Research Article

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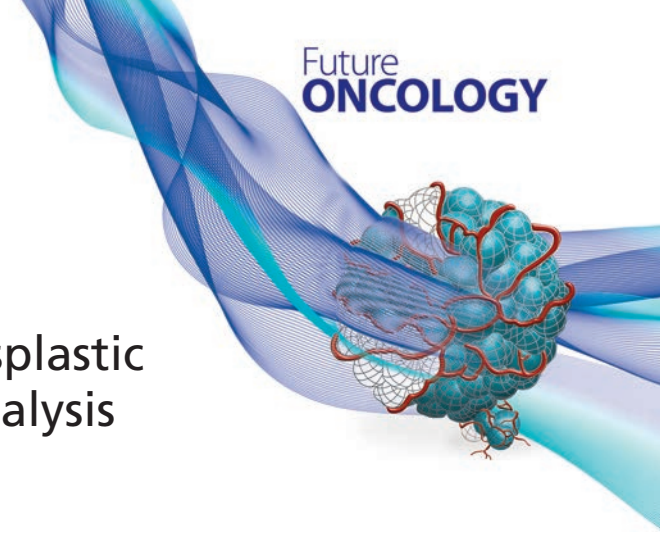

Real-world assessment of myelodysplastic syndrome: Japanese claims data analysis

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Aim: To describe the treatment landscape and associated economic burden for myelodysplastic syndrome in Japan. **Methods:** We studied nationwide retrospective claims data from 2008 to 2019. The study cohort was categorized into patients receiving transfusion, erythropoiesis-stimulating agent, erythropoiesis-stimulating agent + transfusion, azacitidine, azacitidine + transfusion and others. **Results:** Our study found that the azacitidine + transfusion group had the highest medical cost and severity of disease compared with the other groups. In those patients, healthcare resource utilization and the costs of transfusions, including iron chelation therapy, increased medical costs. **Conclusion:** Our retrospective analysis provides a current snapshot of real-world treatment patterns and associated incremental economic costs of iron chelation therapy with the presence of transfusions that drive an increase in total costs.

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Myelodysplastic syndromes (MDS) are groups of clonal hematopoietic malignancies that manifest as ineffective function of hematopoietic stem cells and anemia [1]. MDS primarily affects the elderly, with 86% of patients being ≥60 years old at the time of diagnosis and a reported median age of 76 years [2]. There is unmet medical need for transfusion-dependent (TD) MDS in which transfusion dependency is considered to be a negative prognostic factor; TD patients have a shorter median survival time than non-transfusion-dependent disease, and a primary goal of treatment is to maintain patient quality of life [3]. While Japan is an aging society, an up-to-date Japanese treatment landscape for lower-risk TD and non-TD MDS, as well as related economic outcomes, remains largely undefined due to limited real-world data.

According to the most recent estimates, the crude incidence rate of MDS in Japan increased 12-fold from 0.2 cases per 100,000 patients in 1993 to 3.8 cases per 100,000 male (95% CI: 3.6–4.1) and 2.4 cases per 100,000 female (95% CI: 2.2–2.6) in 2008 [4]. Analysis by the International Working Group for Prognosis of MDS showed that Japanese patients were younger with more severe cytopenia and unique genetic disease markers as compared with Caucasian patients [5].

Recommendations for MDS treatments vary depending on the presence of risk factors such as age, cytogenetic abnormalities and comorbidities. Lower-risk patients may receive blood transfusion as standard supportive care for anemia [6,7]. Cytokine therapy (e.g., erythropoiesis-stimulating agent [ESA]) for anemia, or lenalidomide therapy for patients with chromosome 5q deletion, are recommended to improve transfusion independence [8]. Patients with higher-risk disease are recommended to undergo allogeneic hematopoietic stem cell transplant (allo-HSCT) if possible, with a hypomethylating agent (HMA) such as azacitidine (AZA) or decitabine acting as a bridge to transplantation [7]. Transplantation is the only durable cure for MDS, but less than 5% of MDS patients are reported to be eligible [9].

Furthermore, clinical outcomes including overall survival (OS) marked a median survival of 55 months for patients who underwent allo-HSCT versus 26 months for patients who did not receive such treatment [10]. Patients

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