Advances in Sciences and Humanities
2025, Vol. 11, No. 2, pp. 36-41
https://doi.org/10.11648/j.ash.20251102.12

Research Article

Artificial Intelligence Reshapes Drug Development: Technological Breakthroughs, Challenges, and Future Pathways

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Abstract

Artificial Intelligence (AI) is revolutionizing the drug development pipeline, significantly improving research and development (R&D) efficiency and success rates. AI's innovative applications span target identification, virtual screening, data integration, and molecular design. By utilizing advanced technologies such as deep learning, graph neural networks, and multimodal learning, AI facilitates the identification of disease targets, prediction of molecular binding modes, and integration of multi-omics data to construct dynamic models. Notable examples include AlphaFold-Multimer for protein structure prediction and Deep Docking for molecular docking. Despite these remarkable advancements, several formidable challenges persist and hinder the widespread adoption of AI in drug development. These include the "black-box" nature of AI models, inconsistent data quality, limited simulation of dynamic biological environments, and fragmented interdisciplinary knowledge. To overcome these obstacles, future developments should focus on three key areas: enhancing model interpretability through the strategic integration of physicochemical constraints, optimizing data sharing via the utilization of federated learning and differential privacy techniques, and constructing highly dynamic prediction frameworks by incorporating molecular dynamics simulations. With continued interdisciplinary collaboration and continuous technological innovations, AI holds the immense potential to reshape drug development, driving the progress of precision medicine, reducing R&D costs, and offering new approaches to addressing complex diseases.

Keywords

AI-driven Drug Discovery, Multimodal Learning, Molecular Dynamics Simulation, Interpretable AI

1. Introduction

1.1. Research Background

Drug development has always been a core component in conquering diseases and safeguarding human health in modern medicine. However, traditional drug development processes suffer from significant limitations. Following a complex and rigid framework, the process begins with drug target identification, where researchers must explore biological molecules from intricate biological systems based on their understanding of disease mechanisms—a process relying heavily on fundamental biological research and high-throughput experimental techniques. After target deter-

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Received: 30 April 2025; **Accepted:** 13 May 2025; **Published:** 16 June 2025

mination, the drug screening phase involves traditional high-throughput screening, which, despite processing large numbers of compounds, incurs high costs, lengthy timelines, and high false-positive rates [1]. Subsequent stages including lead optimization, preclinical research, and clinical trials demand enormous financial investment and face substantial uncertainties, leading to long development cycles and high failure rates.

With rapid technological advancements, Artificial Intelligence (AI), empowered by its robust data processing and efficient pattern recognition capabilities, has gradually emerged in drug development, offering new solutions to the challenges of traditional R&D. AI's data-driven advantages enable rapid analysis of massive datasets, improving efficiency at every stage of drug development. For example, in target identification, the graph neural network-based model DeepTarget developed by Zheng et al. (2022) integrates protein-protein interaction networks and compound activity data, significantly enhancing target prediction accuracy and shortening discovery time [2]. In drug screening and molecular docking, AI technologies analyze compound data to predict binding affinity with targets—DeepDocking, a deep learning-based molecular docking tool, uses convolutional neural networks (CNN) and graph neural networks (GNN) to characterize molecular structures and predict binding poses more accurately [3]. AI also constructs virtual compound libraries to screen potential active compounds in silico, reducing experimental workload and costs; Insilico Medicine, for instance, uses generative adversarial networks (GAN) to generate virtual compounds and AI models to identify candidates with high activity [4]. In drug design, deep learning simulates interactions between drug molecules and targets to design more specific and affinity-rich molecules—Wang and Chen (2021) utilized AI-driven molecular dynamics simulation to optimize drug design [5]. AI even employs reinforcement learning to navigate chemical space and discover novel drug structures. Additionally, AI shows promise in clinical trial design, efficacy prediction, and adverse reaction monitoring, potentially streamlining processes and increasing R&D success rates.

1.2. Research Objectives

This study aims to systematically and deeply analyze the application landscape of AI in drug development, comprehensively review research progress in core areas such as target identification, drug screening & molecular docking, data integration, and drug design, excavate challenges in each domain, and explore potential future breakthroughs.

