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Smart Therapeutics: Deep Learning and Machine Learning Models for Target Identification and Drug Candidate Screening in Oncology

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ABSTRACT

By making the process faster, more successful and more economical, Artificial Intelligence (AI) and machine learning (ML) are revolutionizing the field of cancer drug research. Advanced therapeutic approaches are necessary for cancer, a complex and potentially fatal disease characterized by uncontrolled cell proliferation and spread. Conventional drug development techniques are costly, time-consuming and frequently associated with low success rates. This paper emphasizes the role of AI in various phases of cancer drug discovery, including target selection, target validation, drug design and clinical trials. Large biological datasets can be analyzed, potential therapeutic targets can be identified and new compounds with increased efficacy and reduced toxicity can be designed with the aid of AI techniques such as machine learning, deep learning and generative models. Additionally, AI improves monitoring systems, predicts drug responses and optimizes patient selection to increase the efficiency of clinical trials. By enabling individualized treatment based on genetic and molecular data, it also promotes precision medicine. Despite its benefits, challenges including data quality, ethical considerations and model interpretability still exist. In summary, AI is a potent instrument in cancer drug discovery, enhancing human knowledge and accelerating the development of safer, more effective and more affordable treatments. Incorporating AI into pharmaceutical research could greatly improve future outcomes in cancer therapy.

INTRODUCTION

Cancer is a complex condition that arises when specific genes within the body undergo changes, leading to the uncontrolled growth of cells. These rapidly multiplying cancer cells deprive healthy cells of oxygen and nutrients and can often evade detection by the immune system[1]. As a result, cancer has the ability to invade nearby tissues and spread to other areas of the body, a process known as metastasis. Metastasis is the leading cause of mortality among individuals diagnosed with cancer[2].

Around 14.6 lakh (1.46 million) people in India are affected by cancer, according to the Indian Council of Medical Research (ICMR). In 2022, new cancer cases were reported and approximately 8.81 lakh (0.881 million) people died from the disease[3]. The most prevalent cancers in India include breast, lung, cervical, oral and colorectal cancers. It is anticipated that by 2040, there will be more than 20 lakh (2 million) new cases of cancer in India annually. These numbers indicate that cancer is a growing concern and highlight the urgent need to develop effective drugs that can prevent, control, or cure the disease[4].

Developing a new anticancer drug is a lengthy, complex and costly process. The Drug Discovery and Development (DDD) process involves many stages, from understanding the disease and identifying suitable targets to discovering active molecules and testing them in preclinical and clinical studies[5]. On average, this process takes 10-15 years and costs billions of rupees. To reduce this time and cost, artificial intelligence (AI) is now being applied to practically every phase of the cancer drug discovery process[6].

AI is one of the most powerful technologies of the modern era. It enables computers and systems to perform tasks that require intelligent reasoning, learning and decision-making, similar to human capabilities. In cancer research, AI allows scientists to understand how cells, genes and proteins interact within the body. These complex relationships can be represented as biological networks, which AI can analyze more rapidly than humans. Using machine learning (ML) and deep learning, AI can identify potential drug targets, examine disease patterns and design potential drug molecules[7].

AI can also predict whether a compound will be effective as a drug or cause adverse effects, a process referred to as hit triage. In the early stages of drug design, AI-based generative models can create new molecules and predict important properties such as absorption, metabolism and toxicity[8].

Clinical trials in India are also being transformed by AI. It can assist in selecting appropriate patients, hospitals, or trial locations, as well as in monitoring real-time trial data. As a result, the overall process can become faster, more precise and less expensive. In addition to clinical trials, AI is assisting the Indian pharmaceutical sector in pharmaceutical manufacturing, quality assurance and market research[9,10].

With all these advantages, AI has the potential to transform cancer drug research in India. The purpose of this study is to investigate how AI could accelerate the development of effective and affordable cancer medications. This review will first outline fundamental AI and ML methods and then discuss their applications across the various stages of drug discovery, including the identification of novel targets, clinical testing and post-market analysis. Finally, it will highlight the challenges faced by India, as well as the potential applications of AI in cancer research and treatment in the future[11,12].

Humans have been continuously searching for ways to develop medications since ancient times and with the aid of contemporary technology, this search continues today. In the past, it often took 12-15 years or longer to develop a new medication before it was authorized for use.

Additionally, this process is highly costly, typically costing between $1 and $2.6 billion[1,13,14].

In the past, the majority of pharmacological research was conducted using in vivo investigations (experiments on living organisms), particularly between the 1950s and 1980s. To expedite the process, numerous innovative technologies have been developed since the 1980s. These include large compound libraries, structure-based drug design, combinatorial chemistry and *in vitro* screening techniques. The biomedical image analysis discussed below is shown in Fig. 1[15,16].

Despite these developments, fewer new medications have been licensed over the past 20 years. The primary causes of this decline are the increasing complexity of drug development and stringent safety regulations, which make

