Human Monoclonal Antibodies for the Therapy of Transthyretin Amyloidosis Diseases

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Human Monoclonal Antibodies for the Therapy of Transthyretin Amyloidosis Diseases

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VINNOVA

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Research Abstract

Transthyretin (TTR) amyloidosis is a fatal disease caused by deposition of TTR amyloid (ATTR) fibrils in various organs, such as the heart and nerves leading to organ damage and dysfunction. TTR is a tetramer, and the current concept for ATTR is that the tetramer dissociates into monomers that after conformational changes miss-folds and reassemble as amyloid fibrils.Two different forms of the disease is recognised: 1, where the dissociation of the tetramer is facilitated by a TTR mutation (h-ATTR amyloidosis), and 2, where ageing facilitate dissociation of wild type TTR (wt-ATTR amyloidosis).The treatment available today is liver transplantation for patients with h-ATTR amyloidosis aiming at exchanging the variant TTR

producing liver with one that produces wt-TTR. In addition, TTR stabilising agents have been developed to prevent dissociation of tetrameric TTR, however, no available treatment has been proven effective against the disease.It has been shown that patient with a late onset of h-ATTR amyloidosis have high levels of circulating auto-antibodies against miss-folded amyloidogenic TTR. It is therefore reasonable to suspect, that circulating antibodies against miss-folded TTR are part of the natural defence system against circulating amyloidogenic TTR.Since we have a large population of predominantly late onset h-ATTR amyloidosis patients, and also healthy carriers of the TTR V30M mutation, we are able to collect blood samples for identification of antibodies with a high affinity for miss-folded TTR and low affinity for wild type TTR. This should enable a development of antibody treatment of both wt- and h- ATTR amyloidosis, aiming at a vital substrate for amyloid formation, i.e., miss-folded TTR.

Further information available at:

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