Deloitte.

2024 advanced therapy industry report

Contents

Executive summary

Deloitte conducted the second annual advanced therapy (AT) industry survey—on cell, gene, and other transformative therapies—to uncover what industry leaders believe are the headwinds and tailwinds that are poised to shape the industry. More than 350 respondents from across the value chain—advanced therapy developers (ATDs), advanced therapy manufacturing organizations (ATMOs), health care providers, and payers—were surveyed. The findings reveal a continued optimism among stakeholders and a shared sentiment on the most pressing challenges the industry is facing. These findings provide actionable insights to inform decision-making and strategic planning for all players in the AT space.

Survey highlights

Positive outlook: More than 80% of AT leaders surveyed felt optimistic about the performance of the industry in the past 12–18 months, and nearly 90% expressed a positive outlook for the next 12–18 months—both consistent with last year's outlook.

Patient experience: For respondents surveyed, 67% of ATDs highlight patient and caregiver education and onboarding as the most impactful factor for a positive patient experience.

ATD growth strategies: More than 30% of ATDs are actively considering partnerships for growth. Almost a quarter of the respondents show a high level of interest for RNA-based therapies, as opposed to only 1% for induced pluripotent stem cells (IPSCs). Radiopharmacy was an emerging area of interest with 8% of respondents.

Manufacturing challenges: Nearly 90% of ATDs have contracted with external manufacturing organizations—a 30% increase from last year.

Main barrier for commercialization: While "payer and reimbursement challenges" remains the top force affecting the commercialization of advanced therapies in the next 12–18 months, this year it tied for the top position with "improved manufacturing technology," underscoring a push toward consistency and lower cost of goods sold (COGS).

Critical factors for patient access: Whereas reimbursement challenges continue to be a major barrier for patient access, treatment center capacity and constraints was a surprise close second.

Factors for successful clinical trials: Optimizing trial design and maximizing patient recruitment and retention continue to be issues of concern for clinical organizations. Surprisingly, site selection decreased in concern compared to last year. This could be a result of having sufficient experienced treatment centers to meet demand, yet many organizations continue to struggle with meeting patient enrollment numbers in the clinical setting.

Artificial intelligence (AI): More than two-thirds of all ATDs and ATMOs have established an AI strategy. The trend is mostly driven by increased interest in Generative AI capabilities; however, the value of which remains nebulous.

Regulatory ambiguity: Manufacturing continues to be at the top of the list in terms of areas of regulatory ambiguity in the AT value chain.

Introduction

The second iteration of the survey was expanded to capture a broader range of insights—with a total of 376 respondents across key functions. The respondents include a diverse and influential group of C-suite executives, senior leaders, physicians, and other key stakeholders. The represented companies include advanced therapy developers (ATDs), advanced therapy manufacturing organizations (ATMOs), health care providers, and payers: 53% of respondents were ATDs, 7% were payers, and the rest were evenly split across health care providers, ATMOs, and others (academics, consultants, etc.). Of the ATD respondents, 22% had assets in pre-clinical, 44% in clinical, and 34% in the commercial realm. The roles of the respondents span functions, including research and development and clinical operations, manufacturing/supply chain and quality, commercial operations, market access/ patient services, digital/IT, and regulatory affairs. The following sections detail the sentiments, challenges, and opportunities across different aspects of the industry.

