International Journal of Basic & Clinical Pharmacology
Mishra S. Int J Basic Clin Pharmacol. 2026 Nov;15(6):1416-1422
https://www.ijbcp.com
pISSN 2319-2003 | eISSN 2279-0780

DOI: https://dx.doi.org/10.18203/2319-2003.ijbcp20263250

Review Article

Artificial intelligence: transforming drug discovery

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Received: 08 August 2026
Accepted: 24 August 2026

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ABSTRACT

Artificial intelligence (AI) has improved medication research and development by shortening schedules, decreasing costs, and raising success rates. AI uses machine learning (ML), deep learning (DL), and natural language processing (NLP) to analyse large datasets and quickly identify pharmacological targets, forecast chemical efficacy, and optimise medication designs. It speeds up lead development by predicting pharmacokinetics, toxicity, and probable side effects. It also improves clinical trial designs through better patient recruitment and data analysis. Recent advances in protein structure prediction and generative molecular design have further expanded the potential of AI in pharmaceutical research. However, challenges include data quality, algorithmic bias, lack of interpretability, validation, reproducibility, and regulatory concerns remain. Overall, AI represents a powerful complementary technology that may significantly accelerate the discovery and development of safer and more effective medicines. This article highlights the role of artificial intelligence in drug discovery, its applications, challenges and future perspectives.

Keywords: Artificial intelligence, Machine learning, Deep learning, Drug discovery, Pharmacovigilance

INTRODUCTION

Artificial Intelligence (AI) is an area of computer science that aims to create machines that can perform jobs normally done by humans. These tasks involve reasoning, learning, perception, language comprehension, problem-solving, and decision-making. AI systems replicate human cognition through algorithms, data, and computation. AI approaches are widely used for natural language processing, robotics, autonomous vehicles, medical diagnostics, and more. AI is transforming medication design and discovery by bringing revolutionary ways that reduce time and costs associated with old processes.[1] medicine discovery is a complex and resource-intensive process that can take years of research and billions of dollars to get a medicine to market. AI can accelerate drug development by identifying targets, predicting interactions, and optimising chemical architectures. AI integration has revolutionised virtual screening, medication creation, and precision medicine. Virtual screening uses artificial intelligence to predict molecular interactions with target proteins, minimising the requirement for physical testing. De novo drug design uses artificial intelligence to create new molecular structures with desired features, allowing researchers to prioritise potential candidates from the start.[2] AI has become a crucial tool in many fields, enabling systems to accomplish jobs that previously required human intelligence. The AI revolution is driven by various methodologies, each adapted to unique applications and difficulties.

ARTIFICIAL INTELLIGENCE IN DRUG DISCOVERY

Drug discovery is a time-consuming and expensive process that can take decades and billions of dollars to bring a novel medicine to market. AI has become a valuable tool in pharmaceutical research due to the increasing amount of biomedical data and the need for

faster and more efficient development. AI is transforming drug discovery at all stages, including target selection, molecular design, preclinical testing, and trial optimisation. AI techniques such as machine learning (ML), deep learning (DL), natural language processing (NLP), reinforcement learning (RL), and quantum computing (QC) have significant influence. The AI techniques and their applications in drug discovery are illustrated in Table 1.

Table 1: Key AI techniques and their applications in drug discovery.

AI technique	Examples / models	Major applications in drug discovery
ML	Random Forest, Support Vector Machines (SVM), XGBoost	Target identification, QSAR, activity prediction, virtual screening, ADMET prediction.
DL	Convolutional Neural Network (CNN), Recurrent Neural Network (RNN), Graph Neural Network (GNN).	Molecular property prediction, drug-target interaction prediction, virtual screening, toxicity prediction
NLP	BERT, biomedical language models	Extraction of drug-gene disease relationships, target identification, literature and patent mining
RL	Deep RL, policy-gradient methods	Goal-directed molecular generation, optimization of potency, selectivity and drug-like properties
QC	IBM Quiskit, Xanadu pennylane	Molecular simulation, structure optimization

MACHINE LEARNING

Machine learning (ML) is a branch of artificial intelligence that enables computer systems to learn patterns from large datasets and make predictions without being explicitly programmed for every task. In pharmacology, ML is increasingly used to analyze complex biological, chemical and clinical data. Support vector machines (SVMs) and Random forests are supervised learning techniques commonly used to predict drug-target interactions, toxicity profiles, and pharmacokinetic properties (ADMET). ML models may estimate a compound's likelihood of binding to a specific protein, allowing for virtual screening of enormous chemical libraries without costly tests.[3] ML models require huge, high-quality datasets and must be validated and interpreted carefully to avoid biases and overfitting. Machine learning (ML) improves drug discovery efficiency and precision, cutting costs and increasing success rates.

