Federal Trade Commission Microeconomics Conference

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What is Personalized Medicine?

"Getting the right treatment, to the right patient, at the right dose, at the right time."

- When used in the context of R&D, it primarily refers to diagnostics and targeted therapeutics
- When used in a broader context, it expands to include technologies and tools influencing decisions by both clinicians and patients

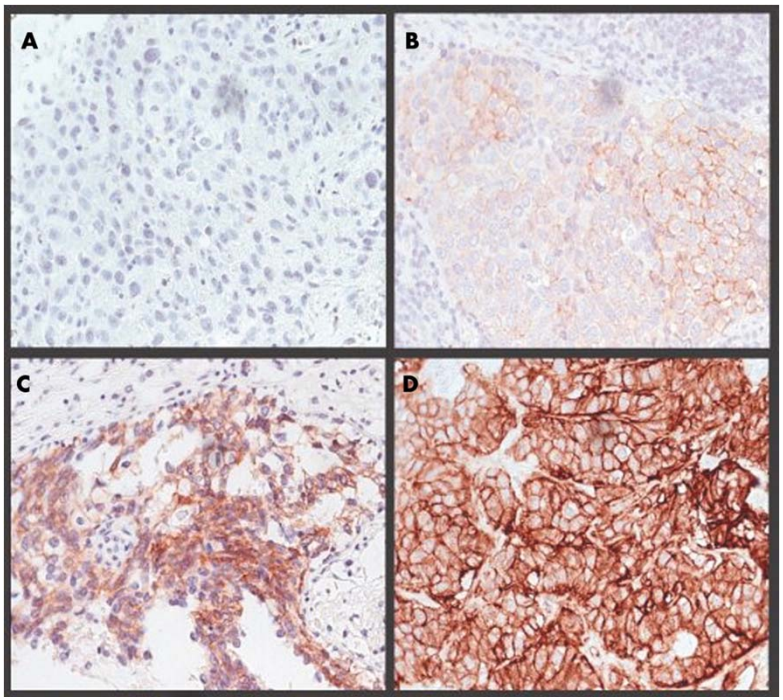
Targeted Treatments: Herceptin

- Her2/neu is over-expressed in 25-30% of breast cancer cases

- Herceptest® is an protein-based test designed to identify subsets of patients with tumors that over-express the Her2/Neu receptor

- Herceptest ®/Herceptin ® approaches have demonstrated a success story for target/therapy combinations

Her2/neu IHC Scoring (0-3)



Images from Ellis et al. (2005) J Clin Path. **58**. 710-4

The Overlap of Personalizing Medicine and Care

Treatment Summary

- Removal of the testicle
- Surgery involving the brain
- **One round of BEP** (Bleomycin, Etoposide and Platinol)
- Three rounds of VIP chemotherapy (Ifosfamide, Etoposide and Platinol.)

Risks Associated with Tx

- 2X Risk of a second cancer
- Bladder or urinary tract toxicities
- Risk of cardiac problems
- Fertility and sexuality concerns
- Ototoxicity (hearing loss, tinnitis)
- Peripheral neuropathy
- **Lung (pulmonary) Complications**

Personalized Medicine and Patient Decision-Making

"**For Gen Y woman with cancer risk, 'it's just a boob'** "

