FY 2019

Real Time Report

pursuant to the

Prescription Drug User Fee Act

as amended by the FDA Reauthorization Act of 2017

Acronyms

BLA – Biologics License Application

CBER – Center for Biologics Evaluation and Research

CDER – Center for Drug Evaluation and Research

FDA – Food and Drug Administration

FDARA – FDA Reauthorization Act of 2017

FY – Fiscal Year (October 1 to September 30)

NDA – New Drug Application

PDUFA – Prescription Drug User Fee Act

Q1 – Quarter 1 (October 1 to December 31)

Q2 – Quarter 2 (January 1 to March 31)

Q3 – Quarter 3 (April 1 to June 30)

Q4 – Quarter 4 (July 1 to September 30)

Background

On August 18, 2017, the FDA Reauthorization Act of 2017 (FDARA) (Public Law 115-52) was signed into law. FDARA amends the Federal Food, Drug, and Cosmetic Act (FD&C Act) to revise and extend the user fee programs for human drugs, biologics, generic drugs, medical devices, and biosimilar biological products.

Section 736B(a)(3) of the FD&C Act, as amended by Section 903 of FDARA, requires the Food and Drug Administration (FDA) to provide 'Real Time' reporting, posted on a quarterly basis, of guidance documents and public meetings related to the process for the review of human drugs and biologics, and the number of new drug and biologics license applications filed, and the number of approvals.[1]

Real Time Reporting Under Section 736B(a)(3) of the FD&C Act

This report provides the PDUFA real time reporting metrics, required under Section 736B(a)(3) of the FD&C Act:

> Not later than 30 calendar days after the end of the second quarter of fiscal year 2018, and not later than 30 calendar days after the end of each quarter of each fiscal year thereafter, the Secretary of Health and Human Services shall post on the internet website of the Food and Drug Administration:
>
> 1) The number and titles of draft and final guidance on topics related to the process for the review of human drug applications and whether such guidances were issued as required by statute or pursuant to a commitment under the letters described in section 101(b) of the Prescription Drug User Fee Amendments of 2017.
> 2) The number and titles of public meetings held on topics related to the process for the review of human drug applications, and whether such meetings were required by statute or pursuant to a commitment under the letters described in section 101(b) of the Prescription Drug User Fee Amendments of 2017.
> 3) The number of new drug applications and biological licensing applications approved.
> 4) The number of new drug applications and biological licensing applications filed.

[1] This report provides information related to human drug applications, which is defined by section 735(1) of the FD&C Act as an application for approval of a new drug submitted under section 505(b) of the FD&C Act or licensure of a biological product under section 351(a) of the Public Health Service (PHS) Act, with certain exceptions including supplemental applications and applications for certain types of drugs and biologics. This report does not include information regarding biosimilar biologic license applications, which is presented in the Real Time Report pursuant to the Biosimilars User Fee Act.

Human Drugs and Biologics

Guidance Documents

Pursuant to Section 736B(a)(3) of the FD&C Act, the table below lists the number and titles of draft and final guidance on topics related to the process for the review of human drug and biologics license applications and whether such guidances were issued as required by statute or pursuant to a commitment under the letters described in section 101(b) of the Prescription Drug User Fee Amendments of 2017. Guidance documents are listed by the quarter in which they were issued and are provided in a cumulative format for fiscal year 2019.

Table 1: Draft and Final Guidance Documents Related to the Process for the Review of Human Drug and Biologics License Applications for FY 2019

Number	Quarter Issued	Title & Website Link	Date Issued	Issued as Required by Statute or Pursuant to Commitment Letter	Statutory or Commitment Letter Citation (if applicable)
1	Q1	Contents of a Complete Submission for Threshold Analyses and Human Factors Submissions to Drug and Biologic Applications www.federalregister.gov/documents/2018/10/01/2018-21243/contents-of-a-complete-submission-for-threshold-analyses-and-human-factors-submissions-to-drug-and	10/1/2018	Other	N/A
2	Q1	Adaptive Designs for Clinical Trials of Drugs And Biologics www.federalregister.gov/documents/2018/10/01/2018-21314/adaptive-designs-for-clinical-trials-of-drugs-and-biologics-draft-guidance-for-industry-availability	10/1/2018	Pursuant to Commitment Letter	J.4.d.
3	Q1	Master Protocol: Efficient Clinical Trial Design Strategies to Expedite Development of Oncology Drugs and Biologics www.federalregister.gov/documents/2018/10/01/2018-21313/master-protocols-efficient-clinical-trial-design-strategies-to-expedite-development-of-oncology	10/1/2018	Other	N/A
4	Q1	Selection of the Appropriate Package Type Terms and Recommendations for Labeling Injectable Medical Products Packaged in Multiple-Dose, Single-Dose, and Single-Patient-Use Containers for Human Use www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM468228.pdf	10/2/2018	Other	N/A
5	Q1	Atopic Dermatitis: Timing of Pediatric Studies During Development of Systemic Drugs www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM603702.pdf	10/3/2018	Other	N/A

