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ONE HUNDRED FIFTEENTH CONGRESS

Congress of the United States
House of Representatives
COMMITTEE ON ENERGY AND COMMERCE
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MEMORANDUM

May 16, 2017

To: Subcommittee on Health Democratic Members and Staff

Fr: Committee on Energy and Commerce Democratic Staff

Re: Mark-up of H.R. 1222, Congenital Heart Futures Reauthorization Act of 2017, H.R. 2410, Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2017, and H.R. ___, the Food and Drug Administration Reauthorization Act of 2017.

On **Thursday, May 18th, at 10:00 a.m., in Room 2123 of the Rayburn House Office Building**, the Committee will hold a mark-up on H.R. 1222, Congenital Heart Futures Reauthorization Act of 2017, H.R. 2410, Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2017 and H.R. ___, the Food and Drug Administration (FDA) Reauthorization Act of 2017 (FDARA). FDARA is a package of the Food and Drug Administration (FDA) user fee agreements regarding prescription drugs, medical devices, generic drugs, and biosimilars. All user fee agreements will be reauthorized for a five year period extending from October 1, 2017 to October 1, 2022.

I. PUBLIC HEALTH LEGISLATION

A. H.R. 1222 – Congenital Heart Futures Reauthorization Act of 2017

The Congenital Heart Futures Act became law as part of the Affordable Care Act in 2010. The bipartisan legislation authorized the Centers for Disease Control and Prevention (CDC) to create a National Congenital Heart Disease Surveillance System and encouraged increased congenital heart disease research at the National Institutes of Health (NIH).

Congressman Bilirakis (R-FL) and Congressman Schiff (D-CA) introduced H.R. 1222, the Congenital Heart Futures Reauthorization Act of 2017 on February 27, 2017. H.R. 1222 builds on existing efforts by requiring CDC to enhance and expand its research, surveillance infrastructure, and public outreach and education programs relating to congenital heart disease. The bill also would direct CDC to submit one or more reports to Congress on a study to improve knowledge of the epidemiology of congenital heart disease. Additionally, the bill would direct CDC to implement a CHD awareness

campaign. Finally, the bill requires NIH to issue a report outlining current and future research plans with respect to CHD. A Manager's Amendment will be introduced that eliminates the requirement that CDC sponsor a CHD awareness campaign.

B. H.R. 2410 – Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2017

Section 712 of the American Jobs Creation Act of 2004 created the Sickle Cell Disease Treatment Demonstration Program. The law directed the Health Resources and Services Administration (HRSA) to make grants available to up to 40 entities for the purpose of improving the prevention and treatment of sickle cell disease.

Congressman Danny Davis (D-IL) and Congressman Michael Burgess (R-TX) introduced H.R. 2410, the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2017 on May 11, 2017. The bill would reauthorize the Sickle Cell Disease Treatment Demonstration Program and allow up to 25 eligible entities to receive grants under the program. H.R. 2410 also would allow the Secretary of Health and Human Services to conduct or support research to help increase understanding of sickle cell disease. Finally, this legislation would allow the Secretary to create a grant program that would support states in conducting surveillance on the prevalence and distribution of the disease, conduct public health initiatives, or to identify and evaluate prevention and treatment strategies.

II. H.R. ___, FDA REAUTHORIZATION ACT OF 2017

A. Title I – Fees Relating to Drugs

Title I puts forth the provisions of the Prescription Drug User Fee Amendments of 2017 (PDUFA VI). PDUFA VI authorizes (FDA to collect user fees from companies producing certain human drug and biological products to ensure the timely review of new drug applications (NDAs) and biologic license applications (BLAs). PDUFA VI contains several modifications which have been previously deliberated and agreed upon by both FDA and industry. Of note, PDUFA VI maintains the current drug review timelines of reviewing 90 percent of applications within 10 months with a 60-day filing date for standard applications, and 90 percent of applications within six months with a 60-day filing date for priority applications.

