An overview of EMA development support to innovative medicines and technologies

EIC – EMA Info Day: Regulatory support for the development of innovative medicines and technologies

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An agency of the European Union

Disclaimer

The presenter does not have any conflict of interests.

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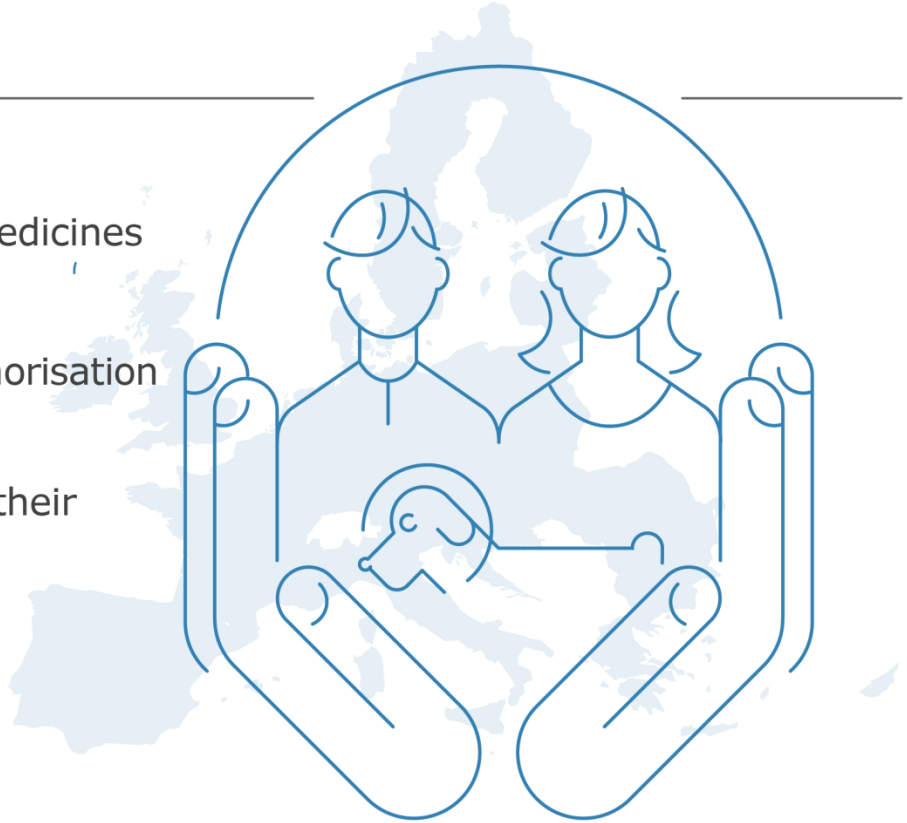
What EMA does?

Protect human and animal health

- Facilitate development and access to medicines
- Evaluate applications for marketing authorisation
- Monitor the safety of medicines across their life cycle
- Provide reliable information on human and veterinary medicines to patients and healthcare professionals



