RECOMMENDATION 5: INVEST IN FDA'S USE AND OVERSIGHT OF ARTIFICIAL INTELLIGENCE AND OTHER ADVANCED COMPUTING TECHNOLOGIES

Key takeaways:

- Artificial intelligence (AI), machine learning (ML) and other advanced computing technologies are revolutionizing medical products—as development tools, as features in the products themselves, and as tools to make FDA more effective and efficient.
- FDA should accelerate modernization of its technical infrastructure and procurement of advanced tools to improve its workflows and make product reviews more efficient and predictable.
- FDA should leverage existing frameworks to facilitate innovative uses of AI in safe and effective medical products, including with respect to potential third-party reviews.
- FDA should update its approach to clinical decision-support software.

Recent advancements in AI, ML, and other advanced computing technologies offer unprecedented opportunities, not only in healthcare, but across the United States economy and government.[175] In January 2025, President Trump declared it the policy of the United States to "sustain and enhance America's global AI dominance in order to promote human flourishing, economic competitiveness and national security," and ordered development of an action plan to advance this policy.[176] FDA has a critical role to play in these efforts, and agency leadership should make its advancement of AI and other advanced computing technologies a core priority.

The agency sits at the intersection of many transformational use cases:

- *FDA as user:* AI, ML and other advanced technologies can help FDA improve its capabilities and the efficiency of its operations across domains such as product reviews, post-market surveillance, inspections and import operations.[177,178]
- *Algorithmic and AI-enabled products:* AI and ML technologies are powering innovative medical products, with applications such as improving detection and diagnosis of disease, personalizing therapies and diagnostics, and improving the functions and user interfaces of a wide range of medical devices.[179]
- *Product development tools:* AI, ML and other advanced tools are being deployed for uses across the product lifecycle that include, for example, accelerating drug discovery by helping identify and research promising drug candidates, improving recruitment and selection of clinical trial participants, optimizing clinical trial sites, managing and analyzing data, improving the manufacturing process, and analyzing post-market surveillance data.[180]

FDA leadership should accelerate and update existing efforts to further the responsible advancement of these use cases and unlock the potential of advanced technologies to improve patient access to safe and effective treatments and diagnostics.

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

In 2019, under the first Trump administration, FDA launched an initiative to modernize the agency's technical infrastructure and enhance its ability to deploy technology to support its mission. This effort, called the Technology Modernization Action Plan,[177] was expanded in 2022 through further efforts to modernize the agency's stewardship and use of data,[181] move the agency away from historically siloed approaches to information technology (IT) and toward enterprise-level IT management across programmatic areas,[182] and strengthen the agency's approach to cybersecurity.[183]

Agency leadership should make it a high priority to continue and build upon the work under these initiatives, with a focus on (1) developing the infrastructure to efficiently work with large volumes of data and deploy cutting-edge tools, (2) procuring solutions (both bespoke and commercially available) to use AI, ML and other advanced technologies to improve FDA workflows, and (3) implementing centralized IT solutions that enable the efficient migration toward more advanced systems.

FDA leadership has already indicated that the agency will work toward consolidating duplicative IT infrastructure[184] and implementing generative AI tools in product reviews.[185]

These initiatives have the potential to be transformative for the agency by improving operations, promoting efficiency, and improving agency-wide governance. As the agency continues to implement these and other initiatives, it should adhere to the following principles:

- *Efforts to better utilize advanced tools should not be limited to product reviews:* In recent years, FDA successfully employed advanced technology in other domains, such as by deploying tools to improve the efficiency of surveillance inspections[129] and more effectively screen certain food imports.[186] FDA should apply the learnings from these and similar experiences and use technology to improve

operations across more domains throughout the agency, including expanded uses in post-market surveillance and enforcement activities.

