EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

# EMA Roundtable meeting

10-year anniversary of the SME Office

Presented by Constantinos Ziogas on 27 November 2015
Head of SME Office (Ad Interim), Corporate Stakeholders Department,
Stakeholders and Communication Division

An agency of the European Union

# Agenda

1 EU SME regulation

2 SME Office remit

3 What the SME Office does

4 Evolution and support to SMEs

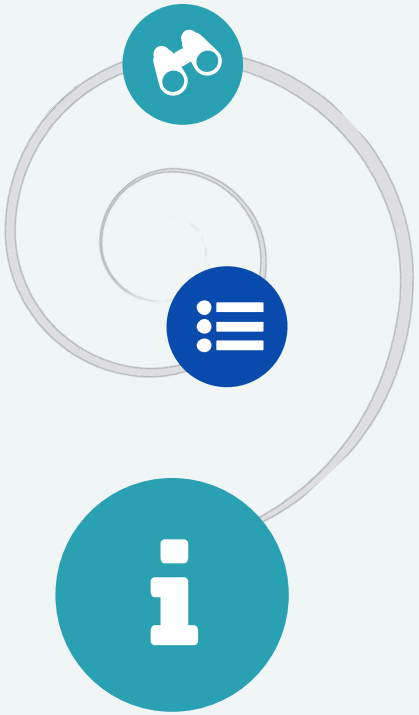
5 A profile of registered SMEs

6 Marketing authorisations

7 Looking ahead

10-year anniversary of the SME Office

# *EU SME regulation*



## Scope

*This Regulation applies to SMEs established in the Community.*
*This Regulation applies to applications concerning medicinal products for human use & veterinary medicinal products.*

## Subject matter

*Establishes the circumstances in which SMEs may pay reduced fees, defer payment of fees, or receive administrative assistance when submitting to the EMA.*

## Submission of information

*An SME wishing to benefit from the provisions of this Regulation shall submit to the Agency the information necessary to demonstrate compliance with the criteria mentioned in Article 2(1).*

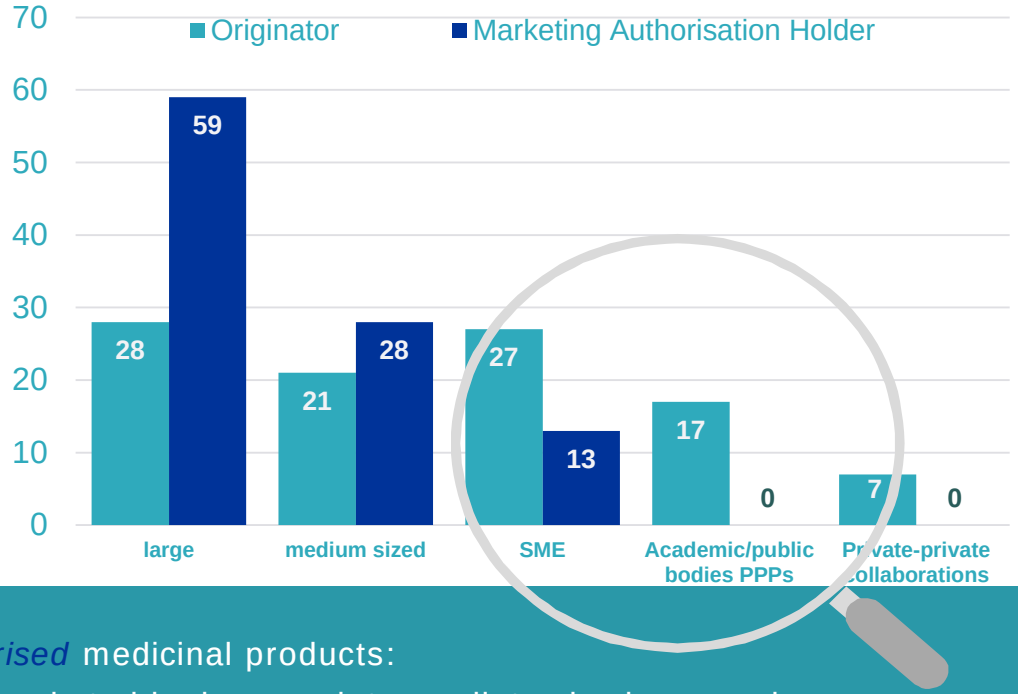
COMMISSION REGULATION (EC) No 2049/2005 of *15 December 2005*

> *The Executive Director of the Agency shall set up dedicated administrative structures and specific procedures for the establishment of an SME Office.*

# Origins of new medicines

## *EU 2010-2012*


*




*

Of 94 novel *authorised* medicinal products:

- Large majority marketed by large or intermediate sized companies.
- SMEs and academia at the origin of innovation.

10-year anniversary of the SME Office

# *SME Office remit*

## SME Office launch
*December 2005.*

## A single interface
*“One-stop-shop.”*

## Assistance to SMEs
*Regulatory, administrative and procedural support.*

## Facilitates communication
*Of SMEs in veterinary and human pharma sector.*

## Coordinating & networking
*Working closely with EU.*

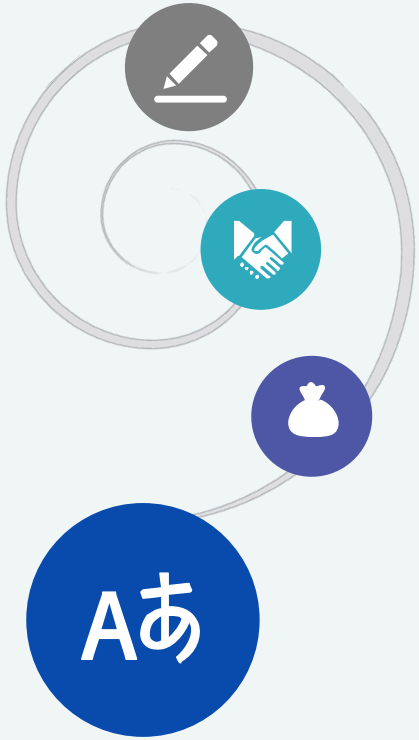
SME Office
tailoring assistance to SMEs

*“A strategic regulatory toolbox to promote innovation and development of new medicines by SMEs.”*

10-year anniversary of the SME Office

# *What the SME Office does*

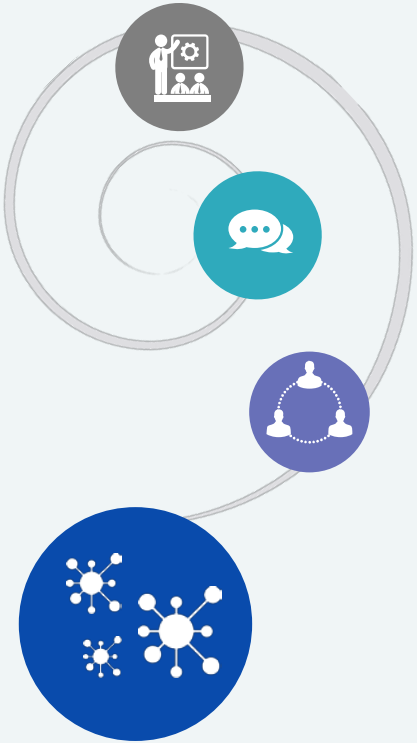
1. Validation
2. Regulatory Assistance
3. Fee Incentives
4. Translation Assistance



