# The mixed potential of AI in drug discovery and development



The potential for artificial intelligence (AI) to transform drug discovery and development is generating a mixture of enthusiasm and skepticism among scientists, investors, and the public. There is a growing interest in leveraging AI technologies to design drugs and optimise clinical trials, particularly as investment and research in this field have surged in recent years. A prominent example includes AlphaFold, a groundbreaking AI platform that won the 2024 Nobel Prize for its ability to predict protein structures and innovate new proteins, showcasing how AI could significantly accelerate drug development processes.

However, not all experts share the same optimism. Some industry veterans have labelled the rush to embrace AI in drug discovery as "nonsense", calling for a "reality check" by underscoring that AI-generated drugs have yet to demonstrate an efficacy that can combat the substantial 90% failure rate of new drugs in clinical trials. They caution that while AI has proven effective in areas such as image analysis, its impact on drug development remains ambiguous and requires further scrutiny.

The article highlights the perspective of professionals experienced in both pharmaceutical science and programme management at the Defence Advanced Research Projects Agency (DARPA). They argue that while AI is not the "game-changer" some propose, nor is it wholly ineffective, it should be seen as a tool that can potentially address fundamental issues contributing to drug failure when utilised properly and competently.

Currently, the drug development process is notoriously lengthy and expensive, taking an average of 10 to 15 years and costing between US$1 billion to $2 billion to bring a single drug to market. AI is being applied across various stages of the drug development pipeline, including identifying biological targets, screening drug candidates, designing drug molecules, predicting toxicity, and selecting suitable clinical trial participants. Between 2010 and 2022, AI-focused startups successfully discovered 158 drug candidates, with 15 progressing to clinical trials. Notably, these candidates achieved completion of preclinical testing in as little as 30 months, a marked reduction compared to the traditional timeline of three to six years.

Nevertheless, there are significant challenges to AI's integration into this field. AI platforms may efficiently identify compounds that perform well in laboratory settings; however, the unpredictability of these candidates' success during clinical trials remains a prevalent barrier, as the majority of drug failures happen at this stage.

The article also notes that, unlike domains where high-quality datasets are plentiful—facilitating robust AI model training—drug development struggles with limited and often inadequate datasets pertaining to compounds and biological responses. This limitation poses a critical challenge that affects the reliability of AI in drug design, particularly given that slight alterations to a drug's structure can significantly influence its therapeutic effectiveness.

There is a concern that the current efforts in drug development, including those utilising AI, may be obscured by "survivorship bias." This refers to an overemphasis on minor improvements in the process rather than addressing the more significant systemic issues leading to drug failures. Historical innovations in drug development, such as computer-aided design and high-throughput screening, have enhanced specific stages over the past four decades; however, they have not effectively reduced overall drug failure rates.

Moreover, the complexities of the drug development pipeline are compounded by a disconnect in expertise. Many AI researchers focus on isolated tasks within the drug development process, often lacking familiarity with the comprehensive nature of drug development. Conversely, scientists adept in drug development may not possess the requisite training in AI and machine learning, leading to challenges in communication and collaboration.

To advance drug development using AI, the article suggests a new approach that tackles three interconnected factors driving most drug failures: dosage, safety, and efficacy. Researchers propose utilising a machine learning system to enhance the selection of drug candidates by integrating novel features including the drug's binding potency to targets and its distribution within different tissues. This could lead to more effective phase 0+ trials, which employ ultra-low doses to evaluate drug behaviours in patients, thereby optimising the selection of drug candidates while reducing costs associated with conventional "test-and-see" clinical trials.

In conclusion, while AI may not singularly revolutionise drug development, it possesses the capacity to address the underlying issues contributing to the high rate of drug failures and streamline the arduous journey to regulatory approval.

Source: [Noah Wire Services](https://www.noahwire.com)

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