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Chapter 10

Artificial intelligence-powered drug discovery and development: Accelerating innovation

Kiran Kumar Maguluri

IT systems Architect, Cigna Plano, Texas, USA.
kirankumar.maguluri.hcare@gmail.com

Abstract

AI-powered drug discovery accelerates the development of new treatments by analyzing vast datasets, predicting molecular interactions, and optimizing drug candidate identification. By reducing time and cost, AI enhances innovation, improves drug efficacy, and supports the discovery of personalized therapies, transforming the pharmaceutical industry and healthcare delivery.

Keywords

AI, Drug Discovery, Innovation, Molecular Interactions, Personalized Therapy, Pharmaceutical Industry

10.1. Introduction

1.1 Background In the rapidly evolving pharma sector, advancements facilitated by innovative technologies play a paramount role. Innovative concepts, especially in the drug discovery and development sector, provide leading pharma companies with a competitive edge. Traditional methodologies of drug development and discovery have a high rate of failure and are extensive in terms of time and cost; therefore, there is an urgent need for innovative methods such as artificial intelligence (AI) in this sphere. Limited studies and understanding exist on the role of AI in drug development and discovery;

hence, this paper aims to provide an extensive understanding of the changes created by AI in drug discovery and development.

The purpose of the study is to delve into the frontiers of mechanism and formulation for AI-based drug discovery and development. This paper will be restricted to one explicit dimension, which is target identification. Target identification is one of the critical aspects of drug discovery. In this paper, a systematic assembly of the entire target identification process for modifying various potential targets involved in a broad spectrum of human diseases will be carried out. The therapy of unmet needs in human diseases such as heart failure, viral infections, or the development of antimicrobial drugs for superbugs can be achieved using drugs that function mechanistically on these targets. This paper portrays the potential targets, their roles in human diseases, and the underlying mechanism. Due to the profusion of prospective and mechanistically robust targets, the plethora of validated drug-target entities propounds the immunotherapeutic modus operandi for vaccine breakthroughs for emerging future infectious diseases. The study is delimited to a descriptive analysis (Nampalli et al., 2024). A comprehensive understanding of the underlying mechanisms of validated targets has been carried out to explore their role in different indications.

While covering this paper, there remain some limitations. This paper was constructed specifically in the backdrop of the pharma sector, seeing the need for various AI-based techniques to surpass the 'Valley of Death', access both small and large pharma, and resolve various issues for vaccine development and characteristic advancements. Primarily, drug discovery and development, including flaviviridae, antivirals, hemorrhagic fever, selective endothelial adhesion, virus assembly, and provocation of protective immune response, have been deliberated as they play a major role not just for novel autoantibody adjuvant antibody discovery but also antibodies with other pharmacological modes. In order to systematically furnish this report and provide insightful drive, we must understand the mechanisms associated with the following therapeutic approaches, comprehensive of drug identification methodologies and techniques, whose detailed review has been presented above, and also the antisense gene therapeutic agents that have received approval for the preemptive treatment of infectious diseases and antimicrobials/glucan synthesis inhibitors.

The complexities associated with the approval of drug candidates have extensively overshadowed the global approval of drugs that reach the promise of enabling potent and approvable targets. Current challenges in drug discovery that lead to the failure of drugs circling the overt passage of the landmine of drug approval can be replaced by innovative and unique targets drilled down by AI (Danda, 2023). An assortment of AI-

empowered tools capable of recognizing a plethora of potential targets, dissecting various facets of human diseases to mechanistic routes and areas, have been discussed.

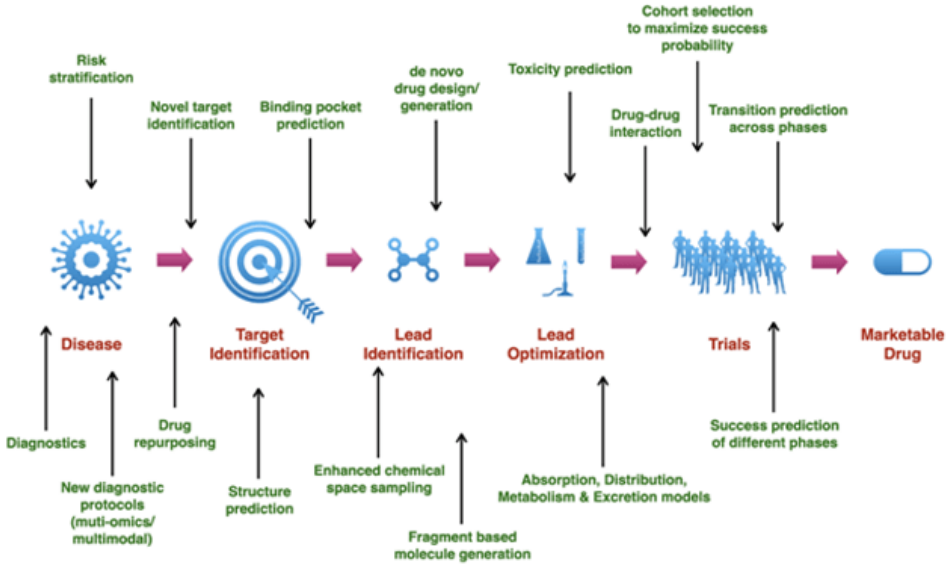

Fig 10 . 1 : AI To Accelerate Drug Discovery.

