EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EU Incentives for Orphan Medicines

M.Carr
19 September 2013

Agenda

- Early dialogue with Regulators
- Scientific Advice & Certification
- Regulatory Assistance & Fee Incentives
- EU Marketing Authorisation

EMA Road Map 2015

EUROPEAN MEDICINES AGENCY

An Agency of the European Union

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Text size: A A A | Site-wide search | GO

Home | Find medicine | Regulatory | Special topics | Document search | News & events | Partners & networks | About us | Quick links

- What we do
- Who we are
- How we work
  - Legal foundation
  - Integrated management system
  - Funding
  - Financial management and budgetary reporting
  - Conflicts of interest
  - Core management documents
  - Procedures
  - Policies
  - Road map to 2015
    - Road map to 2010
  - Business hours and holidays

Home ▸ About Us ▸ How we work ▸ Road map to 2015

Road map to 2015

Email | Print | Help | Share

The Agency's 'Road map to 2015' charts the way forward for the Agency for the five years to 2015.

Building on the progress of its previous five-year strategy, the road map sets out the **Agency's vision** for its further development as a European public-health body in the field of medicines.

It describes three priority areas for the Agency's work:

1. Addressing public health needs

- Stimulating the development of medicines for areas of unmet medical need, neglected diseases and rare diseases, and for all types of medicines for veterinary use;
- Facilitating new approaches to medicine development;
- Applying a more proactive approach to public-health threats where medicines are implicated.

2. Facilitating access to medicines

- Addressing the high attrition rate during the medicine-development process;
- Reinforcing the benefit/risk-balance assessment model;
- Continuing to improve the quality and the regulatory and scientific consistency of outcome of the scientific review.

Related information

- European Medicines Agency publishes final 'Road map to 2015' (26/01/2011)
- Road map to 2015: The European Medicines Agency's contribution to science, medicines and health (26/01/2011)

The various entry doors

Early development Support – dialogue with regulators is key

Development Challenges – seeing through the eye of the customer

Issue:

- (Perceived) barriers to engaging with regulators

Solutions:

- ✓ Easy access through SME Office
- ✓ Early support from Innovation Task Force

Tailoring assistance to SMEs

To promote innovation and development of new medicines by SMEs

SME Office created in December 2005:

- A single interface
- SME assignment, public SME register
- Fee incentives, regulatory assistance, translations
- Facilitate communication
- News bulletins, SME User Guide, Workshops

Innovation Task Force (ITF)

Early support to developers of products based on emerging science

- Objective: informal exchange of information and provision of guidance, complementing formal procedures
- Scope: regulatory, technical and scientific issues
- Applicants: Consortia, Networks, Public/private partnerships, Learned societies, Pharmaceutical industry, Academia
- Free of charge
- Scientific discussions led by experts from Agency network, working parties and committees


Scientific Advice &
Certification


Success
Failure

Protocol Assistance (Scientific advice)

- Strictly confidential
- Scientific Advice can be provided on ANY scientific question
  - Quality, non-clinical and clinical
- At any time point of the development
  - Early advice with subsequent follow-up is recommended
- Broad advice, Conditional approval/Exceptional circumstances
  - On the eligibility or on proposed development
- Qualification of biomarkers and other novel methodologies

Protocol Assistance (Scientific advice)

Voluntary, not mandatory procedure:

- companies ask questions;
- responses are prepared and discussed;
- in 50% of the cases a face-to-face meeting with the company is organised;
- written responses, adopted by the licensing Committee, sent to the company: scientific advice letter;
- short process: 40 days or 70 days.

FAQs in Scientific Advice

Quality / CMC

- comparability, stability, etc.

Non-clinical

- *in vivo* pharmacology for innovative products
- animal models for products with human specific targets, animal models mimicking the human disease, surrogate molecules
- carcinogenicity and reprotoxicity waivers, etc.

Clinical

- PK/PD, dose-finding, interactions
- exploratory & pivotal trials: study endpoints, population, comparator, blinding, statistics (interim A, adaptive/seamless design), safety DB

Compliance with scientific advice is associated with positive MA outcome…

DOI 10.1007/s00228-009-0756-y

SPECIAL ARTICLE

Factors associated with success of market authorisation applications for pharmaceutical drugs submitted to the European Medicines Agency

Jan Regnstrom · Franz Koenig · Bo Aronsson · Tatiana Reimer · Kristian Svendsen · Stelios Tsigkos · Bruno Flamion · Hans-Georg Eichler · Spiros Vamvakas

Received: 25 September 2009 / Accepted: 28 October 2009 / Published online: 20 November 2009

Abstract critical variables. Thirty-nine of these were assessed as

Certification for advanced therapy medicinal products

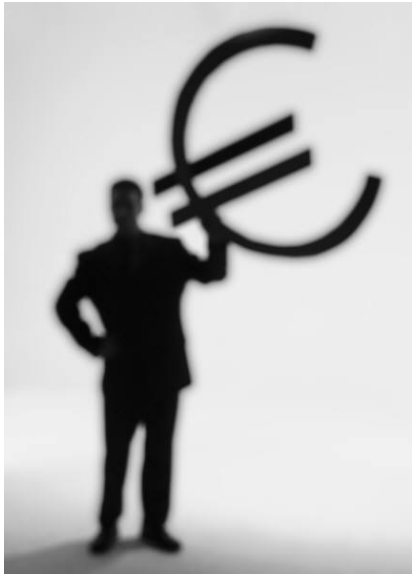
- Scientific evaluation of Quality / Non-clinical data during development
- Available to SME applicants
- Same scientific evaluation standards as for MAA
- Optional tool to potentially facilitate further development of ATMP
- Distinct to prospective scientific advice


Regulatory Assistance
& Fee Incentives

Regulatory Assistance for SMEs

- Direct assistance:
  - Queries dealt with by SME office e- mail/telecon
  - Briefing meetings/telecon on regulatory strategy
- Published SME User Guide on regulatory procedures
- SME News bulletin
- Annual training/workshops tailored for SMEs

Orphan Fee Incentives



SMEs - 100% fee waiver for:

- scientific advice
- scientific services
- marketing autorisation application
- pre-authorisation GMP, GLP, GCP, PhVig inspections
- 1st year post-licensing fees (variations etc.)

Non-SMEs – 40% fee reduction for:

- scientific advice


EU Marketing
Authorisation

Centralised procedure

Mandatory for orphan medicines

- Single scientific opinion
- Max. 210 evaluation days to Opinion
- 1 marketing authorisation valid EU
- 1 (invented) name
- 1 common Product information
- Transparent system (e.g. EPAR)
- 24 languages + IS/NO
- 28 Member States
- Access to potentially 495 million users

Incentive: Market exclusivity

Orphan Market exclusivity for 10 years

= Period of time during which a medicinal product which is similar* to an orphan medicinal product cannot be validated by the Agency, even if based on a full, complete dossier

* Similar means similar principal molecular structure and same mode of action and same indication

Market exclusivity for orphans

Extend Market exclusivity to 12 years (=Paediatric orphan)

= for orphan indication(s) covered by a condition benefiting of 10 years of market exclusivity and for which the paediatric investigation plan (PIP) is completed

Market Exclusivity (Orphan)

Marketing authorisation of reference product

for indication(s) for a separate orphan designation for which the PIP is completed

Strategy for incentive maximisation

Across the life cycle of the product:

- Explore different regulatory strategies to maximise existing legislative incentives
- Engage in early discussions of strategies with the competent authorities and with rapporteurs
- Seek regulatory and scientific advice

Thank you for your attention

Contact for further information:
- smeoffice@ema.europa.eu
- http://www.ema.europa.eu