Advances in Pharmacology and Therapeutics Journal

SHAHID SADOUGHI UNIVERSITY OF MEDICAL SCIENCES

Editorial Article

Translational Pharmacology: Bridging Scientific Discovery and Patient Care

Mohsen Zabihi[1,2*]

[1]Traditional Pharmacy and Pharmaceutical Science Research Center, Faculty of Pharmacy, Shahid Sadoughi University of Medical Sciences, Yazd, Iran.
[2]Department of Toxicology and Pharmacology, Faculty of Pharmacy, Shahid Sadoughi University of Medical Sciences, Yazd, Iran.

***Corresponding author**
Traditional Pharmacy and Pharmaceutical Science Research Center, Faculty of Pharmacy, Shahid Sadoughi University of Medical Sciences, Yazd, Iran.
ORCID: 0000-0002-8929-3684; E-mail: mzabihi1000@gmail.com

Received: 09 Aug 2026; **Accepted:** 23 Sep 2026

Citation: Zabihi M. Translational Pharmacology: Bridging Scientific Discovery and Patient Care. Adv Pharmacol Ther J. 2026;6(2): 68-77. DOI: https://doi.org/10.18502/aptj.v6i2.????

Introduction

The history of pharmacology is fundamentally a history of translation. Every major therapeutic achievement has required the conversion of scientific knowledge into interventions capable of improving human health. From conventional small-molecule drugs to biologics, gene therapies, and precision medicines, pharmacology has continuously evolved with advances in biomedical science.

Nevertheless, scientific discovery does not always lead to clinical success. Identification of a molecular target or demonstration of biological activity in experimental systems does not guarantee development of an effective therapy. The pathway from discovery to clinical application remains associated with high failure rates, uncertainty, and substantial investment.

Traditional drug development follows a sequential pathway involving target identification, preclinical evaluation, clinical trials, regulatory approval, and post-marketing surveillance. Although this approach has generated major therapeutic advances, increasing understanding of biological complexity has revealed important limitations. Human diseases are rarely caused by isolated molecular abnormalities; instead, they result from interactions among biological pathways, genetic factors, environmental influences, and individual patient characteristics.

This complexity is particularly evident in cancer, neurodegenerative disorders, autoimmune diseases, and metabolic conditions. Patients with similar clinical diagnoses may have different molecular profiles and therapeutic responses. Consequently, the concept of developing a single therapy for a broadly

Copyright © 2026 Advances in Pharmacology and Therapeutics Journal. Published by Shahid Sadoughi University of Medical Sciences.

This work is licensed under a Creative Commons Attribution-Noncommercial 4.0 International License (https://creativecommons.org/licenses/by-nc/4.0/).

defined disease population is increasingly being replaced by strategies aimed at identifying the most appropriate treatment for specific patients.

Translational pharmacology represents this evolving paradigm. Translation should no longer be considered a final step after laboratory discovery but rather an integrated process beginning at the earliest stages of therapeutic development and continuing through clinical implementation.

The central challenge of modern pharmacology is therefore shifting from discovering molecules capable of modifying biological pathways toward determining whether these interventions can provide meaningful benefit in real-world clinical settings.

Several emerging approaches are accelerating this transformation. Biomarkers improve patient selection and treatment monitoring. Pharmacogenomics enhances understanding of variability in drug response. Human-relevant models increase confidence in preclinical prediction. Artificial intelligence enables analysis of complex biological datasets. Model-informed drug development improves decision-making, and real-world evidence expands knowledge beyond controlled clinical trials.

However, technology alone cannot overcome the translational challenge. Successful translation requires integration of scientific innovation with clinical expertise, regulatory science, healthcare implementation, and patient perspectives.

The future success of pharmacology will depend not only on generating new discoveries but also on creating reliable pathways through which discoveries improve patient outcomes.

The Translational Gap: From Scientific Promise to Clinical Reality

Despite remarkable progress in biomedical research, translating promising discoveries into successful therapies remains challenging. Many therapeutic candidates demonstrate strong activity in experimental systems but fail during clinical development because of inadequate efficacy, unexpected toxicity, insufficient target validation, or inaccurate prediction of human responses (1, 2).

