The NIH Roadmap for Medical Research

www.nihroadmap.nih.gov

What is the **NIH Roadmap for Medical Research?**

- A framework of priorities that the NIH as a whole must address in order to optimize its entire research portfolio

- A set of initiatives that are central to extending the quality of healthy life for people in this country and around the world

- A vision for a more efficient and productive system of biomedical and behavioral research

Why was there a need for an NIH Roadmap for Medical Research?

- Position NIH to address evolving public health challenges
    - Acute and chronic diseases; changing demographics; health disparities, emerging diseases and biodefense

- Accelerate the pace of discoveries
- Develop more rapid translation from laboratories to patients and back

How was the Roadmap developed?

- Extensive consultations with stakeholders, scientists, health care providers
- Discussions addressed:
  - **What are today's scientific challenges?**
  - **What are the roadblocks to progress?**
  - **What do we need to do to overcome roadblocks?**
  - **What can't be accomplished by any single Institute – but is the responsibility of NIH as a whole?**

Roadblocks
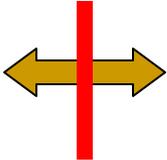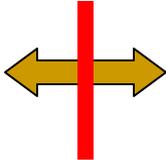
Bench ⟷ **Bedside** ⟷ **Public**

Criteria for Roadmap Initiatives

- Is it 'transforming' -- will it change how or what biomedical and behavioral research is conducted in the next decades?

- Would its outcome enhance the ability of all ICs to achieve their missions?

- Can the NIH afford NOT to do it?

- Does it address key issues of interest to stakeholders, especially the public?

- Is it something that no other entity can or will do?

NIH Roadmap for Medical Research: three themes emerged

- New Pathways to Discovery

- Research Teams of the Future

- Re-engineering the Clinical Research Enterprise

New Pathways to Discovery

Bench ⟺ **Bedside** ⟺ **Public**
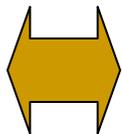
- **Building Blocks and Pathways**
- **Molecular Libraries**
- **Bioinformatics**
- **Computational Biology**
- **Nanomedicine**

Research Teams of the Future

Interdisciplinary Research Teams; Pioneer Awards

Public-Private Partnerships

Bench ⬌ Bedside ⬌ Public

**Building Blocks
  and Pathways
Molecular Libraries
Bioinformatics
Computational Biology
Nanomedicine**

Re-engineering Clinical Research

Interdisciplinary
Research

Pioneer Awards

Public-Private Partnerships

Bench ⟺ **Bedside** ⟺ **Practice**

Building Blocks
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Molecular Libraries
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Computational
  Biology
Nanomedicine

Translational
Research
Initiatives
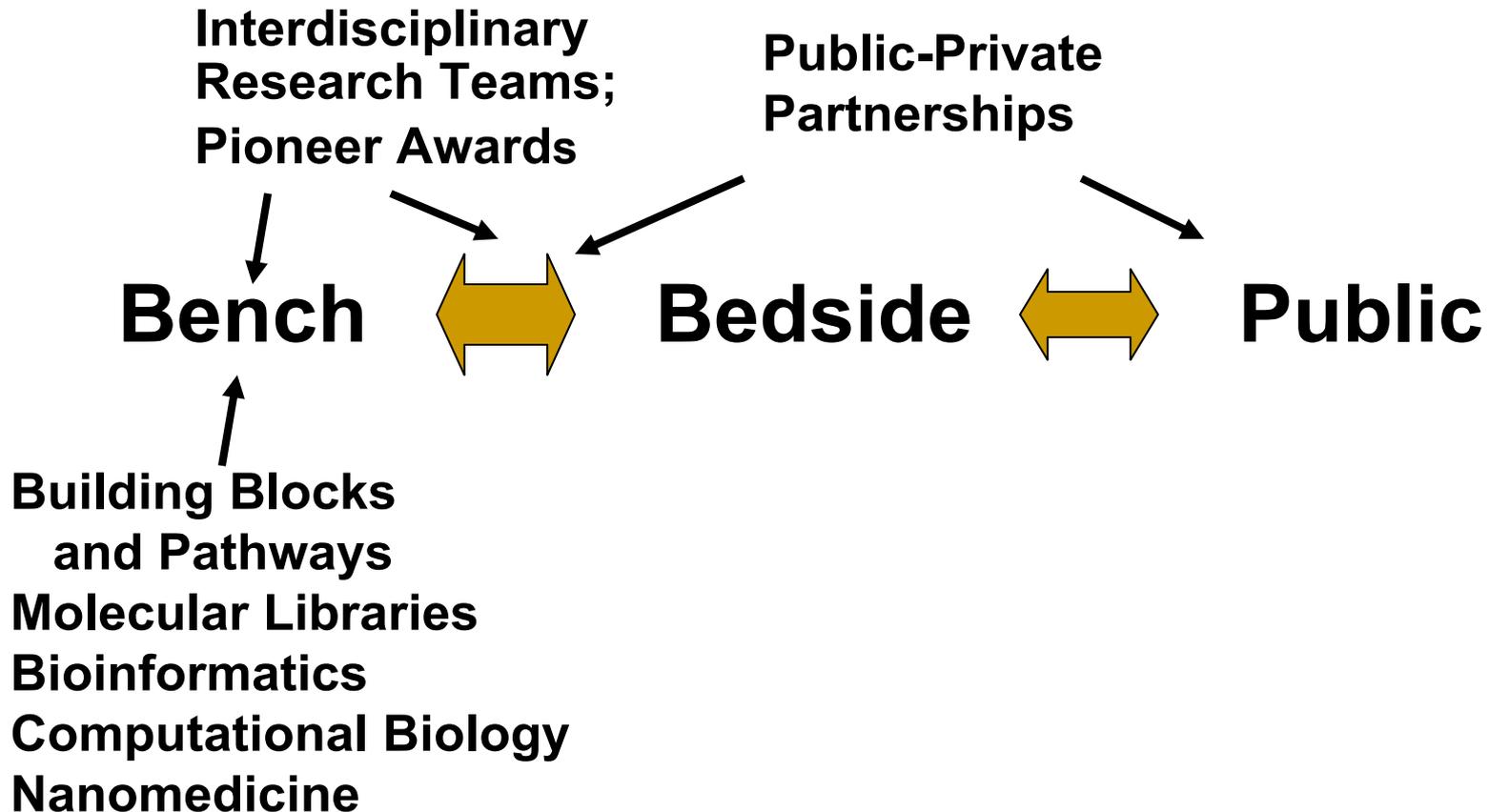
•Integrated Clinical Research Networks
•Clinical Research Informatics
•NIH Clinical Research Associates
•QoLClinical outcomes
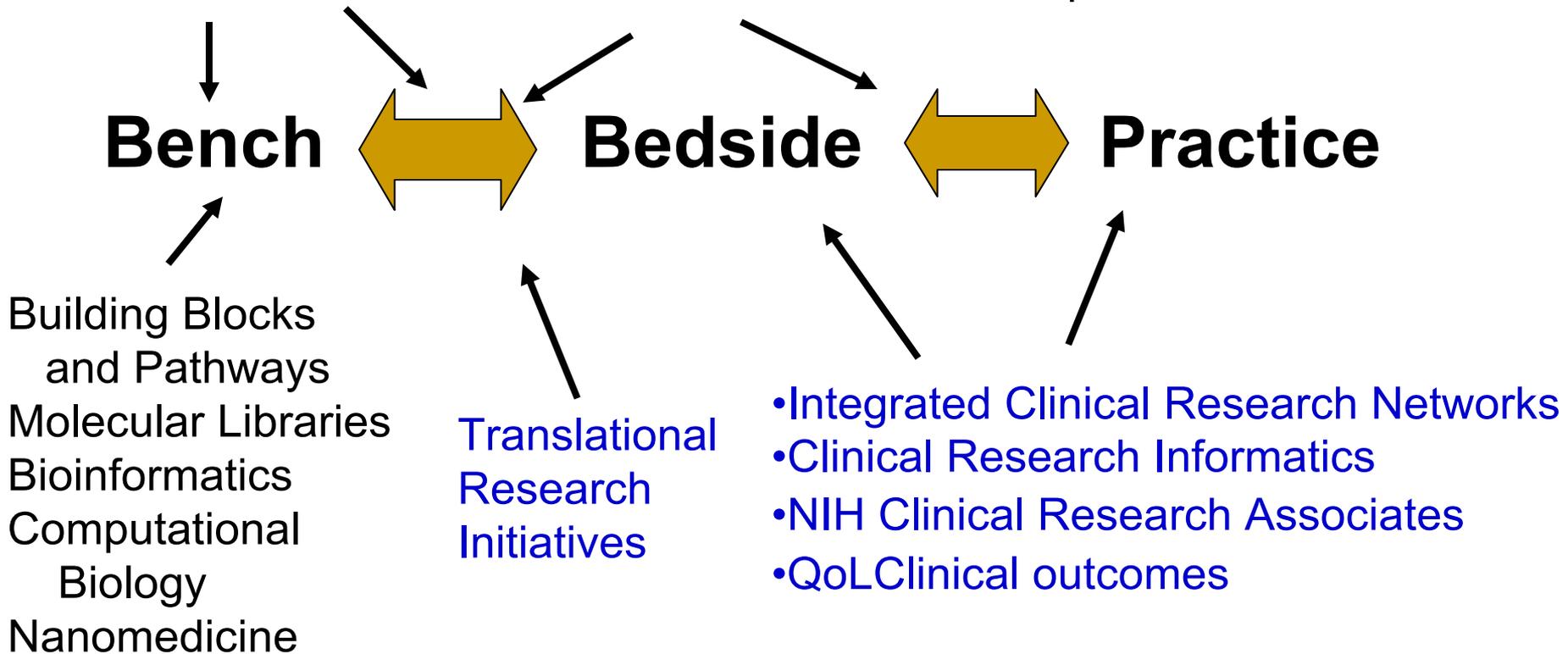
Cross cutting: Harmonization, Training

