Realizing the Dreams of Personalized Medicine

Data-Driven Medicine in the Age of Genomics

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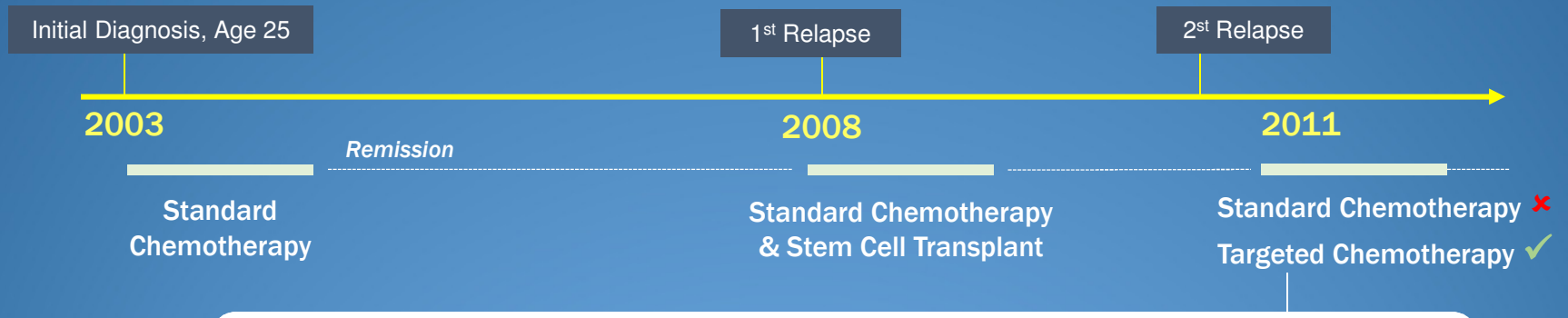
The Promise of Personalized Medicine

"In Treatment for Leukemia, Glimpses of the Future" …

A Patient's Clinical History: Acute Lymphoblastic Leukemia (ALL)



Washington University in St. Louis — The Genome Institute

- Researchers compare whole genomes sequences and RNA of patient's normal & tumor cells
- Gene FLT3 is found to be significantly hyperactive in tumor cells
- FLT3 is targeted with *Sutent*, a drug already approved by FDA for kidney cancer

The New York Times

"I can't overstate the importance of those discoveries that really were driven out of the research lab, but made their way, just in a matter of weeks, from the research lab to helping me as a patient…. With new technology we have today… we're now able to decipher the very small changes that are present in my tumor genome which may look acutely different than someone else's….. Changes in my tumor genome that were unique led to changes in my treatment."

Lukas Wartman, MD , Medical Oncologist
Washington University of St. Louis, July 2012

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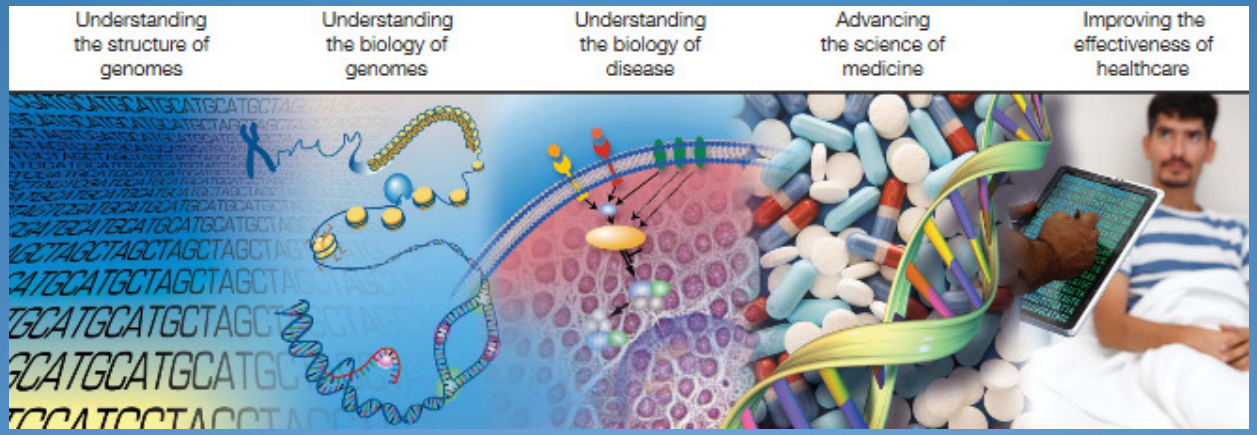
The Path Toward Personalized Medicine

