



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

# PRIME

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SME workshop, 3 October 2016

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An agency of the European Union







# PRIME scheme - Goal & Scope

To foster the development of *medicines with major public health interest*.



## Reinforce scientific and regulatory advice

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



## Optimise development for robust data generation

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



## Enable accelerated assessment

- Facilitated by knowledge gained throughout development
- Feedback of relevant SA aspects to CHMP

Building on existing framework;  
Eligibility according to existing 'Accelerated Assessment criteria'



# Features of the PRIME scheme

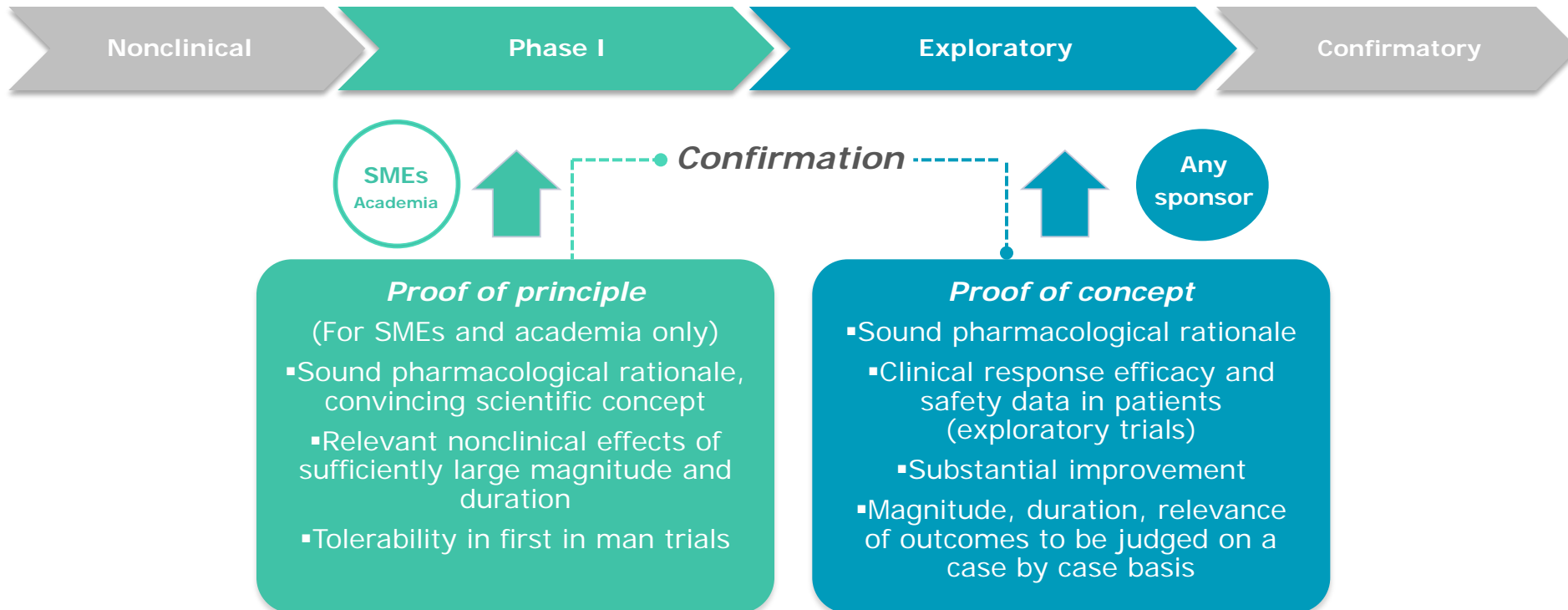
Early access tool, supporting patient access to innovative medicines.



- **Written confirmation of PRIME eligibility** and potential for accelerated assessment;
- **Early CHMP Rapporteur appointment** during development;
- **Kick off meeting** with multidisciplinary expertise from EU network;
- **Enhanced scientific advice** at key development milestones/decision points;
- **EMA dedicated contact point**;
- **Fee incentives** for SMEs and academics on Scientific Advice requests.



# Entry points PRIME eligibility and required evidence







# Overview of PRIME scheme

Early identification of therapeutic innovation in unmet medical needs.

