

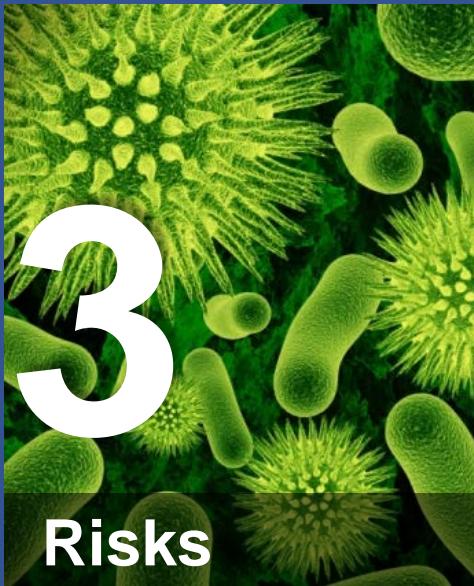
Extraordinary Opportunities in Biomedical Research

Francis S. Collins, M.D., Ph.D.
Director, National Institutes of Health
Seoul National University
September 8, 2015



Global Health Security Agenda

International effort to accelerate progress towards strengthening global capacity to prevent, detect, and respond to infectious disease threats



3

Risks

- Emerging organisms
- Drug resistance
- Intentional creation



3

Opportunities

- Public health framework
- New lab and surveillance tools
- Successful outbreak control



3

Priorities

- Prevent wherever possible
- Detect rapidly
- Respond effectively

Global Health Security Agenda

International effort to accelerate progress towards strengthening global capacity to prevent, detect, and respond to infectious disease threats

Global Health Security Agenda 2015 High-Level Meeting in Seoul

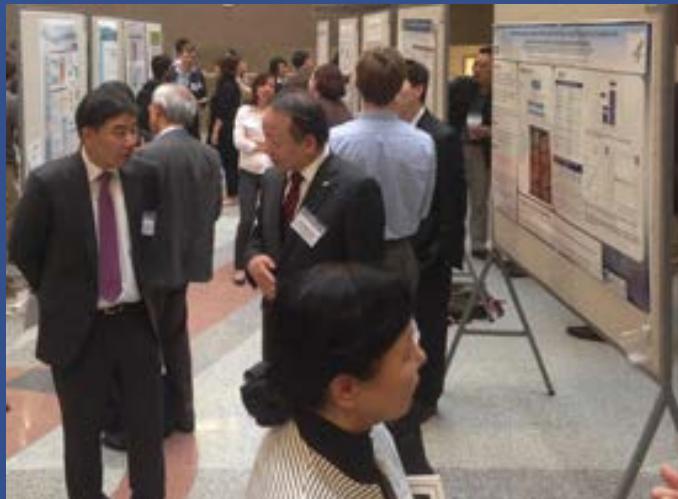
2015년 글로벌보건안보구상 고위급 회의



NIH and Korea: Advancing Biomedicine

- Awards: NIH currently supports 139 awards to Korean researchers partnering with US institutions
- Fellowships: 245* Korean post-doctoral researchers working on NIH campus
- Symposium on NIH-Korea Research

*Fiscal Year 2014



NIH National Institutes of Health
Turning Discovery Into Health

KHIDI
Korea Health Industry Development Institute

NIH-KSA
NIH-Korean Scientists Association

Inaugural Symposium on U.S. NIH-Korea Collaborative Biomedical Research

April 16–17, 2015
Lipsett Amphitheater, FAES Classrooms & Terrace
Clinical Center, Bldg. 10, NIH, Bethesda, MD

Program and Abstracts



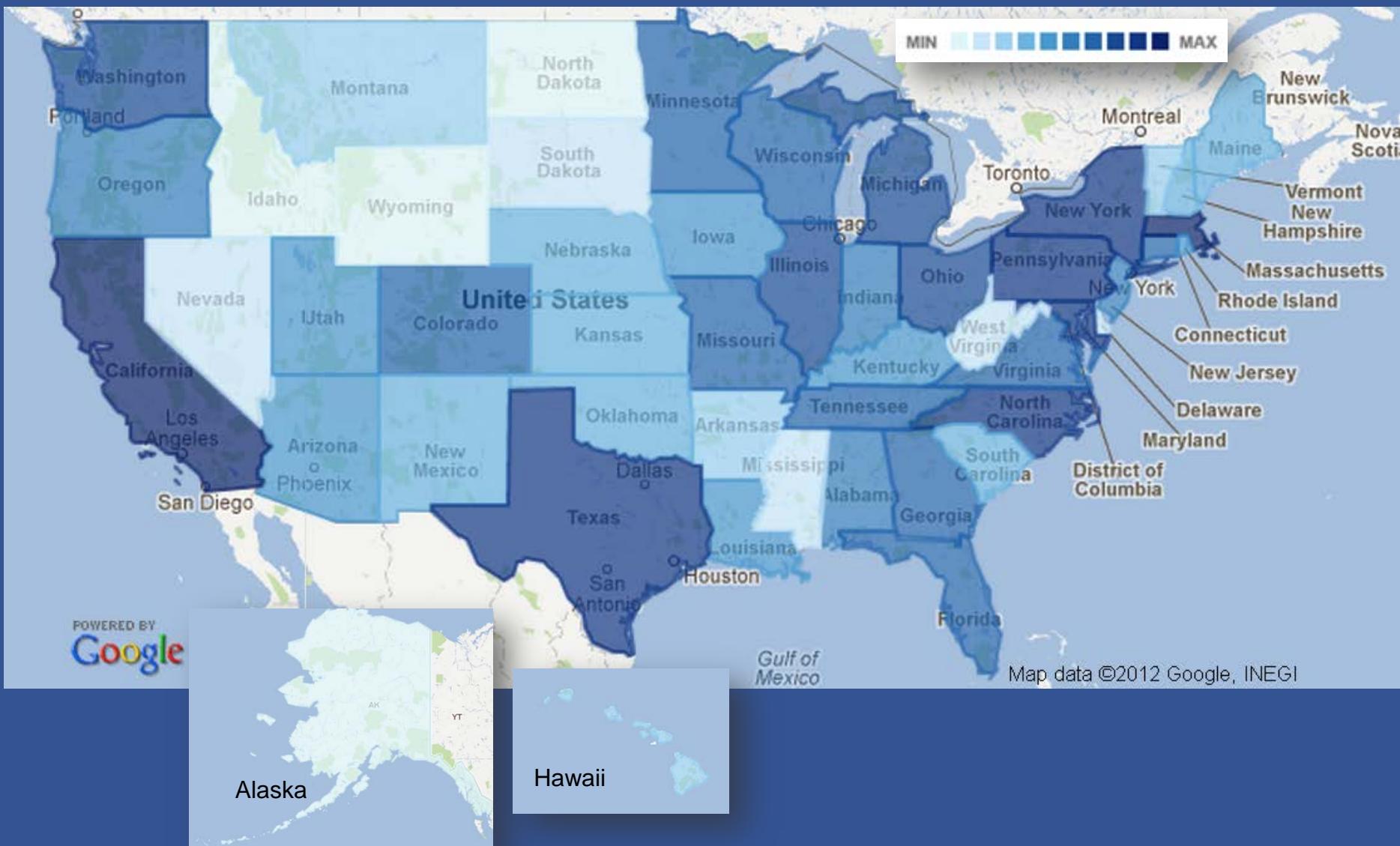
NIH: Steward of Medical and Behavioral Research for the United States



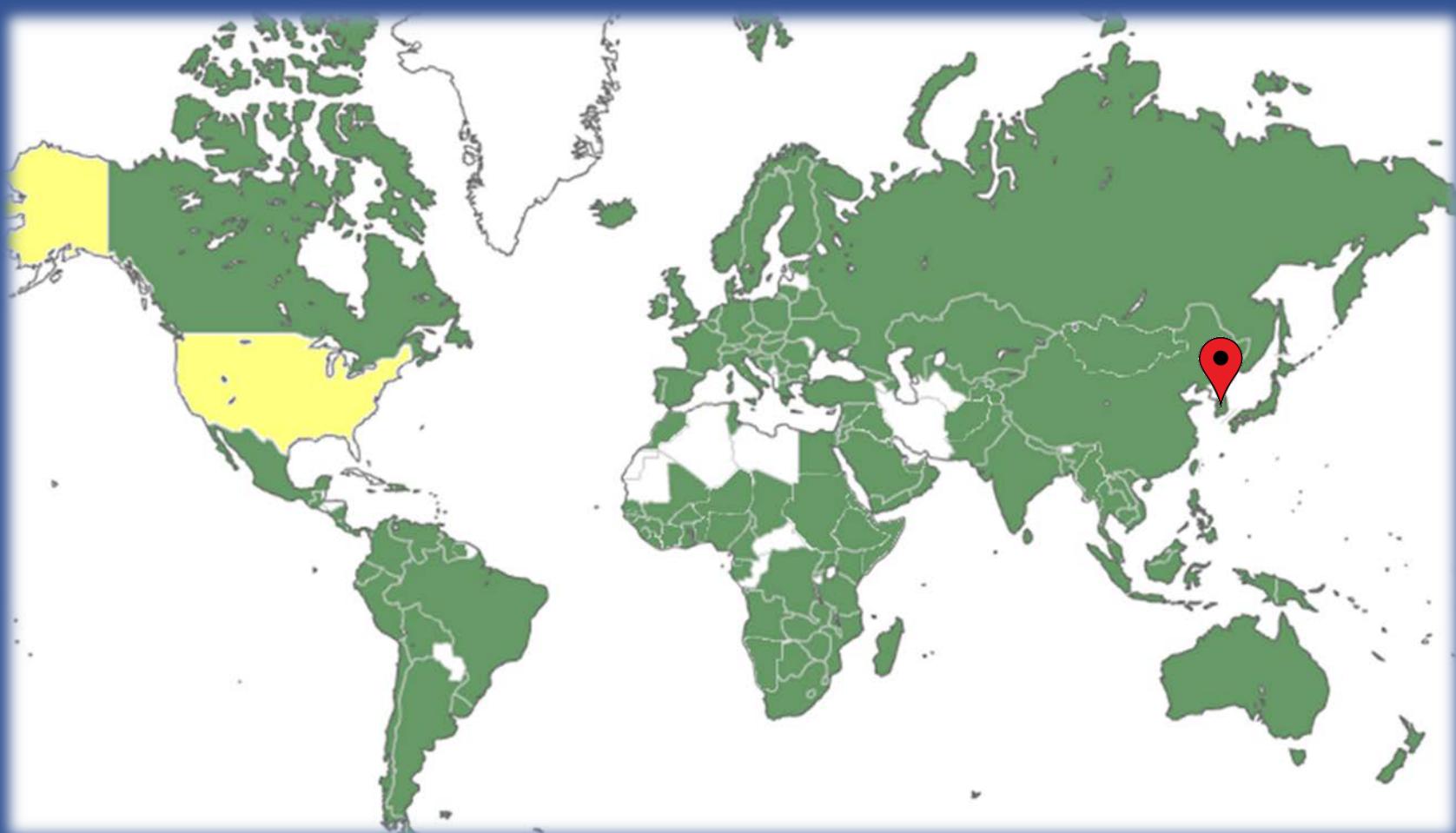
“Science in pursuit of fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to extend healthy life and reduce illness and disability.”



