EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Workshop on quality support to early access approaches (PRIME & Breakthrough)

Perspective from the EMA

Veronika Jekerle, Quality Office & BWP scientific secretariat

Content

- PRIME scheme & goals
- Experience
- Quality challenges

PRIority **ME**dicines scheme (* March 2016)

support the development of medicines with **major public health interest**

Scientific & regulatory advice	Robust data generation	Accelerated access
early interaction	focus the development	discuss filing strategies early on
raise awareness on regulatory & scientific requirements as early as possible	promote robust & high quality data	generate and leverage high quality data for MAA dossier

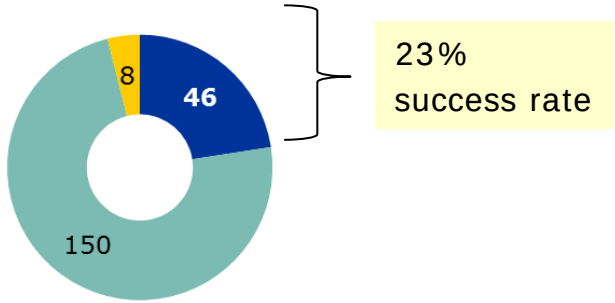
https://www.ema.europa.eu/en/human-regulatory/research-development/prime-priority-medicines

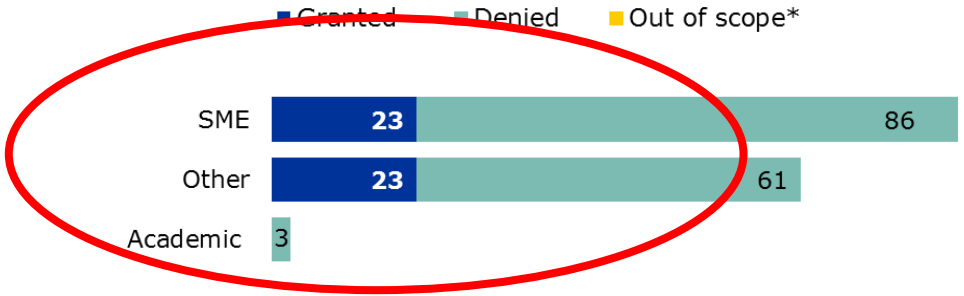
PRIME eligibility criteria

'*a major therapeutic advantage over existing treatments, or benefit patients without treatment options*'

- medicine to show its potential to benefit patients with unmet medical needs based on early clinical data

PRIME eligibility recommendations adopted by 18 October 2018





http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000660.jsp&mid=WC0b01ac05809f8439

PRIME 2 year report: http://www.ema.europa.eu/ema/index.jsp?curl=pages/news_and_events/news/2018/05/news_detail_002953.jsp&mid=WC0b01ac058004d5c1

Features of the PRIME scheme

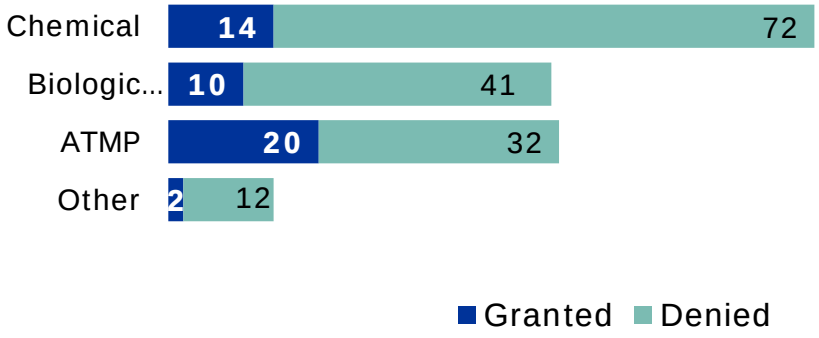
Early access tool, supporting patient access to innovative medicines.

