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Artificial intelligence (AI) for drug discovery and optimization

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ABSTRACT:-
Artificial intelligence (AI) is rapidly transforming drug discovery and development, shifting from traditional trial-and-error approaches to data-driven, predictive, and highly efficient workflows. Early AI applications in the 1980s–1990s focused on simple molecular modeling, evolving through the 2000s with machine learning for improved molecular interaction predictions. The 2010s saw accelerated growth due to advances in big data, deep learning, and large-scale biological datasets, enabling AI integration across target identification, drug repurposing, lead optimization, and virtual screening. AI techniques—including natural language processing, reinforcement learning, swarm intelligence, and generative adversarial networks—enhance molecular design and optimization, overcoming the vast complexity of chemical space. Applications extend to biomarker discovery, clinical trial design, and personalized medicine. While AI improves predictive accuracy, reduces costs, and accelerates timelines, challenges remain, including data quality issues, biological complexity, interpretability, regulatory constraints,and the irreplaceable role of human expertise. Opportunities lie in combining AI with real-world data, improving decision-making, and fostering innovative therapeutic solutions. A balanced approach—leveraging AI's strengths while addressing its limitations—is crucial for its responsible and sustainable integration into pharmaceutical research.

▪ **KEYWORDS: -**
Artificial Intelligence, Clinical Trials, Lead Optimization, reinforcement learning, personalized medicine**.**

I. INTRODUCTION: -

[1]The use of AI in the pharmaceutical industry has developed a lot over the last few decades. In the beginning, during the 1980s and 1990s, AI was only used in simple ways, mainly for modeling molecules and predicting chemical structures. These early tools helped prepare the way for more advanced technologies as computers and algorithms improved.

By the early 2000s, AI became more useful with the rise of machine learning. These new tools could study complex data and helped make drug discovery faster by predicting how molecules would interact and improving drug designs.

The real growth of AI in this field started in the 2010s, thanks to major progress in Big Data, deep learning, and the availability of large datasets from genomics, proteomics, and high-throughput screening. Pharmaceutical companies began using AI in many parts of drug development, including identifying drug targets and designing clinical trials.

In recent years, AI has become a key tool in speeding up drug discovery, improving clinical trials, and creating personalized treatments. This marks a move toward faster and more efficient, data-driven research and development in the pharmaceutical world.

[2]The structure of a drug candidate plays a key role in determining its properties and how it works in the body. However, many of the drugs that pass initial screening still have some flaws that limit their use in actual treatments. Because of this, improving or "optimizing" drug molecules is one of the biggest challenges in drug development. The goal of molecular optimization is to improve important features of a molecule, such as how easily it dissolves, how well it is absorbed by the body, and how specifically it targets the disease.

Since a molecule's structure affects its behavior, scientists created a method called quantitative structure–activity relationship (QSAR). QSAR builds mathematical models that link the structure of a molecule to its activity or effect.

Traditional computer-based approaches for molecular optimization treat a molecule as a combination of smaller parts called fragments. These methods improve molecules by adding, removing, or changing these fragments based on QSAR models. But because there are so many possible fragments and ways to combine them, the

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number of possible molecules is enormous—making it extremely difficult to find the best ones. This is known as an NP-hard problem, meaning it's very complex and time-consuming to solve.

To make this process faster and more efficient, researchers are now using artificial intelligence (AI) techniques. These include methods like natural language processing (NLP), swarm intelligence, reinforcement learning, and generative adversarial networks (GANs) to help design and optimize drug molecules more effectively.

[3]In pharmaceutical research, new artificial intelligence (AI) technologies have gained a lot of attention, especially when deep learning methods showed better results in predicting chemical properties. For example, in the Merck Kaggle and NIH Tox21 challenges, deep neural networks performed better than traditional machine learning techniques. Since then, AI has been used in many more areas of early drug discovery, such as designing new chemical compounds and peptides from scratch, and helping plan how to make them.

