Medicines & Healthcare products Regulatory Agency

MHRA
Regulating Medicines and Medical Devices

# EU early access tools: Overview

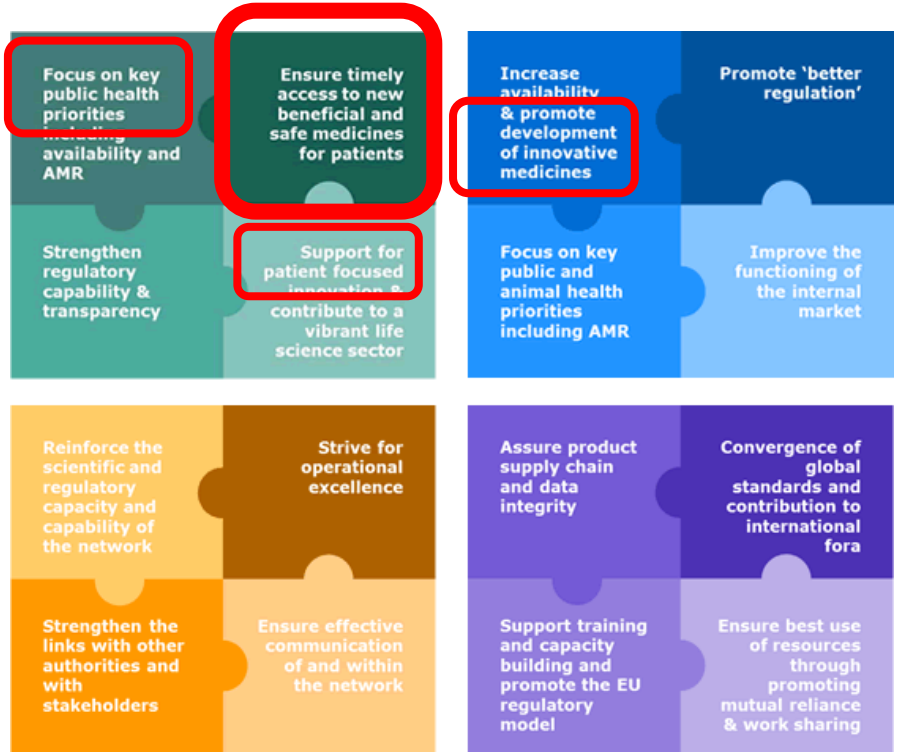
Rob Hemmings, MHRA and CHMP/SAWP, May 2016

# 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.

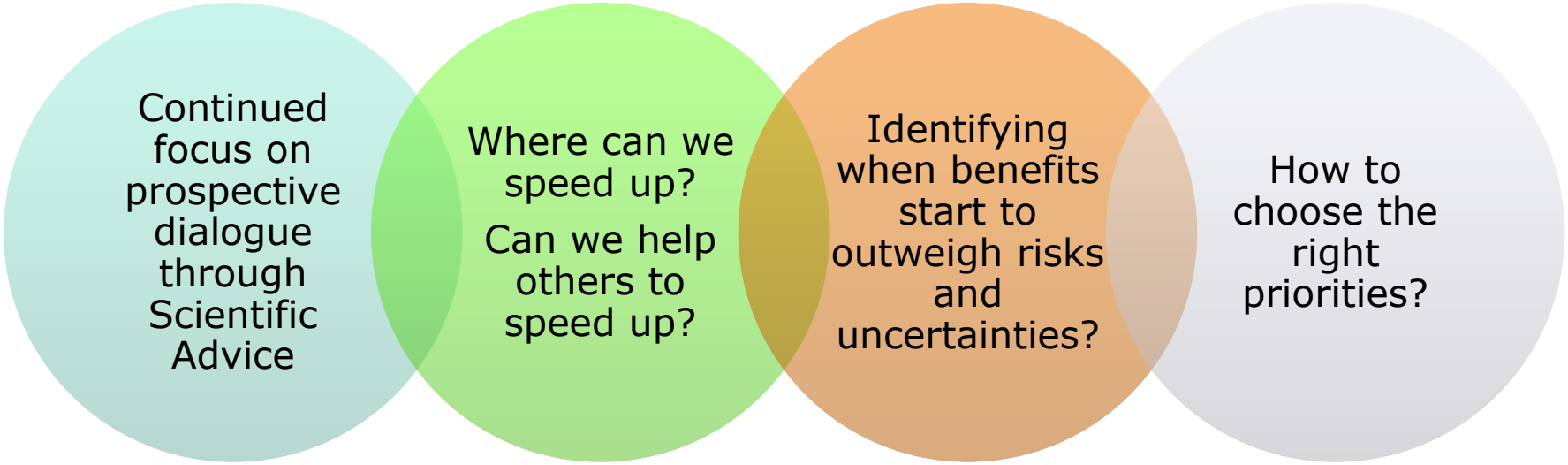
# EU Medicines Agencies Network Strategy to 2020