NIH Funds Scientists Across U.S.



... and around the globe ...



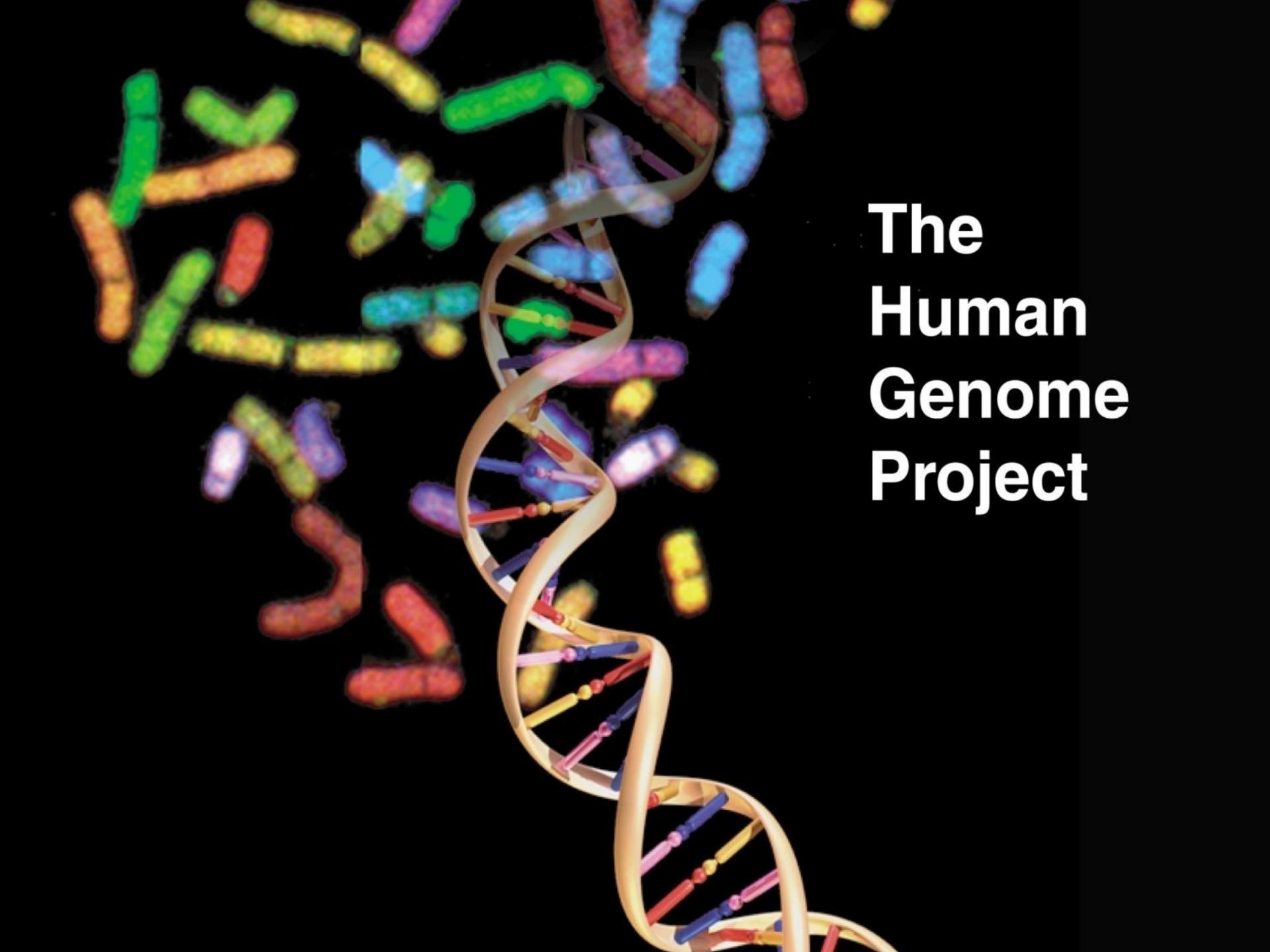
Source: Foreign Tracking System

Extraordinary Opportunities in Biomedical Research

- Unraveling Life's Mysteries through Basic Research
- Translating Discovery into Health
- Global Health, Global Partnerships
- Bringing Biomedicine Full Circle Through Precision Medicine

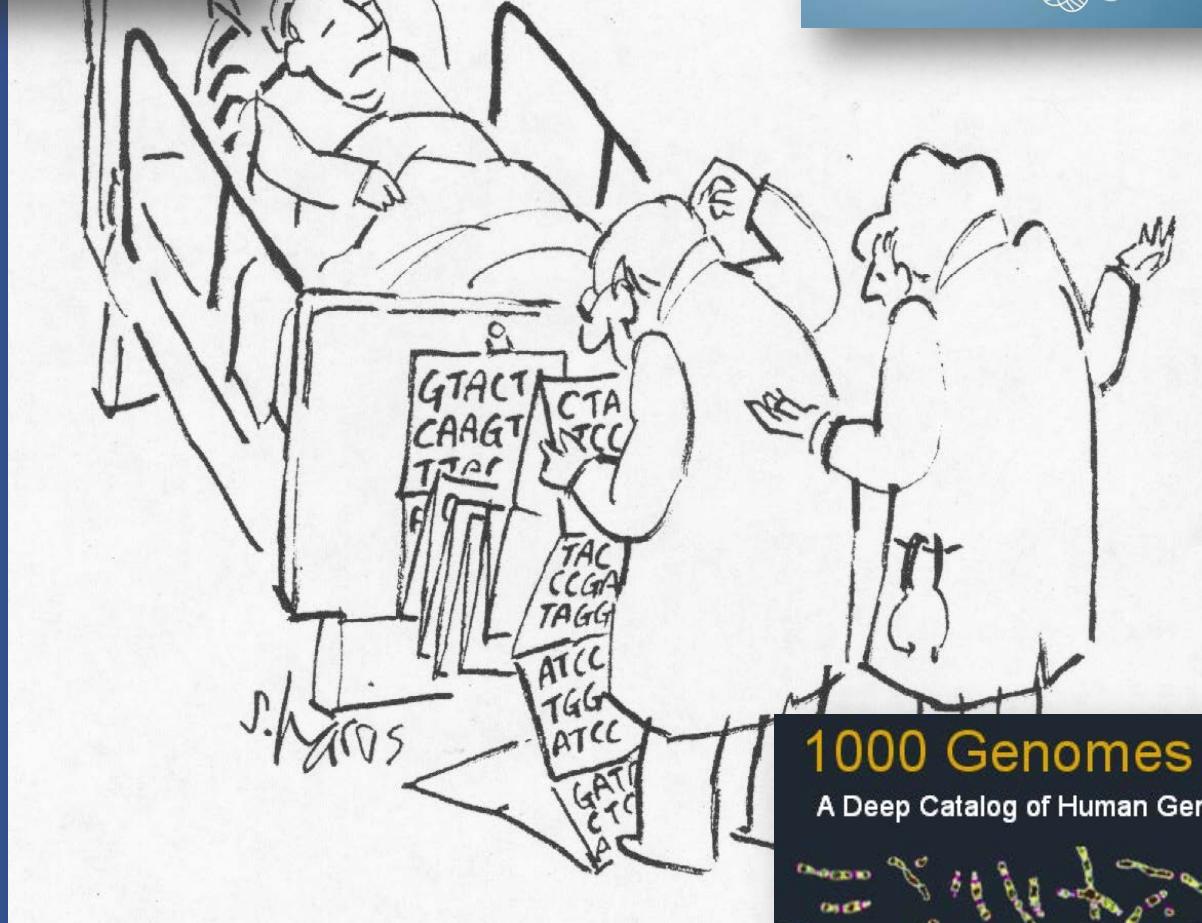
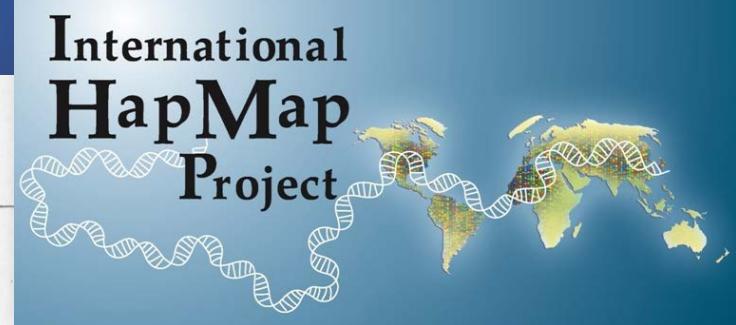
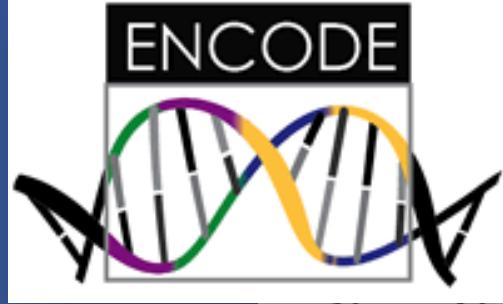