NIH Roadmap for Medical Research

**New Pathways
to Discovery**

NIH
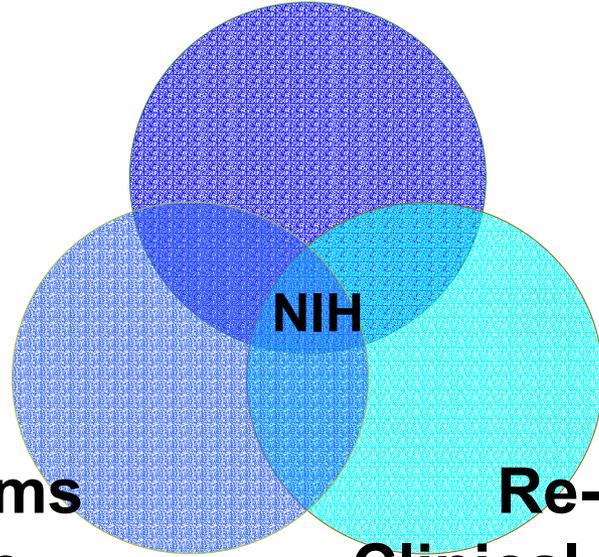
**Research Teams
of the Future**

**Re-engineering the
Clinical Research Enterprise**

National Institutes of Health Roadmap Initiatives

THEMES

NEW PATHS TO DISCOVERY	RESEARCH TEAMS OF THE FUTURES	RE-ENGINEERING THE CLINICAL RESEARCH ENTERPRISE
Implementation Groups	Implementation Groups	Implementation Groups

NEW PATHS TO DISCOVERY

- **Building Blocks, Pathways, & Networks**
- **Molecular Libraries & Imaging**
- **Structural Biology**
- **Bioinformatics & Computational Biology**
- **Nanomedicine**

RESEARCH TEAMS OF THE FUTURES

- **High-Risk Research**
- **Interdisciplinary Research**
- **Public Private Partnerships**

RE-ENGINEERING THE CLINICAL RESEARCH ENTERPRISE

- **Clinical Research**

  -Harmonization of Clinical Research Regulatory Requirements
  - Integration of Clinical Research Networks
  - Enhance Clinical Research Workforce Training
  - Clinical Research Informatics: National Electronic Clinical Trials and Research Network (NECTAR)
  - Translational Research Core Services
  - Regional Translational Research Centers
  - Enabling Technologies for Improved Assessment of Clinical Outcomes