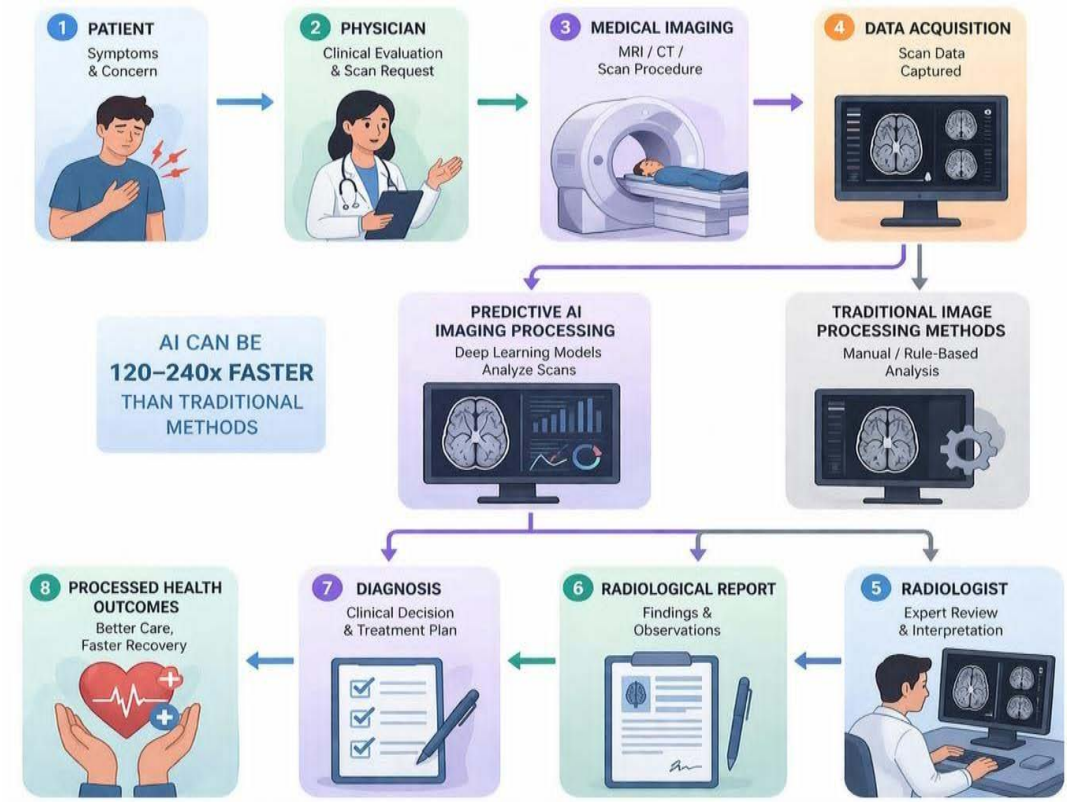


Fig 1: Biomedical image analysis (AI vs traditional techniques)

it more difficult to translate medications from *in vitro* (laboratory) testing to in vivo (living organism) testing[17]. Drug research is currently utilizing computational sciences, particularly artificial intelligence (AI), to address these challenges. AI can analyze large datasets and identify potential new medications that meet clinical needs, making the drug discovery process faster, less expensive and more efficient[10,18,19].

Target identification: A drug target is a biological molecule or site in the body to which a medication binds or acts to treat or prevent a disease, such as a protein, enzyme, or receptor. Identifying a suitable target is a crucial initial step in the development of a novel medication[20].

Drug development has traditionally focused on developing a single medication to treat a specific disease by acting on a single target. However, this is not always the case. Some medications, particularly those derived from natural sources, such as plant-based compounds, contain a wide range of chemical constituents with diverse biological effects and can act on multiple targets simultaneously. Natural products can therefore be particularly valuable for the discovery and development of new medications because of their capacity to interact with multiple biological targets[18,21,22].

However, an important barrier is that researchers still do not fully understand precisely which targets are affected by many of these natural compounds. This knowledge gap has slowed research on and the application of natural products in drug development[23,24]. To address this issue, researchers now use data mining and bioinformatics, which are computer-based approaches for analyzing large amounts of biological data. These tools help identify, select and rank potential disease targets using information from scientific studies, gene expression data, protein analyses, chemical profiles and patents[25]. For example:

- During the COVID-19 pandemic, scientists employed data mining and bioinformatics to rapidly identify potential targets and facilitate the development of drugs and vaccines to combat the virus
- In triple-negative breast cancer with brain metastases (TNBC-BM), drug development is challenging because existing drugs are often too toxic or cannot effectively reach the brain. Although several drugs have entered clinical trials, very few have been approved, despite the identification of numerous potential molecular targets for TNBC-BM. Overall, target identification helps scientists understand where and how a drug should act in the body, while modern computational tools are making this process faster and more accurate[17,26]

Target validation: Once a potential therapeutic target has been identified, scientists must validate it by demonstrating that the target contributes significantly to the disease and can be safely and effectively modulated by a therapeutic intervention. This process involves both in vitro (laboratory-based) and *in vivo* (animal or living organism) experiments to determine how effectively the intervention affects the target and the disease-related processes associated with it[27,28]. Target identification and validation work hand in hand. Using multiple validation approaches increases confidence in the results and reduces the likelihood of failure later in drug development. When these steps are performed carefully, they help ensure that new drugs are more effective and have fewer side effects[29].

Combining phenotypic methods, which study how drugs affect cells or organisms, with target-based approaches helps researchers discover new and potentially powerful drugs, including first-in-class drugs, which represent entirely new types of treatments and best-in-class drugs, which are improved versions of existing therapies[22,30]. A recent study used AI-based methods to identify and evaluate a new anticancer compound called Z29077885, which targets a protein named STK33. The AI system analyzed large public databases and manually collected information on how different compounds interact with diseases. Following the identification of this potential therapeutic compound, researchers investigated its effects in both in vitro (laboratory) and in vivo (animal) models[31].

The findings demonstrated that Z29077885 effectively destroyed cancer cells by inducing apoptosis, or programmed cell death and inhibiting cell proliferation during a particular phase of the cell cycle known as the S phase. Additionally, it induced cancer cell death in specific regions and reduced tumor growth[32].

This study demonstrated how AI-driven methods can accelerate and improve the accuracy of therapeutic target identification and validation, particularly in cancer drug discovery[33].

Stages in drug discovery: Identifying an unmet medical need, that is, determining what type of illness or condition requires improved treatment, is the first step in the process of developing a new medication[30,34]. Scientists then develop theories, or hypotheses, regarding how a novel medication could be superior to existing treatments, either by operating through a different mechanism or by being safer and more effective[34]. After defining the therapeutic objective, scientists examine various substances using appropriate biological techniques to determine whether any show therapeutic potential. Finding a "hit," or a chemical compound that exhibits beneficial biological activity in vitro, is the first significant step[35]. Once a hit is identified, it undergoes preclinical testing, during which its effects on a living system are examined through *in vivo* studies,

primarily in animal models. After that, scientists modify the structure of the original molecule to produce analogues, which are similar compounds intended to increase efficacy and reduce adverse effects. The drug candidate selected for further development is generally the analogue that demonstrates the most favorable overall profile[36,37].

