Session 5: Dealing with Unmet Medical Needs and Support to Innovation

Guidance for R&D programmes	• Scientific Advice • PRIME framework	Stiina Aarum
Early engagement in R&D	• The Innovation Task Force	Falk Ehmann
Special provisions	• Exceptional circumstances • Conditional marketing authorisation	Zigmars Sebris
Special support	• The SME initiative	Leonor Enes

R&D = Research and Development; SME = small and medium enterprise

Structure: 4 presentations followed by 20 minutes for exchange and discussion

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Guidance to R&D programmes: Scientific Advice and the PRIME network

The EU medicines regulatory system and the European Medicines Agency: an introduction for international regulators and non-governmental organisations

18 September 2017

Presented by Stiina Aarum
Product Development Scientific Support Department

This session:

Scientific advice and protocol assistance – Scope, value and current developments

- Parallel EMA/HTA scientific consultation
- Qualification of novel methodologies and biomarkers
- Modelling and simulation
- PRIME-Legal basis, value and experience so far

The typical long road of bringing medicines to patients

Regulatory provisions targeting the risk of development failure and the time to access:

- **Scientific advice**
- Support to small/medium-sized enterprises
- **PRIority MEdicines scheme (PRIME)**
- Conditional marketing authorisation
- Accelerated Assessment
- Compassionate Use

Scientific Advice

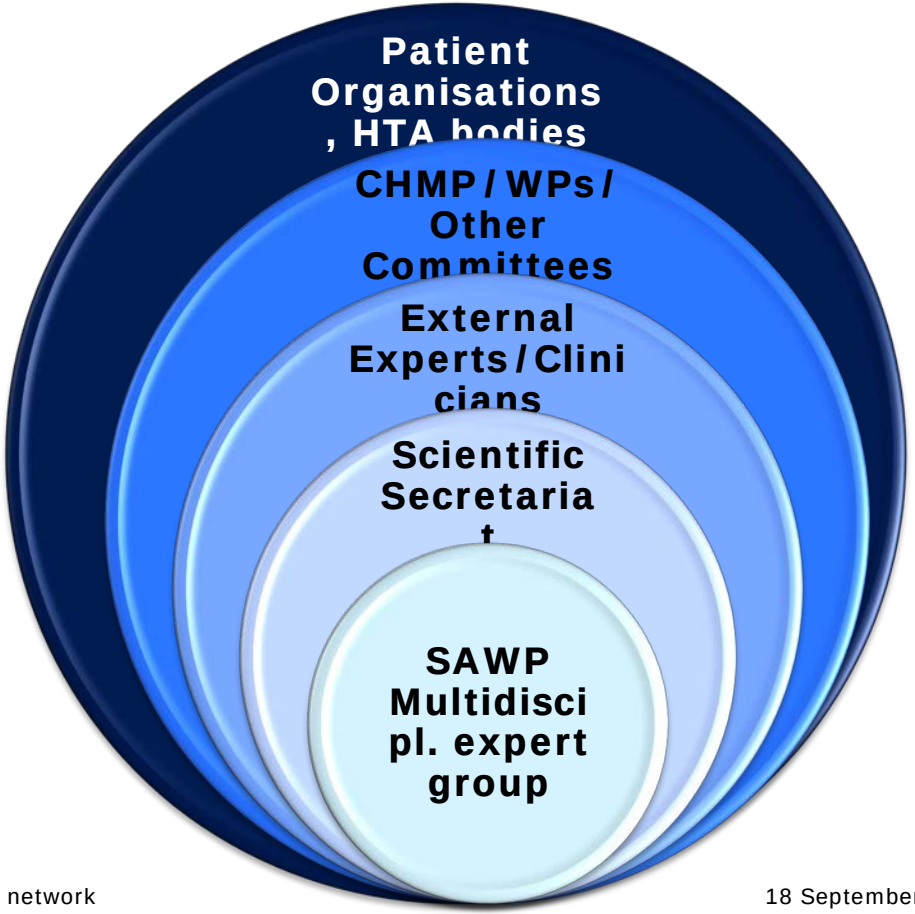
- Legal basis: According to Article 57-1 (n) of Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004
- One of the tasks of the Agency is "advising undertakings on the conduct of the various tests and trials necessary to demonstrate the quality, safety and efficacy of medicinal products".
- Advising Applicants on the scientific requirements for marketing authorisation (MA):
  - Before the first MA: companies ask questions on manufacturing, non-clinical and clinical trials, risk-management plans, ways to develop generics, hybrids and biosimilars; significant benefit for orphan medicines; development in children etc.
  - Post-MA: extension of indication to different age groups and stages of the disease; different conditions; & safety aspects. Line extensions etc.

Scientific Advice

For human medicines, SA and protocol assistance are given by the Committee for Medicinal Products for Human Use (CHMP) on the recommendation of the Scientific Advice Working Party (SAWP).

Prospective in nature- focusing on development strategies rather than pre-evaluation of data to support a MAA.

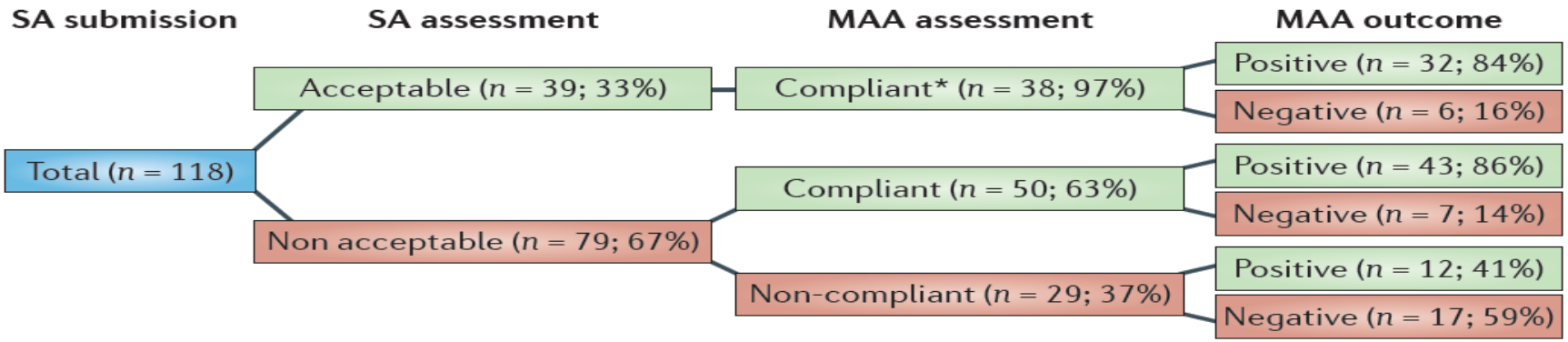
Scientific Advice Network



Scientific Advice Working Party (SAWP)

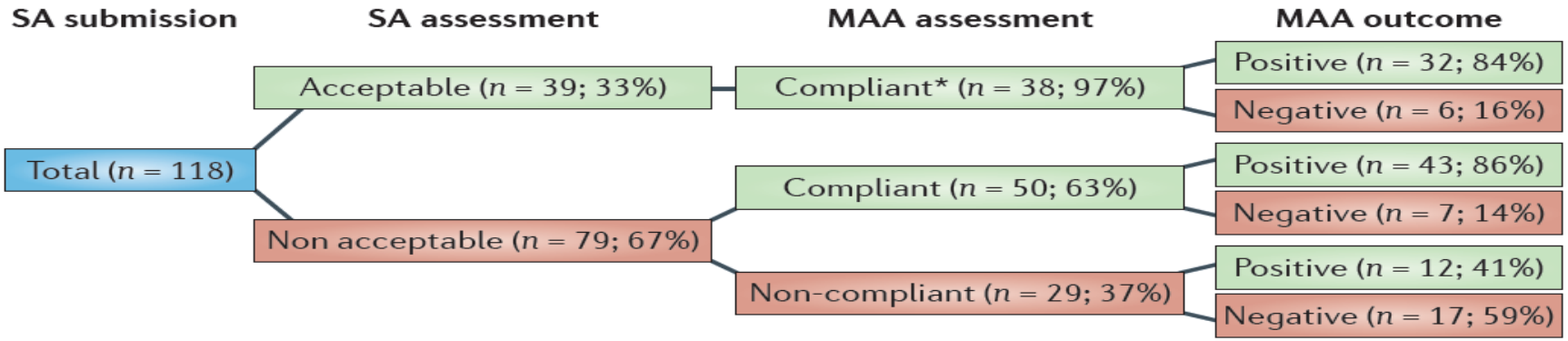
- Experts from national authorities, universities and hospitals selected for expertise: e.g. oncology, cardiology, psychiatry, neurology, immunotherapy, gene and cell therapy, advanced therapies, pediatrics, geriatrics; quality, non—clinical and statistical methodologies.
- Joint members across Committees not only CHMP, but also Paediatrics, Orphan, Advanced Medicinal Products, PRAC
- Scientific and logistic support from EMA secretariat: medical doctors /pharmacists and assistants