5 Training and Awareness

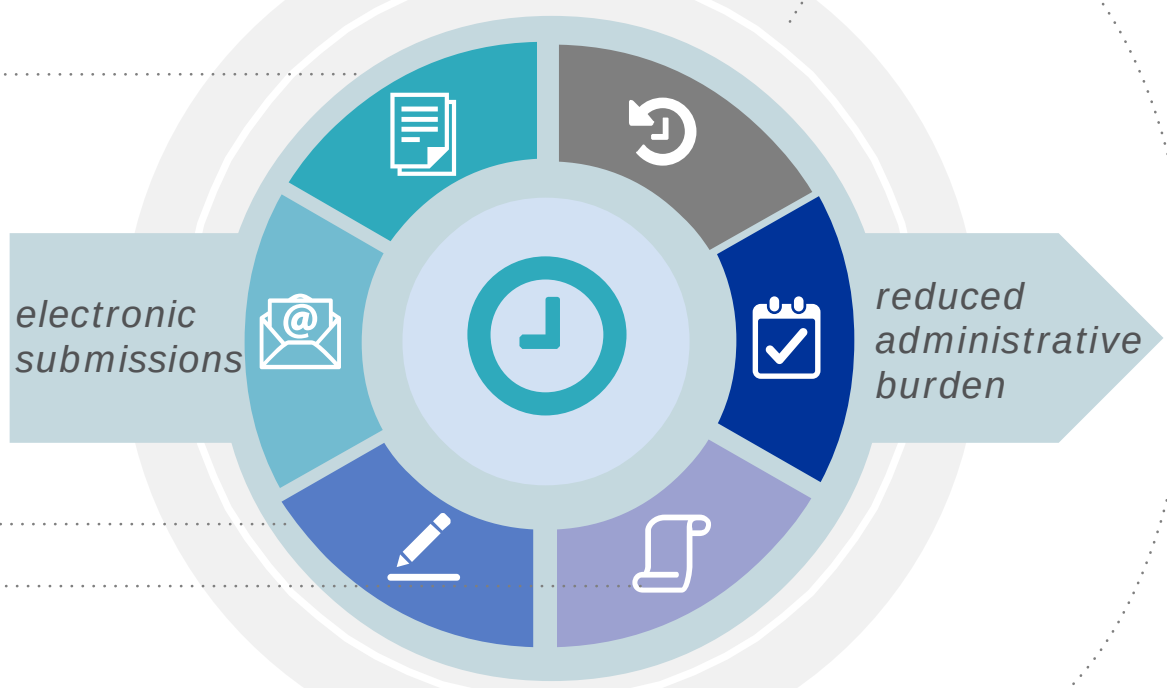
6 Partnering & Networking

7 Support to Advanced Therapies



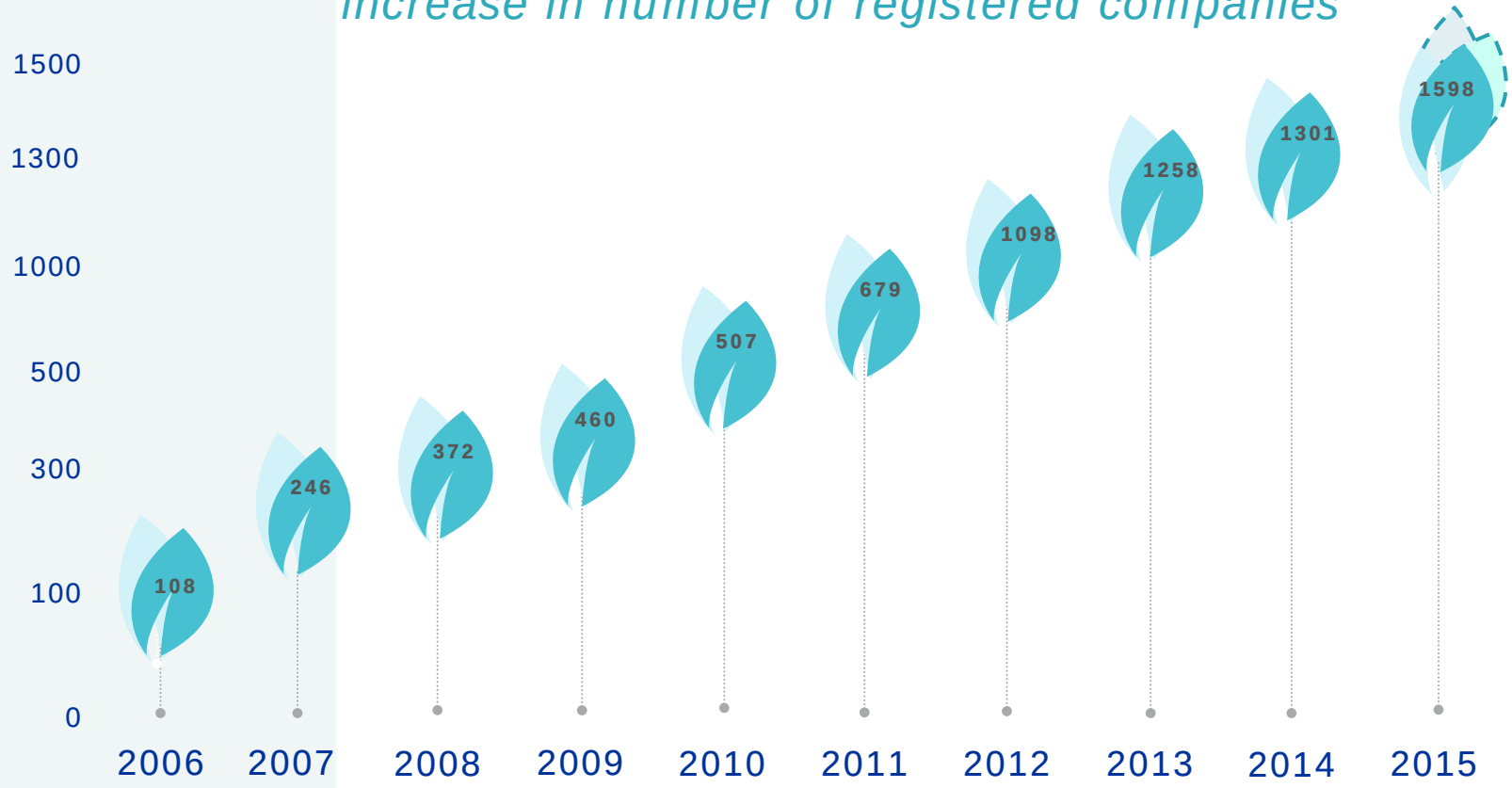
# 01 Validation

1. Submission
2. Review
3. Qualification
4. Renewal



# Validation

*increase in number of registered companies*



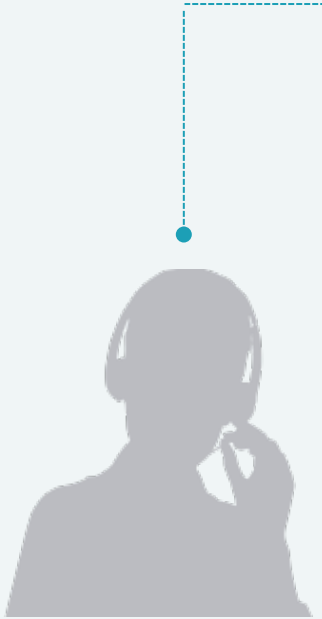
# 02 Regulatory assistance

*Administrative and procedural queries are addressed by email, phone or in meeting.*

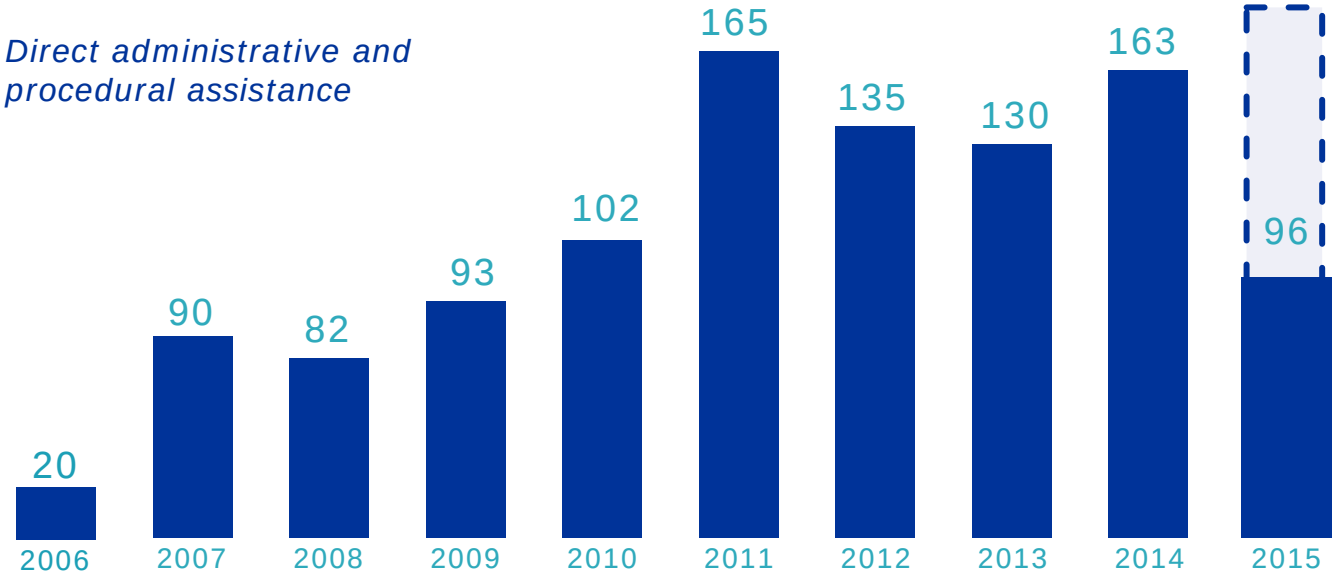
