IMPROVING REGULATOR CAPACITY THROUGH ARTIFICIAL INTELLIGENCE

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Summary

The pharmaceutical industry frequently cites review delays and the limited availability of Food and Drug Administration (FDA) interactions as a key bottleneck in drug approval processes. While raising user fees to hire more staff is one solution, a more sustainable option lies in enhancing the productivity of existing FDA reviewers through artificial intelligence (AI). Proper integration of AI could significantly improve the FDA's capacity, benefiting industry, regulators, and patients.

Challenge and Opportunity

For decades, the FDA has faced resource constraints, resulting in slower review times, low sponsor interaction, and the infamous "drug lag" of the 1980s and 1990s. Recognition of this issue led to the passage of the Prescription Drug User Fee Act (PDUFA)[1] in 1992 to boost FDA's resources through user fees. However, with the coming biotech boom, the volume and complexity of applications may surge, straining the FDA further. Already, one new drug application averages about two million (paper) pages[2]. Simply increasing personnel is neither a sustainable nor scalable solution.

Accelerating the regulatory process has enormous value, as evidenced by the FDA's Priority Review Vouchers (PRV), which expedite the review process by four months, and sell for around $100 million[3] on the open market. Additionally, the COVID-19 pandemic demonstrated the critical importance of frequent, proactive communication between developers and the FDA. Pharma companies repeatedly point[4] to the FDA staff's frequent interactions and proactive guidance as an important driver in the rapid development and approval of COVID-19 vaccines. Enhanced communication with FDA reviewers can catalyze innovation, accelerate development, and help bring treatments to patients faster. Sponsors, who are often risk-averse due to the potential for regulatory delays or denials, tend to default to traditional trial designs. Regular interactions with FDA reviewers can help de-risk new methods and allow sponsors to benefit from the regulatory science expertise of reviewers.

This is where AI offers a transformative opportunity. AI tools have the potential to augment human expertise by automating routine, time-consuming tasks like data entry, document review, and cross-referencing—freeing FDA reviewers to focus on high-level analysis and critical decision-making. However, the introduction of any new technology must be approached with caution. One such concern with AI, particularly large language models, is the potential for "hallucinations,"[5] where the AI generates inaccurate or misleading information. Acknowledging these risks is essential to maintaining regulatory integrity. That said, when AI is carefully implemented with robust safeguards and ongoing human oversight, it might be able to significantly boost both the efficiency and quality of the review process. AI, equipped with appropriate checks, can assist in analyzing vast datasets, performing safety assessments, and identifying patterns that could otherwise be missed. Ultimately, AI should be seen as a complement to human judgment, enhancing the decision-making process without replacing it.

However, to fully harness AI's potential, the FDA must start planning now. This includes ensuring reviewers have access to the necessary tools, building internal expertise, preparing infrastructure for AI integration, and most importantly, maintaining public trust by validating AI assistance against reviewer benchmarks. With proactive measures, AI might vastly improve the FDA's capacity, leading to faster, more transparent approvals, and letting it keep pace with an increasingly AI-powered biotech industry.

Plan of Action

To ensure the FDA is prepared for AI, decisive steps must be taken by both the Agency and Congress. Below are our key recommendations:

Recommendation 1: Congress should require the FDA to report on AI procurement guidelines

To ensure the FDA is equipped to fully integrate AI into its regulatory framework, Congress should require the FDA to produce a report on its current procurement guidelines for AI and related software. This report should assess the availability of advanced AI tools, such as large language models (LLMs), for FDA reviewers. If the report identifies gaps in access, it should propose actionable reforms to streamline procurement processes and address any barriers.

Recommendation 2: The FDA Should Prioritize Data Readiness for AI Integration

To prepare for seamless AI integration, the FDA must prioritize organizing its internal data systems with robust access and permission hierarchies. This will ensure that sensitive regulatory data is properly structured, acces-

1 https://www.fda.gov/industry/fda-user-fee-programs/prescription-drug-user-fee-amendments#:~:text=The%20Prescription%20Drug%20User%20Fee%20Act%20(PDUFA)%20was%20created%20by,of%20certain%20prescription%20drug%20products.

