A proposal by patient advocates members of ACT EU MSP AG

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Presented by:

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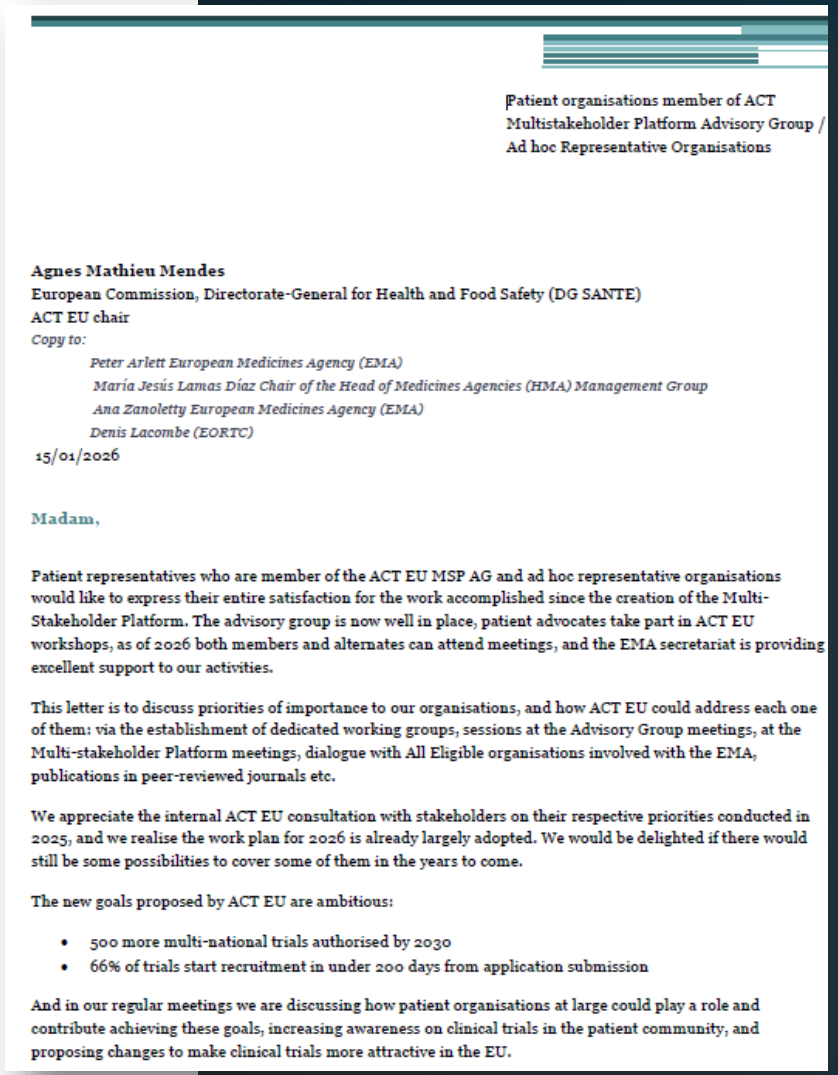
Our 12 priorities

ACT EU MSP AG Patient Advocates letter to ACT EU, 15 January 2026

- Erik Briers (Europa Uomo)
- Nikos Dedes (European Aids Treatment Group)
- François Houÿez (European Organisation for Rare Diseases EURORDIS)
- Amelia Hursey (Parkinson Europe)
- Begonya Nafria, Segolène Gaillard (European Young Person's Advisory Groups Network)
- Michel Rataj, Claudia Louati (European Patient Forum)
- Dina de Sousa (EuroHuntington)
- Juan Ventura (Cancer Patients Europe)



Patient organisations member of ACT
Multistakeholder Platform Advisory Group /
Ad hoc Representative Organisations

Agnes Mathieu Mendes
European Commission, Directorate-General for Health and Food Safety (DG SANTE)
ACT EU chair
Copy to:
Peter Arlett European Medicines Agency (EMA)
María Jesús Lamas Díaz Chair of the Head of Medicines Agencies (HMA) Management Group
Ana Zanoletty European Medicines Agency (EMA)
Denis Lacombe (EORTC)
15/01/2026

Madam,

Patient representatives who are member of the ACT EU MSP AG and ad hoc representative organisations would like to express their entire satisfaction for the work accomplished since the creation of the Multi-Stakeholder Platform. The advisory group is now well in place, patient advocates take part in ACT EU workshops, as of 2026 both members and alternates can attend meetings, and the EMA secretariat is providing excellent support to our activities.

