

**Listing of projects awarded in the frame of ELA call for proposals**
**2019**

	Laboratory	Project	Amount
Bosch Assumpció	Universitat Autònoma de Barcelona, Barcelona, Spain	Gene therapy for Megalencephalic leukoencephalopathy with subcortical cysts in two animal models of the disease	45 600 €
Buffo Annalisa	Neuroscience Rita Levi Montalcini, University of Torino, Orbassano Torino, Italy	Allele-specific siRNAs as therapeutic option for ADLD: in vitro pre-clinical validation on unique human experimental models	100 000 €
Cecchini Marco	NEST, Istituto. Nanoscienze, CNR, Pisa, Italy	nanoERT - Nanoparticle based Enzyme Replacement Therapy for the treatment of Krabbe disease: a pre-clinical study in the Twitcher Mouse	48 000 €
Cohen-Salmon Martine	Interdisciplinary Biology Research Center (CIRB), Collège de France, Paris, France	Megalencephalic leukoencephalopathy with subcortical cysts is an astrocyte disease of the neurovascular unit	49 500 €
Vivien Denis	UMR-S U1237 "Physiopathology and Imaging of Neurological Disorders" PhIND, CIDT, Hospital Center of the University of Caen Normandie, Caen, France		7 500 €
Declèves Xavier	Inserm UMR-S1144, Faculty of Pharmacy of Paris, France		10 500 €
Duncan Ian	University of Wisconsin, Madison WI, USA	The mechanism of oligodendrocyte microtubule accumulation in TUBB4A leukodystrophy	31 348 €
Dent Erik W.	University of Wisconsin, Madison WI, USA		67 994 €
Eichler Florian	Department of Neurology, Massachusetts General Hospital, Harvard Medical School, Boston, MA, USA	AAV-mediated Gene Delivery for the Motor and Sensory Phenotype of Adrenomyeloneuropathy	92 300 €
Elorza Vidal Xabier	University of Barcelona, Barcelona, Spain	Misslocalization of astrocytic VCAM-1 in Megalencephalic leukoencephalopathy	36 359 €
Quinn Ronald	The Eskitis Institute of Drug Discovery Department, Griffith University , Brisbane, Australia	Substrate reduction therapy for Metachromatic leukodystrophy and Canavan disease	64 263 €
Gritti Angela	SR-Tiget, Ospedale San Raffaele, Milano, Italy	Development of chimeric lysosomal enzymes with improved bioavailability to advance gene therapy strategies for globoid cell leukodystrophy	95 000 €
Hamilton Noémie	Infection, Immunity and cardiovascular disease Department, University of Sheffield, Sheffield, United-Kingdom	A zebrafish model to identify new therapeutic strategies for RNaseT2-deficient cystic leukoencephalopathy without megalencephaly	70 851 €
Kemp Stephan	Department of Genetic Metabolic Diseases, Amsterdam UMC, Amsterdam, The Netherlands	Brain organoids to evaluate a new therapeutic strategy for adrenoleukodystrophy	69 345 €
Heine Vivi	Department of Child Neurology, Amsterdam UMC, Amsterdam, The Netherlands		20 000 €
Kuhlmann Tanja	University Hospital Münster, Münster, Germany	Oligodendrocytes: The only stress victims in vanishing white matter disease?	80 370 €
Piguet Françoise	INSERM U1169, Fontenay aux Roses, France	Gene therapy for metachromatic Leukodystrophy	100 000 €
Simas Mendes Marisa-Isabel	Metabolic Unit Department, VU University Medical Center , Amsterdam, The Netherlands	Towards treatment of leukoencephalopathy with brain stem and spinal cord involvement and lactate elevation (LBSL)	54 410 €
van der Knaap Marjo	VU university medical center, Amsterdam, The Netherlands	Targeting the integrated stress response in vanishing white matter	120 893 €
Wrabetz Laurence	SUNY Buffalo/ Hunter James Kelly Research Institute, Buffalo, USA	Selective galactosylceramidase ablation to study the pathogenesis of Krabbe leukodystrophy	71 655 €

New Projects

Renewals