# 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

Continued focus on prospective dialogue through 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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# Accelerated assessment

Where can we speed up?

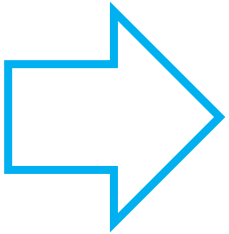
Can we help others to speed up?

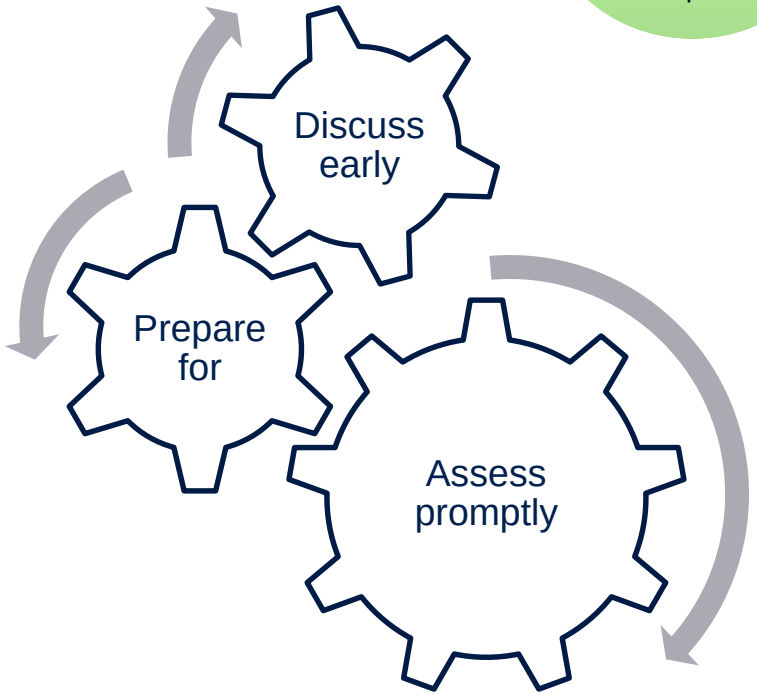
- Increase use
- New timetable
- Challenges

# Speeding up - accelerated assessment

Where can we speed up?

Can we help others to speed up?