DEEP LEARNING

Deep learning (DL) is a subset of AI and a specialized form of machine learning that uses artificial neural networks with multiple layers to learn complex patterns from large datasets. Convolutional Neural Networks (CNNs) excel in interpreting 3D molecular structures and protein-ligand binding sites, allowing for accurate prediction of binding affinities and drug-target interactions. Recurrent Neural Networks (RNNs) and variants analyse sequential data, such as chemical or protein sequences, to uncover patterns that standard models may miss. Graph Neural Networks (GNNs) have gained popularity for its ability to simulate atom connectivity and chemical interactions by representing molecules as graphs.[4] AlphaFold's ability in accurately predicting protein structures, aided by deep learning algorithms, has accelerated target characterisation. Deep learning improves precision and speed for virtual screening, molecular property prediction, and de novo drug creation, contributing to the digital transformation of pharmaceutical research.

NATURAL LANGUAGE PROCESSING

Natural language processing (NLP) is a branch of AI that enables computers to understand, process, analyze, and generate human language. In pharmacology, NLP is particularly useful because a huge amount of pharmaceutical and medical information exists as unstructured text, such as research papers, electronic health records, clinical trial reports, drug labels and adverse drug reaction reports. NLP accelerates information extraction, allowing for quick identification of important chemical entities, biological targets, and experimental results.[5] NLP helps summarise research data, establish clinical trial protocols, and improve decision-making in drug development. NLP improves drug development by identifying hidden knowledge in textual data, bridging information gaps, and expediting research creativity.

REINFORCEMENT LEARNING

Reinforcement learning (RL) is a branch of artificial intelligence in which an agent learns by interacting with an environment. It takes actions, receives rewards or penalties and gradually learns the best strategy to achieve a desired outcome. In pharmacology, reinforcement learning can be used to optimize drug development, treatment selection, dosing and drug discovery. RL is effective for optimising complex multi-step processes like chemical design, synthesis, and pharmacological properties.[6] RL frameworks automate chemical synthesis and molecular modification, allowing for the creation of new compounds by sequential decision-making. In addition to molecular design, RL is used to optimise synthetic routes. The agent

learns efficient chemical reaction paths to synthesise target molecules, decreasing costs and time in the lab. RL's capacity to learn from subsequent interactions and optimise complex objectives makes it a powerful tool for expediting drug discovery pipeline.

QUANTUM COMPUTING

Quantum computing (QC) is a computational approach that uses principles of quantum mechanics to perform certain calculations exponentially faster than classical computers. Quantum algorithms provide exact simulation of molecular electronic structures and protein folding, crucial for understanding drug-target interactions at the atomic scale. This skill can significantly enhance the accuracy of predicted binding affinities, reaction pathways, and physicochemical attributes, leading to faster lead optimisation and fewer costly experimental iterations. Hybrid quantum-classical algorithms, including the Variational Quantum Eigensolver (VQE) and Quantum Approximate Optimisation Algorithm (QAOA), are being used to tackle small-molecule simulations and optimisation problems for drug discovery.[7] Companies and academic groups are using QC to improve molecular docking simulations, protein design, and combinatorial optimisation of chemical libraries. As quantum hardware advances, QC is projected to become a crucial tool for pharmaceutical AI. The various AI techniques in drug discovery are shown in Figure 1.