- Iterative Scientific advice
- Enhanced regulatory guidance
- Incremental knowledge gain
- Proactive dialogue
- Promote use of existing tools

MAA review under accelerated assessment.

Nonclinical

Phase I

Exploratory

Confirmatory

Evaluation

Post-authorisation

SA 1  
(SAWP)

Eligibility  
(CHMP)

SA 2  
(SAWP)

SA n  
(SAWP)

Accelerated  
Assessment  
confirmation  
(CHMP)

SMEs  
Academia

Any  
sponsor

Early CHMP Rapporteur appointment





## PRIME eligibility criteria and request

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# Eligibility to PRIME scheme

Based on 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 method or if method exists, bring a major therapeutic advantage

Introducing new methods or improving existing ones

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



# Justification for eligibility to PRIME

For products under development yet to be placed on the EU market

## Unmet medical need

- Epidemiological data about the disease
- Description of available diagnostic, prevention and treatment options/standard of care, their effect and how medical need is not fulfilled

## Potential to significantly address the unmet medical need

- Description of observed and predicted effects, clinical relevance, added value and impact
- If applicable, expected improvement over existing treatments

## Data required at different stages of development



*Justification assessed by EMA's scientific committees*





# Justification for eligibility to PRIME

Date: \_\_\_\_\_

PRIME eligibility request – Applicant's justification  
s.(Invented) Name>

Product information	
Active substance (INN or common name or company name):	<Chemical> <Biological> <Advanced therapy>
Substance type:	
Description of the product & mechanism(s) of action:	
Therapeutic indication:	
Applicant:	
UFI number/DNA number:	

- Short background on disease & product
- Unmet medical need
  - Epidemiology of disease
  - Available treatment
- Supportive evidence
  - Nonclinical pharmacology
  - Clinical data (eg exploratory efficacy + safety)
  - Conclusion on claim of major public health interest
  - Is there an unmet medical need in the proposed indication?
  - Is the data sufficient to support product's potential to significantly address unmet medical need?