*A lack of experience with the centralised procedure should not impair the development and marketing of new medicinal products. The SME office will facilitate communication and answer practical or procedural enquiries.*

# Regulatory assistance

*tailored to SMEs*



*Direct administrative and procedural assistance*

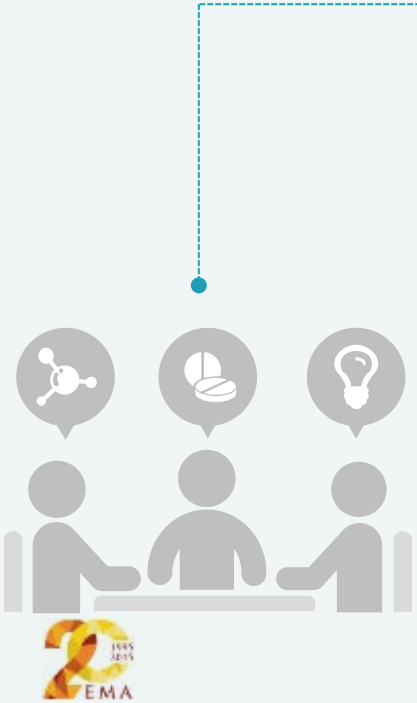


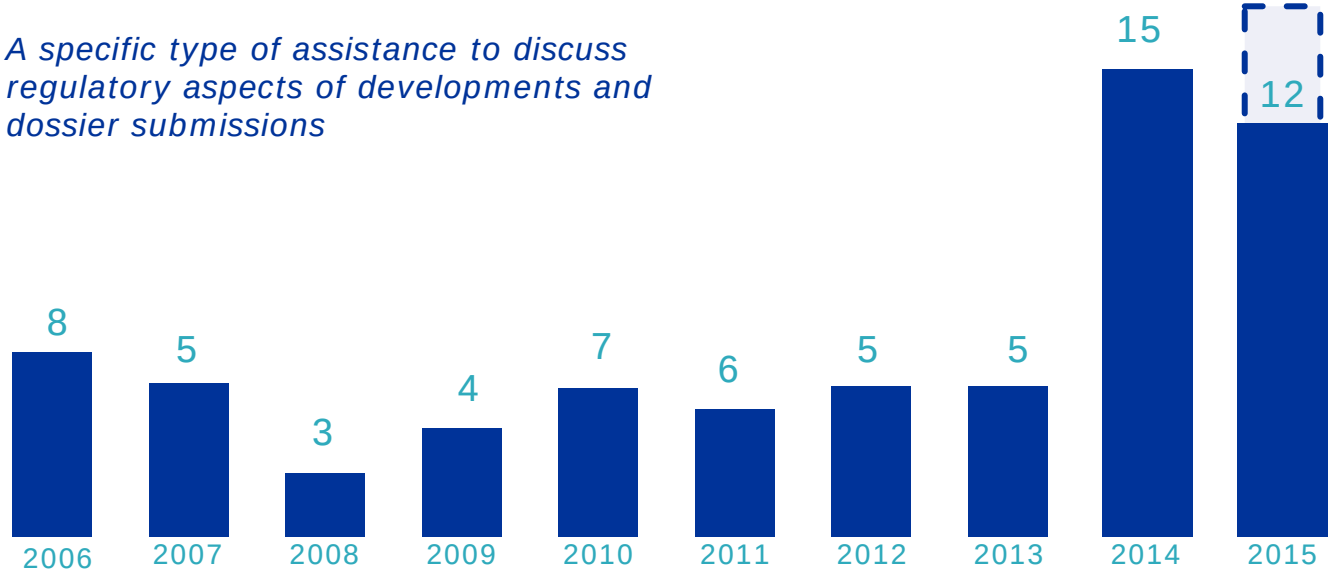

Direct Assistance: total number of queries 2006 - 2015

# Regulatory assistance

## *briefing meetings for SMEs*

*A specific type of assistance to discuss regulatory aspects of developments and dossier submissions*