The European medicines regulatory network

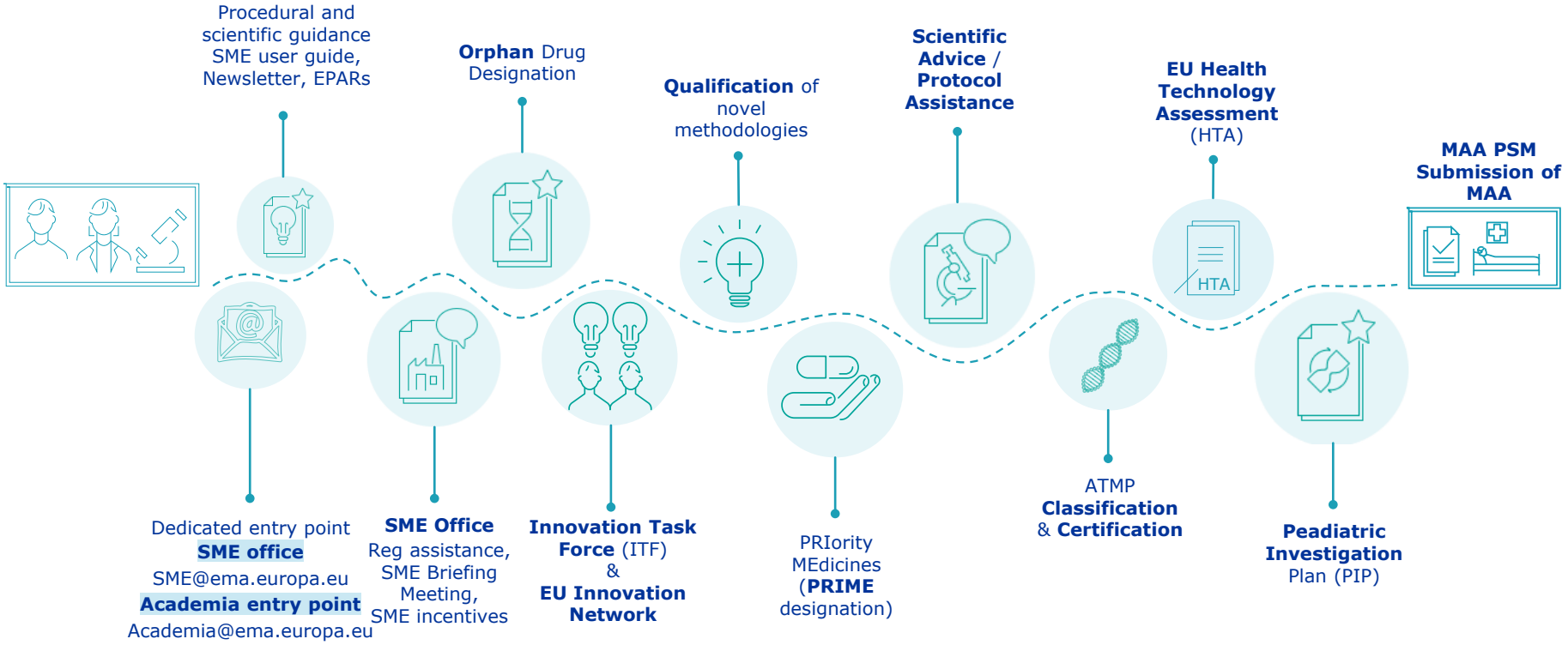
~50 national regulatory authorities

(27 Member States)

European Medicines Agency

European Commission

Support to innovation and opportunities to interact with EMA



Early development services

EMA has developed a consolidated list of available guidance and opportunities for interaction in the development phase of a medicinal product.

Research and development | European Medicines Agency (europa.eu)

Area	Guidance	Key points
Scientific advice for human medicines	Scientific Advice general information	Scientific advice is advice to a medicine developer on the appropriate tests and studies in the development of a medicine
	Guidance for applicants seeking scientific advice and protocol assistance	Protocol assistance is a special form of scientific advice, reserved for medicines with an orphan designation
	How to submit a request	Procedural advice for requestin scientific advice and protocol assistance
	Parallel EMA-United States (US) Food and Drug Administration (FDA) scientific advice	Parallel EMA-FDA scientific advice should focus primarily on important breakthrough drugs or important safety issues
	Parallel consultation with regulators and health technology assessment bodies	Provides feedback from regulators and HTA bodies at the same time, at any point in the developmental lifecycle of medicines.

