EMA
EUROPEAN MEDICINES AGENCY

Achievements of EMA's SME Regulation

20th anniversary of the SME office

Presented by Leonor Enes on 17th October, 2025
SME Office | Regulatory Science and Innovation Task Force (TRS)

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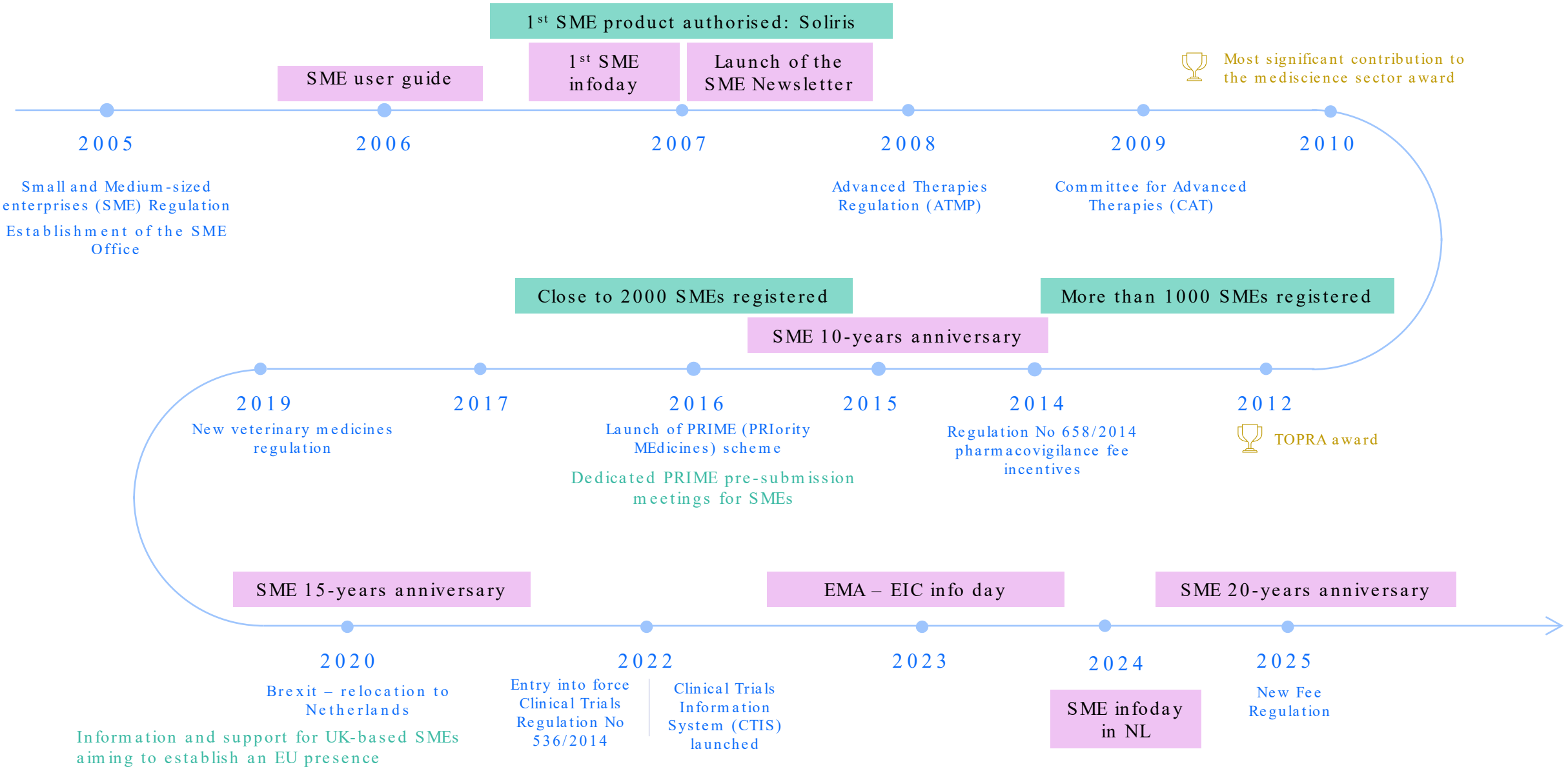
Implementation of the SME regulation

EMA


1st SME product authorised: Soliris
SME user guide
1st SME infoday
Launch of the SME Newsletter
Most significant contribution to the mediscience sector award
2005
2006
2007
2008
2009
2010
Small and Medium-sized enterprises (SME) Regulation
Establishment of the SME Office
Advanced Therapies Regulation (ATMP)
Committee for Advanced Therapies (CAT)
Close to 2000 SMEs registered
More than 1000 SMEs registered
SME 10-years anniversary
2019
2017
2016
2015
2014
2012
New veterinary medicines regulation
Launch of PRIME (PRIority MEdicines) scheme
Dedicated PRIME pre-submission meetings for SMEs
Regulation No 658/2014 pharmacovigilance fee incentives
TOPRA award
SME 15-years anniversary
EMA – EIC info day
SME 20-years anniversary
2020
2022
2023
2024
2025
Brexit – relocation to Netherlands
Information and support for UK-based SMEs aiming to establish an EU presence
Entry into force Clinical Trials Regulation No 536/2014
Clinical Trials Information System (CTIS) launched
SME infoday in NL
New Fee Regulation

SME office: dedicated SME support

Commission Regulation (EC) No 2049/2005

SME@ema.europa.eu

SME Helpline :+31 (0)88 781 8787

Support to SMEs | European Medicines Agency (EMA)

