



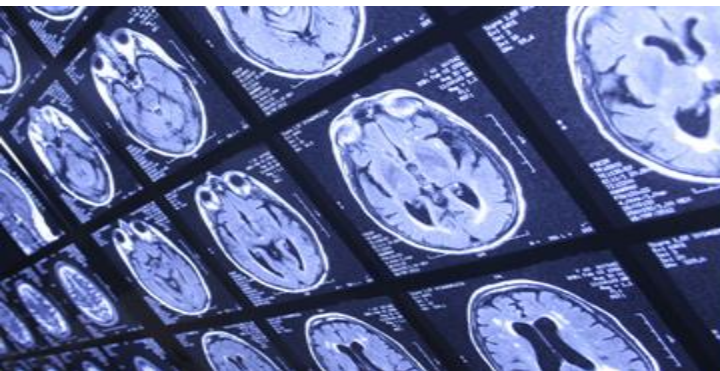
Medicines & Healthcare products
Regulatory Agency



MHRA
Regulating Medicines and Medical Devices

EU early access tools: Overview

Rob Hemmings, MHRA and CHMP/SAWP, May 2016



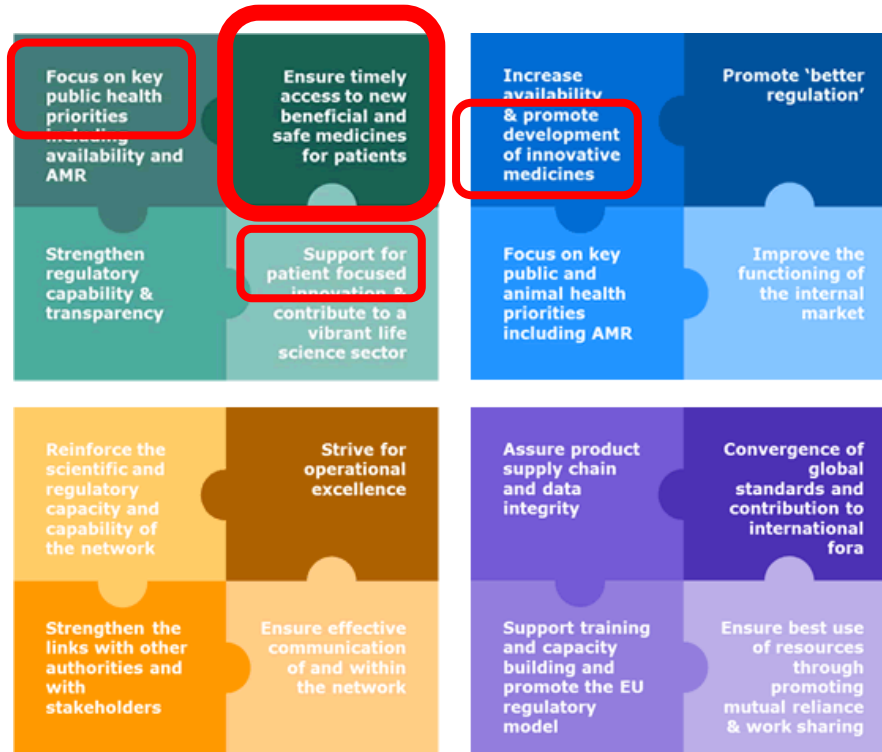
Introduction / Context

*“...**access** to beneficial treatments for the **right patient groups** at the **earliest appropriate time** in the **product life-span** in a **sustainable fashion**”*



- Multi-stakeholder environment
- Balancing patient access with evidence generation
- Need for, and opportunities for, evidence generation after initial MA
- Costs of development are high, especially the cost of failure.

