



FDA Driving Biomedical Product Innovation

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FDA Mission

- FDA is charged with protecting the public health by ensuring the safety, effectiveness, and security of human and veterinary drugs, biological products, and medical devices; ensuring the safety of foods, cosmetics, and radiation-emitting products; and regulating tobacco products.
- Specifically, FDA is responsible for advancing the public health by
 - Helping to speed innovations that make foods safer and make medicines and devices safer and more effective;
 - Ensuring the public has accurate, science-based information they need to use medicines, devices, and foods to improve their health;
 - Regulating the manufacture, marketing, and distribution of tobacco products and reducing tobacco use by minors; and,
 - Addressing the Nation's counterterrorism capability and ensuring the security of the supply of foods and medical products.

Innovation in medicine has depended upon a thriving ecosystem and partnerships





REPORT TO THE PRESIDENT ON
PROPELLING INNOVATION IN
DRUG DISCOVERY, DEVELOPMENT,
AND EVALUATION

Executive Office of the President
President's Council of Advisors on
Science and Technology

SEPTEMBER 2012



Innovation Ecosystem

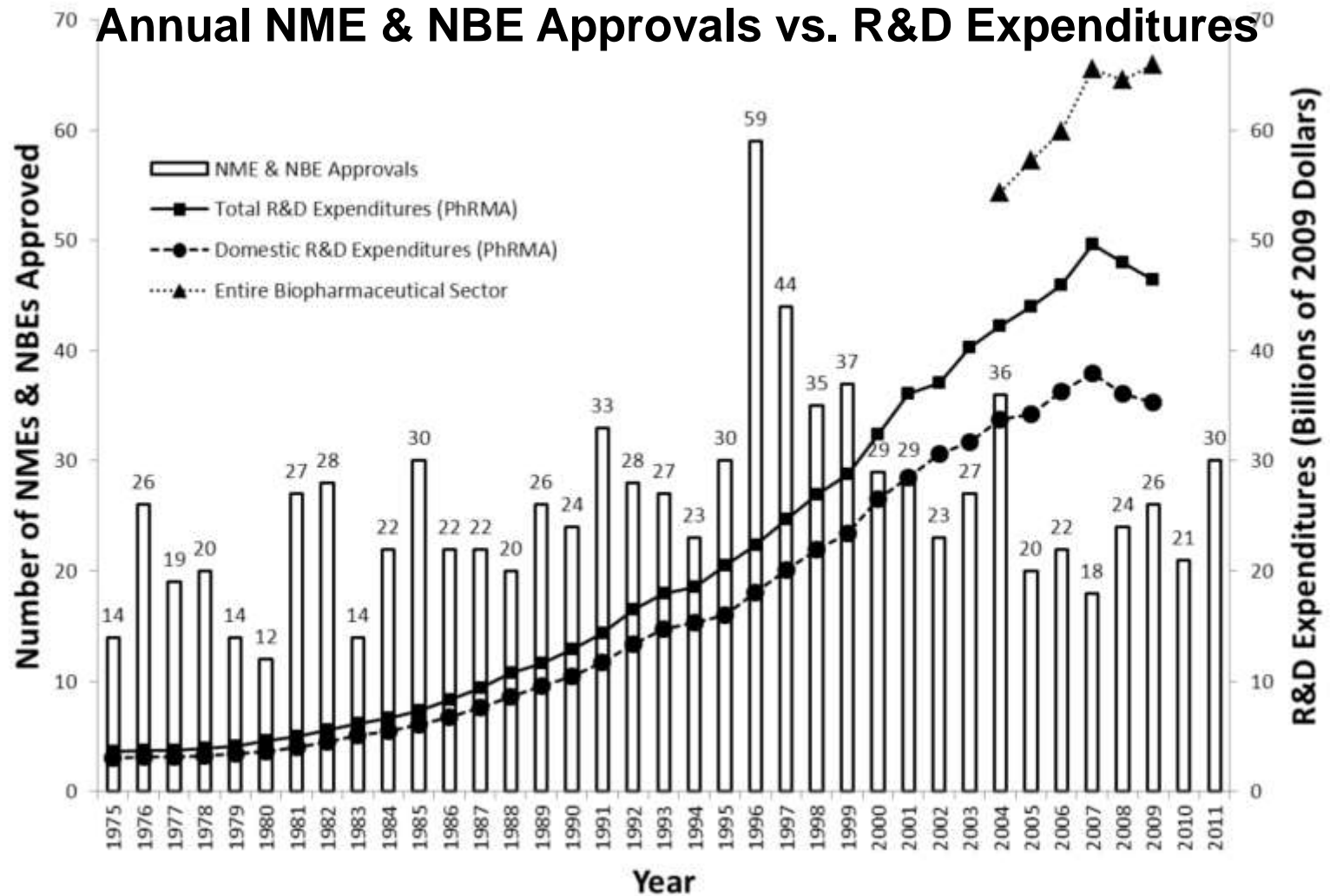
- Innovation Ecosystem is under significant stress and R&D Productivity is declining
 - Largest “patent cliff” in pharma history
 - Drugs with annual sales exceeding \$200B will come off patent in the period 2010-2014 -resulting in loss of more than \$100B in sales to generics.
 - VC investment is declining
 - Companies are exiting important fields of critical public health need (Alzheimer and psychiatric diseases)

