TTIP - An Opportunity to Increase Regulatory Compatibility in the Biopharmaceutical Sector

EU-U.S. High Level Regulatory Cooperation Forum
April 10, 2013
The Innovative Pharmaceutical Industry

• The Pharmaceutical Research and Manufacturers of America and the European Federation of Pharmaceutical Industries and Associations represent the world’s leading research-based biopharmaceutical companies.

• Our shared mission is to advocate for policies that encourage the discovery of life-saving and life-enhancing new medicines for patients.
The Value of the Industry

Medicines extend life, reduce suffering, and enable patients to lead more productive and fulfilling lives.

• Over the last 25 years, prescription medicines have significantly reduced deaths from major diseases and improved quality of life.
  – New medicines helped decrease heart failure and heart attack deaths in the U.S. by 45% in just six years.
  – Improvements in cancer treatment have cut death rates in half. Research finds that medicines alone account for 50-60% of survival increases.
  – Antiretroviral medicines developed in the last 40 years have decreased HIV/AIDS mortality 77% and increased life expectancy for HIV/AIDS patients by 50 years.

• As our populations age and face increased rates of disease, medical advances will be key to alleviating suffering, improving quality of life, and increasing productivity.

Sources: PhRMA Profile (2012); PhRMA Chart Pack (2012)
The Value of the Industry (cont’d)

Medicines extend life, reduce suffering, and enable patients to lead more productive and fulfilling lives.

• The United States and the European Union represent the two largest markets for pharmaceuticals.

• In 2011, PhRMA members alone invested almost $50 billion in the United States researching and developing new medicines for patients. That same year, the innovative biopharmaceutical industry invested €27.5 billion in R&D in Europe.

• Total industry employment in the United States in 2009 totaled approximately 4 million jobs, including direct employment of over 674,000 Americans. In 2011, those employees supported approximately $48 billion in U.S. exports, making the biopharmaceutical industry the fourth largest R&D intensive U.S. exporting industry.

• Similarly, EFPIA members directly employ 640,000 people. 25% of these jobs are in R&D areas. The EU pharmaceutical industry is one of few sectors to contribute positively to the EU’s trade balance. Its trade surplus of €48.3 billion in 2011 was the highest among high-tech industries.

Developing a new medicine is lengthy, risky, and costly. New drug development takes an average of 10–15 years, and costs approx. US $1.3 billion.
The Innovative Pharmaceutical Industry Strongly Supports the Proposed Partnership

• EFPIA and PhRMA support an ambitious and comprehensive agreement that addresses regulatory compatibility initiatives, intellectual property protections, market access and investment provisions, customs and trade facilitation, and public procurement measures.

• Economic analyses estimate that a significant portion of the benefits from a potential agreement will come from tackling non-tariff barriers. As much as 80% of the total potential gains will come from cutting costs imposed by unnecessary bureaucracy and regulations, as well as from liberalizing trade in services and public procurement.

• To this end, we have sought to identify regulatory compatibility proposals that:
  – address regulatory differences and duplicative requirements that can impede efficiency in global drug development, review and evaluation;
  – reduce redundant testing;
  – optimize deployment of limited regulatory agency resources; and
  – expedite patient access to new, innovative medicines.

Source: CEPR, 2013 “Reducing Transatlantic Barriers to Trade and Investment – An Economic Analysis”.

The Benefits of Greater Regulatory Compatibility

- Harmonization of regulations facilitates investment in R&D of new, innovative medicines targeting unmet patient needs around the globe.

- Regulatory guidance produced under the auspices of the Int’l Conference on Harmonization (ICH) has introduced efficiencies in drug development and extended benefits to patients:
  - Harmonized and rationalized preclinical animal studies, eliminating a considerable amount of duplicative work;
  - Standardized safety update reporting during the drug development process, enabling more timely patient safety information which focuses on findings of greatest concern to investigators and patients;
  - Established common structure/design for reporting of safety and efficacy data, helping to reduce excessive documentation; and
  - Consolidated reporting of new candidate drug/biologic submission material via the electronic common technical document.

