



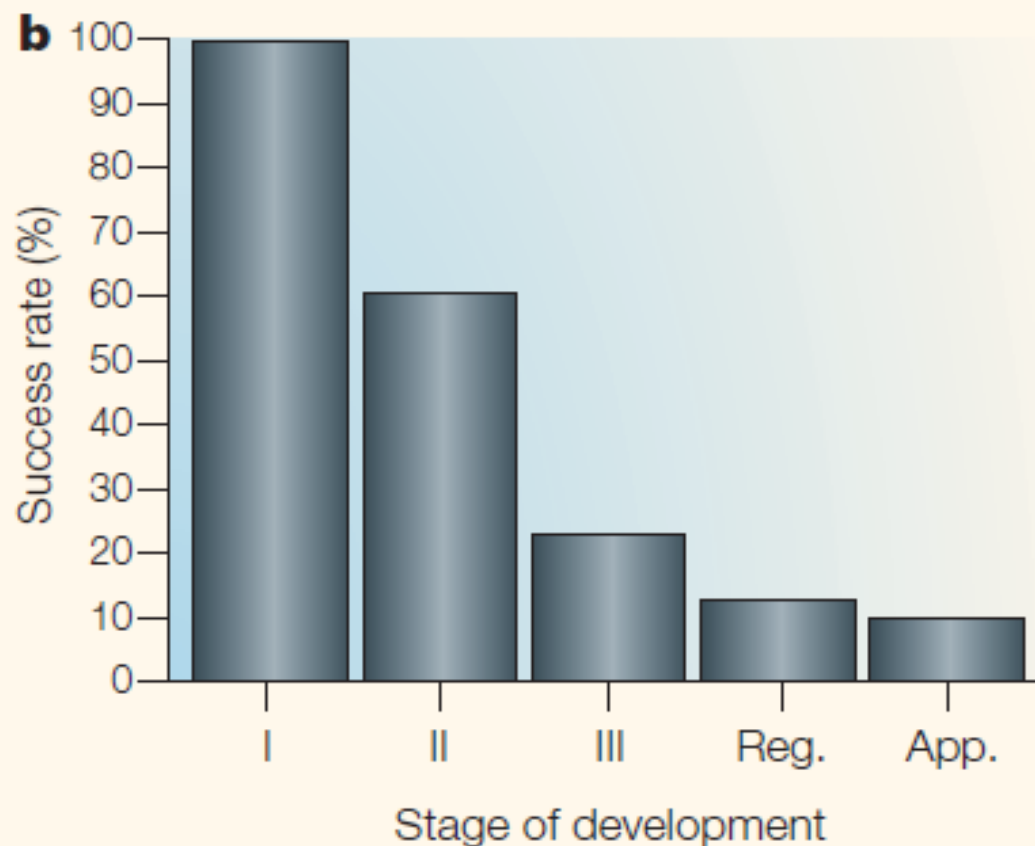
FDASIA and Standardized Electronic Data: Where Are We?

