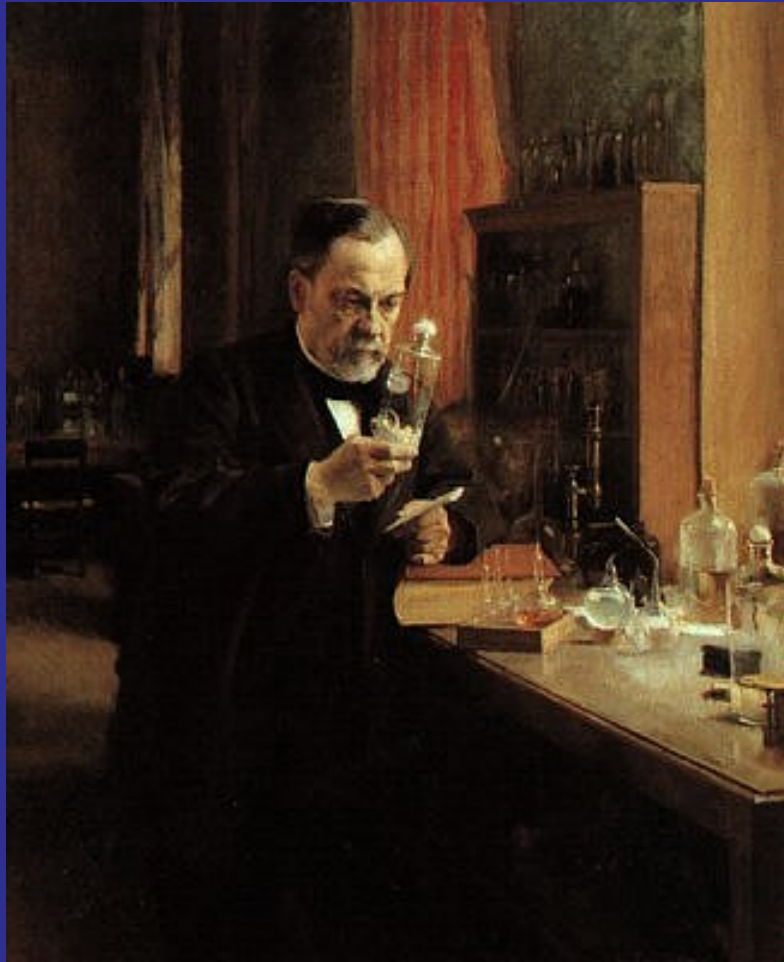


A Vision for Transforming Medicine in the 21st Century

Elias A. Zerhouni, M.D., Director
National Institutes of Health



The Promise of “Scientific” Medicine



Louis Pasteur (1822-1895)



学祖 北里柴三郎博士
(1852~1931)