THE PRECISION MEDICINE INITIATIVE



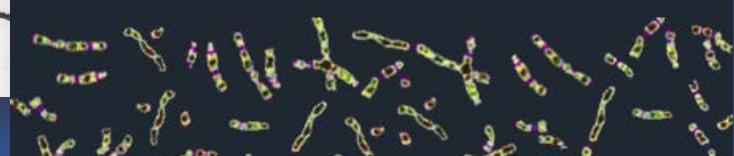
The Human Genome Project

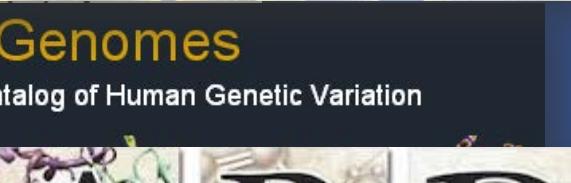
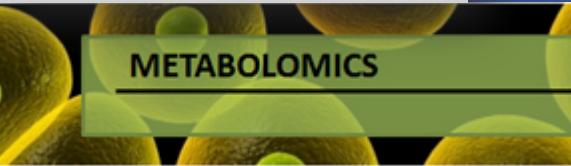
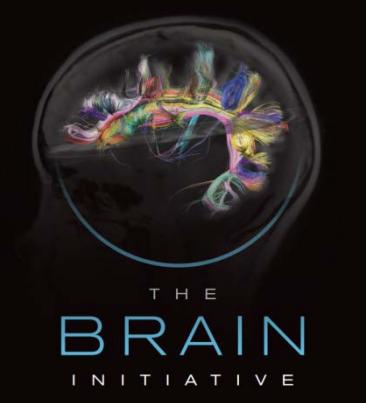
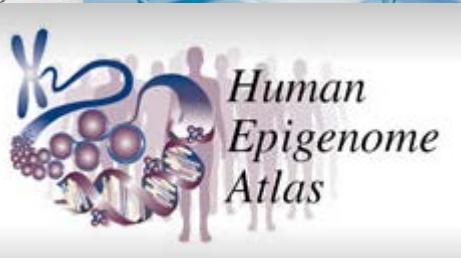
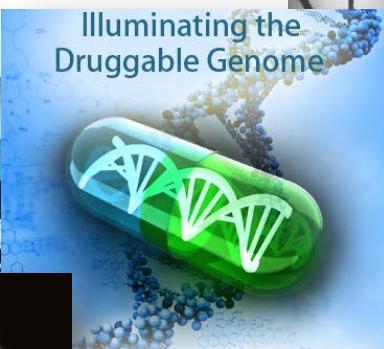
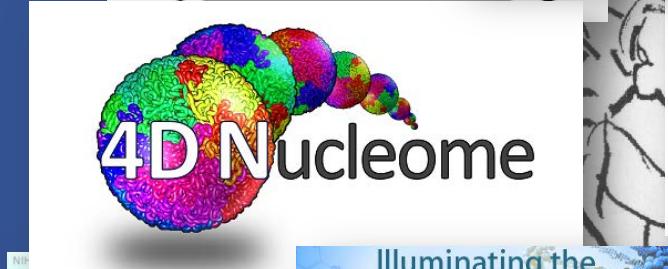
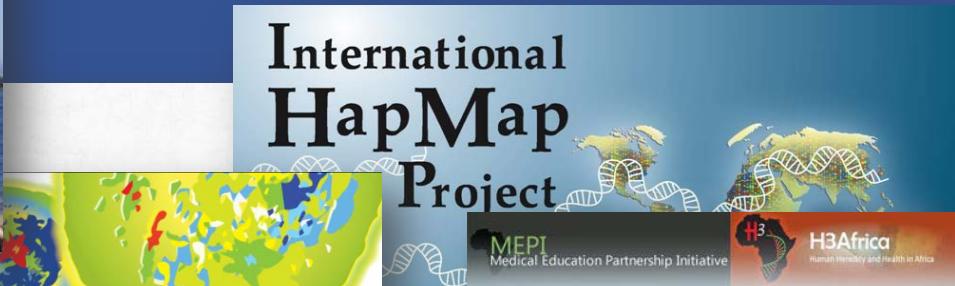
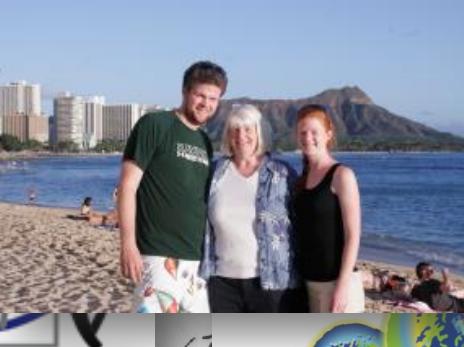




1000 Genomes

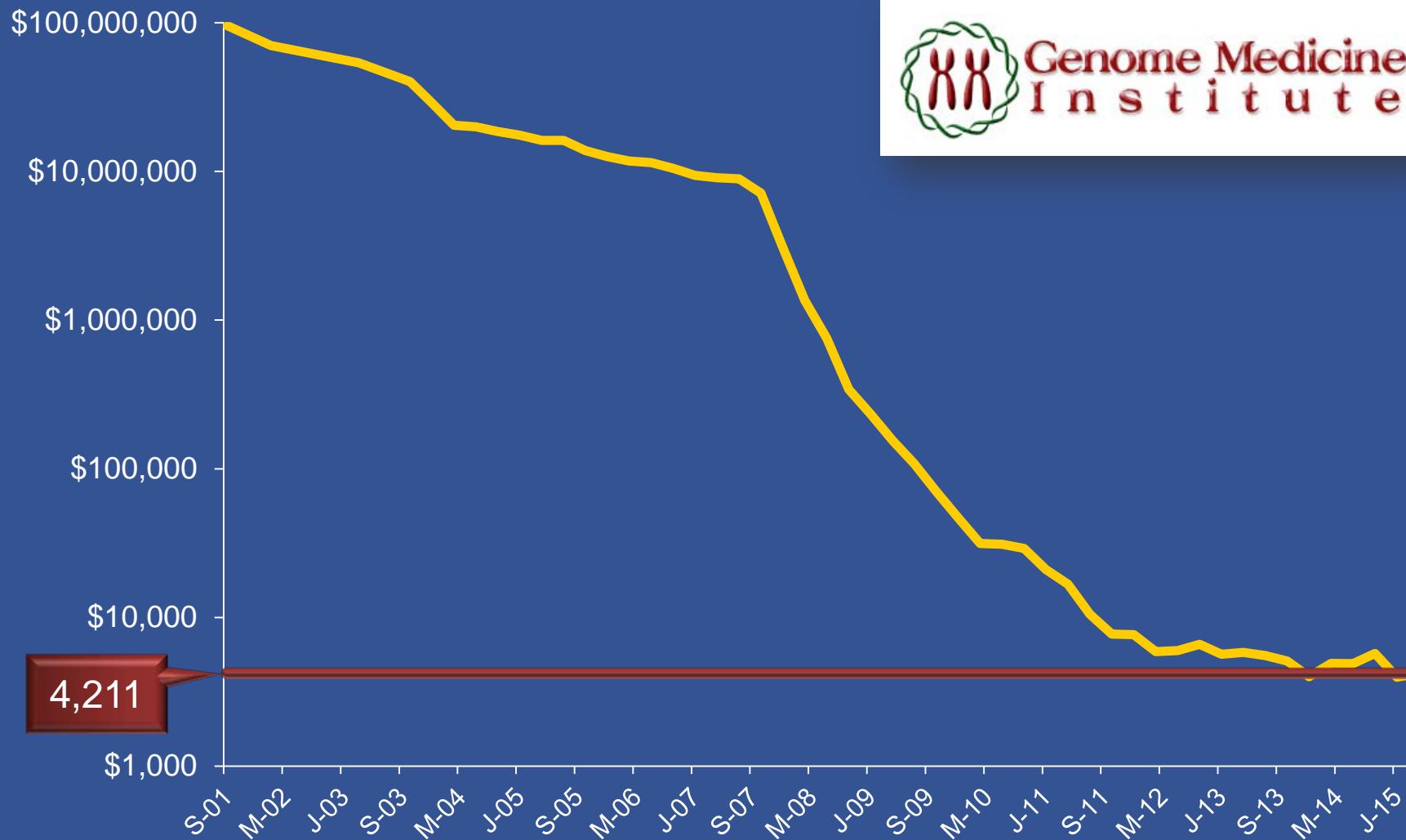
A Deep Catalog of Human Genetic Variation