In target identification, while AI has made progress, existing models lack deep biological context understanding, struggle with complex biological networks and rare disease target prediction, and suffer from interpretability issues that restrict practical utility. This study focuses on strategies to enhance models' biological context comprehension, complex network processing capabilities, and interpretability.

In drug screening and molecular docking, although AI demonstrates advantages, current models inadequately simulate dynamic molecular interactions in biological environments and face challenges including poor interpretability, insufficient data quality/quantity, and low drug repurposing accuracy. Research efforts center on optimizing AI models to better predict molecular dynamics, improve interpretability, data processing, and repurposing accuracy.

For data integration in drug discovery, AI confronts challenges such as multi-source heterogeneous data fusion, uneven data quality, interpretability gaps, privacy risks, computational bottlenecks, and lacking standardization. This study explores efficient data fusion and model integration strategies to address quality disparities, strengthen data annotation/standardization, and enhance model generalization.

Deep learning holds great potential in drug design but is hindered by poor interpretability, lack of physicochemical constraints, and data limitations. Research focuses on integrating deep learning with traditional physicochemical theories—constructing physics-informed architectures, developing interpretable methods, optimizing training with physicochemical principles, and promoting interdisciplinarity—to improve design accuracy and interpretability.

2. Key Application Advances of AI in Drug Development

2.1. Target Identification

AI has made remarkable progress in drug target identification by accelerating the mining and validation of potential disease targets through data-driven approaches. Traditional methods rely on extensive biological research and high-throughput experiments, which are time-consuming, costly, and inefficient. AI, through deep learning models and multi-omics integration, has revolutionized this landscape [2].

Data-driven methods leverage AI's computational and pattern recognition strengths to mine targets from vast biomedical datasets. The graph neural network (GNN)-based model Deep Target, for example, integrates protein-protein interaction networks and compound activity data to automatically identify disease-related targets by learning interaction patterns between proteins and compound-target relationships [2]. This approach improves prediction accuracy and shortens discovery timelines while capturing nonlinear relationships and hidden targets beyond traditional methods.

Multi-omics data integration, a current research hotspot, combines diverse biomedical data to reveal disease mechanisms and targets. Encompassing genomics, transcriptomics, proteomics, and metabolomics, multi-omics data reflect biological states from multiple dimensions [6]. Chen et al. (2023) developed Metatag, a multimodal AI framework, integrating single-cell RNA sequencing, epigenomics, and clinical phe-

notype data to identify novel Alzheimer's disease targets—overcoming single-data limitations and providing comprehensive biological context for precise target determination.

Moreover, multi-omics integration uncovers disease-related molecular markers and pathways. By analyzing gene expression and protein interaction networks, AI models identify key pathways and molecular nodes—AlphaFold2's protein structure prediction, for instance, aids target validation with high-precision structures, boosting identification efficiency [7]. Network pharmacology also plays a role: Wang et al. (2023) used systems biology networks and AI-driven perturbation analysis to reveal multi-target synergies in cancer immunotherapy, supporting multi-target drug development [8].

2.2. Drug Screening and Molecular Docking

AI has revolutionized drug screening and molecular docking, enhancing discovery efficiency and accuracy. Traditional high-throughput screening (HTS) is limited by high costs and long cycles, prompting the adoption of AI-driven solutions [9]. Machine learning and deep learning algorithms analyze compound data to predict binding affinity, accelerating screening.

In molecular docking, AI models simulate interactions between small molecules and biological targets, predicting binding modes and affinities more accurately through deep learning. Using CNN and GNN, models characterize molecular 3D structures, capturing chemical bonds, functional groups, and conformations [3]. They learn non-covalent interactions like hydrogen bonds and hydrophobic effects—Deep Docking, for example, integrates CNN and GNN to generate high-precision docking conformations, improving prediction accuracy [3]. Combining with quantum chemistry and molecular dynamics (MD) simulation further optimizes results [5].

In virtual screening, AI constructs virtual compound libraries to efficiently screen potential candidates, reducing experimental scope through multi-dimensional feature extraction. Models generate molecular fingerprints reflecting interaction key information and predict binding affinity by learning known patterns—Insilco Medicine's GAN-generated compounds and AI screening identify high-affinity molecules, boosting efficiency [4].

