EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Regulatory perspective

Rare Cancers Europe Webinar on "Rare Cancers in All Policies"

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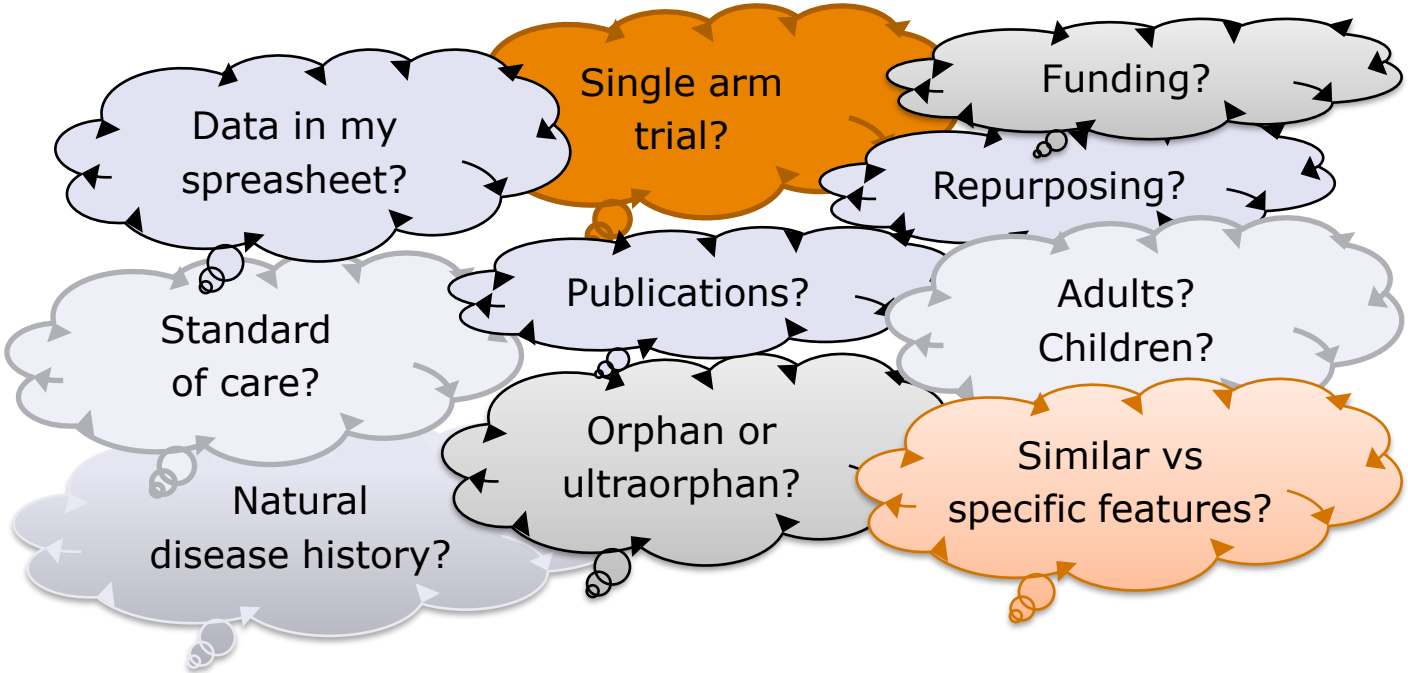
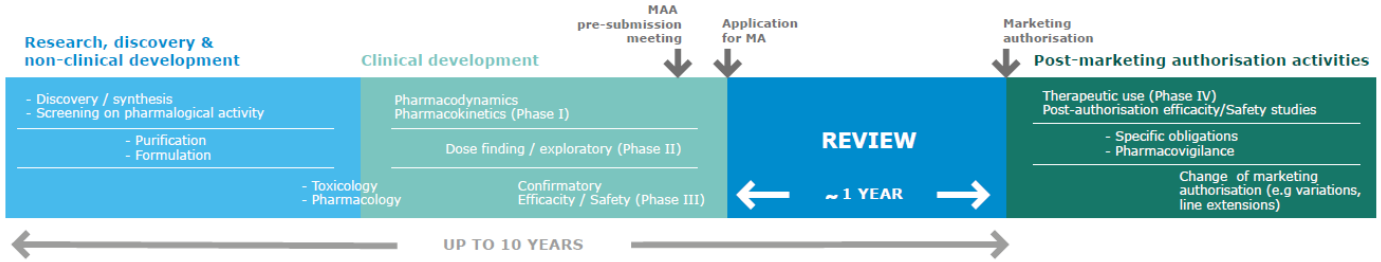
Disclaimer

