Changing Landscape, Changing Needs

Regulatory Demands and Responsibilities

Biotech / Pharma
Blockbuster Boom
1980 – 1990s

Mergers and Acquisitions
1990s – 2000s

Drug Safety Concerns
2004
(ex. Vioxx)

PERFECT STORM!
Decline in Productivity;
Rise in Product Failures;
Unsustainable Paradigm

EMERGING REGULATORY TRENDS:
ICH to Local Heterogeneity
Collaboration amongst Regulatory Agencies
Adaptive Trial Designs
CV Outcomes Studies
REMS Development
Advanced Therapeutic Pipelines
Personalized Medicine
Orphan Drug Indications

Regulatory Science in the Drug Development Process

- Chemistry
  - Synthesis
  - Chemical and analytical characterization
  - Scale up manufacture
- Pharmaceutical
  - Development pharmaceutics
  - Analytical specifications
- Pharmacology
  - Screening
  - Side effects
  - Interaction with other drugs/Genomics
- Toxicology
  - Acute toxicology
  - Subacute toxicology/teratology
  - Chronic toxicology mutagenicity carcinogenicity
- Pharmacokinetics
  - Absorption distribution metabolism excretion// Biomarker
- Clinical trials
- Regulatory affairs
  - Advice on regulatory strategy
  - Preclinical / Biomarker strategy
  - Orphan drug, pediatric, Regulatory agency – novel technologies

On the average, ~8-12 years to develop a drug at a cost of ~£464 million or ~$900 million

"Knowing is not enough; we must apply..." Goethe
Emergence of Regulatory Science

- Bridge the scientific, translational and clinical knowledge into regulatory development planning
  - Become the liaison between multidisciplinary groups
  - Bring clinical reasoning and scientific methodology to a process-driven field
  - Continually educate themselves on international regulations that govern scientific discovery
- Need for precision, prediction, and intelligence in adapting the Regulatory Process to product development
  - Introduce pragmatic methods to proactive approaches to complex situations
  - Incorporate analytical process to improve operational execution
- Infuse business doctrine into strategy
  - Business Development - licensing opportunities, outsourcing strategies and CRO partnerships

“Regulatory science is the science of developing new tools, standards and approaches to assess the safety, efficacy, quality and performance of regulated products…” FDA
Strategic Elements of Regulatory Contribution

- TPP / Product inception
  - Global CTA / IND filings
    - Clinical trial design & conduct
  - Fast track; Orphan drug; Opportunities for acceleration; Manufacturing strategies
- TPP-Driven Regulatory
  - Agency meetings / Consultations & scientific advice
- Special Protocol Assessment
  - Pivotal studies / Endpoint assessment; SRI
- Stakeholder Engagements
  - KOL; HTA / Experts
- Data review; Competitive Benchmarking; Product label;
  - REMS; Benefit /Risk Assessment
- Global Filing & Review of Marketing Applications e-CTD
- Competitive, Globally acceptable, Value-adding, Sustainable Product

Opportunities for Regulatory Science

REGULATORY SCIENCE DEMANDS
- Strategic Regulatory Expertise
- Competitive Regulatory Intelligence
- Therapeutic Expertise
- Regional / Local Expertise
- Operational Excellence

OPPORTUNITIES
- Redefinition of Stakeholders;
- Heightened Regulatory Approval Threshold;
- Increased Patient Access;
- Product Value Proposition / Differentiation;
- Increased Competition;
- Transformation Initiatives
- Simultaneous Global Development / Registration;
- Cost Containment Measures; Rigorous Regulatory Reviews

CHALLENGES
- Redundancy vs. Complementarities to Clinical/Medical/Scientific Affairs teams;
- Clarity of Value-added roles;
- Positioning and Development of Career Advancement vs. Traditional Model

... Differentiation is the name of the game!

Position Profile of a Regulatory Scientist

Profile
• Usually terminal clinical and/or scientific degrees (MD, PhD, PharmD, DDS) with post-doc/fellowship experience
• Active participation in professional organizations and publications
• Background in translational research/medicine
• Therapeutic Area and technical/data review Capabilities are strong assets

Responsibilities
• Combination of **strategic** and **operational** excellence
  - Program development planning
  - Strategic regulatory intelligence
  - Regulatory authority meetings
  - Clinical trial methodologies
  - Global awareness & understanding
  - Matrix-organization supervisory experience

Ukwu, H. Role of Regulatory Scientist in BioPharmaceutical Industry. RAPS Focus perspectives.. March 2003
Industry Model: Regulatory Development

Physician / Scientific
Application of Therapeutic Area expertise and
Strategic Regulatory Intelligence

Regional Strategy & Operational excellence:
Executing on Milestones
Regulatory submission
BOH correspondence

Associate/Principle/Senior Specialist / Analyst

Associate/Senior Manager / Scientist

Associate/Senior/Executive Director

Therapeutic Area Head

Associate/Senior/Executive Vice President

Associate/Senior/Executive Director

Associate/Senior Manager / Liaison

Associate/Senior Coordinator / Specialist

Associate/Senior/Executive Director

Associate/Senior/Executive Director
*Ukwu, H. (2011). Leading a CRO paradigm shift at PPD from operational to strategic regulatory science application to enhance quality of services to clients