The drug candidate's toxicity and safety profile are then evaluated in animal models. If these preclinical studies are successful, a report known as an Investigational New Drug (IND) application is created and submitted to the U.S. Food and Drug Administration (FDA). If the application is approved, the drug can proceed to human clinical trials[37,38]. There are three primary phases of clinical testing:

- **Phase I:** To assess the drug's safety, tolerability and pharmacokinetic properties, a small group of participants, typically healthy volunteers, is evaluated
- **Phase II:** To determine the appropriate dosage and assess the medication's efficacy and safety, patients with the intended disease or condition are evaluated
- **Phase III:** To confirm the medication's efficacy and safety and to obtain more comprehensive data, it is tested in a larger number of patients, often through controlled clinical trials

Even after progressing through all three phases, only a small proportion of drug candidates ultimately receive regulatory approval. Before a new medication is formally approved for medical use, the FDA's experts examine the data generated throughout the various stages of development[39,40].

A portion of the hypotheses that form the foundation of a research project may result in what is known as a "drug candidate" during the "R" phase (research phase). Approximately one out of every 15-25 drug candidates entering the "D" phase (drug development phase) successfully passes the required safety and efficacy evaluations in humans and animals and is ultimately approved for marketing[41].

AI-driven innovations in oncology: Today, the need for faster and more accurate approaches to drug development is growing rapidly. One of the major challenges in modern medicine is cancer, a disease that continues to threaten human health worldwide. The World Health Organization (WHO) reports that cancer is a leading cause of death globally and is among the leading causes of death in many countries[42,43].

Many anticancer medications, including taxol, vinblastine, vincristine, derivatives of camptothecin, etoposide, irinotecan and topotecan, were originally derived from plants.

Ideally, a good plant-based anticancer drug should be potent against cancer cells, have a well-defined mechanism of action and cause minimal side effects. However, many of these natural compounds face challenges such as poor absorption in the body, unfavorable pharmacokinetic properties, low efficacy and high toxicity, which make it difficult for them to progress from laboratory research to clinical trials[40,44,45].

To overcome these challenges, artificial intelligence (AI) is now playing a major role in cancer research and drug development. AI helps scientists rapidly analyze large amounts of data and identify patterns that connect specific cancers with potential treatments, for example, in cancer immunotherapy[46].

Researchers can develop safer, more potent and faster-acting anticancer medications by utilizing AI algorithms and current information technologies. This approach improves the likelihood of discovering more effective treatments for various cancer types while also saving time and reducing costs[47].

AI's potential to accelerate drug discovery, enhance drug efficacy and reduce toxicity: According to recent studies, artificial intelligence (AI) is a key factor in accelerating the development of novel medications and enhancing cancer therapies[47]. AI tools, including Deep Learning (DL) and machine learning (ML), are transforming several areas of cancer research, ranging from new drug design and clinical trial planning to target validation and drug repurposing, in which existing medications are investigated for novel therapeutic indications[48].

These AI techniques assist scientists in rapidly discovering novel chemical compounds, determining optimal dosages and improving therapeutic efficacy while minimizing adverse effects. AI can analyze enormous volumes of data, predict how patients will respond to medications, enhance the precision of medical imaging and facilitate personalized medicine, in which each patient receives a customized course of therapy[49].

AI is currently applied across many sectors but its influence in medicine is particularly noteworthy. It can enhance the accuracy, consistency and efficiency of healthcare professionals. Nevertheless, challenges remain, including ensuring data integrity and understanding how AI models make decisions. Despite these challenges, AI has considerable potential to transform cancer treatment by accelerating drug development, reducing costs and improving the success of therapeutic development. An excellent illustration was provided by a 2025 study published in *Nature Communications*, which demonstrated how AI can identify potent drug combinations for pancreatic cancer[50].

Using experimental data from the testing of 496 combinations of 32 anticancer drugs on pancreatic cancer cells, researchers from the National Center for Advancing

Translational Sciences (NCATS), Massachusetts Institute of Technology (MIT) and University of North Carolina (UNC) developed machine learning models.

To determine which drug combinations would be most effective, the researchers employed several AI techniques, including Random Forest (RF), XGBoost, Deep Neural Network (DNN) and Graph Convolutional Networks (GCN)[51,52].

The MIT model was the most accurate, correctly predicting laboratory test results for 83% of the 1.6 million conceivable drug combinations. Additionally, 307 effective drug combinations were identified. These findings could potentially be translated into therapeutic applications because the study included both experimental and already-approved medications.

This study demonstrates how machine learning can accurately predict which medications work synergistically, thereby assisting in the more effective development of potent new cancer treatments[53].

Subfields of AI for developing new medicines: A subfield of artificial intelligence (AI), machine learning (ML), enables computers to learn from data and make judgments or predictions without being explicitly programmed. The three main categories of machine learning are supervised learning, unsupervised learning and reinforcement learning[54,55].

In supervised learning, the computer learns from examples that contain both input data and known outcomes (outputs). Applications include disease diagnosis (classification) and prediction of a drug's effectiveness or the body's response to it, including its absorption, distribution, metabolism, excretion and toxicity (ADMET)[56]. In unsupervised learning, the computer does not have predetermined answers. Rather, it uses input data to identify patterns, group observations, or organize the data. This technique is useful for identifying novel drug targets through feature analysis or detecting new disease subtypes through clustering[56,57].