A major contributor to translational failure is the limited predictive capacity of conventional preclinical models. Animal models and traditional cellular systems have provided essential insights into disease mechanisms and pharmacology; however, biological differences between experimental systems and humans may limit their ability to predict clinical outcomes.

This limitation is particularly important in complex diseases involving multiple interacting pathways. A therapeutic strategy that appears successful in simplified experimental models may not produce similar results in patients with diverse genetic backgrounds and heterogeneous disease biology.

These challenges have stimulated development of human-relevant approaches, including organoids, organ-on-chip technologies, and patient-derived models, which aim to better represent human physiology and improve prediction of therapeutic responses before clinical exposure (3, 4).

Disease heterogeneity is another major barrier. Traditional classification based mainly on clinical characteristics is increasingly being complemented by molecular and biological information. This transition has accelerated precision pharmacology, in which

treatment decisions are guided by individual patient characteristics rather than disease labels alone.

However, precision medicine also creates new challenges. Large-scale biological data do not automatically translate into improved clinical decisions. The key challenge is converting complex information into validated and practical tools that can guide treatment selection.

Therefore, the translational gap should not be considered only a scientific problem. It reflects challenges involving biology, methodology, regulation, healthcare systems, and implementation. Bridging this gap requires a multidisciplinary approach in which every stage of therapeutic development contributes to the ultimate goal of improving patient care.

Emerging Drivers of Translation: From Discovery-Oriented Pharmacology to Predictive Therapeutics

The future of translational pharmacology will depend not only on discovering new therapeutic candidates but also on improving the ability to predict which interventions are most likely to benefit specific patient populations. The traditional approach based on average treatment effects is increasingly being replaced by strategies that incorporate biological diversity, individual variability, and real-world clinical complexity.

Several emerging approaches are reshaping the pathway from scientific discovery to patient care.

Biomarkers: Moving Toward Clinical Decision-Making

Biomarkers have become essential components of modern translational pharmacology. Their role has expanded from indicators of biological activity to tools that support therapeutic selection, prediction of treatment response, disease monitoring, and identification of patients most likely to benefit from specific interventions.

The impact of biomarker-guided strategies is particularly evident in oncology, where molecular characteristics have transformed therapeutic approaches. Identification of genetic alterations and molecular signatures has enabled targeted therapies and companion diagnostics, demonstrating that biological classification may sometimes be more clinically meaningful than traditional disease categories.

Despite extensive biomarker discovery, relatively few biomarkers have achieved routine clinical implementation. Major barriers include insufficient validation, limited reproducibility, uncertain clinical utility, and challenges in integrating biomarker testing into healthcare systems.

Future biomarker development should therefore focus not only on biological relevance but also on clinical impact. A successful biomarker should improve therapeutic decisions and contribute to measurable patient benefit. The transition from biomarker discovery to clinical implementation remains a critical step in advancing precision pharmacology.

Pharmacogenomics: Toward Individualized Drug Response Prediction

Variability in drug response remains a major challenge in clinical pharmacology. Patients receiving identical treatments may experience different outcomes because of differences in genetic background, drug metabolism, transport mechanisms, immune responses, and environmental factors.

Pharmacogenomics provides an important framework for understanding these differences by evaluating how genetic variation influences drug efficacy and toxicity. Several pharmacogenomic associations have demonstrated clinical relevance, particularly when genetic variants affect drug metabolism or the risk of severe adverse reactions (5).

The development of evidence-based guidelines has supported the integration of pharmacogenomic information into prescribing decisions. However, widespread implementation remains limited by barriers including access to genetic testing, interpretation complexity, clinician education, reimbursement challenges, and integration into clinical workflows.

The future of pharmacogenomics will likely depend on combining genomic information with other biological and clinical datasets. Since drug response is rarely determined by a single genetic factor, integration of genomic, transcriptomic, proteomic, metabolomic, and clinical data may provide a more comprehensive understanding of therapeutic variability.

