



Coave Therapeutics to Collaborate with World-Renowned Institute of Neurodegenerative Diseases of Bordeaux to Develop Gene Therapy Programs Targeting Protein Degradation in Neurodegenerative Disorders

Paris, France, September 14, 2022 - Coave Therapeutics ('Coave'), a clinical-stage biotechnology company focused on developing life-changing gene therapies for CNS (Central Nervous System) and eye diseases, announces it has entered into collaboration with the Institute of Neurodegenerative Diseases (IMN), a joint research unit associating the University of Bordeaux and the French National Centre of Scientific Research (CNRS), to develop gene therapy programs targeting protein degradation in neurodegenerative disorders. The collaboration will explore the development of gene therapy products targeting the transcription factor EB (TFEB) for the treatment of alpha-synucleinopathies, such as Multiple System Atrophy (MSA) and idiopathic Parkinson's disease (PD).

TFEB is a master regulator of the autophagy lysosomal pathway, a central cellular pathway controlling the degradation of toxic protein aggregates. Overexpression of TFEB via gene therapy demonstrates potential to reduce and prevent the accumulation of toxic protein aggregates¹ and to consequently prevent neurodegeneration

A recent paper², authored by eminent scientists at IMN, including Erwan Bézard, IMN and INSERM Research Director and member of Coave's Scientific Advisory Board (SAB), and world-renowned Dr Andrea Ballabio, MD, Scientific Director, Telethon Institute of Genetics and Medicine (TIGEM) and also member of Coave's SAB, demonstrated effective delivery of TFEB AAV-based gene therapy in MSA and PD disease models. Furthermore, the results demonstrated a reduction of alpha-synuclein aggregates, prevention of dopaminergic neuron destruction and recovery of clinical phenotype. Dr Ballabio, who discovered the role of TFEB as a master regulator of lysosomal biogenesis and autophagy^{3,4}, will be an advisor to this collaboration.

Under the collaboration, Coave will use its AAV-Ligand Conjugate (ALIGATER) platform to design, develop and manufacture coAAV viral vectors carrying the TFEB gene for targeted delivery to deep brain structures. IMN will be responsible for carrying out jointly designed *in vivo* studies to evaluate the effect of the gene therapy products in animal models of MSA and PD. The collaboration aims to generate further *in vivo* proof of concept data and enable the selection of therapeutic candidates to enter IND enabling studies.

Erwan Bézard, Research Director, IMN, said: *"Targeting the autophagy lysosomal pathway by using coAAV based gene therapy is a unique approach to address neurodegenerative diseases, such as Parkinson's disease or Multiple System Atrophy. Thanks to the partnership with Coave, IMN scientists have an important opportunity to confirm their scientific findings towards the clinic. We look forward to leveraging our collective strengths to best develop gene therapy programs for neurodegenerative diseases with the potential to improve patient outcomes."*

"We are delighted to be collaborating with IMN to develop coAAVs carrying TFEB and explore these gene therapy constructs for the treatment of neurodegenerative diseases. TFEB is an exciting target and we look forward to working with IMN and Andrea Ballabio to evaluate the effect of our novel gene therapies from our ALIGATER platform for the treatment of MSA and PD,



with the potential to develop further programs.” added **Rodolphe Clerval, CEO, Coave Therapeutics.**

References

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<https://doi.org/10.1016%2Fj.tins.2016.02.002>
2. Arotcarena et al. ***Transcription factor EB overexpression prevents neurodegeneration in experimental synucleinopathies,*** *JCI Insight* 2019 <https://doi.org/10.1172/jci.insight.129719>
3. Sardiello et al. ***A gene network regulating lysosomal biogenesis and function.*** *Science, 2019 325: 473-7.*
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About Institute of Neurodegenerative Diseases (IMN)

The Institute of Neurodegenerative Diseases (IMN) is a joint research unit associating the University of Bordeaux and the French National Centre of Scientific Research (CNRS). The IMN, established in January 2011, was founded by Erwan Bézard to develop new therapeutic approaches for neurodegenerative diseases by facilitating translational research from the laboratory to the patient bed.

About Coave Therapeutics

Coave Therapeutics is a clinical-stage biotechnology company focused on developing life-changing gene therapies for CNS (Central Nervous System) and eye diseases.

Coave Therapeutics’ next-generation AAV-Ligand Conjugate (‘ALIGATER’) platform enables targeted delivery and enhanced gene transduction to improve the effectiveness of advanced gene therapies for rare diseases.

The company is advancing a pipeline of novel therapies targeting CNS and eye diseases where targeted gene therapy using chemically modified AAVs (coAAVs) has the potential to be most effective.

Coave Therapeutics, which is headquartered in Paris (France), is backed by leading international life science and strategic investors Seroba Life Sciences, Théa Open Innovation, eureKARE, Fund+, Omnes Capital, V-Bio Ventures, Kurma Partners, Idinvest, GO Capital and Sham Innovation Santé/Turenne.

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