



## Advanced Therapeutics Progress Key Takeaways

- Advanced therapeutics (cell- and gene-based modalities) no longer exist on the outskirts, and the challenge going forward is less about technical or even clinical vs. commercial risk, namely, how to achieve cost-effectiveness sufficient for optimal pricing, market adoption, and patient access to these efficacious but costly and complex new therapies.
- Manufacturing is no longer a behind-the-scenes component of the value proposition for these novel modalities. Key decision-making considerations include that of whether to build out infrastructure vs. contracting, with whom to partner, what capabilities are regarded as most critical, and which processes to lock in early to obviate downstream regulatory concerns.
- The goal with autoimmune and oncology are complementary: potent and selective silencing vs. activation of the host immune response, respectively. That said, autoimmune is a decade behind oncology in terms of validation, and so-called secondary attributes (dosing/administration, tolerability, logistics) have become increasingly prominent keys to success across both categories.
- Rare/ultra-rare (e.g., monogenic) diseases serve as good arenas for validation of advanced therapeutics but require transformational results and repeat wins for commercial viability, necessitating the push toward broader, heterogenous conditions. Patient population, clinical endpoints, mechanistic rationale, and pricing strategy are keys to expanding beyond rare disease.
- As curative therapies, advanced therapeutics will be increasingly reimbursed via value-based contracting. Payers prefer having their own data but lack the resources or impetus to generate it. Real-world evidence (RWE) is emerging as a tool to enable drug companies to provide payers with data for outcomes that won't happen within a benefit year.

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