Successful first-line treatment of hemophagocytic lymphohistiocytosis with ruxolitinib in a pediatric patient with trisomy 21

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Abstract

Hemophagocytic lymphohistiocytosis (HLH) is an uncommon immunologic disorder associated with high rates of morbidity and mortality characterized by systemic inflammation and multiorgan dysfunction. The standard of care for primary treatment of HLH is chemotherapy (i.e. etoposide), but consideration of alternative therapies is warranted to support treatment goals for critically ill pediatric patients. We present the case of a 7-year-old male with trisomy 21, acute multiorgan failure secondary to infection, and subsequent HLH who was successfully treated with ruxolitinib. This represents the first use of ruxolitinib as a first-line agent for secondary HLH in a critically ill child with trisomy 21.

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