Alzheimer Europe

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CLINICAL TRIALS WATCH

ACCESSIBLE EASY READ INFORMATION ON:

ASPIRE-FTD STUDY

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ASPIRE-FTD study

1. Study Information	
Name of the study	A study to evaluate the safety and effect of AVB-101, a gene therapy product, in subjects with a genetic sub-type of frontotemporal dementia
Study sponsor	AviadoBio Ltd
Disease	Frontotemporal dementia with progranulin mutations (FTD-GRN). It is an early-onset form of dementia, with symptoms resulting from below normal levels of a protein called progranulin in the brain.
Phase	Phase I/II

2. Information about the drug that will be tested in the study	
Name of drug	AVB-101
Administration	Single dose administered intrathalamically (directly to the thalamus in the brain)
Is the drug already on the market for another medical condition?	No
Will all participants receive the same drug?	All participants will receive AVB-101

3. Information about participating in the trial	
What are the researchers trying to find out?	The purpose of the study is to evaluate the efficacy and safety of AVB-101 in people with frontotemporal dementia with progranulin mutations. The three main questions that the study aims to answer are: 1. Is a one-time treatment with AVB-101 safe? 2. Does a one-time treatment with AVB-101 restore progranulin levels to at least normal levels? 3. Could AVB-101 work as a treatment to slow down or stop progression of the disease?
How long will the treatment last?	It is a one-time treatment of AVB-101 with follow-up assessments for 5 years.

What your involvement will entail?	During the study, participants will be asked: • To complete some laboratory tests (e.g. blood, semen for males) to evaluate the emergent adverse effects (unfavourable signs, symptoms or diseases temporally associated with the use of the drug tested in the study) • To undergo brain scans (MRI) • To complete several tests that will assess memory, cognition and suicide rate (i.e. tests like MMSE, CDR, C-SSRS). Further information on the procedures, tests and number of visits can be obtained from the study team.

4. Who can participate in this study?	
Who can participate in the study?	To take part in the study, participants must: • Be between 30 and 75 years old • Carry a pathogenic progranulin mutation • Have a diagnosis of frontotemporal dementia according to the CDR and NACC FTLD global score • Able and willing to comply with all procedures, the study visit schedule and give written informed consent • Have a study partner who has a sufficient contact with the participant, is willing to participate in all study procedures throughout the study duration.
Who cannot participate in the study?	Exclusion criteria include: • Severe dementia or other symptoms that preclude the ability to comply with study procedures and/or pose unacceptable

	safety risk • Any disease that may cause cognitive impairment unrelated to progranulin mutations, such as other causes of dementia, neurosyphilis, hydrocephalus, stroke, small vessel ischemic disease, uncontrolled hypothyroidism, or vitamin B12 deficiency • Clinically significant abnormality on brain scans • Previous treatment with any gene or cell therapy • Previous treatment with any investigational medicinal product within 60 days prior to study drug treatment. The above list is not exhaustive. It includes the most common conditions and diseases that might exclude people from the study.

5. Where and when will the study be conducted?	
European countries involved in the trial	• Netherlands • Poland • Spain
Estimated start date of recruitment	August 2023

6. Information for your doctor			
Clinicaltrials.gov identifier	NCT06064890	EudraCT Number	2022-002568-62
Study contact information	clinicaltrials@aviadobio.com		
Link to full text	https://clinicaltrials.gov/study/NCT06064890		

✓ The information contained in this document is based on information available on public registries (e.g. clinical trials register website) on February 2024.