Harnessing artificial intelligence to advance clinical pharmacology and DMPK in drug development

Authors: Danielle Thomas, MS, PhD[1], Amy Nielsen, PhD[1],Thomas Vargues[2], PhD and Olivier Barberan, PhD[2]

Affiliations: 1. Elsevier Inc, 525 B St Suite 1800 San Diego, CA 92101. 2 Elsevier BV, Radarweg 29 1043 NX Amsterdam, The Netherlands

Introduction

Drug development relies on the ability to efficiently translate complex regulatory, preclinical, and clinical data into actionable insights. Critical insights into drug metabolism, transporter interactions, and toxicological outcomes are often embedded within FDA and EMA approval documents, presents a major challenge to researchers.

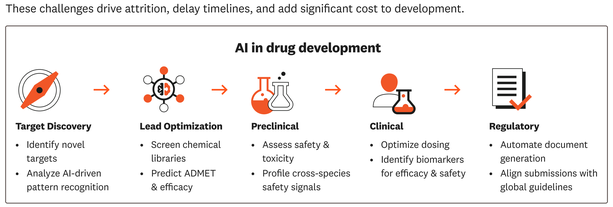
PharmaPendium, Elsevier's regulatory and scientific intelligence solution, integrates these data sources to enable translational research in pharmacokinetics, drug–drug interactions, and mechanistic toxicology. Recent advances in artificial intelligence, particularly natural language processing and machine learning, provide new opportunities to interrogate unstructured biomedical content at scale.

By applying AI to PharmaPendium, it is now possible to uncover mechanistic associations, identify emergent safety signals, and accelerate hypothesis generation for xenobiotic metabolism and disposition. This poster introduces the integration of AI-enhanced approaches within PharmaPendium and demonstrates their potential to improve predictive safety assessments, support regulatory decision-making, and advance drug discovery and development.

Ai in drug development: Opportunities and pitfalls across the pipeline

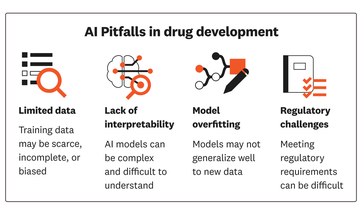
AI is increasingly applied to overcome long-standing bottlenecks in drug development. **DMPK scientists** face difficulties translating preclinical findings into reliable human predictions, while **clinical pharmacologists** must determine safe first-in-human doses and evaluate complex drug–drug interactions. **Toxicologists** work to identify subtle safety signals early, and regulatory scientists are burdened by the vast volume of FDA and EMA documents needed for precedent and compliance.

These challenges drive attrition, delay timelines, and add significant cost to development.

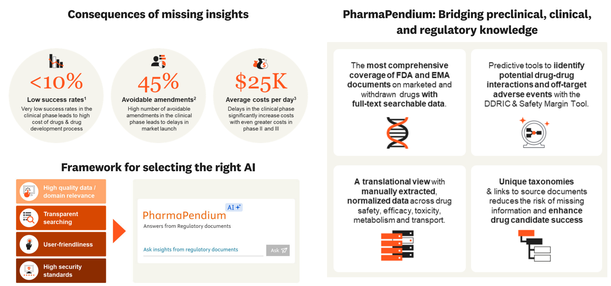


AI supports drug development by automating data mining, extracting insights, and enabling predictive modeling. NLP speeds precedent retrieval, machine learning aids clearance scaling and DDI prediction, and deep learning strengthens PK/PD modeling.

Still, AI depends on data quality, risks bias and can lack transparency. With proper oversight, it improves accuracy, accelerates decisions, and frees experts to focus on interpretation.



From regulatory data to actionable intelligence: PharmaPendium AI workflow



Accelerate discovery with the power of PharmaPendium AI

Background

Tenosynovial Giant Cell Tumor (TGCT) is a rare disease where abnormal tissue growth affects joints, often making surgery difficult. Currently, **Pexidartinib** is the only approved drug for patients who cannot undergo surgery, but it comes with safety concerns—particularly risks to the liver. A hypothetical Our new therapy candidate, also targeting TGCT, shows promise as a **safer and more effective alternative**.

Current challenges with Pexidartinib

- **Liver risk:** Can cause serious liver injury.
- **Strict safety rules:** Patients and doctors must follow a special FDA program requiring ongoing liver monitoring.
- **Patient burden:** Safety protocols and monitoring can make treatment difficult to manage.

Opportunities with a new therapy

- **Safer dosing:** Start with lower or optimized doses to reduce liver risk.
- **Better monitoring:** Use new tools (like imaging or biomarkers) to catch problems early.
- **Stronger results:** Explore combinations with other therapies or target specific patient groups for better outcomes.
- **Simplified care:** Make treatment and monitoring easier to encourage patient adherence and wider access with TKIs.

