European Medicines Agency support to SMEs and Academia

Workshop on Support for Orphan Medicines Development

Presented by Hélène Casaert on 30 November 2020
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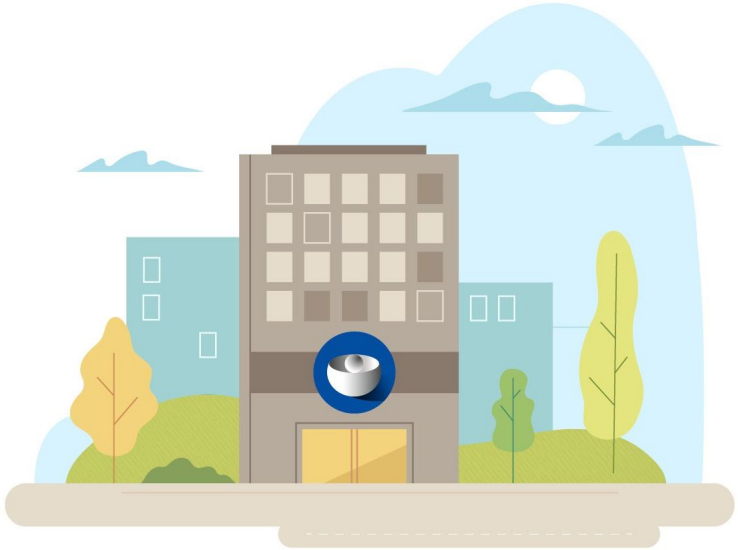
Disclaimer

The presenter does not have any conflict of interests.

Understand how EMA can support SMEs and Academia

1. Specific support for SMEs and academia
2. EMA support to innovation
3. Tips and recommendations



Specific support for SMEs and academia

EMA SME Office

SMEs are an important source of innovation

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

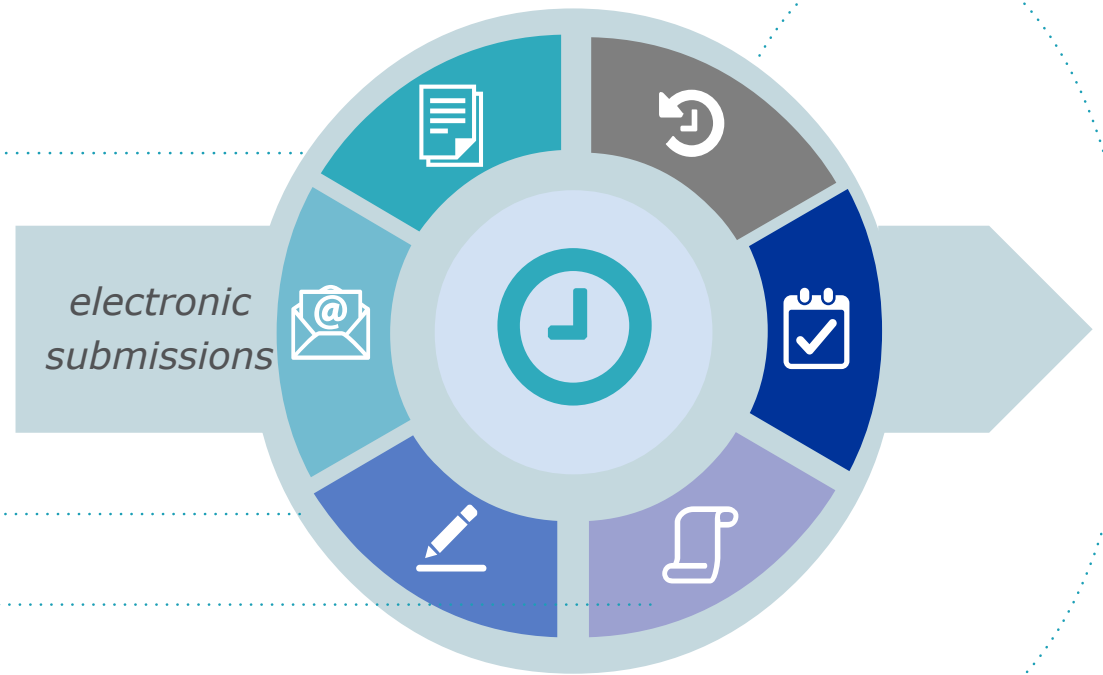
Aims to promote innovation and development of new medicines by SMEs

EMA SME Office launch in December 2005

- Dedicated contact point
- Assistance to SMEs
  Regulatory, administrative and procedural support.
- Facilitates communication with SMEs
- Engage with EU bodies and industry stakeholders

Assignment of SME status

1. Apply for EMA SME status
2. Review by SME Office
3. SME qualification
4. Renewal



Assignment of SME status

1866 registered SME at 17/11/2020

23% of orphan developers

EU SME Definition - Commission recommendation 2003/361/EC

SME thresholds

Size and ownership

Regulatory assistance

Administrative, regulatory and procedural queries from SMEs
SME@ema.europa.eu
SME Helpline :
+31 (0)88 781 8787