2 https://www.accumulus.org/

3 https://sites.fuqua.duke.edu/priorityreviewvoucher/value/

4 https://www.nber.org/system/files/chapters/c15005/c15005.pdf

5 https://www.ibm.com/topics/ai-hallucinations

sible, and secure for future AI training and utilization. AI tools, particularly those designed for regulatory tasks, depend on clean, well-organized data to perform effectively. By categorizing and tagging datasets with clear access controls, the FDA can ensure that AI models are fine-tuned for specific regulatory needs while maintaining the highest standards of data security and privacy. This will also guarantee the confidentiality that sponsors expect from the FDA.

Recommendation 3: The FDA should Conduct an FDA Reviewer Benchmark Test

Before the FDA integrates LLMs into its submission review process, it must first validate their effectiveness in maintaining high regulatory standards. To do this, the FDA's Office of Digital Transformation (ODT[6]) and the Center of Excellence in Regulatory Science and Innovation (CERSI[7]) should conduct a formal benchmark test[8]. This evaluation would compare AI-assisted reviews to human-only reviewer decisions by applying AI tools to previously approved drug submissions. Similar tests have been used in other fields; for example, former FDA Commissioner Scott Gottlieb and Shani Benezra informally tested[9] LLMs on the United States Medical Licensing Examination (USMLE) Step 3 medical licensing exam, demonstrating AI's potential for complex decision-making, and many of the leading models have published performance benchmarks on similar medical knowledge questions. Publishing the results of such tests would foster transparency and build trust in AI's role within the FDA, ensuring both efficacy and reviewer buy-in.

Recommendation 4: HHS should bring AI experts into the FDA

Some of the aforementioned recommendations are within the FDA's current capabilities. Constructing a reviewer benchmark, however, may require specialized AI expertise that the Agency currently lacks. Thus, the U.S. Department of Health and Human Services (HHS) should establish an AI Corps (similar to the existing Department of Homeland Security AI Corps[10]), with dedicated experts placed across its ten agencies, including the FDA, to ensure the Agency is fully leveraging AI to improve regulatory processes and healthcare outcomes. This AI Corps member would bring technical expertise, ensuring that AI deployment is aligned with regulatory needs and that FDA reviewers can benefit from tools that optimize workflow, enhance data analysis, and reduce bottlenecks in the approval process.

Recommendation 5: The FDA should Foster AI Adoption through Targeted Reviewer Engagement

The FDA should actively engage its reviewers in AI adoption by hosting regular workshops that introduce AI tools and their potential to enhance regulatory processes. These sessions would create a space for AI experts and reviewers to collaborate on practical applications, ensuring AI policies are developed with reviewer input in mind. By familiarizing staff with emerging technologies, the FDA empowers reviewers to shape how AI can be effectively integrated within the Agency.

Conclusion

AI has the potential to transform the FDA's capacity, making it faster, more efficient, and better equipped to handle the growing complexity of medical submissions. By laying the groundwork now for AI integration, Congress and FDA can ensure that the FDA is not only prepared for the challenges ahead but also maintains its position as a global leader in regulatory innovation. Proactively adopting AI will enhance the FDA's ability to protect public health, foster innovation, and ensure that life-saving treatments reach patients without unnecessary delays.

6 https://www.fda.gov/about-fda/office-commissioner/office-digital-transformation

7 https://www.fda.gov/science-research/advancing-regulatory-science/centers-excellence-regulatory-science-and-innovation-cersis

8 https://www.ibm.com/think/topics/llm-benchmarks

9 https://www.aei.org/op-eds/how-well-can-ai-chatbots-mimic-doctors-in-a-treatment-setting-we-put-5-to-the-test/

10 https://www.dhs.gov/news/2024/06/25/dhs-hires-first-10-experts-ai-corps-recruiting-sprint

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