SA can help to guide changes in the pivotal clinical development…



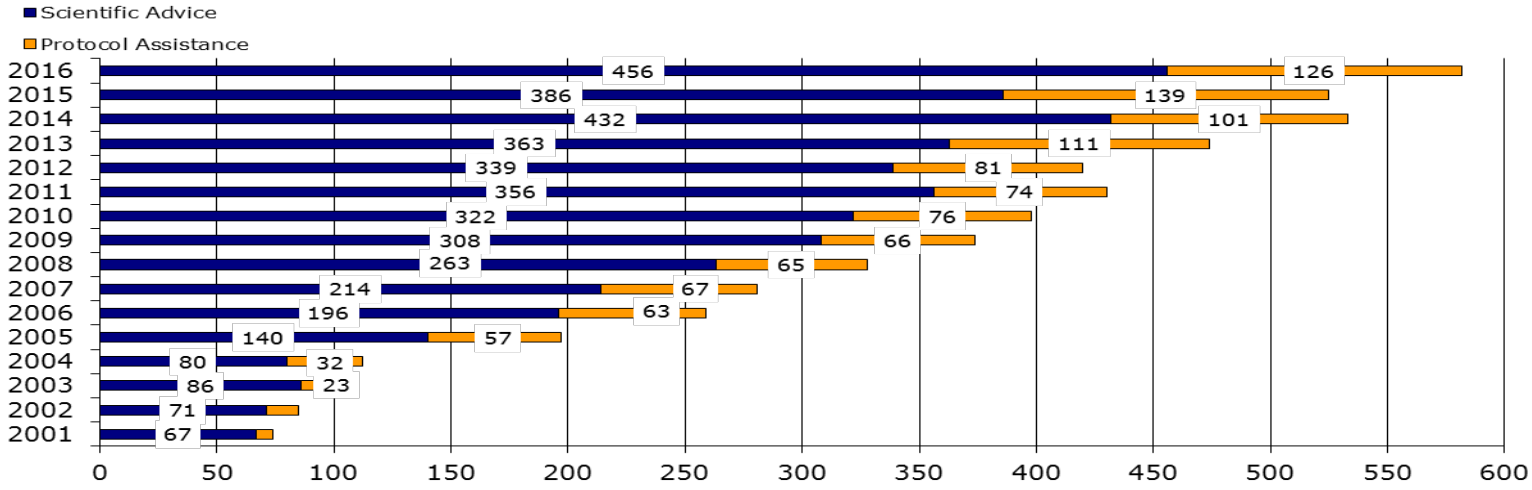
SA/PA submitted in 2008–2012, Hofer et al. Nat Rev Drug Discov. 2015

SA can help to guide changes in the pivotal clinical development towards improved regulatory acceptability



- Obtaining and complying SA is strongly associated with a positive outcome of a MAA: almost 90% of those who obtain and follow SA receive a positive opinion compared to 40% for those who do not follow SA; *Hofer et al. 2015*

Scientific Advice main activity so far: scientific advice and protocol assistance



Parallel EMA/HTA scientific consultation

Starting point: Newly licensed medicines do not reach all patients in need

Regulators and HTAs

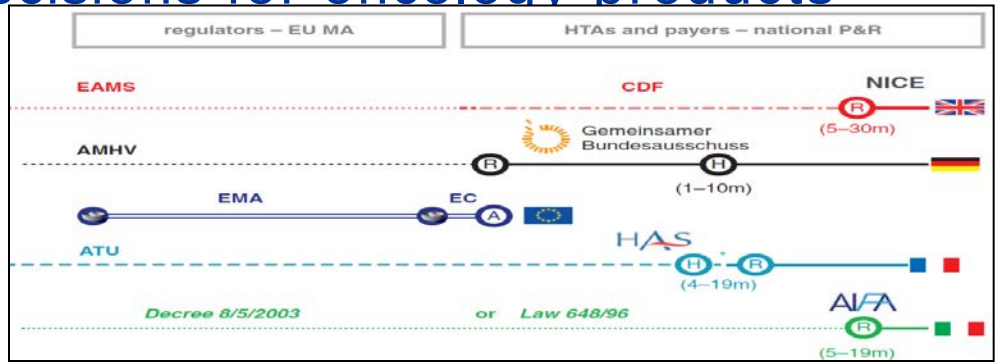
- answer different questions
- have different requirements in terms of evidence

Aim: decision makers come together early to discuss

- the planned development including populations / comparators / design of trial /endpoints
- the requirements for post-licensing evidence generation

Expectation: Optimised development plan ➔ Improve access for patients

Reality check: from EU regulatory approval to national HTA/P&R decisions for oncology products



Drug	Indication	EU MA Approval	Time for HTA/P&R after MA (month)			
bosutinib (Bosulif)	chronic myeloid leukaemia	03/2013	7	7	11	18
vismodegib (Erivedge)*	basal cell carcinoma	07/2013	n/a	7	5	20
cabozantinib (Cometriq)*	medullary thyroid cancer	03/2014	n/a	10	8	n/a

Martinalbo et al., Early access to cancer drugs in the EU. *Ann Oncol* 27: 96–105, 2016

Align regulatory and HTA thinking; what constitutes success?

Tripartite understanding of roles, remits and standards

Common language

Common understanding of methodology

Common understanding of science and methodology; different application?

Evidence generation without undue delay: avoid sequential thinking

Alignment of the perspectives of EU regulators and HTA bodies published: Tafuri et al, Br J Clin Pharmacol (2016):

Studied population, comparator, endpoints, overall package for E and S, other study design characteristics

Qualification of novel methodologies and biomarkers

Vision: Speed up/optimise drug development and utilisation, improve public health

Procedure to guide the development of new more efficient ways to develop drugs, e.g. development of new endpoints for clinical trials

Examples:

- Methods to predict toxicity; IC to enrich a patient population for a clinical trial: Volume of certain brain structures and level of certain biochemicals in the cerebrospinal fluid for trials in Alzheimer's disease
- Surrogate clinical endpoints: new sensitive scales to measure efficacy of a new drug instead of hard clinical endpoints
- Patient and caregiver reported outcomes

Qualification of Novel Methodologies for drug development

CHMP Qualification Advice on future protocols and methods for further method development towards qualification.

CHMP Qualification Opinion on the acceptability of a specific use of the proposed method (e.g. use of a biomarker) in a research and development (R&D) context (non-clinical or clinical studies), based on the assessment of submitted data.

Who can apply? Consortia, Networks, Public / Private partnerships, Learned societies, Pharmaceutical industry.

117 procedures since start in 2008

Modelling and simulation- regulatory value

Early: Enable early informed discussion with sponsors regarding study designs, endpoints, dose regimens, paediatric questions, data needed to support benefit risk decisions

At MAA: Support benefit risk decisions by investigating uncertainties & untested scenarios, and their clinical consequences

Translate benefit risk from the population to individual

Inform SmPC especially for special populations

Support Subgroup analysis

Post Marketing: Inform the contents of the RMP

Lifecycle management of products

Eligibility to PRIority Medicines (PRIME) scheme

Legal base-accelerated assessment

(Recital 33 and Article 14(9) of Regulation (EC) No 726/2004)

Medicinal products of major public health interest and in particular from the viewpoint of therapeutic innovation.

