EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Horizon Scanning for pharmaceuticals

EMA – Payer Community meeting, 19 September 2017

Presented by Michael Berntgen
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The foundation: what do we mean?

Elements that drive the approach to horizon scanning:

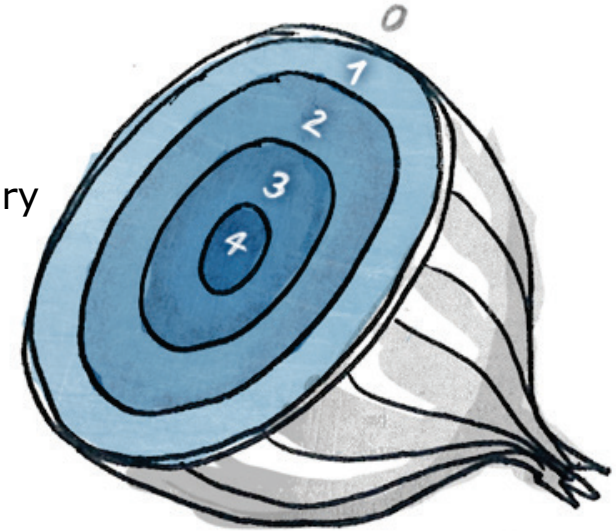
- Objectives / desired impact
- Scope of technologies / interventions
- Observation period
- Data sources and detection methodology
- Triage
- Reporting mechanisms

"Horizon scanning" means different things to different people.

Understanding the payers' needs

Using the KCE report* as (exemplifying) basis:

- Focus on pharmaceuticals (4 listings)
  - mostly in later development and during the regulatory approval process plus some specific early development (e.g. orphan designations)
  - considering the impact on health care system, i.e. financial, clinical or organisational impact
- Need for voluntary information from companies (e.g. face-to-face pipeline meetings)

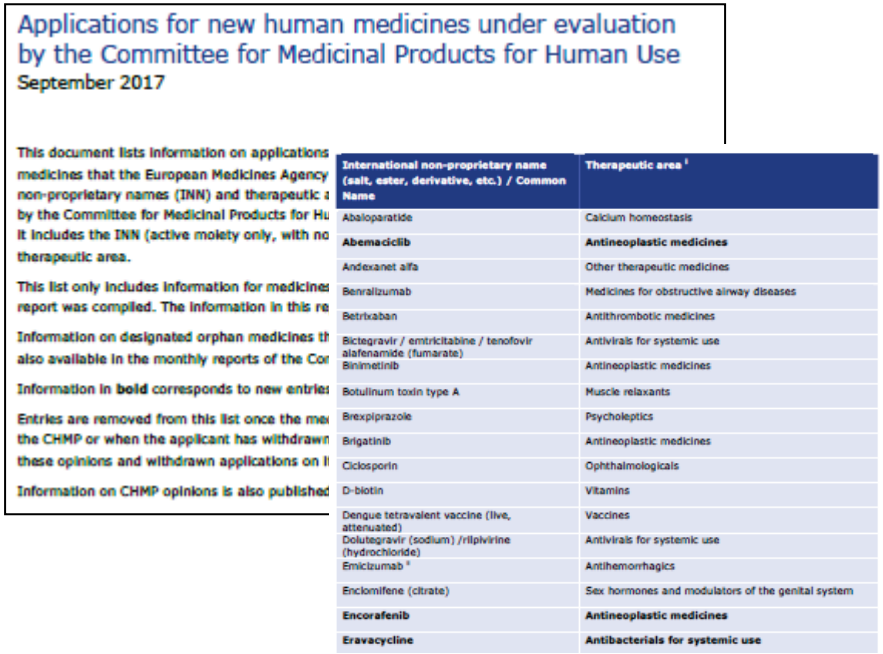


* Horizon scanning for pharmaceuticals: proposal for the BeNeLuxA collaboration

Information that can be used as source data (1/2)

During the regulatory review of the marketing authorisation application:

- Monthly listings of "Medicines under evaluation"
- Progress updates through milestones in published CHMP agendas/minutes



Applications for new human medicines under evaluation by the Committee for Medicinal Products for Human Use
September 2017

This document lists information on applications
medicines that the European Medicines Agency
non-proprietary names (INN) and therapeutic a
by the Committee for Medicinal Products for Hu
It includes the INN (active moiety only, with no
therapeutic area.