Structural Biology:
Life in three dimensions

- Proteins that reside in cell membranes – the next frontier

- Long term goal: the ability to predict shape and function of any protein from sequence
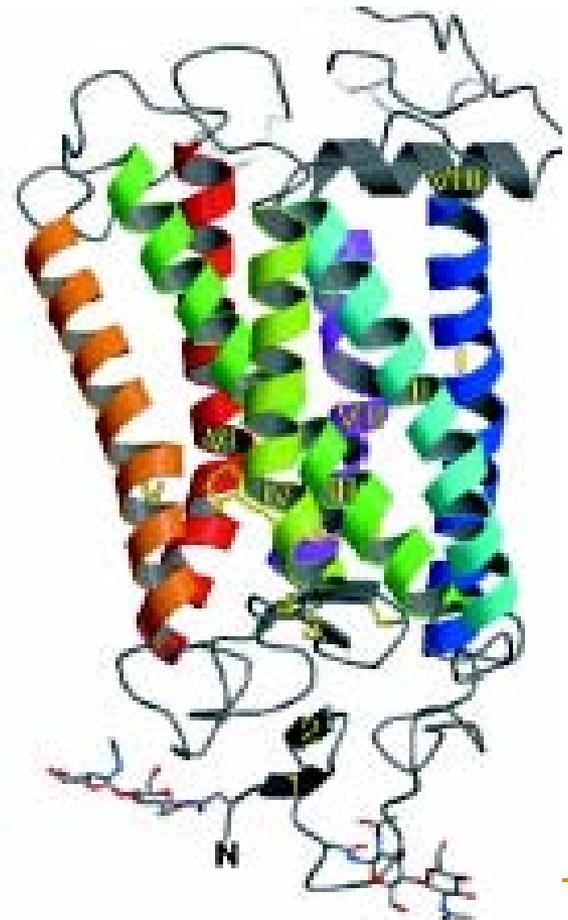
Molecular Libraries and Imaging:
Putting Chemistry to Work for Medicine

- National public sector screening centers for small molecules

- Public database for "chemical genomics" and imaging probes

- Improve technologies for high throughput screening

- Develop imaging probes that increase sensitivity 10 to 100 fold

Computational Biology:
Modeling the Cell's Information Superhighway

- National Centers for Biomedical Computing
- National software engineering system
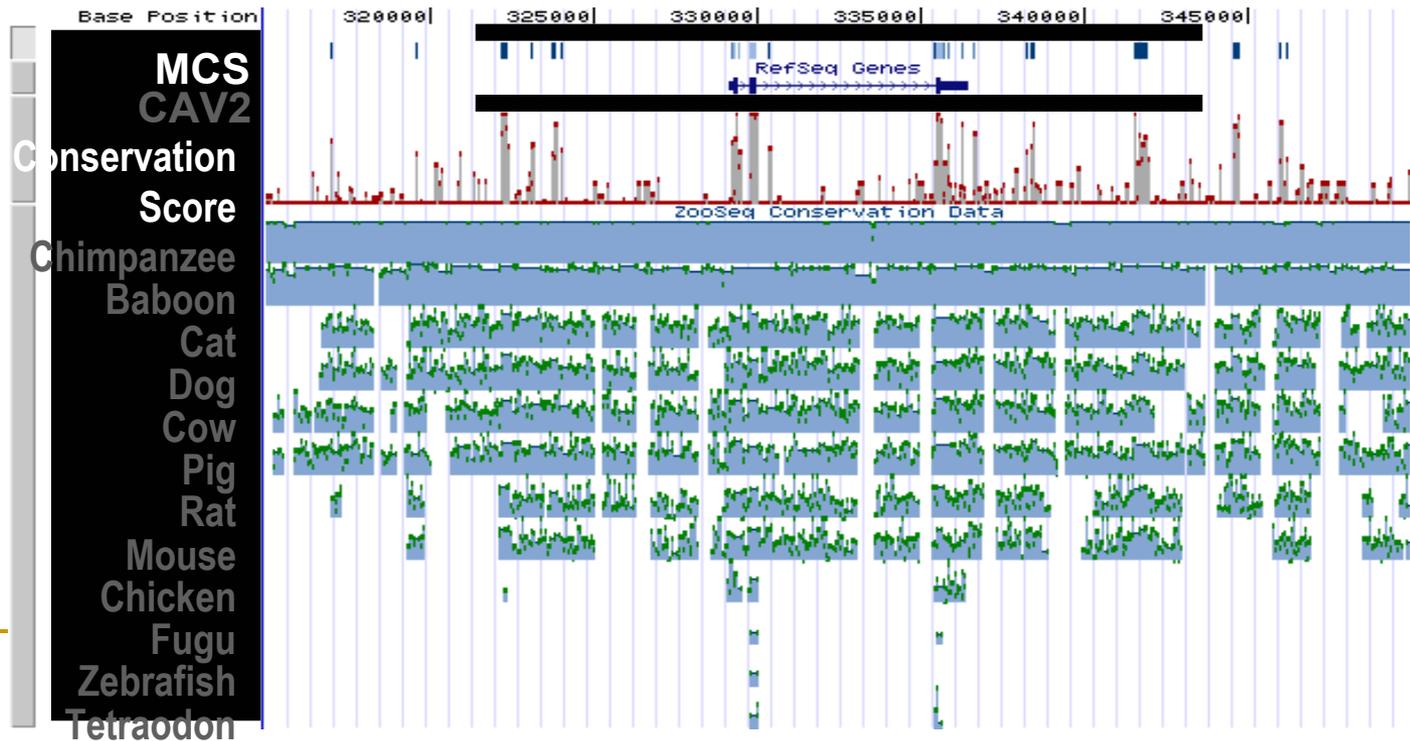
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◆ **Building Blocks, Pathways, & Networks** ◆ **Molecular Libraries & Imaging** ◆ **Structural Biology** ◆ **Bioinformatics & Computational Biology** ◆ **Nanomedicine**	◆ **High-Risk Research** ◆ **Interdisciplinary Research** ◆ **Public Private Partnerships**	◆ **Clinical Research** -Harmonization of Clinical Research Regulatory Requirements - Integration of Clinical Research Networks - Enhance Clinical Research Workforce Training - Clinical Research Informatics: National Electronic Clinical Trials and Research Network (NECTAR) - Translational Research Core Services - Regional Translational Research Centers - Enabling Technologies for Improved Assessment of Clinical Outcomes

The Challenge

- **NIH seen by some as being risk-averse**

- **Peer- review typically values likelihood of success more than potential impact**

- **Funding decisions are too conservative and too slow**

- **NIH's research investments are not sufficiently diversified**

This threatens to deplete the NIH of a vital set of investments that are critical to its future successes

The Challenge

- The strategy:

  Develop **NIH Pioneer Awards**

Challenges to Interdisciplinary Research

- **The current system of academic advancement in science favors the independent investigator.**

