Alzheimer Europe

Making dementia a priority:
changing perceptions, practice and policy.

CLINICAL TRIALS WATCH

ACCESSIBLE EASY READ INFORMATION ON:

FOCUS-C9 STUDY

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FOCUS-C9 study

1. Study Information	
Name of the study	Study of WVE-004 in patients with C9orf72-associated Amyotrophic Lateral Sclerosis (ALS) or Frontotemporal Dementia (FTD) (FOCUS-C9)
Study sponsor	Wave Life Sciences Ltd.
Disease	Frontotemporal Dementia, Amyotrophic Lateral Sclerosis or both
Phase	Phase Ib/IIa

2. Information about the drug that will be tested in the study	
Name of drug	WVE-004
Administration	The study drug is given as an injection through a thin needle into the spinal canal (lumbar puncture).
Is the drug already on the market for another medical condition?	No
Will all participants receive the same drug?	Participants will be selected by chance to receive one of the following options: • WVE-004 (Dose A, B, C or D) • Placebo (also called a dummy treatment which is an inactive substance identical in appearance to the drug being tested with no active therapeutic effect). Neither the participant nor their doctor will know if the person is receiving the investigational drug or the placebo.

3. Information about participating in the trial	
What are the researchers trying to find out?	• The purpose of the study is to evaluate the safety and tolerability of WWE-004 in adults with documented mutation in the C9orf72 gene (GGGGCC repeat expansion) who have been diagnosed with Frontotemporal Dementia or Amyotrophic Lateral Sclerosis.
How long will the treatment last?	• 9-10 months

What your involvement will entail?	• During the study, participants will be asked to complete some laboratory/biological tests to evaluate the emergent adverse effects (unfavourable signs, symptoms or diseases temporally associated with the use of the drug tested in the study) as well as to understand how WVE-004 moves through and impact the body • Participants with Frontotemporal Dementia and its study partner will complete a test called CDR® plus NACC FTLD, which assesses memory, orientation, judgement, problem solving, personal care, community affairs, behaviour, and language • Participants will be asked to undertake brain scans (MRI) or lumbar punctures (for CSF sampling and administration of study drug) to see changes in biomarkers in the brain. Participants with mixed disease (Amyotrophic Lateral Sclerosis and Frontotemporal Dementia) need not undergo MRI if their Amyotrophic Lateral Sclerosis symptoms prevent it. Further information on the 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 18 to 80 years old Participants with Frontotemporal Dementia must: • Have a score of 0.5 or 1 in the Clinical Dementia Rating-Global Score- Frontotemporal Lobar Degeneration (CDR® plus NACC FTLD). This would suggest that the person has an impairment in their memory that is at a very mild stage • Able to undergo brain scans and lumbar puncture.

	Participants with Amyotrophic Lateral Sclerosis must: • Be diagnosed with Amyotrophic Lateral Sclerosis based on clinical disease • Clinically diagnosed possible, laboratory supported probable, probable, or definite criteria for diagnosing Amyotrophic Lateral Sclerosis according to the World Federation of Neurology revised El Escorial criteria • If the participant is taking riluzole, the dose must be stable for ≥30 days • If the participant is taking edaravone, he must have received it for a minimum of 1 cycle (28 days). Participants with mixed disease (Amyotrophic Lateral Sclerosis and Frontotemporal Dementia) must: • Meet above criteria for both Amyotrophic Lateral Sclerosis and Frontotemporal Dementia.
Who cannot participate in the study?	Exclusion criteria include: • A disease or medical condition that may interfere with the study assessments and will make the participant unsuitable for participation in or completion of the trial procedures • Any other investigational drug, biological agent, or device within 1 month and received an investigational oligonucleotide, within the past 6 months. The above list is not exhaustive.

5. Where and when will the study be conducted?	
European countries involved in the trial	• Belgium • Ireland • Netherlands • Sweden • UK
Estimated start date of recruitment	June 2021

6. Information for your doctor			
EudraCT Number:	2020-005193-94	**Clinicaltrials.gov identifier**	NCT04931862
Study contact information	clinicaltrials@wavelifesci.com		
Link to full text	https://clinicaltrials.gov/ct2/show/NCT04931862		

- ✓ The information contained in this document is based on information available on public registries (e.g. clinicaltrials.gov website) on November 2022.
- ✓ This document has been reviewed by the pharmaceutical company running this trial.