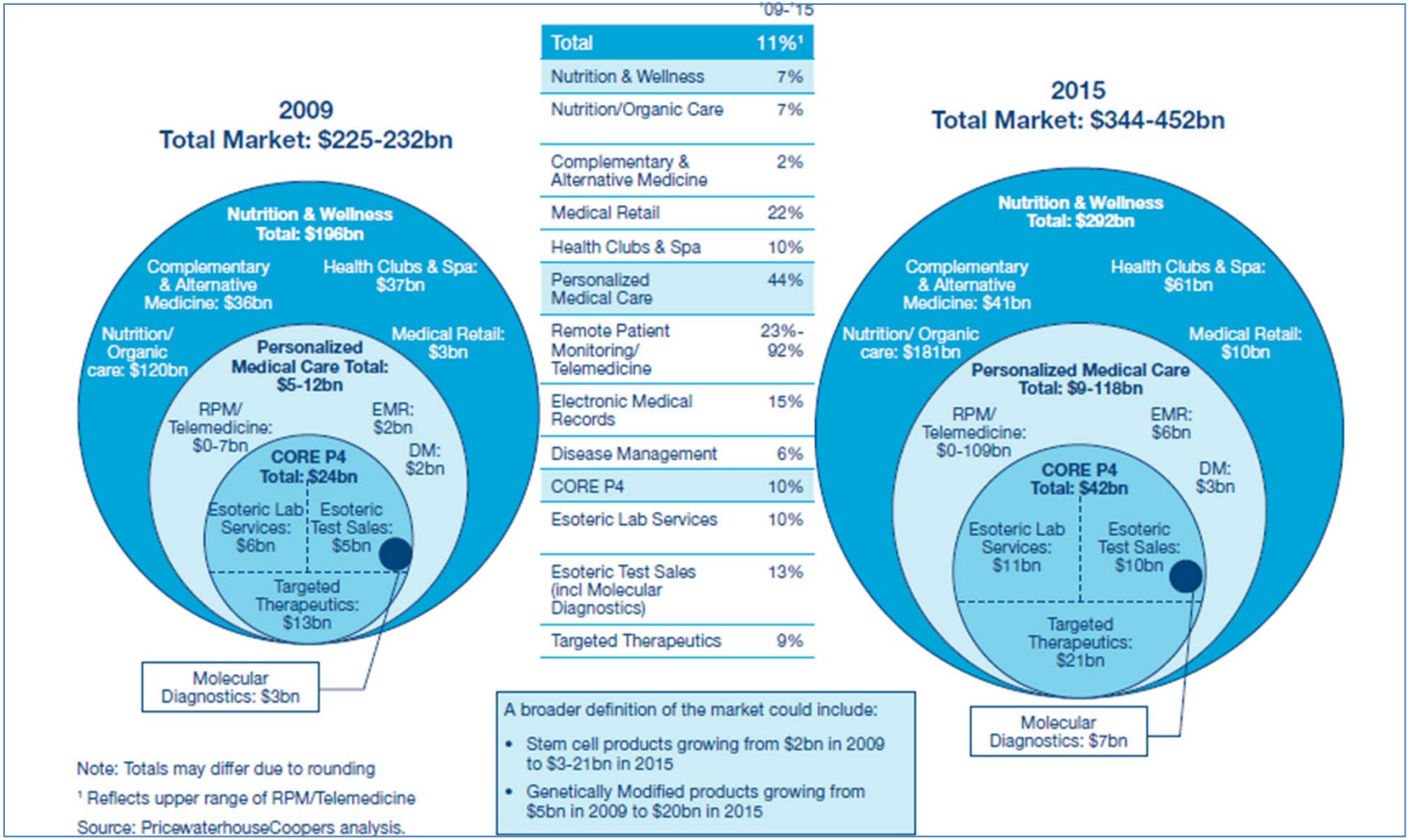
(CNN.com article July 17, 2009)

BILATERAL PROPHYLACTIC MASTECTOMY

- 90% risk reduction in occurrence in high-risk women and BRCA carriers
- Pain, discomfort, and loss of sexual enjoyment commonly reported
- Feelings of regret are uncommon