This list only includes information for medicines
report was compiled. The information in this re

Information on designated orphan medicines th
also available in the monthly reports of the Cor

Information in **bold** corresponds to new entries

Entries are removed from this list once the me
the CHMP or when the applicant has withdrawn
these opinions and withdrawn applications on l

Information on CHMP opinions is also published

International non-proprietary name (salt, ester, derivative, etc.) / Common Name	Therapeutic area [1]
Abaloparatide	Calcium homeostasis
Abemaciclib	**Antineoplastic medicines**
Andexanet alfa	Other therapeutic medicines
Benralizumab	Medicines for obstructive airway diseases
Betrixaban	Antithrombotic medicines
Bictegravir / emtricitabine / tenofovir alafenamide (fumarate)	Antivirals for systemic use
Binimetinib	Antineoplastic medicines
Botulinum toxin type A	Muscle relaxants
Brexpiprazole	Psycholeptics
Brigatinib	Antineoplastic medicines
Ciclosporin	Ophthalmologicals
D-biotin	Vitamins
Dengue tetravalent vaccine (live, attenuated)	Vaccines
Dolutegravir (sodium) /rilpivirine (hydrochloride)	Antivirals for systemic use
Emicizumab *	Antihemorrhagics
Enclomifene (citrate)	Sex hormones and modulators of the genital system
Encorafenib	**Antineoplastic medicines**
Eravacycline	**Antibacterials for systemic use**

Making it real: examples of available information

Case 1: accelerated assessment agreed

Case 2: evaluation milestone achieved

Brexpiprazole	Psycholeptics
Brigatinib	Antineoplastic medicines
Ciclosporin	Ophthalmologicals
D-biotin	Vitamins
Dengue tetravalent vaccine (live, attenuated)	Vaccines
Dolutegravir (sodium) /rilpivirine (hydrochloride)	Antivirals for systemic use
Emicizumab [‡]	Antihemorrhagics
Enclomifene (citrate)	Sex hormones and modulators of the genital system

Emicizumab - Orphan - H0004406

Roche Registration Limited; indicated for routine prophylaxis to prevent bleeding or reduce the frequency of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) with factor VIII inhibitors, can be used in all age groups.

Scope: Briefing note and Rapporteurs' recommendation on the request for accelerated assessment

Action: For adoption

The CHMP agreed to the request for accelerated assessment and adopted the briefing note and Rapporteurs' recommendation on the Request for Accelerated Assessment.

Minutes of the CHMP meeting in May 2017

- brigatinib - EMEA/H/C/004248

treatment of anaplastic lymphoma kinase (ALK)-positive non-small cell lung cancer (NSCLC)

Scope: Day 120 list of questions

Action: For adoption

The Committee discussed the issues identified in this application.

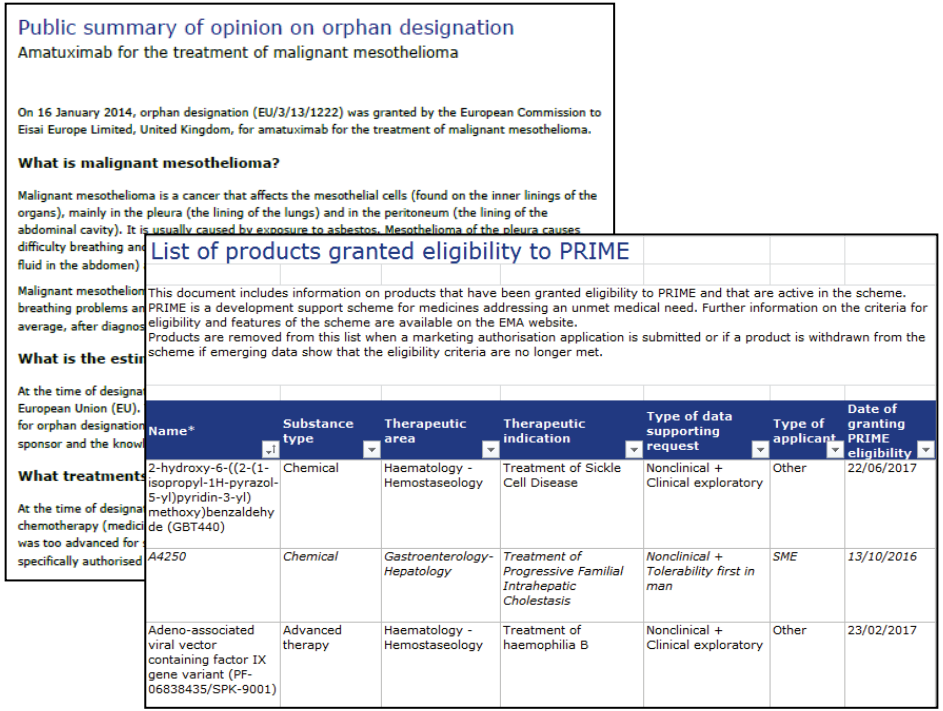
The Committee adopted the CHMP recommendation and scientific discussion together with the list of questions.

Minutes of the CHMP meeting in June 2017

Information that can be used as source data (2/2)

During the development phase:

- Products that received an orphan designation, including details of the condition and high-level information about the development stage
- Products with confirmed eligibility for the PRIority MEdicines scheme (PRIME)



Public summary of opinion on orphan designation

Amatuximab for the treatment of malignant mesothelioma

On 16 January 2014, orphan designation (EU/3/13/1222) was granted by the European Commission to Eisai Europe Limited, United Kingdom, for amatuximab for the treatment of malignant mesothelioma.

