Scale-Up and Commercialization of Advanced Therapies

tensentric

ZERO DOUBT

Advanced Therapies have Disruptive Potential

Disruptive innovation happens when a new technology creates a previously nonexistent market (for example, curing previously uncurable diseases) or enters at the bottom of an existing market by providing a different set of values which ultimately overtakes the incumbent technology in it's original market.[1]

DISRUPTIVE INNOVATION

An innovation that **creates a new market** or enters at the bottom of an existing market **by providing a different set of values**, which ultimately (and unexpectedly) overtakes incumbents.

Advanced therapies (treatments that are based on genes, tissues, or cells, including cell & gene therapies or CGTs) have enormous disruptive potential due to their ability to harness and direct the power of molecular and cell biology to fight the underlying causes of disease at a mechanistic level. Rapid acceleration in research, development, and commercialization attempts in the advanced therapies market in the last 3-5 years has revealed that these unique treatments don't fit the mold or of the technologies that they are disrupting, such as traditional chemical, radiative, surgical, or even biologic therapies like monoclonal antibodies.

To truly disrupt the treatment paradigm for complex, systemic diseases such as cancer, those developing advanced therapies need to solve the entire clinical value chain of delivering the right treatment to the right patient at the right time for the right cost.

TRUE CLINICAL VALUE

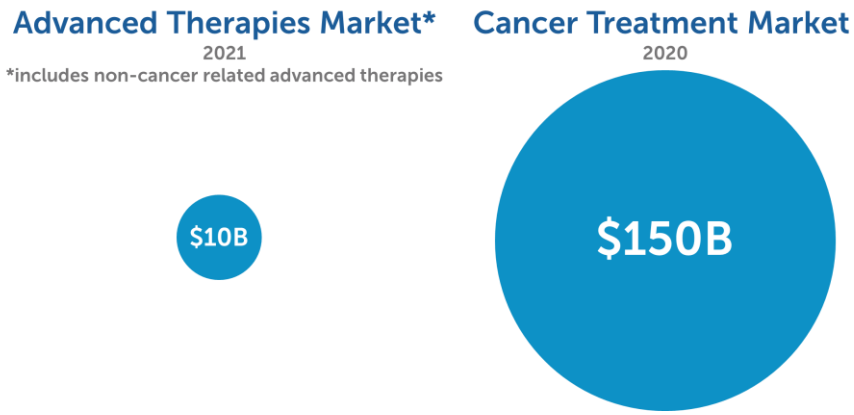
RIGHT TREATMENT **RIGHT PATIENT** **RIGHT TIME** **RIGHT COST**

[1] Christensen, Clayton M. *The Innovator's Dilemma: When New Technologies Cause Great Firms to Fail*, Harvard Business School Press, 1997.

While biotech and biopharma companies in the CGT space are highly competent at developing the right treatments for the right patients using innovative biology and chemistry at the bench top, custom bioprocessing tools arising from cross-disciplinary integration of engineering teams and scientists is needed to scale up and commercialize these therapies to realize the promise of advanced therapies in solving the full value chain for patients, providers, and payers.

Novel Technology Does Not (Always) Equal Disruptive Technology

Despite rising merger & acquisition activity in the advanced therapies market and widespread investor interest[2], the vast majority of these technologies have yet to enter the market. Most cancer patients today are faced with the same legacy treatment options including some version of chemo, radiation, and surgery. A fraction (~13%) of cancer patients may be eligible for 'genomically targeted' therapies such as antibody treatments based on specific tumor expression profiles, but only 7% of cancer patients benefit from these treatments, which are the precursors to even more advanced CGTs.[3]



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Novel biochemical and immunological technologies in the advanced therapies space, while they portend revolutionary outcomes in treating some of the most complex and difficult diseases humanity has yet come across, remain for the most part novel, not yet truly disruptive in the marketplace. For example, the cancer treatment market in 2020 was estimated to be approximately $150 billion, while the entire advanced therapies market in 2021 including non-cancer treatments was only $10 billion.[4,5]

A major obstacle to the advanced therapies industry is solving this gap between novelty and disruption for scale-up and manufacturing. Because advanced therapies are typically complex products that can be combinations of living biological components, chemical components, and devices, scale-up and manufacturing is not as straightforward as either biopharmaceutical or medical device companies are used to.