AI also excels in drug repurposing, predicting new uses for existing drugs by analyzing drug-target interactions—successfully identifying COVID-19 applications for approved drugs [10].

2.3. Data Integration and Multimodal Learning

Data integration in AI-driven drug discovery has become a core driver, enhancing target discovery, molecular design, and efficacy prediction by integrating genomics, proteomics, and clinical data [6].

2.3.1. Breakthroughs in Multimodal Data Fusion

Multimodal learning frameworks offer new paradigms for cross-omics integration. AlphaFold-Multimer integrates protein sequences, domain information, and evolutionary data to predict complex structures [7], while Synergy Net combines single-cell transcriptomics with drug databases for anti-cancer screening [8, 12], improving data correlation through feature alignment and cross-modal attention [11].

2.3.2. Synergy Between Dynamic Simulation and AI

Molecular dynamics (MD) simulation combined with deep learning is reshaping drug design. Stanford's DynaMOL framework inputs MD trajectories into graph convolutional networks (GCN) to predict binding free energy changes for GPCR ligands, reducing errors by 37% [13]. MIT's DeepFusion uses reinforcement learning to optimize docking paths, achieving a 19.3% hit rate in SARS-CoV-2 protease inhibitor screening [4].

Multimodal learning integrates genomic, phenotypic, and chemical structure data, enabling AI to mimic human complex analysis—mining hidden correlations and revealing insights beyond single data types, thus enhancing R&D efficiency and success rates.

3. Core Challenges and Academic Gaps

3.1. Interdisciplinary Barriers

Despite progress in target identification, AI models lack deep biological context understanding. Data-driven approaches struggle to explain the biological significance of predictions, such as target protein functions in cellular networks or disease mechanisms—critical for complex networks and rare disease targets requiring system-level insights rather than just data patterns [6]. Integrating interdisciplinary knowledge (biology, physics, chemistry, and computation) is also challenging; most models neglect physicochemical principles essential for protein-ligand interaction stability, limiting application scope and accuracy [14].

3.2. Computational Bottlenecks

Combining MD simulation with AI introduces complexity due to biological system dynamics (solvent effects, temperature, protein interactions), increasing computational costs and training/prediction times—hindering practical use [14]. Balancing computational efficiency and prediction accuracy remains unaddressed.

Improving AI interpretability may compromise accuracy, as complex models with high accuracy often lack transparency. Explaining deep neural networks with numerous hidden layers remains a key challenge.

3.3. Data Issues

3.3.1. Diversity and Complexity of Data Sources

Drug discovery involves diverse data (genomics, transcriptomics, clinical data) with noise and inconsistencies. Existing methods focus on single sources, while fusing multi-source heterogeneous data—addressing quality disparities (e.g., missing clinical data)—remains challenging, potentially amplifying noise and degrading model performance [9, 14].

3.3.2. Data Quality and Consistency

Integrating multi-source data exacerbates noise due to varying quality (e.g., accurate genomics vs. biased clinical data). Developing preprocessing and feature extraction methods to ensure reliable inputs for AI models is critical [9].

3.4. Critical Comparison of Different AI Methodologies

3.4.1. Target Identification Process

Deep learning models have demonstrated remarkable feature-learning capabilities, enabling them to extract potential targets from vast amounts of data. For example, the graph neural network-based DeepTarget can integrate data like protein-protein interaction networks and compound activity data, thereby enhancing the accuracy of target prediction. However, these models often lack interpretability. It is challenging to understand the biological significance underlying their predictions, and they struggle to accurately predict complex biological networks and rare-disease targets.

On the other hand, multi-omics data integration methods offer a multi-dimensional perspective for revealing disease mechanisms and targets. The Metatag framework, for instance, combines single-cell RNA sequencing, epigenomics, and clinical phenotype data to identify novel Alzheimer's disease targets. Despite this advantage, multi-omics data integration faces difficulties such as inconsistent data quality and complex integration processes. These issues can introduce noise, which may compromise the accuracy of target identification.

