durante o Transplante de Células Hematopoiéticas (TCH). O objetivo deste trabalho foi comparar os fatores de risco para a MO e o desequilíbrio nutricional durante o TCH em pacientes pediátricos com Doenças não Malignas (DNM) e Doenças Malignas (DM). Foram selecionados 132 prontuários de pacientes pediátricos com idades entre 0 e 18 anos, que foram submetidos a TCH, tratados no Centro de Transplante de Medula Óssea do Hospital Israelita Albert Einstein, entre os anos de 2012 e 2019. Foram coletados dados, incluindo dentre outras informações: idade, sexo, doença primária, tipo de transplante, regime de condicionamento, profilaxia para Doença do Enxerto Contra o Hospedeiro (DECH), toxicidade gastrointestinal, gradação de mucosite oral, porcentagem de perda e ganho de peso corporal, reposição nutricional e Sobrevida Global (SG). Os dados foram comparados entre pacientes com DNM (n = 70) e DM (n = 62). Observou-se que a MO teve gravidade semelhante entre os dois grupos. O principal fator de risco no grupo DNM foi o regime de condicionamento com bussulfano, enquanto no grupo DM foi a profilaxia para DECH com ciclosporina e metotrexato. A MO não teve impacto na perda ou ganho de peso corporal em nenhum dos grupos. No grupo DNM, o ganho de peso corporal devido à sobrecarga de fluidos foi mais pronunciado e associado a uma faixa etária menor. A OS foi semelhante entre os grupos e não foi afetada pela MO. Conclui-se que o padrão de MO foi semelhante em pacientes pediátricos com ou sem DM, mas os fatores que determinaram essas lesões orais foram diferentes. Houve disparidades nas mudanças de peso corporal entre os dois grupos, e essas mudanças não foram associadas à MO.

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GVHD PROPHYLAXIS WITH METHOTREXATE IN HAPLOIDENTICAL HCT USING POSTTRANSPLANT CYCLOPHOSPHAMIDE: A PHASE IB/II CLINICAL TRIAL (NCT04622956)

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Introduction: In haploidentical hematopoietic cell transplantation (Haplo-HCT), the prevailing Graft-Versus-Host Disease (GVHD) prophylaxis in Brazil consists of posttransplant cyclophosphamide (PTCY) with cyclosporine and Mycophenolate Mofetil (MMF). While comparisons between MMF and Methotrexate (MTX) for GVHD prophylaxis have sparked debate in other donor types, some large studies indicate that MTX is linked to a reduced risk of GVHD and improved long-term outcomes. It's worth noting that MMF, a potent inhibitor of NK cells, could potentially interfere with the graft-versus-leukemia effect in Haplo-HCT. Additionally, IV MMF is not available in Brazil, which poses challenges for patients undergoing HCT. **Objective:** Our aim was to conduct a phase Ib/II study to assess the efficacy of MTX in adult patients with hematologic malignancies undergoing Haplo-HCT with PTCY. Herein, we present the findings from the phase I portion of this investigation. **Methods:** This ongoing single-arm, multicenter phase 1/2 study evaluates MTX administered on D+6 and +9 alongside PTCY at 50 mg/kg/day on D+3 and +4, with cyclosporine starting on day +5 until D +90 or beyond in the event of GVHD. The phase 1 part enrolled eligible adult patients with hematologic malignancies undegoing myeloablative Haplo-HCT. A 3+3 escalation design was employed to assess the safety and tolerability of three MTX dose levels: 0 (10 mg/m^2 on D+6 and 7.5 mg/m^2 on D+9), +1 (10 mg/m^2 on D +6 and +9), and +2 (15 mg/m^2 on D+6 and 10 mg/m^2 on D+9). The primary objective was to determine the MTX dose for the phase 2 part of the study. **Results:** A total of 15 patients were enrolled: 6 at Level 0, 6 at Level +1, and 3 at Level +2. Most patients had acute leukemia or chronic myeloid leukemia. All patients received mobilized peripheral stem cells, except for two patients in the Level +1 group who received bone marrow grafts. Dose-Limiting Toxicities (DLTs) included transient increases in ALT/AST or total bilirubin at Level 0 and Level +1. The Maximum Tolerated Dose (MTD) was not reached. Febrile neutropenia and grade 3–4 transplant-related toxicities occurred in 6/6, 4/6, and 3/3 patients at Levels 0, +1, and +2, respectively. The rates of grade 3-4 infectious complications, excluding febrile neutropenia and CMV reactivation, were 0.43 and 0.21, 0.37 and 0.34, and 0.23 and 0.46 events per patient per 100 days at risk at Levels 0, +1, and +2, respectively. Other adverse events were consistent with this patient population. Neutrophil engraftment was achieved in all patients, with median times of 19.5 days (range 17–25), 19 days (17–22), and 20 days (17–22) for Levels 0, +1, and +2, respectively. Grades 2 and 3 acute GVHD up to day +90 occurred in 3 (50%)/2 (33%), 1 (17%)/1 (17%), and 1 (33%)/1 (33%) patients at Levels 0, +1, and +2, respectively. Moderate to severe chronic GVHD was diagnosed in one patient (17%) at Level 0 and two patients (34%) at Level +1. One patient at Level 0 with ALL who had prior anthracycline exposure developed heart failure on day +6 and died on day +56. Another patient at Level +2 experienced disease relapse and died. To date, all other patients are alive and remain disease-free. **Conclusions:** GVHD prophylaxis with MTX in Haplo-PTCY appears to be safe and well-tolerated in this myeloablative HCT population, with no MTD reached. These findings support the continuation of the phase II portion of the study, which is currently accruing with MTX doses of 15 mg/m^2 on day +6 and 10 mg/m^2 on day +9. Updated data will be presented at the forthcoming meeting.

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