Number	Quarter Issued	Title & Website Link	Date Issued	Issued as Required by Statute or Pursuant to Commitment Letter	Statutory or Commitment Letter Citation (if applicable)
6	Q1	Hematologic Malignancies: Regulatory Considerations for Use of Minimal Residual Diseases in Development of Drugs and Biological Products for Treatment www.federalregister.gov/documents/2018/10/16/2018-22436/hematologic-malignancies-regulatory-considerations-for-use-of-minimal-residual-disease-in	10/16/2018	Other	N/A
7	Q1	Rare Diseases: Early Drug Development and the Role of Pre-Investigational New Drug Application Meetings www.federalregister.gov/documents/search?conditions%5Bterm%5D=FDA-2018-D-3268	10/16/2018	Other	N/A
8	Q1	Developing Targeted Therapies in Low-Frequency Molecular Subsets of a Disease www.federalregister.gov/documents/2018/10/16/2018-22437/developing-targeted-therapies-in-low-frequency-molecular-subsets-of-a-disease-guidance-for-industry	10/16/2018	Other	N/A
9	Q1	Considerations for the Development of Dried Plasma Products Intended for Transfusion; Draft Guidance for Industry www.fda.gov/downloads/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/Blood/UCM624461.pdf	10/20/2018	Other	N/A
10	Q1	Testicular Toxicity: Evaluation During Drug Development www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm455102.pdf	10/25/2018	Other	N/A
11	Q1	Biopharmaceutics Classification System-Based Biowaivers; International Council for Harmonisation www.federalregister.gov/documents/2018/10/26/2018-23425/biopharmaceutics-classification-system-based-biowaivers-international-council-for-harmonisation	10/26/2018	Other	N/A
12	Q1	Chronic Hepatitis B Virus Infection: Developing Drugs for Treatment www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM624695.pdf	11/2/2018	Other	N/A
13	Q1	Hypertension: Developing Fixed-Dose Combination Drugs for Treatment www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM593825.pdf	11/7/2018	Other	N/A
14	Q1	Nonmetastatic, Castration-Resistant Prostate Cancer: Considerations for Metastasis-Free Survival Endpoint in Clinical Trials www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM625703.pdf	11/14/2018	Other	N/A

Number	Quarter Issued	Title & Website Link	Date Issued	Issued as Required by Statute or Pursuant to Commitment Letter	Statutory or Commitment Letter Citation (if applicable)
15	Q1	Noncirrhotic Nonalcoholic Steatohepatitis With Liver Fibrosis: Developing Drugs for Treatment www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM627376.pdf	12/4/2018	Other	N/A
16	Q1	Interpretation of the Deemed to be a License Provision of the Biologics Price Competition and Innovation Act of 2009 www.federalregister.gov/documents/2018/12/12/2018-26854/interpretation-of-the-deemed-to-be-a-license-provision-of-the-biologics-price-competition-and	12/12/2018	Other	N/A
17	Q1	The Deemed to be a License Provision of the BPCI Act: Questions and Answers www.federalregister.gov/documents/2018/12/12/2018-26855/the-deemed-to-be-a-license-provision-of-the-bpci-act-questions-and-answers-draft-guidance-for	12/12/2018	Other	N/A
18	Q1	Data Integrity and Compliance With Current Good Manufacturing Practice Guidance for Industry www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM495891.pdf	12/12/2018	Other	N/A
19	Q1	Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data www.federalregister.gov/documents/2018/12/21/2018-27657/developing-and-submitting-proposed-draft-guidance-relating-to-patient-experience-data-draft-guidance	12/21/2018	Other	N/A

Public Meetings

Pursuant to Section 736B(a)(3) of the FD&C Act, the table below lists the number and titles of public meetings held on topics related to the process for the review of human drug and biologics license applications and whether such meetings were required by statute or pursuant to a commitment under the letters described in section 101(b) of the Prescription Drug User Fee Amendments of 2017. Public meetings are listed by the quarter in which they were held and are provided in a cumulative format for fiscal year 2019.