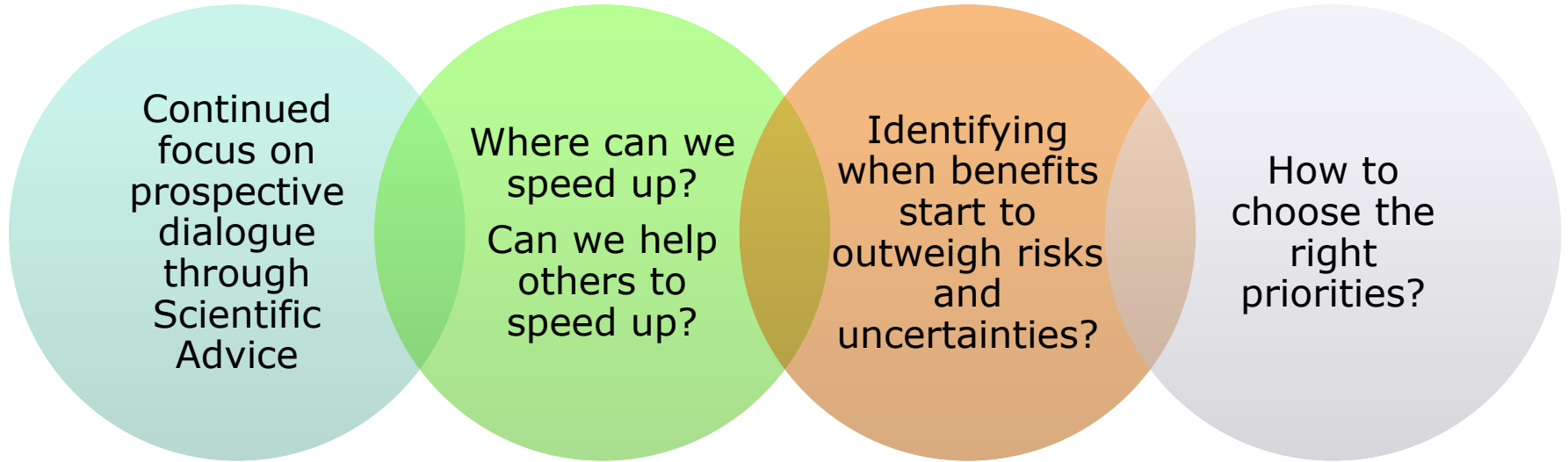
EU Medicines Agencies Network Strategy to 2020



EU Medicines Agencies Network Strategy to 2020

- Ensure timely access to new beneficial and safe medicines for patients
 - **Better understanding of existing tools (conditional MA, accelerated assessment...) and prospective planning of their use**
- Support for patient focused innovation and contribute to a vibrant life science sector in Europe
 - **Facilitate innovation to ensure patient access to new medicines**
 - **Greater collaboration across network to support innovation**
 - **Consider further incentives to support beneficial innovation, including a European early stage innovative medicines designation, with subsequent optimisation of development**

Role of regulators to foster early access



Regulatory/HTA Parallel Scientific Advice

Continued
focus on
prospective
dialogue
through
Scientific
Advice

- Aim is to generate data that meets needs of all stakeholders as efficiently as possible – preferably in one trial design/ one development plan
- Understand where the divergences lie, and explore solutions whilst adhering to remits
- Increased numbers in 2015; levels of agreement seem high.
- Report on the pilot issued on the EMA website.
- The ‘model’ for the procedure remains interim.
- Challenges identified in the current discussions include capacity and a focus on pre-authorisation plans.

A word on Conditional MA

Early dialogue and prospective planning

Discuss in advance the overall development plan and design of studies to be completed before and after authorisation

Prospective scenario building, planning the impact of future outcomes on next steps (including on proposed specific obligations)

Consider seeking accelerated assessment, since products addressing unmet medicinal needs are expected to be of major public health interest

Encouraged to engage with Health Technology Assessment bodies during the development, e.g. through a parallel scientific advice

Expected benefits

Successful CMAs

Optimised development timings

Avoiding delays in assessment procedure

Enable accelerated assessment

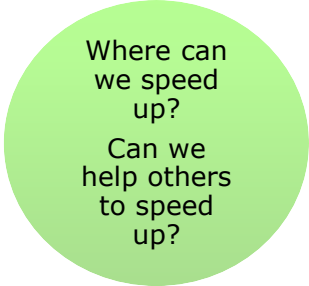
Be prepared for maintenance of orphan designation

