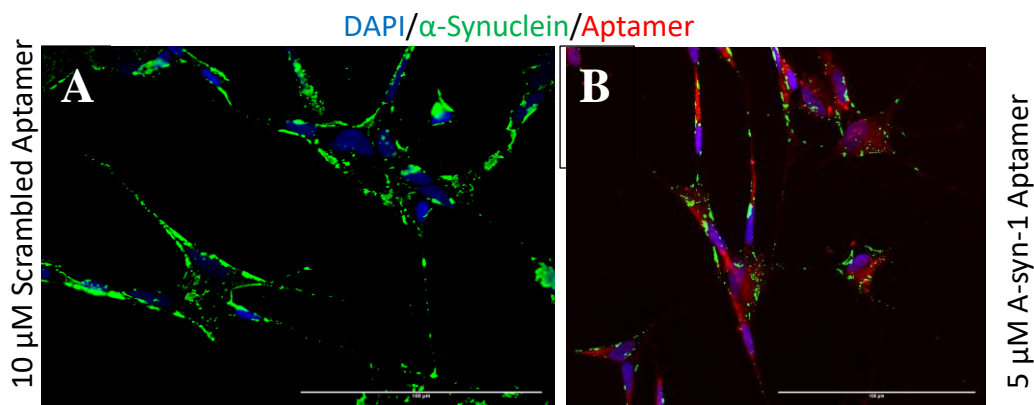


# Aptamers as a Therapeutic Tool to Prevent Protein Aggregation in Neurodegenerative Disease

<p><b>The challenge</b></p>	<p>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.</p>
<p><b>The solution</b></p>	<p>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 <i>in vitro</i> and <i>in vivo</i>, using a mouse model for PD.</p>
<p><b>Key Benefits</b></p>	<ul style="list-style-type: none"> <li>✓ Compared with conventional antibodies, aptamers are not typically recognized by the immune system and are neither immunogenic nor toxic.</li> <li>✓ Aptamers can discriminate between different conformations of the same target protein.</li> <li>✓ Can be easily generated by chemical synthesis</li> </ul>
<p><b>Development Stage</b></p>	<p>Concept validation complete</p>



**Confocal microscopy images of DNA aptamer treatment on alpha-synuclein aggregation in SH-SY5Y cells.** SH-SY5Y cells treated with (A) scrambled DNA aptamer; (B) DNA aptamer targeting  $\alpha$ -synuclein. Cells treated with  $\alpha$ -syn-1 DNA aptamer exhibit reduced green fluorescence compared to the scrambled DNA aptamer treatment, suggesting inhibition of  $\alpha$ -synuclein aggregation by the  $\alpha$ -syn-1 DNA aptamer

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## **Details**

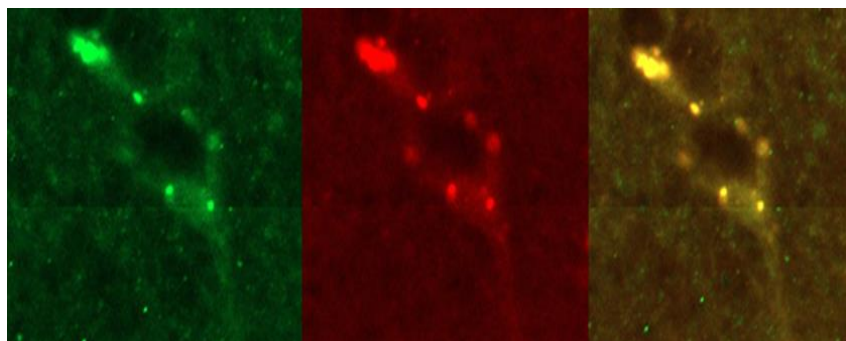
- Using immunofluorescence, Inventors have confirmed both delivery to the brain and co-localization of DNA aptamer in the with an antibody specific for phosphorylated alpha synuclein monomer.
- DNA aptamers with different sequences can bind different sizes and morphologies of alpha synuclein protein.
- 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**

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



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  $\alpha$ -syn aptamer (middle) confirm the delivery and binding affinity of  $\alpha$ -syn

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

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