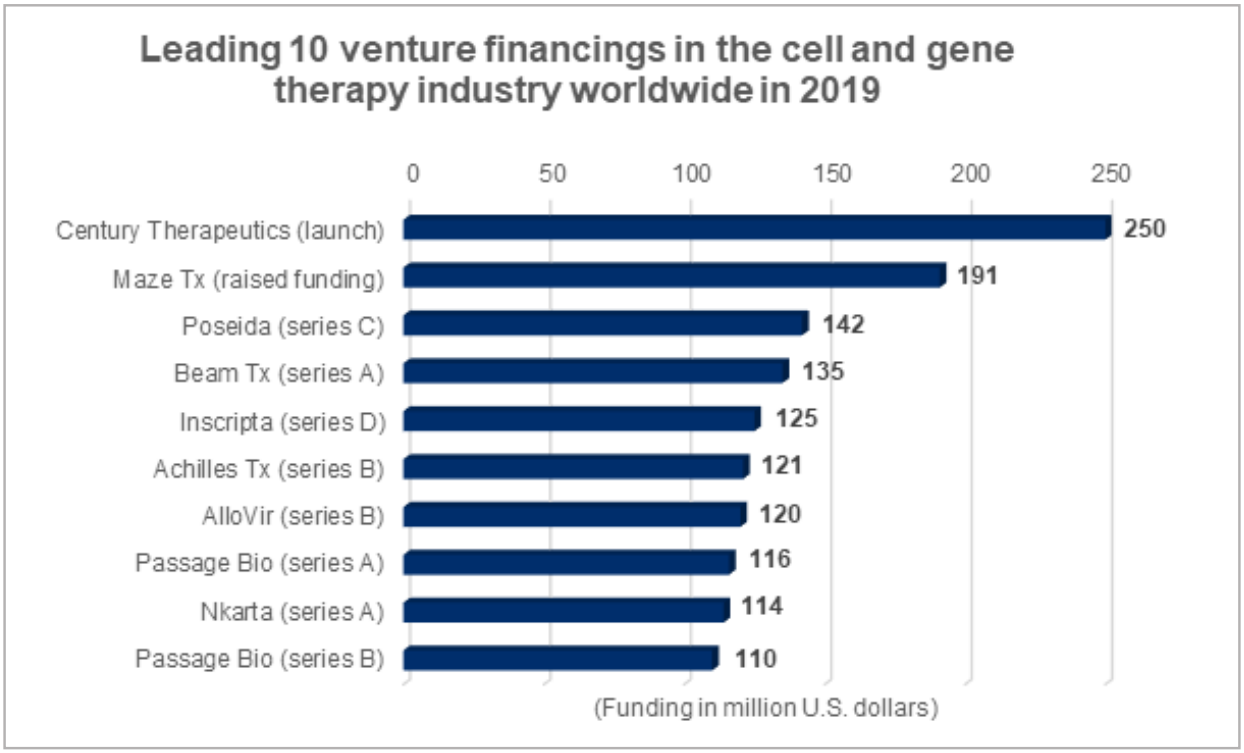
THREE KEYS TO VALUE FOR UNLOCKING THE PROMISE OF CELL THERAPIES

Faruk Abdullah,
Head of US Management Consulting,
EVERSANA MANAGEMENT CONSULTING

EVERSANA™
MANAGEMENT CONSULTING

eversanaconsulting.com

For several years cell therapies have been commanding the headlines with their novel scientific approach and remarkable results in improving patient outcomes. The allure of cell therapy has raised expectations not only for what it could do from a patient standpoint but also how it could potentially transform the way we think about treating and curing patients with life-threatening diseases. This has led to hundreds of millions of dollars of investment in research-stage cell therapy companies as well as a multitude of partnerships and acquisitions with the expectation of yielding significant clinical and commercial benefits.



Source: Statista

Many of the top 25 big pharma companies have placed heavy bets on being part of the cell therapy revolution. Celgene's acquisition of Juno therapeutics for $9 billion and Gilead's acquisition of Kite Pharma for $11.9 billion are powerful examples of the recognition and promise cell therapies have for saving lives and improving current treatment paradigms.

