U.S. FOOD & DRUG ADMINISTRATION

Regulatory Education for Industry (REdI) Conference: What can CDER do for You?

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5.29.19

Welcome

- Who is CDER?
- Our Role in Drug Development and Regulation
- Importance of Small Businesses in Drug Development
- Challenges for Small Businesses
- Support for Small Businesses

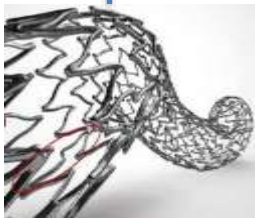
Office of the Commissioner

Office of Regulatory Affairs

Center for Food Safety & Applied Nutrition

Center for Drug Evaluation & Research

Center for Biologics Evaluation & Research



Center for Devices & Radiological Health

Center for Veterinary Medicine

National Center for Toxicological Research

Center for Tobacco Products

The Food and Drug Administration Mission

- Protects the public health by assuring that foods are safe, wholesome, sanitary and properly labeled
- Ensures that human and veterinary drugs, and vaccines and other biological products and medical devices intended for human use are safe and effective
- Protects the public from electronic product radiation
- Assures cosmetics and dietary supplements are safe and properly labeled
- Regulates tobacco products
- Advances the public health by helping to speed product innovations

https://www.fda.gov/AboutFDA/Transparency/Basics/ucm194877.htm

Center for Drug Evaluation and Research (CDER)

- ~5100 people,
  - ~3000 scientists and professionals:
    - MDs - 600
    - Pharmacists – 549
    - Pharmacologist/ Toxicologists - 550
    - Chemists - 660
    - Statisticians – 210

  - Communications staff - 25

- Director: Dr. Janet Woodcock

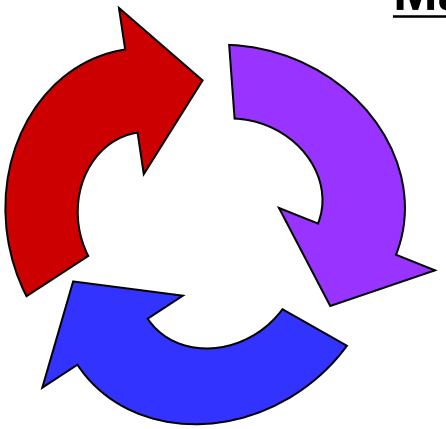
What CDER Does: Focus on Core Business Functions

Pre-Market Review

Assessment of safety and effectiveness of new medical technology & safety of new food ingredients

Product Manufacturing Quality

Inspection of manufacturing facilities and products to assure safety, quality & compliance with FDA regulations