AND HERE ARE MY INDEPENDENT CO-WORKERS !
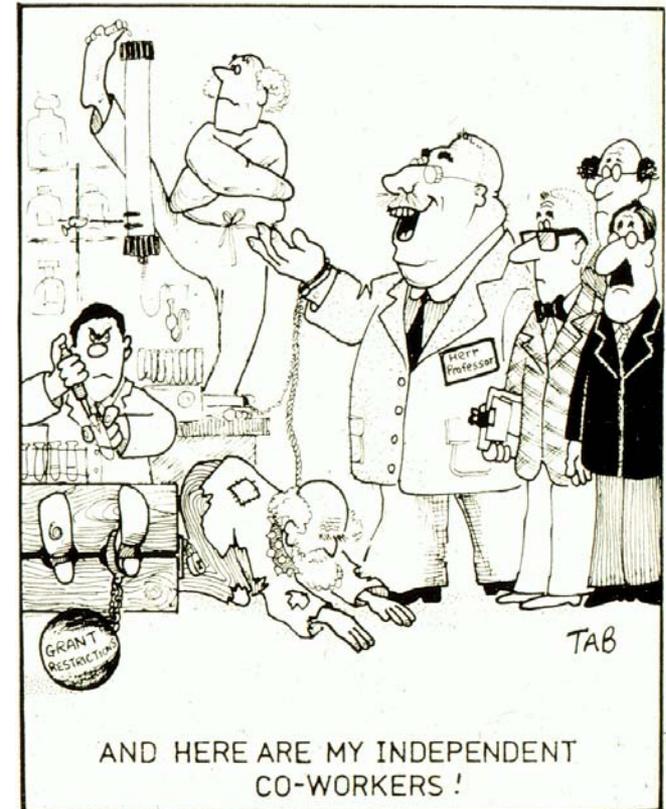
Challenges to Interdisciplinary Research

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Challenges to Interdisciplinary Research

- **The current system of academic advancement in science favors the independent investigator.**

- **Most research institutions house scientists in discrete departments.**

- **Interdisciplinary research teams take time to assemble and require unique resources to be maintained.**

Challenges to Interdisciplinary Research

The strategy:

Promote paradigms for the formation of
INTERDISCIPLINARY RESEARCH TEAMS

Re-engineering Clinical Research

Interdisciplinary
Research

Pioneer Awards   Public-Private Partnerships

Bench   ⟺   **Bedside**   ⟺   **Practice**

Building Blocks
 and Pathways
Molecular Libraries   Translational   Integrated Research Networks
Bioinformatics   Research   Clinical Research Informatics
Computational   Initiatives   NIH Clinical Research Associates
 Biology   Clinical outcomes
Nanomedicine
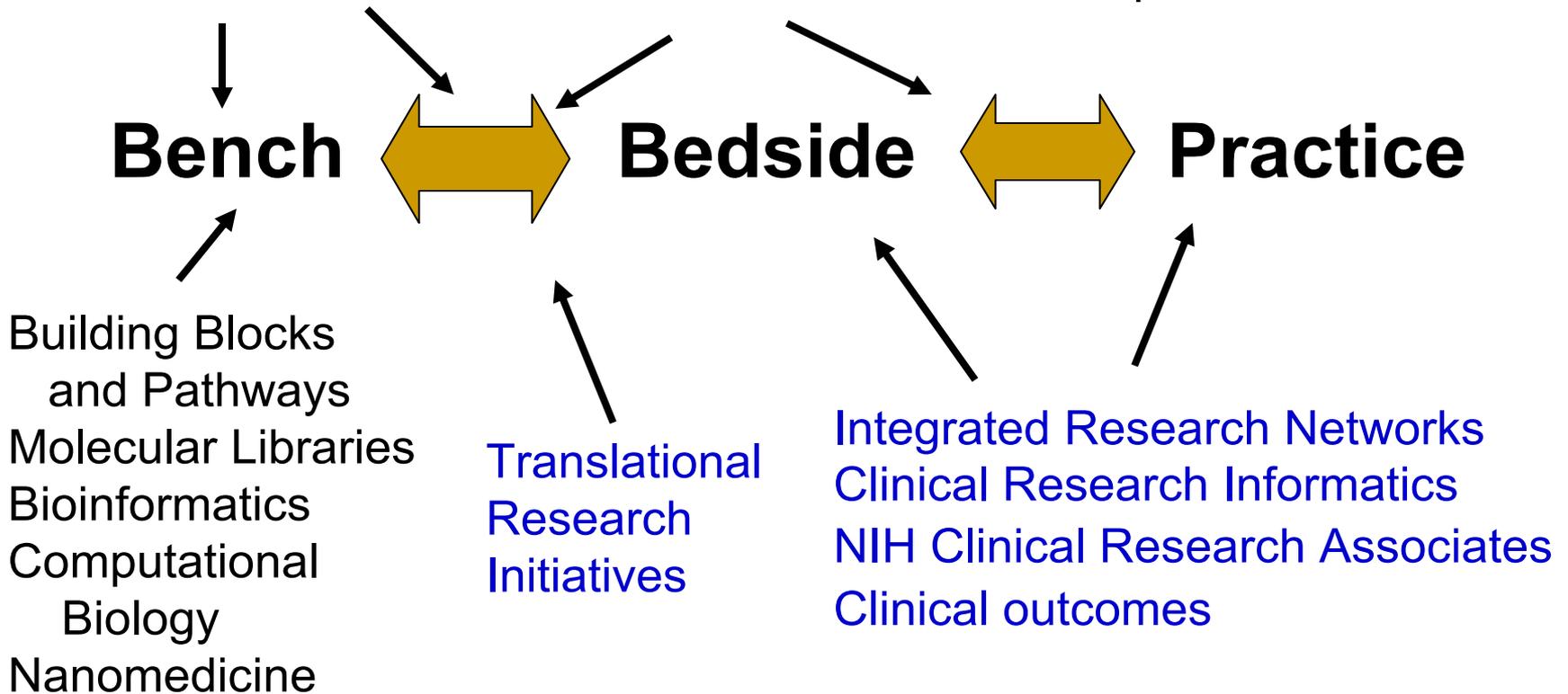
Cross cutting: Harmonization, Training

The Challenge:

Basic discoveries must be transformed more quickly into practical, preventative and therapeutic approaches

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The Strategy:

Provide the training and tools to accelerate the translation of new knowledge from the bench to the bedside to clinical practice

Harmonization of Clinical Research Regulatory Processes

Goal: **Harmonize and (?) simplify requirements for clinical research in ways that enhance public trust**