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[2]Mooraj, Gupta, Kawalekar, & Shah. *Cell and gene therapies: Delivering scientific innovation requires operation model innovation.* April 17, 2020. Deloitte Insights. https://www2.deloitte.com/us/en/insights/industry/life-sciences/operating-models-for-gene-cell-therapy-manufacturing-process.html

[3] Haslam, A, Kim, M. S., & Prasad, V, *Updated estimates of eligibility for and response to genome-targeted oncology drugs among US cancer patients, 2006-2020*, Annals of Oncology, 2021 (https://doi.org/10.1016/j.annonc.2021.04.003)

[4] *Advanced Therapy Medicinal Products Market Size, Share & Trends Analysis Report, 2021-2028*, Grand View Research

[5] *Global Industry Analysis, Size, Share, Growth, Trends, Regional Outlook, and Forecast 2022-2030*, Precedence Research

From Novelty to Disruption: Unique Cell & Gene Therapies Require Custom Scale-Up and Manufacturing

Every advanced therapy is unique and requires a unique approach to scale up and manufacturing. A recent advanced therapies industry review by Deloitte put it bluntly: "Drug discovery and approvals will continue, but if manufacturing and/or therapy delivery is prohibitively difficult or expensive, patients will likely not benefit." [2] To transform novel science from the bench to a commercialized product that reaches patients and provides clinical value, companies developing these therapies should pay close attention to their toolset. Particularly, they should identify opportunities where custom solutions may be preferable to off-the-shelf technologies for scale-up and manufacturing early on and establish partnerships to develop these custom solutions in parallel with clinical pipelines so that the manufacturing processes for these advanced therapies are well controlled and understood early on in development, limiting process variability and uncertainty throughout clinical studies and commercialization.

Additionally, there may be opportunities for adding value to a given advanced therapy by developing a combined solution to a wider scope of the clinical value chain through developing a custom tool or device that makes the logistics, clinical administration, or use of the product upstream or downstream from the therapy manufacturing easier and more well suited to the clinical environment.

Engaging an engineering firm fluent in medical, life sciences, and CGT device design to develop custom supporting hardware to advanced therapies is a robust strategy to increasing disruptive potential and chances of commercial success.

Owning The Advanced Therapy Manufacturing Process from Vein to Vein

Notwithstanding the advantages of custom manufacturing and scale up solutions for CGT discussed above, companies competing in the rapidly evolving advanced therapies market can also differentiate by widening their technological portfolio. Advanced novel biochemistry is important to the CGT market, but so too are custom platforms which enable the disruptive potential of therapies under development. CGT developers should consider widening and diversifying their IP portfolio across disciplines, from custom engineered equipment and software for bioprocessing and therapy administration all the way to the core biochemical technologies they seek to commercialize.

Summary

- Advanced therapies are widely novel, not yet widely disruptive, because they do not yet solve the entire spectrum of true clinical value.
- Scale-up and manufacturing of advanced therapies presents unique challenges not yet solved by either biopharmaceutical or medical device industries; Some of these challenges are best addressed by cross-disciplinary teams of scientists and engineers.
- Engaging an engineering firm fluent in medical, life sciences, and CGT device design to develop custom supporting hardware to advanced therapies is a robust strategy to increasing disruptive potential and chances of commercial success.

Tensentric is a team of highly experienced engineers developing a wide range of cell & gene therapy bioprocessing systems, medical devices, in vitro diagnostic systems, and life sciences instruments. Tensentric has completed over 300 development projects for clients since the company's inception in 2009 and is ISO 13485:2016 certified for design and manufacturing.