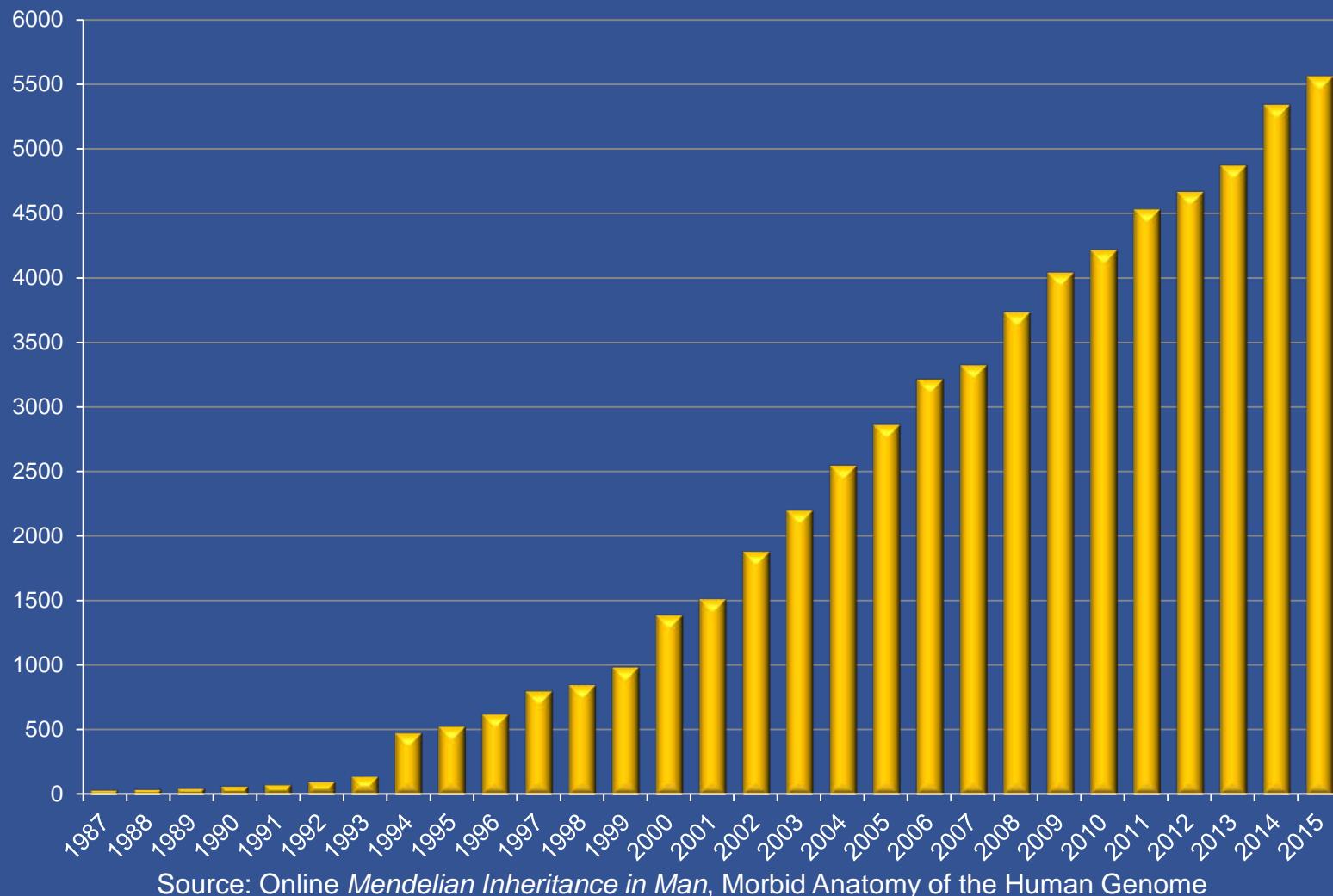


Cost of Sequencing a Human Genome

September 2001–April 2015



Disorders with Known Molecular Basis



Undiagnosed Diseases and NIH

- Undiagnosed Diseases Program (UDP)
 - Patients with longstanding, undiagnosed medical conditions are seen at NIH Clinical Center
 - UDP's NIH-wide staff, led by Dr. William Gahl, has:
 - Evaluated ~3,000 medical records
 - Accepted ~700 cases
 - Determined some diagnosis in ~25%

HARD CASES: INVESTIGATING RARE & TOUGH DISEASES

Dr. William Gahl is one of the last, best hopes for people suffering from rare, debilitating, and undiagnosed medical conditions

2013
FEB 25

CBSNews.com / CBS Evening News / CBS This Morning



60 MINUTES



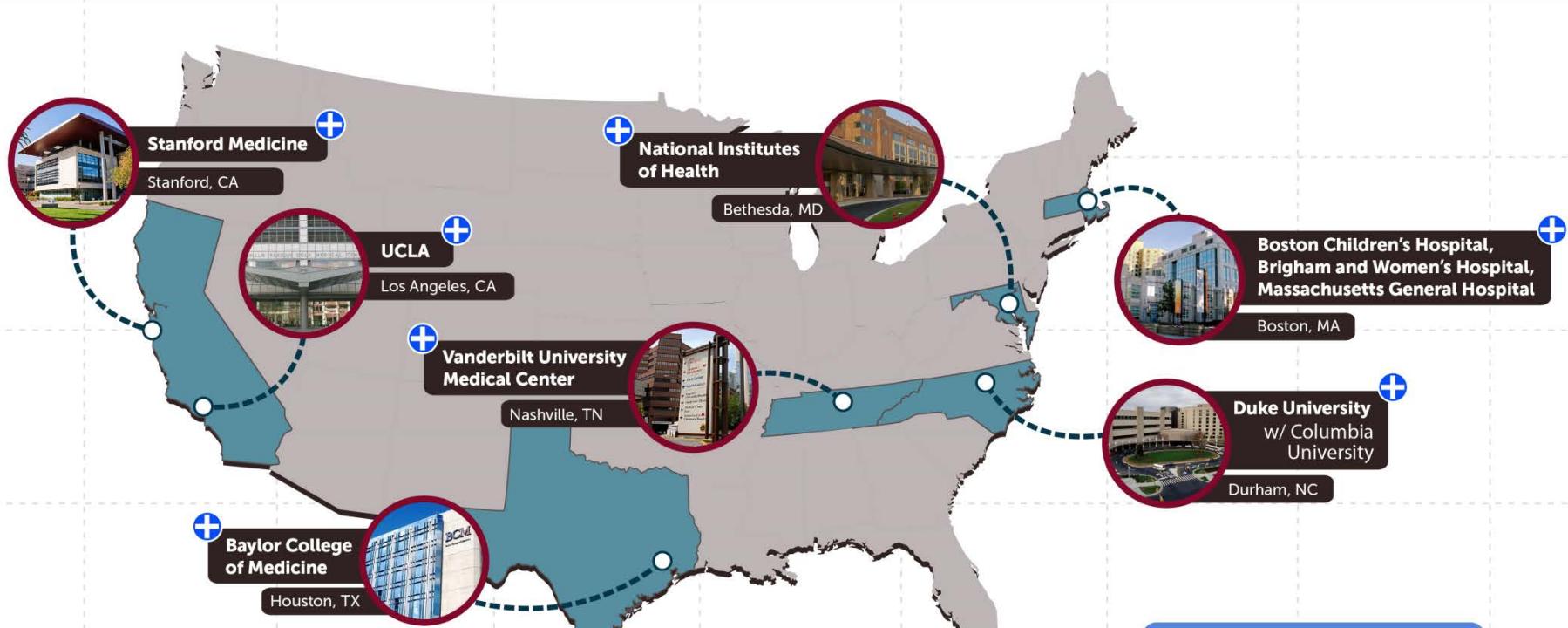
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- From UDP to “UDN”: expanding the model ...