Be ready for reimbursement discussions

Continued focus on prospective dialogue through Scientific Advice

Accelerated assessment

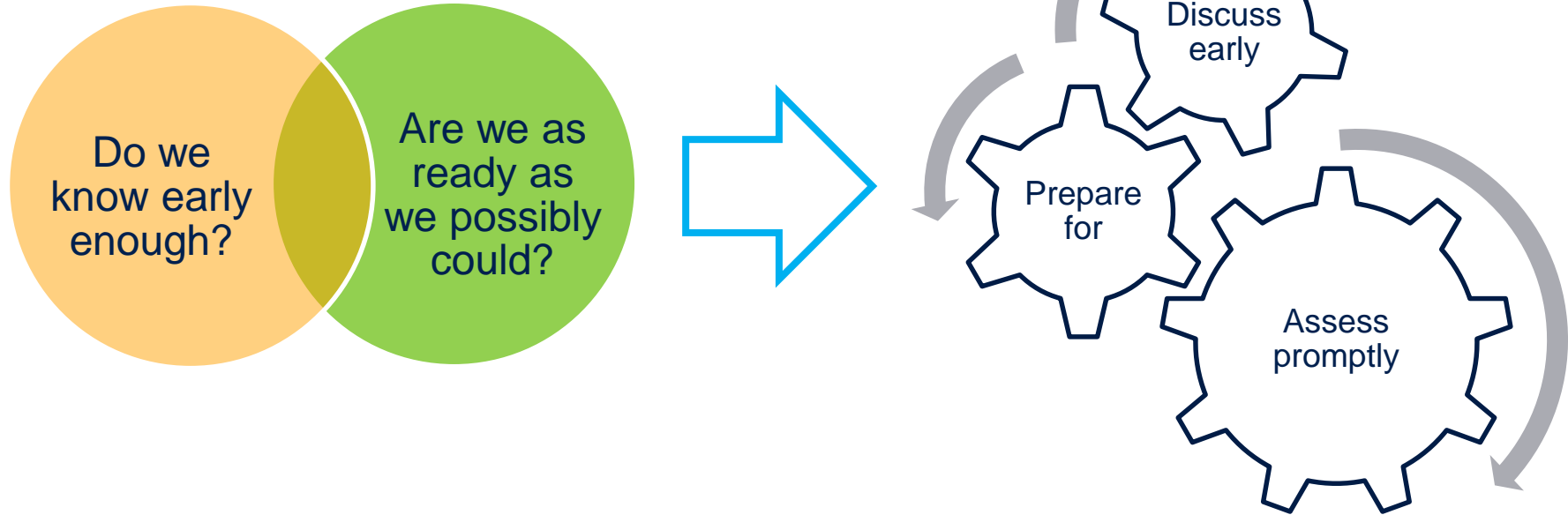
- Increase use
- New timetable
- Challenges



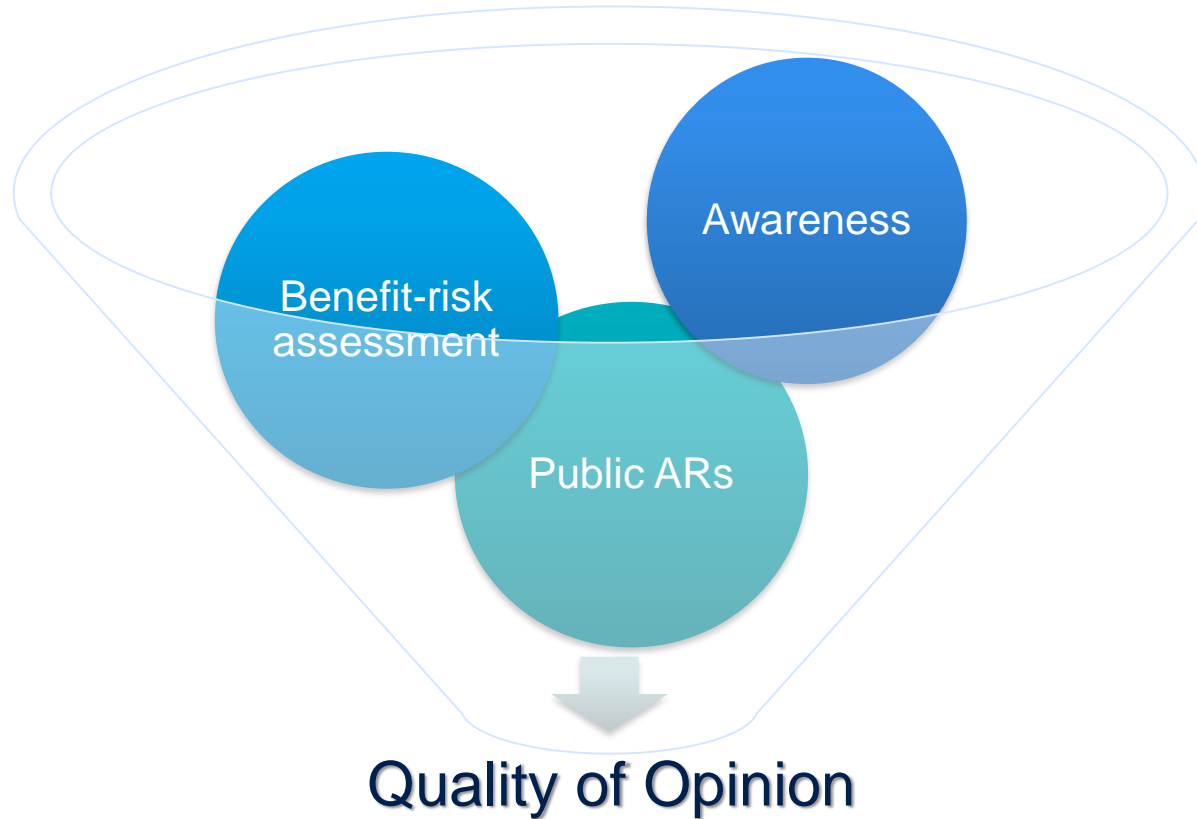
Where can
we speed
up?
Can we
help others
to speed
up?

