Report on

Scientific webinar October 27, 2021

11h00	**Welcome**	Evy Reviers - EUpALS
11h05	**Role EMA and how patients are involved**	Maria Mavris - EMA Public and Stakeholder Dept.
11h50	**Update on start Amylyx PHOENIX trial**	Machelle Manuel and Stéphanie Hoffmann - Amylyx
12h00	**Update on closing Alexion CHAMPION trial**	Walter Atzori - Alexion
12h10	**Introduction to Effik**	Claudia De Wannemaeker - Effik
12h15	**Closing**	Evy Reviers - EUpALS

As the European Medicines Agency (EMA) is the key player in the regulatory process leading to potential market authorisation of ALS therapies under clinical development, we are very grateful to **Maria Mavris of the EMA Public and Stakeholder Department**, for having presented an **introduction to EMA and how patients can be involved in EMA activities**.

The integral EMA presentation can be found here.

Most important **questions that were addressed to EMA** were:

- Q : What is the time gain that an Orphan Designated Medicine can make as compared to a medicinal product that follows the regular pathway?

  A : There is no specific time gain for a medicine that receives Orphan Designation, so it does not speed up the evaluation process. It gives however certain benefits and incentives. The developing small or medium sized company or academic group can receive scientific advice at reduced cost or even for free. Also, when receiving market autorisation, the Orphan Designated medicine gets 10 years of market exclusivity.
  If a company likes to have an excellerated review for its medicine, it can follow the PRIME patway.

- Q : Are you aware of any application to obtain Orphan Designation for a potential ALS Medicine that was not designated? If so, what was the main reason?

  A : If the EMA COMP signals to the company that it intends to give a negative advice to the European Commission, the company can withdraw its application. As a result, only few non-Orphan Designations appear in the public domain.
  Positive opinions for Orphan Designation must meet certain criteria like :
    - the product must prevent, diagnose or treat a life-threatening or debiliating condition
    - not affecting more than 5 in 10.000 persons in the European Community
    - showing significant benefit over already autorised medicine(s) for that condition

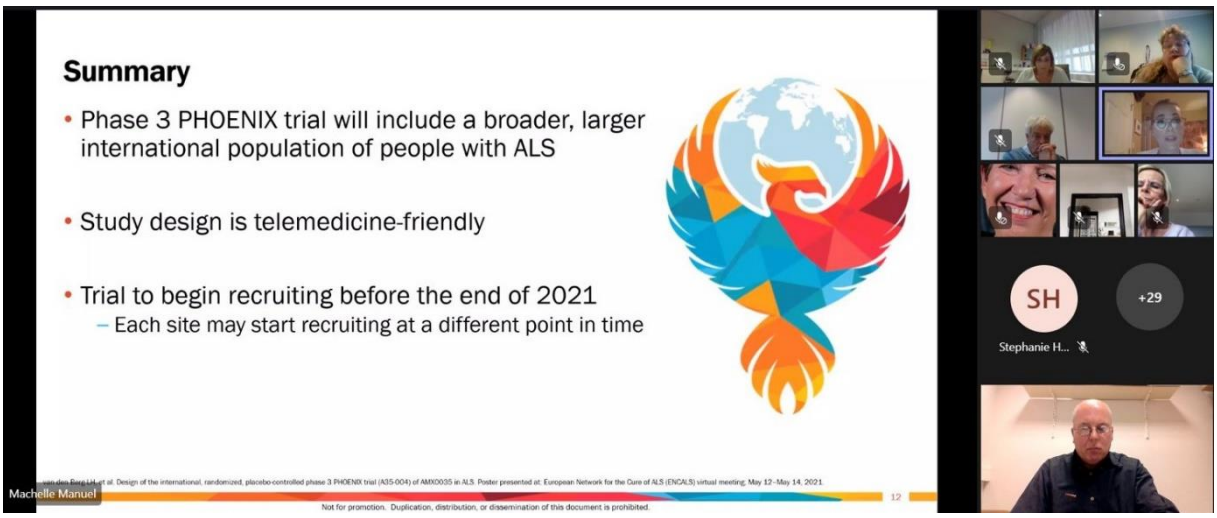
- Q : Has EMA advice for the ALS patients that engage with them, who feel hughe (social media) pressure from their fellow-patients to get additional information and to speed up clinical development of potential ALS medicines?