Human-Relevant Experimental Models: Improving Preclinical Prediction

Enhancing the predictive accuracy of preclinical research remains a major priority in translational pharmacology. Although traditional experimental models have contributed significantly to biomedical progress, their limitations in representing human disease biology are increasingly recognized.

Human-relevant models, including organoids, organ-on-chip systems, and patient-derived cellular models, provide new opportunities to investigate disease mechanisms and therapeutic responses in more clinically relevant environments (3, 4).

These approaches may be particularly valuable for complex diseases in which conventional models fail to capture essential aspects of human pathology. Patient-derived systems may also support individualized therapeutic evaluation by allowing assessment of treatment responses based on specific patient characteristics.

However, these technologies should complement rather than replace established pharmacological methods. Their successful adoption requires improvements in standardization, reproducibility, scalability, cost-effectiveness, and regulatory acceptance.

The ultimate value of these models will depend on demonstrating that they improve prediction of clinical outcomes and contribute to more efficient therapeutic development.

Artificial Intelligence and Data-Driven Pharmacology

Artificial intelligence (AI) has emerged as a transformative technology in drug discovery and development. Machine learning approaches can analyze large-scale biological, chemical, and clinical datasets to identify patterns that may not be recognized through conventional methods.

AI has demonstrated potential in target identification, molecular design, virtual screening, prediction of pharmacological properties, and optimization of development strategies (6, 7). These applications may accelerate discovery and improve efficiency in identifying promising therapeutic candidates.

However, AI should be integrated with scientific caution. Artificial intelligence cannot replace biological understanding, experimental validation, or clinical expertise. The reliability of AI predictions depends on the quality, diversity, and representativeness of available data.

A major challenge is model interpretability. For clinical applications, researchers and clinicians need confidence not only in predictive performance but also in understanding the biological basis of predictions.

The future role of AI in pharmacology will likely be as a decision-support technology that enhances human expertise rather than replacing scientific judgment.

Model-Informed Drug Development: Transforming Data into Decisions

The increasing complexity of modern therapeutics requires more advanced approaches for decision-making throughout drug development. Model-informed drug development (MIDD) integrates pharmacokinetic, pharmacodynamic, disease progression, and clinical trial data to support rational development decisions (8, 9).

MIDD approaches can improve dose selection, optimize trial design, predict therapeutic responses, and reduce uncertainty during development. These advantages are particularly valuable for biologics, advanced therapies, and precision medicines.

By incorporating quantitative modeling into development programs, MIDD enables a transition from descriptive analysis toward predictive decision-making. This represents an important conceptual advancement in pharmacology.

Successful implementation requires multidisciplinary collaboration among pharmacologists, clinicians, statisticians, computational scientists, and regulatory authorities, together with reliable datasets and validated models.

Real-World Evidence: Completing the Translational Pathway

Randomized controlled trials remain the foundation of evidence-based medicine; however, they represent only one stage of a therapy's lifecycle. Trial populations are often highly selected and may not fully reflect the diversity of patients treated in routine clinical practice.

Real-world evidence (RWE), generated from electronic health records, registries, healthcare databases, and digital health platforms, provides complementary information regarding effectiveness, safety, treatment patterns, and long-term outcomes (10, 11).

RWE is particularly valuable for rare diseases, advanced therapies, and populations that are difficult to study through conventional randomized trials. It can also support post-marketing surveillance and evaluation of treatment performance over time.

Nevertheless, real-world data require rigorous interpretation. Confounding factors, treatment selection bias, incomplete data, and differences in healthcare practices may influence conclusions. Therefore, robust analytical methods and transparent reporting standards are essential.

The future of translational pharmacology will depend on integration of multiple evidence sources. Clinical trials, mechanistic studies, computational models, and real-world data should function as

complementary components of a modern therapeutic development ecosystem.

Regulatory Science and Clinical Implementation: Creating Pathways for Successful Translation

Scientific innovation achieves its greatest value only when it results in therapies that are safe, effective, accessible, and clinically meaningful. Successful translation requires not only scientific discovery but also appropriate regulatory frameworks, healthcare implementation strategies, and continuous evaluation of outcomes.