Response by email or phone

Interaction with other EMA offices

Meetings

Dedicated entry point for Academia

Academia@ema.europa.eu

Example of topics addressed

- SME definition and incentives
- Scientific advice /protocol assistance: how/when to apply
- Orphan designation/PRIME: how/when to apply
- Criteria for orphan designation, market exclusivity
- Regulatory topics (e.g. eligibility to centralised procedure, legal basis)
- Paediatric requirements

SME briefing meetings

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

10 SME briefing meetings in 2020

4 covering orphan aspects

Topics covered

- Scientific advice / protocol assistance
- Regulatory and procedural aspects
- Orphan drug designation
- Paediatric requirements
- PRIME eligibility

Therapeutic indications

Oncology, rare diseases, anti-infectives and CNS.

Stage of development

Majority at early stage.

Focus on fee incentives

Scientific advice / protocol assistance

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

Marketing authorisation application

Fee exemption for orphan designated products developed by SME

Post-authorisation procedures

Fee exemption for designated orphan products during the first year after marketing authorisation for SME

Inspection (pre-authorisation)

Fee exemption for orphan designated products developed by SME

Inspection (post-authorisation)

90% fee reduction for SME

MedDRA

Fee exemption for micro and small-sized enterprises

Pharmaco vigilance

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

Full detail on fee incentives is available here

Other SME incentives

Training & awareness
Facilitating access to regulatory information

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

Engage
with EU bodies and industry stakeholders

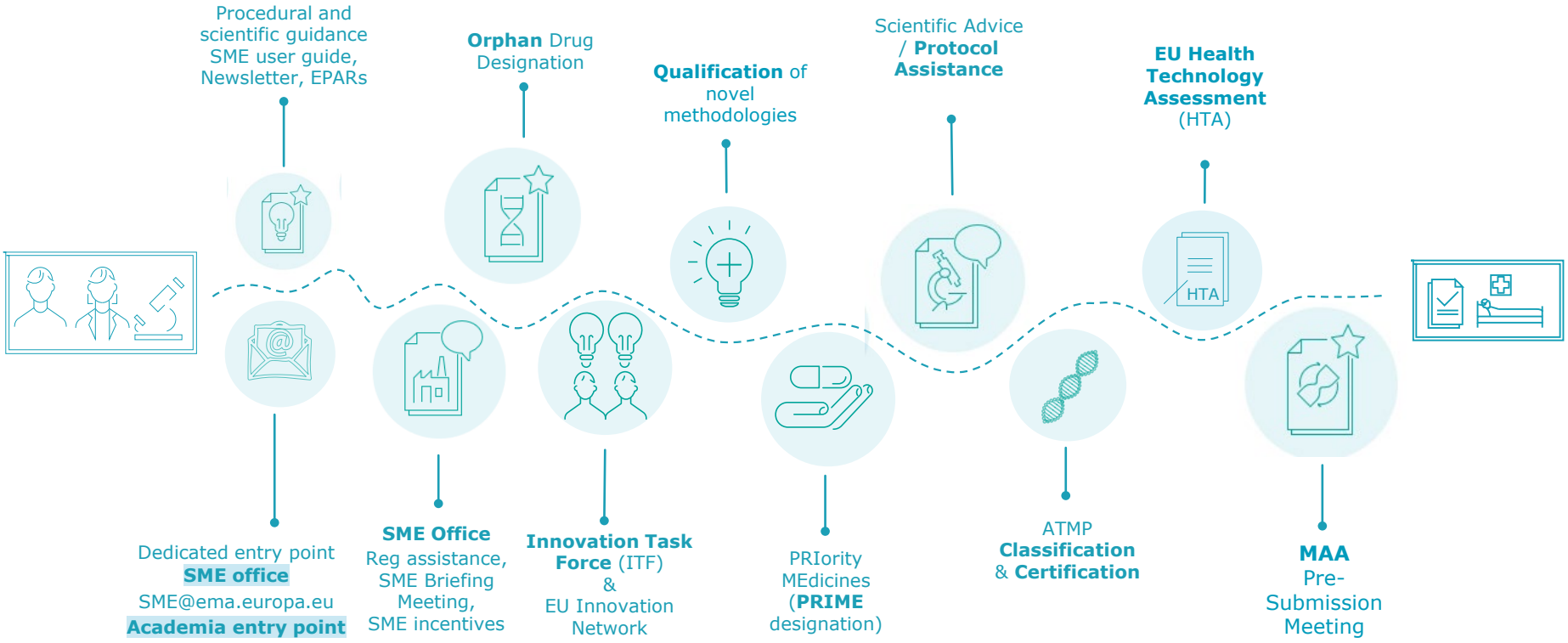
- SME Register
- Participation to conferences and events

