TITOLO: **DEM-CHILD A Treatment-Oriented Research Project of NCL Disorders as a Major Cause of Dementia in Childhood**
DATA INIZIO: 1 ottobre 2011
DURATA: 36 mesi
DIPARTIMENTI CHE PARTECIPANO: Dipartimento di Scienze Neurologiche, Neuropsicologiche, Morfologiche e Motorie
RESPONSABILE DEL PROGETTO: Alessandro Simonati
FINANZIAMENTI o FONDI DI RICERCA GESTITI DALL'ATENEO:

Unione Europea

- IMPORTO TOTALE: 357.120 €
- TIPO: richiesta contributi UE

OBIETTIVI: The DEM-CHILD project will combine the expertise of (i) recognized European research teams with (ii) high-technology SMEs, and will (iii) collaborate with Indian experts on the following objectives:
(1) High-technology SMEs will develop innovative cost- and time-effective testing and screening methods for all NCLs in order to ensure early diagnosis and thereby prevention.
(2) DEM-CHILD will collect the worlds largest, clinically and genetically best characterised set of NCL patients in order to study disease prevalence and precisely describe the natural history of the NCLs leading to the development of an evaluation tool for experimental therapy studies.
(3) Novel biomarkers and modifiers of NCL will be identified to support the development of innovative therapies.
(4) Focussing on the development of therapies for NCLs caused by mutations in intracellular transmembrane proteins, two complementary therapeutic strategies will be used and compared in eye and brain of mouse models: a) viral-mediated gene transfer and b) neural stem cell-mediated delivery of neuroprotective factors.
ABSTRACT: The DEM-CHILD project focusses on the main cause for childhood dementia in Europe, the neuronal ceroid lipofuscinoses (NCLs). The NCLs are neurodegenerative diseases characterized by dementia, blindness, epilepsy and physical decline leading to an early death of the patients. Since no cure is currently available, these disorders represent a serious social, medical, and economic challenge. To date, eight NCL genes have been characterised. There is evidence suggesting that further gene loci remain to be identified. NCLs are under-diagnosed in many countries around the world as there is an overall lack of research, early diagnosis, treatment and expert availability. Furthermore, due to their broad genetic heterogeneity it is difficult to collect large numbers of genetically similar patients. As such, large therapeutic studies required for advances in treatment are difficult to initiate.