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New antibody development brings treatment for amyloidosis a step closer

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Scientists funded by the Medical Research Council (MRC) have devised a new approach to treatment of amyloidosis, according to new research published today. Systemic amyloidosis is a serious and usually fatal condition that can affect virtually any organ in the body. It kills one in a thousand people in developed countries.

Amyloid is composed of abnormal protein fibres that are deposited in the body's tissues, damaging their structure and function. Diagnosis is often difficult and delayed so that, by the time the disease is recognised, most patients already have irreversible organ damage. A protein from blood, called SAP, accumulates in amyloid deposits and contributes to their formation and persistence.

In work supported by the MRC spanning 30 years, Professor Mark Pepys and his team from University College London discovered the role of the SAP protein in amyloidosis and have been developing new treatments aimed at it. Initial work with Roche led to the development of a small molecule drug, called CPHPC, which removes SAP from the blood but only partly clears it from amyloid deposits.

The latest development uses CPHPC to first remove SAP from the blood so that antibodies to SAP can then be safely given to target the residual SAP in the amyloid deposits. In experimental models closely resembling human disease, this treatment swiftly eliminated all of the amyloid deposits. The next stage will be to test the treatment in patients, and work towards clinical trials is now proceeding in collaboration with GlaxoSmithKline.

Professor Pepys, University College London, said:

"Our findings open up the prospect of a successful treatment for patients with amyloidosis and we are looking forward to developing the drugs for testing in the first human studies."

The Medical Research Council has supported Professor Pepys since 1969, providing over £15.5 million of funding during this time and is specifically supporting the work on SAP and collaboration with GSK.

A core focus of the MRC's strategy is to fund research that unravels the complex biology of disease. By supporting collaborations with industry, the MRC aims to bring these findings from the laboratory bench to patient bedside more quickly.

The paper "Antibodies to human serum amyloid P component eliminate visceral amyloid deposits" is published on 4th November in Nature.

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