The report concludes there are two critical needs:

1. Scientist need better methodologies and tools for translating basic biological insights into validated therapeutic targets and leads- a gap in the drug discovery and development pipeline that academic scientists often view as “too applied” and pharmaceutical companies often eschew as “too basic” to justify private investment

The report concludes there are two critical needs, cont:

2. Pharmaceutical developers and regulators need to incorporate new efficiencies into clinical trials of candidate medicines –complex and costly human studies that today constitute fully 40 percent of the biopharmaceutical industry's R&D budget



Recommendations from PCAST report

- Support Federal Initiatives to Accelerate Therapeutics
- Catalyze the Creation of a Broad-based Partnership to Accelerate Therapeutics
- Expand the Use of Practice of FDA's existing Authorities for Accelerated Approval and for Confirmatory Evidence
- Create a New Pathway for Initial Approval of Drugs shown to be Safe and Effective in a specific Subgroup of Patients
- Explore Approaches for Adaptive Approval via Pilot Projects under existing pathways
- Improve FDA's Tool for Monitoring and Communication of Clinical Benefits & Risks
- Reform Management Practices at FDA
- Study Current and Potential Economic Incentives to Promote Innovation in Drug Development



Public-Private Partnerships

Partnerships

Collaboration through partnerships can promote innovation through synergistic problem solving

- Partnership for Comparative Effectiveness Sciences (ARRA –funded)
- Project with MIT (EKG)
- Centers of Excellence in Regulatory Science
 - Three Main Components:
 - Regulatory science collaborative research, focused on FDA Priority areas
 - Staff training and scientific exchange (bi-directional)
 - Core dedicated infrastructure



Health.Data.gov, the one-stop place to get free, publicly available data, launched 2/2011

Welcome to the Health Data Community

You've found a public resource designed to bring together high-value datasets, tools, and applications using data about health and health care to support your need for better knowledge and to help you to solve problems. These datasets and tools have been gathered from agencies across the Federal government with the goal of improving health for all Americans. Check back frequently because the site will be updated as more datasets and tools become available.

