

Speeding up research to improve the lives of people with ALS



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Advances in research over the past few years have provided a greater understanding of the pathophysiology, genetics, and risk factors for amyotrophic lateral sclerosis (ALS). Patient activism is also gaining momentum, with the aim of raising awareness and bolstering funding for further research. The urgent need to accelerate drug development and clinical trials has been the impetus behind the creation of several European initiatives, including the UK Motor Neuron Disease Research Institute (UK MND RI), the European Network for the Cure of ALS (ENCALS), and The European ALS Coalition.

The UK MND RI mission was co-developed by patient campaigners and researchers to target the gap between fundamental research and clinical trials, with three core aims: to create a drug discovery and development programme; to deliver a sustainable platform for conducting clinical trials; and to implement a research programme to understand the disease and the patients' experience. The first phase of this work began in January, 2023, at six academic centres in the UK. The coordinated national nature of the UK MND RI has enabled the generation of collaborations where none existed before. This consortium has also launched projects that would not otherwise have been funded. For example, a unique cell model institutional network has been created, so that a finding in one laboratory can be validated in another using a single set of cell lines and a harmonised protocol for deriving cell types relevant to ALS. Another project, EXPERTS-ALS, a clinical platform for the study of experimental drugs, will begin enrolling participants in 2024 and will enable expedited screening of candidate drugs in people with ALS in an open-label design (ie, to identify drugs to be tested in larger clinical trials). The platform is expected to provide an efficient method to prioritise drugs for phase 3 trials, give people with ALS more rapid access to new therapies by increasing the speed of research, and address any concerns of participants regarding trial duration and methods.

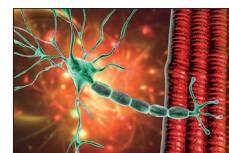
The UK MND RI complements and is closely linked with ENCALs, a network of ALS centres in Europe focused on clinical practice and research. Population registry and biomarker data, as well as genomics and epigenetics data, are shared between the UK MND RI and a major project of ENCALs, PRECISION ALS, which is a platform to collect multimodal patient data, with the aim to

provide granular information about ALS across Europe. This initiative aims to harmonise all data definitions and standard operating procedures, for example, and enable data sharing across Europe and the UK. Clinical trials are supported across both the UK MND RI and ENCALs through the TRICALS Consortium, the largest European research initiative for ALS to date, involving a subset of 48 centres from ENCALs that are focused on research.

To speed up the translation of research findings into clinical advances, ALS must become a priority on the European policy agenda. To this end, a policy paper was launched on Sept 7, 2023, by The European ALS Coalition at the European Parliament in Brussels, Belgium. The Coalition, which brings together experts with varied experience and different perspectives on ALS in Europe, was initiated by the patient advocacy group EUPALS in collaboration with six Members of the European Parliament. In its policy paper, the Coalition draws attention to the challenges that patients face when receiving a diagnosis of ALS, highlights gaps in research and unmet needs in relation to diagnosis, care, and prognosis, and provides actionable policy recommendations to address these needs. Ten policy recommendations, intended as a roadmap for policymakers at the European and national level, are provided to address the requirements for optimised diagnosis, a proactive model of care that matches the needs of people living with ALS, and improved prognosis. The paper also draws attention to the need for coordinated funding across Europe, and discusses the regulatory review by the European Medicines Agency of submissions for approval of new therapeutic agents, emphasising the importance of including people with specific expertise in ALS as members of the review panels, which is not always the case currently.

Policy changes, such as those called for by the European ALS Coalition, remain crucial to improve the lives of people with ALS and their families. However, with strong research infrastructures, such as those of the UK MND RI and ENCALs, excellent collaboration between academia and industry in partnership with people with ALS, and expedited regulatory approval, a range of new disease-modifying therapies for ALS could be available in the not-too-distant future.

■ *The Lancet Neurology*



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For more on **research advances** see **The Lancet Neurology Series on amyotrophic lateral sclerosis** <https://www.thelancet.com/series/amyotrophic-lateral-sclerosis>

For more on **patient activism** see **Feature Lancet Neurol 2023; 22: 204-05**

For more on the **UK MND RI** see <https://ukmndri.org/>

For more on **ENCALS** see <https://www.encals.eu/>

For more on the **European ALS Coalition** see <https://www.alscoalition.eu/>

For more on **EXPERTS-ALS** see <https://ukmndri.org/2023/09/01/new-programme-to-move-potential-mnd-drugs-into-clinical-trials-faster/>

For more on the **PRECISION ALS initiative** see www.precisionals.org/

For more on the **TRICALS Consortium** see <https://www.tricals.org/en/>

For the **European ALS Coalition policy paper** see <https://www.alscoalition.eu/European-ALS-Coalition-Policy-Paper-2023.pdf>

For more on **EUPALS** see <https://als.eu/>