- *FDA should maintain strong governance principles, especially when deploying AI to support regulatory decision-making.* These principles should include rules (1) to ensure that "algorithmic-informed" decisions are made ultimately by humans who understand the risks and limitations of AI systems, (2) to minimize unnecessary duplication of systems across FDA's centers and programs, and (3) to provide appropriate transparency to users and the public into how results are generated.[175,182]
- *FDA should articulate clear objectives and use cases for deploying AI and other advanced tools in product reviews:* The most impactful opportunities go beyond merely summarizing data and may include, for example, using analytical platforms and other tools to improve the agency's ability to receive, manage and analyze the increasingly large datasets that are submitted in support of product applications.[177] Such tools could, among other things, enable reviewers to detect falsified data and other data quality issues more effectively and in a fraction of the time, or run analyses that otherwise might require substantial line coding by a statistician or computer engineer. Clearly articulating the anticipated use cases will enable stakeholders to understand how AI is being used in reviews and what controls are being used to mitigate risks.
- *FDA should articulate a clear plan for how it will use the efficiencies it generates to improve product reviews:* Among other things, more efficient reviews would help the agency keep up with the ever-increasing volume of applications, which is expected to rise substantially as developers continue to build AI and ML tools into their own processes.[177] In addition, increased efficiency would enable FDA reviewers to devote less time and attention to rote tasks and instead focus more on human-centric activities like ensuring that product reviews are consistent with agency policy and prior precedent—an area where the agency has historically struggled.[187,188]

Funding the infrastructure and procurement to drive these improvements should be a budgetary priority—internally within the agency, as part of the annual budget request to Congress and as part of the agency's requests for industry funding in negotiations for upcoming renewals to relevant user fee programs.

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

Innovative uses of AI and ML in medical products—both to enable the products themselves and as tools to improve the development process and other activities throughout the product life cycle—present extraordinary new opportunities to advance patient health. At the same time, they present novel challenges for FDA as the regulator tasked with ensuring that the products are safe and effective, such as (to the extent relevant and consistent with FDA's authority) ensuring that the tools are trained on appropriate data, applying validation models to software that may rely on decision-making processes that are not readily understandable, and addressing adaptive technologies that continue to learn and evolve over time.[180,189–191]

As FDA continues the work to address these challenges and advance innovation, it should aim to maximize its use of existing tools and frameworks where possible in order to minimize the disruption and uncertainty associated with developing wholly new frameworks. While new approaches may be needed to address certain novel issues, FDA's existing tools and authorities provide a solid foundation for future efforts. For example:

- FDA has already used its existing authorities to support a significant volume of product development. The agency has authorized more than 1,000 AI- and ML-enabled medical devices, a body of precedent that includes health-tracking features on wearable devices such as smartwatches, sleep-monitoring software, complex radiological devices, and many other products.[192]
- FDA has also advanced significant policy development regarding how its tools and authorities can be applied to address novel questions in this space, and has published draft or final guidance on topics such as submitting marketing applications for devices with AI- and ML-enabled software functions,[193] how marketing submissions for AI- and ML-enabled devices should address anticipated changes over time (including through continuous learning),[179] and using AI to produce information or data to support FDA decisions about drugs and biological products.[194] A considerable portion of this guidance was still out for public comment in draft form at the change in presidential administrations, which means that the agency will have the opportunity to consider public feedback in the context of new administration priorities and executive actions, including with respect to emerging issues related to generative AI.

As FDA builds upon this foundation, it should pay particular attention to the potential use of third-party resources. Agency leaders have expressed concern about whether FDA has sufficient in-house expertise and bandwidth to effectively assess the various technologies that will come before it for review[195] and have begun exploring the concept of using a network of AI "assurance laboratories," possibly based in academic medical centers, to support validation and other vetting activities.[196,197] This model could significantly expand FDA's capacity, but the concept has proved controversial. Critics have argued, among other things, that the large institutions needed to support this effort would be too prone to conflicts of interest, are ill-equipped to operate across geographically diverse (and locally regulated) healthcare settings, and could impede innovation.[198,199]

FDA will need to navigate this ongoing debate in the context

of evolving national strategies on advancing AI. In doing so, the agency should look to existing frameworks, including the core principles of other third-party review programs that FDA has previously implemented. For example, the 510(k) Third Party Review Program allows device manufacturers—on a voluntary basis—to have certain marketing applications reviewed by accredited Third Party Review Organizations before FDA makes a final determination, which can streamline the review process and conserve agency bandwidth.[200] While the 510(k) Third Party Review Program has struggled to provide consistent and high-quality reviews or reduce workload relative to regular submissions,[201] FDA should consider whether some variation on its voluntary framework could provide a useful model for third-party assessments of AI technologies — for example, by establishing a system in which using outside assessors is an available option alongside other, equally viable pathways. The agency should also consider, a designation program for validation methods akin to the program for designating platform technologies,[45] in which an approach intended to be used across multiple products could be evaluated and authorized by the agency to facilitate streamlined development of products deploying that approach.

