é um distúrbio autossômico recessivo extremamente raro, mais comum em países mediterrâneos e asiáticos, sendo a consanguinidade um dos principais fatores para a ocorrência da doença. Sua fisiopatogenia está relacionada com mutações nos genes LMAN1 e MCFD2 que fazem parte da cascata intracelular na formação destes fatores. O diagnóstico se firma com a confirmação da deficiência através da aferição dos fatores V e VIII, sendo geralmente 10 a 20% do valor normal. Os sintomas geralmente são leves e incluem frequentemente sangramento excessivo durante ou após trauma, cirurgia e trabalho de parto, além de epistaxe, sangramento gengival, hematomas e menorragia. O tratamento depende da gravidade do sangramento, podendo utilizar plasma fresco congelado e concentrado de FVIII ou desmopressina. **Conclusão:** Assim, mesmo se tratando de um distúrbio muito incomum, a deficiência combinada de fator V e fator VIII deve ser um dos diagnósticos diferenciais em pacientes com histórico de sangramento, principalmente em mulheres jovens com metrorragia. Ademais, é de suma importância o acompanhamento e a orientação destes casos, a fim de evitar grandes sangramentos e de agregar uma maior qualidade de vida aos pacientes.

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DRAWBACKS OF PERSONALIZATION OF PROPHYLAXIS AMONG ADULT MEN WITH HEMOPHILIA A

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Personalization of prophylaxis applying population pharmacokinetic (PK) tools has recently rendered fewer bleeding episodes for people with hemophilia A (PwHA). However, despite PK data (e.g., terminal half-life – t– and clearance), many other variables should be considered when proposing an individualized replacement therapy, such as bleeding phenotype, arthropathy, physical exercise, and adherence to treatment. All these should be considered, especially among adult PwHA who have lived for many years with exclusive episodic treatment. We evaluated factor VIII (FVIII) consumption among adult PwHA who had been treated with exclusive episodic therapy and had changed to prophylaxis, when personalization was provided. Men with severe (FVIII < 1%) or moderate (FVIII 1%-5%) HA and inhibitor negative who were treated at the Universidade Estadual de Maringá (Maringá/Brazil) signed the Consent Form to participate in the study. Anthropometric and hemophilia-related data were obtained using a standardized form. PK analysis was performed according to the WAPPS-Hemo tool, based on two blood samples (3 and 24 h after infusion and one-step coagulometric test). After PK analysis, PwHA were approached to adjust their regimen (time, dose, and frequency of infusion) according to bleeding phenotype, arthropathy, physical exercise, and adherence to treatment. Bleeding episodes, regimen, and FVIII disposal (from now called consumption) were assessed 12 months before and after treatment adjustment. The estimated FVIII consumption was calculated by multiplying the daily dose by the number of infusions in a month, based on the prescription. The percentage of the quotient between the actual FVIII consumption per the estimated FVIII consumption for prophylaxis was considered a proxy for adherence to treatment. Plasma-derived FVIII (Beriate®, CSL Behring, and Fahndi®, Grifols) were prescribed during the 2-year analysis. Five men with HA (2 receiving Beriate®, 1 moderate and 1 severe; and 3 receiving Fanhdi®, 2 moderate and 1 severe) were included. All PwHA received exclusive on-demand infusions for at least 30 years and tertiary prophylaxis was started in the last 10 years. They presented hemophiliac arthropathy, ranging from 1 to 6 affected joints. None practiced physical exercise. None had HIV and 3 had HCV. PK analysis was performed at ages ranging from 38 to 61 years and twere 10.3 and 16.5 h for those receiving Beriate®and 8.8 to 13.5 h for those receiving Fanhdi®. All PwHA were advised about the best time to infuse FVIII, but only 2 PwHA had an increment of their regimens. Pre-adjustment PK-estimated trough levels varied 0.0 to 1.2 IU/dL from baseline which we had access. During this period, the monthly actual FVIII consumption for prophylaxis ranged from 12.8% to 74.5% of the estimated FVIII consumption. During the year following the adjustment, this relation increased from 28.0% to 93.8%. However, the total annual bleedings did not change between these moments. We believe personalization of therapy is important to guarantee joint health and quality of life of PwHA. However, based on the analyses of the individual cases we reported, adherence to treatment may be an important impacting factor to consider among adult PwHA.

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ELECTROCARDIOGRAPHIC ALTERATIONS AMONG ASYMPTOMATIC ADULTS WITH HAEMOPHILIA

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