Bench to bedside: Accelerate cell and gene therapy adoption

Equipping pharma companies with the tools to expedite oncology and rare disease drug development and manufacturing for faster commercialization and time-to-market.

Global market potential

The advent of precision medicine, especially cell and gene therapy, is opening new frontiers where pharma companies can identify genetic variations and molecular signatures indicative of specific illnesses. At its core, the field leverages advancements in genetics and genomics to tailor interventions to a patient. Furthermore, cell and gene therapy helps predict cancer susceptibility, diagnose conditions very early, and build timely interventions that address the underlying molecular mechanisms that drive the cancer's progression. These therapies analyze a wealth of data to create personalized treatment plans that are effective, efficient, and targeted and have the potential to enrich patient experiences tremendously, improving the industry's sluggish pace of adopting this technological innovation on a wide scale.

The cell and gene therapy market was $4.72 billion in 2023. The market will reach $24.85 billion by 2032, growing at a CAGR of 20.4% between 2024–2032.

With such exponential growth projected, pharma companies must strategically invest in development and commercialization efforts within the cell and gene therapy space to position themselves as leaders in the evolving landscape of precision medicine. Integrating cutting-edge technologies into cell and gene therapy holds immense promise since it will help pharma players enhance their manufacturing processes and roll drugs into the market faster.

Opportunity for pharma players

CAR-T cell therapy (or chimeric antigen receptor T cell therapy) represents a dynamic market that is evolving with new research and smart clinical trials exploring its applications in various types of cancer. It has had a transformative impact on cancer immunotherapy and offers hope to patients with certain types of advanced cancer. The significant markets in the US and Europe represent a lucrative opportunity for crucial pharma and biotech players to shape the market landscape. Key considerations that these organizations must address include:

- **Ensuring product availability**
- **Improving reimbursement scenarios**
- **Finding production sites for CAR-T therapy**
- **Expanding the patient pool to include cohorts of relapsed cancer patients**

Challenges in implementing precision medicine for CAR-T cell therapy

The manufacturing process of CAR-T cells is very complex. The therapies pass through multiple hands, from production facilities to hospitals and pharmacies. Another area of concern is the treatment cost and reimbursements. Healthcare insurance companies don't have specific insurance packages covering these treatments. Let's examine five particular challenges in implementing precision medicine for CAR-T cell therapy.

Fig 1: Challenges of cell and gene therapy

How to approach cell and gene therapy with precision medicine

Cell and gene therapy orchestration entails a 5-step strategy that underscores a long-term joint vision.

Step I: Creating favorable dialog conditions.
Step II: Implementing precision medicine incrementally.
Step III: Addressing data interoperability and security.
Step IV: Reforming regulations and payment systems.
Step V: Redefining drug manufacturing processes with cell and gene therapy.

Step I: Creating favorable dialog conditions.

A joint long-term vision of precision medicine can emerge from a fruitful dialog among stakeholders, requiring policymakers to engage with scientists, patients and citizens, regulators, payers, and the medical and clinical sectors. The intent is to establish political will and societal buy-in based on trustworthy partnerships.

Engaging with the health sector

- Enable healthcare professionals to partner with other healthcare partners by helping individuals become active participants in their health.
- Enable medical professionals to become 'big data wranglers' to reflect the new process of collecting, analyzing, administrating, and working with large amounts of data.

Engaging with industry stakeholders and actors from the non-medical domain

- Define roadmaps and develop new business models that make it rewarding for healthcare partners to participate in precision medicine.
- Continue keeping the business interests of patients and society as aligned as possible.
- Introduce actors from outside the medical domains, like technology firms and big data and telecommunication companies, to participate and innovate with healthcare professionals.

Engaging with citizens

Engage and enable citizens in a two-way dialog to make informed decisions and set expectations about the benefits of precision medicine and the related challenges, both for them and society, regarding:

Ethical implications

- Discuss and define ethical frameworks with citizens.
- Create a legitimate space (boundaries) for precision medicine to develop.

Data collection and sharing

- Define methods to correlate biomedical data and their scientific implications to translate into medical practice.
- Link genomic and other '-omics' to health, lifestyle, and environment data (big data).
- Define frameworks to resolve any problems related to consent and data collection, access, use, and sharing, as well as the ethical and economic considerations around privacy and security.

Engaging with patients

Engaging patients will require communication between physicians and patients to substantiate and explain the following:

- The continuum between health and disease.
- To trigger behavioral change.
- Co-develop a new taxonomy of diseases.

