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Review Paper

AI In Drug Repurposing

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ABSTRACT

Artificial Intelligence (AI) has emerged as a transformative force in drug repurposing, offering innovative solutions to accelerate the discovery of new therapeutic applications for existing drugs. This approach significantly reduces development time and costs, particularly in addressing unmet medical needs in rare diseases and complex conditions (Álvarez-Machancoses & Fernández-Martínez, 2019; Cortial et al., 2024). AI-driven drug repurposing leverages machine learning algorithms, deep learning techniques, and big data analytics to systematically identify potential drug candidates across various stages of the drug development process (Abbas et al., 2024; Tanoli et al., 2021). The integration of AI in drug repurposing has led to the development of sophisticated computational methods that can analyze diverse data types, including genomics, electronic health records, and chemical informatics (Pan et al., 2022; Zong et al., 2022). These methods enable researchers to uncover novel therapeutic targets, predict drug-target interactions, and optimize lead compounds more efficiently than traditional approaches (Abbas et al., 2024; Farghali et al., 2021). Notably, AI has demonstrated its potential in addressing urgent medical needs, as evidenced by its application in identifying potential treatments for COVID-19 (Cong & Endo, 2022; Pan et al., 2022). While AI shows great promise in revolutionizing drug repurposing, challenges remain, including data quality, model interpretability, and ethical considerations (Abbas et al., 2024; Kokudeva et al., 2024). Despite these hurdles, the growing interest and investment in AI-driven drug repurposing underscore its potential to significantly enhance the drug discovery process and improve patient outcomes across a wide range of diseases (Cortial et al., 2024; Huang et al., 2024). As the field continues to evolve, the convergence of multi-omics data and AI technologies is expected to further accelerate innovation in drug development and repurposing strategies (Cong & Endo, 2022; Farghali et al., 2021).

INTRODUCTION

Drug repurposing, the process of identifying new uses for existing drugs beyond their original

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indications, has emerged as a cost-effective and time-efficient strategy in drug development (Cortial et al., 2024; Tanoli et al., 2021). This approach has gained significant traction in recent years, particularly in addressing unmet medical needs in rare diseases and responding to urgent health crises like the COVID-19 pandemic (Cong & Endo, 2022; Cortial et al., 2024). Artificial Intelligence (AI) and Machine Learning (ML) have revolutionized the field of drug repurposing by enabling researchers to systematically analyze vast amounts of data from diverse sources, including electronic health records (EHRs), genomics, and large-scale gene expression profiles (Saberian et al., 2019; Tanoli et al., 2021; Zong et al., 2022). These advanced computational methods have accelerated the drug discovery process by identifying potential drug candidates more efficiently and accurately than traditional methods (Abbas et al., 2024; Farghali et al., 2021). The integration of AI in drug repurposing has opened up new possibilities for addressing complex human diseases. By leveraging multi-omics data and applying sophisticated algorithms, researchers can now explore drug-disease relationships, predict drug-target interactions, and identify novel therapeutic applications for existing drugs (Cong & Endo, 2022; Dotolo et al., 2020). This approach has been particularly valuable in the rapid response to the COVID-19 pandemic, where AI-based drug repurposing strategies have been employed to identify potential treatments in a shorter timeframe (Dotolo et al., 2020; Kaushal et al., 2020).

METHODS

AI-based methods for drug repurposing encompass a wide range of techniques, including machine learning, network-based models, and structure-based approaches. Machine learning methods, such as clustering, dimensionality reduction, and supervised learning, are commonly used to analyze large-scale datasets and identify potential drug candidates (Cong & Endo, 2022). These methods can process diverse data types, including electronic health records (EHRs), which provide rich longitudinal and pathophysiological information (Zong et al., 2022). Network-based models, which can be further categorized into clustering and propagation approaches, are used to identify patterns, functionally associated proteins, and novel drug-disease or drug-target relationships (Dotolo et al., 2020). Structure-based approaches focus on identifying small chemical compounds that can bind to macromolecular targets, evaluating drug-target interactions to find new applications for existing drugs (Dotolo et al., 2020). Knowledge Graphs (KGs) have also emerged as a powerful tool for drug repurposing, integrating complex datasets and leveraging advanced algorithms to accelerate the identification of viable drug candidates (Perdomo-Quinteiro & Belmonte-Hernández, 2024). In conclusion, AI methods for drug repurposing are diverse and continually evolving. While these approaches have shown promise, challenges remain, such as the need for validation, accessibility, and understanding of data sources like EHRs (Zong et al., 2022). The integration of multiple AI techniques, along with the use of comprehensive target activity profiles and functional testing of patient cells, may lead to more effective and tissue-aware drug repurposing strategies (Quazi, 2021; Tanoli et al., 2021). As AI in drug development continues to mature, collaboration between pharmaceutical scientists, computer scientists, statisticians, and physicians will be crucial in realizing its full potential for accelerating drug discovery and repurposing (Farghali et al., 2021).