Seven clinical site locations

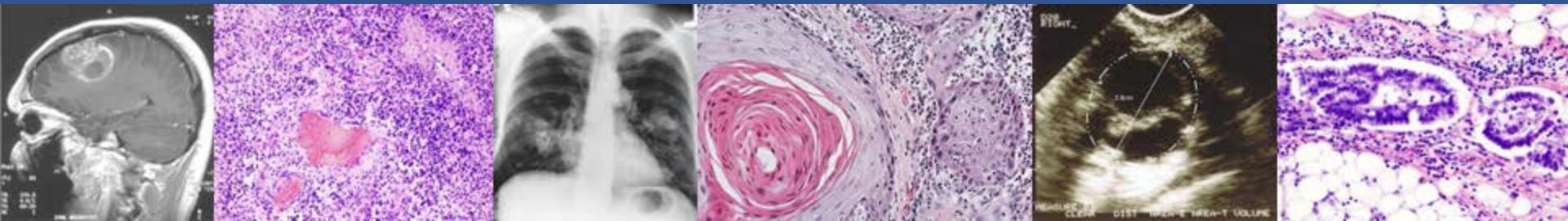


Seven UDN Clinical sites

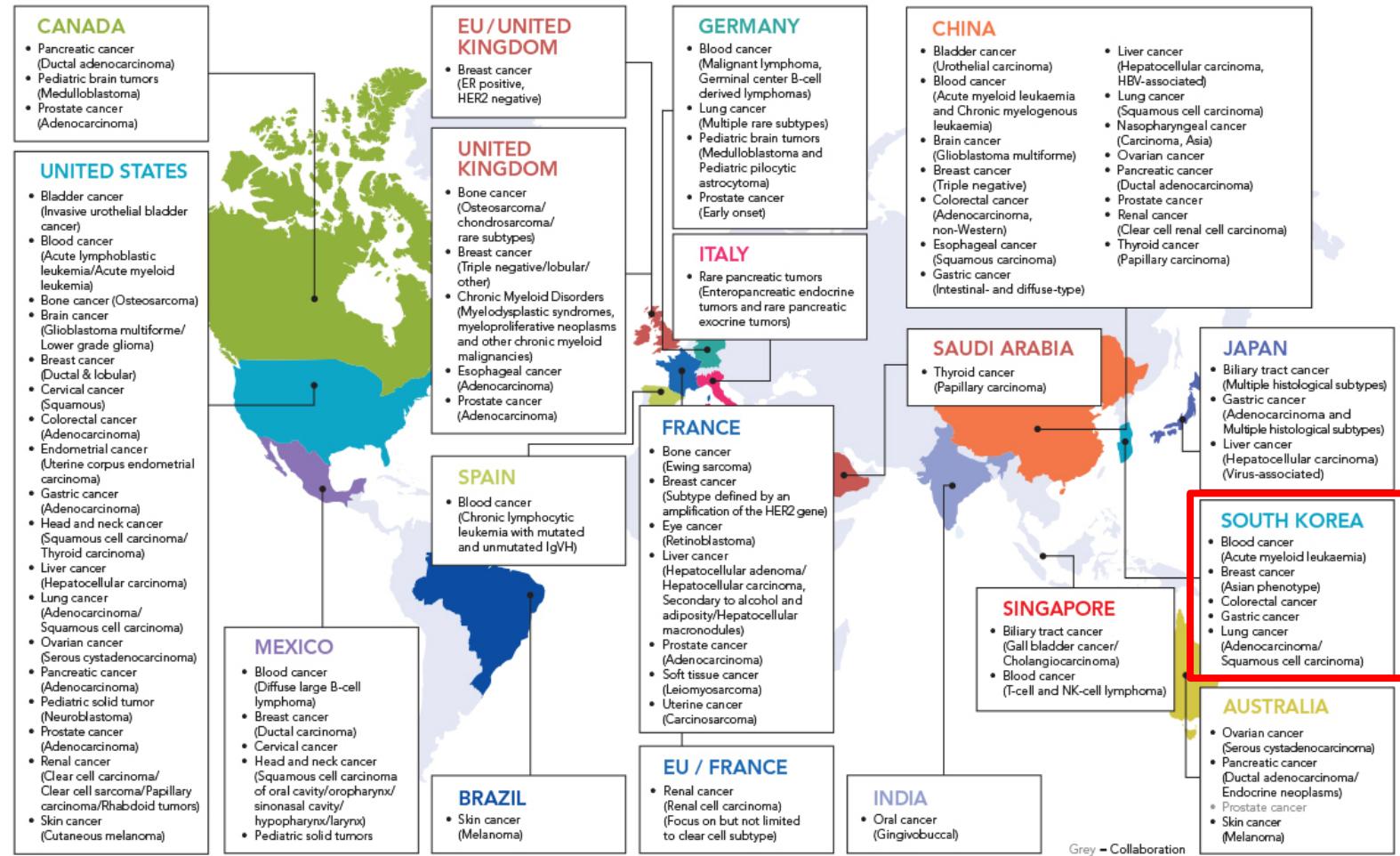
The NIH site will continue to enroll about 150 patients per year, each of the clinical sites will ultimately enroll about 50 patients per year.

The Cancer Genome Atlas (TCGA)

- Coordinated effort to accelerate understanding of cancer through genome analysis to improve diagnosis, treatment, prevention
- Complete characterization of ~35 adult cancers
 - ~20 common cancers at 500 cases each
 - ~15 rare cancers at 50-150 cases each
 - Seoul National University Hospital investigator contributed samples for Acute Myeloid Leukemia study
- Project ending in 2016 – future projects to use infrastructure