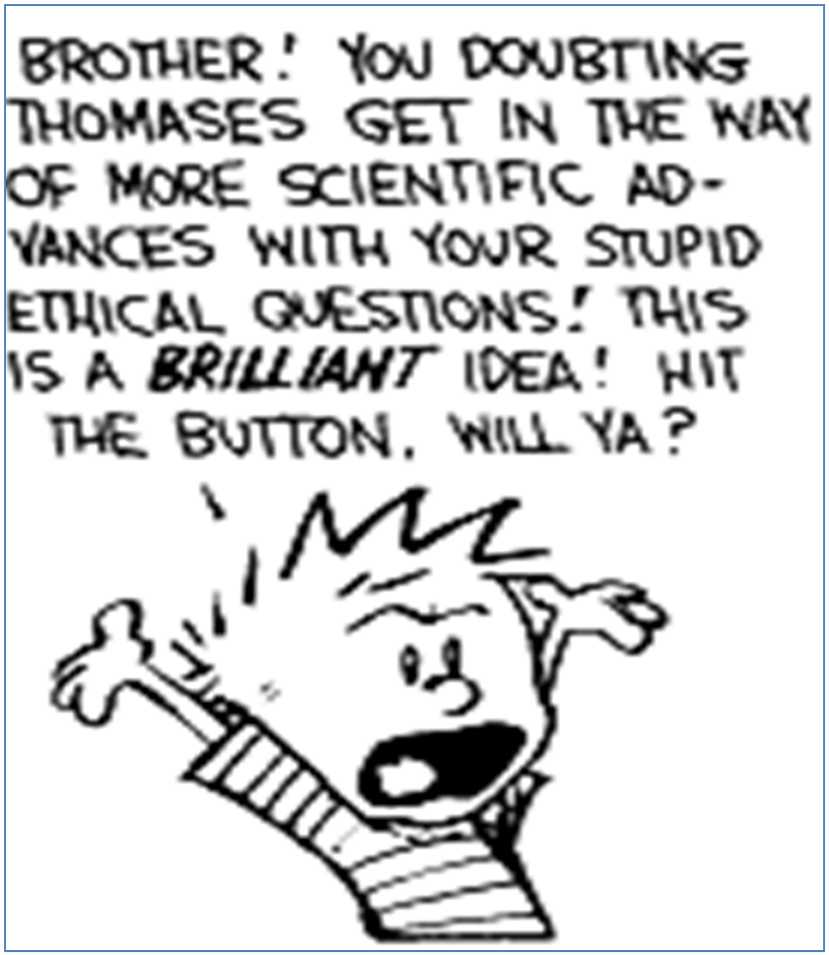
Tuttle. 2010.Curr Onc Rep

Broad Market for Personalized Med.



PriceWaterhouseCoopers. (2009) The New Science of Personalized Medicine.

