Pathways to Personalized Medicine

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“Personalized Medicine: the use of marker assisted diagnosis and targeted therapies designed from an individual molecular profile”

Ginsburg and McCarthy 2001
The Promise:

- Target specific therapy
- Avoiding toxic results
- Targeting susceptible populations
- Reducing drug development costs
- Treating the primary cause rather than symptoms
Some Victories:

- Gleevac – Leukemia
- Herceptin – Breast Cancer (HER-2 Screening)
- Warfarin – Metabolism
- Childhood Leukemia – Drug Metabolism
Capabilities:

- Rapid sequencing
- DNA, mRNA, proteomics analysis
- Advances in structural analysis
- Designer structures
- Intracellular modeling
The Practicalities:

- Rarely a single target
- Economics of drug development
- Market size reduction
- Cost per person
The History:

- War on cancer 1972
- Genome project $\rightarrow$ cures
- Gene therapy
- Decrease in new drugs to FDA
Ethical Legal Issues:

- Genetic screening – everyone predisposed to something?
- Laboratory accuracy
- Screening without interventions?
- Anxiety without benefit
- Insurance implications of predisposition
- Limiting profile to available therapies
- Legal protections for information
Opportunities:

- Can we avoid the hype?
- Progress usually incremental until a breakthrough technology
- Group products versus individual application
- Advance the basic understanding of structure and function
- Cohort studies lead to good questions – rarely definitive answers
- Support good ideas
Strategies:

Interdisciplinary Approaches
- Molecular biology
- Mathematics
- Chemistry
- Physics
- Structural biology
- Computational biology
- Computer science
- Pharmacology
- Clinical investigation
- Engineering
Strategies:

- Collaborative resources
- Computing capacity
- Structure technologies
- Imaging
- Clinical trials
- Populations
- Engineering
- Modeling concepts