



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Executive Director

Letter of support for Patient Data Platform for capturing patient-reported outcome measures for Dravet syndrome

On 09 December 2015 the applicant Dravet Syndrome Foundation Spain requested qualification opinion for Patient Data Platform as an electronic tool for capturing patient reported outcomes in paediatric epilepsies, pursuant to article 57(1)(n) of regulation (EC) 726/2004 of the European Parliament and of the Council.

During its meeting held on 11-14 April 2016, the SAWP agreed on the qualification advice to be given to the applicant. During its meeting held on 25-28 April 2016, the CHMP adopted the advice to be given to the Applicant.

The sponsor seeks qualification opinion for their proposed "Patient Data Platform" (PDP) as a patient-reported outcome measure (PROM) to be used within drug development for paediatric epilepsies.

The Patient Data Platform has been designed by a patient organization with patient needs in mind, and is primarily a tool to improve comprehensive patient care by facilitating patient data capture and integration as well as to produce reports and summaries that can be shared with physicians. As such, it is patient-friendly and brings direct benefit to patients and caregivers. We believe that using the Platform for capturing PROs in the context of drug development will not only provide high quality patient-reported data but also reduce the burden on patients and caregivers to complete separate questionnaires or surveys during clinical trials, therefore improving compliance.
(http://www.ispor.org/workpaper/patient_reported_outcomes/Coons.pdf)

The idea is that it will support longitudinal tracking of patient symptoms (including PROs) and treatment and therefore be a useful tool for clinical trials in paediatric epilepsy.

It is proposed to be used through a computer or mobile phone interface so that patients or caregivers would be able to store and manage medical data in a single place. The platform consists of a number of 'data modules' and is intended to allow caregivers and/or paediatric patients with epilepsy themselves to collect/store not only medical records and genetic as well as phenotypic information, but also daily dynamic information on seizure count, type and duration, use of medications, temperature and sleep quality, etc.

As stated by the applicant the proposed data modules have been designed by caregivers of paediatric patients with epilepsy in order to capture the most relevant clinical data and outcomes for their disease, but the modular nature of the Platform will permit that new modules can be added with specific data requirements in an ad hoc basis.

The idea of systematically and proactively collect "from the inside" (the patient's side) disease related information in nearly real time and in a user (patient/caregiver)-friendly way so that it can be pooled



and shared “online” deserves full consideration. An approach to incorporate up to date communication technologies to these type of situations within the regulatory context was, perhaps, long overdue. For this reason the current proposal, sponsored by a patients’ organization, is welcome. Their elaborated project aims not only to collect the information but to make it easily manageable, within-built mechanisms to elicit it, to ensure completeness and to facilitate consistency and usability.

EMA encourage the primary objectives of the applicant and has decided to issue a Letter of Support to the Dravet Syndrome Foundation Spain to encourage the further development and validation of the proposed Patient Data Platform.

Sincerely,

Guido Rasi
Executive Director