Reinforcement learning is based on decision-making and operates in a distinct way. By experimenting with different actions and refining its performance in response to rewards or feedback, the system learns. Using tools such as modeling and quantum chemistry to guide the process, reinforcement learning can aid in the design of novel (de novo) drugs and the planning of experiments[58,59].

Deep Learning (DL), a specialized area of machine learning, has grown in significance as databases have increased in size and complexity. The DL learns from vast volumes of data using artificial neural networks, which are computational systems inspired by the structure and function of the human brain. Deep learning is particularly powerful for drug development and medical research because it can adapt to complex datasets, identify intricate patterns and improve predictive accuracy[59].

Example of AI use in drug discovery: In 2025, a study employed deep learning (DL) and machine learning (ML) methods to aid in the discovery of novel medications. To identify new therapeutic candidates, the study integrated AI-based drug discovery with multi-omics data, including information from genetics, proteins and other biological levels. TNFRSF10A/TRAILR1 was investigated as a therapeutic target for pancreatic ductal adenocarcinoma (PDAC), an aggressive form of pancreatic cancer[60].

The researchers hypothesized that medications including capivasertib, ergotamine and temsirolimus could function as TRAILR1 modulators, thereby helping to regulate or inhibit the activity of this target. TNFRSF10A/TRAILR1 was found to be highly active in PDAC tumor stroma and cancer cells[61,62].

The study suggested an antagonist strategy, in which the drugs act by inhibiting the target's function, rather than the typical agonist strategy, which activates a target.

Additionally, the researchers demonstrated that integrating molecular dynamics simulations, AI methods (ML and DL) and multi-omics data can improve the efficiency and precision of drug discovery[63].

The study concluded that integrating genomics, systems biology and artificial intelligence technologies may provide valuable insights into difficult-to-treat malignancies such as PDAC and contribute to the development of novel and potentially more effective treatment approaches[64].

AI vs. conventional approaches to the development of new drugs: Although, conventional drug development techniques have proven effective in the past, they also have significant drawbacks. The process is often slow and costly because these approaches frequently rely on trial-and-error studies and may have difficulty accurately predicting how new medications will behave. However, artificial intelligence (AI) is increasingly being applied across many aspects of drug research and development.

AI can assist with:

- Identifying and evaluating drug targets
- Designing novel drugs
- Repurposing existing medications to treat new diseases
- Accelerating research and development (R&D)
- Analyzing biomedical data
- Improving decision-making

A comparison of classical drug development and AI-driven drug development is shown in Fig. 2.

AI tools can address uncertainties in conventional procedures, increase process efficiency and reduce human error and bias. AI is becoming increasingly useful in drug research owing to recent developments such as data augmentation, explainable AI and the integration of AI with laboratory-based drug discovery studies[65].

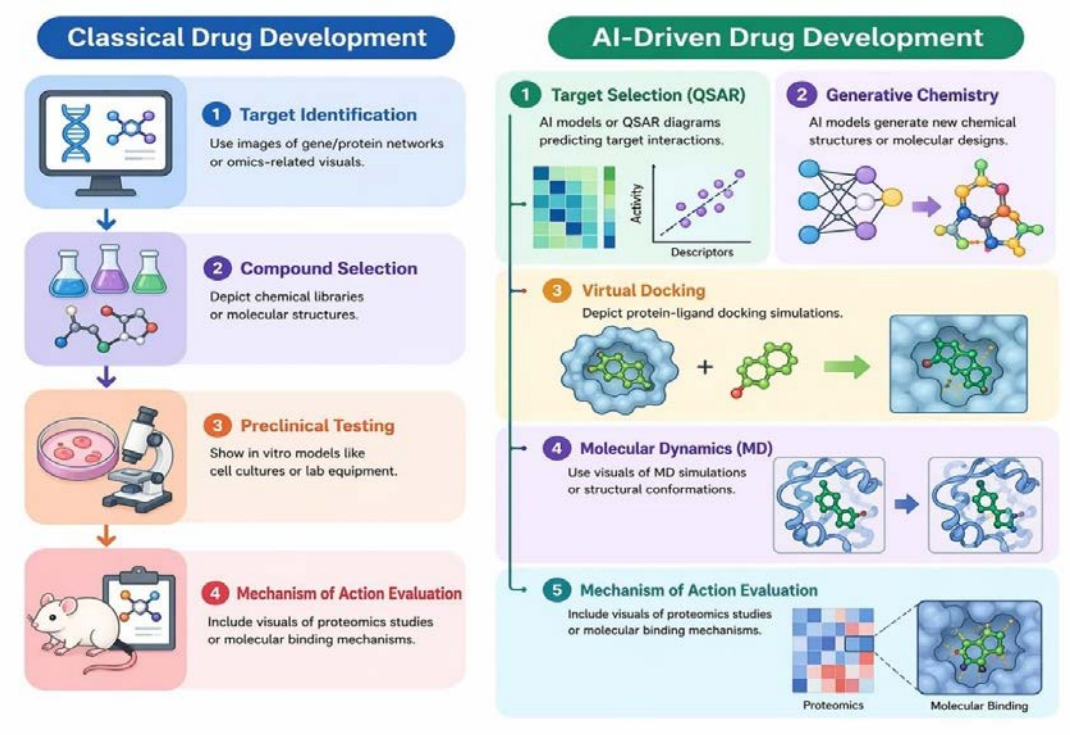


Fig 2: Description of classical versus AI-driven drug development

AI is not flawless, however. The availability of high-quality data is a determining factor and ethical issues must also be taken into consideration. Although AI has assisted in the discovery of novel medications, it still cannot replace scientists' expertise and judgment [65,66].