Reflections and future sentiments toward the AT industry

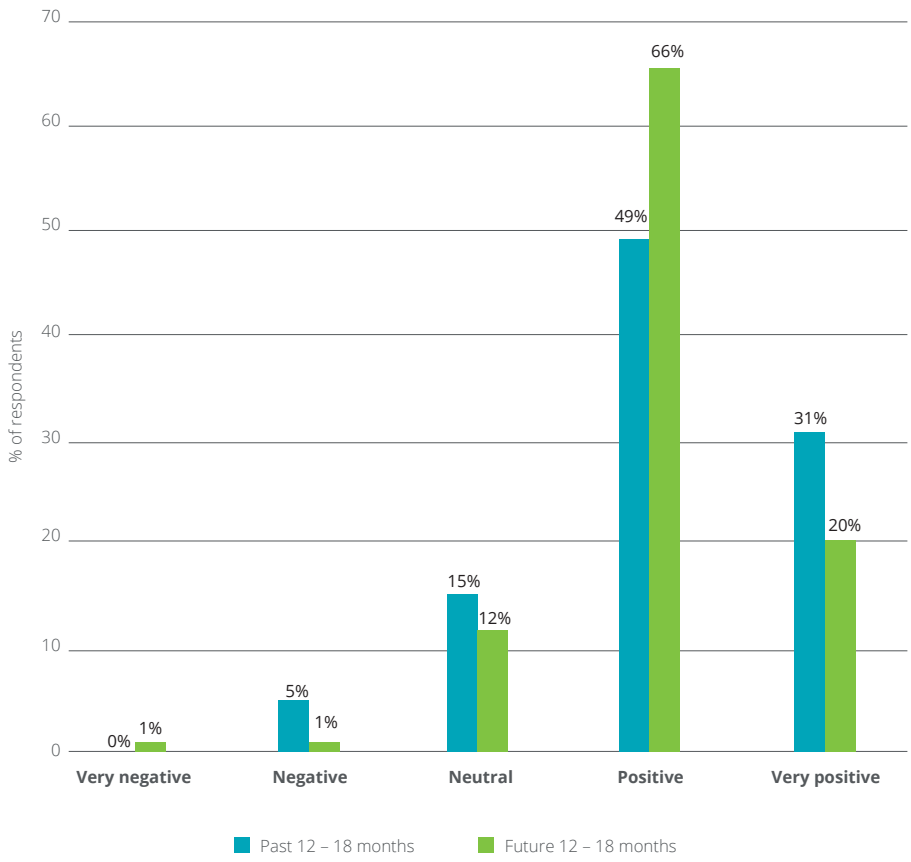
Looking ahead, nearly 90% of leaders have a positive or very positive outlook for the next 12–18 months, driven by ongoing innovation and a strong pipeline of therapies nearing commercialization. There was a 20% increase in those having a very positive outlook compared to last year's survey, indicating growing confidence in the industry's ability to overcome challenges. Positivity remains high among all stakeholder groups—health care providers (83%), ATDs (85%), payers (90%), and ATMOs (91%)—suggesting stakeholders anticipate continued progress and confidence in the evolving market. This positive sentiment may also be reflective of the messages the FDA continues to send as well as the increased capital flow over the past year in the space.

Respondents had a more favorable outlook for gene therapies and autologous cell therapies versus allogeneic cell therapies. Specifically, 82% and 77% of respondents, respectively, expressed a positive outlook. This could be because we have yet to see allogeneic therapies demonstrate efficacy.

As organizations continue to invest in their advanced therapy portfolios, the survey revealed interest in pursuing novel treatment modalities with one-fourth of ATDs looking into RNA-based therapies, reflecting the growing interest due to its versatility and recent successes, such as mRNA vaccines.

Figure 1:

Past and future sentiment regarding the development and commercialization of all advanced therapies



Research and development

In considering advanced therapy trials, respondents identified the top three factors for achieving successful trial outcomes to be:

1. Trial design.
2. Patient recruitment and retention.
3. Regulatory alignment on endpoint selection.

A majority (72%) of respondents cited trial design as the most important factor. This marks a shift from last year's results, in which regulatory alignment on endpoint selection was identified as the primary factor for successful trial outcomes.