Assignment and renewal of SME status

Review of SME declaration

Regulatory assistance

Guidance & advice

SME briefing meetings

Early dialogue and discussion on a regulatory strategy for a product development

Fee incentives

e.g. Scientific advice, inspections

Translation of product information in all EU languages

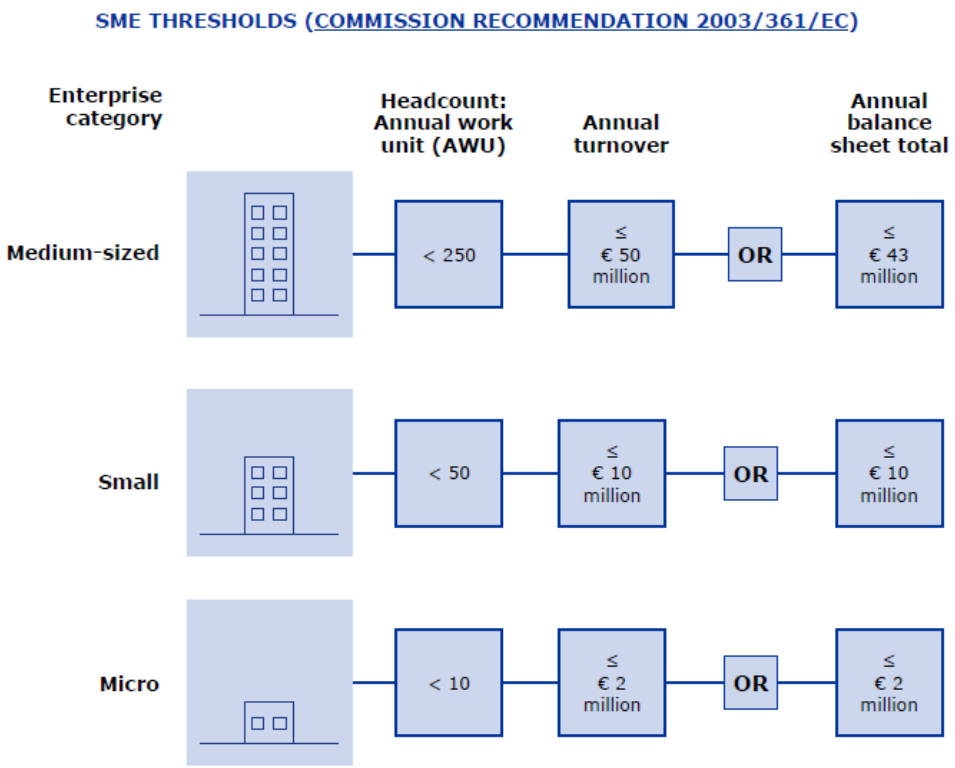
In all EU languages free of charge via EU Translation Centre, at time of marketing authorisation

Training and engagement

Info days, SME newsletters and mailings targeted at SMEs

SME definition

EU SME Definition – Commission recommendation 2003/361/EC



Established in EEA

How to apply?

sme@ema.europa.eu

- Declaration on the qualification of an enterprise as a micro, small or medium-sized enterprise (SME) - link
- Most recent annual accounts covering 12 months
- Information on ownership
- Proof of establishment in the EEA

Full assessment for new applications, simplified risk-based review of SME status renewals.

✓ Registered SME are listed in the SME Register

Regulatory assistance

Key topics

SME definition and incentives

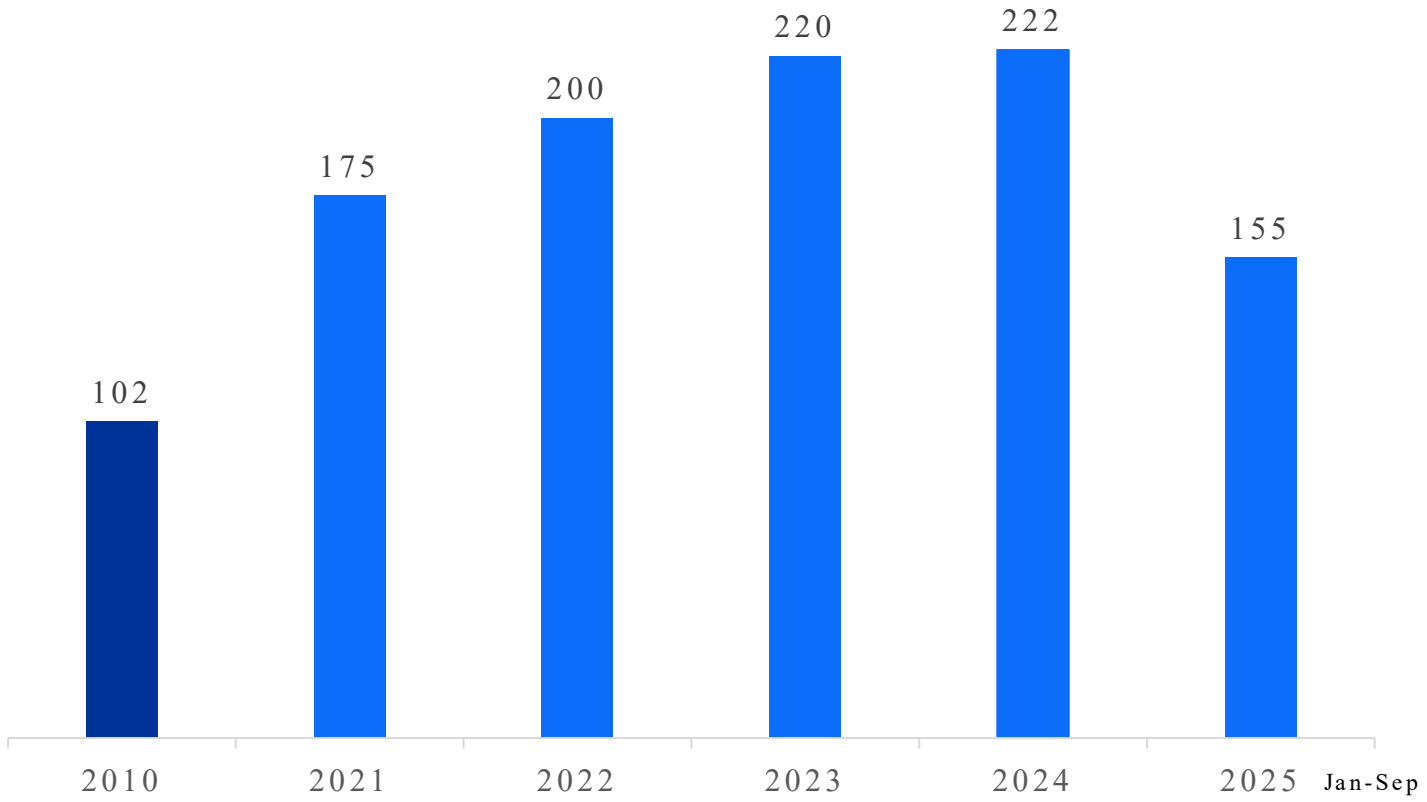
Early development advice (e.g. scientific advice, ITF, PRIME)

