Update on the ground-breaking Genistein clinical trial for MPS III, Sanfilippo disease

January 2014 - With thanks to the support from the international MPS community and the amazing fundraising efforts of MPS families affected by MPS III, Sanfilippo, we are now only £120,000 short of our £650,000 target.

What is MPS III?
MPS III or Sanfilippo disease is an inherited disease caused by the lack of an enzyme that breaks down complex sugars. Sanfilippo disease results in progressive neurological deterioration, severe behavioural difficulties and a greatly shortened lifespan due to storage of these complex sugars in the brain. No treatment currently exists for Sanfilippo children and young adults, aside from palliative care, therefore there is an urgent unmet clinical need.

What is Genistein Aglycone?
The University of Manchester have trialled a drug called Genistein aglycone at a high dose and it reduced the amount of complex sugars stored in the brain of mice with Sanfilippo disease, much improving brain function. A compound of Genistein is found in soy foods, it is non-toxic, can be taken by mouth and is relatively cheap. Before purified Genistein aglycone can be prescribed for children with Sanfilippo disease at a higher dose it must be assessed in a double blind placebo controlled clinical trial of children with Sanfilippo disease to determine the correct and safe dose, the effect of the drug on delaying the progression of the disease and how it improves symptoms.

Who will be affected by this treatment?
At present there are approximately 130 individuals in the UK living with Sanfilippo disease A, B and C. With no current treatments available the outlook is bleak. If successful, this treatment will be available to both UK and worldwide sufferers today and into the future.

Where and when will the Clinical Trial be carried out?
The Clinical Trial for Genistein Aglycone will be carried out at the Manchester Children’s Hospital. The trial opens for recruitment from January through to Spring 2014.

What does the clinical trial involve?
24 children with MPS III Type A, B and C will be recruited. The clinical trial is for 1 year duration with 1 year extension and will not involve any crossover. The total duration of the clinical trial is 42 months.

How will funding this clinical trial help other Sanfilippo children?
At the end of the trial the results will be analysed and published. If the results demonstrate safety and significant benefit of Genistein Aglycone, the evidence would be presented to regulators for marketing approval, providing an essential treatment whilst Gene Therapy is developed.

Our fundraising target
In 2012 in collaboration with Dr Brian Bigger of the University of Manchester, the MPS Society began fundraising £650,000 to fund a clinical trial of high dose oral Genistein Aglycone in Sanfilippo disease. Although originally the cost of the trial was £800,000 the MPS Society working with Dr Bigger was able to reduce the cost of the trial to £650,000.

It is truly amazing the support received in our quest to raise £650,000 to fund the Genistein clinical trial in Manchester. We are very grateful for the fantastic support of a small number of affected families in the UK, the National MPS Society, grants pledged from trusts and contributions being raised by the MPS Societies around the world.

However, we still need to raise £120,000.

The Manchester Children’s Hospital have agreed to start the clinical trial to help the children but also subject to the MPS Society raising the rest of the money. If we don’t, the trial will run out of money.

On behalf of the Board of Trustees, the UK MPS Society would like to thank its members, Share A Gift, the National MPS Society in the USA, Austrian MPS Society, Hong Kong MPS Society, German MPS Society, Japanese MPS Society, Swiss LSD Patient Organisation, Irish MPS Society, Australian MPS Society, VML France, Lysosomal Diseases New Zealand and the Spanish MPS Society for their support in enabling us to start this important clinical trial.

Please help us to raise a further £120,000
To donate directly to the MPS Society’s Genistein Appeal please visit http://www.mpssociety.org.uk/research/latest-news/mps-iii-genistein-clinical-trial/

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