Pharmacogenetic Testing: What's Different, What's Not ?

An International Perspective on Pharmacogenetics: The Intersections between Innovation, Regulation, and Health Delivery

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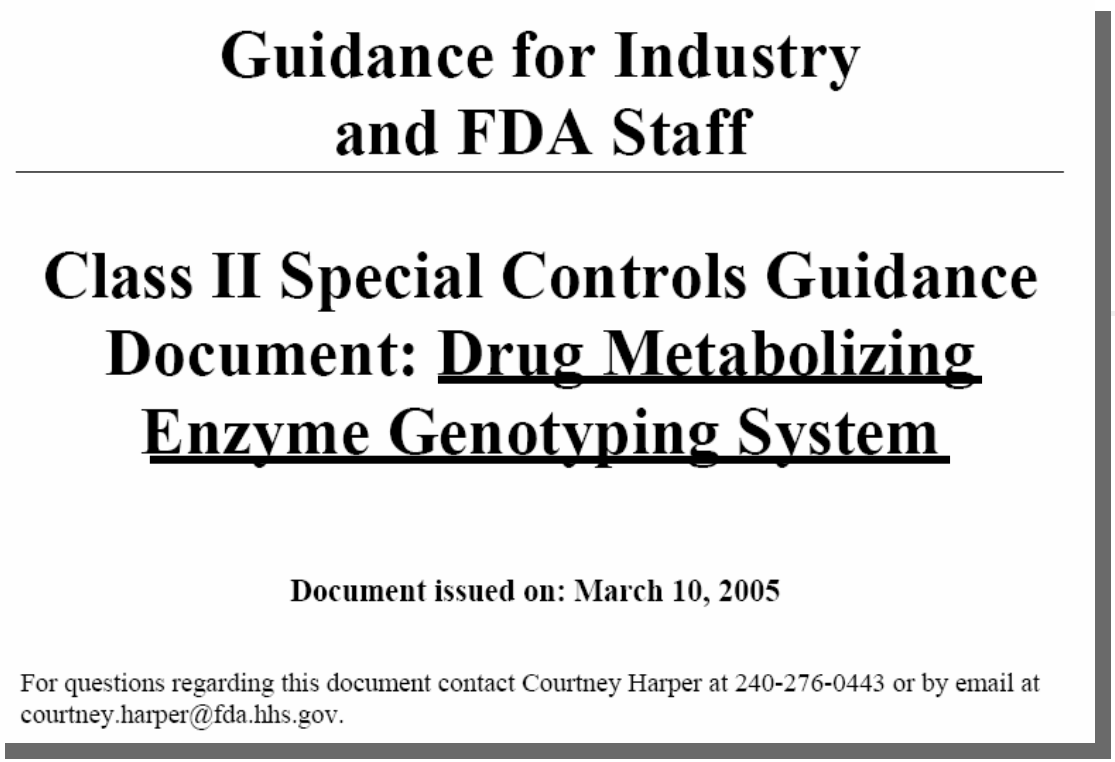
What's Different, What's Not ?

- Genetic vs. Pharmacogenetic testing
- Free-standing test vs. drug-test co-development
- Investigational test vs. marketed test
- Prospective vs. retrospective use of data (clinical utility)