- **Adverse event reporting**
- **Human subjects protection**
  - **DSMB-IRB interactions**
  - **Consent procedures**
- **Auditing and monitoring clinical trials**
- **HIPAA, privacy, conflict of interest policies**
- **Investigator registration, financial disclosure**
- **Standards for electronic data submission/reporting**

NIH Career Development Programs

College Professional Grad School Post-Doc Prof. Advancement
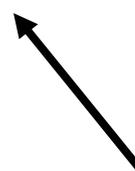
NIH Clinical Research Training Pgm; Advanced Degree Pgm? **Trans-NIH K12 Career Dev; Loan Repayment**

Trans-NIH Multidisciplinary K12 Career Development Program (RFA)

Goal:
- Promote development of investigators from a variety of disciplines (MD, PhD, RN, MPH, DC …)
- To be trained in multidisciplinary team settings

Features:
- Up to 5 years of training
- Core didactic courses, Project-specific training
- Mentored research experience in team settings
- Faculty/mentor support to protect their time
- Tuition support
- Annual meetings

National Clinical Research Associates

Goal:
- **Diverse national group of trained and certified community healthcare providers**
- **Will enroll and follow their own patients**
- **Accelerate translation of results into practice**

Steps:
- **Determine feasibility: Barriers? Communities? Incentives needed?**
- **Inventory training methods, best practices**
- **Develop core competencies, certification**
- **Future: Training programs**

Translational Research

- Regional Translational Research Centers

- Tools - Pre-clinical drug synthesis, toxicity testing

- Enabling Technologies for Improved Assessment of Clinical Outcomes

Enabling Technologies for Improved Assessment of Clinical Outcomes

Goals:	■Improve and validate QoL measures ■Improve validation of surrogate markers of disease outcomes
Features:	■Improved QoL instruments to provide tools for comparing outcomes ■Better enabling technologies to facilitate the translation of basic findings to the clinic

Integration of Clinical Research Networks

- **Link existing networks so clinical studies and trials can be conducted more effectively**

- **Ensure that patients, health care providers, and scientists form true "Communities of Research"**
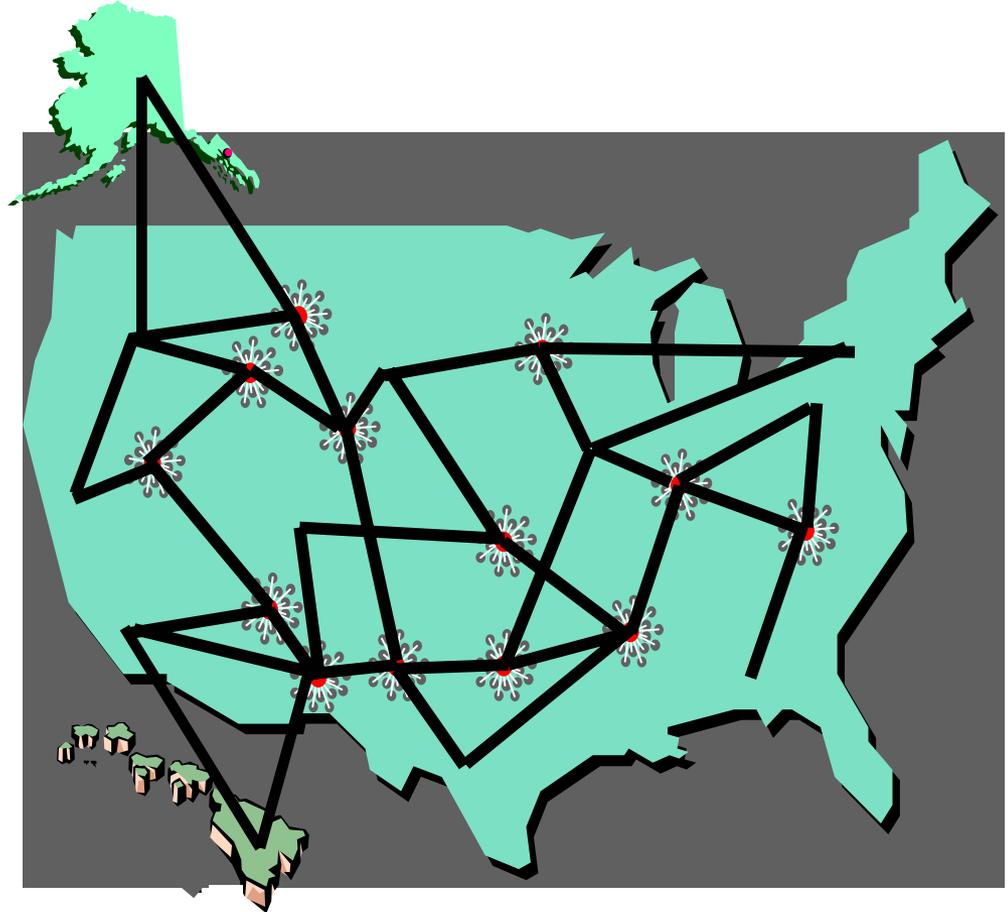
# Integration of Clinical Research Networks

Goal:

Establish a resource consisting of integrated and interoperable networks where clinical studies and trials can be addressed in an efficient, effective, and economical manner