What is malignant mesothelioma?

Malignant mesothelioma is a cancer that affects the mesothelial cells (found on the inner linings of the organs), mainly in the pleura (the lining of the lungs) and in the peritoneum (the lining of the abdominal cavity). It is usually caused by exposure to asbestos. Mesothelioma of the pleura causes difficulty breathing and

Malignant mesothelion
breathing problems ar
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What is the esti

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for orphan designation
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What treatment

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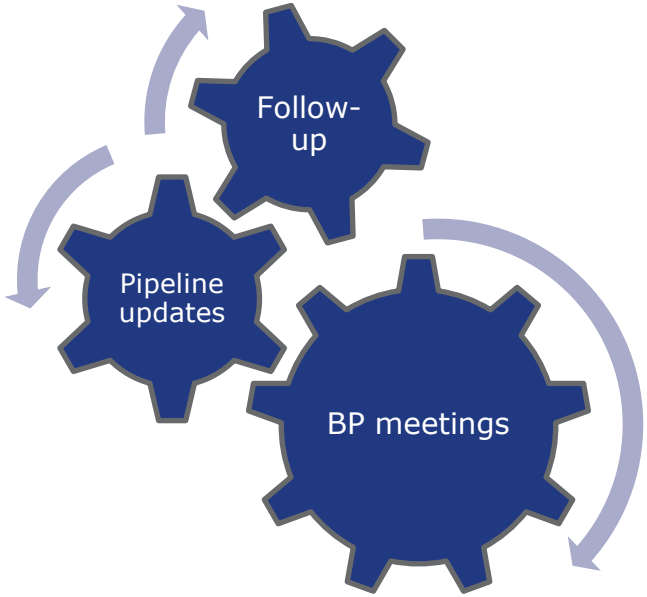
chemotherapy (medici
was too advanced for
specifically authorised

List of products granted eligibility to PRIME

This document includes information on products that have been granted eligibility to PRIME and that are active in the scheme. PRIME is a development support scheme for medicines addressing an unmet medical need. Further information on the criteria for eligibility and features of the scheme are available on the EMA website.
Products are removed from this list when a marketing authorisation application is submitted or if a product is withdrawn from the scheme if emerging data show that the eligibility criteria are no longer met.

Name*	Substance type	Therapeutic area	Therapeutic indication	Type of data supporting request	Type of applicant	Date of granting PRIME eligibility
2-hydroxy-6-((2-(1-isopropyl-1H-pyrazol-5-yl)pyridin-3-yl) methoxy)benzaldehyde (GBT440)	Chemical	Haematology - Hemostaseology	Treatment of Sickle Cell Disease	Nonclinical + Clinical exploratory	Other	22/06/2017
A4250	*Chemical*	*Gastroenterology-Hepatology*	*Treatment of Progressive Familial Intrahepatic Cholestasis*	*Nonclinical + Tolerability first in man*	*SME*	*13/10/2016*
Adeno-associated viral vector containing factor IX gene variant (PF-06838435/SPK-9001)	Advanced therapy	Haematology - Hemostaseology	Treatment of haemophilia B	Nonclinical + Clinical exploratory	Other	23/02/2017

EMA experience with business pipeline meetings



Development of a framework in which companies voluntarily provide information on their pipeline

- Meeting frequency depending on needs
- Confidential provision of information (commercially sensitive)
- "e-updates" to provide additional data
- Instrumental in contributing to preparedness

EMA's aims with business analysis and forecasting

To support for "the near future"
- operational workload
- expertise needed

based on type of applications, therapeutic areas, medicinal products, indications etc. (not: prices / medicines budget)

To facilitate development activities by identifying 'road blocks', signpost companies to interaction opportunities and support reviews of trends (environmental analysis)

Additional observations

- "Horizon scanning" has been identified as an area for collaboration between regulators and HTAs
  - The EMA/EUnetHTA work plan has a dedicated item
  - The Synergy group from the HTA Network is formulating an action plan
- Opportunity to link up activities on European level, capitalising from synergies in data needs whilst recognising differences in objectives (due to different mandates)

To start the debate: where to go from here?

- Part of the data needs identified by payers can be addressed already:
  - Jointly explore how to optimise the reporting of available data
  - Payers to perform product selection based on their needs
- Benefit from experience with setting up a business pipeline monitoring :
  - Exchange on learnings from development and conduct of company meetings
  - Possibility to reach out to industry to see readiness to engage
- Opportunity to see how data held by different actors can be mutually beneficial
- Importance to avoid duplication of efforts hence proposal to link up with other activities from payers and from HTAs on European level