



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

# Lifecycle Pharmacovigilance planning for public health: key to product development and safe and effective use of products

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Tenth Stakeholder forum on the Pharmacovigilance legislation  
21 September 2016

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**EMA**

An agency of the European Union





# Planning is key to product development and safe and effective use of products

## Key messages

- Pharmacoepidemiology and pharmacovigilance play critical roles in medicines regulation: regulator as gate-keeper and enabler
- Planning data collection and integrating knowledge starts in early development and is life-long
- Embrace the evidence spectrum: different data and methods are best to address different questions
- Use of real world evidence holds great promise to support drug development and in the fulfilment of unmet needs
- Collaboration will be critical to realising that promise.



# Planning is key to product development and safe and effective use of products

In this session: Explore the critical link between pharmacoepidemiology and risk management planning and the development of innovative medicines to fulfil the unmet needs of patients.

- Scientific Advice delivers planning during development - Spiros Vamvakas, EMA
- Epidemiology as an enabler for health - Alison Cave, EMA
- Actions from the Risk Minimisation Measures Workshop in 2015 - Jamie Wilkinson, PGEU
- Open panel discussion

## Health Protection and Promotion – two sides of the same coin



### **Protect 'safety'**

Through detection and  
management of side effects

### **Promote 'efficacy'**

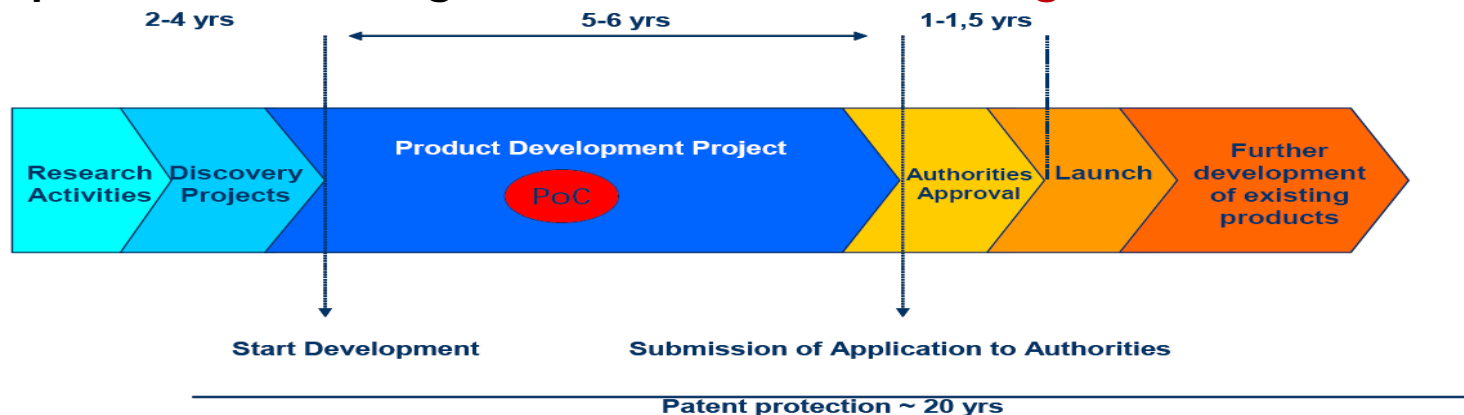
Fulfil unmet medical needs

**Better information throughout the product lifecycle and  
patient journey**



# How do pharmacovigilance and epidemiology support innovation?

- **Proactive enabler:** planning for data collection, management of risk monitoring of use
- **Reactive responder:** analysis and integration of various data streams – updated advice to optimise use – better patient outcomes and sustainable B/R management.
- Provides: assurance that data will be available once a product is on the market to allow actions for risk min and benefits optimisation
- **Development → Licensing → Utilisation → Monitoring.**





# Experience with new EU PV legislation

Access provided to Medicines and Healthcare Products Regulatory Agency by Information Services

**nature** DRUG DISCOVERY  
**REVIEWS**

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**CORRESPONDENCE** [LINK TO ORIGINAL ARTICLE](#)

## Proactively managing the risk of marketed drugs: experience with the EMA Pharmacovigilance Risk Assessment Committee

Peter Arlett, Geraldine Portier, Roberto de Lisa, Kevin Blake, Noel Wathion, Jean-Michel Dogne, Almath Spooner, June Raine and Guido Rasi

challenges in the optimization of their safe and effective use.

