DESIGN OF AN INTERNATIONAL, PHASE 3 OPEN-LABEL EXTENSION STUDY TO INVESTIGATE THE LONG-TERM SAFETY OF DAILY ORAL EDARAVONE (FNP-122) IN ALS: THE ADOREXT STUDY

Leonard van den Berg(1), Ruben van Eijk(2)
Carla Varona(3), Esther Miralles(4), Claudia Roca(5), Tania Nadal(6), Cristina Tarragó(7), Nuria Albareda(6)

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Objetive

To describe/illustrate the study design of ADOREXT, a phase 3 open-label (OLE) study assessing the long-term safety of daily oral edaravone (FNP-122) in people living with ALS (pALS)

Effective treatment of ALS continues to be a significant unmet need.

Edaravone is thought to be a potent antioxidant and may slow the progression of ALS by protecting nerve damage.

ADOREXT trial design includes the voice of pALS

The ADOREXT protocol has been developed considering pALS' needs and perspectives collected in an ALS Patient Representatives Advisory Board (PAB)

8 patient organizations from Europe and North America participated.

The aim was to appraise the pALS' perception on the clinical protocol to improve accessibility to the trial to patients as well as to validate endpoints and study design.

- pALS Needs & Expectations
- Hospital Visits
- Study Design
- Safety Reporting & Additional Questions
- Treatment
- Questionnaires

Inclusion criteria

All pALS who completed the full study period in the main ADORE study and whom the investigator has no concern and judges tolerable for receiving treatment with FNP-122 from a risk and benefit point of view.

Endpoints

Primary endpoint

- *Nature, frequency, and severity of Treatment Emergent Adverse Events in the pALS*

Secondary endpoints

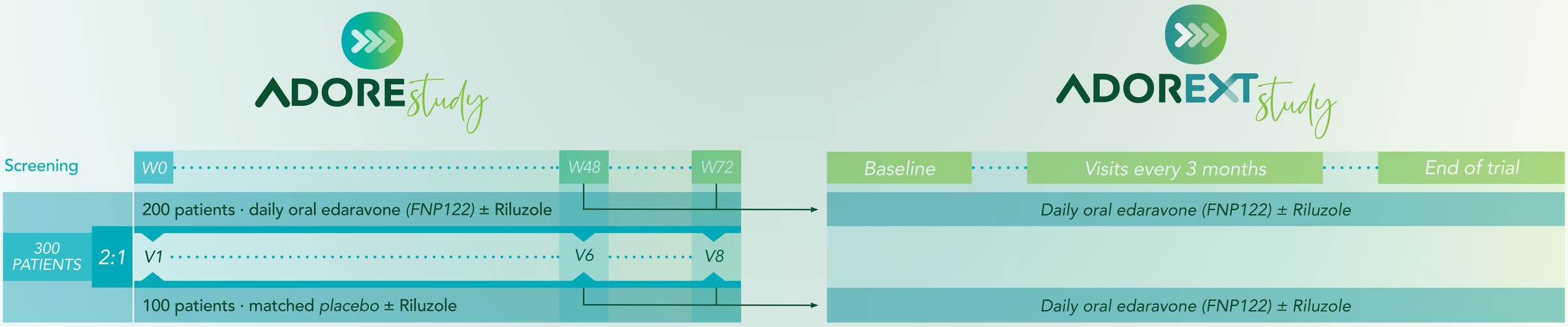
- **Mortality** · *mortality-adjusted change from baseline in ALSFRS-R total score until the end of the study*
- **Overall Survival**
- **Pulmonary function** · *Change from baseline in SVC*
- **Biomarkers** – *Change from baseline (neurofilament light, creatinine, creatinine kinase, 8-OHdG and Urinary p75ECD)*
- **QoL** · *Change from baseline in ALSAQ-40 and EQ- 5D-5L scores, VAS*
- **Cognition** · *ECAS*

Safety endpoints

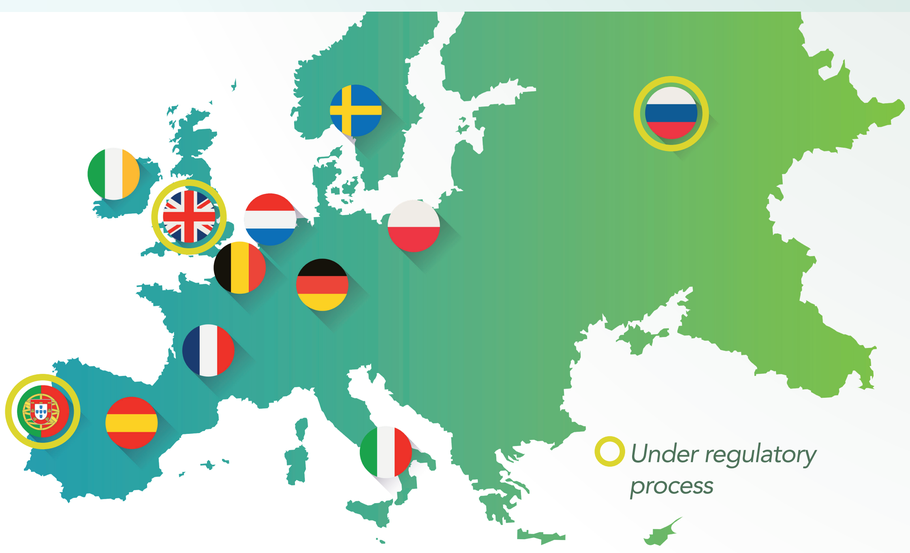
Study design

The ADOREXT study offers the possibility of receiving FNP-122 to all ADORE participants that complete the full study period