Shibasaburo Kitasato



Current Public Health Challenges: We Need a Global Culture of Science



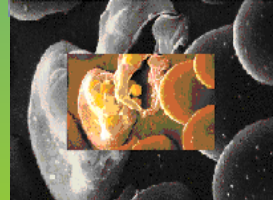
Acute to Chronic Conditions



Aging Population



Health Disparities

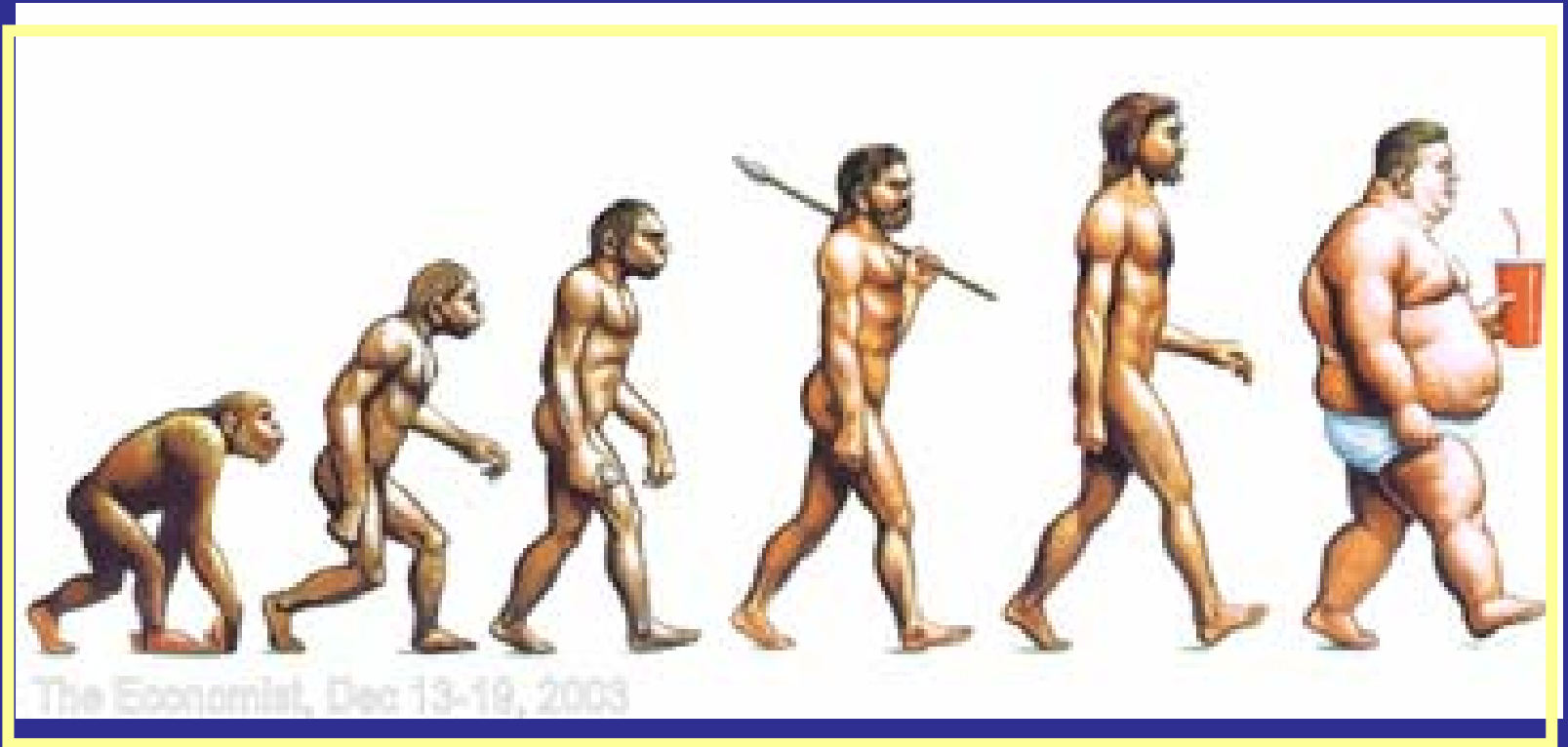


**Emerging and Re-emerging
Infectious Diseases**



**Emerging Non-communicable
Diseases**

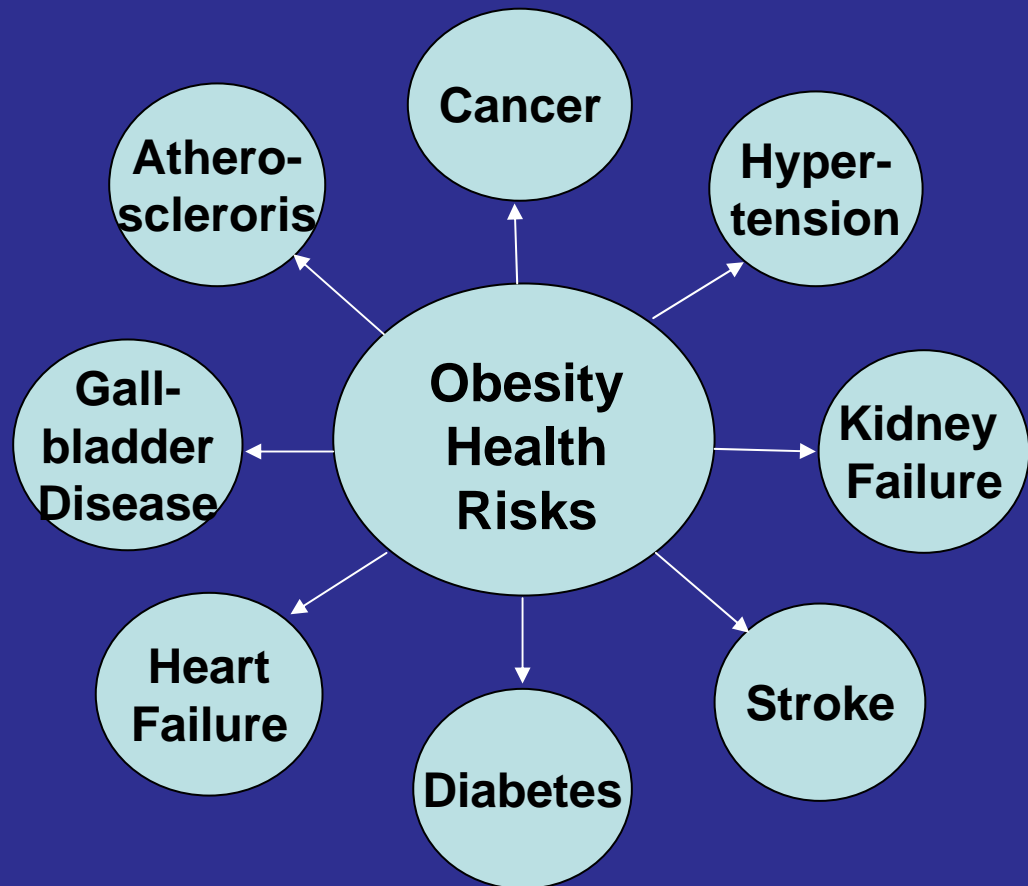
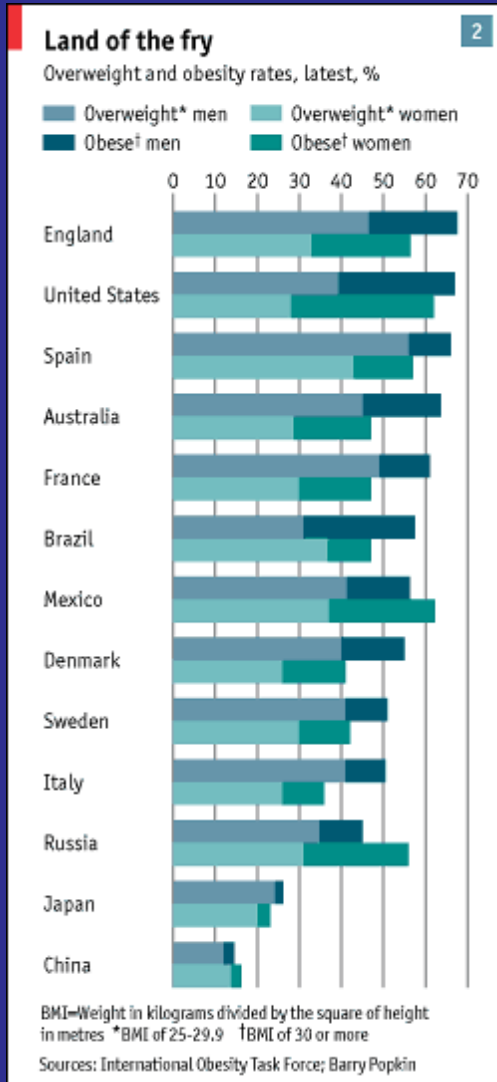
The Shape of Things to Come



