EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Rare Diseases Day 2024

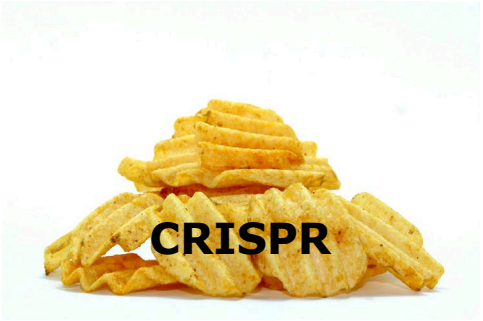
Ask the regulator

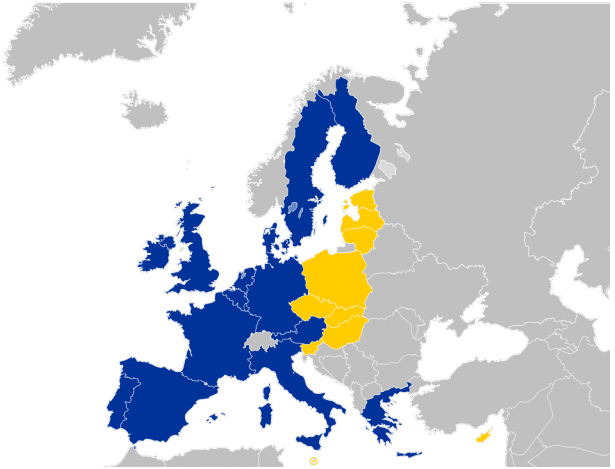
Webinar

Presented by Kristina Larsson on 29 Feb 2024
Head of Orphan Medicines Office, Human Evidence Generation, EMA

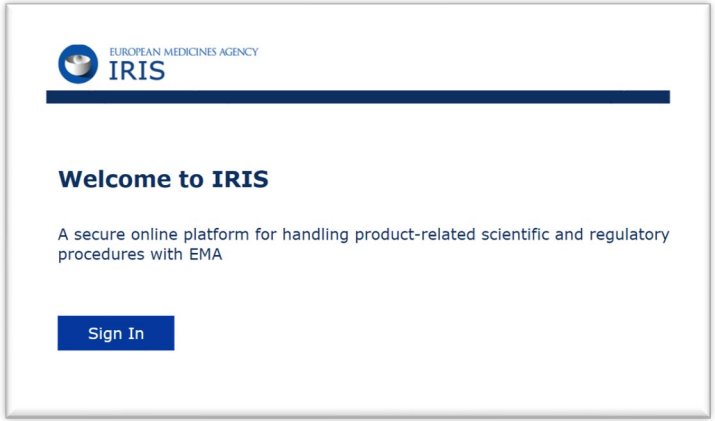
An agency of the European Union

2000 vs now…





Orphan designation submissions



2000 → 2024

Designation criteria, regulation (EC) No 141/2000

RARITY (prevalence) / RETURN OF INVESTMENT**, Art 3.1 (a)**

- Medical condition affecting not more than 5 in 10,000 in the Community (around 250,000 people)
- Without incentives it is unlikely that the marketing of the product would generate sufficient return to justify the necessary investment

SERIOUSNESS

- Life –threatening or chronically debilitating

ALTERNATIVE METHODS AUTHORISED, Art 3.1(b)

- If satisfactory method exist the sponsor should establish that the product will be of significant benefit

Incentives for orphan medicines

- ❑ Fee reduction / exemptions
  - Extended incentives for Small and Medium sized Enterprises (SMEs)
- ❑ 10-year market exclusivity (+ 2 if paediatric)
  - Protection against similar products structure mech of action same indication
  - Three derogations: Sponsor's consent, lack of supply, clinical superiority
- ❑ Product development
  - Protocol assistance, reduced fee
- ❑ Community marketing authorisation (all EU and EEA member states)

European regulatory input along drug life cycle

pharm | Non-clinical | 1 | 2 | 3 | MAA | Post market

Benefit/risk

Paediatric investigation plan

Orphan designation

Orphan maintenance

Scientific advice / Protocol assistance

PhV

Communication

- EMA orphan designation website
  - Q&A (general public)
  - Guidance documents
- COMP minutes
- Scientific publications
- Orphan Maintenance Assessment Report (OMAR) - published with EPAR

Rare diseases, orphan medicines

Getting the facts straight

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Support to development

PRIME

Innovation Task Force

Orphan designation

Paediatric development

Scientific advice and Protocol assistance

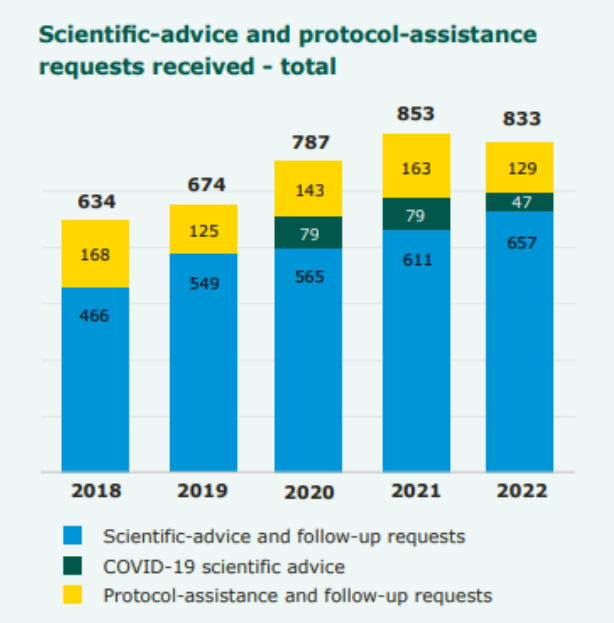
Qualification advice and opinion

Protocol assistance

Scientific Advice Working Party.

Advising companies on specific questions they have during development of medicines to meet regulatory and scientific requirements:

- how to manufacture them;
- how to test them first in the tube and in experimental animals; and
- most importantly: how to test them in humans in clinical trials.
- COMP responds to the question on Significant Benefit



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Contact points

Patients and carers

Academia

SME office

Some things do not change

- Our devotion and support to developments of rare diseases
- Our work to improve the experience for stakeholders working with EMA

Remember:

- The importance of early interaction with EMA and the benefits of early orphan designation
- Ask for protocol assistance (or scientific advice)
- Use the suitable contact points, we are here to help you!