



Prescription Drug User Fee Act (PDUFA) VII Reauthorization

Stakeholder Meeting with FDA | Meeting Summary

December 11, 2020 | 10:30am-12:30pm

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.

Meeting Start Time: 10:30 AM

CBER Cell and Gene Therapy Review Programs

After welcoming stakeholders, FDA kicked off the meeting with a presentation from the Office of Tissues and Advanced Therapies (OTAT) providing a regulatory perspective of the CBER cell and gene therapy review programs. FDA provided an overview of what constitutes cell and gene therapies, as well as examples of approved therapies under these programs and the scientific advances are spurring growth in research and development (R&D) of advanced therapies.

FDA also presented information on the cell and gene therapy program resources and CBER modernization. FDA provided an overview and projection of expected resource needs for this program based on the current trajectory of volume of cell and gene therapy development work include including resources to address the current stress and strain on the cell and gene therapy program, sustain the regenerative medicine advanced therapies program, prepare for increased postmarket workload, enhance outreach and guidance development, and regulatory science to support novel technologies. FDA also provided an overview of CBER modernization efforts, including need for IT modernization.

PDUFA Financial Status Update

FDA provided a brief background on financial changes implemented in PDUFA VI and the financial status of the current PDUFA program. This includes a revised user fee structure and development and implementation of resource capacity planning (RCP) including workload projection models and full time reporting by FDA staff to inform the RCP model. FDA presented on RCP development milestones achieved to date, and how it relates to capacity planning adjustments. FDA concluded that these changes have helped enhance financial

predictability, minimize inefficiency, and improve FDA’s ability to manage program resources and engage in effective long-term planning.

CDER Modernization - New Drug Review Information Infrastructure & Reviewer Talent Management

FDA provided an overview of FDA’s New Drug Regulatory Program Modernization initiative and its strategic objectives. This effort was undertaken to address the rapid and sustained growth in the volume of drug development activity, the increased complexity of innovative therapies under development, greater availability of observational and other “real world” data, increased public engagement in FDA activity, persistent budget constraints and perennial talent shortage. FDA outlined six goals for the effort including: scientific leadership, integrated assessment, benefit-risk monitoring, managing talent, operational excellence and knowledge management. The presentation highlighted recent modernization efforts in the integrated assessment of marketing applications, internal efforts to identify knowledge management priorities, and efforts to better develop and manage talent including a recent pilot program for reviewer development and assessment.

Wrap-Up and Topics for Upcoming Meetings

FDA shared that topics for the next meeting on January 15, 2021 would include the remaining ones identified and prioritized by public stakeholders following the initial meeting in September 2020. These include an overview of FDA efforts to make clinical trials more inclusive and diverse, recent FDA guidance on the use of decentralized clinical trials and other potential clinical trial flexibilities, and an overview of the strength and reach of patient and rare disease programs, including rare disease cures accelerator to improve diversity in patient engagement and encourage greater data sharing.

Meeting End Time: 12:30 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 | No |
| 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 |

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| 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 | Yes |
| 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 | Yes |
| Eric Gascho | National Health Council (NHC) | Yes |
| Victoria Gemme | Cystic Fibrosis Foundation | No |
| 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) | No |
| Melissa Laitner | Society for Women's Health Research | No |
| 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 |
| Gabriel Miller | American Osteopathic Association | Yes |
| Steven Newmark | Global Healthy Living Foundation | Yes |
| Sarah Parvanta | ALS Association | 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 |

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| 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 | 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

Joshua Barton
 Robyn Bent
 Wilson Bryan
 Kevin Bugin
 Jason Bunting
 Meghana Chalasani
 Dat Doan
 Laura Lee Johnson
 Christopher Joneckis
 Andrew Kish
 William Lewallen
 Allison Lyndaker
 Theresa Mullin
 Khushboo Sharma
 Mary Ann Slack
 Mary Thanh Hai
 Graham Thompson
 Patrick Zhou