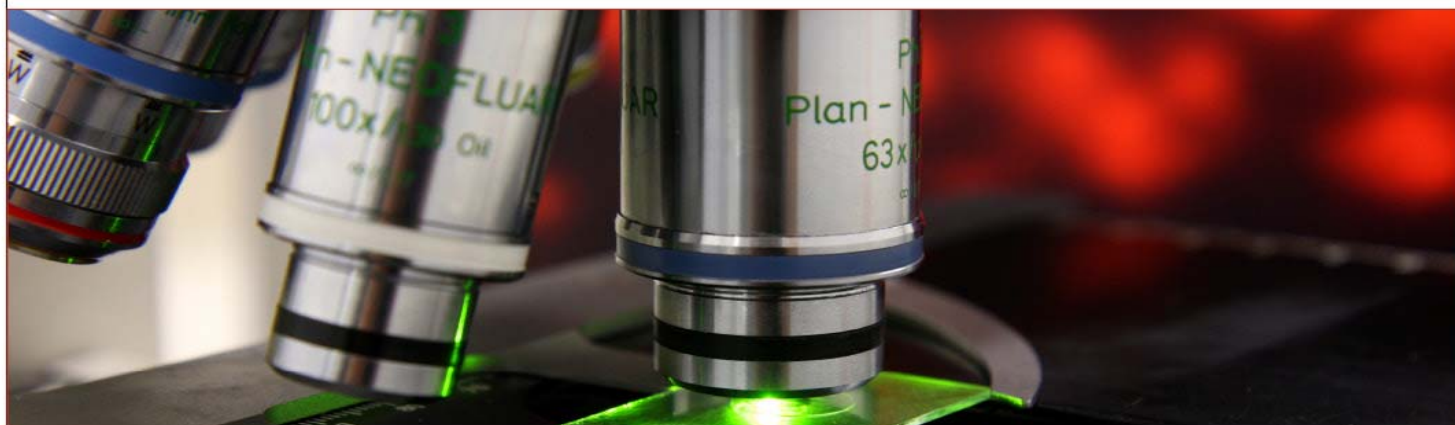


SÉMINAIRES ET CONFÉRENCES



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« Triplet repeats RNA toxicity: from foci to pharma »

Genomic expansions of triplet repeats are involved in a large number of neuromuscular and neurodegenerative disorders. RNAs transcribed from repeat expansions exert toxic gain-of-function effects by interfering with normal cellular regulatory pathways. One of the best known triplet repeats pathology is the neuromuscular disease myotonic dystrophy type 1 (DM1), which is caused by an expansion of CTG repeats in the DMPK gene. Mutant DMPK mRNA accumulates into aberrant nuclear RNA aggregates, leading to the sequestration of essential RNA-binding proteins involved in mRNA splicing, translational control and decay. I will present work from my laboratory exploring the mechanism of triplet repeats RNA aggregation, provide novel insights into the pathogenesis of DM1 and present our effort to develop a therapy for this disease.



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Le lundi, le 18 septembre 2017, 11:30

**Pavillon Roger-Gaudry
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Invité par Gérardo Ferbeyre

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