PDUFA VI modifies the current fee structure under PDUFA to merge product fees and establishment fees into a PDUFA program fees. The program fee will be paid for every marketed product by the drug sponsor. Historically, fees were derived equally from facility fees, various application fees, and product fees. The new structure is derived 20 percent from application fees and 80 percent program fees. Supplement fees and facility fees are eliminated. The fee setting methodology will be modified to create an annual base revenue amount. In fiscal year, the base fee amount will be $878,590,000. New methodology known as a capacity planning adjuster will be implemented so that fees may be adjusted to more accurately reflect application workload. Performance and financial reports continue to be due to Congress every year while HHS is required to provide recommendations to Congress by January 15, 2022 after a series of public meetings.

PDUFA VI also complements many of the policy provisions included in 21st Century Cures. The agreement will continue to support patient-focused drug development through the development of five guidance documents focused on the following topics: patient data collection, including methods, nomenclatures and how to collect representative data; methods and approaches to collect information regarding patient preferences and how the agency expects to incorporate such information into risk-benefit assessments; assessment of disease impact in order to better facilitate the incorporation of patient outcomes into clinical trial designs; clinical patient assessments; and structuring the risk-benefit assessment, including through incorporation of patient perspectives and data from the product lifecycle. The agreement will also build on biomarker qualification through enhancing staff capacity for biomarker review, developing and issuing guidance on the taxonomy for biomarkers and the needed standards of evidence, and publishing a list of submitted biomarkers for qualification. FDA will also hold public workshops and initiate pilot projects to assess how real world evidence can be used to support regulatory decision-making. In addition, the agency will issue guidance on innovative trial design and establish a voluntary pilot program for drug sponsors wishing to utilize innovative trial designs.

B. Title II – Fees Relating to Devices

Title II puts forth the provisions of the Medical Device User Fee Amendments of 2017 (MDUFA IV). MDUFA IV authorizes FDA to collect fees from medical device companies to support the work of processing company registrations, applications, notifications, and other types of submissions.

Other than the addition of de novo submission fees, MDUFA IV does not contain any changes to the established fee structure. Over the course of the agreement, the medical device industry will pay $999.5 million, building from a base fee amount for FY2017 of $130,184,348 to $213,687,660 in FY2022. These user fees will also support 217 new employees, in addition to funding the other activities laid out in the agreement. Performance and financial reports continue to be due to Congress every year while HHS is required to provide recommendations to Congress by January 15, 2022 after a series of public meetings.

MDUFA IV makes a number of key improvements to the medical device user fee program. First, it reduces the number of days in the "total time to decision" goals and incorporates new goals for the review of de novo devices. For 510(k) medical devices, the total time goal now is currently 124 days, which would be lowered to 108 under MDUFA IV. For premarket applications, the total time goal now is 385 days, which would be lowered to 290 under MDUFA IV. The agreement also continues to incorporate the patient perspective in the regulatory process through developing internal expertise for the review of patient preference information and patient reported outcomes, and holding a public workshop on how to incorporate such information in submissions and study design. FDA will also establish a pilot to audit and certify device testing laboratories for purposes of determining whether or not medical devices are in compliance with certain consensus standards. The agency will also tailor the third party device review program to clarify which device types are appropriate for third party review and to improve the quality of third party reviews. Finally, MDUFA IV also commits resources for pilot projects utilizing the National Evaluation System for health Technology, or NEST, which would be used to collect real world evidence data for purposes of supporting new indications for use, or new clearances and approvals.

C. Title III – Fees Relating to Generic Drugs

Title III puts forth the provisions of the Generic Drug User Fee Amendments of 2017 (GDUFA II). GDUFA II establishes a user fee schedule for generic drug applications to be paid by industry for the review of their applications and other activities. These fees are collected to ensure that FDA has the necessary resources and manpower to review applications in a timely manner.