APPLICATIONS

AI has emerged as a powerful tool in drug repurposing, offering innovative approaches to identify new therapeutic applications for existing drugs. Supervised machine learning and AI methods utilize publicly available databases and information resources to systematically identify drug repurposing leads, accelerating and de-risking the drug development process (Tanoli et al., 2021). These methods include network-based models, structure-based approaches, and AI-based networks, which have been applied to various diseases, including COVID-19 (Dotolo et al., 2020). Interestingly, while AI has shown promise in drug repurposing, its application is not without challenges. The scarcity of clinical patient data and the current focus on genetic aberrations as primary drug targets may limit the performance of anticancer drug repurposing approaches that rely solely on genomics-based information (Tanoli et al., 2021). Additionally, AI-based networks currently appear less relevant for COVID-19 drug repurposing due to the need for more data (Dotolo et al., 2020). In conclusion, AI has demonstrated significant potential in accelerating drug repurposing efforts across various diseases. The convergence of multi-omics and AI-based drug repurposing offers new insights and opportunities for innovation (Cong & Endo, 2022). However, challenges such as data quality, generalizability, and ethical considerations must be addressed to fully realize the benefits of AI in drug repurposing (Abbas et al., 2024). As the field continues to evolve, AI is expected to play an increasingly important role in identifying new applications for existing drugs, potentially revolutionizing the drug discovery and development process.

DRAWBACKS

- AI models, especially deep learning, can be difficult to interpret, making it hard to understand how they reach their conclusions.
- This "black box" nature can hinder clinical acceptance, as clinicians need to understand the rationale behind treatment decisions.
- Regulatory bodies also demand explanations for AI predictions, which can be challenging to provide if the model's decision-making process is opaque.

2. Data Quality and Availability Issues:
   - AI algorithms require large, high-quality datasets for training, but such datasets may be limited in certain areas, especially for rare diseases.
   - Data can also be biased, either intentionally or unintentionally, leading to inaccurate or harmful predictions.
   - Incomplete or inconsistent data can also affect the accuracy and reliability of AI predictions.
3. Ethical and Regulatory Concerns:
   - Data privacy and security are major concerns, as AI models may inadvertently collect and store sensitive data.
   - Ethical considerations regarding the use of AI in healthcare, such as algorithmic bias and fairness, need to be addressed.
   - Regulatory hurdles can be significant, especially for drug repurposing, where strict validation and approval processes are required.
4. Cost and Expertise:
   - Implementing and maintaining AI systems in drug repurposing can be expensive, requiring specialized expertise in both AI and pharmaceutical sciences.
   - The cost of data acquisition, model development, and validation can be substantial.
   - The need for specialized personnel can also be a barrier to adoption.

DISCUSSION

Through this mini-review, we have been able to characterize the proportion represented by rare diseases in the literature describing the application of AI for drug repurposing. Compared to the other diseases categorized (e.g., COVID-19, cancer, or neurodegenerative diseases), rare diseases only represent 2.63% of the overall literature, which can also be found regarding the broader use of AI in rare diseases, where, compared to other application domains (e.g., disease diagnosis, gene identification, and drug discovery), drug repurposing is also a relatively less prominent area of focus (27, 28). This low representation of rare diseases in the literature on AI-driven drug repurposing may be attributed to several factors, including the complexity of rare diseases, the small number of patients affected by rare diseases, the limited availability of data, the variability in symptoms, the genetic diversity among patients, or even the lack of funding priorities in rare diseases, making research in rare diseases not only challenging but also economically unappealing. However, different applications have demonstrated that AI in drug repurposing is promising and that it will benefit patients suffering from rare diseases. Indeed, six concrete applications illustrated diverse strategies regarding drug repurposing in rare diseases: Ekins et al. combined HTS and ML to identify potential treatments for PTHS; Sosa et al. (13) used literaturebased graph embedding to categorize drug repurposing candidates in various rare diseases. Esmail and Danter (14) generated artificial brain organoids to identify promising drug combinations for the treatment of MLD. Cong et al. used gene expression data to match diseases with potential drug candidates, while Foksinka et al. (16) used AI reasoning tools to identify therapeutics based on gene regulation, and, finally, Zhu et al. (17) constructed a rare disease KG to identify potential drugs and SMs for repurposing in various rare diseases. This momentum surrounding AI-driven drug repurposing in rare diseases is also reflected in the various current initiatives transcending core research. Scientists have developed algorithms, platforms, and libraries to inform and assist the scientific community. Companies have made it their business to identify and obtain regulatory approvals for promising drugs, while public bodies and big pharma's involvement is already being manifested.

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