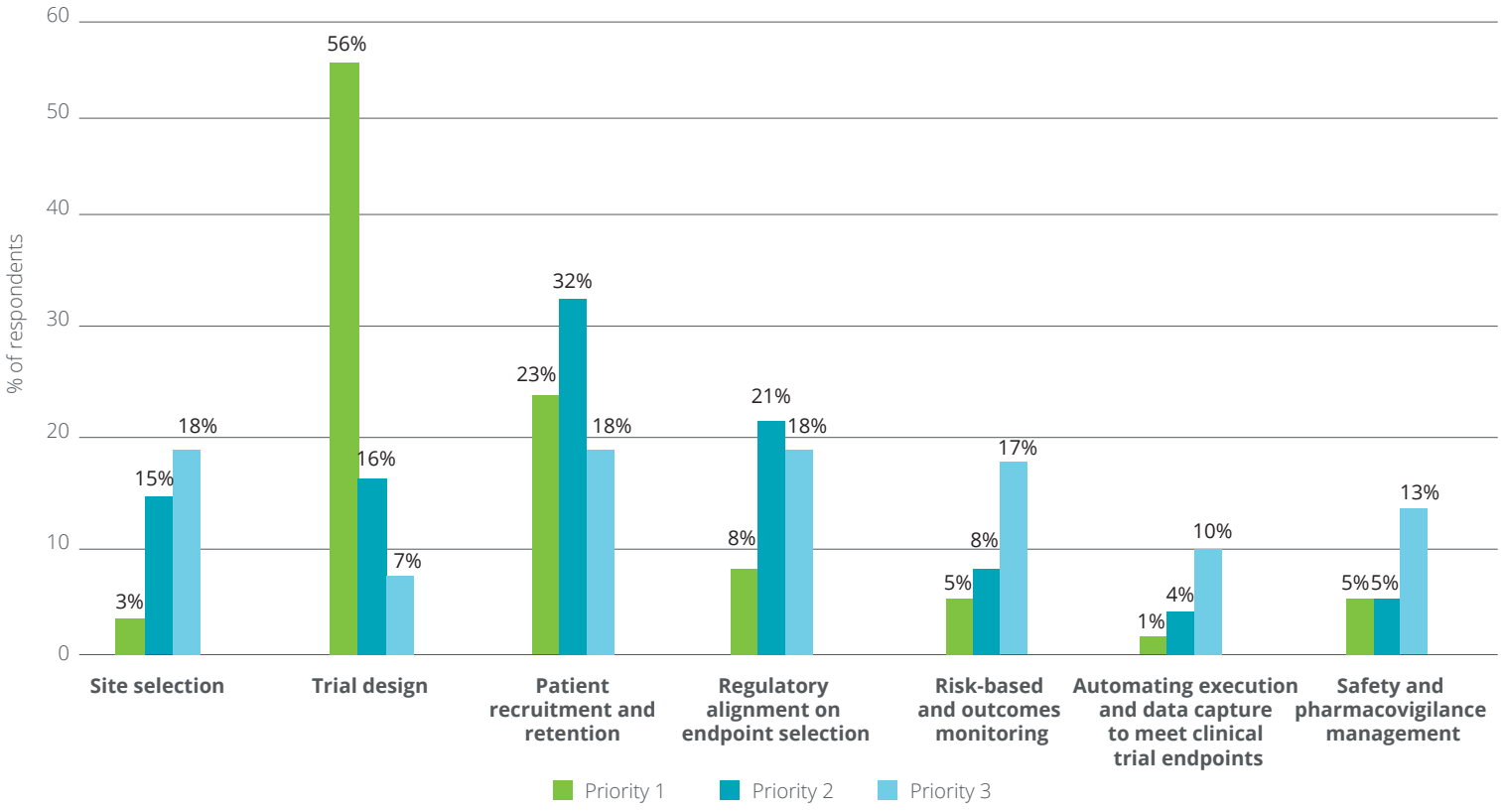
While last year's findings highlighted the critical role of alignment with regulatory authorities, this year's results highlight the importance of establishing a robust foundation through trial strategy and design. Such a foundation is essential for improving favorable outcomes in regulatory alignment.

ATDs identify the size of the eligible patient population as the primary challenge in clinical trials with respect to patient identification/recruitment. Conversely, providers point to the complexity of the patient journey as the most significant obstacle. This highlights the fact that ATDs primarily struggle with small, geographically dispersed pools of eligible patients, while health care providers face challenges in identifying patients at the right stage of disease progression to ensure maximum benefit of ATs.

The results show that robust decision-making with regard to trial design, patient engagement, and regulatory collaboration is key for the success of advanced therapy trials.

Figure 2:

What are the most critical success factors in ensuring successful trial outcomes?



Manufacturing

The results revealed that more than 40% of respondents do not have confidence in meeting demand in the next two to three years. This reflects a continued uncertainty stemming from challenges faced when scaling manufacturing. The top three challenges identified with regards to scaling manufacturing were:

1. Lack of talent.
2. Capital access.
3. Lack of standardized processes.

When asked about preferred manufacturing strategies that are being evaluated, a majority responded in favor of centralized manufacturing. However, there was a large cohort (40%) that was very optimistic about decentralized and distributed manufacturing. There was lackluster support among respondents for point-of-care (POC) manufacturing, mainly because of quality control and potential operational challenges per our anecdotal discussions.

