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Review Article

Artificial Intelligence in Drug Discovery: Trends, Challenges, and Future Perspectives

Aura Rusu*

Pharmaceutical and Therapeutic Chemistry Department, Faculty of Pharmacy, George Emil Palade University of Medicine, Pharmacy, Science, and Technology of Targu Mures, Targu Mures, Romania

***Correspondence to**: Aura Rusu, Pharmaceutical and Therapeutic Chemistry Department, Faculty of Pharmacy, George Emil Palade University of Medicine, Pharmacy, Science, and Technology of Targu Mures, Targu Mures, Romania; Email: aura.rusu@umfst.ro

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ABSTRACT

Artificial intelligence (AI) has evolved from experimental applications to a central tool of modern drug discovery and development. This editorial reviews AI's current impact across the pipeline, from target identification and generative molecular design to preclinical prediction, drug repurposing, and clinical trial optimisation, highlighting advances in multi- omics integration, generative chemistry, and absorption, distribution, metabolism, excretion, and toxicity (ADMET) modelling. Novel directions include hybrid AI-quantum approaches, regulatory modernisation, and collaborative frameworks that accelerate translation from discovery to patient benefit. The challenges in data quality, interpretability, governance, and system integration highlight the need for robust infrastructure and ethical governance. By aligning technical innovation with transparent, interoperable platforms and responsible practices, AI can transform drug design into a faster, more predictive, and patient-centric process.

Keywords: Artificial Intelligence; Drug Discovery; Generative Models; Target Identification; Multi-Omics Integration; ADMET Prediction; Drug Repurposing; Clinical Trial Optimisation; Regulatory Modernisation; Quantum Computing in Drug Design.

INTRODUCTION

For decades, drug discovery has been de ined by long timelines, high costs, and overwhelming failure rates. The average time from idea to market can span more than a decade and cost more than $2.5 billion, while only a small fraction of clinical candidates ever succeed. Within this context, AI has moved from promise to practice, becoming a decisive force across the entire pipeline, from target identi ication and molecular design to preclinical prediction, clinical trial optimisation, and even regulatory review. This inaugural issue coincides with a critical in lexion point in the ield, from isolated proof-of-concept applications to fully integrated, production-grade AI systems embedded within routine research and development work lows. Understanding where AI is already delivering and where it must improve is essential to discussing the next phase of innovation [1,2,3]. The following sections brie ly explore these dimensions, beginning with computational approaches for target identi ication and validation, then advancing to generative techniques for chemical innovation, preclinical prediction strategies, and AI-driven drug repurposing. We then examine AI's role in clinical trials and regulatory modernisation, collaborative frameworks shaping the industry, and the operational challenges of data governance and system integration. The subsequent discussion considers emerging paradigms, such as hybrid AI-quantum pipelines, and the ethical imperatives expected to shape the future of drug design.

COMPUTATIONAL APPROACHES TO TARGET IDENTIFICATION AND VALIDATION

Target identification, once dominated by labour-intensive methods like affinity pull-down and genome-wide screening, is now accelerated by AI. By integrating multi-omics data and building biological networks, AI uncovers disease-related patterns and predicts candidate targets using natural language processing (NLP) embeddings, graph deep learning, and knowledge graphs. Platforms such as PandaOmics exemplify this progress, while real-world data adds contextual depth despite its noise. Challenges remain in data integration, interpretability, and bias, but ongoing advances promise faster and more accurate early-stage drug discovery [2].

AI's impact begins upstream, in translating complex, multi-omic datasets into tractable hypotheses. Machine learning excels at recognising patterns across genomics, proteomics, transcriptomics, and clinical data to propose

previously overlooked targets and biomarkers. In oncology and neurodegeneration, platforms have demonstrated the ability to surface novel targets by fusing pathway knowledge with patient-derived evidence, a direction illustrated by BenevolentAI's work in target discovery and by broader industry analyses that highlight AI's unique pattern-recognition strengths [1,2,4]. Although these approaches expand hypothesis generation, they impose stricter demands on data quality, traceability, and harmonisation. Reviews consistently note that AI is only as strong as the data foundation, and that model interpretability remains critical when linking predictions to biological plausibility and experimental validation [1,3].