First-generation cell therapies have been in development and marketed for decades. Recently, however, we are seeing the proof-of-concept signals of the patient and commercial value that is being realized by the latest-generation cell therapies. In 2017, Novartis launched Kymriah, indicated for the treatment of lymphoblastic leukemia. Kite/Gilead launched Yescarta in the same year, indicated for the treatment of refractory large B-cell lymphoma. Kite/Gilead then followed up in 2020 with the approval of Tecartus, indicated for the treatment of relapsed or refractory mantle cell lymphoma.

Several other cell therapy products are on deck to be launched very soon and are anticipated to dramatically alter the standard of care for various indications, predominantly in oncology.

DRUG	COMPANY	TARGET	TIMELINE FOR APPROVAL
Potential 2020 Approvals			
Gene Therapy			
Zynteglo	Blubird bio	Beta thalassemia	Rolling BLA filed
nadoferagene firadenovec	FerGene	Bladder cancer	BLA filed (Dec. 2019)
Cell-Based Immuno-Oncology			
liso-cel	Bristol Myers Squibb (Celgene)	Diffuse large B-cell lymphoma (DLBCL)	BLA filed (Dec. 2019)
Cell Therapy			
Ryoncil	Mesoblast	Acute graft versus host disease	BLA filed (Jan. 2020)
Potential 2020 Filings			
Gene Therapy			
AT132	Audentes Therapeutics	X-linked myotubular myopathy	BLA filing expected mid-2020
Valrox	BioMarin	Hemophilia A	BLA filed (Feb. 2020); Received CRL
Lenti-D	bluebird bio	Boys cerebral adrenoleukodystrophy	BLA filing expected mid-2021
OTL-101	Orchard Therapeutics	ADA-SCID	BLA filing expected in 1H20 but delayed due to COVID
OTL-200	Orchard Therapeutics	Metachromatic leukodystrophy	BLA filing expected in 2H20 or 1H21
GT-AADC	PTC Therapeutics	AADC deficiency	BLA filing expected in 2H20
Cell-Based Immuno-Oncology			
ide-cel	bluebird bio /BMY	Multiple myeloma	BLA filed (July 2020)
omnidubicel	Gamida Cell	High-risk hematologic malignancies	BLA filing expected in 4Q20
Vigil-EWS	Gradalis	Ewing's Sarcoma	Phase III trials
Lifileucel	Iovance	Advanced metastatic melanoma	BLA filing expected in late 2020
LN-145	Iovance	Advanced metastatic cervical cancer	BLA filing expected in 2021
P-BCMA-101	Poseida Therapeutics	Multiple myeloma	BLA filing expected in 2021
Cell Therapy			
tab-cel	Atara Biotherapies EBV-PTLD		BLA filing expected in 2H20
MultiStem	Athersys	Multiple indications (Ischemic stroke/ARDS)	Phase 2/3 trials ongoing
PLX-PAD	PluriStem	Critical limb ischemia	Phase 3 data due 4Q20
MPC-06-ID	Mesoblast	Chronic low back pain	Phase 3 data expected mid-2020
MPC-150-IM	Mesoblast	Heart failure	Phase 3 data expected mid-2020

Source: Stephens Industry Note. Cell/Gene Therapy Takes from World Orphan Congress, Updated Approvals to Watch. August 28, 2020.

There is no question about the incredible clinical impact cell therapies can have on the lives of patients. However, this impact is the result of innovation that does not equate to a simple, straightforward commercialization process. The commercialization and management requirements of these treatments are just as complex as their innovative science.

Cell therapy manufacturers must think differently about their approach to and corresponding capability investments in commercialization and market readiness. This will require leveraging the latest thinking and best practices from traditional big pharma while also thoughtfully crafting novel approaches and keys to success. The list of "keys to value" is ever growing, and they will require creative thinking along with the application of disciplines from industries not necessarily associated with pharma or healthcare. Below, we will highlight a few standout keys to value that cell therapy leadership teams will have to navigate in a best-in class manner despite an arena with a high degree of complexity and uncertainty.