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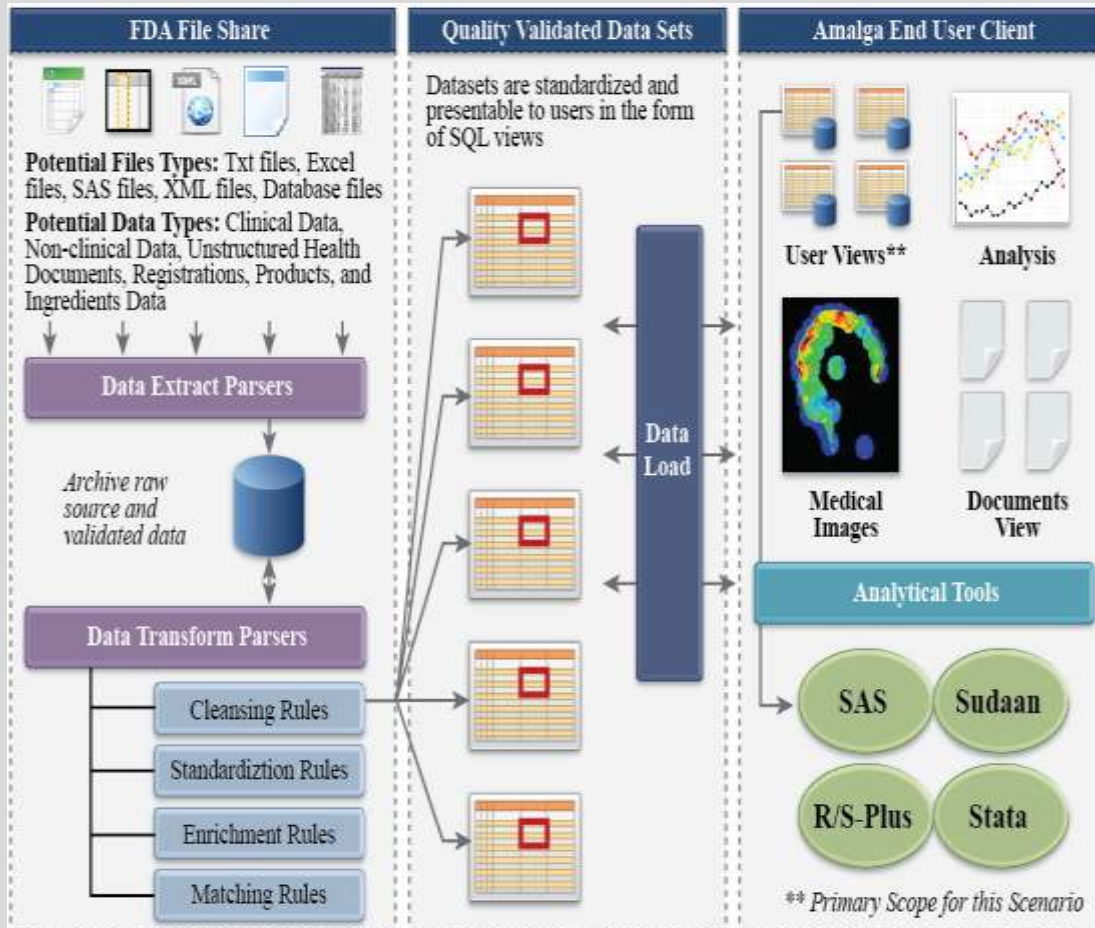
What's New



Challenges



Amalga™ as a platform enables end-users to view data from disparate sources, generate dashboards and reports, and export the corresponding data using the web interface

