

Artificial Intelligence and Biopharmaceutical Innovation



What Is Artificial Intelligence?

Artificial intelligence (AI): A machine-based system that can, for a given set of human-defined objectives, make predictions, recommendations, or decisions influencing real or virtual environments. Artificial intelligence systems use machine- and human-based inputs to perceive real and virtual environments, abstract such perceptions into models through analysis in an automated manner, and use model inference to formulate options for information or action.ⁱ



Opportunities for AI to Enhance Drug Discovery, Development and Manufacturing

A recent study showed that there has been an increasing number of drug and biologic submissions to the U.S. Food and Drug Administration (FDA) that incorporate AI elements over the past few years.ⁱⁱ AI is used as a tool to perform a variety of tasks throughout the drug development process with human oversight, including:

- Informing drug discovery, including new molecule design or screening existing molecules
- Enhancing clinical trial design (e.g., better matching of participants for trial recruitment)
- Dose optimization, which may include more efficiently finding a dose of a medicine that enables the best result for patients
- Endpoint optimization, which may include establishing potential novel endpoints associated with desired patient outcomes
- Increasing clinical trial efficiency, which may include analyzing outcome assessments, improving patient recruitment efforts, or creating a digital twin to predict what would happen to a specific participant in a clinical trial if they had received a placebo
- Post-marketing surveillance (e.g., safety monitoring)
- Advanced pharmaceutical manufacturing



The Biopharmaceutical Industry Is Leading the Way

There are a host of ways biopharmaceutical companies use AI as a tool to help scientists and researchers be more efficient and accurate, transforming processes across a drug's life cycle. Below are some examples of how various innovative biopharmaceutical companies have used AI:

Drug Discovery

- Partnered with AI platforms to use foundation model technologies to support the discovery of new antibody therapeuticsⁱⁱⁱ and created partnerships with AI companies to power drug discovery.^{iv}

Nonclinical Research and Drug Development

- Partnered with an AI company for early preclinical research to discover and create protein therapeutics across multiple therapeutic areas and modalities.^v
- Entered into a multiyear research effort with a computing capability company to optimize and accelerate the “lab in a loop,” where data from the lab and clinic feed AI models and algorithms to identify trends, make predictions, and generate new molecules.^{vi}
- Established a partnership with an AI company to help with mRNA research by using digital models to predict the strongest choice of particles to deliver vaccines to target cells.^{vii}

Clinical Research

- Collaborated to study the ability of an AI tool to identify individuals with cognitive impairment who may develop dementia, potentially leading to early detection and appropriate intervention.^{viii}
- Used AI to assist with maintaining data quality in the analysis of its COVID-19 vaccine clinical trial data so that it was ready to be analyzed by researchers within 22 hours – a process that can take more than 30 days.^{ix}
- Partnered with an AI company to analyze tissue samples to gain insight into disease progression and how an individual patient might react to various treatments.^x

Post-market Safety

- Innovative biopharmaceutical companies recognize that AI tools have the potential to help manage the use of chemotherapy drugs and help predict the optimal treatment regimen for cancer patients. AI can also assist with matching patients with the most effective treatments.^{xi}
- Used AI to significantly improve the efficiency of collecting and analyzing post-market safety data, ensuring collection of robust safety monitoring once a product is on the market.^{xii}

Manufacturing

- Partnered with a cloud computing company to leverage AI technology to boost operations and sustainability at an assembly and final product packaging facility.^{xiii}





Challenges for AI in Drug Development

Some of the current challenges for biopharmaceutical companies as they continue to use and incorporate AI in drug development include variability in the quality and representativeness of datasets for training and validation.ⁱ As such, data used to develop AI models should be relevant and reliable for the intended purpose. Not all AI models are explainable. Detailing the process and data used to develop an AI tool can help provide transparency. An AI model's performance can change over time, which necessitates life cycle maintenance to ensure such models remain credible.



Driving Toward a System that Enables Innovation

Opportunities to move AI forward in the development of medicines abound and must include investing in robust and fit for purpose data sets.^{xiv} As companies continue to use AI as a tool in drug development, additional considerations for the FDA to foster innovation in AI and protect public health include:

- **A risk-based regulatory framework:** As the FDA further considers its regulatory approach to the use of AI to support regulatory decision-making, it will be important for the FDA to maintain a sufficiently flexible, risk-based regulatory framework to foster continued innovation and support a patient-centric approach to drug development.
- **International harmonization:** Given the global nature of drug development, driving alignment across global regulators and achieving harmonization, where feasible, would help foster innovation by minimizing duplicative, overlapping, or inconsistent approaches to regulation. To accomplish this, the FDA can continue to work with other regulators, through appropriate venues such as the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH), the International Coalition of Medicines Regulatory Authorities (ICMRA), the International Medical Device Regulators Forum (IMDRF), and Pharmaceutical Inspection Co-operation Scheme (PIC/S).
- **Standards for the use of AI:** The use of standards can help enable transparency and understanding in drug development and review. The FDA can work with the National Institute of Standards and Technology (NIST) and other U.S. government entities in developing and/or adopting best practices and standards that can be used to facilitate understanding and share case studies across industries.
- **Coordination and stakeholder engagement:** FDA's ability to review AI-generated data and associated algorithms will require an integrated approach across FDA centers and a robust infrastructure. Continued engagement with relevant stakeholders to help inform the agency's use of AI, its approach to AI within its regulatory purview, and to disseminate learnings from the agency's experience with AI will be essential as well.

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