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

Plenary 6 - The use of information in regulatory convergence

Martin Harvey, European Medicines Agency (EMA)
IX CPANDRH, San Salvador, 24-26 October 2018
### 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

### External contribution:
- Patient, healthcare professional and academia engagement
- Public hearings for pharmacovigilance
- EU experts, including academia
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

2017
Involve young people

Ongoing...
Systematic inclusion of patient input along medicine lifecycle

INTERACTION WITH PATIENTS: A PROGRESSIVE JOURNEY
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
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

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

<table>
<thead>
<tr>
<th>Type of published procedure</th>
<th>Published documents</th>
</tr>
</thead>
<tbody>
<tr>
<td>Initial marketing authorisation</td>
<td>Module 1.9</td>
</tr>
<tr>
<td></td>
<td>81</td>
</tr>
<tr>
<td>Extension of indication</td>
<td>Module 2.5</td>
</tr>
<tr>
<td></td>
<td>40</td>
</tr>
<tr>
<td>Line extension</td>
<td>Module 2.7.1–2.7.4</td>
</tr>
<tr>
<td></td>
<td>5</td>
</tr>
<tr>
<td>Total number of published procedures</td>
<td>Module 5.3 (CSR)</td>
</tr>
<tr>
<td></td>
<td>126</td>
</tr>
<tr>
<td></td>
<td>167</td>
</tr>
<tr>
<td></td>
<td>395</td>
</tr>
<tr>
<td></td>
<td>5,965</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Published documents</th>
<th>Total number of documents</th>
<th>Total number of pages</th>
</tr>
</thead>
<tbody>
<tr>
<td>Module 1.9</td>
<td>126*</td>
<td>2,977,612</td>
</tr>
<tr>
<td>Module 2.5</td>
<td>167</td>
<td></td>
</tr>
<tr>
<td>Module 2.7.1–2.7.4</td>
<td>395</td>
<td></td>
</tr>
<tr>
<td>Module 5.3 (CSR)</td>
<td>5,965</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Total number of documents</th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Total number of documents</td>
<td>6,653</td>
<td></td>
</tr>
<tr>
<td>Total number of pages</td>
<td>2,977,612</td>
<td></td>
</tr>
</tbody>
</table>

(*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
Gracias, Thank You

Para más información / For more information:
www.ema.europa.eu/contact
EMAIInternational@ema.europa.eu

Website: www.ema.europa.eu
Follow us on @EMA_News
An overview of Request for information activities in the EMA

RFIs received 2014-2017

<table>
<thead>
<tr>
<th>Year</th>
<th>RFIs</th>
</tr>
</thead>
<tbody>
<tr>
<td>2014</td>
<td>4,625</td>
</tr>
<tr>
<td>2015</td>
<td>4,573</td>
</tr>
<tr>
<td>2016</td>
<td>4,843</td>
</tr>
<tr>
<td>2017</td>
<td>6,735</td>
</tr>
</tbody>
</table>
An overview of Access to Documents activities in the EMA

ATD Requests received 2014-2017

- Initial requests
- Confirmatory applications

Pages released 2014-2017

- Pages released
- Documents released

<table>
<thead>
<tr>
<th>Year</th>
<th>Initial Requests</th>
<th>Confirmatory Applications</th>
<th>Pages Released</th>
<th>Documents Released</th>
</tr>
</thead>
<tbody>
<tr>
<td>2014</td>
<td>377</td>
<td>39</td>
<td>167,309</td>
<td>2,807</td>
</tr>
<tr>
<td>2015</td>
<td>683</td>
<td>18</td>
<td>333,999</td>
<td>2,876</td>
</tr>
<tr>
<td>2016</td>
<td>817</td>
<td>6</td>
<td>380,911</td>
<td></td>
</tr>
<tr>
<td>2017</td>
<td>844</td>
<td>21</td>
<td>487,092</td>
<td></td>
</tr>
</tbody>
</table>