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

custos de hospitalização, características sociodemográficas e mortalidade. **Metodologia:** Trata-se de um estudo epidemiológico, descritivo e retrospectivo, de análise quantitativa, cuja fonte de dados foi o Sistema de Morbidade Hospitalar (SIH-SUS) do Ministério da Saúde, tabulados em gráficos e tabelas no programa Microsoft Excel 2016. **Resultados:** Foram registradas 9.066 internações por afecções hemorrágicas e outras doenças do sangue e dos órgãos hematopoéticos no estado da Bahia, com um aumento de 103,97% de 2010 a 2019 e uma redução de 25,57% entre 2019 e 2020. O tempo médio de permanência nas internações foi de 8,0 dias, com diminuição de 22,5% no período analisado, e o valor médio por internamento foi de R$752,79. A taxa de mortalidade foi de 5,67 óbitos/100 internações, com maior letalidade para o sexo feminino (55,25%) e indivíduos com mais de 60 anos. Além disso, 51,14% das internações ocorreram no sexo feminino e 35,7% ocorreram na cor parda. A faixa etária predominante foi de 1-4 anos (17,14%) seguida de 5-9 anos (11,9%). Ademais, 85,5% das internações possuíam caráter de urgência. **Discussão:** A classificação das afecções hemorrágicas e outras doenças do sangue e dos órgãos hematopoiéticos compreende um grupo vasto de enfermidades que possuem, muitas vezes, etiologia, quadro clínico, diagnóstico, tratamento e prognóstico diferentes. No período analisado, houve um aumento expressivo no número de internações por essas doenças e foi possível observar sua relevância tanto para o público entre 1-9 anos, em que nota-se a maior incidência de casos, quanto para o público com mais de 60 anos, já que essas enfermidades em questão, comumente, têm a taxa de mortalidade aumentada conforme o avanço da idade. A alta prevalência em pessoas pardas não se configura como fora do normal, por conta da constituição étnica majoritária do país e, principalmente, do estado da Bahia. Os dados que diferenciam as internações por sexo não são significativas já que a doença não se relaciona diretamente com essa variável. Além disso, o fato de a maior parte dos internamentos possuir caráter de urgência denuncia o diagnóstico tardio, o que pode estar relacionado com a elevada taxa de mortalidade. **Conclusão:** Diante disso, as estratégias de diminuição da incidência das afecções hemorrágicas e outras doenças do sangue e dos órgãos hematopoiéticos relacionam-se diretamente ao diagnóstico precoce e diferencial, a partir da compreensão das alterações clínicas e laboratoriais compatíveis com as características de cada doença. Isso pode ser realizado através de uma anamnese bem feita juntamente com interpretação dos exames laboratoriais, sem dispensar a necessidade da orientação e do acompanhamento após o estabelecimento do diagnóstico.

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RESULTS FROM A 52-WEEK, NONINTERVENTIONAL STUDY OF BRAZILIAN INDIVIDUALS WITH SEVERE HAEMOPHILIA A RECEIVING PROPHYLAXIS: RATES OF BLEEDING, FVIII USE, AND QUALITY OF LIFE

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Regular prophylaxis with exogenous Factor VIII (FVIII) is recommended for individuals with severe haemophilia A (HA), but standardised data on bleeding, FVIII use, and quality of life (QoL) for this population in routine clinical practice are scarce. In a prospective, multinational, noninterventional study, male participants age ≥18 years with severe HA (FVIII ≤1 IU/dL) receiving prophylactic FVIII were followed for at least 6 months. Bleeding and FVIII use were reported weekly. Annualised bleeding rate (ABR) and FVIII infusion rate were calculated for participants with ≥6 months follow-up and combined with retrospective data from 6 months prior to baseline. Adverse events (AEs) were collected monthly. Participants completed the haemophilia-specific health-related QoL questionnaire for adults (Haemo-QoL-A) at baseline. This abstract presents a sub analysis of the study's Brazilian population. For all 54 Brazilian participants, median (range) age was 27 (18–47) years. For the 41 (76%) Brazilian participants with ≥6 months follow-up, mean (standard deviation [SD]; median) ABR for treated bleeds was 2.42 (4.05; 0.80) bleeds per year and mean (SD; median) annualised FVIII infusion rate was 167.87 (60.22; 155.17) infusions per year. Mean (SD; median) ABRs of treated traumatic, spontaneous, and joint bleeds were 0.90 (1.6; 0.0), 1.52 (3.3; 0.0), and 1.91 (3.29; 0.0) bleeds per year, respectively. Median (range) percentage of follow-up time adherent with prescribed prophylaxis frequency in Brazilian participants was 94.7%(49%-100%). Mean (SD) Haemo-QoL-A total transformed score (0–100) score was 70.3 (15.1) The lowest mean (SD) domain scores (representing lower QoL) in all Brazilian participants were for treatment concern 45.7 (30.85), physical functioning 66.9 (21.33), and consequences of bleeding 68.1 (22.92). AEs were reported in 22 (40.7%) participants, with the most common AEs being haemophilic arthropathy, nasopharyngitis, and synovitis, each reported in 2 (3.7%) Brazilian participants. Two serious AEs (oesophageal haemorrhage and haematoma) were reported,