Cell-ebrate Innovation: Navigating the Next Frontier in Cellular, Molecular, and Bispecific Therapies

Matthew Hathcock M.S.[1], Brooke Gentzler M.S. [1], Aaron Maixner M.A. [2], Jenny Fahse M.B.A. [2], Hollie Stegemann M.A. [1]

[1]Department of Hematology, [2]Department of Laboratory Medicine and Pathology

INTRODUCTION

The FDA has approved 45 Cellular, Molecular, and Bispecific (CMB) products since 2010, with 42 receiving FDA approval since 2014. The approval of CMB products has increased in recent years, with ten products receiving FDA approval in 2022 and seven products in 2023 and 2024. These products introduced new challenges with escalating wholesale acquisition costs (WAC) and increased supportive care requirements (Table 1). We continue to see products receive FDA approval that challenge and strain healthcare system resources, with Zolgensma (2019) and Hemgenix (2022) being multimillion-dollar gene therapies administered in the outpatient setting, and more recent multimillion-dollar genetically edited stem cell products administered in the inpatient setting. In addition to the growing FDA approvals, there is significant growth in the clinical trial setting. Currently, there are more than 4,000 CMB clinical trials, with 2,093 gene therapies and 885 cellular therapies[1].

TABLE 1: Median cost of CMB products with costs data publicly available through web search.

Modality	Products[1]	Median Cost
Bispecifics[2]	10	$431,000
Cell Therapy	9	$468,088
Gene Therapy	20	$2,475,000
RNA Therapy	6	$121,688

[1]Does not represent the number of products with cost data publicly available.

[2]Estimated annual cost for bispecific antibody therapy

OBJECTIVE

The diverse portfolio of therapies and rapidly changing environment requires a complex healthcare management strategy. Four key objectives for the management of CMB products were identified:

1. Resource allocation for administration and patient care.
2. Clinical trial resource management and selection.
3. Strategic selection of CMB therapies.
4. Monitoring of patient outcomes.
5. Centralizing financial risk and performance monitoring.

IMPLEMENTATION

Stakeholders from critical groups support the CMB Program through a Hub and Spoke Model of Care (figure 1). The Hub and Spoke Model placed the CMB Program at the center, working with various specialists to ensure patient care. The CMB Steering Committee was created to provided oversight, with two subcommittees focusing on different therapy types: outpatient molecular therapies and inpatient cell engager therapies. These committees collaboratively monitored CMB therapies, ensuring financial security and high-quality patient outcomes.

RESULTS

- Established two committee group for review and approval of therapies.
- Since implementation, the CMB steering committee has reviewed and approved seven products.
- Successfully integration of Committee to provide oversight of CMB product review and approvals.
- Successful implementation of the hub and spoke model.

Figure 1 The Hub and Spoke Model of Care for Cellular, Molecular, and Bispecific Therapies

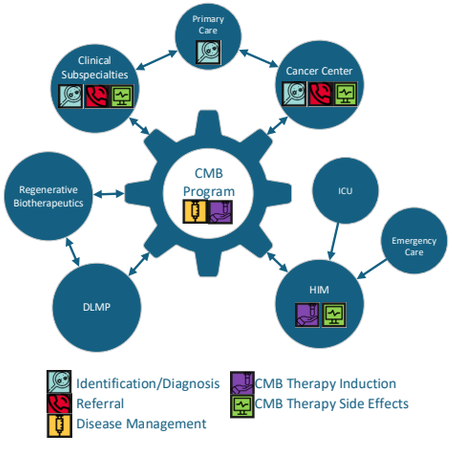
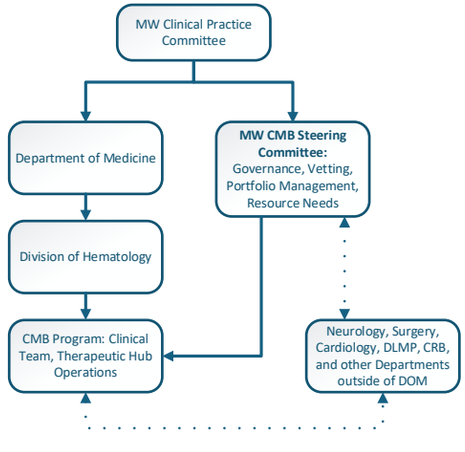


Figure 2 Committee reporting and collaborating structure



DISCUSSION

- These therapies are transformative and have notable financial risk.
- The ethical considerations should be at the forefront of monitoring CMB products.
- The financial profile introduces significant challenges.
- The complexity of accreditation, compliance, and quality is complex when considering FDA approved products and clinical trial products.

CONCLUSIONS

- Comprehensive management and oversight model is required for these therapies.
- A multi-committee approach is required to monitor the complex therapy portfolio.
- Standard of Care and Clinical Trails must fall under the same oversight structure.

REFERENCES

1. Barrett, D. Gene, Cell, & RNA Therapy Landscape Report, Q3 2024 Quarterly Data Report. American Society of Gene + Cell Therapy. Citeline.
2. U.S. Food and Drug Administration. Approved Cellular and Gene Therapies. Updated November 21, 2024. Accessed December 2, 2024.
3. Shukla V, Seoane-Vazquez E, Fawaz S, Brown L, Rodriguez-Monguio R. The Landscape of Cellular and Gene Therapy Products: Authorization, Discontinuations, and Cost. Hum Gene Ther Clin Dev.