



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.

Andrea Gasper, on behalf of the European ALS Coalition

Conference on Rare diseases and the European reference networks. Side event on “Amyotrophic lateral sclerosis – The perspective of patients' associations”

Bilbao, 10th of October 2023

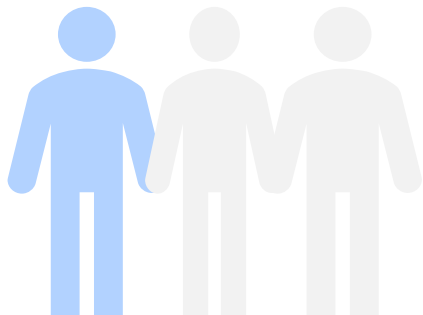
Why ALS?

Amyotrophic Lateral Sclerosis is characterised by a relentless and progressive loss of muscle function and strength

Universally fatal.

~**32,000** people are living with ALS.

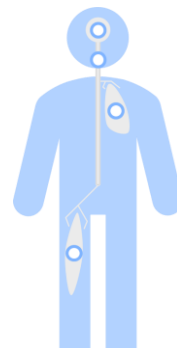
1 in every 300 will develop ALS in their lifetime.



Multifaceted presentation.

Impacting ability to **speak, swallow, walk, and breathe.**

Highly complex to manage.



Extremely rapid progression.

Average life expectancy for someone with ALS is **2 – 5 years** after symptom onset.



The European ALS Coalition represents multistakeholder views with the aim of fostering a positive policy environment to support access to optimal diagnosis and care for PLWALS



A **diverse range of experts** from across **Europe**, supported by MEPs (ambassadors for the initiative), form the European ALS Coalition including:



Academic researchers



Healthcare professionals



PAGs and PLWALS



Multidisciplinary care experts



European Commission representatives



Former HTA representatives



Payers



Pharmaceutical industry representatives



Ethicist



The **first initiative** of the European ALS Coalition was to produce a **policy paper** that aimed to:



Raise awareness and build a better understanding of the disease burden and unmet needs in ALS among key policy stakeholders



Provide policy recommendations and create a sense of urgency for improvement in holistic care and access to treatments for PLWALS across Europe

The policy paper was launched on the 7th of September 2023 in the European Parliament

Name	Role / Title	Country
Prof. Dr. med. Julian Grosskreutz	Coalition Chair, Physician Chair of Precision Neurology, University of Lübeck	Germany
Olivier Goy	Coalition Vice-Chair, PAG Patient representative and advocate	France
Sheela Upadhyaya	Coalition Moderator, HTA expert & Payer Life Sciences Consultant in Rare Diseases	UK
Dirk De Valck	Patient organisation representative Staff member, EUpALS	EU
Karolina Koucká	Patient organisation representative Deputy, ALSA Czechia	Czech Republic
María José Arregui	Patient organisation representative Executive President, Fundación Luzón	Spain
Tatiana Foltánová	Patient organisation representative Slovak Alliance for Rare Diseases (Aliancia ZCH)	Slovakia
Andrea Gasper	MDT carer Coordinator Hospice and Palliative Care Network Bonn/Rhein-Sieg, Case Manager Special Outpatient Clinic for Amyotrophic Lateral Sclerosis University Hospital Bonn	Germany
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Prof. Orla Hardiman	Physician Professor of Neurology/Head of Academic, Clinical Medicine, Trinity College Dublin	Ireland
Antoni Montserrat Moliner	EU policy-maker Active Senior on Public Health, European Commission	EU
Prof. Fabrizio Gianfrate	Payer Professor of Health Economics and Outcome Research, Universities of Rome and Ferrara; Market Research Consultant; Past AIFA Member; Former Director of Ministry of Health	Italy
Prof. Pier Luigi Canonico	Payer Professor of Pharmacology, University of Piemonte Orientale; actual president of ISPOR Italy	Italy
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European ALS Coalition ambassadors

Member of European Parliament	Party	Country
István Ujhelyi	Progressive Alliance of Socialists & Democrats	Hungary
Jordi Cañas	Renew Europe	Spain
Ondrej Knotek	Renew Europe	Czechia
Stelios Kympouropoulos	European People's Party	Greece
Susana Solis Perez	Renew Europe	Spain
Tomislav Sokol	European People's Party	Croatia

What do people with ALS need?