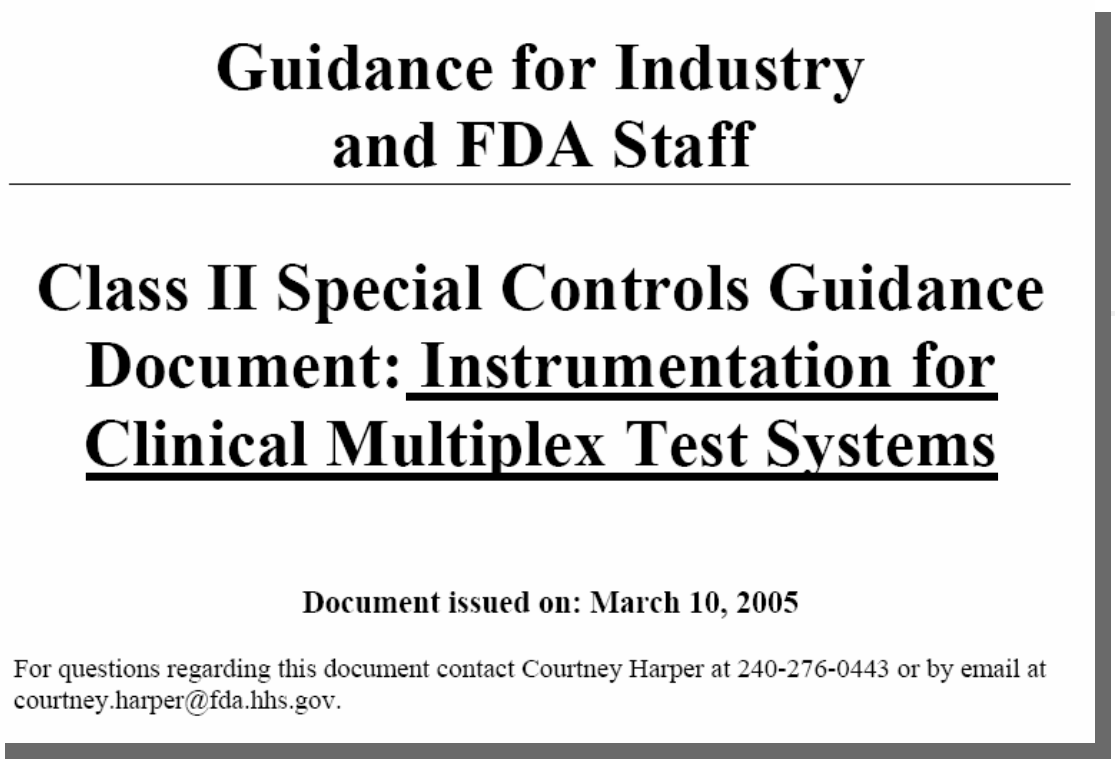
Guidance for Industry and FDA Staff

Class II Special Controls Guidance Document: Drug Metabolizing Enzyme Genotyping System

Document issued on: March 10, 2005

For questions regarding this document contact Courtney Harper at 240-276-0443 or by email at courtney.harper@fda.hhs.gov.

A drug metabolizing enzyme genotyping system is a device intended for use in testing DNA to identify the presence or absence of human genotypic markers encoding a drug metabolizing enzyme. This device is used as an aid in determining treatment choice and individualizing treatment dose for therapeutics […].



Guidance for Industry and FDA Staff

Class II Special Controls Guidance Document: Instrumentation for Clinical Multiplex Test Systems

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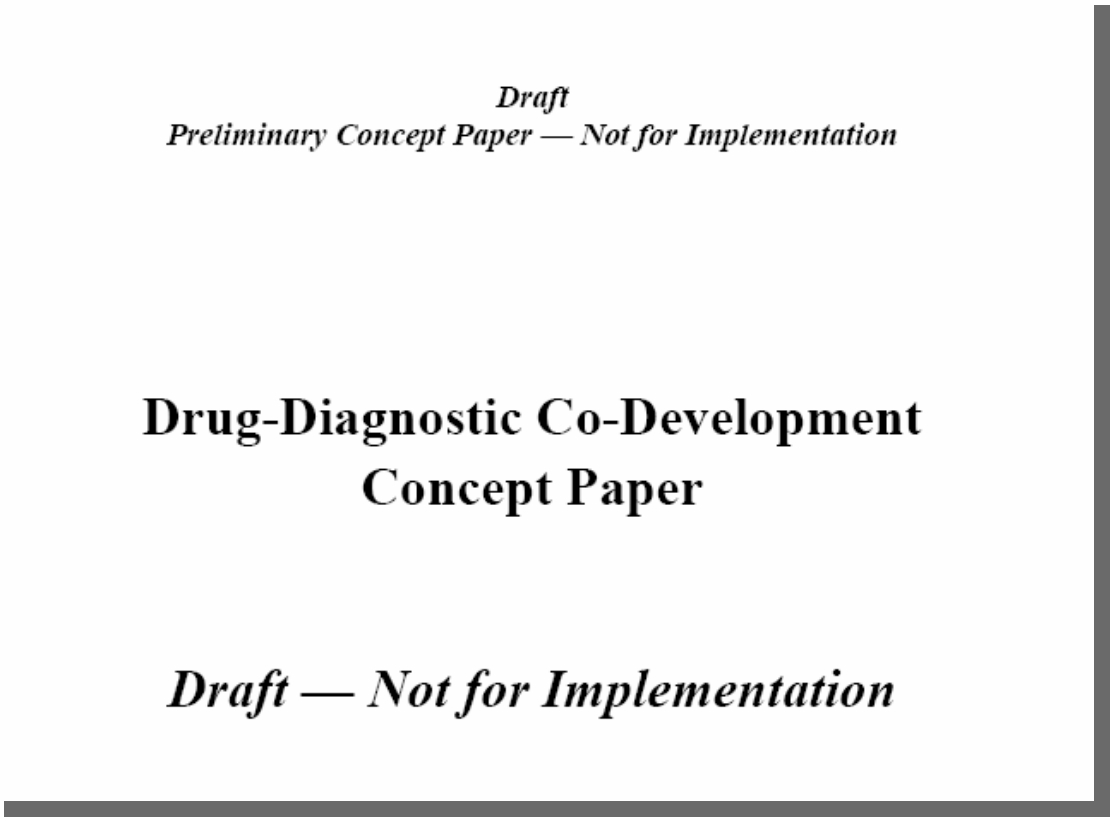
Instrumentation for clinical multiplex test systems is a device intended to measure and sort multiple signals generated by an assay from a clinical sample. This instrumentation is used with a specific assay to measure multiple similar analytes that establish a single indicator to aid in diagnosis.

