

# FDA Public Hearing on Electronic Submissions and Public-Private Partnerships

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Pharmaceutical Research and Manufacturers of  
America (PhRMA):

e-Submissions – Ed Tripp, Abbott

Public-Private Partnerships – Bill Rosen, Pfizer

# PhRMA

- PhRMA represents companies who spent \$39.4 billion on pharmaceutical research in 2005
- Long-standing advocates of automation
  - CANDA applications in mid-1990s
  - Major partners with FDA at ICH and with eCTD
  - Active participants in HL7 eHealth standards organization
  - Committed to improving patient healthcare through automation and information access

# PhRMA Positions

- PhRMA fully endorses a planned move to an all-electronic submissions environment
- PhRMA is an engaged advocate of Public-Private Partnerships
- Our experience reinforces the need for a well-managed development and implementation process for standards

# Top Goals on Electronic Submissions

- Implement a common, electronic-only, end-to-end regulatory environment
- Improve assessment of products' risk/benefit profile by enhancing the collection and analysis of safety information
- Establish a rolling 5-year standards and information technology plan
- Harmonize standards across FDA Centers, ICH regions, and across Healthcare domains

# How would an all-electronic submission environment benefit you?

- An all-electronic environment requires standards to be effective
- Benefit of Harmonized Standards Implementation
  - Reduces multiple formats; creates efficiencies
  - Facilitates information exchange among business partners
  - Improves information access, query, and retrieval
  - Allows focus on content (science) not format
- Benefit of Standards to Public Health
  - Helps speed new products to patients
  - Facilitates data aggregation for risk / benefit analysis
  - Lays foundation for global electronic health records

# Standards Improve Transparency and Consistency

- Interoperability and portability of standards is imperative
  - Between systems operated by various stakeholders
  - Throughout the end-to-end process of information creation, analysis and exchange
- Stakeholders include
  - Regulatory agencies / health authorities
  - Clinical researchers and healthcare payers, providers & patients
  - Other governmental agencies with healthcare related missions (e.g. VA, CDC, DOD)
  - Biopharmaceutical industry
  - Third party providers (e.g., CROs)

# **Would an all-electronic submission environment change your ability to initiate in a timely manner the studies supporting your regulatory submission?**

- An improvement is possible only if the electronic submission format is designed to support a more efficient clinical trial startup business process
  - Need to consider the impact on upstream (sponsor) and downstream (health authority) processes
  - Implies we do not simply automate the legacy processes
- More important question: Can a sponsor improve the conduct and analysis of a clinical trial with an all-electronic submission environment? – YES!

# What are the major impediments to an all-electronic submission environment?

- Having a clearly stated, compelling business case for e-Submissions encourages adoption
- Standards churn
  - There is a critical need for harmonization of standards and their planned implementation
    - “The nice thing about standards is that there are so many to choose from” - Andrew Tanenbaum
    - Different groups are driving related but different standards
  - Creates a lack of clarity
  - There is a need for a standards lifecycle
    - Retire old standards when new standards are mature
    - Notice to withdraw eNDA / eBLA guidance is positive step
  - A lack of specificity (too many options how to submit)
    - Consistent method of migration and conversion is needed

# Are there enough entities available to provide such services or tools in support of this effort?

- The recent standards implementation efforts do not encourage early adoption
  - Scenario-based testing (piloting or prototyping) is needed and would increase confidence
  - Often the initial focus is on “safer” conversion/migration services
  - A robust marketplace is needed that encourages vendors to create new tools that address both Agency and Industry needs
- The marketplace cannot keep pace with recent and more complex standards
  - Release of standards prematurely causes frequent software re-coding
  - Vendors are reluctant to offer products quickly until the specifications mature to minimize costly changes

# What additional costs are associated with implementing a particular format or standard for an electronic pre-market submission?

- Moving from eNDA to eCTD to RPS results in substantial costs
- Preparation of an electronic submission is more complex than preparing a paper submission *but* it is a richer and more capable product
- Need to limit the amount of information resubmitted in different formats (e.g. link existing INDs to an NDA without resubmission; multiple label formats)
- Automating an antiquated paper process in an electronic environment based on new standards is not effective and can create added burden
- Must be sure to add value when adding costs – value for all parties

# Are there parts of a product application that are more costly to convert to an electronic format than others?

- It's not just about replacing paper!
- Source data formats are more costly to change
  - Cascades throughout sponsor's internal systems and processes and impacts vendors
    - Clinical data, Pre-clinical data, manufacturing floor
    - Image formats (MRIs, ECGs, X-rays, scans, etc)
  - Benefits realized only after systemic industry change that considers technology and business process, and it engages all stakeholders

# How much time would you need to make a smooth transition to a new electronic system?

- Requires sufficient time to develop, test, pilot and implement new standards and processes
  - Start with a long-term plan
  - Freeze standards for a period of time to stabilize systems and processes (difficult to hit a moving target)
  - Minimize dependencies with other systems for data collection
  - Prepare a robust implementation guide
- Reacting to meet incomplete standards is problematic

# Goals for Electronic Information Exchange

- Advance the concept of shared services through third party entities by establishing a public-private partnership for a broad stakeholder healthcare community - CRIX
- Assure the broad stakeholder community is involved in the establishment and governance of the shared services environment
- Demonstrate the value of a Public-Private Partnership (PPP) by establishing an initial application for speeding clinical study start up by providing a common area for investigator registration - FIREBIRD

# On Public-Private Partnerships

- The concept as we understand it:
- A secure, sustainable infrastructure providing services across the biopharmaceutical and eHealthcare community
  - Facilitates electronic submissions
  - Promotes information exchange and sharing amongst Sponsors, Regulatory Authorities, the healthcare community and vendors
  - Provides an opportunity to optimize business processes for all participating parties
- Must assure security and intellectual property protection

# **What are your general viewpoints on a third party entity or entities providing services related to such an electronic platform?**

- PhRMA supports the creation of a PPP for a shared technology infrastructure for information exchange
- PhRMA supports a not-for-profit consortium to implement and manage the infrastructure
- Implementation must be affordable and allow for broad participation
- Must allow for global participation
- Prefer a pragmatic approach to implementation
- Health Authorities must be engaged, provide oversight, and help identify priorities
- Smaller companies and smaller countries can leverage a shared infrastructure

# Benefits

Value gained from a PPP:

- Improves workflow efficiencies, lowers process & resource costs for all participants
- Reduced infrastructure expenses
- Faster submission notification and delivery
- Automates high-volume administrative submission transactions
- Enables business continuity in a disaster

# It's All About Information

- Sharing information seamlessly, in a controlled, secure manner is the goal
  - Enhances visibility to all concerned: patient, patient advocate, health worker, authority, sponsor, CROs, allied health providers
  - Allows integration with national, regional and global e-Health records
  - Health information is our most critical, unmanaged data type today

# Potential Barriers

- Engagement across a broad spectrum of users
  - A “critical mass” of partners is required to move the reluctant from observer to active participant
  - Requires members of all sectors to participate
- Need to understand the full range of potential partners within the healthcare community.
  - Limited or narrowly focused initiatives may have a limited lifespan
- Requires a compelling business case

# Conclusions

- Electronic information exchange is the future
  - A long-range plan is required to establish an end-to-end electronic environment
  - Standards must be driven by business process improvements
  - ERS recognizes the need to address regionally specific regulatory requirements, but every effort should be made to establish globally harmonized standards
- Public-Private Partnerships
  - ERS has been engaged in an effort to develop and implement a PPP for the past 6 years
  - A PPP brings an improved business model to the healthcare community

**Thank You!**