CLINICAL BENEFITS OF ROPEGINTERFERON ALFA-2B IN THE TREATMENT OF YOUNG HIGH-RISK PATIENTS WITH ESSENTIAL THROMBOCYTHEMIA: SINGLE CENTER EXPERIENCE

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Introduction: Despite Interferon's (IFNs) well known effectiveness in the treatment of patients with BCR-ABL1- negative myeloproliferative neoplasms (MPNs), its use has been hampered in recent decades because of its side effects and lack of official regulatory approval for this specific indication. However, due to its already established ability to induce not only hematologic but also molecular responses in this setting of patients, the introduction of novel pegylated IFN formulations in recent years, renewed the interest. Ropeginterferon alfa-2b has emerged as an important therapeutic tool, currently approved only for treatment of patients with Polycythaemia Vera (PV). Nevertheless, many studies are evaluating its efficiency in Essential Thrombocythemia (ET) and Primary Myelofibrosis (PMF) as well, with final intention to expand its usage. In order to further clarify these observations, we present our experience with ropeginterferon alfa-2b in 16 young patients with MPNs, diagnosed and treated at the University clinic of hematology in Skopje, R. of North Macedonia.

Material and Methods: This is a retrospective observational study in which 16 patients with BCR-ABL negative MPN, treated with ropeginterferon alfa-2b were included. Responses were evaluated by ELN, IWG-MET and EUMNET standardized criteria sets and occurrence of side effects was recorded.

Results: In our cohort 11pts were female (69%), with average age at diagnosis at 36 years (17-51). In terms of diagnosis, 12 patients had ET, one had PV and three patients had hypercellular phase of PMF. According to mutational status, JAK2V617F mutation was detected in 10 patients (62.5%), CALR mutation in three (19%), and three patients were designated as triple-negative cases (19%). Decision for treatment was made using the revised IPSET score by which they were classified as high risk patients. Bone marrow biopsy before start of treatment was performed in all of the patients in order to establish the eventual presence and grade of fibrosis. Splenomegaly was present in four patients (25%) while three patients underwent splenectomy because of splenic vein thrombosis. In seven pts (43.7%) of which five ET, one PV, and one with prefibrotic phase of PMF, ropeginterferon alfa-2b was used as first line treatment. The other nine pts. were previously treated with another therapy, Interferon-α and hydroxyurea. Evaluation of clinicohematologic response was performed every two weeks along with the drug administration. Complete hematological response was observed in 12 patients (75%), with average time to blood count normalization at 8 weeks using average dose of 150mcg (100-250). In terms of side effects only flu-like symptoms were observed in one patient, without need of drug discontinuation.

Conclusion: Our study confirmed that ropeginterferon alfa-2b is a powerful therapeutic tool in this setting of patients and supports the need to expand its use in the treatment of young high risk patients with ET and PMF.

Keywords: myeloproliferative neoplasm, essential thrombocythemia, polycythaemia vera, ropeginterferon, primary myelofibrosis