Insight Brief

Navigating Autoimmune Disease

A Playbook for Cell and Gene Therapies

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Introduction

Cell and gene therapies (CAGT) are redefining how we treat autoimmune diseases, offering new possibilities for conditions that have been notoriously difficult to manage. As the industry pivots its focus from oncology to autoimmune indications, strategies for clinical development and commercialization must adapt to navigate the complexities of this evolving field.

In this webinar, we examined the rapid advancements in CAGT for autoimmune disease states, highlighting the challenges unique to this shift from oncology development. Our discussion focused on the importance of enhancing site readiness, prioritizing patient-centric approaches and emphasizing early planning and data-driven decisions. This brief summarizes the key takeaways and providing insight into how the industry is adapting to such a transformative change.

The shift from oncology to autoimmune therapies

Historically, oncology has been the primary focus of CAGT, with breakthrough treatments such as CAR-T cell therapies demonstrating clinical success. Recently, the field has turned its attention to autoimmune diseases, with companies applying what they've learned from oncology to develop better treatments.

This shift is more than just a trend—it's reflected in the increasing number of clinical trials dedicated to autoimmune diseases. Once dominated by oncology, the pipeline is now seeing a surge in non-oncology studies, particularly in autoimmune research. This evolution speaks to the broader dynamics in the industry, as investors, sponsors and clinicians actively explore new opportunities in this space.

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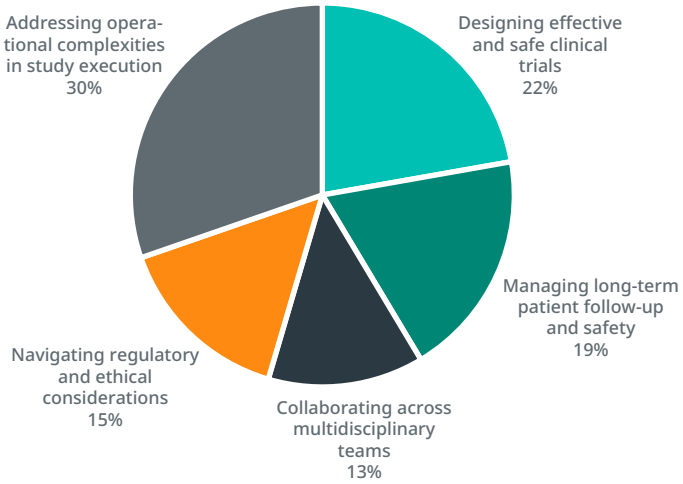
What do you see as the biggest challenge in developing cell and gene therapies for autoimmune diseases?

Poll results captured from webinar



Building a roadmap for success

With the growing emphasis on autoimmune diseases, navigating this evolving landscape requires a strategic approach. A well-defined playbook is essential for guiding sponsors through the complexities of clinical development, regulatory compliance and commercialization. To achieve success, the following key factors should be considered:

Regulatory strategy and compliance

Understanding the global regulatory landscape is crucial, as requirements for gene-modified therapies vary across regions. This includes obtaining Institutional Biosafety Committee (IBC) approvals and complying with Good Clinical Practice (GCP) standards. Proactive engaging with regulatory authorities can help avoid delays and streamline the approval processes.

Clinical development expertise

Running successful clinical trials requires a mix of medical, scientific, and operational expertise. Designing complex studies, managing adjudicated endpoints, and overseeing long-term follow-ups all require coordination. Given the variability of autoimmune diseases, trials often need to accommodate personalized treatment plans, adding another layer of complexity to execution.

Site readiness and multidisciplinary coordination

Preparing clinical sites is a key factor in trial success. Unlike oncology centers, which may already have experience with advanced therapies, autoimmune trials often involve collaboration between cell therapy specialists and disease-specific experts, such as rheumatologists or neurologists. Building strong partnerships with well-equipped centers and providing the necessary resources is essential.

Patient engagement and recruitment

Recruiting patients for autoimmune clinical trials calls for a patient-centered approach. Autoimmune patients often evaluate the risk-benefit profile of participating in trials differently than oncology patients, especially given the chronic nature of many of these diseases. Sponsors must develop patient engagement strategies that address these concerns, including flexible schedules, travel support, and clear communication about trial objectives and potential benefits.

