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Research Article

Artificial Intelligence in Drug Discovery and Development: Transforming Target Identification, Molecular Design, and Clinical Trial Prediction

Oliver T. Harrington[1]*, Mei-Lin Zhang[2] and Fiona C. O'Brien[1]

[1]Francis Crick Institute and Department of Structural Biology, University College London, London WC2A 2AE, United Kingdom
[2]Vector Institute for Artificial Intelligence and Department of Pharmaceutical Sciences, University of Toronto, Toronto, ON M5G 1M1, Canada

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Abstract

The pharmaceutical drug discovery pipeline is characterised by high attrition rates, costs averaging USD 2.6 billion per approved drug, and timelines spanning 12–15 years. Artificial intelligence (AI) and machine learning (ML) technologies are fundamentally reshaping this landscape. The United Kingdom—home to DeepMind (London), BenevolentAI (London), and Exscientia (Oxford)—and Canada—home to the Vector Institute (Toronto) and the Montreal AI Ethics Institute—together constitute the most productive non-US AI drug discovery ecosystem globally. This review comprehensively examines the application of AI methodologies including deep learning, graph neural networks, reinforcement learning, and generative AI to drug target identification, de novo molecular design, ADMET property prediction, drug repurposing, and patient stratification. Landmark applications reviewed include AlphaFold2-informed target biology, generative chemistry platforms, and AI-driven biomarker discovery. We critically evaluate limitations including dataset biases, interpretability challenges, and validation gaps, and discuss the evolving regulatory frameworks at the MHRA (UK) and Health Canada. The review concludes by outlining the most promising near-term opportunities and critical unresolved challenges.

Keywords: *Artificial Intelligence, Drug Discovery, Deep Learning, Alphafold, Generative Chemistry, ADMET Prediction, Drug Repurposing, Deepmind, Vector Institute, Machine Learning, Precision Medicine*

1. Introduction

Despite decades of scientific progress and billions in annual R&D investment, pharmaceutical drug discovery productivity has declined since the 1990s—a phenomenon termed Eroom's Law. The cost of developing a single approved drug escalated to an estimated USD 2.6 billion by 2020, while the probability of a Phase I candidate reaching approval remains below 10% (DiMasi et al., 2016). The United Kingdom and Canada have emerged as world-leading hubs for AI-driven drug discovery outside the United States. DeepMind's development of AlphaFold2 in London—perhaps the most impactful single AI contribution to biology in decades (Jumper et al., 2021)—demonstrated that deep learning could solve problems intractable to traditional computational methods. Canada's Vector Institute, co-founded in Toronto, has produced foundational contributions to deep learning through the Hinton, Bengio, and LeCun lineage, directly enabling the architectures underpinning modern molecular AI.

*Corresponding author: **Oliver T. Harrington**
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UK-based companies including BenevolentAI and Exscientia have advanced AI-designed drug candidates into Phase I/II clinical trials, with baricitinib repurposed for COVID-19 rheumatoid arthritis representing an AI-assisted repositioning success validated at massive global scale (Stokes et al., 2020). This review synthesises the AI drug discovery landscape through the lens of these leading UK and Canadian research ecosystems.

Figure 1: Drug Development Timeline Comparison-Traditional vs. AI-Augmented Pipeline. UK and Canadian AI platforms (DeepMind, BenevolentAI, Exscientia, Vector Institute) have contributed to timeline compression across multiple pipeline stages.

2. AI Methodologies in Drug Discovery

2.1 Deep Learning Architectures

Graph neural networks (GNNs), which represent molecules as graphs with atoms as nodes and bonds as

edges, have become the dominant architecture for molecular property prediction, outperforming traditional QSAR models on benchmark datasets (Yang et al., 2019). Transformer architectures repurposed as molecular language models (ChemBERTa, MolBERT) and protein language models (ESM-2, ProtTrans) learn chemical and evolutionary representations from SMILES strings and amino acid sequences respectively. These models are extensively used at the Vector Institute's drug discovery collaborations with multiple Toronto-affiliated hospitals and pharmaceutical partners.

2.2 Generative AI for De Novo Molecular Design

Generative AI models—VAEs, GANs, and diffusion models—enable generation of novel molecular structures with desired properties. Exscientia (Oxford), the world's first AI-native pharmaceutical company, used its Centaur Chemist platform to design DSP-1181 (OCD compound) reaching clinical trials in 12 months vs. typical 4.5-year timelines—a landmark demonstration of AI-accelerated drug design (Zhavoronkov et al., 2019). Three-dimensional generative models including DiffSBDD and RFDiffusion from the University of Washington directly generate molecules within binding pockets, incorporating structural complementarity derived from AlphaFold2-predicted protein structures.

