SUMMARY OF RECOMMENDATIONS

1. Modernize Evidence Generation

1.1 Expand FDA's efforts to facilitate novel trial designs.
FDA should update its pilot programs to allow more programs to benefit, disseminate learnings more rapidly and better encourage the appropriate use of external control arms.

1.2 Encourage the use of patient preference information to "right-size" clinical trials.
FDA should expand its approach of encouraging patient perspectives in medical device applications to all medical products. This would improve trial design by informing endpoint selection and statistical considerations, allowing trials to better fit the needs of patients.

1.3 Develop a framework for addressing privacy considerations related to FDA's review of real-world data sources.
FDA should update its privacy framework to enable submissions with more reliance on data from real-world clinical sources.

1.4 Eliminate unnecessary burdens relating to data formatting.
FDA should eliminate the requirement to convert all real-world data into the same format as clinical trial data, which requires significant effort relative to benefit and discourages the use of relevant and reliable data.

2. Advance Innovation for Rare Disease

2.1 Provide greater specificity, consistency and predictability as to how FDA will assess the evidence for rare disease products.
FDA should standardize evidence assessment for rare disease products across all FDA centers and review divisions, potentially supporting legislation to clarify and improve consistency of regulatory approaches.

2.2 Modernize pathways for extremely rare and "n of 1" diseases.
FDA should take action to foster more scalable product development, including by leveraging its new authority to designate platform technologies.

2.3 Enable greater use of external controls in studying rare disease.
FDA should update its guidance on external controls to better facilitate their use in rare disease contexts, including in combination with other novel trial designs (such as trials involving master protocols).

3. Enhance Supply Chain Oversight

3.1 Use all available tools to clear the COVID-19 inspection backlog.
FDA should prioritize clearing the inspection backlog that developed from pausing in-person activities during the pandemic, and strategically use remote inspection tools to manage the workload.

3.2 Designate foreign manufacturing oversight a core leadership priority and evaluate options for third-party support.
FDA should prioritize foreign inspections at the leadership level and explore partnerships with nongovernmental third parties to supplement FDA's oversight capacity for long-standing foreign inspection challenges.

3.3 Develop a rating system to incentivize quality manufacturing maturity.
FDA should develop facility ratings based on advanced technology adoption beyond minimum requirements to reduce supply disruption risks, guide inspection priorities and inform payor decisions.

3.4 Incentivize and de-risk investment in advanced manufacturing technologies.
FDA should reduce the regulatory risk of using advanced manufacturing technologies (AMTs) by clarifying how existing frameworks that were designed for conventional manufacturing techniques apply to new technologies, and update its guidance on AMT designation to expand incentives for using this new statutory program.

4. Strengthen the Accelerated Approval Pathway

4.1 Facilitate more data from real-world clinical practice in confirmatory studies.
FDA's efforts to improve timely follow-through on post-market requirements should include efforts to facilitate more confirmatory studies that draw on data from real-world clinical practice.

4.2 Pursue reform strategies that address programmatic concerns while prioritizing early availability to patients.
FDA should continue reforming the accelerated approval program, including by regularizing its procedures and updating the processes for withdrawing approval and using advisory committees, while monitoring new policies to ensure they do not unnecessarily delay patient access.

4.3 FDA should minimize unnecessary duplication with other agencies.
FDA should enhance the transparency of its decisions to enable agencies such as the Centers for Medicare and Medicaid Services to minimize duplicative review and improve regulatory predictability.

5. Invest in FDA's Use and Oversight of Artificial Intelligence (AI) and Other Advanced Computing Technologies

5.1 Accelerate modernization of FDA technical infrastructure and procurement of advanced tools to improve FDA workflows. FDA should accelerate its technology modernization to improve internal operations and product reviews, shifting staff time from manual tasks to ensuring consistency with agency policy and precedent.

5.2 Build upon existing frameworks to facilitate innovative uses of AI in safe and effective medical products, including with respect to potential third-party reviews.
FDA should build upon existing frameworks for AI in medical products rather than creating entirely new regulatory approaches.

5.3 Update FDA's approach to clinical decision-support software.
FDA should revise its guidance on clinical decision support software to better reflect congressional intent and facilitate development of fit-for-purpose tools.

6. Advance Drug Competition

6.1 Continue the Drug Competition Action Plan and Biosimilars Action Plan, and update them to account for changes under the 2022 Inflation Reduction Act (IRA).
FDA should devote sufficient resources to continue activities with a successful track record and update its plans to account for IRA provisions that may reduce incentives for generic and biosimilar development.

6.2 Further streamline the pathway for interchangeable biological products.
FDA should update its policies to provide a clearer pathway for licensing interchangeable products without the need for switching studies.