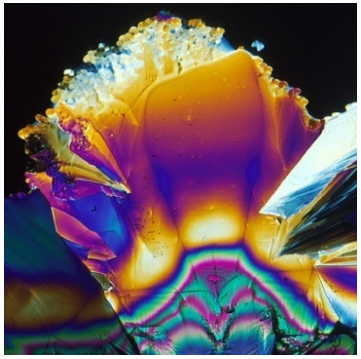


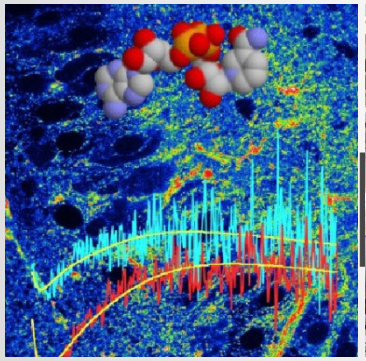
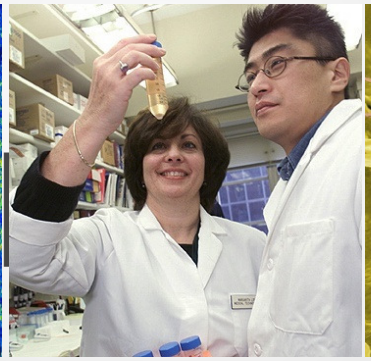
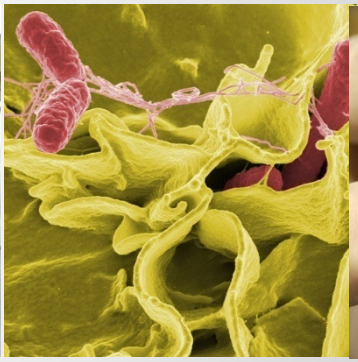


FDA Driving Biomedical Innovation

Health TechNet

January 20, 2012

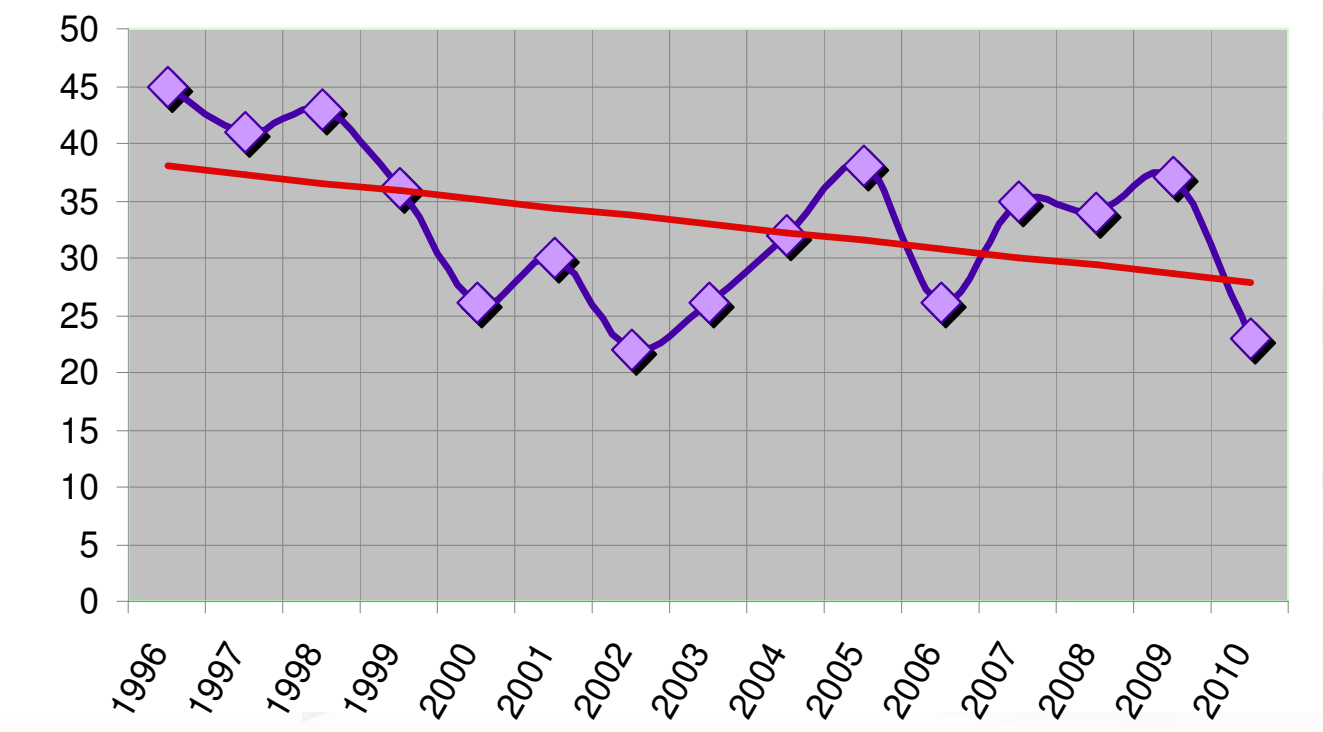
Vicki Seyfert-Margolis, Ph.D.
Senior Advisor for Science, Innovation, and Policy
Office of the Commissioner
U.S. Food and Drug Administration