AI collaborates with human researchers rather than replacing them. It helps by providing data-driven predictions but the results still need to be interpreted and verified by specialists. Therefore, it is critical for pharmaceutical scientists and AI specialists to work closely together to develop improved drug discovery tools and algorithms[67]. This collaboration can assist in:

- Discovering new drug targets
- Improving the effects of existing medicines
- Improving people's quality of life

In clinical trials, AI can also help by rapidly analyzing data to:

- Identify patterns and potential side effects
- Improve accuracy
- Make healthcare more affordable and accessible

One of AI's main roles is to help design new drugs with specific properties. For instance, a deep learning system trained on a database of currently available medications may suggest a new drug with desirable solubility and activity. AI can also predict a drug's potential toxicity or efficacy, thereby aiding the development of safer and more effective therapies[68]. AI is used in medicinal chemistry to analyze vast volumes of data and identify patterns that humans might overlook. This allows AI to suggest potential new medicines more rapidly and with fewer side effects. For instance:

- Deep learning (DL) models can predict how new drug molecules may behave based on historical data
- Training AI models using databases of known safe and toxic drugs can help identify potential safety concerns and prevent harmful side effects in newly developed drugs

In contrast, traditional methods still rely heavily on slow and expensive laboratory testing and the results may vary and are not always reliable.

How AI is used in developing cancer drugs: AI is being widely used in the discovery and development of drugs for cancer treatment. Some of the most important applications include:

- **CADD, or computer-aided drug discovery and design:**
  - AI enables computers to collaborate with scientists to develop new medications more rapidly and precisely
  - This process uses large digital datasets containing medical and chemical information

Table 1: AI Models Used in Oncology: Methods and Restrictions

AI System	How it works (methodology)	Utilization in oncology (application)	Example/Outcome	Limitations (cons)
Computer-Aided Drug design (CADD)	Uses computer and statistical methods to study biochemical data and identify potential drug targets. It helps check a drug's safety, effectiveness and toxicity	Finds protein binding sites carry out virtual screening (SBVS) and predicts drug absorption, distribution, metabolism, excretion and toxicity (ADMET)	Helped discover Halicin, an antibiotic later discovered to possess anticancer properties Designed derivatives of anthraquinone with reduces toxicity and improved outcomes. KRAS inhibitor drug targets that have been developed	1. Accuracy still needs 2. Validation. Binding affinity 3. scoring may be inaccurate Oversimplifies biological systems, leading to low-quality data
Generative Artificial Intelligence (GAI)	Uses generative models trained on existing data to create new molecules with desired chemical and biological properties	Helps design new anticancer drugs for breast and prostate cancers. Analyzes pharmacogenomi c variations to predict how a patient's genes affect cancer treatment response[69]	Develops new drug candidates with improved molecular activity	1. Can produce false results and unrealistic 2. suggestions. 3. Struggles to generalize new candidates. 4. Difficult to interpret outputs for decision making. It depends heavily on the quality of training data[69,70]
High volume screening (HVS)	Tests of biologically active compounds ranging from thousands to million at the cellular and molecular levels and ranks them by performance	Improves treatment selection by identifying the most effective therapy for specific cancers	Speeds up the drug discovery process	1. Over-reliance on AI prediction may result in a lack of practical insights. Can reinforce bias from existing data. Oten gives high false-positive results

- **AI can:**
  - Identify drug targets
  - Help design drugs using virtual tools, known as in silico tools.

Although, newer approaches such as high-throughput screening (HTS) are now widely used, CADD remains an important approach because:

- It helps identify and select promising drug candidates
- It reduces the likelihood of drug candidates failing at later stages of development

Predicting how and where a drug will bind to a target using extensive databases is one of the most common applications of CADD. This approach is particularly useful in the development of new anticancer drugs[68]. Table 1 presents the AI models used in oncology, along with their methods and limitations. The application of AI in drug development is shown in Fig. 3.

Antibody design: AI, especially deep learning (DL), is now used to develop biological drugs such as:

- Monoclonal antibodies
- Peptides
- Oligonucleotides

In cancer treatment, antibodies are used to target specific cancer cells

AI can help discover new antibody sequences by analyzing:

- Their chemical properties
- The arrangement of amino acids

Algorithms such as generative adversarial networks (GANs) and variational autoencoders (VAEs) are used to:

- Learn from existing antibody data
- Suggest new antibody designs with improved targeting abilities

This opens new possibilities for creating advanced cancer treatments in the future[71,72].

Treatment in oncology: Artificial Intelligence (AI) is increasingly being employed in cancer treatment, particularly in complex technologies such as CyberKnife, which uses AI to support highly precise radiation therapy. Machine learning (ML) algorithms have also been trained to evaluate various cancer medications according to their effectiveness in preventing the growth of cancer cells[73].

Combining AI with robotics for radiotherapy, which helps administer radiation to tumors more precisely while conserving healthy tissues, is an important application of AI