The Economist, Dec 13-19, 2003



Obesity: A Worldwide Issue



We Need a Global Culture of Science

AIDS makes orphans.

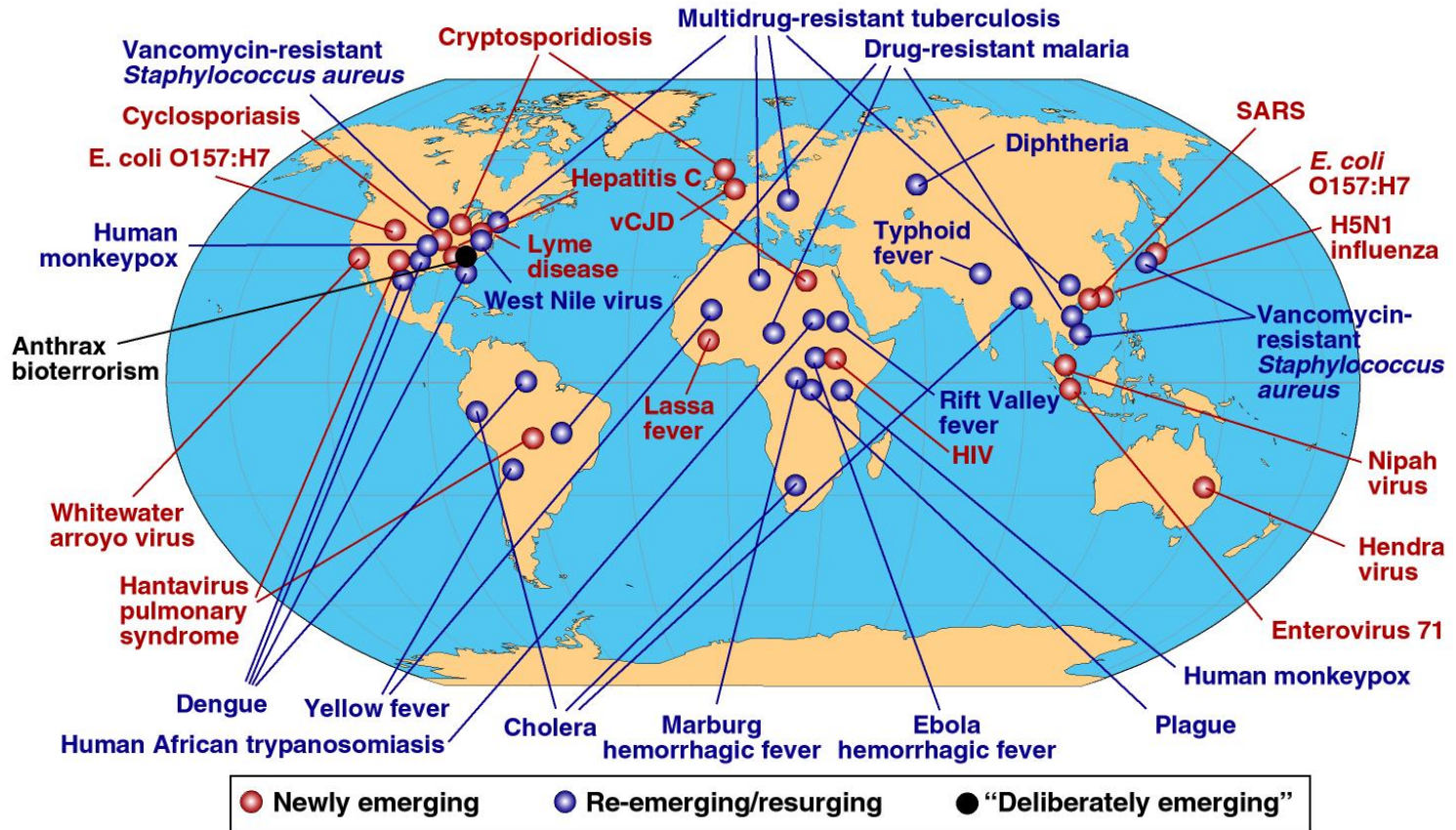


<http://www.ecdgroup.com>

- Emerging and re-emerging diseases create social instability



Global Examples of Emerging and Re-Emerging Infectious Diseases



Toward a “Universal” Influenza Vaccine?

