Do Breakthrough Therapies Demonstrate Breakthrough Value? An Evaluation of US Hemophilia B Gene Therapy Access Policies

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Background

- Gene therapies are hailed as potentially curative treatments; given this potential benefit, regulators tend to classify them as Breakthrough products
- However, payers may not perceive the same value of these products, considering the high cost of these treatments
- This study sought to assess payer–regulator alignment on the value of Hemophilia B gene therapies

Methodology

- Approved Hemophilia B gene therapies were selected for review and key data abstracted from various sources (Fig. 1)
- Policy restrictiveness was assessed by comparing coverage criteria against (1) the FDA labeled indication and (2) pivotal trial inclusion / exclusion criteria
- Policy publication dates were compared to FDA approval dates
  - If exact publication dates were not found, the first of the publication month was used for analysis

Results

- The study included two FDA-approved gene therapies: Hemgenix (FDA Approval: November 2022) and Beqvez (FDA Approval: April 2024)
- All 12 payer policies reviewed included additional criteria for treatment eligibility compared to the population in the approved FDA label
- Furthermore, 67% (8/12) limited treatment eligibility to a population meeting (n=3) or exceeding (n=5) each product's trial criteria (Fig. 2)
- Level of restrictiveness was similar for each therapy, with exception of BCBS Michigan, which listed Beqvez as a "preferred product" (Fig. 3)
- Most payers (4/6) took longer to publish their Beqvez policy, though mean time to publication was similar (Fig. 4) (92.5 days Hemgenix, 97 days Beqvez)

Conclusions

- All US payer policies reviewed defined a narrow patient population for Hemophilia B gene therapies compared to the approved FDA labels
- For the majority of policies, patients also had to meet or exceed trial criteria
- Misalignment between payers and regulators on the value of Hemophilia B treatments suggests that manufacturers must better communicate breakthrough product value to payers

References

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Hemophilia B gene therapy **coverage policies are more restrictive than regulatory labels,** suggesting that payers do not perceive price / value alignment for these Breakthrough products.

Manufacturers must better communicate Breakthrough product value to payers.

Figure 1: Key Data Sources and Abstracted Information

	FDA Approved Products Database	Clinical Trials Website	Payer Websites
Information Abstracted (accessed December 19, 2024)	FDA approval date, labeled indication (PI)	Pivotal trial inclusion / exclusion criteria	Coverage policies, access criteria, and publication dates

Key payers included in analysis: aetna, cigna healthcare, HCSC, Blue Cross Blue Shield of Michigan, Elevance Health, United Healthcare

Figure 2: Policy Restrictiveness vs. Trial Criteria

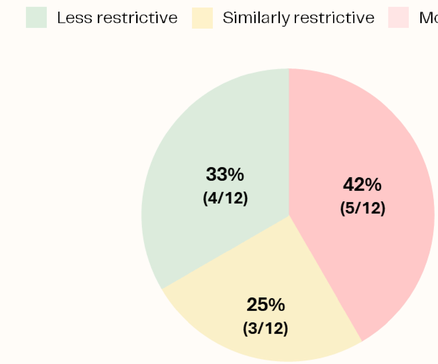
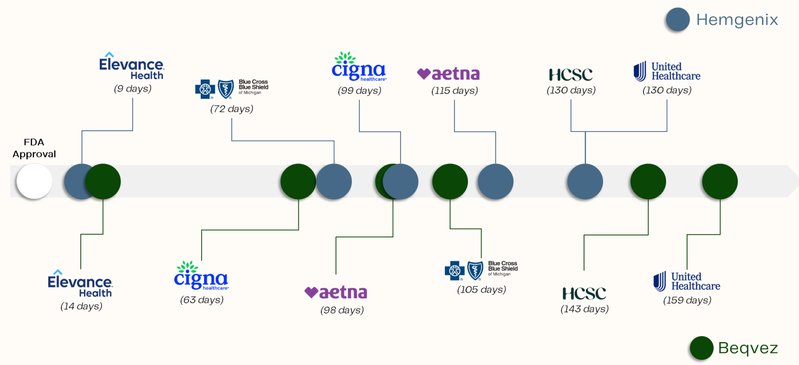


Figure 3: Individual Payer Coverage vs. Trial Criteria

Less restrictive · Similarly restrictive · More restrictive

	aetna	Blue Cross Blue Shield of Michigan	cigna healthcare	Elevance Health	HCSC	United Healthcare
Hemgenix						
Beqvez						

Figure 4: Time to Policy Publication from Approval



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