Pharmacogenomics, Market Opportunities and Barriers

Presenter: Patrick J. Hurd, Esq.
Senior Counsel

Moderator: James P. Anelli, Esq.
Shareholder

January 13, 2011
Welcome. Due to the number of attendees, please note all lines have been muted for the event. Q&A can be posted at the right of your screen, but any questions (time permitting) will be addressed at the end of the event. If using Q&A – please send to both the host and the presenter. You can send direct questions (including request for copy of slides) to seminars@leclairryan.com with Pharma in the subject for reply after the event.
Some news on LeClairRyan

- New talent joins LeClairRyan in Rochester, NY, San Francisco, Chicago, Washington, D.C. and Northern Virginia – approx. 34 attorneys an professional administrative staff including patent agents
- Expands LeClairRyan’s depth and breadth in intellectual property, commercial and securities litigation, and bankruptcy and restructuring.
- In particular, the new additions to our intellectual property team is nationally recognized in patent prosecution, interferences, reissues, and reexaminations, and patent litigation in the biotech, pharmaceutical, software, chemical, electrical and physical science fields.
Pharmacogenomics

- What is Pharmacogenomics?
- How does FDA regulate Pharmacogenomic products?
- What are the BioPharma and Diagnostic market potentials?
- What are the market barriers?
- What is on the horizon?
Pharmacogenomics

- What is Pharmacogenomics?
  - How inherited variations in genes dictate a person’s reaction to a drug
  - Combines pharmaceutical sciences and gene sciences
  - Outgrowth of the Human Genome Project
  - Sometimes referred to as “Personalized Medicine”
Pharmacogenomics

- “Personalized Medicine”
  - Variations in genetic makeup, environmental conditions and lifestyle influence the severity and type of disease and responses to therapeutics
  - Genetic tests that assist in determining targeted drug therapies and treatment protocols to minimize side effects and improve outcomes
  - Genetic tests that determine disease susceptibility
Pharmacogenomics

- Some articulated goals:
  - Shift emphasis to prevention, not reaction
  - Enhance drug safety
  - Optimize therapy
  - Improve patient compliance
  - Reduce healthcare costs
Pharmacogenomics

- Developments in FDA’s Regulation of Pharmacogenomics
  - Center for Drug Evaluation establishes Voluntary Data Exchange Program in 2003
    - Companies to submit genomic data collected during drug development process
    - Reluctance at first, but voluntary submissions increasing

- Critical Path Initiative to validate biomarkers
Pharmacogenomics

- FDA (continued)
  - Statutory authority and regulatory scheme differs for drugs/biologics and medical devices
    - Genetic tests are devices
    - Drug efficacy and safety may depend on genetic test precision
    - Some genetic tests may help target variety of drug therapies
    - Specific drug efficacy tied to specific genetic test identifying specific biomarker
Pharmacogenomics

- FDA (continued)
  - Office of Combination Products
    - Established in 2002 & focused on more traditional drug/device, drug/biologic and device/biologic products
      - Drug eluting stents
      - Antibiotic bone cement
      - Transdermal patches
    - Office now plays role in evaluating genetic test/drug therapy products
Pharmacogenomics

- FDA (continued)
  - Some early examples of agency actions involving “personalized” drugs
    - Warfarin
    - Abacavir
    - Erbitux & Vectibix
    - Cisplatinum
Pharmacogenomics

- FDA (continued)
  - Warfarin
    - Known for serious and frequent adverse events
    - 2007 FDA approved label change to list genetic tests relevant to dosage
    - 2010 FDA approved another label change to match specific genetic test results to specific initial doses
  - Abacavir
    - Certain patients suffered serious adverse reactions
    - Genetic variant HLA-B 5701 in at risk patients and FDA required label warning
Pharmacogenomics

- FDA (continued)
  - Erbitux & Vectibix
    - Patients with K-RAS gene mutation unlikely to benefit from these drugs
    - Label recommendation now required
  - Cisplatinum
    - Potential to treat children with leukemia
    - Genetic allele differences used to vary dose or select alternative drug therapy
Pharmacogenomics

- FDA (continued)
  - FY2011 Budget for Advancing Regulatory Science for Public Health
    - Build scientific capacity to assist in pharmacogenomic product approvals
    - Develop product evaluation standards
    - Establish Office of Science and Innovation to coordinate scientific expertise especially in emerging technologies
Pharmacogenomics

- FDA (continued)
  - Office of Clinical Pharmacology
    - Integrated Review Process for Pharmacogenomics
    - Pharmacometrics and Genomics staff to manage quality and consistency of regulatory reviews
    - Good Review Management Principles (GRMP) to shorten review time periods and increase efficiencies
    - See “An Integrated Genomics, Pharmacometrics and Clinical Pharmacology review Process,” MAPP 5100.5
Pharmacogenomics

- FDA (continued)
  - Office of the CDER Director Interdisciplinary Pharmacogenomics Review Group (IPRG), see MAPP 4180.2 (March 16, 2005)
  - “Guidance for Industry, Pharmacogenomics Data Submissions,” (March 2005)
  - “E15 Definitions for Genomic Biomarkers, Pharmacogenomics, Pharmacogenetics, Genomic Data and Sample Coding Categories (April 2008)
Pharmacogenomics

