Assay Management for the Treatment of Hemophilia A and B: Experience from the Gulf States Pharmacy (GSP)

Stephanie Gustafson, Margaret Lee, Aroub A. Khleif, Nidra Rodriguez, **Miguel A. Escobar**

The University of Texas Health Science Center at Houston, Gulf States Hemophilia & Thrombophilia Center
Houston, TX, USA

INTRODUCTION

- While there have been substantial improvements in the treatment of hemophilia during the past three decades, these advancements have come at a high cost, and hemophilia remains among the most expensive and challenging diseases to manage.

OBJECTIVES

- The primary objective of this study was to determine the difference between a patient's physician-prescribed factor dose, and the actual dose that was dispensed based on assay availability.

METHODS

- This was a prospective study that collected prescription data among patients on a prophylaxis regimen who utilized the GSP (340B program) during March and June 2013.
- Assay selections were based on the manufacturers' weekly factor assay availability reports.
- Information regarding the prescribed product and dose, available assays closest to the prescribed dose along with the number of vials required to reach that dose, and the actual assays and number of vials dispensed to the patient were collected.
- While the guidelines for filling prescription orders are recommended to be between ± 5% to 10% of the prescribed dose without using an excessive number of vials, the GSP strives to manage doses of factor to achieve less than a 5% variance from the number of prescribed units.

Table 1. Selected characteristics of patients utilizing the Gulf States Pharmacy between March and June 2013

Characteristics	n	%
All Patients	80	
Age in years		
Median (IQR)	16.5 (10.5 - 29)	
Race/Ethnicity		
Caucasian (Non-Hispanic)	37	46.2
Hispanic	28	35.0
African American	5	6.3
Asian Pacific Islander	10	12.5
Hemophilia disease		
A	63	78.8
B	17	21.2
Severity		
Mild	2	2.5
Moderate	6	7.5
Severe	72	90.0
Number of vials dispensed to reach optimal dose		
Mean ± SD	1.8 ± 0.85	

IQR, interquartile range
SD, standard deviation

RESULTS

- A total of 129 prescriptions for 80 patients were dispensed during the study period.
- The majority of the sample had hemophilia A and was less than 17 years of age.
- The difference between the patient's prescribed and actual dose dispensed was calculated taking into account the available assays, and the number of vials required to reach as close to the prescribed amount as possible.
- The results of this study showed that greater than 70% of the prescriptions dispensed were within ± 5% of the physician's prescribed factor dose (median = 1.7%, IQR: 1%, 2.8%).
- Greater than 90% were within ±10% of the prescribed dose. Additionally, 41% of these prescriptions required only 1 vial for patients to reach their optimal dose.

CONCLUSIONS

- The availability of factor assays drives the factor dose that is dispensed to patients.
- Precise management of factor concentrate is essential in minimizing waste and reducing healthcare costs.
- Patient's requests for one vial to reach optimal dose may not always be feasible due to lack of suitable assay strengths.
- Managing hemophilia requires a coordinated effort by the patient, treatment center, and the pharmacy.

REFERENCES

1. Blankenship CS. To manage the costs of hemophilia, patients need more than just clotting factor. Biotechnol Healthc 2008; 5(4): 37– 40.
2. Manufacturer Assay Availability Lists (Bayer, Baxter, CSL, Pfizer, Kedrion) March 2013 and June 2013.
3. National Hemophilia Foundation. MASAC recommendations regarding standards of service for pharmacy providers of clotting factor concentrates for home use to patients with bleeding disorders. MASAC Document #188.
4. Manco-Johnson MJ, Abshire TC, Shapiro AD, et al. Prophylaxis versus episodic treatment to prevent joint disease in boys with severe hemophilia. New Engl J Med. 2007;357:535–544.

DOI: 10.3252/pso.eu.WFH2014.2014