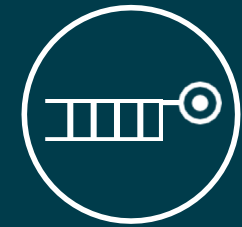


Beta Thalassemia

Group of hereditary blood disorders characterized by decreased production of beta chains of haemoglobin, frequently resulting in anaemia and iron overload.

Drug candidate
SLN124



Causes¹

Beta Thalassemia is caused by a deficiency in the production of the β -globin protein component of haemoglobin, leading to anaemia and dysregulated iron homeostasis. Often patients need life-long blood transfusions which further exacerbates tissue iron overload.



Prevalence²

60,000

Patients in EU and US



Symptoms and Complications²

- > Chronic Fatigue
- > Delayed growth
- > Poor appetite
- > Enlarged spleen, liver or heart
- > Bone problems
- > Jaundice
- > Vascular dysfunction



Treatments³

Patients receive daily iron chelation therapy to prevent iron overload, in addition to regular transfusions to control their anaemia. In severe cases allogenic bone marrow transplantation may be considered, but this is hampered by the availability of HLA-match donors



Advantages

SLN124 provides a safe, durable and easy to administer approach to modulate a key intrinsic mechanism to prevent iron overload and improve anaemia. It will reduce or abolish the need for transfusions, prevent the onset of secondary complications and improve overall quality of life.



¹Thalassaemia. Taher et al. The Lancet, 2017; 391 (10116): 155 - 167

²Beta-thalassemia. Galanello and Origa. Orphanet journal of rare diseases, 2010; 5:10

³How I treat Thalassemia. Rachmilewitz and Giardina. Blood, 2011; 118(13):3479-88