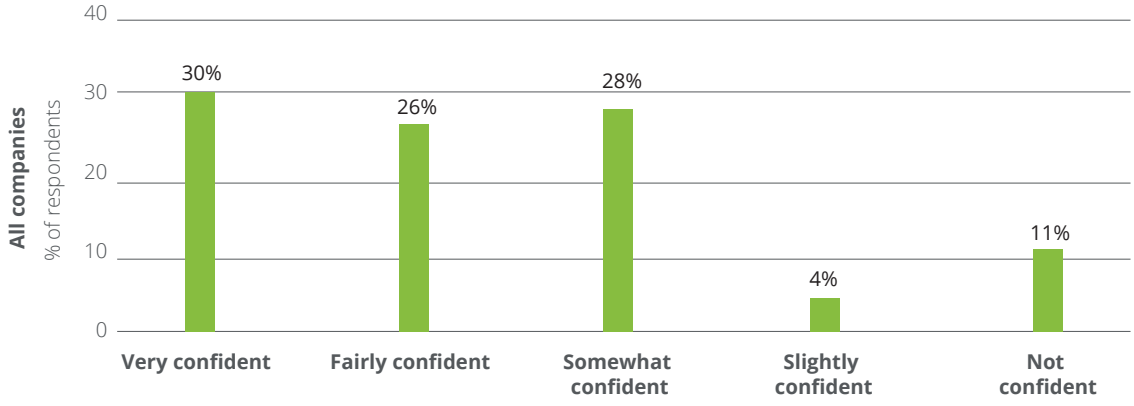
Nearly 90% of ATDs have contracted with external manufacturing organizations, a 30% increase from last year. This reflects a strategic shift away from investing in manufacturing capabilities to leveraging specialized expertise and infrastructure to accelerate time to market. The results revealed the top external manufacturer selection criteria to be:

1. Regulatory compliance.
2. Service and talent consistency.
3. Market reputation.

This highlights the importance of ensuring high-quality manufacturing standards, reliable service delivery, and having a trustworthy partner that aligns with stringent industry regulations.

Figure 3:

How confident are you that your manufacturing and production capacity/capabilities will meet the global demand for your products in the next 2-3 years?



Commercialization

Consistent with the findings from last year's survey, "payer and reimbursement challenges" was identified by all respondent groups as the top force affecting the commercialization of advanced therapies. The results revealed the other top two forces affecting commercialization to be: improved manufacturing technology and federal drug pricing controls.

Improved manufacturing technology is seen as a crucial enabler, particularly given the persistent challenge of out-of-spec manufacturing rates among even mature ATMOs. Advanced manufacturing technology will address quality issues and help reduce the COGS, potentially lowering prices and improving access. This is especially important in the emerging markets where pricing controls and restrictions abound.

Federal drug pricing controls introduce uncertainty into a market that already struggles with complex financing models. When considering financing options for high-cost therapies, more than 80% of respondents believe value-based outcomes and annuity models are the payment models with the most likelihood for adoption. These models have the potential to align incentives across stakeholders and can drive cost-effectiveness and patient-centric care.

Furthermore, more than 30% of ATD respondents said they are actively exploring partnerships for growth. This is mainly driven by smaller, innovative players leveraging large biopharma networks and capabilities to establish commercial success and bring their asset to market. Other players leveraging partnerships for growth are nontraditional players entering the cell and gene therapy space and investing in building an advanced therapy pipeline through acquisitions and partnerships.

When looking at patient access to these transformative therapies, the results revealed the top three barriers to patient access to be:

1. Reimbursement challenges.
2. Treatment capacity constraints.
3. Concerns over safety and efficacy.

More than 50% of respondents identified reimbursement issues as the first or second most significant barrier to patient access, highlighting the need for innovative payment models and collaboration with payers to ensure access to and affordability of these therapies. Additionally, reimbursement issues was the only barrier recognized by all stakeholders, further underscoring its significance on patient access to innovative therapies.

Patient and caregiver education and onboarding was highlighted by 67% of ATDs as the most impactful factor of a positive patient experience. Educating patients ensures they feel informed and supported, which helps with adherence to treatment protocols and achieving better outcomes. This support is especially important in this space given the burden on patients and caregivers of the longer therapy journey, such as apheresis, hospitalization, and long-term follow-up care.