Completion of the Human Genome Project in 2003 and a significant decline in the cost of whole genome sequencing jump-started the rapid expansion of research on genomics in disease diagnosis, treatment, and prevention



Green, ED et al (2011). Charting a course for genomic medicine from base pairs to bedside. *Nature* 470: 204-213

Change in personalized medicine investment from 2005 to 2010 [1]

↑ 75%

Biopharmaceutical companies investing in personalized medicine research in 2010 [1]

94 %

Prominent personalized medicine treatments & diagnostics available [2]

13 in 2006 ▶ **113** in 2014

[1] Tufts Center for the Study of Drug Development, 2010; [2] Personalized Medicine Coalition, 2014

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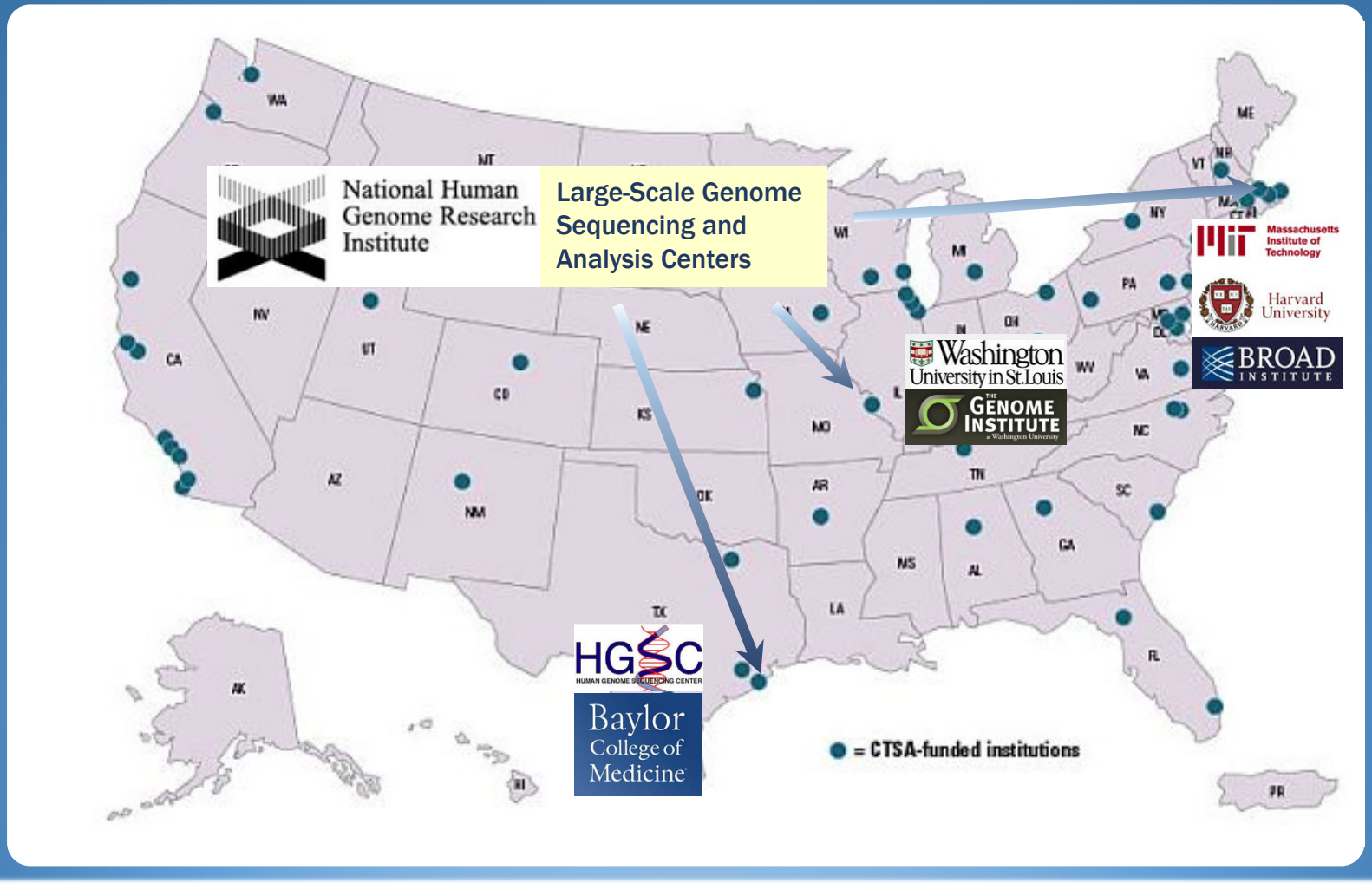
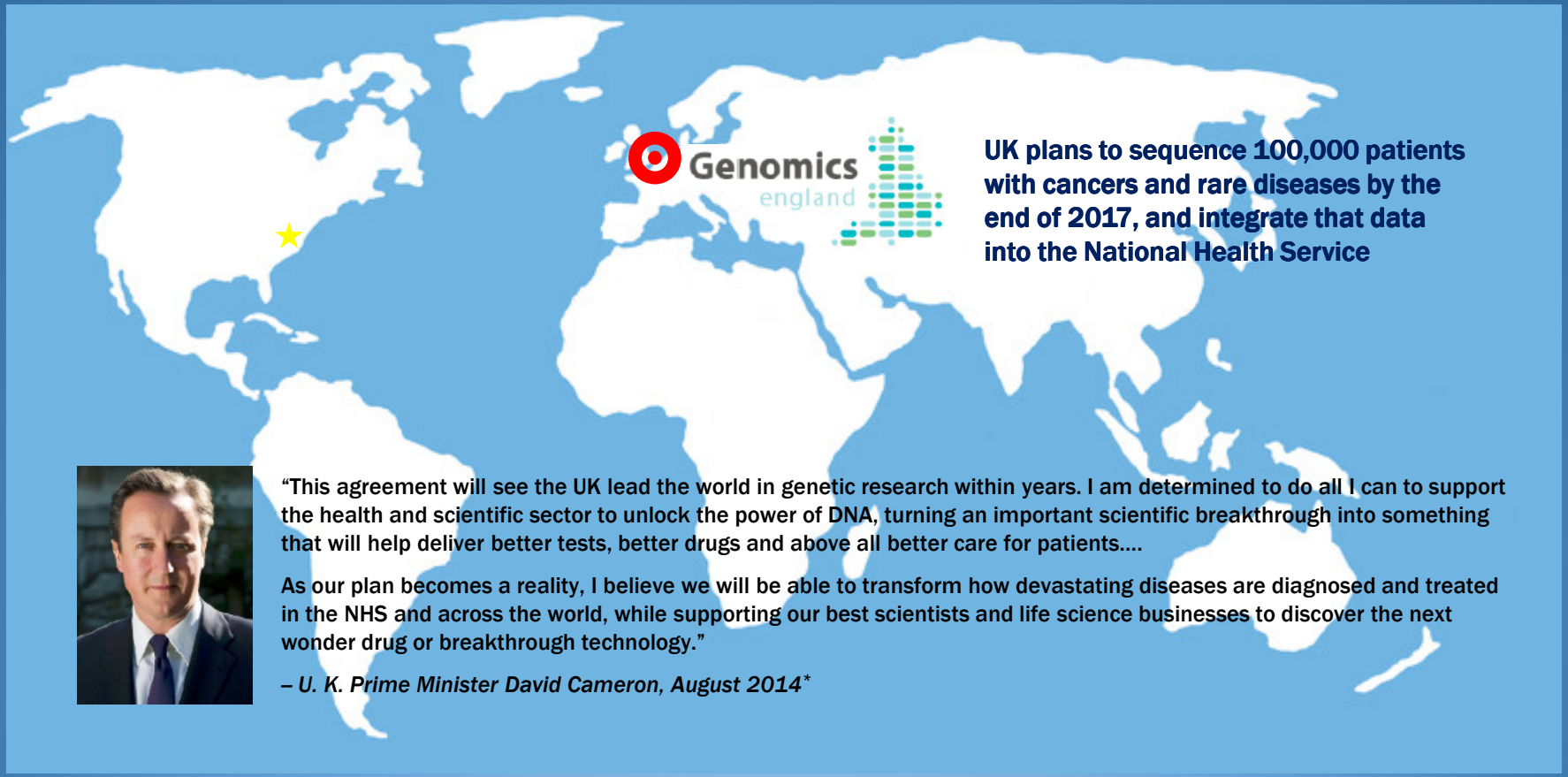
Personalized Medicine Research in the U. S.