EVOLUTION OF GENERATIVE TECHNIQUES FOR CHEMICAL INNOVATION

AI-driven computer-aided synthesis planning and automation are transforming chemical synthesis from a labour-intensive, constrained process into an intelligent, streamlined process [2]. Perhaps the most visible advances lie in de novo molecular design. Generative models, spanning recurrent networks, transformers, reinforcement learning, diffusion, and curriculum learning, now synthesise candidate molecules that optimise multiple objectives simultaneously: potency, selectivity, physicochemical properties, and synthetic accessibility. Open-source frameworks like REINVENT 4 have crystallised best practices for library design, scaffold hopping, and R-group optimisation, making state-of-the-art methods broadly accessible to both industry and academia [5].

Newer multi-objective approaches, such as IDOLpro, push the frontier further by guiding diffusion models with differentiable scoring functions to explore uncharted chemical space and generate ligands that outperform strong baselines on binding and synthesizability, often at a fraction of the cost and time of exhaustive virtual screens [6]. Generative AI is no longer operating in a vacuum. Hybrid experimentation-computation platforms (e.g. Terray Therapeutics) now couple massive, high-quality wet-lab datasets and billions of measured interactions with foundation models that "speak" the language of chemical structure, closing the loop between learning, generation, and empirical validation [7,8].

PRECLINICAL PREDICTION AND THE PUSH BEYOND ANIMAL MODELS

The predictive gap between animal data and human outcomes has long constrained the preclinical stage. AI-enabled in silico modelling is helping reduce reliance on animal studies by simulating absorption, distribution, metabolism, excretion, and toxicity (ADME-Tox) pro iles, lagging liabilities earlier, and prioritising compounds with more favourable pharmacology. A broadening set of companies combines physics-based simulation and machine learning (ML) to predict binding modes and toxicity. At the same time, regulators increasingly acknowledge "new approach methodologies" that complement or replace animal testing in speci ic contexts [9].

ADMET prediction is vital for drug safety and efficacy. AI has shifted from manual feature engineering to deep learning models (transformers, convolutional neural networks (CNNs), and graph neural networks (GNNs)) that learn directly from molecular representations such as SMILES and graphs. Although signi icant progress has been made, challenges remain in data scarcity, interpretability, and generalizability, but self-supervised learning and multimodal approaches show promise for more robust predictions [2].

These developments extend beyond technical innovation; they are fundamentally reshaping ethical frameworks and economic paradigms. Faster, more accurate preclinical readouts reduce unnecessary animal use and trim costs, but they also demand rigour in model transparency and external validation to ensure safety is never compromised [3,9].

AI IN DRUG REPURPOSING

AI is accelerating drug repurposing by uncovering new therapeutic uses for approved drugs through large-scale biomedical data analysis, reducing time and cost compared to traditional discovery. Techniques include deep learning on real-world data, such as electronic health records and insurance claims, as well as omics-based approaches that classify drugs based on transcriptional signatures. Platforms leveraging perturbation data, interpretable models, and high-content imaging (e.g., MitoReID) have demonstrated success in identifying mechanisms of action and novel drug combinations. Although progress has been substantial, persistent issues in data integrity, model transparency, validation expense, regulatory compliance, and computational scalability demand continued methodological re inement for widespread adoption [2].

CLINICAL TRIALS AND REGULATORY MODERNIZATION

AI has become essential in modern clinical research, improving patient recruitment, protocol design, and real-time monitoring through data streams from electronic health records and wearables. The payoff is better-powered studies, earlier detection of safety signals, and shorter timelines [10]. On the regulatory front, agencies are piloting AI to streamline scienti ic reviews. The FDA's recent generative-AI initiatives have reportedly cut repetitive review tasks from days to minutes, signalling a pragmatic embrace of tools that free expert time for high-judgment decisions. Parallel perspectives from policy analysts and industry observers suggest broader global alignment on integrating AI while maintaining robust oversight [11,12,13].

