

NATIONAL
ACADEMIES

Sciences
Engineering
Medicine

Developing Regenerative Medicine Therapies with Artificial Intelligence: A Workshop

November 18, 2025

Convened by the Forum on Regenerative Medicine
In Collaboration with the Forum on Drug Discovery, Development, and
Translation

Anne Plant, Workshop Co-Chair
Nabiha Saklayen, Workshop Co-Chair



SESSION I: Opening Remarks & Keynote

- **Core Applications:** AI is transforming therapy development by automating the creation and review of regulatory and clinical trial documents, enhancing product quality, and streamlining manufacturing. It also helps identify true biomarkers in biology.
- **Building Trust:** Trust in AI models is foundational and requires the use of explainable AI and mandatory AI auditing.
- **Legal and Ethical Governance:** Legal and ethical issues necessitate checks and balances from experts, with regulators setting boundaries for non-explainable models and addressing liability for AI-driven mistakes.
- **Data Foundation and Regulation:** The FDA is fostering the use of AI, but the appropriateness and quality of data used for model training is key. This aligns with broader NIH initiatives focused on big data, open data, and harmonized metadata.
- **Human Role:** Human intervention remains an essential safeguard and opportunity in clinical decision-making to maintain oversight and manage uncertainty.

SESSION II: AI in the Pre-Clinical Development of Regenerative Medicine Therapies

- **Fundamental Biological Modeling:** AI/ML is used to model cellular networks and biological mechanisms by analyzing relationships between cell states and image-derived measurements.
- **AI for Cell Therapy (CAR-T):** Models are trained on experimental data to infer how protein structure dictates cell function, allowing for the identification of desirable and undesirable cell populations.
- **AI for AAV Therapy:** High-throughput assay data, combined with in vivo data, provides key structural information for developing physiologically effective protein-based therapeutics.
- **Regulatory Adoption:** The FDA has already approved over 500 products that incorporate the use of AI.

SESSION III: AI in Regenerative Medicine Clinical Trials and Manufacturing

- **AI for Clinical Trial Efficiency:** Large Language Models (LLMs) significantly speed up rare disease clinical trials by detecting protocol errors, drafting regulatory documents, and testing statistical implications of small data sets to design clinical trials that will provide useful data.
- **Machine Learning for Manufacturing Quality:** ML models and autonomous robotics are essential for quality control and scaling the manufacturing of personalized cell therapies like iPSCs and CAR-T.
- **CAR-T Scale and Failure Challenges:** The industry must address the need for scale and affordability in CAR-T, especially with high patient/batch failure rates and limited treatment locations. Can success for each patient be accurately predicted?
- **Data as a Critical Risk:** The complexity of living medicines and the pressing need for data quality and standardization are major risks.

SESSION IV: Laying the Data Groundwork for Regenerative Medicine AI Tools

- **Strategic Prioritization:** Focus AI strategy on pharma/regulatory partners' unmet needs to guide solution design and data requirements.
- **Complex Data Groundwork:** Passively collected data (e.g., from personal devices) requires cleaning and transformation to ensure quality and standardization for AI models.
- **Overcoming Data Sharing Hurdles:** Data accessibility is challenging (due to investment/relationships), requiring privacy protection methods like federated learning.
- **Building Trust and Governance:** Foster public trust through active patient participation, open data access, transparency, and ensuring all decisions include public input.

SESSION V: Building Trust in AI for Regenerative Medicine

- **Defining Trust:** Trustworthiness is difficult to define but one way could be through consensus-based AI product qualifications (like those from the AI Safety Institute) and by rigorously assuring both data quality and model quality in applications.
- **Trust as a Value Proposition:** Confidence in AI involves a practical risk assessment, viewing it as another technology tool whose use must be justified by a clear value proposition.
- **Transparency and Dialogue:** Establishing trust requires transparency across the entire AI/Regenerative Medicine supply chain, alongside vital education and open dialogue to anticipate risks and realize benefits.

SESSION VI: Final Reflections and Future Opportunities

- **Capital investment:** need to lower barriers to applications of AI in regenerative medicine. AI can reduce costs and increase the success of clinic trials and therapies.
- **Workforce:** hard to retain AI talent because of slow rate of development in this field. Encourage more computer scientists to pursue biology and medicine.
- **Advance AI techniques:** to achieve a better understanding and more trust.
- **Data generation:** More emphasis on datasets that are computer-ready (ex: AICS)
- **Digital twin:** simulations of biological systems should be pursued though more challenging than physics simulations.
- **Sharing data:** Could the FDA share data?

Final Takeaways For The Day

- **AI models are improving efficiencies** in documentation and regulatory compliance. Using AI models for predictions or decision-making is more complicated and still under development.
- **Predicting** a successful CART product from patients' cell characteristics can reduce the risk of unrecovered hospital costs, effectively improving therapy efficiency.
- **Data sharing** is critical, and most models require substantial data. Small clinical trials can use AI to help select a statistically meaningful clinical trial model.
- **Investment in AI** within the context of regenerative medicine has not been sufficiently robust.
- **Standards** are needed across many facets: data sharing (metadata, data formats, and protecting personal information); evaluating reliability and bias (AI regulatory and consensus ethical considerations. What serves as ground truth?

Next steps

- Please complete our post-workshop survey
- Slides and videos will be posted to the workshop webpage within a couple of weeks
- A proceedings-in-brief will be published in the coming months to capture the discussions here today
- Please leave your name badge holders on the registration table on your way out

Thank you for participating with us!

Post-Workshop Survey



NATIONAL
ACADEMIES

Sciences
Engineering
Medicine

Thank you for attending!

Developing Regenerative Medicine Therapies with Artificial Intelligence: A Workshop



November 18, 2025

Please scan the QR code to take our survey
and provide feedback on the workshop