10.1.1. Background and Significance

Innovative new drugs are essential for public health and have been used frequently to improve the quality of human life. Since the 18th century, drug discovery has historically experienced a slow process from random discovery to accurate scientific research. Until the 20th century, with the progress of molecular biology, genomics, and artificial intelligence technology, the paradigm of drug innovation has shifted. The rapid development of computer technology has promoted the digitization of various research information for drugs, changed these cumbersome operations, and provided convenience for people to synthesize new drug molecules by predicting their pharmacological properties in advance. At present, many pharmaceutical companies have applied machine learning technology to the discovery and development of lead compounds for cardiovascular, oncological, neurological, and metabolic diseases. More and more machine learning models have been disclosed, marking the progress of drug discovery-aided innovation into the clinical stage.

There are several key challenges in traditional drug discovery and development. As diseases become increasingly complex, new mechanisms of disease are overlapping signaling pathways, and drug discovery is unpredictable due to the uncertainty of the characteristics of druggable disease targets. These complicate the matching exploration of active compounds to compounds, delaying the traditional drug discovery and development process for 10-15 years, increasing the risk of investment and the cost of returning drugs, and the time cost of drugs from targeting diseases to landing marketing or being unknown on the market. The rise of computational biology has revolutionized the multidisciplinary approach to answering important questions in biology, materials science, and more. The multi-scale modeling and multidisciplinary analysis of transphenomic data have become the focus of some computational biologists, and biophysical models collaborating with models at other scales are widely used. At the same time, quantitative structure-activity relationships by combining molecular dynamics and virtual screening have been used in the drug industry and academic institutions to discover new therapies. But as technology continues, computational biology can still help us find more effective therapeutics, understand disease mechanisms, and move from academic publications to industrial application. Bioinformatics, in conjunction with artificial intelligence, will reduce assay time and screening time by finding drug candidates that are more likely to be toxic (Syed, 2023).

10.1.2. Purpose of the Paper

This research paper aims to provide an overview of how AI can be leveraged to empower different phases of drug discovery and development. Specifically, we aim to identify the potential applications of AI at various stages of drug discovery and development and provide a holistic understanding from drug target identification and validation to drug formulation and repurposing. We believe that our paper will be beneficial for researchers seeking to go deeper into the specific concepts and algorithms of biological applications of machine learning, as well as practitioners and policymakers in the pharmaceutical field who aspire to solve current challenges and bottlenecks in the drug discovery and development pipeline. To achieve our purpose, for the first three sections, we focused on discussing the main principles that form the theoretical backbone of AI in drug discovery and development. Beginning from the fourth part, instead, we present some practical application case studies that directly respond to the potential research and development challenges we have identified in the previous sections.

Given the abovementioned backdrop, in the following pages, we will try to justify the potential value that may be unlocked by deploying AI in drug discovery and development. We will thus discuss the most relevant techniques that fall within the wider array of artificial intelligence methods, as well as present some applications of these techniques in industry and research. This paper is structured as follows: '2. Theoretical Frameworks' explains drug discovery and development processes and identifies AI applications. In line with our purpose of identifying potential applications of AI, we describe the processes in terms of their sub-stages, where mutations in diseases are defined as natural environmental factors affecting the gene. For each AI technology, its possible application area is specified. In '3. Case Studies', we showcase some cases where AI has been used to solve drug discovery and development problems.

10.1.3. Scope and Limitations

This paper will focus on two major aspects of the use of AI in drug design that show the most promise for revolutionizing how drugs are developed and how early they can progress along the pipeline. The first part of the study discusses a range of AI methods utilized to rationally design new drugs, including methods that predict atomistic interactions in the complex, fragment screening, and drug design. Several companies and research groups are enjoying great success in this space, and we believe it is ripe for success in accelerating early drug discovery efforts. The second part of the paper discusses AI in analyzing the bioactivity that flows from testing entire compounds in biological assays. By using a form of transfer learning, bioactivity can now be predicted from molecular structure and random data. This gives us a data-driven understanding of drug-target interactions, and future medicines may have been tested against dozens or hundreds of putative targets at an early stage, substantially derisking failures further along the pipeline.

A stated aim of this study is that readers should be aware that their chosen datasets and model architecture may have dramatically improved or fallen out of style since the time of writing. However, as the merit of raised prediction accuracy by a small margin, a true validation dataset for molecular bioactivity is often determined in a biological assay, and there is therefore an absence of a 'ground truth' data in this space. In a similar vein, the state of the art for recreating protein-ligand interactions in silico is an active, rapidly developing field, and the most effective model in use now may be obsolete in six months.

10.2. Current Challenges in Drug Discovery and Development

The pharmaceutical industry invests an enormous amount of resources in drug discovery and development. It is estimated that the cost of bringing a new drug to the market may exceed USD 1.5 billion and 20 years of effort. Traditional strategies in drug discovery and development are usually inefficient and can lead to a high failure rate. By far, they make drug development an expensive enterprise for pharmaceutical companies. Traditional drug discovery can take a decade or more from target identification to approval of the drug (Nampalli, 2024). Animal models play a critical role in drug development, but a drug that is effective in animals may not necessarily also be effective in humans, and many substances are abandoned at this stage.

Given the proven healthcare and economic benefits of bringing new therapies and medications to the market, one of the most important goals for pharmaceutical companies is to reduce the time taken to bring pharmaceuticals to patients. There is tremendous pressure to find potential innovative molecules while reducing the time and cost of the process. Over the years, researchers have begun to understand that dealing with the fundamental challenges in targeting and validating potential molecules could pay dividends in drug development by shortening the process. Two of the most promising areas are target regulation and the use of AI. AI has become an area of interest for all pharmaceutical companies. It is one of the most efficient ways to choose a drug candidate and thus reduces the time and cost associated with drug discovery.