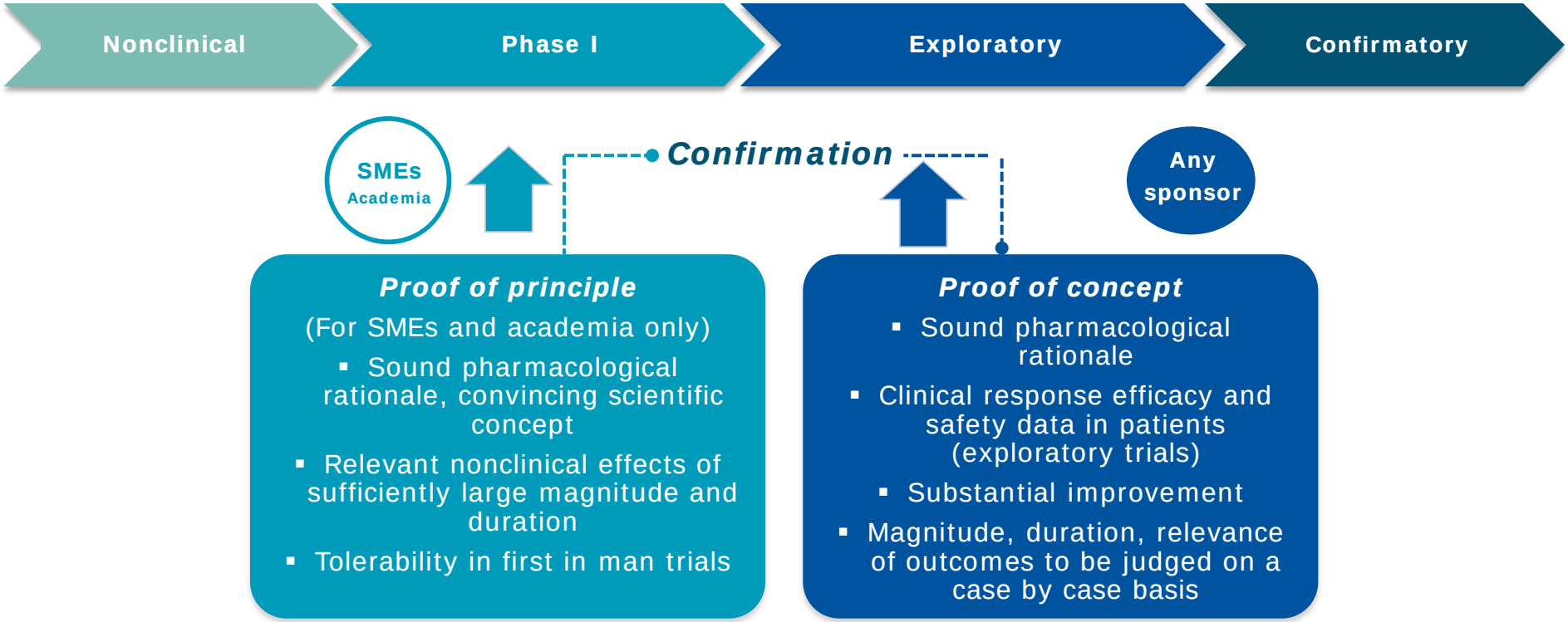
- Potential to address to a significant extent **an unmet medical need**
- Scientific justification, based on **data and evidence** available from nonclinical and clinical development to address the UMN

No satisfactory method or if method exists, bring a major therapeutic advantage

Introducing new methods or improving existing ones

Meaningful improvement of efficacy (impact on onset, duration, improving morbidity, mortality)

Entry points PRIME eligibility and required evidence



Features of the PRIME scheme

Early access tool, supporting patient access to innovative medicines.

- **Written confirmation of PRIME eligibility** and **potential for accelerated assessment**;
- **Early CHMP Rapporteur appointment** during development;
- **Kick off meeting** with multidisciplinary expertise from EU network;
- **Enhanced scientific advice** at key development milestones/decision points;
- **EMA dedicated contact point**;
- **Fee incentives** for SMEs and academics on Scientific Advice requests.

PRIME eligibility recommendations adopted by 20 July 2017

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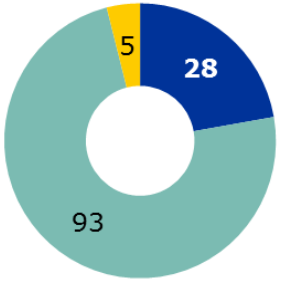
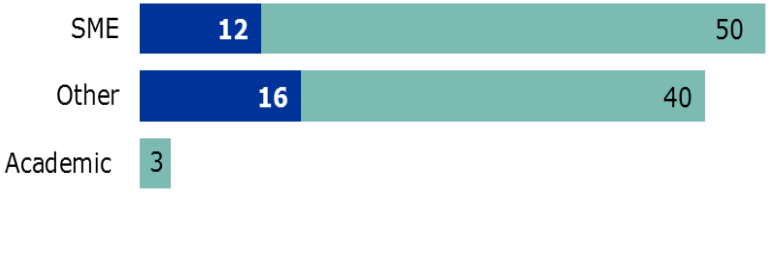


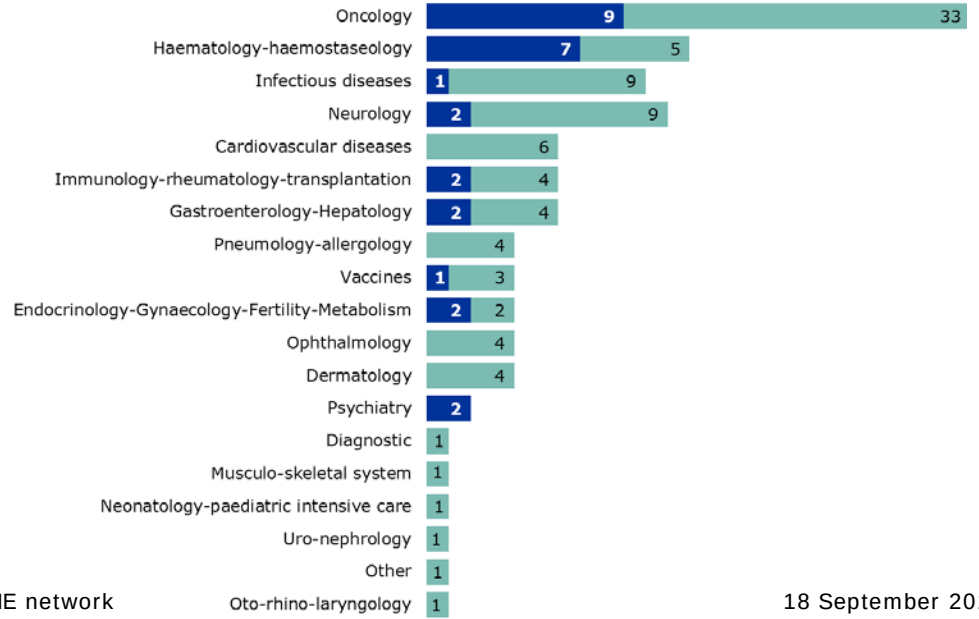
> 120 eligibility requests

28 granted*

~ 50% SMEs

~ 50% Advanced therapies





Take home message- Scientific Advice and PRIME

- **Key tool to promote the collection of robust data on the benefits and risks of medicines**
- **Benefits patients as it promotes the generation of robust data and protects them from participating in badly designed or irrelevant clinical trials**
- **Key platform for our collaboration with health technology assessment (HTA) bodies** which aims to facilitate patients' access to new medicines
- **Central activity to stimulate innovation**
- **Regulatory incentive via PRIME is possible for medicinal products of major public health interest** and in particular from the viewpoint of **therapeutic innovation**

Thank you for your attention

Further information

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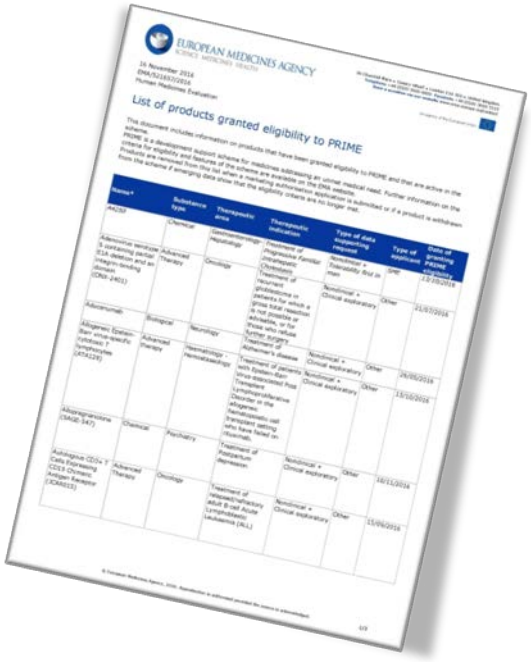
Backup/extra slides

