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AI's Potential to Deliver Better Patient Care and Accelerate Drug Discovery

Russ Altman[1]
Stanford University

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Executive Summary

AI is already improving care for patients and accelerating drug discovery. Properly deployed, these tools can make patient care more personalized, fair, and affordable, and sharpen drug discovery and development pipelines. However, healthcare systems need frameworks to evaluate and deploy safe AI tools while the drug discovery field needs stronger data sharing, adequate infrastructure, and an expert workforce. To realize the full potential of AI, policymakers should focus on several key areas.

AI to Improve Patient Care

- **Augmenting diagnosis and treatment:** The use of AI to analyze medical images is already well established. Beyond imaging, health systems are rolling out tools that read images and analyze records and notes to flag likely conditions for timely follow-up — prioritizing patients and surfacing risks earlier.
- **Improving the physician-patient relationship:** AI scribes that generate draft summaries of clinic visits within minutes can ease after-hours charting, reduce burnout, and help physicians focus their attention on patients.
- **Increasing patients' understanding and control of their care:** Specialized medical LLMs that can explain lab results in plain language or estimate the likelihood of developing a disease enable patients to better understand diagnoses and treatments.
- **Evaluating clinical effectiveness and safety:** We will only fully realize the benefits of AI tools if healthcare systems thoroughly vet them. Interdisciplinary evaluation, such as Stanford Health Care's multistakeholder process, should be standard.

[1] Kenneth Fong Professor and Professor of Bioengineering, of Genetics, of Medicine, of Biomedical Data Science and (by courtesy) Computer Science, Stanford University; Senior Fellow, Stanford Institute for Human-Centered Artificial Intelligence (HAI); Faculty Lead, Stanford Responsible AI for Safe and Equitable Health (RAISE-Health).

AI to Accelerate Drug Discovery

- **Improving privacy-preserving data collection and sharing:** Incentivizing hospitals nationwide to contribute secure, de-identified datasets would improve generalizability while protecting patients.
- **Investing in public computational infrastructure and research capacity:** Ensuring that government and academic scientists have adequate resources to build biomedical AI tools and pursue curiosity-driven research is critical to maintaining America's leadership in life-saving therapeutic innovation.
- **Strengthening regulatory evaluation capacity:** The FDA needs expertise to assess the novel ways drugs are developed with AI assistance. Academic partnerships offer one way for the FDA to obtain this specialized knowledge and expertise.
- **Developing an interdisciplinary workforce:** Training programs for biologists, clinicians, and computer scientists to validate and audit AI-generated predictions will be crucial to ensuring that AI augments human expertise rather than replaces it.

Statement

Chairman Cassidy, Ranking Member Sanders, and members of the committee, it is an honor to speak before you today.

As a board-certified physician who treats patients and a research professor who has spent three decades studying how AI can transform medicine, I am excited by the ways AI can improve healthcare. I also believe we have significant opportunities to develop this technology in ways that are responsive to the unique needs of this sector and to all the patients who depend on it.

Today I want to share insights on two critical areas: the state of AI applications for delivering better patient care and how AI is accelerating the drug-discovery process. I will highlight opportunities for congressional action to make these efforts stronger, safer, and human-centered. I should note that I am here in my personal capacity, and the views I share reflect my own professional experience.

The Promise of AI to Improve Patient Care

The use of AI to analyze medical images — detecting cancer in lung X-rays or melanoma in pictures of skin — were the first to herald the revolution of AI in medicine. However, there are now myriad ways AI is being applied at various points of the patient-care journey, from scheduling and triage to documentation support and patient communications. Properly validated and deployed, these tools will help care and treatment become more personalized, fair, and

affordable. Let me highlight three areas where AI has the potential to, and — in many cases — is already used to, transform patient care:

First, like many, I am excited about AI's ability to augment clinical diagnosis and treatment. Over 75% of FDA-cleared AI tools are for medical imaging — reflecting strong performance in pattern recognition.[2] However, AI's use in healthcare can be much broader. At Stanford Health Care, as in many systems, we are seeing the careful rollout of tools that both read images and analyze patient records and clinical notes to flag likely conditions, such as cancer and peripheral artery disease, for timely follow-up. Used this way, AI helps prioritize patients, surface risks earlier, and focus clinicians' attention where it matters most, without replacing professional judgment. My group has prototyped an AI system that uses electronic medical records and ancillary data to predict a patient's risk for every disease; our work is a first step toward a dashboard where a physician can not only see the patient's current diseases, but also those mostly likely to develop in the future.[3] We have also used AI to process genetic information to choose the medication most likely to work for an individual.[4]

Second, healthcare AI applications can improve the relationship between physicians and their patients. Hospitals and physicians across the country are rapidly adopting AI scribe tools that within minutes can generate transcripts and summaries of clinical conversations. Initial pilots have not only shown that these tools significantly reduce the burden of after-hours documentation work, which often contributes to physician burnout, but also improve the physician-patient relationship.[5] Many patients are already noticing that their physician is paying more attention to them during clinic visits, while physicians feel they can provide more effective and personalized patient care. The days when physicians pay more attention to a computer screen than to their patients may soon be over.