Traditional regulatory pathways were primarily developed for conventional small-molecule drugs. However, the emergence of biologics, gene therapies, cell-based therapies, RNA-based medicines, and personalized treatments has challenged conventional approaches.

Advanced therapies differ fundamentally from traditional medicines because they may involve complex biological mechanisms, individualized manufacturing, and long-lasting effects. Therefore, regulatory science must evolve from a process focused only on approval toward a broader framework supporting innovation while maintaining scientific rigor.

Modern regulatory approaches increasingly emphasize adaptive evaluation, lifecycle monitoring, integration of diverse evidence sources, and continuous reassessment of benefit–risk relationships.

A therapy should not be viewed as a static product evaluated only at approval. Instead, it should be considered an evolving intervention whose clinical value becomes clearer through continuous evidence generation.

Redesigning Clinical Trials for the Era of Precision Medicine

Clinical trials remain the cornerstone of therapeutic evaluation; however, traditional trial designs were developed primarily to determine average treatment effects in relatively homogeneous populations. This approach is increasingly challenged by the biological diversity of diseases and variability in individual treatment responses.

Precision medicine has encouraged the development of innovative clinical trial designs, including adaptive trials, basket trials, umbrella trials, and platform trials. These approaches allow more efficient evaluation of targeted therapies, facilitate modification of trial strategies based on emerging evidence, and improve identification of patients most likely to benefit.

This transformation is particularly evident in oncology, where molecular characteristics increasingly guide therapeutic selection. Patients with different clinical diagnoses may share similar biological vulnerabilities, whereas patients with the same diagnosis may respond differently because of molecular differences.

Nevertheless, innovation in clinical trial methodology must be accompanied by scientific rigor. Accelerated development should not compromise evidence quality. Appropriate statistical methods, predefined adaptation criteria, transparent reporting, and close collaboration with regulatory authorities remain essential.

Future clinical trials will become increasingly dynamic, data-driven, and patient-centered. Their success will depend not only on demonstrating

efficacy but also on evaluating outcomes that reflect meaningful improvements in patients' lives.

Advanced Therapies and the Need for New Regulatory Thinking

The emergence of advanced therapies represents one of the most important developments in modern pharmacology. Gene therapies, cell-based therapies, and other biological interventions have created opportunities to treat diseases previously considered difficult or impossible to manage.

However, these therapies introduce unique scientific and regulatory challenges. Unlike conventional drugs with relatively predictable pharmacological profiles, advanced therapies may produce prolonged or permanent biological effects.

Gene therapy illustrates this complexity. Although a single administration may provide durable benefit, important questions remain regarding long-term safety, immune responses, durability of therapeutic effects, and delayed adverse events (12, 13).

Similarly, cell-based therapies require careful evaluation of product characteristics, manufacturing consistency, biological activity, and patient-specific factors. These products represent complex biological systems rather than simple chemical entities.

Future regulatory approaches must therefore evaluate the complete therapeutic pathway, including manufacturing processes, patient selection criteria, delivery systems, biomarkers, and long-term follow-up strategies.

The Expanding Role of Real-World Evidence in Regulatory Decision-Making

Real-world evidence (RWE) has become an increasingly important component of modern regulatory science. While randomized clinical trials provide high-quality evidence under controlled conditions, they may not fully represent treatment performance in routine clinical practice.

Real-world data from electronic health records, registries, healthcare databases, and digital health platforms can provide valuable information regarding long-term effectiveness, safety signals, adherence patterns, and outcomes in diverse patient populations (10, 11).

This approach is particularly important for rare diseases and advanced therapies, where large randomized trials may be challenging. RWE can also support post-approval monitoring and evaluation of additional therapeutic applications.

However, observational data require careful interpretation. Confounding factors, treatment selection bias, missing information, and differences in clinical practice may affect conclusions. Therefore, advanced analytical methods and transparent reporting standards are essential.

The future regulatory environment will depend on an integrated evidence ecosystem in which clinical trials, biological models, computational predictions, and real-world observations collectively inform therapeutic decisions.