Putting it Together: Drug-Test Co-Development



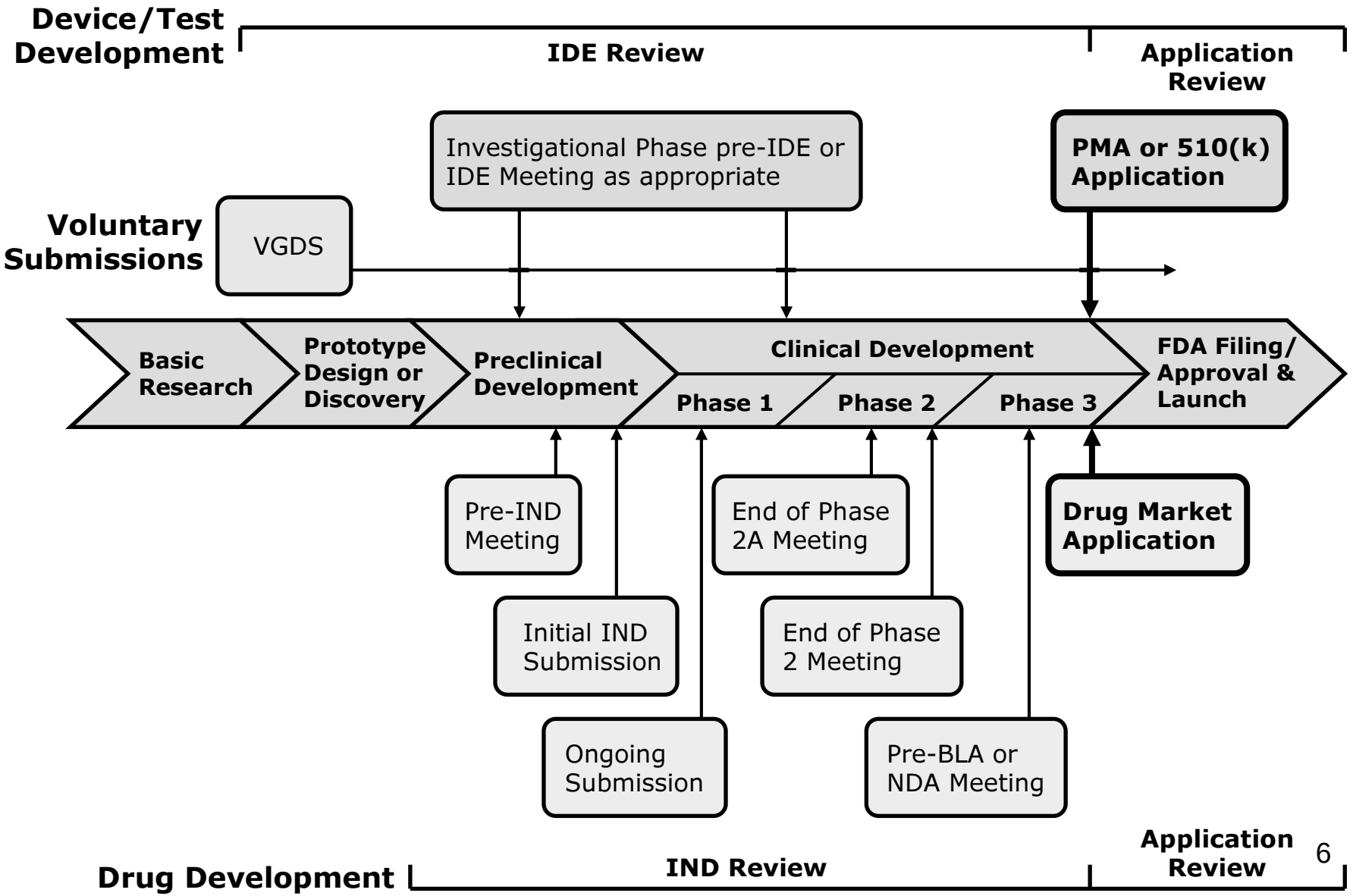
Draft
Preliminary Concept Paper — Not for Implementation

