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VIRAL VECTORS FOR THE TREATMENT OF LAFORA DISEASE

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Summary

Lafora disease is an inheritable neurodegenerative condition affecting children. The onset of the disease is in adolescence, in apparently healthy teenagers, with headaches, seizures, and insidious decline in cognitive function. No effective treatment is known, and the disease progresses rapidly with amplification of seizures, loss of neurologic functions and dementia, inevitably leading to the death of the patient 5-10 years after the onset. This great-unmet clinical need is the source of considerable effort and the focus of our research (1,2).

Biologics therapeutics based on the delivery of enzymes or polynucleotides is steadily becoming the new standard in the pharmaceutical industry. Despite their potential, their application remains hindered mainly due to the recognition by the immune system and its clearance from the body before they can exert their therapeutic function. Many elegant strategies emerged to overcome these drawbacks, such as embedding polynucleotides in viral vectors (3).

During this master thesis, you will design and synthesize new treatments for Lafora disease based on viral nanovectors encapsulated with (co)polymers. The size, composition, stability, and morphology of these nanovectors will be assessed using state-of-the-art physicochemical techniques involving scattering radiation and microscopy. Furthermore, you will test the efficiency of your designed systems in cells and, possibly, in animal models.

References

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