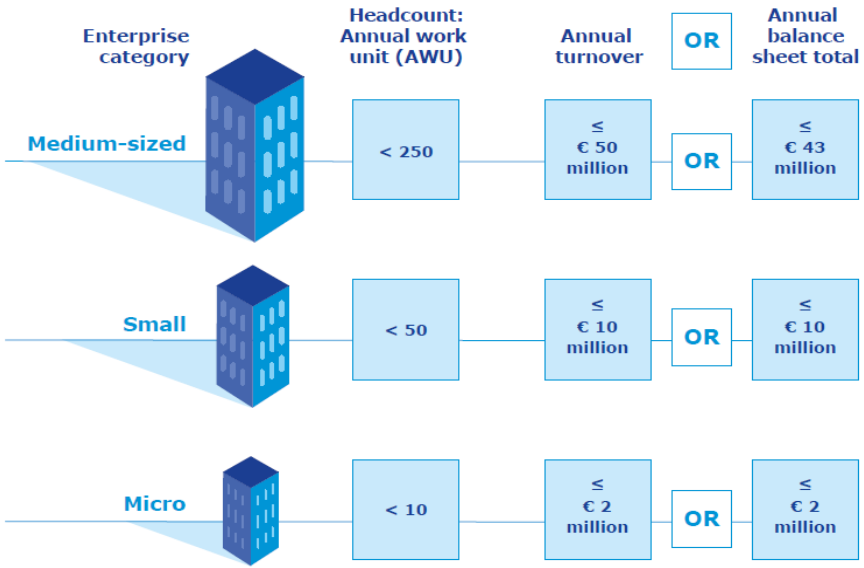
Assistance to SMEs

EU SME regulation(EC) No 2049/2005 of 15 December 2005

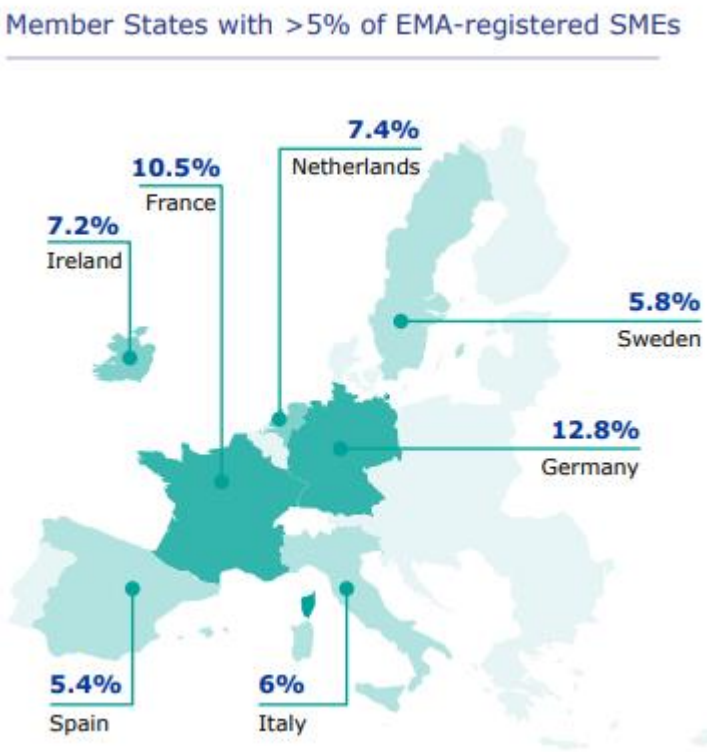
- Assignment and renewal of SME status
- Regulatory assistance
- SME briefing meetings
- Fee incentives
- Translation assistance for the product information
- Training and awareness via info days, SME newsletters and mailings targeted at SMEs
- Partnering and networking

SME Definition Commission recommendation 2003/361/EC

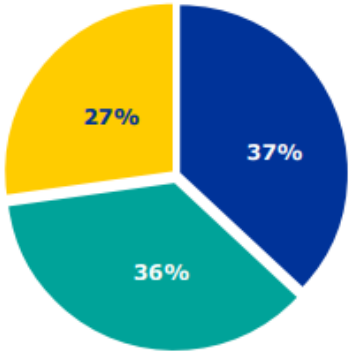
SME thresholds



SMEs registered with EMA (2021)



1780 SMEs registered with EMA at the end of 2021



Micro-sized
Headcount <10; annual turnover or balance sheet total ≤€2 million

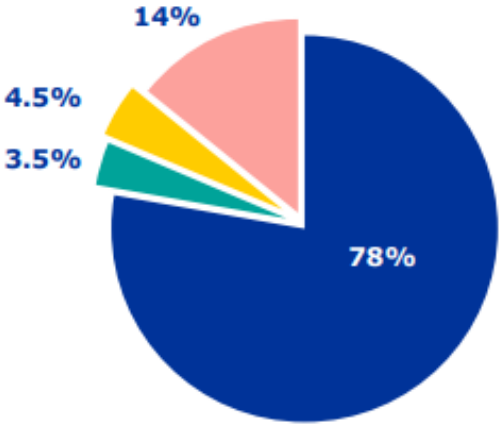
Small-sized
Headcount <50; annual turnover or balance sheet total ≤€10 million

Medium-sized
Headcount <250; annual turnover ≤€50 million or balance sheet total ≤€43 million

SMEs registered with EMA (2021)

Company activity

- Human medicines
- Veterinary medicines
- Human and veterinary medicines
- Service providers and regulatory consultancies



Profile

11% Academic spin-offs

13% SMEs incorporated over the last three years:
31% newly created entities
69% new subsidiaries