International Cancer Genome Consortium



78 projects in 17 jurisdictions

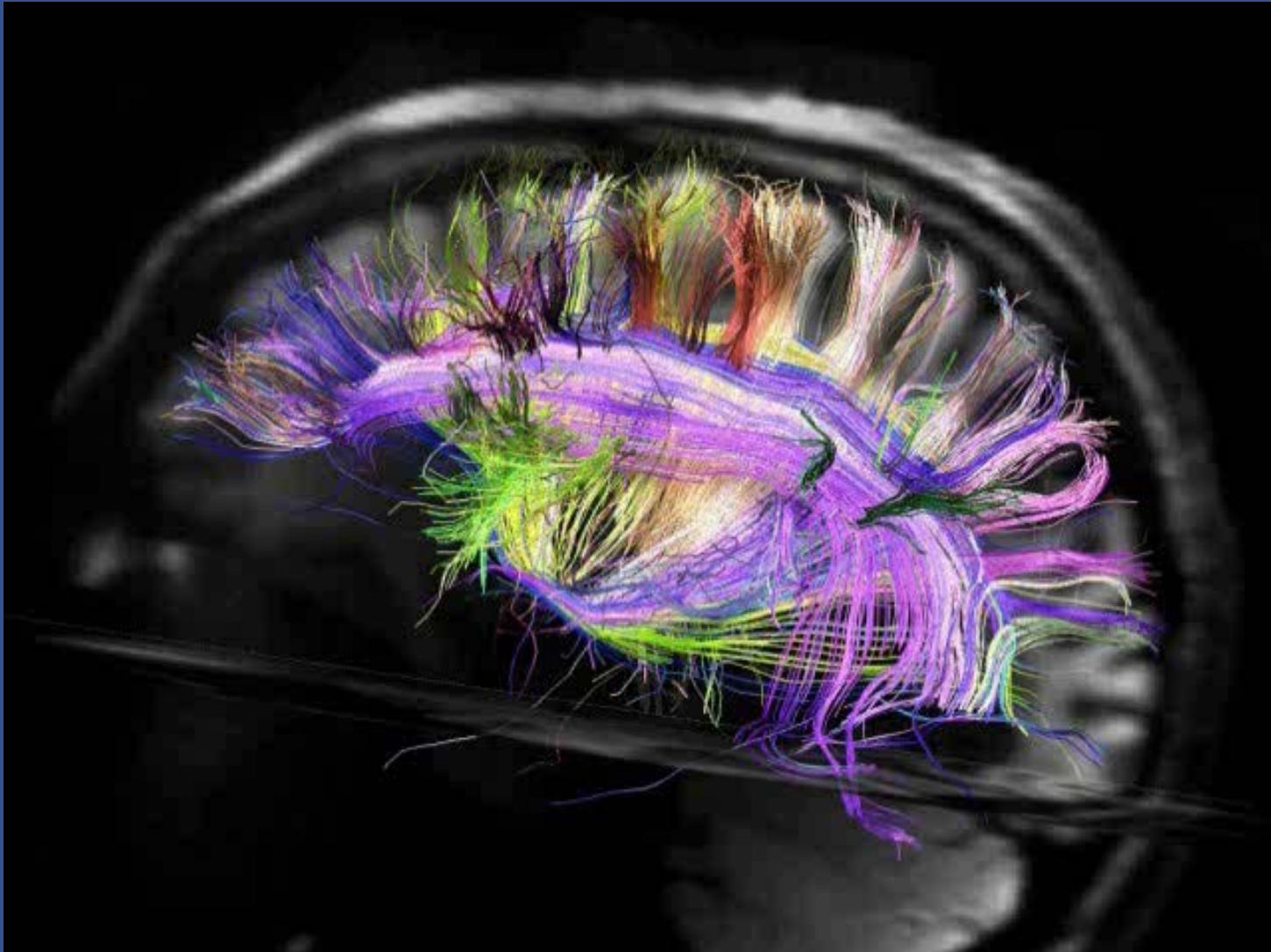


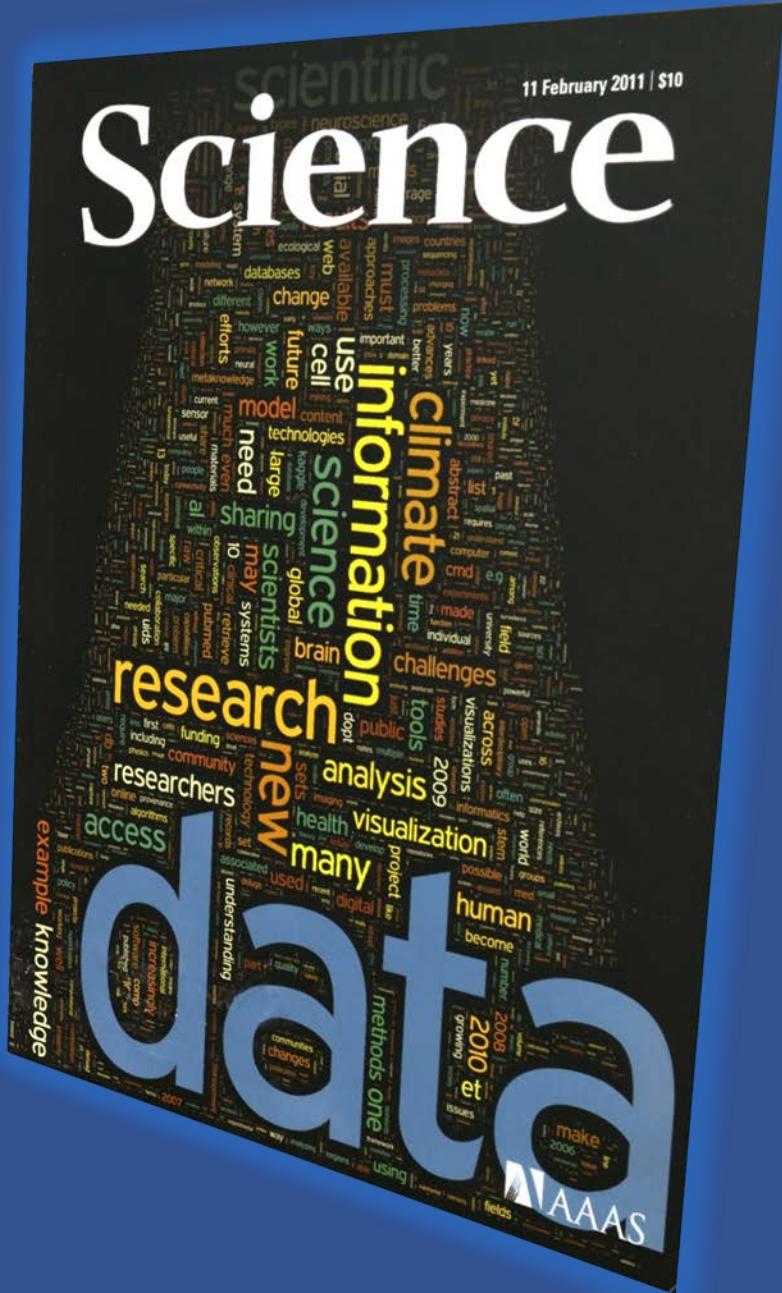
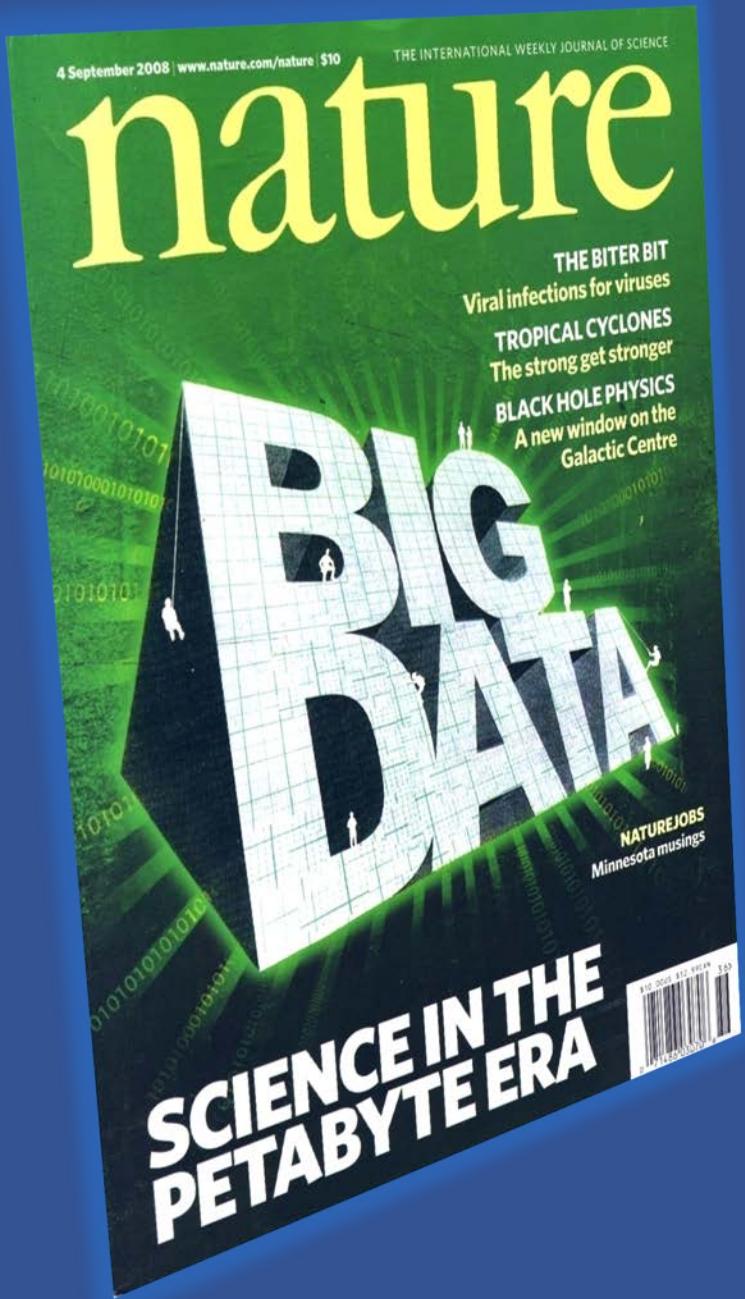
ICGC

Understanding the Brain ... Capturing the Imagination



Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative





Harnessing Data to Improve Health: BD2K (Big Data to Knowledge)

NIH's 6-year initiative *to use data science to foster an open digital ecosystem that will accelerate efficient, cost-effective biomedical research to enhance health, lengthen life, and reduce illness and disability*

Programs and activities:

- Advance discovery for biomedical research
- Facilitate use and re-use of biomedical data
- Develop analytical methods and software
- Enhance biomedical data science training



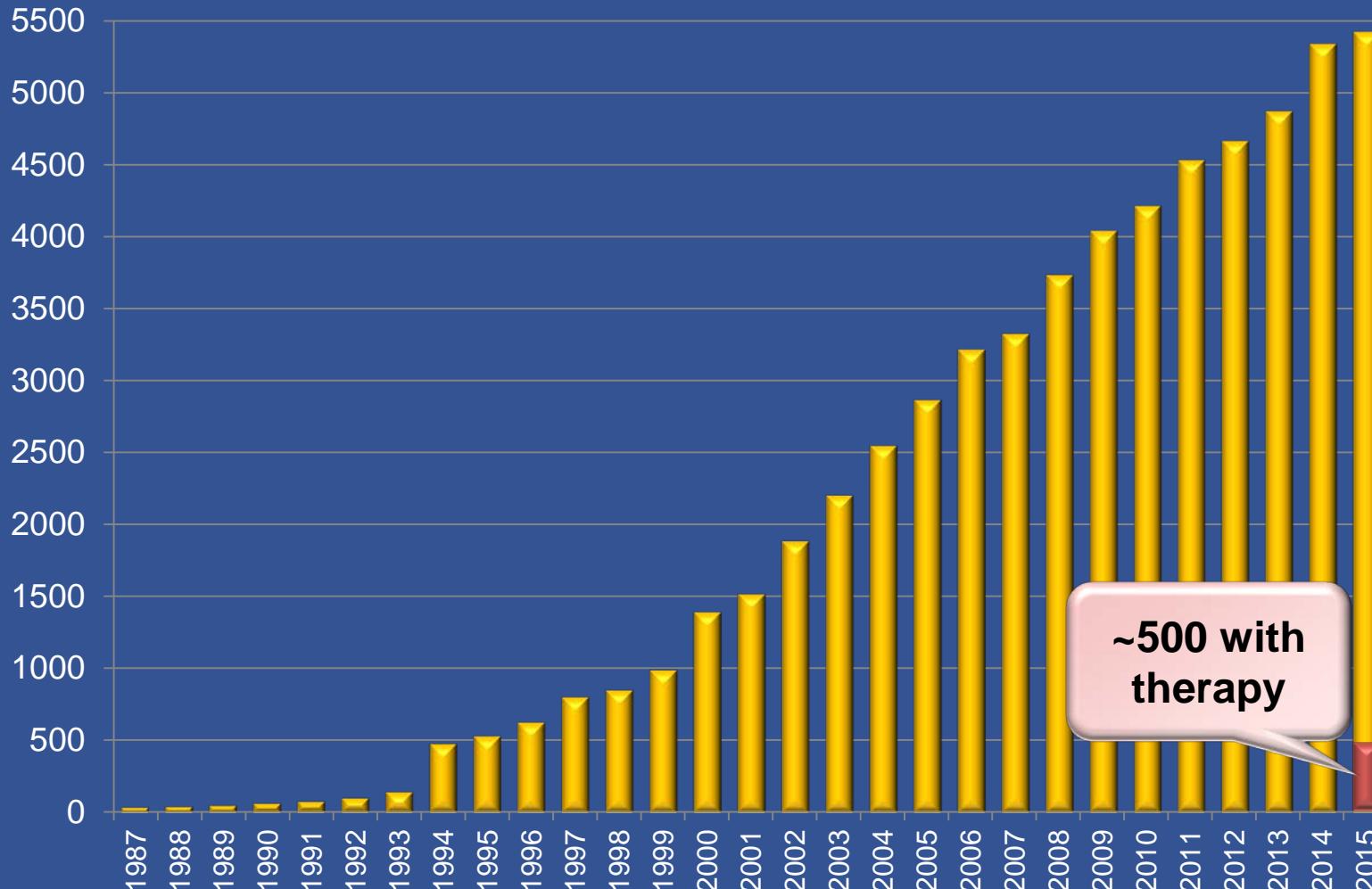
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THE PRECISION MEDICINE INITIATIVE

Disorders with Known Molecular Basis



Source: Online Mendelian Inheritance in Man, Morbid Anatomy of the Human Genome

~500 with
therapy

National Center for Advancing Translational Sciences (NCATS)

Mission:

To catalyze the generation of innovative methods and technologies that will enhance the development, testing, and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions.

<http://ncats.nih.gov/>



Therapeutics for Rare and Neglected Diseases (TRND) Program

To speed the development of new drugs for rare and neglected diseases

- Model: collaboration between NIH labs and extramural labs with appropriate expertise
- Projects:
 - May enter at various stages of development
 - Taken to stage needed to attract external organization to adopt for final clinical development
 - Serve to develop new generally applicable platform technologies and paradigms
- Encourages creative partnerships and novel approaches to intellectual property



Cancer Immunotherapy: *Science* Breakthrough of the Year (2013)



What stops the body from waging its own “war on cancer”?

- New approaches unleash the power of the immune system to search and destroy cancer cells
 - Blocking protein receptors that hinder immune response
 - CTLA-4
 - Anti-PD [programmed death] 1
 - Genetically engineering immune cells to target tumor cells

How the Promise of Immunotherapy Is Transforming Oncology

By Ron Winslow

The Wall Street Journal

Dec. 4, 2014



Carter getting immunotherapy for cancer in brain

By Todd Ackerman | August 20, 2015 | Updated: August 20, 2015 11:21pm

Former President Jimmy Carter, who announced Thursday that his cancer had spread to his brain, will be treated with a promising approach.

