Deferoxamine

Essential medicine status

Section: 10. Medicines affecting the blood > 10.3. Other medicines for haemoglobinopathies

EMLc | ATC codes: V03AC01

Indication	Other specified sickle cell disorders or other haemoglobinopathies — ICD11 code: 3A51.Y
INN	Deferoxamine
Medicine type	Chemical agent
List type	Complementary Deferasirox oral form may be an alternative, depending on cost and availability.
Formulations	Parenteral > General injections > unspecified: 500 mg in vial powder for injection (mesilate)
EML status history	First added in 2011 (TRS 965)
Sex	All
Age	Also recommended for children
Therapeutic equivalence	The recommendation is for this specific medicine
Patent information	Patents have expired in most jurisdictions Read more about patents.
Wikipedia	Deferoxamine
DrugBank	Deferoxamine

Summary of evidence and Expert Committee recommendations

The Committee recognized that sickle-cell disease (SCD) is an important public health problem in many parts of the world. The Committee noted that treatment with hydroxycarbamide can significantly decrease the incidence of painful crises and can be effective in the treatment of acute chest syndrome, priapism, and in reducing overall mortality in adult patients (1, 2). The Committee noted that evidence from a systematic review (3) supported the safety and efficacy of hydroxycarbamide for the treatment of SCD in children aged 1 to 14 years. The Committee noted that although hydroxycarbamide is potentially mutagenic and carcinogenic, there are no definitive data to suggest that the incidence of malignancy is increased in adult SCD patients who receive hydroxycarbamide. The Committee concluded that the risk of death due to SCD-related complications is greater than the potential for hydroxycarbamide induced leukaemia in adults. The Committee noted that there are no evidence-based guidelines for the treatment of SCD-associated acute pain episodes, but the commonly used medicines for pain management included NSAIMs, such as ibuprofen, paracetamol, and morphine and these are already included in the WHO Model List. Deferoxamine is used to treat iron overload resulting from chronic red blood cell infusion in SCD patients with acute chest syndrome, refractory pain, or following a stroke and is already listed on the current Model List. Deferoxamine would be added under this new section, with a note about oral alternatives. The Committee noted that prophylactic penicillin is recommended for the prevention of infections by S. pneumoniae in children with SCD (4) and that pneumococcal vaccination should be given to children with SCD to reduce the risk of bacteraemia with S. pneumoniae. Penicillin and pneumococcal vaccine are both listed in the current EML. Although the Committee expressed concern about the addition of a new section specifically for sickle-cell disease, it was concerned about this selection being less visible if listed in Section 8.2 Cytotoxic medicines. The Committee therefore decided to add a subsection under Section 10, as 10.3 Other medicines for haemoglobinopathies and to list hydroxycarbamide for adults and children, based on the evidence of its safety and efficacy. Hydroxycarbamide would be added to the Complementary List. 1. Steinberg MH et al. Effect of hydroxyurea on mortality and morbidity in adult sickle cell anemia. Risks and benefits up to 9 years of treatment. Journal of the American Medical Association, 2003, 289:1645–1651. 2. Saad ST et al. Follow-up of sickle cell disease patients with priapism

treated by hydroxyurea. American Journal of Hematology, 2004, 77:45–49. 3. Strouse JJ et al. Hydroxyurea for sickle cell disease: a systematic review for efficacy and toxicity in children. Pediatrics, 2008, 122:1332–1342. 4. Gaston MH et al. Prophylaxis with oral penicillin in children with sickle cell anemia. A randomized trial. The New England Journal of Medicine, 1986, 314:1593–1599.