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Contributions: MC was the principal investigator of the study. JAR was the scientific director of the study. JAR, FL, MD, LL and LJ were responsible for patient care and follow-up. JAR, EM and AM supervised the clinical assessments and sample harvesting. EM and AM analysed the data. JAR, EM and CC helped to write the manuscript. CLP designed and conducted the in vivo experiments and wrote the manuscript. OR analyzed and interpreted RNA-Seq data. LW prepared some patient samples. HS performed and performed the FACS analyses. TF performed experiments (purification of CD34+ HSPCs, total RNA extraction and FACS analyses). AS and JMT submitted the protocol to the French drug agency, coordinated the clinical trial and collected the data. AM designed and supervised the RNA-Seq experiments and wrote the manuscript. JAR and FL helped to design the study. IAS collected and analyzed the data. MC and IAS designed and supervised the overall study and wrote the manuscript.