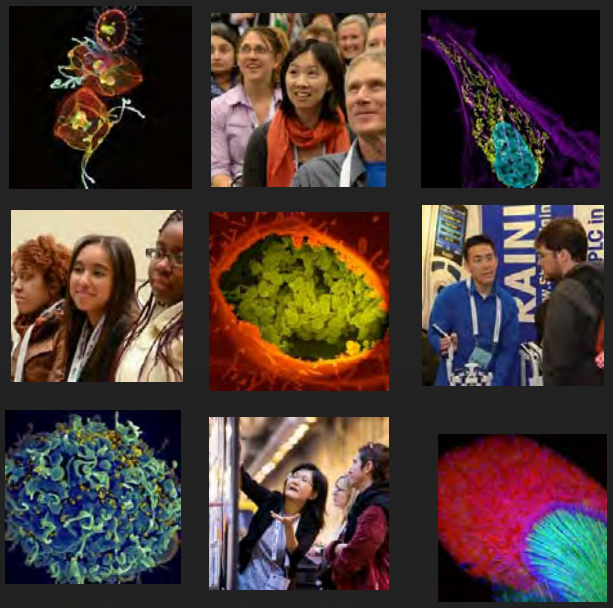
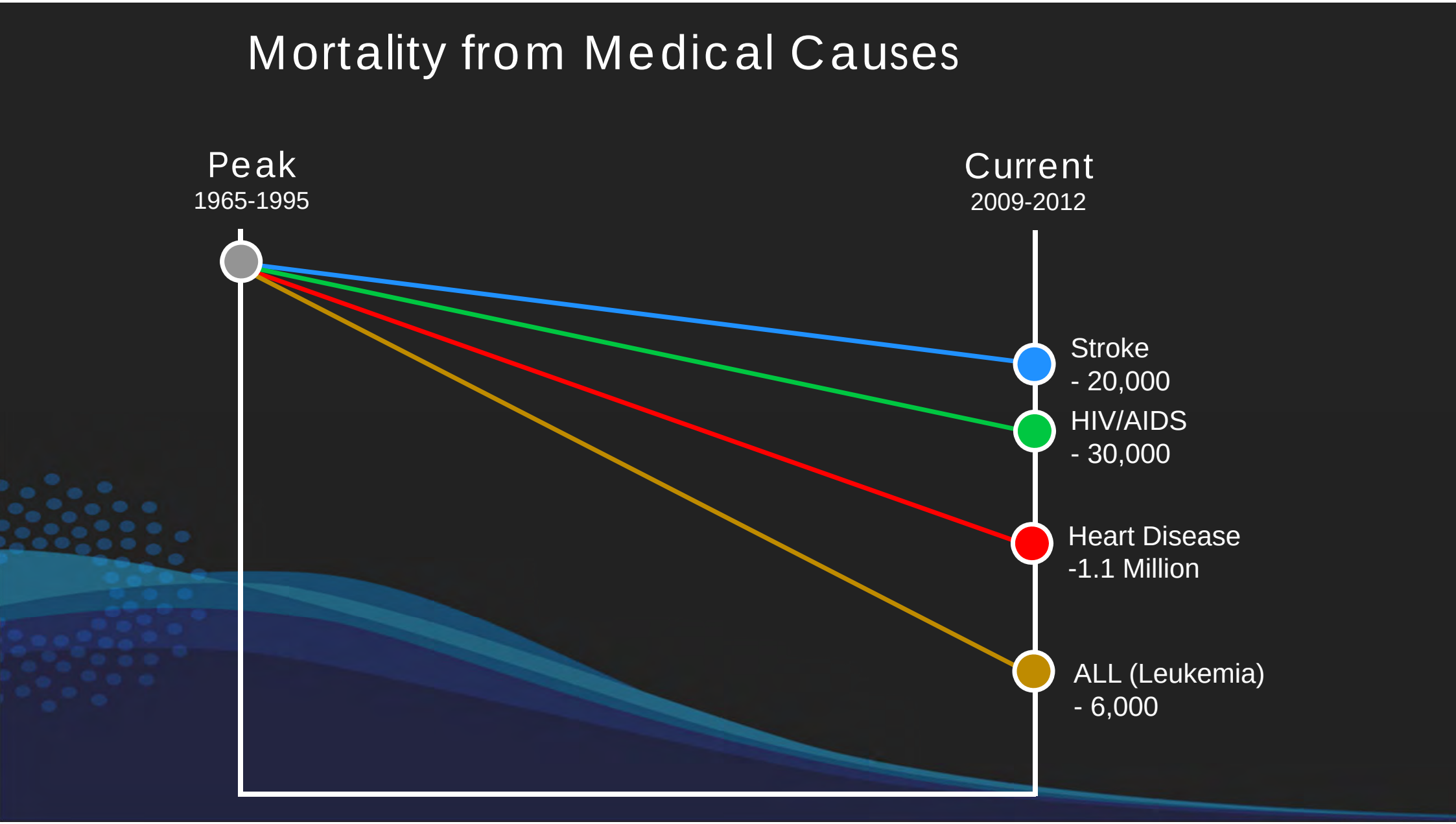


ascb
an international forum for cell biology

Il futuro della ricerca biomedica. Sfide ed opportunita'