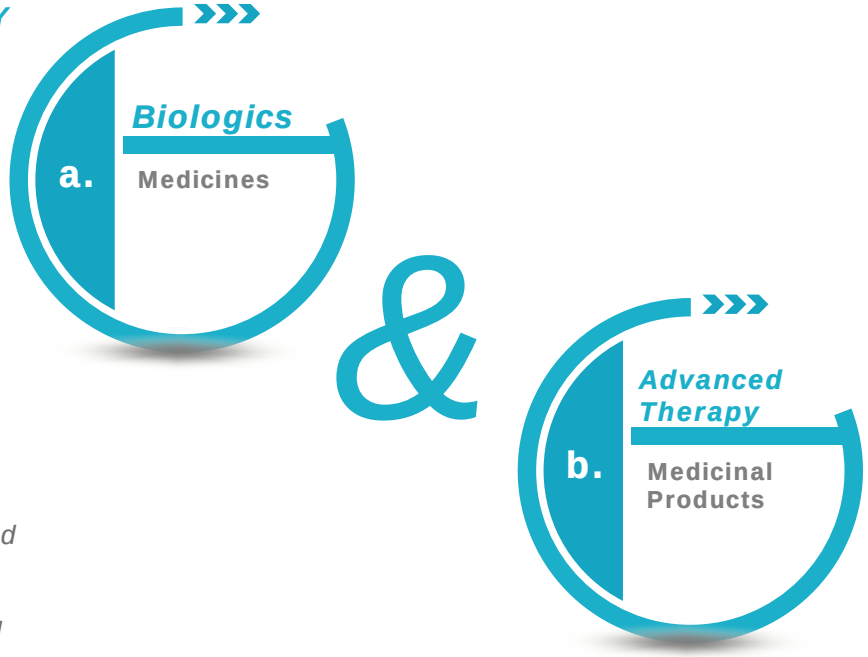

Total number of briefing meetings 2006 - 2015

# Regulatory assistance

## Top 10 Most frequent areas

### ADMINISTRATIVE, PROCEDURAL & REGULATORY

1. *Eligibility to centralised procedure, preparation and submission of dossier*
2. *SME definition, incentives and translations*
3. *EMA and national fee queries*
4. *GCP/GMP inspection*
5. *Packaging and labelling requirements*
6. *Assistance on legal basis, including conditional marketing authorisation, approval under exceptional circumstances, dossier reviews under accelerated conditions, paediatric use marketing authorisations and adaptive pathways*




### SCIENTIFIC ADVICE, ORPHANS & PAEDIATRICS

7. *Guidance on scientific advice procedure and dossier contents*
8. *Assistance on identification and interpretation of CHMP/CVMP/ICH guidelines*
9. *Procedural assistance on PIP and requirements/waivers*
10. *Assistance and guidance on contents of Orphan designation applications*

# 03 Fee incentives

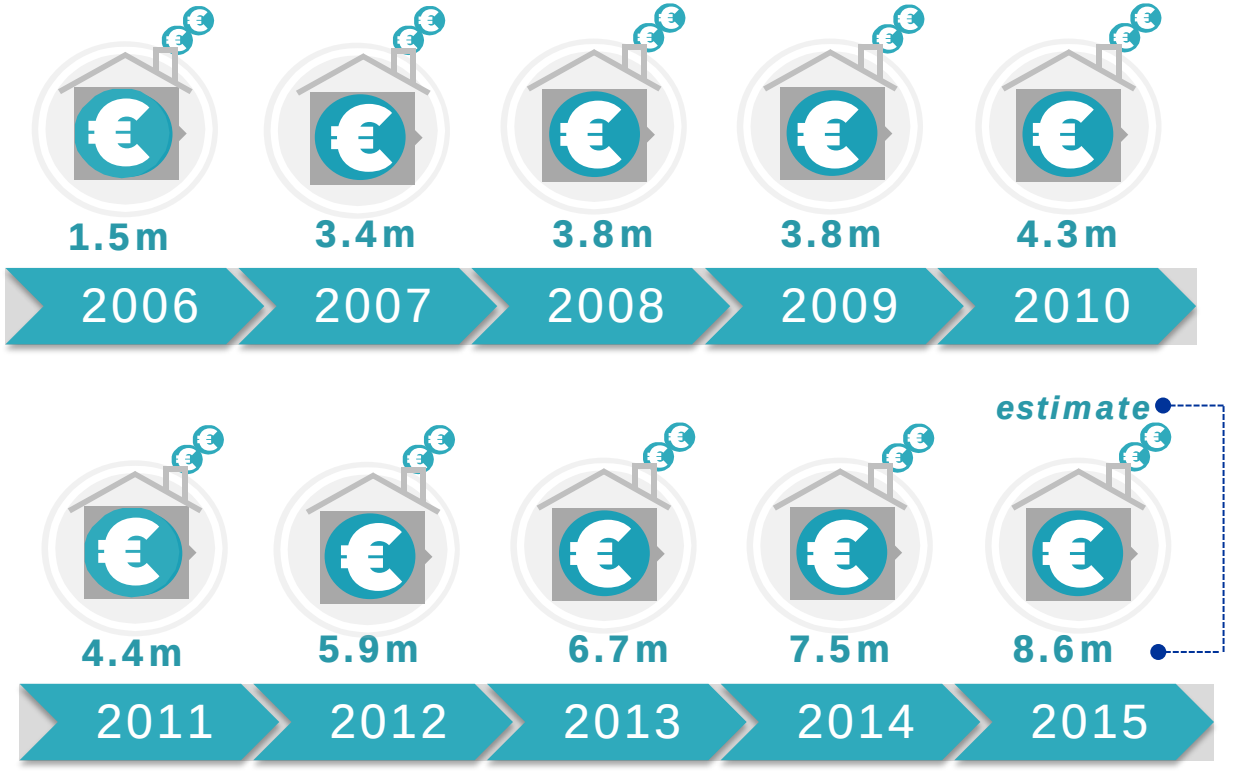
*Fee reductions for SA, scientific services, Inspections & MRLs; Fee exemptions; Fee deferrals; Waiver of the MedDRA licensing fee; Post-authorisation fee incentives*

