Slides for Break Out Session

Strategic Planning Process

- Develop program portfolio
  - Near-term 25%
  - Mid-term 50%
  - Long-term 25%
- Near-term program determined by industry workshops
- Mid-term projects combination of longer time scale near term needs and strategic projects
- Long-term projects determined by strategic vision of where industry is headed

Key Trends on the horizon for the Pharmaceutical Industry

- **<u>Personalized Healthcare</u>**
  - Getting the right medicine to the right patients at the right time

- **<u>Continued Drive for Highly Innovative New Medicines</u>**
  - Better understanding of disease pathways

- **<u>Discovery and Enablement of the "Next big thing" in drug discovery</u>**
  - Historical example : Monoclonal Antibodies
  - Future opportunity : Therapeutic RNAi?

Technology Platforms that could influence the Trends

- **Imaging**

  - Goal is to use technologies that are non-invasive to the patient
  - Biomarkers can provide the predictive information of disease and patient stratification
    - Currently use plasma samples, tumor biopsy and gene arrays with the exact method depending on the disease area

  Any increase in image resolution and quantitative measurement tools associated with imaging techniques may aid in improved disease diagnosis and treatment

- **Mass Spectrometry Technologies**

  - Analysis of proteins which is important for biomarkers and path ways for new medicine
  - We can analyze gene expression (RNA) with gene array chips, but no equivalently robust platform for proteomics
    - We cannot survey post translational modifications with adequate speed and sensitivity

  Need increased sensitivity and analyze multiple samples in parallel,
  Other technology platforms might also be applicable to proteomics

- **Delivery Technology for Oligonucleotides**

  - Examples of what is being looked at to do this are liposomes and nanoparticles

  All efforts to improve ability to deliver olignoculotides is key for new areas of disease management

Personalized Medicine

- The approach is to truly understand disease at the individual patient level

- Driving Forces
  - Improved efficacy to every patient and reduce side effects
  - Minimize or eliminate drugs going to patients where it will not help based on their individual biology
  - Cost of health care and insurance focus on not paying for care if efficacy can not be demonstrated

Innovative New Medicines

- Driving Force:
  - The biological pathways underlying several of the major diseases are still not understood
  - The sequencing of the human genome and the development of gene chips for analysis of messenger RNA has facilitated assessing the underlying biology, however, measurement of post translational protein modifications is still tedious

  Opportunity for Mass Spectrometry, protein on chips, antibody arrays and other Proteomics Technologies

21st Century Diagnostic Medicine

- **Predict:** Determine disease proclivity
  - DNA SNP pattern
  - Family history
- **Screen:** Detect early for effective intervention
  - Imaging
  - Body fluid trace analysis
- **Diagnose:** Precisely specify disease phenotype to choose optimal therapy
  - Molecular analysis
  - Imaging
- **Monitor:** Measure response to therapy and provide early detection of recurrence
  - Imaging
  - Body fluid trace analysis

Predict Disease Proclivity

- DNA SNP and mutation analysis
  - Nucleic acid sequencing
- Protein biomarkers in blood
  - Protein mass spec and correlations
- Metabolites in blood, urine, and tissue
  - Trace element analysis

Screen

- Imaging
  - Cancer early detection
    - CT, MRI, US, OCT quantitative imaging
  - Coronary artery disease
  - Embolisms
- Blood testing
  - Hematology
    - Immune system
      - Flow cytometry
  - Blood chemistry
    - Protein biomarkers of disease
      - Protein Mass Spec
  - Metabolics
    - Diabetes
      - ELIZA, blood chemistry analysis

Diagnose

- Tumor molecular phenotype analysis
  - Microgenomics
    - DNA microarrays, Q-PCR, NA amplification
  - Micro-proteomics
    - Protein mass spec, protein arrays, ELIZA
- Imaging
  - Tumor size and shape
    - Feature extraction and quantitation
    - Functional characteristics
      - Functional biomarkers for MRI and CT

- DNA mutation analysis
  - SNP arrays, high throughput sequencing, amplification
- Blood testing
  - Trace chemical analysis
    - Mass Spec

Monitor Treatment response

- Imaging
  - Tumor size/morphology changes for drug response
    - Quantitative imaging
- Blood tests
  - Chronic disease management
    - Aids: Flow cytometry, viral load
    - Diabetes: Glucose levels
    - Stroke: Blood clotting levels
    - Cancer: Rare cell detection, proteomics (?),