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October 16, 2026

Division of Dockets Management
Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

**RE: Prescription Drug User Fee Amendments (PDUFA VIII); Public Meeting;
Request for Comments [FDA-2026-N8163]**

Dear Sir/Madam:

The Personalized Medicine Coalition (PMC), an education and advocacy organization comprised of more than 150 institutions from across the health care spectrum, appreciates the opportunity to provide insights on the importance of the Prescription Drug User Fee program to personalized medicine and to highlight how the agreement reflects PMC's priorities for the reauthorization of the Prescription Drug User Fee Amendments (PDUFA) for fiscal years 2028 through 2032.¹

Personalized medicine is an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By combining data from diagnostic tests with an individual's medical history, circumstances, and values, health care providers can develop targeted treatment and prevention plans with their patients. Personalized medicine is helping to shift the patient and provider experiences away from trial-and-error toward a more streamlined process for making clinical decisions, which will lead to improved patient outcomes, a reduction in unnecessary treatment costs, and better patient and provider satisfaction.

Personalized medicine-related developments at the U.S. Food and Drug Administration (FDA) in 2025 show that scientific innovation continues to move the health system toward the utilization of individual patient information to improve patient outcomes and make clinical care more efficient. A well-resourced, focused, transparent, and flexible FDA is essential to achieving PMC's mission of bringing forward the best treatment and prevention strategies for each patient. User fees supplement the agency's budget authority from Congress and ensure the FDA has the resources and workforce needed to complete the timely evaluation of personalized medicine products while upholding the agency's high standards for ensuring safety and effectiveness. Without this funding, many FDA activities that support personalized medicine would lag the technological progress we are seeing.

Statement of Neutrality

Many of PMC's members will present their own responses to the FDA's request for comments on the Prescription Drug User Fee Amendments (PDUFA VIII) and will actively advocate for those positions. PMC's comments are designed to provide feedback so that the general concept of personalized medicine can advance and are not intended to impact adversely the ability of individual PMC members, alone or in combination, to pursue separate comments with respect to PDUFA VIII.

Importance of the PDUFA Program to Personalized Medicine

User fee-funded activities now account for more than three-quarters of the agency's drug review budget. The PDUFA program is a critical mechanism that has helped improve the timeliness of drug reviews, encourage innovation in drug and biologic development, and promote initiatives at the FDA that leverage the best science. As in previous reauthorization cycles, PMC has engaged at multiple points throughout the negotiations and will engage in the legislative process to ensure that the FDA is well-resourced and can continue to review and approve lifesaving products.

Research on the genetic and biological underpinnings of disease have contributed to development of more than 300 personalized treatments and over 140,000 diagnostic products benefiting patients today. PMC's analyses have shown that personalized medicines account for more than a quarter of all new drugs approved annually by the FDA. That number has steadily increased from 5 percent in 2005 when PMC started evaluating annual personalized medicine approvals. More than half of new personalized treatments approved recently have been for indications outside of oncology.ⁱⁱ

Initiatives advanced by the FDA in recent years have fostered many notable regulatory firsts including the approval of the first cell and gene therapies, n-of-one therapies, and tissue-agnostic therapies. We believe that enhancements included in the PDUFA VIII agreement will allow for continued progress in advancing personalized medicine and that the PDUFA program will continue yielding benefits for a wide range of patients, including those with unmet medical needs.

Continued Support for Provisions in PDUFA VIII

PDUFA VII meaningfully built upon commitments made under previous authorizations, particularly in the areas of regulatory science, real-world evidence (RWE), digital health technologies (DHTs), and patient engagement. We appreciate the FDA's efforts to enhance reviewer training on patient experience data, facilitate externally led Patient-Focused Drug Development meetings, and provide guidance supporting the development and submission of clinical outcome assessments (COAs).

At the beginning of the PDUFA VIII negotiations and throughout the monthly stakeholder consultation process, PMC offered suggestions to the FDA on proposed enhancements for the PDUFA program that would bolster the progress already made through PDUFA VII pilot programs for generating data, improving sponsor interaction with the agency, implementing complex innovative trial designs, and expanding guidance on DHTs and COAs. We further encouraged negotiations focused on consistency in review practices across FDA divisions, increased transparency and communication, structure for clinical outcome assessment development, and utilization of primary disease biomarkers as acceptable endpoints. **PMC is encouraged to see many of these recommendations embedded in the current PDUFA VIII agreement.**