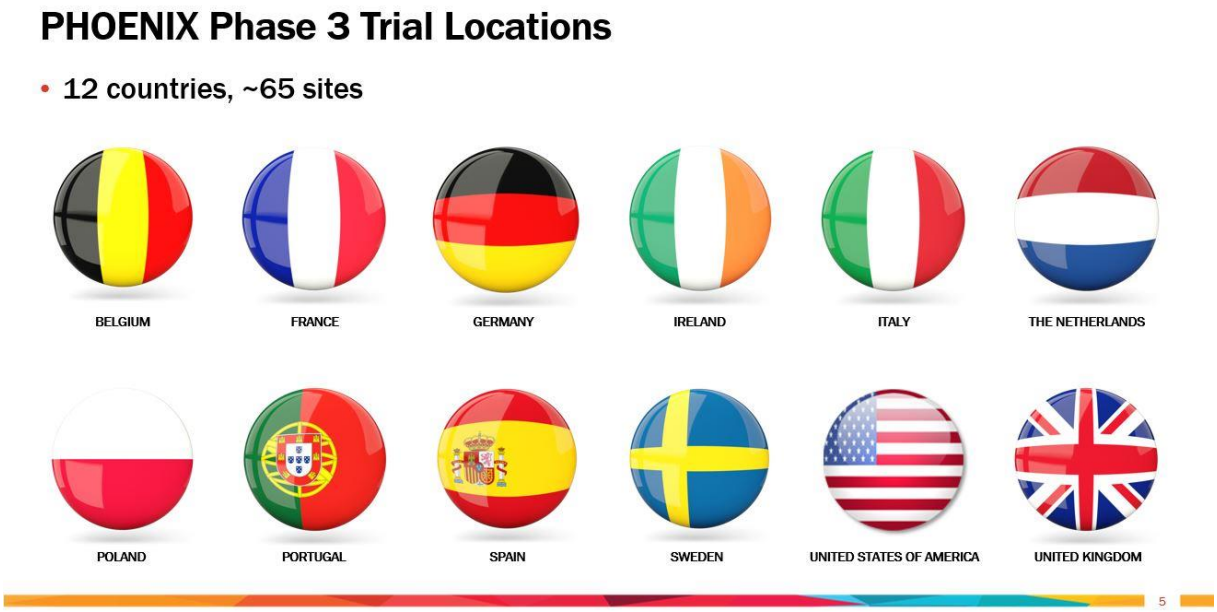
  A : EMA understands the sense of urgency to develop ALS medicines as quickly as possible. But managing the time line is not only at the level of EMA, but also at the level of the developing companies. It is a big network that has to work together.

- Q : Can EMA – to a certain extend – understand that such fellow-patientsunjustly think that 'too little, too slow' is happening in clinical development of an ALS medicine?

  A : Yes, but the risk-benefit balance should be safeguarded by a regulatory agency.

Amylyx presented an update on the start of the PHOENIX Phase 3 trial with AMX0035 in ALS.





Most important **questions that were addressed to Amylyx** were:

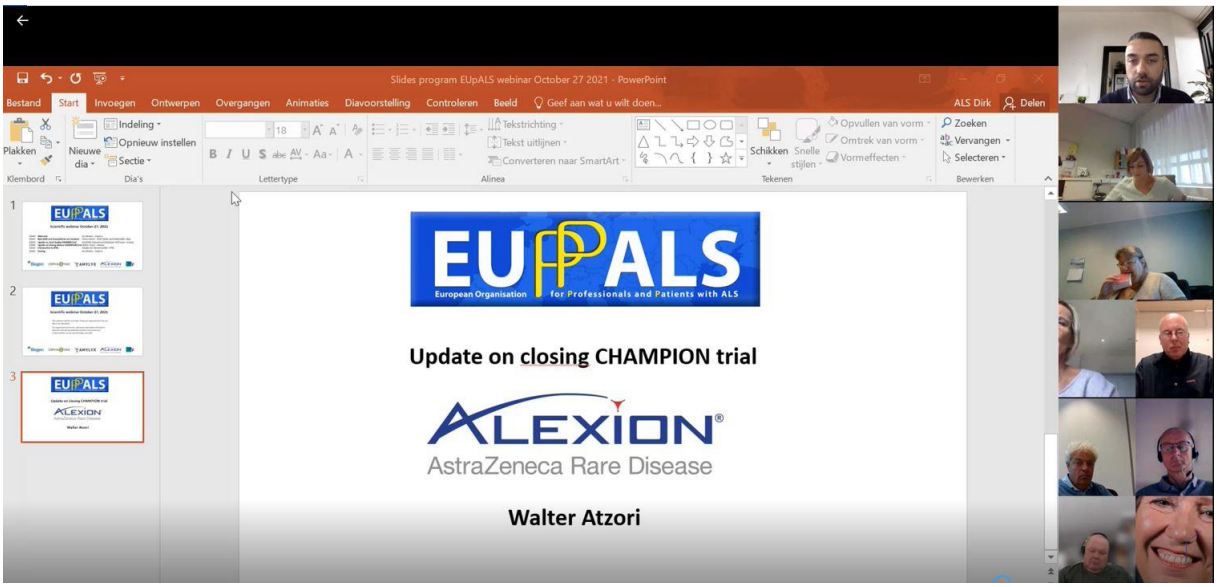
Q : Are the site names in the above listed countries already known?

A : Not yet. They will gradually be added to the 'contacts and locations' section of the ClinTrial.gov PHOENIX trial form as soon as they get approval.

Q : Where can ALS patiënts outside the above listed countries find information on the PHOENIX trial?

A : They should speak to their treating physician in first place. Furthermore, a specific PHOENIX website will be launched. Also, they can talk directly to Amylyx.

Alexion presented an update on closing of the CHAMPION trial with Ultomiris in ALS.



Most important **question that was addressed to Alexion** was:

Q : Will study participants be informed of whether they were on placebo or intervention arm?

A : Some people in the study have already been informed by their doctor if they were on the intervention group or not because they are potentially considering joining a different study and therefore need to know if they can be eligible. We will inform anyone who has not already been told what their treatment assignment was when the final study close takes place.

Finally, **EFFIK provided an introduction to the company**

Most important **question that was addressed to EFFIK** was:

Q: Is Teglutik – the liquid formulation of riluzole that EFFIK has commercially available – accessible for all ALS patients in Europe?

A: Teglutik is already available in Belgium, Germany, Spain, Italy, Greece, Switzerland, UK and France. In the Netherlands we are busy to launch, and also in Luxembourg it will soon be available.

We thank all presenters and attendees.
Stay tuned for future editions of EUpALS webinars!