10.2.1. Traditional Methods and Limitations

Subsection 2.1. Traditional Methods and Limitations

Drug discovery is the process of identifying new drug candidates that show promising therapeutic effects, usually through high-throughput screening and rational drug design. High-throughput screening is a valuable experimental technique in biology that is used to rapidly assay the biological function that drives disease or is responsible for pathology in cells. One of the primary disadvantages of traditional drug discovery is that it is usually a trial-and-error process that can take some time to identify all possible dosages. Pharmaceutical research and development can also begin to erode profitability due to failed drugs and their high experimental costs. Controversial spending is one of the primary disadvantages of pharmaceutical research and development. The average approved drug in 2020 costs $359 million to develop, a significant increase from previous years. The estimated peak costs to bring a drug to market ranged between $6.4 billion and

$26.8 billion. In addition, various drugs pose a standard risk of low return on investment to the pharmaceutical industry. It is estimated that for every $10 billion spent on clinical trial phase I, only $1.2 billion translates to positive net present value and an internal rate of return of 11.4%, belying doubts that pharmaceutical drugs are a losing financial proposition in their developmental phase (Ramanakar, 2023).

The leading cause of drug failures is various aspects of drug development or the drug candidate itself. According to benchmark and statistical analysis, a significant percentage of drugs in phase I fail to enter the market, amounting to a failure rate of 14%. Some common barriers include a lack of efficacy and low impact of the marketed drug, including marketing, research and development, production, patents, and government intervention. These factors help ensure medications don't reach the market. Many of these failed drugs have valuable information that is not published, and new technologies developed by pharmaceutical companies and bottlenecks are one way to find them. Overall, the drug target has a high attrition rate that can take anywhere from 10 to 15 years to develop and further attract attention. Regulatory challenges are connected to the varying concentration of the asset from upstream to downstream assets. With low stated costs, the top assets are appreciated. There is no secret formula or pathway for drug development. Any potential drug discovery-oriented company can be particularly unpredictable or seem to have a lack of information or knowledge on the potential adverse or toxic effects of the drug candidate. During drug development, this is identified as a problem – not a negative regulatory response or a prominent information set, as used on a scale to assess progress and risks.

Equation 1 : Target Identification

$$S_{\text{target}} = \sum_{i=1}^{n} w_i \cdot f(X_i)$$

S_{target}: Target relevance score.

X_i: Feature i (e.g., gene expression, protein structure).

w_i: Weight assigned to feature i by the AI model.

$f(X_i)$: Contribution function for feature X_i.

10.2.2. The Role of AI in Overcoming Challenges

Service functions have become significantly complex, moving from being manual operators to advanced knowledge workers and often using a systems biology approach. AI is playing a critical role in assisting these workers in enhancing creativity and decision-making, conceptually providing an unfair advantage. By augmenting professionals with AI, we are eliminating the need to replace jobs and are introducing them as collaborators with AI. In this age of scientific complexity, the technological advances in AI and their applications to life sciences are taking us further and faster. Over the next 5 to 7 years, AI-based digital disruption is expected to create new applications, enabling drug discovery teams to identify new drugs in just 4 to 5 years, where one drug may currently take 10 to 12 years. This will not only be possible through AI-based simulations for drug development but also through predictive analytics for understanding therapeutics.

Drug discovery is slow, expensive, and often prone to failure. Artificial intelligence focuses on replicating human thought processes and applying advanced decision-making capabilities in medical settings, which can assist in accelerating and optimizing drug discovery and demystifying chronic diseases (Kothapalli et al., 2022). Techniques like deep learning and machine learning are being used to mine available data or supplement it by providing hypotheses from medical research repositories, thus enhancing research. Though still in their early days, many of these techniques have been used successfully by more than 200 startups that have emerged worldwide. Big pharma, as well as established IT giants, have all developed AI platforms to quicken the processes. Several successful case examples exist where AI is helping. One important example is how a biotech company identified potential targets and subsequently zeroed in to predict vaccine development within six days, without any external funding. They attempted wet lab tests for the treatment of melanoma in 2018, which led to a reduction in drug development costs by 50%.

10.3. Applications of AI in Drug Discovery

Within drug discovery, several applications of AI are emerging, spanning across the pharmaceutical R&D value chain. From de novo design to drug repurposing, the versatile functionalities of AI are being utilized by players in the field at numerous stages of the drug development process to perform these tasks more efficiently and help bring new medicines to market. Largely, AI is being utilized within drug discovery in one of two approaches: a services-based approach in which pharmaceutical companies outsource

a specific AI-based discovery workstream, or a partnership/licensing-based approach in which a pharmaceutical company forms a partnership with or licenses technology from an AI-based drug discovery company (Subhash et al., 2022).

Within the process of drug discovery, access to a more unparalleled and diverse set of potential drug targets is culminating in an ever-increasing demand within the R&D environment. In the last decade, the potential drug target space has continued to expand due to such initiatives. Computational and AI approaches can be used to more accurately identify and qualify potential therapeutic targets. AI tools can harness and analyze multimodal data and apply deep learning to achieve more precise and accurate target discovery and validation. Once a suitable target has been qualified and selected, the use of AI can help chemical bioassays with the co-crystal-based perspective to potentially reduce non-clinical pharmacokinetic risk. The number of potential drug-binding sites can also be expanded with the use of AI, enabling wider predictions of the druggability of new protein targets. In essence, AI is allowing scientists to consider a more extensive set of drug targets in a much shorter period than would have been possible with traditional computational discovery approaches.

Fig 10 . 2 : AI's Role in Drug Discovery.