Federal support such as the NIH-sponsored Clinical & Translational Science Award and NHGRI Genome Sequencing Programs makes progress in Personalized Medicine possible



An International Healthcare Priority: United Kingdom

The UK invests £300M in the 100,000 Genome Project – a nationwide push to encourage regional life science investments and make the UK the worldwide leader in Personalized Medicine



UK plans to sequence 100,000 patients with cancers and rare diseases by the end of 2017, and integrate that data into the National Health Service

"This agreement will see the UK lead the world in genetic research within years. I am determined to do all I can to support the health and scientific sector to unlock the power of DNA, turning an important scientific breakthrough into something that will help deliver better tests, better drugs and above all better care for patients....

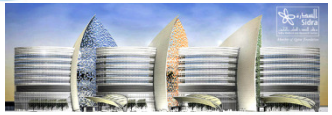
As our plan becomes a reality, I believe we will be able to transform how devastating diseases are diagnosed and treated in the NHS and across the world, while supporting our best scientists and life science businesses to discover the next wonder drug or breakthrough technology."

*– U. K. Prime Minister David Cameron, August 2014**

* U. K. Government Press Release, August 1, 2014, https://www.gov.uk/government/news/human-genome-uk-to-become-world-number-1-in-dna-testing

An International Healthcare Priority: Qatar

Qatar Foundation launches the Qatar Genome Project as the nation's leadership makes the development of an international Center of Excellence in Personalized Medicine a national priority



مؤسسة قطر
Qatar Foundation

Qatar plans to sequence the genomes of large populations of Qatari nationals in order to develop genome-based diagnostics and therapies for illnesses common to communities in the Gulf Region