Regulatory topics (e.g. legal basis, data protection)

Criteria for orphan designation, market exclusivity

Paediatric requirements

Clinical trials, CTIS



Tools

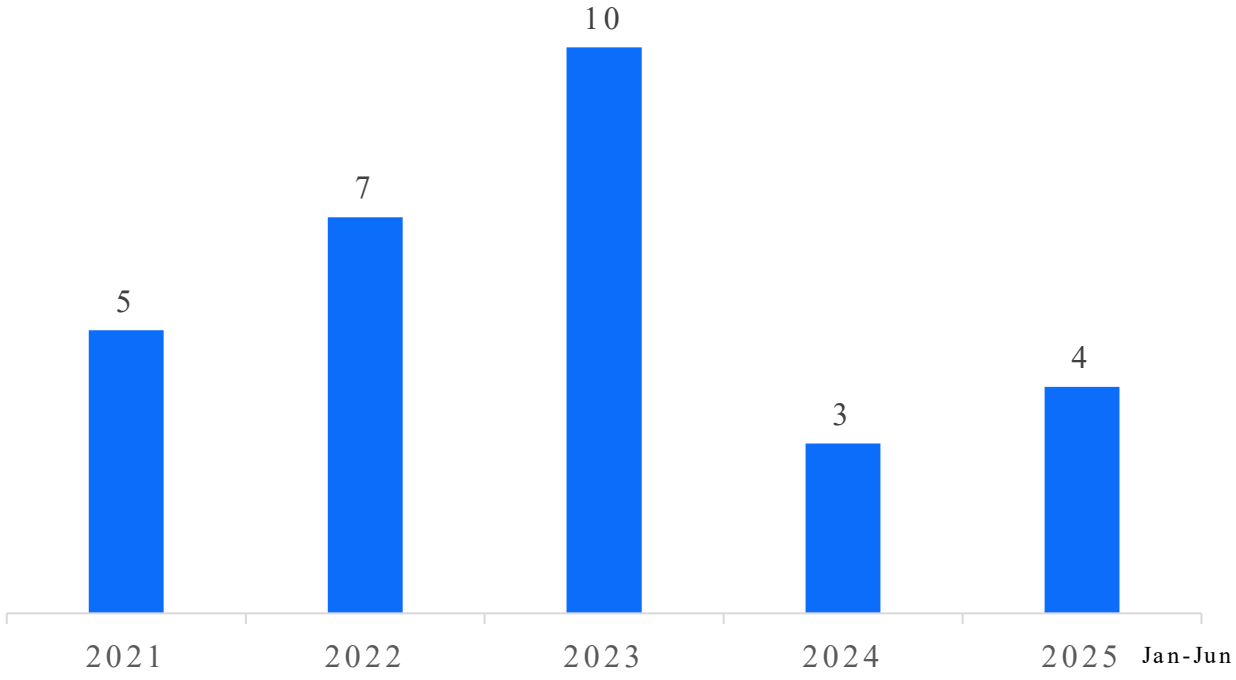
Via email SME@ema.europa.eu

Via SME helpline + 31 (0)88 781 8787

SME briefing meetings

- Discussion on a regulatory strategy for the development and authorisation of a medicinal product
- Multidisciplinary EMA team
- Free of charge

Topics: e.g Scientific Advice, Orphan Drug Designation, Pediatric investigation plan, PRIME.



Year	Average N. of meetings
2006-2010	5
2011-2015	9
2016-2020	13
2021-2024	6

Fee incentives for SMEs

Scientific advice :
90% fee reduction / 100% for designated orphan products

Marketing authorisation application :
Fee deferral until the outcome
100% fee reduction for designated orphan products
Conditional fee exemption

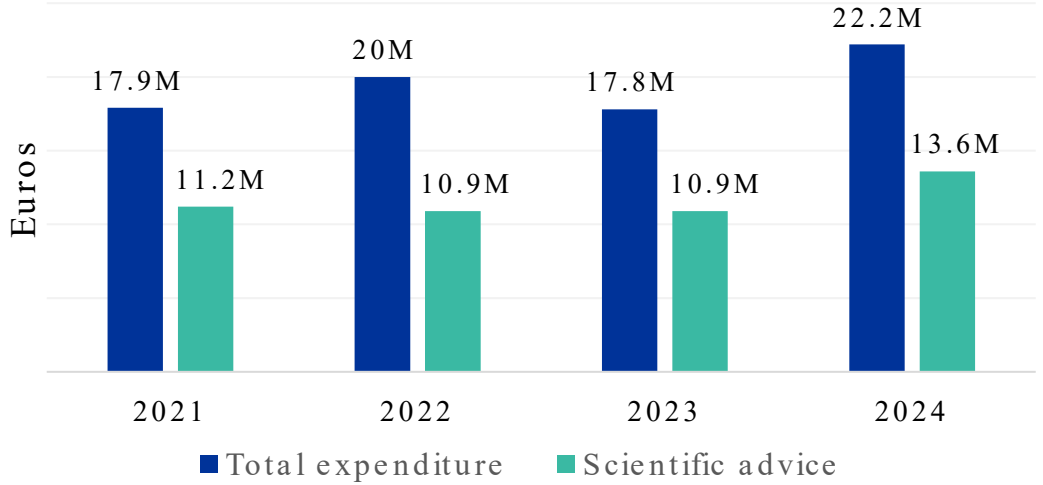
Inspections (pre-authorisation) :
90% fee reduction and deferral
100% fee reduction for designated orphan products

Inspections (post-authorisation) :
90% fee reduction
100% fee reduction for designated orphan products