Speeding up - accelerated assessment

Where can we speed up?
Can we help others to speed up?



Quality of Opinions



Where can we speed up?
Can we help others to speed up?

Another word on Conditional Marketing Authorisation

Identifying when benefits start to outweigh risks and uncertainties?

When is B/R balance positive based on incomplete data?

What if long-term effects are targeted?

Which of two products with similar early results is better?

Will an early authorisation lead to early access?

Will missing data be generated after authorisation?

Examples of other activities to foster application of early access tools in practice

Identifying when benefits start to outweigh risks and uncertainties ?

Adaptive pathways

Scientific concept of iterative development and data generation with use of real-life data.

Collection of real-world data

EMA patient registry initiative. IMI projects. Other EU and national initiatives.

Parallel advice with HTA bodies

To provide feedback from regulators and HTA bodies at the same time, early in the development.

Features of the PRIME scheme

Early access tool, supporting patient access to innovative medicines.

How to
choose
the right
priorities
?



- **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.

PRIME vs Adaptive Pathways

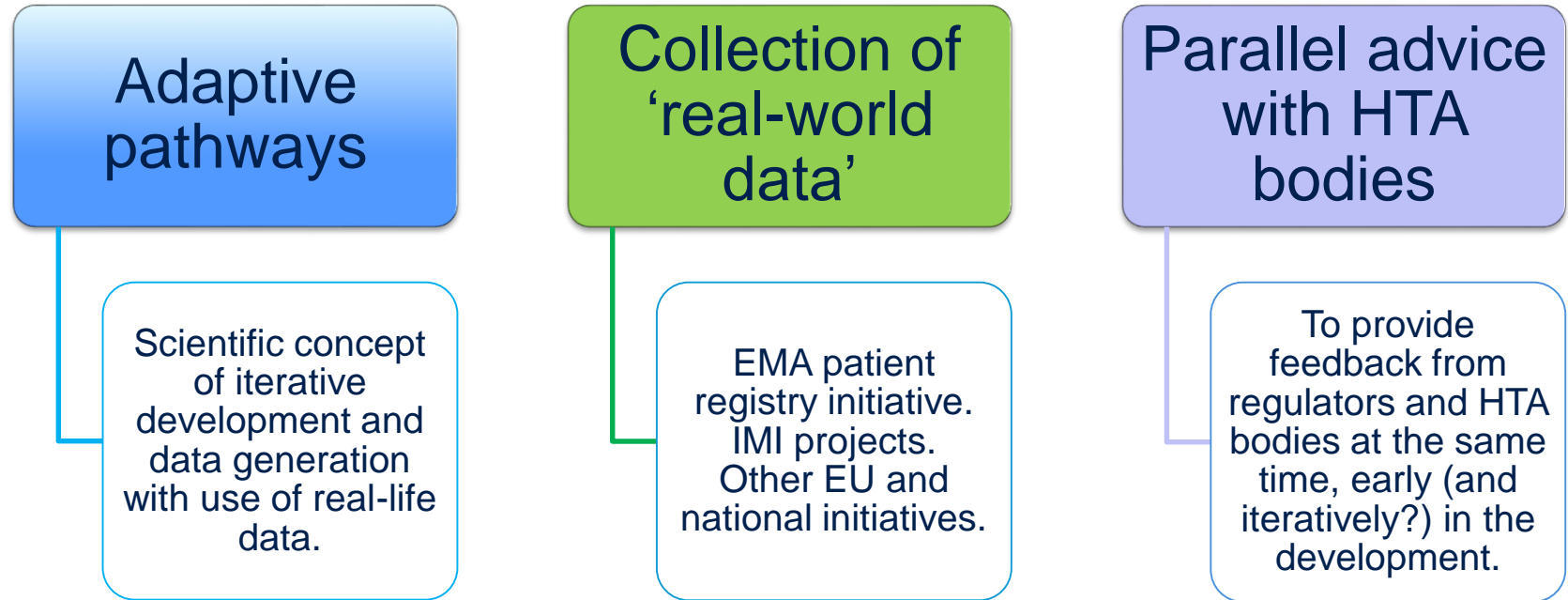
- **PRIME: Early and enhanced scientific and regulatory support** to medicine developers to optimise the generation of robust data and enable accelerated assessment.
 - Regulatory procedure with eligibility and designation.
- **Adaptive pathways: Scientific concept of data generation, medicines development, authorisation and access**

Compassionate Use

How to
choose
the right
priorities
?

