

Objectives: This study aims to compare clinical parameters and outcomes between patients with aplastic anaemia undergoing immunosuppressive treatment (IST) who received more than 10 units of blood components prior to IST and those who did not.

Methods and materials: This cross-sectional study included patients diagnosed with aplastic anaemia and treated at Hospital Geral de Fortaleza. The treatment regimen comprised anti-thymocyte globulin, cyclosporine, and, in some cases, eltrombopag. Data were collected on various clinical parameters, including age, time to IST, haemoglobin (Hb), white blood cell count (Wbc), neutrophils (Neut), lymphocytes (Linf), platelet count (Plat), creatinine (Cr), urea (Ur), and lactate dehydrogenase (LDH). Patients were divided into two groups based on whether they received more than 10 transfusions before IST. Variables were summarised using medians and interquartile ranges (IQR). Comparisons between groups were evaluated using non-parametric tests, specifically the Mann-Whitney U-test, with p-values < 0.05 considered statistically significant. Overall survival (OS) was assessed using the Kaplan-Meier method, with a 95% confidence interval and p-values < 0.05 deemed statistically significant. **Results:** Between 2021 and 2024, 11 patients were diagnosed with aplastic anaemia and deemed suitable for IST. Patients who did not receive more than 10 transfusions had a median age of 20 years (IQR: 20-26.5), compared to 40.5 years (IQR: 35.5-42) for those who did. Time to IST was shorter for the transfusion group, with a median of 21 days (IQR: 11.5-25) versus 44 days (IQR: 34-54) for the non-transfusion group. The transfusion group had lower median levels of Hb (4.8 g/dL), WBC (1250 cells/ μ L), Neut (77 cells/ μ L), and plat (10500 cells/ μ L). Cr and urea levels were slightly higher in the transfusion group, with medians of 0.88 mg/dL and 35 mg/dL, respectively. LDH data were unavailable for the transfusion group. Only two patients received eltrombopag. There was no significant difference between the two groups in terms of age, blood count values at diagnosis, and sex (U-test > 0.05). Overall survival was 75%, with better outcomes in patients who had fewer than 10 transfusions, although this difference was not statistically significant (p-value: 0.257). **Discussion:** The analysis revealed significant clinical differences between the two groups. Patients who received more transfusions were older, had a shorter time to treatment initiation, and exhibited worse cytopenias. Although Cr and Ur levels were slightly higher in the group with more transfusions, LDH data were unavailable. Only two patients received eltrombopag, and there was no significant difference between the groups in terms of age, blood count values at diagnosis, and sex. The overall survival rate was 75%, with better outcomes in patients who had fewer transfusions, although this difference was not statistically significant. These findings suggest that multiple transfusions before IST may be associated with a more severe clinical profile, yet they do not significantly impact overall survival. **Conclusion:** The overall survival observed in this study was lower than international data. There was no significant difference between the groups analysed; however, the low patient count in the study should be considered when interpreting the lack of impact on outcomes. The use of IST with eltrombopag should be evaluated sequentially to determine its impact on individuals treated within the public health system.

PANCITOPENIA SECUNDÁRIA À ANEMIA DE FANCONI: RELATO DE CASO

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Introdução: Anemia de Fanconi (AF) é uma rara doença genética, multissistêmica, caracterizada por insuficiência da medula óssea e consequente pancitopenia, malformações somáticas e predisposição a cânceres, como leucemias agudas em 13 % dos casos até os 50 anos de idade e lesões em cabeça e pescoço. O diagnóstico é feito através da análise genética das mutações no gene FANCL, associado com quadro clínico e evidência de pancitopenia em biópsia de medula óssea. Relato de caso: feminino, 29 anos, foi encaminhada ao serviço de hematologia do IMIP-PE por quadro de pancitopenia e passado de síndrome HELLP precoce. Em antecedentes familiares, apresenta irmão falecido por Anemia de Fanconi, irmã falecida aos 15 anos de idade por neoplasia gástrica e irmão falecido aos 35 anos por neoplasia a nível de sistema nervoso central. Realizada investigação inicial, sem anormalidades, com os seguintes exames: dosagem de vitamina B12, cinética do ferro, pesquisa de HPN, sorologias para hepatite B, C, HIV, sífilis, citomegalovirose, Herpes, doença de chagas, pesquisa para doenças tireoidianas e reumatológicas. Procedida pesquisa com deb test, sendo esta positiva para o gene FANCL com heterozigose de 50,88%, confirmando o diagnóstico de AF. Após o diagnóstico, paciente vem apresentando quadro de fraqueza muscular progressiva, com hiperreflexia e babinski positivo em ambos os MMII, com RNM evidenciando lesões intra axiais sem etiologia definida. Optada por biópsia das lesões, pendente anatomopatológico. **Discussão:** Tratamento de escolha, quando disponível, é o transplante de medula óssea alogênico com depleção linfocitária, sendo a única modalidade com potencial curativo. Androgênios são usados para manejo da anemia e plaquetopenia, com taxas de resposta individuais de 50%. O rastreio periódico de neoplasias secundárias e seu tratamento é imperativo. O rastreio familiar, como no caso em questão, especialmente nas mutações ligadas ao X, também é recomendado. **Conclusão:** A anemia de fanconi, apesar de congênita, pode ter diagnóstico tardio, sendo de suma importância o deb test. Desse modo, deve-se lembrar da apresentação de pancitopenia sem etiologia clara em pacientes jovens. No caso em questão, a paciente apresenta sinais e sintomas neurológicos sem definição de diagnóstico até o momento, sendo esta portanto, outra provável manifestação da doença.

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