[4] This review aims to help readers understand how artificial intelligence (AI) is being used in pharmacology. It gives a detailed and critical overview of current research and explores how AI might be used in the future. To do this, we searched for relevant studies using keywords on PubMed, Google Scholar, and ScienceDirect. The main goal was to look at how AI and precision medicine work together. This combination has the potential to transform healthcare by creating personalized treatments and improving patient outcomes.

II. DRUG DISCOVERY IN THE AI ERA:

[5] A Historical Perspective on the Evolution of Drug Discovery.

Traditional methods of drug discovery have mainly depended on trial and error, where large collections of chemical compounds are carefully tested to find those with the desired biological effects. However, this process is slow, labor-intensive, and often results in compounds that have unwanted side effects or toxic properties.

- Drug Repurposing.

AI plays an important role in finding new uses for existing drugs. By analyzing large sets of medical data, AI can suggest which approved drugs might work for other diseases. For example, remdesivir, originally developed for Ebola, was identified by AI as a potential treatment for COVID-19 (Kim et al. 2020).

- Target Identification

AI helps identify proteins and molecules in the body that are linked to diseases. By studying large amounts of genetic and protein data, AI can point out possible targets for new drugs (Sahayasheela et al. 2022).

- Lead Optimization

AI improves the process of developing drug candidates by designing new compounds that are more effective, safer, and more likely to succeed in clinical trials (Blanco-Gonzalez et al. 2023).

- Virtuall Screening

AI speeds up virtual screening by quickly analyzing huge libraries of chemical compounds to find those most likely to have the desired effect on the body (Ren et al. 2023).

[6] Instead of just searching through existing chemical libraries, modern drug design goes further by exploring the huge space of all possible small molecules — a number estimated to be between 10^{30} and 10^{60}. Drug design follows a process called the design-make-test-analyze (DMTA) cycle, which involves repeatedly creating new compounds and testing their properties.

To explore this vast chemical space more efficiently, a method called quantitative drug design has been used since the late 1970s. At its core, drug design revolves around two main questions:

1. Can we predict how a molecule behaves just by looking at its structure?
2. Which parts of the structure affect specific properties?

The first question supports the idea behind virtual screening (VS), while the second is what quantitative structure–activity relationship (QSAR) models aim to answer. So, drug design builds on VS and focuses on predicting molecule properties and generating new molecules — both of which are key goals in today's AI-powered drug discovery.

[7] Different types of AI algorithms—such as supervised and unsupervised learning, reinforcement learning, and rule-based or evolutionary methods—can help solve complex problems in drug discovery. These algorithms work by analyzing large amounts of data in various ways For example, they can predict how effective or

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toxic a new drug might be, often more accurately and efficiently than traditional methods.

AI can also help find new drug targets, such as specific proteins or genes involved in diseases. This opens up new possibilities for drug development that go beyond what older methods can achieve, potentially leading to better and more innovative treatments.

While traditional drug research has had success, it often depends on trial-and-error and struggles to predict how new compounds will behave. In contrast, AI-based methods can make the drug discovery process faster and more accurate, helping create more effective medicines.

[8] Biomarker Discovery and Validation, In modern medicine, finding biomarkers helps improve the drug development process. This involves collecting and analyzing a large number of samples in a consistent way. Once a potential biomarker is found, it must be validated to make sure it gives reliable and repeatable results, with good accuracy and precision. Artificial intelligence (AI) can assist in this stage. In drug development, biomarkers are used to measure how well a treatment works in clinical trials and to identify and confirm drug targets. This helps ensure that each patient receives the most suitable treatment based on their biomarker results

III. CHALLENGES: -

[9] The human role in drug discovery

People still play an important role in drug discovery. In both universities and pharmaceutical companies, medicinal chemists often make the final decisions on which compounds to test in the design-make-test (DMT) cycle, using their own experience and judgment instead of relying only on AI. Teams may carefully review and vote on the order in which compounds are made and tested.