Transparency

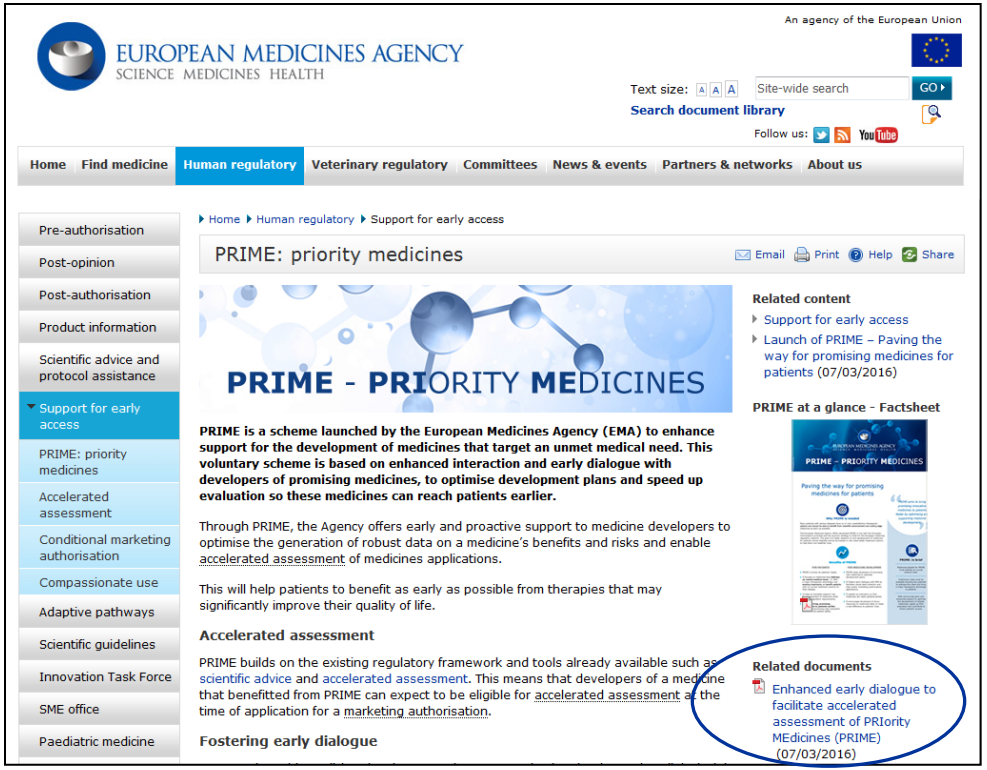
Publication of monthly reports

- Broad characteristics
- Active substance (for eligible products only)
- High-level statistics

List of products granted eligibility to PRIME



PRIME webpage and supporting documents



Factsheet in lay language

Q&A, templates, application form for applicants

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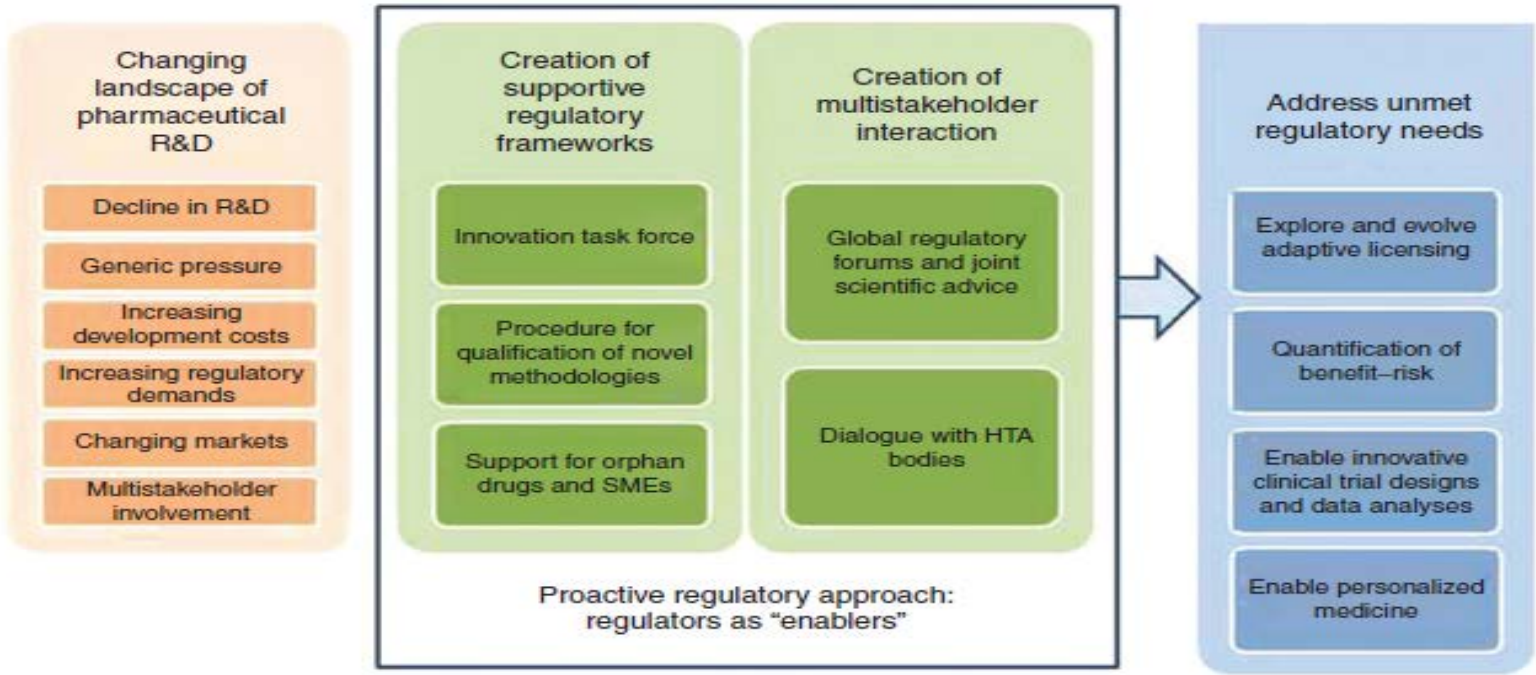
Early engagement in R&D: The Innovation Task Force (ITF)

The EU medicines regulatory system and the European Medicines Agency: an introduction for international regulators and non-governmental organisations

Presented by: Falk Ehmann
Science and Innovation Office; Human Medicines Research and Development Support Division; EMA

Regulators became gatekeepers and enablers...



Clinical pharmacology & Therapeutics; Advance online publication 3 April 2013. doi:10.1038/clpt.2013.14 ; F Ehmann, M Papaluca Amati, T Salmonson, M Posch, S Vamvakas, R Hemmings, HG Eichler and CK Schneider

Innovation Task Force (ITF)

Multidisciplinary platform

for **preparatory dialogue and orientation on**

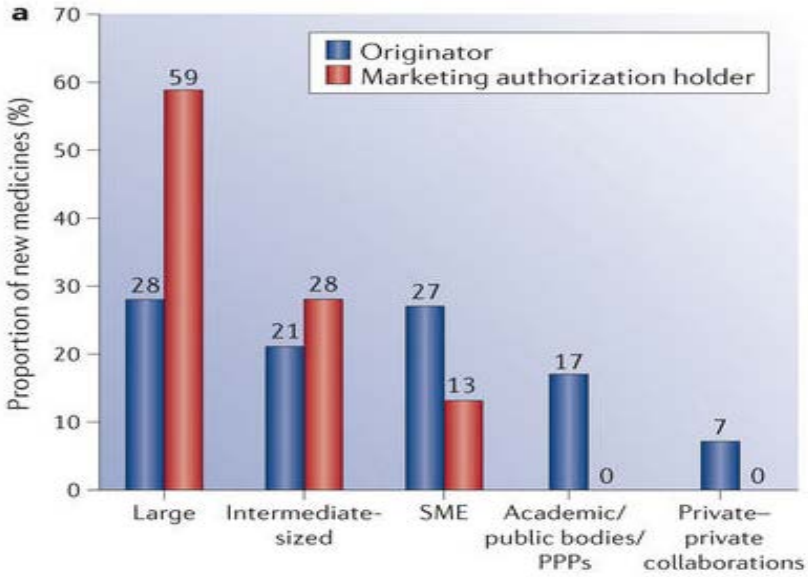
innovative methods, technologies and medicines

