SCIENTIFIC SEMINAR

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A "morbus morbum sanans" strategy to cure prion diseases

Transmissible spongiform encephalopathies or prion disorders are a group of fatal neurodegenerative diseases affecting humans and other mammals for which no treatment exists. The causal agent of these disorders is an abnormally folded isoform (PrP^{Sc}) of the cellular prion protein (PrP^{C}). Once misfolded, it accumulates in amyloid plaques in the Central Nervous System (CNS) and causes neurodegeneration. Our *in vitro* screening of compounds able to inhibit the misfolding of PrP^{C} and therefore, prion propagation and disease progression, revealed a natural porphyrin as the most powerful anti-prion compound known. This natural metabolite accumulates in patients affected by Congenital Erythropoietic Porphyria (CEP), a rare disease that is chronic thanks to the palliative treatments available. Since prion misfolding takes place mainly in the CNS, any compound with anti-prion capacity requires crossing the blood-brain barrier in order to reach an inhibitory concentration in brain, which in the case of this porphyrin is impossible through direct exogenous administration. Thus, we have designed a new approach to allow a sufficient amount of this metabolite to get into the brain for long enough. In this study, we explore the efficiency of this "morbus morbum sanans" treatment, which consists of curing an invariably fatal disease by inducing another chronic disease with treatable symptoms.

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Friday
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Atrio 800
10.00H

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