Table 2: Public Meetings Held Related to the Process for the Review of Human Drug and Biologics License Applications for FY 2019

Number	Quarter Held	Title	Date Held	Held as Required by Statute or Pursuant to Commitment Letter
1	Q1	Patient-Focused Drug Development Guidance: Methods to Identify What Is Important to Patients and Select, Develop or Modify Fit-for-Purpose Clinical Outcome Assessments; Public Workshop	10/15/2018	Pursuant to Commitment Letter
2	Q1	FDA Oncology Center of Excellence - Society for Immunotherapy of Cancer Public Workshop: Immune-modified Response Criteria in Cancer Immunotherapy Clinical Trials	11/8/2018	N/A*
3	Q1	Quantitation of AAV-Based Gene Therapy Products	12/7/2018	N/A
4	Q1	Drug Development Tool Process Under the 21st Century Cures Act and Prescription Drug User Fee Act VI; Public Meeting	12/11/2018	Pursuant to Commitment Letter

* Note: This meeting was a co-sponsored

New Drug and Biologics License Applications

The figures in the tables below represent filed and approved New Drug Applications (NDAs) and Biologics License Applications (BLAs) during FY 2019. Figures are calculated based on the same criteria used in the annual PDUFA Report to Congress. The filed figures are based on when the application was received and include applications that are still within the 60-day filing date and have not yet been filed.[2] The approved figures include applications that have received an approval or tentative approval action. All data is as of December 31, 2018, including data previously provided.

Quarterly filed figures are preliminary.

Table 3: The number of NDAs and BLAs filed* in FY 2019 (as of December 31, 2018)

Application Type	Q1	Q2	Q3	Q4	Cumulative
NDAs	53				53
BLAs	4				4
Total	**57**				**57**

* Data excludes applications that are unacceptable for filing due to nonpayment of user fees, have been withdrawn within 60 days of receipt, or have been refused to file.

Table 4: The number of NDAs and BLAs approved in FY 2019 (as of December 31, 2018)

Application Type	Q1	Q2	Q3	Q4	Cumulative
NDAs	37				37
BLAs	7				7
Total	**44**				**44**

[2] FDA only files applications that are sufficiently complete to permit a substantive review. The Agency makes a filing decision within 60 days of an original application's receipt.

Glossary of Terms Included in This Report

Approval – An official action by FDA, communicated via letter to a NDA or BLA applicant, that the applicant has satisfied the requirements of the statute for approval and allows the commercial marketing of the product.

BLA – The BLA must contain specific information on the manufacturing processes, chemistry, pharmacology, clinical pharmacology, and the clinical effects of a biologic product. If the information provided meets FDA requirements, the application is approved, and a license is issued allowing the firm to market the product.

NDA – When the sponsor of a new drug believes that enough evidence on the drug's safety and effectiveness has been obtained to meet FDA's requirements for marketing approval, the applicant submits to FDA a new drug application. The application must contain data from specific technical viewpoints for review, including chemistry, pharmacology, medical, biopharmaceutics, and statistics. If the NDA is approved, the product may be marketed in the United States.

Refuse to File – An official action from FDA, communicated via letter to a NDA or BLA applicant, stating that the FDA has made a threshold determination that the application is not sufficiently complete to permit a substantive review

Tentative Approval – An official action by FDA, communicated via letter to a NDA applicant, stating that the NDA otherwise meets the requirements for approval, but that it may not be legally marketed in the U.S. until the market exclusivity and/or patent term of the listed drug upon which the application relies, has expired.

Unacceptable for Filing – An official action by FDA, communicated via letter to a NDA applicant, stating that the application is not accepted by the FDA for review. Note: PDUFA requires this action when the applicant has not submitted payment for the application, or when the applicant is determined to be in arrears for non-payment of annual program fees.