Recommendation 5.3: Update FDA's approach to clinical decision-support software

As FDA updates its AI policies, one immediate priority should be to revise the guidance it finalized in 2022 on clinical decision support (CDS) software.[202] That guidance—which deviates sharply from the approach the agency proposed in 2019 during the first Trump administration—has been highly controversial. Stakeholders have expressed concern that the agency is subjecting beneficial software functions to regulatory burdens that Congress did not intend and, as a result, causing developers to make their software less useful to healthcare providers (HCPs), and limit innovations that would benefit patients in order to avoid triggering FDA pre-market review.[203–205] FDA should revise its approach to avoid this outcome and better align its policies with congressional intent.

CDS software can encompass a wide range of functions that support HCPs' decision-making in the course of delivering clinical care, such as tools that analyze information about a patient to help an HCP make a diagnosis, identify potential drug-drug interactions and match patient-specific information to relevant treatment guidelines. These tools have enormous potential to improve patient health outcomes by reducing errors and driving better treatments decisions.[206] One recent study found that a commercial large language model (LLM) AI chatbot was able to significantly outperform doctors using conventional tools when making a diagnosis,[207] which shows significant potential for these and other tools to support HCP decision-making when properly deployed.

Many of these CDS software functions are, by statute, exempt from FDA regulation as a medical device. In the 21st Century Cures Act of 2016, Congress created a safe harbor that exempted CDS software functions from FDA medical device regulation as long as they met certain criteria.[208] To qualify, a software function must:

- Display, analyze, or print medical information about a patient or other medical information (such as peer-reviewed clinical studies and clinical practice guidelines);
- Support or provide recommendations to an HCP about prevention, diagnosis, or treatment of a disease or condition;
- Enable the HCP to independently review the basis for the recommendations so that it is not intended for the HCP to rely primarily on any of the recommendations to make a clinical diagnosis or treatment decision regarding an individual patient; and
- Not be intended to acquire, process, or analyze medical images or signals from an in vitro diagnostic device or a pattern or signal from a signal acquisition system.[209]

Exempt software functions are still potentially subject to regulation under other authorities, such as state-level regulation of the practice of medicine, but they do not need to meet FDA's requirements for medical devices.

FDA's 2022 final guidance significantly narrowed the safe harbor.[202] For example, the guidance:

- States that, in order to limit the risk of "automation bias" (i.e., the tendency for humans to over-rely on suggestions from automated systems), FDA is applying the safe harbor only if the software recommends multiple options, as opposed to a single, specific recommendation. This distinction does not appear in statute.
- Imposes restrictions on the type of information that exempt software can analyze. According to the guidance, the safe harbor applies only if the software analyzes information about a patient of a type that would normally be communicated in a conversation between HCPs, or between patients and HCPs—an ambiguous restriction that does not exist in statute—or other medical information that is "independently verified and validated," a limitation that does not appear in the statute and might potentially exclude information from reliable real-world data sources, such as patient registries.

This approach would benefit from reassessment. First, by introducing limits on the safe harbor that do not appear in statute, the approach in the guidance may be legally vulnerable. In addition, the guidance could have the unintended impact of leading HCPs to use less fit-for-purpose AI tools to support their decision-making. As commercial AI tools proliferate, an increasing number of clinicians are using general-purpose tools to support their decisions; in one recent survey, a majority of physicians reported using general-purpose LLMs in clinical decision-making, including for uses like diagnosis support and checking drug interactions.[210] If developers decide to forgo releasing beneficial software functions due to the risk of regulation, HCPs may turn instead to tools not designed or optimized specifically for CDS use.