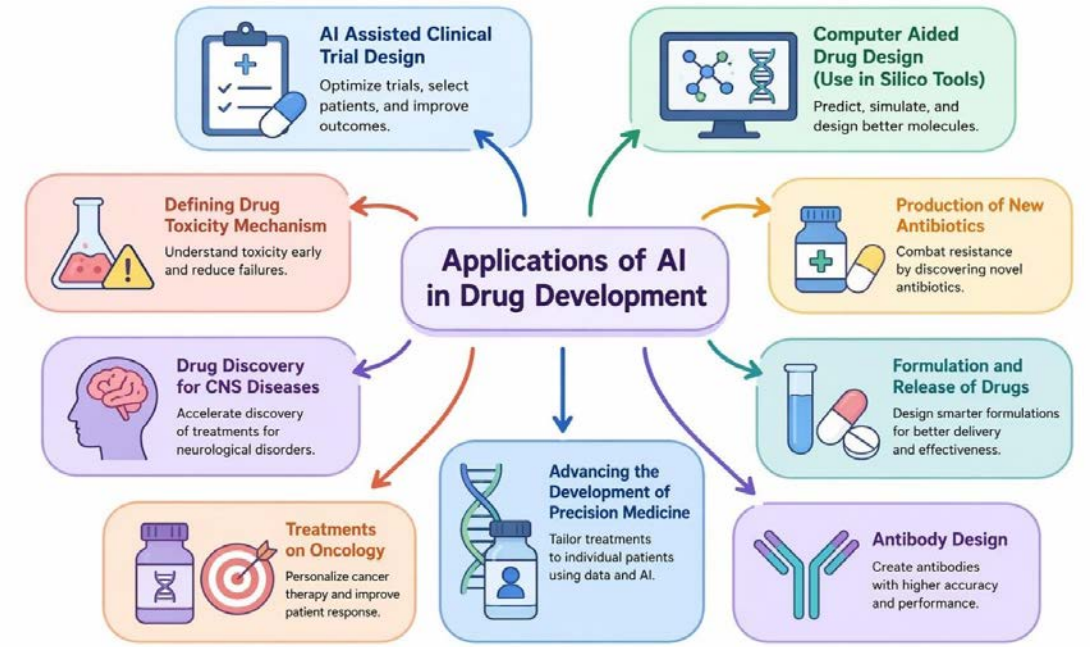


Fig 3. Applications of AI in drug development

in oncology. AI also has considerable potential in cancer imaging, assisting medical professionals in identifying and evaluating tumors more rapidly and accurately.

AI is also essential to the development of novel anticancer medications. It can help researchers overcome several obstacles in conventional drug development by analyzing massive datasets and predicting which compounds might be most effective against particular cancer types[74].

AI-assisted clinical trial design: Approximately 90% of clinical trials fail, representing a significant obstacle to the development of novel medications. AI-based solutions have been developed to address this challenge by enhancing several crucial processes, including patient selection, recruitment, assignment and monitoring.

For instance, by examining a patient's biomarkers and medical history, AI may evaluate patient data and predict how well a patient will respond to a treatment. Additionally, it can employ phenotyping, which aids in grouping patients with similar characteristics to improve the precision and efficacy of clinical trials[75].

AI also enhances patient monitoring in clinical studies. It can monitor whether patients are taking their medications as prescribed, which is crucial because trial outcomes can be affected by medication adherence. AiCure, an AI platform that tracks whether patients take their medication, is a prime example. Compared with conventional approaches, it improved medication adherence by 25% in a phase II trial for schizophrenia[76].

AI can help reduce side effects, optimize drug dosages and lower patient dropout rates owing to safety concerns, thereby making clinical trials safer and more effective.

Promoting the advancement of precision medicine: Rather than employing the same approach for everyone, precision medicine focuses on providing the appropriate treatment to the appropriate patient. Doctors require comprehensive information on every patient, including their medical history, lifestyle, test findings, imaging data and genetic (omics) data, to achieve this successfully.

AI assists by gathering and evaluating this vast quantity of data to develop a customized treatment plan for each patient. Research has demonstrated that AI can link a patient's proteomic, phenotypic and genetic data with their medication response to develop more effective treatment strategies[73,74].

Additionally, AI increases precision, sensitivity and specificity when predicting a patient's response to a particular medication. This enhances the effectiveness of precision medicine, assisting physicians in selecting appropriate treatment strategies and improving patient outcomes.

Generative artificial intelligence (GAI) in drug discovery: One of the newest and most sophisticated AI technologies that makes use of deep learning (DL) is generative artificial intelligence (GAI). From various types of training data, including images, sounds and chemical molecules, it can learn complex patterns. Based on what it has learned, it can then generate new data or make predictions.

In drug discovery, GAI is used to identify new molecules (de novo molecules) and predict how a drug will interact with its target in the body.

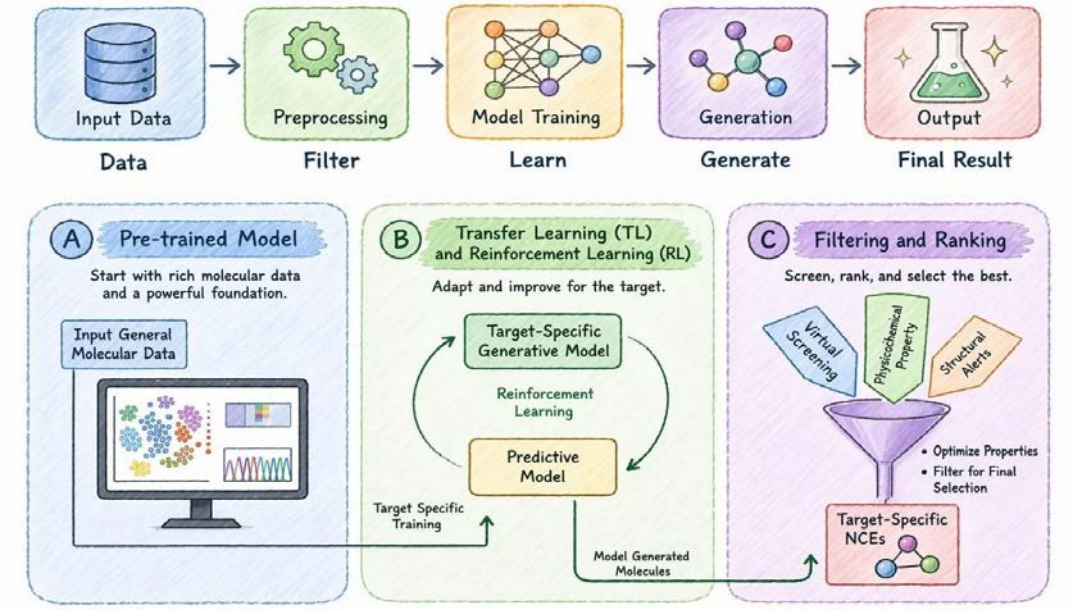


Fig 4: De novo molecular design workflow

Unlike traditional AI models, which can make predictions based primarily on existing data, GAI can create completely new compounds, even when the desired molecules are not present in the chemical library. By learning from existing chemical patterns, GAI can design novel molecules that may have potential as effective drugs[74].

