Prescription Drug User Fee Act (PDUFA) VII Reauthorization

Stakeholder Meeting with FDA | Meeting Summary

January 15, 2021 | 12:30pm-2:30pm

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 15th included three topic areas for discussion based on the priority topics identified by the public stakeholders in the initial consultation meeting in September 2020. The three topics included:

- Enhancing Diversity in Clinical Trials
- Digital Health Technology and Decentralized Clinical Trials
- Strength and reach of rare disease programs in patient engagement and encouraging greater data sharing

Meeting Start Time: 12:30 PM

Enhancing Diversity in Clinical Trials

After welcoming stakeholders, FDA kicked off the meeting with a presentation from the Office of Medical policy providing a regulatory perspective on enhancing diversity of clinical trial populations. FDA provided an overview of recent progress in this area, including a public meeting held in 2018, draft guidance published in 2019 titled *Enhancing the Diversity of Clinical Trial Populations, Eligibility criteria, Enrollment Practices, and Trial Designs*, and the final version of this guidance published in 2020.

FDA then provided an overview of its recommendations on inclusive trial practices. These included but were not limited to: developing new representative eligibility criteria for each trial; enrolling participants from clinically relevant populations; using adaptive clinical trial designs; considering inclusion of a broader participant group even in enriched clinical trials; making trials less burdensome; using more inclusive strategies for public outreach and education; designing clinical trial protocols along with patients, advocates, and caregivers; and engaging with rare disease patients and their advocates early in the trial design process. FDA and participants then held a discussion based on questions posed by public stakeholders related to this topic including: how representation affects requirements for powering trials to be able to detect signals; how operational burden related to representativeness can be reduced; how patients

and advocacy groups can get involved in this effort; and what kind of future guidance or research might be expected in this area. FDA noted that interested parties who wished to further discuss and be involved in this area should reach out to the FDA Office of Minority Health and the Office of Patient Affairs.

Digital Health Technology and Decentralized Clinical Trials

FDA provided a brief history of FDA's efforts to support technology in clinical trials, including digital health technologies (DHTs), which include technologies that use computing platforms, connectivity, software, or hardware, including sensors for health care and related uses. FDA also provided an overview of the most important characteristics of DHTs from a regulatory perspective, including a description of the DHT, verification in the laboratory, validation in the field, usability determination, whether or not patients are using their own device, and capturing endpoints using DHTs. FDA also discussed some of the challenges related to determining whether the quality of the evidence collected from DHTs in clinical trials is adequate to support regulatory decision making.

FDA then presented on decentralized clinical trials (DCTs), which are clinical investigations where some or all trial-related activities take place at locations remote from the investigator. FDA discussed possible benefits of such trials, including patient convenience, ability to recruit and study patients in widespread locations, and gaining more and potentially better data during the trial. FDA then discussed various aspects of DCT design and conduct, including trial setup, remote study visits, oversight, safety, shipment of medical products, and inspection. FDA concluded that digital trial technologies and decentralized trials may change the way trials are done due to the potential advantages of these approaches for patient convenience, inclusivity, continuous monitoring, and trial efficiency. FDA also noted that this would require thoughtful dialogue with drug developers, patients, disease experts, and regulators to advance these approaches in a judicious way.

FDA and participants then held a follow-up discussion based on questions posed by public stakeholders related to this topic including how these DHTs are developed and validated by industry; how data is captured and tracked by DHTs and how proprietary design and analyses factor into that data collection; whether DHTs might be able to capture patient experience data as well as outcomes; and how patient input can help with design of DHTS.

Strength and Reach of Rare Disease Programs in Patient Engagement and Encouraging Greater Data Sharing

FDA has a number of programs and initiatives under way to help support rare disease drug development and was able to present on two of these efforts, namely the efforts of the FDA Office of Orphan Products (OOPD) and those under way as part of the FDA Rare Disease Cures Accelerator (RDCA).

