



UCL



Gene therapy at UCL: a powerful new way to treat haemophilia A

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Discipline

Arts & Humanities
Built Environment
Engineering
Laws

Life & Medical Sciences

Mathematical & Physical Sciences
Social & Historical Sciences

Grand Challenges

Global Health

Human Wellbeing

Intercultural Interaction
Sustainable Cities

Mechanism

Business Support
Consultancy
Placements

Licence

Partnerships
Public Outreach
Research Collaborations & Studentships
Spin-outs
Student Engagement
Subsidiary

An urgent medical challenge

Haemophilia A is a life-threatening genetic condition that affects the blood's ability to clot and causes increased or spontaneous bleeding. Mostly affecting men, it occurs in about 1 of every 6,000 male births in the UK and crosses all racial and ethnic groups, with approximately one boy in every 5,000 born with it worldwide.

Haemophiliacs lack the blood-clotting protein Factor VIII, which is vital for blood coagulation. There is currently no cure for the condition, and treatment is demanding both in terms of time and cost. This means that its availability is limited to a subset of patients in the most economically advanced countries.

New frontiers

A long-awaited breakthrough occurred in February 2013, when UCL Business (UCLB) signed a licensing agreement with US biotechnology firm BioMarin to develop a new Factor VIII gene therapy programme. The agreement came after a 2011 trial in which the Royal Free London NHS Foundation Trust reported that six people with haemophilia B saw a reduction in symptoms after gene therapy on liver cells. This breakthrough might pave the way for potentially curative life-saving treatment to become widely available.

Gene therapy goes global

Gene therapy inserts therapeutic DNA into a patient's cells, usually using DNA that encodes a functional gene to replace a mutated one. It's a relatively new way of treating diseases, functioning as a direct treatment to the root cause. The

Royal Free trial was the first ever to report positive results using it. The agreement with BioMarin, as Professor Amit Nathwani of the UCL Cancer Institute and Director of the Katherine Dormandy Haemophilia Centre and Thrombosis Unit at the Royal Free London NHS, says: "brings us an ability to scale up everything we're doing so that a larger number of patients can benefit from the advances we've made in the laboratory."

The right conditions for treatment

The deal was brokered by UCLB, who supported it by taking Professor Nathwani's team through negotiations and contracts, helping them rapidly find an agreed basis for continued collaboration.

Cengiz Tarhan, Managing Director of UCL Business says,

"This is an excellent partnership for UCL Business, which combines the world-class translational research strengths of Professor Nathwani and his team with the significant development and commercialization capabilities of BioMarin to progress this groundbreaking therapy for haemophilia A."

This achievement was recognised in May 2014 when Professor Amit Nathwani won the UCL Business Award of the Year at the UCL Awards for Enterprise 2014.



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About us

About UCL

UCL (University College London) was established in 1826 and is ranked as one of the world's top-ten universities. The university is a modern, outward-looking institution, with more than 4,000 academic and research staff committed to engaging with the major issues of our times. It has a global reach, with 34% of its students coming from outside the UK, from almost 140 countries.

www.ucl.ac.uk

UCL Business

UCL Business PLC (UCLB) is a leading technology transfer company, which supports and commercialises research and innovations arising from UCL, one of the UK's leading research-led universities. UCLB has a successful track record and strong reputation for identifying and protecting promising new technologies and innovations from UCL academics. It invests directly in development projects to maximise the potential of the research and manages the commercialisation process of technologies from the laboratory to be market ready. UCLB supports UCL's Grand Challenges of increasing UCL's positive impact on and contribution to Global Health, Sustainable Cities, Intercultural Interaction and Human Wellbeing.

UCL Business is affiliated with UCL Enterprise, which provides UCL's structures for engaging with business for commercial and societal benefit.

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About Licence

For businesses seeking specific technology solutions, UCL Business (UCLB) provides the commercial expertise, legal advice and administrative support required to broker licensing agreements, allowing companies to fully exploit unique technologies with the knowledge that exclusivity and market advantage is preserved.

Through UCLB, academics wishing to license technology have access to comprehensive support services, with Business Managers assisting throughout the process from initial negotiation to concluding contracts.