Transparency and information for decision making: patient engagement and publication of clinical data

Plenary 6 - The use of information in regulatory convergence

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IX CPANDRH, San Salvador, 24-26 October 2018

TRANSPARENCY AND INFORMATION FOR REGULATORY DECISION-MAKING IN THE EU SYSTEM

External contribution:

- Patient, healthcare professional and academia engagement
- Public hearings for pharmacovigilance
- EU experts, including academia

Assessment:

- Sharing the same dossier with all regulators
- Sharing and peer reviewing assessment reports and inspection reports

For the public:

- Public assessment reports for all products
- Same SPC and package leaflet in all 24 official EU languages
- Access to documents
- Publication of clinical data

INTERATION WITH PATIENTS: A PROGRESSIVE JOURNEY

1995
EMA created

1996
Dialogue with patients

2000
Patients become Committee members

2003
Working group with patients

2005
Framework of interaction with patient & consumer organisations

2006
Patient & Consumer Working Party (PCWP)

2014
Public Engagement Department

2017
Public Hearings

Involve young people

Ongoing…
Systematic inclusion of patient input along medicine lifecycle

A STEP-BY-STEP APPROACH

COMP members → Board members → PDCO members → CAT members → PRAC members

Working group of patients → Patient and Consumer Working Party

Review orphan summaries → Review of PLs → Review of medicine summaries → Review of safety communications → Review of herbal summaries

Protocol Assistance → All scientific advice

Pilot in expert meetings → Systematic involvement

PAHO

PATIENT ENGAGEMENT: BENEFITS BUT ALSO SOME CHALLENGES

Engaging with patients has benefits:

- Brings everyday aspects of living with a disease into scientific discussions
- Helps bridge the gap between clinical trial data and real world data
- Increases transparency, awareness and understanding
- Leads to more meaningful outcomes

But can have some challenges:

- Finding suitable patients (e.g. language barrier, availability)
- Ensuring comprehensive, tailored support to enhance participation
- Clear definition of patients' role to manage expectations
- Managing potential conflicts of interest
- Gathering information that is considered representative

Website – www.ema.europa.eu/en/patients-carers

PUBLICATION OF CLINICAL DATA

Purpose

- Transparency (EMA commitment)
- Enables public scrutiny (establishes trust, confidence in outcomes)
- Reduce clinical trials duplication
- Enhances scientific knowledge, facilitates secondary analysis

Website - https://clinicaldata.ema.europa.eu

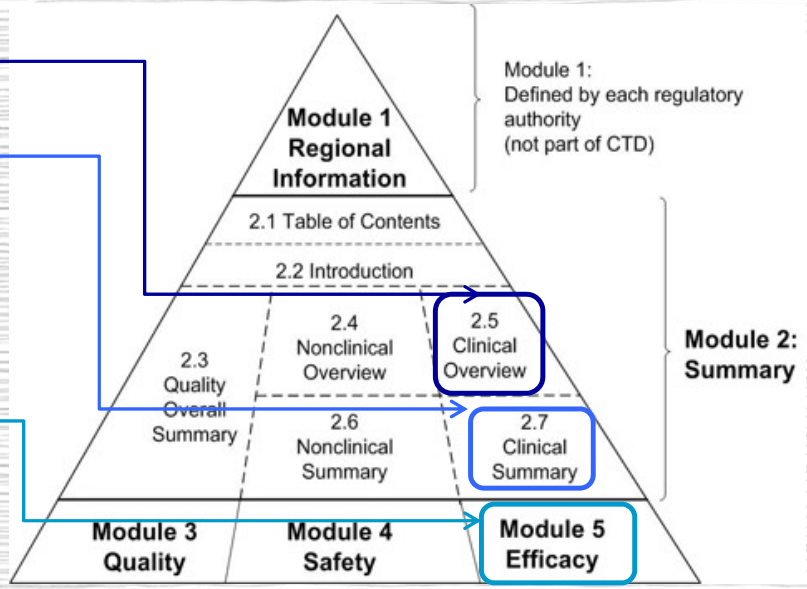
WHAT CLINICAL DATA DO WE MEAN?