*The SME initiative has been designed to offer financial incentives to SMEs. Such assistance will encourage SMEs to seek advice from the EMA, with a view to maximising the chances of a successful marketing authorisation.*

# Fee incentives

*2006-2015*

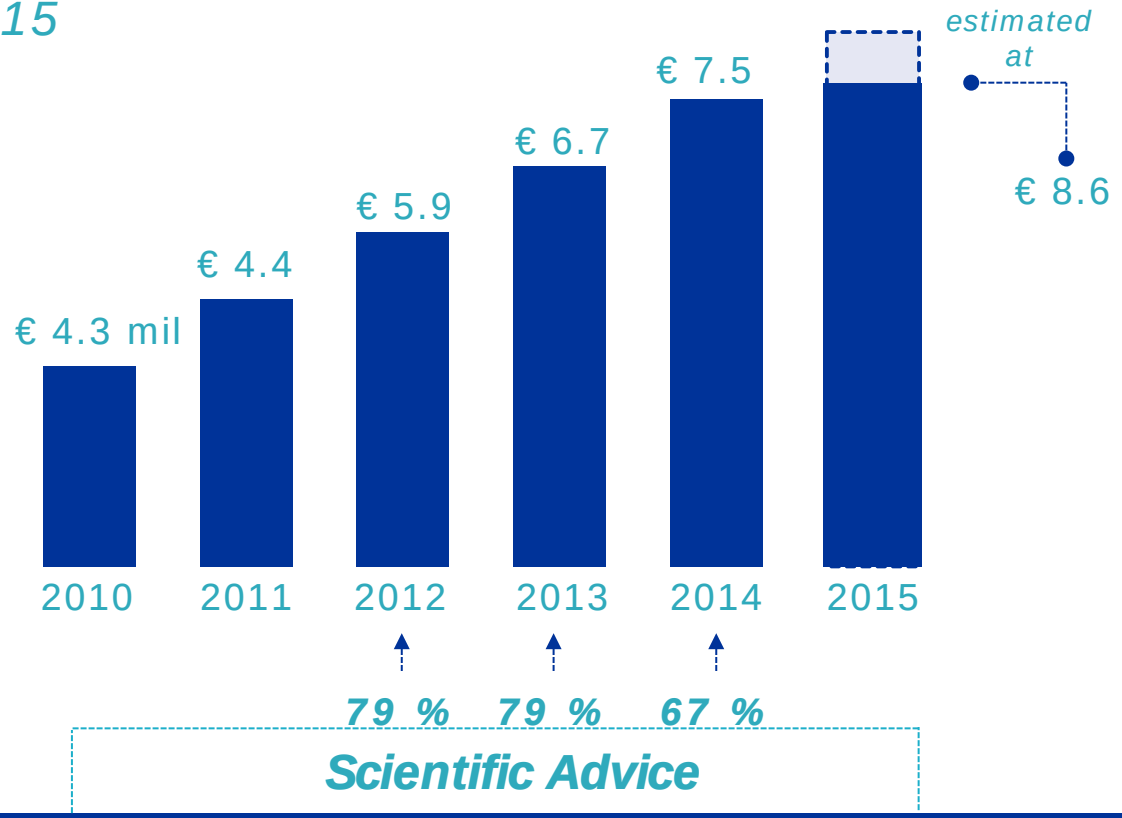
*"... the main financial and administrative entry hurdles for SMEs are the various steps involved in pre-marketing authorisation procedures. ...The fees for the marketing authorisation application and the related inspections conducted for the purpose of assessing the application could constitute a significant financial constraint for SMEs. ... it is appropriate to defer the payment of these fees until the end of the procedure. ... SMEs seeking marketing authorisation should be facilitated through fee reductions."*



# Fee incentives

- *Fee deferrals* ***non orphans***
- *Conditional fee exemptions* ***8***

*2010-2015*



# 04 Translation assistance

*Assistance with translations of the product information documents submitted in the application for marketing authorisation.*

*The EMA will provide translations of product information required to grant an EU marketing authorisation. Translation into EU official languages will be provided free of charge by the Agency.*

# Translation assistance

## *budget for translations*

*Assistance with*

**54**

*product translations*

**Translations cost (Human & Vet)**

| 2010 | 2011 | 2012 | 2013 | 2014 | **2015** |
|---|---|---|---|---|---|
| 300 k€ | 392 k€ | 292 k€ | 776 k€ | 332 k€ | **365 k€** |
| 6 | 8 | 5 | 7 | 6 | 4 |

*as at 21 October 2015*

**Average translation cost per file 68,250 €**

# 05 Training & awareness

### *Workshops*
*Annual or Biannual regulatory training course tailored for SMEs.*

### *Newsletters*
*Circulated quarterly. Published on the EMA Website.*

### *Announcements*
*Ad hoc information sent by email to SMEs and stakeholders.*

### *User Guide*
*Updated regularly.*


*The SME Office shall have the following tasks:*

*(b) to organise workshops and training sessions for applicants on the administrative and procedural steps necessary to comply with the requirements laid down in Regulation (EC) No 726/2004.*

# 06 Partnering & networking

*The register was created in consultation with SME stakeholders aiming …*


- *to facilitate and promote interaction, partnering and networking between SMEs*
- *to increase information available to SMEs and their stakeholders*
- *to provide a source of information for EU institutions, agencies and Member States.*

# 07 Support to advanced therapies

*Certification of quality non-clinical data for Advanced therapy medicinal Products (ATMPs) intended for human use.*

**6** *ATMP certifications* ***FINALISED***

*"As an incentive to develop ATMPs, an SME can submit the results of studies carried out to demonstrate the quality and non-clinical safety of ATMPs and request evaluation and certification of the data, independently of any MAA."*

