Prescription Drug User Fee Act (PDUFA) VII Reauthorization

Stakeholder Meeting with FDA | Meeting Summary

November 20, 2020 | 11:00am-12:45pm

Virtual Format (WebEx)

PURPOSE

To continue the process of FDA periodic consultation with representatives of patient and consumer advocacy groups, to discuss topics prioritized by patient and consumer participants, and to continue discussing their views on the reauthorization and their suggestions for changes to the user fee program performance goals.

Meeting Start Time: 11:00 AM

Enhancement and Modernization of the FDA Drug Safety System

After welcoming stakeholders, FDA kicked off the meeting by providing an overview of the PDUFA VI drug safety overarching goal, which is to continue to use user fees to enhance and modernize the current U.S. drug safety system. FDA then provided an overview of other commitments related to timely and effective evaluation and communication of postmarket safety findings. These commitments include supporting the review, oversight, and tracking of postmarket safety issues, making improvements to FDA's current process for capturing and tracking information, updating existing policies and procedures concerning tracking safety signals, and conducting an assessment of how its data systems and process support review and communication of safety issues. FDA also presented on how it provides the public with important information about the safety and availability of drug and biological products. FDA and stakeholder participants then had a follow-up discussion on topics including how FDA post-market processes address health disparities and representativeness and how new information and additional research affects FDA review of black box warnings.

Update on the Sentinel Initiative

FDA then provided a brief background on the history and impact of the Sentinel initiative, as well as an overview of the PDUFA VI goals and commitments related to Sentinel. These commitments included expanding sources of data and enhancing core capabilities, enhancing communication with sponsors and the public on methodologies for Sentinel queries, and facilitating public and sponsor access to Sentinel. The commitments also included holding a public meeting to engage stakeholders establishing policies and procedures to facilitate informing sponsors about the planned use of Sentinel, facilitating integration of Sentinel into the human drug review program, developing a comprehensive training program for review staff

on Sentinel, and analyzing and reporting on FDA's use of Sentinel for regulatory purposes. FDA also presented on the Active Risk Identification and Analysis (ARIA) system and the Biologics Effectiveness and Safety (BEST) Program.

FDA's Real-World Evidence (RWE) Program

FDA provided a brief background on RWE and real-world data (RWD), including relevant provisions in the 21st Century Cures Act and the commitments under PDUFA VI. These commitments included initiating appropriate activities to address key issues in the use of RWE for regulatory decision-making purposes and publishing draft guidance on how RWE can contribute to the assessment of safety and effectiveness in regulatory submissions. FDA presented on a wide range of topics related to its experience to date with RWE, including the FDA RWE framework, RWD fitness for use, regulatory considerations, RWE study design, the state of the science of RWE, engagement with stakeholders, demonstration projects, and guidance development. FDA concluded by stating that the RWE program is moving forward, ongoing efforts can identify attributes that promote generation of reliable and relevant RWD as well as valid RWE, and that alternative study designs can support and augment—but are not intended to replace—clinical trials for regulatory decision-making. FDA and stakeholder then had a follow-up discussion on topics including the link between database availability and potentially medically useful information, and how FDA currently is engaged on gaining insights from databases.

Wrap-Up and Topics for Upcoming Meetings

FDA stated that topics for the next meeting on December 11, 2020 would include recent work to modernize new drug review information infrastructure, an overview of the cell and gene therapy review programs, and a discussion of the financial aspects and enhancements in PDUFA reauthorization under FDARA.

Meeting End Time: 12:45 pm

PARTICIPANTS

Registered Public Stakeholders

Name	Organization	Attended
Michael Abrams	Public Citizen	No
Devon Adams	American Cancer Society Cancer Action Network, Inc.	No
Lynn Albizo	Immune Deficiency Foundation	Yes
Emily Anderson	Physicians Committee for Responsible Medicine	Yes
Elizabeth Baker	Physicians Committee for Responsible Medicine	Yes
David Balto	Coalition to Protect Patient Choice	No
Elizabeth Barksdale	LUNGevity Foundation	Yes
Andre Barlow	Coalition to Protect Patient Choice	No
Wendy Begolka	National Eczema Association	Yes
Cynthia Bens	Personalized Medicine Coalition	Yes