The presenter does not have any conflict of interest

The views expressed in this presentation are the personal views of the author(s) and may not be understood or quoted as being made on behalf of or reflecting the position of the European Medicines Agency or one of its committees or working parties.

New medicines to treat a rare cancer: where to start?





Medicine development

Opportunities for dialogue with EMA

Orphan Drug Designation and incentives
Orphan designation/incentives for products being developed and authorised for rare conditions.

PRIME Scheme
Reinforced scientific and regulatory support. Early rapporteur appointment and early identification of candidates for accelerated assessment.

Paediatric requirements and incentives
Paediatric investigation plan (PIP), deferral or waiver, PIP modification, MA compliance, s… and post-authorisation requirements.

Certification procedure for ATMPs
Scientific evaluation of quality/ non clinical data.

Scientific Advice/Protocol Assistance
Scientific advice for quality/safety/efficacy issues. Protocol assistance for orphan products.Parallel scientific advice with health-technology-assessment bodies.

Classification as ATMPs
Confirmation that the product falls within the definition of an advanced therapy medicinal product.

Innovation Task Force
Briefing meetings. Provision of guidance early in the development process.

SME status
Incentives and regulatory assistance, briefing meetings for SMEs.

Product discussions

Product discussions

…duct discussions

Workshop with study groups, patients, companies

See „User guide" https://www.ema.europa.eu/documents/regulatory-procedural-guideline/user-guide-micro-small-medium-sized-enterprises_en.pdf

Small populations: different regulatory frameworks built for purpose

Orphan conditions (valid for products that received an orphan designation)	•Orphan designation •Protocol Assistance •PRIME
Paediatric conditions (some are rare, need dedicated research)	•Scientific advice •PIP, potentially PUMA •PRIME
Subsets of diseases (e.g. biomarker driven indications)	•ITF •Scientific advice •PRIME
Personalised medicine (innovative trials, out of the box proposals for regulators)	•ITF •Scientific advice •PRIME

Medicines recommended for approval in the last 3 years

	2018	2019	2020
Medicines recommended for approval	**84**	**66**	**97**
Total Solid and Blood Cancer	**24**	**13**	**32**
Rare Solid Cancer + Rare Blood Cancer	**6**	**2**	**8**
Rare disease (orphan designation at the time of CHMP opinion)	21	7	22
Conditional marketing authorisation	**3**	**0**	**3**
Exceptional circumstances	**0**	**1**	**2**

Conditional marketing authorization:

- unmet medical needs
- less complete clinical data than normally required
- specific post-authorisation obligations

Exceptional circumstances:

- comprehensive data cannot be obtained (e.g. only very few patients with the disease)
- specific post-authorisation obligations and monitoring

Present and Future Challenge

- "*Clinical **research** in cancer is challenging and especially so for the many rare and paediatric cancers. There is an opportunity for the EU to optimise the research effort by ensuring access to clinical trials and facilitating participation in trials when considered the best option. For this, we need to leverage the resources for conducting high quality clinical research in Europe.*
- "*Rare cancers will benefit from facilitating access to **clinical trials** that would otherwise take prohibitively long time to conduct……..need to encourage and support collaborative clinical trials leveraging collaboration between academia and network scientists to address rapidly emerging regulatory science research questions as well as treatment optimisation."*
- "*the challenge is in creating and supporting the right infrastructure across Europe for systematically collecting, federating, and **sharing of key data** from different sources"*

Emer Cooke- Executive Director EMA

Thank you for your attention

Further information

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