- Compassionate use programmes are coordinated and implemented by Member States, which set their own rules and procedures. National Competent Authorities can ask EMA for an opinion on how to administer, distribute and use certain medicines for compassionate use.
- EAMS and PRIME are distinct; EAMS is to give patients with life threatening or seriously debilitating conditions access to medicines that **do not yet have a marketing authorisation**; PRIME is enhanced regulatory support for development **to get marketing authorisation**.
- **What challenges? What synergies?**

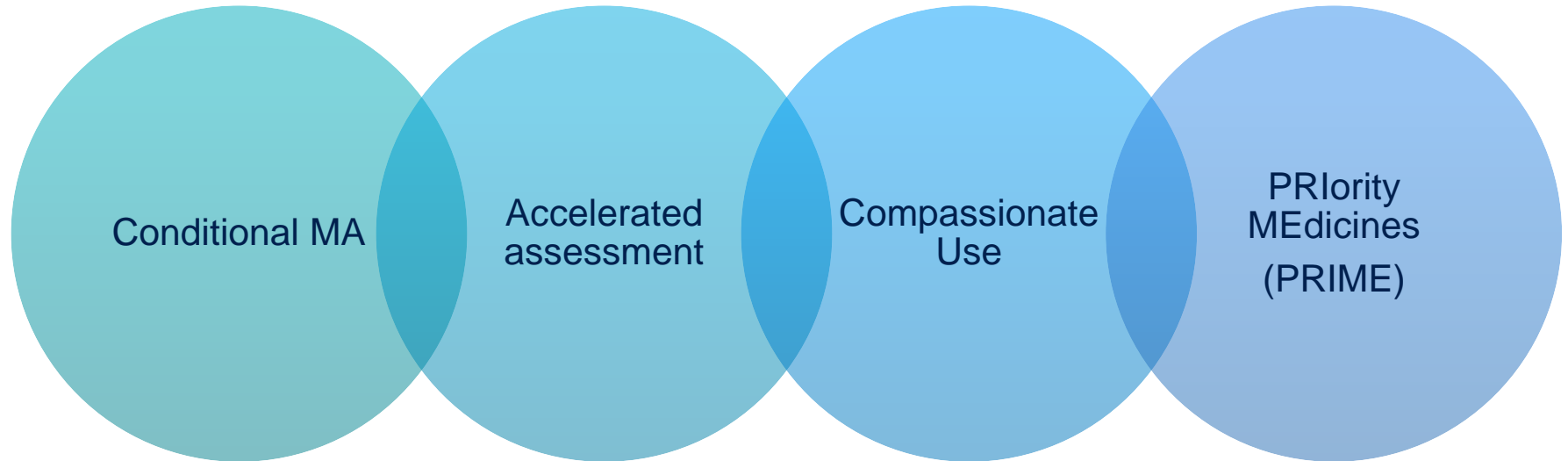
Activities to foster application of early access tools in practice



Scientific initiatives

- Enhanced role of modelling and simulation
 - For understanding, for planning, to replace clinical trials, to address uncertainties
- Extrapolation
- ICH E series
 - E6, E9, E11, E17 etc.
- EMA Initiatives on Patient Registries and Patient Involvement

What EU processes to foster early access?



Launch of PRIME and updated guidelines



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Support for early access

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The European Medicines Agency (EMA) is committed to enabling early patient access to new medicines, particularly those that target an unmet medical need or are of major public health interest. The Agency seeks to support the medicine development process from an early stage and to offer regulatory mechanisms to help promising new medicines reach patients as early as possible. Companies developing such medicines can apply to EMA for their products to make full use of these regulatory opportunities.

