Prescription Drug User Fee Act (PDUFA) VII Reauthorization

Stakeholder Meeting with FDA | Meeting Summary

October 30, 2020 | 9:00am-11:00am

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: 9:00 AM

Discussion on Patient Focused Drug Development (PFDD)

After welcoming stakeholders, FDA kicked off the meeting by providing an overview of the PDUFA VI goals and commitments related to PFDD and the progress made to date. These commitments included strengthening staff capacity, developing a series of guidance documents, creating and maintaining a repository of publicly available tools, revising internal processes to incorporate an increased patient focus in public meetings, and conducting a public workshop. FDA then discussed the current status of the methodological guidances, public workshops, and the repository of publicly available tools. FDA also shared information on some of its other ongoing efforts to sustain and improve integration of the patient's perspective . FDA and stakeholder participants then had a follow-up discussion on topics including how to further encourage industry engagement of patients in drug development, how to ensure that patient experience data submitted to FDA is "fit for purpose" and can be used in regulatory decision making, how to encourage use not only of PROs but also other COAs, and how to expand longitudinal collection of patient experience data.

Discussion on Model-Informed Drug Development (MIDD)

FDA then provided a brief background on MIDD, as well as an overview of the PDUFA VI goals and commitments related to advancing MIDD and the progress made to date. These commitments included developing regulatory science and expertise in MIDD, convening a series of workshops, conducting a pilot program for MIDD approaches, publishing draft guidance on MIDD, and revising internal processes to incorporate guidelines for evaluating MIDD. FDA shared its experience to date with the MIDD workshops, guidance documents, and pilot program, and other efforts to further advance MIDD. FDA and Stakeholders then had a follow-

up discussion on topics including converting the pilot to a full program and the value that the program has had.

Complex Innovative Designs for Clinical Trials (CID)
FDA provided a brief background on CID, including an overview of the PDFUA VI goals and commitments. These commitments included developing staff capacity to facilitate appropriate use of CID, conducting a pilot program for highly innovative trial designs, convening a public workshop, and publishing draft guidance on complex adaptive designs. FDA shared its experience and progress to date, including submissions accepted under the pilot program, conducting public workshops, and publishing guidance documents. FDA and Stakeholders then had a discussion on topics including whether the paired meeting pilot would continue, how to ensure adequate representation of under-represented populations in CID trials, the value of the CID program and potential benefits for drug development, getting representative populations for study cohorts, how innovative trial designs are still intended to be randomized well-run clinical trials and not a departure from the FDA's standards for trials, and how information about CID gets shared with the public.

Discussion on Other Areas of Regulatory Science: Advancing Translational Models & Tools (ATMT)
The final topic area discussed, ATMT, was raised by FDA as a potential area of interest under PDUFA VII. FDA provided an overview of its Division of Applied Regulatory Science, the type of work it covers, and its goal to move new science into drug review and close the gap between scientific innovation and drug review. FDA then provided examples of some of the projects being worked on by this division, and how additional enhancements might be able to move this area forward. FDA and Stakeholders then had a brief follow-up discussion on topics such as the resources currently available and what might be most helpful for this area.

Wrap-Up and Topics for Upcoming Meetings
FDA noted that in follow up to the initial kickoff meeting, a survey was emailed to stakeholders to obtain their priority ranking of the agreed short list of already identified topics and to obtain input on any additional topics of interest that were not included in the discussion. FDA stated that these responses were used in order to identify topics for upcoming stakeholder meetings.

Meeting End Time: 11:00 am

PARTICIPANTS

Registered Public Stakeholders

Name	Organization	Attended
Michael Abrams	Public Citizen	Yes
Devon Adams	American Cancer Society Cancer Action Network, Inc.	Yes
Lynn Albizo	Immune Deficiency Foundation	No
Emily Anderson	Physicians Committee for Responsible Medicine	Yes

Elizabeth Baker	Physicians Committee for Responsible Medicine	No
David Balto	Coalition to Protect Patient Choice	No
David Balto	dcantitrustlaw.com	Yes
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.	No
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	Yes
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	Yes
Mark Fleury	American Cancer Society Cancer Action Network, Inc.	Yes
Betsy Foss-Campbell	American Society of Gene and Cell Therapy	No
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	No
Bennie Johnson	JDRF	Yes
Joyce Johnson	American Osteopathic Association (AOA	Yes
RADM Joyce Johnson	The American Osteopathic Association (AOA)	No
Stephen Karpen	Critical Path Institute	Yes
Sean Kassen	Ara Parseghian Medical Research Fund	No
Samantha Kay	American Society of Gene and Cell Therapy	No
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	Yes
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	No

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

FDA

Joshua Barton
Boris Brodsky
Dat Doan
Amanda Edmonds
Laura Lee Johnson
Chris Joneckis
Andrew Kish
William Lewallen
Allison Lyndaker
Rajanikanth Madabushi
Theresa Mullin
Dionne Price
Khushboo Sharma
Mary Ann Slack
David Strauss
Graham Thompson
Theresa Toigo
Patrick Zhou