# Vesper Bio announces completion of single ascending dose stage in trial of its lead candidate VES001, a potentially disease-modifying treatment for fronto-temporal dementia

**Copenhagen, Denmark, 30 May 2024** – Vesper Bio ApS ("Vesper" or "the Company"), a clinical stage biotech and world leader in sortilin receptor biology, today announces completion of the single ascending dose stage of its first-in-human trial of VES001 in healthy volunteers.

VES001 is the first oral, brain penetrant, small molecule sortilin inhibitor designed as a potentially disease-modifying treatment for the neuro-cognitive disorder fronto-temporal dementia (FTD(GRN)). Data returned from this study (NCT06226064) demonstrate the safety and tolerability of VES001 across the full range of doses tested and show it has excellent pharmacokinetics and distribution to relevant parts of the brain. The data predict a once or twice daily efficacious dose.

Importantly, volunteers who received VES001 experienced significant and robust increases in levels of progranulin, demonstrating target engagement. Progranulin is a protein known to play a key role in promoting neuron survival, growth and function. People with FTD(GRN) have inherited gene mutations associated with low progranulin levels.

**Mads Kjølby, Chief Medical Officer of Vesper Bio, said**: "These excellent early-stage results are highly encouraging, and spur us on to develop VES001 as a highly effective new treatment for FTD(GRN). Vesper Bio will continue to seek ways to bring this exciting discovery to benefit the lives of patients and their families."

He added: "We look forward to building on these exciting data and accumulating further evidence of the significant potential that sortilin inhibition has as a disease-modifying strategy - not only for FTD(GRN), but also for a range of other central nervous system (CNS) diseases characterised by progranulin deficiency, neuroinflammation and cell death, such as Parkinson's."

Sortilin is a receptor found on the surface of neurons that competes with progranulin receptors for progranulin binding. When internalised by a sortilin receptor, progranulin is degraded – potentially leading to neuron damage and cell death. VES001 works by blocking sortilin receptors, so progranulin is internalised normally by progranulin receptors.

**Paul Little, Chief Executive Officer of Vesper Bio, said**: "It is a real credit to the unremitting work of the Vesper team - and the steadfast support of Lundbeckfonden BioCapital - that we continue to make rapid progress in our mission to translate breakthrough science into a new class of potentially revolutionary CNS drugs to help address major unmet clinical needs."

**ENDS**

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**Notes to Editors**

**About Vesper Bio**
Vesper is a clinical stage biotech and world leader in sortilin receptor biology. Its lead program uses a sortilin inhibitor to rebalance levels of progranulin in patients where the sortilin receptor would otherwise reduce circulating and extracellular progranulin, contributing to disease. Progranulin is a protein that the body uses to regulate cell growth, survival, repair and avoid inflammation. Low progranulin levels are believed to be a factor in cell dysfunction and damage in a range of indications across neurology. By normalizing progranulin levels, Vesper believes its compounds will have a disease modifying effect, protecting and preserving the remaining cells.

Its lead compound, VES001, is a patient friendly, first-in-class, brain penetrant, oral treatment which targets progranulin deficiency, a major underlying cause of fronto-temporal dementia (FTD). As an orally delivered small molecule, VES001 is able to cross the blood-brain barrier and is an ideal dosing method among these patients due to their rapidly declining mental state.

**About fronto-temporal dementia (FTD)**
Fronto-temporal dementia (FTD), also known as fronto-temporal lobar degeneration (FTLD), is a group of brain disorders that cause degeneration in the frontal and temporal lobes of the brain. FTD impacts a person's behavior, judgement, communication and ability to participate in all activities of daily living. It is the most common cause of dementia in people under the age of 60, and is often misdiagnosed as Alzheimer's. FTD(GRN) is a form of FTD caused by mutations of the progranulin gene (PGRN), resulting in low progranulin levels. FTD(GRN) is thought to account for a quarter of FTD cases.

For further information please visit, https://www.vesperbio.com/.