10.3.1. Drug Target Identification and Validation

Identifying and validating a druggable target is a critical early stage in the drug discovery process. Tools that utilize big data integrated from multiple sources to predict novel disease targets prior to clinical observations have increased the chance of success for early-stage drug development. Several types of existing evidence can be utilized to infer a disease target, but few data are actually strong evidence for a drug inducing a desirable phenotype. Machine learning has shown strong performance in terms of predictive ability, but the real value of AI is seen as an enabling technology that can enhance efficiency, reliability, and discovery of novel target biology, reflecting the performance of technology to examine AI at each stage of the drug's lifecycle. This involves removing potential targets and/or transitioning additional targets to increase the chance of reaching a clinical outcome, such as lowering adverse effects.

Data integrated from a variety of public and licensed sources can be used to perform structured and unstructured predictions, and analysis of all appropriate data points is used to address target identification. We cannot afford to rely only on previously documented evidence to identify new disease targets and are increasingly involved in commercial targets. This process efficiently discards irrelevant targets that have been well-documented or known to be unreliable against the prediction properties of macromolecules. While additional tools will provide target validation evidence, literature search after such a dismissal step is still fruitful. We aim to provide on-target evidence for known targets and new hypotheses, associated biology, or disease indications for follow-up. This includes targets for which we own molecule-based relevance, but under license, an indication could be launched.

We are currently examining the predictive results from the recent prediction models. Machine learning has been utilized to combine input data and algorithms. This early version shows the development of a number of new targets that can now help prioritize the input data-dependent drug development pipeline. This process ultimately helps projects with a greater likelihood of success in developing new medicines faster and at a reduced cost. Given that one cycle of compound interest is estimated to be worth €1 billion, the early stage of drug discovery has the potential to reduce financial losses by up to 40% (Sondinti et al., 2023). Drug-target identification and validation. Target identification and validation are defined as follows:

- Drug target identification aims to isolate the pathogen from polymers coding and affected by disease directly or indirectly that can be cured by drug intervention.

Drug target validation is the process of basing a clinical model on the prediction of a drug target. Identifying and validating a correct target have been shown to be essential to confirm the predictive power of the technique. The early phase of medication development has the characteristics of the invention where unwanted targets are eliminated to increase the chances of developing the effect of a drug.

10.3.2. Compound Screening and Optimization

Drug development begins with the identification of a chemical entity that can modulate a defined biological target, typically a protein that is implicated in a given disease. Traditional drug discovery involves the high-throughput chemical screening of a large chemical library, comprising millions of compounds, to identify 'hits', i.e., those compounds that bind to the target. The compound, once optimized to possess desired characteristics, may lead to a new drug. Compound optimization includes assessing efficacy to ensure it still has an effect when used in the body, and the assessment of the safety profile for its performance as an experimental drug. By utilizing predictive modeling and simulation, AI technologies have the potential to accelerate the screening and predict the behavior and risk of new drug candidates.

The assessment of a new drug candidate regarding whether it has good safety profile properties and a low risk of failure is fundamental early in the process. In this context, AI can be an effective tool for compound optimization. AI can utilize large data sets from assays, chromatography, mass spectrometry, etc., to develop a safety profile using predictive models in silico comparisons with existing synthesized compounds. AI can be used to enhance lead compound characteristics within a chemical series, which will allow for the improvement of the properties of new drug candidates. The drug candidate should be predicted to be metabolized well in the body and have a lower risk of inducing an adverse effect due to the formation of unusual metabolites. In the dental field, AI has optimized drug compound structures for oral pain relief, which would be a treatment option for oral and maxillofacial surgery pain. It is reported that the new drug candidate has several strategies to reduce the standard dosage of opioids to avoid side effects. It has been optimized that safety studies are yielding desirable results. By integrating AI technology, the drug compound screening and optimization is where a significantly large cost-saving can be realized. The presented AI-powered methodology has the potential to significantly expedite improvements through reduced screening times and a reduction in non-viable projects. Compound optimization will yield performance-improved material properties to develop and facilitate a variety of other research

endeavors and commercialization opportunities. AI technologies can expedite lead compound selection and design before high-throughput synthesis, as well as preclinical efficacy and safety testing (Vankayalapati et al., 2023).

10.3.3. Clinical Trial Design and Patient Selection

Scientists face enormous challenges when evaluating whether a molecule has the potential to become a drug. Central to the process is quantifying if the molecule is safe and efficacious in relevant animal models before being tested, initially on healthy individuals and later in patients suffering from the disease of interest during clinical trials. Unfortunately, nine out of ten molecules fail during clinical development. The use of AI has the potential to allay some of these issues during the process of clinical trial design through trial simulations. One of the main challenges of clinical trial design is ensuring that the clinical trial population is representative of the target disease population. AI, in particular deep machine learning, is currently being utilized for predictive analytics in specific fields to predict whether patients will carry a certain genetic mutation several years before they do and whether that mutation is associated with a better patient outcome and potential therapeutic opportunities. This could be applied to estimate whether enough patients could be recruited to warrant the cost of running the trial.

Concurrently, the vast quantitative clinical data generated by healthcare systems is being utilized using AI to predict patient response. Importantly, it is already much quicker and cheaper to model a population of patients in a computer than in a clinical trial, thus trials could be optimized at an early stage to increase the chance of success by those most likely to respond to treatment. It should be noted that extensive ethical and practical considerations apply to patient selection for clinical trials, and time for gaming of developing clinical trials must also be factored in as these models become more accurate. From a business perspective, many life science groups are choosing to set up their own datasets; these have potential utility for the development of precision medicine clinical trials. By reducing the time and cost of the trial along with increasing the chance of success, AI can be used to aid the developers with patient group selection, increasing drug development, and clinical trial success.

10.4. Case Studies and Success Stories

There is a clear need to accelerate innovation and cut development costs associated with the discovery and development of new therapeutic interventions. Given the significant investment in AI in general and machine learning in particular, and the growing number of companies offering solutions in this space, there are few tangible success stories to substantiate these claims. Especially within the pharmaceutical research and development space, there remains a great deal of skepticism from analysts and industry professionals.