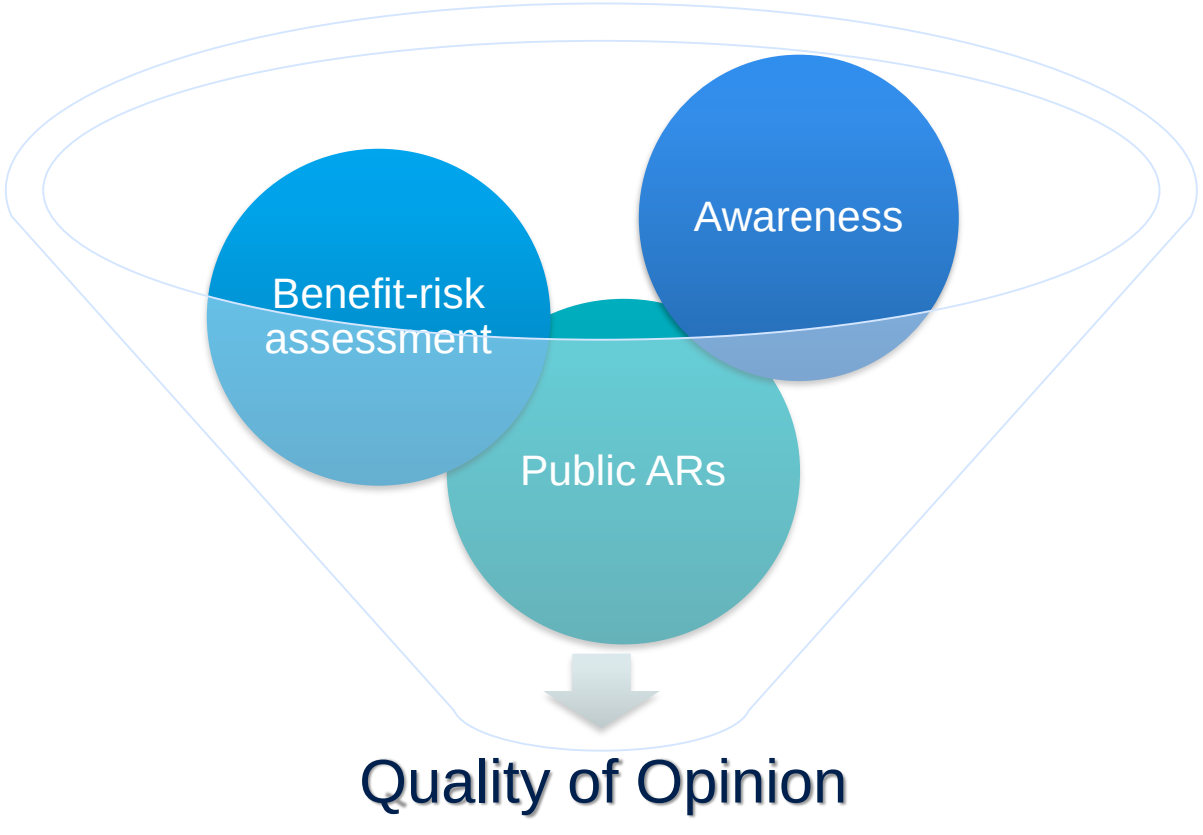





# Quality of Opinions

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

How to choose the right priorities ?

## 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.

# 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.

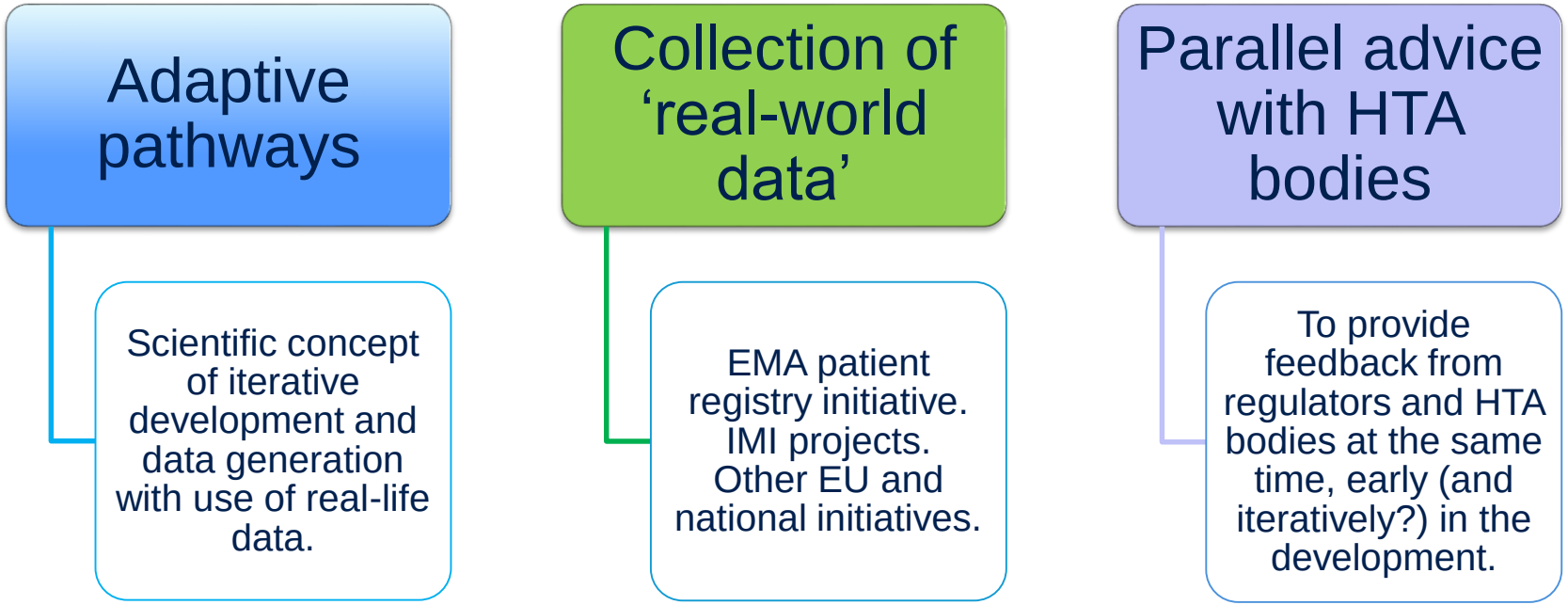
- **Adaptive pathways: Scientific concept of data generation, medicines development, authorisation and access**