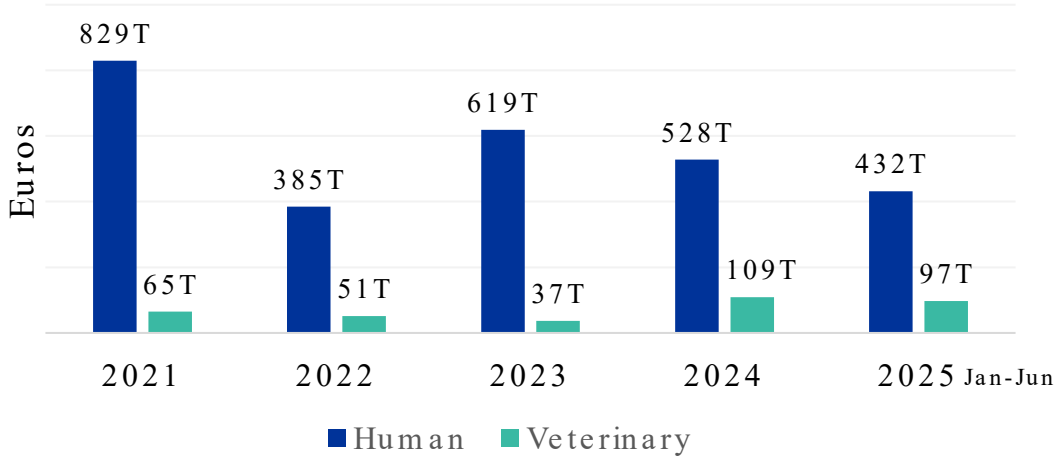
Pharmacovigilance :
100% fee reduction for micro-sized enterprises, 40% fee reduction for small or medium-sized enterprises

Post-authorisation procedures :
100% fee reduction for micro-sized enterprises, 40% fee reduction for small or medium-sized enterprises

SME Fee incentives (Millions)

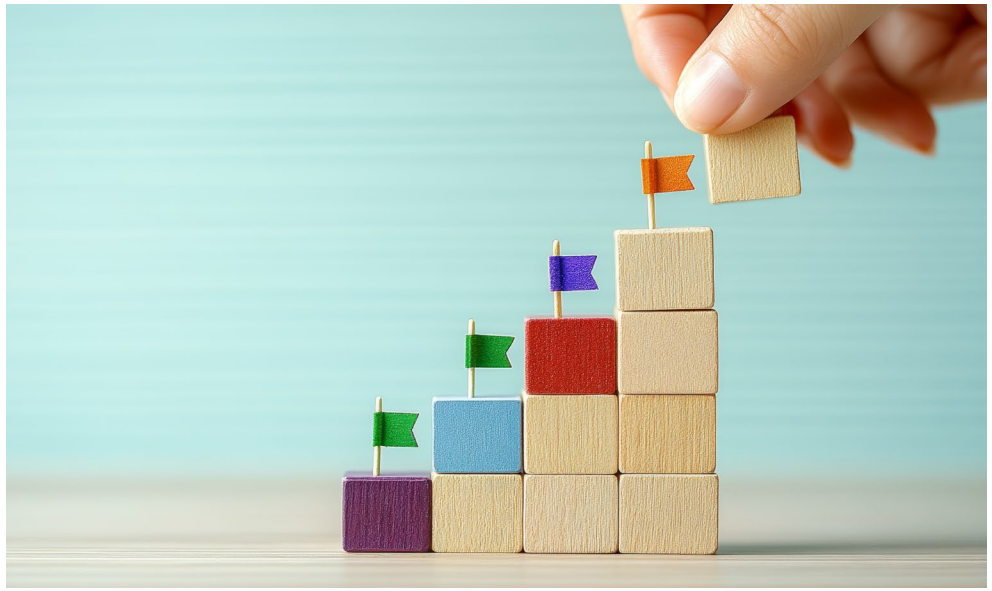


Translation assistance costs (Thousands)



Who are the registered SMEs?

Who are the ~2,000 SMEs registered?



2015

Field of activity

Large majority: Human use (76%)
Veterinary medicines: 4%
Consultancies: 15%

Category of enterprise

Micro: 42%
Small: 35%
Medium: 23%

Type of enterprise

Linked: 40%
Linked/partner: 6%
Partner: 3%
Autonomous: 51%

2024

Field of activity

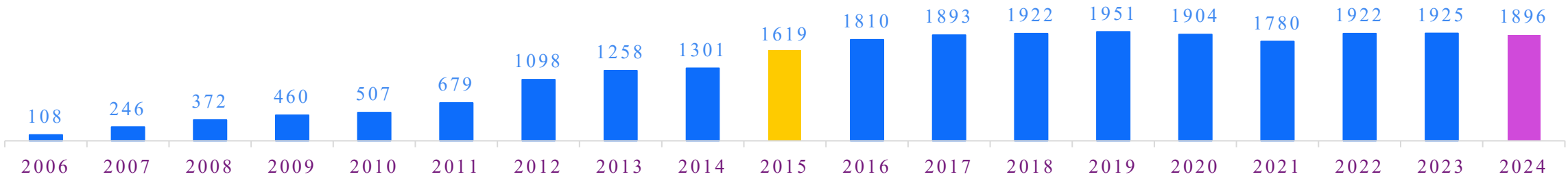
Large majority: Human use (78.6%)
Veterinary medicines: 4.2%
Consultancies: 13.1%

Category of enterprise

Micro: 36%
Small: 37%
Medium: 27%

Type of enterprise

Linked: 48%
Linked/partner: 6%
Partner: 3%
Autonomous: 43%



Who are the ~2,000 SMEs registered?