There are two major modifications to the GDUFA user fee structure. First, annual program fees will replace individual application fees for sponsors with one or more approved ANDAs. This will improve predictability of the fee base and will better align sponsor fees with program costs. This annual fee schedule will be divided into three tiers of application holders: Large (companies with 20 or more approved ANDAs), Medium (companies with 6-19 approved ANDAs), and Small (companies with 5 or fewer approved ANDAs). Finished Dosage Form (FDF) and Active Pharmaceutical Ingredient (API) facilities will continue to pay annual fees as agreed upon in GDUFA I. Second, the Prior Approval Supplement (PAS) fee will be eliminated. Ultimately, 33 percent of the total revenue will come from application fees, 20 percent of the revenue will come from generic drug facility fees, 7 percent will come from active pharmaceutical ingredient facility fees, and 35 percent will come from a new generic drug applicant program fee. The base fee amount is updated from the FY2017 amount of $299,000,000 to $493,600,000 in FY2018, providing FDA with over $2.6 billion over the course of the five years of the agreement.

GDUFA II also establishes simplified review goals for ANDAs. FDA will review and act on 90 percent of standard generic applications within 10 months after the date of submission and 8 months for priority applications. An action means an approval, refuse-to-receive, complete response, withdrawal or denial. Priority status will be provided by FDA for submissions identified as eligible for expedited review. This currently includes applications for drugs in shortage, first generics, sole source generics, or drugs that meet a public health need. FDA has also committed in GDUFA II to increasing communication with sponsors of complex products through an optional pre-ANDA submission process to provide sponsors with greater clarity around regulatory expectations earlier in order to assist with the development of complete submissions. GDUFA II also increases transparency and communication between the agency and applicants during the review process through use of Information Requests (IRs) and Division Review Letters (DRLs). These communications will detail any deficiencies about an application to a sponsor around the midpoint of the review cycle so as to help decrease the number of review cycles.

Finally, GDUFA II also addresses the ANDA backlog. During GDUFA I, ANDA review goals were complex and difficult to operationalize. While it allowed FDA to organize the workload and make headway, it still resulted in a large gap between negotiated goals and stakeholder expectations. Despite this, FDA has met the goal of acting on 90 percent of the pre-GDUFA I backlog. Further, FDA has assigned a target action date (TAD) to ensure action on all applications in the pre-GDUFA I backlog by the end of 2017. All TADs will be converted to goal dates under GDUFA II with FDA committed to reviewing within the GDUFA II goal timeframes.

D. Title IV – Fees Relating to Biosimilar Biological Products

Title III puts forth the provisions of the Biosimilar User Fee Amendments of 2017 (BsUFA II). BsUFA II allows FDA to assess and collect fees for biosimilar products. BsUFA II it is proposed that an annual BsUFA program fee will be assessed for approved product. BsUFA II will retain the initial, annual, and reactivation biosimilar biological product development (BPD) fees while removing the

supplement fee and establishment fee. Sponsors will be limited to five BsUFA Program fees for a fiscal year application. HHS will determine the appropriate percentage of revenue that will come from each of the fees, and each fee amount annually. The base fee amount is updated from $20,000,000 in FY 2017 to $45,000,000 in FY2018. BsUFA II also maintains the existing reauthorization process. Performance and financial reports continue to be due to Congress every year while HHS is required to provide recommendations to Congress by January 15, 2022 after a series of public meetings.

E. Title V – Reauthorization of Other Programs

Section 501 reauthorizes HHS to grant exclusivity for drugs containing single enantiomers until October 1, 2022. Section 502 reauthorizes rules regarding the development of devices for rare pediatric conditions until October 1, 2022. Section 503 reauthorizes the Critical Path public-private partnership from October 1, 2018 until October 1, 2022 at current authorization levels. Section 504 reauthorizes FDA to issue grants to consortia working to develop devices for pediatrics at current authorization levels until October 1, 2022. Section 505 reauthorizes FDA to issue grants for orphan drug development until 2022. Section 506 reauthorizes funding for National Institutes of Health to conduct pediatric trials not being conducted by drug sponsors.

F. Title VI – Additional Provisions

Section 601 makes technical changes to H.R. 34, the 21st Century Cures Act (Public Law No. 114-255).