Typical NIH Network
## Academic Health Center Sites
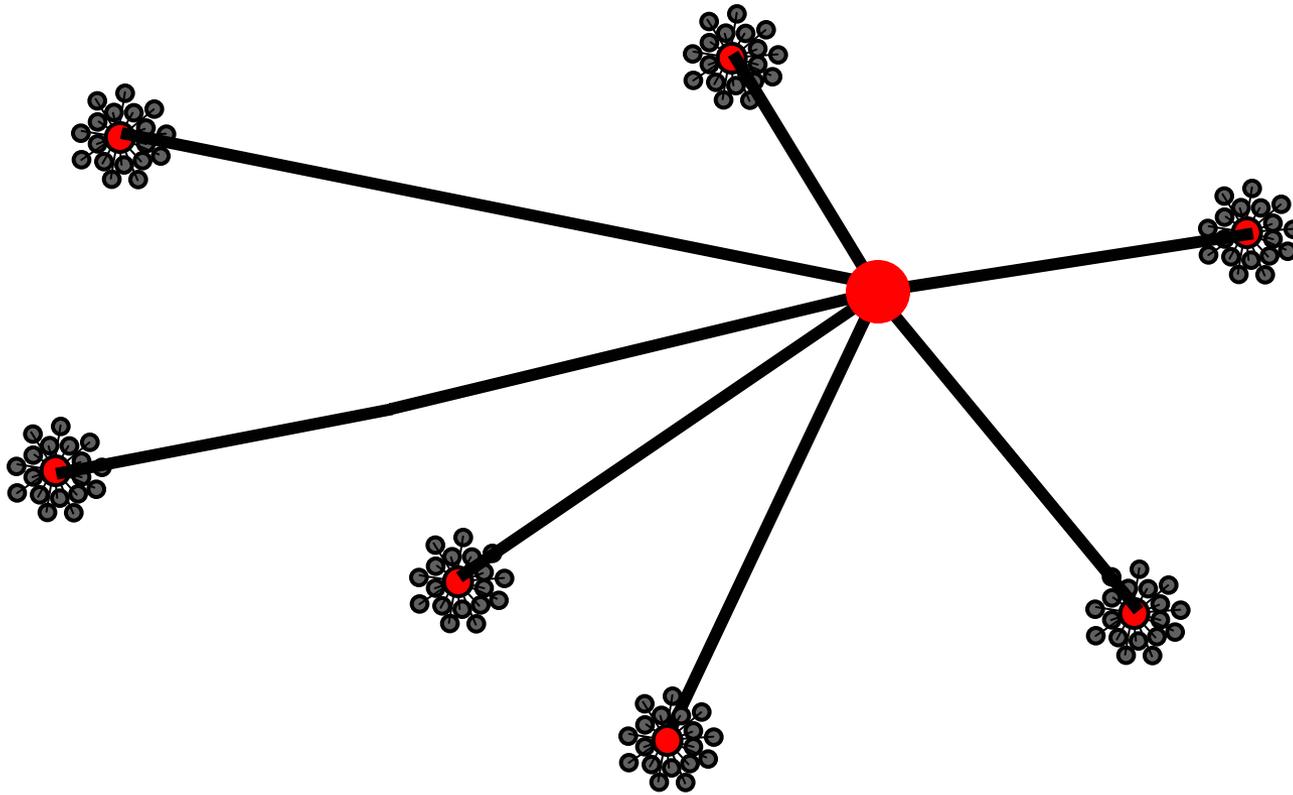
Two National NIH Networks
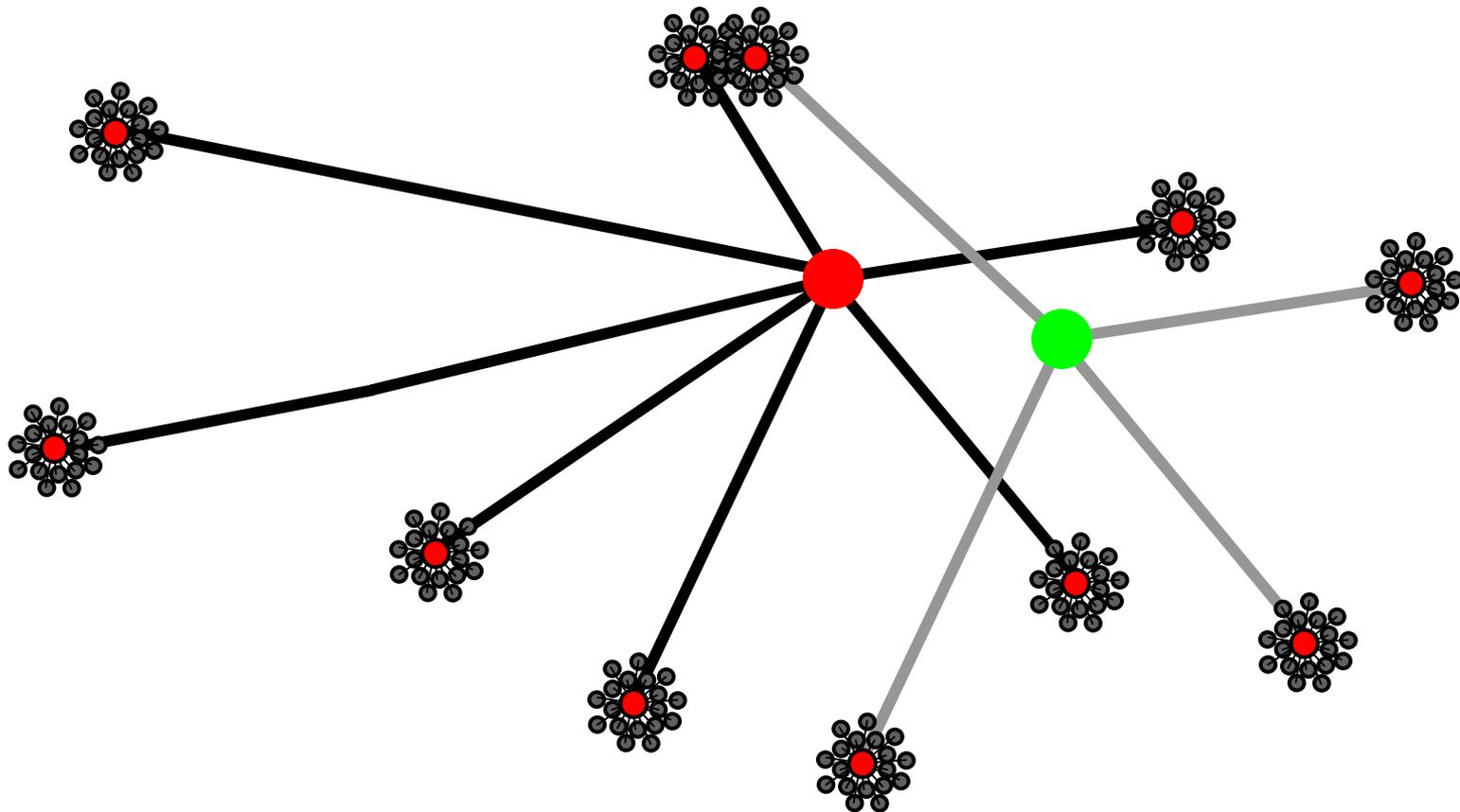
Interoperable Networks
Share Sites