Enhancing Consistency in Review Practices Across FDA Divisions

Many divisions within the Center for Drug Evaluation and Research (CDER) and the Center for Biologics Evaluation and Research (CBER) generally employ high-quality review practices that guide and assist sponsors through the regulatory process. New reviewers often lack the necessary experience to review certain specialized products and regulatory approaches whereas reviewers in some therapeutic areas, such as oncology and rare disease, have broader experience with innovative regulatory science approaches. There is a need for these innovative approaches to be well understood and supported by reviewers in non-rare and chronic disease therapeutic areas as well. **To support a more consistent regulatory approach across CDER and CBER, PMC recommended that the FDA strengthen its reviewer training activities by increasing reviewer education on topics such as novel statistical approaches, innovative clinical trial designs, use of RWE in regulatory decision-making, assessing patient experience data, and combination products. The PDUFA VIII agreement commits FDA to ongoing reviewer training across these domains.**

PMC supports the PDUFA VIII agreement’s emphasis on transparency regarding RWE that contributes to regulatory decisions. Aggregate annual reporting can help stakeholders understand where RWE is being used and the types of methods that have been successful, while links to relevant public review documents can provide additional context for individual approvals. This approach can improve shared learning without compromising confidential commercial information and may be particularly useful to smaller sponsors and academic investigators that do not have experience across a large portfolio of FDA interactions. Where RWE is used as confirmatory evidence alongside a pivotal investigation, as external or supplemental evidence in rare disease development, or to support post-approval requirements, the same principle should apply.

With respect to patient experience data, the systematic approach to collecting and using robust and meaningful patient and caregiver experiences, perspectives, needs, and priorities to inform drug development and regulatory decision-making, FDA made several commitments. Under PDUFA VIII, FDA would continue raising awareness regarding how patient experience data can be submitted with an NDA or BLA and considered in regulatory decision-making, including labeling; to publish case studies at least twice by the end of FY 2032; to convene a public meeting by the end of FY 2030; to continue internal and external training; and to maintain a brief public statement in review documents regarding patient experience data submitted and reviewed with an application. **PMC supports these commitments because they respond directly to a recurring challenge of when particular forms of patient experience data are sufficiently relevant and fit for purpose for a specific regulatory question.**

Under PDUFA VIII, FDA would also transition the Complex Innovative Design program into standard review practice. This shift would allow the FDA to maintain capacity and subject matter expertise for adaptive, Bayesian, and other novel designs, while allowing sponsors to seek advice through existing formal meeting pathways. PDUFA VIII expands the Model-Informed Drug Development (MIDD) meeting program as well by increasing the number of eligible proposals beginning in FY 2028 and working toward the elimination of its quarterly cap by the end of FY 2030. MIDD meetings would be led by quantitative medicine leadership in partnership with relevant clinical, statistical, and other disciplines. Model-informed approaches can reduce unnecessary studies, improve dose selection, support extrapolation, and help make development programs more efficient. **PMC believes that these commitments can further integrate scientifically appropriate innovative designs into normal regulatory practice.**

Increasing Transparency and Communication

Effective communication across review disciplines and with various stakeholders is a critical part of the review process. Consistency is especially important to personalized medicine when considering the need for cross-center collaboration on co-developed drugs and diagnostics, as well as the acceptance of endpoints by multiple

centers to support accelerated review pathways in the same disease setting. One example of where inconsistent regulatory expectations are creating uncertainty for sponsors developing products intended to achieve similar clinical outcomes is FDA's treatment of minimal residual disease (MRD) as a surrogate endpoint in multiple myeloma. While CDER has recognized MRD as a pathway to support accelerated approval in multiple myeloma, uncertainty remains regarding the acceptance of MRD for cell therapies reviewed within CBER despite growing evidence supporting its relevance in the same disease setting. In order to mitigate inconsistent review practices, avoidable delays, and differences in the quality of feedback received by sponsors, FDA should consider training reviewers on the quality of their communication with sponsors and other stakeholders.

While communication between FDA review staff and sponsors has improved through expanded meeting types introduced under PDUFA VII, stakeholders have reported inconsistent implementation of meeting formats across review divisions. In addition to reviewer training, we recommended at the beginning of the negotiation that the agency consider whether certain meeting pathways can be made more predictable and whether internal review timelines can be optimized to reduce scheduling bottlenecks without compromising rigor or transparency.