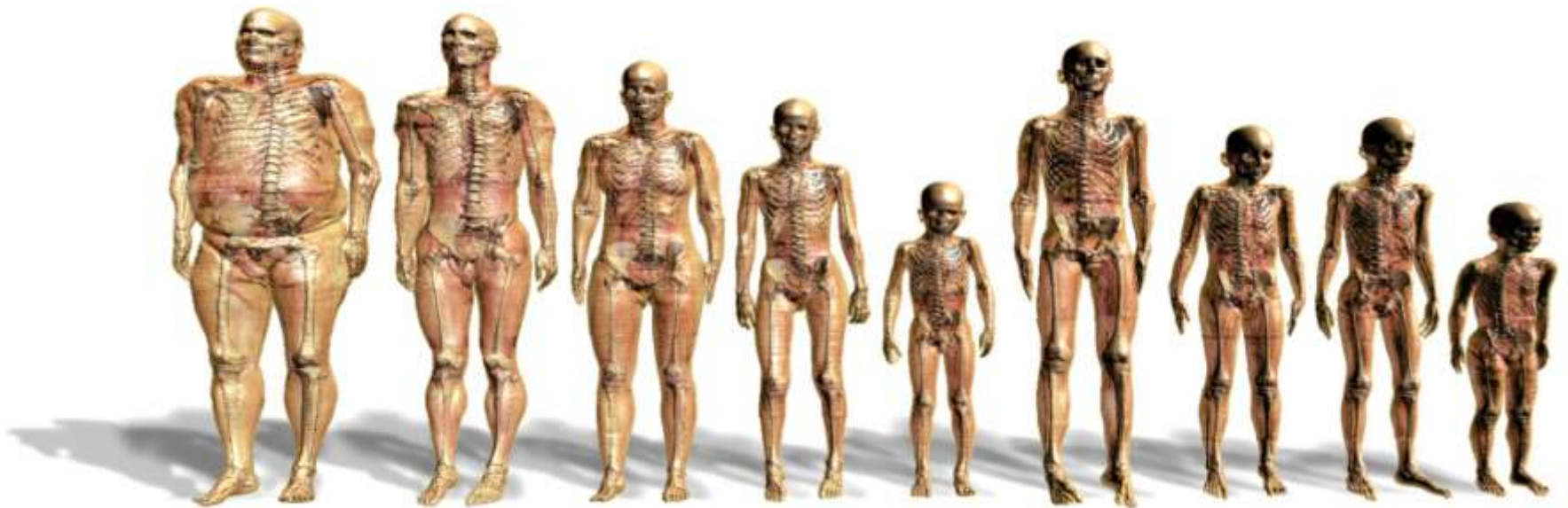


- Amalga™**
- ▶ Amalga™ is a commercial off-the shelf (COTS) product that leverages customized, built-in message parsers to ingest disparate data sources and images, generates dashboards and reports of the reconciled information, and exports the corresponding material using a web interface
 - ▶ Historically, utilized in Electronic Health Records (EHR) and in-patient care settings, this was the first instance where Amalga™ was implemented in a regulatory environment
 - ▶ The one year pilot, which culminated in the successful integration and analysis of disparate regulatory data sets encompassing the medical product lifecycle, demonstrated the feasibility of leveraging Amalga™ to enhance the quality, efficiency, and accuracy of FDA reviews

** Primary Scope for this Scenario

The Virtual Family

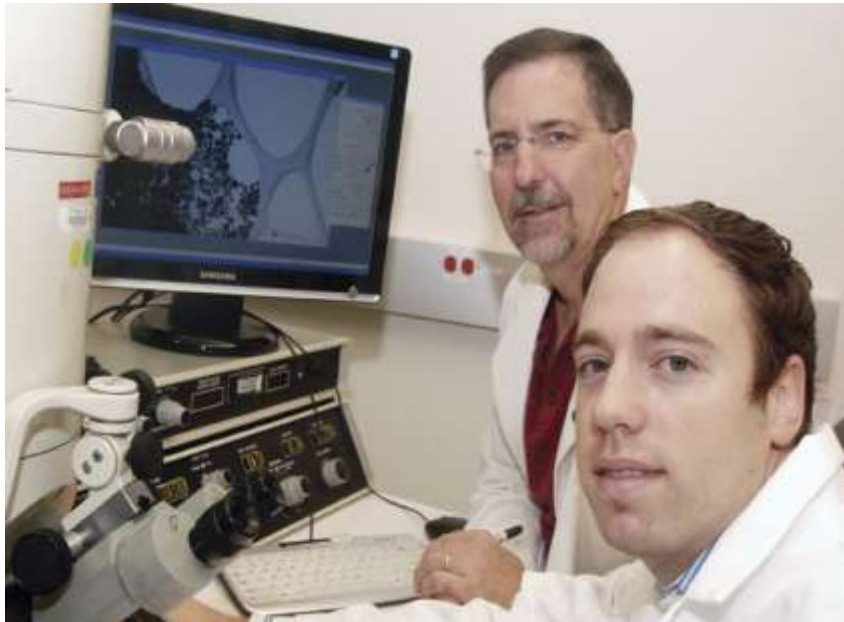
Spurring innovation through modeling device use *in silico*