Even in companies that use AI, chemists can reject compounds that don't meet certain standards, even if AI models or toxicology tests show no problems. This shows that human expertise, personal bias, and time limits still have a big influence in the early stages of drug discovery—sometimes more than AI.

An early example of combining data science with drug discovery is the Pfizer Rule of 5 (Ro5, also called Lipinski's rules). These rules look at factors like hydrogen bonding ability, the octanol/water partitioning coefficient (logP), and molecular weight to help narrow down the vast number of possible chemical compounds. Ro5 has shaped medicinal chemistry around the world, but its influence has been slowly decreasing over time.

[10] Current AI methods cannot replace traditional lab experiments or the knowledge and skills of human researchers.AI can only make predictions based on the data it is given, and these predictions must still be checked and understood by human experts . However, using AI alongside traditional experiments can make drug discovery more effective. By combining AI's prediction abilities with human expertise. the process of finding and developing new medicines can be improved and sped up.

[11]AI and machine learning are most advanced in the field of diagnosis, with many smart algorithms now approved by the FDA at a growing pace. This quick progress has been possible because diagnostic data are collected in a consistent way across different sources and technologies. Researchers have also been willing to share and organize large datasets, and many algorithms from other industries—such as image and electrical signal analysis—have been adapted for medical diagnosis. In some cases, a single hospital or healthcare system had enough data to test and confirm an algorithm's accuracy. These tools are now advanced enough that researchers are beginning to study how to measure and handle uncertainty in their results.

[12]Although AI offers many benefits for drug discovery, it still faces major hurdles, such as poor or inconsistent data quality, the intricate nature of biological systems, strict regulatory and ethical requirements, and significant implementation costs. Overcoming these obstacles is essential for effectively integrating AI into pharmaceutical research.

IV. OPPORTUNITY: -

[13]Opportunities for AI in Drug Discovery.

Acceleration of the Drug Discovery and Development Continuum.

The conventional drug discovery paradigm represents a protracted and multifaceted process, encompassing extensive experimental workflows and exhaustive data interrogation. Within this framework, researchers are tasked with the identification and prioritization of bioactive candidate molecules from a vast chemical space comprising tens of thousands of compounds. Subsequent to this selection, comprehensive multi-phase clinical trials are undertaken to rigorously evaluate pharmacological safety profiles and therapeutic efficacy. This traditional trajectory is

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inherently resource-intensive, both in temporal and financial terms.

The advent and integration of artificial intelligence (AI) technologies have markedly enhanced efficiency across this continuum. Equipped with advanced deep learning architectures and high-throughput data mining capabilities, AI systems can distill actionable insights from expansive and heterogeneous biomedical datasets—encompassing genomic sequences, proteomic architectures, and mechanistic models of disease pathogenesis. Through sophisticated computational interrogation of such data, AI enables the delineation of more precise and strategically informed research trajectories, thereby mitigating empirical redundancy, minimizing non-strategic experimental iterations, and ultimately expediting the progression from molecular conception to clinical application.

[14] Future Directions and Opportunities in AI-Assisted Drug Discovery

The global market for AI-enabled drug discovery is anticipated to experience substantial expansion in the forthcoming years. Progressive innovations in artificial intelligence—encompassing machine learning, natural language processing, and deep learning—have facilitated the creation of highly sophisticated computational frameworks capable of interrogating intricate biological datasets and forecasting the molecular interactions between pharmacological agents and their respective biological targets.

[15] With the rapid advancement of technological innovations, the integration of artificial intelligence (AI) into the domain of drug discovery has expanded at an unprecedented pace, introducing both transformative opportunities and formidable challenges to pharmaceutical research and development. This work delineates the principal applications of AI within the drug discovery paradigm, encompassing molecular design and virtual drug screening, the optimization of drug development workflows, and the refinement of clinical trial design coupled with advanced data analytics.

