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Efficacy of thrombopoietin receptor agonists in patients with immune thrombocytopenia and megakaryocytes with myelodysplastic features

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Immune Thrombocytopenia (ITP) is a diagnosis of exclusion that may require bone marrow (BM) examination to rule out a unilinear myelodysplastic syndrome (MDS). In a few cases, BM examination shows signs of MK dysplasia with no other criteria compatible with MDS. We performed a retrospective analysis on 16 patients diagnosed with ITP from 2011 to 2021 of median age at diagnosis 58.5y, M:F ratio of 1.3:1 whose BM examination showed signs of MK dysplasia, treated with thrombopoietin receptor agonists (TPO-RAs) Eltrombopag (ETP, n=8) or Romiplostim (RPS, n=8) as 2nd (or further) line treatment, from 2016 to 2021. All patients included that were not treated with TPO-RAs on first relapse ultimately developed resistance or intolerance to steroids, some of them were also unresponsive to Rituximab and splenectomy. We evaluated response to TPO-RAs at 2 weeks, 3 months, 6 months and 1 year from start of treatment. Three patients started TPO-RAs in 2nd line. Twelve patients started TPO-RAs in 3rd or further lines due to loss of response to previous treatments. Two patients treated with ETP experienced sustained response. Limited to the small population sample, this study suggests the efficacy of long-term treatment with TPO-RAs in patients with underlying MK myelodysplastic features in the BM observed at diagnosis. The overall response to immunosuppressive treatments such as Rituximab or steroids appears to be short lived and hard to maintain in the analyzed cohort, thus suggesting a role of MK dysplasia in the mechanism of thrombocytopenia in addition to autoimmunity. Due to their mechanism of action, TPO-RAs actually represent the optimal treatment for these patients, as they can stimulate thrombopoiesis while also inducing a degree of immunotolerance. Furthermore, this study shows the possibility of a sustained response in patients affected by ITP with signs of MK dysplasia.

Biography

Riccardo Tomasello is currently a third-year resident in haematology since 2019 at the University of Palermo. He completed his medical studies at the University of Palermo in 2018. The main research field are autoimmune hematological diseases, primarily immune thrombocytopenia, but he is also interested in other clinical research on various other hemostasis and platelets diseases. He is a member of the Italian thrombosis and hemostasis society SISET.