Virtual Family and Virtual Classroom

http://www.itis.ethz.ch/index/index_humanmodels.html

Nanomaterials

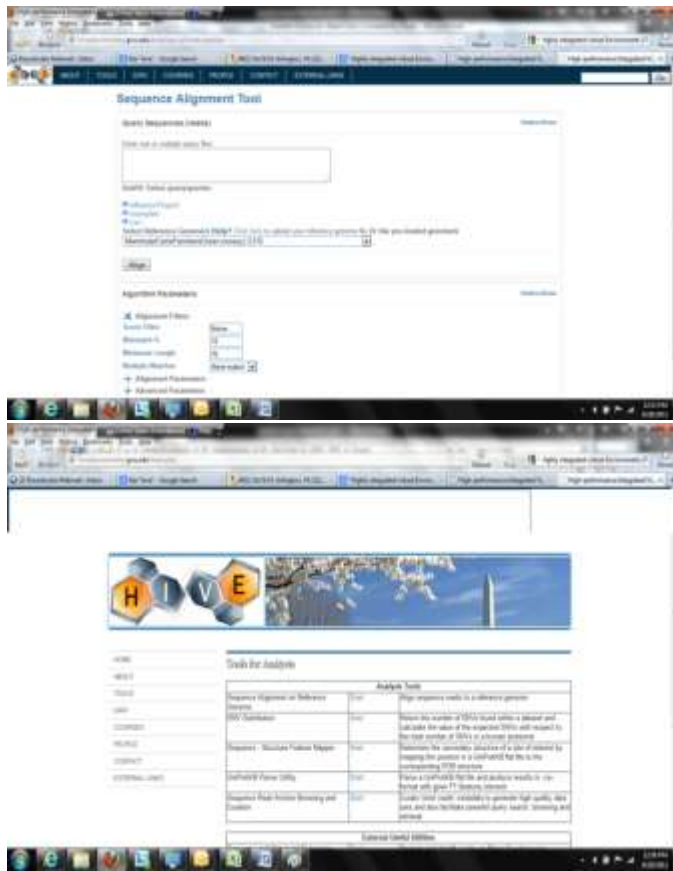


- Nanotech has the potential to revolutionize medical devices
- CDRH is developing methods to measure size, uniformity, and toxicity
- Methods will help developers understand safety questions regarding nanotech devices