PDUFA VIII makes several meaningful changes to formal meetings, including mechanisms for multi-divisional meetings, clearer treatment of sponsor requests for face-to-face or written responses, preliminary-response timing goals for certain meeting types, a formal opportunity to submit clarifying questions after meeting minutes or a written response, and an independent assessment of selected enhancements. PMC supports consistent implementation of these reforms across review divisions. We urge FDA to bring considerations for companion diagnostics and surrogate endpoints into early meetings, especially for divisions that may see fewer biomarker-selected programs.

PDUFA VIII also provides for a third party to evaluate first-cycle outcomes including the timing and basis of complete responses and major amendments, adherence to key process milestones, labeling communications, pivotal-protocol interactions, and the timing and substance of FDA-sponsor communications across review divisions. **PMC strongly supports this assessment because it may help in understanding issues and process patterns that require adjustment, practices that could lead to timelier resolution, and offer explanations to variations in decisions.**

Improving Frameworks for COA Development and Utilization of Primary Disease Biomarkers

PMC appreciates the FDA's efforts to support the development and submission of COAs through guidance and public workshops. COAs are essential to measuring how a patient feels, functions, or survives, and for determining whether a drug provides clinical benefit. COAs identify, measure, and evaluate symptoms or functions that are meaningful to patients, a task that can be particularly challenging given the heterogeneity of signs, symptoms, and functional impairment that patients exhibit in numerous disease areas, including rare diseases. Stakeholders continue to report confusion about which types of data are acceptable for regulatory use and what evidentiary thresholds apply. To improve clarity and reduce sponsor burden, PMC recommended consolidating duplicative guidance and harmonizing terminology related to patient experience data, patient preference studies, and COA development. **PDUFA VIII continues substantial investment in regulatory science**, including structured benefit-risk assessment, complex innovative designs, digital health technologies, biomarker/COAs, and model-informed drug development. The agreement also commits FDA to ongoing reviewer training across these tools and to hold a public meeting by the end of FY 2028 on best practices for communicating how regulatory science approaches are used in drug development and regulatory decision-making. **PMC supports this integrated approach because the value of innovative methods depends partly on whether review teams understand them, sponsors can access appropriate expertise, and experience gained in one program can inform future work.**

Furthermore, the development of drugs intended for use in small patient populations, an increasingly common occurrence in the field of personalized medicine, presents unique challenges. For rare diseases without well-characterized natural histories specifically, drug development tools are often lacking. Primary disease biomarkers are critical to understanding and evaluating disease processes and drug response throughout rare disease drug development. They are essential to development programs for illnesses where traditional clinical endpoints measuring decline are neither feasible nor ethical. FDA should maintain regulatory flexibility to accept some primary disease biomarkers as endpoints, however, in addition to case-by-case acceptance there is a need for a widespread framework that will apply across CDER and CBER outlining criteria for how to qualify biomarkers as surrogate endpoints in rare diseases. Early surrogate-endpoint consultation can also create value when FDA ultimately concludes that additional evidence is necessary. A sponsor that understands at an early stage what is known about the biomarker, what uncertainty remains, and what evidence would be needed to establish its relationship to clinical benefit can design a development program around those questions rather than discovering them after a pivotal study is underway.

PDUFA VIII would retain resources for intensive FDA guidance and senior-management involvement in breakthrough programs and would provide a meeting pathway for early discussion of novel surrogate endpoints. PMC supports these resources because they are particularly relevant mechanisms for programs targeting serious and life-threatening diseases in which development time, uncertainty about endpoint acceptability, and limited opportunities for conventional evidence generation can directly affect whether a promising therapy advances. We encourage the FDA to use guidance, case examples, and cross-program learning to make the surrogate-endpoint consultation pathway increasingly predictable.

Further Considerations for PDUFA VIII

Bolstering Cell and Gene Therapy Capacity

FDA has approved 34 cell or gene-based therapies (CGTs). In 2025, FDA's CBER approved five new CGTs. This further underlines the potential of this class of personalized treatments.ⁱⁱⁱ **Because CGT development relies heavily on regulatory predictability and early scientific discussion between the FDA and industry, PMC strongly supports efforts in PDUFA VIII to bolster the agency's Cell and Gene Therapy Program.** CGT development can depend on highly specialized manufacturing and assay capabilities, complex comparability questions, long-term follow-up, limited patient populations, and rapidly evolving scientific knowledge. Regulatory predictability depends on continuity of expertise and opportunities for early engagement are essential. The PDUFA VIII agreement focuses on sustaining specialized CBER staffing, training, policy development, external outreach, stakeholder education, post-market safety work, and collaboration on manufacturing, testing, bioinformatics, artificial intelligence, standards, and new approaches to evidence generation. It also directs continued attention to rare diseases, small populations, novel trial designs, surrogate endpoints, natural histories, RWE, and methods for capturing post-approval safety and efficacy.