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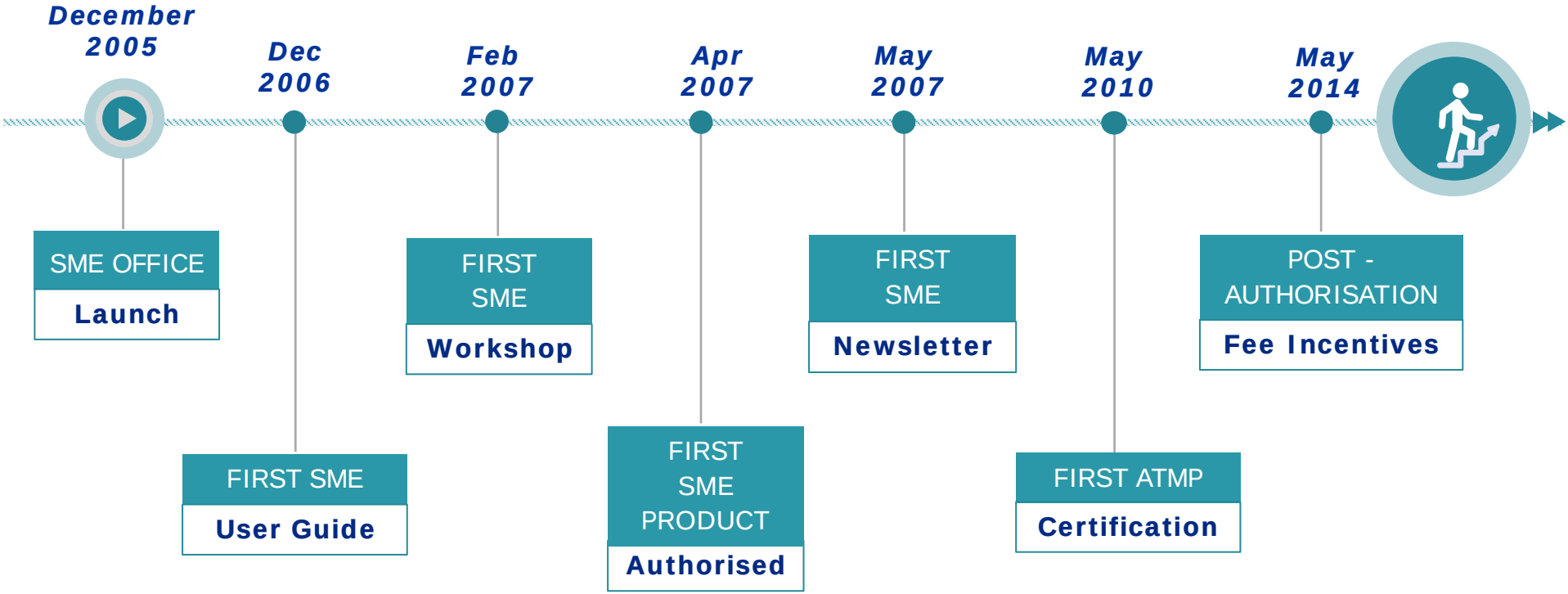
# *Evolution and support to SMEs*


December 2005
SME OFFICE
Launch
Dec 2006
FIRST SME
User Guide
Feb 2007
FIRST SME
Workshop
Apr 2007
FIRST SME PRODUCT
Authorised
May 2007
FIRST SME
Newsletter
May 2010
FIRST ATMP
Certification
May 2014
POST - AUTHORISATION
Fee Incentives

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# *A profile of registered SMEs*


December
2006


November
2015

# Company profiles


## 2006

### Field of activity
Large majority: Human use

### Category of enterprise
Micro: 24%
Small: 37%
***Medium: 39%***

### Type of enterprise
***Linked: 58%***
Partner: 2%
Linked/Partner: 3%
Autonomous: 37%

### Geographical distribution
1. UK
2. France
3. Germany
4. ***Denmark***
5. ***Sweden***

## 2015

### Field of activity
Large majority: Human use

### Category of enterprise
***Micro: 42%***
Small: 35%
Medium: 23%

### Type of enterprise
Linked: 40%
Linked/Partner: 6%
Partner: 3%
***Autonomous: 51%***

### Geographical distribution
1. UK
2. Germany
3. France
4. ***Italy***
5. ***Spain***

**General profile of SMEs**

# Product profiles


## TYPES OF PRODUCTS

| | |
|---|---|
| Chemicals | 2 / 3 |
| Biologics | 1 / 3 |

**NO CHANGE IN DISTRIBUTION**

*Same % of companies developing ATMPs as 2010*

## DEVELOPMENT STAGE

**2010**

| | |
|---|---|
| Marketing stage | 11 % |

**2015**

| | |
|---|---|
| Marketing stage | 56 % |

*Change due to PhVigilance legislation*

## PRODUCT CATEGORIES

**2012**

| | |
|---|---|
| Therapeutics | 76 % |
| Vaccines | 17 % |

**2015**

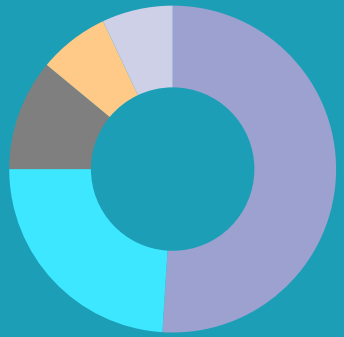
| | |
|---|---|
| Therapeutics | 84 % |
| Vaccines | 5 % |

# Ownership


**221** newly created companies since January **2012**

**10 %** *is academic spin-off*

| | |
|---|---|
| Natural Persons | 51 % |
| Corporate | 24 % |
| Private investment | 11 % |
| Venture capital | 7 % |
| Other | 7 % |



**2015**

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# *Marketing Authorisations*

# Marketing authorisations

## *humans medicines MAA outcomes*

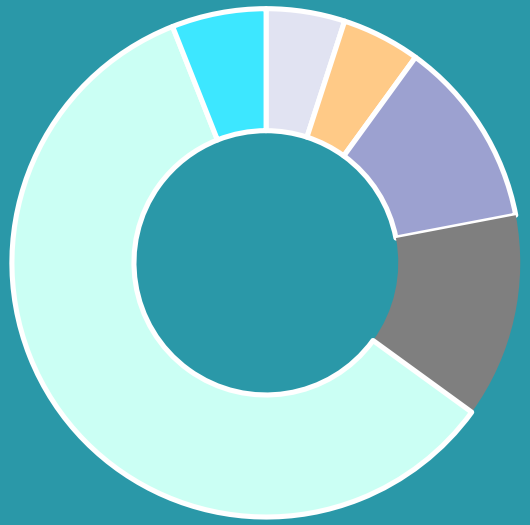

| | Positive | Negative | Withdrawn | Success rate |
|---|---|---|---|---|
| 2006 | 0 | 1 | 1 | *0 %* |
| 2007 | 1 | 1 | 3 | *20 %* |
| 2008 | 5 | 2 | 6 | *38 %* |
| 2009 | 8 | 0 | 6 | *57 %* |
| 2010 | 4 | 1 | 1 | *67 %* |
| 2011 | 8 | 0 | 3 | *73 %* |
| 2012 | 5 | 0 | 3 | *63 %* |
| 2013 | 10 | 3 | 4 | *59 %* |
| 2014 | 6 | 1 | 3 | *60 %* |
| 2015 | 5 | 1 | 1 | *71 %* |