Personalized Medicine and Regulation

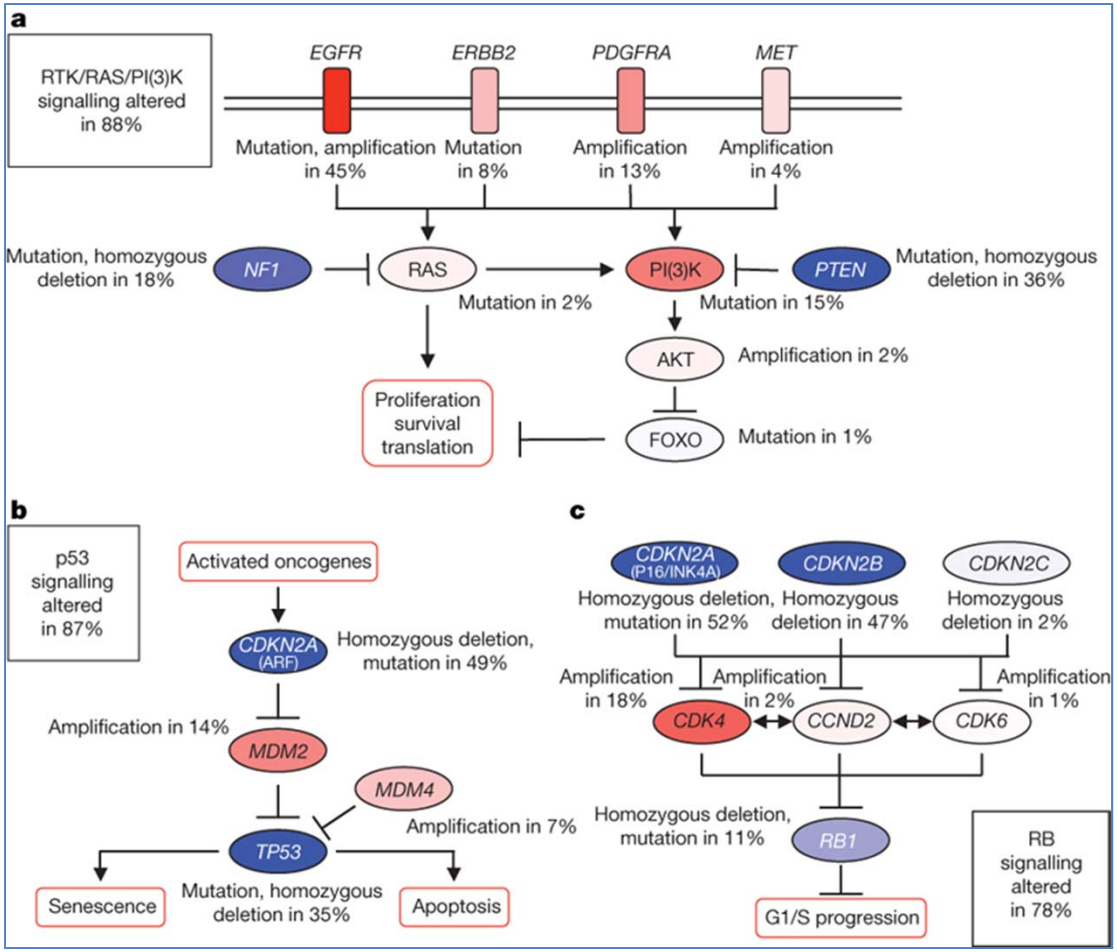


- Is the existing institutional/regulatory framework set up to promote the development of personalized medicine?

  - Very complex question
    - Clinical research: sub-populations, responders versus non-responders, complex analytics/statistics in clinical trial design
    - FDA: Risk/Benefit, “Safe and Effective”
    - CMS: What should be paid for?
    - Intellectual property – what data can be shared and who owns it
    - Market forces: What is the incentive to create a new drug targeted to smaller populations

CURRENT ISSUES: WHO OWNS THE GENOME?

CURRENT ISSUES: DEVLOPING RATIONALE DRUG COMBINATIONS



Nature **455**, 1061-1068(23 October 2008)

CURRENT ISSUES: GENOMICS IS HERE…

JNCI Journal of the National Cancer Institute Advance Access published October 21, 2010

NEWS

FDA To Regulate Direct-to-Consumer Genetic Tests

By Vicki Brower

On July 22, the U.S. Food and Drug Administration told Congress that the agency was planning to regulate genetic tests sold directly to consumers. Jeff Shuren, M.D., J.D., director of the FDA's center for devices and radiological health, testified at a House subcommittee hearing that problems with direct-to-consumer (DTC) genetic tests included faulty data analysis, exaggerated clinical claims, fraudulent data, lack of traceability, poor clinical study design, and poor clinical performance.

On the same day, the Government Accountability Office issued a highly critical report of the tests, citing, among other problems, misleading test results, deceptive marketing, and other questionable practices.

Controversy has long simmered about whether, or how, to regulate genetic tests, in particular DTC tests (*see* sidebar). But the events on July 22 brought the issue to a head. In the months that followed, articles in *Nature*, the *New England Journal of Medicine*, *The Economist*, and other publications debated DTC genetic testing. Critics pointed to all the issues raised in the hearing and report, and more broadly, the government's responsibility to protect consumers; supporters stressed an individual's right to unfettered access to genetic information. And with FDA regulation of some sort a virtual certainty, both sides began to focus on what aspects of the industry should be regulated and just how far the regulations should go.

"A DTC test may test for a high-risk condition, such BRCA1/2 status, or a low-risk condition, such as type of earwax, and should be regulated according to risk, solely on how it's marketed

Rapid Increase

It took nearly two decades for the debate, and the FDA, to reach this point. Back in 1990, when the Human Genome Project began, genetic tests were available for about 100 diseases—most of them caused by just one faulty gene, such as the mutations that cause Tay-Sachs and cystic fibrosis. As genomic technologies matured and sequencing be began develop of complex, m These were in used by phys dozens of com such tests on a