Drug-Diagnostic Co-Development Concept Paper

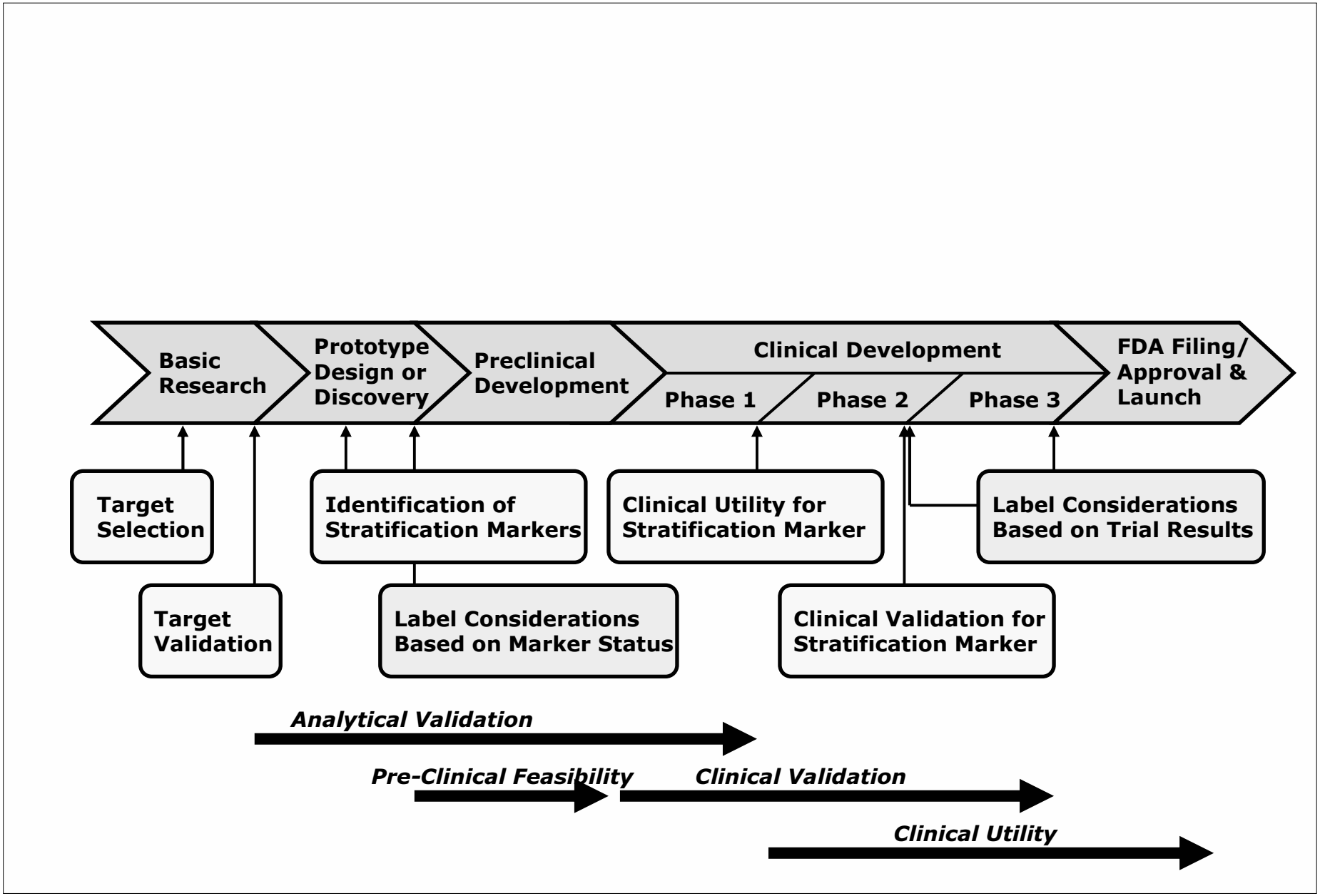
Draft — Not for Implementation

April 8, 2005

Drug-Test Co-Development Process:



Drug-Test Co-Development Process:



Drug-Test Co-Development

- Clinical phase of drug development program to provide the evidence of clinical utility (i.e., value) of the diagnostic test
- Claim for test would be for use with drug
- Drug cross-labeled for use with diagnostic
- However, other parts of drug and diagnostic development programs (e.g., analytical validation) would proceed as usual

Drug-Test Co-developed Products: Benefits

- Co-development of drug/test combination products
  - Patient stratification (safety/efficacy)
  - Enrichment in clinical trials (efficacy)
- Product label and/or marketing
  - Should a patient be treated (safety/efficacy)?
  - What is the best dose (efficacy)?
- Can be critical for bringing product to market
- Can save drugs from withdrawal
- Can rescue candidate drugs

Drug-Test Co-developed Products: Issues

- Strategy (use during drug development only)
- Competitive advantage (i.e. ID responders)
- Timing (development, approval)
- Cost (development, reimbursement)
- Availability of alternative therapy (what if none?)
- Platform (platform change)
- Complexity (point-of-care vs. service laboratories)
- *Clinical usefulness* (i.e. therapeutic area, marketability)

Genomics at FDA
Regulatory Information

Guidances

- Guidance for Industry: Pharmacogenomic Data Submissions
- Guidance for Industry: Formal Meetings With Sponsors and Applicants for PDUFA Products
- Class II Special Controls Guidance Document: Instrumentation for Clinical Multiple Text Systems
