2018 Rising Leaders Conference on Healthcare Policy

The FDA in the Gottlieb Era: An Early Report Card

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Agenda

- The FDA Drug & Biologics Approval Process
- Right-to-Try/Expanded Access
- Antibiotic Incentives
- Orphan Drugs

I

The FDA Drug & Biologics Approval Process

Approval Routes - Drugs

- **"Full" New Drug Application (NDA) – 505(b)(1)**
  - **Includes "full reports" of studies to prove safety and effectiveness**
- **505(b)(2) Application – 505(b)(2)**
  - **NDA where applicant does not have rights to some of the "full reports" necessary for approval**
- **Abbreviated New Drug Application (ANDA) – 505(j)**
  - **No requirement for "full reports"**
  - **Approval based on showing of similarity to previously approved drug product, including bioequivalence**

Approval Route - Biologics

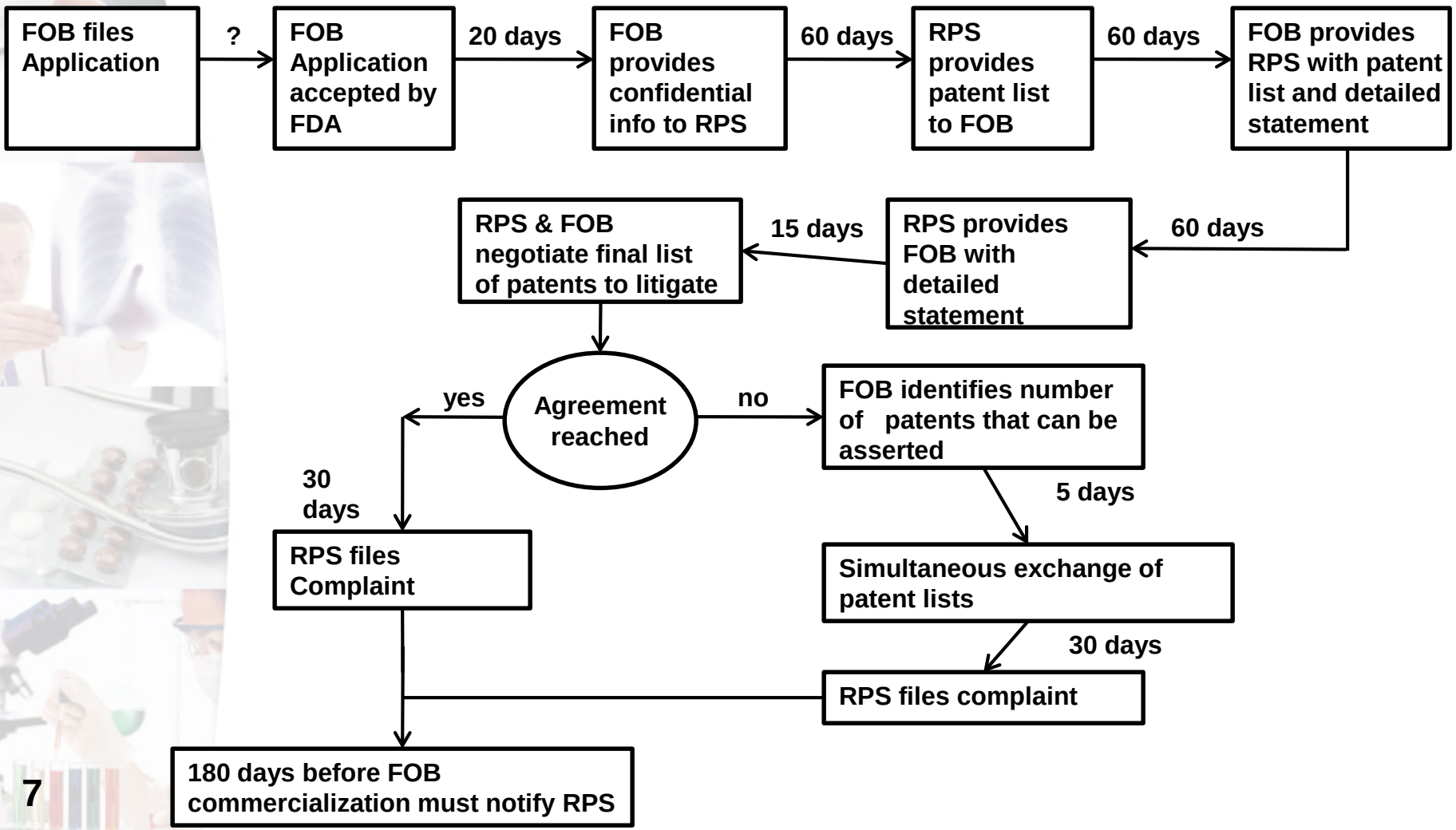
- **Biologics License Application (BLA)**
  - **Public Health Service Act 351**
- **The BPCIA passed as Title VII, Subtitle A of the Patient Protection and Affordable Care Act, Pub. L. No. 111-148, 124 Stat. 119, § § 7001-03.**
- **Signed into law**

on March 23, 2010.

