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A Review on the Significance of Artificial Intelligence in Drug Discovery and Pharmacology

Ravi Kumar Bogireddy*, Naga Venkata Divya Bokka, Samshad Nafisha Mohammad, Anil Kumar Bondalapati

Department of Pharmaceutics, GIET School of Pharmacy, Rajahmundry, Andhra Pradesh, India.

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Corresponding Author:
Mr. Ravi Kumar Bogireddy
Department of Pharmaceutics,
GIET School of Pharmacy,
Rajahmundry,
Andhra Pradesh, India.
Email: ravikumarbogireddy@gmail.com

ABSTRACT

Drug discovery is associated with enormous expenses in terms of financial resources required to introduce one innovative medicine into market. For instance, introducing new drugs is estimated to cost more than $2.6 billion dollars while the process may take up to 10-15 years and 90% of drugs fail during clinical trials. There is another challenge associated with drug discovery. Bacteria are developing increasing resistance to current antibiotics. Therefore, there is an urgent need to develop innovative approaches to drug discovery and development, which have been facilitated by the rapid development of artificial intelligence. Machine learning, deep learning, natural language processing and reinforcement learning are some applications of AI relevant for pharmaceutical industry. Those methods can be used for target identification and validation, molecular designing, virtual screening and docking, ADMET prediction, clinical trials, etc. This systematic review aims to analyze the AI applications in the process of drug discovery. It becomes evident from this systematic review that artificial intelligence has become an integral part of drug discovery and pharmacology. The existing literature have demonstrated some improvements in drug discovery process, achieved by using AI methods such as discovery speed, hit rates, ADMET prediction accuracy, or clinical trial optimization, among others. While there are various challenges to overcome in the field of AI-assisted drug discovery, such as data biases, lack of interpretability, experimental validation gap, or regulatory considerations, the best way going forward is to look at AI as an augmentative intelligence, rather than replacement for medicinal chemists.

Introduction

Drug discovery is associated with enormous expenses in terms of financial resources required to introduce one innovative medicine into market. For instance, the estimated cost to introduce a new drug into the market is more than $2.6 billion dollars while the process may take up to 10-15 years and 90% of drugs fail during clinical trials. There is another challenge associated with drug discovery. Bacteria are developing increasing resistance to current antibiotics. Therefore, there is an urgent need to develop innovative approaches to drug discovery and development, which have been facilitated by the rapid development of artificial intelligence [1].

Machine learning, deep learning, natural language processing and reinforcement learning are some applications of AI relevant for pharmaceutical industry. Those methods can be used for target identification and validation, molecular designing, virtual screening and docking, ADMET prediction, clinical trials, etc. This systematic review aims to analyze the AI applications in the process of drug discovery [2].

AI Applications in Drug Discovery

i. Target Identification and Validation

Target identification is considered to be the first step of the drug discovery process. At this step, AI has a huge potential as it facilitates the identification of the most effective treatment targets. The integration of various genomic, proteomic and phenotypic datasets through graph neural network models has shown that

machine learning can help identify drug targets 35% more accurately when compared to traditional bioinformatics methods.

Natural language processing techniques can be useful at the stage of target validation. Transformer-based natural language processing algorithms (BioBERT and PubMedBERT) can be used to identify disease-gene associations and formulate novel mechanistic theories based on the mining of large biomedical literature datasets.

The AlphaFold2 model created by DeepMind has revolutionized the field of structural biology by achieving extremely high accuracy in predicting 3D structures of proteins, making structural modeling available for target validation when experimental data was lacking [3].

ii. Molecular Design and De Novo Drug Generation

Generative AI models offer completely new approaches for molecular design and synthesis. Variational autoencoders (VAEs), generative adversarial networks (GANs) and transformer architecture (MolGPT) are capable of designing novel molecular compounds with desirable pharmacological properties. Large databases of known chemical entities (ChEMBL and ZINC) form the basis for training generative models that produce novel drug-like molecules meeting given criteria.

Reinforcement learning algorithms can also be used to optimize molecule generations according to various objectives, such as binding affinity, synthetic feasibility, metabolic stability, etc. In one of the landmark studies in this area, researchers managed to synthesize AI-created inhibitors of DDR1 kinase in just 46 days after designing them which is about 10x faster than usual. Diffusion models for molecule generation represent the latest advancement, as they generate 3D conformers [4].

iii. Virtual Screening and Molecular Docking

Traditional high-throughput virtual screening, involving docking of large compound libraries with hundreds of millions of molecules, can be significantly improved through the use of ML-based scoring function models that replace or complement physicochemical scoring functions, resulting in huge increases in speed and hit rates.

There is no need to dock molecules when using advanced deep learning tools, such as EquiBind and DiffDock, which are capable of blind docking and achieving similar accuracy as computationally expensive techniques.

