Follow-Up Survey of stakeholder actions following the EMA patient registry workshops

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Study Finalised

Administrative details

EU PAS number

EUPAS28593

Study ID

32041

DARWIN EU® study

No

Study countries

United Kingdom

Study description

The Initiative for Patient Registries, launched in September 2015 by the European Medicines Agency (EMA), explores ways of expanding the use of patient registries by introducing and supporting a systematic and standardised approach to their contribution to the benefit-risk evaluation of medicines within the European Economic Area. Objectives of the Initiative include facilitating the use of existing patient registries as well as the establishment of new registries if none are available or adequate. At a consultative meeting in October 2016, expert stakeholders who included registry holders, patients, health care professionals (HCPs), regulators, marketing authorisation holders and applicants (MAHs/MAAs), health technology assessment (HTA) and reimbursement bodies, and European Commission representatives participated in discussions to share their views on barriers and facilitators to registry use and on optimising the use of registries for regulatory assessments. Subsequently EMA hosted four disease-specific patient registry workshops: Cystic Fibrosis (14th June 2017), Multiple Sclerosis (7th July 2017), CAR T-cell therapy Registries (9th February 2018) and Haemophilia Registries (8th June 2018). These disease areas were chosen because there was ongoing product development with new products recently approved or undergoing assessment and registries had requested support for harmonisation. Following each workshop, a report was published that included the recommendations and actions arising. Participants, who represented all of the stakeholder groups, contributed to the drafting of the reports.During October-November 2018, a survey was conducted to assess the impact of the workshops on stakeholder registry-related activities and to identify further EMA activities that could be explored to facilitate stakeholders’ work.

Study status

Finalised

Research institutions and networks

Institutions

European Medicines Agency (EMA)

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Institution

Contact details

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Primary lead investigator

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Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 13/08/2018

Actual: 13/08/2018

Study start date

Planned: 13/08/2018

Actual: 13/08/2018

Date of final study report
Planned: 13/12/2018

Actual: 13/12/2018

Sources of funding

- No external funding

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Other

Study topic, other:

Survey of stakeholder

Study type:

Non-interventional study

Scope of the study:

Other

If ‘other’, further details on the scope of the study

Survey of actions taken after registries workshops

Data collection methods:

Primary data collection

Main study objective:

The aims were:1) to determine if the recommendations and actions agreed in each of the workshops by the stakeholders had been achieved, were under consideration, or if stakeholders were actively working on measures to be put in place in the short/long term2) to assess if views on the value of registries had changed following the workshops.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Survey

Population studied

Short description of the study population

EMA Patient Registries Initiative team's regulatory colleagues who had attended at least one workshop.

Age groups

- Adults (18 to < 46 years)
- Adults (46 to < 65 years)
- Adults (65 to < 75 years)
- Adults (75 to < 85 years)
- Adults (85 years and over)

Estimated number of subjects

194

Study design details

Data analysis plan

The survey was anonymous. Participants were asked to provide their primary stakeholder group (registry holder, patient representative, regulator, marketing authorisation holder/applicant (MAH/MAA), health technology assessment (HTA) and/or reimbursement body) and the workshop attended. The survey consisted of common questions to be answered by all the participants and specific questions based on the stakeholder group and/or a specific workshop. Most questions asked respondents to choose a single answer from a list of 3-5 options. Broadly, the questions enquired about the status of workshop actions and recommendations, impact on views about registries, and collaboration between stakeholders including any new alliances or early dialogue between registries and MAH/MAAs on studies or protocols. Free text space was provided for respondents to expand on their answers, for example, to explain why

recommendations from the workshop had or had not been implemented.

Documents

Study report

EMA Follow-Up Survey Report.pdf (431.41 KB)

Study publications

McGettigan P, Alonso Olmo C, Plueschke K, Castillon M, Nogueras Zondag D, Bahri…

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data sources (types)

Other

Data sources (types), other

The survey was conducted by the EMA Patient Registries Initiative. The survey was sent to the participants from the 4 workshops hosted by the EMA.Please find the link to the EMA Patient Registries

webpage:https://www.ema.europa.eu/en/human-regulatory/post-authorisation/patient-registries

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No