Genomics and health literacy

- Ensure patients understand the implications of DNA testing, genomic analysis, and probabilistic predisposition.

Patient empowerment and responsibility

- Empower patients to make informed decisions and incentivize them through adaptive medical insurance schemes.
- Drive active participation in patient monitoring programs using sensor-based medical devices.

Engaging with regulators and insurers

Decisions by those who want to develop precision medicine must result from engaging with:

- Rule makers (legislators and regulators) to decide, design, implement, and enforce the rules like relevant health data and data interoperability.
- Payers to pay or reimburse diagnostics, prevention, and therapies based on comparative cost-benefits or cost-efficiency analyses.
- Insurers who face difficult conditions due to the liabilities of practitioners of precision medicine.

There are two aspects worthy of mention in the context of a roadmap:

Firstly, translate the complexities of genomics and other data-driven biomedical sciences into tangible clinical applications. Secondly, create a robust risk assessment matrix that addresses the quality, efficacy, and safety concerns surrounding collecting and sharing citizens' data within databases. The assessment includes implementing trustworthy sharing schemes to ensure transparency and accountability. By addressing these priorities, we can foster meaningful dialog and collaboration among stakeholders, ultimately advancing the integration of cutting-edge science into clinical practice while safeguarding data privacy and security.

Step II: Implementing precision medicine incrementally.

Horizon 1: Ensure comprehensive data collection, sharing, and analytics.

- Enable genomic data organization and shareability of large-scale biomedical data collection for an interoperable framework of data streams.
- Design and implement large-scale programs for DNA testing and medical and health records collection and sharing.
- Enable doctors, data wranglers, and scientists to collaborate and conduct research, identify patterns of medical conditions, and improve diagnostics and predictions.

Horizon 2: Develop precision diagnostics and treatment strategies for tailored healthcare interventions for individual patients. Maintain continuity of experimenting and communicating the benefits of precision medicine to:

- Showcase the benefits of precision medicine, like its cost-effectiveness and conventional approach.
- Account for broader aspects of conventional medicine and expenses like health and medical costs, overtreatments, untreated or uncontrolled side effects, or quality of life.

Horizon 3: Implement personalized prevention measures via biomedical profiling to proactively address health risks and promote well-being.

- Targeted prevention measures to maintain and develop health and wellness.
- Develop measures to fund genetic testing and patient counseling.

Horizon 4: Focus on tackling complex traits and exploring novel therapies based on emerging biomedical data. This phased approach ensures a systematic progression toward achieving precision medicine's full potential, enhancing patient care outcomes while driving innovation in healthcare.

Step III: Addressing data interoperability and security.

In developing a roadmap for CAR-T cell therapy, addressing critical data issues in the following ways is imperative:

- Implement robust data management practices prioritizing privacy and confidentiality and define clear protocols for data custodianship and access control to sensitive information.
- Establish a framework for secure data sharing among relevant stakeholders, fostering collaboration while safeguarding patient privacy.

• Standardize data formats to facilitate the development of biobanks and ensure interoperability, enabling seamless integration and data exchange across diverse healthcare systems.

By tackling these data-related challenges, stakeholders can pave the way for the effective implementation of CAR-T cell therapies while upholding the highest data security and integrity standards.

Step IV: Reforming regulations and payment systems.

In the realm of healthcare reforms, there exists a pressing need to overhaul regulations and payment systems to better align with the evolving landscape of medical treatments. This strategy entails a comprehensive redesign of methods and metrics used to assess the safety and efficacy of therapies, and the associated payment models. Key components include planned adaptive licensing, which enables flexibility in regulatory approval processes to accommodate emerging evidence and real-world data.

Performance-based metrics: Payments must be tied to the effectiveness of treatments in adaptive clinical practice by shifting toward performance outcome-based reimbursements. Moreover, embracing pay-for-diagnostics and data models incentivizes precision medicine investments by rewarding the value of accurate diagnostics and insightful data analysis.

Post-market surveillance: Robust surveillance mechanisms are essential to monitor the safety and performance of medical interventions post-approval continuously. Through these strategic reforms, stakeholders can foster a more dynamic and responsive healthcare ecosystem that prioritizes innovation, patient outcomes, and value-based care.

Diagnostics and data analytics regulations: Organizations must identify critical quality attributes to measure and demonstrate consistency, reproducibility, and safety clearly for each therapy, creating pathways for regulatory submissions. Real-time monitoring with data being collected and stored at each touchpoint would be essential to improve manufacturing efficiency and ensure predictable outcomes.