The European Union (EU) pharmaceutical legislation includes several provisions to foster patients' early access to new medicines that address public health needs and are eligible for the centralised procedure such as:

- ▶ **accelerated assessment**: reduces the timeframe for review of an application for marketing authorisation for medicines of major public health interest and in particular from the viewpoint of therapeutic innovation;
- ▶ **conditional marketing authorisation**: grants marketing authorisation before complete data are available;
- ▶ **compassionate use**: allows the use of an unauthorised medicine for patients with an unmet medical need. The Committee for Medicinal Products for Human Use (CHMP) issues an opinion on criteria and conditions, which national patient access programmes can consider when making such medicines available.

Related content

- ▶ Adaptive pathways
- ▶ Innovation Task Force
- ▶ Scientific advice and protocol assistance
- ▶ Scientific guidelines
- ▶ SME office

Related EU legislation

- ▶ Regulation (EC) No 726/2004

Related documents

- ▶ Development support and regulatory tools for early access to medicines (07/03/2016)

Medicines approved since 2006 using early access tools

28

Opinions for conditional marketing authorisations

22

Medicines evaluated under accelerated assessment

- SME office
- Paediatric medicine
- Geriatric medicine
- Orphan designation
- Herbal products

1 March 2016
EMA/726/2004/2015
Human Medicines Research and Development Support Division

Development support and regulatory tools for early access to medicines

The EU pharmaceutical legislation includes a number of provisions in Regulation (EC) No 726/2004 aimed at fostering patients' early access to new medicines that address public health needs and are eligible to the centralised procedure, such as:

- **accelerated assessment procedure** which reduces the timeframe for review of an application for marketing authorisation from a maximum of 210 days to 150 days for medicinal products of major public health interest and in particular from the viewpoint of therapeutic innovation;
- for certain categories of medicinal products, the possibility to obtain a **conditional marketing authorisation** on the basis of less complete data than is normally the case and subject to specific obligations and additional comprehensive data to be provided post-authorisation. Conditional marketing authorisations are valid for one year on a renewable basis;
- the possibility for a **compassionate use opinion** by the CHMP defining at European level the criteria and conditions for use of medicinal products which are made available to patients through national patients' access programmes (prior to a marketing authorisation).

To optimise the use of the above regulatory tools, EMA has launched the **PRIME** scheme to support development of medicinal products of major public health interest through early and enhanced scientific and regulatory dialogue. This tool targets support to certain types of products eligible for accelerated assessment and falling within the scope of the centralised procedure. It builds also on existing regulatory tools in place within the European Union (EU) legal framework, including scientific advice/protocol assistance.

The table overview provides a high-level overview of the above legislative and development support tools to help sponsors identify where and how to use them.

However, there are a number of other development support activities, not covered in this tabular overview, carried out by the Agency including the following:

- ▶ The **Excellence Task Force (ETF)** which is a multidisciplinary group providing a forum for informal early dialogue with applicants, in particular micro, small and medium enterprises (SMEs) and academic sponsors, to proactively identify scientific, technical and regulatory issues related to emerging therapies and technologies.