- Class II Special Controls Guidance Document: Drug Metabolizing Enzyme Genotyping System

Concept Papers

- Drug-Diagnostic Co-Development — Preliminary Draft Concept Paper (4/8/2005)
- Drug Interaction Studies — Study Design, Data Analysis, and Implications for Dosing and Labeling: Preliminary Concert Paper

Manual of Policy and Procedures (MaPP)

- Management of the Interdisciplinary Pharmacogenomics Review Group (IPRG) MaPP 4180.2
- Processing and Reviewing Voluntary Genomic Data Submissions (VGDSs) MaPP 4180.3

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www.fda.gov/cder/genomics

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Discussion Points

- What may influence the pace at which pharmacogenetic tests are integrated into clinical practice?
- What is needed to turn hypothesis into valid data that can verify the clinical validity of pharmacogenetic tests?
- If studies indicate that pharmacogenetic testing should become standard practice, is widespread testing feasible?
- What are the incentives for the co-development of new drugs and pharmacogenetic tests?

Discussion Points, cont'd

- What are the incentives for retrospective pharmacogenetic test development?
- What are the needs for international standards and how can these be addressed?
- What incentives exist for disclosure of information and for data sharing?
- Are current regulatory frameworks across OECD countries adequate to deal with co-development?
- What are the respective roles and responsibilities of public and private sector in supporting the establishment of common standards for pharmacogenetic testing and related platforms?