November 16, 2005

Vaccine

**PROTECTION AGAINST
MULTIPLE INFLUENZA A
SUBTYPES BY
VACCINATION WITH
HIGHLY CONSERVED
NUCLEOPROTEIN**

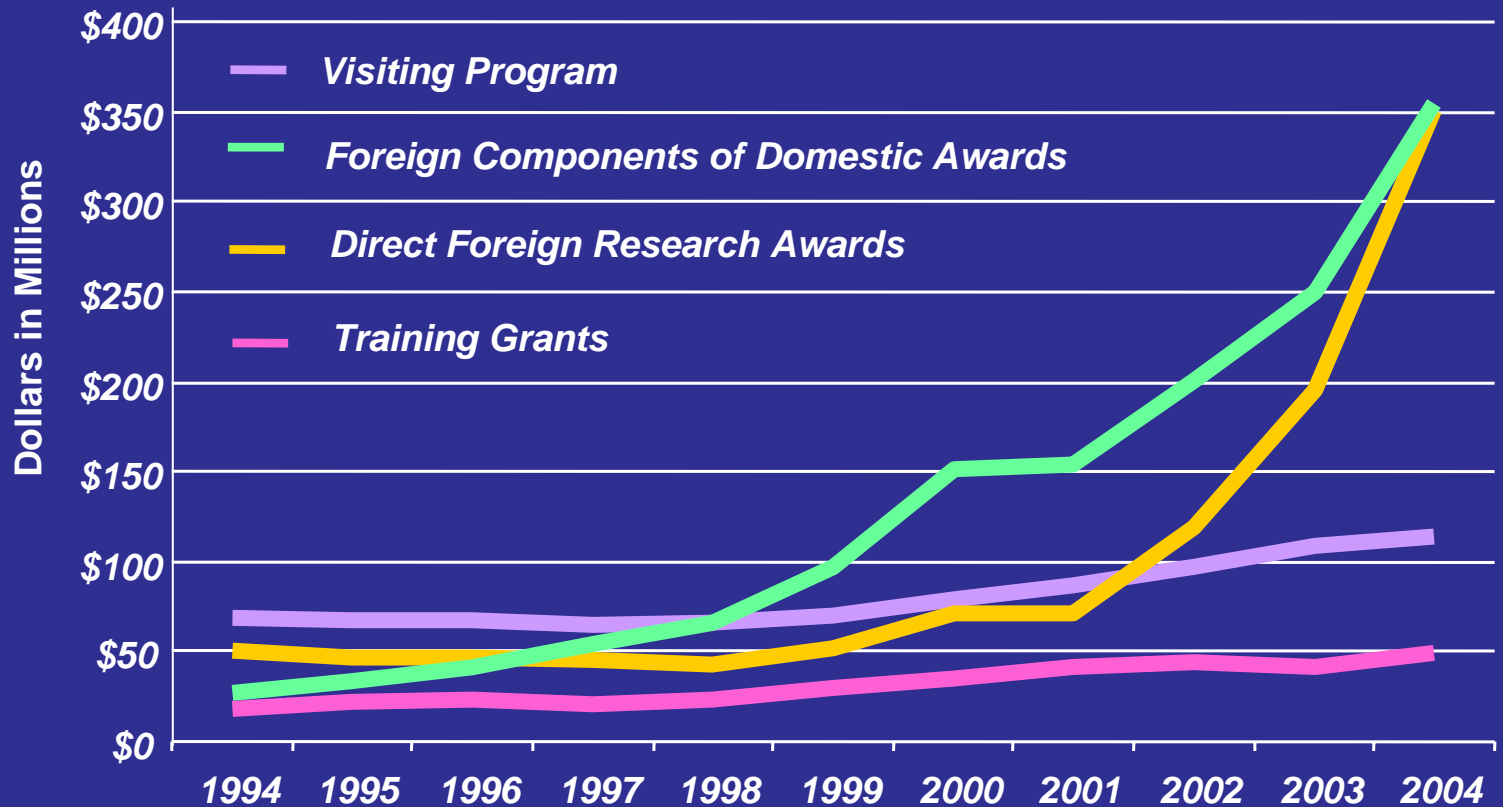
SL Epstein, WP Kong, JA Mispion,
CY Lo, TM Tumpey, L Xu, GJ Nabel

- DNA prime-recombinant adenoviral boost immunization to nucleoprotein (NP).
- Strong antibody and T cell responses were induced.
- Vaccination protected against lethal challenge with highly pathogenic H5N1 virus.