From Regulatory Approval to Meaningful Clinical Impact

Regulatory approval represents a major milestone in drug development but should not be considered the final measure of therapeutic success. A medicine may demonstrate efficacy in clinical trials yet have limited real-world impact because of cost, accessibility barriers, complex administration requirements, or challenges in healthcare implementation.

The ultimate objective of translational pharmacology should extend beyond approval toward meaningful improvement in patient outcomes. This requires collaboration among researchers, clinicians, regulatory authorities, healthcare systems, industry partners, and patients.

Future therapeutic evaluation should increasingly incorporate quality-of-life measures, patient-reported outcomes, and long-term effectiveness data. The success of a therapy should be assessed not only by statistical significance but also by its ability to improve survival, function, wellbeing, and daily life.

Regulatory science should not be viewed as a barrier to innovation. Instead, it should provide the framework that enables innovation to reach patients responsibly, efficiently, and safely.

Future Perspectives: Redefining the Next Generation of Translational Pharmacology

The coming decade may represent a defining period for pharmacology. The field is moving from an era focused primarily on discovery toward one centered on prediction, integration, and personalization.

Future therapeutic development will depend on the ability to anticipate treatment response before exposure, identify appropriate patients, minimize unnecessary toxicity, and ensure that innovation generates measurable clinical value.

From Precision Medicine Toward Predictive Therapeutics

Precision medicine has already transformed several therapeutic areas, particularly oncology and rare diseases. However, the next stage requires moving beyond molecular classification toward comprehensive prediction of therapeutic response.

Integration of genomics, transcriptomics, proteomics, metabolomics, imaging data, clinical characteristics, and environmental factors may provide a more complete understanding of disease biology.

Nevertheless, generating large datasets alone will not guarantee better medicine. The essential challenge is converting complex biological information into practical tools that clinicians can apply in real-world decision-making.

Future predictive therapeutics will require models that are accurate, interpretable, clinically validated, and accessible.

Patient-Centered Innovation as the Ultimate Goal

The ultimate purpose of translational pharmacology is not simply the development of new therapeutic products but the improvement of human health. Traditional measures of success, including regulatory approval and statistical efficacy, do not always capture the full value of a therapy from the patient perspective.

Patient-reported outcomes, digital health technologies, wearable devices, and remote monitoring platforms provide new opportunities to evaluate treatment benefits more comprehensively. These approaches can capture clinically meaningful outcomes such as symptom improvement, functional recovery, treatment burden, adherence, and quality of life.

Patient involvement in therapeutic development is also increasingly important. Incorporating patient perspectives can improve the relevance of research objectives, acceptability of risk–benefit assessment, and practical implementation strategies.

A patient-centered approach requires redefining therapeutic success. A statistically significant

improvement does not always represent a meaningful improvement in daily life. Future pharmacology should focus increasingly on outcomes that matter to patients, caregivers, and healthcare systems.

Ensuring Equitable Access to Future Therapeutics

Scientific innovation must be accompanied by strategies ensuring equitable access to new therapies. Advanced technologies, including precision medicines, genomic testing, biological therapies, and artificial intelligence-based healthcare solutions, may increase healthcare disparities if access remains limited.

Addressing this challenge requires early consideration of affordability, accessibility, healthcare infrastructure, and implementation strategies. These issues should be integrated into therapeutic development rather than addressed only after regulatory approval.

Diversity in clinical research is also essential. Clinical trials should include populations reflecting the patients who will ultimately receive these treatments because genetic background, environmental factors, and healthcare access may influence therapeutic response and safety.

The future of translational pharmacology must therefore combine scientific excellence with a commitment to global health equity.

The Evolving Identity of the Translational Pharmacologist

As pharmacology becomes increasingly interdisciplinary, the role of the translational pharmacologist is also evolving. Future experts will require knowledge extending beyond classical pharmacology to include molecular medicine, computational biology, clinical trial methodology, regulatory science, data interpretation, and healthcare implementation.

The translational pharmacologist will increasingly serve as an integrator who connects discoveries from multiple scientific domains and guides them toward clinical application.

Educational programs should adapt by promoting interdisciplinary training, quantitative skills, data literacy, and collaboration among laboratory scientists, clinicians, computational experts, engineers, and regulatory specialists.

