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

Re: Expedited Investigational New Drug Pilot Program Request for Information (Docket No. FDA-2026-N-4699)

To whom it may concern:

The Massachusetts Biotechnology Council (MassBio) appreciates the opportunity to comment on the Food and Drug Administration's (FDA) proposal to establish the Expedited Investigational New Drug (IND) pilot program.

MassBio represents more than 1,700 members spanning the full spectrum of the life sciences, from the smallest startups to the largest biopharmaceutical companies, alongside academic institutions, research hospitals, contract research and manufacturing organizations, and service providers. Our mission is to advance Massachusetts' leadership in the life sciences to grow the industry, add value to the healthcare system, and improve patient lives.

MassBio supports FDA's effort to shorten the time from drug identification to first-in-human (FIH) study, and we commend the FDA for pursuing a concrete reform aimed at keeping early-stage biomedical development in the United States. We offer these comments from a particular vantage point. Massachusetts stands as a leading hub of biotech research and development, responsible for 15.7 percent of the total U.S. drug development pipeline and 6.4 percent of the global pipeline. A substantial share of MassBio's membership consists of early-stage companies, many of which pre-clinical, first-time sponsors operating with small teams. These are precisely the sponsors for whom the time and predictability of reaching FIH can determine whether a program advances at all, and precisely the sponsors that a well-designed Qualified Research Institution (QRI) model could help the most. At the same time, Massachusetts is home to some of the world's leading academic medical centers, research hospitals, and universities that are among the strongest candidates to serve as QRIs under this pilot.

We support the pilot's goals. Our comments are offered to help ensure that the pilot delivers meaningfully for the smaller and first-time sponsors who make up so much of the innovation ecosystem. We respectfully offer the following responses to the RFI questions.

A. Pilot Program Design and Implementation

1. QRI Qualification and Capabilities

i. What specific changes, if any, would you recommend regarding FDA's thinking on the capabilities, infrastructure, and/or leadership expertise research institutions must demonstrate to qualify for the pilot program?

MassBio's primary recommendation on QRI qualification is that FDA standardize the procedures, review standards, and documentation expectations that QRIs must follow, so that the quality and predictability of the pathway do not vary depending on which QRI a sponsor engages. A pilot built around multiple independent institutions carries an inherent risk of divergence: different QRIs may apply different interpretations of what constitutes an adequate FIH submission, different levels of rigor, and different working practices, producing inconsistent experiences and outcomes for sponsors. That inconsistency would be most damaging to the smaller and first-time sponsors this pilot could most help, who depend on clear and uniform expectations and lack the resources to adapt to idiosyncratic, QRI-specific requirements.

We therefore recommend that FDA, in defining the capabilities, infrastructure, and leadership expertise that QRIs must demonstrate, pair those qualification criteria with standardized operating procedures and common review standards that all participating QRIs are required to follow. This should cover, at a minimum, the scope and format of QRI review, the criteria for a complete FIH submission, documentation and record-keeping practices, and the nature of the recommendation a QRI provides to FDA and the sponsor.

v. To ensure faster FIH initiation, should QRIs be required to have a self-owned and operated IRB and/or clinical trial site? If not, would a partnership with an IRB and/or trial site network be sufficient?

FDA should not require QRIs to own and operate their own Institutional Review Board (IRB) and/or clinical trial site as a condition of participation. An ownership requirement would unnecessarily narrow the QRI pool, excluding smaller, specialized, or highly capable institutions that possess significant scientific and regulatory expertise but do not maintain their own IRB or site infrastructure.

2. Pre-IND and IND Review Process

iv. How should expedited INDs during the pilot differ in requirement from standard IND submission, if at all?

Expedited INDs under the pilot should be governed by a risk-based, phase-appropriate framework. FDA should also publish clear eligibility criteria and a working definition of a “low-risk” FIH study, addressing product characteristics, preclinical safety data, study design, and patient population, so that smaller sponsors without large regulatory departments can readily assess whether their program qualifies.

3. Drug Eligibility and Participation

i. Should all drug products be considered in the pilot or are there specific types of drug products and/or specific disease or conditions that are better suited for the pilot (e.g., small molecules, large molecules, cellular & gene therapies, nucleic acid-based therapies, combination products, etc.)?

MassBio supports broad consideration across CDER and CBER and recommends FDA not confine the pilot to a single modality.

ii. How should drug products be prioritized for participation in the pilot and why?

MassBio recommends that FDA prioritize participation for first-time sponsors, small teams, and early-stage companies that lack the in-house regulatory, CMC, and toxicology capacity to independently produce a high-quality FIH IND submission. These sponsors stand to gain the most from partnering with a QRI, and for them a delay in reaching FIH might impact future investment that would otherwise sustain it.

Prioritizing this population would maximize the pilot's marginal benefit, generate the most informative data on where QRI support adds value, and directly advance the national goal of retaining early-stage development in the United States.

5. Implementation and Feasibility

i. What resources would QRIs need to develop and maintain IND review and recommendation capabilities (staff, quality systems, technology infrastructure, estimated costs)?