Facilitating Innovation through Bioinformatics tools

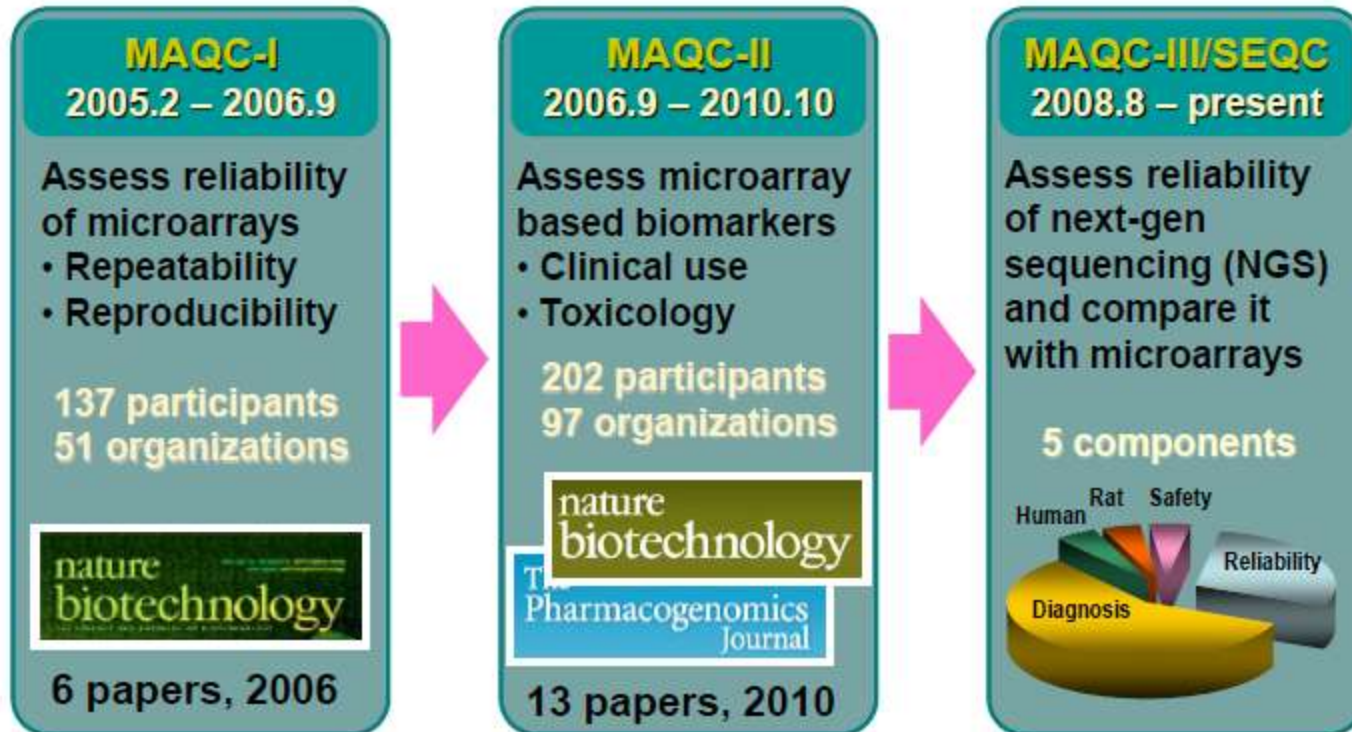


- High throughput sequence data analytics:
 - Live virus vaccine consistency
 - Use of stem cell-derived products by providing analytic tools to look at epigenetic stability, RNA expression, etc
 - Collaboration efforts on Genome-Wide Association Study (GWAS) to identify genetic bases of rare vaccine-related AEs-moving towards personalized medicine



MicroArray Quality Control (MAQC)

An FDA-led community wide consortium effort to assess technical performance and application of genomics technologies (microarrays, GWAS and next-gen sequencing)



ArrayTrack™ – A Bioinformatics Solution for Genomics Research and Review at FDA

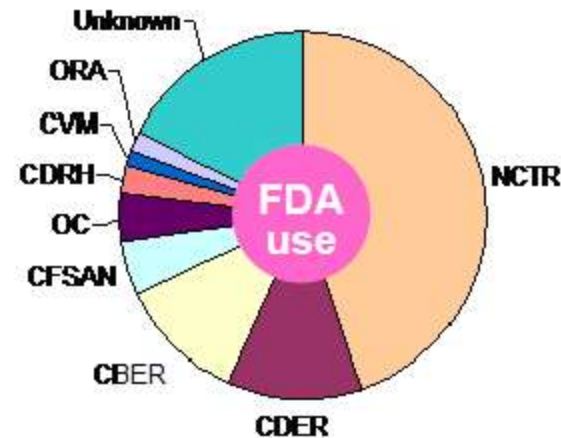
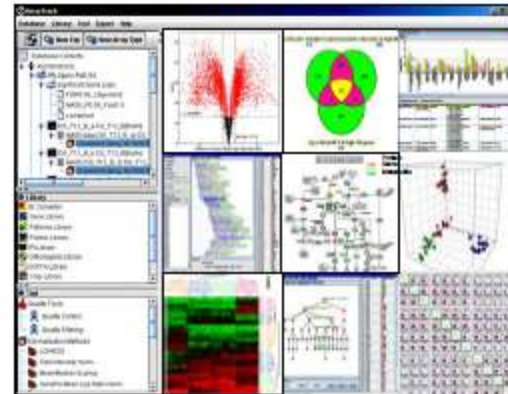
An integrated solution for microarray data management, analysis and interpretation

