# Evaluation of a Quantity Limit on Hemophilia Factor Products in a Large Managed Care Organization: Pharmacy Cost and Utilization Outcomes

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## Background

- Hemophilia is among the most expensive disease states to manage as published estimates suggest that mean healthcare costs for patients with hemophilia in the United States reach upward of $140,000 per year.[1,2]
- Among hemophilia costs, clotting factor concentrate (CFC) used for prophylaxis has usually accounted for over 90% of the cost of hemophilia care. [2]
- Utilization manage programs can help ensure patients are using clotting factor optimally as overutilization provides no additional benefit and can increase annual treatment costs anywhere from 12-25%.[3]
- In April 2022, Blue Cross NC implemented a quantity limit (QL) for three of their Hemophilia policies allowing coverage for a maximum of 3 on-demand doses and up to a 5%-unit variance for dispensed factor product.

## Objective

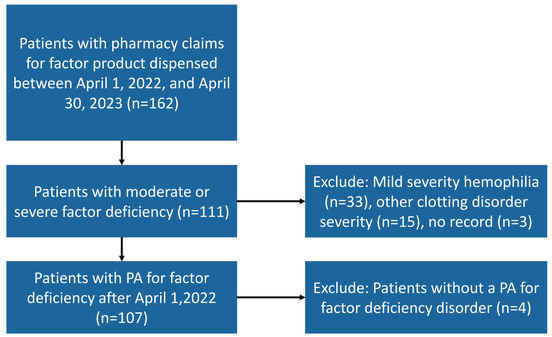
- To assess the clinical and economic impact of a Blue Cross NC quantity limit among commercial plan members treated with factor products using medical records and claims data.

## Methods

**Study design and data:**

- Retrospective analysis of factor product utilization using prior authorization determinations, electronic health records, and pharmacy claims data 12-months pre/post of the QL implementation.

**Study population:**




**Data analysis:**

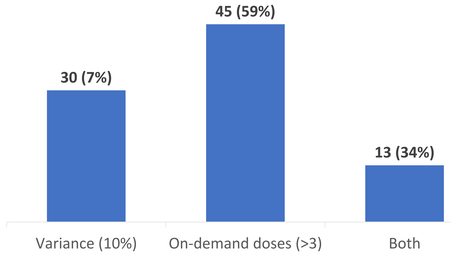
- Descriptive statistics were calculated for all variables of interest.
- Cost avoidance was calculated by multiplying the difference in requested vs. approved units and multiplying by the payer's product-specific avg. $/unit

## Results

### Table 1. Population Characteristics

| Characteristics | Variable | Moderate (n = 17) | Severe (n = 90) |
|---|---|---|---|
| Demographics | Age, years (median) | 28 | 28.5 |
| | Male, n (%) | 17 (100) | 89 (98.9) |
| ICD10 Description | Hemophilia A - **Factor VIII Deficiency** | 12 (70.6) | 78 (86.7) |
| | Hemophilia B - **Factor IX Deficiency** | 5 (29.4) | 10 (11.1) |
| | Other | - | 2 (2.2) |

### Figure 1. Cost Savings by QL Variable




### Table 2. Approval/Denial Rates Summary

| Status | Indication | Factor VII | Factor VIII | Factor IX | Hemlibra |
|---|---|---|---|---|---|
| **Approved (253, 91%)** | Prophylaxis (PPX) | - | 15 | 3 | 75 |
| | On-demand (PRN) | 3 | 70 | 2 | - |
| | PPX + PRN | 3 | 42 | 12 | - |
| | Active Bleed | - | 6 | 2 | - |
| | Peri-Operative | 1 | 10 | 2 | - |
| | Other Combination | - | 6 | 1 | - |
| | **Grand Total** | **7 (2.8)** | **149 (58.9)** | **22 (8.7)** | **75 (29.6)** |
| **Denied (25, 9%)** | **Grand Total** | **1 (4)** | **13 (52)** | **5 (20)** | **6 (24)** |