Case studies showing successful applications of AI in drug discovery at distinct stages are few and far between. Nevertheless, those that do exist showcase the dramatic impact of integrating AI technologies in the overall efficiency of drug discovery processes, as well as the specific cost savings claimed. These successes encourage further interest and confidence in AI's potential among R&D professionals in the industry. Individually, these case studies alone can steer future bioinformatics studies as good practice examples, helping scientists to generate useful predictions from big data. In the remainder of this chapter, compilations of existing case studies utilizing AI and machine learning in drug design are presented. First, a qualitative description of a variety of AI applications is provided across a spectrum of both biological pathways and disease areas. Secondly, the cost savings and/or time reductions associated with the applications detailed are summarized. Finally, there is a summary of the conclusions drawn, and examples of common best practice approaches to the development of AI technologies relevant to drug discovery and development within the case studies discussed.

10.4.1. Examples of AI-Driven Drug Discovery Platforms

The above-mentioned parameters are driving the leading pharmaceutical companies in the world to make significant investments in AI-driven drug discovery. Many new players across the world are now emerging as innovative start-ups to address the opportunities and challenges facing the industry. Known as CRO 2.0 or CRO+, these platforms are using data and AI-driven approaches to support or potentially replace big pharma in delivering a full drug development pipeline. The ability to analyze significant amounts of data, powerful algorithms such as deep learning, machine learning, and so on, are at the core of these platforms. Automation of large-scale molecular design and screening based on high-quality data, predictive algorithms, and human-incorporated

structural and biological knowledge is also at the core of each platform (Maguluri et al., 2022).

There are chemists who believe these approaches mentioned above lack creativity and the unique way the human mind is able to think. It is best when these approaches are used in conjunction with a traditional medicinal chemistry campaign. Nonetheless, below they are detailed to showcase the arguments for and against them. Each example below is given merely to showcase the toolbox of AI approaches and not as a full comprehensive or exhaustive list. The key companies described had to be mentioned as they were bought out for substantial amounts by major pharma companies and showcase their innovation capabilities by recognizing the potential of AI.

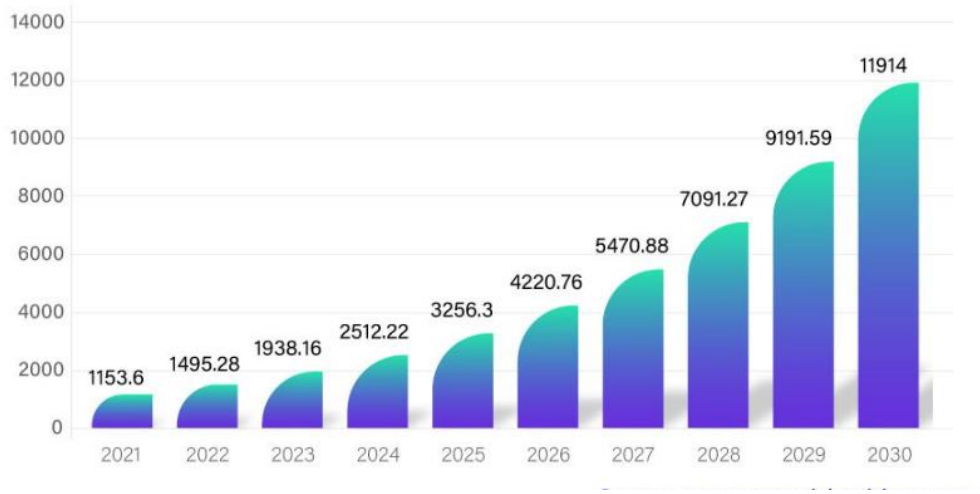

Fig 10 . 3 : AI-Powered Drug Discovery and Development.

10.4.2. Impact on Time and Cost Savings

AI is of particular interest to the pharmaceutical sector as it can aid the discovery of new drugs. Numerous reports have been published on the time savings and cost efficiency that AI stands to bring to the development and design phase of the pharmaceutical sector. Besides qualitative impacts, AI can reduce development timelines by using predictive analytics to define a fast-fail approach through early decisions. AI also facilitates adaptive approaches, which provide up-to-date information to the sponsor on treatment effects and help with tasks that enable just-in-time manufacturing and

distribution. Progress from one stage to the next can greatly benefit from the ability to quickly make critical decisions based on predictive analytic models and rapidly scale resources and budgeting to support a raft of candidates for package inclusion and filing, rather than having to go to mass production in costly clinical trials or ramping production at the time of the NDA submission. Clearly, time savings also translate directly to reduced costs, as development staff and allocated resources required are minimized while generating a higher interaction with the regulatory agencies to reduce potential rework. A notable effort in the field of AI of this type was realized at a company, where a 15% reduction in development time and cost was achieved relative to applying standard efficient portfolios. A recent report highlighted that the pharmaceutical and biopharmaceutical market is now more siloed and price-sensitive than ever before, with companies actively seeking value-adding processes to their development pipelines. In adhering to these trends, the report noted that companies employing a 'silo-busting' model drive the market and can leverage AI to achieve breakthrough efficiency improvements in both cost and time. Overall, robust evidence exists to support a significant decrease in the time and cost of drug development efforts by leveraging AI.

