

[2617] The Incidence and Impact of Thrombocytopenia in Myelodysplastic Syndrome (MDS).

Session Type: Poster Session, Board #795-II

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Background: MDS is a heterogeneous stem cell malignancy characterized by ineffective hematopoiesis and increased risk of transformation to acute myeloid leukemia. Thrombocytopenia and platelet dysfunction are recognized to contribute to hemorrhagic complications, however, reliable data regarding the frequency and consequences of thrombocytopenia in MDS are lacking. *Objective:* To assess thrombocytopenia in MDS based on literature and chart review. *Methods:* MDS literature (January 1980 to November 2005) was identified using MEDLINE, EMBASE, and Cochrane databases, society websites, and hand searches of bibliographies. In addition, a retrospective review of all patients referred to the MD Anderson Cancer Center (MDACC) with MDS since 1980 was performed. *Results:* A total of 85 references were identified describing clinical consequences of thrombocytopenia, n=16; efficacy and safety of MDS therapies, n=60; guidelines, n=9. Because of variable definitions of thrombocytopenia, a platelet count $<100 \times 10^9/L$ was applied as the threshold point in the literature analysis. The prevalence of thrombocytopenia in MDS ranged from 40% to 65%. Baseline thrombocytopenia rates were reported in 19/60 (32%) references; the median frequency of thrombocytopenia prior to MDS therapy was 65% (range, 23% to 93%). The MDACC database review identified 1605/2410 (67%) patients with thrombocytopenia ($<100 \times 10^9/L$) at referral. Severe thrombocytopenia ($<20 \times 10^9/L$) was observed in 425 (18%) patients. Using the International Prognostic Scoring System (IPSS), 399/2410 (17%) patients had intermediate-2 or high risk disease; 83% and 25% of these patients had platelet counts of $<100 \times 10^9/L$ and $<20 \times 10^9/L$, respectively. Corresponding event frequencies decreased to 21% and 3% in patients with low or intermediate-1 risk MDS (264/2410, 11%). The reported incidence of MDS treatment-related thrombocytopenia was $>50\%$ in studies involving lenalidomide, azacitidine, or a combination of idarubicin, cytarabine and topotecan. Treatment-related thrombocytopenia was also observed in studies involving tipifarnib, linomide, decitabine, all-trans retinoic acid, and sirolimus. The reported incidence of hemorrhagic complications ranged from 3% to 53%, and the frequency of hemorrhagic deaths ranged from 14% to 24%. At MDACC, 460 patients had a coded cause of death: hemorrhage as a cause of death, 20%; hemorrhage as the only cause of death, 10%. *Conclusions:* Thrombocytopenia is common in MDS with a higher prevalence in higher risk IPSS categories. Many approved and investigational MDS therapies cause or exacerbate pre-existing thrombocytopenia. The incidence of severe bleeding in MDS was higher than reported in current guidelines or reviews. Abstract #2617 appears in Blood, Volume 108, issue 11, November 16, 2006

Keywords: Platelet count|Bleeding|Thrombopoiesis

Sunday, December 10, 2006 9:00 AM

Poster Session: Myelodysplastic Syndromes: Diagnosis, Classification, and Prognosis (9:00 AM-8:00 PM)