Medicine Technical Panel

Facilitated Session Results

October 22, 2008

Medicine: Future Characteristics/Vision

- What the future holds
  - Personalized medicine will be a reality – a physician will be able to integrate an individual's genotype and phenotype, diagnose disease based on a signature, select type of drug, dose and monitoring needed
  - Dynamics of measures over time
  - New computational approaches to analysis
  - Standardization in sample data collection
  - Standardization for how signatures are characterized
  - Commerce in info and ideas to the medicine enterprise
  - More affordable care
  - Turn the way medicine is practiced on its head (e.g., fund prevention)

Medicine: Highlights of Broad Challenges and Barriers to Reaching our Vision

- Science and Biology
  - Lack of accepted standard phenotype description off disease and normal populations
  - What assays to use for testing health or disease
- Info/Data Management
  - Massive quantities of non-comparable data
  - Data management computational barriers
- Resources and Incentives/Economics
  - Lack of funding
  - Competing priorities – science, economic, risking health care costs

Medicine: Highlights of Broad Challenges and Barriers to Reaching our Vision

- Standards
  - Lack of standardized unit of measure ("Gold Standard") for comparing, compiling and analyzing across instrumentation technologies, platforms, etc.
  - Standards / reference materials for all medical tests (portability)
- Education/communication
  - Lack of collaboration between industry and academia on a larger scale
- Technology
  - Technical standardization of samples, assays, protocols, data, etc.
- Health care policy
  - Privacy – Will people agree?
- Regulatory

Medicine: Approaches to Selected Priority Measurement & Standards Barriers

- "Phenotype" standardization
  - Objectives: define what phenotype means for disease, perturbed or normal pathways, biologic correlates of descriptive characteristics; consider severity, changes over time in disease activity and environmental or therapeutic outcomes in phenotype
  - Rationale: correlates between phenotype and underlying pathways and biological mechanisms in health and disease

- Prioritization/Resource allocation
  - Conclusion – we don't know which analytes are most important/relevant
  - Define and promote health
  - Develop tools to define health
  - Different prioritization scheme for therapeutics – funding

  Therapeutics
  - Understanding clinical and biologic phenotypes
  - Understand pathobiology, identify novel targets, improve trial design
  - Long term goal of targeted or even personalized medicine

Medicine: What We Need to Measure and Why

- Outcomes measurement
  - Good quantitative measures of clinical phenotypes – Without this, the diagnostic is not much use
  - Consistency of input information – Retrospective analysis
  - Longitudinal data (biomarkers and clinical information) – To better characterize individual patients and populations
  - Decrease morbidity, greater quality of life measure – What you actually get from what you put in
- "What" to measure
  - DNA, RNA, protein, metabolites, lipids, cell function (blood, others), organs, organization, etc.
  - Imaging-- Xray, MRI, CT,

Medicine: What We Need to Measure and Why

- Dynamics of measures over time: Longitudinal Testing
  - Measure molecular structure and dynamics at resolutions beyond current technologies—provide basis for comparison
- Validation & standardization of measurement
  - Variables that are not platformdependent
  - Sample integrity
  - Statistical standards
  - Process integrity and quality
  - Measure biologically validated markers
- Cost effectiveness
  - Health care improves…for all

Medicine: Selected Priority Measurement & Standards Barriers

- Clinical phenotype and outcomes
  - Define and standardize phenotypes –consensus to avoid inconsistency
  - Define disease categories and dynamic disease symptoms /characteristics
- Prioritization
  - Predicting the future
  - Evolving knowledge base
- Therapeutics
  - Detection and identification of molecule processes at high time and space resolution
- Simplify technology
  - Point of care device – Reference material and controls?

Medicine: Selected Priority Measurement & Standards Barriers

- Sample integrity/preparation
  - Standardized and validated methods for sample processing
- Standardization across platforms
  - Comparability between technologies
  - Time, money
- Reference Materials
  - Traceability of biological measurement results to an absolute or relative reference
  - Missing definition of a “normal”, “healthy” reference value and range when a patient is own control

Medicine: Approaches to Selected Priority Measurement & Standards Barriers

- Sample integrity and Preparation
  - Objectives: consistent sample collection and storage so samples can be used across platforms, maintain integrity over time, and provide for traceability and meaningful results
  - Rationale: to provide for quality results to achieve long term vision of personalized medicine and provide internal integrity markers, automated annotation of time, temperature, etc., clinical data integration tools
- Standardization across Platforms/Reference Materials
  - Objectives: Comparability of data across platforms and creation of a platform independent reference material
  - Rational: Enables cross validation of platforms, facilitates innovation and increases confidence in the measurement

Summary

- Personalized medicine will be a reality – a physician will be able to integrate an individual's genotype and phenotype, diagnose disease based on a signature, select type of drug, dose and monitoring needed
- Healthcare system will require a transition to wellness monitoring
- Impact on healthcare costs will be the measure of our moral compass.

Medicine: Approaches to Selected Priority Measurement & Standards Barriers

- Phenotype" standardization
  - Objectives: define what phenotype means for disease, perturbed or normal pathways, biologic correlates of descriptive characteristics; consider severity, changes over time in disease activity and environmental or therapeutic outcomes in phenotype
  - Rationale: correlates between phenotype and underlying pathways and biological mechanisms in health and disease

  - Prioritization