This technology has the potential to transform the drug development process by saving time and reducing costs, as it can automate many steps that traditionally require years of laboratory work.

The de novo molecular design workflow is shown in Fig. 4.

How GAI works: The ability of GAI to create completely new compounds with particular desired properties, a process known as de novo molecular generation, is one of its most intriguing capabilities. In this process, neural networks are trained to predict the properties of molecules and generate new molecules that possess the desired properties.

For example, a model called REINVENT combines a neural network that predicts molecular properties with another network that generates new molecules. This system has been used successfully to design molecules that can inhibit Janus kinase 2 (JAK2), a protein involved in cancer growth[75,76].

GAI can also create chemical libraries containing molecules that focus on one or more desired traits, such as low toxicity or high activity.

Another study showed that, after training a GAI model on natural products, the AI generated new compounds that acted as Retinoid X Receptor (RXR) modulators with potential applications in cancer treatment. When tested, two of the four AI-generated compounds were found to have properties similar to those of natural compounds[77].

GAI in lung cancer drug discovery: In one study, scientists employed machine learning and deep learning to create novel small-molecule antitumor drugs for lung cancer.

Several AI methods were employed in their framework, including:

- Using deep learning to examine protein interactions and gene expression
- Using generative adversarial networks (GANs) to generate novel chemical structures
- Using LASSO linear regression for selecting key features
- Using BERT-based AI tools for identifying chemical names and entities

Five of the 37 candidate compounds identified by the model as potentially targeting specific proteins in lung cancer cells were selected as promising therapeutic candidates following further investigation. The study demonstrated how AI can expedite the discovery and development of targeted cancer medicines by analyzing genetic data and generating novel compounds in silico (through computer-based simulations), although it did not determine whether these molecules could be successfully synthesized in the laboratory[77].

DrugGPT and the new generation of AI models: Similar drug discovery methods, such as DrugGPT, have recently been inspired by tools such as ChatGPT. These approaches employ GAI to generate novel chemical molecules. The primary categories are based on how molecules are generated:

- **Distribution learning:**
  - New molecules that occupy a similar "chemical space" to the training data are generated by the AI
  - Mathematical metrics such as the Kullback–Leibler (KL) divergence and Fréchet ChemNet Distance (FCD) can be used to evaluate the similarity between generated molecules and the original dataset
  - KL divergence can be used to determine how closely the distribution of generated molecules resembles that of the original dataset

- **Goal-directed generation:**
  - The system focuses on specific objectives, such as reducing toxicity or increasing biological activity.
  - Reinforcement learning (RL) and scoring functions are used to progressively generate molecules with improved properties
  - When the AI generates molecules with desired characteristics, such as high bioactivity or structural similarity to known drugs, it receives "rewards"

- **Conditional generation**
  - This approach combines distribution learning with goal-directed generation
  - It enables AI to generate molecules that satisfy specific requirements or "prompts," such as a target protein, a specific three-dimensional (3D) structure, or a gene expression pattern
  - These molecules can be generated end-to-end using methods such as conditional recurrent neural networks (RNNs), reducing the need for independent scoring systems[76,77]

Successful case studies of AI-powered drug discovery: By training deep learning (DL) models on large datasets of known anticancer compounds and their biological activities, artificial intelligence (AI) has been successfully employed to identify new anticancer drug candidates. These AI models can predict novel compounds that may possess anticancer properties by learning chemical and biological patterns. As a result, researchers have identified several promising drug candidates that may have potential applications in cancer treatment.

For example, although the identification of effective MEK inhibitors, a class of proteins targeted in cancer therapy, is challenging, machine learning (ML) algorithms have been used to identify novel small molecules that inhibit the MEK protein. These findings demonstrate how AI can help address some of the challenges associated with traditional drug design[78].

Another example concerns ouabain, a compound originally developed for the treatment of heart failure. Researchers evaluated almost 10,000 compounds using AI-powered high-throughput small-molecule screening and identified compounds that were effective in association with microRNA-34 (miR-34), which functions as a tumor suppressor and helps regulate the proliferation of cancer cells. Researchers observed that combination therapy was required to prevent toxicity associated with high doses of miR-34, despite the AI system demonstrating high accuracy, with 91% sensitivity[78].

A more recent example is the highly specific A2A receptor antagonist EXS-21546. Phase I clinical studies in healthy volunteers have been completed for this drug, which was co-developed using AI by Exscientia and Evotec SE.

EXS-21546 was developed for patients with solid tumors exhibiting a strong adenosine signature, a molecular marker observed in some types of cancer. Its potential use is being investigated in renal cell carcinoma, non-small-cell lung cancer and other advanced solid tumors[78,79].

These findings demonstrate how AI can accelerate the development of precision medicines specifically designed to address the molecular characteristics of particular patient populations. Table 2 presents an overview of effective case studies demonstrating the application of AI models in oncology.

AI in cancer disease modelling: Kumar's 2024 study investigated the use of AI for modelling and predicting cancer progression, therapeutic response and patient outcomes. The study demonstrated that AI algorithms can analyze high-dimensional genetic data to identify biomarkers, which are biological indicators that can be used to predict a patient's response to treatment or the course of a disease. Cancer patients were also categorized according to their molecular profiles using machine learning (ML) and deep learning (DL) models[77,78].

One of the study's main accomplishments was the development of adaptive treatment strategies using neural networks and reinforcement learning. These algorithms can modify cancer treatments in real time based on the distinct genetic characteristics of individual patients. Overall, the study demonstrates how AI-driven disease modelling can enhance patient outcomes, treatment planning and cancer detection, moving medicine closer to a genuinely individualized approach[78].

