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Hope on nerve diseases



The research focused on one particular protein

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“ **Discovering how to interfere with these protein folding events at a cellular level offers enormous potential** ”

Dr Richard Harvey

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Many diseases

Symptoms include numbness, muscle weakness, and - in advanced cases - failure of part of the nervous system.

Transthyretin is made up of four sub-units that bind to each other.

Sometimes mutant forms of the sub-units are produced which undermine the binding process, and cause the protein to fold up in an abnormal way.

The new approach counters this by exposing transthyretin to tiny designer molecules which bind to its sub-units, and make it less likely that they will not bind together.

Lead researcher Professor Jeffery Kelly said: "I'm very excited about pursuing these potential therapeutic opportunities.

"The same approach may also work with other amyloid diseases.

"Any protein that misfolds and causes pathology could, in principle, be targeted."

Significant finding

Dr Richard Harvey, director of research at the Alzheimer's Society, said it was becoming increasingly clear that a range of degenerative neurological diseases all have problems with the folding of proteins at the molecular level at their core.

"Even though these are all different diseases, and are caused by the accumulation of different neurotoxic proteins, it is the way these proteins fold up that determines whether or not they cause disease.

"Discovering how to interfere with these protein folding events at a cellular level offers enormous potential for preventing, halting or even reversing some of the most disabling degenerative neurological diseases that affect hundreds of thousands of people in the UK alone.

"Unfortunately, almost all of this research is still in the test tube. What is now needed is greatly increased funding from governments, industry and from the public through charities to translate these exciting findings into the treatments of tomorrow."

Harriet Millward, deputy chief executive, Alzheimer's Research Trust, described the

research as "novel and interesting".

But she said: "It may be difficult to produce similar results for Alzheimer's due to the nature of the particular amyloid protein associated with the disease.

"There is a continued need to fund major research into the possible causes of Alzheimer's so we can find an answer to this terrible disease."

The research is published in the journal Science.

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