## Haematologica HAEMATOL/2020/255620 Version 4

Treatment with emapalumab in an ADA-SCID patient with refractory hemophagocytic lymphohistiocytosis-related graft failure and disseminated BCGitis

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Disclosures: Disclosure of conflict of interest: MPC is PI of the long-term follow up study sponsored by Orchard Therapeutics for patients treated with Strimvelis and AA is PI of clinical trials sponsored by Orchard Therapeutics. VA is an employee of Sobi; CdM is consultant for Sobi

Contributions: Contribution: FT, VG followed the clinical course of the patient, collected data, analyzed results and wrote the paper; FB, FF, MM, VC, MD, MPC, ESF, CO followed the clinical course of the patient and collected data; ZK and SG followed the patient before referral to our Institution; SG collected peripheral blood hematopoietic stem cells from the donors; MZ, CP and RM performed graft manipulation; BM performed chimerism analyses; CB and MB performed radiological evaluations; DMC performed fecal PCR for detection of Mycobacterium bovis; VA and CDM provided emapalumab as per CU, performed PK/PD emapalumab evaluations, critically read the manuscript and helped with scientific discussion; AA and FC critically revised the manuscript and helped with scientific discussion; MEB advised on the design the study, analyzed data and contributed to the final writing of the paper.