This letter is to discuss priorities of importance to our organisations, and how ACT EU could address each one of them: via the establishment of dedicated working groups, sessions at the Advisory Group meetings, at the Multi-stakeholder Platform meetings, dialogue with All Eligible organisations involved with the EMA, publications in peer-reviewed journals etc.

We appreciate the internal ACT EU consultation with stakeholders on their respective priorities conducted in 2025, and we realise the work plan for 2026 is already largely adopted. We would be delighted if there would still be some possibilities to cover some of them in the years to come.

The new goals proposed by ACT EU are ambitious:

- 500 more multi-national trials authorised by 2030
- 66% of trials start recruitment in under 200 days from application submission

And in our regular meetings we are discussing how patient organisations at large could play a role and contribute achieving these goals, increasing awareness on clinical trials in the patient community, and proposing changes to make clinical trials more attractive in the EU.

First priority:

Patient involvement in clinical trial design and conduct

- ICH E8 guideline on general considerations for clinical studies
  - “Engaging stakeholders in study design and informing how patients’ representatives were engaged”
- Industry sponsors set their own advisory boards with patients and clinicians, public research organisations sometimes also
  - Often limited to the review of information and consent letters
  - In paediatric trials, (young) patients are much more rarely involved
- Patients’ organisations developed their own independent approach in the 90s (haemophilia, cancers, AIDS)
  - Setting their own **Community Advisory Boards**
  - Cf ACT EU workshop on ICH E6 in February 2025, Sally Hofmeister (Duchenne CAB) and Hilde de Keyser (CF-E CAB)

CAB MEMBERS

Patient experts:
- Trained on clinical research
- Can discuss all aspects of the trial relevant to patients
- From its design to practical aspects

A group of patients who offer their expertise to public or private sponsors of clinical research

Is the medicine needed? Fow whom?

Overall programme development, a single clinical trial

PROMs, Compassionate use, pricing and access, side effects…

The same patients advise several sponsors in their field

Avoids selection of patients' representatives by the sponsor

With or without clinicians? Patients' representatives tend to fade into the background in their presence

Agenda and secretariat driven by the patients

Guidelines, SOPs, templates, decision tracker etc.

Companies engage with CABs to discuss unmet needs, impact of the disease, endpoints etc.: are mutual expectations met?

Quality of trial but also recruitment and retention are improved, trials can be authorised faster

A proposal

A pilot, under the auspices of the EMA as organiser

Adapting the concept of Community Advisory Board

For 2 or 3 Community Advisory Boards

Following a call for expression of interest? Other approach?

More than 2 patients invited to Scientific Advice, between 10 to 20

With possible discussion on innovative trial designs

Platform trial, basket trial, Bayesian approach…

Or on innovative technologies

Eg Technology platform / Regulatory Sandbox as per Biotech Act

Many questions

A need for guidance on CABs (for POs)? Other models than CABs?

Some protocols are global, how to organise the involvement of patients? Are they experiences with global or with regional approaches?

What do regulators expect to see reported by sponsors in terms of patient involvement?

When do we know patient involvement is meaningful, or pure tokenism?

How to incentivise patient involvement? Patient-experts and sponsors

Same principles for for-profit versus academic research?

Should Data Safety Monitoring Board include patients? For all trials, or some?

Beyond the design and the conduct, can patient and their organisations be involved in the explanation of results to participants?

Can trial participants contact CAB members, or patients involved with sponsors? In which situations, how?

Can CABs engage with regulators or ethics committees if needed?

During the authorisation phase of a clinical trial, patients are not consulted. How could authorities engage with them?

Can we measure impact?

Thank you!

And dicussion