Coave Therapeutics announces Positive 12-months Data from Ongoing Phase I/II Clinical Trial of CTx-PDE6b in Patients with Retinitis Pigmentosa Caused by Bi-allelic Mutations in PDE6b

- *CTx-PDE6b demonstrated clinically meaningful benefit in visual functions at the highest dose and exhibits a good safety profile*

- *Positive results from the study to date support preparations for a registrational trial for CTx-PDE6b in PDE6b Retinitis Pigmentosa*

- *Regulatory approval received to expand the current study to include younger patients with earlier stages of disease who are expected to benefit most from Coave's gene therapy to preserve visual function*

Paris, France, May 31, 2023 - Coave Therapeutics ('Coave'), a clinical-stage biotechnology company focused on developing life-changing gene therapies for CNS (Central Nervous System) and eye diseases, today announces the positive 12-month results from its Phase I/II trial evaluating the safety and efficacy of its innovative gene therapy, CTx-PDE6b, for retinitis pigmentosa (RP) caused by bi-allelic mutations in the PDE6B gene (PDE6b RP). These positive data support Coave's preparations for a registrational trial with CTx-PDE6b in this indication.

In addition, Coave has received regulatory approval to expand the ongoing trial to include a new cohort of six younger patients (aged 13-25 years) with earlier stages of disease to further explore the clinical benefit of CTx-PDE6b in a broader patient population. The first patient is expected to receive treatment by July 2023.

To date, CTx-PDE6b has been administered to 17 patients aged 18 years and older presenting an advanced form of PDE6b RP using two ascending doses. The treatment was administered in the more affected eye while the other eye served as an untreated control.

Following the 12-month study period, both doses were well tolerated (n=17). A subgroup of patients (n=6) with less advanced disease who received the higher dose showed positive efficacy results, consistently measured across all five clinical endpoints (BCVA - Best Corrected Visual Acuity, Visual Field, Microperimetry, Full Field Sensitivity Test and Mobility test) and the retinal anatomical evaluation by OCT (Optical Coherence Tomography). In this sub-group, using microperimetry, a significant favorable progression of sensitivity of the four central loci of the retina was observed in the treated eyes compared to the untreated eyes.

Furthermore, the full-field stimulation test in blue light assessing rod function shows an improvement of the light perception threshold in favor of the treated eyes, which is considered clinically meaningful.

These data support a positive effect of CTx-PDE6b on visual functions and provide a strong rationale to pursue the product's clinical development with the preparation of a registrational trial.

The Phase I/II study design and data will be presented at upcoming conferences.

"*We are highly encouraged by the safety and efficacy data observed so far in patients who have been treated with CTx-PDE6b. This therapy has shown a good safety profile and promising benefit in visual functions within the treated eye after a 12-month follow-up period,*" **said Rodolphe Clerval, Chief Executive Officer**. "*These initial findings provide strong support for expanding this Phase I/II study to include patients with less advanced disease while we begin our preparations for a registrational trial with CTx-PDE6b.*"

"*PDE6B retinitis pigmentosa is a progressive and irreversible inherited degenerative disease that leads to significant visual impairment and blindness. These initial safety and promising efficacy results are of great medical interest and could represent a significant step towards providing a specific treatment for patients with this devastating disease,*" **commented Professor Michel Weber, MD, PhD, Professor at Nantes University Hospital (France) and Guylène Le Meur, MD, PhD, MCU-PH, Associate Professor and Head of Ophthalmology at Nantes University Hospital.**

Coave is responsible for the global development of CTx-PDE6b and retains commercial rights to the product in the US, Japan, South Korea, China and other territories outside Europe. In Europe and certain other countries, CTx-PDE6b is being co-developed by Coave and Théa Open Innovation ('TOI') under a license and development agreement with exclusive rights granted to TOI to commercialize CTx-PDE6b in these territories.

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About CTx-PDE6b

CTx-PDE6b is a clinical stage AAV5-based gene therapy for the treatment of retinitis pigmentosa (RP) caused by bi-allelic mutations of the PDE6b gene (PDE6b RP). RP is a type of inherited retinal dystrophy that leads to blindness by midlife and is characterized by a progressive loss of photoreceptors. There are currently no approved treatments for PDE6b RP.

CTx-PDE6b is designed to deliver a full-length non-mutated copy of the functional human PDE6b gene into the subretinal space where it rapidly induces robust transgene expression and synthesis of functional PDE6b protein in photoreceptive rod cells. By providing these cells with the functional missing protein, CTx-PDE6b is expected to stabilize or halt retinal degeneration in PDE6b-deficient patients.

About Clinical Trial NCT03328130

CTx-PDE6b (AAV2/5-hPDE6B or HORA-PDE6b) is being evaluated in a Phase I/II, monocentric, open-label, dose-ranging clinical trial to assess its safety and efficacy in patients with PDE6b retinitis pigmentosa (RP). Seventeen patients aged 18 years and above across three cohorts and two doses have completed the 12-month study period. The study will now enroll a further cohort of six patients aged 13-25 years with earlier stage disease.

About Coave Therapeutics

At Coave Therapeutics, we are leading the transition of genetic medicine from rare to prevalent conditions, in neurodegenerative and eye diseases.

The Company's proprietary AAV-Ligand Conjugates platform ('ALIGATER') introduces chemical modifications onto AAV capsids, overcoming the limitations of current vectors on efficacy, safety, and manufacturability. With low doses and optimized routes of administration, our conjugated AAV vectors have demonstrated markedly improved transduction and biodistribution in the central nervous system and the eye across different species.

Our diverse pipeline of novel genetic medicines can potentially transform the lives of people afflicted by rare and prevalent neurodegenerative and ocular diseases – including genetically and non-genetically defined indications.

Headquartered in Paris, France, Coave Therapeutics is backed by leading international life sciences investors. For more information about the science, pipeline, and people, please visit www.coavetx.com and follow us on LinkedIn.

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