



Uniting European ALS Associations to cooperate better



EUfpALS objectives

- Defending the rights of European people living with ALS (PLWALS)
- Creating awareness on European scale
- Providing access to & information about ALS research
- Creating same rules for ALS research across Europe

28 ALS Member Associations



22 European countries



17 Industry Partners





Academic Partners



ENCALS European Network to Cure ALS

Close collaboration



EUpALS Board of Directors



- Evy Reviers (ALS Liga Belgium; Chairwoman)
- Gudjon Sigurdsson (MND Iceland; Secretary)
- Sabine Turgeman (ARSLA France; Treasurer)
- Christian Lunetta (Post Fata Resurgo Italy; Director)
- Joaquin de la Herran (Fundacion Luzon Spain; Director)
- Aylin Yaman (ALS/MND Association Turkey; Director)
- Limore Noach (Stichting ALS The Netherlands; Director)

EUpALS Patients and Carers Expert Board



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ALS Sweden



Jan Bastiaens

ALS Liga Belgium



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ALS Liga Belgium



Angélique van der Lit –
van Veldhuizen

ALS patiëntenvereniging



Josefina Garcia Pastor

Fundació Miquel Valls Spain



Alper Kaya

ALS/MND Association Turkey

Input and feedback from a PLWALS perspective provided for:

- Clinical trial design
- Patient brochure
- Informed consent
- Market authorisation application
- Early Access programme

EUpALS Scientific Expert Board



Prof. Philip Van Damme
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Prof. Markus Weber
ALS Clinic, Kantonsspital
St.Gallen, Switzerland.
Professor of Neurology.
Neuromuscular Diseases unit/

Scientific advice on recent evolutions in ALS research; drafting opinion papers

EUpALS Working Groups



Genetic Testing

EUpALS
European Organisation for Professionals and Patients with ALS

Position Paper

Need of EU harmonization of ALS patient genetic testing

Hope emerges for ALS patients as ASO-based therapies for genetic subgroups become successful

After decades of repeatedly negative clinical trial results, hope is emerging for ALS patients as a clear milestone in ALS research has been reached, showing that ALS is a treatable disease after all.

Indeed, positive data from the Biogen's VALOR OLE study of the antisense oligonucleotide (ASO) Tofersen in patients with ALS based on an SOD1 mutation were published in the prestigious journal *The New England Journal of Medicine* (edition 2022; 387:1099-1110). The study shows that the therapy reduces motor nerve cell damage and that patients show stabilization in their disease progression. Based on these study results, the FDA approved QALSOLOGY (Tofersen) on April 25, 2023.

Tofersen is currently under evaluation by EMA for market authorization in the EU. Meanwhile, Biogen initiated a Compassionate Use - Early Access Program for Tofersen in the EU.

Having shown the therapeutic potential of an ASO to treat ALS, Biogen and several other companies are currently conducting similar ASO-based clinical trials in other genetic subgroups of ALS patients.

My genetic profile: an ALS patient's access key to ASO-based clinical trials and therapy

To gain access to an ASO-based trial and therapy, it is critical to an ALS patient to know his/her genetic profile. In addition to previous scientific knowledge that gene mutations are the underlying cause of familial ALS only (approximately 10% of patients), it becomes nowadays widely accepted that genes also play a role in sporadic ALS (90% of patients), as some of the ALS genes have a reduced penetrance.

Genetic profiling should therefore be offered to all ALS patients as part of the standard clinical practice at diagnosis.

Need of EU harmonization of ALS patient genetic testing

In accordance with the right of freedom of movement of EU citizens, genetic profiling should be harmonized throughout all EU member states, and preferentially expanded to the additional EEA and EFTA countries, as well as the UK.

In practice, results performed by a certified ALS genetic testing facility of a certain EU member state should be accepted in all other member states. Most appropriate, this can be implemented through an existing European network of centers of excellence, e.g. ENCALS.

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Market Access



In progress

Quality of Life

ALS COALITION

Amyotrophic Lateral Sclerosis, a rare neurodegenerative disease: European landscape assessment and policy recommendations for improved diagnosis, care, and treatment.

Let's act together now. Time is precious and running out fast for people living with ALS.

Co-authors and European ALS Coalition members:

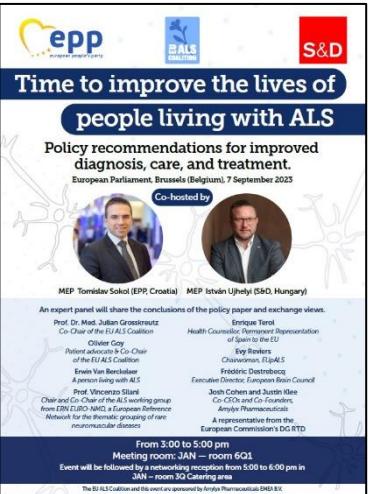
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Role of EUpALS in the EU ALS community



- EU ALS community: many stakeholders
 - patient organisations, academic researchers, clinicians, industry, ...
- EU ALS community: many initiatives
 - TRICALS, ENCALS, EU ALS Coalition, ...
 - EUpALS partner
 - Input of the PLWALS voice
 - Coordinator role: aligning the different initiatives
- Communication and feedback to the 28 EUpALS Member Associations
 - News from first line
 - Same communication at the same time

EUpALS and EU policy impact





More about EUpALS

- Website [www\(ALS.eu](http://www(ALS.eu)
- Facebook.com/EUpALS/
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