Abram Bielauskas	The ALS Association	Yes
Lauren Bloch	Lupus Foundation of America, the Crohn's & Colitis Foundation, and the Ara Parseghian Medical Research Fund.	Yes
Karin Bolte	American Pharmacists Association	Yes
Remy Brim	American Society of Gene and Cell Therapy	Yes
Sarah Buchanan	Crohn's & Colitis Foundation	No
Magdalena Bujar	CIRS - Centre for Innovation in Regulatory Science	No
Ryne Carney	Alliance for Aging Research	Yes
Emily Conron	Global Health Technologies Coalition	Yes
Kim Czubaruk	Cancer Support Community	Yes
David Davenport	Personalized Medicine Coalition	Yes
Ryan Fischer	Parent Project Muscular Dystrophy	No
Mark Fleury	American Cancer Society Cancer Action Network, Inc.	Yes
Betsy Foss-Campbell	American Society of Gene and Cell Therapy	Yes
Erin Frey	CureDuchenne	Yes
Eric Gascho	National Health Council (NHC)	Yes
Victoria Gemme	Cystic Fibrosis Foundation	Yes
Niles Godes	UsAgainstAlzheimer's	Yes
Jason Harris	Lupus Foundation of America	Yes
Kimberly Haugstad	N/A	Yes
Veronica Hood	Dravet Syndrome Foundation	No
Brenda Huneycutt	FasterCures	Yes
Bennie Johnson	JDRF	Yes
Joyce Johnson	American Osteopathic Association (AOA	Yes
Stephen Karpen	Critical Path Institute	Yes
Sean Kassen	Ara Parseghian Medical Research Fund	No
Samantha Kay	American Society of Gene and Cell Therapy	Yes
Annie Kennedy	EveryLife Foundation for Rare Diseases	Yes
Amanda Klein	Critical Path Institute	Yes
Ian Kremer	Leaders Engaged on Alzheimer's Disease (LEAD Coalition)	Yes
Melissa Laitner	Society for Women's Health Research	No
Debra Lappin	UsAgainstAlzheimer's	No
Trevan Locke	American Association for Cancer Research	Yes
Laura Maliszewski	Harvard-MIT Center for Regulatory Science	No
Paul Melmeyer	Muscular Dystrophy Association	Yes
Brittany Meyer	The Michael J. Fox Foundation	Yes
Steven Newmark	Global Healthy Living Foundation (GHLF	Yes
Russ Paulsen	UsAgainstAlzheimer's	No
Jason Resendez	LatinosAgainstAlzheimer's Coalition	No
Jon Retzlaff	American Association for Cancer Research	No
Leslie Ritter	National Multiple Sclerosis Society	Yes
Monica Ruse	Harvard – MIT Center for Regulatory Science	No
Sanjyot San-god-kar	Lupus Foundation of America	Yes
Kristen Santiago	LUNGevity Foundation	Yes
Kathleen Sheehan	The ALS Association	No

Rachel Sher	National Organization for Rare Disorders	Yes
Shimere Sherwood	Association for Clinical Oncology	Yes
Kanwaljit Sign	Critical Path Institute	Yes
Andrew Sperling	National Alliance on Mental Illness	Yes
Daniel Spirn	American Academy of Neurology	Yes
Laura Thornhill	Alzheimer's Association	No
James Valentine	Global Genes	Yes
Michael Ward	Alliance for Aging Research	Yes
Richard White	National Organization for Rare Disorders	Yes
Kael White	Critical Path Institute	No
Patrick Wildman	Lupus Foundation of America	No
Phylicia Woods	American Cancer Society Cancer Action Network, Inc.	No
Marc Yale	International Pemphigus and Pemphigoid Foundation (IPPF	No
Jill Yersak	The ALS Association	No

FDA

Robert Ball
Joshua Barton
Robyn Bent
Boris Brodsky
John Concato
Amanda Edmonds
Richard Forshee
Laura Lee Johnson
Andrew Kish
William Lewallen
Allison Lyndaker
Madabushi Rajanikanth
Theresa Mullin
Paul Phillips
Dionne Price
Sarah Riordan
Khushboo Sharma
Mary Ann Slack
Graham Thompson
Julia Tierney
Theresa Toigo
Patrick Zhou