# 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





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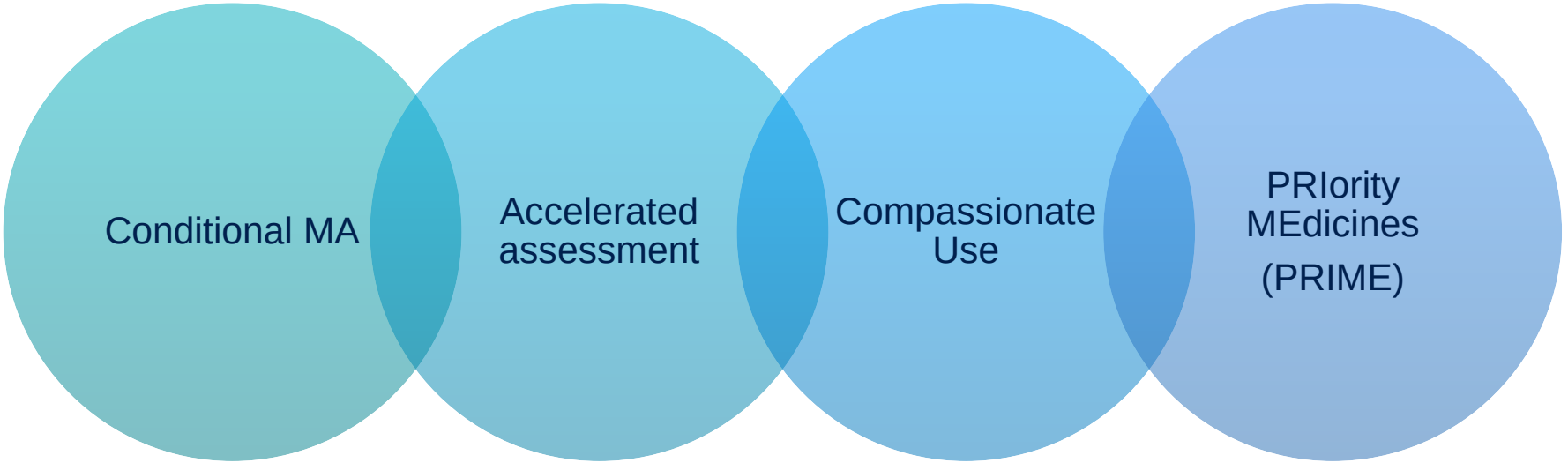
# Scientific initiatives

- 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?





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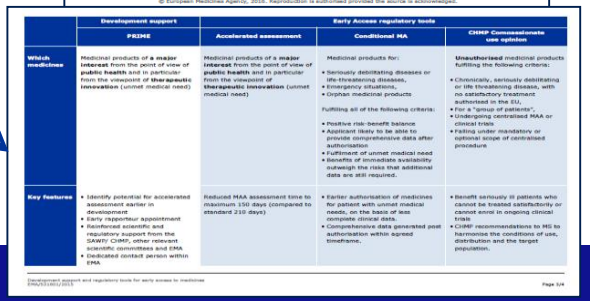
# Launch of PRIME and updated guidelines