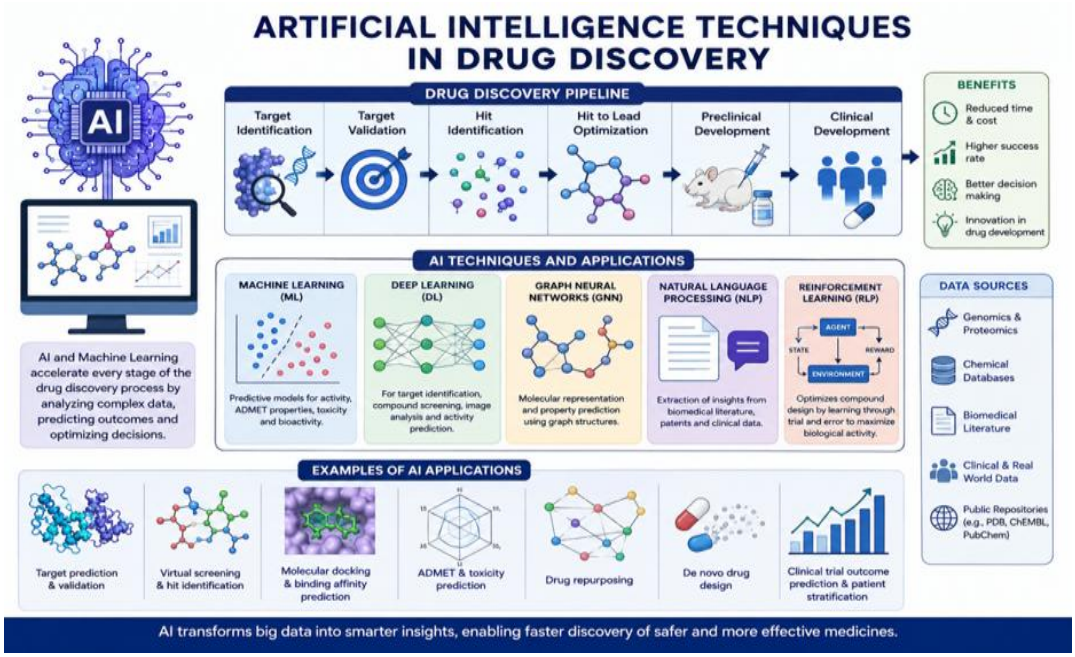


Figure 1: Artificial intelligence techniques in drug discovery.

Figure 1 illustrates major AI approaches, including machine learning, deep learning, natural language processing, reinforcement learning, and quantum computing and their applications in target identification, virtual screening, molecular design, drug-target interaction prediction, toxicity prediction, and optimization of drug candidates.

AI IN DRUG DESIGNING AND DISCOVERY

Target identification and validation

AI improves drug target prediction by analysing a wide range of biological data. AI systems can identify new targets more efficiently than traditional methods by combining genomes, proteomics, and other sources. AI may analyse genomic data to find disease-related genetic variants and prospective targets, such as genes and proteins.[8] AI can analyse proteomic data, including protein structures and interactions, to uncover disease pathways and evaluate druggability. ML models have emerged as powerful tools for understanding gene-disease relationships and discovering new biomarkers.

These models may analyse complicated datasets, including gene expression profiles, SNPs, and protein-protein interaction networks, to identify patterns and associations that traditional statistical methods may miss. Using labelled gene expression and illness status datasets, supervised learning algorithms like SVMs and random forests can predict disease risk and discover genes associated with disease susceptibility. Deep learning (DL) methods, such recurrent neural networks (RNN) and CNN, can analyse complicated genomic and proteomic data to accurately predict illness outcomes. Datasets with 10,000-15,000 entries have been used for target proteins including Mpro (the major protease of SARS-CoV-2) in antiviral drug development and hERG (human Ether-à-go-go-Related Gene) to assess cardiotoxic effects.[9]

Drug screening and lead discovery

AI-powered virtual screening and in silico techniques have transformed the identification of possible lead compounds in drug discovery. These methods use computational tools to analyse large chemical libraries faster and at lower costs than traditional high-throughput screening

These techniques rely heavily on machine learning algorithms. QSAR models can predict the biological activity of substances based on their chemical structures.[10]

These models can scan chemical libraries and prioritise compounds that are most likely to bind to the target of interest.