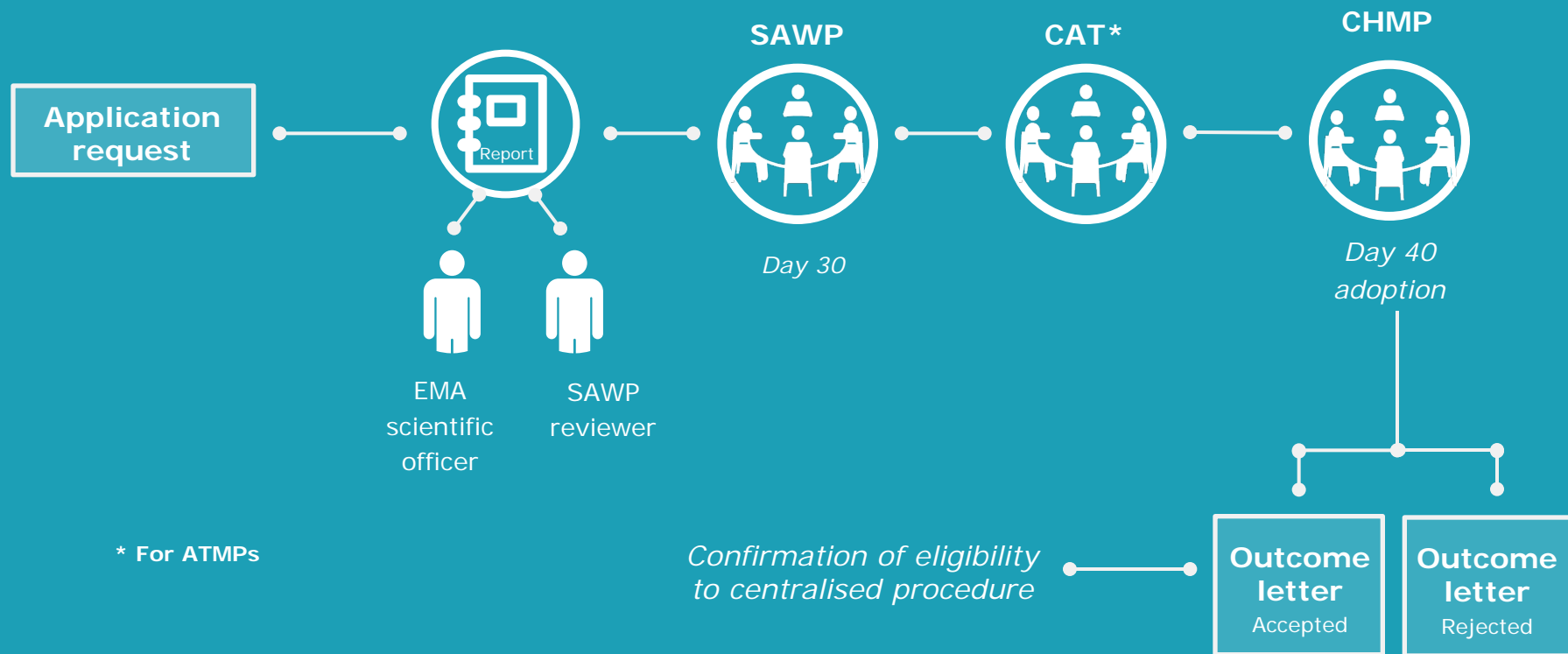


## Focus on nonclinical data & proof of principle

- Sound pharmacological rationale, convincing scientific concept
  - But, new pharmacological target or mechanism of action is not sufficient
- Relevant nonclinical effects
  - in vitro and in vivo data from relevant models, with comparison to results from other products if possible
  - Observed effects: sufficiently large and/or of long duration
  - Compelling results to outweigh many uncertainties of very early stage
- Early clinical data from first in man trials
  - Acceptable tolerability
  - PK to confirm sufficient exposure so that nonclinical effect may be observed in man



# Assessment of Eligibility: 40-day procedure





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27 July 2016  
EMA/424591/2016  
Investigative

Recommendations on eligibility to PRIME scheme  
Adopted at the CHMP meeting of 18-21 July 2016

Eligibility granted				Type of data supporting request	Type of applicant
Issue*	Substance type	Therapeutic area	Therapeutic indication		
Autologous CD4 and CD8 T cells transfused with functional vector	Advanced Therapy	Oncology	Treatment of HLA-A*0201, HLA-B*0702, or HLA-B*0206 class positive patients with comparable or metastatic epidermal sarcoma who have received prior chemotherapy and whose	Nonclinical + Clinical exploratory	SME

Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant
Biological	Oncology	Treatment of relapsed and refractory multiple myeloma.	Nonclinical + Clinical exploratory	Other
Biological	Oncology	Treatment of diffuse intrinsic pontine glioma (DIPG)	Nonclinical + Clinical exploratory	SME
Biological	Oncology	Treatment of myeloid leukaemia A	Nonclinical + Clinical exploratory	Other
Biological	Haematology + Haematology + Haematology + Haematology	Treatment of iron overload in patients with beta thalassemia	Nonclinical + Clinical exploratory	SME
Chemical	Haematology + Haematology	Treatment of active moderate to severe Crohn's Disease (CD)	Nonclinical + Clinical exploratory	SME
Advanced Therapy	Gastroenterology + Hepatology	Treatment of active moderate to severe Crohn's Disease (CD)	Non clinical + tolerability first in man	Other
Chemical	Infectious Diseases	Treatment of invasive fungal infections	Clinical exploratory	Other
Biological	Immunology-Allergy	Treatment of peanut allergy		

Small and medium-sized enterprises registered with the Agency's SME office. Other type of applicants are those not qualifying or not registered as

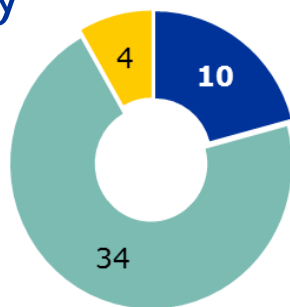
**Biological**  
SME applicants are micro-, small- and medium-sized enterprises or not fulfilling the definition of academic sponsors.

