BATTEN DISEASE FAMILY ASSOCIATION

Together we WILL make a difference

Registered Charity In England and Wales 1084908 - Scotland SC047408

Despite the hopeful news of a European licence for Brineura - the new and only treatment for children diagnosed with CLN2 (Late Infantile Batten disease) - NHS England has now denied access to this only hope for children and families who will receive this devastating diagnosis. While NICE takes time to consider whether the treatment should receive future funding, newly diagnosed families will not have vital interim access to the drug which has been shown to slow down the devastating progression of symptoms in these children.

Children with a diagnosis of CLN2 (Late Infantile Batten disease) are born seemingly healthy before going on to rapidly develop seizures, dementia, losing their sight and their ability to walk, talk and eat before becoming completely dependent on carers for all of their daily needs. There is no cure for the disease and children may not survive into their early teens. Brineura provides the only hope for families where previously there was none.

Andrea West, Chief Executive of the BDFA added “Alongside fearless families, we have worked with the pharmaceutical company, BioMarin to give much longed for hope for a future for these children. To hear that newly diagnosed children will not receive treatment whilst the NICE process continues is devastating, when we know that even just weeks of delay can make a critical difference to these children’s and families lives.”

We are again faced by a situation where UK families take part in ground-breaking clinical trials and research only to see access to life saving treatment delayed or denied whilst watching the same treatment available to families in other European countries and worldwide.