Furthermore, it elucidates the strategic advantages afforded by AI, such as the acceleration of drug discovery and development timelines, the enhancement of predictive accuracy and therapeutic efficacy, and the mitigation of both financial expenditures and developmental risks. These benefits position AI as a pivotal driver of innovation in contemporary drug research.

Nevertheless, the implementation of AI in this sector is not without its constraints, necessitating a balanced approach that harnesses its capabilities while rigorously addressing its inherent limitations, potential biases, and ethical considerations. For AI to contribute effectively to the advancement of human health, sustained investigative efforts and proactive measures are imperative to navigate and resolve these challenges, thereby fostering its responsible and sustainable deployment in drug discovery.

[16] Artificial intelligence (AI) has the potential to transform drug discovery by shifting drug screening from physical laboratory experiments to virtual simulations. This transition allows for faster screening processes and the identification of promising drug targets with less manual effort and fewer resources. However, the adoption of advanced AI-based simulation and modeling techniques faces notable challenges, including limitations in data quality, difficulties in interpreting complex AI models, and the need for rigorous validation and reproducibility. Despite these hurdles, AI can significantly enhance the efficiency of drug discovery, support unbiased investigation of potential compounds, and improve predictive accuracy—ultimately lowering costs and increasing the chances of identifying successful drug candidates. For AI to be effectively integrated into drug discovery, these challenges must be addressed, with particular attention to ensuring the reliability and transparency of AI systems.

V. APPLICATION: -

▫ APPLICATIONS OF AI: -

[17] The integration of artificial intelligence (AI) and real-world data (RWD) is increasingly prevalent in contemporary drug development paradigms.Among the most prominent applications are adverse event surveillance, optimization of clinical trial recruitment, and identification of novel therapeutic uses for existing drugs.Current research exhibits critical limitations, particularly in the domains of data integrity, causal inference, and the generalizability of findings across diverse populations.Extensive national and international initiatives are progressively enhancing the accessibility of RWD.Innovative distributed analytical methodologies are required to fully leverage the potential of RWD in advancing pharmaceutical research and development.

[18] Conventional drug design entails substantial investment of both time and financial resources in research and development.

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[19] The aim of this study was to examine the impact of artificial intelligence (AI) techniques on drug development.

[20] Gene therapy, designing tiny carriers for drugs, finding new medicines from natural sources, and managing the business side of pharmaceuticals, including marketing studies and running clinical trials.

[21]The chances that come up and the plans needed to make the most of AI's ability to change the pharmaceutical industry.

[22]Companies and other tools that help reduce the effort needed in the drug discovery process.

REFERENCE: -

[1]. Serrano, D. R., Luciano, F. C., Anaya, B. J., Ongoren, B., Kara, A., Molina, G., ... &Lalatsa, A. (2024). Artificial intelligence (AI) applications in drug discovery and drug delivery: Revolutionizing personalized medicine. Pharmaceutics, 16(10), 1328.

[2]. Xia, Y., Wang, Y., Wang, Z., & Zhang, W. (2024). A comprehensive review of molecular optimization in artificial intelligence-based drug discovery. Quantitative Biology, 12(1), 15-29.

[3]. Hessler, G., &Baringhaus, K. H. (2018). Artificial intelligence in drug design. Molecules, 23(10), 2520.

[4]. Singh, S., Kumar, R., Payra, S., & Singh, S. K. (2023). Artificial intelligence and machine learning in pharmacological research: bridging the gap between data and drug discovery. Cureus, 15(8).

[5]. Gupta, U., Pranav, A., Kohli, A., Ghosh, S., & Singh, D. (2024). The contribution of artificial intelligence to drug discovery: Current progress and prospects for the future. Microbial data intelligence and computational techniques for sustainable computing, 1-23.

[6]. Deng, J., Yang, Z., Ojima, I., Samaras, D., & Wang, F. (2022). Artificial intelligence in drug discovery: applications and techniques. Briefings in Bioinformatics, 23(1).