Patients who stopped treatment (for other than safety reasons) but completed the study period will be also invited to participate in ADOREXT



Roll over from the ADORE study to the ADOREXT study started in March 2023



TRICALS
The highway towards a cure

European Study conducted with TRICALS support

All sites that recruited pALS for the pivotal phase 3 ADORE study, most of them from the TRICALS Network, can participate in the OLE study ADOREXT.

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Conclusions

The ADOREXT study will provide relevant safety and long-term survival data on this daily oral formulation of edaravone (FNP-122)

TRICALS: Treatment Research Initiative to Cure ALS; **ALSFRS-R**: Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised; **SVC**: slow vital capacity; **pALS**: people living with ALS; **cALS**: caregivers for people with ALS; **PAB**: Patient Representatives Advisory Board; **ALSAQ-40**: Amyotrophic Lateral Sclerosis Assessment Questionnaire; **ECAS:** The Edinburgh Cognitive and Behavioural ALS Screen; **VAS**: visual analogue scales

REFERENCES: A Multicenter, Randomized, Double-blind, Placebo-controlled Study to Investigate the Efficacy and Safety of FAB122 in Patients With Amyotrophic Lateral Sclerosis (ADORE). ClinicalTrials.gov [Internet]. January 5, 2022. Accessed June, 2023. https://clinicaltrials.gov/ct2/show/NCT05178810; A Multicenter, Open-label Extension Study to Investigate the Long-term Safety of FAB122 in Patients With Amyotrophic Lateral Sclerosis (ADOREXT). ClinicalTrials.gov [Internet]. May 19, 2023. Accessed June, 2023. https://clinicaltrials.gov/ct2/show/NCT05866926.

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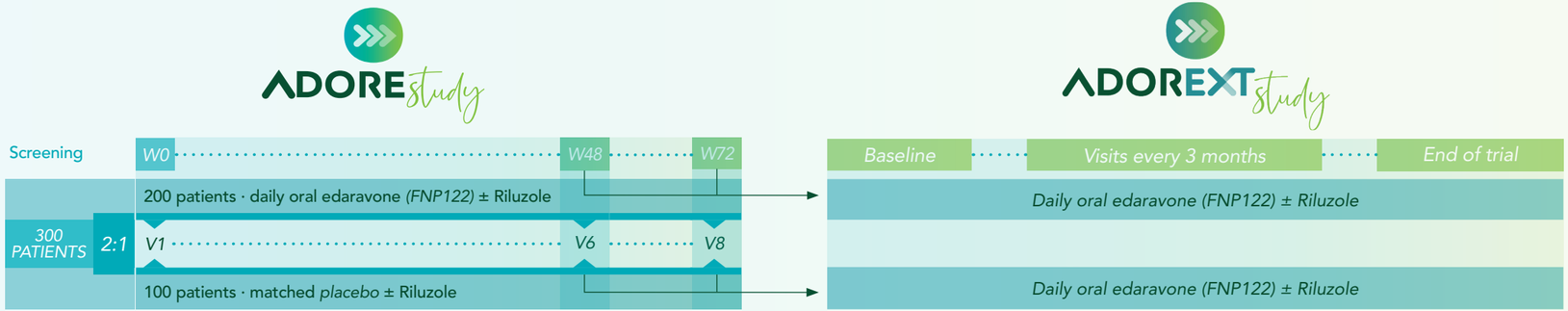

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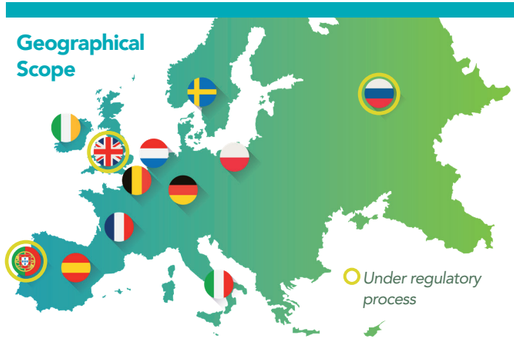
Geographical Scope



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ADOREXT study
ALS TRIAL WITH DAILY ORAL EDARAVONE EXTENSION

Graphical Abstract

Access the Graphical Abstract presented at the ENCALS 2023 meeting to describe the design of a Phase 3 trial, assessing the safety and the efficacy on disease progression and survival of once daily oral edaravone in patients with ALS

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ADOREXT study
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