THINK BEYOND THE THERAPY:
Demystifying the Complexities of Cell and Gene Therapy Commercialization

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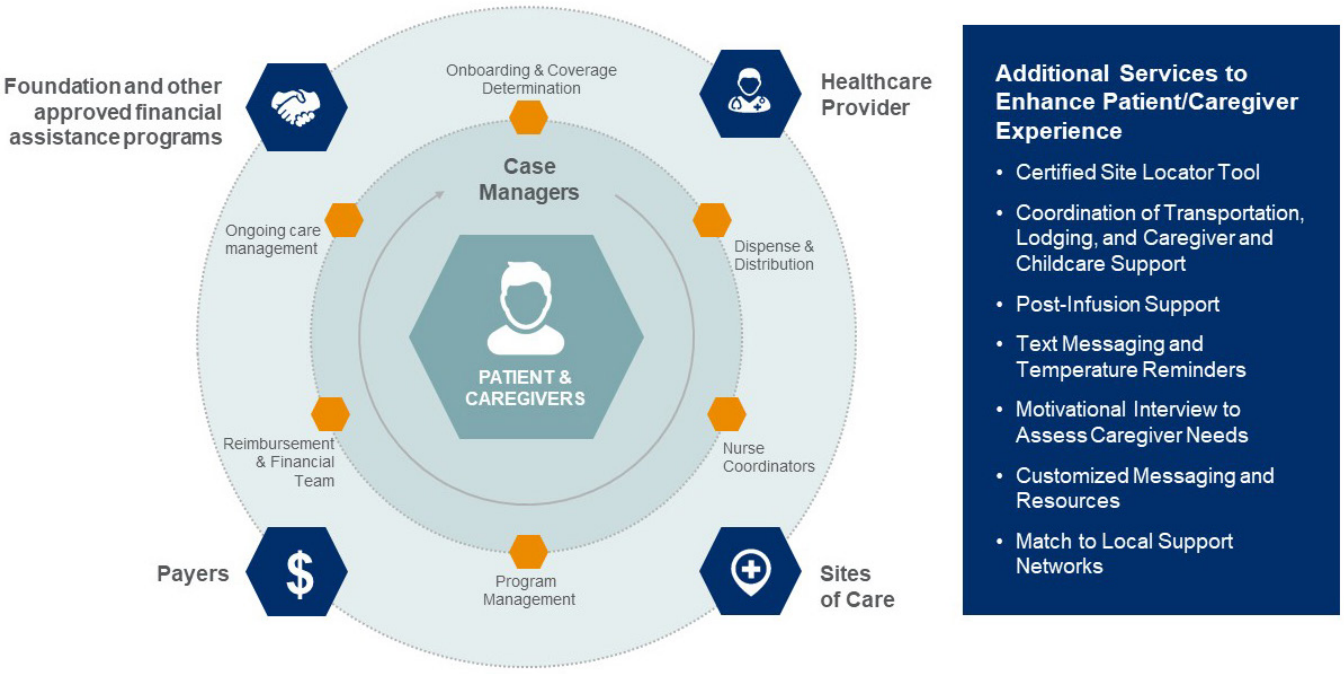
While much attention has been given to the novel science behind cell and gene therapies, ultimately the success of every precision medicine starts and ends with the patient. Whether coordinating a cell or gene therapy clinical trial or product launch, even the best-planned efforts can be derailed without a clear strategy for facilitating access to the therapy and supporting patients through the treatment journey. This is true for almost every specialty product, but it is especially important for cell and gene therapies, given the high cost and complexity of administering these innovative new therapies.

Figure 1

At a High Level, Cell and Gene Therapy Product Flow Sounds Simple. Behind the Scenes, Many Time-Critical Events Need Precise Coordination.

On the surface, the commercialization process for cell and gene therapies may seem simple. However, many time-critical events need to be precisely coordinated and planned across multiple facilities and stakeholders. Individualizing the treatment experience for patients (and their caregivers) is just as important as personalizing the therapy itself. This requires providing tailored, high-touch patient services throughout the treatment journey (*Figure 1*).