FDA use

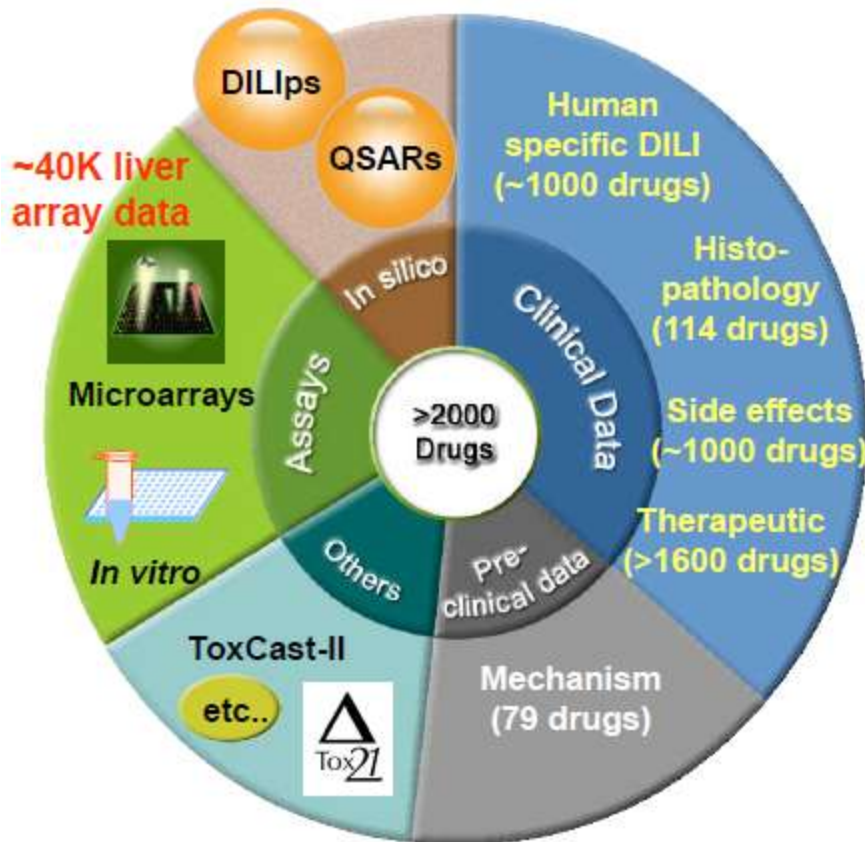
- A tool for the FDA Voluntary eXploratory Data Submission (VXDS) program
- >200 FDA reviewers and scientists have participated in the training

Public use

- ~5000 user entries each year; # users have increased steadily
- ~120 new accounts for the past 2 years
- Hosts >50,000 array data from >1600 experiments



Liver Toxicity Knowledge Base (LTKB)



Study of drug-induced liver injury (**DILI**) with emphasis on **marketed drugs**

Objectives:

- Predictive models to prioritize drug candidates for DILI potential
- Centralized repository of diverse data for DILI study

Potential applications:

- For drug development
- For the FDA to utilize and reference when liver toxicity issues arise during the various stages of the regulatory review process.



FDA's Role

- Bringing together stakeholders to identify and overcome the challenges ahead
- Implementing reforms that adapt to the changing scientific and technological landscape
- Assuring modern, streamlined regulatory pathways



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THANK YOU

