Haematologica HAEMATOL/2018/189357 Version 3

Short-hairpin RNA against aberrant HBB[IVSI-110(G>A)] mRNA restores β -globin levels in a novel cell model and acts as mono- and combination therapy for β -thalassemia in primary hematopoietic stem cells

Petros Patsali, Panayiota Papasavva, Coralea Stephanou, Soteroulla Christou, Maria Sitarou, Michael N. Antoniou, Carsten W. Lederer, and Marina Kleanthous

Disclosures: The present study was co-funded by the European Union's Seventh Framework Program for Research, Technological Development and Demonstration under grant agreement no. 306201 (THALAMOSS), and by the Republic of Cyprus through the Research Promotion Foundation under grant agreement ΥΓΕΙΑ/ΒΙΟΣ/0311(ΒΕ)/20 and through core funding of The Cyprus Institute of Neurology and Genetics.

Contributions: Contributions: PeP performed molecular and cell culture work, designed molecular tools for this study and prepared initial text and figure drafts. PaP performed cell culture work and scored cytocentrifugation samples and HPLC analyses. CS assisted in cloning and cell culture work. SC and MS coordinated sampling and hematological analyses. MNA guided study design and progress. CWL wrote the grants, conceived and supervised the study, performed HPLC analyses and wrote the manuscript. MK hosted the research and guided study design. PeP, PaP, CS, MNA, CWL and MK contributed to the final version of the manuscript. All authors have approved the final version of the article.