Figure 2



Strategic Considerations

Access to cell and gene therapy is associated with unique challenges and patient support needs (*Figure 2*). Cell and gene therapies require treatment at certified centers. Whereas commercial products can be administered at both academic centers of excellence (COEs) and non-academic sites, during the clinical trial stage treatment will likely be restricted to academic COEs. As a result of the limited treatment venues, a high need exists for transportation, lodging and caregiver and childcare support. Transportation and lodging support are perceived by customers and their patients as a critical offering, and caregiver-specific support and education remains a key unmet need in the current cell and gene therapy market.

For commercialized products, meanwhile, there is the added challenge of insurance navigation. Navigation of the coverage and access to the therapy — including steps like prior authorization (PA) documentation — can pose a burden and create barriers to treatment.

This means that manufactures should be aware of the following challenges when planning for commercialization of their cell or gene therapy:

- Investing in creating personalized, strong support relationships with each patient
- Providing intensive support to help patients navigate reimbursement challenges
- Ensuring alignment among the patient services program, the logistics team and the site of care
- Leveraging data to inform the patient support strategy and needs of the patient, healthcare provider (HCP) and caregiver
- Starting early when planning your patient support strategy

The Patient Journey

The patient journey for cell therapies can have multiple treatment steps and can be confusing for those families navigating the process. That is why it is critical to have a strong case management approach to assist at every stage of treatment. When the provider identifies the patient as a best fit for these therapies, the patient

journey becomes a literal one, as they may need to physically travel to and stay near (for extended periods of time) an approved site of care. This is where the path diverges for those patients being treated with autologous cell and gene therapies because only certain U.S. centers are trained on the process, preparation, treatment and ongoing care for these therapies.

When working toward understanding the different steps in the manufacturing and treatment process, manufacturers must consider a range of questions, including:

- When is apheresis?
- How long does it take for a therapy to be ready for the patient?
- What type of administrative prep work has to happen to prepare them to receive the therapy?
- What type of monitoring or follow-up needs to occur post-administration, and for how long?

When the patient is part of the manufacturing process, as it is with 1:1 cell and gene therapies, the patient support solution must follow the steps to ensure access and timely patient treatment. The key steps in the patient journey are illustrated in *Figure 3*.

Referral

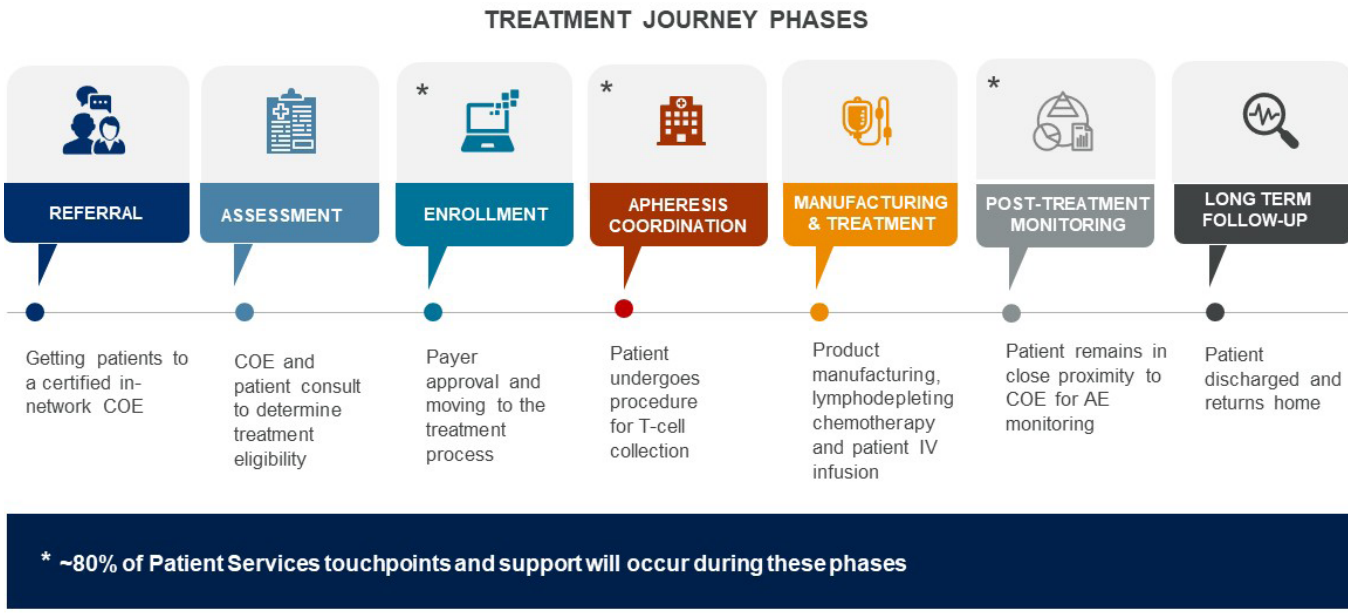
The referral process involves getting patients to a certified COE. A complicated treatment journey that is not well understood by both patient and care provider can lead to fear, apprehension and perceived difficulty to access treatment, as they face the issues of time, cost and travel. At this stage, the manufacturer must build trust with the patient and care provider, easing their fears through education.