77% of companies declared activities in the pharmaceutical sector

20% in the pharmaceutical and medical devices sectors

3% in the medical devices sector

64% of companies operating in the pharmaceutical sector have products at development stage

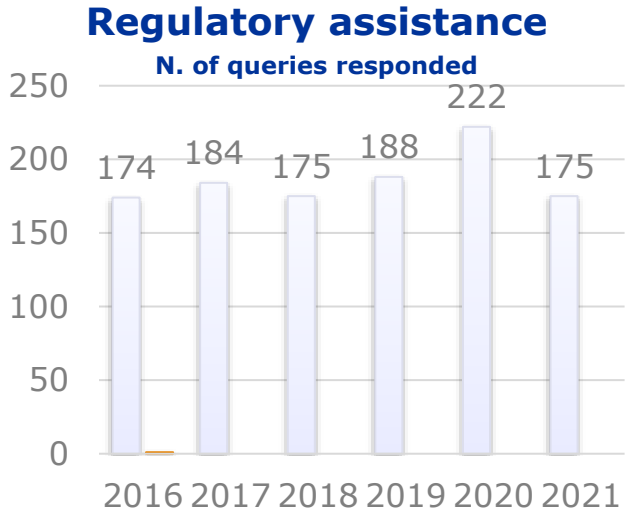
Regulatory assistance

SME@ema.europa.eu
SME Helpline :
+31 (0)88 781 8787

- Response by email or phone
- Interaction with other EMA offices
- Meetings → SME briefing meetings

Common topics

- SME definition & SME incentives
- Scientific advice /protocol assistance: how/when to apply
- PRIME designation: how/when to apply and eligibility
- Orphan designation and market exclusivity
- Paediatric requirements
- Regulatory topics (eligibility to centralised procedure, legal basis, data protection)
- Clinical trials (CTIS)



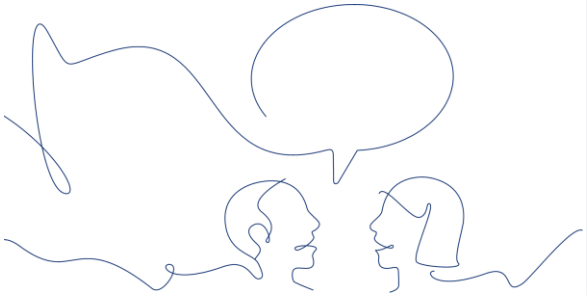
SME briefing meetings

- Platform for **early dialogue** with SMEs
- Discuss the **regulatory strategy** of a medicinal product development
- Navigate the range of procedures and incentives available
- Multidisciplinary EMA group
- Human and veterinary topics
- **Free of charge**

→ 5 SME briefing meetings in 2021

Most common Therapeutic indications

Oncology, rare diseases, anti-infectives and CNS.



Stage of development

Majority at early stage.

Topics covered

- Scientific advice / protocol assistance (quality, non-clinical, clinical) including procedural and types of advice available
- Regulatory and procedural aspects
- Access to SME incentives
- Orphan drug designation, Paediatric requirements
- PRIME eligibility
- Pre/post-licensing evidence generation

'Feedback from companies shows high level of satisfaction'

Fee incentives

Scientific advice / protocol assistance

- 90% fee reduction for SMEs
- Fee exemption for orphan designated products developed by SME or academia

Marketing authorisation application

- Fee deferral to 45 days after EC decision (positive/negative/withdrawn)
- Conditional fee exemption where EMA Scientific advice followed and dossier not successful
- Fee exemption for orphan designated products developed by SMEs
- **Translation of the product information at time of opinion**

Post-authorisation procedures

- Fee exemption for micro sized enterprise
- 40% fee reduction for small or medium-sized enterprises

Inspections

Pre-authorisation

- 90% fee reduction for SME + deferral to 45 days after EC decision
- Fee exemption for orphan designated products developed by SME

Post-authorisation

- 90% fee reduction for SMEs

Pharmacovigilance

- Fee exemption for micro-sized enterprises
- 40% fee reduction for small or medium-sized enterprises

MedDRA

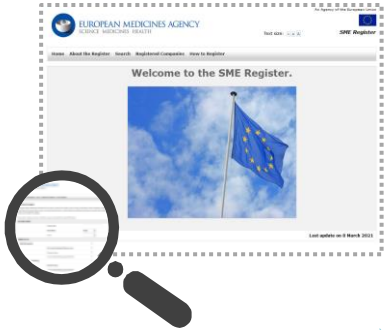
Fee exemption for micro and small-sized enterprises

