**Title:**

Bleeding severity and haemostatic management of inherited Rare Bleeding Disorders (RBDs) at an Australian Haemophilia Treatment Centre.

**Aim:**

Rare bleeding disorders (RBDs) refer to inherited deficiencies of coagulation factors other than VIII and IX. We sought to investigate the relationship between factor activity level and bleeding severity in patients with RBDs at a large Australian Haemophilia Treatment Centre (HTC).

**Method:**

A cross-sectional analysis was performed of patients registered in the Australian Bleeding Disorders Registry (ABDR) at the Alfred Hospital HTC with factor V, VII, X, XI or XIII deficiency as of March 31, 2023. Fibrinogen disorders are currently the subject of a separate project at our institution and so were excluded. Bleeding episodes were retrospectively assessed using both the International Society of Thrombosis and Haemostasis bleeding assessment tool (ISTH-BAT) and European Network of Rare Bleeding Disorders (EN-RBD) severity category. Linear regression analysis was used to determine the association between factor activity level (dependent variable) versus ISTH-BAT and EN-RBD grade (independent variables).

**Results:**

61 patients had adequate records for analysis as tabulated below:

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
|  | **FV****(n = 4)** | **FVII****(n = 19)** | **FX****(n = 4)** | **FXI****(n = 28)** | **FXIII****(n = 6)** |
| Age | 38 (28-33) | 52 (30-62) | 34 (29-36) | 54 (41-79) | 31 (27-32) |
| Male (%) | 25 | 63 | 50 | 54 | 83 |
| Baseline factor activity | 2 (2-11) | 11 (5-29) | 12 (1-26) | 2.5 (1-6) | 8.5 (2.5-10) |
| ISTH-BAT score | 5 (4-7) | 3 (0-4) | 3 (2-5) | 3 (1-6) | 7 (6-11) |
| EN-RBD grade (%)No bleedingGrade 3 (severe) | 2525 | 4216 | 025 | 292 | 017 |
| ISTH-BAT vs factor activity (r2) | 0.5525 | 0.1597 | 0.5717 | 0.0187 | 0.3447 |
| EN-RBD vs factor activity (r2) | 0.8596 | 0.0487 | 0.2173 | 0.0112 | 0.4402 |
| Peri-procedural prophylactic haemostatic agent use (%) | 25 | 31 | 25 | 43 | 20† |

All data are median (IQR), unless otherwise stated.

† 5 of the 6 patients with FXIII deficiency were already receiving regular prophylactic FXIII concentrate

**Conclusion:**

The relationship between factor activity and bleeding severity by both tools was strongest for FV, FX and FXIII and weakest for FXI, similar to previous reports. Prophylactic use of FXI concentrate is common, despite FXI deficiency having the mildest bleeding phenotype at presentation and weakest correlation between factor activity and bleeding.