



## BDFFA Statement on LINCL Gene Therapy 13.05.08

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### NYP/Weill Cornell gene therapy clinical trial yields promising results for Batten disease

[http://www.eurekalert.org/pub\\_releases/2008-05/nyph-ncg050908.php](http://www.eurekalert.org/pub_releases/2008-05/nyph-ncg050908.php)

This report describes a study that was designed to assess the safety of a new treatment for late infantile NCL, also called late infantile Batten disease. Four or five children in the UK are diagnosed with this condition each year in the UK.

Late infantile Batten disease is an inherited condition caused by mutations in a gene called CLN2 that results in loss of an essential enzyme. Children are born healthy but show symptoms in early life. These include seizures and slowing of development. Children lose skills including the abilities to walk, talk, play and feed themselves. They gradually deteriorate until they are completely dependent on others for everything. Death occurs in middle childhood and there is currently no cure.

The new approach uses a Ogene therapy<sup>1</sup> vector to surgically deliver a healthy gene into several different areas of the brains of affected children. It is hoped that the vector can Oinfect<sup>1</sup> brain cells and make them produce the missing enzyme.

This new study has shown that in a very small number of children with late infantile Batten disease, the gene therapy procedure is practical and safe. Perhaps more importantly for families, in some children who received this particular form of gene therapy their disease did not progress as quickly as might be expected. However, it must be remembered in such rare conditions it is difficult to make direct comparisons with untreated children who have the disease. This makes these findings harder to interpret and it remains to be seen how long lasting these effects will be.

Much remains to be done before this kind of treatment can be offered safely and routinely to children newly diagnosed with late infantile Batten disease. Nevertheless, this is an important step forwards in research aiming to find a cure for late infantile Batten disease. Indeed, these findings will raise the hope of families affected by all kinds of inherited neurological conditions and will pave the way for improving this method further. The group at Cornell University have already begun working with new generations of gene therapy vectors and better ways to tell if they have had any long-term benefit.

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**Registered Charity No 1084908**



The BDFA is a registered charity which provides a supportive, informative, national networking organisation for the families, carers and professionals giving care to children and adults with Batten Disease and promoting awareness of, and research into, the disease. Further information of the activities of the BDFA can be obtained from its website <http://www.bdfa-uk.org.uk/>.

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