Location

12%

11%

8%

6%

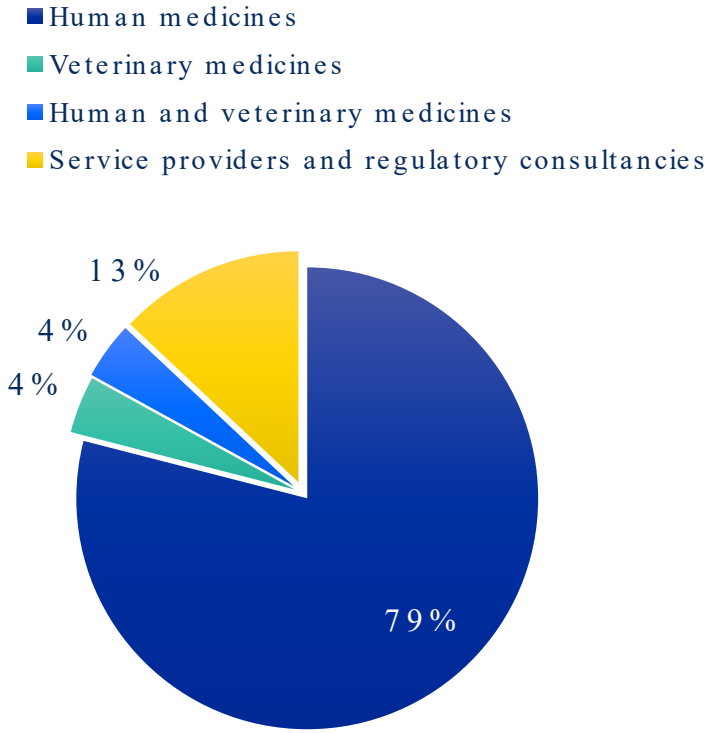
6%

6%

6%

9% non-EU entities associated to EU based SME regulatory consultancies

Company activity



Profile

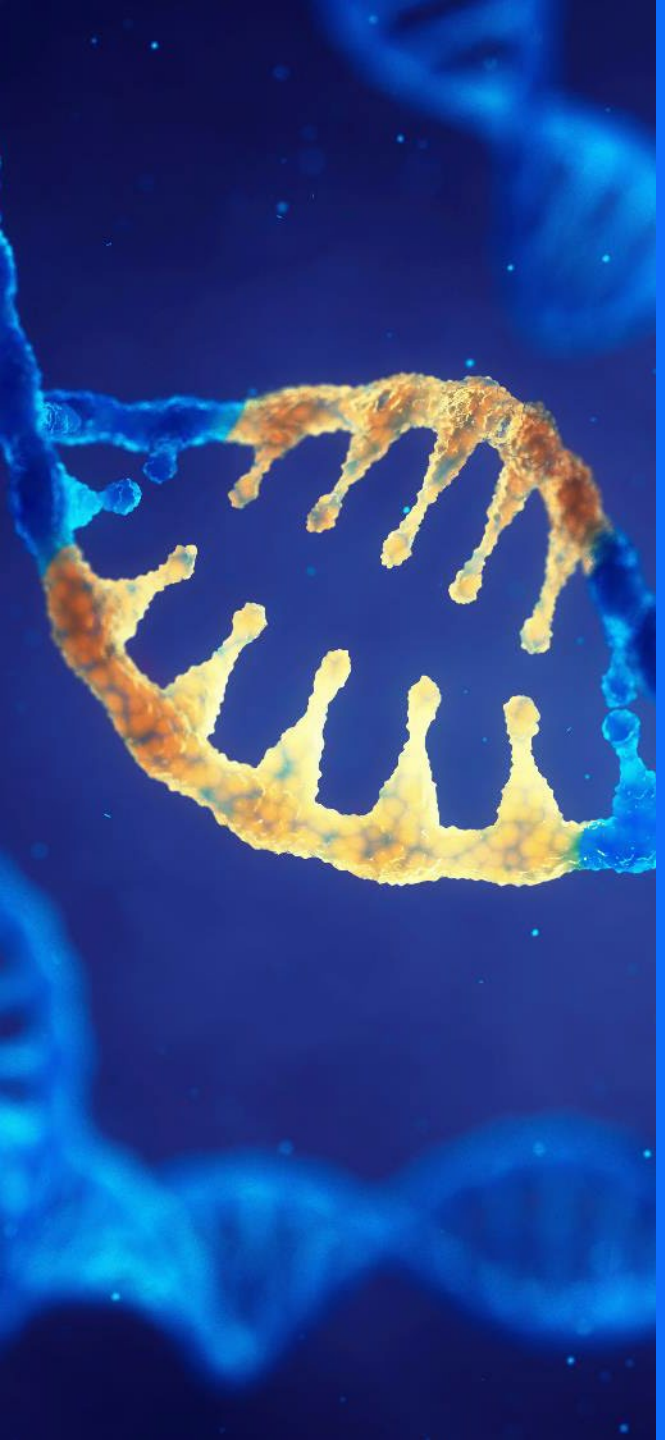
13% Academic spin-offs

80% of companies' activities in the pharmaceutical sector

17% in the pharmaceutical and medical devices sector

3% in the medical devices sector

59% of companies operating in the pharmaceutical sector have products at development stage



SMEs driving innovation in pharmaceuticals

PRIME – Priority Medicines

- Support for the development of medicines that target an unmet medical need.
- Enhanced interaction and early dialogue with developers of promising medicines, to optimise development plans and speed up evaluation so these medicines can reach patients earlier.

