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Lenalidomide for the Treatment of Low- and Int-1-Risk MDS with Del(5q): Efficacy and Quality of Life Study.

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Abstract

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Introduction:

Chronic anemia of myelodysplastic syndromes (MDS) is associated with poor quality of life (QoL) and an inferior clinical course. Transfusion dependence in lower-risk patients is associated with reduced survival as a result of iron overload, heart failure, and progression to acute myeloid leukaemia. Lenalidomide is

approved for the treatment of transfusion-dependent anemia in patients with International Prognostic Scoring System (IPSS) Low- or Intermediate (Int)-1-risk MDS with deletion 5q [del(5q)]. Rapid and durable responses include transfusion independence with a rise in Hb, suppression of the del(5q) clone, and improvement in bone marrow morphological features. We present preliminary results of a prospective single-arm trial investigating the effect on QoL, efficacy, and safety of lenalidomide in the treatment of 49 adult patients with IPSS Low- and Int-1-risk MDS with del(5q) with/without additional cytogenetic abnormalities and Hb < 10 g/dL.

Methods:

Exclusion criteria include: ANC < 500/mm3; PLT count < 50,000/mm3; prior chemotherapy; and ongoing treatment with rHuEpo. Lenalidomide was administered orally at a starting dose of 10 mg/day. If necessary, dosing was reduced to 5 mg/day or 5 mg on alternate days. Treatment will be continued for a maximum of 12 months or until evidence of unacceptable non-hematological adverse events, lack of response, disease progression, or relapse following erythroid improvement. QoL was assessed at study entry and weeks 8, 12, and 24 using the QOL-E v.2 questionnaire. QoL scores are standardized in a 0–100 scale with lower scores representing a worse QoL. Response was evaluated according to the modified International Working Group (IWG) response criteria.

Results:

Twenty patients (5 M, 15 F, mean age 72 ± 10 years) are evaluable for erythroid responses and cytogenetic changes at 12 weeks and 13 patients have reached a 24-week follow-up. At baseline, mean disease duration was 3.4 ± 2.3 years. Seventeen patients were transfusion dependent (TD), 3 were transfusion free (TF). ECOG performance status was 0 in 14 patients and 1 in 6 patients. After 12 weeks from study entry, 17 (85%) patients obtained an erythroid response with a mean Hb level increase from baseline 8.6 ± 0.9 g/dL to 11.1 ± 2.4 (p=0.001). By 24 weeks, 11 of the 13 patients re-evaluated were erythroid responders obtaining transfusion independence and significant improvements in Hb (mean change from baseline 3.7 ± 2.7 g/dL, and increase to mean 11.1 ± 2.4 g/dL (p<0.001). Eight out of 20 cases (35%) reached normal Hb levels after 12 weeks and 8 out of 13 patients (62%) by 24 weeks.
A cytogenetic response (at least 50% reduction in del[5q]) was observed in 5 responders out of 13 patients evaluated at 24 weeks. Additional cytogenetic abnormalities were observed in 4 responders.
A progressive improvement in QoL was experienced in responders in the first 24 weeks of treatment. Physical QoL scores increased from 35 ± 9 at baseline to 69 ± 25 at week 24 (p = 0.086). Social-QoL scores significantly changed from 29 ± 20 at baseline to 83 ± 20 at week 12 (p = 0.021). Changes in physical QoL correlated with improvements in Hb (r = 0.768, p=0.001). Drug interruption followed by reduction to 5 mg/day was required in 16 patients within the first 8 weeks due to significant neutropenia,

which was associated with thrombocytopenia in 3 patients and hospitalization because of infection in 2 patients. One patient withdrew from treatment because of progressive anemia.

Conclusions:

Preliminary results confirm that in Low- and Int-1-risk MDS patients with del(5q) lenalidomide induces clinically significant erythroid responses and transfusion independence. Most patients require a dose reduction mainly due to neutropenia. Responders experience improvements in physical and social QoL.

Disclosures:

Oliva: *Celgene:* Consultancy. **Finelli:** *Celgene:* Consultancy.

Author notes

* Asterisk with author names denotes non-ASH members.