Regulatory considerations

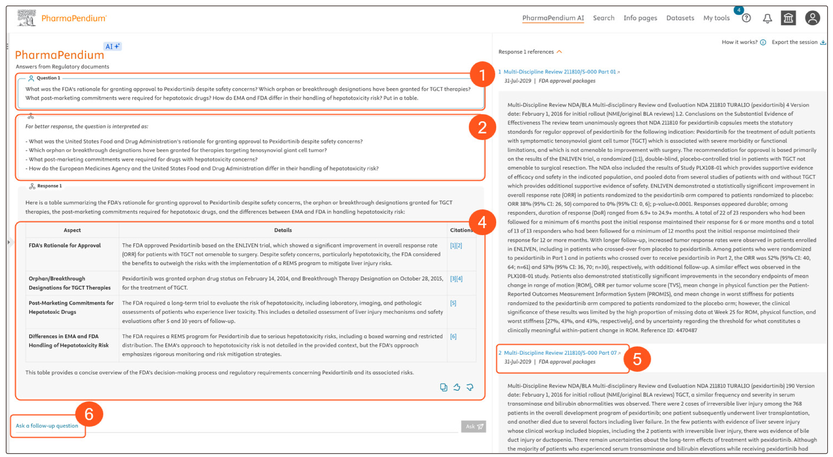
To bring this new therapy to market, success will depend on:

- Learning from **regulatory precedents** (how FDA and EMA approved and monitored Pexidartinib).
- Gaining **special designations** (like orphan or breakthrough status) to speed up approval.
- Designing robust **safety programs** that address liver concerns before approval and after launch.

How PharmaPendium AI helps

PharmaPendium AI supports drug developers by:

- **Discovery & preclinical:** Comparing new therapies with existing drugs to identify strengths and risks.
- **Safety evaluation:** Analyzing patterns of liver injury and helping design safer dosing and monitoring strategies.
- **Regulatory strategy:** Summarizing past FDA/EMA decisions to guide designations, approvals, and post-marketing requirements.
- **Competitive edge:** Benchmarking outcomes against Pexidartinib and identifying opportunities to improve patient experience.



Intuitive experience

1. Ask natural language questions in multiple languages including English, Japanese, Korean, French and Spanish.
2. Request responses in a variety of formats including table, free text, bullet points and CSV.
3. Easily search through previous responses within your history

Transparent searching

4. Review how a question was interpreted to gain clarity and improve responses.
5. Provides in-line references to allow users to dive into the original regulatory document and verify the response.
6. Ask follow-up questions refine your search and gather more regulatory insights.

Conclusion

- To compete with Pexidartinib, a new TGCT therapy must deliver **better safety, simplified patient care, and equal or stronger results**—backed by a smart regulatory strategy. **PharmaPendium AI acts as the intelligence engine** that helps anticipate challenges, optimize development, and accelerate market success.
- When coupled with high-quality regulatory data from PharmaPendium, AI becomes a powerful tool for clinical pharmacologists and DMPK scientists in supporting evidence-based, regulatory-compliant drug development. It facilitates proactive risk mitigation, improves the accuracy of early human dose projections, and aids in comprehensive DDI evaluations.

References

1. Sharma, P. (2023, December 13). Identifying druggable therapeutic targets: Unveiling promising avenues in drug discovery. Excelra. https://www.excelra.com/whitepaper/identifying-druggable-therapeutic-targets-unveiling-promising-avenues-in-drug-discovery/
2. Getz, K. A., Stergiopoulos, S., Short, M., Surgeon, L., Krauss, R., Pretorius, S., & Dunn, D. (2016). The impact of protocol amendments on clinical trial performance and cost. Therapeutic Innovation & Regulatory Science, 50(4), 436–441. https://doi.org/10.1177/2168479016632271
3. Smith, Z. P., DiMasi, J. A., & Getz, K. A. (2024). New estimates on the cost of a delay day in drug development. Therapeutic Innovation & Regulatory Science, 58(6), 855–862. https://doi.org/10.1007/s43441-024-00667-w
4. Tap, W. D., Wainberg, Z. A., Anthony, S. P., Ibrahim, P. N., Zhang, C., Healey, J. H., ... & Dubois, S. G. (2019). Structure-guided blockade of CSF1R kinase in tenosynovial giant-cell tumor. New England Journal of Medicine, 380(19), 1729–1740. https://doi.org/10.1056/NEJMoa1816738
5. PharmaPendium. (n.d.). Pexidartinib hydrochloride – FDA data. Retrieved September 13, 2025, from https://www.pharmapendium.com/browse/fda/Pexidartinib%20Hydrochloride

Copyright © 2026 RELX Intellectual Properties SA. All rights reserved.
PharmaPendium, RELX Group and the RE symbol are trademarks of RELX Intellectual Properties SA, used under license.