FDA began with an overview of the work of OOPD which includes rare disease product designations, including Orphan Drug Designation (ODD) (and Exclusivity), Rare Pediatric Disease (RPD) Designation, and Humanitarian Use Device (HUD) Designation, and OOPD IT

modernization to support this work. FDA provided an overview of the wide range of its internal and external outreach, such as the orphan products policy council, the rare disease council, meetings with other regulatory agencies, patient listening sessions, and public meetings. FDA also discussed the orphan products grants program, which has a budget of $17.7M with a goal of advancing the development of orphan products (drugs, biologics, devices, or medical foods) that demonstrate promise for the diagnosis or treatment of rare diseases or conditions. FDA stated that while efforts to develop products in this area are ongoing, developing rare disease treatments is challenging, the vast majority of rare diseases currently do not have approved treatments, and the key to further advancement is to have collaboration and patient involvement.

FDA then provided an overview of the rare diseases cures accelerator (RDCA) and key activities to address areas of major challenge for the development of drugs for rare diseases. These challenges include characterization of disease, getting patient perspectives on their disease and treatment and developing meaningful clinical outcome measures and endpoints, and the design and conduct of clinical studies for new treatments for rare disease populations. FDA stated that there is a compelling need for centralized and standardized infrastructure to support and accelerate rare disease characterization, including a rare disease natural history study data platform. FDA then provided an overview of the RDCA Data and Analytics Platform, developed by the Critical Path Institute with collaboration from the National Organization for Rare Diseases and supported by a cooperative agreement with FDA, to promote the sharing of critically important data across rare diseases in order to accelerate the understanding of disease progression and optimize clinical trial design.

Finally, FDA presented on its grant program to support development of standard core Clinical Outcome Assessments (COA) and their related endpoints. FDA stated that the standard core COA and endpoints pilot grants aim to help address the lack of coordination of COA development across researchers and sponsors within a given disease area, noting that reviewers currently may see multiple independent efforts with duplication of effort and variable quality of tools and resulting data. The goal of this program is to ensure development of standard core sets of measures of disease burden and treatment burden for a given area, that FDA considers fit for purpose and that will be publicly available to all drug developers at nominal or no cost. FDA provided a brief overview of grants it has been funding in this area with a particular focus on those supporting development of drugs for rare disorders.

FDA and participants then had a follow-up discussion based on questions posed by public stakeholders related to this topic including how patient groups, coalitions, and independent registries can be supportive of these efforts and what opportunities for coordination with FDA exist. FDA discussed how it is currently looking at how to integrate data from external sources into these efforts, and that this was a topic that may benefit from further discussion at a future public workshop that could focus on issues related to further advancement of patient focused drug development.

Wrap-Up and Topics for Upcoming Meetings

FDA then solicited suggestions on topics for the next meeting on February 19, 2021. Participants identified a range of topics, including further information on how patient experience data can be collected, measured, and communicated, the provisions FDA has in place to ensure avoidance of conflict of interest with this user fee-supported review program, and sharing of information related to what is expected to be included in the proposed recommendations for program enhancement from the PDUFA VII discussions with regulated industry.

Meeting End Time: 2:30 pm

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	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	No
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	No
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	No
Erin Frey	CureDuchenne	Yes
Eric Gascho	National Health Council (NHC)	Yes
Pamela Gavin	National Organization for Rare Diseases	
Victoria Gemme	Cystic Fibrosis Foundation	Yes
Niles Godes	UsAgainstAlzheimer's	Yes
Jason Harris	Lupus Foundation of America	No

Kimberly Haugstad	N/A	Yes
Veronica Hood	Dravet Syndrome Foundation	No
Brenda Huneycutt	FasterCures	Yes
Lihamm Jaffer	American Cancer Society Cancer Action Network, Inc.	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	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	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	No
Sarah Parvanta	ALS Association	No
Russ Paulsen	UsAgainstAlzheimer’s	No
Jason Resendez	LatinosAgainstAlzheimer's Coalition	Yes
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	No
Andrew Sperling	National Alliance on Mental Illness	Yes
Daniel Spirn	American Academy of Neurology	Yes
Mark Stewart	Friends of Cancer Research	Yes
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	Yes
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

Robyn Bent
Michelle Campbell
Meghana Chalasani
Amanda Edmonds
Jamie Gamerman
Laura Lee Johnson
Christopher Joneckis
William Lewallen
Allison Lyndaker
Janet Maynard
Theresa Mullin
Leonard Sacks
Khushboo Sharma
Mary Ann Slack
Mary Thanh Hai
Graham Thompson
Julie Tierney
Theresa Toigo
Patrick Zhou