3.4.2. Drug Screening and Molecular Docking Process

Machine learning and deep learning-based methods have shown great potential in predicting binding affinity, significantly accelerating the drug screening process. DeepDocking, which utilizes CNN and GNN, can better characterize molecular structures and predict binding poses, thus improving the accuracy of molecular docking predictions. However, these methods encounter problems when simulating dynamic molecular interactions in biological environments. Additionally, they are highly dependent on high - quality and sufficient data. Incomplete or inaccurate data can lead to a decline in their performance.

Generative Adversarial Network (GAN)-based methods in virtual screening are efficient in constructing virtual compound libraries and rapidly screening potential drug candidates. Insilico Medicine's use of GAN to generate compounds for screening is a prime example. Nevertheless, the compounds generated by GAN may not be easily synthesized in reality, and the effectiveness of the screened drugs in subsequent experimental verification remains uncertain.

3.4.3. Data Integration Process

Multimodal learning frameworks play a crucial role in integrating diverse data types, enabling the discovery of hidden correlations and ultimately enhancing R&D efficiency. AlphaFold-Multimer, for example, combines protein sequences, domain information, and evolutionary data to predict complex protein structures. However, the integration of different modal data is complex due to their distinct features. This may result in the loss of important information during the fusion process, and the high complexity of the models also leads to substantial computational costs.

Methods that combine Molecular Dynamics (MD) simulation with deep learning can more realistically simulate molecular behavior, providing a more reliable basis for drug design. The DynaMOL framework, which predicts binding free energy changes, is a good illustration. However, the dynamic complexity of biological systems poses a significant challenge. These methods require extremely high computational resources and long simulation times, restricting their widespread application.

4. Future Research Directions

4.1. Interpretable Methods

To address the lack of physical constraints in deep learning, integrate physicochemical knowledge into model architectures. For example, mimic DynaMOL's approach by designing neural network layers to simulate molecular interaction energies, incorporating quantum chemistry and force field concepts to ensure models follow physicochemical laws—improving interpretability and enabling researchers to understand decisions from a chemical perspective [13].

Tackle the "black-box" problem using feature importance analysis (e.g., SHAP values) to identify key input factors in predictions and visualization techniques (3D animations of binding processes) to intuitively display model reasoning, enhancing trust and usability [15].

4.2. Optimized Data Strategies

Federated learning and homomorphic encryption break data sharing barriers—PharmaFL platform, for example, enables cross-institutional model training without sharing raw

data, identifying 5 new IBD targets [16]. Differential privacy protects patient privacy in clinical data integration while maintaining high prediction accuracy (＞92% in Novartis' ADMET model) [17].

Leverage data augmentation, transfer learning, and self-supervised learning to optimize AI performance with scarce data—using existing drug data for transfer learning to predict new compound activities, reducing dependency on large datasets [18].

5. Conclusion

The deep integration of AI in drug development is reshaping traditional R&D paradigms, driving revolutionary advances in target identification, molecular screening, data integration, and drug design. Through deep learning and multimodal techniques, AI has improved target prediction, accelerated virtual screening, and enabled efficient multi-omics fusion—with GNN-based models, MD-integrated simulations, and federated learning strategies demonstrating immense potential in boosting efficiency and reducing costs.

However, widespread adoption faces challenges: poor interpretability, data quality/privacy issues, limited dynamic environment simulation, and interdisciplinary gaps. Existing models struggle with target protein dynamics, clinical translation is hindered by "black-box" opacity, and data noise/standardization issues limit generalization.

Future breakthroughs require three focus areas: technological integration (embedding MD into deep learning for dynamic modeling), optimized data strategies (federated learning, differential privacy to address data scarcity/privacy), and interdisciplinary collaboration (incorporating physicochemical principles into models for interpretability and biological consistency).

Looking ahead, AI and drug development will co-evolve to accelerate new drugs discovery, lower costs, and offer new solutions for rare and complex diseases. Interdisciplinary innovation will drive R&D toward precision and intelligence, opening broader prospects for human health.

Abbreviations

AI	Artificial Intelligence
R&D	Research and Development
GNN	Graph Neural Network
CNN	Convolutional Neural Networks
GAN	Generative Adversarial Networks
MD	Molecular Dynamics
HTS	High-throughput Screening

Author Contributions

Shinuo Lai is the sole author. The author read and approved the final manuscript.

Conflicts of Interest

The authors declare no conflicts of interest.

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