COLLABORATIVE FRAMEWORKS FOR AI-ENABLED DRUG DEVELOPMENT

Recent strategic collaborations highlight the accelerating progress within the ield. Large pharma's and biotech's are building GPU-powered "AI factories", engaging in billion-dollar partnerships, and expanding access to sovereign AI supercomputing for peptide and protein design, developments that are shaping the operational foundations required for scalable, validated AI deployment in drug discovery. Examples include Eli Lilly's work with NVIDIA, Sano i's partnership with Insilico, and Zealand Pharma's engagement with Denmark's Ge ion supercomputer for advanced peptide simulations [14,15,16].

Eli Lilly and NVIDIA established a co-innovation AI lab that links automated wet labs with computational models in a continuous-learning loop powered by BioNeMo and hyperscale GPUs. The lab's 24/7 feedback system rapidly retrains models from real experimental data, cutting iteration times from weeks to hours and setting a new benchmark for AI-driven experiments. The initiative also extends AI use beyond discovery into clinical development, manufacturing, and digital-twin supply chains [17].

Sano i uses Insilico's Pharma.AI platform that integrates target identi ication, generative molecule design, and prioritisation into a single work low. With 22 preclinical candidates since 2021 and positive Phase IIa results for rentosertib, the collaboration shows that generative AI can deliver clinically advancing small molecules. The resulting scale and clinical traction position Pharma.AI as a platform-level benchmark rather than a project-speci ic tool. Insilico and Qilu expanded their relationship from software licensing to full research co-development in cardiometabolic disease, applying Pharma.AI to design small-molecule inhibitors in a deal worth nearly $120 million. The shift to shared pipeline development has already advanced several assets from hit discovery toward investigational new drug (IND)-enabling stages, establishing a model for AI-driven co-development alliances [18].

Zealand Pharma is leveraging Denmark's Ge ion AI supercomputer to accelerate peptide discovery for metabolic diseases using national-scale graphics processing unit (GPU) infrastructure. Its unique strength is a sovereign, peptide-optimised compute platform that sets a benchmark for country-level high-performance computing (HPC) applied to therapeutic design. The collaboration also aligns with Zealand's Metabolic Frontier 2030 strategy [19]. NVIDIA's BioNeMo platform offers large, biology- and chemistry-focused foundation models with lexible cloud-to-supercomputer deployment, enabling teams to unify structure prediction, generative design, and docking within a single system. Its key advantage is infrastructure standardisation, which provides a replicable framework for scaling multimodal AI across discovery, manufacturing, and digital-twin operations [17].

Terray integrates billions of proprietary high-throughput measurements with foundation models to operate a closed-loop Design–Build–Test–Learn cycle that continually refines generative outputs. Its unusually dense wet-lab data (far richer than typical public datasets) improves prioritisation accuracy and significantly shortens medicinal-chemistry iteration times [20].

Exscientia showed that AI-designed molecules can advance into human trials, with candidates like DSP-1181 reaching Phase I on accelerated timelines, despite some programs not succeeding. Its end-to-end automated workflow helped establish the expectation that generative AI candidates can be clinically viable, while highlighting the need for strong attrition management [8]. The growing adoption of AI platforms, exemplified by Insilico's software licensing deals with 13 of the top 20 pharma companies and major pipeline out-licensing agreements with groups like Exelixis and Menarini (totalling over $2 billion), shows that AI has moved beyond pilots into enterprise-scale toolchains. The combination of licensed platforms and co-development models is setting a new benchmark for scalable, validated AI deployment across therapeutic portfolios [18].