***Overall success rate for any applicant*** **72%**

# Marketing authorisations

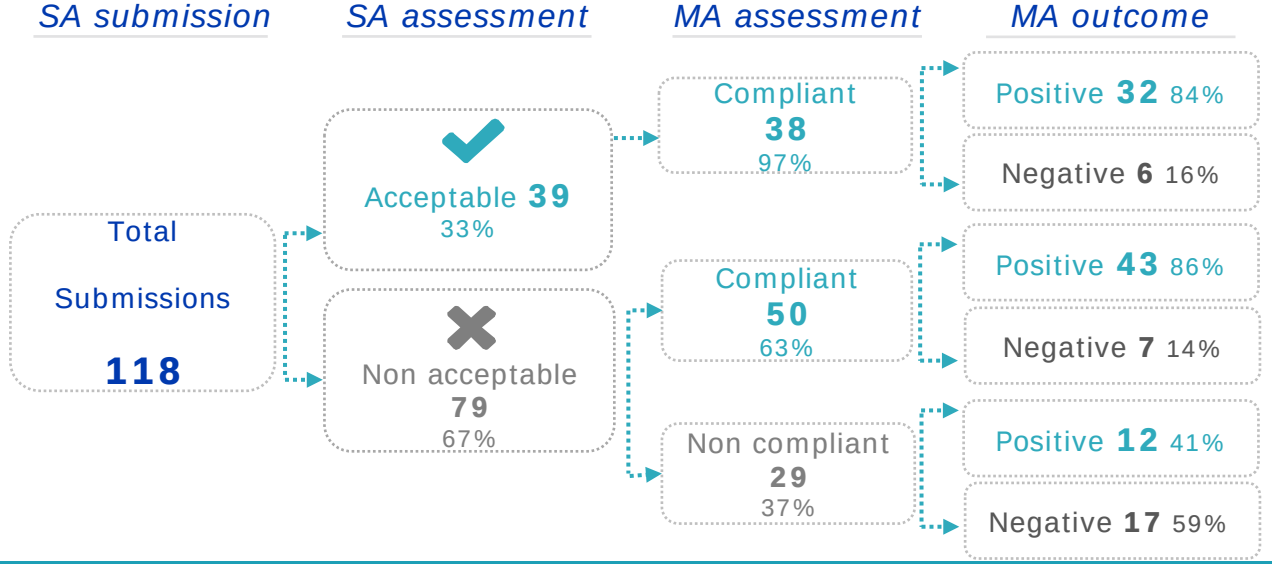
## *Legal Basis*


| | | |
|---|---|---|
| *Art 8(3)* | New active substance | 59% |
| *Art 8(3)* | Known active substance | 13% |
| *Art 10(3)* | Hybrid application | 12% |
| *Art 10a* | Well established use | 6% |
| *Art 10(1)* | Generic application | 5% |
| *Art 10b* | Fixed combination | 5% |



# MA applications outcome

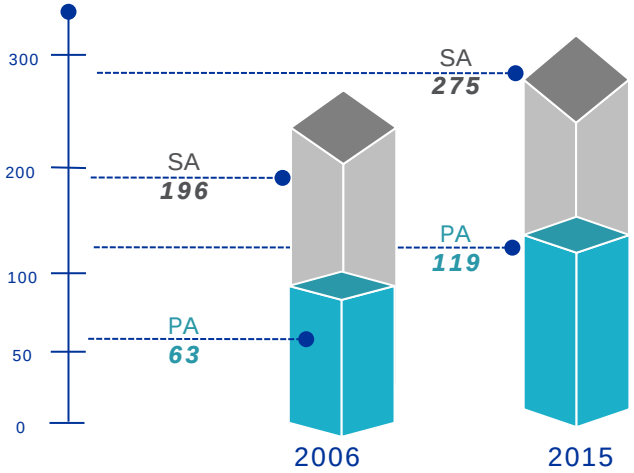
## Scientific Advice

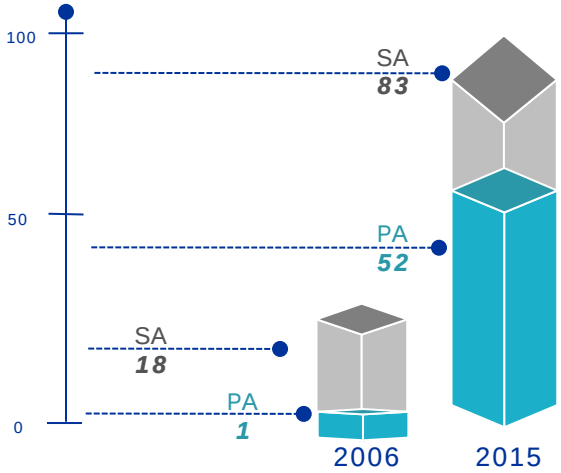




**2008-2012**

| | MAA Procedure average days | Total MO * Quality/Pre-Clinical | Clin. Efficacy | Clin. Safety | Total |
|---|---|---|---|---|---|
| Compliance | 367 | 1.64 | 2.66 | 0.86 | 5.16 |
| Non-Compliance | 428 | 4.25 | 3.91 | 1.42 | 9.58 |

# Scientific advice – protocol assistance


**5** ***Parallel HTA Advice by SMEs***

***35%*** ***of SA by SMEs***

***44%*** ***of PA by SMEs***

# Post-marketing authorisation - Adis, PSUR

*for HUMAN medicines only*


*Licensing activity in **90%** of products (**19/21**)*

| EU Countries | Non-EU Countries |
|---|---|
| Out-licensed | Out-licensed |
| 53% | 47% |
| 9 products | 10 products |

***Authorised products are marketed in* 6 *countries of the EU/EEA***

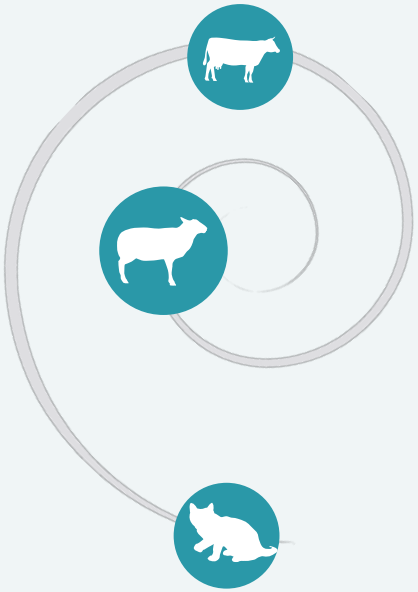
***Average time to market is* 7 *months***

