

CONCLUSION

The recommendations in this paper offer a set of actions, across multiple domains, that FDA can take to facilitate medical product innovation, expand products' availability to patients, and foster improved access. In some cases, the recommended action involves a new policy or a change in direction; in others, the recommendation is to continue with (or expand upon) a policy or program that has been successful. Particularly in the face of current uncertainty regarding FDA's resources and structure, identifying both types of priorities—and ensuring that the agency has what it needs to deliver on them—is important.

Identifying both types of priorities is also important because it provides medical product developers with greater clarity and predictability regarding FDA's future regulatory expectations. Uncertainty regarding what policies the agency will pursue, or the extent to which it will continue with existing initiatives, adds unnecessary risk to development programs. A proactive policy agenda, pursued energetically and communicated clearly, promotes the public health not only through the policies themselves, but by fostering a predictable regulatory environment in which developers are better able to make the big bets that fuel innovation.

POLICY IMPLEMENTATION PRIORITIES

Effective implementation of the recommendations in this white paper requires a strategic, phased approach that balances impact with feasibility. The prioritization framework presented below considers four key factors: ease of implementation, speed of potential execution, resource requirements, and expected impact on public health and FDA's mission. Given recent resource constraints and organizational changes, particular attention has been paid to identifying recommendations that can deliver meaningful results without requiring significant additional staffing or funding. The recommendations have been organized into three tiers to guide implementation planning and resource allocation.

Tier 1: Quick Wins (Months 1–6)

These recommendations can be implemented relatively quickly with existing resources while delivering significant benefits to FDA stakeholders. They represent opportunities for early momentum and visible progress.

Recommendation 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.

Recommendation 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.

Recommendation 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.

Recommendation 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.

Recommendation 6.1: Continue the Drug Competition Action Plan and Biosimilars Action Plan, and update them to account for changes under the 2022 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.

Tier 2: Strategic Initiatives (Months 6–12)

These recommendations require moderate investment of time and resources but offer substantial benefits to FDA's core mission. They build upon existing programs and authorities while addressing critical needs.

Recommendation 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.

Recommendation 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.

Recommendation 2.1: FDA should provide greater specificity, consistency and predictability as to how it 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.

Recommendation 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).

Recommendation 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.

Tier 3: Long-Term Projects (Year 2+)

These recommendations require substantial resources, coordination with other agencies or longer timelines for implementation, but represent critical investments in FDA's future capabilities and effectiveness.

Recommendation 2.2: Modernize pathways for extremely rare and “n of 1” diseases

FDA should take action to foster more scalable product development, including by facilitating greater use of its new authority to designate platform technologies.

Recommendation 3.2: Designate foreign manufacturing oversight as a core leadership priority and evaluate options for third-party support

FDA should prioritize foreign inspections at leadership level and explore partnerships with nongovernmental third parties to supplement FDA's oversight capacity for long-standing foreign inspection challenges.

Recommendation 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.

Recommendation 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.

Recommendation 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 processes for withdrawing approval and using advisory committees, while monitoring new policies to ensure they do not unnecessarily delay patient access.

Recommendation 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 (CMS) to minimize duplicative review and improve regulatory predictability.

Recommendation 5.1: Accelerate the modernization of FDA technical infrastructure and procurement of advanced tools to improve FDA workflows

FDA should accelerate FDA technology modernization to improve internal operations and product reviews, shifting staff time from manual tasks to ensuring consistency with agency policy and precedent.

Recommendation 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 utilize existing frameworks and third-party expertise for AI in medical products rather than creating entirely new regulatory approaches.

This prioritization framework provides a roadmap for implementing the recommendations in this white paper in a manner that balances impact with feasibility. While the timing may be adjusted based on evolving circumstances, the overall approach ensures that FDA can make meaningful progress toward enhancing innovation and access while operating within resource constraints.