Full detail on fee incentives is available here

Training, regulatory awareness and engaging

Training & awareness
Facilitating access to regulatory information

- SME user guide
- Info days
- SME newsletters
- Mailings / announcements



- SME Register

Increase information available on SMEs
Facilitate and promote interaction, partnering and networking between SMEs
Provide a source of information for EU institutions, agencies and Member States

Engage
with **EU** bodies and **industry stakeholders**

- Participation to conferences and events

Research and development - support to SMEs

SME annual report 2021

Innovation Task Force (ITF)

11 briefing meetings with ITF

10 on human medicines
1 on veterinary medicine

PRIME

3 out of **11 PRIME positive** eligibility recommendations from SMEs

Advanced therapies

26 recommendations for **advanced therapy classification**

The full report is available here

Scientific advice

19%

118 out of 611 requests from SMEs

Protocol assistance

36%

58 out of 163 requests from SMEs

Scientific advice for PRIME products

25%

15 out of 59 requests from SMEs

COVID-19

11%

9 out of 79 requests from SMEs

Qualification of novel methodologies

48%

12 out of 25 requests from SMEs

Veterinary scientific advice

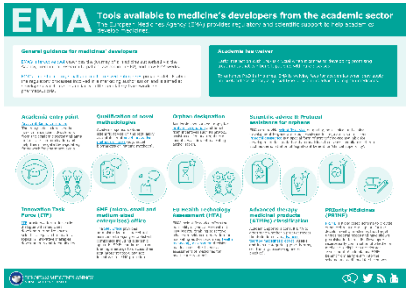
26%

6 out of 23 requests from SMEs

4. EMA engagement with academia

Framework for collaboration with academia since 2017. It aims to:

- Promote and further develop regulatory support for translating academic research into novel methodologies and medicines
- Ensure best scientific expertise and research is available
- Collaborate on areas of research on regulatory science



Dedicated entry point for Academia: Academia@ema.europa.eu

Financial incentives: protocol assistance free of charge to academic organisations developing orphan medicines

EMA pilot offers enhanced support to academic and non-profit developers of advanced therapy medicinal products

Regulatory Science Research Needs initiative: calling on stakeholders

- EMA seeks to stimulate researchers to consider addressing these needs
- EMA invites funders to support identified topics
- 110+ topics identified by EMA and EU network that need research into regulatory science
- List v1.0 published December 2021, can be updated with new topics

Orphan designation

- Orphan designation **(cumulative) criteria:**
  - **Prevalence**
    - Rare condition affecting less than 5 per 10,000 persons in the EU, or
    - [exceptionally] unlikely that the medicine would generate sufficient returns to justify the investment
  - **Seriousness of condition:** Life threatening and/or chronically debilitating condition
  - **Comparison to available treatments**
    - Unless no satisfactory methods of diagnosis, prevention or treatment exist
    - Otherwise need to demonstrate significant benefit vs. existing methods
- Assessment by COMP followed by EC decision on designation
- All designations included in Community Register of orphan medicinal products for human use (link).
- Incentives include 10 years **market exclusivity and fee incentives.**

https://www.ema.europa.eu/en/human-regulatory/research-development/orphan-designation-research-development

Paediatric requirements, rewards and incentives

- **Paediatric investigation plan** (PIP): **a development plan** to ensure necessary data are obtained in the conditions in which a medicinal product may be authorised to treat children.
  - Early paediatric interaction meetings.
  - Should be submitted around end of Phase I studies, start of Phase 2 POC
  - Outlines timing and measures (studies, formulations, etc) to be undertaken.
  - Deferral or waiver, if applicable.
- Rewards include extension of their supplementary protection certificate and additional 2 y of market exclusivity for orphans
- Incentives include scientific advice free of charge
- Submission and publication of study results conducted in children with authorised medicines by marketing authorisation holders.

