U.S. FOOD & DRUG ADMINISTRATION

FDA High-Level Update

WCBP Conference, January 31, 2018
Regulatory ICH Countries Workshop Plen-shop

2017 CDER/FDA Approvals

- 46 novel drug approvals CDER
  - 39% rare or orphan diseases
  - 39% Fast Track
  - 37% Breakthrough Therapy
  - 61% Priority Review
- 21 BLA approvals
  - 7 breakthrough products
  - 2 Antibody Drug Conjugates
  - 2 rare diseases
  - 5 biosimilars
- **Nine** 351(k) BLAs for biosimilar products have been approved:
  - Zarxio (filgrastim-sndz)
  - Inflectra (infliximab-dyyb)
  - Erelzi (etanercept-szzs)
  - Amjetiva (adalimumab-atto)
  - Renflexis (infliximab-abda)
  - Cyltezo (adalimumab-adbm)
  - Mvasi (bevacizumab-awwb)
  - Ogivri (trastuzumab-dkst)
  - Ixifi (infliximab-qbtx)

OPQ Strategic Priorities 2018

- **Strengthen OPQ's collaborative organization**
  - Leverage a collaborative culture, an engaged and empowered workforce, streamlined processes, and effective teaming to ensure an efficient, high-performing, innovative, and results-oriented organization

- **Promote availability of better medicines**
  - Minimize barriers to encourage innovation within FDA and in the pharmaceutical sector through sensible oversight, research, risk-based decision-making and continuous process improvement
  - Emerging Technology Team: CDER-ETT@fda.hhs.gov

- **Elevate awareness and commitment to the importance of pharmaceutical quality**
  - Effectively communicate the importance of quality and that the American public can trust their drugs

- **Strengthen partnerships and engage stakeholders**
  - Build productive relationships with business partners within and outside FDA and jointly foster effective stakeholder engagement to meet the needs of the American public

CBER Strategic Goals

1. Increase the nation's preparedness to address threats as a result of terrorism, pandemic influenza, and emerging infectious diseases
2. Improve global public health through international collaboration including research and information sharing
3. Utilize advances in science and technology to facilitate development of safe and effective biological products
4. Ensure the safety of biological products
5. Advance regulatory science and research
6. Manage for organizational excellence and accountability

CBER's Strategic Plan is located at:

http://www.fda.gov/downloads/aboutfda/centersoffices/cber/ucm266867.pdf

Selected FY 2017 Product Approvals

Kymriah and **Yescarta**

- CD19-directed chimeric antigen receptor (CAR) T cell immunotherapies
- Kymriah - indicated for treatment of patients up to 25 years of age with B-cell precursor acute lymphoblastic leukemia refractory or in second or later relapse [*FDA News Release, August 30, 2017*]
- Yescarta - indicated in adult patients with large B-cell lymphoma after at least two other kinds of treatment failed, including DLBCL, primary mediastinal large B-cell lymphoma, high grade B-cell lymphoma and DLBCL arising from follicular lymphoma [*FDA Press Release, October 18, 2017]*

Selected FY 2017 Product Approvals

Luxturna

- An adeno-associated virus vector-based gene therapy expressing the gene for human retinal pigment epithelium 65 kDa protein (hRPE65)
- Indicated for treatment of patients with confirmed biallelic mutation in gene encoding hRPE65 associated with retinal dystrophy, a disorder affecting 1000 – 2000 people in the United States that may cause a complete blindness in certain patients
- First gene therapy approved for the treatment of a genetic disease [*FDA Press Release, December 19, 2017]*

Regenerative Medicine Advanced Therapy (RMAT) Designation

- 21st Century Cures Act, section 3033 (implemented in Dec, 2016)
- A drug is eligible for designation if:
  - It is a regenerative medicine therapy;
    - Includes cell therapies, genetically modified cells, gene therapies producing durable effects, therapeutic tissue engineering products, human cell and tissue products, or any combination product using such therapies or products
    - The drug is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and
    - Preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such disease or condition
- CBER has Granted 13 RMAT Designations Since Program Inception (data as of Dec 29, 2017):
  - All 13 are Cell Therapy products
  - 9 have Orphan Product designation
  - 5 also have Fast Track designation
- CBER awarded a contract in September 2017 to support the coordination of standards development process (draft guidance is available)
  - Includes standards for manufacturing processes and controls
  - Through a public process (in consultation with NIST, industry, academia, and other stakeholders)