Pharmacotherapeutic Report on Hydroxycarbamide (Siklos®) for the Indication Sickle Cell Disease

Summary

The Medicinal Products Reimbursement Committee has issued a report for the medicinal product hydroxy carbamide (Siklos®). In order to determine the therapeutic value of hydroxy carbamide, the medication has been compared to best supportive care. The committee has concluded the following:

Treatment with hydroxy carbamide may accomplish a reduction of the number of (hospitalizations by) crises, like the acute chest syndrome, for patients with three or more crises per year in comparison to treatment with placebo. It has not been shown whether hydroxy carbamide has an effect on survival.

The main adverse effect of hydroxy carbamide is dose-dependent bone marrow suppression (especially neutropenia). Cases of azoöspermia or oligospermia may occur incidentally. Long term usage does not appear to result in cumulative toxicity. It is not know what the effects on the development of malignancies (especially leukemia) and the growth and development of children will be in the long run. These effects need to be examined more extensively. Presently, the incidence towards the development of malignancies has not yet increased.

The experience with hydroxy carbamide for the indication sickle cell disease is limited.

It is not permitted to administer hydroxy carbamide to patients with serious loss of liver or renal function. Usage is discouraged during conception, pregnancy, lactation and for children of 2 years of age or younger. Also, hydroxy carbamide should not be administered when myelosuppression has reached toxic levels.

In Conclusion

Preventive usage of hydroxycarbamide has a therapeutic advantage when compared to best supportive care in the treatment of sickle cell disease for patients who had three or more vaso-occlusive crises in the previous year.

Recommendations by the CFH

Administration of hydroxycarbamide reduces the number of (hospitalizations by) vaso-occlusive crises, such as the acute chest syndrome, in patients with sickle cell disease (with three or more vaso-occlusive crises in the previous year) by approximately half. The main adverse effect is bone marrow suppression. Clinical studies have not shown a significant effect on survival.