

87. Casu C, Presti VL, Oikonomidou PR, et al. Short-term administration of JAK2 inhibitors reduces splenomegaly in mouse models of beta-thalassaemia intermedia and major. *Haematologica* 2018; **103**: e46-e9.
88. Taher AT, Karakas Z, Cassinerio E, et al. Efficacy and safety of ruxolitinib in regularly transfused patients with thalassaemia: results from a phase 2a study. *Blood* 2018; **131**: 263-5.
89. Matte A, Federti E, Winter M, et al. Bitopertin, a selective oral GLYT1 inhibitor, improves anemia in a mouse model of β -thalassaemia. *JCI Insight* 2019; **4**: e130111.
90. Taher AT, Viprakasit V, Cappellini MD, et al. Haematological effects of oral administration of bitopertin, a glycine transport inhibitor, in patients with non-transfusion-dependent beta-thalassaemia. *Br J Haematol* 2021; **194**: 474-7.
91. Casu C, Oikonomidou PR, Chen H, et al. Minihepcidin peptides as disease modifiers in mice affected by beta-thalassaemia and polycythemia vera. *Blood* 2016; **128**: 265-76.
92. Casu C, Chessa R, Liu A, et al. Minihepcidins improve ineffective erythropoiesis and splenomegaly in a new mouse model of adult β -thalassaemia major. *Haematologica* 2020; **105**: 1835.
93. Motta I, Bou-Fakhredin R, Taher AT, Cappellini MD. Beta thalassaemia: new therapeutic options beyond transfusion and iron chelation. *Drugs* 2020; **80**: 1053-63.
94. Guo S, Casu C, Gardenghi S, et al. Reducing TMPRSS6 ameliorates hemochromatosis and beta-thalassaemia in mice. *J Clin Invest* 2013; **123**: 1531-41.
95. Schmidt PJ, Toudjarska I, Sendamarai AK, et al. An RNAi therapeutic targeting *Tmprss6* decreases iron overload in *Hfe(-/-)* mice and ameliorates anemia and iron overload in murine beta-thalassaemia intermedia. *Blood* 2013; **121**: 1200-8.
96. Porter J, Taher A, Viprakasit V, et al. Oral ferroportin inhibitor vamifeportin for improving iron homeostasis and erythropoiesis in beta-thalassaemia: current evidence and future clinical development. *Expert Rev Hematol* 2021; **14**: 633-44.
97. Manolova V, Nyffenegger N, Flace A, et al. Oral ferroportin inhibitor ameliorates ineffective erythropoiesis in a model of beta-thalassaemia. *J Clin Invest* 2019; **130**: 491-506.
98. Nyffenegger N, Flace A, Doucerain C, et al. The oral ferroportin inhibitor VIT-2763 improves erythropoiesis without interfering with iron chelation therapy in a mouse model of beta-thalassaemia. *Int J Mol Sci* 2020; **21**: 873.
99. Richard F, van Lier JJ, Roubert B, et al. Oral ferroportin inhibitor VIT-2763: first-in-human, phase 1 study in healthy volunteers. *Am J Hematol* 2020; **95**: 68-77.
100. Baronciani D, Casale M, De Franceschi L, et al. Selecting beta-thalassaemia patients for gene therapy: a decision-making algorithm. *Hemasphere* 2021; **5**: e555.
101. Leibowitz ML, Papatheanasiou S, Doerfler P, et al. Chromothripsis as an on-target consequence of CRISPR-Cas9 genome editing. *Nat Genet* 2021; **53**: 895-905.

