Personalized Medicine - Implications for Medical Technology

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Introduction

The vast implications of genetics and genomics are beginning to sink in. Many sectors in the healthcare industry are rethinking their fundamental business model.

• What impact will genetics and genomics and personalized medicine have on medical devices?
• How will personalized medicine—with its targeted therapies, individualized treatment, niche markets, and gene-based treatments—affect how devices are developed, marketed, and commercialized?
• And what impact will it have on customer demands and expectations?
Issues in Personalized Medicine

• Reimbursement for new diagnostics
• Regulation
• Payer reactions
• Social challenges/racial/cultural
• Implications of nanotechnology
• Implications of regenerative medicine/stem cells
• Clinical workforce adoption
The Healthcare System is Expanding in Scope and Shifting Toward Personalized Medicine

**Today’s Healthcare System:**

- Healthy
- Vulnerable
- Affected
- Sick

**Future Healthcare System:**

- Healthy
- Vulnerable
- Affected
- Sick

*Wider scope of focus; blurring of lines between populations; increased personalization*
In the future, patients will need to navigate a health system that is:

- Broader in scope
- Different in substance with far more complexity
- More options for engagement
- More specialized in resources
- More diverse in payment sources
Today, doctors and hospitals are the primary axes in the healthcare system, but in the future they will be nodes in a large and complex network. Fundamentally new ideas need new organizational structures.
The future of medicine has a wide range of stakeholders

All of these players need to come together to create changes in the healthcare model.

Change will require substantial investment: both in time and money.

Personalized Medicine will generate new health and business models.
What is next in personalized medicine?

Case Studies - Current initiatives - from practice to proposal......

• Molecular Profiling Institute – Dr. Dan Von Hoff
• Partnership for Personalized Medicine – Dr. Lee Hartwell, Dr. Jeff Trent and Dr. George Poste
• Mayo Clinic strategy – Dr. Frank Prendergast
• P4 Medicine - Dr. Lee Hood
Molecular Profiling Institute (Caris MPI)

- A reference lab that offers comprehensive patient information in a personalized format for physicians and for clinical research sponsors.
- Originally created by the International Genomics Consortium (IGC) and the Translational Genomics Research Institute (TGen).
- Caris MPI introduces discoveries made in the research lab to clinical patient care.
- Currently offering tests to help oncologists to better understand and treat cancer patients with personalized treatment plans based on the molecular characteristics of their tumors.
Molecular Profiling Institute (Caris MPI)

- Caris MPI's *Target Now* offers advanced molecular tumor analysis in the research setting and provides potential therapeutic options to cancer patients for whom several standard therapies have failed.

- Based on the molecular profile of a patient's tumor, the program generates potential treatment options that would likely otherwise not be considered (e.g. a breast cancer drug for pancreatic cancer).
Partnership for Personalized Medicine

- The Partnership for Personalized Medicine addresses two critical issues in healthcare:
  - Improving patient outcomes
  - Reducing healthcare costs

“The challenge we face is to improve patient outcomes while stabilizing or reducing the costs of healthcare.”

Lee Hartwell
Partnership for Personalized Medicine

- Model is to create partnerships leveraging the full suite of genomic and proteomic capabilities provided by the PPM partnering with dedicated healthcare systems to complete demonstration projects that integrate four key elements:
  1) A cohesive and interactive partnership between health insurance providers, clinicians and researchers;
  2) Epidemiologic, clinical and economic analysis to identify critical intervention points in disease management;
  3) Systematic and empirically-based discovery, development and validation of new diagnostic tests to improve patient outcomes and reduce system costs;
  4) Collaborative, prospective and evidence-based evaluation of the test within health systems to validate and implement the new test in patient management.
Mayo Clinic Individualized Medicine Diagnostic/Prognostic Development

- **Biomarker Discovery**
  - Basic biology
  - Differential genomics
  - Proteomic detection

- **Biomarker Assay**
  - Samples/sops
  - Analyte detection
  - Method development
  - Panels

- **Assay Validation**
  - Quantification
  - Controls
  - Precision/reproducibility
  - Accuracy
  - Standardization

- **Diagnostic Validation**
  - Clinical assessment
  - Stratification
  - Diagnostic clinical trials
  - Diagnostic “teams”
  - Biomarkers
  - Surrogate endpoints

- **Launch**
  - Reagents
  - Kits
  - Instrumentation
  - Reimbursement
P4™ Medicine

• Predictive

• Personalized

• Preventive

• Participatory
Predictive, Personalized, Preventive and Participatory Medicine (P4 Medicine)

• **Predictive:**
  – Probabilistic health history--DNA sequence
  – Biannual multi-parameter blood protein measurement

• **Personalized:**
  – Unique individual human genetic variation mandates individual treatment
  – Patient is his or her own control
  – Perturb blood cells for dynamic measurements
  – Go directly to patient and skip doctor--patient will have all medical information

• **Preventive:**
  – Strategies for re-engineering the behavior of disease- perturbed networks with drugs
    Vaccines
    Focus on wellness

• **Participatory:**
  – Patient understands and participates in medical choices
Blood Protein Diagnostics

New diagnostics for new biology
Organ-specific blood proteins will make the blood a window into health and disease

*Pharmaco-proteomics*

- Use for determining appropriate individual drug dosage
- Follow response to therapy
- Assess drug toxicities
- Use for prediction & assessment of drug toxicity
- Stratify the individual’s response to drugs
- Assess toxicity of combinations of two or more drugs

* Quantification of organ-specific blood fingerprints will provide insights into drug toxicity, effective use, appropriate dose, organ interactions etc.
Deal for *In vitro* molecular diagnostics:

*Integrated nanotech/microfluidics platform*

Separate plasma & rapidly quantitate protein biomarker panels
- Profile health status of individual organs
- Select appropriate therapies or combination therapies
- Profile positive & adverse responses to therapies

Panel of protein biomarkers measured in a single microfluidics channel
(15 min assay time)

Organ 1  Organ 2  Tox response  inflammation

Dynamic range--$10^8$
Sensitivity--high atmole

Jim Heath, et al
In vitro blood protein diagnostics

Perhaps 50 major organs or cell types--each secreting protein blood molecular fingerprint.

The levels of each protein in a particular blood fingerprint will report the status of that organ and thus distinguish health from disease--and if disease, which disease. Probably need perhaps 50 organ-specific proteins per organ.

Quantitate 2500 organ-specific proteins to:
- identify disease;
- stratify disease;
- progression of disease;
- response of disease to therapy etc.
“Science and technology are beginning to provide revolutionary insights into medicine through a comprehensive molecular understanding of human health and disease.”

Lee Hartwell