1 Financing

Talk of the promise and clinical success of cell therapies is accompanied by debate and thought leader discussions around the pricing and financing of these novel (and often expensive) treatments. The average price range of currently commercialized therapies is $370,000 to $450,000. When accounting for the total cost of care associated with delivering and managing these treatments, costs can increase to $1 million per patient.

Cell therapy manufacturers are building the case for these price ranges based on the curative nature of these products and the severe unmet need these treatments are addressing. Demonstrating true value in a real-world setting for these treatments will be critical to provide sufficient evidence that not only supports the pricing expectations but also provides further clarity on specific patient populations that will respond disproportionately better than other populations. Enhanced real-world monitoring and analytics of cell therapy patients is required to draw the correlative and causative factors that will drive clinical performance. However, this will necessitate technology investments that will span manufacturer, provider, patient and payer stakeholders.

Another aspect of the pricing and financing discussion relates to how these therapies will be funded and how the expenses will be distributed across payers and providers. Financing mechanisms, such as outcomes-based contracts, amortization schedules, reinsurance and payment pools, are in consideration to create an appropriate financial system of payment. Naturally, cell therapy manufacturers will have to collaborate with private insurance companies, hospitals and state and federal agencies to craft a set of financial approaches that provide the appropriate incentives for all parties while minimizing any financial burden on patients.

To further address patient financial burden, it will also be important for cell therapy companies to thoughtfully craft their patient support and assistance programs in addition to their drug pricing models. One major opportunity among cell therapy manufacturers is their patient assistance and support offerings. This will allow manufacturers to increase patient access by alleviating the financial burden for patients utilizing their treatments.

2 Provider Engagement (But Not What You Think)

Stating that provider engagement is another key to value is not only obvious but also a significant understatement in the context of cell therapies. Unlike when dealing with traditional pharmaceutical products, providers need a much greater level of support in order to successfully operationalize and provide cell therapies, and they look to manufacturers for help.

One example of this major difference is onboarding hospitals to be cell therapy ready. First, a great deal of effort will be required to assess the current capabilities of hospitals interested in using cell therapies by developing a deep understanding of how providers will use each unique therapy. Next, an onboarding process will need to be designed that assists hospitals in preparing to safely steward patients through a complex journey. This journey involves collecting and shipping patients' cells to the manufacturer; tracking the manufacturing process; receiving, storing, and administering the therapies; and, finally, providing post-infusion patient monitoring. It cannot be overstated how important and challenging it will be for cell therapy manufacturers to develop partnerships with providers and deliver the appropriate resources and training across multiple channels (e.g., in person, digital) to support care via cell therapies.

3 Supply Chain and Operations

Cell therapy manufacturers will need to bring a very different lens and set of capabilities to the supply chain and operations in order to successfully commercialize their products. Orchestrating activities across various stakeholders throughout the process of patient identification, product development, manufacturing, distribution, etc. will require drawing from the leading practices of other industries, such as shipping, retail and consumer packaged goods. The stakes are even higher because there is truly no room for error on late shipments, sub-quality product or poor execution of treatment protocols.

The answer to these challenges is not simply for each manufacturer to create a comprehensive software/ workflow management tool and standard operating procedures that their customers can access. Hospitals have already expressed concerns about using different software applications and resources that are tailored for specific cell therapy manufactures. The market is signaling a desire for a unified platform of technologies, tools and operational processes that can be a ubiquitous resource for any cell therapy manufacturer, hospital provider, payer and patient to access in order to maximize the value and care of using these treatments.

The three keys to value outlined above represent just a few of the requirements for realizing the true potential of cell therapies. Though the challenges are intimidating, they are not insurmountable. Leadership teams of cell therapy manufacturers need to revisit their approach to these issues, including how they define the keys to value; collaborate, design and construct solutions with all stakeholders in the process; and implement with capabilities that frequently will need to be developed from scratch. Ultimately, solving these issues will help patients realize the full value from the promise of cell therapy.

SOURCES:

https://www.statista.com/statistics/1088722/major-venture-financings-in-cell-and-gene-therapy-industry/

Stephens Industry Note. Cell/Gene Therapy Takes from World Orphan Congress, Updated Approvals to Watch. August 28, 2020.