NIH Increased International Research Expenditures

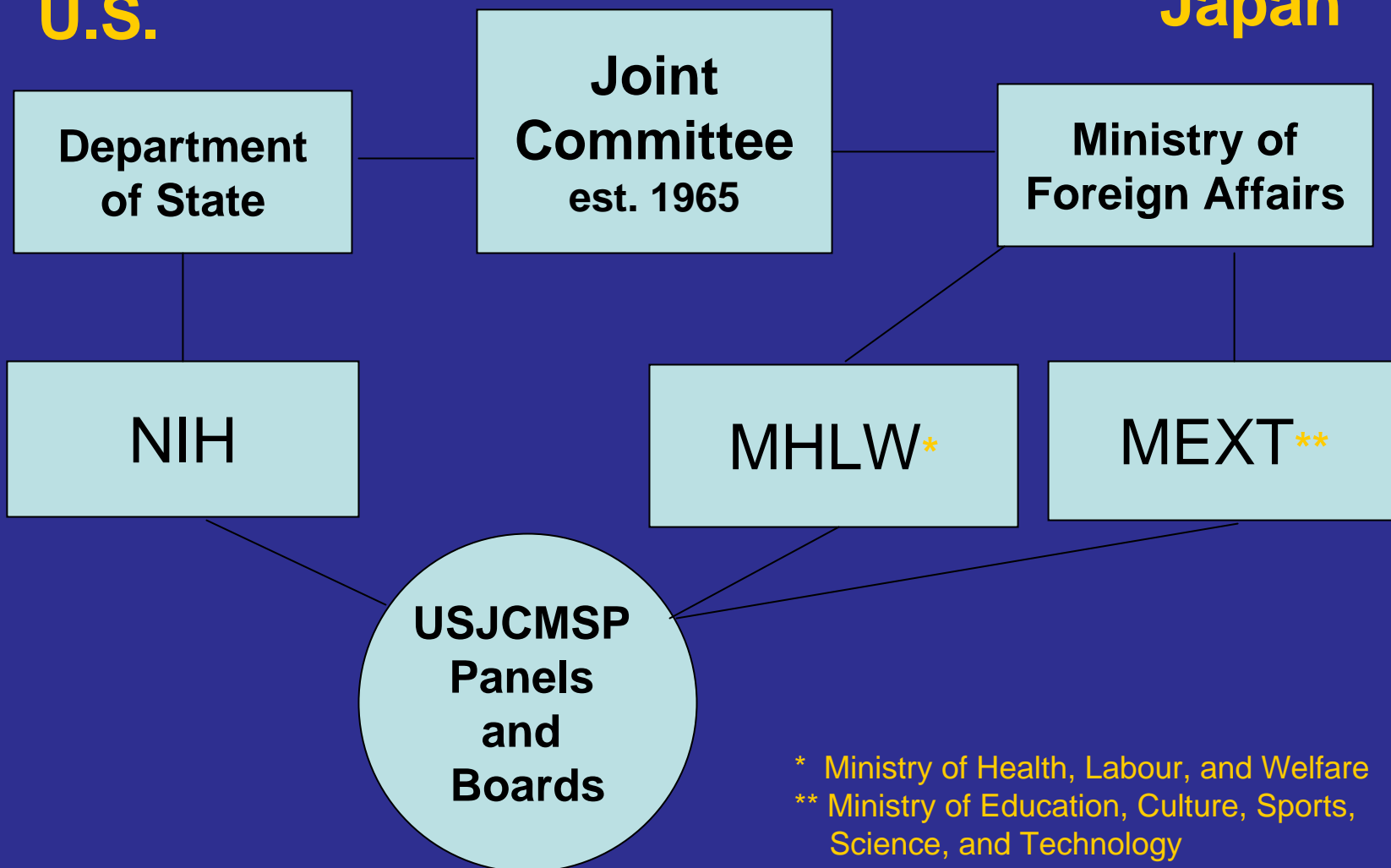
Source: Fogarty International Center, Nov. 2005



U.S.-Japan Cooperative Medical Science Program (USJCMSP)

U.S.

