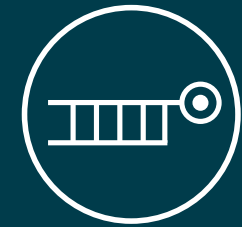


Iron overload disorders

A group of conditions characterised by increased blood and tissue iron levels resulting in damage to the liver and other organs.

Drug candidate
siRNA-GaINAc



Causes

Iron overload can result from a genetic condition called hereditary haemochromatosis or from repeated blood transfusions in patients with transfusion-dependent anaemias (e.g. thalassaemias, sickle cell anaemia).



Prevalence¹



Symptoms and Complications²

- > Fatigue
- > Abdominal pain
- > Joint pain
- > Low energy
- > Low libido
- > Erectile dysfunction



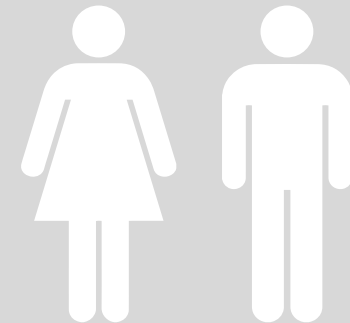
Treatments^{3,4,5}

Daily iron chelation, in some cases performed by intravenous or intramuscular injection, can cause side effects and can lead to low compliance rates.



Advantages

Our subcutaneous drug will minimise patient burden and require less frequent administration, while being highly effective at targeting the underlying causes of the disease.



¹ Global prevalence of putative haemochromatosis mutations. Merryweather-Clarke et al. J. Med. Genet., 1997

² A survey of 2,851 patients with hemochromatosis: symptoms and response to treatment. McDonnel et al. Am J Med, 1999

³ How I treat hemochromatosis. Adams and Barton, Blood, 2010

⁴ A Report on Chelating Therapy and Patient Compliance by Determination of Serum Ferritin Levels in 243 Thalassaemia Major Patients. Pedram et al. Iranian Journal of Pediatric Society, 2010

⁵ Iron-Chelating Therapy for Transfusional Iron Overload. Brittenham, N Engl J Med, 2011