Drug optimization and design

AI-driven approaches optimise medication characteristics like solubility, stability, and bioavailability. Machine learning algorithms can accurately anticipate critical characteristics by analysing large datasets of chemical structures and properties. QSAR predicts water solubility with 1000-5000 data points, while DL models predict drug stability under different conditions. Predictive models let researchers quickly find and optimise drug candidates with improved physicochemical qualities, boosting the likelihood of successful clinical translation.[11]

Preclinical and clinical development

Predictive modelling of PK, PD, and toxicity profiles is essential for effective drug development. AI approaches, notably machine learning (ML), have considerably improved these capabilities. ML algorithms can analyse large datasets of chemical compounds and their PK/PD properties to predict crucial parameters like ADME and drug-drug interactions. DL models effectively estimate drug absorption across biological membranes, while others simulate drug distribution within the body. Furthermore, AI can predict drug interactions and toxicity profiles. ML algorithms can find correlations between chemical structures and toxicity endpoints, helping researchers prioritise safer treatment options and reduce the likelihood of unanticipated side effects.[12] AI has transformed clinical trial design, patient recruiting, and data analysis, resulting in more efficient and successful research. The various applications of AI in drug discovery are shown in Figure 2.

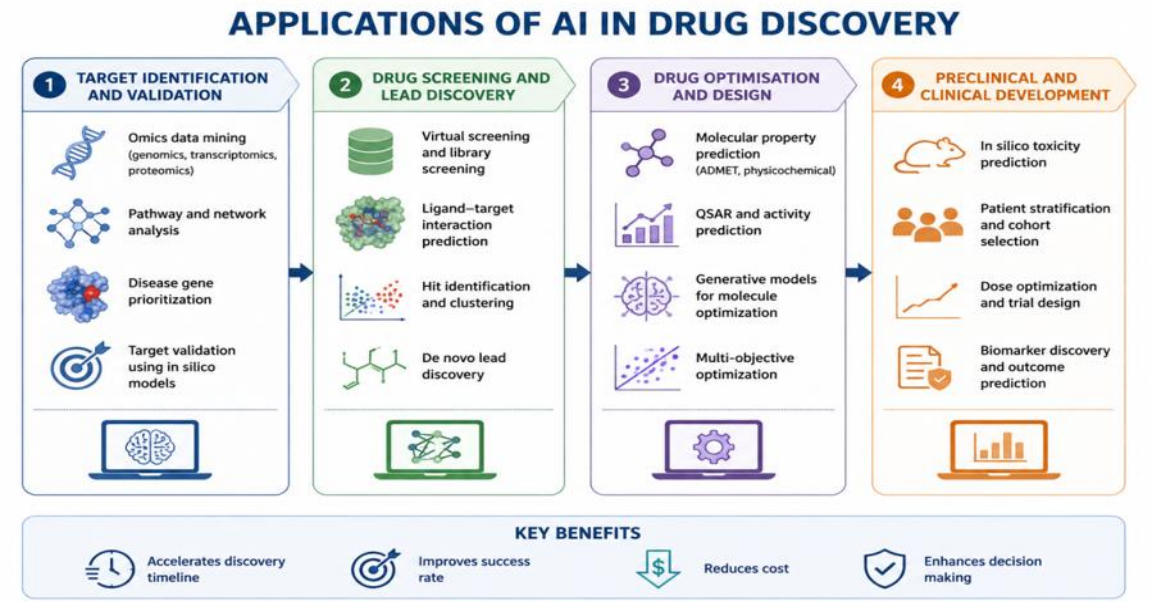


Figure 2: Applications of AI in drug discovery.

Figure 2 illustrates the applications of artificial intelligence in drug discovery. The various applications are target identification and validation, drug screening and lead discovery, drug optimization and design, preclinical and clinical development.

Generative AI in drug design

Gen-AI solutions like ChatGPT have enormous potential in healthcare education and clinical practice. They can improve pharmacy productivity by assisting with prescription reviews, drug interactions, and adverse reaction monitoring, resulting in better patient care. However, limited research on implementation problems highlights the need for additional exploration of their use in pharmacy education.

Apart from this, generative AI models like variational autoencoders (VAEs), generative adversarial networks (GANs) and transformer-based models can generate novel drug like molecules.[13] Their applications include de novo drug design, lead optimization, molecular property

optimization, target based molecular generation and designing new chemical scaffolds while maintaining important pharmacophoric features.

