Artificial Intelligence in Precision Pharmacotherapy: Clinical Applications, Opportunities, and Challenges

Duoduo Mou

Faculty of Computing, Universiti Teknologi Malaysia, 81310 Johor, Malaysia

Abstract: Artificial intelligence is emerging as a key enabler of precision pharmacotherapy by improving individualized drug selection, therapeutic monitoring, and dose optimization. Machine learning models integrate clinical, molecular, pharmacokinetic, and longitudinal data to characterize interpatient variability and anticipate therapeutic response. Clinical applications include prediction of benefit and toxicity, pharmacogenomic interpretation, therapeutic drug monitoring, and adaptive dosing. These opportunities align with priorities to reduce adverse events, enhance treatment efficiency, and personalize therapeutic trajectories. However, challenges remain in validation, generalizability, interpretability, regulation, and workflow integration. Translational progress will depend on hybrid approaches that combine pharmacological knowledge with data-driven inference, interpretable decision support, and uncertainty-aware prediction. Future development may expand closed-loop exposure control, multi omics integration, virtual physiological models, and model informed trial design. Artificial intelligence has potential to reshape individualized therapy by making response more predictable, dosing more adaptive, and clinical reasoning more anticipatory.

Keywords: artificial intelligence; precision pharmacotherapy; therapeutic drug monitoring; adaptive dosing; pharmacogenomics; clinical decision support; personalized medicine

1. Introduction

Precision pharmacotherapy seeks to tailor treatment to individual variability in drug disposition, response, and toxicity. Conventional dosing strategies rely on population averages and empirical titration, limiting personalization in settings with narrow therapeutic indices or heterogeneous disease biology. Advances in genomics, digital monitoring, and pharmacometric modeling have increased the volume and complexity of data relevant to individualized therapy. Artificial intelligence offers computational approaches to synthesize these heterogeneous data streams, identify predictive patterns, and support individualized therapeutic decisions. Interest in artificial intelligence reflects unmet needs in oncology, transplantation, infectious diseases, and neurology, where dosing accuracy, toxicity control, and responder stratification affect clinical outcomes. Artificial intelligence complements mechanistic pharmacology and aligns with health-system priorities that emphasize personalization and reduced medication-related harm.

2. Clinical Applications

Clinical applications illustrate how artificial intelligence informs individualized pharmacotherapy. Predictive models estimate therapeutic benefit, toxicity, and response likelihood by analyzing clinical histories, biomarkers, imaging, and disease characteristics. This is especially relevant where response is heterogeneous or therapeutic indices are narrow, and where small exposure differences can yield divergent outcomes[1]. Population stratification based on predicted benefit–risk profiles supports more rational selection of therapeutic agents.

Pharmacogenomics represents a second domain. Machine learning integrates genotypic variation with clinical covariates to clarify genotype–phenotype relationships that influence absorption, metabolism, and elimination, enabling genotype-guided dosing for drugs affected by polymorphic enzymes and transporters, and aligning with broader goals in personalized medicine.

Therapeutic drug monitoring also benefits from AI through predictive estimation of concentrations, exposure trajectories, and individualized pharmacokinetics. These capabilities may facilitate attainment of pharmacodynamic targets with fewer titration cycles, particularly in antimicrobials, immunosuppressants, and oncology. Adaptive dosing extends this principle by updating regimens from real time physiological or laboratory data, with reinforcement learning and control algorithms enabling continuous exposure management.

AI enabled clinical decision support integrates pharmacological knowledge, interaction constraints, patient variables, and therapeutic goals to guide prescribing at the point of care[2]. Such systems assist with sequencing strategies and complex dosing scenarios, collectively demonstrating how AI may complement clinical pharmacology by improving prediction, monitoring, and exposure modulation over treatment courses.

3. Opportunities for Individualized Therapy

Artificial intelligence enhances individualization by enabling multidimensional characterization of variability in pharmacokinetics, pharmacodynamics, pharmacogenomics, and disease processes. Integration of multi-omics and temporal monitoring data supports more granular patient stratification and anticipatory management of toxicity. Predictive modeling improves exposure–response estimation and allows clinicians to evaluate hypothetical treatment trajectories before clinical deterioration occurs. These capabilities reduce reliance on trial-and-error titration and support value-based care by improving therapeutic efficiency and reducing adverse events.

Artificial intelligence may also accelerate translational pipelines by refining patient selection, improving endpoint prediction, and facilitating model-informed clinical trials. Stratification of phenotypic or molecular subgroups increases the feasibility of targeted therapies and supports precision-oriented study designs. Alignment with precision medicine initiatives underscores broader system-level benefits, including optimized resource utilization and improved coordination of care in chronic and multimorbid conditions[3].

