



Academic Directorate of Communicable  
Diseases and Specialised Medicine



Please speak to your health care professional if you are interested in any of the trials listed below or contact the directorate research coordinator Sarah Birch [sarah.birch15@nhs.net](mailto:sarah.birch15@nhs.net) or 0114 2713696

### **Haemophilia and Thrombosis Centre Research**

Study Title	Local PI	Description	Link
The UK TTP Registry	Dr VanVeen	Data collected on patients with newly presenting TTP	<a href="https://www.uclh.nhs.uk/OurServices/ServiceA-Z/Cancer/CBD/TP/TTPRegistry/Pages/Home.aspx">https://www.uclh.nhs.uk/OurServices/ServiceA-Z/Cancer/CBD/TP/TTPRegistry/Pages/Home.aspx</a>
A phase I/II open label, multi centre, ascending single dose safety study of a novel adenoassociated viral vector in patients with haemophilia B	Professor Makris	Gene therapy provides hope for people living with haemophilia B to prevent bleeds and eliminate the need for frequent infusions. The study aims to characterise the safety of the new gene therapy, FLT180a, and to investigate its potential to produce normal levels of FIX following a single administration of the vector.	
A-SURE	Dr Hampton	A study to evaluate the effectiveness of Elocta compared to conventional factor products in the prophylactic	<a href="https://www.hra.nhs.uk/planning-and-improving-research/application-summaries/research-summaries/sobiecto-002-_a-su">https://www.hra.nhs.uk/planning-and-improving-research/application-summaries/research-summaries/sobiecto-002-_a-su</a>

		treatments of patients with haemophilia A	<a href="#">re/</a>
Explorer 6	Dr Hampton	A non-interventional study in haemophilia A and B patients treated according to routine clinical practice	
Explorer 7 and 8	Dr Hampton	The efficacy and safety of concizumab prophylaxis in patients with haemophilia A or B with and without inhibitors	
Dynamo	Professor Makris	The dynamic interplay between bleeding phenotype and baseline factor level in moderate and mild haemophilia	

Updated 07/11/2019