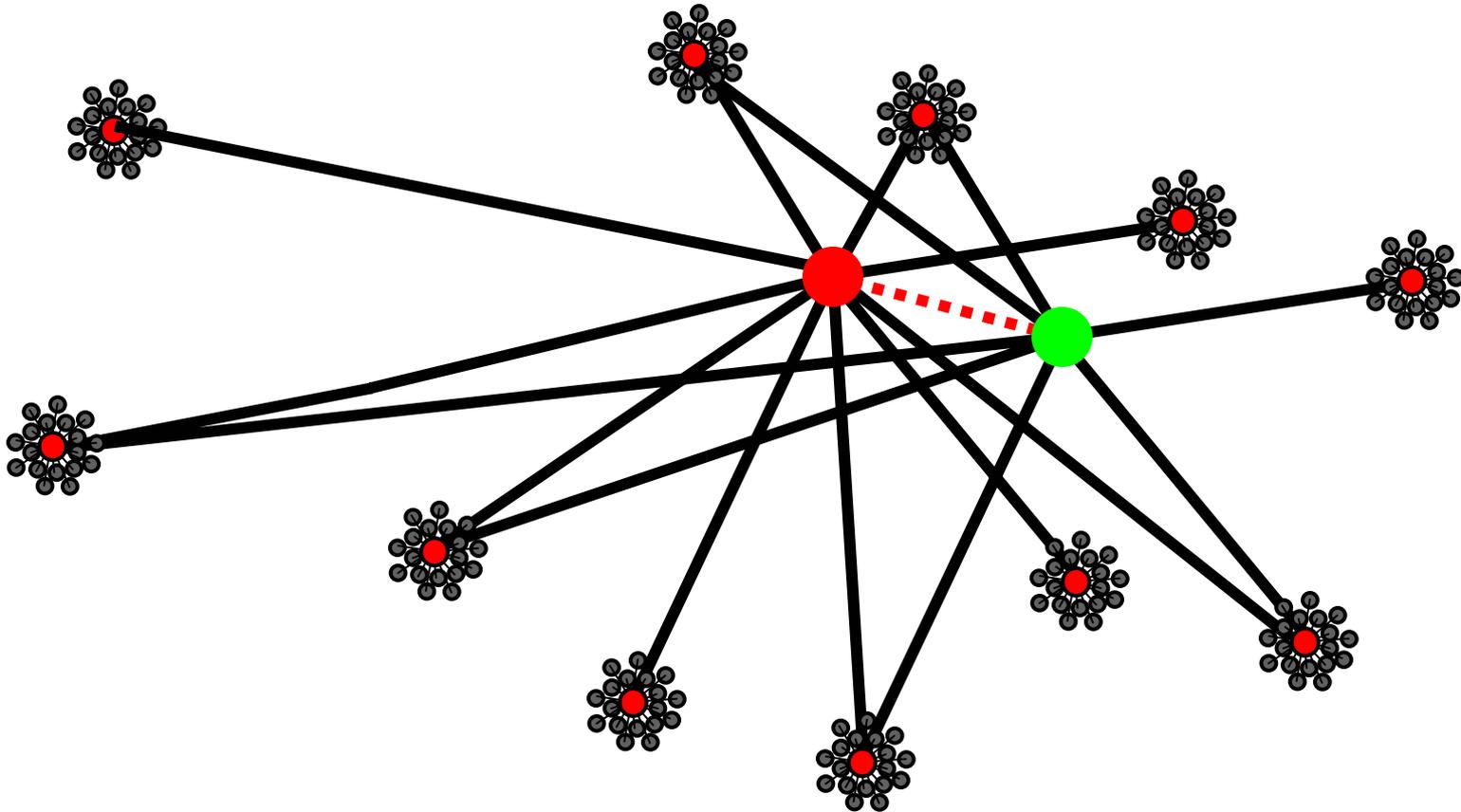
Interoperable Networks with National Clinical Research Corps
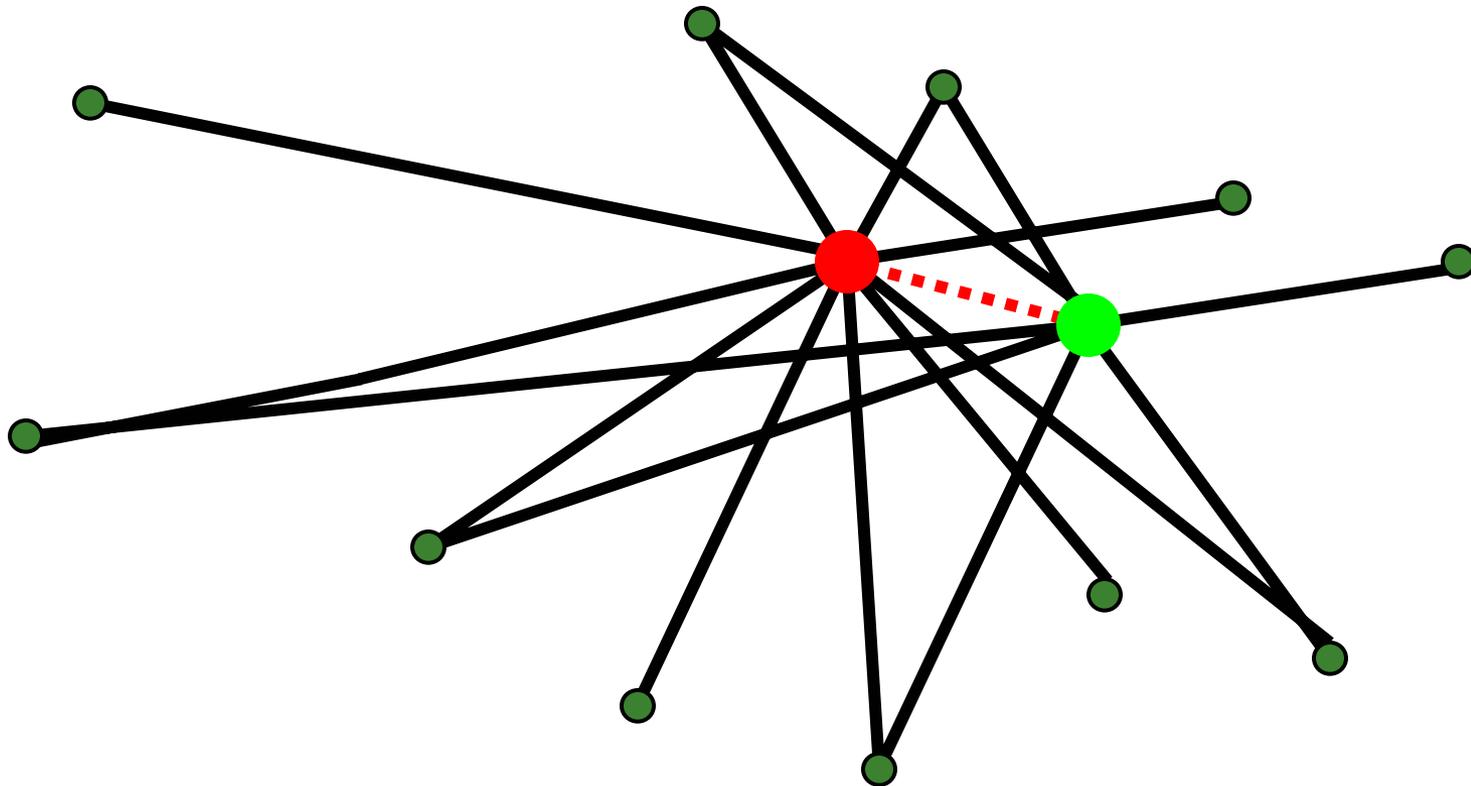
Need NCRC; Interoperable Networks; Informatics