ITF objectives (ASAP):

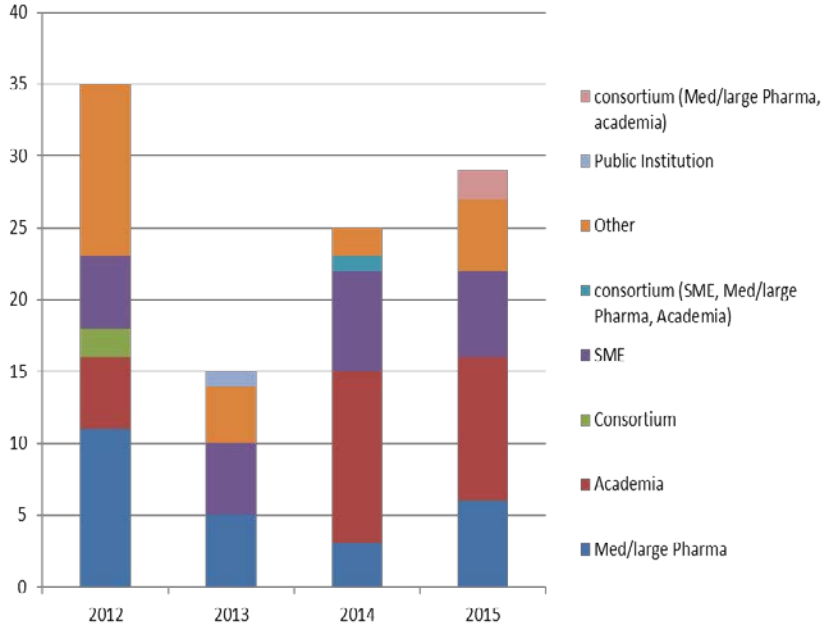
- **Assist Knowledge exchange** on innovative strategies involving **regulatory network**
- **Support drug development** via early dialogue on
  - **Scientific, legal and regulatory** issues
  - Products, **methodologies and technologies**
- **Address the impact of emerging therapies and technologies** on current regulatory system
- **Preparing for formal procedures**

Users of the Innovation Task Force

Originator and the marketing authorization holder for 94 approved products evaluated, divided according to organization type



Regulatory watch: Where do new medicines originate from in the EU? Nature Reviews Drug Discovery Volume: 13, Pages: 92–93; Published online 31 January 2014



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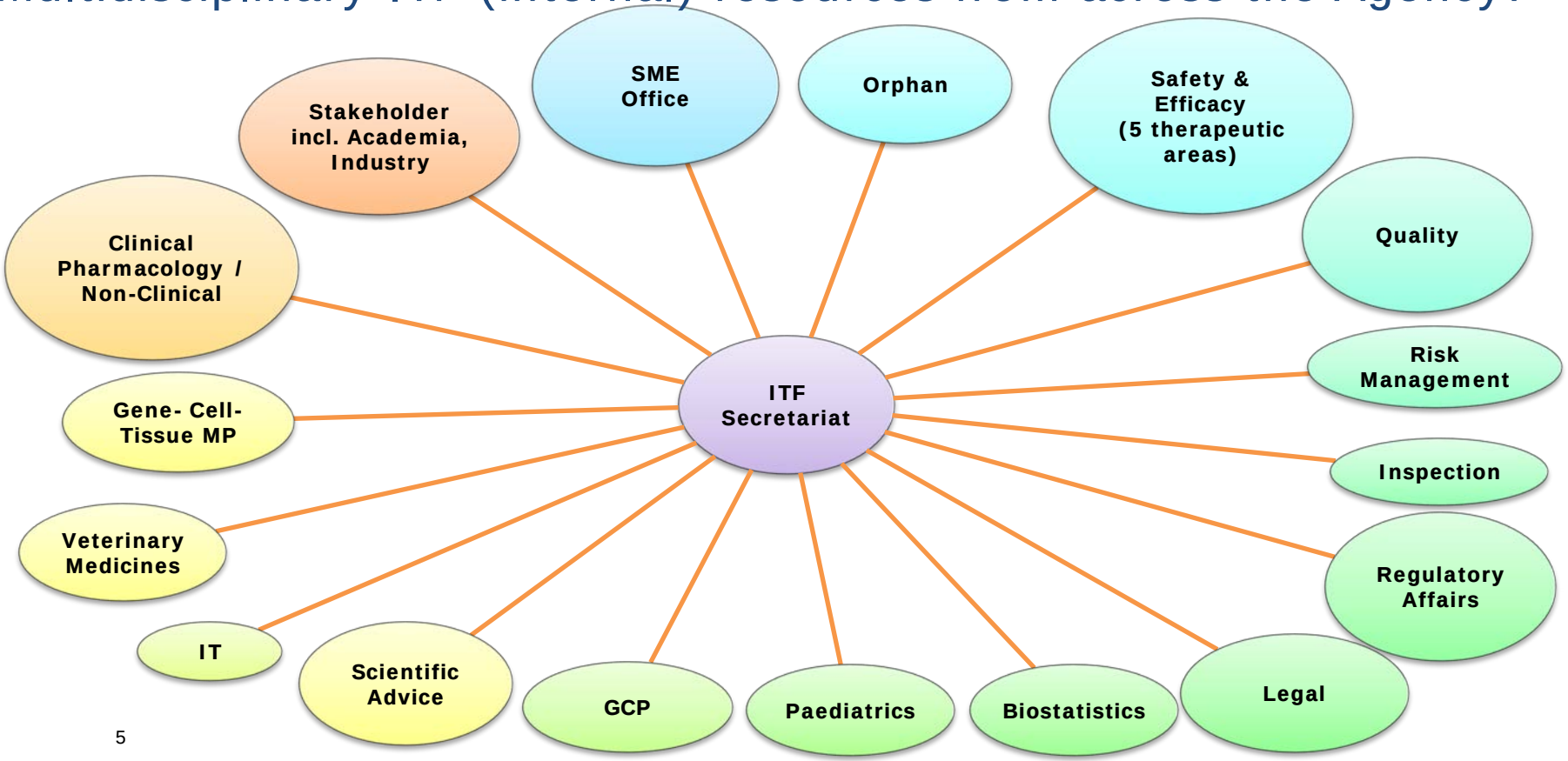
ITF users 2012-2015

Multidisciplinary ITF (internal) resources from across the Agency:



ITF (external) ITF resources from EU and beyond:

- EU **regulatory network i**ncluding **Committees, WPs and experts**
- **Research and other EU Public Institutions** (Karolinska, Italian Nano Centre, Max-Planck, Frauenhofer)
- **EU Institutions** e.g. Joint Research Centre, EFSA, ECHA, EDQM, DG Research, -Sante, -Growth
- Expertise from International Regulators, e.g. FDA, PMDA/MHLW, HC, Swissmedic, TGA
- International Institutions (US-Nano Characterisation Laboratory, Mayo Clinic)
- Other bodies within the EU (ECDC, Medical device authorities)

Main tasks of the Innovation Task Force (ITF)

- Coordination of ITF briefing meetings
- ATMP classification review
- Art. 57 Scientific Opinion

→ With focus on:

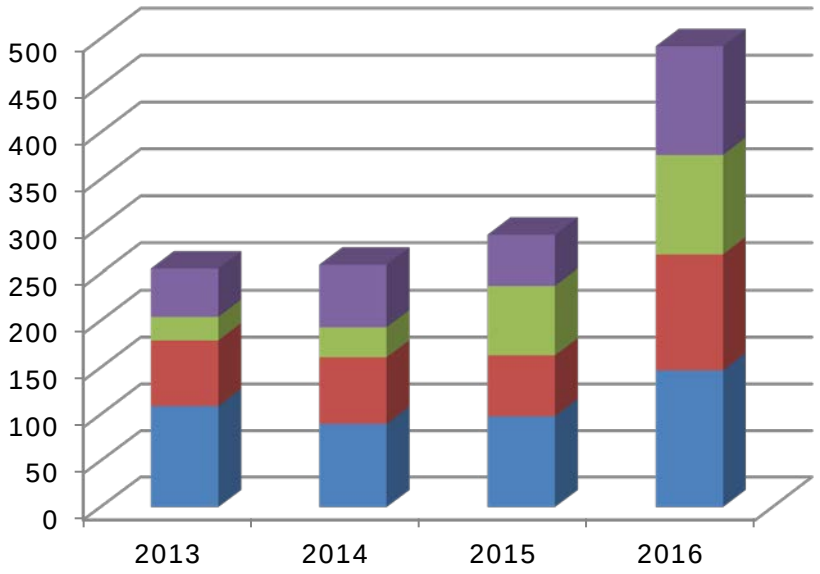
Emerging therapies and technologies

e.g. Nanomedicines, Synthetic Biology, Epigenetics, Biomaterial, Health technologies (e- and m-health)

Borderline and combination products

e.g. devices, cosmetics, food

Involvement in ITF Briefing Meetings (internal and external):



Year of meetings	2013	2014	2015	2016
Number of meetings	23	27	33	41
ITF attendees	51	66	54	116
EMA attendees (non ITF)	25	32	74	106
WP experts from EU Regulatory Network	70	71	65	123
Industry attendees	109	90	98	147
Total	255	259	291	492

Impact of Innovation Task Force on other EMA procedures:

92 ITF Briefing meetings organised between 2014 – 2016, of which **80%** were submitted by **academia, SMEs and consortia** (ITF support focus)

- 15% are Advanced Therapies (Gene, Cell, Tissue engineered products)
- 14% consider seeking EU Orphan Drug designation (rare diseases)
- 20% consider interaction with the EMA Paediatric Committee (PDCO)
- 30% of applicants consider applying a formal scientific advice request
- 11% consider Qualification of methodology (e.g. Biomarker qualification)
- 10% consider Marketing Authorisation Application within foreseeable future

ITF Outcomes: Intel gathering and dissemination

ITF Briefing meetings and minutes ATMP classifications Art. 57 opinions

- ITF-BM **Tracking database** as constant tracking and intel gathering tool
- Annual **intelligence gathering including stakeholder consultation**

- Monthly briefing and **feed-back** provided **to CHMP** and **other Committees**
- **Trainings organised (internal and external)**
- **Awareness sessions broadcasted** via EU-NTC
- Recommendations for the organisation of **workshops, expert meetings**
- Recommendations for **Drafting guidance**
- **Input in Horizon Scanning and EU Innovation Network**

Take home messages

- Regulators became gatekeepers and enablers
- The EMA is open to discuss scientific, regulatory and technical aspects of innovative developments
- The ITF is the Regulator's tool for informal early engagement and feed-back

Further information

See: http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000334.jsp&mid=WC0b01ac05800ba1d9

Contact us at: ITFsecretariat@ema.europa.eu

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Dealing with Unmet Medical Needs and Support to Innovation

Marketing authorisation under exceptional circumstances, Conditional Marketing Authorisation and Adaptive pathways

The EU medicines regulatory system and the European Medicines Agency: an introduction for international regulators and non-governmental organisations

Presented by Zigmars Sebris on 18 September 2017
Regulatory Affairs Office, Human Medicines Evaluation Division

Regulatory tools and initiatives aimed at unmet medical needs

Tools in legislation

Conditional Marketing authorisation

- Based on less comprehensive data and with obligation to generate these data post-authorisation

Marketing authorisation under exceptional circumstances

- When comprehensive data can not be general at all, subject to specific obligations

Accelerated assessment

- Reduced assessment time for products of major public health interest

Compassionate use

- Products made available to patients prior granting of marketing authorisation

Other tools and initiatives

PRIME

- Support scheme with early and enhanced scientific dialogue

Adaptive pathways

- Scientific concept of medicines development and data generation

Innovation task force

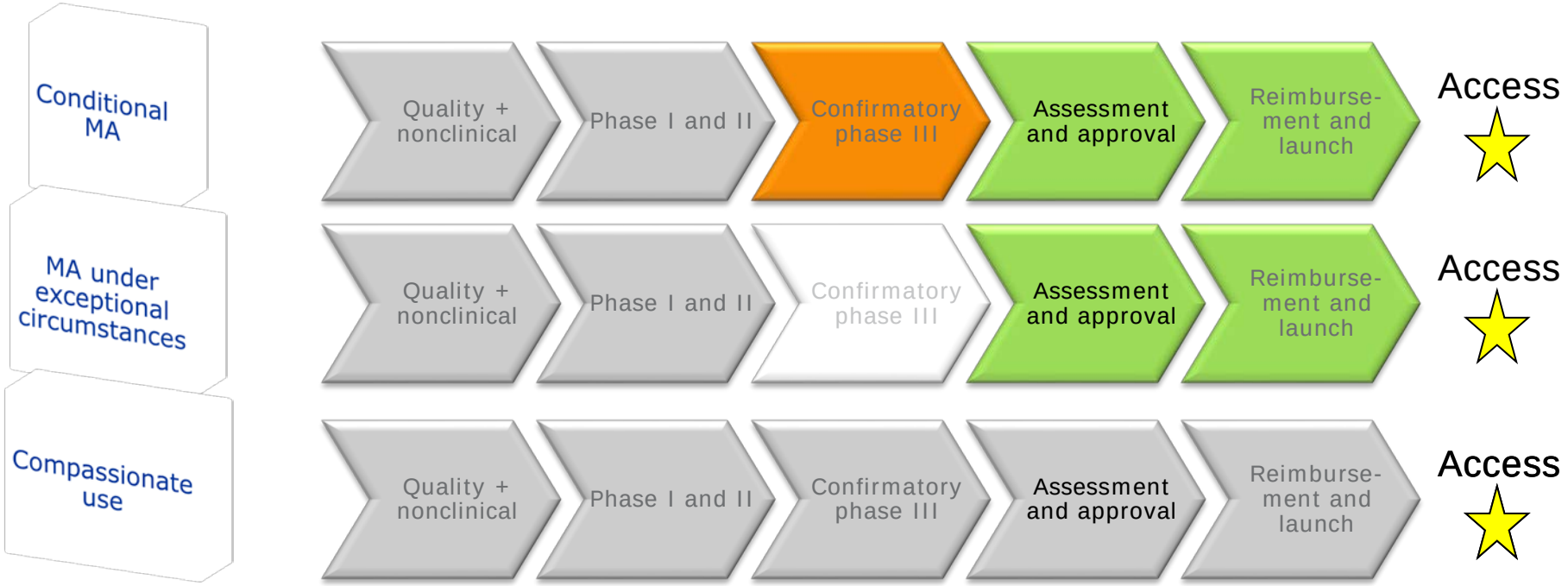
- Forum for informal early dialogue

Etc.