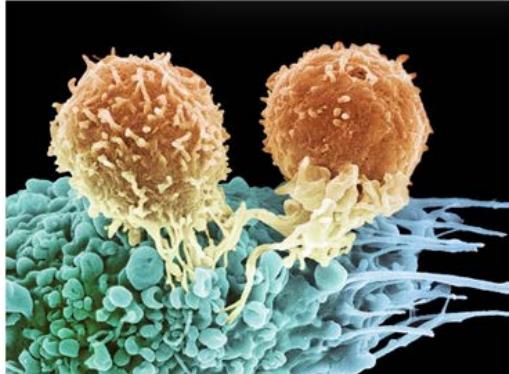


PRESS RELEASE

Immune Design Announces Phase 2 Cancer Immunotherapy Trial Collaboration

Published: Aug 12, 2015 8:00 a.m. ET

Plan to Evaluate Combination of CMB305 and Genentech's Atezolizumab in Patients With Soft Tissue Sarcoma



STEVE GSCHMEISSNER/SCIENCE SOURCE

Multiple boosts for cancer immunotherapy

By Mitch Leslie

26 November 2014 3:30 pm



Leukemia & Lymphoma

Immunotherapy Brings New Hope to Cancer Fight

Nov. 4, 2014

By Sonya Collins

WebMD Health News

SCIENCE

NEWS | IN DEPTH

16 JANUARY 2015 • VOL 347 ISSUE 6219

BIOMEDICINE

Google^[x] searches for ways to boost cancer immunotherapy

At gathering, researchers brainstorm “10x” ideas

By Jon Cohen, in Mountain View, California

Google^[x], a semisecretive branch of the company that's famous for making information as accessible as possible, thinks big—and wants others to do the same. When it comes to human disease, there are few bigger challenges than cancer. So last week, the corporate headquarters MEDPAGE TODAY^{*} hosted a gathering of researchers, entrepreneurs, and investors to brainstorm “10x” ideas for fighting cancer.

Founded that company's National Genetics Institute for blood screening and clinical research, he asked participants to think “very un-NIH-like.”

They were happy to do so. NIH's National Cancer Institute “hasn't recognized this area of science nearly enough,” meeting co-chair Phillip Sharp, a Nobel laureate based at the Massachusetts Institute of Technology in Cambridge, told AAAS, in inter-

ONCOLOGY/HEMATOLOGY 08.19.2015

Combo Immunotherapy Promising for Melanoma: Clin Onc News Report

— Also, two generic drugs can improve breast cancer survival

by Clinical Oncology News Staff

Together, immunotherapies ipilimumab and nivolumab increased progression-free survival in melanoma patients by almost 60% compared to ipilimumab alone, oncologists report.

Extraordinary Opportunities in Biomedical Research

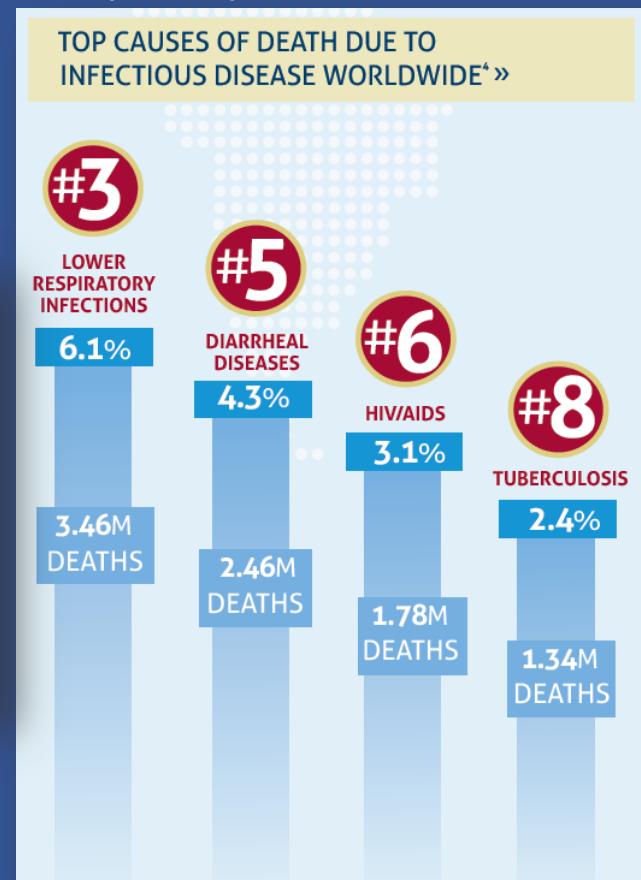
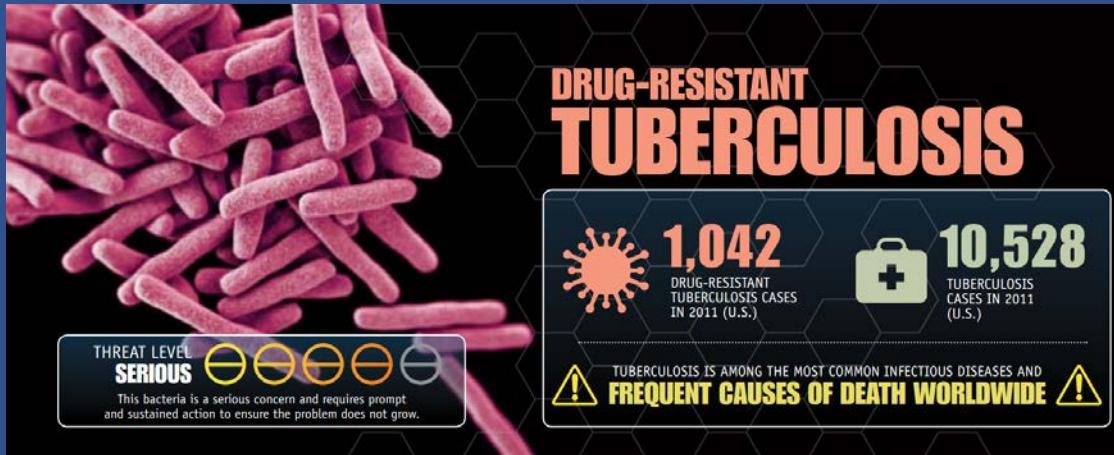
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THE PRECISION MEDICINE INITIATIVE

Global Challenges, Global Partnerships: Korea, NIH, and Tuberculosis

- Tuberculosis: a continuing global threat ...
 - And a rapidly-widening global crisis: development of multi-drug resistant (MDR); extremely drug resistant (XDR), strains

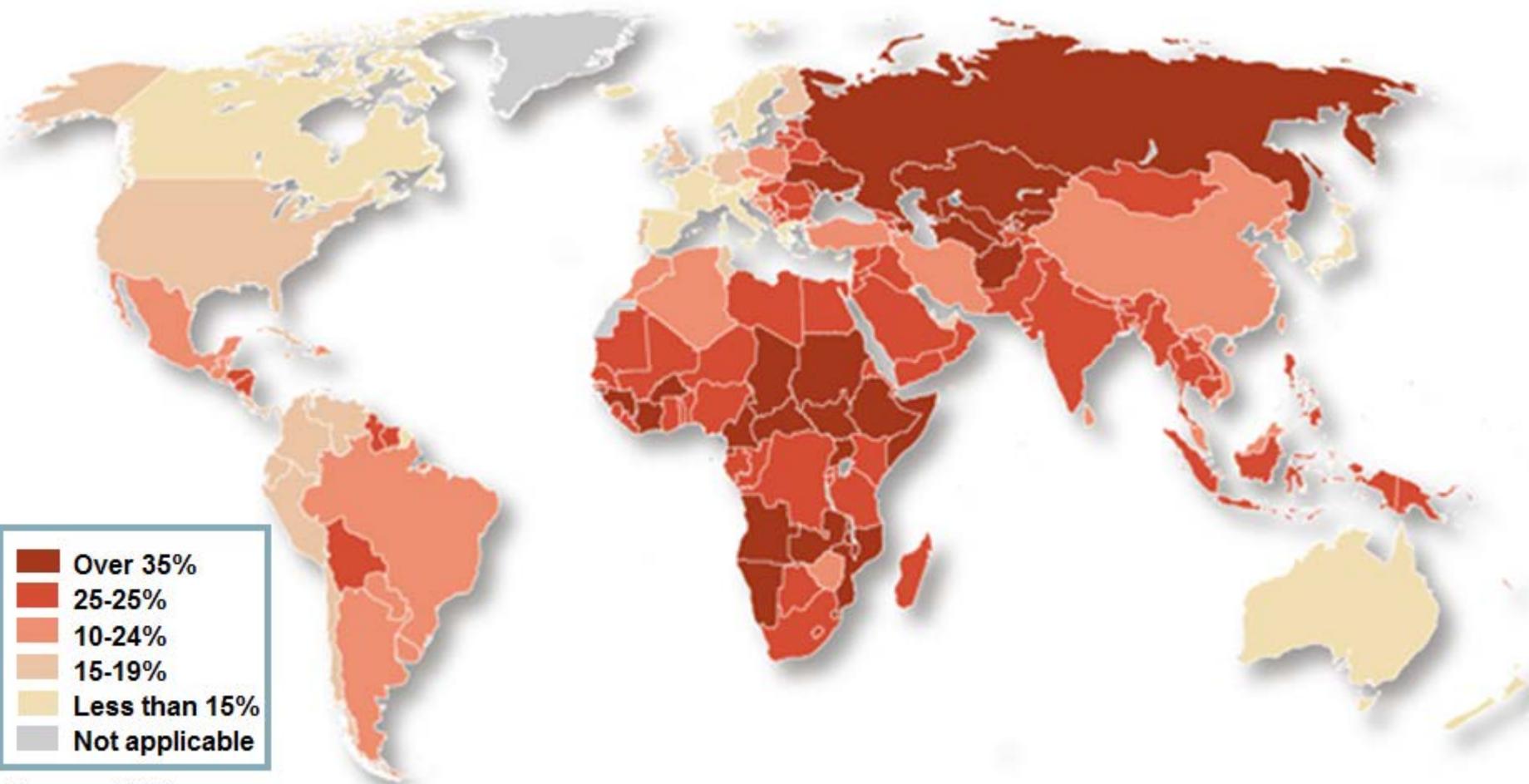


Global Challenges, Global Partnerships: Korea, NIH, and Tuberculosis

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 - And a rapidly-widening global crisis: development of multi-drug resistant (MDR); extremely drug resistant (XDR), strains
 - Urgent need to monitor, understand, limit resistant strains
- NIH and Korea: rising to TB's challenges
 - To develop new drug regimes for effective treatment; led to:
 - Successful study of utility of Linezolid in XDR-TB patients
 - Critical validation studies of Cepheid GeneXpert diagnostic – led to WHO global endorsement
 - To evaluate early stage diagnostics in TB-endemic countries
 - South Korea is part of NIH-funded Tuberculosis Clinical Diagnostics Research Consortium

