



**GREAT ORMOND STREET
INSTITUTE OF CHILD HEALTH**

Great Ormond Street 
Hospital for Children

NHS Foundation Trust

Joint Research and Development Office
Division of Research and Innovation

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05 February 2018

Dear MHRA,

Study Title: Phase II clinical trial to evaluate safety and efficacy of mobilisation and collection of CD34+ cells after treatment with plerixafor and filgrastim in patients with Fanconi anaemia for subsequent transduction with a lentiviral vector carrying the FANCA gene and reinfusion into the patient

Sponsor: Great Ormond Street Hospital for Children NHS Foundation Trust

Sponsor Protocol number: 14MI10

MHRA Reference: 17328/0223/001-0001

REC Reference: 15/LO/0126

Eudract Number: 2014-004272-29

Product: Mozobil

Chief Investigator: Professor Adrian Thrasher

For the above study please find enclosed the End of Study Notification Form.

The study was approved by the MHRA on 02 March 2015 and by NRES Committee London - West London & GTAC on 30 March 2015.

The study was never formally opened to recruitment following receiving the above approvals. The decision has been taken to progress straight to the gene therapy trial for Fanconi Anaemia patients as the data that would have been obtained from this mobilisation study has already been obtained by the research team in Spain who we are collaborating with. The Fanconi Gene Therapy study (EudraCT number: 2011-006197-88) was approved by the London - West London & GTAC Research Ethics Committee on 25 October 2017 and is due to be submitted to the MHRA in February 2018.

Please do not hesitate to contact me if you require any further information.

Kind regards,

**Professor Adrian Thrasher
Chief Investigator**