In the first 18 months of its operation, the PRAC has considered risk management plans for 160 medicinal products. In this work the PRAC has focused on ensuring feasible, evidence-based and risk-proportionate planning<sup>4</sup>.

The collection of individual reports of suspected adverse drug reactions (ADRs) is one of the foundations of drug surveillance, and the reporting rules have been strengthened. These now include the formal

*"After 18 months of operation of PRAC*

*We suggest more safe & effective drugs can be made available*

*Through planning, engagement and transparency, as well as rapid assessment"*

2014 Nat Rev Drug Disc

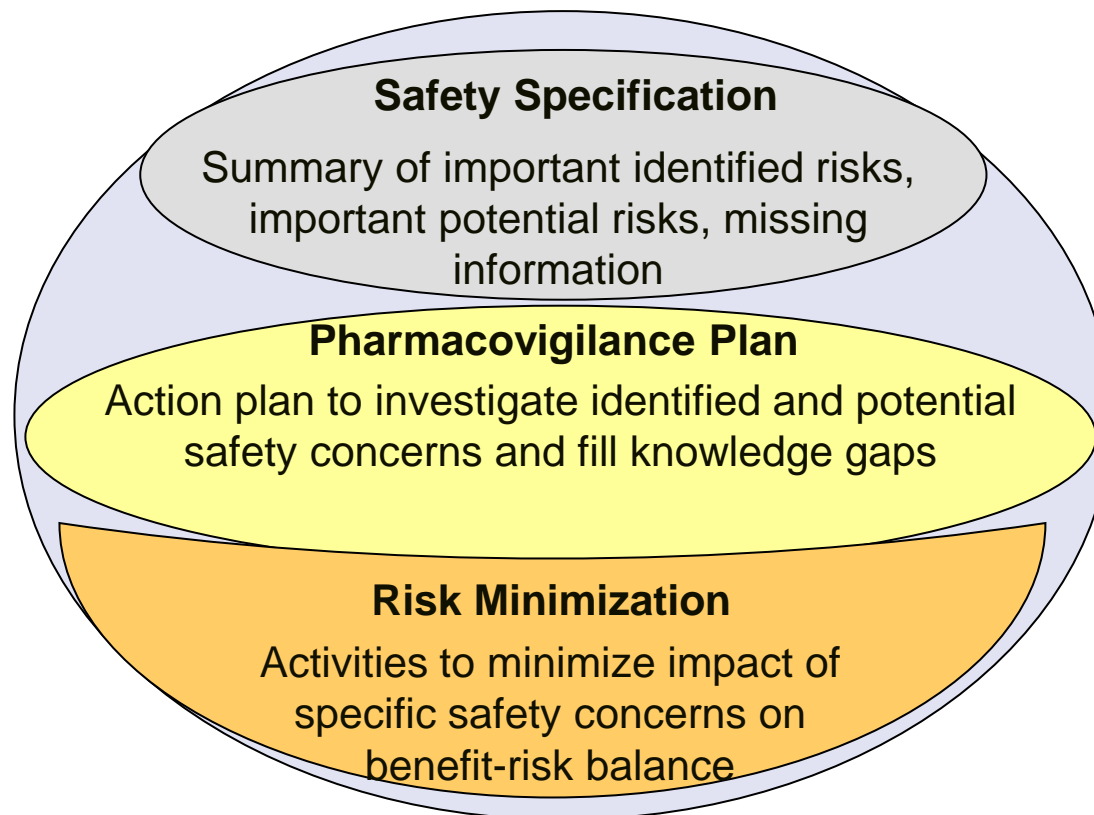


# Proactive Risk Management Planning





# EU Risk Management Plan







# Advice on RMP at early stage



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19 September 2014  
EMA/691788/2010 Rev. 7  
Human Medicines Research and Development Support

