

October 16, 2026

Dockets Management Staff (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

**Re: Reauthorization of the Prescription Drug User Fee Act (PDUFA VIII), FY 2028–2032
(Docket No. FDA-2026-N-8163)**

To whom it may concern:

The Massachusetts Biotechnology Council (MassBio) appreciates the opportunity to comment on the Food and Drug Administration's (FDA) proposed recommendations for reauthorization of the Prescription Drug User Fee Act (PDUFA) for fiscal years 2028 through 2032. MassBio strongly supports timely reauthorization and welcomes the constructive direction of the proposed PDUFA VIII commitment letter. We are encouraged by provisions intended to strengthen communication between FDA and sponsors, improve predictability throughout development and review, and make regulatory processes more efficient without compromising the Agency's rigorous standards for safety and effectiveness.

MassBio represents more than 1,700 members across the life sciences ecosystem, from emerging biotechnology companies to global biopharmaceutical firms, academic institutions, research hospitals, and service providers. Of our membership, 80% of our biopharma members are small companies with fewer than 50 employees. Our 2026 Industry Snapshot shows that Massachusetts is home to 22.3 percent of the nation's biopharma research and development workforce and 2,175 drug candidates in development, representing 17 percent of the U.S. pipeline.^[1] The state's innovation ecosystem illustrates the importance of a regulatory framework that supports companies at every stage of development, particularly small and first-time sponsors working to translate promising discoveries into new treatments.

PDUFA VIII presents a defining opportunity to strengthen U.S. leadership in biomedical innovation. For small and emerging biotech companies operating with limited capital, a predictable regulatory process is essential to their success. Clear feedback, timely meetings, and consistent expectations can determine whether a company initiates a clinical trial, secures the investment needed for its next milestone, or advances a promising therapy for patients. While MassBio supports the broader commitments to improve regulatory efficiency and predictability, we highlight three priority recommendations for your consideration, discussed in greater detail below:

MassBio's priority recommendations

- Strengthen U.S. clinical trial competitiveness beyond financial incentives. MassBio supports the proposed 50 percent application-fee reduction for submissions including data from a qualifying U.S.-anchored Phase 1 trial, while emphasizing that financial incentives alone are unlikely to drive decisions about where companies initiate and conduct clinical trials. Regulatory speed and predictability remain critical to where small sponsors initiate trials.
- Assess the impact of proposed orphan drug fee changes. MassBio urges careful review of proposals to narrow the orphan application fee exception and charge a fee for the first supplement seeking a non-orphan indication, which could discourage or delay development of additional indications for promising treatments.
- Pilot a navigation service for first-time clinical trial sponsors. MassBio recommends FDA explore establishing a dedicated concierge-style service to help first-time sponsors navigate the IND process, understand submission requirements, prepare for meetings, and connect with appropriate FDA reviewers. This effort could build on existing FDA sponsor-engagement programs such as INTERACT while providing more tailored support to small and emerging biotechnology companies.

Strengthening timely and meaningful FDA–sponsor engagement

MassBio welcomes the commitments to improve formal meeting management and clarify when a sponsor may request a face-to-face meeting, including a virtual meeting, rather than a written response only. For small and first-time sponsors, the opportunity for direct discussion with FDA reviewers is especially valuable when addressing novel scientific questions, complex development plans, or uncertainty about regulatory expectations. We appreciate the proposed requirement that FDA provide a specific rationale when it determines that a written response is sufficient for certain meeting requests, and we encourage the Agency to apply this process consistently across review divisions.

We also strongly support the proposed process allowing sponsors to seek clarification of FDA meeting minutes or written responses, with a defined 20-calendar-day period for FDA to respond to qualifying questions. Clarifying a narrow but consequential point should not necessarily require a new formal meeting. These provisions, together with the existing performance goals for responding to meeting requests, scheduling meetings, and providing timely preliminary written feedback, can give emerging companies greater confidence in planning their clinical and financing milestones.

MassBio further welcomes recognition of multi-divisional meetings for eligible programs involving multiple investigational new drug applications across therapeutic areas. Coordinated feedback can reduce duplicative interactions and help sponsors understand how guidance from different divisions fits together. As FDA updates its formal-meetings guidance, we encourage practical examples of when interactive or multi-divisional meetings are appropriate, clear instructions for first-time sponsors, and an implementation approach that does not create additional paperwork or procedural complexity.

Increasing predictability at critical development and review milestones

MassBio supports the new pivotal-protocol prioritization process. Earlier, actionable feedback on a protocol intended to support a primary efficacy claim can help a smaller sponsor avoid costly revisions after a study has begun. We encourage FDA to communicate clearly how sponsors should identify qualifying submissions, document critical questions, and understand when they can expect a response under applicable review procedures. Consistent implementation across review divisions will be essential to realizing the value of this commitment.