Successful translation requires not only discovery but also communication, collaboration, and the ability to transform complex scientific information into clinically meaningful decisions.

Conclusion

Translational pharmacology has become a central framework for transforming biomedical discoveries into meaningful therapeutic advances. The field has evolved from a simple connection between laboratory research and clinical medicine into an integrated discipline combining molecular science, precision medicine, computational approaches, regulatory innovation, and patient-centered care.

Despite substantial progress, major challenges remain, including biological complexity, limitations of current experimental models, variability in treatment response, regulatory uncertainty, and unequal access to innovative therapies.

Emerging strategies such as biomarker-guided development, pharmacogenomics, human-relevant models, artificial intelligence, model-informed drug development, and real-world evidence provide unprecedented opportunities to improve the

predictability, efficiency, and clinical relevance of therapeutic development.

The future of pharmacology will not be determined solely by the number of scientific discoveries generated, but by the ability to ensure that these discoveries reach the right patients, at the right time, with measurable clinical benefit.

Translation should no longer be considered the final step of drug development; rather, it should be integrated throughout the entire process of therapeutic innovation. The ultimate goal is to transform scientific progress into accessible, meaningful, and sustainable improvements in human health.

Translational pharmacology provides the scientific foundation and collaborative framework required to achieve this objective.

Declarations

Conflict of Interest

The author declares no conflict of interest.

Funding

No external funding was received for the preparation of this editorial.

Ethical Approval: Not applicable. This editorial is based on previously published literature and does not involve new experimental studies, human participants, or animal subjects.

Use of Artificial Intelligence

The author declares that no generative AI tools were used in the writing of this manuscript.

References

1.Wong CH, Siah KW, Lo AW. Estimation of clinical trial success rates and related parameters. Biostatistics. 2019;20(2):273-86.
2.Seyhan AA. Lost in translation: the valley of death across preclinical and clinical divide – identification of problems and overcoming obstacles. Translational Medicine Communications. 2019;4(1):18.
3.Low LA, Mummery C, Berridge BR, Austin CP, Tagle DA. Organs-on-chips: into the next decade. Nat Rev Drug Discov. 2021;20(5):345-61.
4.Ingber DE. Human organs-on-chips for disease modelling, drug development and personalized medicine. Nat Rev Genet. 2022;23(8):467-91.
5.Roden DM, McLeod HL, Relling MV, Williams MS, Mensah GA, Peterson JF, et al. Pharmacogenomics. Lancet. 2019;394(10197):521-32.
6.Vamathevan J, Clark D, Czodrowski P, Dunham I, Ferran E, Lee G, et al. Applications of machine learning in drug discovery and development. Nat Rev Drug Discov. 2019;18(6):463-77.
7.Paul D, Sanap G, Shenoy S, Kalyane D, Kalia K, Tekade RK. Artificial intelligence in drug discovery and development. Drug Discov Today. 2021;26(1):80-93.
8.Marshall SF, Burghaus R, Cosson V, Cheung SY, Chenel M, DellaPasqua O, et al. Good Practices in Model-Informed Drug Discovery and Development: Practice, Application, and Documentation. CPT Pharmacometrics Syst Pharmacol. 2016;5(3):93-122.
9.Zhao P, Zhang L, Grillo JA, Liu Q, Bullock JM, Moon YJ, et al. Applications of physiologically based pharmacokinetic (PBPK) modeling and simulation during regulatory review. Clin Pharmacol Ther. 2011;89(2):259-67.
10.Franklin JM, Schneeweiss S. When and How Can Real World Data Analyses Substitute for Randomized Controlled Trials? Clin Pharmacol Ther. 2017;102(6):924-33.
11.Corrigan-Curay J, Sacks L, Woodcock J. Real-World Evidence and Real-World Data for Evaluating Drug Safety and Effectiveness. Jama. 2018;320(9):867-8.
12.High KA, Roncarolo MG. Gene Therapy. N Engl J Med. 2019;381(5):455-64.
13.Bulaklak K, Gersbach CA. The once and future gene therapy. Nature Communications. 2020;11(1):5820.