

**LERU STudent REseArch Mobility Programme (STREAM)  
Project proposal**



**Host University:**  
Università degli studi di Milano



**Field:**  
Medicine/biology

**Specified field, subject:**  
Molecular biology, neurology, neurobiology

**Research project title:**  
Novel approaches for the treatment of Rett syndrome



Possible starting month(s):

Sep	Oct	Nov	Dec	Jan	Feb	Mar	Apr	May	Jun	Jul	Aug
				x							



Possible duration in months:

1	2	3	4	5	6
		x	x	x	x



**Suitable for students in:** 2<sup>nd</sup> cycle (Master students)

**Prerequisites:** Required exams: molecular biology. Students with certified knowledge in neurobiology and cell biology will be preferred. Previous attendance to a laboratory course is appreciated.

**Restrictions:** none



**Description:**

Rett syndrome (RTT) is a devastating neurological disorder that to date is still lacking any treatment to combat the primary pathology; current options are geared only to ameliorate secondary phenotypes, such as seizures, behavioural/emotional states and breathing disturbances. It has, however, been established that the RTT phenotype, at least in mice, is reversible and that its severity can be ameliorated by restoration of normal MeCP2 levels in brain, therefore giving hope that rationally-designed therapeutic measures can ameliorate, fix or prevent RTT. The aim of this project is to evaluate the therapeutic efficacy of human neural stem cells in Rett syndrome, by combining in vitro and in vivo experiments. By using co-culture systems, we will analyze whether naive or genetically modified glial cells differentiated from neural stem cells can ameliorate/rescue the morphological and molecular phenotypes that characterize Mecp2-null primary neurons. In parallel, the same stem cells will be intracerebrally transplanted in a mouse model of Rett syndrome and the therapeutic potential will be assessed performing several behavioural, histological and biochemical assays.

**Faculty or Department** Department of Medical Biotechnology and Translational Medicine - Università degli Studi di Milano

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