Operational and logistical planning

Managing the logistics of CAGT, including autologous treatments, is complex. Cold chain management, chain of custody, and site training all need attention. In autoimmune trials, these logistics become even more complicated due to the specialized handling of materials and the need to maintain regulatory compliance throughout the supply chain. Establishing standardized protocols and providing site training can enhance efficiency, ensure compliance, and maintain product

Overcoming challenges in autoimmune therapy development

Developing therapies for autoimmune diseases requires a deep understanding of the disease biology. Unlike oncology, where treatments target malignant cells, autoimmune therapies focus on modulating the immune system—typically by targeting specific cells or pathways driving pathological immune responses.

For example, B-cell targeted therapies, which have been successful in treating certain autoimmune conditions, must carefully consider the role of long-lived plasma cells in disease persistence. Targeting these cells effectively can lead to more durable responses, but also requires sophisticated approaches to avoid unintended immunosuppression or adverse effects.

To successfully bring new autoimmune therapies to market, sponsors must address multiple challenges:

COMPLEX STUDY DESIGNS

Autoimmune clinical trials frequently involve complex designs with multiple endpoints and long-term follow-up requirements. Sponsors must balance the need to demonstrate safety and efficacy with the practical considerations of patient burden and study feasibility. Adaptive trial designs and leveraging real-world evidence can help optimize development and improve efficiency.

PATIENT AND SITE DYNAMICS

Sites conducting autoimmune trials must manage both the cell therapy components and the specific needs of autoimmune patients. This demands clear protocols, thorough site training, and an increased level of

coordination between specialty areas within healthcare facilities. Sponsors can support sites by providing resources such as clinical trial educators and home health services.

COMMERCIALIZATION PATHWAYS

As the market for autoimmune CAGT becomes more competitive, sponsors also need to think about commercialization strategies early on. This includes understanding market access requirements, preparing for potential competition, and engaging with payers to highlight the value and benefits of new therapies. Considering these factors early supports a smooth progression from development to market entry.

LOOKING AHEAD

As more therapies progress from clinical trials and into clinical practice, there's a growing opportunity to improve patient outcomes and quality of life. Companies like IQVIA Biotech are at the forefront of this progress, investing in research and operational capabilities, expanding global site networks, strengthening training programs, and developing innovative service solutions to support continued growth.

With the right playbook, the industry can navigate the complexities of autoimmune therapy development and deliver transformative treatments to patients with unmet needs. By combining scientific expertise, operational excellence, and patient-centric strategies, the CAGT sector is poised to make meaningful advancements in the management of autoimmune diseases.

Want to learn more? Watch the full webinar on-demand: Building a Playbook for Cell and Gene Therapies for Autoimmune Disease: Navigating a Rapidly Evolving Environment.

About IQVIA Biotech

IQVIA Biotech is a biotech-specialized CRO delivering flexible clinical development solutions for biotech and emerging biopharma companies. Our clinical solutions are built on 25 years of unmatched experience with therapeutically aligned expertise, uniquely designed to deliver full-service solutions on a global scale.

About the authors

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As Vice President of Immuno-Oncology and Cell & Gene Therapy at IQVIA Biotech, Erin leads the strategic efforts to ensure high-quality services and innovative solutions for biotech sponsors. With over 20 years of experience in global clinical research and drug development, Erin's therapeutic expertise guides, sponsors through the dynamic landscape of immuno-oncology and cell and gene therapies.

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Jessica Knight-Perry, MD, is a board-certified pediatric hematologist oncologist with additional expertise in hematopoietic stem cell transplantation and delivery of cell and gene therapies. In her current position as Senior Director, Medical Strategy Lead, Cell & Gene Therapy Center of Excellence at IQVIA, Jessica supports the development of cell and gene therapy assets for pharmaceutical and biotech companies.

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Emily Smith is a therapeutic strategy lead at IQVIA's Cell & Gene Therapy Center of Excellence and holds over 10 years of experience specializing in trial design and feasibility focusing on non-oncology cell and gene therapy studies. She co-leads the with cell therapy and autoimmune disease working group collaborating with experts to develop comprehensive strategies for complex therapies.

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