Optimised diagnosis



- **Shorten time** to diagnosis
- **Reduce** misdiagnoses
- **Expert-led** and continuous

Matched care



- **Proactively match** needs
- **Plug gaps** in care
- **Strengthen** social care

Better prognosis



- **Prioritise** approval and access
- **Minimise** barriers
- **Ignite** further innovation

Key insights



12-month diagnostic period from symptom onset



3 physicians before diagnosis confirmed, delays due to:

- Relatively low levels of **ALS knowledge** and training amongst GPs
- **Fear** in giving devastating diagnosis



Unnecessary tests delay timely initiation of care and increase healthcare costs



Frequent **lack of continued observation** post-diagnosis



Once ALS diagnosis confirmed:

- PLWALS and their families **fearful and uncertain** of what is to come
- **Immediate changes to work, home, and everyday life**
- **Significant psychological impact** of processing ALS diagnosis



Little help or information provided on available management and support options



It's a shock and from this shock nothing will be better, in fact I have to learn to live a life in which death reigns.






— Olivier Goy, *person living with ALS*

Policy recommendation

① Enhance capabilities of primary care physicians and other first line health providers to conduct timely referrals to NMD/ALS specialists, and ensure expert-led diagnostic assessment, subtype characterisation, and continued evaluation is conducted from point at which ALS is suspected

② Provide counselling and support to PLWALS and their families on ALS, receiving an ALS diagnosis, and on disease management options

Key insights

-  **Shortage of specialists** and expected **20% increase of PLWALS** by 2040
-  **Specialist centres are scarce** and concentrated in **urban areas**
-  Care is often **reactive**, resulting in mismatch in needs with type and timing of care
-  Social care system typically have limited capacity and places administrative hurdles in way of accessing **assistive technology devices** (ATDs)
 - **Lack of consistency in criteria** for granting disability cards and **slow** provision
 - Significant **variability in time** taken to receive aids
 - If ATDs not supplied on time or insufficient, PLWALS often pay **out-of-pocket**
-  **Home assistance is limited** in some countries and existing long-term care options are typically not appropriate
 - **Little respite** available, and caregiving often becomes a **full-time job**
 - **Emotional, physical and financial toll** on families and caregivers



The mismatches between patient needs and the care they receive may result in unnecessary complications and further reduces the little time they have with their families.

— Prof. Dr. med. Julian Grosskreutz, Chair of Precision Neurology, University of Lubeck

Policy recommendation

- 3 Increase co-ordination between ALS and non-ALS specialists, and involvement of essential multidisciplinary professionals, leveraging alternative approaches to care, collaboration, and communication
- 4 Speed up access to fully reimbursed assistive technological devices for PLWALS
- 5 Improve availability of non-hospital-based care for PLWALS unable to remain in their home as their disease progresses
- 6 Recognise the essential role of family and caregivers in the care of PLWALS and provide appropriate social, psychological, and financial supports

Key insights



Only one treatment for ALS approved in **Europe** more than 25 years ago



Challenging conditions for R&D for rare and complex diseases such as ALS, due to disease complexity and limited understanding, and heterogenous population



Progress is being made, with an **imminent pipeline** of promising treatments



PLWALS should have **opportunities to participate** in clinical trials and **logistical barriers** should be minimised



Little understanding of ALS disease mechanism, with research implications:

- **Difficult to identify and select** appropriate clinical trial design elements
- Frequent **disconnect** between research in laboratory and clinical practice, and in what is considered **acceptable** by decision-making bodies



Evidence generated may not meet standards of all decision-makers and cause avoidable delays for or limit patient access



Solutions tackling urgency and high unmet needs in **regulatory and national processes** needed



The latest innovative treatments bring hope of invaluable months of extra life... except their authorization is slow in Europe, to the great displeasure of the patients who often don't have time to wait.

— Olivier Goy, person living with ALS

Policy recommendation

7 Augment research to further disease understanding and treatments for ALS and increase awareness of and accessibility to ALS clinical trials

8 Improve alignment between researchers, clinical experts, medicine developers, regulatory and payers on trial design (incl. acceptable clinical trial endpoints) accounting for patient evidence and opinions

9 Expedite and support the approvals of new treatments for PLWALS considering the urgency and breadth of their unmet needs, ensuring ALS expertise is accounted for in drug evaluations

10 Provide timely access to new treatments targeting life-threatening diseases with extremely high unmet need via fast-track and conditional reimbursement processes, that consider the holistic value of medicines

**People living with ALS have no time to wait,
we need to act now!**

**Optimised
diagnosis**



**Matched
care**



**Better
prognosis**



If we solve the challenges in ALS, we can tackle other severe and complex neurological disorders!



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