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
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.
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”

- **FOB files Application**
- **FOB Application accepted by FDA**
- **FOB provides confidential info to RPS**
- **RPS provides patent list to FOB**
- **FOB provides RPS with patent list and detailed statement**

**RPS & FOB negotiate final list of patents to litigate**

- **Agreement reached**
  - yes: **RPS files Complaint**
  - no: **FOB identifies number of patents that can be asserted**

- **Simultaneous exchange of patent lists**
  - 5 days
  - 30 days

- **RPS files complaint**
- **180 days before FOB commercialization must notify RPS**

- **20 days**
- **60 days**

- **Simultaneous exchange of patent lists**
  - 30 days
  - 5 days
  - 60 days

- **30 days**
- **5 days**
- **60 days**
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 . . . .
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 or life-threatening infections, including those caused by resistant, novel or emerging pathogens
  - FDA is required to maintain a list of qualifying pathogens
• **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
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.
• “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.”
Orphan Drug- Ongoing Controversy

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THANK YOU!!
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