We also appreciate the proposed third-party assessment of first-cycle review processes and FDA–sponsor communications. Identifying opportunities to reduce avoidable delays and improve efficiency are particularly important for small companies, which may have limited resources to sustain additional review cycles. MassBio encourages FDA to seek input from emerging and first-time sponsors as part of the assessment and to translate its findings into practical improvements in the timing, clarity, and consistency of feedback during development and application review.

Expanding access to regulatory science and manufacturing support for emerging companies

MassBio is encouraged by the proposed continuation and integration of regulatory science initiatives, including approaches involving model-informed drug development, complex innovative trial designs, rare disease endpoints, and real-world evidence. These tools offer meaningful potential for companies pursuing emerging modalities or treatments for conditions where conventional development approaches may be difficult. As the programs are incorporated into established meeting pathways, we encourage FDA to provide straightforward, consolidated guidance on eligibility, meeting requests, and expectations for sponsor submissions so that access, and success, do not depend on a company having a large in-house regulatory team.

We similarly support the risk-based manufacturing-facility lifecycle approach, including the proposed Chemistry, Manufacturing, and Controls (CMC) Facility Pre-submission meeting and opportunities for discussion following certain pre-approval or pre-license inspections. Manufacturing readiness is an important challenge for smaller companies, many of which rely on contract development and manufacturing partners. Earlier communication about facility expectations and potential deficiencies can help sponsors address issues before they jeopardize an application's review. We encourage FDA to ensure that these engagement opportunities are workable for applicants that do not own their manufacturing facilities and to provide practical implementation guidance.

Sustaining FDA capacity for rare disease and advanced therapy development

Massachusetts is home to one of the world's largest concentrations of rare disease research, and MassBio welcomes FDA's continued investment in the tools that support it. We support the continuation of the Rare Disease Endpoint Advancement (RDEA) Program, the commitment to hold rare disease workshops throughout the PDUFA VIII period, and the inclusion of dedicated rare disease staff on review teams. We encourage FDA to monitor interest in the program closely and to expand capacity if qualified requests exceed available slots, so that the shift to a standing meeting type does not create a period of constrained access for sponsors developing therapies for rare conditions.

MassBio similarly urges FDA to sustain its commitment to the Cell and Gene Therapy Program. Massachusetts companies account for a substantial share of cell and gene therapy development worldwide, and the program has been important in building the specialized review expertise these products require. We hope the PDUFA VIII commitment letter signals the Agency's continued commitment to this program and to growing it alongside the field it serves. As the pipeline matures and more products advance toward approval, we encourage FDA to ensure that CBER retains and expands the specialized staff, scientific capacity, and review flexibility these therapies require, including continued work on approaches to demonstrating effectiveness in very small patient populations.

MassBio also urges careful consideration of the proposed changes to the orphan drug fee exception. Under the proposal, the exception would apply only to products approved solely for orphan indications, and the first supplement seeking approval for a non-orphan indication would carry a fee equal to 50 percent of the full application fee. Many rare disease therapies are first approved for a narrow indication and later studied in broader populations, and the sponsors pursuing that path are frequently the same small and emerging companies PDUFA VIII otherwise seeks to support. We are concerned that this change could discourage sponsors from pursuing additional indications that would benefit patients or delay those programs at the point when the supporting evidence is strongest. We encourage FDA and Congress to evaluate its likely effect on rare disease development before the change is finalized.

Encouraging U.S.-based clinical trials through financial incentives and regulatory efficiency

MassBio supports positive financial incentives that encourage companies to conduct early-stage clinical research in the United States, including the proposed changes to the PDUFA VIII fee structure that would provide a 50 percent reduction in application fees for sponsors whose submissions include data from at least one Phase 1 clinical trial anchored in the United States and initiated after October 1, 2027. However, financial incentives alone are unlikely to determine where companies choose to initiate clinical trials.

For small and emerging biotechs operating with limited capital, speed and predictability are paramount. Decisions about where to conduct early-stage trials are often made years before a marketing application is submitted, and the ability to initiate a trial quickly, manage development costs, and reach the next milestone can be far more consequential than potential savings realized later in development. If the United States is to remain a global leader in early-stage clinical research, it must compete not only through financial incentives but also through a regulatory environment that enables companies to advance promising therapies efficiently.

