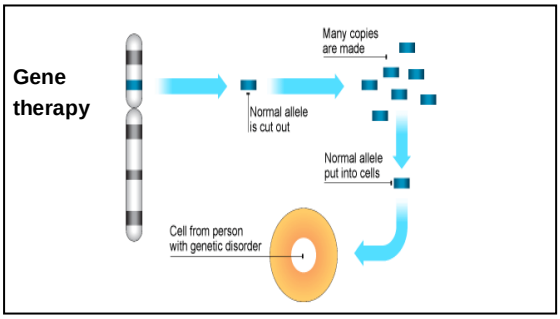
Gene Therapy for Ataxia Telangiectasia

AT society
awareness research care
Research



Why is this project needed?

- Gene therapy involves using a functional healthy gene to replace or correct a mutated gene.
- If we could get a functioning gene into the cerebellum, it ought to be possible to stop cells dying off and ataxia developing.
- The healthy DNA is enclosed in a 'vector' a mechanism which is used to transport the gene into the cells in the body.
- The ATM gene is quite large, which has made it hard to find a vector to use in gene therapy to treat A-T.

Summary:

Professor Molina's team have already developed both an ATM minigene – a compact but functioning version of the gene – and a vector. The vector that they have chosen is called a 'lentiviral' vector.

They have successfully used the vector in the laboratory to insert the ATM minigene into cells taken from people with A-T and have also shown that the minigene can produce ATM protein in the cell. However, they do not yet know whether this protein is fully functional.

One major problem is that the ATM minigene is very big (for a minigene). It is very close to the limit of what a lentivirus will carry. It is also becomes difficult to produce the loaded vector in quantity when it is so big. There is also the danger that the immune system will attack it.

If the team are able to overcome these challenges, and they are very major ones – the next issue is safety. Before one can consider trying this approach on humans, there are a lot of safety questions which will need to be answered. Nevertheless, the team are very optimistic...

Status of the project: Currently underway

Main Researcher: Iganacio Molina at the University of Granada, Spain

Total costs: £80,200 provided jointly by the A-T Society, Action for A-T and Sparks

Start date: This is a two-year project starting in May