FDA/PhUSE Computational Science Symposium

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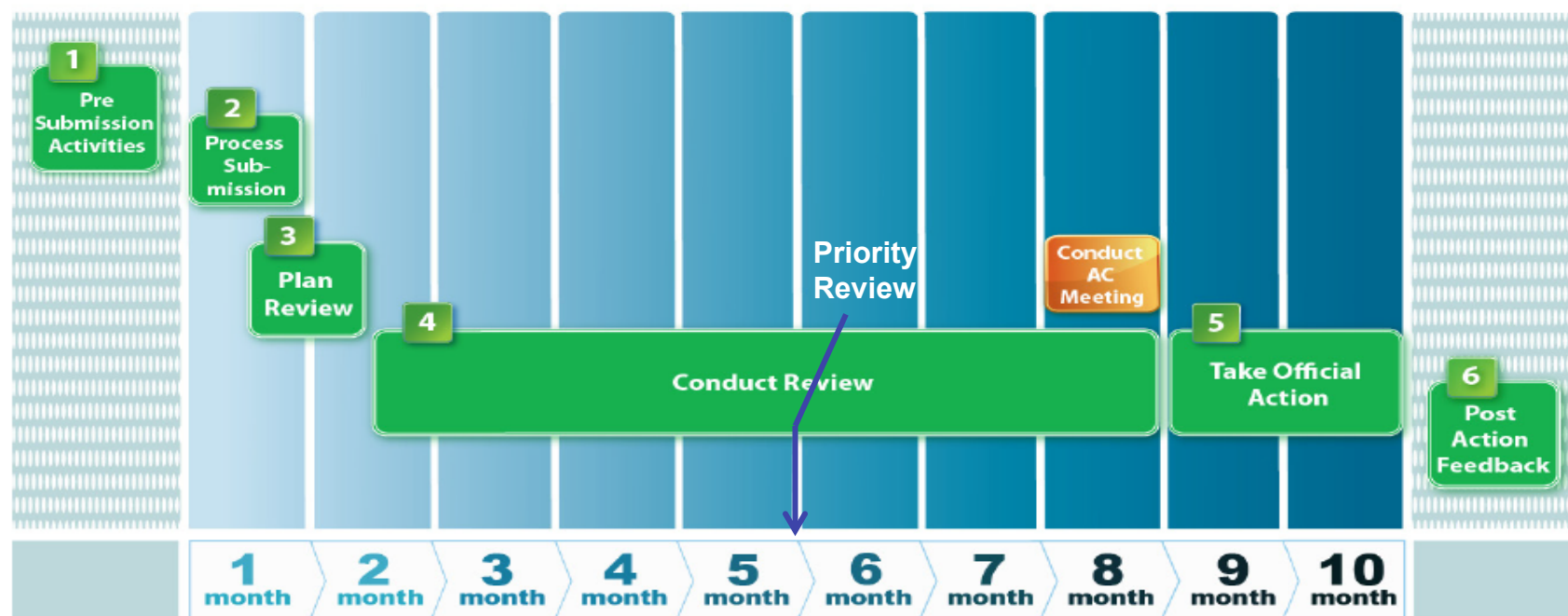
Clinical Trials Suffer High Failure Rates – Costly in Time and \$\$



Rate of Attrition for Drugs Entering Each Phase

Kola I, and Landis J. Can the pharmaceutical industry reduce attrition rates? *Nature Rev. Drug Discov.* 3:711-715, 2004.

Efficient, Consistent Review Is Challenging



New Drug Application and Biologic License Application Reviews
(NDA/BLA Review Process)

Version 2011.01.04

*In fiscal 2012, CDER received about 662 study datasets per week, up to 10GB in size
43% of effort in a review is spent on data management and primary analysis*

How Do We Improve This?

- Data Standards
 - Prioritize development based on key elements to support reviewer decision-making
- Communication
 - Collaborate & with other government agencies, industry, other stakeholders and SDOs
- Policy
 - Clarify FDA requirements and set expectations
- Business process
 - Incorporate best-practice use of new tools and methods
- Technical architecture
 - Must be able to efficiently support e-review
- Resources
 - Sustained support and management attention

FDA Safety and Innovation Act (FDASIA)

- Reauthorizes the fifth instance of Prescription Drug User Fee Act (PDUFA V)
- Authorizes the new Generic Drug User Fee Act and Biosimilars User Fee Act
- FDASIA Section 1136:
 - Allows FDA to require standardized fully electronic submissions related to marketing applications no less than 24 months after publishing final guidance
- Phased-in through guidance to industry

Data Standards Action Plan (CDER's Standards Program Portfolio) – Ready To Publish

Initiatives align with the Center's data standards strategic goals:

- **Policy and Process** – Key activities to establish essential standards-related policy or process (e.g., standards development & adoption, guidance development and schedule, specific guidance)
- **Standards Development and Implementation** – Projects to develop, test, and implement a standard to meet a regulatory need
- **Study Data Standards** – Projects that develop, test, or implement advancements in study data terminology and content standards
- **Research and Development** – Projects to assess a potential approach to meet a standards-related need without immediate intent to implement; these are to inform future direction