Cives
Piacenza, October 30, 2015

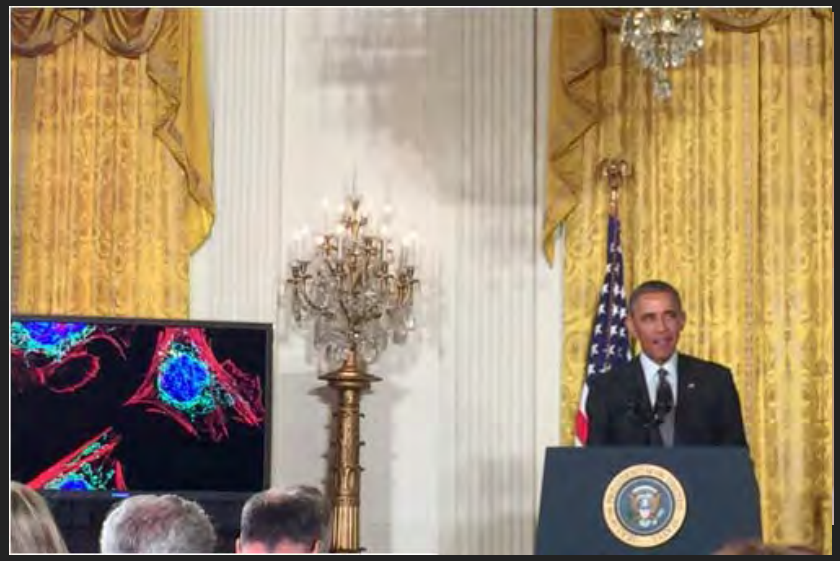
Stefano Bertuzzi, Ph.D., M.P.H.
Executive Director
American Society for Cell Biology


Mortality from Medical Causes
Peak
1965-1995
Current
2009-2012
Stroke
- 20,000
HIV/AIDS
- 30,000
Heart Disease
-1.1 Million
ALL (Leukemia)
- 6,000

A BIG Project:
Precision Medicine

A Rapidly Evolving Landscape

Industry	Today	Tomorrow
Products	Few	Many
Focus	Phenotypes	Genotypes
Sales	Blockbusters	Many Smaller Sales
Patient Target	Diseased	Prevention/Early Diagnosed
Partnerships	Acquisitions of Biotech	Wide Networks



Friday, January 30, at the White House, the President Announces the Precision Medicine Initiative

Precision medicine announcement in the press

Precision Medicine—What is it?

- An emerging approach for disease prevention and treatment that takes into account people's individual variations in genes, environment, and lifestyle

- It will generate the scientific evidence needed to move the concept of precision medicine into clinical practice.

Targeting Lung Cancer Treatments In Patient Subsets To Improve Outcomes

Crizotinib: A potent and selective oral inhibitor of MET and ALK

... initially being developed for MET mechanism

Academic discovery of new patient segment redefined lung cancer

10-15% of non small cell lung cancer (NSCLC) patients with fusion oncogene ELM4-ALK are unresponsive to conventional EGFR inhibitor treatment

New Phase I trial targeting advanced NSCLC patients harboring ALK rearrangement

Approved by FDA on August 26, 2011 for non-small cell lung cancers that express the abnormal anaplastic lymphoma kinase (ALK) gene

