Patients Involved – Patient organisation promotes Phase I clinical trial

Introduction

Involvement of the charitable foundation ‘Children with SMA’ (spinal muscular atrophy) to establish contact with the academic institution ‘Institute of Neurology, Psychiatry and Narcology of NAMS Ukraine’ to design a pilot clinical trial addressing issues in SMA clinical trials.



Where in the process? – Phase I

When does it happen? – Phase I

Description of the case

Although specific medications with clear clinical benefit is absent from treatment of patients with spinal muscular atrophy (SMA), both doctors and patients’ families widely use numerous off-label medications and physiotherapeutic interventions in Ukraine. A second problem is SMA-patients’ lack of mobility due to health risks and infrastructure restrictions. Also there are commonly accepted difficulties with treatment outcome measurement in SMA.

The problems mentioned above are complicated by unwillingness of pharmaceutical companies to conduct either clinical trials or preclinical research in Ukraine, as well as lack of state investments in rare disease research.

Our organisation, CSMA, established contacts with the academic institution ‘Institute of Neurology, Psychiatry and Narcology of NAMS of Ukraine’, which provided us with an expert-volunteer who designed a pilot clinical trial addressing most of the issues mentioned before in SMA clinical trials. Our collaboration also provided us with access to an institutional Ethic Committee which allowed independent ethical assessment of our projects by experts.

Main objectives of the trial to start in January – February 2015, is to prove the possibility of conduction SMA clinical trials in Ukraine, and to find most convenient forms and designs for their organisation. The protocol of the trial has been already approved by the Ethic Committee.

Type(s) of patient (advocates) involved

- Patients with personal disease experience
- Expert patient / patient advocate with good expertise on disease and good R&D experience.

Benefits of patient involvement

Our project’s main benefits are the promotion of R&D in SMA, optimisation of study designs, and developing collaboration between academic / research institutions and patient organisations in Ukraine. As far as such experience is absent in Ukraine, we look forward to obtain valuable information about specific national pitfalls and obstacles in SMA research.

Challenges and barriers

Most obvious challenges and barriers are lack of specific experience in SMA R&D in Ukraine, low motivation of researchers or relevant institutions, and incompetence of representatives mainly of patient community in evidence based medicine principles, as well as in general issues of R&D.

Learnings

At this stage of the project it is possible to state that despite of sufficient scientific and medical infrastructure in Ukraine, motivation for SMA R&D, both in industry and in academic researchers, is low; a key factor for successful advance in SMA research is sufficient number of experienced and motivated researchers as well as patient representatives and R&D EU experts.

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Attachments

- Patients Involved Case Report: Patient organisation promotes Phase I clinical trial
  Size: 856,309 bytes, Format: .pdf
  An infographic describing a case of involvement of a spinal muscular atrophy foundation with an academic institution to design a pilot clinical trial addressing issues in SMA clinical trials.