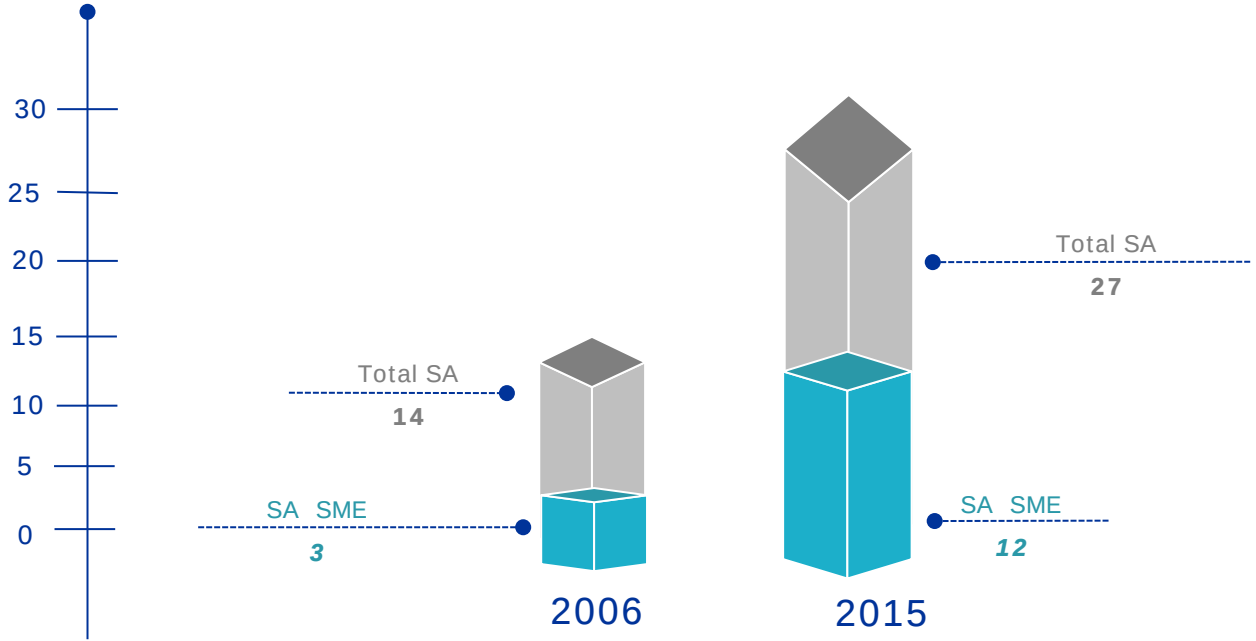
# Marketing authorisations


| | Positive | Negative | Withdrawn |
|---|---|---|---|
| 2006 | 1 | 0 | 0 |
| 2007 | 1 | 0 | 0 |
| 2008 | 2 | 0 | 0 |
| 2009 | 1 | 0 | 0 |
| 2010 | 2 | 0 | 0 |
| 2011 | 4 | 0 | 0 |
| 2012 | 0 | 0 | 2 |
| 2013 | 1 | 0 | 0 |
| 2014 | 2 | 0 | 1 |
| 2015 | 2 | 1 | 0 |

# Veterinary SMEs

## *Scientific Advice*

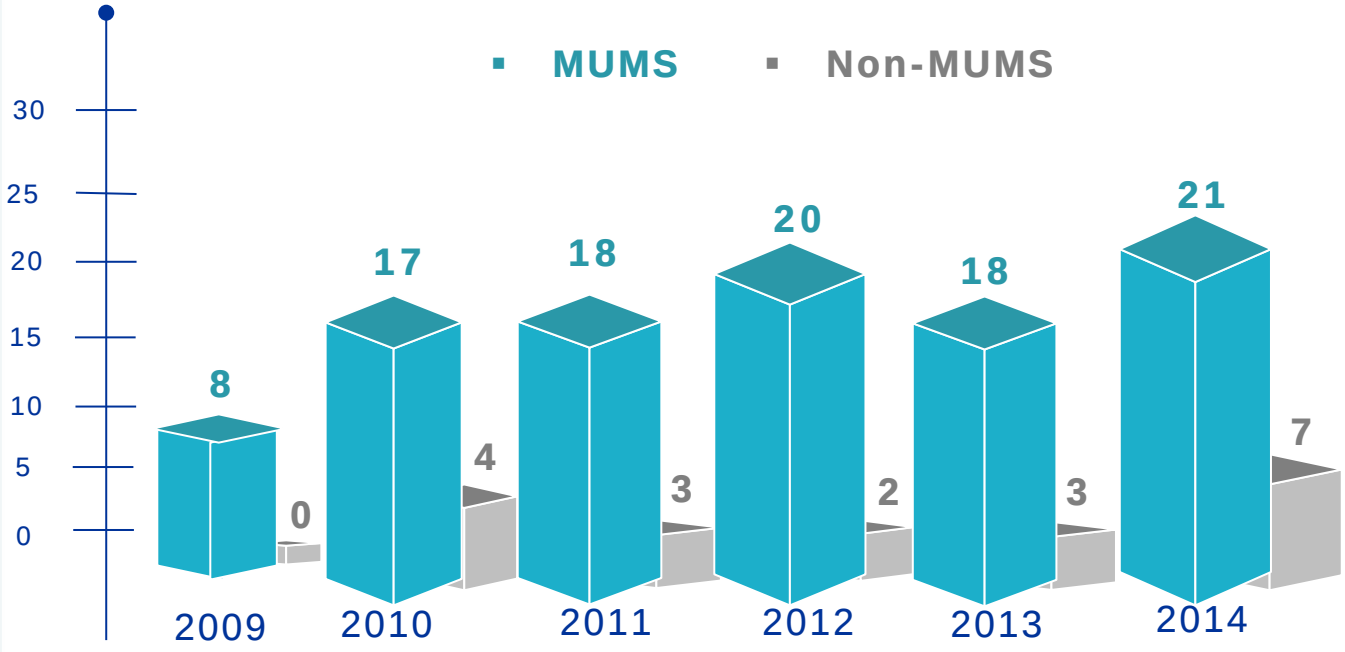




# Classifications

## *Veterinary MUMS/limited market*

- *Policy started September 2009 (EMA/308411/2014 Adopted) - updated in 2014*
- *2009-2015 57 out of 151 requests for classification under policy were received from SMEs*
- *Annual reports published on website*



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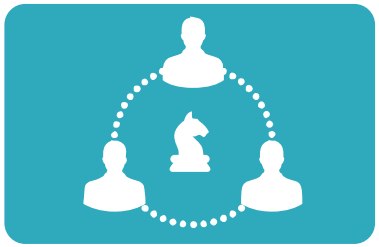
# *Looking ahead*

# Strategic vision and work programme

**EMA Work Priorities 2016-2017**

*Priority area 2:*
*'Supporting availability of medicines, timely access, and facilitating innovation'*



**NCA Network Strategy & Objectives**

*'Supporting value added innovation'*
*'Developing support to SMEs'*

**EC Communication WP 2016**

*'No time for business as usual'*
*'We are focused on helping SMEs and start-ups to grow by addressing regulatory obstacles and facilitating access to finance'*

- *Role of academia and collaborative public/private partnerships*
- *Future changes in innovation for medical devices and veterinary regulations*

# Opportunities

**1** *Enhance communication and outreach to wider SMEs stakeholders involved in pharma innovation. (innovation support agencies, finance stakeholders, business incubators, networks and clusters at the National, European and International level).*

**2** *Increase efficiencies and develop synergies through greater work-sharing and exchange of best practices with bodies offering support to SMEs at the National, European and International level.*

**3** *Improve guidance and systems for optimal use of the full range of available regulatory tools for SMEs.*

Acknowledgements:
Aliki Synodinou
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## Further information

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