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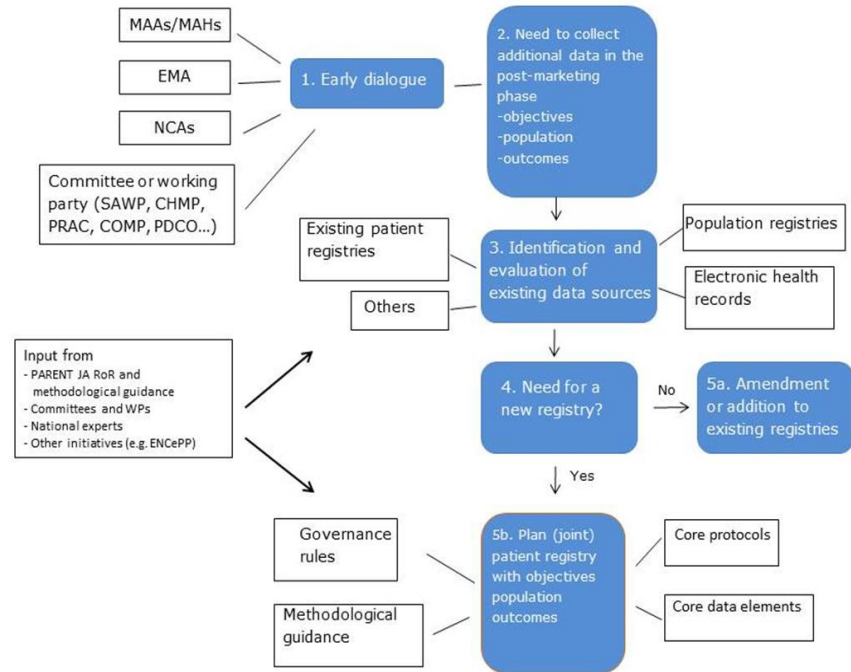
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	Development support	Accelerated assessment	Early Access regulatory tools	Other Commission use options
PRIME				
Which medicines	Medicinal products of a major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation (unmet medical need)	Medicinal products of a major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation (unmet medical need)	Medicinal products for: <ul style="list-style-type: none"> • Seriously debilitating diseases or life-threatening situations; • Orphan medicinal products Fulfilling all of the following criteria: <ul style="list-style-type: none"> • Positive risk-benefit balance • Applicant likely to be able to provide comprehensive data after authorisation • Fulfilment of unmet medical need • Benefits of immediate availability outweigh the risks that additional data are still required. 	Unauthorised medicinal products fulfilling the following criteria: <ul style="list-style-type: none"> • Orphan drugs, seriously debilitating or life-threatening diseases, with an orphan indication • Undergoing centralised RAA or centralised • Fulfilling under mandatory or voluntary scheme of reference procedure
Key features	<ul style="list-style-type: none"> • Identify potential for accelerated assessment based on early development • Early interactive assessment • Accelerated scientific and regulatory support from the SANIT/CHMP, other relevant scientific committees and EMA • Dedicated contact person within EMA 	Reduced RAA assessment time to maximum 150 days (compared to standard 210 days)	<ul style="list-style-type: none"> • Earlier authorisation of medicines for patient with unmet medical needs, on the basis of less complete data • Comprehensive data generated post-authorisation • Earlier availability of medicines to patients 	<ul style="list-style-type: none"> • Access to patients who cannot be treated satisfactorily or cannot access in ongoing clinical trials • CHMP recommendations to MS to harmonise the conditions of use, distribution and the target population

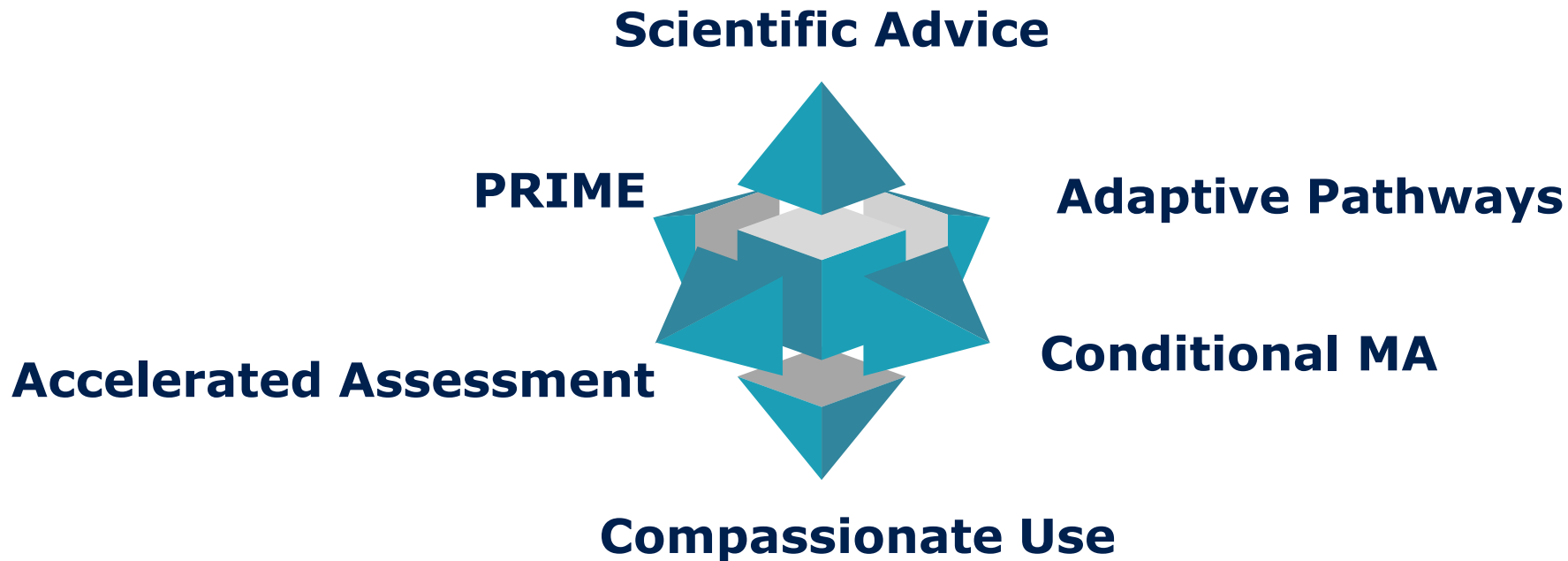
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000856.jsp&mid=...