- Monthly report in CHMP highlights
- Broad characteristics
- Active substance/INN for eligible products
- High-level statistics regularly updated



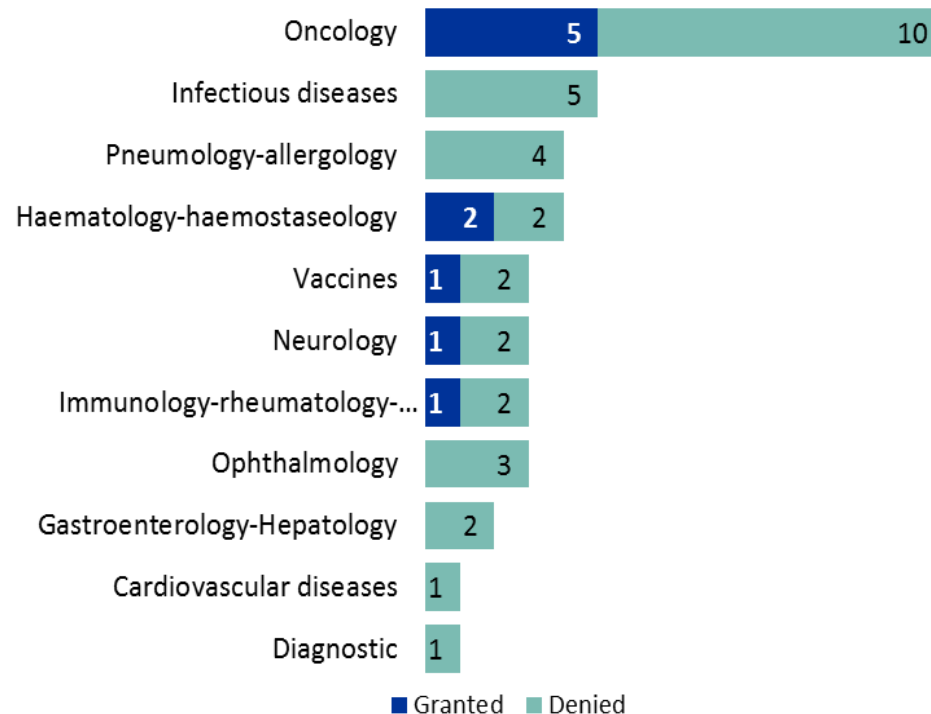


# PRIME eligibility requests received as of 29 June 2016



■ Granted ■ Denied ■ Out of scope\*

## By type of applicant







## Support to eligible products

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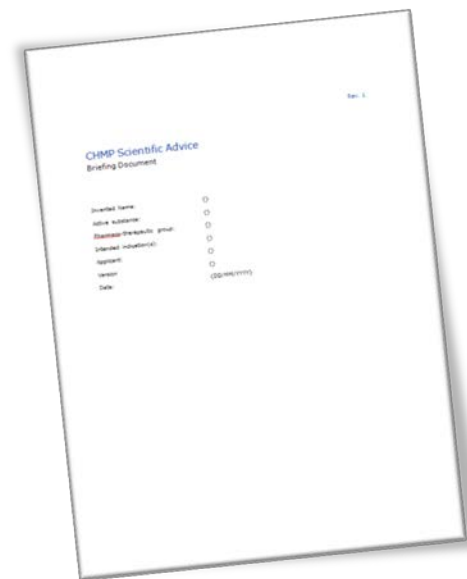
# What happens next?



**Rapporteur  
nomination**



**Kick-off meeting**



**Scientific advice**





## Kick-off meeting

- Multi disciplinary meeting with relevant experts from SAWP and CHMP and other committees;
- To take place, at EMA, shortly after eligibility confirmation;
- Facilitate initial interaction between applicant and EU regulatory network;
- Introduction of product and development status by applicant;
- Discuss the overall development plan and regulatory strategy;
- Regulatory guidance and awareness on requirements
- Provide recommendation on milestones and identify issues for scientific advice;
- Plan interactions with regulators.