Efforts Are ‘Risk Focused’

The standards activities and projects are defined with the major program risks in mind. This risk focus includes:

- Strong, consistent communications with stakeholders (e.g., SDOs, Industry, Academia, other government agencies, medical professional societies)
- Transparency through solicitation and receipt of public input
- Predictability through use of consistent process
- Assuring use-ability for regulatory review

A Sample of Relevant Efforts

- Published Communications Plan, Data Standards Strategy, and soon the Action Plan
- Executing requirements gathering and modeling for FDA “core” needs plus TAs
- Developing standards testing/readiness approach
- Conducting forward-looking activities (e.g., requirements and alternatives for data exchange needs, sustainability over time)

PDUFA Sec XII – Some Key Guidance-Related Activities

(electronic submissions)

- Issue guidance for standardized electronic submissions (i.e. eCTD); draft by end of year, final within 12 months of receiving comments
-- PUBLISHED
- Guidance will ultimately apply to all NDA, BLA, ANDA and commercial IND submissions (except for those described in section 561 of the FD&C Act)

(clinical/nonclinical study data)

- Develop plan for standardizing study data for Therapeutic Areas
 - **DRAFT, anticipated publication by June 30, 2013**
- Develop standardized clinical data terminology through open, consensus-based standards development organizations
 - **UNDERWAY**
- Periodically issue final guidance specifying the completed clinical data standards for use in sponsor application submissions
 - **“Parent” Guidance in DRAFT**

Study Data Standards

Number	TA	Roadmap Grouping ¹	Commenced	Start Date	Added to List ²	Removed from List ²	Tier ³	Stage ⁴	Lead Group ⁵	Notes
1	Cardiovascular (CV)	1	Y				Tier 1	Development	CDISC	
2	CV Imaging	1	Y		2/1/12			Development	Duke University	
3	Pain	1	Y				Tier 1	Public Release, v1.0 Provisional	CDISC	
4	Parkinson's	1	Y				Tier 1	Public Release, v1.0 Provisional	CDISC	
5	Polycystic Kidney Disease	1	Y				Tier 1	Public Release	CDISC	
6	Schizophrenia	1	Y				Tier 1	Development	Duke University	
7	Tuberculosis	1	Y				Tier 1	Public Release, v1.0 Provisional	Critical Path Institute	
8	Virology	1	Y		2/1/12			Public Release, v1.0 Provisional	CDISC	
9	Anti-diabetic agents	2					Tier 1	Initiation	FDA/CFAST	Not yet started
10	Gastroesophageal Reflux Disease	2					Tier 2	Initiation	FDA	Not yet started
11	Major Depressive Disorder	2	Y	9/15/12			Tier 2	Development	Duke University	
14	Acne	3	Y	10/1/12			Tier 1	FDA Requirements	FDA	
29	Lipid-altering Drug Groups	6	N	January			Tier 2	FDA Requirements	FDA	
32	Asthma	7	Y				Tier 2	Development	CFAST	
42	Treatment of Hepatitis C	9	Y	1/10/13			Tier 1	FDA Requirements	FDA	
45	Treatment of Overactive Bladder	10	Y	11/1/12			Tier 2	FDA Requirements	FDA	
	Alzheimer's		Y				Tier 1	Public Release, v1.0	CDISC	

Links & Contacts

Data Standards Program Website:

<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/FormsSubmissionRequirements/ElectronicSubmissions/ucm249979.htm>

Study Data Standards Resources and Data Standards Catalog:

<http://www.fda.gov/ForIndustry/DataStandards/StudyDataStandards/default.htm>

Common Issues Document:

<http://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/FormsSubmissionRequirements/ElectronicSubmissions/UCM254113.pdf>

Questions: CDER-edata@fda.hhs.gov