NIH
Small Businesses
Academia
Biotech/ Pharma
U.S. Food and Drug Administration
Payor
Physicians
Public
Patients

15-Year NME Applications



Calendar Year	Applications Filed
1996	45
1997	41
1998	43
1999	36
2000	26
2001	30
2002	22
2003	26
2004	32
2005	38
2006	26
2007	35
2008	34
2009	37
2010	23

NME applications to CDER are not increasing. If the number of applications does not increase, CDER does not expect to see much of a year-to-year increase in approvals.

*2004-2010 represents applications for New Molecular Entities (NMEs) filed under New Drug Applications (NDAs) and therapeutic biologics filed under Original Biologic License Applications (BLAs). 2001-2003 represents NMEs but not therapeutic biologics.

FY 2011 Innovative Drug Approvals

Released November 3, 2011

U.S. Food and Drug Administration
Advancing Regulatory Science

Notable FY11 Approvals

Drug	Target	Review Pathway	Approval Time
Zytiga	Late-stage Prostate Cancer	Priority Review	4.2 months
Zelboraf	Late-stage Melanoma	Fast Track/Priority Review	3.6 months
Xalkori	Late-stage Lung Cancer	Fast Track/Priority Review/Accelerated Approval	4.9 months
Yervoy	Late-Stage Melanoma	Fast Tack/Priority Review	9.0 months
Adcetris	2 Types of Lymphoma	Fast Track/Priority Review/ Accelerated Approval	5.7 months
Caprelsa	Thyroid Cancer	Fast Track/Priority Review	9.0 months
Halaven	Metastatic Breast Cancer	Fast Track/Priority Review	7.6 months
Victrelis	Chronic Hepatitis C	Fast Track/Priority Review	5.9 months
Incivek	Chronic Hepatitis C	Fast Track/Priority Review	6.0 months
Benlysta	Systemic Lupus	Fast Track/Priority Review	9.0 months
Pradaxa	Reduce Risk of Stroke	Priority Review	6.0 months
Brilinta	Reduce Cardiovascular Death and Heart Attack	Priority Review	20.1 months
Teflaro	MRSA	Fast Track	10.0 months
Nulojix	Prevent Rejection of Transplanted Kidneys	Fast Track	23.5 months

Common Themes from Discussions with Stakeholders:

- Need to do more to help small businesses navigate the regulatory process.
- Need to adapt current FDA policies to address personalized medicine.
- Need to take advantage of cutting-edge IT and scientific computing.
- Need to address regulatory uncertainty.
- Need to streamline FDA policies and procedures.
- Need to develop more efficient regulatory pathways for companion diagnostics.
- The need to build regulatory science capacity within FDA and the broader medical device community.

Driving Biomedical Innovation: Initiatives to Improve Products for Patients

Released October 5, 2011

U.S. Food and Drug Administration
Advancing Regulatory Science

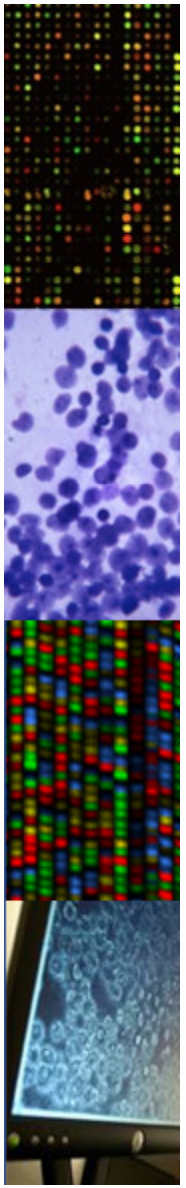
Steps FDA is Taking to Address Most Immediate Concerns:

- Rebuilding FDA's small business outreach services.
- Building the infrastructure to drive and support personalized medicine.
- Creating a rapid drug development pathway for targeted therapies.
- Harnessing the potential of data mining and information sharing.
- Increasing consistency and transparency in the medical device review process.
- Training the next generation of innovators.
- Streamlining and Reforming FDA regulations.