Japan



* Ministry of Health, Labour, and Welfare

** Ministry of Education, Culture, Sports, Science, and Technology



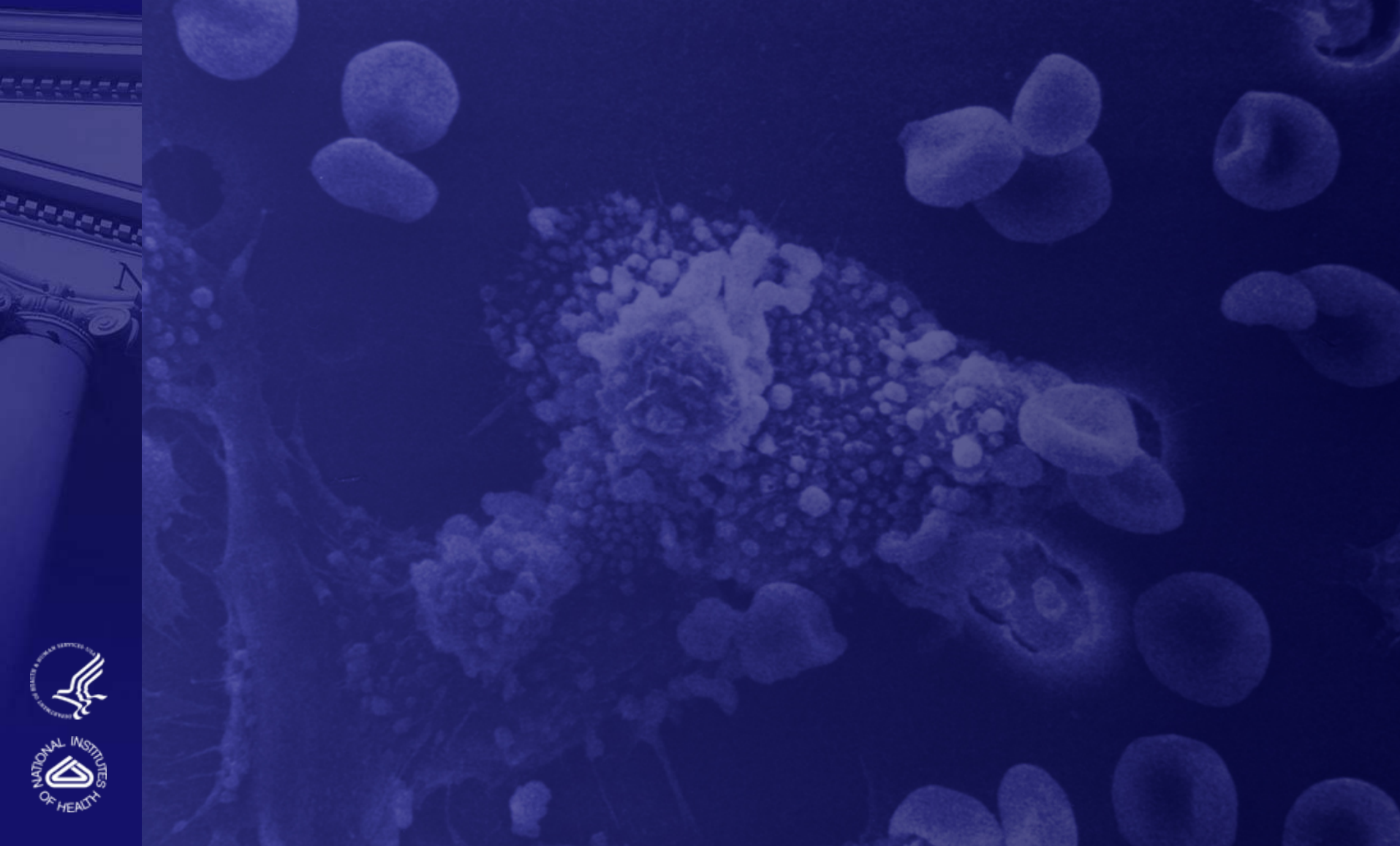
Need to Transform Health and Medicine in the 21st Century

20th Century	21st Century
Treat disease when symptoms appear and normal function is lost	Intervene before symptoms appear and preserve normal function for as long as possible
Did not understand the molecular and cellular events that lead to disease	Understanding preclinical molecular events and ability to detect patients at risk
Expensive in financial and disability costs	Orders of magnitude more effective



The Future Paradigm: The 4 P's

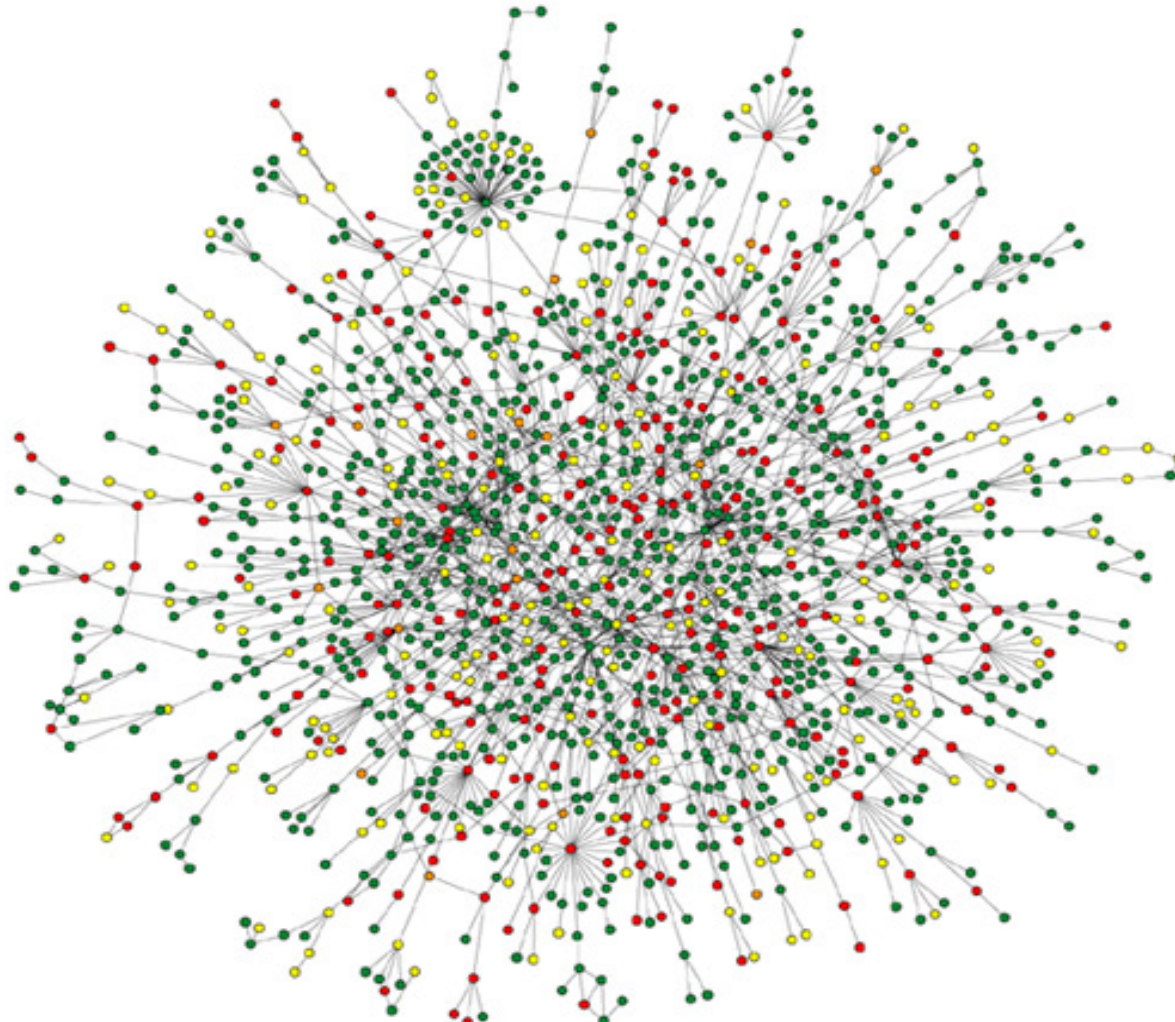
Transform Medicine from Curative to Preemptive