Assessment

At the assessment stage of the patient journey, the COE and patient consult to determine treatment eligibility. Here, the patient faces emotional and logistical challenges in the potential transition of care to a new HCP/facility. With commercial products, patients face the additional burden and complexity of high treatment costs and the need to determine coverage.

Manufacturers can help ease the transition by working as a partner, providing a single point of contact for navigating facility, cost and travel logistics/support as well as caregiver-specific education and resources to help guide responses to diagnostic, assessment, patient eligibility criteria and/or caregiver roles and responsibilities.

Figure 3



Enrollment

Enrollment can take one of two forms. Patients being considered for clinical trials will need to enroll in the study according to protocol. Commercial patients will need payer approval. Patients, caregivers and their families face further logistical, time and cost burdens, while the treatment itself can be challenging for the patient and caregiver to endure.

Here, the manufacturer can work to provide coverage and access services, such as eligibility screening for patient assistance programs for drug and ancillary support services to help offset out-of-pocket expenses for the patient. Specific turnaround times for benefit verification, eligibility and coverage details can be communicated to the site of care and HCP, in addition to providing prior authorization and appeal support and billing and coding guidance.

Apheresis Coordination, Manufacturing and Treatment

At this stage, the patient undergoes the procedure for T-cell collection, which is followed by product manufacturing, lymphodepleting chemotherapy and patient IV infusion. Again, the patient and caregiver face logistical complexities (distance, time and cost) that all factor into the patient's decision-making process.

High-touch service points are needed throughout the treatment journey, including navigators to coordinate initial logistics and when changes occur (i.e., lodging, meals, transportation, childcare). Program resources can also be structured to help offset treatment and/or ancillary costs.

Post-Treatment Monitoring and Long-Term Follow-Up

T-cell-related toxicity post-treatment is a concern and requires monitoring and management, including 24/7 caregiver support for a period of time. Adverse event monitoring is particularly rigorous during the clinical trial stage. The patient must remain in close proximity to the COE for this time (which may be as long as 45 days). Even once the patient is discharged and returns home, check-ins and follow-ups are needed.

Again, manufacturers can assist through their programs to provided continued logistics support, education and tools to help the patient and caregiver in this post-treatment phase.

Conclusion

Thinking beyond the therapy and having the opportunity to fully understand the challenges each patient faces is critical to manufacturers.

1) Invest in creating personalized, strong support relationships with each patient.
2) Provide intensive support to help patients navigate reimbursement challenges.
3) Ensure alignment among the patient services program, the logistics team and the site of care.
4) Leverage data to inform the patient support strategy and needs of the patient, HCP and caregiver.
5) Start early when planning your patient support strategy.

Many of the challenges patients and their caregivers face are present whether the patient is enrolling in a clinical trial or is starting on an approved commercial product. Regardless of the path a patient is on (clinical trial or commercial), engaging with all stakeholders early and often is key to a successful treatment plan and administration.

For commercial patients, while payers' familiarity with cell and gene therapy grows as more treatments reach the marketplace, overall, there is still a need for payers to understand these unique types of advanced therapies and the challenges they present.

Not only is this education needed post-approval, but manufacturers need to collaborate with payers about ultimate coverage and payment decisions much earlier in the development life cycle. They should be specifically asking about — and then subsequently building into their phase 2 and phase 3 clinical trials — endpoints that are meaningful to payers and that are specifically designed to be used to support the value of their products.

A customer-centric support model is needed to ensure a simplified, enhanced experience for all treatment stakeholders. With the right tools and solutions, manufacturers can enable customer choice, remove burden from patients and HCPs and accelerate the patient's speed to therapy.