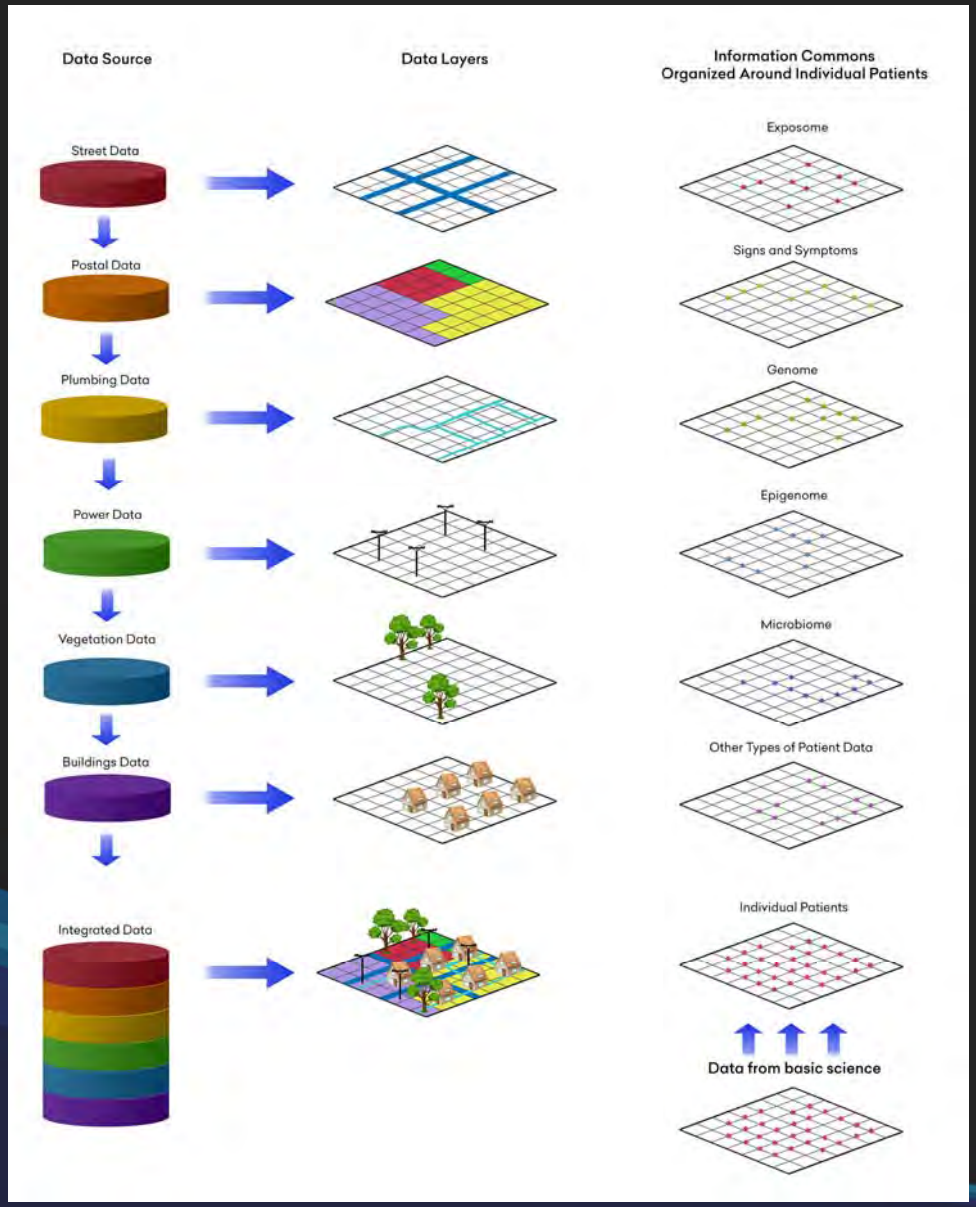
Highly effective therapy

Overall response rate = 65%

Disease control rate = 84% at a median of ~24 weeks

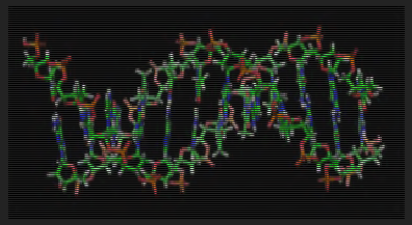
Accelerated clinical activities

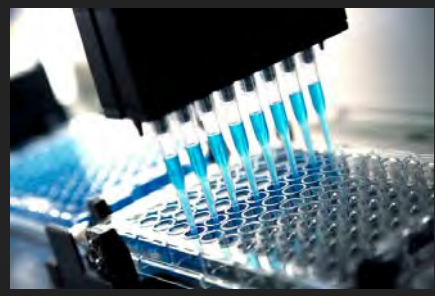
Phase 3 trial based on Phase 1 results, bypassing Phase 2 and accelerating development timeline


Data Source
Data Layers
Information Commons
Organized Around Individual Patients
Street Data
Postal Data
Plumbing Data
Power Data
Vegetation Data
Buildings Data
Integrated Data
Exposome
Signs and Symptoms
Genome
Epigenome
Microbiome
Other Types of Patient Data
Individual Patients
Data from basic science

Why now? Why not before? Why not tomorrow?