European Medicines Agency Guidance for applicants  
seeking scientific advice and protocol assistance

Scientific Advice Working Party  
includes PV expertise – PRAC  
members

Early discussion of risk  
management plans including  
design of studies



# EC Report on 3 years of EU pharmacovigilance



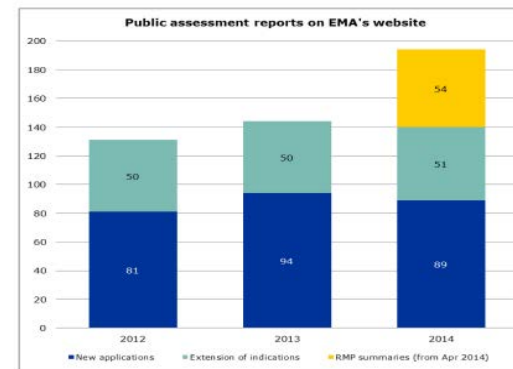
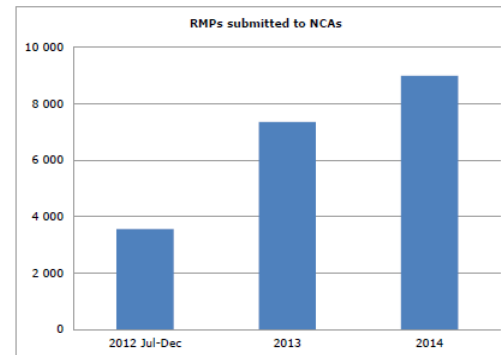
Brussels, 8.8.2016  
SWD(2016) 284 final



**COMMISSION STAFF WORKING DOCUMENT**  
*Accompanying the document*

**Commission Report**

**Pharmacovigilance related activities of Member States and  
the European Medicines Agency concerning medicinal products for human use  
(2012 – 2014)**





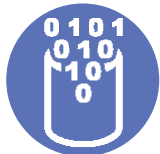
# PRIME scheme - Goal & Scope

Medicines with major public health interest



## Reinforce scientific and regulatory advice

- Foster and facilitate early interaction
- Raise awareness of requirements earlier in development



## Optimise robust data generation

- Focus efficient development
- Promote generation of robust and high quality data



## Enable accelerated assessment

- Facilitated by knowledge gained throughout development
- Building on existing regulatory framework





# Eligibility for PRIME scheme

Based on EU Accelerated Assessment criteria



Medicinal products of major public health interest and in particular from the viewpoint of therapeutic innovation.

- Potential to address to a significant extent **an unmet medical need**
- **Scientific justification**, based on data and evidence available from nonclinical and clinical development

No satisfactory treatment or if method exists, brings a major therapeutic advantage

Introducing new methods- or improving existing ones

Meaningful improvement of efficacy - impact on onset, duration, improving morbidity, mortality



## First 4 products granted eligibility

### **CCX-168**

Treatment of patients with active ANCA-associated vasculitis (GPA and MPA)  
*Orphan*

### **KTE-C19**

Treatment of lymphomas (DLBCL, PMBCL, TFL)  
*Orphan*

### **Emapalumab**

Treatment of primary haemophagocytic lymphohistiocytosis (HLH)  
*Orphan*

### **Aducanumab**

Alzheimer's disease



# Prime Web-page and supporting documents

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Post-opinion  
Post-authorisation  
Product information  
Scientific advice and protocol assistance  
**Support for early access**  
PRIME: priority medicines  
Accelerated assessment  
Conditional marketing authorisation  
Compassionate use  
Adaptive pathways  
Scientific guidelines  
Innovation Task Force  
SME office  
Paediatric medicine

Home > Human regulatory > Support for early access

PRIME: priority medicines

**PRIME - PRIORITY MEDICINES**

**PRIME is a scheme launched by the European Medicines Agency (EMA) to enhance support for the development of medicines that target an unmet medical need. This voluntary scheme is based on enhanced interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier.**

Through PRIME, the Agency offers early and proactive support to medicine developers to optimise the generation of robust data on a medicine's benefits and risks and enable accelerated assessment of medicines applications.