- Other tools and initiatives

Dealing with Unmet Medical Needs - CMA, MA under exceptional circumstances, Adaptive Pathways

Tools in the Legislation



Conditional Marketing authorisation

Comprehensive data not yet available

Scope (at least 1)

- Seriously debilitating or life-threatening diseases
- Emergency situations
- Orphan products

Criteria (all)

- Positive benefit-risk balance
- Comprehensive data can be provided after authorisation
- Unmet medical needs will be addressed
- Benefits of immediate availability outweigh the risks

'**unmet medical needs**' means a condition for which there exists no satisfactory method of diagnosis, prevention or treatment authorised in the Community or, even if such a method exists, in relation to which the medicinal product concerned will be of major therapeutic advantage to those affected

Commission Regulation (EC) No 507/2006

Marketing Authorisation Under Exceptional Circumstances

Impossible to provide comprehensive data on the efficacy and safety of the medicinal product under normal conditions of use, for objective, verifiable reasons

Criteria (at least 1)

the indications are encountered so rarely that it be expected to obtain comprehensive evidence, or

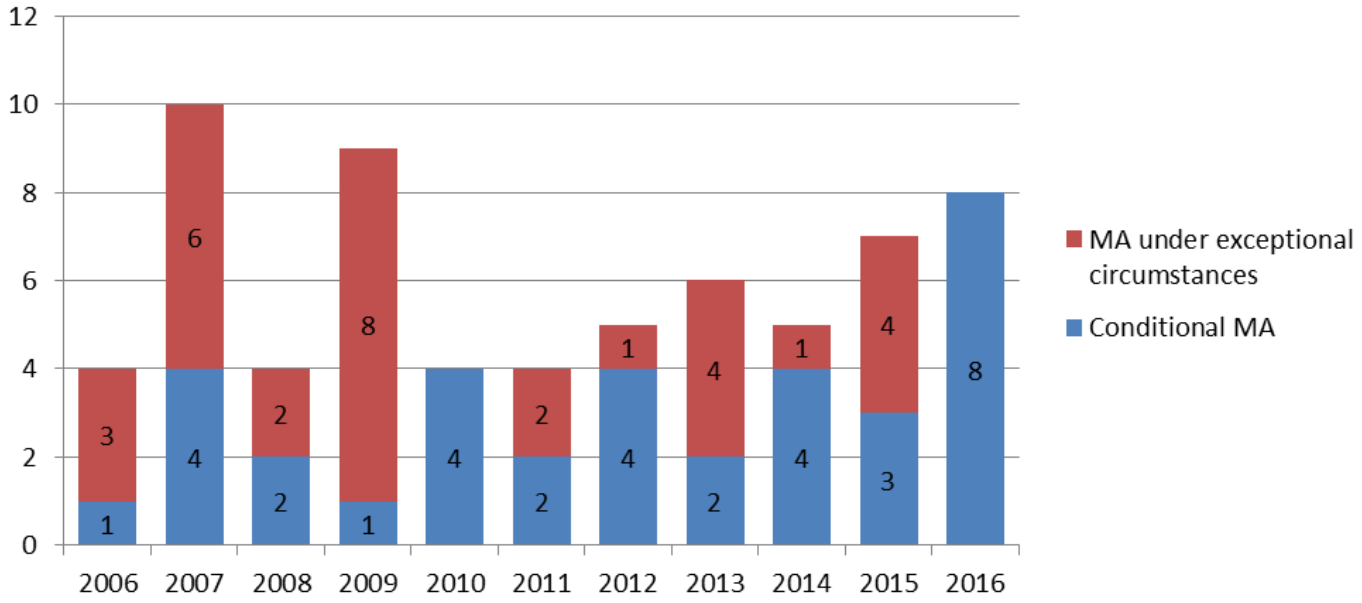
in the present state of scientific knowledge, comprehensive information cannot be provided, or

it would be contrary to generally accepted principles of medical ethics to collect such information

Comparison

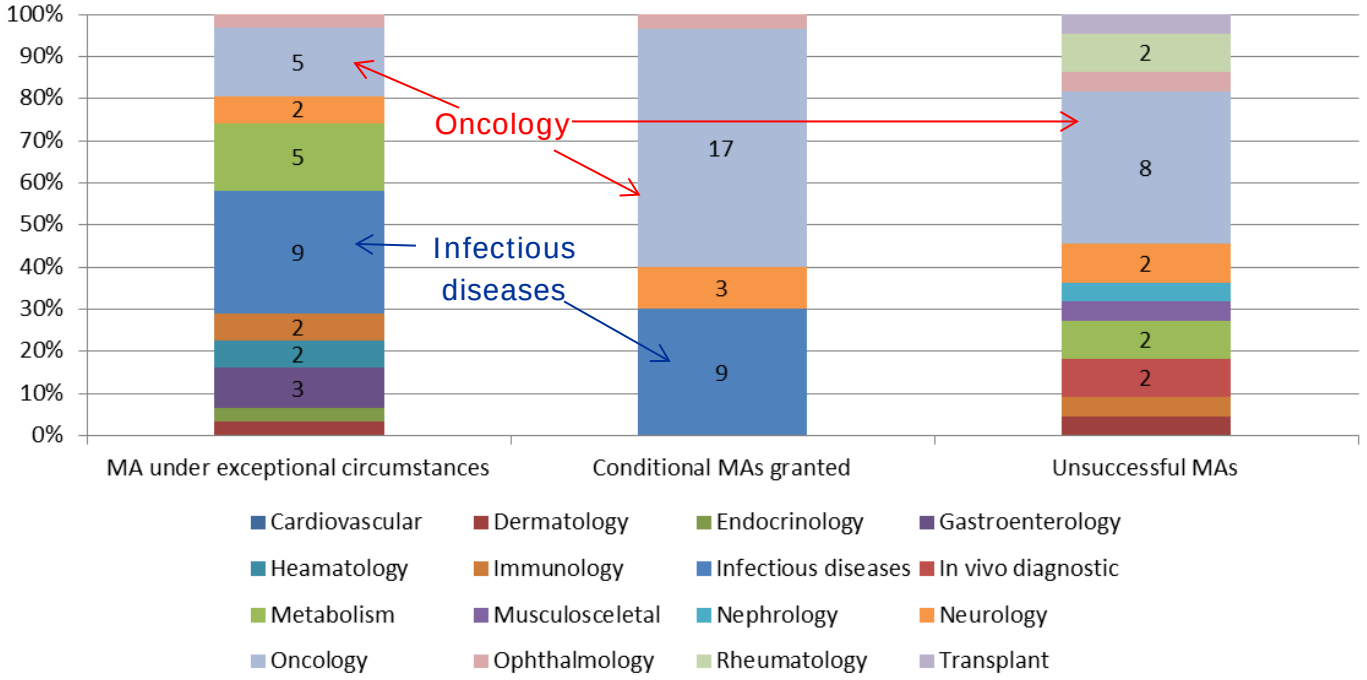
Conditional MA	MA under exceptional circumstances
Comprehensive data **after authorisation**	Comprehensive data **not possible**
To later **switch** to 'standard' MA	To remain such **indefinitely**
Valid for **1 year** only (annual renewals)	Valid for **5 years** (renewable) + annual re-assessment
Possible in centralised procedure only	Possible in all registration procedures
Specific Obligations + may have conditions	**Specific obligations** + may have conditions

