Future Challenges and Opportunities Emerging from the Mapping of The Human Genome

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Topics

- Opportunities and Challenges of Genomics
- 21st Century -- Fundamental Change
- 6 Challenges
Opportunities from Genomics and Related Trends

- Predict disease likelihood
- Predict therapeutic effectiveness & safety
- Detect disease early
- Enhance therapeutics
- Improve clinical development and regulation
- “Conquering diseases”
- Design out illness, enhance humans
- Bring health and health care to all
21st Century — Fundamental Change

- 21st Century -- The Design Century
  - Technology and society
  - The environment
  - Health
  - Health care
  - Humans and humanity
21st Century -- Fundamental Changes in Health Care

- **Scientific and research advances**
  - Genomics enhancing understanding and control options
  - Additional Advances of disease and behavior
  - Biomonitoring adding specific detail

- **Shifting paradigms in health care**
  - Enhanced, sophisticated self-care
  - Complementary and alternative approaches
  - Wellness and enhancement choices
  - Forecast, prevent and manage paradigm
Anticipated Benefits of Pharmacogemomics (NIGMS)

- More powerful medicines
- Better and safer drugs the first time
- More accurate methods of determining appropriate drug dosages
- Advanced screening for disease
- Better vaccines
- Improvements in the drug discovery and approval process
- Decrease overall cost of health care
Challenges from Genomics and Related Trends

1. Pharmaceutical economics
2. Smarter markets
3. Drug Regulation
4. Public attitudes and policy
   - Privacy, discrimination, intellectual property
   - Equity
5. Governing evolution
6. Harnessing Visionary Potential
Challenge 1: Pharma Industry Economics

- Targeting shrinks many markets
  - Some blockbusters remain
- Need/demand for lower costs of R&D and FDA approval
- New pharma players, especially in discovery and development
- Marketing costs rise and fall
Challenge 2: Smarter Markets

- Markets and marketplaces which give consumers more sophisticated choices and customized information for their decisions.
- By 2010 for most products there will be global awareness of efficacy, cost effectiveness, and price
- Broader criteria for choice will be included
Smarter Markets in Health and Pharma

- Buyers, large and small, will have increasing intelligence
- Outcome measures will be available
  - systems, individual health care
  - providers, therapy systems and components
- Report cards will become ubiquitous and well used
- Consumers will judge quality, price, and other values
Smarter Markets: Broader Values

- Pharma and other genomic companies will be judged by multiple bottom lines
  - Broader Quality measures
Smarter Markets: Broader Quality

- **Inherent quality**
  - Strength, purity; USP standards
- **Functional quality**
  - Safety, efficacy, cost-effectiveness
- **Contextual quality**
  - Currently: Worker safety = ISO 9000; Environmental protection = ISO 14000
  - Emerging: Sustainability, equity
    - e.g., Social Accountability (SA) 9000; Natural Step; WHO & Health for All
Contextual Quality: “Health For All”

- Contextual quality: What does “goodness” mean for health — what determines the right things?

  - “Health For All,” the WHO/PAHO vision as a “gold standard” — Health with these values:
    - Equity, solidarity, ethics, gender and human rights
    - US Healthy People 2010 Objectives
Challenge 3: Reinventing Drug Regulation

- Current system does not guarantee “safety and efficacy” in use
  - 1994 2 million hospitalized patients with serious reactions and 106,000 deaths (Lazarou in JAMA 1998)
  - 6.7% of people prescribed drugs have severe adverse reactions — 5th leading Cause of death in the US in 1997 (CDC, 2000)
Factors Accelerating Reinventing Drug Approval

- Empowered patients
- Patient groups and disease voluntary organizations
- Lower clinical trial costs for health providers
  - Information systems, electronic medical records, low cost biomonitoring,
- Possible changes in intellectual property
- “Smarter Markets”
Genomics and Clinical Development

- Virtual organs, patients and cohorts
- Genotype and phenotype focused studies
  - Including phenotypes from Ayurveda, Oriental Medicine
- Greater access to clinical trials
  - Disease groups play active role in design and recruiting
Reinventing Drug Regulation

- Congress will need to rewrite FDA’s drug process approval process
  - New system will need to be more:
    - Customized and predictive
    - Capable of handling multiple factors, learning from post approval data
    - Flexible and adjustable as options improve
  - Option to lower entry to market threshold, requiring greater post-market capture of efficacy, side effects; Consumers choose how much risk they want to take
Virtual Organs, Selves and Cohorts

- Virtual organs will provide "in silico" models that embed our understanding of human physiology and that can be used to test new medicines.

- Many individuals will have personalized virtual selves with their organs modeled for continuous updating and testing against new compounds.

- Use of virtual cohorts will grow.
Challenge 4: Public Attitudes and Policy

- Public reaction - depends on how well genomics is applied
  - GMO’s in foods not a good start
- Policies & protections need to be resolved
  - Privacy
  - Discrimination
  - Intellectual Property
  - Equity
Challenge 5: Guiding Human Evolution

- Momentous directions
  - Eliminate disease
  - Enhance human capacities
    - 2050 - 2 Olympics
  - Near immortality
  - Designing humans and human societies
- Private and public decisions
- Corporate players are players
Challenge 6: Harnessing Visionary Potential

- Real opportunity for generating significant health gains
- Opportunity to use these to bring “Health For All”
- Great threats, risks, in the immediate market
- Vision needed to ensure companies provide highest value added and remain competitive.
Challenge 6: Harnessing Visionary Potential

- Genomics forces all players to consider our fundamental contribution -- what type of healthcare system, health and health gains, and society we are seeking to build.
- This discussion contributes to finding value added -- this discussion is occurring in several sectors.
Value-Added is defined

Why/What

Percentage of Person/Organization’s Time

Present
Orientation
Future

Reactive
Proactive

VISION
STRATEGY
TACTICS/OPERATIONS
Challenge 6: Harnessing Visionary Potential

- Harnessing visionary potential will require:
  - All players in genomics ensuring their own vision is up to the challenges.
  - The community as a whole (the U.S. and global communities) have a shared vision and strategies that enable genomics to make its optimal contribution to “Health For All” and to conscious evolution.
Conclusion

Genomics
- gives us great power
- will make health care and pharma more complex
- can contribute to significant health gains
- invites all players to rise to the challenges, especially achieving “Health For All”