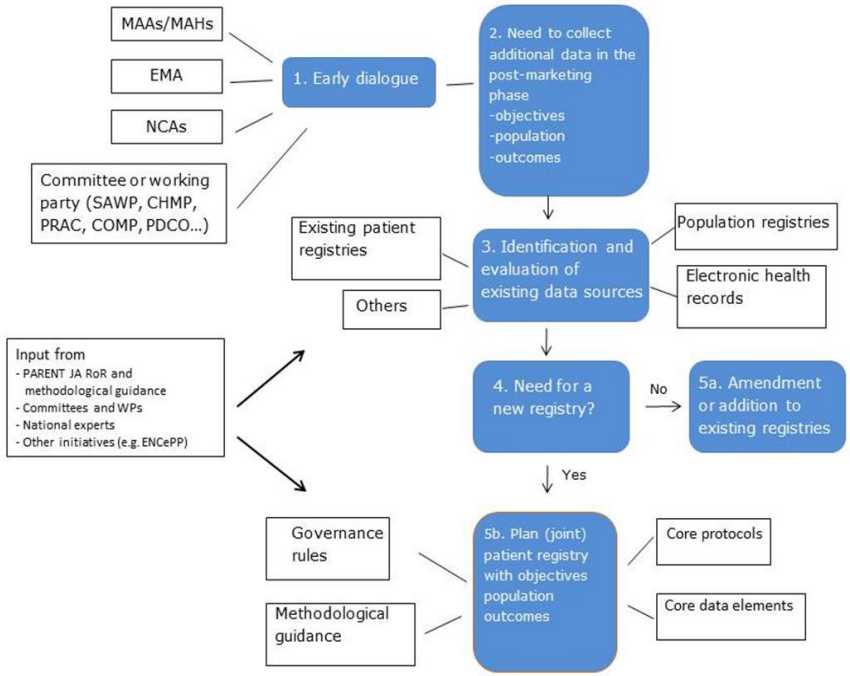




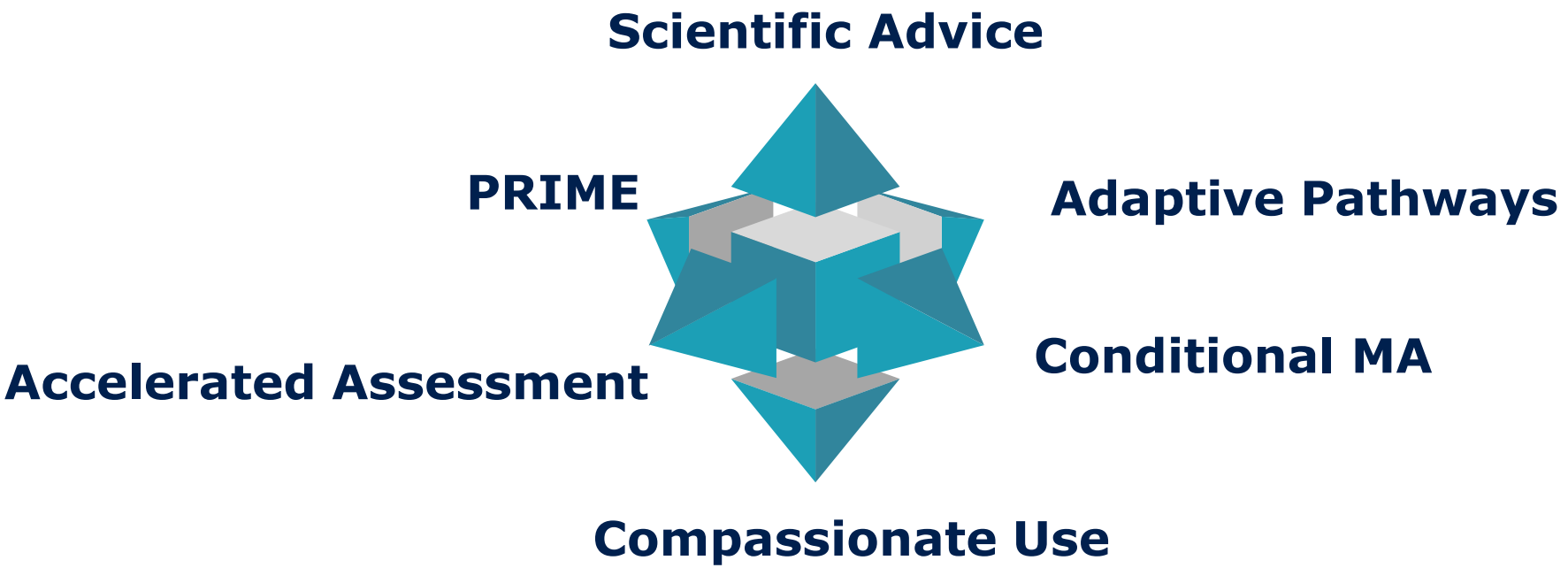

http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000856.jsp&mid=WC0b01ac058096f643

