



Prescription Drug User Fee Act (PDUFA) VII Reauthorization

Stakeholder Meeting with FDA | Meeting Summary

September 25, 2020 | 11:00am-1:00pm

Virtual Format (WebEx)

PURPOSE

To initiate the process of FDA periodic consultation with representatives of patient and consumer advocacy groups to continue discussions of their views on the reauthorization and their suggestions for changes to the user fee program performance goals in follow up to the initial public meeting discussion.

Meeting Start Time: 11:00 AM

Welcome and FDA Introductions

FDA kicked off the meeting by welcoming stakeholders and laying out the purpose of these meetings as part of the reauthorization provisions, as specified in statute. These meetings are meant to continue the discussions of stakeholder perspectives that began at the July 23, 2020 public meeting. Reauthorization of PDUFA focuses on enhancements to the drug review process, not FDA policy.

Background on PDUFA and Review of Stakeholder Perspectives

FDA provided a brief historical perspective on user fee legislation for prescription drugs and highlighted the commitments and goals incumbent upon FDA as a result of PDUFA VI. FDA reviewed its performance to date related to the metric goals and commitments and commented on the successes achieved. FDA overviewed the impact of the COVID-19 pandemic, including receiving a significant amount of new drug development programs and trials since the emergency began. FDA presented a summary of perspectives on PDUFA VII by patient advocates, consumer advocates, healthcare professionals, and representatives from regulated industry that were shared at the July 23, 2020 public meeting and in the docket.

Stakeholder Introductions and Topics

A representative from each stakeholder organization offered an introduction and highlighted their group's primary topics pertaining to the PDUFA VII. Some of the themes frequently cited by stakeholders included enhancing the incorporation of patient voice in drug development and regulatory decision making, modernizing FDA's infrastructure, ensuring FDA has adequate resources to recruit and retain qualified staff including in the areas of cell and gene therapy, increasing the strength and reach of patient and rare disease programs including improving

diversity in clinical trials and patient engagement, enhancing FDA’s use of regulatory science (e.g. COAs, MIDD, RWE), and improving the integration of and guidance for the use of real-world evidence (RWE). The sets of topics identified by different stakeholders were then discussed and consolidated into a short list of topics that had been most commonly cited.

Wrap-Up and Overview of Future Meetings

FDA requested that stakeholders indicate areas of greatest interest for further discussion at future meetings based on the areas identified by stakeholders during the meeting as well as the input received in the July 2020 meeting summarized by the FDA. In addition to the areas related to user fee program goals, stakeholders identified topics including decentralized trials and COVID-19 lessons learned.

FDA noted that in follow up to the meeting, a survey would be 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. The responses to this survey would be used by FDA to plan for topics for discussion in future meetings.

Meeting End Time: 12:57 pm

PARTICIPANTS

Registered Public Stakeholders

Michael Abrams	Public Citizen	Attended
Devon Adams	American Cancer Society Cancer Action Network, Inc.	No
Emily Anderson	Physicians Committee for Responsible Medicine	No
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	Yes
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	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
Victoria Gemme	Cystic Fibrosis Foundation	Yes
Niles Godes	UsAgainstAlzheimer's	Yes
Jason Harris	Lupus Foundation of America	Yes
Kimberly Haugstad	Global Genes	Yes
Veronica Hood	Dravet Syndrome Foundation	No
Brenda Huneycutt	FasterCures	Yes
Bennie Johnson	JDRF	Yes
Joyce Johnson	American Osteopathic Association (AOA)	No
Stephen Karpen	Critical Path Institute	Yes
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	Yes
Debra Lappin	UsAgainstAlzheimer's	No
Treva 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)	(Zoe on behalf)
Russ Paulsen	UsAgainstAlzheimer's	No
Jason Resendez	LatinosAgainstAlzheimer's Coalition	No
Jon Retzlaff	American Association for Cancer Research	Yes
Leslie Ritter	National Multiple Sclerosis Society	Yes
Sanjyot San-god-kar	Lupus Foundation of America	No
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	No
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	Yes
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

Joshua Barton
Jason Bunting
Meghana Chalasani
Amanda Edmonds
Christopher Joneckis
Andrew Kish

William Lewallen
Theresa Mullin
Paul J. Phillips
Carol Rehkopf
Sarah Riordan
Mary Ann Slack

Graham Thompson
Theresa Toigo
Yonatan Tyberg
Patrick Zhou