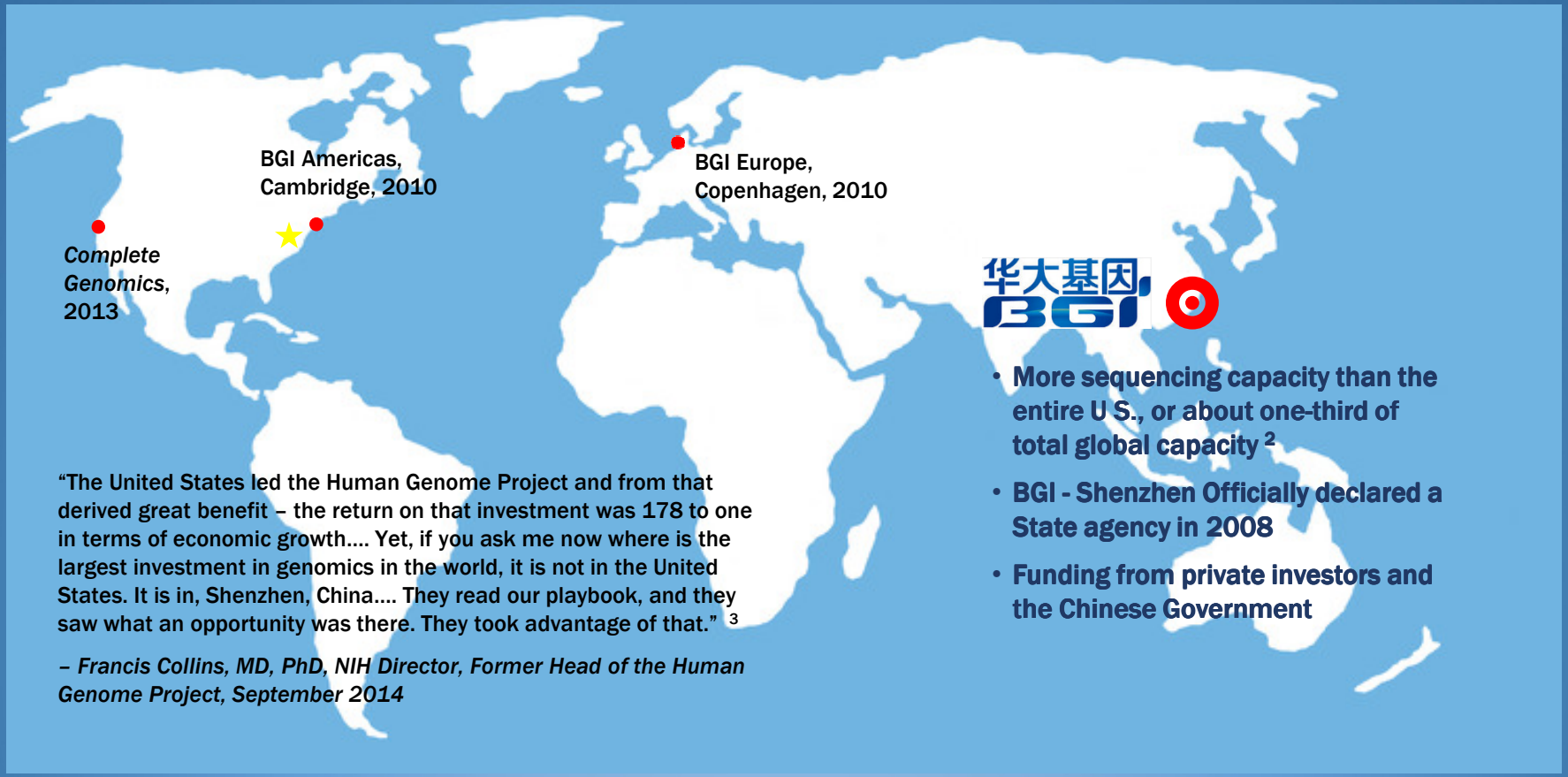
"We're creating an environment conducive to research and education, and linking research to clinical practice. I see personalised medicines as a revolutionary way to diagnose and treat diseases in the future. And this is the vision of Qatar."*

– Her Highness Sheika Mosa bint Nasser, Chairperson of Qatar Foundation & Founder of the World Innovation Summit for Health, December 2013

* *Qatar Tribune, December 11, 2013*

An International Healthcare Priority: China

Beijing Genomics Institute leads the world in genomics investments, capturing at least 25% of market share [1] as the only genome services organization with a global footprint