# 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



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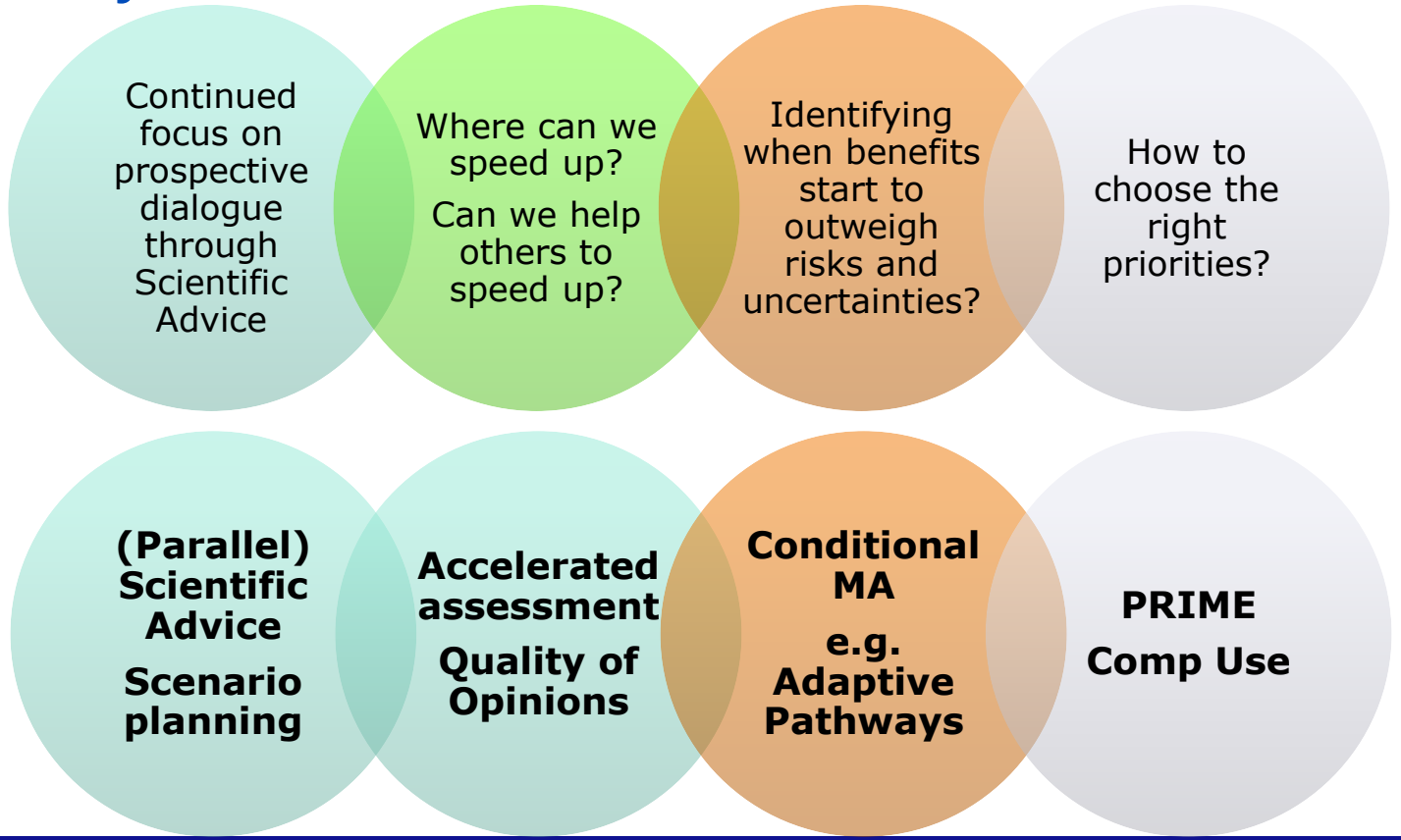

# Thank you for your attention