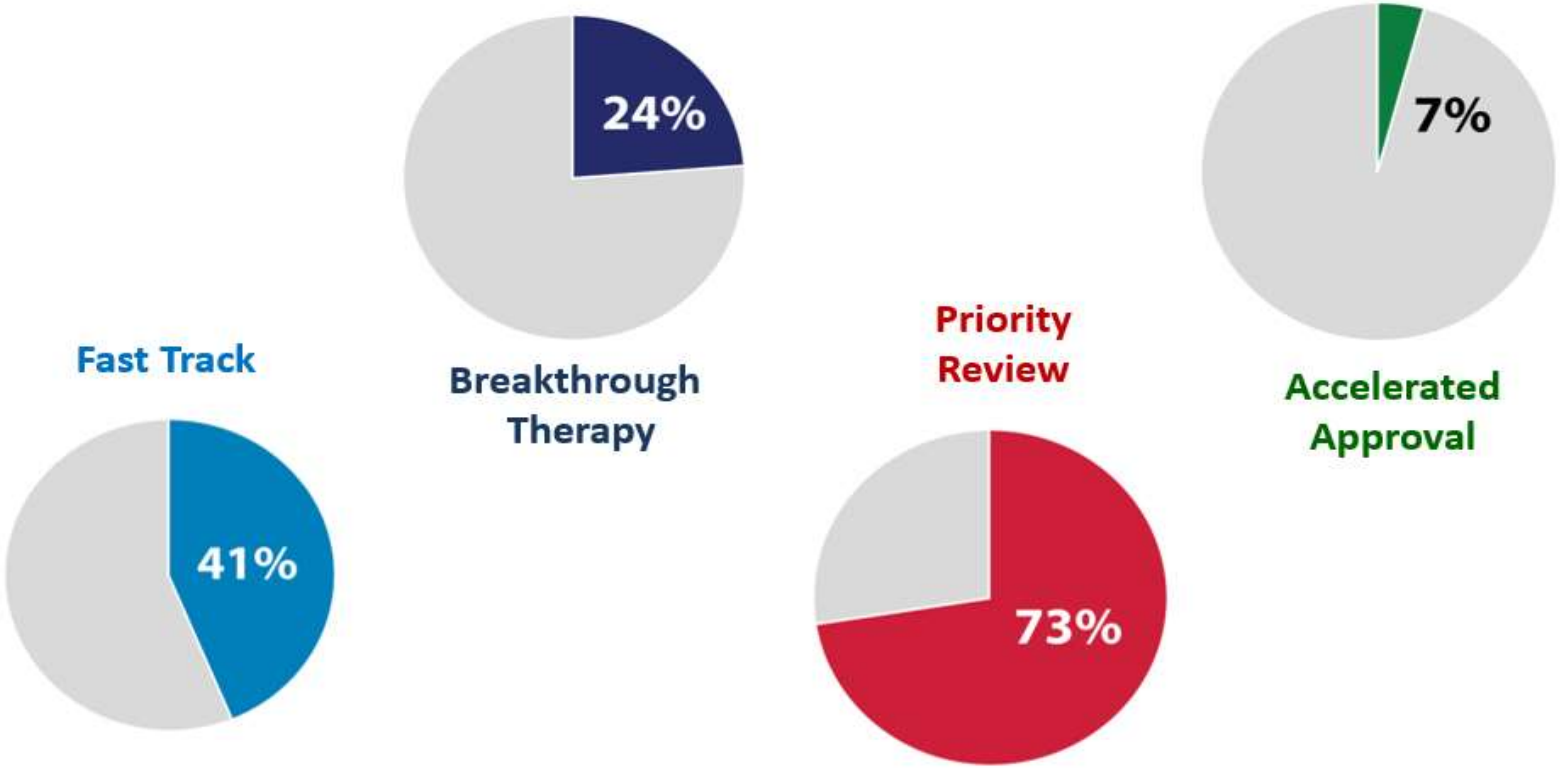
Consumer & Patient Safety

Post-marketing surveillance to ensure the safety of consumers & patients who use FDA-regulated products

What CDER Does: Promote the Availability of Safe and Effective New Drugs

- Develop infrastructure and tools for product development (not focus on development of specific products but rather areas of need)
- Encourage collaborative efforts among government, academia, industry, and patient groups
- Develop relevant data standards and regulations
- Build support for relevant academic science
- Create opportunities to share existing knowledge and databases

How CDER Supports Innovation



Reference: https://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/DrugInnovation/UCM629290.pdf

Results: 2018 Drug Approvals

- 59 novel drugs, either as new molecular entities or as new therapeutic biologics
- Seven new biosimilars
- The Office of Generic Drugs approved over 1000 new generic drugs
- First generics made up nearly 10% of 2018's approvals

Notable Product Approvals

- The first drug that contains a purified drug substance derived from marijuana (Cannabidiol or CBD), for the treatment of childhood seizures associated with two rare and severe forms of epilepsy

- A new class of drugs for the prevention of **migraine**

- The first drug for the treatment of patients with **smallpox**, which was approved under the FDA's Animal Rule, that allows efficacy findings in animal studies to support FDA approval when it is not feasible or ethical to conduct clinical trials in humans

Critical Role of Small Business in Innovation

- From 2008 to 2018 (past 10 years), among the 381 novel drugs approved by CDER, **approximately one-fifth (~22%)** were sponsored by small companies (fewer than 500 employees.)
  - Of these, **the majority of the companies (~89%)** had <u>not</u> previously brought an approved product to market

Importance of Small Businesses

- Provide important support to developing new indications for old drugs
  - New formulations of Naloxone for opioid overdose
- Small companies make up an important portion of generics and OTC manufacturers
- Small companies often involved in drug-device development
  - Vascular wraps to prevent dialysis graft occlusion
- Small companies sources of important innovative energy– needs to be supported!

Meeting the Challenges of Medical Product Development

Challenges for Small Businesses

- Drug Development has many challenges:
  - Scientific
  - Financial
  - Regulatory
- Little Room for Error
  - Trial data
  - Manufacturing data on quality
  - Adverse events reporting after marketing
  - Promotion of products scrutinized

