NCI's Bio-repositories, Future Needs and the National Biospecimen Network Model

NCI: Meeting Post-Genomic Bio-repository Research Needs

Tissue Repositories in the United States

- More than 300 million specimens are stored from >150 million cases, and over 20 million new specimens are collected each year*

HOWEVER…

- Advancing the science of genomics and proteomics depends on the availability of uniformly collected, processed, annotated and stored biological samples. Several lines of evidence indicate that this is rapidly becoming a major barrier.

* *Handbook of Human Tissue Sources*, RAND 1999

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Bio-Specimen Access Issues – Post Genomic Research

- Tissue collection and/or storage not uniformly compatible with genomic analysis – data comparison difficult to meaningless
- Concerns from patients on genetic privacy - HIPAA
- “Ownership” barriers - impede tissue sharing among researchers
- Role of pathology
- No unified system with broad access
- Data from tissues sold to companies is not in the public domain

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Recent Recommendations for Biospecimen Resources to Support Genomic/Proteomic Research

- NCI's Progress Review Groups (PRGs), particularly the Brain, LLM, GYN and Lung PRGs
- State of the Science Meetings
- NDC Research Team Forum I

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U.S. and International Major Bio-repository Initiatives

- Other countries
  - UK National Cancer Tissue Resource (~ $8 mil/5yrs)
  - European Human Frozen Tissue Bank (TuBaFrost)
  - UK Biobank (~ $73 mil/10 yrs)
  - Biobank Japan (~ $180 mil/5 yrs)
  - Icelandic Health Sector Database (~ $135-250 mil)
- U.S. Private Tissue Acquisition Companies

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NCI's Interest in Major Bio-repository Model to Support Post-Genomic Research

- Examples of NCI's tissue resources (Centers, SPORES, EDRN, Cohort Studies, Clinical Trials, CHTN, etc.)
  - Estimated (internal) cost of NCI Tissue Resource Activities for FY 2001 = ~ $40.6 M*
- Need for consistency in collection, storage, annotation, bioinformatics, access, etc.
- Leadership in genomic- and proteomic-based development of new cancer drugs and diagnostics
- Collaborated on development of NBN model with NDC Research Team – available to community

*Figure includes resources in Cancer Diagnosis Program, CTEP, Centers, SPORES, DCCPS and DCEG

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Overall Goal for a National Post-Genomic Bio-repository

A new national, pre-competitive, regulatory compliant and genetic-privacy protected, standardized, inclusive, highest quality network of biological sample(s) banks; supported by and developed via novel financial and other partnerships with cancer survivors and advocates, the private sector and non-profit organizations as appropriate; that is shared, readily accessible, and searchable using state-of-the-art informatics systems (e.g., amenable to molecular profiling capability).

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NBN Blueprint Input

- Over 100 experts from all sectors participated in design
- Informed by a “best practices” study performed by RAND
- The NBN report is available to all interested individuals and institutions
- The Blueprint and the RAND study is accessible from the NDC website - available for public comment

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Strategy for Developing the NBN Concept (Two Studies)

- RAND Case Studies of Existing Human Tissue Repositories: “Best Practices” for a Biospecimen Resource for the Genomic and Proteomic Era
- National Biospecimen Network Blueprint

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Goal: Model for a national biospecimen resource to optimally support genomic and proteomic research and and accelerate the development of new cancer interventions

RAND study of "Best Practices"

NBN Blueprint

Organizational Framework:

Business Plan and Operations

Overarching Requirements:

Researcher Needs

Ethical and Legal

Operational Issues:

Biospecimen and Data Collection and Distribution

Bioinformatics and Data Management

Communications

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Goal: Model for a national biospecimen resource to optimally support genomic and proteomic research and accelerate the development of new cancer interventions

RAND study of "Best Practices"

- Standardized interview instrument administered by RAND staff
- 12 repositories interviewed through site visits and teleconferences
- Includes 50-60 "best practices" specifically focused on supporting genomic and proteomic research

NBN Blueprint

- Model developed by the NBN "Design Team," *experts*, and Constella Health Sciences
- Supported by needs assessment questionnaire
- Provides detailed requirements for building the NBN

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RAND Study - Repositories Analyzed

- Ardais Corporation
- AFIP
- CHTN
  - TARP
- EDRN
- Genomics Collaborative Inc.
- NHLBI (BBI Biotech)
- Philadelphia Familial Breast Cancer Registry
- SPOREs (Duke Breast, UAB Breast and Ovarian, Mayo Clinic Prostate)
- University of Pittsburgh Medical Center (CPCTR, EDRN, Lung SPORE)

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Examples of RAND Study Findings- NBN Response

RAND Findings	NBN Concept
Repositories serve many purposes: few common standards observed across repositories	Standardization of biospecimen collection, storage, distribution, etc.
Variable and inconsistent data associated data collected with biospecimens	Standardization of data collected about biospecimens, including longitudinal data
Variable ethical clearance obtained for biospecimens	Standardization of ethical clearance appropriate for genomic and proteomic investigations

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Examples of RAND Study Findings- NBN Response (cont.)