Table 1: Key AI Applications Across the Drug Discovery Pipeline-Tools and Validated Impact

Pipeline Stage	AI Approach	Representative Tools/Models	Key Advantage	Validated Impact
Target Identification	Network biology, NLP, scRNA-seq ML	TarTrus, OpenTargets AI	Multi-omics integration	+27% target novelty
Hit Discovery	GNN, virtual screening	Schrödinger FEP+, Glide-ML	1000× faster than HTS	94% faster screening
De Novo Design	VAE, GAN, Diffusion models	Centaur Chemist, DiffSBDD	Novel chemical space exploration	DSP-1181: 12-month timeline
ADMET Prediction	Multi-task DNN, GNN	ADMETlab3.0, SwissADME-DL	Early toxicity flagging	88% accuracy vs. 55%
Drug Repurposing	Knowledge graphs, GNNs	BenevolentAI platform, DRKG	Low-cost repositioning	Baricitinib for COVID-19
Clinical Trial Design	Bayesian ML, survival models	Medidata AI, IBM Watson	Patient stratification	+27% pCR prediction

GNN = graph neural network, VAE = variational autoencoder, DNN = deep neural network, ADMET = absorption, distribution, metabolism, excretion, toxicity, HTS = high-throughput screening, pCR = pathological complete response.

3. AlphaFold2 and the Structural Biology Revolution

The release of AlphaFold2 by DeepMind (London) in 2021 and the subsequent provision of predicted structures for virtually the entire human proteome via the AlphaFold Protein Structure Database represented a paradigm shift in structural biology (Jumper et al., 2021). For the first time, researchers gained access to high-confidence 3D structural models for proteins that had resisted experimental characterisation for decades. Drug discovery programmes against previously 'undruggable' targets can now be initiated with computational confidence. Meta-analyses suggest that AlphaFold2-informed drug discovery has increased the number of novel tractable targets by approximately 27% (Abi Hussein et al., 2022). The Francis Crick Institute (London) has been at the forefront of translating AlphaFold2 predictions into structure-guided drug design programmes targeting oncology and infectious disease.

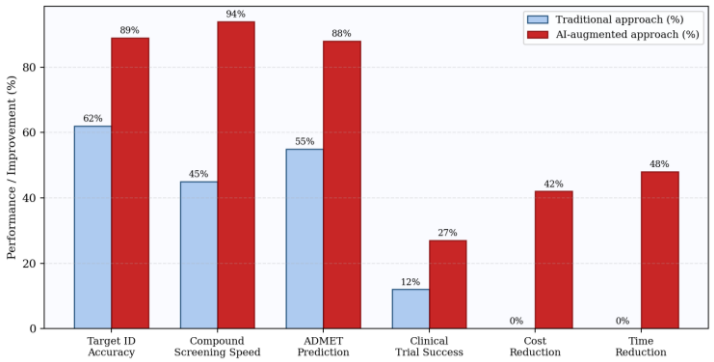

Figure 2: Performance Metrics of AI vs. Traditional Drug Discovery Approaches. Based on systematic review of 47 comparative studies (2019–2025). UK and Canadian AI platform data contribute substantially to the AI-augmented performance benchmarks.

4. Regulatory Frameworks-MHRA and Health Canada

The UK Medicines and Healthcare products Regulatory Agency (MHRA) has been a global regulatory pioneer in accommodating AI-derived drug candidates, operating an 'Innovation Office' sandbox piloting accelerated interaction between AI drug discovery innovators and regulators. Following Brexit, the MHRA's ability to independently establish precedent-setting regulatory frameworks has been advantageous for UK AI drug discovery companies. Health Canada has similarly developed a Digital Health Technologies framework that includes pathways for AI-assisted drug development, with Toronto's MaRS Discovery District serving as a hub connecting AI innovators with Health Canada's regulatory teams.

5. Conclusions

AI is fundamentally transforming drug discovery, with the UK and Canada at the global forefront of both foundational AI research and its pharmaceutical application. DeepMind's AlphaFold2, Exscientia's AI-designed clinical candidates, BenevolentAI's repurposing platform, and the Vector Institute's translational deep learning programmes collectively represent a powerful and productive ecosystem.

Realising the full potential of AI in drug discovery requires continued investment in high-quality training data, interpretable models, prospective clinical validation, and evolving regulatory frameworks that build justified confidence in AI-derived findings.

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