TREATMENT WITH ZYNTEGLO

ZYNTEGLO (autologous CD34+ cells encoding $\beta^{A\text{-}T87Q}$-globin gene*) is the first and only one-time gene therapy that gives patients with transfusion-dependent β-thalassaemia (TDT) the potential to achieve transfusion independence[1†]

*Full common name: A genetically modified autologous CD34+ cell–enriched population that contains haematopoietic stem cells transduced with lentiviral vector encoding the $\beta^{A\text{-}T87Q}$-globin gene.

†In clinical trials, transfusion independence was defined as a weighted average haemoglobin of ≥9 g/dL with no red blood cell transfusions for a continuous period of ≥12 months at any time during the study after infusion of ZYNTEGLO.[1]