RAND Findings	NBN Concept
No uniform informatics framework for submitting research results to a central database	State-of-the-art informatics system for analyses and comparisons of genomic and proteomic data – building *in-silico* capability
Only a few of the repositories examined provide extensive biospecimen access to the broader research community	Timely, open, peer-review based access of biospecimens and associated data

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Results from RAND Study

- Many individual elements of the NBN model are present in existing resources, but they are not standardized or integrated into a single system with broad peer-reviewed access
- Many of the NBN objectives could be met by implementing the “blueprint” through capabilities resident in existing systems - provided that these resources can meet the overarching access, technology, quality, and other goals of the NBN

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Key Specifications for the New NBN Model

Theme	Requirement(s)
Researcher Needs	• Biospecimens collected in a defined manner from a diversity of cancer types and populations based on a continual review of researcher needs • Timely and widespread access of biospecimens and associated data through a centralized peer-review process
Ethical and Legal	• Privacy issues are important, but manageable • NBN biospecimens and data are provided to the research community free of intellectual property restrictions
Biospecimen and Data Collection and Distribution	• Standardization of biospecimen and data collection methodologies • Detailed pathology and clinical annotation of biospecimens, including longitudinal data – potential to include technology characterization
Bioinformatics and Data Management	• State-of-the-art bioinformatics system to support data analysis and submission of research results – cumulative data base
Communications	• Communication and outreach efforts directed at basic and clinical researchers, patients and the general public

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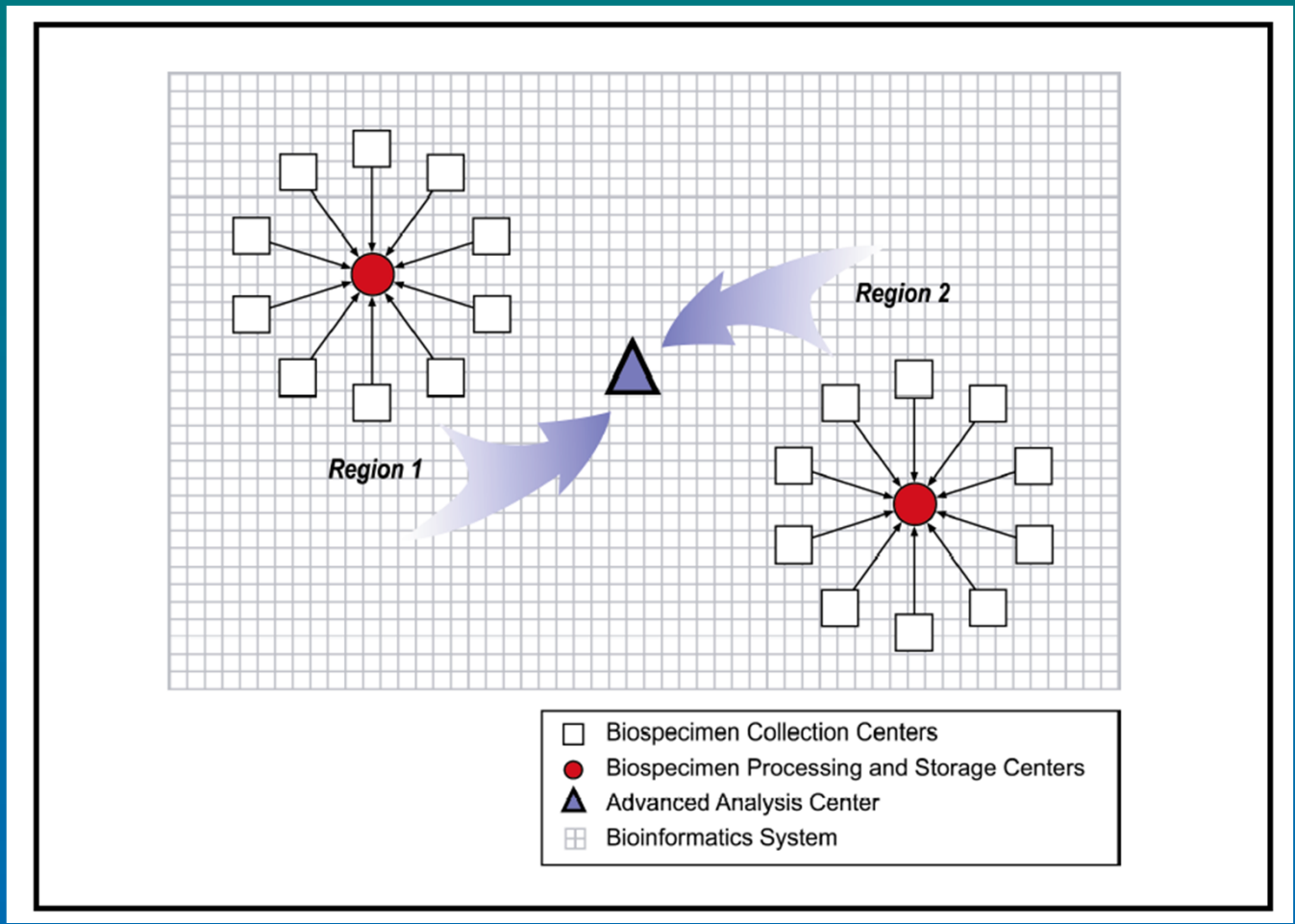
Hypothetical Model for NBN Biospecimen Collection Networks and Distribution



