Pharmacokinetic (PK)-guided prophylaxis for people with hemophilia A (PwHA) treatment has been related to fewer bleeding and cost savings have been reported. myPKFiT®is a populational PK tool available for this purpose, developed for α-octocog (Advate®, Takeda) analysis. We evaluated the impact of myPKFiT®-guided prophylaxis in PwHA on replacement costs and bleeding episodes. Men with severe (FVIII < 1%) or moderate (FVIII 1%-5%) HA without inhibitors and treated with Advate®were invited at two Southern Brazilian Hemophilia Treatment Centers. Inclusion criteria involved ≥ 50 exposure days, age 1-65 years, weight 12-120 kg, bleeding-free for ≥ 2 weeks, and last surgical procedure ≥ 6 months. PwHA who developed inhibitor (> 0.6 BU/mL at two time-points) during the study period were excluded. Anthropometric and hemophilia-related data were obtained using a standardized form. PK analysis was performed according to myPKFiT®recommendations, using one-step test. After PK analysis, PwHA were approached to adjust their regimen according to bleeding phenotype, arthropathy, and physical exercise. Replacement regimen and FVIII disposal (from now called consumption) were assessed before and after treatment adjustment. PwHA younger than 15 years were evaluated for 6 months, while those 15 years or older were evaluated for 12 months. Annualized bleeding rate (ABR) was calculated from the reported treated bleeding episodes. FVIII disposal (from now reported as consumption) was adjusted by weight (kg) and monthly. The cost of 1 IU of Advate®was based on the median date of the evaluated periods, which was July/2019. We converted the price in Real into American Dollar, considering the currency at the date that the Brazilian Ministry of Health purchased the product. A total of 37 PwHA were included. In the younger group (n = 20), 75% were severe, and 65% had no hemophiliac arthropathy. Half of these patients were on primary prophylaxis. Two PwHA had a previous history of successful immune tolerance induction. Among the older group (n = 17), 76% were severe, 1 PwHA was treated exclusively on-demand before adjustment, none were on primary prophylaxis, and 12% had no hemophiliac arthropathy. One participant developed inhibitor during the study. Median [interquartile range, IQR] tranged from 9.5 [8.0-10.0] h, among younger individuals, to 10.5 [9.0-11.0] h, among the older ones. Three PwHA from the older group were excluded from the cost analyses: 1 developed inhibitor during follow-up, 1 was treated exclusively on-demand before PK-analysis, and 1 was prescribed plasma-derived FVIII few months after adjustment. From the remaining 34 PwHA, all individuals were advised about behavior and time of infusion, according to their bleeding phenotype, arthropaties, and physical exercise. Among younger PwHA, monthly total FVIII replacement cost increased after PK-based adjustment ($p < 0.0001$), while reducing costs of episodic therapy ($p = 0.05$) and increasing costs of prophylaxis ($p < 0.0001$). Median [IQR] ABR reduced from 3.0 [0.5-10.0] to 1.0 [0.0-2.0]. Among older PwHA, although monthly total and prophylactic costs did not change between the periods, costs of episodic treatment reduced ($p = 0.039$). ABR did not change either. PK-adjusted prophylaxis was related to increased treatment costs among PwHA < 15 years, but not among individuals ≥ 15 years. Although ABR reduced after adjustment among younger PwHA, no ABR change was evidenced among older participants.

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PREVALENCE OF HEMATURIA IN PATIENTS WITH HEMOPHILIA A AND B AND ITS ASSOCIATION WITH COMORBIDITIES: EXPERIENCE FROM A BRAZILIAN CENTER

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Background: Hemophilia A and B are X-linked inherited bleeding disorders. Hematuria is a common manifestation among patients, resulting in a worsening of quality of life. Results of previous studies regarding the association between hematuria and hemophilia have been inconsistent, leading to the need for further analysis. **Aims:** Determine the prevalence of macroscopic or microscopic hematuria in patients with hemophilia, as well as its association with comorbidities. **Methods:** Transversal unicenter study with adult patients (≥18 years old) bearing hemophilia A or B under active outpatient clinic in a reference center over the year of 2019. Hematuria was defined as any macroscopic episode or ≥3 red blood cells per high power field in urine analysis. Using simple and multivariable logistic analysis, age, diagnosis type, disease severity, comorbidities and creatinine clearance have been studied as potential characteristics associated with the prevalence of hematuria. **Results:** In total, 179 male patients were included. Mean age was 26 years (sd = 15), the majority of them were diagnosed with hemophilia A (76%) and 66% presented a severe disease. Despite our young population, the prevalence of comorbidities was high, mainly hypertension (10%), dyslipidemia (10%), diabetes (5%) and obesity (10%). The prevalence of hematuria was 39% (95% CI 32% - 47%), of these a half presented at least one macroscopic episode. Age (OR = 1.03[1.01-1.05]), hemophilia B (OR = 0.47[0.21-0.99]), hypertension (OR = 3.88[1.45-11.55]), obesity (OR = 2.92[1.11-8.24]) and creatinine clearance (OR = 0.99[0.98-1.00]) were associated with hematuria in univariable analysis. No independent factors were found in multivariable analysis. **Conclusions:** Hematuria is common among patients with hemophilia and further studies are needed to elucidate the relationship between hematuria and increased morbidity.

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PREVALÊNCIA DE AFECÇÕES HEMORRÁGICAS E OUTRAS DOENÇAS DO SANGUE E DOS ÓRGÃOS HEMATOPOIÉTICOS

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Objetivos: Descrever as internações hospitalares por afecções hemorrágicas e outras doenças do sangue e dos órgãos hematopoéticos no estado da Bahia, através da lista de morbidade do CID-10 (D65-D77) no período de 2010 a 2020, quanto aos