- Market Potentials
  - 2004-2005 Study at William & Mary Center for Public Policy, led by L.F. Rossiter, PhD.:
    - Summarized both marketed products and those in premarket approval stage
    - Interviewed industry and academic representatives to identify mechanisms for bringing pharmacogenomics to market
    - Studied financial and organizational aspects of healthcare impacting pharmacogenomics
Pharmacogenomics

- Market Developments
  - Rossiter study findings:
    - Few products on market at that time, but many in premarket approval stage
    - Stakeholders include genetic analysis companies, molecular diagnostic companies, research and manufacturing companies and distributors
    - Competition exists at market and physician levels
    - Physician role in development remains undefined
    - Private investors key to future
Pharmacogenomics

- Market Developments

  - Rossiter Study Conclusions:
    - Healthcare payment system not conducive to development and market entry of pharmacogenomics
    - Funding for cost-effectiveness research should be increased
Pharmacogenomics

- **Market Developments**
  - November/December 2010 Tufts Center for the Study of Drug Development Impact Report, Christopher Paul-Milne, author
    - Focusing more on “personalized medicine” in research and development protocols/approaches for new products
    - 12-50% of Pharma companies developing “personalized medicine” facets of their overall R&D programs and organizational structures
Pharmacogenomics

- Market Developments
  - Tufts Impact Study
    - High costs require multiple external partners
    - Biomarkers used more frequently in clinical trials but of little utility in drug approval process until FDA acts
    - Oncology leads market for existing and developing products
    - Cardiovascular, CNS and Immunologic Therapies also receiving greater focus of product development efforts
Pharmacogenomics

- Market Developments
  - Academic Centers and Research Hospitals play pivotal role in product development
    - Ohio State, Cleveland Clinic, Harvard, Stanford, Fox Chase Cancer Center, for example
  - Public-Private Partnerships may also be important vehicles for funding necessary research and development
Pharmacogenomics

- Market Barriers
  - FDA Regulatory Approval Process for Pharmacogenomics remains a work in progress
  - Complexity of the Science
  - Need for further refinement of drug company R&D
  - Intellectual Property Issues
  - Costs and Uncertainty of Reimbursement
**Pharmacogenomics**

- **Market Barriers**
  - FDA Approval Process
    - Some changes implemented but uncertainty remains
    - Consistent, comprehensive and integrated approach yet to materialize
    - Expectations for clinical trials need clarity
    - Inconsistencies in evidence necessary to demonstrate safety and efficacy
    - Communication channels between agency and industry need improvement
Pharmacogenomics

- Market Barriers
  - Complexity of the science
    - Depends on single nucleotide variations of which there are millions
    - Difficult to identify which genes are associated with what drug response
    - Varying gene effects and overall inherent biological diversity create statistical problems in establishing efficacy and safety
    - Data not replicated across clinical trials
Pharmacogenomics

- Market Barriers
  - Need to Refine R&D
    - Limited drugs available to treat a specific condition
    - Costly and Time consuming to change from “one size fits all” to many and varied alternative drugs
    - Selecting and assuring effective partnerships with genetics testing entities
    - Role of healthcare provider community in R&D yet to be clearly established
Pharmacogenomics

Market Barriers

• Intellectual Property Issues
  – Patentability of Pharmacogenomics
    • See also Prometheus Laboratories, Inc. v. Mayo Collaborative Services, 08-14-3
    • See also Association for Molecular Pathology v. USPTO, United States District Court, Southern District of NY, 09 Civ. 4515 (March 29, 2010) now on appeal at the U.S. Court of Appeals for the Federal Circuit.
  – Ownership and Control issues among joint partners in research and development
Pharmacogenomics

- Market Barriers
  - Intellectual Property Issues
    - Access to clinical trial data and other key technical information from drug development for use in genetic test development
    - Balancing the protection of Trade Secrets and CBI with the need to share information among collaborators and consortiums
    - Increasing knowledge and awareness of IP issues among academic and healthcare provider researchers
Pharmacogenomics

- Market Barriers
  - Costs and Uncertainty of Reimbursement
    - Small targeted disease populations and high costs of R&D may limit the economic viability of many products
    - No “bright lines” for measuring successful progress in clinical trials phases may hinder investor interest
    - Insurers consider pharmacogenomics “experimental” and remain reluctant to approve coverage for such testing/therapies
What’s on the horizon?

- Pharmacogenomics products continue to move along the premarket approval process, adding to the knowledge base as to how FDA views such innovative technology
- BioPharma industry continues to trend toward increased focus on Pharmacogenomics R&D
- Physician and Patient Education improves, creating greater market interest in “personalized medicine”
Pharmacogenomics

“It’s far more important to know what person the disease has than what disease the person has.” Hippocrates
Thank You.

Patrick J. Hurd
patrick.hurd@leclairryan.com
757.441.8931

James P. Anelli
james.anelli@leclairryan.com
973.491.3550
Disclaimer

- This presentation provides general information and is not legal advice and should not be used or taken as legal advice for specific situations. You should consult legal counsel before taking any action or making any decisions concerning the matters in this presentation.

- This communication does not create an attorney-client relationship between LeClairRyan, A Professional Corporation, and the recipient.

- Copyright 2011 LeClairRyan, A Professional Corporation. All rights reserved.