MassBio therefore encourages FDA to view the proposed fee reduction as one component of a broader strategy to make the United States a more attractive destination for clinical research. The other commitments in PDUFA VIII, including improvements to sponsor engagement, more timely and predictable regulatory feedback, and efforts to streamline clinical development, are equally critical to achieving that goal. Successful implementation of these commitments, particularly for small and first-time sponsors, will help address the practical barriers that influence where companies choose to conduct their earliest trials. Together, these efforts can strengthen U.S. leadership in clinical research and expand opportunities for American patients to access promising new treatments.

Ensuring PDUFA VIII delivers for small and first-time sponsors

The success of PDUFA VIII is dependent on efficient and committed implementation. MassBio appreciates the proposed continued attention to resource planning, hiring and retention transparency, and technology modernization. Experienced and adequately staffed review teams, consistent application of guidance, and dependable submission systems are essential to the timely, high-quality interactions on which emerging companies rely.

As FDA moves forward, MassBio encourages the Agency to consider the experience of small and first-time sponsors when evaluating new meeting processes and other PDUFA VIII improvements. Where feasible and appropriate, assessments should examine whether sponsors of different sizes experience comparable access to meetings, timely feedback, and consistent guidance. FDA should also solicit input from emerging companies during guidance development and implementation so that process improvements reduce, rather than shift, administrative burdens onto sponsors.

Establishing a dedicated navigation resource for first-time sponsors

FDA's Initial Targeted Engagement for Regulatory Advice on CBER/CDER Products (INTERACT) meetings already provide an important entry point for sponsors facing novel questions in early development, and for many emerging companies they are the first substantive interaction with the Agency. MassBio appreciates that the proposed commitment letter preserves INTERACT, and we encourage FDA to maintain adequate resources for the program throughout PDUFA VIII so that first-time sponsors can engage the Agency early on the questions that most benefit from real-time discussion.

MassBio also recommends building on INTERACT and the proposed enhancements to FDA—sponsor engagement by exploring the establishment of a dedicated concierge-style service to help first-time clinical trial sponsors navigate the regulatory process. Small and emerging biotechnology companies often lack the in-house regulatory expertise and resources of larger sponsors, making the process of preparing an Investigational New Drug (IND) application and initiating a first-in-human clinical trial particularly challenging. Even when regulatory requirements are clear, navigating the process for the first time can introduce unnecessary delays, uncertainty, and costs that may prevent promising treatments from advancing.

Under this approach, FDA could establish a dedicated point of contact for first-time sponsors to help them navigate the early stages of clinical development, understand submission requirements, identify appropriate regulatory pathways, and connect with the relevant FDA offices and reviewers. The service could provide practical assistance on preparing for pre-IND meetings, addressing common submission deficiencies, and understanding expectations for initiating clinical trials.

MassBio believes this type of service would complement the PDUFA VIII commitment letter's emphasis on improved communication, more predictable timelines, and greater efficiency. We encourage FDA to consider piloting such a program with particular attention to reducing the time and administrative burden associated with initiating first-in-human studies for first-time sponsors. By making it easier for emerging companies to navigate the regulatory process, FDA can help ensure that the United States remains an attractive location for early clinical development while accelerating the translation of promising scientific discoveries into new treatments for patients.

Conclusion

The recommendations in this letter share a common premise: the benefits of PDUFA VIII should reach sponsors of every size. We urge FDA to apply the new meeting and feedback processes consistently across review divisions; to issue consolidated, practical guidance so that access to these programs does not depend on a sponsor's in-house regulatory capacity; to sustain its investment in the rare disease and cell and gene therapy programs on which much of the Massachusetts pipeline depends; and to evaluate carefully the proposed changes to the orphan drug fee exception before they are finalized. We also encourage the Agency to pilot a dedicated navigation resource for first-time sponsors, which would advance the commitment letter's goals of clearer communication, greater predictability, and a more efficient path from discovery to first-in-human study.

MassBio stands ready to work with FDA, our member companies, and other stakeholders to help put these commitments into practice. We would welcome opportunities to share examples from small and emerging biotechnology companies, participate in public workshops and listening sessions, and help ensure that the perspectives of first-time sponsors are reflected as new procedures are developed.

PDUFA has long provided an important foundation for predictable, science-based drug review. The proposed PDUFA VIII agreement offers an opportunity to build on that foundation and reinforce the United States as the premier location to discover, develop, and deliver new medicines. MassBio supports this effort and looks forward to working collaboratively with FDA to ensure that the next five years of progress benefit companies of every size, and, ultimately, the patients they serve.

Sincerely,



Kendalle Burlin O'Connell
President & Chief Executive Officer
MassBio

^[1] <https://www.massbio.org/industry-reports/industry-snapshot/>