Rebuilding FDA's Small Business Outreach Services

- Small Business Liaison (SBL) program
  - Business people with experience starting and running biomedical companies and successfully navigating the FDA regulatory process.
  - Responsible for establishing and coordinated more effective interactions between FDA staff and small businesses.
  - Will provide FDA staff with a better understanding of inherent challenges in small business to enhance communications.
- Young Entrepreneurs program
  - Four- to six-month fellowships for business, engineering, or science students.
- Partnership with the Small Business Administration

Building the Infrastructure to Drive and Support Personalized Medicine



- Supporting Personalized Medicine through Regulatory Science.
  - Focused on approaches that use novel clinical trial designs and statistics.
- Facilitating Personalized Medicine through FDA Policies and Procedures
  - Issued Draft Guidance on Companion Diagnostics
  - Developing Draft Guidance for Co-Development
- Created a Deputy Commissioner for Medical Products.

New Drug Development Pathway for Targeted Therapies

- FDA will hold a series of meetings with stakeholders to address important questions and achieve a common understanding of steps that can be taken.

- Based on these discussions, CDER will publish a draft guidance on an expedited drug development pathway.

Harnessing the Potential of Data Mining and Information Sharing

- Establishing science enclaves to analyze large, complex datasets.
- Modernizing the IT infrastructure to support scientific computing.
- Building an infrastructure for patient-centered outcomes research.
- Creating opportunities through public-private partnerships.

Increasing Consistency and Transparency in Medical Device Review

- Development of a new expedited review program.
- Identification of emerging trends in science and technology to prepare for new breakthroughs.
- Establishment of CDRH Entrepreneurs-in-Residence pilot program.
- Increasing consistency through improved staff training.
- Providing additional clarity to industry through new guidance documents.

Future Innovators Program

- Competitive, two-year program to bring practical regulatory science and policy training together to meet the scientific and technological demands of the 21st century.
- FDA will hire qualified candidates who show outstanding promise in their fields for a short-term position within the Agency.

Improving FDA Regulations

- Proactive steps to reform existing regulations based on public feedback.
- Identifying burdensome, unclear, obsolete, ineffective, or inefficient regulations.
- FDA will hire qualified candidates who show outstanding promise in their fields for a short-term position within the Agency.

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

References

- **CL, Barton.** *Innovative Strategies and Models for R&D Success: The Evolving Networked Pharma Company*. s.l.: Business Insights Ltd, 2008.
- **The Pharmaceutical Research and Manufacturers of America (RhRMA**). Issues: Research and Development. [Online] March 16, 2010. [cited: August 23, 2010] http://www.phrma.org/news/news.
- **Elder, M.** *Pharmaceutical and Biopharmaceutical Market to 2015 Pipeline Report.* Kalorma Information a division of MarketResearch.com, Inc. 2010.
- *Pharma Embraces Open Source Models.* **Strauss, S.** 7, July 2010, Nature Biotechnology, Vol. 28.
- **Ernst & Young.** *Progressions:* Pharma 3.0. 2010
- **Taylor, Payl.** *Drug Discover Collaborations between Academia and the Pharmaceutical Industry: Cultural Factors, Intellectual Property Considerations, Case Studies and Future Trends*. s.l.: Business Insights Ltd., 2010.
- **International Expert Group on Biotechnology, Innovation, and Intellectual Property.** *Toward a New Era of Intellectual Property: From Confrontation to Negotiation.* Centre for Intellectual Property Policy, McGill University.
- *Open Source Science to Enable Drug Discovery.* **Edwards, A.** 17/18, s.l.: Drug Discover Today, 2008, Vol. 14
- *Open Innovation Networks between Academia and Industry: An Imperative for Breakthrough Therapies.* **Melese, T, Lin, SM and Chang JI, Cohen NH.** 5, May 2009, Nature Medicine, Vol. 15.
- *Open Access Chemical and Clinical Probes to Support Drug Discovery.* **Edwards, AM, et al.** 7, July 2009, Nature Chemical Biology, Vol. 5, pp. 426-440.
- Biotech reinvented. Where do you go from here? Price Waterhouse and Coopers 2010.
- Can open source Drug R&D re power pharmaceutical ? Munos, B, Nature Vol 87 No 5 May 10.
- How to improve R&D productivity: the pharmaceutical industry @ grand challenge. Paul S et al. Nature Reviews Drug Discovery Vol 9 March 10.
- Lessons from 60 years of pharmaceutical innovation, Munos B., Nature Reviews Drug Discovery Vol 8 Dec 09, pg 967.
- Federov et al, Nature Chem Biol 2010.
- Panagis et al Nature 2010
- DiMasi et al 2002.

Questions?

Vicki.Seyfert-Margolis@fda.hhs.gov