### Table 3. Estimated Cost Avoidance

| Policy | Type of PA Requests (#) | Total Requests (%) | Estimated Total Cost Savings through 7/31/23 |
|---|---|---|---|
| Factor IX | On-demand (2)<br>Prophylaxis (1)<br>Both (7) | 10 (14%) | $4,798,120 |
| Factor VIII | On-demand (39)<br>Prophylaxis (4)<br>Both (17) | 60 (85%) | $4,787,053 |
| Factor VII | On-demand (1) | 1 (1%) | $239,760 |
| **Grand Total** | | **71** | **$9,824,934** |

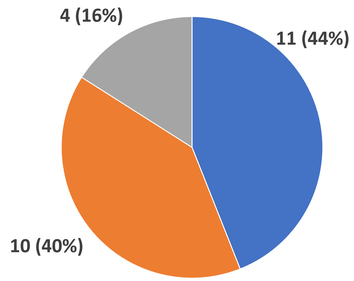
### Table 4. Top Hemophilia Spend

| | Pre-QL (4/1/2021-3/31/2022) | | | Post-QL (4/1/2022-3/31/2023) | | | % Change | | |
|---|---|---|---|---|---|---|---|---|---|
| **Policy** | **Rxs** | **Patients** | **Total Plan Spend** | **Rxs** | **Patients** | **Total Plan Spend** | **Rxs** | **Patients** | **Total Plan Spend** |
| FACTOR IX | 256 | 31 | $ 7,627,949 | 254 | 24 | $ 8,567,618 | -0.79% | -29.17% | 10.97% |
| FACTOR VIII | 807 | 122 | $ 33,880,546 | 908 | 109 | $ 33,672,732 | 11.12% | -11.93% | -0.62% |
| FACTOR VII | 15 | 7 | $ 465,416 | 20 | 4 | $ 1,762,398 | 25.00% | -75.00% | 73.59% |
| **Grand Total** | **1078** | **160** | **$ 41,973,911** | **1182** | **137** | **$ 44,002,748** | **8.80%** | **-16.79%** | **4.61%** |

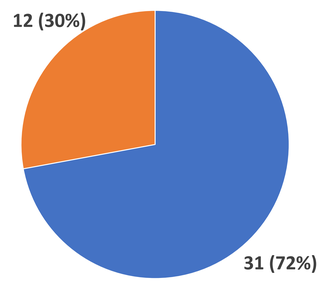
### Table 5. Top 5 Hemophilia Drug Spend

| | Pre-QL (4/1/2021-3/31/2022) | | | | Post-QL (4/1/2022-3/31/2023) | | | | |
|---|---|---|---|---|---|---|---|---|---|
| **Rank** | **Drug** | **Rxs** | **Patients** | **Total Plan Spend** | **Rank** | **Drug** | **Rxs** | **Patients** | **Total Plan Spend** |
| 1 | HEMLIBRA | 620 | 56 | $ 21,883,376 | 1 | HEMLIBRA | 702 | 51 | $ 25,343,560 |
| 2 | NUWIQ | 256 | 16 | $ 17,124,363 | 2 | NUWIQ | 308 | 18 | $ 17,798,524 |
| 3 | ELOCTATE | 107 | 8 | $ 4,584,179 | 3 | ALPROLIX | 102 | 9 | $ 4,276,352 |
| 4 | ALPROLIX | 105 | 19 | $ 4,509,865 | 4 | ELOCTATE | 119 | 16 | $ 4,178,599 |
| 5 | ADYNOVATE | 99 | 14 | $ 3,135,168 | 5 | NOVOEIGHT | 58 | 5 | $ 2,920,358 |
| **Grand Total** | | 1187 | 113 | $ 51,236,953 | | | 1289 | 99 | $ 54,517,394 |

### Figure 2. Denial Reasons




### Figure 3. Provider Follow-Up




## Conclusions

- The findings from this study suggest that on-demand regimens are more likely to be impacted by QL than prophylaxis or prophylaxis/on-demand regimens.
- Incorporating a QL strategy for hemophilia factor products led to a high potential for payer cost savings.
- However, increased factor utilization contributed to a higher plan spend overall.
- Alternative methods for assessing the impact of UM strategies for hemophilia are necessary as payers are faced with managing costs for emerging non-factor and gene-therapy products.

## References

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3. Blankenship CS. To manage costs of hemophilia, patients need more than clotting factor. Biotechnology healthcare. 2008 Nov;5(4):37.

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