

One step closer in finding treatment for amyloidosis

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Image: Professor Mark Pepys

University College London scientists funded by the Medical Research Council (MRC) have devised a new approach to treatment of amyloidosis, according to new research published yesterday in *Nature*.

Systemic amyloidosis is a serious and usually fatal disease that can affect virtually any organ in the body. It is the cause of death in one per thousand of the population 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. The SAP protein from the blood accumulates in amyloid deposits and contributes to their formation and persistence. In work supported by the MRC spanning 30 years, Professor Mark Pepys FRS and his team from UCL discovered the role of SAP in amyloidosis and have been developing new treatments aimed at it. Initially they partnered with Roche to develop a small molecule drug, called CPHPC, which removes SAP from the blood but only partly clears it from amyloid deposits. This treatment stopped the accumulation of new amyloid but did not clear the existing deposits.

The latest development uses CPHPC to first remove SAP from that 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 the amyloid. 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, Director of the UCL Centre for Amyloidosis and Acute Phase Proteins, 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."

More information: The paper "*Antibodies to human serum amyloid P component eliminate visceral amyloid deposits*" was published yesterday in *Nature*.

Provided by University College London

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