Strengthening Rare Disease Expertise

Treatments for rare diseases continue to make up a growing percentage of personalized medicine approvals each year. Last year alone almost 30 percent of new FDA-approved personalized medicines were intended for use by patients with rare diseases.^{iv} However, **CDER and CBER review teams require additional expertise to meet the unique challenges associated with rare disease product applications and strategies for addressing them. We believe PDUFA VIII begins to address this need** by committing FDA to continue integrating rare disease expertise into review teams and reporting on participation of rare disease staff in review programs, stakeholder engagement, use of expedited programs, and regulatory flexibility in rare disease approvals. Additionally, FDA will continue convening Rare Disease Innovation, Science, and Exploration (RISE) workshops, which have brought together thousands of stakeholders to advance regulatory science for rare

disease treatment development, including approaches to individualized therapies.^v Recognizing that limited regulatory precedent, small trial populations, and limited understanding of disease natural history create challenges in identifying efficacy endpoints for many rare disease therapies, PDUFA VIII continues the Rare Disease Endpoint Advancement (RDEA) program transitioning from a pilot in FY 2028 and FY 2029 to a formal program under the PDUFA framework between FY 2030 and FY 2032.

Increasing Resources for Expertise and Infrastructure

PMC supports the PDUFA VIII agreement's emphasis on rebuilding and maintaining the scientific workforce required for the human drug review program. FDA acknowledges high attrition among technical and scientific experts during PDUFA VII and commits to quarterly updates on PDUFA hiring, including designated positions associated with model-informed drug development, rare diseases, and others. PDUFA VIII also includes substantial investments related to information technology, data exchange, bioinformatics, cloud infrastructure, and emerging technologies. FDA plans to maintain specialized expertise, software, storage, computing capacity, and global data-standards work to help ensure that technical limitations do not become barriers. **PMC supports FDA's commitment to modernization because outdated systems can make it more difficult for reviewers to analyze increasingly complex datasets. The bioinformatics commitments are particularly important to personalized medicine programs as genomic, multi-omic, computational, and other high-dimensional data become more common.**

Conclusion

Thank you for your work to ensure patients have timely access to safe and effective prescription drugs and biologic medicines so that every patient may have the opportunity to benefit from a personalized approach to health care. PMC looks forward to collaborating with the FDA and Congress as the user fee reauthorization process advances. If you have any questions about the content of this letter, please contact me at 202-499-0986 or cbens@personalizedmedicinecoalition.org.

Sincerely,



Cynthia A. Bens
Senior Vice President, Public Policy

ⁱ U.S. Food and Drug Administration. Reauthorization of the Prescription Drug User Fee Act; Public Meeting; Request for Comments. August 14, 2026. <https://www.federalregister.gov/documents/2026/08/14/2026-16650/reauthorization-of-the-prescriptiondrug-user-fee-act-public-meeting-request-for-comments>. (accessed October 9, 2026).

ⁱⁱ Personalized Medicine Coalition. Personalized Medicine at FDA: The Scope & Significance of Progress in 2023. February 29, 2024. <https://www.personalizedmedicinecoalition.org/wp-content/uploads/2024/02/report-3.pdf> (accessed October 9, 2026).

ⁱⁱⁱ Personalized Medicine Coalition. Personalized Medicine at the FDA: The Scope & Significance of Progress in 2025. May 14, 2026. https://www.personalizedmedicinecoalition.org/wp-content/uploads/2026/05/9920-PMC_PM_at_FDA_The_Scope_Significance_of_Progress_2025_v8_Digital.pdf. (accessed October 9, 2026).

^{iv} Personalized Medicine Coalition. Personalized Medicine at the FDA: The Scope & Significance of Progress in 2025. May 14, 2026. https://www.personalizedmedicinecoalition.org/wp-content/uploads/2026/05/9920-PMC_PM_at_FDA_The_Scope_Significance_of_Progress_2025_v8_Digital.pdf. (accessed October 9, 2026).

^v Personalized Medicine Coalition. Personalized Medicine at the FDA: The Scope & Significance of Progress in 2025. May 14, 2026. https://www.personalizedmedicinecoalition.org/wp-content/uploads/2026/05/9920-PMC_PM_at_FDA_The_Scope_Significance_of_Progress_2025_v8_Digital.pdf. (accessed October 9, 2026).