Early clinical outcomes from AI-identi ied candidates progressing to Phase I in markedly reduced timelines, and from programs advancing toward Phase III, indicate that accelerated discovery can translate into tangible patient bene its, highlighting the role of these collaborations in shortening feedback loops and improving translational readiness [14,21]. Collectively, these developments signal a fundamental shift from isolated AI experiments toward integrated, industry-scale infrastructures that are capable of sustaining reproducible, validated, and clinically relevant innovation across the drug discovery pipeline. By aligning computational capacity, data quality, and cross-sector collaboration, these strategic partnerships are not only accelerating discovery timelines but also rede ining the operational standards by which AI is translated into therapeutic outcomes. Thereby, they represent not just technological progress, but an emerging blueprint for how the ield can mature responsibly and effectively in the years ahead.

CHALLENGES IN DATA QUALITY, GOVERNANCE, AND SYSTEM INTEGRATION

Recent developments reveal that the challenge is not the models themselves, but the operational systems required for their effective deployment. Many generative-AI pilots failed to produce measurable impact because they were disconnected from real workflows, lacked clean data pipelines, or were deployed without durable governance. AI-enabled drug discovery programs often encounter recurrent categories of failure modes that highlight the practical vulnerabilities of these approaches. First, model reproducibility issues frequently arise when training data lack proper lineage or when evaluation pipelines are not consistently version-controlled, leading to results that

cannot be replicated across teams or time. Second, pipeline failures occur when prototype models are integrated into real-world workflows without sufficient observability or change control, allowing silent performance degradation. Third, many generative-AI pilots remain unrealized, not because the methods lack promise, but because they are disconnected from wet-lab validation cycles or depend on data infrastructures that are not yet mature. Acknowledging these categories of setbacks highlights the importance of transparency, prospective validation, and governance frameworks that ensure AI systems behave reliably once deployed. The next phase will centre on platform thinking, observability, versioning, and change control, acknowledging that AI behaves like infrastructure once embedded [22].

Regulatory and legal frameworks must evolve concurrently. IP strategies for AI-enabled discovery require careful identi ication of protectable elements across the stack (models, data, lab automation, and integration), while compliance and safety demand transparent documentation of how AI contributes to decisions from hit-to-lead through clinical evidence generation [23].

INTEGRATING AI AND QUANTUM FOR NEXT-GENERATION DRUG DESIGN

As computing and algorithms advance, hybrid AI-quantum pipelines are emerging to tackle challenging targets and vast chemical spaces with improved efficiency. In the near term, deployable capabilities remain limited to hybrid, quantum-inspired optimisation techniques and classical simulations that have demonstrated measurable performance in benchmarking studies. These approaches represent the technologies currently in operational use, supported by early published performance metrics and prototype workflows. In contrast, the application of true quantum hardware to drug discovery remains an early-stage, exploratory trajectory.

Early demonstrations suggest that quantum classical methods can enhance sampling and optimisation for challenging oncologic targets. However, these results are still experimental and contingent on future advances in qubit stability, error correction, and scalable architectures. This distinction clari ies that quantum-accelerated generative design remains a forward-looking possibility rather than an immediately deployable capability [24]. At the same time, the profession must commit to responsible scale: bias mitigation, fairness across diverse populations, transparent model reporting, and rigorous prospective validation. Regulators will continue to modernise, but trust will hinge on the community's ability to explain and verify AI's contributions in human-relevant terms [3,11].

CONCLUSION

AI has transitioned from a peripheral proof-of-concept to an integral component of contemporary drug design. The most important shift is cultural and infrastructural: treating AI as an integrated system that is owned, monitored, and validated within the scienti ic process. If we match technical progress with robust data foundations, interoperable platforms, and responsible governance, we can translate speed into quality and reduce the distance between discovery and patient bene it. This inaugural issue provides a critical platform for advancing discourse beyond rhetoric, fostering the exchange of operational, methodological, and ethical frameworks that will shape the future of drug design.

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CONFLICT OF INTEREST

The author declares no conflicts of interest.

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