Third, AI can increase patients' understanding and control of their care using AI tools. Patients are eager to better understand their diagnoses, the progression of diseases, treatment plans, and medication side effects. We know that patients are going to general chatbots for medical information, much as they have used search engines in the past. Meanwhile, we physicians are often not as good as we think at explaining complex conditions in simple terms. Specialized medical LLMs that explain lab results in plain language or provide patients with an

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[2] Windecker D, Baj G, Shiri I, et al. Generalizability of FDA-Approved AI-Enabled Medical Devices for Clinical Use. *JAMA Netw Open*. 2025 Apr 1;8(4):e258052. doi:10.1001/jamanetworkopen.2025.8052.
[3] Yang L, Wang S, Altman RB. POPDx: an automated framework for patient phenotyping across 392 246 individuals in the UK Biobank study. *J Am Med Inform Assoc*. 2023 Jan 18;30(2):245-255. doi:10.1093/jamia/ocac226.
[4] McInnes G, Dalton R, Sangkuhl K, et al. Transfer learning enables prediction of CYP2D6 haplotype function. *PLoS Comput Biol*. 2020 Nov 2;16(11):e1008399. doi:10.1371/journal.pcbi.1008399.
[5] Shah SJ, Devon-Sand A, Ma SP, et al. Ambient artificial intelligence scribes: physician burnout and perspectives on usability and documentation burden. *J Am Med Inform Assoc*. 2024 Dec 5;32(2):375-380. doi:10.1093/jamia/ocae295.

estimate of the likelihood that they will develop a certain disease could be powerful tools for transforming patients' ability to feel empowered in their healthcare.[6]

While I am optimistic about these applications, **we will only fully realize their benefits if healthcare systems build interdisciplinary teams that thoroughly evaluate tools for clinical effectiveness and safety.**

At Stanford Health Care, we have established such a process where an interdisciplinary team of AI and medical experts works collaboratively to identify promising AI products and vet them before use in our healthcare system. We developed a novel process for evaluating model risk that uses a multistakeholder lens to weigh an AI technology's advantages and disadvantages, and to recommend whether it should be used in a clinical setting.[7] Our patient and family advisory council participates as a key stakeholder in these assessments, inviting a holistic conversation about the different trade-offs associated with an AI model. Recommendations don't necessarily stop deployment of a tool, but they do create a more robust conversation about how to train users and monitor it for efficacy.

Our tried-and-tested process shows that internal governance mechanisms are effective, scalable, and necessary. Every healthcare organization should put in place a similar process to guide the integration of AI tools in clinical settings.

Unique Challenges With Drug Discovery and How AI Can Help

The United States has long been a major source of novel therapeutics, benefiting patients and fueling the bioeconomy. But the drug discovery process has a high failure rate, which increases the expense and delays successful therapies. AI has the potential to dramatically accelerate the discovery and development of drugs. Indeed, we are already seeing it used by most pharmaceutical companies at different points in their discovery and development pipelines.

I am often asked, "Has AI discovered a new drug yet?" That is not the right question. The right question is, "Is AI being used appropriately and strategically to accelerate the creation of new drugs?" There, the answer is yes. AI helps scientists read and connect vast bodies of evidence, clarify how diseases work, and focus attention on the most promising ideas. The goal is not to replace expert judgment, but to sharpen decisions.

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[6] AI tool assists doctors in sharing lab results. Stanford.edu. Published 2025; Shmatko A, Jung AW, Gaurav K, et al. Learning the natural history of human disease with generative transformers. *Nature*. 2025 Sept 17:1-9. doi:10.1038/s41586-025-09529-3.

[7] Callahan A, McElfresh D, Banda JM, et al. Standing on FURM Ground: A Framework for Deploying Fair, Useful, and Reliable AI Models in Health Care Systems. *NEJM Catalyst*. 2024 Sept 18;5(10):CAT-24. doi:10.1056/cat.24.0131.