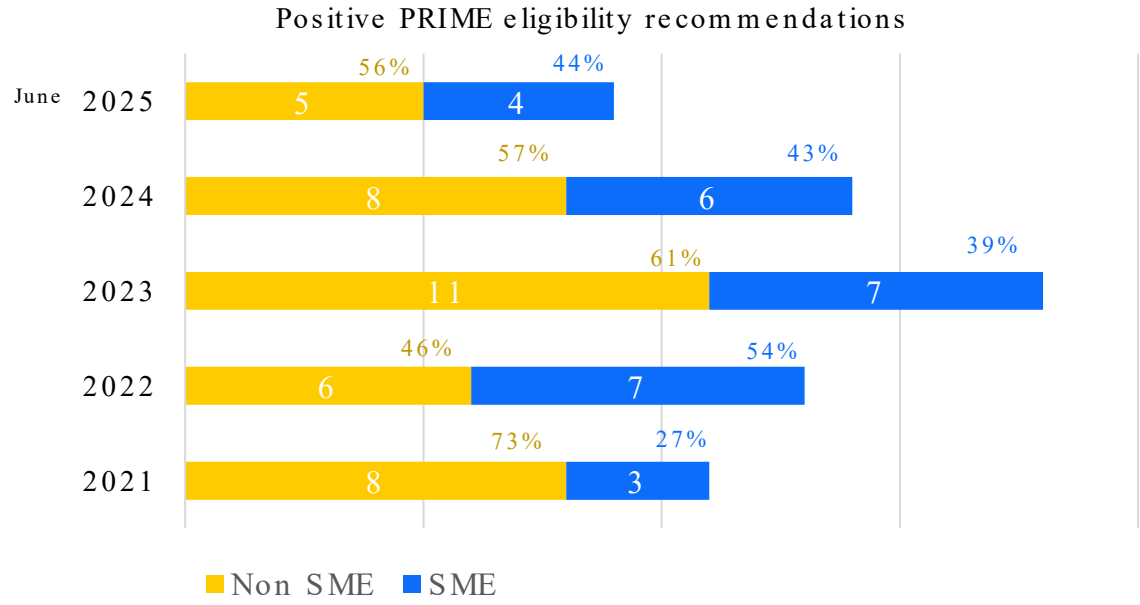
Jan 2021 - Jun 2025

23

out of 48 PRIME positive eligibility recommendations from SMEs

4

Human medicines authorisations under PRIME scheme



Innovation Task Force briefing meetings (ITF BMs)

What are ITF BMs?

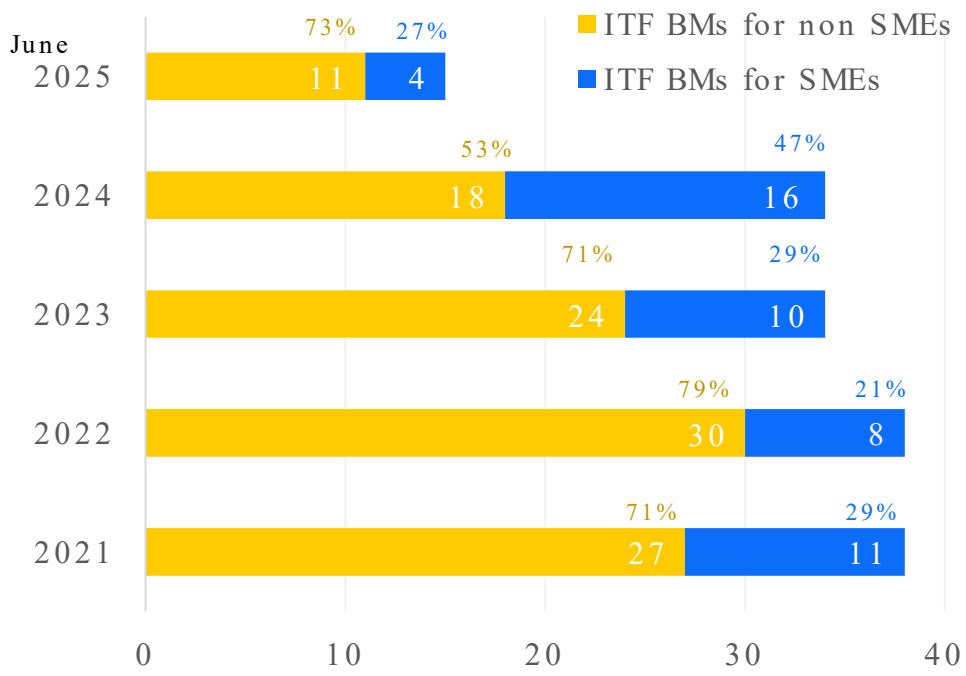
Early informal dialogue on innovative methods, technologies and medicines

1,5-hour discussion – free of charge

Involvement of EU network

Tools

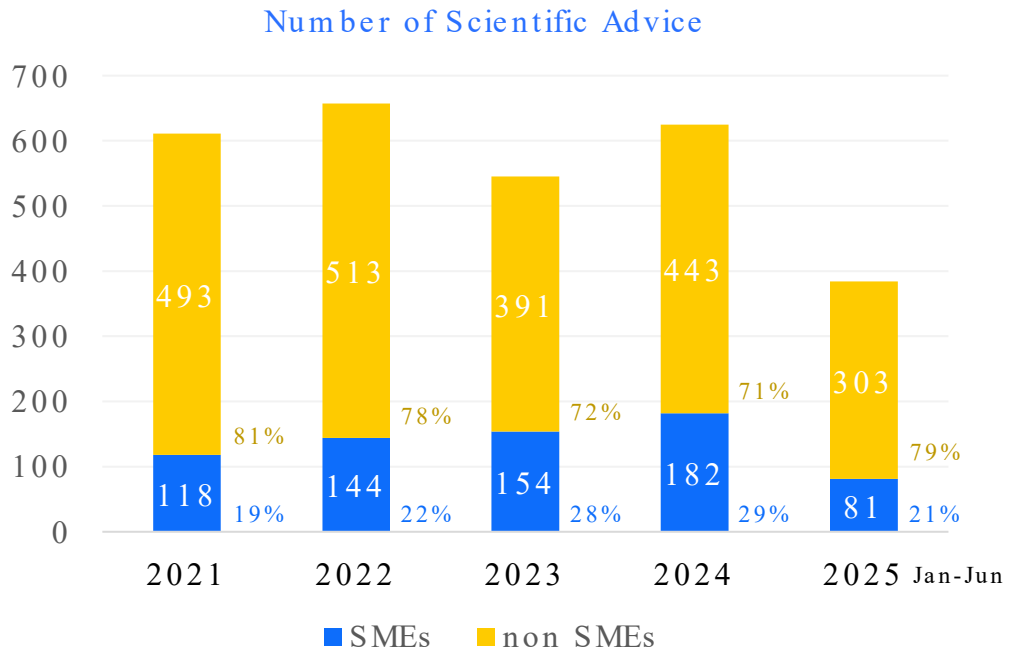
Send request form via email
ITFSecretariat@ema.europa.eu