10.5. Ethical and Regulatory Considerations

There are some ethical considerations that must be taken into account with the application of AI in drug discovery. Recent studies indicate that AI algorithms are as biased as the historical data used to train them. Although the application of AI to drug discovery is just beginning, researchers in this field need to be attentive to potential biases in their AI algorithms to ensure that future patients are not treated unfairly. Furthermore, preclinical AI models, particularly those intended for coronavirus disease 2019, do not yet have any ethical or regulatory approval. Privacy and data sharing are also areas of concern. Machine learning, particularly the use of deep learning and neural networks that learn intricate structures from complex data, is often used in early-stage drug discovery. The training data required for these methods can be vast, but it is unclear whether many patient data sets could be shared with the commercial sector to train such AI models.

The evolving regulatory landscape for AI/ML-based technologies is still taking shape. A recent survey has been conducted for the preparation of a discussion paper on artificial intelligence in the pharmaceutical regulatory environment. A new regulatory framework for the development and approval of machine learning in image analysis in the pharmaceutical industry is also being developed. Although there are no specific AI regulations, ethical guidelines exist for the use of AI across different disciplines. One

pioneer international institution has released principles and recommendations on responsible innovation in neurotechnology. These state-of-the-art principles offer unique frameworks for safe and acceptable AI innovation by underlining the need for transparency in data and AI systems and proactive stakeholder dialogue between researchers, regulators, policymakers, industry, healthcare professionals, and the general public. Guidance for innovation in machine learning and artificial intelligence has been developed to encourage and oversee responsible use of electronic health data in research. The application of AI in healthcare is expected by these guidelines to comply with both the ethical considerations that feed into data protection and the regulatory standard inherent in good clinical practice. In essence, researchers and their hosts have long had to engage with a set of general ethical and legal principles to ensure the responsible use of AI in drug discovery.

10.5.1. Privacy and Data Security

Any pharmaceutical research will involve the handling of personally identifiable or otherwise sensitive data. A wealth of information on patients might be stored in an institution's or company's systems. For example, data can relate to their treatment, treatment information, treatment effect, and comorbid diseases. Especially in orphan drugs, a small pool of patients can be profiled and triangulated through the published data. Therefore, we can expect that patient sensitive data can be partially uncovered by matching public data with real-world evidence.

Today, having a breach of this data is a significant risk for companies from at least a public relations perspective, but in the case of directly identifiable medical data traces being in the systems, it is also a risk for the patients. In fact, the requirement for information and systems security, right after patient privacy, are ethical obligations. Thus, it is important for both companies and involved researchers that generating and handling patient data should be done in accordance with at least the existing regulations. Here, suitable IT infrastructure to maintain compliance is key; a contractual hold harmless clause will not change the ethical responsibility. Offenses are usually related to not informing or not properly pseudonymizing the data of the research and not informing the responsible authority of this. Not having traceability, for example, electronic patient case files that are not pseudonymized, brings pressure and legal problems to involved companies and researchers. Two of the most mentioned are observing pseudonymization and data controller requirements. For these reasons, the AI system should not, at this stage, be able to leave traces for re-identification of the patients. If a more powerful algorithm

or supercomputer is available to the attacker, user privacy can be completely compromised. With the data developing, experts are thinking about privacy-respecting technologies to incorporate with the AI development.

10.5.2. Bias and Fairness in AI Algorithms

Utilizing AI in drug discovery can enhance the identification and development of new drugs in the biopharmaceutical industry. The use of large-scale big data, coupled with innovative machine learning algorithms and powerful computation, offers new insights and opportunities for drug discovery and cancer therapeutics. However, one downside to using such technology is the rise of bias in the predictive models. Bias can potentially be introduced at three development phases of machine learning algorithms: data collection, data processing and integration, and training of algorithms. For example, training with an unrepresentative sample can further increase model bias. In drug discovery and implementation, the discriminatory use of biased algorithms can have serious implications on patient care and outcomes. Currently, due to systemic issues in this space, the onus lies within the model developers to prevent the creation of biased algorithms. Since AI is becoming more sophisticated, there needs to be parallel sophistication in techniques to detect and mitigate biases. One current best practice is to develop inclusionary datasets and metrics as a preventive measure against biased algorithms. Alongside this, there is also high interest in using fairness constraints or adversarial attacks to identify and mitigate algorithmic biases in health care, including pharmaceutical spaces.

AI, machine learning, and deep learning are already making rapid strides in informatics and technology adoption in the pharmaceutical industry. It is thus prescient to track the ethical considerations of integrating AI- and machine learning-powered systems within the drug discovery process. In this context, it is especially pressing to make sure the use of these algorithms does not result in biased decision-making, as it would exacerbate pre-existing concerns in current drug discovery systems. As AI continues to evolve and amass new datasets and new inferences, there remains a need for continuous re-evaluation for biases within these learning systems. These strategies can pivot to becoming integrated social change processes that can contribute to proactively addressing systemic health inequities.

10.5.3. Regulatory Approval and Compliance

Regulatory Approval and Compliance. Recent years have witnessed the fast development of regulations and standards governing the development, deployment, and application of AI technologies by food and drug regulatory agencies. AI applications are also increasingly mentioned by these regulatory agencies in their published guidelines, directives, reports, and strategic plans. They offer valuable insights into guiding good practices in developing and evaluating ML models in the pharmaceutical industry. However, it will be critical for the academic community and private sector to establish ongoing engagement with these regulatory bodies to gain a greater understanding of the evidence requirements for ML models and how to demonstrate compliance during the drug development and decision-making processes.

There are regions globally and other criteria where variations in regulatory approvals may occur; for example, ease of application, cost, time elapsed, and effectiveness. However, established regulatory guidelines and continued dialogue with regulatory agencies can also help CRO companies and pharmaceutical companies make strategic decisions around the governance of data, workload management, and the business value of technologies and methodologies. Current guidance from regulatory agencies also sets the standard for effective validation of outcomes, versus established paradigms, against falsifiable targets, through the underpinning theory and empirical evidence. Given the current trajectory of ML drug discovery, it is anticipated that the regulatory landscape governing the use of ML in the pharma industry will continue to evolve, and maintaining flexibility as well as the ability to adapt and incorporate these developments into study design is essential. Regulatory agencies rightly seek to ensure that AI-based systems not only perform safely and effectively in the real-world setting intended but are also in line with consumers' expectations and ethical standards.

