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## Haemophilia A Gene Therapy

**Case ID:**

94-075

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**Category(s):**

Bio-Pharmaceutical Therapy

**Description:**

**Haemophilia A Gene Therapy using a highly-expressing FVIII Sequence**

**Available For:** Licensing

**Summary:**

We have developed a gene therapy for Haemophilia A that utilises a novel promoter and a highly-expressed sequence for the clotting protein Factor VIII.

Building on our expertise in Haemophili B treatment with a Factor IX gene therapeutic, we are currently in pre-clinical development of our FVIII medicament. Planning is underway to take this technology into the clinic.

**Market Opportunity**

Haemophilia A is caused by a deficiency of the clotting factor "Factor VIII". This is the most frequent clotting disorder, and has an incidence of approximately 1 in 5,000-10,000 male births. The direct (clotting factor therapeutics) and indirect (in-patient treatment) costs of treating Haemophilia A are substantial.

**Further Information**

Please contact Chris Loryman, UCL Business PLC. T +44 (0)20 7679 9000 E [c.loryman@uclb.com](mailto:c.loryman@uclb.com)

**For Information, Contact:**

Chris Loryman  
Business Manager  
UCL Business PLC  
020 7679 9000  
[c.loryman@uclb.com](mailto:c.loryman@uclb.com)

**Inventors:**

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Edward Tuddenham  
Amit Nathwani  
Adrian Thrasher  
John McVey

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