[7]. Blanco-Gonzalez, A., Cabezon, A., Seco-Gonzalez, A., Conde-Torres, D., Antelo-Riveiro, P., Pineiro, A., & Garcia-Fandino, R. (2023). The role of AI in drug discovery: challenges, opportunities, and strategies. Pharmaceuticals, 16(6), 891.

[8]. Kokudeva, M., Vichev, M., Naseva, E., Miteva, D. G., &Velikova, T. (2024). Artificial intelligence as a tool in drug discovery and development. World Journal of Experimental Medicine, 14(3), 96042.

[9]. Hasselgren, C., &Oprea, T. I. (2024). Artificial intelligence for drug discovery: are we there yet?. Annual Review of Pharmacology and Toxicology, 64(1), 527-550.

[10]. Blanco-Gonzalez, A., Cabezon, A., Seco-Gonzalez, A., Conde-Torres, D., Antelo-Riveiro, P., Pineiro, A., & Garcia-Fandino, R. (2023). The role of AI in drug discovery: challenges, opportunities, and strategies. Pharmaceuticals, 16(6), 891.

[11]. Baldoni, J., Begoli, E., Kusnezov, D. F., & MacWilliams, J. (2020). Solving hard problems with AI: dramatically accelerating drug discovery through a unique public-private partnership. Journal of Commercial Biotechnology, 25(4).

[12]. Mettleq, A. S. A., Akkila, A. N., Alkahlout, M. A., ALmurshidi, S. H., Abu-Nasser, B. S., & Abu-Naser, S. S. (2024). Revolutionizing Drug Discovery: The Role of Artificial Intelligence in Accelerating Pharmaceutical Innovation.

[13]. Wang, W. (2024). The application of artificial intelligence in drug discovery: opportunities and challenges. Computational Molecular Biology, 14.

[14]. Visan, A. I., &Negut, I. (2024). Integrating artificial intelligence for drug discovery in the context of revolutionizing drug delivery. Life, 14(2), 233.

[15]. Blanco-Gonzalez, A., Cabezon, A., Seco-Gonzalez, A., Conde-Torres, D., Antelo-Riveiro, P., Pineiro, A., & Garcia-Fandino, R. (2023). The role of AI in drug discovery: challenges, opportunities, and strategies. Pharmaceuticals, 16(6), 891.

[16]. Visan, A. I., &Negut, I. (2024). Integrating artificial intelligence for drug discovery in the context of revolutionizing drug delivery. Life, 14(2), 233.

[17]. Chen, Z., Liu, X., Hogan, W., Shenkman, E., & Bian, J. (2021). Applications of artificial intelligence in drug development using real-world data. Drug discovery today, 26(5), 1256-1264.

[18]. Xia, Y., Wang, Y., Wang, Z., & Zhang, W. (2024). A comprehensive review of molecular optimization in artificial

intelligence-based drug discovery. Quantitative Biology, 12(1), 15-29.

[19]. Staszak, M., Staszak, K., Wieszczycka, K., Bajek, A., Roszkowski, K., &Tylkowski, B. (2022). Machine learning in drug design: Use of artificial intelligence to explore the chemical structure–biological activity relationship. Wiley Interdisciplinary Reviews: Computational Molecular Science, 12(2), e1568.

[20]. Mottaghi-Dastjerdi, N., &Soltany-Rezaee-Rad, M. (2024). Advancements and applications of artificial intelligence in pharmaceutical sciences: A comprehensive review. Iranian Journal of Pharmaceutical Research: IJPR, 23(1), e150510.

[21]. Rahate, K. P., & Mondal, R. (2024). Applications of AI in drug discovery: Its challenges, opportunities, and strategies. Approaches to Human-Centered AI in Healthcare, 86-120.

[22]. Visan, A. I., &Negut, I. (2024). Integrating artificial intelligence for drug discovery in the context of revolutionizing drug delivery. Life, 14(2), 233.