Module 2.5 - Clinical Overview

Module 2.7.1 to 2.7.4 - Clinical Summary

- ***Module 5.3 Clinical Study Reports (CSR) – Body of the reports***
- ***Module 5.3 Clinical Study Reports – 3 appendices per CSR***
  - ***16.1.1 (protocol and protocol amendments)***
  - ***16.1.2 (sample case report form)***
  - ***16.1.9 (documentation of statistical methods)***

+ Anonymization Report



- *For all applications falling within the scope of 'Policy 0070' whether studies were conducted in or outside the EU*
- *No Individual Patients Data (IPD) listings*

DOCUMENTS PUBLISHED SO FAR

Type of published procedure	
Initial marketing authorisation	81
Extension of indication	40
Line extension	5
Total number of published procedures	**126**

Published documents	
Module 1.9	126*
Module 2.5	167
Module 2.7.1 – 2.7.4	395
Module 5.3 (CSR)	5,965
Total number of documents	**6,653**
Total number of pages	**2,977,612**

*(*125 Anonymisation reports + 1 Annex)*

Publication began in October 2016, data as of 7 September 2018

CONCLUDING REFLECTIONS

- Transparency and information-sharing is not cost-neutral – there are resource implications
- However there are significant benefits to achieving our overall missions by being as transparent and open as possible
- Sharing information with our stakeholders builds trust, confidence in our decisions and shows we are prepared to stand by our actions
- Involving different stakeholders in our activities can bring new perspectives that enrich our outputs, and improve quality of outcomes for patients

PAHO/WHO

Gracias, Thank You

Para más información / For more information:

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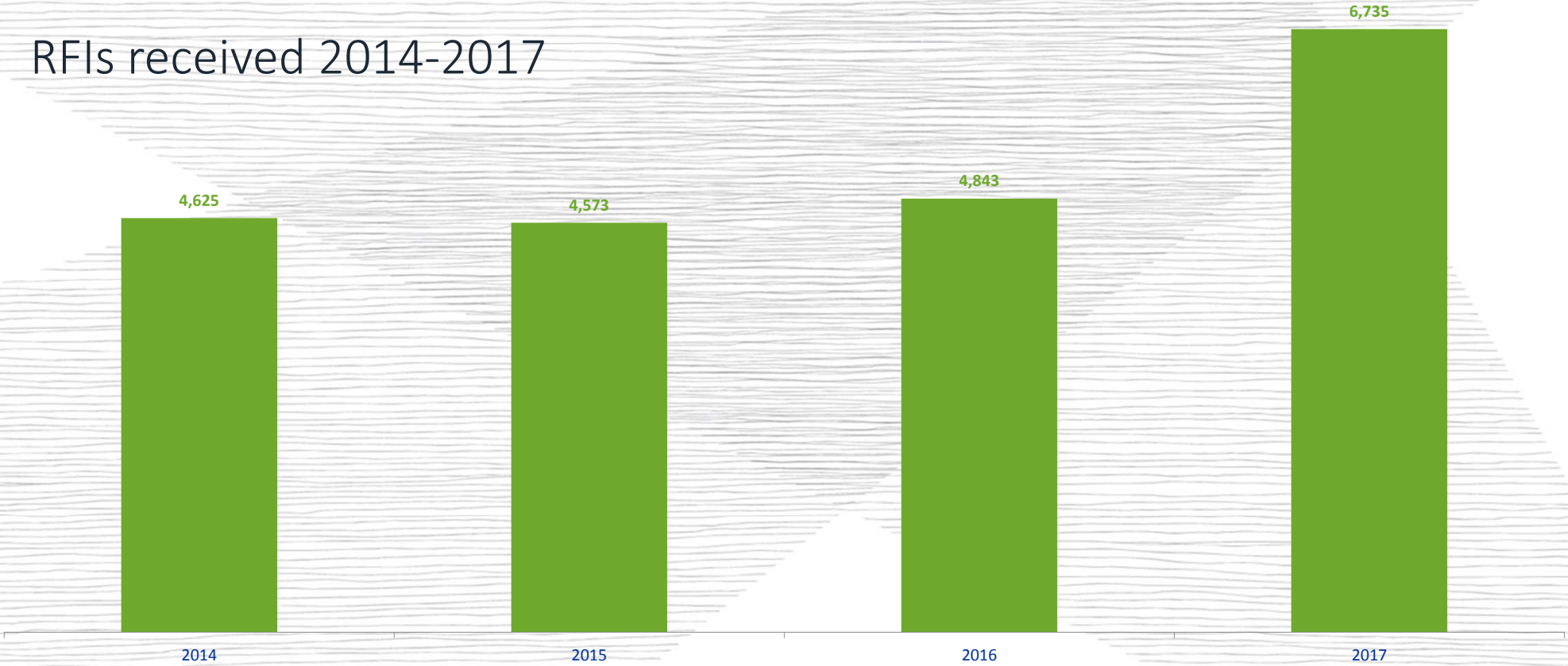
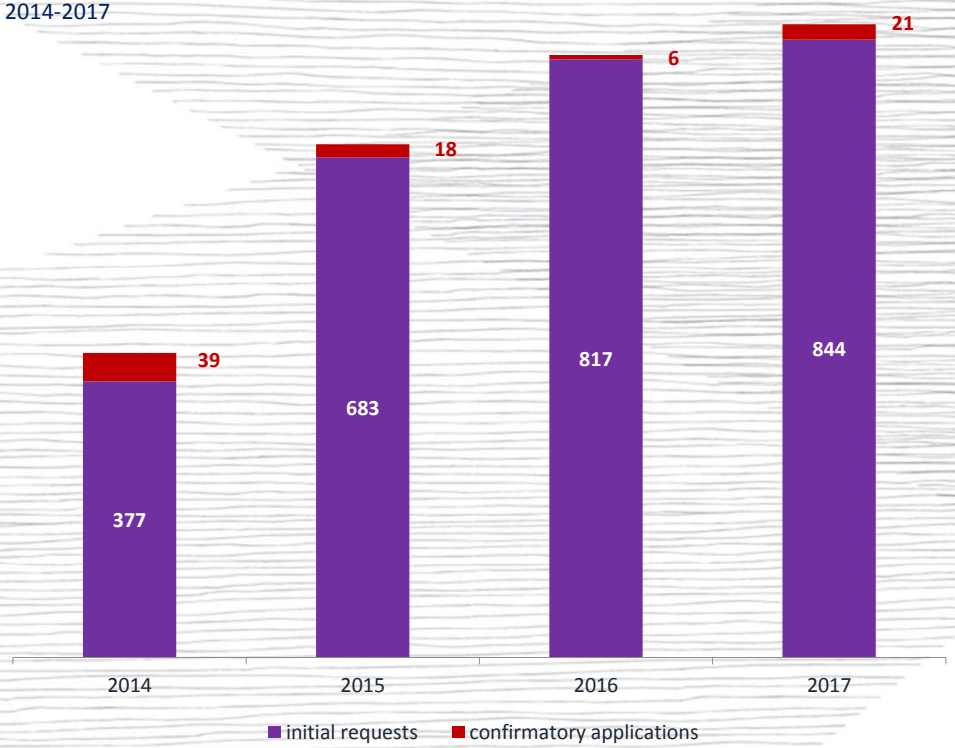
An overview of Request for information activities in the EMA

RFIs received 2014-2017



An overview of Access to Documents activities in the EMA

ATD Requests received
2014-2017



Pages released
2014-2017