"The United States led the Human Genome Project and from that derived great benefit – the return on that investment was 178 to one in terms of economic growth.... Yet, if you ask me now where is the largest investment in genomics in the world, it is not in the United States. It is in, Shenzhen, China.... They read our playbook, and they saw what an opportunity was there. They took advantage of that." [3]

– Francis Collins, MD, PhD, NIH Director, Former Head of the Human Genome Project, September 2014

- More sequencing capacity than the entire U S., or about one-third of total global capacity [2]
- BGI - Shenzhen Officially declared a State agency in 2008
- Funding from private investors and the Chinese Government

[1] ISI Group, *Forbes*, August 28, 2013
[2] National Institutes of Health, http://www.nih.gov/about/impact/impact_global.pdf
[3] Testimony before U. S. Congress, September 10, 2014; http://www.rsc.org/chemistryworld/2014/09/us-genomics-lead-being-lost-china

A Complex Technical Challenge

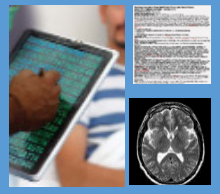
Understanding linkages between genomic variation, disease treatments, and clinical outcomes requires innovative technical solutions to speed discovery

1 'Big' Data

Data from 1000s of Patients ...

'Omics

Clinical Records



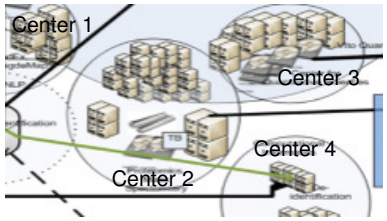
Security for PHI + *"HIPAA – compliant"*

... and thousands of scientific documents

2 Data Silos

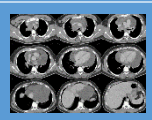
Across geographies ...

... and within organizations



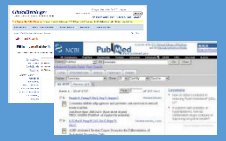
3 Unstructured Data

From clinical notes ...



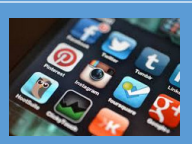
... to medical images ...

... to peer-reviewed journals



... to websites

... to social media



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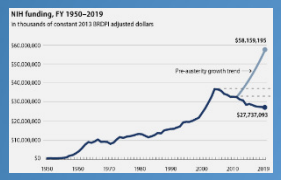
A Challenging Business Environment

Technical innovation and regulatory policies must enable and encourage progress amid growing life science R&D costs and greater revenue threats

Extended R & D Timelines

- Shrinking pool of drug targets requires shift to complex therapeutic areas
- Increasingly complex eligibility requirements extend clinical trials
- More stringent regulatory requirements delay trials approval process

Revenue Uncertainty



- Any increases in public & private life science R&D funding not commensurate with the cost of conducting scientific research
- Shifting models related to medication access and reimbursement

Greater Competition

- Shorter timelines for data exclusivity and IP protection reduce barriers to entry for competitors with similar products

Tackling the Challenge of Unstructured Information

IBM Watson extracts scientific and clinical information from large volumes of unstructured text and transforms it into a structured format for analysis

1 Omics Data

Sample: Annovar

```
exonic NOD2 16 … a frameshift … SNP
… exonic GJB2 13 … associated with
hearing loss … exonic CRYL1,GJB6 13 …
a 342kb deletion encompassing GJB6,
associated with hearing loss …
```