Today, a fundamental scientific barrier is our limited ability to study complex and dynamic biological systems in health or disease!



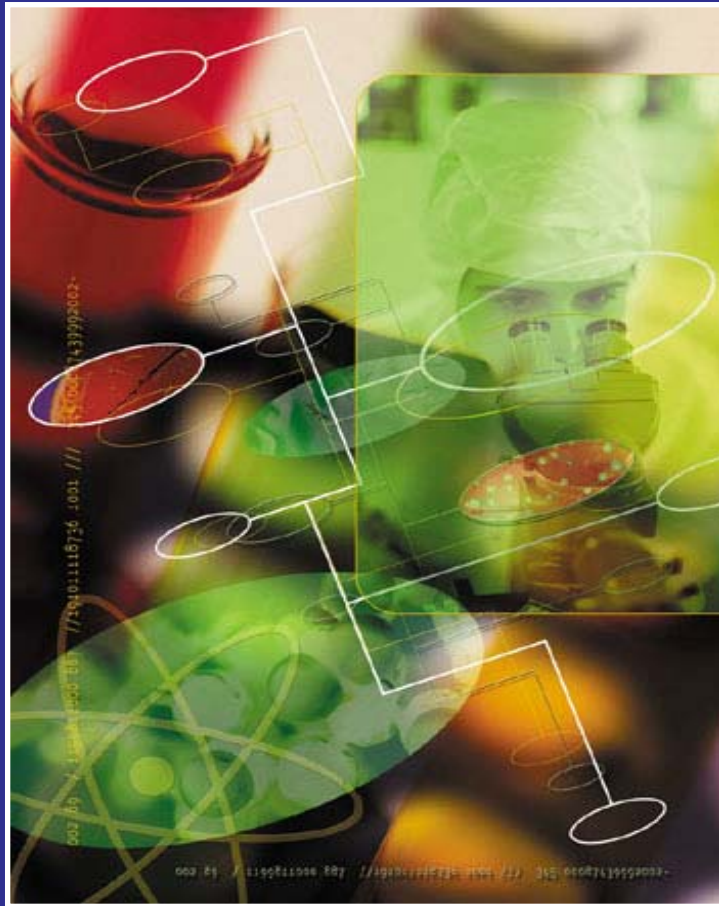
Complexity of Biological Networks



MOLECULAR INTERACTIONS ARE SCALE FREE

Nature Reviews | **Genetics**

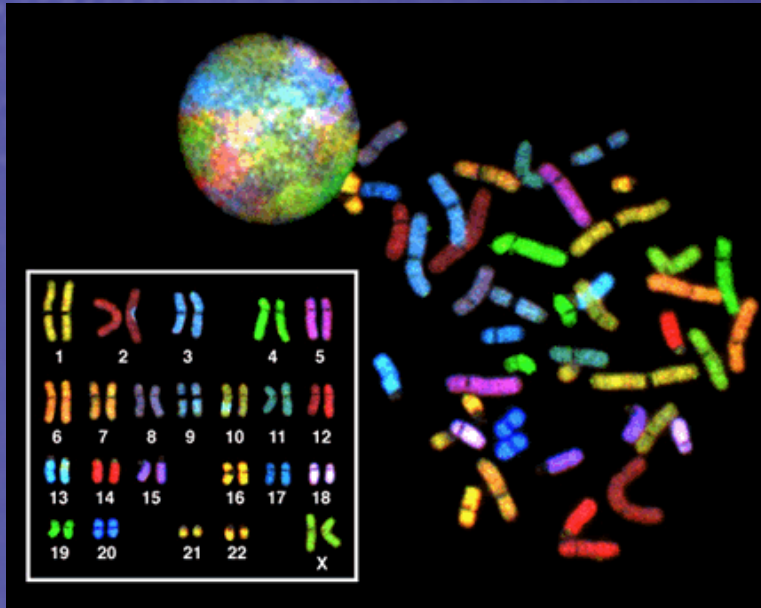
New Pathways to Discovery



- Building blocks, biological pathways, and networks
- Molecular tools libraries
- Structural biology
- Bioinformatics and computational biology
- Systems biology
- Molecular reclassification of diseases



