



# Prescription Drug User Fee Act (PDUFA) VII Reauthorization

## Stakeholder Meeting with FDA | Meeting Summary

February 19, 2021 | 10:00am-11:30am

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. The stakeholder meeting on January 15<sup>th</sup> included three topic areas for discussion based on the priority topics identified by the public stakeholders in the initial consultation meeting in September 2020 and in subsequent meetings. The two topics included:

- Review of Basis for and Management of User Fee Funded Drug Review
- Themes and Highlights of Anticipated Set of PDUFA VII Proposed Recommendations

**Meeting Start Time:** 10:00 AM

### Review of Basis for and Management of User Fee Funded Drug Review

After welcoming stakeholders, FDA kicked off the meeting with a short presentation from the CDER Office of Strategic Programs providing an overview of the user fee process. Key elements of the user fee process discussed included how Congress directed FDA to establish a user fee program for human drug review, how fee funds are added to non-fee appropriated funds, and that funds are intended to increase staffing and other resources to speed and enhance the review process. Finally, FDA provided an overview of what topics are included in user fee discussions with industry, including new and enhanced processes and resources needs, and not including discussions of policy substance.

FDA and participants then engaged in a discussion based on questions posed by public stakeholders related to this topic, including on how FDA distinguishes process items from policy items in the fee discussions. FDA explained that while industry can request that FDA describe or explain its requirements or how it thinks about various policy issues, the fee discussions cannot include attempts to shape the agency's regulatory policy .

### Themes and Highlights of Anticipated Set of PDUFA VII Proposed Recommendations

FDA first provided an overview of the overall themes of the PDUFA VII recommendations. FDA emphasized that the recommendations were not final and still required ratification.

CBER-specific recommendations included enhancing CBER's capacity to guide development and review innovative products such as cell and gene therapies. This includes additional staff capacity and capability to support development and review of such therapies, as well as support for engagement with key stakeholders.

Pre-market recommendations focused on introducing new approaches to improve efficiency and expand communication in the human drugs review program. This includes new processes for postmarketing requirements, new meeting types during the drug development process, a pilot program to advance rare disease development, and a pilot program to improve quality and acceptability of real-world evidence.

Regulatory decision tool recommendations aimed to continue application of innovative methods and tools to enhance regulatory decision-making. This includes continuing to advance incorporation of the patient voice in drug development, continuing the model-informed drug development paired meeting program, and continuing the complex innovation trial designs paired meeting program.

Manufacturing recommendations focused on facilitating manufacturing readiness and use of innovative manufacturing technologies, including enhancement of communication during application review.

Post-market recommendations focused on ensuring safe use of medicines through continued enhancements to our drug safety system, including modernization of REMS activities and continued support of the Sentinel initiative.

Digital health and informatics recommendations focused on leveraging modern technology and bioinformatics to support enhanced and streamlined drug development and review. This includes enhancing use of digital health technologies, enhancing transparency, and providing additional resources and staff capacity.

Other recommendations highlighted by FDA included enhancing financial management and transparency and focusing on the strategic hiring and retention of world-class technical and scientific staff.

FDA also provided an overview of next steps in the fee discussion and clearance process, which include review and input by the Department of Health and Human Services, the Office of Personnel Management, and others in the Administration.

FDA and stakeholder participants engaged in a discussion based on questions posed by public stakeholders, including how public stakeholders can provide input on the proposed recommendations. FDA noted that after review and clearance, following the statutory

provisions for PDUFA reauthorization, the proposed recommendations would be published in the Federal Register for public comment, and FDA would also conduct a public meeting to obtain public input on the proposed performance commitments related to program reauthorization. FDA would make any further revisions based on this public input.

### Wrap-Up

FDA stated that as user fee discussions have concluded, this was the final public stakeholder meeting. FDA thanked participants for their input throughout the periodic consultation process and encouraged submissions further comments to the docket when the proposed recommendations are published in the Federal Register.

**Meeting End Time:** 11:30 am

## PARTICIPANTS

### Registered Public Stakeholders

<b>Name</b>	<b>Organization</b>	<b>Attended</b>
Michael Abrams	Public Citizen	Yes
Devon Adams	American Cancer Society Cancer Action Network, Inc.	Yes
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	LUNGevery 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	No
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	No
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	No
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	Yes
Erin Frey	CureDuchenne	No
Eric Gascho	National Health Council (NHC)	No
Pamela Gavin	National Organization for Rare Diseases	No

Victoria Gemme	Cystic Fibrosis Foundation	Yes
Niles Godes	UsAgainstAlzheimer's	Yes
Jason Harris	Lupus Foundation of America	No
Kimberly Haugstad	N/A	No
Veronica Hood	Dravet Syndrome Foundation	No
Brenda Huneycutt	FasterCures	No
Lihamm Jaffer	American Cancer Society Cancer Action Network, Inc.	No
Bennie Johnson	JDRF	No
Joyce Johnson	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	Yes
Annie Kennedy	EveryLife Foundation for Rare Diseases	Yes
Amanda Klein	Critical Path Institute	No
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	No
Laura Maliszewski	Harvard-MIT Center for Regulatory Science	Yes
Marjana Marinac	JDRF	Yes
Paul Melmeyer	Muscular Dystrophy Association	Yes
Brittany Meyer	The Michael J. Fox Foundation	No
Gabriel Miller	American Osteopathic Association	Yes
Steven Newmark	Global Healthy Living Foundation	Yes
Sarah Parvanta	ALS Association	Yes
Russ Paulsen	UsAgainstAlzheimer's	No
Julien Rashid	Global Health Technologies Coalition	Yes
Jason Resendez	LatinosAgainstAlzheimer's Coalition	No
Jon Retzlaff	American Association for Cancer Research	No
Leslie Ritter	National Multiple Sclerosis Society	No
Joe Rothblatt		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	No
Andrew Sperling	National Alliance on Mental Illness	Yes
Daniel Spirn	American Academy of Neurology	No
Mark Stewart	Friends of Cancer Research	No
Laura Thornhill	Alzheimer's Association	No
James Valentine	Global Genes	No
Michael Ward	Alliance for Aging Research	No
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**

Jason Bunting  
Meghana Chalasani  
Amanda Edmonds  
Laura Lee Johnson  
Andrew Kish  
William Lewallen  
Rajanikanth Madabushi  
Janet Maynard  
Theresa Mullin  
Paul Phillips  
Sarah Riordan  
Mary Thanh Hai  
Graham Thompson  
Theresa Toigo  
Patrick Zhou