This will help patients to benefit as early as possible from therapies that may significantly improve their quality of life.

**Accelerated assessment**

PRIME builds on the existing regulatory framework and tools already available such as scientific advice and accelerated assessment. This means that developers of a medicine that benefitted from PRIME can expect to be eligible for accelerated assessment at the time of application for a marketing authorisation.

**Fostering early dialogue**

**Related content**

- Support for early access
- Launch of PRIME – Paving the way for promising medicines for patients (07/03/2016)

**PRIME at a glance - Factsheet**

**Related documents**

- Enhanced early dialogue to facilitate accelerated assessment of Priority Medicines (PRIME) (07/03/2016)

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**PRIME – PRIORITY MEDICINES**

Paving the way for promising medicines for patients

**Why PRIME is needed**

Many patients with serious diseases have no or only unsatisfactory therapeutic options and should be able to benefit from scientific advancement and cutting-edge medicines as early as possible.

The European Medicines Agency (EMA) developed PRIME to show with this European Commission how the path to a medicine intended for adult patients of unmet medical need can be shortened for patients whose diseases cannot be treated or who need better treatment options to help them live longer lives.

**Benefits of PRIME**

FOR PATIENTS	FOR MEDICINE DEVELOPERS
<ul style="list-style-type: none"> <li>PRIME is driven by patients' needs.</li> <li>It focuses on medicines that address an unmet medical need, i.e. other existing treatments, or health conditions with limited treatment options for their diseases.</li> <li>It helps to maximise medical data on the development of medicines while enabling regulatory interactions.</li> <li>It aims to bring groundbreaking medicines to patients earlier, without compromising high scientific standards and patient safety.</li> </ul>	<ul style="list-style-type: none"> <li>PRIME helps development of promising new medicines to optimise development plans.</li> <li>It helps you dialogue with EMA to optimise real data collection and high quality scientific information for regulators.</li> <li>It speeds up evaluation so that medicines get reach patients earlier.</li> <li>It allows you to interact with EMA reference to previous PRIME medicines to inform your reference to current PRIME medicines.</li> </ul>

**PRIME is brief**

Medicine developers for PRIME need to apply for a PRIME medicine.

Medicine developers should be prepared to address the need and bring a major therapeutic advantage to patients.

EMA will provide early and collaborative support to facilitate the development of adult medicines, based on their interaction and contribution to society patients' needs.

## Factsheet in lay language

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7 March 2016  
EMA-11164-1611  
Human Medicines Research and Development Support Division

European Medicines Agency Guidance for applicants seeking access to PRIME scheme

This guidance document addresses questions that applicants seeking support through the PRIME scheme may have.

The guidance also explains the scope and features of PRIME. It provides an overview of the procedure to obtain support through the scheme and gives guidance to companies in preparing their requests.

This guidance will be updated regularly to reflect new developments as experience is gained with the scheme.

It should be read in conjunction with:

- [Enhanced early dialogue to facilitate accelerated assessment of Priority Medicines \(PRIME\) Guidance on accelerated assessment](#)
- [European Medicines Agency Guidance for applicants seeking scientific advice and protocol assistance](#)

If you require further information on any of the included topics, do not hesitate to send your request to [prime@ema.europa.eu](mailto:prime@ema.europa.eu) and we will deal with your enquiry in a timely manner.

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## Q&A, templates, application form for applicants



# Planning is key to product development and safe and effective use of products

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## Some dates for the diary

WebRADR workshop 19<sup>th</sup> October

EMA Registries workshop 28<sup>th</sup> October

EMA Big Data Workshop 14-15<sup>th</sup>  
November

ENCePP plenary  
22<sup>nd</sup> November

EMA Pharmacovigilance Impact  
Workshop  
5<sup>th</sup> - 6<sup>th</sup> December





# Thank you for your attention

## Further information

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