Emerging Challenges: Global Increase in Non-Communicable Disease Deaths

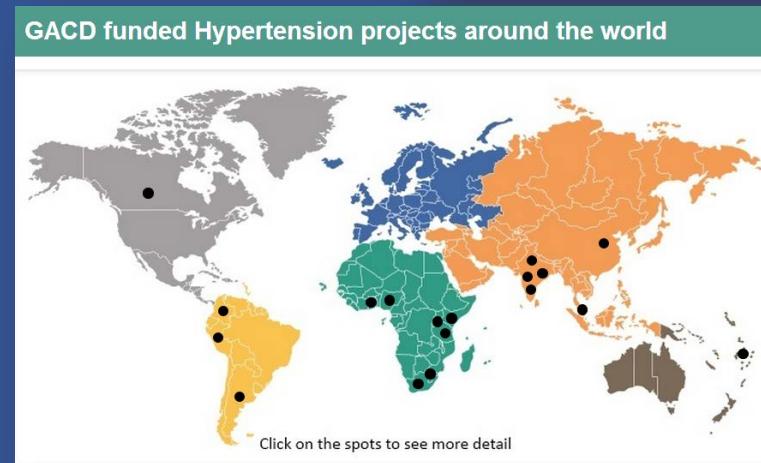
Probability of dying from a non-communicable disease
Between the ages of 30 and 70, 2008, %



Source: WHO

Global Alliance for Chronic Diseases

- Collaboration of world's major research funding agencies to address growing global burden of chronic disease
 - Representing ~80% of all public health research funding worldwide
 - Launched June 2009
 - Supports multi-country, multidisciplinary research focusing on needs of low- and middle-income countries; vulnerable populations
- Priority Areas:
 - Hypertension 2013
 - Diabetes 2014
 - Lung Disease 2016



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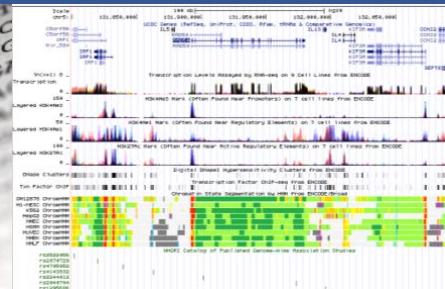
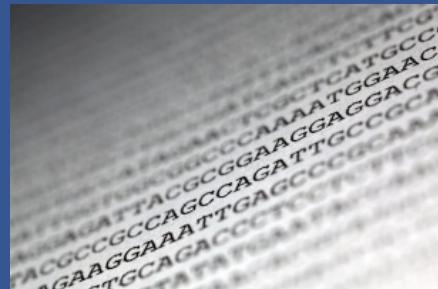
“Tonight, I’m launching a new Precision Medicine Initiative to bring us closer to curing diseases like cancer and diabetes – and to give all of us access to the personalized information we need to keep ourselves and our families healthier.”

President Barack Obama
State of the Union Address, January 20, 2015



Patient Partnerships

EHRs



Technologies

Genomics



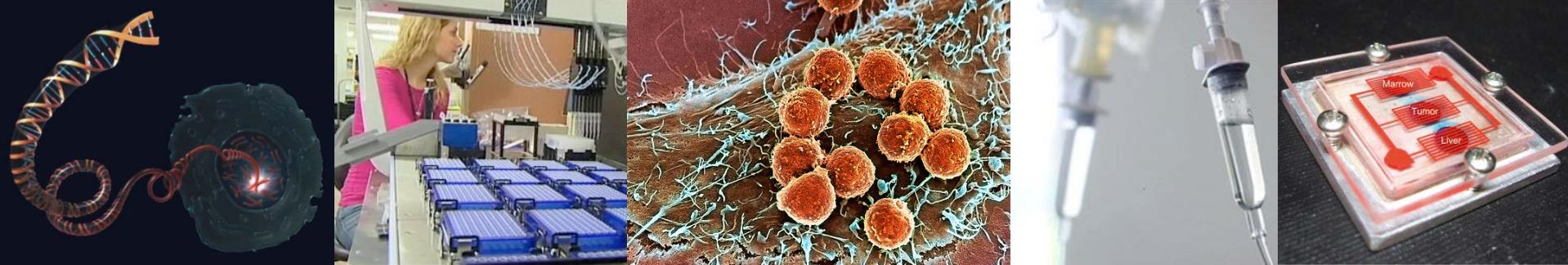
Data Science

Precision Medicine Initiative (PMI) for Oncology

Goal: Apply tenets of precision medicine to cancer

To reach this goal, PMI-Oncology will enable research to:

- Use NCI clinical trials as models
 - NCI-MATCH: solid tumors, lymphomas (multi-drug, multi-arm)
- Identify new cancer subtypes, therapeutic targets
- Partner with private sector to test multiple targeted drugs
- Test combination therapies – to go beyond remission to cure?
- Understand and combat drug resistance



PMI: National Research Cohort

- ≥1 million U.S. volunteers
 - Numerous existing cohorts (many funded by NIH)
 - Outreach to underrepresented groups
 - New volunteers
- Participants will be:
 - Centrally involved in design, implementation
 - Able to share genomic data, lifestyle information, biological samples – all linked to their electronic health records
 - Can choose how, when to participate in research studies
- Will forge new model for scientific research that emphasizes:
 - Engaged participants
 - Open, responsible data sharing with privacy protections



National Research Cohort: Possible Uses

- Provide data to reclassify disease categories
- Test pharmacogenomics: the right drug at the right dose
- Provide unbiased quantitative determination of disease risk
- Study factors contributing to disease resistance/resilience; possible source of new therapeutic targets
- Offer powerful test bed for:
 - Incorporating patient-reported outcomes
 - Improving utility of EHRs
 - Evaluating wide array of mHealth applications
 - Assessing consequence of environmental exposures



Pathways to Engage with NIH

- Investigator-initiated grant applications
 - Building academic partnerships through competitive research grant awards
 - grants.nih.gov
- Post-doctoral fellowship opportunities at NIH
 - Open to all foreign postdocs: NIH Visiting Scientist Program
 - training.nih.gov
 - Programs exclusive to Korean postdocs—jointly supported by Korea and NIH:
 - Korean Visiting Scientist Training Award (KVSTA)
 - Korean Biomedical Scientist Fellowship Program (KBSFP)
 - Contact Tina Chung (chungt@mail.nih.gov)



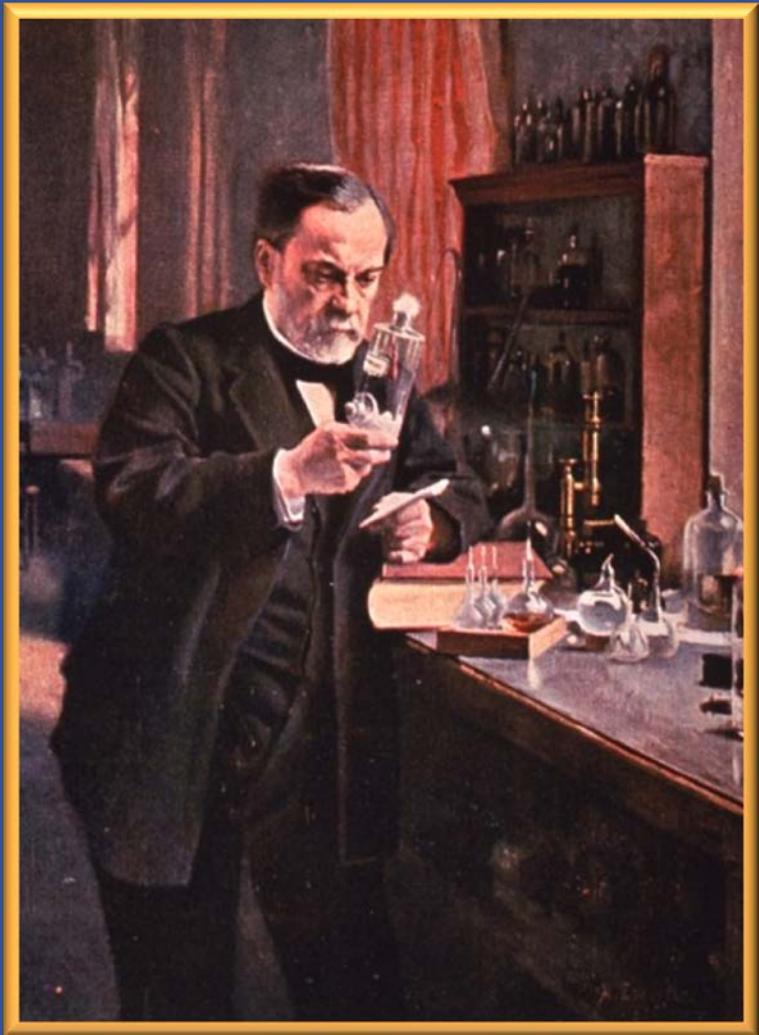
JANUARY/FEBRUARY 2013

New partnership models emerge for research training

New models of research training partnerships are emerging in the global health arena, reflecting the increased ability



For the first time, South Korea will fund 12 post-doctoral trainees at the NIH. Above, NIH Director Dr. Francis S. Collins and Korean official Dr. Kyung-Hwa Ko, hold the signed agreement.



Science knows no country, because knowledge belongs to humanity, and is the torch which illuminates the world

– *Louis Pasteur*



NIH... Turning Discovery Into Health

directorsblog.nih.gov

@NIHDirector 