Drug discovery involves multiple complex stages: (1) understanding physiological defects that lead to disease, (2) identifying molecular targets, (3) refining therapeutic interventions, and (4) extensive testing from computer simulations through clinical trials. At each step, AI augments the abilities of biologists, chemists, engineers, and clinicians. My lab has developed AI systems that predict drug-target interactions,[8] identify existing FDA-approved drugs that could treat new diseases,[9] and analyze genetic markers to predict who will respond well or poorly to specific medications.[10]

General AI tools created by major vendors are powerful but not always well suited to detailed biological reasoning and complex data integration. To unlock the full potential of AI in drug discovery, we need more specialized AI systems built with reliable and plentiful data. This is where government support becomes crucial. I recommend four specific areas for congressional action:

First, we must improve privacy-preserving data collection and sharing. AI systems need representative biological and clinical data to identify drug targets and predict treatment responses. Yet for clinical data, research shows over 50% of AI systems are trained on data from just three states: New York, Massachusetts, and California.[11] Incentivizing hospitals nationwide to contribute secure, de-identified datasets under clear governance would improve generalizability while protecting patients. In parallel, ensuring that published biomedical literature (and the data upon which it is based) is openly available with full text will enable innovators to leverage AI for understanding biological knowledge.

Second, we must invest in public compute infrastructure and research to advance public-interest drug discovery. Academic and government labs pursue curiosity-driven research that can yield breakthrough treatments for rare diseases and conditions that industry overlooks. Yet these labs often lack the compute and funding needed for modern AI-driven drug discovery, for developing biomedical AI tools, and for basic science that uncovers disease mechanisms. Ensuring that government and academic scientists have these resources is critical — without them, we risk losing the public-benefit research that has historically transformed medicine and maintained America's leadership in life-saving therapeutic innovation.

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[8] Torng W, Altman RB. Graph Convolutional Neural Networks for Predicting Drug-Target Interactions. *J Chem Inf Model*. 2019 Oct 3;59(10):4131-4149.

[9] Sosa DN, Derry A, Guo M, Wei E, Brinton C, Altman RB. A Literature-Based Knowledge Graph Embedding Method for Identifying Drug Repurposing Opportunities in Rare Diseases. *Pac Symp Biocomput*. 2020;25:463-474; Sosa DN, Altman RB, Sosa DN, Altman RB. Contexts and contradictions: a roadmap for computational drug repurposing with knowledge inference. *Brief Bioinform*. 2022 Jul 18;23(4):bbac268. doi:10.1093/bib/bbac268.

[10] Smith DA, Arteaga SA, Sadler MC, Altman RB. Identifying DNA methylation sites affecting drug response using electronic health record-derived GWAS summary statistics. *Pac Symp Biocomput.* 2025;30:457-472. doi: 10.1142/9789819807024_0033.

[11] Kaushal A, Altman R, Langlotz C. Geographic Distribution of US Cohorts Used to Train Deep Learning Algorithms. *JAMA*. 2020 Sep 22;324(12):1212-1213. doi:10.1001/jama.2020.12067.

Third, we must strengthen the public sector's capacity to evaluate AI-accelerated drug discoveries. The FDA needs expertise to assess drugs developed with AI assistance, as AI will generate novel types of evidence for safety and efficacy. Academic partnerships offer one effective approach to building this capacity. The FDA-supported Center of Excellence in Regulatory Science and Innovation (CERSI), which I help lead, exemplifies this model — my group has worked directly with FDA scientists to develop AI tools for drug review. These collaborations provide the specialized knowledge, exposure, and experience needed to accelerate safe drug approvals while maintaining rigorous standards.

Fourth, we must develop a workforce equipped for safe and robust AI-driven drug discovery. Successful AI drug development requires professionals who understand biological science, medicine, and AI capabilities. We need training programs that teach biologists and clinicians not only how to use AI tools, but also how to validate and audit AI-generated predictions. Similarly, computer scientists must understand the complexities of drug development and the critical need for transparency in life-or-death decisions. This interdisciplinary workforce will guide the creation of AI tools that augment human expertise rather than replace it, ensuring patient safety throughout.

Conclusion

In conclusion, the promise of AI in healthcare is not distant — it is here today, improving how we care for patients and accelerating therapeutic discovery. But realizing this promise requires thoughtful collaboration among innovators, researchers, healthcare providers, users and patients, and policymakers. Policymakers can encourage stronger data sharing and invest in infrastructure and capacity to ensure that AI models are properly developed for specific use cases in the drug discovery process. And healthcare systems can better position themselves to take full advantage of AI's benefits for the patient experience by establishing interdisciplinary teams to evaluate AI tools.

Thank you and I welcome your questions.