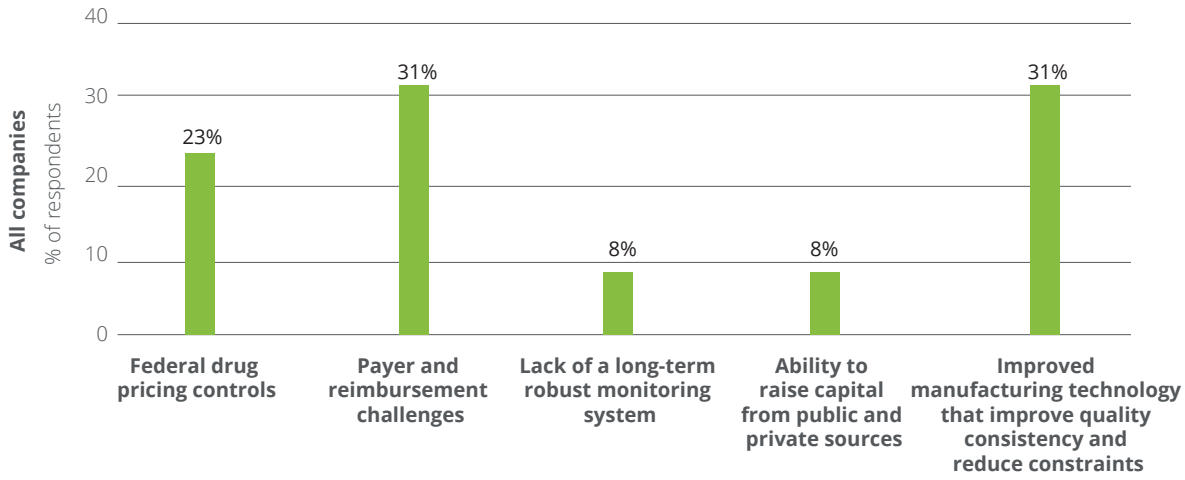
When considering the most important elements to site selection, the top three factors were:

1. Existing engagement between site and patients with the indication of study.
2. Site having existing accreditations (e.g., FACT, AABB, or other health authority).
3. Region/location of the site.

More than one-third of respondents prioritize sites having existing engagement with patients (for the indication they are pursuing) as a key element of the site's ability to understand the patient journey and to support patient recruitment and trial execution.

Figure 4:

Which of these forces do you believe will have the most significant impact on the commercialization of advanced therapies in the next 12-18 months?



Technology

The results revealed that more than two-thirds of all ATDs and ATMOs have established an AI strategy. Notably, a higher percentage of ATDs (approximately 75%) have defined an AI strategy compared to ATMOs (about 63%). This finding surpasses expectations for an industry characterized by numerous emerging startups and highlights the sector's proactive approach in harnessing AI's potential. The trend is mostly driven by increased interest in Generative AI capabilities; however, where the value may yet be proven, both growing and established organizations are treading carefully on the applicability of AI technologies.

Respondents said the top three goals their organizations are targeting with AI are:

1. Improving efficiency and productivity.
2. Reducing costs.
3. Encouraging innovation and growth.

While 80% of respondents identified efficiency and productivity improvements as their major goal, surprisingly only 10% of respondents were focused on uncovering data and insights with their AI investments.

Reaching a certain scale renders manual planning and execution unsustainable, necessitating better solutions to reduce the cost of goods manufactured. The results also revealed that 40% of ATDs and 65% of ATMOs have planned to stand up digital slot allocation and capacity planning capabilities.