Scientific advice/protocol assistance to SMEs

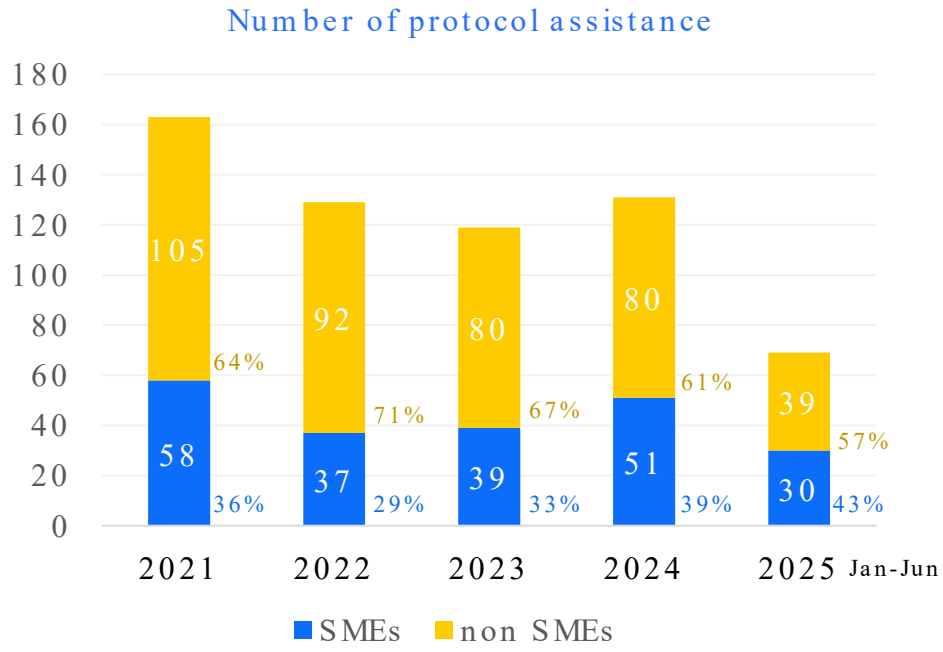
Scientific advice – Human medicines

Supports the timely and sound development of high-quality, effective and safe medicines, for the benefit of patients



Protocol assistance – Human Medicines

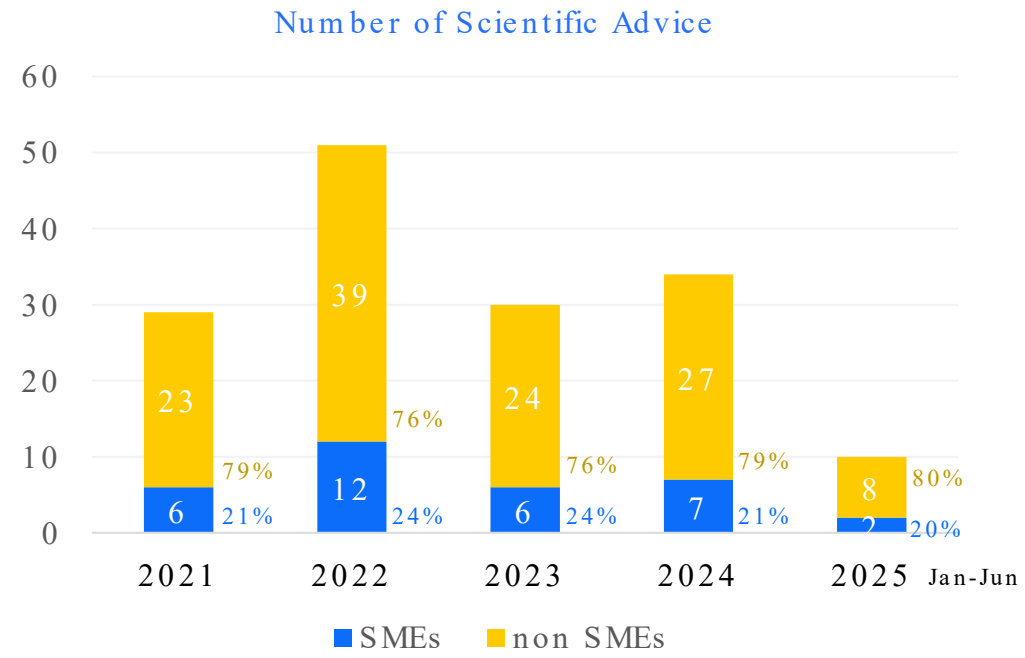
Special form of scientific advice available for developers of designated **orphan medicines** for rare diseases



Scientific advice/protocol assistance to SMEs

Scientific advice – Veterinary medicines

- Scientific advice to companies on the appropriate tests and studies in the development of veterinary medicines
- Facilitates the development and availability of high-quality, effective and acceptably safe medicines



