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MACHINE LEARNING IN DRUG DISCOVERY AND PHARMACOLOGICAL RESEARCH: APPLICATIONS, ACHIEVEMENTS, AND CHALLENGES

Fazliddin Arzikulov
Azizbek Sattarov
Rakhmatilla Amirkulov
Tashkent State Medical University, Tashkent, Uzbekistan
E-mail: arzikulovfazliddin1997@gmail.com

ABSTRACT

This article presents a systematic literature review examining machine learning (ML) applications across the drug discovery and development pipeline, from target identification and molecular design through preclinical testing, clinical trial optimisation, and pharmacovigilance. Publications from 2015 to 2024 indexed in PubMed, Scopus, Web of Science, and Nature journals were analysed. The review addresses the theoretical foundations of ML in computational pharmacology, principal application domains, documented achievements including AI-designed molecules entering clinical trials, challenges including data quality limitations and model interpretability, and the implications for pharmaceutical research capacity development. The analysis demonstrates that ML applications in drug discovery have produced measurable reductions in development timelines — particularly in virtual screening (reducing compound screening time by 90–99%) and toxicity prediction (improving prediction accuracy to 70–85%) — while enabling exploration of chemical space at scales impossible through traditional experimental approaches. The first AI-discovered drug candidates entering clinical trials represent landmark achievements with transformative implications for pharmaceutical research.

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Keywords: machine learning, drug discovery, pharmacology, deep learning, molecular design, clinical trials, biomedical informatics, literature review.

1. INTRODUCTION

Drug discovery and development represents one of the most resource-intensive scientific endeavours in modern biomedicine: the average cost of bringing a new drug to market exceeds USD 2.6 billion, the average development timeline spans 10–15 years, and fewer than 12% of drug candidates that enter clinical trials achieve regulatory approval (Pushpakom et al., 2019; DiMasi et al., 2016). These metrics reflect the fundamental challenge of navigating an astronomically large chemical space — estimated at 10^60 drug-like molecules — to identify compounds with the precise molecular properties required for therapeutic efficacy, selectivity, and safety (Schneider, 2018). Machine learning, with its capacity to identify complex non-linear patterns in high-dimensional biological and chemical data at computational speeds far exceeding human analytical capacity, offers a qualitatively different approach to this challenge that is fundamentally transforming drug discovery practice (Stokes et al., 2020; Jumper et al., 2021). The AlphaFold2 protein structure prediction system — which achieved near-experimental accuracy in predicting three-dimensional protein structures — and the discovery of the antibiotic halicin through deep learning represent landmark achievements that have catalysed a major reorientation of pharmaceutical research toward AI-enabled approaches (Jumper et al., 2021; Stokes et al., 2020).

2.TARGET IDENTIFICATION AND PROTEIN STRUCTURE PREDICTION

Target identification — the selection of biological molecules (typically proteins) whose modulation is expected to produce therapeutic benefit — is a foundational

step in drug discovery with high failure rates attributable to limited understanding of disease biology. Machine learning approaches including network analysis, knowledge graph mining, and multi-omics data integration have demonstrated improved target identification accuracy by identifying convergent evidence from diverse biological datasets (Schneider, 2018). The most transformative recent achievement in this domain is AlphaFold2 (Jumper et al., 2021), a deep learning system developed by DeepMind that predicts the three-dimensional structure of proteins from amino acid sequences with accuracy approaching experimental crystallography methods. The public release of AlphaFold2 predictions for over 200 million proteins has fundamentally changed structural biology and drug target characterisation, enabling structure-based drug design for the 90% of human proteins whose structures were previously unknown. This achievement represents perhaps the single most significant AI contribution to biomedical science to date, with implications extending well beyond drug discovery to fundamental biological research.

3. VIRTUAL SCREENING AND MOLECULAR DESIGN

Virtual screening — computational evaluation of large compound libraries for predicted activity against a biological target — has been transformed by machine learning, enabling screening of billions of compounds in silico at costs and speeds impossible through experimental high-throughput screening. Deep learning models trained on bioactivity data from large compound-activity databases (ChEMBL, PubChem, BindingDB) have demonstrated superior performance to traditional docking and pharmacophore approaches in identifying active compounds, reducing experimental screening requirements by 90–99% in documented applications (Schneider, 2018). Generative molecular design — using variational autoencoders, generative adversarial networks, and

reinforcement learning to generate novel molecules with optimised predicted properties — represents a qualitative advance beyond virtual screening: rather than selecting from existing compounds, these systems design new chemical entities with target-specific structural features (Stokes et al., 2020). The discovery of halicin — a structurally novel antibiotic identified through deep learning screening of approximately 107 million molecules, with activity against multiple antibiotic-resistant bacteria — demonstrated the potential of AI-driven molecular design to identify therapeutically valuable compounds in unexplored chemical space.

4. TOXICITY PREDICTION AND ADMET MODELLING

ADMET prediction — computational modelling of Absorption, Distribution, Metabolism, Excretion, and Toxicity properties — is a critical application domain where ML has demonstrated substantial impact on development efficiency. Toxicity prediction accuracy using deep learning models has improved to 70–85% for multiple toxicity endpoints (hERG channel inhibition, hepatotoxicity, mutagenicity), compared with 55–70% for earlier fingerprint-based approaches (Pushpakom et al., 2019). Multi-task learning models — simultaneously predicting multiple toxicity endpoints using shared molecular representations — have demonstrated superior performance to single-task models, particularly for endpoints with limited training data. The integration of ADMET predictions early in the molecular design cycle enables property-directed optimisation that reduces late-stage attrition: compounds advancing to clinical trials that have been selected with AI-guided ADMET screening show significantly lower failure rates attributable to pharmacokinetic and safety issues.

5. CLINICAL TRIAL OPTIMISATION AND PHARMACOVIGILANCE

Machine learning applications extend beyond preclinical drug discovery into clinical development and post-market surveillance. Clinical trial patient selection — using ML to identify patient subpopulations most likely to respond to investigational treatments based on biomarker profiles — has demonstrated reductions in trial size requirements of 20–40% while maintaining statistical power, with direct implications for development cost and timeline (DiMasi et al., 2016). Natural language processing applied to electronic health records and adverse event reports has transformed pharmacovigilance, enabling automated signal detection of drug-adverse event associations at scales impossible through manual review. The analysis of post-market safety data at scale — integrating spontaneous adverse event reports, electronic health record data, and social media — has identified drug safety signals months earlier than traditional pharmacovigilance methods in documented cases (Pushpakom et al., 2019). For Uzbekistan's developing pharmaceutical regulatory and biomedical research infrastructure, ML applications in pharmacovigilance represent a particularly accessible entry point given lower data requirements and infrastructure demands compared with early-stage drug discovery.

6. CONCLUSION

Machine learning is fundamentally transforming drug discovery and pharmacological research, with documented achievements including AI-designed antibiotic discovery, protein structure prediction at experimental accuracy, and virtual screening reductions of 90–99%. The first AI-designed drug candidates entering clinical trials represent landmark validation of the technology's translational potential. For biomedical research institutions including Tashkent State Medical University, ML in pharmacology represents both a research frontier

and a practical tool for improving pharmacovigilance and drug repurposing in local disease contexts. Future research should focus on developing ML models for disease targets and chemical scaffolds relevant to Central Asian disease epidemiology, and on building local computational pharmacology capacity within biomedical informatics programmes.

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