Ensuring the effectiveness of drug development systems is a key priority in regulatory decision-making. The ability to understand and do due diligence on AI platforms will be particularly important from a regulatory perspective, to evaluate AI-driven technology as a proposed system for future integration. To do this, discussions with the regulatory body can help shape the evidence requirements. Regulatory advice at an early stage can also identify potential limitations of a later trial to support a license, either explaining why the study would not meet the licensing threshold and should be replaced with a more suitable approach or highlighting what further data will be required to allow the drug developer to be confident in the result. Furthermore, data governance is a key focus as academic researchers begin to apply AI research within an industrial complex system, where analyses are employed to make decisions, and the outputs are submitted

back as evidence into regulatory systems. Supporting the translational pathway for AI research early, and therefore engaging with companies developing drug discovery AI models, is recommended, both to guide research and likely requirements for approval in the clinic and to promote the highest standards of data governance.

Equation 2 : Virtual Drug Screening

$$P_{\text{bind}} = \frac{1}{N} \sum_{j=1}^{N} P_{\text{AI}}(B_j = 1 | M_j, T)$$

P_{bind}: Predicted average binding probability of candidate molecules.

$P_{\text{AI}}(B_j = 1 | M_j, T)$: AI-predicted probability of binding for molecule M_j with target T.

N: Number of molecules screened.

10.6. Future Directions and Opportunities

Although AI has overturned previous assumptions on the relevance of drug discovery and development, future trends may lead to further questioning of the way in which the pharmaceutical industry and other industries engaged in similar activities function. AI areas, such as analysis of complex systems, robust optimization, and pooling of capabilities and initiatives in AI technologies are likely to play an increasingly relevant and disruptive role in drug development and discovery. Instead of outsourcing specific steps, there may be a move towards collaboration for the design of novel clinical trial paradigms. A particular form of possible evolution could consist in the development of AI entrepreneurs in pharma, possibly coming from expansions of traditional AI-powered pharma companies, or in the closer association between these companies and academic institutions and consortia to explore more specific commercial opportunities.

Techniques and applications of AI usually require large datasets. In principle, one would imagine biotech and pharmaceutical companies making more investments in data generation as a result of the AI revolution. Such data, coming from a wider variety of information sources, may in principle enable a shift to personalized or more patient-based medicines. In the future, AI may enable the effective and efficient integration of genomics, transcriptomics, lipidomics, proteomics, and metabolomics. This in turn may improve the qualitative aspects of drug design, identifying new targets or defining phenotype subsets that inform repurposing strategies. It should be noted that there will

still be a continuous need to study new drug-like molecules and their targets in order to truly understand the potential of a patient-personalized medicine. Such domains are only partially realized in current AI and will require further exploratory research focusing on population-level modeling.

10.6.1. Emerging Trends in AI and Drug Discovery

1. Machine Learning Models: Machine learning has become popular for its applications in drug discovery and pharmaceutical scientific research. Faster improvements are expected in drugs under design and innovation for the creation of innovative products. 2. Natural Language Processing: Natural language processing is another area under active research. As more knowledge is generated for various pharmaceutical and biotech companies, its applications in repurposing drugs for various diseases and the study of pharmacogenomics will skyrocket. Today, NLP techniques have already discovered diverse complex relations that reflect potential uses, targets, diagnostics, and adverse events, and have dramatically improved molecule design. 3. Data Analytics: Access services, publications, financial reports, and press releases to predict development trends. AI and relevant analytical data can improve R&D and advance the pharmaceutical pipeline, including effective consumer product management, improved turnaround time from drug discovery to market, enhancing the drug assessment process, improving disease modeling and scientific research, and determining potential alternative models. To this end, these innovations deepen the knowledge base of new drug possibilities in which genetic information plays key roles—much earlier than classical model building becomes possible. Search and execution of relevant research roadmaps, including the review of the potential uses of technology, require ongoing learning from people interested or active in drug discovery and the pharmaceutical community in industry, academia, startups, etc. Aims and Scope: AI is facing global health challenges, reducing the time and effort of drug discovery and increasing scientific research. Driven by the related community and industrial stakeholders in drug discovery and relevant initiatives, the journal seeks to collect quality papers from researchers, developers, and entrepreneurs to provide a platform. Established and emerging AI technologies have undergone significant improvements, shaped and transformed, and have been used in these applications in recent pharmaceuticals, which address the evaluation, identification, design, mortality rate estimation, diagnosis, and healthcare issues, and personalizing therapeutic predictions based on personalized genomic data relating to drug use, drug repositioning, drug adverse events, drug modeling, target identification, market adoption, and enhanced knowledge generation. A precious place is given to alternatives.

10.6.2. Collaborations and Partnerships in the Industry

In a very innovative and specialist field such as AI for the pharmaceutical and healthcare industry, there is a fast-growing number of collaborations between different pharmaceutical and AI companies, as well as with academic institutions and other entities in both fields. The provision of both data and access to infrastructure or a directed development process are two important drivers of these collaborations. There has been a shift from companies looking to manage everything in-house to integrating the algorithms that drive artificial intelligence to optimize individualized value chains through strategic alliances that bring together different, complementary expertise. Interest in new, faster ways to prove the utility of potential drug candidates is a strong motivator for these collaborations in the pursuit of novel approaches to a high unmet medical need.