CDER Helping to Navigate the Regulatory Path for Small Business

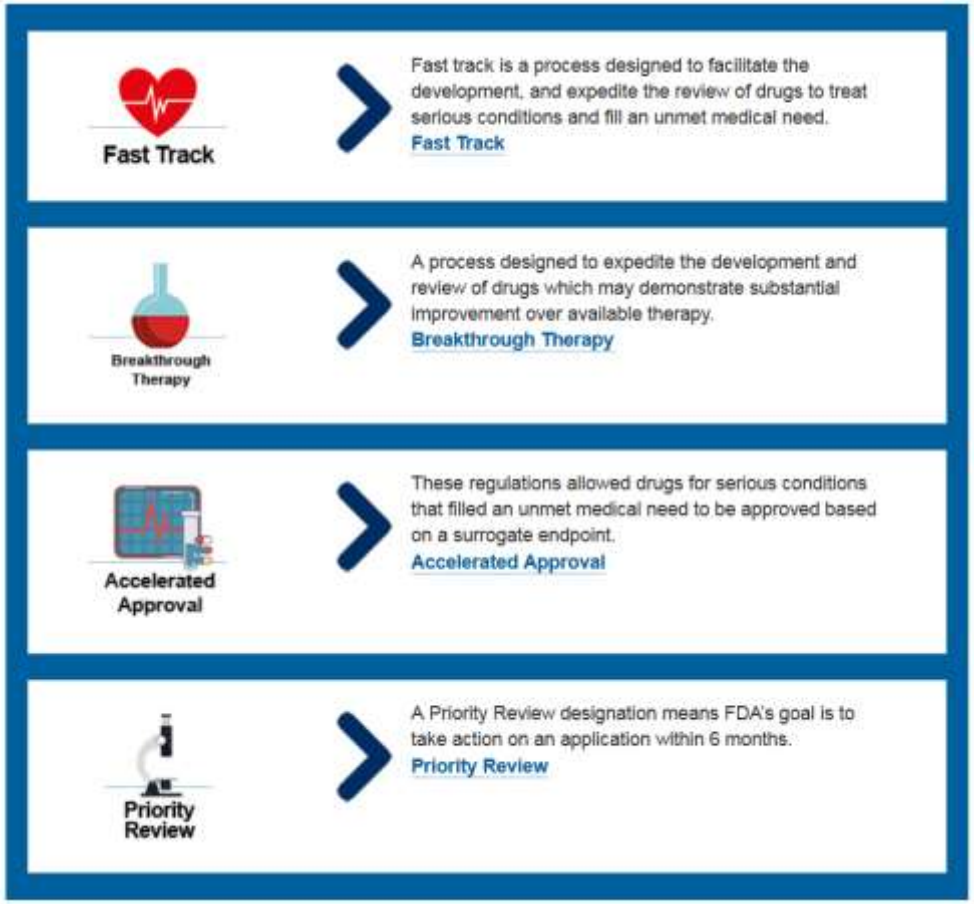
- CDER Small Business Assistance Group
  - Program Director--Captain Brenda Stodart
    - CDER Website--CDERSBIA@fda.hhs.gov
    - FDA Website—https://www.fda.gov/industry/small-business-assistance

CDER Helping to Navigate the Regulatory Path for Small Business

- Provides assistance for aspects of drug development:
  - In 2018 answered 9,710 inquiries from developers
  - Help deciding where to send submissions
  - Overviews of common complex challenges
    - Good Clinical Practice, Generics and OTC Drug Review, Biosimilars, Drug Master Files
  - Info on potential economic incentives
    - NIH funding, Orphan Products Program, PDUFA User Fees
  - Info on potential accelerated development pathways
    - https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/breakthrough-therapy

Accelerated Development Pathways

- Regulatory programs and expediting pathways for important drug development
  - Fast Track Designation, Breakthrough Therapy Designation, Accelerated Approval, and Priority Review of NDAs



Example: Breakthrough therapy designations

Recognize and support products with potential to be gamechangers and focus CDER resources on expediting their assessment, 22 awarded in 2019

CDER SBIA Helping to Navigate the Regulatory Path for Small Business

- Provides variety of meetings and info touching critical aspects of drug development:
  - Hosted 6 conferences/workshops on drug development topics
  - Produced 6 webinars.
    - Worldwide attendance: current conference has 1,055 non-US registrants
  - Issued 6 articles; Sent 174 messages to investigators
  - Clinical Investigator Training Course –
    - Co-sponsorship with University of Maryland Center of Excellence in Regulatory Science and Innovation
  - Good Clinical Practice Workshop with the UK's Medicines and Healthcare Products Regulatory Agency (MHRA-UK)
  - Targeted meetings on specific development issues
    - https://www.fda.gov/drugs/cder-small-business-industry-assistance-sbia/regulatory-education-industry-redi-complex-generic-drug-product-development-workshop-september-12-13

Supporting Drug Development Crucial to Addressing CDER Priorities

- **Policy and Process Improvements**
  - **CDER Modernization of the New Drug Regulatory Program**
    - Reorganization
    - Streamlined process
  - **Clinical Trial Modernization**
    - Clinical trial design efficiencies
    - Natural history data
  - **Use of Real World Data for regulatory decision-making**

Supporting Drug Development Crucial to Addressing CDER Priorities

- Advancing new product areas
  - Combination Products (e.g., drug-device combos)
- Responding to public health crises
  - Opioids Crisis
  - Drug Shortages
  - Compounding

Other CDER Assistance for Drug Development

- SBIA augments, but does not replace meetings with the Therapeutic Divisions (e.g., Pulmonary Drugs Division, Anti-Infectives Division) and industry on individual product development

Summary

- Helping companies innovate is part of the FDA public health mission
- This is a transformational time in the healthcare system. Expectations, science, resources, and challenges all changing
- CDER recognizes the critical role of small businesses in confronting these challenges
- CDER has programs in place to support small business
  - Process
  - Financial
  - Regulatory
- **CHALLENGE US**

Thank You