

# Challenges & Opportunities for the Use of Health Economic Information in Decision Making on New Technologies

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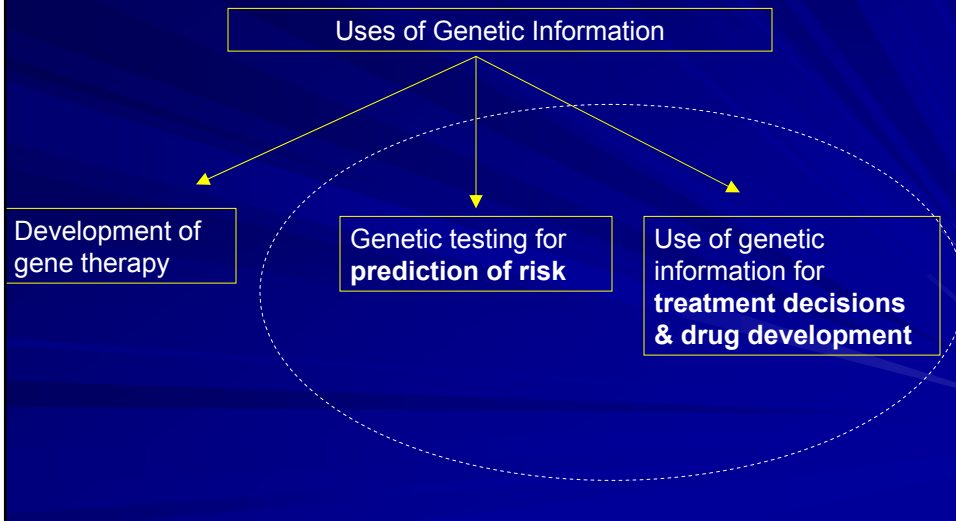
## Objective

- To examine how economics is being used in decision making on new technologies
- To focus on the example of personalized medicine

# What is Personalized Medicine?

- Targeting healthcare based on genomic information (“Genetically-enabled healthcare”)
  - Stratified medicine
    - Includes targeting of drugs (pharmacogenomics) & targeted therapies (drugs based on molecular targets)
  - Not “individualized”
  - Genomics = inherited (germline) & acquired (somatic) mutations

# Uses of Genetic Information



## Revolution or Train Wreck?

- *“In 20 years we will have “predictive, personalized, preemptive” health care”*
  - NIH Director Zerhouni
- Hype? YES BUT - inevitable trend towards greater stratification & targeting
  - Knowledge of human genomics
  - Emphasis on safety
  - High drug costs
- Regardless of whether hype or not
  - Inevitable that will change landscape of health care

## The Train Has Left The Station

- Many genetic tests & targeted drugs available or coming
  - 1700 clinics/labs using genetic tests for > 1500 diseases
  - 70 tests under consideration for review by national advisory committee (EGAPP)
  - Most activity in oncology but more coming in CHD, asthma, diabetes, mental health, AIDS
    - W/in cancer, over 62 tests available & 104 in development (as of 07)

## The Train Has Left the Station

- Historically, genetic testing has been used more frequently for prenatal/postnatal diagnosis & rare, highly penetrant genes, e.g., BRCA 1/2 (breast cancer), HNPCC (colorectal cancer)
- But personalized medicine is being or could be used in clinical practice increasingly for common and/or chronic diseases

## The Train Has Left The Station

- Cancer
  - HER2/neu testing for trastuzumab (Herceptin)
  - Gene expression profiling for cancer (Oncotype)
  - CYP2D6 testing for tamixofen
  - TPMT testing for leukemia, etc
  - UGT1A1 testing for irinotecan (Camptosar)
- Coronary heart disease
  - CYP2C9 & VKOR testing for coumadin (Warfarin)
  - Beta1-adrenergic receptor testing for beta-blockers (bucindolol)
- Psychiatric illness
  - CYP2D6 testing for SSRI's
- AIDS
  - HLA-B\*5701 screening for abacavir

## The Train Has Left the Station

- Industry using data for drug development
- FDA pursuing initiatives to promote personalized medicine
- Health plans & employers looking for approaches to better target interventions – and personalized medicine offers hope
- Government looking to personalized medicine to provide better care at lower cost

## Challenges/Opportunities for Personalized Medicine

Aligning Incentives

Determining Value  
&  
Reimbursement



Balancing Innovation  
& Regulation

Building an Evidence  
Base

## Using Economics

- What is ‘value’ in genomic-based translational research, specifically personalized medicine?

- How to measure economic value?

- Evaluating cost-of-illness
- Criteria for cost-effectiveness of PGx
- Criteria for PGx to be economically viable in the marketplace

- Challenges to assessing value and implications for translation of new technologies

## The Bottom Line

- How should value be defined and by whom?

- Value does not equal cost

- FDA? CMS? Health Plans? Employers? Professional groups? Government agencies? Pharma/diagnostic industry?

- What should be considered sufficient “evidence”?

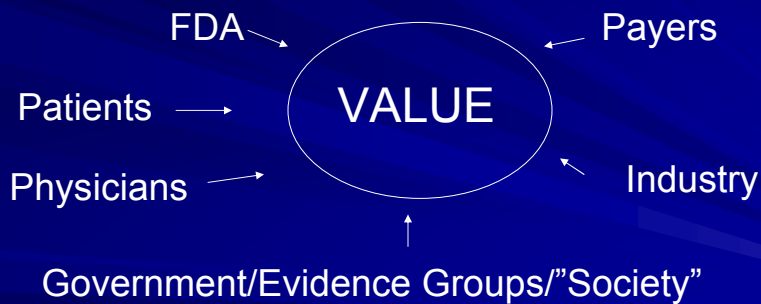
- Who should pay for that evidence?

# Adoption Requires Evidence & Value

- For successful adoption into clinical practice, a test should demonstrate:
  - Analytic validity – accuracy of test for genotype
  - Clinical validity – accuracy for clinical outcome
  - Clinical utility – ability to inform clinical decision making, prevent adverse outcomes, predict outcomes
  - Economic value - impact on health sector, cost-effectiveness, and commercial viability

- Secretary's Advisory Committee on Genetics, Health and Society, 2007

## “Value” is in Eyes of Beholder



## Challenges to Establishing Value: “The Poor Step-Child”

- Diagnostic industry has historically been “secondary” to pharma industry – but now playing increasingly important role
  - Gene expression profiling test - Oncotype (Genomic Health) – now darling of industry
  - Requires integration of historically divided industries and regulatory mechanisms
  - Can require early consideration of diagnostics in drug development process

## Challenges to Establishing Value: “Flying Under the Radar”

- Reimbursement system is challenging
  - Traditionally not “value-based” reimbursement for diagnostics
  - Personalized medicine can be either “screening” or “diagnosis” or both
  - Payers can’t track use of diagnostics in claims data

## Challenges to Establishing Value: “The Black Box”

- Little data on clinical utility of diagnostics
  - Few RCTs
- Few economic analyses
- Demonstrating value requires linking ability to target to improved outcomes
  - Testing then treatment then outcomes
  - Impact on family members

HER2/neu & trastuzumab (Herceptin):  
How Even a Successful Product Generates Questions