- These successes motivate industry to continue to pursue regulatory harmonization and compatibility to foster continued innovation to address unmet patient needs.

- It is vital that all stakeholders – regulators, industry, policymakers and healthcare providers – collaborate to achieve streamlined regulatory processes which preserve patient protections while eliminating inefficiencies/redundancies, in order that new cures move more rapidly from test-tubes to trial testing to therapeutic use.
Mutual Recognition of Inspection Findings

• With global regulatory standards converging, it makes sense for regulators to undergo common training and to trust and trade information garnered from clinical and manufacturing audits.

• The FDA and EMA have taken steps to coordinate inspections assessing compliance with Good Manufacturing Practices (GMP) and Good Clinical Practices (GCP).

• The EU and U.S. should go a step further by mutually recognizing each other’s GMP and GCP inspections.

• This recognition could encompass inspections by European inspectors within the European Economic Area (EEA), FDA inspections of U.S. sites, and inspections that both European and U.S. inspectors conduct outside the U.S. and EEA.

• In line with the EU’s longstanding approach of conducting risk-based inspections, the regulatory authorities could work together to identify systematically high-risk sites and coordinate inspection schedules.
Parallel Scientific Advice

- Parallel scientific advice offers the opportunity for sponsors pursuing applications in both regions to conduct the necessary clinical trials based on a common approach and for the learnings from those studies to be shared with both regulators.

- Although the FDA and EMA have established a program to provide parallel scientific advice, the sponsor has no right to such advice and may obtain it only in limited circumstances.

- This program could be better utilized by amending the current policies to:
  1. Expand its applicability to all medicines; and
  2. Grant sponsors the right to receive parallel scientific advice upon request.
Regulatory Convergence on QbD Applications

• The FDA and EMA are piloting an effort to conduct parallel assessment of “Quality by Design” (QbD) applications that is intended to allow for the parallel evaluation of relevant development and manufacturing quality components that are submitted to both agencies.

• The program should be formally adopted by the two agencies, with the following expected benefits:
  • Better deployment of resources;
  • Improved implementation of post-approval changes; and
  • Elimination of country/region specificities for release.

• Overall benefits:
  • Reduced complexities in the supply chain, prevention of drug shortages; and
  • Improved patient access to medicines.
Increased Collaboration
Under the Auspices of the ICH

• Work together to achieve greater regulatory compatibility in the scope, content and timing of submission of pediatric plans, so that companies are required to prepare only a single plan for submission in both territories, and thereby drive greater research efficiencies and speed the completion of pediatric trials.

• Add a pharmacovigilance cluster to conduct work on such topics as post-market testing and risk management requirements and format and deadlines for adverse event reporting.

• Address duplicative clinical testing requirements via revision of ICH guidance E5 (design of multiregional trials).

• Establish a common framework and methodology benefit-risk assessment, while retaining authority to make different risk-benefit judgments under their individual approval schemes.
Additional Proposals for Collaborative Processes

• To establish a harmonized list of clinical trial result data fields, and agree on which of these data fields may be disclosed to the public.

• Development of therapeutic area guidelines, beginning with the establishment of a procedure addressing scientific and regulatory guidelines for specific treatment areas.

• The EU and U.S. should work together to ensure national/regional coding systems are based on common standards for the use of unique identifiers, developed using non-proprietary, harmonized international standards.
Conclusion

• The TTIP offers an opportunity for us to expand and extend well-grounded and rational ICH and bilateral initiatives beyond their current scope.

• Together we can:
  – Refocus/re-invigorate - build on prior successes and expand on the solid infrastructure of the ICH process;
  – Recalibrate - address current divergence in practices and upgrade current practices;
  – Re-energize our two regions engagement in harmonization efforts; and
  – Restore new hope for patients – by breaking new ground (new initiatives in benefit-risk, pediatrics, etc.).
Thank you!

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