Moreover, the representation of molecules as graphs and applying them to MPNN (Message Passing Neural Networks) models allows capturing more molecular attributes than traditional fingerprint descriptions. AI enhanced virtual screening algorithms showed an average enrichment factor of 8.2 over random screening and 2.5 over traditional docking approaches, as reported in various reviewed papers [5].

iv. ADMET Prediction and Drug Safety

Early prediction of pharmacokinetic and toxicological characteristics is crucial for avoiding failure of a drug candidate during the later phases of its development, such as clinical trials. Numerous AI models have been created for estimating various ADMET parameters, using large curated datasets from FDA, ChEMBL and ToxCast. Multi-task learning methods have proven to be superior to other models because they predict multiple targets simultaneously.

When using Graph Convolutional Networks (GCNs) in the context of ADMET prediction, average AUC-ROC reached 0.87. Particularly, the predicted liver toxicity models have shown sensitivities up to 80%. Therefore, AI is becoming a key instrument in the drug design pipeline and predictive models for drug safety are gradually becoming prominent in the process of drug discovery [6].

AI in Clinical Pharmacology and Trial Optimization

AI technologies are also increasingly becoming significant in various phases of clinical trials. AI algorithms are capable of patient stratification by analyzing electronic health records (EHRs) and genomic data, facilitate finding biomarker-defined patient populations that are most responsive to a specific therapy. This greatly increases power and decreases the number of participants required in trials.

In addition, predictive models for estimating the likelihood of successful completion of a clinical trial can help make important portfolio decisions. Using such predictive models on the data from ClinicalTrials.gov, researchers reached ~70% accuracy of predicting the passage of the trial from phase II to phase III. Other applications of AI in clinical trials include adverse event prediction and post-marketing surveillance via NLP (Natural Language Processing) models for FDA Adverse Event Reporting System (FAERS). Federated learning can be used in training of models on distributed clinical data, without the risk of breaching patient confidentiality [7].

Challenges and Limitations

i.Data Quality and Bias

AI is essentially dependent on the availability and quality of data that it is trained on. Public databases of biological activity data contain biases, including those related to assay variability, publication bias favoring positive results and overrepresentation of specific classes of drugs and molecular scaffolds. Biases in data lead to underrepresentation of particular types of targets, including those involved in neglected tropical diseases.

ii.Model Interpretability and Explainability

The "black-box" problem of advanced AI techniques, such as deep learning architectures, remains a considerable issue preventing their implementation in regulated pharmaceutical industry. Med chemists and regulatory authorities require explanation for predictions provided by a model in terms of mechanisms. Currently, SHAP (SHapley Additive exPlanations) methods, attention visualizations and GCNExplainer models are developed to achieve better explainability. However, interpreting these explanations in the chemical context is still a challenging task.

iii.Experimental Validation Gap

One of the problems that persist among reviewed studies is the low proportion of works that involve experimental validation of predictions made. While AUC-ROC and enrichment factors can indicate performance of an algorithm, they are only proxies for the real drug discovery value. Bridging the gap between computation and experiment in drug discovery requires the development of benchmarks, validation platforms and cooperation between academia and industry.

iv.Regulatory Considerations

The issue of regulatory considerations cannot be ignored in discussing AI-assisted drug discovery. Regulatory bodies such as FDA or EMA are currently working on guidelines for AI-based software as a medical device (SaMD) and AI-supported drug development projects. The FDA's AI Action Plan (2025) offers initial guidance in this regard. One of the main challenges is that of ensuring reproducibility, avoiding model drift and explaining the workings of AI model in regulatory submission [8-14].

Future Directions

One direction in which AI will continue to develop in drug discovery is multimodality. Combining multiple sources of information in the same deep learning architecture, such as genomic, proteomic, structural and metabolomic data,

along with clinical data is expected to yield promising results. Another future direction pertains to foundation models, which are trained on large databases of molecules and then can be fine-tuned on small datasets for specific purposes. Physics-informed neural networks, which are capable of modeling molecular systems based on quantum chemistry, thermodynamics and kinetic laws, will help increase reliability of AI-generated molecular compounds.

The combination of AI and automation of experimental processes, such as robotic synthesis, HTS and automated bioassays will allow creating closed-loop discovery platforms. Quantum machine learning models are expected to bring unprecedented computational power to drug discovery tasks, although it is still premature to assess their utility. From the translational standpoint, there should be increased use of federated learning and creation of shared benchmark datasets, such as MoleculeNet or TDC (Therapeutic Data Commons).

Conclusion

It becomes evident from this systematic review that artificial intelligence has become an integral part of drug discovery and pharmacology. The existing literature have demonstrated some improvements in drug discovery process, achieved by using AI methods such as discovery speed, hit rates, ADMET prediction accuracy, or clinical trial optimization, among others. While there are various challenges to overcome in the field of AI-assisted drug discovery, such as data biases, lack of interpretability, experimental validation gap, or regulatory considerations, the best way going forward is to look at AI as an augmentative intelligence, rather than a replacement for medicinal chemists.

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