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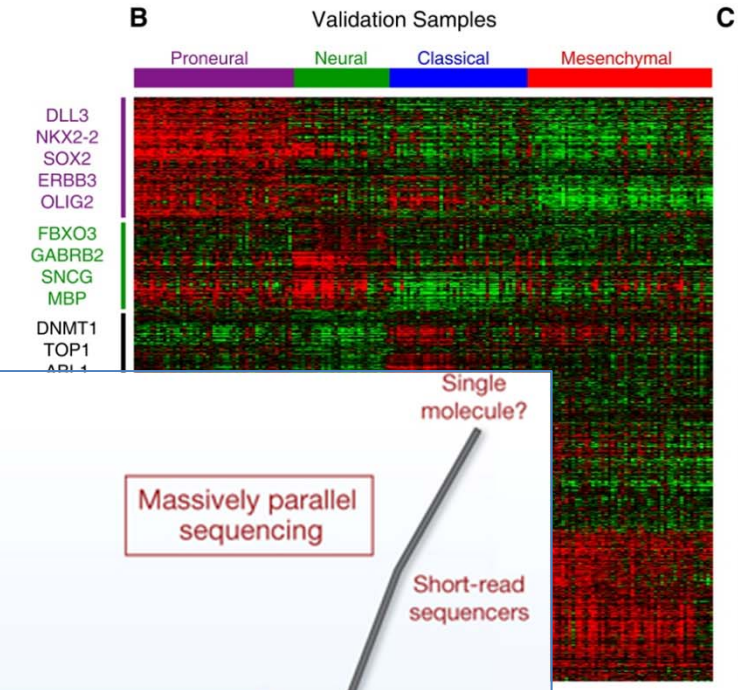
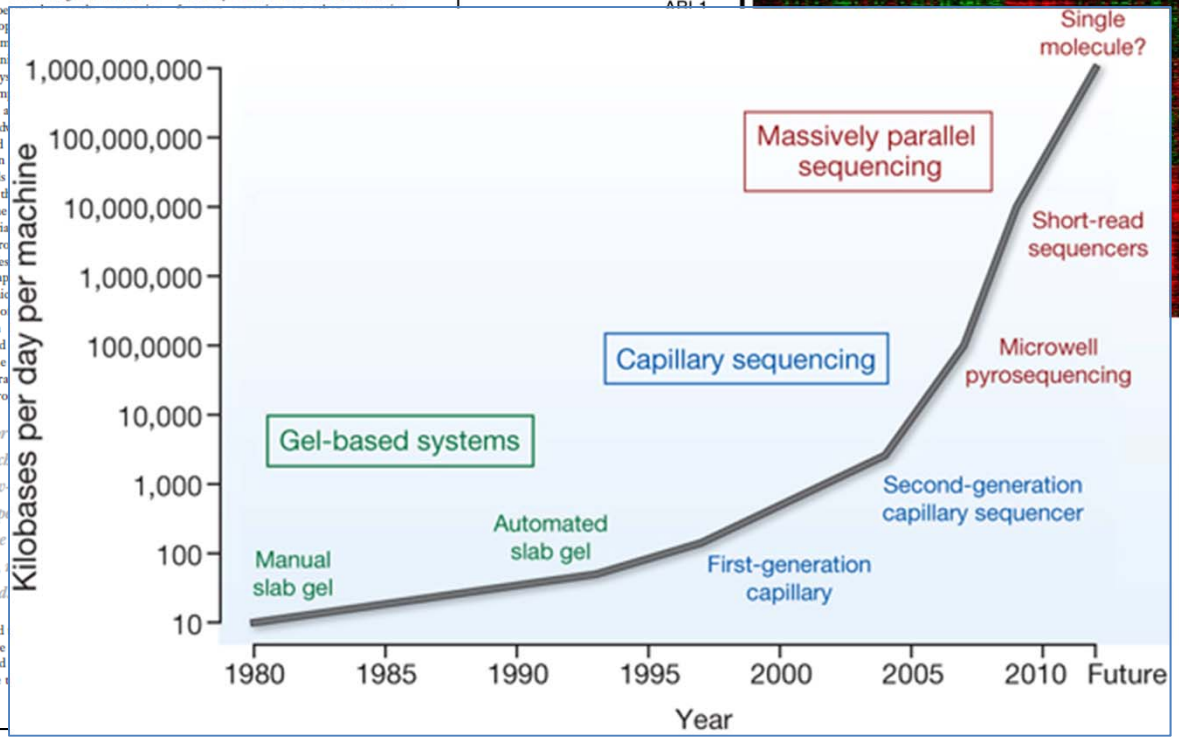
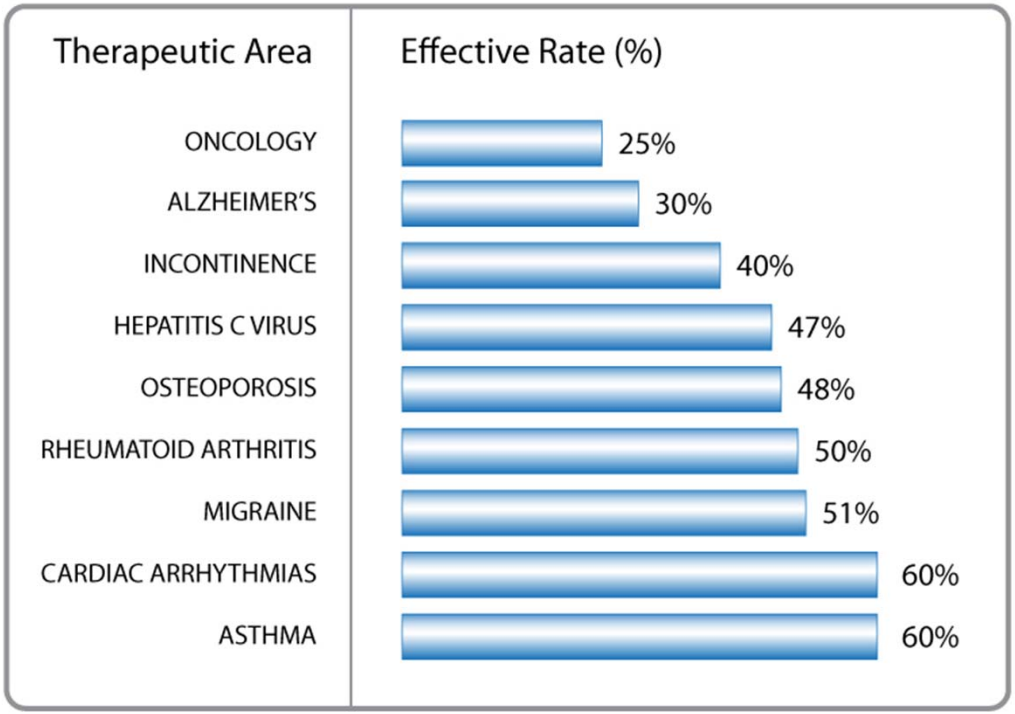
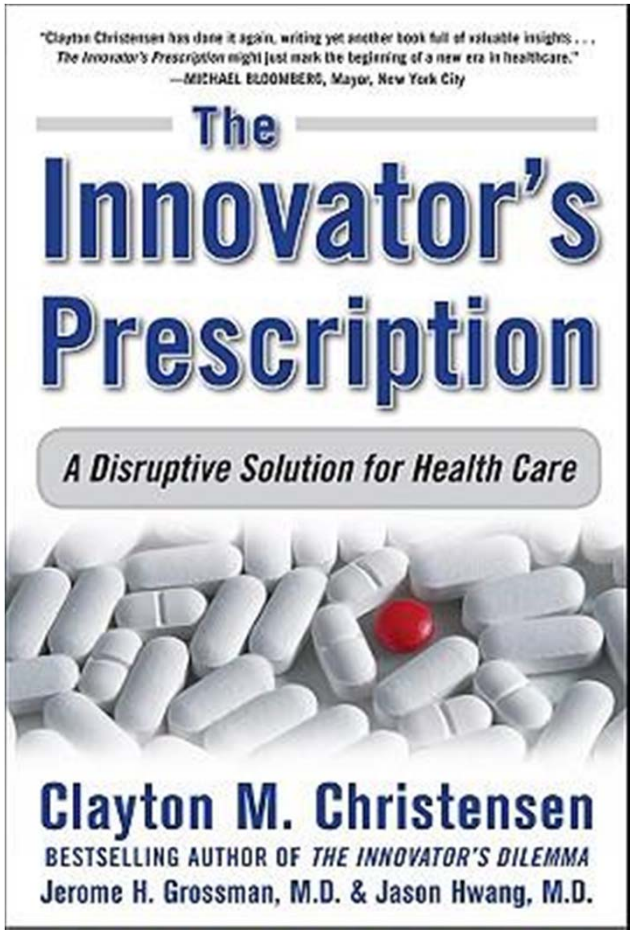




Disruptive Innovation and Precision Medicine





Spears et al. "Clinical Application of Pharmacogenetics", Trends in Molecular Medicine Vol. 7 No. 5, May 2001
Image taken from www.nodality.com