
• Mission: Pursue innovative basic, applied, and translational research leveraging technical expertise, physical infrastructure, and FFRDC status
Defining Characteristics of Frederick National Laboratory for Cancer Research

• **Unique combination** of scientific expertise & operational capability to support all aspects of applied biology and translational medicine

• **Agile**: adapt to changes in NCI priorities

• **Honest Broker**: integrate with government agencies, extramural community, and industry partners

• **Accessible**: technologies and contractor expertise is available to intramural, academic, and industrial biomedical concerns
Supporting the NCI Mission in Cancer and AIDS Research

• **Technology Development and Application**
  - Genomics, Proteomics, Advanced biomedical computing, Biomedical imaging & microscopy, Laboratory animal sciences, Small animal imaging, Clinical Assay technology

• **Accelerate Preclinical Development**
  - Nanotechnology (NCL), Genetically Engineered Mouse Models of cancer (CAPR)

• **Clinical development support**
  - Clinical Assay Development Center, Biopharmaceutical Development Program, Diagnostics and Pharmacodynamics

• **AIDS & Cancer Virus Program**
Nanotechnology Characterization Laboratory (NCL)

- NCL was established in 2004 as an interagency collaboration among NCI, NIST, and FDA. The lab’s mission is to accelerate the translation of promising nanotech cancer drugs and diagnostics.

- NCL performs preclinical characterization of nanomaterials, including:
  - physicochemical characterization
  - in vitro experiments
  - in vivo testing for safety and efficacy

90% of NCL’s efforts support the extramural community
Why NCL Is Needed

- Most nanomaterials come from academic labs focused on materials science
  - Investigators have little experience with oncology, pharmacology, drug development, or regulatory requirements

- Collaboration with NCL allows investigators to take advantage of ‘lessons learned’:
  - Trends in biocompatibility
  - Gives investigators a heads-up on regulatory requirements

Success Stories: NCL-aided Submissions to Clinic

ATI-1123: PEGylated nanoliposomal formulation of docetaxel
Phase I safety study in patients with advanced solid tumors complete in 2012.

IND 2009

BIND-014: docetaxel-encapsulated PLGA nanoparticle-aptamer conjugates
Binds PSMA expressed on prostate cancer cells
Phase I safety study in patients with advanced or metastatic cancer ongoing.

IND 2011

Silica-core gold-shell particle for photothermal ablation with NIR irradiation
Pilot safety study in head and neck cancers ongoing; efficacy study in lung tumors to start in 2012.

IDE 2008

ProNAi

PNT2258: liposome-encapsulated oligonucleotide for breast and lung cancer.
Phase I safety study in patients with advanced solid tumors ongoing.

IND 2010

Aurlmune®: PEGylated colloidal gold nanoparticle-TNFα conjugates
Phase II study in combination with Taxotere to start in 2012.

Phase 1
Completed 2008
### The **NCI Experimental Therapeutics Program (NExT)**

- NExT is led by the Division of Cancer Treatment and Diagnosis to create a coordinated cancer therapeutics discovery and development pipeline with the external scientific community
  - Projects evaluated by extramural Special Emphasis Panel
- SAIC-F provides operational and dedicated technical support to all phases of NExT programs

|-------------------|-----------|-------------|-----------|-------------|-------------|------|-------|--------|-------|

- **Small Molecule Repository**
  - Chemical Biology Consortium
  - Molec. Charact. Lab
  - Bioph.Dev.Prgm.(BDP)
- **Clin. Target Valid. Lab**
- **Clinical Assay Development Program**
  - BDP Production, Quality Assur.
**Concept**: ch14.18 marks neuroblastomas for killing by the immune system by binding to an overexpressed antigen called GD2

- Due to complexity of process and small market, no commercial vendor would make the antibody

Children’s Oncology Group Phase III trial in patients with high-risk neuroblastoma demonstrated clear event-free survival benefit

With the success of the trial, a commercial vendor has been found and our process transferred
Quantitative Molecular Diagnostics Core

- State of the art capabilities for monitoring virus levels in blood and tissues in NHP models
  - Real-time qPCR/qRT PCR, droplet digital PCR
- National reference lab
- Critical support of high impact AIDS vaccine studies

LETTER

Profound early control of highly pathogenic SIV by an effector memory T-cell vaccine

LETTER

Vaccine-induced CD8+ T cells control AIDS virus replication
NCI-Frederick Advisory Committee
Building for the Future

- **NFAC charge** - review the state of research at FNLCR and make recommendations for the best use of its capabilities and infrastructure

- **15 member committee**

  - Zachary Hall, Ph.D. Former Director, NINDS Former President; Institute of Regenerative Medicine, UCSF Emeritus Professor, UCSF

  - C. Barrett  
  - D. Botstein  
  - L. Garraway  
  - J. Gray  
  - B. Hahn  
  - M. Justice  
  - T. Look  
  - L. Marnett  
  - J. Mesirov  
  - G. Nolan  
  - K. Olden  
  - J. Pietenpol  
  - S. Rosen  
  - C. Willman
Expanding the Partnering Base
Development of Contractor Cooperative Research and Development Agreement (c-CRADA)

- Enables SAIC-Frederick to partner directly with extramural scientists and organizations for access to our science and technology know-how

- Use full CRADA authority under CRADA statutes
  - c-CRADAs for Research, Development, and Testing collaborations
  - “Technical Service Agreement” for tactical evaluation of proprietary partner materials, AIDS testing kits, etc.

- Intellectual property rights
  - SAIC-F is the custodian of joint or sole IP emerging from the CRADA
  - Streamlined assignment of exclusive commercialization rights
  - Any royalty streams support FFRDC R&D efforts

- Processes
  - Focus on speed
  - Local government review and approval with external input as appropriate
New Partnering Initiatives
Expanding access to FNLCR Resources

• Cooperative Research and Development Agreements (cCRADA)
  – Two partnerships received initial concept approval
  – Five additional agreements in development

• Technical Service Agreement (TSA)
  – Seven distinct assays approved for external offering
  – Three additional assays submitted for approval, 11 in preparation
  – One agreement signed with UCSF, 4 in progress

• External-facing FNLCR website operational and evolving
  – http://frederick.cancer.gov/
 FNLCR Strategic Direction Initiatives

• Identification and Implementation of “Big Ideas”
  – Fulfill the “National Laboratory” vision
  – Variety of NCI, FNLCR, and external workgroups contributed ideas
    • “Hub-and-spoke” model likely
  – Funding strategies within the existing FNLCR budget under discussion
  – Communication plan under development

• FNLCR Laboratory Director (NCI)
Conclusions

• **Frederick National Laboratory for Cancer Research** is a unique resource within the national biomedical research community

• **Program partnerships** facilitate basic and translational research achievements

• **New partnering opportunities** expand the impact of FNLCR science

• **New “big idea” research programs** will strengthen the identity and impact of FNLCR as a National Laboratory