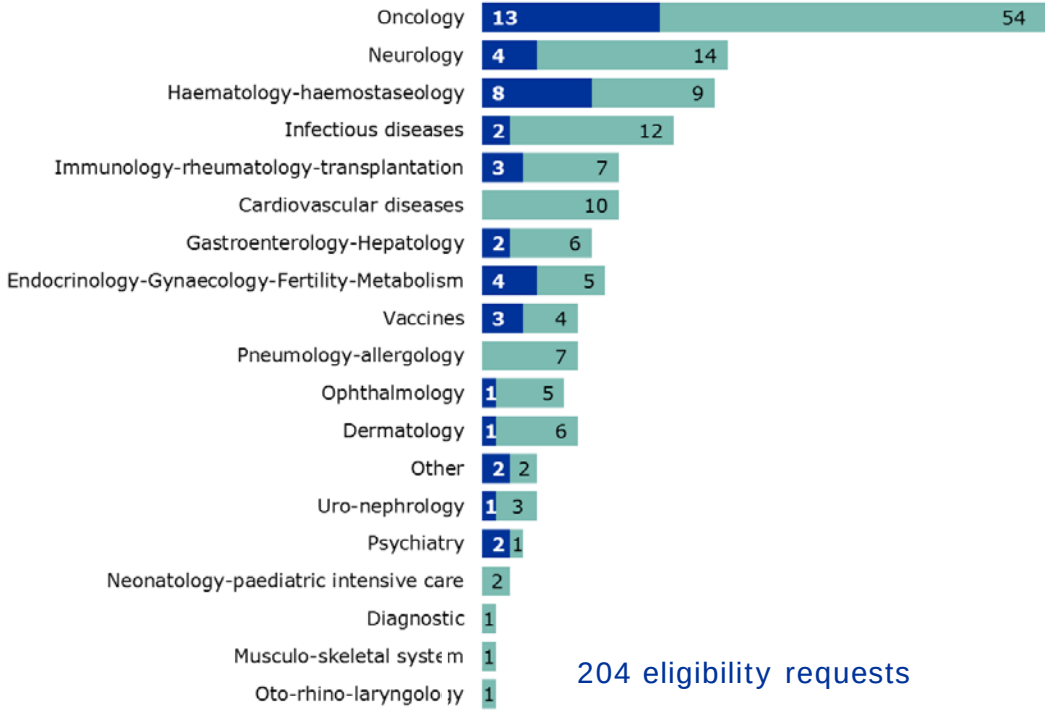
- **Written confirmation of PRIME eligibility** and potential for accelerated assessment;
- **Early CHMP Rapporteur appointment** during development;
- **Kick off meeting** with multidisciplinary expertise from EU network;
- **Enhanced scientific advice** at key development milestones/decision points;
- **EMA dedicated contact point**;
- **Fee incentives** for SMEs and academics on Scientific Advice requests.

Slide by Z. Hanaizi

Eligibility requests (Mar 16 – Nov 18)

Product classes





204 eligibility requests

46 granted*

Challenges

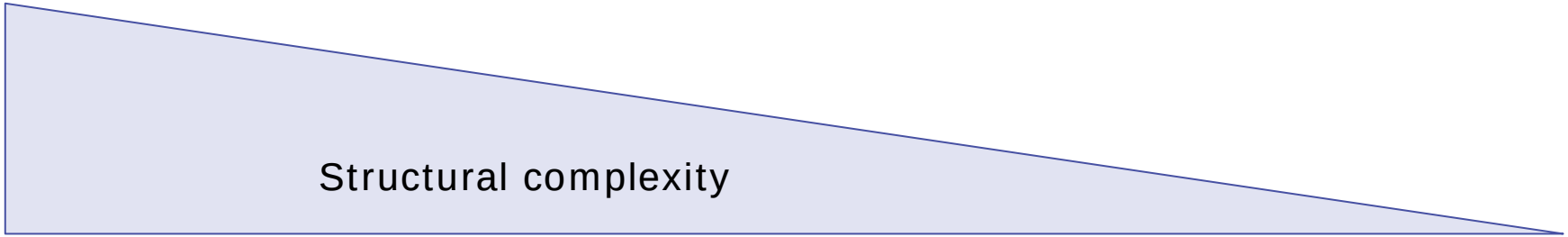
- **Timelines** (e.g. commercial manufacturing sites/description, validation data, stability, control strategy)
- **Innovation & complexity** (e.g. product characterisation, potency, comparability)
- **Global development** (e.g. comparability, manufacturing sites, batch release testing)

→ Module 3 data requirements in line with scientific guidelines and technical requirements according to the EU legislation

(**Annex I of Dir. 2001/83/EC**, *Chemical, pharmaceutical and biological information for medicinal products containing chemical and/or biological active substances*)

Product complexity

Genetically modified cells	Cell/Tissue engineered products	Viral vectors GTMPs	Viral & other vaccines	Recombinant proteins	Synthetic peptides / oligos	Chemically derived small molecules



Designated PRIME candidates (Oct 2018):

10	1	**9**	2	**10**	2	**12**

Scientific challenges of PRIME candidates

areas - scientific advice requests (data up to July 2018)

Critical areas
Number of question > 7

API/ Substance

Areas	Raw materials	Orphan similarity	Cell banks	**Starting materials**	GMP/ site
# Q	**2**	**2**	**4**	**8**	**6**

Process

Areas	Process development	**Comparability**	Change management	**Validation**
# Q	**4**	**22**	**2**	**8**

Control

Areas	Potency assay	**Analytical control strategy**	**Specifications**	Adventitious agents	**Stability**	Product-rel. impurities
# Q	**6**	**14**	**7**	**3**	**9**	**5**

Regulators perspective

- PRIME is a support scheme for development: product quality should not be compromised
- Flexibility can be considered in terms of **when** the quality data comes in (partly post-authorisation) (→ **not if)**
- Risk-based approach to justify the available quality data vs. requirements
- Alternative datasources (e.g. platform/pilot scale data) etc. can be considered provided the relevance is established (see EMA Prior knowledge workshop: Meeting report - Prior knowledge workshop)
- Quality to be considered in the context of the benefit/risk assessment (at CHMP)

Organising Committee (EMA & US FDA)

EU ad-hoc expert group

Marcel Hoefnagel (NL), BWP member
Mats Welin (SE), BWP member
Sean Barry (IE), BWP member
Jobst Limberg (DE), QWP member
Kristofer Olofsson (SE), QWP member
Tone Agasoster (NO), QWP member
Giampiero Lorenti (IT), IWG member

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Thank you for your attention

Further information

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