



Pharmacoeconomic analysis of rFVIIa versus PCC in the treatment of minor-to-moderate bleeds in hemophilia patients with inhibitors in China

Jiaqi Liu¹, Zhen Ruan², Junchao Feng¹, Shunping Li^{*1}

1.Centre for Health Management and Policy Research, School of Public Health, Cheeloo College of Medicine, Shandong University, Jinan, 250012, China.

2.State Key Laboratory of Quality Research in Chinese Medicine, Institute of Chinese Medical Sciences, University of Macau, Macao SAR, China.

Objective

- In China, treatment options for bleeding episodes in hemophilia patients with inhibitors most often involve recombinant factor VIIa (rFVIIa) or prothrombin-complex concentrate (PCC).
- The aim of this study was to compare the cost and effectiveness of two most commonly used treatment regimens : rFVIIa followed by PCC (rFVIIa - PCC) and PCC followed by rFVIIa (PCC - rFVIIa) for the treatment of minor-to-moderate bleeds in hemophilia patients with inhibitors.

Methods

- An observational study was conducted in China from September 2020 to January 2021. We applied the online questionnaire collecting patients’ treatment information over the past year.
- The cost included direct medical cost, direct nonmedical cost and indirect cost. Clinical outcomes included the hemostatic efficiency and re-bleeding rate within 12 hours.

Results

- Overall, 32 bleeds occurred in 26 hemophilia patients with inhibitors (12 adults; mean age=20.4±13.2 years; 25 patients with high response inhibitors; 21 patients with severe hemophilia, 4 patients with moderate hemophilia) were included.
- There were 13 bleeds episodes treated with rFVIIa - PCC and 19 bleeding episodes with PCC - rFVIIa. Median cost of bleed treated with rFVIIa - PCC and PCC - rFVIIa were CNY 38,935(direct medical cost, direct nonmedical cost and indirect cost were CNY 37620, 115 and 1200, respectively) and CNY 47,280 (direct medical cost,direct nonmedical cost and indirect cost were CNY 45620, 660 and 1000, respectively).

Table 1: Demographic characteristics of patients with inhibitors

Characteristics	N (%)
Age	
> 18	12 (46.2)
≤ 18	14 (53.8)
Mean age ($\bar{x}\pm s$)	20.4±13.2
Severity[n (%)]	
Severe(>5%, ≤40%)	1 (3.8)
Moderate(1%~5%)	4 (15.4)
Mild(<1%)	21 (80.2)
Inhibitor levels (BU/ml)	
Low inhibitors titres (≤5BU/ml)	1 (3.8)
High inhibitors titres (> 5BU/ml)	25 (96.2)
Annual income (CNY)	
≤10000	4 (15.3)
10001-50000	10 (38.5)
50001-100000	10 (38.5)
> 100000	2 (7.7)

Results

- The hemostasis efficiency of rFVIIa - PCC was 77%, and PCC - rFVIIa was 63%.
- The re-bleeding rate within 12 hours of rFVIIa - PCC (8%) was lower than PCC - rFVIIa (32%).

Table 2: Comparison of two regimes

	Direct medical cost	Direct nonmedical cost	Indirect cost	Re-bleeding rate within 12 hours	Hemostatic efficiency
rFVIIa - PCC	37620	115	1200	8%	77%
PCC- rFVIIa	45620	660	1000	32%	63%

Conclusion

- The rFVIIa - PCC treatment regimen was cheaper than PCC - rFVIIa with higher hemostatic efficiency and lower re-bleeding rate within 12 hours.
- Therefore, rFVIIa - PCC was found to be dominant (increasing clinical outcome and decreasing costs) compared with PCC - rFVIIa.

References

1. Jardim L L, Chaves D G, Rezende S M. Development of inhibitors in hemophilia A: An illustrated review[J]. Res Pract Thromb Haemost, 2020, 4(5): 752-760.

2. Kanters TA, Bouwmans CAM, van der Linden N, Tan SS, Hakkaart-van Roijen L (2017) Update of the Dutch manual for costing studies in health care[J]. PLoS ONE, 2017,12(11):e0187477.