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

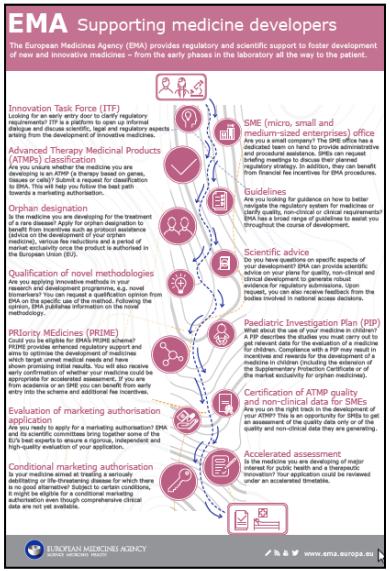
7. Advanced Therapy Medicinal Products (ATMPs)

ATMP classification

- Companies can consult EMA to confirm that their product is classified as an ATMP
- EMA's Committee for Advanced Therapies (CAT) delivers scientific recommendations on ATMP classification
- The outcome is published (summary of scientific recommendation)

ATMP certification - Specific incentive for SMEs

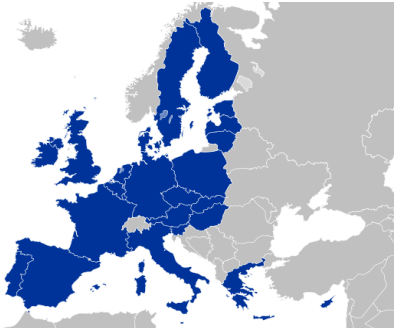
- Certification of quality and non-clinical data for ATMPs (human use)
- At any stage of the ATMP development process
- Aims to identify any potential issues early on, so that these can be addressed prior to the submission of a marketing-authorisation application



Evolution of EU clinical trials regulation

Pre 2004: No harmonisation

National rules, different processes in each Member State.
Resulted in **delays** and **complications**



Clinical Trials Directive
(EU 2001/20/EC)

Some harmonisation, but **national systems & processes varied**

Entered into application 1 May 2004

Clinical Trials Regulation
(No.536/2014)

Full harmonisation, collaborative assessment of **multinational trials**, single EU portal & database

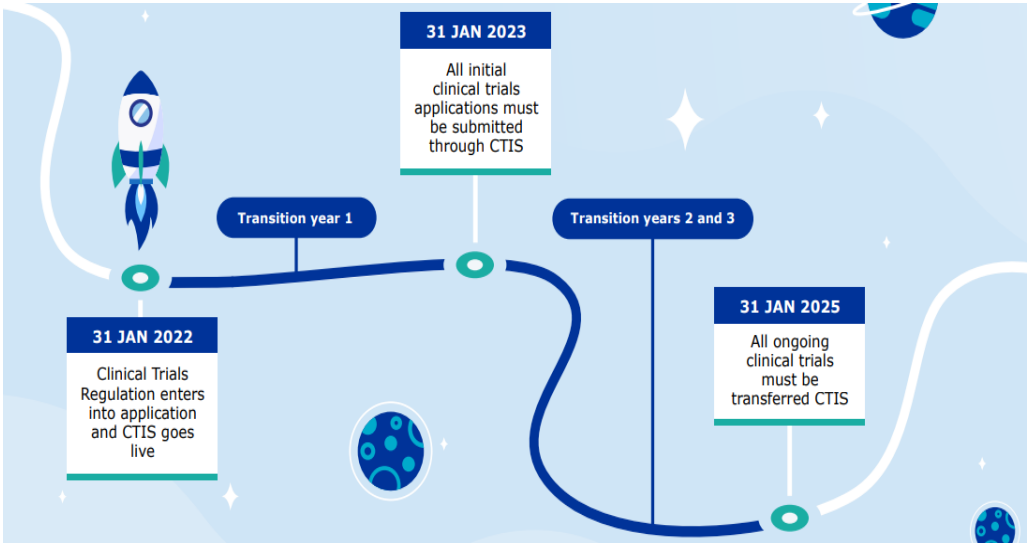
Applies as of **31 January 2022**

(3y transition period)

The Clinical Trials Information System (CTIS)

CTIS is the business tool of the Clinical Trials Regulation.

CTIS **harmonises the submission, assessment and supervision of clinical trials** in the EU/EEA.



Go to euclinicaltrials.eu to learn more and to access the CTIS secure Sponsor workspace

Take home messages

- EMA provides extensive guidance on support to innovation
- EMA offers several opportunities for interaction in the development phase of a medicinal product to support development of innovative medicinal products
- Academia and SMEs benefit from dedicated contact points

Thank you for your attention

Further information

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