

How Can AI Speed Life-Saving Cures to Patients?

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For patients facing cancer, heart disease, diabetes, or Alzheimer's, the wait for new therapies can feel endless. A promising discovery in a laboratory today could take a decade or longer to become an approved treatment. Tragically, many people who could benefit from tomorrow's cures do not have the time to wait. Such bottlenecks are familiar: Researchers painstakingly test and try – running experiment after experiment, discarding countless dead leads before a viable pathway emerges. Data collection and analysis demand extraordinary attention to detail. Even once a breakthrough occurs, peer review and regulatory processes add years. That methodical system safeguards patients but slows progress.

Imagine if the entire process could be compressed. Possibilities and hypotheses could be tested against each other in seconds instead of months. Data analysis could be accelerated by orders of magnitude. And researchers could spend less time buried in paperwork and more time pursuing new ideas. That is the goal of an emerging array of artificial intelligence (AI) tools.

AI IN ACTION

Major biopharmaceutical organizations and companies are investing accordingly into AI technology. Insilico Medicine has advanced an AI-designed small molecule into phase 2 trials for idiopathic pulmonary fibrosis (1). Sanofi's US\$5.2 billion collaboration with Exscientia continues to hit milestones, and a three-way effort with Sanofi, OpenAI, and Formation Bio is producing concrete tools for drug development (2, 3). Meanwhile, the AI-infrastructure layer is maturing: Recursion's NVIDIA-powered stack and Generate:Biomedicines' first-in-human AI-generated proteins compute meaningful biological insights (4). On the clinical-operations side, Sanofi, Formation Bio, and OpenAI have begun rolling out their Muse model to accelerate subject recruitment and streamline late-stage trial execution, including phase 3 multiple-sclerosis studies (5). The implications are profound. By compressing discovery cycles and lowering attrition, AI could shorten patent lives as competition arrives earlier than ever before, creating an access dividend for patients.

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AI also can reopen neglected frontiers. In the antibiotics sector, AI-guided discovery recently surfaced abaucin, a narrow-spectrum compound active against *Acinetobacter baumannii* — a World Health Organization (WHO) priority pathogen — reviving a field long considered to be uneconomical (6). And in protein therapeutics, generative models are informing first-in-human studies and facilitating billion-dollar partnerships, suggesting that design space — not just screening throughput — is changing (7).

But acceleration alone will not save lives. AI can generate promising leads faster than trial systems can validate them, risking an evidence backlog. Regulators are drawing the outlines of a remedy. The US Food and Drug Administration's (FDA's) 2025 draft guidance explains how AI-generated information can support decisions about the safety, efficacy, and quality of drugs and biologics (8). Similarly, the FDA's 2023 discussion paper on AI in drug manufacturing anticipated concerns for model validation, data provenance, and cloud oversight (9). Those materials mark the beginning of an AI-ready playbook, including expectations for model life-cycle management (e.g., documentation of training data, drift monitoring, and change control when algorithms and inputs evolve).

Biomanufacturing is becoming AI-native as well (10). Cell-line development (CLD) is shifting from incremental design of experiments (DoE) to model-informed design. Process analytical technology (PAT) combined with machine learning (ML) is moving monitoring toward prediction, and “digital twins” of upstream and downstream steps are starting to support real-time decision-making and accelerated deviation root-cause analysis (11). Real progress will be measured on factory floors: AI that can shorten technology transfer, flag scale-up risks before they can reduce yields, and accelerate batch-record deviation triage already is emerging in practice. Such capabilities augment rather than replace quality systems.

Access to computing capability will matter as AI technologies become prevalent. Computing power is a new kind of scientific capital, but it should not be a gating factor. Philanthropic investments are helping: the Chan Zuckerberg Initiative (CZI) is building one of the world's most powerful nonprofit graphics processing unit (GPU) clusters for life-science research, a step toward democratizing discovery tools (12). And the field is embracing open challenges: the 2025 Alzheimer's Insights AI Prize invited teams to build agentic AI that could turn existing data into new hypotheses — another way to accelerate breakthroughs without reinventing data collection from scratch (13).

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INNOVATION.

The future of AI in biopharmaceuticals will not be about machines replacing humans. It will be about “centaurs” – teams through which humans and AI work together, each amplifying the other’s strengths. Companies can treat every experiment as data for the next iteration. Regulators can codify standards for AI-native facilities and AI-assisted manufacturing. Funders can keep investing in shared computing power and open datasets. Clinicians can demand transparent tools that they can trust and explain.

AI already has delivered antibiotic candidates and first-in-human proteins – goals that were once thought to be unreachable. But the true promise lies in more than rapid discovery; it includes translating that speed into evidence, regulation, and adoption. The biopharmaceutical sector can transform from a business of serendipitous breakthroughs into one of systematic, global, and equitable innovation. For patients, that could mean something more important than price and profit: time.

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