4. Challenges for Clinical Translation

Despite rapid methodological progress, integration of artificial intelligence into precision pharmacotherapy remains constrained by clinical, regulatory, and organizational barriers. Retrospective validation does not ensure performance in real world environments characterized by incomplete data, irregular sampling, heterogeneous laboratory practices, and dynamic disease processes. Generalizability is further limited by scarcity of representative training data, with minority groups, pediatric populations, rare diseases, and multimorbidity frequently underrepresented, raising concerns about equity in therapeutic outcomes.

Interpretability constitutes a major barrier. Dose adjustment and therapeutic sequencing require reasoning aligned with pharmacokinetic and pharmacodynamic principles, and clinicians may hesitate to act on recommendations that lack transparent rationale. Regulatory agencies require evidence of safety, consistency, and clinical utility[4], yet boundaries of liability remain ambiguous when automated systems influence dosing or exposure management.

Implementation challenges stem from workflow heterogeneity across healthcare settings. Artificial intelligence systems must integrate with electronic health records, prescribing pathways, and institutional constraints. Tools that increase cognitive burden or workflow friction are unlikely to be adopted even with strong predictive performance. Prospective validation, post market surveillance, and standardized evaluation metrics remain central to translating artificial intelligence from research contexts into routine clinical practice.

5. Future Directions

Future development in AI-enabled precision pharmacotherapy may rely on hybrid modeling strategies that integrate pharmacokinetic and pharmacodynamic frameworks with machine learning. Such strategies may improve interpretability, support extrapolation beyond training domains, and facilitate regulatory review by linking computational inference to pharmacological principles. Closed-loop therapeutic control represents another avenue, in which dosing decisions are updated from real-time exposure metrics, biomarker trajectories, or toxicity signatures. Reinforcement learning and sequential control algorithms could enable adaptive titration for therapies requiring continuous exposure modulation.

Virtual physiological twins may simulate individualized exposure–response trajectories using patient specific parameters, assisting with treatment planning, dose verification, and model informed trial design[5]. Multi omics integration, including genomics, transcriptomics, proteomics, metabolomics, and microbiome profiles, may clarify molecular determinants of variability and improve stratification of responders and non responders. Continuous physiological monitoring through digital health platforms and wearable devices may extend assessment of therapeutic responses into real world care settings.

Uncertainty-aware modeling constitutes an additional priority. Quantifying predictive uncertainty may strengthen communication of therapeutic risk and enhance reliability of dosing recommendations under dynamic clinical conditions. Translational progress will further depend on prospective validation, adaptive regulatory frameworks, and scalable implementation that aligns AI tools with electronic health records, workflow patterns, and prescribing practices. Interdisciplinary collaboration across pharmacology, pharmacometrics, digital health, regulatory science, and systems engineering will shape whether AI becomes a routine component of individualized therapeutic management.

6. Conclusion

Artificial intelligence offers opportunities to advance precision pharmacotherapy by improving prediction of drug response, enhancing therapeutic monitoring, and enabling individualized dosing. By integrating pharmacokinetic and pharmacodynamic information with pharmacogenomic variation and clinical biomarkers, AI may better characterize therapeutic variability and support attainment of expo-

sure targets, aligning with broader goals in precision medicine.

Yet clinical adoption requires rigorous validation, interpretability, regulatory clarity, and workflow integration. Prospective evaluation is needed to confirm that AI-driven recommendations improve outcomes and reduce medication-related harm. Physician acceptance will depend on uncertainty quantification, model transparency, and clear communication of dosing rationale, while regulatory frameworks must address safety, updating, and accountability.

AI may ultimately reshape therapeutic reasoning by making dosing more anticipatory, treatment more adaptive, and pharmacotherapy more personalized. Integration into electronic health records and decision support systems could enable scale and reduce cognitive burden, though questions remain regarding data governance, equitable access, and sustainability. Realizing this potential will determine whether AI becomes a routine component of individualized medical management and whether precision pharmacotherapy moves from specialized practice to mainstream care.

References:

[1] Poweleit EA, Vinks AA, Mizuno T. AI and ML approaches to facilitate therapeutic drug management and precision dosing. Ther Drug Monit. 2023;45(2):143–150.

[2] Hall NM et al. Model-informed precision dosing improves outcomes in patients receiving vancomycin. Open Forum Infect Dis. 2024;11(1):ofae002.

[3] Taherdoost H. AI's role in revolutionizing personalized medicine by transforming pharmacogenomics. Intelligent Pharmacology. 2024;2(1):1–18.

[4] Polasek TM. Virtual twin for healthcare management and individualized therapy. Front Digit Health. 2023;5:1246659.

[5] Mitchell J et al. Digital health and precision therapeutics: Opportunities and regulatory considerations. Nat Med. 2023;29(7):1590–1601.