More generally, interdisciplinary collaborations, especially those between a specialist pharmaceutical company and an AI company, bring together the specialist knowledge of researchers with original ideas and domain expertise, the data science expertise of researchers who know a lot about AI and the ways of integrating it with data, the new "-omics" experts who, for instance, understand the science of genomics and epidemiology, and learned professionals who are skilled in coding in sophisticated programming languages, creating and interpreting machine learning algorithms, or teaching computers to see patterns in large volumes of data. These collaborations are also about shared outputs such as co-authored papers, which may be seen as a way of boosting the visibility and reputation of collaborators, and inventions in drug discovery that combine the knowledge of all parties. One of the main advantages of partnering and absorbing the different body of knowledge of collaborators is the absorption of new ways of thinking that could be mutually beneficial.

10.7. Conclusion

AI technologies stand to revolutionize the entire drug development process; this could be the medicine to cure the ailing pharmaceutical industry. Our review of how AI can be used to revolutionize not only the drug discovery process, but also development, faces the many challenges and roadblocks inherent in conducting research in artificial intelligence and medicine. The many in silico approaches discussed offer new insights into tackling old and new problems facing the development of therapeutics. We also discuss the major efforts being taken to understand the "black-box" of AI and legislation updates. AI in general is a rapidly evolving field and the new techniques and technologies

that will be developed in the next few years will hopefully continue to push back these boundaries. The high returns these techniques are currently showing are likely to capture the attention of pharmaceutical industry stakeholders who remain skeptical to such all-encompassing solutions as AI.

Fig 10 . 4 : AI and Drug Discovery: Accelerating the Development of New Pharmaceuticals

In conclusion, AI has the potential to not only revolutionize the drug discovery process and lessen the burden of high-throughput experimentation, but to also simultaneously improve the efficiency of drug development. There are many approaches and tools which can enable a step-change in our understanding of drug action and design. These improvements would accelerate the path towards not only more efficacious and personalized treatments but also eventually a new generation of therapeutics. Drug discovery is a combination of art, craft, and chemistry, and there is unlikely to be a once size fits all solution, rather, a combination of many techniques and integrated platforms and consolidated knowledge for future research. More community participation and sharing of successful pipelines and tools, and less fear, would further accelerate translation and implementation of the findings discussed.

10.7.1. Summary of Key Findings

Key Findings AI technologies offer the potential to revolutionize drug discovery and development and have garnered significant attention and investment across the healthcare, technology, academia, and government sectors. These technologies have the potential to generate highly specific leads that would otherwise remain undiscovered through conventional methods, predict drug toxicity and benefit in the human body, create new treatment options by exploring the use of existing pharmaceuticals in new combinations, or expanding their intended use to a new disease. While prediction-based technologies have the most traction to date, computational solutions across the innovation space are expected to take the lead as they become more clinically validated. AI is expected to provide more targeted and efficient R&D pursuits by identifying patients and target populations that are more likely to display clinical benefits, thus reducing the risk of failure. Efficiency gains are also expected through the collaboration between industry development and technology companies, despite some ethical concerns over collaboration between technology and patient care. Furthermore, AI is being increasingly employed in the recruitment of patients for clinical trials, while data sharing between technology and development companies is being encouraged to support the co-development of companion diagnostics. However, a degree of investment in computation technology is required to ensure that findings in silico models can be translated into clinical practice. In all the applications discussed, the ethical implications of employing AI are a fundamental consideration. A full SWOT analysis is provided in the following section.

10.7.2. Implications for the Future of Drug Discovery

The current study yielded a number of interesting insights, contained illustrative case studies, and discussed the inherent trade-offs of a variety of methodological approaches in AI-driven drug discovery. However, it had limitations. Given the rapid pace of innovation in the area, the relationship presented between the types of data and the classification of analytics methods by vendors is a bit simplistic. It is likely to become out of date as more advanced AI capabilities are integrated with more complex, unstructured data sources. Consequently, other classification criteria may need to be brought in to identify not only the current state of drug discovery but also to identify the more innovative initiatives that integrate, for example, robotics, cheminformatics, or simulation data. Finally, the effectiveness of a number of these innovations is still under active investigation.

Traditionally, AI's main contribution to the pharmaceutical industry has been in screening large datasets for potential hits with desirable qualities. It offers those pharmaceutical companies able to make an investment in its capabilities as a way to identify promising avenues for research in less time than the more traditional approaches. However, AI is forecast to start dramatically reshaping drug discovery and development more broadly as it accelerates integration into existing pharmaceutical workflows. Investing in this direction is an opportunity for pharmaceutical companies to innovate and differentiate themselves by using AI to develop new technologies and identify drug candidates that can prove suitability for clinical development to a greater degree than is possible with today's research, effectively moving faster through the central dogma of therapeutics. A shift from retrospective to prospective research focused on solving the problems of ascent within the pipeline would move AI closer to the beginning of the central dogma of therapeutics, offering companies an even earlier window on the suitability of potential drug candidates emerging from their research programs. Given the application of AI is forecast to offer the ability to run more experiments, analyze more data, and gain a more detailed understanding of safety and efficacy, it has the potential to offer companies a paradigm shift in delivery, which has the promise to deliver on those patient needs most pertinent to the field. Regulating AI in pharmaceutical research is a substantial challenge. The process starts with a deep understanding of how the technology functions and what its potential is relative to more traditional research. At the time of writing, it is unclear if robust ethical AI research guidelines or systems for auto-regulating AI outputs in pharmaceutical research exist; however, the industry is in perpetual dialogue on this issue. Recognizing this, the prediction is that we will see a consensus public meeting in 8–10 years that will set out best practices and considerations for AI use in this area.

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