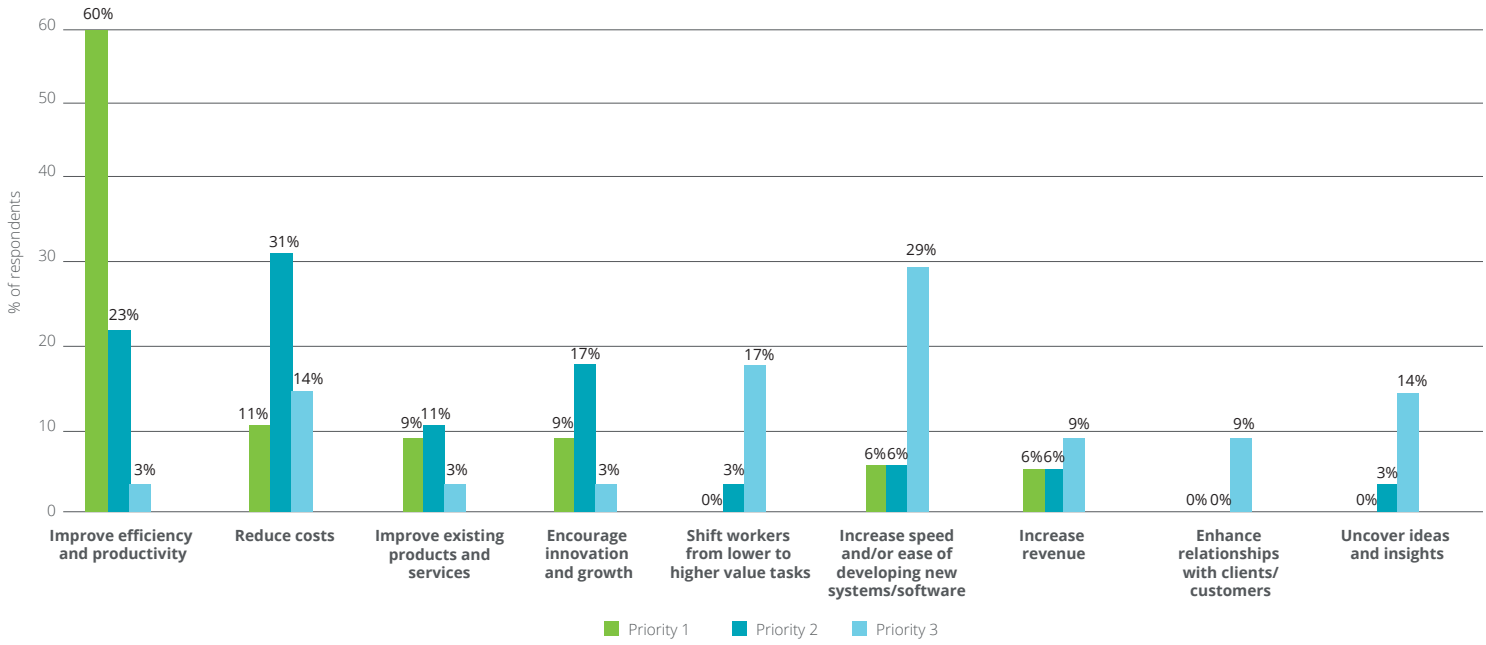
Respondents identified the top four processes they are looking to digitally enable in the next 12–18 months to be:

1. Chain of identity (COI) and chain of custody (COC) and labeling (80%).
2. Financial operation (60%).
3. Procurement and inventory operations (60%).
4. Materials planning and manufacturing capacity management (60%).

These findings highlight continued investment in foundational COI/COC and labeling capabilities, driven by regulatory guidelines.

Figure 5:

What are you hoping to achieve with this AI strategy?



