AMCP
Optimizing medicine.
Improving lives.

October 28, 2021

Lauren K. Roth
Acting Principal Associate Commissioner for Policy
Food and Drug Administration
Department of Health and Human Services
10903 New Hampshire Avenue
Silver Spring, MD 20993

Re: Reauthorization of the Prescription Drug User Fee Act; Public Meeting; Request for Comments [FDA-2021-N-0891]

Dear Acting Commissioner Roth,

AMCP thanks the Food and Drug Administration (FDA) for the opportunity to provide comments on the "*Reauthorization of the Prescription Drug User Fee Act; Public Meeting; Request for Comments."* We appreciate the opportunity to leverage our members' expertise in offering feedback on the proposed commitment letter for the reauthorization of the Prescription Drug User Fee Act (PDUFA), particularly on the issues of the use of real-world evidence for regulatory decision-making and on cell and gene therapies.

AMCP's diverse membership of pharmacists, physicians, nurses, biopharmaceutical professionals, and other stakeholders leverage their specialized expertise in clinical evidence and economics to optimize medication benefit design and population health management and help patients access cost-effective and safe medications and other drug therapies. AMCP members improve the lives of nearly 300 million Americans served by private and public health plans, pharmacy benefit management firms, and emerging care models.

K. Enhancing Regulatory and Expediting Drug Development – Advancing Real-World Evidence [RWE] for Use in Regulatory Decision-Making

This section describes the establishment of a pilot program, the "Advancing RWE Program," intended to identify approaches to generating RWE that "meet regulatory requirements in support of labeling for effectiveness (e.g., new indications, populations, dosing information) or for meeting post-approval study requirements."

> *AMCP Comment:* AMCP supports the FDA's pursuit of identifying approaches to using RWE to meet regulatory requirements for labeling and post-approval study requirements, particularly for drugs approved through accelerated approval pathways. As such, we are encouraged by the establishment of this pilot program.

While we believe the results of a pilot program such as this would help to encourage the collection of this valuable data, AMCP does not believe that submission of this type of evidence should be in lieu of the completion of confirmatory clinical trials, unless directed by the FDA. The completion of confirmatory trials is critical to ensure safe and appropriate access to treatments for patients, while other types of real-world evidence can help bridge the gap from the launch of a drug approved through accelerated pathways and confirmatory trial completion, which can take several years.

FDA should consider taking further action to ensure the completion of confirmatory studies for drugs approved through accelerated pathways, such as by requiring the sunsetting of market authorization for an accelerated approval drug after a specified amount of time, set at the time of approval, in the event that confirmatory evidence not be available to the FDA for review. A formal sunsetting policy would serve to incentivize drug manufacturers to complete confirmatory studies and help to protect the FDA from external pressure to change market withdrawal decisions. A system for determining whether to grant extensions of this timeframe for legitimate reasons, such as needing to resolve scientific or clinical issues with confirmatory trials, should be contemplated in order to eliminate the possibility of withdrawing market authorization prematurely.[1]

O. Enhancing CBER's Capacity to Support Development, Review, and Approval of Cell and Gene Therapy Products

This section proposes to strengthen staff capacity in the Cell and Gene Therapy Program (CGTP) at the Center for Biologics Evaluation and Research (CBER) in order to meet the increasing demands for and challenges in reviewing and approving emerging cell and gene therapies.

AMCP Comment: AMCP strongly supports this proposal to strengthen staff capacity in the CGTP. As more of these complex treatments are researched and developed and come to the FDA for approval, CGTP needs to be fully resourced in order to meet the demand to get these promising treatments to patients. Cell and gene therapies provide highly individualized treatments to patients and have the potential to address many diseases for which no treatment currently exist, which is why it remains imperative for CGTP to be prepared to meet increasing demands.

Conclusion

AMCP appreciates the opportunity to comment on the "*Reauthorization of the Prescription Drug User Fee Act; Public Meeting; Request for Comments [FDA-2021-N-0891]*" commitment letter. We are committed to be being a valuable resource to FDA on improving access to prescription drugs at

[1] https://icer.org/wp-content/uploads/2021/04/Strengthening-the-Accelerated-Approval-Pathway-_-ICER-White-Paper-_-April-2021.pdf

lower costs. If you have any questions regarding AMCP's comments or would like further information, please contact me at 703-684-2600 or scantrell@amcp.org.

Sincerely,

Susan A. Cantrell. RPh, CAE
Chief Executive Officer

675 N Washington Street| Suite 220
Alexandria, VA 22314

703 684 2600 | www.amcp.org |@amcporg