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Dockets Management
Food and Drug Administration
5630 Fishers Lane, Rm 1061
Rockville, MD 20852

Docket Number: **FDA-2023-N-3742**

Dear FDA Dockets Management Staff,

I am submitting comments to Docket Number FDA-2023-N-3742, **Scientific Challenges and Opportunities To Advance the Development of Individualized Cellular and Gene Therapies: Request for Information** on behalf of the National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL). NIIMBL, a part of the ManufacturingUSA network, is a public private partnership of approximately 200 members in academia, government service, and across the biopharmaceutical supply chain. NIIMBL collected feedback on this Request for Information from its membership and aggregated the responses.

We appreciate this opportunity to comment on the thoughtful and important questions posed in this RFI. We understand the preparation of CGTs as an exercise in manufacturing rather than practice of medicine, that CGTs will be regulated under existing statutes of the FD&C and Public Health Service Acts and associated Guidance to Industry, and fully support these positions as matters of policy. While the questions in this RFI are directed at the unique challenges in developing CGTs, we feel that many of the questions apply more broadly to the challenges of developing commercial biologics in general and are addressed in existing Guidance. All biologics are developed on aggressive timelines to meet patient needs, must manage patient populations of different sizes and demographics, and must manage the interconnected complexities of translational science, commercialization, and basic research. We would however advocate, to the extent possible, for a harmonization of review, inspection practices, regulatory considerations, and exception within the FDA. Divergence of practices leads to uncertainty surrounding CMC expectations for regulatory compliance. Rather than identify more and more exceptions and subclasses of consideration, we suggest that the Agency would be better served to affirm that established requirements for INDs, BLAs, and phase-appropriate application of the GMPs are fully relevant to CGTs and to work to align expectations for submission, review, and inspection across different Centers and Offices within the Agency.

We appreciate the concerns detailed in the specific questions that were included as part of the RFI. However, it seems that most of these concerns are similar to those faced by current biologics or at least by a subset of them such as orphan therapies, ADCs with a very short half life, etc. Responses from our members echo that these are indeed pain points for the industry, however there are no quick or obvious solutions. We believe that collaboration between manufacturers, suppliers, and regulators will be critical to finding solutions to

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challenges associated with very small batch sizes, limited patient populations, and the balance between inventory required for testing versus patient supply. At the heart of these discussions should be patient-centered risk management and a focus on safety and clinical relevance. Public-private partnerships could be effective venues to facilitate collaboration and data sharing in a safe, precompetitive space where manufacturers, suppliers, non-profits, academics, and regulators can work together.

We appreciate this opportunity to provide feedback to this request and would be happy to follow up.

Kind Regards,
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NIIMBL Senior Fellow

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relationship between FDA and NIIMBL's Federal Sponsors is expanded upon in MOU 225-21-006 dated January 15, 2021.