

New collaboration to develop treatments for liver disease

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A new collaboration based at the University of Cambridge will aim to discover and develop new medicines to treat liver disease.

The partnership, between the University and global pharmaceutical company GlaxoSmithKline (GSK), will build upon the work of researchers who have identified a [molecular mechanism](#) that plays a major role in the life-threatening liver disease that develops in a population of patients with a particular [mutant gene](#).

Alpha-1 antitrypsin (A1AT) deficiency is one of the most common genetic disorders in the UK, affecting approximately one in 2,000 people. The A1AT protein is produced mainly in the liver and circulates to the lungs, where it serves a protective function against enzymes which can break down [lung tissue](#).

In patients with A1AT deficiency, the protein cannot circulate freely and accumulates in the liver, leading to potentially life-threatening liver conditions including neonatal hepatitis, [cirrhosis](#) and hepatocellular carcinoma. Additionally, without A1AT circulating to the lungs, lung tissue can break down, predisposing patients to early onset emphysema. Currently, the only available treatments are [liver transplantation](#) for cirrhosis and protein replacement therapy for emphysema.

The mechanism that underlies protein accumulation in the livers of individuals with A1AT deficiency has been defined by the team led by Professor David Lomas in the University's Cambridge Institute for

Medical Research (CIMR). The collaboration with GSK will combine Professor Lomas' long-term research with GSK's expertise in drug discovery and development in order to develop new therapeutics. Work on the project will be carried out both at GSK and at the CIMR.

"Currently, the only option for patients with [liver disease](#) as a result of alpha-1 antitrypsin deficiency is transplantation," said Professor Lomas. "This partnership brings together our collective expertise in [target](#) biology and drug discovery to tackle an unmet medical need and by teaming up with GSK, we have a great opportunity to turn our research into effective treatments."

This alliance is part of GSK's Discovery Partnerships with Academia (DPAc) initiative, designed to bring together the complementary skill sets of GSK and individual academic groups in the search for [new medicines](#). Dr Pearl Huang, who leads GSK's Global DPAc, said, "Through this collaboration, we'll be able to combine the substantial knowledge and insight of the Cambridge University scientists working in this field with GSK's drug discovery expertise. By combining our strengths in this way and creating an integrated partnership we will have in place a strong framework for discovering new medicines for patients."

Dr Emma Barker of Cambridge Enterprise, the University's commercialisation group said: "This is a great example of translating academic research. We are delighted to be involved with GlaxoSmithKline's DPAc alliance with the goal of developing & commercialising medicines to treat a clear unmet medical need."

Under the terms of the agreement, the University and Cambridge Enterprise will receive success-based financial support from GSK linked to reaching agreed milestones, as well as an undisclosed upfront payment and royalties on sales from any product that is successfully commercialised out of the collaboration.

Provided by University of Cambridge

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