

Executive Briefing #2

EMA Support for Early Access

David Schwicker, January 2017

The **EMA's Support for Early Access** is focussed on **life-threatening and debilitating** diseases with a major impact on **quality of life**, and on medicines with a **credible promise** of significant improvements in clinical benefit and patient-relevant outcome(s). Additionally, these medicines are eligible to the **centralized EMA procedure**.

A number of the **innovative initiatives** listed below emphasise **early dialogues**, the early involvement of multiple **stakeholders**, **iterative** development in a **life-cycle approach**, and an **expanded toolbox** for evidence generation, with pragmatic and real-world studies complementing RCTs in areas where the collection of data via traditional routes is difficult. The tools are not mutually exclusive and can be used in combination.

Accelerated Assessment (AA)

- Reduces the timeframe for review of an application for marketing authorisation.

Conditional Marketing Authorisation (CMA)

- Earlier authorisation of medicines fulfilling a positive benefit/risk balance for patients with unmet medical needs, on the basis of less complete clinical data, and where the benefits of immediate availability outweigh the risks that additional data are still required.
- Subject to specific obligations for comprehensive clinical and real world evidence to be generated within an agreed timeframe; valid one year, renewable.

PRIority Medicines (PRIME)– “early dialogues”

- Early, proactive and reinforced scientific, joint regulatory and HTA support at key milestones in development to identify potential for accelerated assessment.
- Access to dedicated EMA resources and early rapporteur appointment.

Compassionate Use Opinion by the EMA (CHMP)

- Facilitates early market access through a centralised compassionate use opinion by the CHMP, for unauthorised products, aimed at harmonising the conditions of use, distribution and the target population across the EU.
- To benefit seriously ill patients that cannot be treated satisfactorily or that cannot enrol in ongoing clinical trials.

Parallel scientific advice with regulatory and HTA bodies

- Early in development, establishes the evidence needed for determining a medicine's risk/benefit balance and value (PRIME and AP are potential lead-ins to joint advice).

Adaptive Pathways (AP)

- A prospectively planned, iterative scientific development concept in areas where collection of data via traditional routes is difficult (e.g. rare diseases).
- Permits a stepwise approval in tightly defined patient populations with a gradual extension of the target population as more data become available. The standards of regulatory approval remain unchanged.
- Involves collaborating with all relevant stakeholders very early in the development process, particularly with HTA bodies and patients.
- “Safe harbour”, informal, non-committal entry to explore alternative development routes.
- Expands the toolbox for evidence generation, with pragmatic and real-world studies complementing RCTs.

Initiative for Patient Registries – “late dialogues”

- Aims to facilitate the use of existing and the establishment of new registries to generate refined real-world based benefit/risk and value assessments.
- Informal, collaborative “safe harbour” approach for protocol design that utilises EMA resources and collaborations and will not interfere in the regulatory process.
- Opportunity for interactions across committees and regulatory bodies (CHMP, PRAC, COMP, SAWP, HTAs).

Orphan Designation for Medicines for Rare Diseases

- To qualify for orphan designation, a medicine must meet a number of criteria: developed for the treatment, prevention or diagnosis of a life-threatening or chronically debilitating disease, the prevalence of which in the EU must not be more than 5 in 10,000, and it must address an unmet medical need and/or be of significant benefit to patients.
- Medicines obtaining an orphan designation benefit from a number of incentives: 10 years market exclusivity, access to the centralised procedure, early access, scientific advice specific to designated orphan medicines, and reduced fees for regulatory procedures.
- Many products fulfilling the criteria for orphan designation will also qualify for early access. Therefore, the feasibility of orphan drug designation should be evaluated as part of any early access strategy, and, equally, the potential benefits of early access should be considered as part of a decision to seek orphan designation.

Small- and Medium Sized Enterprise office (SME) and Innovation Task Force (ITF)

- Micro-, small- and medium sizes enterprises (e.g. startups) as well as academic innovators receive enhanced administrative, regulatory, scientific and financial EMA support, particularly for development in rare diseases and orphan designations.

Patients' and Consumers' Working Party (PCWP)

- Early and systematic inclusion of real life patient experience and views regarding benefit/risk and value in regulatory and HTA output.

Further Information

Further information on current early access tools, programs, and initiatives is available at:

<https://www.orphastrategy.com/early-access/>

EMA Support for Early Access:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000856.jsp&mid=WC0b01ac0580b18c78

About ORPHA Strategy Consulting

In rare diseases and for transformative medicines, **early access strategies, benefit/risk and value demonstrations** are often **uncharted terrain**, requiring highly specific expertise and experience.

ORPHA Strategy's principal, David Schwicker (<https://www.orphastrategy.com/biography/>), has industry consulting expertise spanning more than 25 years, and has gained a unique understanding of how early access programs, initiatives, and rare disease and orphan drug incentives can benefit a client's transformative medicine to **prospectively accelerate marketing authorization and market access**. To this is added a focus on **innovative development pathways** that emphasise the use of **real-world evidence**.

Thank you for your interest. To start a strategic discussion on early access, please contact:

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