



# Aptamers as a Therapeutic Tool to Prevent Protein Aggregation in Neurodegenerative Disease

## **The challenge**

Alpha-synuclein is a protein implicated in Parkinson's disease. There is evidence that the aggregation of this protein into large oligomers and fibrils is a part of the underlying mechanism of this disease.

## **The solution**

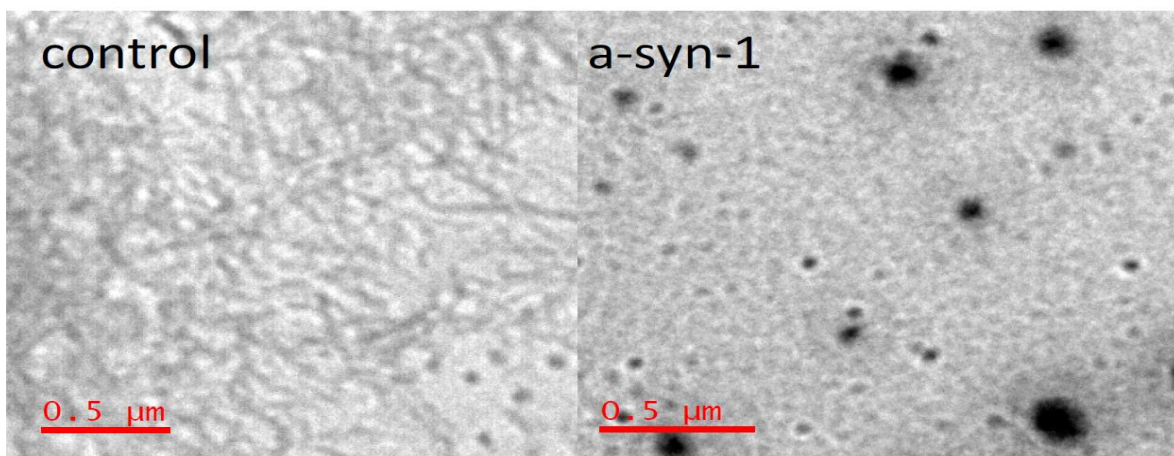
A DNA aptamer sequence that binds to alpha-synuclein monomers and inhibits formation of larger oligomers and fibrils. The DNA aptamer has been packaged in a liposomal vehicle capable of crossing the blood brain barrier *in vitro* and *in vivo*, using a mouse model for PD.

## **Key Benefits**

- ✓ Compared with conventional antibodies, aptamers are not typically recognized by the immune system and are neither immunogenic nor toxic.
- ✓ Aptamers can discriminate between different conformations of the same target protein.
- ✓ Can be easily generated by chemical synthesis

## **Development Stage**

Concept validation complete



**Figure 1: Transmission electron microscopy images of pre-formed fibrils generated from alpha-synuclein monomer (control) in comparison to the minimal aggregation observed in the presence of the inhibitory aptamer (a-syn-1).**



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## **Details**

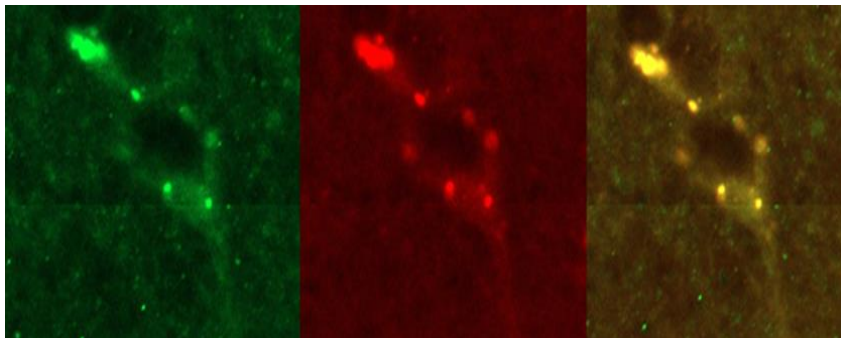
- Using immunofluorescence, Inventors have confirmed both delivery to the brain and co-localization of DNA aptamer in the with an antibody specific for phosphorylated alpha synuclein monomer.
- DNA aptamers with different sequences can bind different sizes and morphologies of alpha synuclein protein.
- DNA aptamers have a very high potential in both therapeutic formulations and diagnostic applications for neurodegenerative disease.

## **Research Team**

Leads: Professor Maria DeRosa and Professor Matthew Holahan  
Co-inventors: McConnell, E; Ventura, KV; Callahan, JP; Hunt,VHD

## **Patents**

- US application 16/758,090 (filed Apr. 22, 2020)
- CA application 3079909 (filed Apr. 21, 2020),
- PCT/CA2018/051335 (filed Oct. 22, 2018), published as WO2019/079887



**Figure 2: Colocalization (right) of the green fluorescence from the secondary antibody recognizing the anti- $\alpha$ -syn antibody (left) and the red fluorescence from injected Cy3.5-labeled a-syn aptamer (middle) confirm the delivery and binding affinity of a-syn**

***For more information about licensing and development opportunities, contact***

**Theresa C. White, PhD**

Manager—Innovation Transfer, Contracts and Agreements

Industry and Partnership Services

[theresawhite3@cunet.carleton.ca](mailto:theresawhite3@cunet.carleton.ca)