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The Food and Drug Administration Amendments Act of 2007 and Their Impact on Transparency of Pharmaceutical Research

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FDAAA Contains Some Sweeping New Authorities

- Reauthorization of PDUFA
- A New DTC User Fee Program to Provide FDA with Additional Resources (**on hold**)
- Drug Safety
- Enhanced Provisions on Clinical Trial Registration
- New Provisions for Clinical Trial Disclosure

Key Issues on CT Registration and Disclosure

- All Trials Except Phase One now must be Registered
- Increased Amount of Descriptive Information now Required
- Results Databank Established at NLM
- Certification to FDA Requirement Established

What does all this mean for PhRMA Companies?

- It's useful to step back and look what the industry has done over the past several years.
- It's important to keep in mind what the public's needs are regarding CT transparency.

WHAT THE PUBLIC WANTS (and maybe expects)

- All Drugs work All the Time for All People
- All Drugs are Safe All the Time for All People
- All Drugs are Cheap All the Time for All People

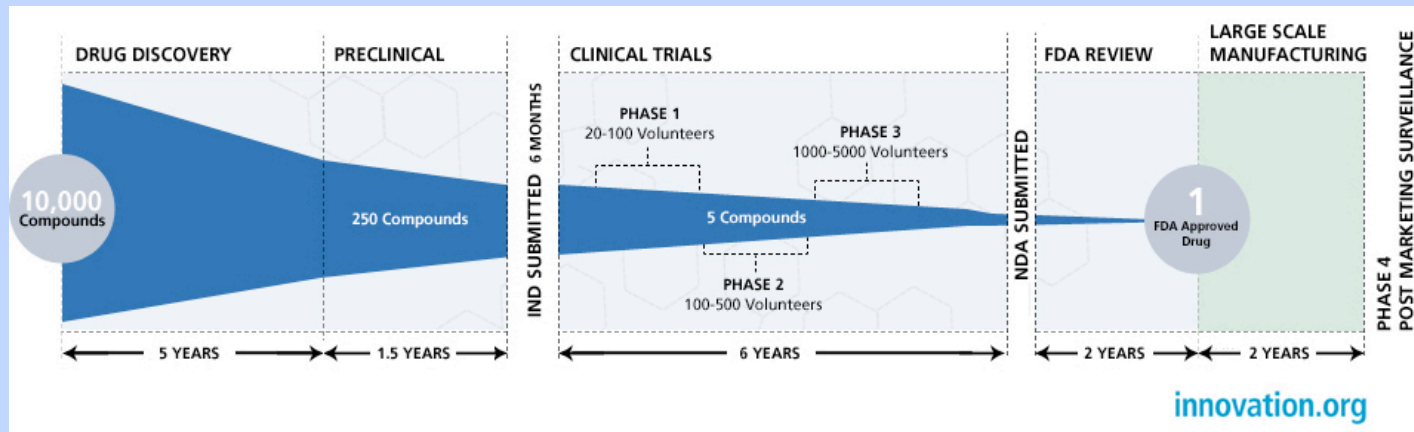
The Reality is that none of the above three are true.

Here is the context that PhRMA Companies work within

- Cost – Current Estimate to Bring a New Molecule to Market is Approximately \$800 Million
- Time – Because of Scientific Uncertainties and Regulatory Compliance it takes 12-14 years from Discovery to Licensure

Despite this effort, we don't fully know the drug's safety profile (or for that matter the full efficacy)

Drug Development Time and Attrition



Drug Development does not take Place in a Vacuum

- Significant Interaction with FDA to assure that CT protocols will yield useful data
- Multi-center CT reduces investigator bias
- CT compliance with GCP ethical standards
- Auditing CT sites to assure quality of data
- NDA review of safety/efficacy data by FDA
- Post-market commitments as appropriate

IS THE UNIVERSAL BLACK BOX WARNING THE SOLUTION????

This drug poses both known and unknown risks. Some of these risks may be severe and even result in death. The known risks are described in this product label. The unknown risks are still unknown.

Two Aspects of Clinical Trial Disclosure

- Registration of Clinical Trials at Their Inception – Provides patients and healthcare providers with information on participation in clinical trials and, given a unique ID number can link to the results of the trial when completed
- Disclosure of Clinical Trial Results – Provides healthcare providers (and patients) information on all clinical trials conducted by the sponsor regardless of outcome (and whether the information is on the FDA-approved drug label)

Past PhRMA Initiatives

- Revised PhRMA Clinical Trial Principles (June 2004)
- PhRMA Clinical Study Results Database (October 2004)
- PhRMA Clinical Trial Registry Policy (January 2005)
- Joint Industry Position on Disclosure of Clinical Trial Information (January 2005)

A Brief History . . .

- “PhRMA Principles on Conduct of Clinical Trials and Communication of Clinical Trial Results” adopted in October 2002
- PhRMA Principles express strong commitment to transparency of clinical trial results – but that there might be a need to protect proprietary information in early stage drug development

PhRMA Principles - Disclosure

“We commit to timely communication of meaningful results of controlled clinical trials of marketed products or investigational products that are approved for marketing, regardless of outcome.”

PhRMA Principles - Communication

- Peer-Reviewed Journal Article
- Presentation at Scientific Meeting (poster or oral presentation)
- “. . . making results public by some other means.”

Criticisms of Industry Position

- Negative studies are not published in peer-reviewed journals
- Even though negative studies are presented at medical meetings, they are available only to the meeting participants

Clinical Study Results Database

- Natural Outgrowth of PhRMA Principles
- Central, easily accessible repository for meeting commitment to communicate clinical trial results
- Broader dissemination than, e.g., presentations at medical meetings
- www.clinicalstudyresults.org (as of 1/9/2008 there is information on 497 drugs)

Clinical Study Results Database

Applicability

- Hypothesis-testing trials
- For marketed drug products
- Studies completed after October 2002

Regardless of outcome

- Posting commitment applies to studies regardless of whether the results are positive, negative or inconclusive

Clinical Study Results Database

Contents:

- Bibliography of published studies
- Summary of unpublished studies (ICH E3)
- Approved Labeling

Timing:

- Studies should be posted within one year of completion
- Extension available if publication is sought

NIH Databank Issues

- Won't be fully operable because of necessary rulemaking
- Initial issues with NIH draft guidance and statutory authority
- Will information in the databank be any more useful than that on the drug label?
- Complicated extension request policy & question of timing for company to post results at the end of the trial
- Will information on unapproved products be required?

FDA Certification Requirements

- NDA submissions require that all databank requirements have been met...However, do other submissions to the agency require certification as well???
- Will FDA issue clarifying guidance?

Will These New Requirements Add Clarity or Confusion?

- Purpose of the Registry was to inform patients and healthcare providers of clinical trial opportunities
- Purpose of the PhRMA clinical studies result database was to increase transparency but continue to focus on the drug label as the primary source of information