Navigating the marketing authorisation landscape

2005-2025

> 140 approvals

For human medicines

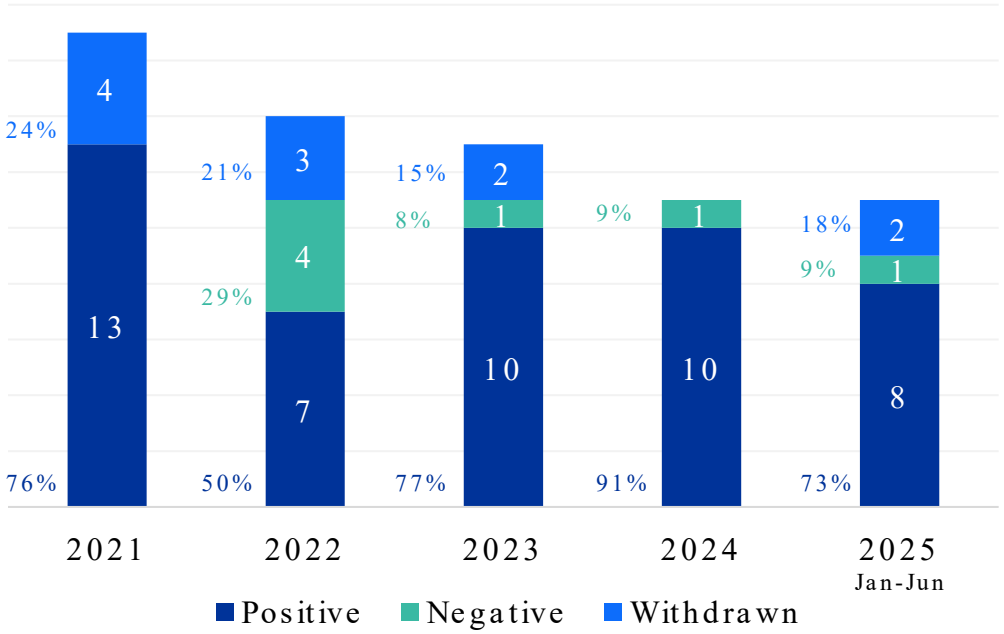
> 40 approvals

For veterinary medicines

H and V medicines marketing authorisations

Jan 2021 - Jun 2025

SMEs – MAA outcome per year (H and V)



2021-2025: 73% success rate

Approved Human medicines: (n=37)

41% (15)
New Active Substance

16% (6)
Accelerated assessment

11% (4)
under PRIME scheme

35% (13)
MA with Orphan Drug Designation

Approved Veterinary medicines: (n=11)

36% (4)
MUMS
minor use / minor species

45% (5)
New Active Substance

Product out-licensing / Mergers & Acquisitions

Analysis of a total of 56 human and veterinary MAAs from SMEs with a positive opinion between 2021 to June 2025

56
MAAs with SME as applicant

MAA submission

8 (14%) of MAs applicants lost SME status or related incentives

48
SME status maintained

Marketing authorisation

23 (48%) of MAs holders lost SMEs status or related incentives

25
SME status maintained

June 2025

As of June 2025, out of 56 MAAs:

- 45 % (25) of MAs holders with maintained SME status
- 55 % (31) of MAs applicants/holders lost SME status/incentives (e.g merger/acquisition, growth above thresholds, product out-licensing to a non-SME)

Selection of products developed by SMEs

Bylvay (H)

May 2021

First treatment for progressive familial intrahepatic cholestasis (PFIC) in patients aged 6 months or older

Orphan
PRIME

SME at MA

Imcivree (H)

May 2021

Treatment of obesity and the control of hunger associated with genetic deficiencies of the melanocortin 4 receptor (MC4R)

Orphan
PRIME

SME at MA

Strangvac (V)

June 2021

Vaccine given to horses from 8 months of age to reduce clinical signs of the acute stage of strangles

SME

Zokinvy (H)

May 2022

First treatment for children with progeroid syndromes, an ultra-rare genetic disease which causes premature aging and death

Orphan

SME at MA

Pedmarqsi (H)

March 2023

Prevention of ototoxicity induced by cisplatin chemotherapy in children from one month up to 18 years of age with localised, non-metastatic, solid tumours

SME at MA

Loargys (H)

October 2023

Treatment of hyperargininaemia

Orphan

SME

Aqumeldi (H)

October 2023

Treatment of heart failure in children from birth to less than 18 years

SME at MA

EURneffy (H)

June 2024

First nasal spray emergency treatment against allergic reactions, providing an alternative to traditional injections

SME at MA

Emcitate (H)

December 2024

First treatment for peripheral thyrotoxicosis in patients with Allan-Herndon Dudley syndrome

Orphan

SME

Biobhyo (V)

June 2025

First vaccine against swine dysentery disease authorised in Europe

SME

Zemcelpro (H)

June 2025

Treatment of adult patients with haematological malignancies requiring an allogeneic haematopoietic stem cell transplantation following myeloablative conditioning for whom no other type of suitable donor cells is available

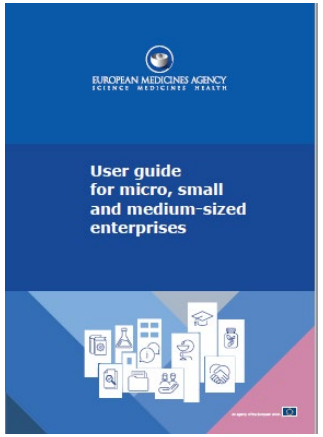
Orphan
ATMP
PRIME

SME

Empowering through training and engagement

Training & education

SME user guide



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The past 5 years:

SME Info Day on VETS
28.10.2021
EMA support for innovative veterinary medicines, vet regulation, PV, UPD

SME and academia webinar on CTR - CTIS
29.11.2021
CTR, CTIS functionalities

EMA-EIC Info day
31.01.2023
EMA support to innovation (PRIME, SA, ITF)

Awareness session for SMEs new pharma legislation
24.11.2023
Pharma package, impact on SMEs, Q&A

SME Info day
18.10.2024
Clinical trials, HTA, Medical devices, NFR, shortages

Training and engagement with stakeholders

Conferences and lectures to raise awareness on EMA support to innovation

IBE Leiden certificate program

Karolinska Institutet Master in Bioentrepreneurship in Stockholm

TOPRA annual symposium

Start-me up 2025, Pasteur Insitut

AREIPS Master, faculty of Pharmacy

Bio International Convention

Basel University, European center of Pharmaceutical Medicines

Collaboration with EU institutions

European Investment Bank

Health innovation

European Innovation Council

EIC - EMA cooperation deliverables

European Commission

HaDEA Horizon Europe – Training to beneficiaries

Mutual exchange and support on SME definition

REA

Sharing Experience on SME Support

efsa EUROPEAN FOOD SAFETY AUTHORITY

Sharing Experience on SME Support

ECHA EUROPEAN CHEMICALS AGENCY

enterprise europe network

EEN Annual Conference, meeting of the Friends of the EEN

European Commission

DG Grow / DG Sante
Mutual exchange and support on SME definition
EU Biotech and biomanufacturing Hub

EMA

The SME Regulation has proven to be a powerful driver of innovation, growth, and collaboration across the European pharmaceutical landscape

EMA remains strongly committed to supporting SMEs throughout their journey

Think small first!

EMA

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Thank you

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Special thanks to Eglantine Legros for preparing the slide deck

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