Licensed Biosimilars

- **IXIFI (infliximab-qbtx) (December 2017)**
- **OGIVRI (trastuzumab-dkst) (December 2017)**
- **MVASI (bevacizumab-awwb) (September 2017)**
- **CYLTEZOC (adalimumab-adbm) (August 2017)**
- **RENFLEXIS (infliximab-abda) (April 2017)**
- **AMJEVITA (adalimumab-atto) (September 2016)**
- **ERELZI (etanercept-szzs) (August 2016)**
- **INFLECTRA (infliximab-dyyb) (April 2016)**
- **ZARXIO (filgrastim-sndz) (March 2015)**

The BPCIA "Patent Dance"



II

Right-to-Try/Expanded Access

“Expanded Access”

- **Single-Patient IND/“Compassionate Use” IND/Emergency Use**
- **Intermediate-Size Patient Populations**
- **Treatment IND/Treatment Protocol**
- **Open-Label Protocols**
- **“Group C” Oncology Exception**
- **Parallel Track IND**

Right -to-Try

- **A legislative initiative to provide patients with an acute terminal prognosis or other terminal illness access to drugs that have completed Phase 1 clinical trials under existing FDA rules.**
- **Right to Try Act of 2018**
  - **The bill's process offers a new alternative pathway for access to investigational treatments, in addition to ones currently offered under FDA regulations.**
  - **On the way to President Trump . . . .**

III

Antibiotic Incentives

QIDP Exclusivity

- New exclusivity provision enacted in 2012 for Qualified Infectious Disease Products (QIDP)
- Intended to provide incentives for the research and development of new antibiotic and antifungal drug products
- Adds 5 years of exclusivity for qualifying products to existing exclusivity periods

QIDP Exclusivity - Scope

- **Designation as QIDP before NDA submission**
- **Approval of NDA (not BLA) on or after July 9, 2012**
- **QIDP Defined:**
  - **Antibacterial or antifungal drug for human use intended to treat serious of life-threatening infections, including those caused by resistant, novel or emerging pathogens**
  - **FDA is required to maintain a list of qualifying pathogens**

QIDP Exclusivity - Requirements

- **Adds 5 years of exclusivity to:**
  - **NCE exclusivity**
  - **3-year exclusivity**
  - **Orphan drug exclusivity**
  - **In addition to pediatric exclusivity**

- **Limitations: QIDP exclusivity does not apply to:**
  - **Supplements for which QIDP exclusivity already granted**
  - **Subsequent application for certain changes (new use, dosage form, strength, etc.)**
  - **Products that fail to meet definition of QIDP**

IV

Orphan Drugs

Orphan Drug Designation

- **The Orphan Drug Act provide two routes for obtaining designation of a drug for a rare disease or condition (i.e., an "orphan drug").**
  - **A request can be made either on the basis that a product is intended to treat a disease or condition that has a prevalence of less than 200,000 affected persons in the United States, or . . . .**
  - **If a disease or condition affects over 200,000 individuals, then if a sponsor can show that there is no reasonable expectation that the costs will be recovered.**

Orphan Drug Exclusivity

- **Seven Years of Marketing Exclusivity**
  - **The FDC Act provides a seven-year period of exclusive marketing to the first sponsor who obtains marketing approval for a designated orphan drug.**
  - **The scope of orphan drug exclusivity is broad; it prevents FDA approval of ANDAs, "full" 505(b)(1) NDAs, 505(b)(2) applications, and BLAs**
  - **Orphan drug exclusivity begins on the date that a marketing application is first approved for the designated orphan drug.**

Orphan Drug- Ongoing Controversy

- **“Critics in Congress and in the pharmaceutical industry and patient groups say that while the [Orphan Drug Act] has generally worked, it has proved to be a bonanza for the makers of some very big drugs, allowing them to charge higher prices than there would have been with competition.”**

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  - **April 1990; New York Times ; “Orphan Drug Law Spurs Debate”**

THANK YOU!!

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