EMA Initiative on Patient Registries

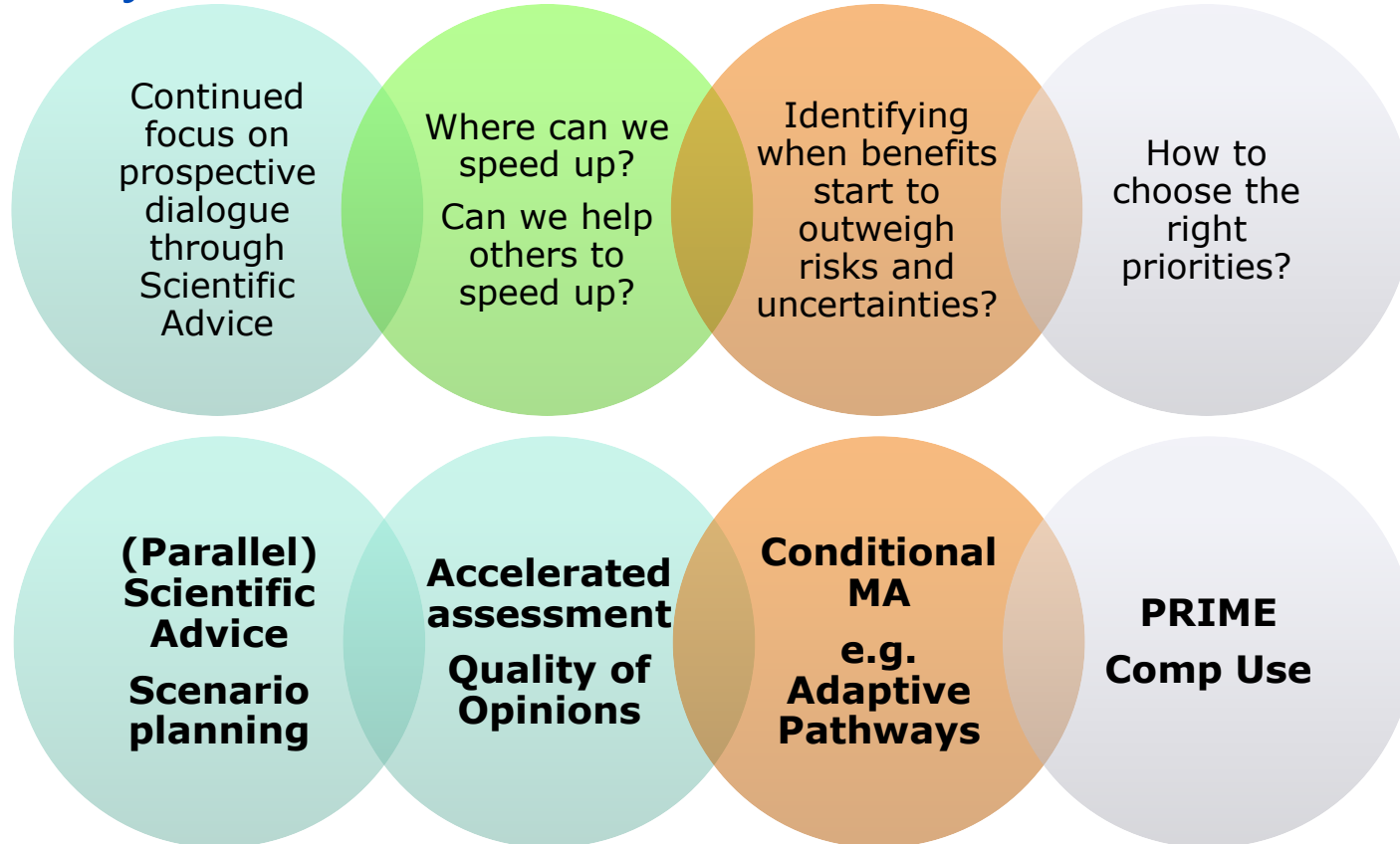
To propose an approach for an EU collaborative framework for patient registries that facilitates use of existing patient registries and setting-up new registries if none are available or adequate, in order to collect and analyse high quality data informing regulatory decisions and the evaluation of the benefit-risk profile of medicinal products



The improved toolkit



EU early access tools: Overview



Thank you for your attention
