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EFFECTIVENESS OF DEFIBROTIDE FOR PROPHYLAXIS AND TREATMENT OF SINUSOIDAL OBSTRUCTION SYNDROME IN CHILDREN: A SYSTEMATIC REVIEW OF CLINICAL STUDIES

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Introduction. Sinusoidal obstruction syndrome (SOS), previously known as veno-occlusive disease, is a rare life-threatening complication of chemotherapy, mostly associated with hematopoietic stem cell transplantation. SOS occurs in approximately 22% of children and 30% of infants undergoing treatment for oncological diseases, which is 2-3 times more often than in adults. Defibrotide (DF) is currently the only drug that is approved for the treatment of SOS, but due to the rarity of the disease and lack of prospective studies, the evidence of DF efficiency remains uncertain. Several studies suggest the benefit of SOS prophylaxis with DF.

Objective. Systematically assess the effectiveness of the use of DF in children for prophylaxis and treatment of SOS in clinical trials, published within the last 10 years.

Methods. Literature search in PubMed and Google Scholar was performed between the 1st of November 2023 and the 1st of December 2023. We used “defibrotide”, “sinusoidal obstruction syndrome”, “veno-occlusive disease”, “pediatric” and “children” as keywords. The systematic review was performed in accordance with the Preferred Reporting Items for Systematic Reviews and Meta-Analyses (PRISMA) guidelines. Case reports, reviews, meta-analyses, studies published more than 10 years ago and studies with no children were excluded.

Results. 15 publications – 10 retrospective studies, 3 prospective studies, one randomized clinical trial, and one controlled clinical trial - met the inclusion criteria and were analyzed in this review. In total, 7655 patients were included into the studies, with 3121 (40,7%) children <18 y. Eight studies evaluated only SOS treatment with DF, four studies described only SOS prophylaxis with DF, and three studies - both prophylaxis and treatment. The DF dose range was between 12,5 and 60,0 mg/kg/day, with the mean dosage of 25 mg/kg/day. Three studies showed the benefit of SOS prophylaxis with DF while three studies concluded that the prophylaxis is not useful. The studies evaluating DF treatment reported overall survival rates ranging from 25% to 90.4%. All studies supported the benefit of DF for the treatment of SOS.

Conclusions. This systematic review suggests that the treatment of SOS with DF in children is effective and should be applied in clinical practice. However, the most recent studies do not show enough evidence that SOS prophylaxis with DF has significant benefits.

Keywords: sinusoidal obstruction syndrome, veno-occlusive disease, pediatric, defibrotide, hematopoietic stem cell transplantation