Integration of Clinical Research Networks

- **Create an interoperable 'Network of Networks'**
  - **<u>N</u>ational <u>E</u>lectronic <u>C</u>linical <u>T</u>rials/<u>R</u>esearch Network (NECTAR)**
  - **Common data standards, interoperable informatics systems**
  - **Software application tools for protocol preparation; IRB management; adverse event reports; data collection, analyses & reporting**
- **Use existing networks to rapidly address questions beyond their traditional scope**
- **?? Link to Nat'l Clinical Research Corps of community (practice)-based caregivers**

# NECTAR: Development and Feasibility Studies and Demonstration Projects

- Common informatics standards
- Inter-operable interfaces
- Informatics tools that link patient care data and clinical research data
- Standard core data elements across network(s)
- Standard definitions of diseases, conditions, and adverse events
- Sharing of data, images, specimens

Linking Databases and Data Mining

- Linking imaging and other databases with software tools

- Biomedical imaging, genomic, gene expression, and patient medical records data for personalized medicine

- Data integration, knowledge extraction, and clinical interpretation, of heterogeneous clinically relevant data

- Database development for software validation/FDA approval

Biomedical Informatics for Clinical Decision Support

Software for Clinical Decision Support

Biology Data: Genomics and Proteomics

⬅➡
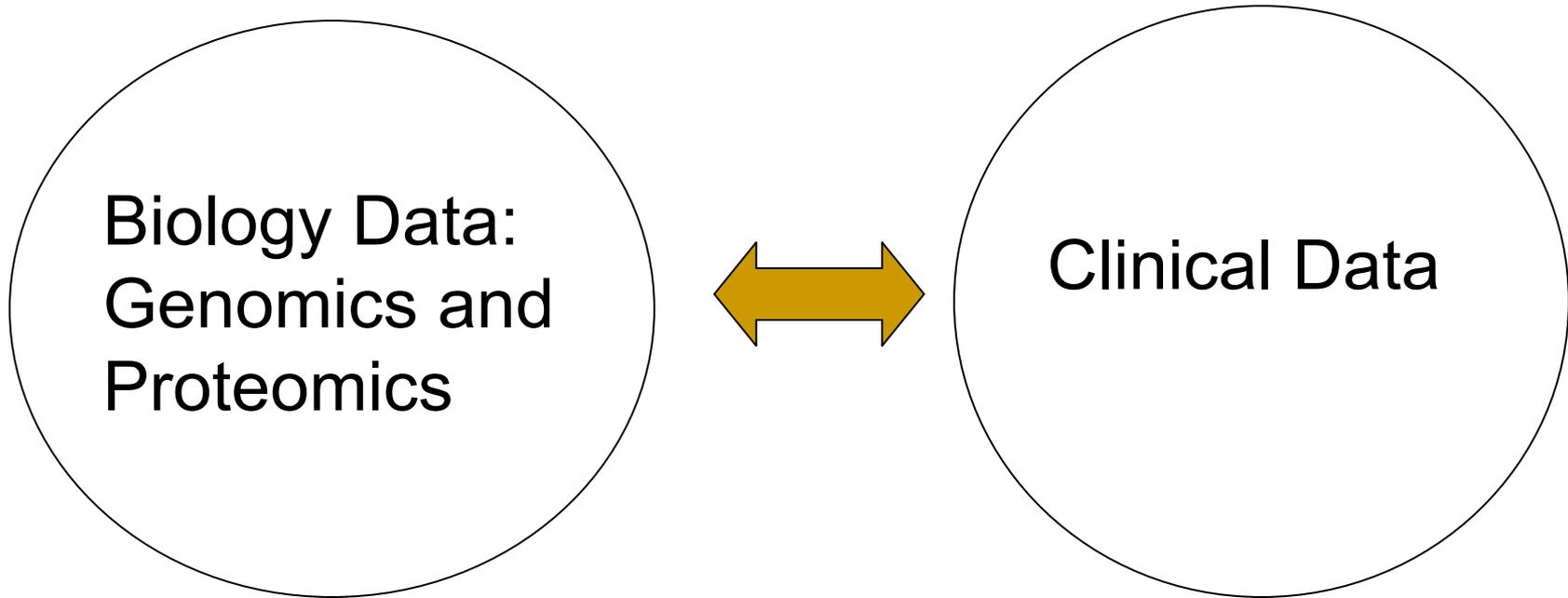
Clinical Data

Translational software tools and methods that extract and integrate heterogeneous clinical and molecular biology data

Research Opportunities

- Build on NECTAR feasibilities studies, develop software tools for clinical trial networks, enable more standardized methods for meta data analysis

- Develop software tools for analysis across heterogeneous clinical and scientific databases

- Academic-industry partnerships are feasible for translational research

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Roadmap Funding

- All Institutes and Centers committed to invest jointly in a pool of resources to support current and future Roadmap initiatives

- $128 M in FY 2004 (DDF funds and ~ 0.34% each ICs budget)

- Cumulatively over $2 B by FY 2009

Roadmap Funding

(dollars in millions)

|	FY04	FY05	FY06	FY07	FY08	FY09	Total
Pathways to Discovery	64	137	169	182	209	188	948
Research Teams	27	39	44	92	96	93	390
Clinical Research	38	61	120	174	214	227	833
Total	128	237	332	448	520	507	2,172

Consultation

Participation, consultation, collaboration, and funding are needed from patients, health care providers, foundations, industry, academia, Federal partners …all stakeholders

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The NIH Roadmap:
A Work in Progress

NIH

**Ideas
People
Resources**