Drug design, selection and optimization enhanced by AI: According to a review paper, the use of in silico (computer-based) techniques and Artificial Intelligence (AI) can significantly contribute to improving drug development. These techniques use virtual screening (VS) and machine learning (ML) to assist scientists in discovering and evaluating novel drug-target interactions from large chemical databases.

The VS uses experimental data together with the physicochemical characteristics of molecules to develop

Table 2: An overview of the effective case studies that have been found using AI models in the field of oncology

Research title	Publication year	Purpose	AI framework	Results	Summarization
Computational methods for drug discovery and target identification[55]	2016	To demonstrate the significance of combining IT, cheminformatic s and human knowledge to handle and analyze large biomedical information effectively	Computer-assisted drug design (CADD)	Computation al tools helped find, design and optimize new drug molecules. Ligand-based CADD finds new compounds similar to active ones. QSAR connects the structure of molecules to target reaction. Structure-based CADD helps study protein behavior and supports docking and breakage based drug design	All computational techniques continue to improve. Their combination will make drug discovery more accurate and economical in the big data era
Artificial Intelligence (AI) in Pharmaceutic al Sciences[62]	2023	To show how AI is being used in pharmaceutical sciences, especially for drug and target discovery	Various models (ML and DL)	Machine learning methods like OCLR helped discover 13 new genes that could be targets for LUAD cancer treatment. AlphaFold2 and Deep Learning models such as DLAB, DeepAb and DeepH3 helped predict and understand protein structures, improving antibody design	AI is widely used in discovering and developing new drugs. It helps manage biological complexity and supports faster and more accurate drug development
Assessing the Efficiency of Artificial intelligence in Forecasting Adverse Drug Reactions in Cancer Patients[58]	2022	To test how well AI models can predict side effects in cancer patients	Models: GNB, ANN, ANN-I, XGBoost, RT, KNN, GB	Artificial intelligence (AI) algorithms predicted drug toxicity by analyzing biomarkers. Sensitivity values between 0.82-0.84 showed good accuracy in identifying patients at risk of side effects Performance depends on the quality and diversity of training data	AI can accurately predict adverse reactions, improving patient safety and cancer treatment decisions However, more standardized research is needed for consistent results

prediction models. These models aid in the identification of novel therapeutic candidates for particular diseases. Drug repurposing, or identifying new uses for medications that have already received approval, is another application of VS.

According to the paper, deep learning (DL) algorithms used in VS can outperform conventional techniques in predicting drug-target interactions. The VS is typically performed before high-throughput screening (HTS) in the drug development process to exclude unlikely drug candidates. This can save both time and financial resources[79].

However, prediction accuracy, data quality and computational cost remain important challenges. These issues may be addressed in the future as drug design and artificial intelligence continue to advance.

Molecular modelling and simulations: High-performance computers have made it possible for scientists to conduct virtual experiments. One of the main applications of these computers is molecular modelling, which aids in the design and study of new drugs. Previously, molecular modelling primarily focused on examining how drugs interacted with cellular components. However, with the aid of Artificial Intelligence (AI), big data and molecular simulations, researchers can now better understand how drugs interact with their targets, such as receptors and design more effective drugs with fewer side effects.

For instance, Alghamdi et al.[80] used molecular modelling to examine the anticancer effects of fenugreek seed extracts. The researchers investigated the interactions between fenugreek compounds and tubulin, a protein that is an important target in cancer therapy. They determined binding affinities and predicted the absorption, distribution, metabolism and excretion (ADME) characteristics of the compounds. This study demonstrated how combining computational techniques with laboratory experiments can aid in the identification of natural anticancer compounds[79,81].

***In silico* drug-target interaction modelling:** Identifying an appropriate drug target, usually a protein, is one of the most important steps in drug development. AI-powered computational approaches can be used to facilitate this process[82].

When the three-dimensional structure of a target protein is known through techniques such as crystallography, nuclear magnetic resonance (NMR), or cryo-electron microscopy (cryo-EM), researchers can employ structure-based approaches to examine drug interactions with the target. When the target structure is unknown, ligand-based approaches can be used to identify compounds that resemble known active molecules[83,84]. AI is particularly effective in ligand-based modelling because it can detect complex relationships between chemical structures and biological activities that may be difficult to identify using conventional approaches[80].

Alghamdi et al.[85] employed a variety of *in silico* tools to predict and examine the characteristics and functions of compounds derived from the *Calotropis procera* plant. They used online tools such as PASS, SwissTargetPrediction, SwissADME and ProTox-II to investigate biological activity, drug-likeness and toxicity. The study demonstrated that in silico tools can contribute to early-stage cancer drug discovery by predicting potentially effective and safe compounds before experimental validation[86,87].

CONCLUSION

Artificial Intelligence (AI) has become an important tool in contemporary cancer drug discovery. Through the integration of computational technologies and biological insights, AI can accelerate various stages of the drug development process, from target discovery and validation to clinical trials and precision medicine. Unlike traditional methods, which are often slow, expensive and limited by the scale of human analysis, AI can analyze enormous volumes of data, identify complex patterns and predict drug-target interactions rapidly and efficiently. Scientists are now able to develop new compounds, redesign existing medications and model disease processes more accurately through advanced techniques such as machine learning (ML), deep learning (DL) and generative AI (GAI). AI has also shown potential for improving patient recruitment and clinical trial design and for predicting adverse effects, which may contribute to improved safety and efficiency in drug development.

The integration of AI into cancer research offers substantial opportunities to improve the efficacy, affordability and accessibility of cancer therapies. Although, challenges related to data quality, model interpretability, computational requirements and ethical considerations remain, continued collaboration among data scientists, medical researchers and pharmaceutical experts may help advance the application of AI in oncology. In conclusion, AI is a powerful tool that can complement human expertise rather than replace it. Its growing contribution to cancer research represents an important step toward more rapid, data-driven and personalized drug development, with the potential to facilitate earlier cancer detection, more effective treatments and improved patient outcomes.

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