## Support to be channelled through Scientific Advice by SAWP/CHMP

- Discuss detailed development plan, design of pivotal studies
- Discuss key issues for MAA, at major milestones
- Expectation of iterative advice, higher frequency of interactions
- Continuity will facilitate sharing of knowledge from development to life-cycle
- Fee waivers for SMEs and academia

## Role of SAWP coordinator

- One SAWP coordinator to follow all iterative Scientific Advice
- Recommend next milestones for SA requests

## Early CHMP Rapporteur appointment

- Continuity with life-cycle approach
- Dialogue on regulatory pathway/MAA requirements
- Promote use of tools/initiatives
- Knowledge gained during development to facilitate accelerated assessment





## In summary

- PRIME aims at **strengthening support** to medicines that target an unmet medical need.
- For medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients with no treatment options.
- EMA will offer early, proactive and enhanced scientific and regulatory support to optimise the generation of robust data and enable accelerated assessment.
- This will allow **patients to benefit from therapies** that may significantly improve their quality of life as early as possible



# PRIME webpage and supporting documents

An agency of the European Union

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## 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 documents**

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

## 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 need new medicines as early as possible.

The European Medicines Agency (EMA) created PRIME in 2015 for the European Commission's marketing and the common strategy to 2020 for the European Commission's marketing. The goal is to foster research and development of medicines for patients whose diseases cannot be treated or who need better treatment options to help them live better lives.

**Benefits of PRIME**

**FOR PATIENTS**

- PRIME is aimed to support needs
- PRIME focuses on medicines that address an unmet medical need
- PRIME is a joint early dialogue with EMA and the developer
- PRIME helps to coordinate early dialogue with EMA and the developer
- PRIME allows to bring promising medicines to the market earlier
- PRIME allows to bring promising medicines to the market earlier

**FOR MEDICINE DEVELOPERS**

- PRIME provides a platform for early dialogue with EMA and the developer
- PRIME allows to bring promising medicines to the market earlier
- PRIME allows to bring promising medicines to the market earlier
- PRIME allows to bring promising medicines to the market earlier
- PRIME allows to bring promising medicines to the market earlier

**PRIME in brief**

PRIME is a scheme for medicines that address an unmet medical need. It is a joint early dialogue with EMA and the developer. It allows to bring promising medicines to the market earlier.

## Factsheet in lay language

primate@ema.europa.eu and we will deal with your query in a timely manner.'"/>

## European Medicines Agency Guidance for applicants seeking access to PRIME scheme

1 March 2016  
EMA/110/15/15  
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.

This 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.

The 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)
- 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: [primate@ema.europa.eu](#) and we will deal with your query in a timely manner.

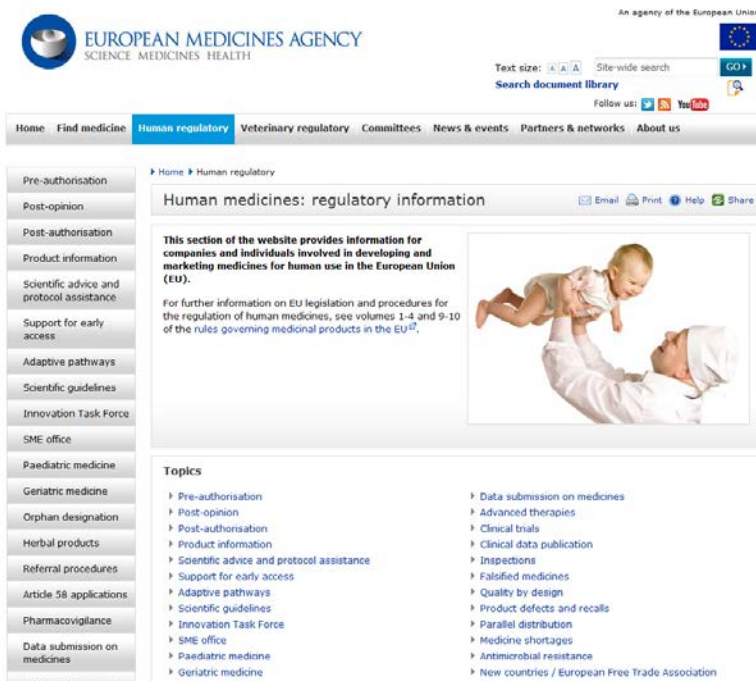
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## Q&A, templates, application form for applicants





# If PRIME is not the right tool



EMA still can  
provide support  
through...







# Thank you for your attention

## Further information

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