Managed Care and Clinician Insights on Optimizing the Management of Inherited Blood Disorders in a Changing Market:

Findings from AMCP Market Insights Expert Interviews

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BACKGROUND

Inherited blood disorders, including coagulation disorders like hemophilia and hemoglobinopathies such as sickle cell disease (SCD) and beta-thalassemia, significantly impact blood cell formation, architecture, or function. These disorders, with well-understood genetic bases, are prime candidates for gene therapy. Concerns about the long-term effectiveness and high initial costs of gene therapies have tempered the enthusiasm for their transformative potential.

OBJECTIVE

To expand on the 2022 AMCP Market Insights on managing inherited blood disorders, identify additional managed care and clinician views on the clinical appropriateness of gene therapy, potential outcomes from value-based contracting (VBC), and the challenges patients face with health insurance coverage.

METHODS

In Fall 2023, AMCP Market Insights engaged in six in-depth interviews with managed care experts overseeing over 77 million individuals and conducted an online survey among Hemophilia Treatment Centers (HTCs) in 18 states, serving around 13,000 patients.

In-depth Interview Demographics

National Electronic Survey of HTC Centers of Excellence (COE)

Responses from **27 HTCs**
Across **18 states**
Serving **13,000 patients** with bleeding disorders

The interview guide and survey were built on insights from the 2022 AMCP Market Insights findings on optimizing the management of inherited blood disorders.[1]

RESULTS

Table 1: Managed Care Perspectives on Gene Therapy Clinical Appropriateness and Possible Value-based Contracting (VBC) Outcomes

Hemophilia Gene therapy is generally considered clinically appropriate for individuals with severe or moderate hemophilia who have experienced frequent bleeding episodes and have not responded well to standard treatments, and coverage will likely align with the treatment on the FDA label. VBC outcomes of interest include reducing or eliminating the need for regular clotting factor infusions and reducing the number of bleeds.
Sickle Cell Disease (SCD) Gene therapy will likely be appropriate for all individuals with SCD, particularly those with severe symptoms and frequent complications. Payers are interested in seeing outcomes such as reducing the frequency and severity of pain crises, organ damage, and hospitalizations as potential value-based measures.
ß-Thalassemia Gene therapy is generally considered for individuals with severe beta-thalassemia who require regular blood transfusions or experience complications associated with the disease. Payers are interested in reducing or eliminating the need for regular transfusions and improving hemoglobin production.

Table 2: National Electronic Survey of HTC Centers of Excellence (COE)

Twenty-seven centers across 18 states participated in the bleeding disorder COE survey. Four kinds of COEs were surveyed and represented (university-based, 41%; standalone facilities, 26%; integrated delivery system, 26%; and children's hospital, 15%), with the majority (89%) of COEs reporting they treat patients with hemophilia and von Willebrand disease, whereas significantly less also treat SCD (7%) and β-thalassemia (4%).

National Electronic Survey of HTC Centers of Excellence (COE) (selected questions)		
Question	***Response***	***Response % (n/total)***
Do you find that as a physician you must tailor treatment choices not only to a patient's clinical needs, but also to their health insurance plan?	Yes	100 (18/18)
	No	0 (0/18)
What are the most common access challenges your patients experience due to their health insurance coverage?	Choice of provider	41 (7/17)
	Approval of prescribed therapy	88 (15/17)
	Mental health services	41 (7/17)
	Pain management services	41 (7/17)
What do you see as potential limitations specifically of gene therapy for hemophilia, sickle cell disease, and/or beta-thalassemia?	Cost Impact	17 (2/12)
	Willingness of payer to cover gene and cell therapies	75 (9/12)
	Patient burden	8 (1/12)
Is your organization currently using or considering using value-based or alternative payment models for high-investment treatments?	Yes	33 (4/12)
	No	66 (8/12)

CONCLUSIONS

Research indicates a strong agreement on the clinical benefits of gene therapy for inherited blood disorders among experts. However, widespread adoption faces obstacles such as insurance barriers, access to specialized care, and reimbursement models, which must be addressed to fully leverage these advanced treatments.

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1. Optimizing The Management Of Inherited Blood Disorders Findings From An AMCP Market Insights Program. Published January 2023. JMCP website. https://www.jmcp.org/pb-assets/Outserts/MarketingInsights_IBD-1670961133577.pdf

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