



Psychiatry and Neuroscience Seminar Series 2021



Pr Nicole DEGLON

(Host D Zala)

*Neuroscience Research Center (CRN), laboratory of Cellular and
Molecular Neurotherapies (LCMN), Lausanne, Switzerland*

Neurodegenerative diseases in the age of genetic engineering

Friday, September 3rd, 2021, noon

R04-45, 102-108 rue de la santé - 75014 Paris & VISIOCONFERENCE

Pr Nicole DEGLON

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Molecular Neurotherapies (LCMN), Lausanne, Switzerland**

Genetic engineering technologies have not only revolutionized basic neuroscience research, but also animal modeling and clinical developments. Single-cell transcriptomic and epigenetic analysis have, for example, revealed cell-type-specific functions and profiles in pathological and non-pathological contexts. Recently, we showed that in Huntington's disease (HD) opposite mechanisms govern the transcriptional regulation of striatal neurons and glial cells. To further decipher the cell-type specific contribution in neurodegenerative disorders, we developed gene transfer strategies with viral vectors to selectively silence, inactivate or overexpress target genes in astrocytes and/or neurons.

We are presently combining these tools to develop disease-altering therapies for HD, with in particular the CRISPR/cas9 system. We demonstrated that huntingtin (HTT) inactivation in differentiated human HD-iPSC and mouse models of HD, is leading to an improvement in key markers of the disease. Finally, we developed a self-inactivating system ensuring transient expression of the Cas9 protein with on-target performance similar to CRISPR/Cas9 and improved biosafety profile.

Keywords:

Neurodegeneration

Neuroscience

Neurobiology and Brain Physiology

Neuroprotection

Neurobiology

Neurodegenerative Diseases

Parkinson's Disease

Neurological Diseases

Cellular Neuroscience

Molecular Neuroscience

ZOOM Meeting ID: 889 1921 6636/ Passcode: 112233

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