Regulatory

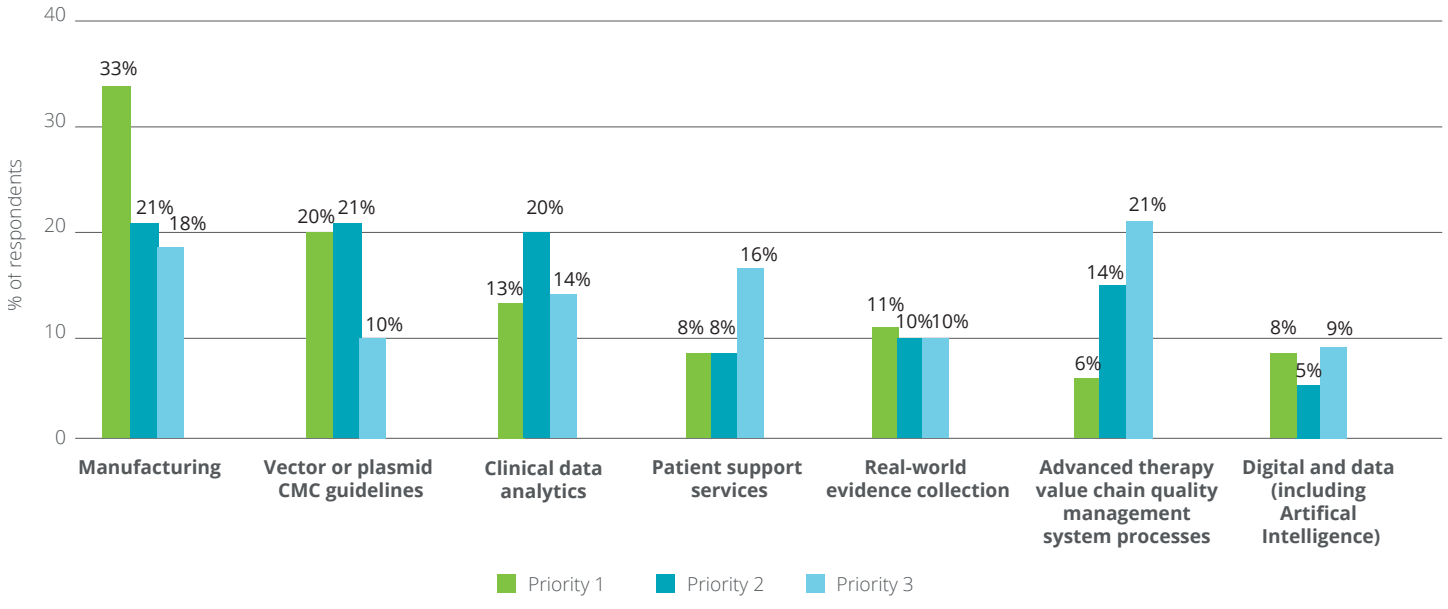
The results revealed that the top three areas of regulatory ambiguity in the AT value chain are:

1. Manufacturing.
2. Vector or plasmid chemistry, manufacturing, and control guidelines.
3. Clinical data analytics.

These areas present significant challenges in developmental timelines due to the rapid evolution of technology in ATs, which often outpaces established regulatory guidelines. Additionally, varying regulatory guidelines across geographies further complicate compliance. Regulatory agencies have demonstrated willingness in supporting developers to navigate the available pathways. At the 2023 Deloitte NextGen Therapies Industry Working Group (IWG) in Washington, D.C., Dr. Peter Marks from the FDA commented on the agency's efforts to harmonize regulatory requirements where possible by collaborating with global regulatory agencies, and to leverage accelerated approval pathways when applicable to accelerate access for patients. Close and frequent coordination with regulators can help innovators better understand expectations and develop a comprehensive regulatory strategy, facilitating faster development and commercialization of advanced therapies, as Dr. Marks shared was the case for Operation Warp Speed during the COVID-19 pandemic.

Figure 6:

In your opinion, which aspects of the advanced therapy value chain have the largest regulatory ambiguity?



Conclusion

Overall, responses highlight a sector poised for transformative growth, driven by optimism and strategic innovation. Stakeholders across the industry—ATDs, ATMOs, health care providers, and payers—share a positive outlook, fueled by scientific advancements.

While key challenges such as reimbursement hurdles, scalability of manufacturing operations, and regulatory ambiguities remain, the industry is increasingly adopting collaborative approaches and using digital technologies to enhance automation for operational efficiencies and innovation.

Embracing innovative payment models, enhancing patient education, and establishing robust clinical trial designs are essential strategies for the AT sector to overcome financial, adherence, and regulatory challenges, thereby securing the future success of the industry.

As the advanced therapy sector approaches a critical juncture in its growth, the industry's ability to navigate these challenges will define its trajectory and potential to revolutionize treatment paradigms.

Contacts

Hussain Mooraj
Principal, NextGen Therapies Practice lead
Deloitte Consulting
+1 508 561 9266
hmooraj@deloitte.com

Jessica Faust
Managing Director
Deloitte Consulting LLP
+1 617 813 4259
jfaust@deloitte.com

Amit Agarwal
Managing Director
Deloitte Consulting LLP
+1 415 783 6691
amitagarwal2@deloitte.com

Alex Haig
NextGen Therapies Consultant
Deloitte Consulting LLP
+1 646 203 1468
ahaigmontes@deloitte.com

Deloitte.

8450353