

## press release

### **Novo Nordisk obtains exclusive worldwide licence to EpiDestiny's sickle cell disease programme**

**Bagsværd, Denmark, 5 April 2018** – Novo Nordisk and EpiDestiny today announced that Novo Nordisk has obtained an exclusive worldwide licence to EpiDestiny's sickle cell disease (SCD) programme, EPI01.

EpiDestiny is eligible to receive more than 400 million US dollars in upfront, development and sales milestone payments and will get royalties on net sales. EpiDestiny and Novo Nordisk will collaborate to develop EPI01 in SCD and beta-thalassaemia. EpiDestiny retains all rights to continue development of EPI01 in oncology.

Increasing levels of foetal haemoglobin (HbF) have important clinical benefits in SCD and beta-thalassaemia patients<sup>1</sup>. Elevated HbF correlated with increased red blood cell half-life, reduced number of pain crises and increased life expectancy<sup>1</sup>. EPI01 is a novel, orally available, disease-modifying therapy to increase HbF and interrupt SCD pathophysiology.

EpiDestiny recently completed a phase 1 trial with EPI01 in SCD patients demonstrating increased HbF expression and safety after eight weeks of administration in a small patient cohort<sup>2</sup>. The clinical observations demonstrated the potential for EPI01 to serve as a safe and highly meaningful disease-modifying therapy for SCD.

"This is a great opportunity for Novo Nordisk to enter into a new therapeutic area closely related to our existing biopharmaceutical business and thereby utilise our core R&D and commercial capabilities to make a significant difference for patients living with a serious chronic disease. We are looking forward to working closely with EpiDestiny and their great network among sickle cell disease experts and the sickle cell community. We are confident that together we can make a significant difference for SCD patients and their families globally," said Mads Krogsgaard Thomsen, executive vice president and chief science officer of Novo Nordisk.

"We would like to extend our gratitude to all the dedicated patients, physicians and nurses who will participate in the clinical studies of EPI01 in SCD and beta-thalassaemia. Further, the collaboration with Novo Nordisk represents an important step in the continued development of EPI01 for patients with this underserved life-threatening disease. We are looking forward to sharing this programme with Novo Nordisk which has a long history of successfully developing and commercialising products within chronic specialty care across the globe", said Santhosh Vadivelu, PhD, president and chief executive officer of EpiDestiny.

“The support we receive from Novo Nordisk will allow EpiDestiny to invest and explore the full potential of EPI01 within oncology and to pursue our other pipeline compounds in oncology and other indications.”

### **About sickle cell disease and beta-thalassaemia**

Sickle cell disease (SCD)<sup>3</sup>, is among the most common inherited diseases affecting mostly people of African and Asian origin<sup>4</sup>, with an estimated 30 million cases worldwide. SCD is caused by mutations in the gene for the haemoglobin beta chain that carries oxygen in red blood cells. The resulting sickle haemoglobin molecule forms large fibrils in red blood cells which renders red blood cells rigid and deformed (sickle-shaped). The sickle-shaped red blood cells tends to aggregate and can block small blood vessels, lead to chronic anaemia, decreasing oxygen delivery and damaging multiple tissues and organs. Damage to organs such as the spleen impairs immunity and causes high risk of infections such as pneumonia. Large vessels in the brain can be affected by the rigid red blood cells and anaemia, leading to overt strokes in 10-15% of children and silent strokes in many more. Chronic anaemia also affects the heart and lungs, contributing to a life expectancy which may be reduced by several decades, since there are limited medicines that address the root cause of the disease.

Beta-thalassaemia<sup>5</sup> is a related group of red blood cell diseases, also arising in human evolution because of malaria, and also caused by mutations in the gene for the haemoglobin beta chain. Instead of producing a mutated haemoglobin molecule, not enough of the haemoglobin chain is produced, leading to varying degrees of anaemia and decreased oxygen delivery. The most severe cases of beta-thalassaemia, thalassaemia major, require regular blood transfusions. The blood transfusions, although initially life-saving, eventually cause too much iron to accumulate in the body; this affects the heart and eventually causes death.

### **About EPI01**

EPI01 is an oral, fixed dose formulation of a DNA methyl-transferase enzyme 1 and cytidine deaminase inhibitor, decitabine and tetrahydrouridine, which has potential to work by increasing the amount of foetal haemoglobin that can substitute the defective haemoglobin in SCD patients, thereby intending to prevent the deformation of the red blood cells and improving the oxygen level in the blood. EpiDestiny has been granted Rare Pediatric Disease, Fast Track and Orphan Designations by the U.S. Food and Drug Administration (FDA) for EPI01.

## About EpiDestiny

*EpiDestiny is a privately held, clinical stage biopharmaceutical company. Our goal is to spearhead a new era of gene control therapies that use small molecules to treat inherited and acquired genetic diseases such as sickle cell disease, beta-thalassaemia and cancer. Our mission is to provide access to disease modifying therapies to patients and transform the way patients are treated worldwide.*

*For more information, visit [epidestiny.com](http://epidestiny.com), Facebook, Twitter, LinkedIn, YouTube.*

## About Novo Nordisk

*Novo Nordisk is a global healthcare company with 95 years of innovation and leadership in diabetes care. This heritage has given us experience and capabilities that also enable us to help people defeat obesity, haemophilia, growth disorders and other serious chronic diseases. Headquartered in Denmark, Novo Nordisk employs approximately 42,100 people in 79 countries and markets its products in more than 170 countries. For more information, visit [novonordisk.com](http://novonordisk.com), Facebook, Twitter, LinkedIn, YouTube.*

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## References

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