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T cell gene therapy for perforin deficiency corrects cytotoxicity defects and prevents Haemophagocytic Lymphohistiocytosis manifestations

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ACCEPTED MANUSCRIPT

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7 8 9 10 11 12 13 14 15 16 17 18 19 20 21 22 23 24 25 26 27 28 29 30 31 32 33 34 42 43 44 45 46 47 48 48 48 48 49 49 49 49 49 49 49 49 49 49 49 49 49	Affiliations: a Infection, Immunity, Inflammation, Molecular and Cellular Immunology Section, UCL Great Ormond Street Institute of Child Health, London, UK b Department of Pediatric Oncology, Hematology and Clinical Immunology, Medical Faculty, Center of Child and Adolescent Health, Heinrich-Heine-University, Duesseldorf, Germany c Institute of Experimental Hematology, Hannover Medical School, Hannover, Germany d Section of Immunobiology, Division of Inflammation and Immunology, Department of Medicine, Faculty of Medicine, Imperial College, London, UK e Center for Chronic Immunodeficiency, University Medical Center, University of Freiburg, Germany f Department of Paediatric Haematology and Oncology, Division of Paediatric Stem Cell Transplantation and Immunology, University Medical Centre Hamburg-Eppendorf, Germany Corresponding address: Professor Bobby Gaspar GOSHCC Professor of Paediatrics and Immunology Consultant in Paediatric Immunology Infection, Immunity, Inflammation, Molecular and Cellular Immunology Section, UCL Great Ormond Street Institute of Child Health, 30 Guilford Street, London WC1N 1EH T; +44 (0)207 905 2319/2809 (direct/secretary) / F; +44 (0)207 905 2810 E; h.gaspar@ucl.ac.uk Title: T cell gene therapy for perforin deficiency corrects cytotoxicity defects and prevents HLH manifestations Text word count: 4508 Abstract word count: 231 Number of figures: 5 Number of figures: 5 Number of figures: 5 Number of references: 37 Funding This work has been funded by grants from the German Research Foundation — Deutsche Forschungsgemeinschaft grant no. GH 154/1-1 (S.G.) and SFB1160, TP1 (S.E.), the Histiocytosis Research Trust and the UCL Therapeutics Innovation Fund (M.C.), the Wellcome Trust (A.J.T.), the DAAD and the German Federal Ministry of Research and Education (A.S.), the European Commission's 7 th Framework Program Contract 261387 (CELL-PID) and grant_MR/I012855/1 from the Medical Research Council. The authors would like to acknowledge the support of Great Ormond Street Hospital Children'
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