



June 9, 2023

## Open Letter by the EUpALS Patients and Carers Expert Board

### **European people living with ALS/MND strongly hope that EMA CHMP's trending negative opinion for AMX0035 can be reversed**

Dear Madam, Dear Sir,

We are the Patients and Carers Expert Board of EUpALS - the European organisation for Professionals and Patients with ALS.

We suffer from Amyotrophic Lateral Sclerosis (ALS) / Motor Neurone Disease (MND), or care for a loved one who is living with this neuro-muscular disease that is characterized by progressive paralysis that precedes death on average 33 months after diagnosis.

There is still no cure for ALS. The only medicine at our disposal is riluzole, a drug with a limited effect on slowing the devastating symptoms of our disease. Since its approval by the European Medicines Agency (EMA) back more than 2 decades ago, the broad community of about 50.000 European people living with ALS eagerly await additional therapies to combat loss of mobility, talking, swallowing, eating and breathing.

Needless to say that we cherish strong hope that the EMA Committee for Medicinal Products for Human Use (CHMP) provides a positive opinion on conditional marketing approval of AMX0035, an investigational ALS therapy under development by Amylyx Pharmaceuticals.

However, with great concern we learned that the CHMP is trending towards a negative opinion\*.

Any new potential therapy that benefits the quality of life and ideally also the prognosis of people living with ALS – even based on phase 2 clinical trial results while waiting for confirmation by a phase 3 trial - is therefore eagerly awaited by the broad community of patients and their families. Delay in availability of additional ALS therapies would therefore be disastrous for the entire generation of European people living with ALS that fight their disease today.

From the everyday life perspective of people living with ALS, changes rated on functionalities that may be only subtle to the healthy person, make a world of difference to patients. Indeed, even clinical effects that are not (yet) statistically proven relevant can keep us independent, maintain

our quality of life, and avoid a lot of anxiety and stress for us as well as our loved ones. Because images say more than words, we invite you to watch our enclosed video that compiles testimonies of people living with ALS.

As passionate advocates for people living with ALS, we strongly hope that EMA CHMP's trending negative opinion for AMX0035 can be reversed. In that regard, broadly distributing our message to target the European policy making community is of utmost importance.

Sincerely,

The EUpALS Patients and Carers Expert Board

### About EUpALS and the EUpALS Patients and Carers Expert Board

The European Organisation for Professionals and Patients with ALS (EUpALS; [www.ALS.eu](http://www.ALS.eu)) unites 28 national ALS associations from 22 European countries. We create equal rights for all European ALS-patients by facilitating broad access to ALS clinical trials in Europe.

To ensure that the patients' needs are properly addressed, EUpALS established the Patients and Carers Expert Board. It brings together ALS-patients in different stages of the disease, complemented with informal caregivers. The composition can be viewed at [https://als.eu/expert\\_board](https://als.eu/expert_board).

### Source

\* Press release 'Amylyx Pharmaceuticals provides update on ongoing regulatory review of AMX0035 for the treatment of ALS in the European Union' via <https://investors.amylyx.com/press-releases>

### Endorsed by



### European organization for Professionals and Patients with ALS (EUpALS) ivzw

Registered office: Vaartkom 17, B-3000 Leuven, Belgium

VAT: BE 0684.923.631 – Commercial Tribunal of Leuven

Tel: +32 (0)16-23 95 82 – [info@ALS.eu](mailto:info@ALS.eu) – [www.ALS.eu](http://www.ALS.eu)