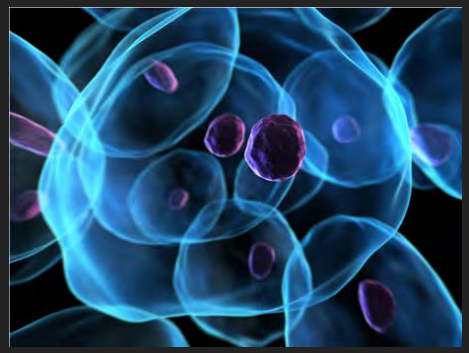
- Sequenced human genome
- New technology in biomedical research
- High computational power and analysis
- Electronic Medical records

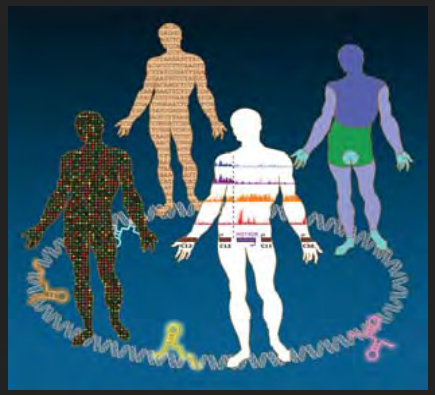




Goals

- Focus on cancer, at least initially
- Create a research cohort of > 1 million American volunteers who will share genetic data, biological samples, and diet/lifestyle information, all linked to their electronic health records if they choose.
- Pioneer a new model for doing science that emphasizes engaged participants, responsible data sharing, and privacy protection.





Goals (continued)

- Advance pharmacogenomics, the right drug for the right patient at the right dose
- Identify new targets for treatment and prevention
- Test whether mobile devices can encourage healthy behaviors
- Lay scientific foundation for precision medicine for many diseases

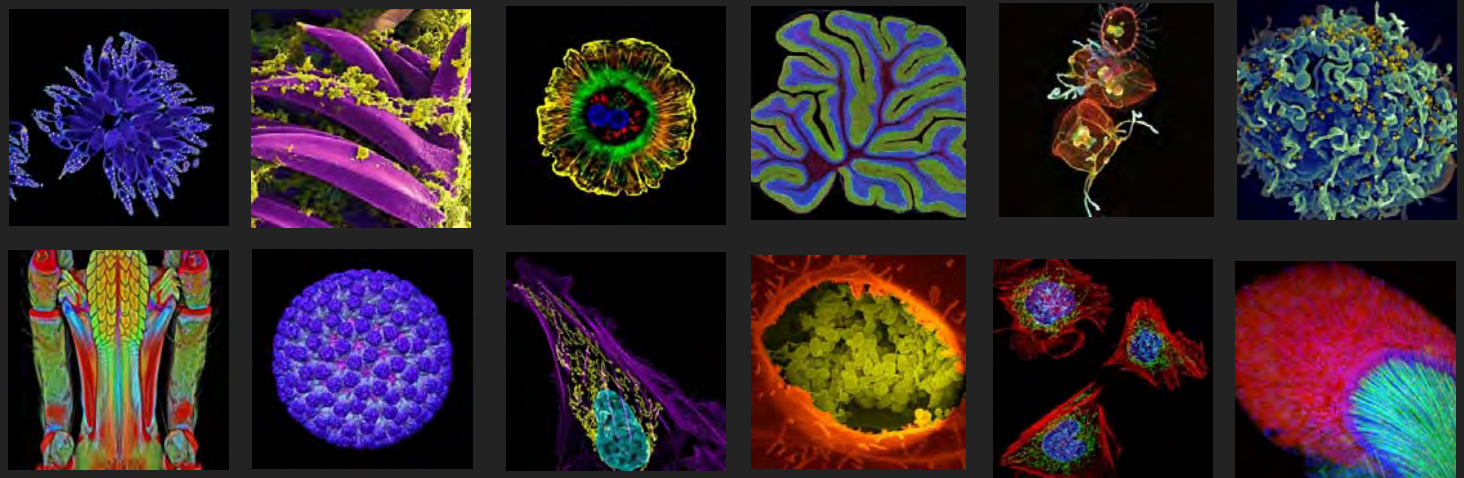

HMP
HUMAN
MICROBIOME
PROJECT

- International effort
- Take advantage of large scale, high through multi ‘omics analyses
- Study the microbiome in human health
- Community resource program
- Generation of rich, comprehensive, and publicly available datasets of the microbiome
- Integrated microbiome

Bottom line

- Integration
- Multi-discipline
- Specialization is not enough anymore
- Hyper-technological
- High-cost
- Cost-saving? Perhaps. But more likely cost-effective
- New job skills
- Fewer jobs? Certainly different jobs

Thank you!



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