FDA has indicated that, following the pilot, sponsors will pay fees directly to QRIs and FDA will not be involved in the setting or collection of fees. If QRI engagement proves expensive, or if qualification requirements drive QRIs toward high fixed costs that are passed through to sponsors, the pathway could become inaccessible to the very small and early-stage companies it could most help, converting a competitiveness reform into a premium service for well-resourced sponsors.

We urge FDA to be transparent, as early as possible, about the anticipated cost of QRI engagement, to clarify how costs will be handled during the pilot phase, and to consider mechanisms that keep the pathway accessible to emerging companies (for example, published fee ranges or reduced-cost participation options). FDA should also establish guardrails ensuring that QRI involvement reduces timelines rather than adding duplicative review, operational complexity, or cost that outweighs the benefit.

B. Risks, Oversight, and Evaluation

1. Risks and Safeguards

iii. Could this pilot create inequitable access favoring well-resourced sponsors over smaller companies?

As currently framed, participation is voluntary and, after the pilot, sponsors pay QRI fees directly. Absent affirmative safeguards, those features could favor larger, better-resourced sponsors who can most easily absorb QRI costs and dedicate staff to navigating a new process, entrenching rather than leveling the existing advantage. That outcome would be at odds with the pilot's stated competitiveness rationale.

MassBio urges FDA to design the pilot affirmatively for equitable access: reserve a meaningful share of pilot capacity for emerging and first-time sponsors; provide transparency on QRI costs; minimize administrative and documentation burden; and track small-sponsor participation as a core evaluation metric.

2. Oversight and Accountability

iii. How can the pilot ensure transparency while safeguarding the sponsor's confidential commercial, trade secrets, and other sensitive information?

Confidentiality protections are especially critical for smaller sponsors, whose entire enterprise value often rests on a single asset or platform and for whom inadvertent disclosure can be existential. Under the pilot, QRIs would have access to highly sensitive nonclinical, clinical, and CMC information. MassBio strongly recommends that FDA establish robust, enforceable confidentiality and conflict-of-interest standards applicable to QRIs and their personnel and partners. To the extent the pilot generates public-facing materials, such as case studies or lessons learned, those materials should be developed collaboratively with participating sponsors to ensure that no proprietary or commercially sensitive information is disclosed.

3. Success Metrics and Evaluation

ii. What metrics should define pilot success? Please address time, quality, safety, and specify any others.

Time to FIH study initiation, measured against a clear pre-pilot baseline for comparable Phase 1 programs, should be the primary success metric. FDA should also track the rate of clinical holds and information requests, submission quality, and participant safety outcomes. In addition, MassBio recommends that FDA expressly measure participation and outcomes among first-time and small-team sponsors. The pilot should be judged in part on whether it broadened access to high-quality regulatory support, not on aggregate speed alone. We encourage FDA to establish and share its baseline data and its expectations for acceptable outcomes before the pilot launches, so that stakeholders can provide informed input.

C. Additional Considerations

i. What additional/alternative approaches could achieve similar goals of accelerating Phase 1 FIH IND study initiation in the U.S.?

The pilot is a valuable step, and MassBio encourages FDA to pursue it promptly. Its benefits would be reinforced by complementary reforms that reside within FDA's existing authority and that help all sponsors. FDA should strengthen and accelerate existing sponsor-engagement mechanisms, such as pre-IND and INitial Targeted Engagement for Regulatory Advice on CBER/CDER ProducTs (INTERACT) meetings, with faster and more predictable timelines; issue clear, phase-appropriate CMC guidance; and establish a time-bound process that gives sponsors a meaningful opportunity to resolve potential deficiencies before a clinical hold is imposed.

iv. Are there important considerations or concerns not addressed in the questions above?

FDA should account for its own resource constraints. The pilot adds new oversight, evaluation, and QRI-coordination responsibilities. FDA should ensure that standing up the pilot does not divert reviewer capacity away from the standard IND pathway, on which the majority of

sponsors will continue to rely. User-fee resources should not be redirected from FDA's core review functions and performance commitments to support pilot infrastructure.

Finally, MassBio notes that Massachusetts offers a natural proving ground for this pilot, combining a deep pool of potential QRIs with the small and first-time sponsors who would benefit most. We and our members stand ready to serve as a resource to FDA as it refines and implements the program.

Conclusion

MassBio commends FDA for advancing a reform aimed at accelerating first-in-human research and keeping early-stage innovation in the United States. We share the FDA's goal of speeding patients' access to safe and effective therapies while strengthening the nation's biomedical innovation ecosystem. We urge FDA to design and implement the pilot so that its benefits reach the smaller and first-time sponsors who make up so much of that ecosystem, and we stand ready to work with the you to that end.

We appreciate your consideration of these comments and look forward to continuing to work with you.

Sincerely,

Kendalle Burlin O'Connell
CEO & President
Massachusetts Biotechnology Council (MassBio)