

## EXECUTIVE SUMMARY

New leadership at the United States Food and Drug Administration (FDA) are reevaluating policy at a time when scientific and technical advancements are rapidly accelerating development of innovative products that hold remarkable promise for patients. These tools and approaches—including innovative trial designs, sophisticated methods for developing evidence from real-world clinical practice, and systems powered by machine learning and artificial intelligence—are bringing medical products to market faster while meeting the same rigorous regulatory standards.

For the U.S. to fully realize the promise of these advancements, it is critical for FDA to update its regulatory frameworks to adapt to new technologies and methods, provide clear guidance about its regulatory expectations so that product sponsors can develop them with less uncertainty, eliminate unnecessary regulatory barriers, and ensure efficient and effective agency operations. This is an ambitious set of priorities under any circumstances, but executing them today is more complex in light of unprecedented challenges for the agency:

- The aftershocks of the COVID-19 pandemic continue to disrupt the agency, which faces a significant backlog in critical functions like inspecting manufacturing facilities<sup>1</sup> and a deficit in public trust.<sup>2,3</sup>
- Changes in the legal landscape for administrative agencies, including how courts evaluate agencies' interpretation of statutes,<sup>4,5</sup> mean that many FDA decisions will be subject to increased scrutiny, and the agency may need to reexamine existing rules.
- FDA as an organization has faced substantial cuts to its staff and programs, and a significant reorganization plan has been discussed.<sup>6,7</sup> The agency will need to find new ways to leverage its resources to accomplish core objectives without stalling medical product development through delayed feedback to product sponsors or creating unnecessary uncertainty regarding how reviews will be conducted.<sup>8</sup>
- Patients continue to face difficulties accessing many approved medicines, including challenges related to drug shortages and the time and cost associated with development and approval of new products.<sup>9,10</sup>

In the face of these challenges, agency leadership should pursue a proactive policy agenda that creates a regulatory environment responsive to both prongs of FDA's dual mission: to “protect the public health” by ensuring medical products are safe and effective, and “promote the public health” by acting “promptly,” “efficiently,” and in a “timely manner.”<sup>11</sup> While maintaining rigorous standards, FDA should ensure that its programs do not create unnecessary barriers to development of medical products that benefit patients or patients' ability to access them.

Promoting innovation and access is a core part of FDA's mission, and should be a core part of the agency's medical product agenda. By defining and enforcing standards for bringing products to market, developing evidence requirements and ensuring post-market oversight, FDA fundamentally shapes medical product innovation.<sup>12</sup> FDA policies directly affect which products are developed, how quickly they become available, and how they satisfy patient demand for effectiveness, quality and safety. FDA should exercise these authorities not only to protect the public from unsafe or ineffective products, but also to promote the public health by enabling innovation to flourish.

FDA should also pursue policies that promote patient access. While FDA does not regulate prices of medical products, its policies have a significant impact on patients' ability to access and afford them—by affecting costs of developing and marketing products and by managing the supply of competing products in the market.<sup>13</sup> Policies that reduce time and cost of bringing novel products to market, while balancing innovation with competition, can deliver significant improvements to patient access and the health ecosystem.

We propose a set of recommended actions FDA should take to accelerate development of transformative medical technologies and enhance patient access. In six areas, we identify opportunities to build on existing FDA work and change course, as appropriate, as part of a proactive medical product policy agenda.

These recommendations are focused on medical product policy. The paper does not address issues in other areas of FDA's jurisdiction, and it generally does not focus on broader institutional issues, like FDA's structural organization, budget or headcount. While organizational factors affect implementation, our focus is upstream—defining policy priorities applicable in any political or budgetary environment.