

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

**First published:** 28/02/2019

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Study

Finalised

## Administrative details

### EU PAS number

EUPAS28593

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### Study ID

32041

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### DARWIN EU® study

No

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### Study countries

 United Kingdom

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### 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.

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### **Study status**

Finalised

## **Research institutions and networks**

# Institutions

## European Medicines Agency (EMA)

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Institution

## Contact details

### Study institution contact

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Study contact

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### Primary lead investigator

Xavier Kurz

Primary lead investigator

## Study timelines

### Date when funding contract was signed

Planned: 13/08/2018

Actual: 13/08/2018

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### Study start date

Planned: 13/08/2018

Actual: 13/08/2018

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### **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

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

Not applicable

## Methodological aspects

### Study type

### Study type list

### Study topic:

Other

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**Study topic, other:**

Survey of stakeholder

**Study type:**

Non-interventional study

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**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

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**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

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**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.

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### **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)
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### **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...](#)

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## 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](#)

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### 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

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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### **Check logical consistency**

Unknown

## Data characterisation

### **Data characterisation conducted**

No