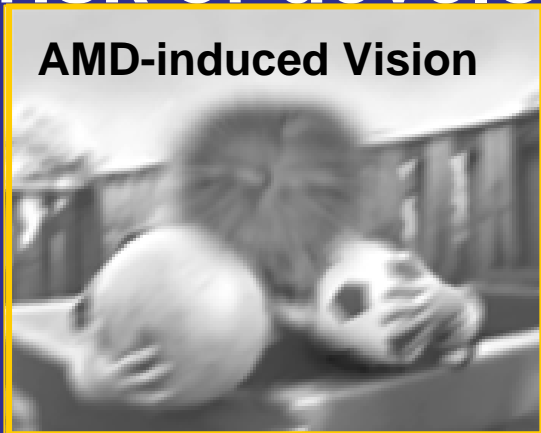
Human Genome Project and HapMap Are Complete: *Where Do We Go From Here?*



- Plummeting cost of DNA sequencing
- New discoveries accelerating revolution of the practice of medicine
- **Genes, Environment, and Health Initiative**
 - Identify roots of 10 most common diseases
 - Devise new ways of monitoring personal environmental exposures

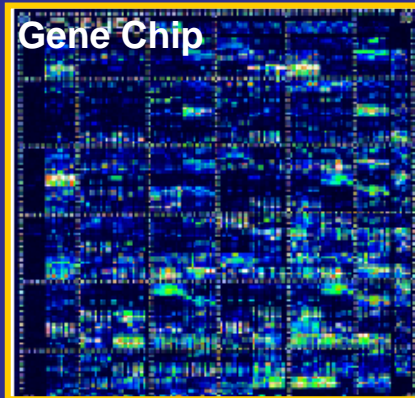
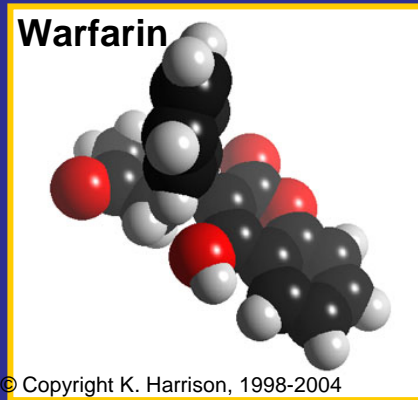


Discoveries in Age-related Macular Degeneration can **PREDICT** who is at risk of developing the diseases



- AMD is the leading cause of blindness in people over age 60
- Variations in genes involved in inflammation can predict the risk of developing AMD
 - 56 % of the unaffected individuals had a variant that conferred protection to AMD
 - 74 % of those with AMD had no protective variants
 - **THOSE WITH THE “WRONG” GENES HAVE 100 FOLD GREATER RISK**

Pharmacogenomics Discoveries Make it Possible to “Personalize” Treatment



- Warfarin: An anticoagulant drug used to reduce the risk of clots causing strokes or heart attacks
- Effective daily dose ranges from 0.5 mg to 60 mg
- Too little: clots, stroke
- Too much: bleeding/death
- Genomic experiments revealed mutations that help predict best dose for individual patients

Cancer Treatment Gets Personal: Potential New Model of Cancer Treatment



“Advances in understanding genetic basis of cancer have led to promising new therapies, which have fueled discussions about a future model of cancer care-- treatment decisions are guided by the molecular attributes of the individual patient.”

CANCER GENOME PROJECT

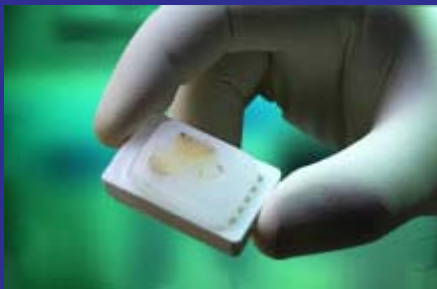
<http://www.sciencemag.org/sciext/cancer/>



New Discoveries Make it Possible to “Personalize” Cancer Treatment



*Identified 16
informative
genes*



*Test tumor
samples for
mutations in
these genes*

Impact:

- 100,000 women *each year* can make a more informed choice
- 70,000 women may not have to undergo chemotherapy
- Reduces routine cost of treating these patients

**Recurrence Score helps
predict which patients
need chemotherapy**



Preemptive: HPV Vaccine



© Rex Features

- Human Papillomavirus (HPV) infects over 80% of 15-50 year old women and can cause cervical cancer
- Prevent sexually transmitted HPV infection = prevent cervical cancer
- Anti-Viral Vaccines are among the most cost effective public health interventions (e.g., smallpox, polio, & measles)
- NIH has two vaccines currently in clinical trials

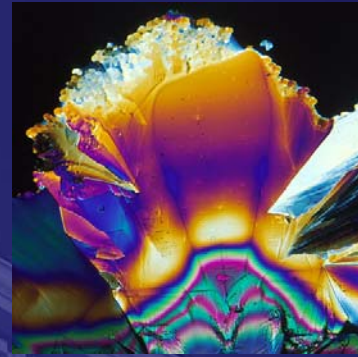
Participatory: Community Involvement



Jackson Heart Study

- Community participation
- Community education
 - Health awareness
 - Student outreach
 - Encourage involvement
- Identify minority risk factors for cardiovascular disease





NIH *Transforming medicine and health through discovery*