Step V: Redefining drug manufacturing processes with cell and gene therapy.

In the pursuit of redefining manufacturing processes, a strategic approach is essential to address the inherent challenges to implement the roadmap successfully. This strategy involves two key components. Firstly, defining the best practices of manufacturing processes to optimize efficiency, quality, and scalability. Identify and standardize the procedures that yield optimal results while adhering to top regulatory standards and industry norms. Secondly, it resolves challenges of custody and identification throughout the entire manufacturing supply chain. By implementing robust systems for tracking and tracing materials and products, stakeholders can mitigate risks associated with custody issues, ensure product integrity, and enhance transparency across the manufacturing processes. Organizations can navigate complexities, streamline operations, and drive innovation through these proactive measures.

Framework for managing CAR-T cell therapy

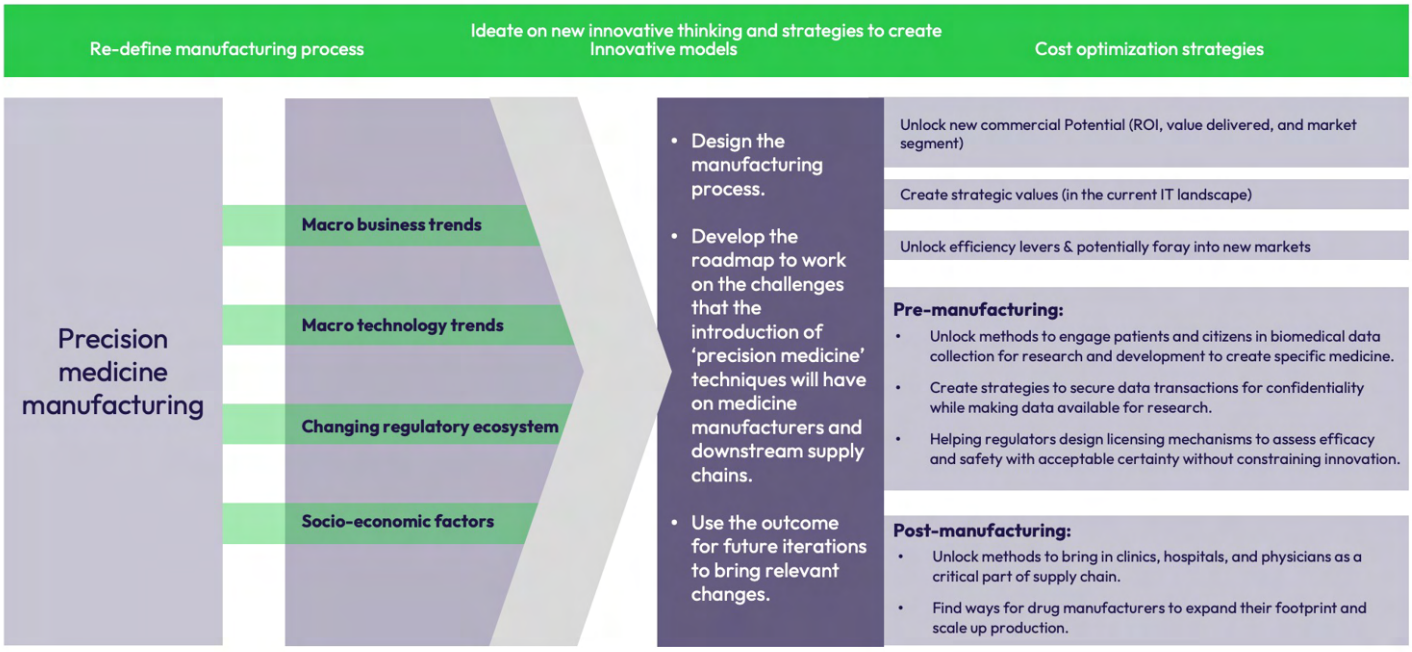

Fig 2: Rethinking the drug development process

What are the challenges?

The key stakeholders in the manufacturing of CAR-T cell therapy are transport logistics, patients, physicians, case managers, collection staff, and manufacturing managers. Stakeholders need to collaborate, but they can't since they use siloed systems and software, which leads to redundant workflows, data errors, problematic hand-offs, and compromised patient safety.