AI in drug repurposing

Repurposing existing medications for novel therapeutics is a cost-effective method that accelerates drug development and reduces trial costs [102]. AI accelerates drug repurposing by analysing large datasets and identifying possible therapeutic uses for new ailments. DeepPurpose and MultiDCP are AI-driven frameworks that use DL models with customisable architectures to predict drug-target interactions, allowing for high-throughput screening and ranking of repurposed drug candidates. Baricitinib, an oral Janus kinase JAK1/JAK2 inhibitor, was initially approved for rheumatoid arthritis effectively repurposed as a COVID-19 therapy with the use of AI.[14]

CURRENT CHALLENGES AND LIMITATIONS

Data quality and availability

AI in pharmacology relies on large, diverse, and high-quality datasets. However, biomedical and pharmacological data is often fragmented, incomplete, biassed, or unstandardized. Electronic health records (EHRs) often contain missing or inconsistent values, which can mislead algorithms unless carefully addressed through imputation or robust model design.[15] Pharmacovigilance databases, such as the FDA's FAERS, suffer from similar issues.

Hidden stratification and data shift

Hidden stratification arises when subgroups in datasets are not properly represented or labelled, resulting in models that perform well overall but fail in certain subpopulations. In pharmacology, factors such as genetics, comorbidities, and age can significantly impact medication responses.[16]

Reproducibility and transparency

AI models in pharmacology sometimes lack reproducibility and interpretability due to their black box nature. Studies often show excellent accuracy on internal datasets but fall short when examined outside. Transparent techniques, such as open-source code, unambiguous reporting, and independent validation, are necessary for successful translation into real-world pharmacotherapy.[17]

Explainability

Explainable AI (XAI) is key to clinical acceptance. To make key decisions such as drug dosing or safety alerts, pharmacologists and physicians must understand why a model recommends a specific option. Saliency maps and attention mechanisms offer interpretability, but they are not without limitations.[18] Real-world deployment of pharmacological AI systems poses safety and reliability challenges. Early research found that even well-calibrated machine learning models can fail to generalise across populations, resulting in unexpected clinical outcomes. Under-reporting of adverse drug reactions (ADRs) is a well-known issue that restricts the accuracy of AI models trained on pharmacovigilance data. Databases like Drug Bank and STITCH provide curated linkages between drugs, targets, and adverse drug reactions, making them valuable foundations for AI-based pharmacology models.

Regulatory hesitancy

Regulators are still responding to the specific difficulties of artificial intelligence. AI algorithms have the potential to evolve over time, rendering static regulatory approval insufficient.[19] Although the FDA and EMA have issued recommendations, frameworks remain fragmented, particularly for pharmacological applications like adaptive dosage and trial optimisation.

Lack of standardized frameworks

While reporting standards like SPIRIT-AI and CONSORT-AI exist, they have not been widely used.

Cost-effectiveness and scalability

AI solutions may be costly to create and implement. Smaller pharmaceutical companies and public pharmacology departments may not have access to the necessary computational resources for advanced AI.

ETHICAL CHALLENGES

Algorithmic bias

Unrepresentative datasets and faulty design can lead to bias in AI systems. Biassed models in pharmacology can exacerbate health disparities, resulting in improper dose, under-reporting of adverse events, and exclusion of vulnerable populations.[20]

Accountability and liability

Legal accountability for AI-driven pharmaceutical judgements remains unclear. If an AI-based dosing system causes an adverse event, who is liable: the physician, the hospital, the software vendor, or the regulator?

Privacy and security

Pharmacological AI often requires access to sensitive patient-level data. This raises issues of data protection, consent, and cybersecurity. The various challenges of AI in pharmacology are shown in Figure 3.