2 Clinical Data

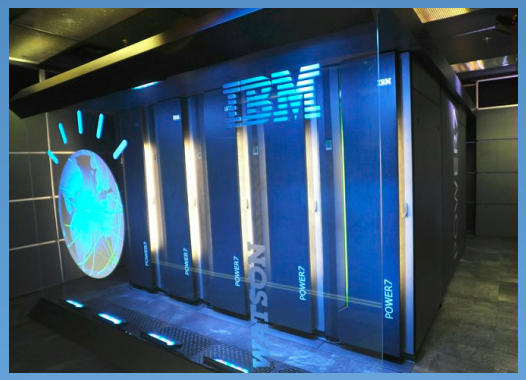
Sample: Patient History

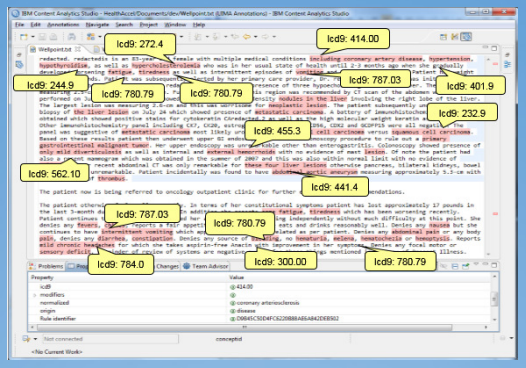
```
…was in good health until 2-3 months
ago when she gradually developed
fatigue and intermittent epigastric
pain, … most recent colonoscopy was
within normal limits…
```

3 Knowledge Base

IBM WATSON

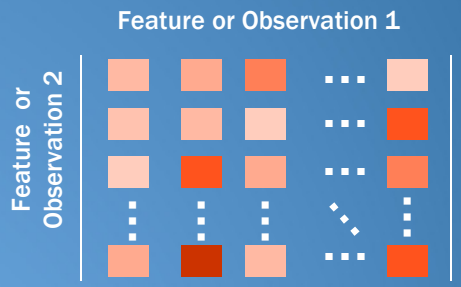
Natural Language Processing





Structured Format

Concept Associations

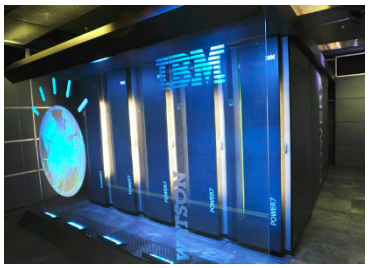


IBM Watson as a Tool for Clinical Decision Support

IBM Watson helps oncologists at a leading Cancer Center mine millions of pages of peer reviewed literature for possible individualized treatments

Memorial Sloan-Kettering Cancer Center

IBM Watson helps fight cancer with evidence-based diagnosis and treatment suggestions



Medical information doubles in volume every five years, and physicians practicing in the rapidly changing field of oncology are challenged to remain current with medical literature, research, guidelines and best practices. Research centers such as MSKCC publish innovative findings in peer-reviewed journals, which are the most common medium doctors use to gather new medical information. Nevertheless, keeping up with the medical literature can take as many as 160 hours a week. It's not surprising that only about 20 percent of the knowledge that clinicians use today is evidence-based.

MSKCC began looking for a way to expand the accessibility and usability of medical evidence to improve patient outcomes across the field of oncology. It wanted to find a technology solution that could provide personalized diagnosis and treatment suggestions for individual patients.

"By sharing our experience and knowledge, coupled with the power of **Watson**, we can help physicians around the world understand and mine the subtleties of each person's illness and make evidence-based treatment decisions."

-Mark G. Kris, Attending, Memorial Sloan Kettering Thoracic Oncology Service and Lead Physician for IBM Watson Oncology

http://www.mskcc.org/cancer-care/watson-oncology

Keys to Accelerating Scientific Breakthrough

Leading biomedical research organizations investigating personalized medicine are asking for solutions that will give them a competitive advantage in therapeutic discovery

Policies must support:

- ✓ **Standardization of clinical and life science data** across geographic areas
- ✓ **Development of technology solutions** capable of processing the rapidly growing quantities of genomic and other types of complex life science data
- ✓ **Scientific collaboration** and **data sharing** across organizations and geographic boundaries in a **secure environment** appropriate for Protected Health Information
- ✓ **Intellectual Property protection** and **financial incentives** that will enable pharmaceutical R&D organizations to continue innovation

Thank You

Any Questions?
Please Contact:

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