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Suggested NBN Governance and Operational Model

Board of Governors

(Oversees Operations Center)

NBN Operations Center

(Manages Business Units)

Business Units

- **Research Administration and Support**
- **Specimen Acquisition**
- **Storage and Distribution/ Basic Analysis**
- **Advanced Analysis**
- **Bioinformatics and Data Management**
- **Patient Relations**

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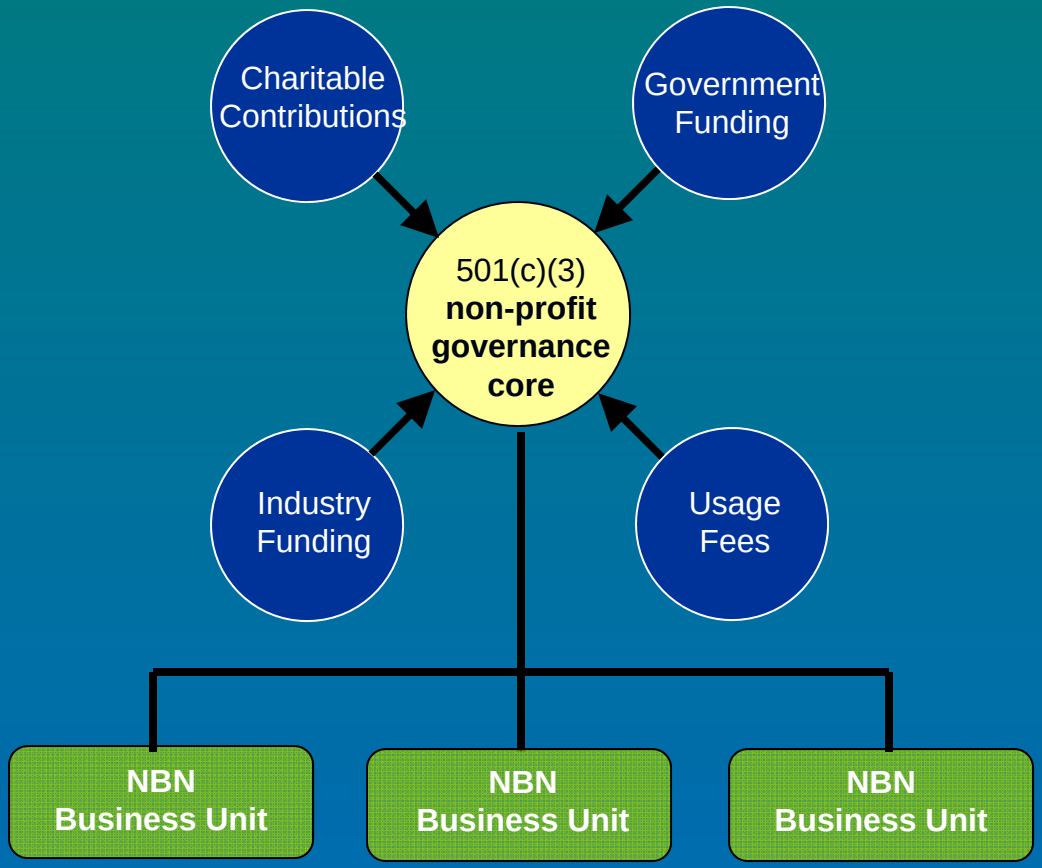
Example of NBN Funding Model



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NBN - Next Steps

- Distribution of reports – public comment period
- Consideration of a potential collaborative pilot project by several groups
- Possible strategy for pilot project
  - Perform basic functions of the NBN
  - Address specific challenges identified by the Design Team (i.e. collecting longitudinal data, encouraging submission of research results)
  - Scale rapidly to operate on a large, national level

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Timeline for the NBN Blueprint Report Release

Release of Reports to NDC Board and NDC Research Team	Completed
Beginning of Public Comment Period for Blueprint Report	Mid-November 2003
Official Broad Release of RAND and Blueprint Reports	Late December 2003
Potential Pilot Project	TBD

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NBN Blueprint and RAND Reports

- Available from the NDC website at: www.ndoc.org

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Value of the NBN to NCI

- Best practices chosen by large group of cross-sector experts
- Opportunity to tie resources together and optimize resources, access, and outcomes
- Could serve the entire community
- Prepared by a broad cross section of community – not dominated by NCI – broad ownership
- Excellent “blueprint” for unique national biospecimen resource and database

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NCAB: Recommended Next Steps

- Re-activate, expand NCAB bio-repository subgroup
- Develop complete inventory of current NCI bio-repositories
- Evaluate NBN and RAND reports
- Establish community needs
- Collaborate on strategy for developing and managing overall resources through NBN or some other model