Framework highlights

Chain of identity

- Regulatory compliance and patient safety—maintain a chain of identity from collection to administration.
- Workflow-driven, consistent product handling reduces the risk of errors impacting product quality.
- Consistent, compliant labels to minimize the mistakes.
- Compliance with major country-specific security regulations governing the use of IT systems for medical applications.
- Role-based access control allows users to view only information relevant to them.
- Configure the journey to match the supply chain precisely for each personalized therapy.

Chain of custody

- Complete supply chain visibility includes tracking and controlling the chain of custody for rapid identification of issues and resolution.
- Prescriptive data capture, driven by workflows, ensures that the correct data is captured in the proper format.
- Real-time alerts ensure that vital communication is maintained across all stakeholders as products scale up and out.
- Integrations already established with third-party service providers and systems allow complete supply chain visibility and control.

Scheduling

- Streamline the supply chain by efficiently coordinating collection, logistics, manufacturing, and treatment administration on a single platform.
- When exceptions occur, manage schedules with ease and update relevant stakeholders.
- Apply rules and constraints to configure scheduling based on your product and supply chain requirements.
- Maximize your manufacturing capacity and throughput by tracking and managing each workflow step.

What outcomes can you expect by leveraging our framework?

We emphasize the importance of technology and systems in ensuring quality control, tracking, and coordination among all stakeholders involved in the therapy process.

Patient evaluation and selection:

- System to record and store test results and monitor treatment outcomes for each patient
- System to record CAR-T therapy, patient evaluation, and selection criteria

T-cell extraction and preparation:

- Quality management program to track key performance indicators
- System to record transportation and shipping along with the integrated details of all the stakeholders involved
- Document control systems
- Electronic tracking infrastructure
- Mobile application for labeling
- Record entry of patients and tracking of cells

Genetic engineering and expansion:

- System to enable coordination between the manufacturing center and the stakeholders
- Status tracking of the current stage of genetic engineering

Condition therapy and infusion:

- Record hospital pharmacy audit results and share them with stakeholders
- Complaint traceability, identification, and reporting
- Patient assessment and pre-infusion results capture and traceability

Post-treatment and recovery:

- System to record post-treatment regulatory reporting
- Manage patient discharge plan and day-to-day activities
- Manage follow-up appointments

Pioneering breakthroughs

How our cell and gene therapy strategies elevated pharmaceutical success

Success story 1:

Recipe authoring for a large pharma enterprise

What the client was looking for: The client's current manufacturing execution system (MES) strategy and solution did not address new company critical success factors of flexibility to deploy across its manufacturing technologies, simplicity to address process improvements, and reduced cost to implement new product introductions. The client wanted to modernize their MES, which included batch processing of their drugs.

What we did: We delivered a new-age application by bringing in the right mix of user experience with modern engineering processes to ensure they culminate in a great customer experience and better ROI, leveraging our deep expertise in platform transformation and agile execution.

What was the business impact?

3x reduction: End-to-end admin and recipe author flow, reduction in time to author recipes from 6–8 months to a few weeks.

Agility: Faster release cycles across sites due to importing and exporting recipes across environments.

Enhanced UX: Streamlined UX flow for easy navigation, allowing junior authors to use the system with less training.

Success story 2:

Delivering precision with a cell and gene therapy orchestration platform

What the client was looking for: The client wanted to improve productivity and accelerate time to market through the early detection of issues in the release life cycle for their flagship therapy orchestration platform.

What we did: We developed project test cases, documented defects, and improved application performance through early detection. We collaborated to create manual test cases and used Salesforce and Codebeamer to track and improve productivity and uptime.

What was the business impact?

20% reduction in the release cycle.

Enhanced productivity and uptime of client's application.

Reusability of all test cases.

About Brillio

Brillio is one of the fastest growing digital technology service providers and the partner of choice for many Fortune 1000 companies seeking to turn disruptions into competitive advantages through innovative digital adoption. We help clients harness the transformative potential of the four superpowers of technology: cloud computing, Internet of Things (IoT), artificial intelligence (AI) and mobility. Born digital in 2014, we apply our expertise in customer experience solutions, data analytics and AI, digital infrastructure and security, and platform and product engineering to help clients quickly innovate for growth, create digital products, build service platforms, and drive smarter, data-driven performance. With 17 locations across the US, the UK, Romania, Canada, Mexico, and India, our growing global workforce of nearly 6,000 Brillians blends the latest technology and design thinking with digital fluency to solve complex business problems and drive competitive differentiation for our clients. Brillio was certified by Great Place to Work in 2021, 2022 and 2023.

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