Translation assistance

- Translation of product information
- for the initial marketing authorisation
- into all EU official languages and free of charge

EMA support to innovation

Support to innovation and opportunities to interact with EMA



Early support to innovation

Innovation task force (ITF)

- Multidisciplinary group, works in co-operation with EMA's working parties
- Forum for early dialogue with applicants on innovative aspects of medicine development
- Scientific, legal and regulatory aspects of emerging therapies, novel technologies and borderline products

EU Innovation Network

- Strengthened cooperation between Innovation Offices and EMA
- Make the regulatory support for medicines developers that is available at national and EU levels more visible and attractive to innovators

In 2019 :
29 ITF briefing meetings for human products

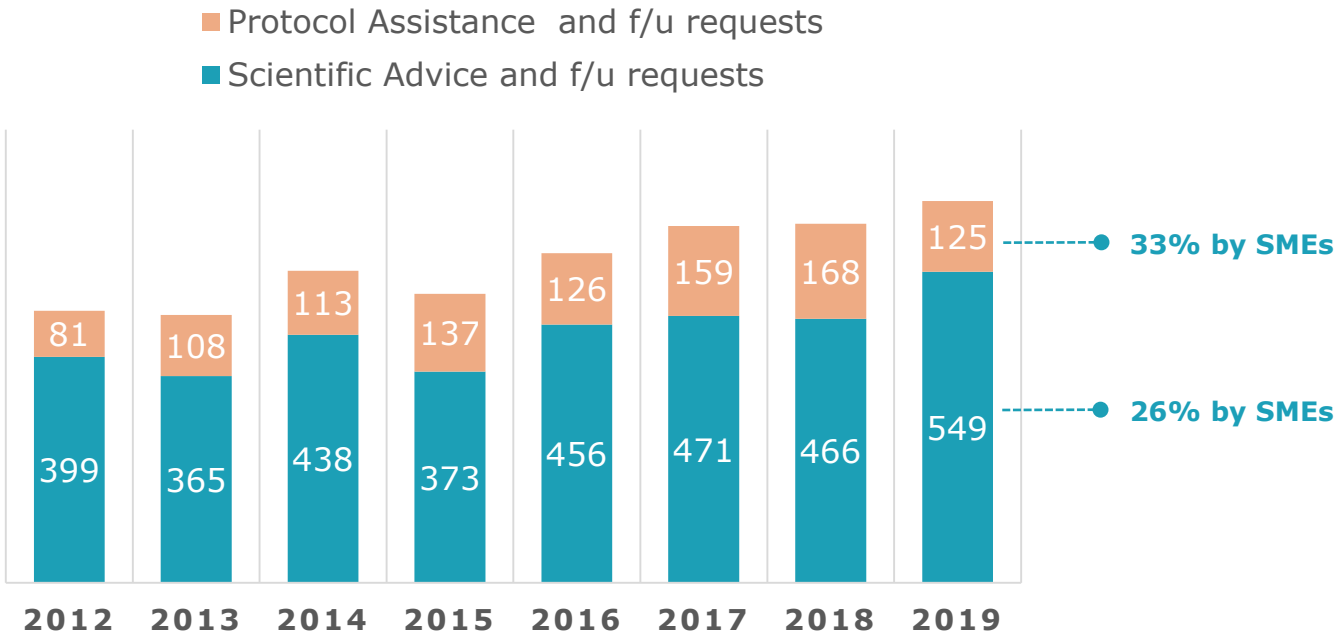
13 for SME

8 for academia

72% of ITF briefing meetings were for SMEs/academia

Scientific advice for designated orphan medicinal products = protocol assistance

Human medicinal Products



PRIME scheme

- For **new human medicines with major public health interest**
- Foster development, facilitate timely access to patients
- **Early entry point for SMEs and academia**:
  - at the proof of principle stage
  - on the basis of compelling non-clinical data and tolerability data from initial clinical trials.

PRIME designation per November 2020

SMEs

- High interest
- Almost 50% of products in the PRIME scheme are from SME
- Few SMEs granted early entry into the PRIME scheme
- SME success rate of applications for PRIME: 20 %
- 63% orphan medicines, 43% ATMPs
- Therapeutic areas : oncology/haematology, neurology, infectious diseases

Academia

- 4 requests received from Academia since launch
- No PRIME eligibility granted

Tips and recommendations

Tips and recommendations for SMEs and academia

Look for guidance

- European Public Assessment Reports are useful source of information.
- Consult available guidance (procedural and scientific) and SME User Guide.

Specific support

- Qualify as SME to benefit from SME incentives.
- Specific fee incentives for SMEs and academia, and orphan designated products
- Regulatory assistance to SMEs and Academia, including SME briefing meeting

Come early and come often

- Make use of available support (ITF, EU innovation network, PRIME scheme, scientific advice…)
- Importance of early regulatory and scientific advice to minimising the most frequent hurdles, especially on quality aspects.

Thank you for your attention

Any questions ?

Further information

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