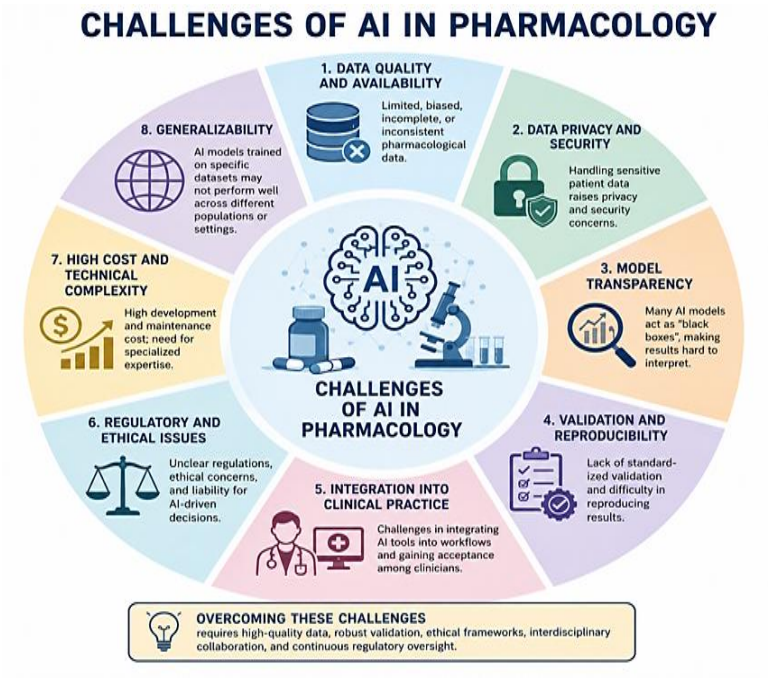


Figure 3: Challenges of AI in pharmacology.

Figure 3 illustrates challenges associated with the application of artificial intelligence in pharmacology. The figure highlights major challenges, including data quality and availability, data privacy and security, model transparency, validation and reproducibility, integration into clinical practice, regulatory and ethical concerns, high cost and technical complexity, and limited generalizability across populations and clinical settings.

FUTURE PROSPECTS

Blockchain and AI in pharmacology

Blockchain and artificial intelligence are complementary technologies that can improve the security, reliability, and efficiency of pharmacological research and drug development. Blockchain is a decentralized digital ledger in which information is stored in linked, tamper-resistant records. In pharmacology, it can provide securable, traceable, and transparent records of research and clinical data.

Blockchain-based clinical trial registries can prevent selective reporting and data manipulation, leading to increased responsibility among sponsors and regulators.[21] When combined with AI, these systems can automatically analyze trial data, identify abnormalities, and optimize endpoints. In pharmacovigilance, blockchain-enabled allows patient to report adverse events directly, with AI aggregating and analyzing these inputs in real time.

Federated learning and privacy preserving AI

The sensitive nature of patient-level data poses a significant hurdle to AI adoption in pharmacology. Federated learning allows AI models to be trained across institutions without centralised data. This strategy protects privacy while increasing the diversity of training data and eliminating algorithmic bias. Federated learning in pharmacogenomics can pool sequencing data from global populations while protecting sensitive information locally. Collaborative analysis of multi-country data in pharmacovigilance can discover safety signals ahead of time without infringing privacy rules like GDPR in the EU or HIPAA in the US.

Digital twins in pharmacology

Digital twins, or virtual patient models, have gained popularity in medicine and pharmacology. A digital twin combines clinical data, genetics, pharmacokinetics, and environmental variables to mimic medication responses and predict consequences before real-world use. Digital twins in oncology can mimic tumour growth and evaluate chemotherapy regimens in silico, enabling personalised treatment. Twins in clinical pharmacology can optimise dose in populations with high variability, such as paediatrics or geriatrics.

Multi-omics integration

AI can manage complicated multi-omics data, including genomics, transcriptomics, proteomics, metabolomics, and microbiomics, and integrate it with PK/PD analysis.

Deep learning models can identify correlations between microbiome profiles and drug metabolism, shedding light on why some patients experience poor response or toxicity.

Integrating proteome and metabolomic data improves biomarker identification and therapeutic efficacy prediction. Integrating AI into drug research and repurposing processes is a promising future area. AI-based models can uncover novel targets, predict chemical interactions, and create new compounds in silico, reducing the time and expense of medication development.

CONCLUSION

Artificial intelligence is transforming drug discovery by enabling faster and more efficient target identification, virtual screening, drug design, optimization, and prediction of drug properties. By analysing large and complex datasets, AI can reduce the time, cost, and failure rates associated with conventional drug development. Despite challenges, such as data quality, model interpretability, validation, ethical and regulatory concerns, continued advances in AI are expected to strengthen its role in developing safer, more effective, and personalized therapies.

Funding: No funding sources
Conflict of interest: None declared
Ethical approval: Not required

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Cite this article as: Mishra S. Artificial intelligence: transforming drug discovery. Int J Basic Clin Pharmacol 2026;15:1416-2.