Last 10 years in numbers

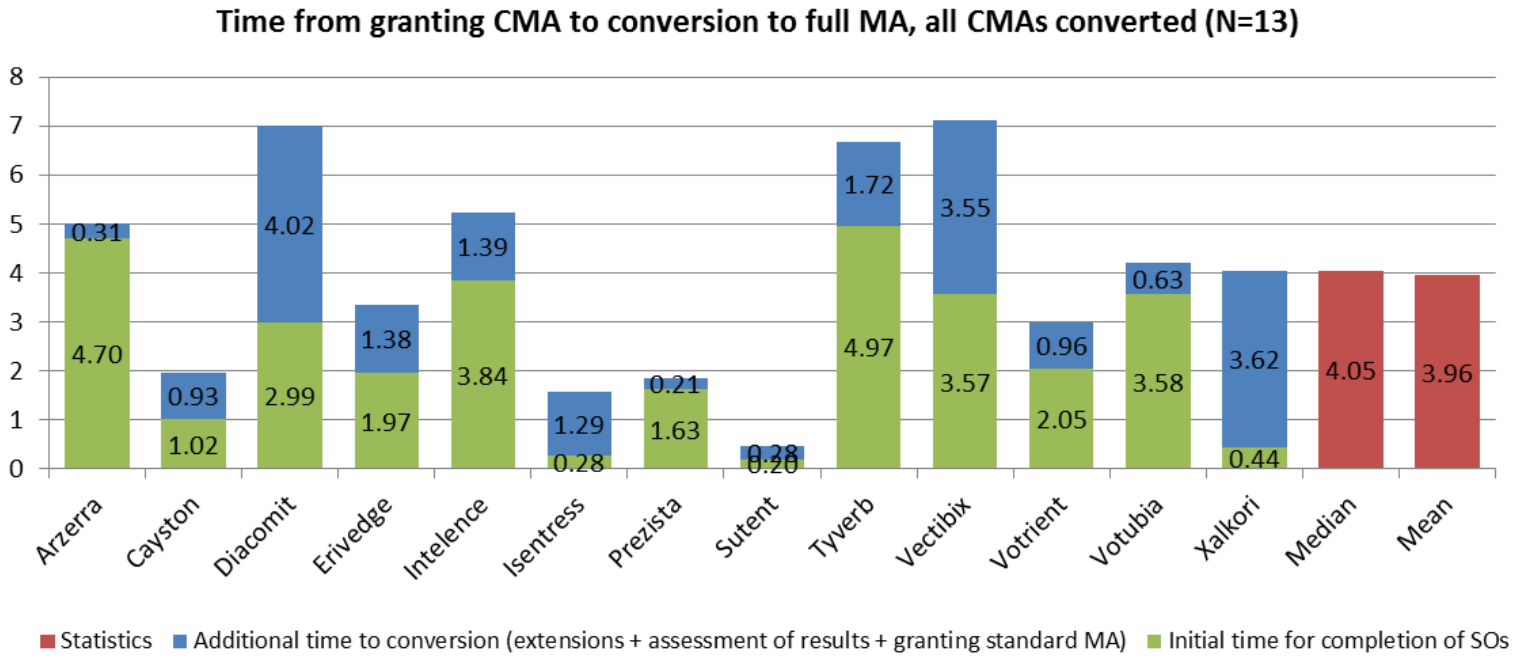


Data updated with DLP 31 Dec 2016

Therapeutic areas



Conversion of Conditional MA to standard MA



On average **within 4 years** a conditional MA is converted into a standard MA

Data updated with DLP 31 Dec 2016

Conditional MA – everolimus

Votubia

For subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis complex

In patients not amenable to surgery

Unmet need: lack of non-invasive pharmacological treatments (no satisfactory method)

Initial evidence

Phase II single arm study (28 pts)

Ongoing phase III double blind placebo controlled randomised study (117 patients)

Reduction in SEGA volume demonstrated

Safety database: 143 pts

Specific obligations

Final results from main part of the pivotal phase III study

Long-term follow up from both pivotal studies

No changes in scope or deadlines

Conditional MA – raltegravir

Isentress

For treatment of HIV-1 infection

In case of HIV-1 replication despite ongoing anti-retroviral therapy

Unmet need: activity against HIV that is resistant to many other antiretroviral agents

Approved following accelerated assessment

Initial evidence

16 and 24 week results from two ongoing phase III studies (350 + 349 pts.)

+ supportive studies

Results showed that addition of raltegravir can provide suppression of HIV replication that is sustained to 24 weeks

Safety database: 1214 pts.

Specific obligations

48 week data from pivotal studies

Implementation of a plan to monitor resistance and of observational safety data collection

Due date for one obligation extended by 10 months to duet o need to revise proposal based on CHMP comments

MA under Exceptional Circumstances – lomitapide

Lojuxta

For patients with homozygous familial hypercholesterolaemia

Rare, life-threatening autosomal dominant genetic disease characterised by marked elevations in low density lipoprotein cholesterol

Criterion: rarity of the indication (comprehensive data cannot reasonably to be expected)

Initial evidence

Unblinded single arm study (29 patients in primary analysis)

Complemented by short term supportive studies

Consistent lipid lowering efficacy shown

Total safety database: 845 patients

Specific obligations

Long term prospective observational study to systematically collect information on the safety and effectiveness outcomes

- Annual reports provided each year
- No completion date for the activity

Information on EMA's website

Home | Find medicine | Human regulatory | Veterinary regulatory | Committees | News & events

Human medicines
European public assessment reports

Home ▸ Find medicine ▸ Human medicines

European public assessment reports

Name	Active substance	Therapeutic area	Date of authorisation / refusal	▼	O	C	E	⚠	Status
Natpar	parathyroid hormone	Hypoparathyroidism	2017-04-24	▼	O	C			Authorised
Alecensa	alectinib hydrochloride	Carcinoma, Non-Small-Cell Lung	2017-02-16	▼		C			Authorised
Ocaliva	obeticholic acid	Liver Cirrhosis, Biliary	2016-12-12	▼	O	C			Authorised

Name	Active substance	Therapeutic area	Date of authorisation / refusal	▼	O	C	E	⚠	Status
Brineura	cerliponase alfa	Neuronal Ceroid-Lipofuscinoses	2017-05-30	▼	O		E		Authorised
Dinutuximab beta Apeiron	dinutuximab beta	Neuroblastoma	2017-05-08	▼	O		E		Authorised

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

1 March 2016
EMA/531801/2015
Human Medicines Research and Development Support Division

Development support and regulatory tools for early access to medicines

The EU pharmaceutical legislation includes a number of provisions in Regulation (EC) No 726/2004 aimed at fostering patients' early access to new medicines that address public health needs and are eligible to the **centralised procedure**, such as:

- **accelerated assessment** procedure which reduces the timeframe for review of an application for marketing authorisation from a maximum of 210 days to 150 days for medicinal products of major public health interest and in particular from the viewpoint of therapeutic innovation,
- for certain categories of medicinal products, the possibility to obtain a **conditional** marketing authorisation on the basis of less complete data than is normally the case and subject to specific obligations and additional comprehensive data to be provided post-authorisation. Conditional marketing authorisations are valid for one year on a renewable basis,
- the possibility for a **compassionate use opinion** by the CHMP defining at European level the criteria and conditions for use of medicinal products which are made available to patients through national patients' access programmes (prior to a marketing authorisation).

To optimise the use of the above regulatory tools, EMA has launched the **PRIME** scheme to support development of medicinal products of major public health interest through early and enhanced scientific and regulatory dialogue. This tool targets support to certain type of products eligible for accelerated assessment and falling within the scope of the centralised procedure. It builds also on existing regulatory tools in place within the European Union (EU) legal framework, including scientific advice/protocol assistance.

The table overleaf provides a high-level overview of the above legislative and development support tools to help sponsors identify when and how to use them.

However, there are a number of other development support activities, not covered in this tabular overview, carried out by the Agency including the following:

- The Innovation Task Force (ITF) which is a multidisciplinary group providing a forum for informal early dialogue with applicants, in particular micro, small and medium enterprises (SMEs) and academic sponsors, to proactively identify scientific, technical and regulatory issues related to emerging therapies and technologies.

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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Special Support: The Small and Medium enterprise (SME) initiative

The EU medicines regulatory system and the European Medicines Agency: an introduction for international regulators and non-governmental organisations

Presented by: Leonor Enes
SME Office; Stakeholders & Communication Division; European Medicines Agency

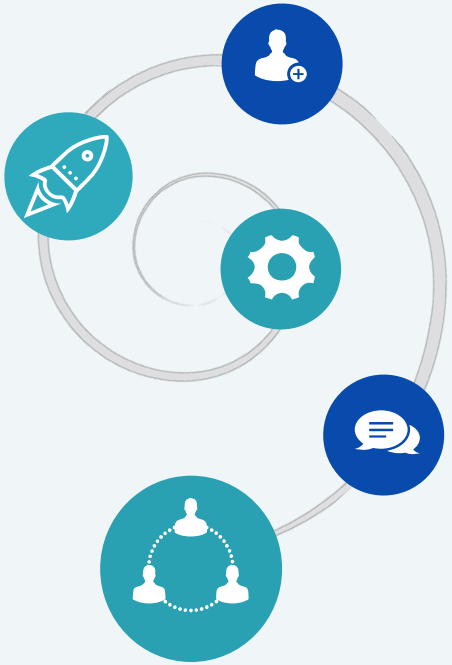
1 SME Regulation

2 What the SME Office does

3 Support to innovation – stats

4 Marketing authorisations

1 SME Regulation



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

***Aim**: to promote innovation and the development of new medicines for human and veterinary use by SMEs*

SME Office launch in December 2005

- A single contact point
- Assistance to SMEs
  Regulatory, administrative and procedural support
- Facilitates communication
  With SMEs in veterinary and human pharma sector
- Coordinating & networking
  Working closely with EU, SME partners and stakeholders

2 What the SME Office does

- Assignment of SME status
- Regulatory Assistance & SME briefing meetings
- Fee Incentives
- Translation Assistance
- Training and Awareness
- Partnering & Networking
  SME Register
- Reporting and Planning
  SME Office annual report 2016
  SME action plan (2017-2020)

Assignment of SME status: SME definition

- Commission Recommendation 2003/361/EC defines micro, small and medium-sized enterprises

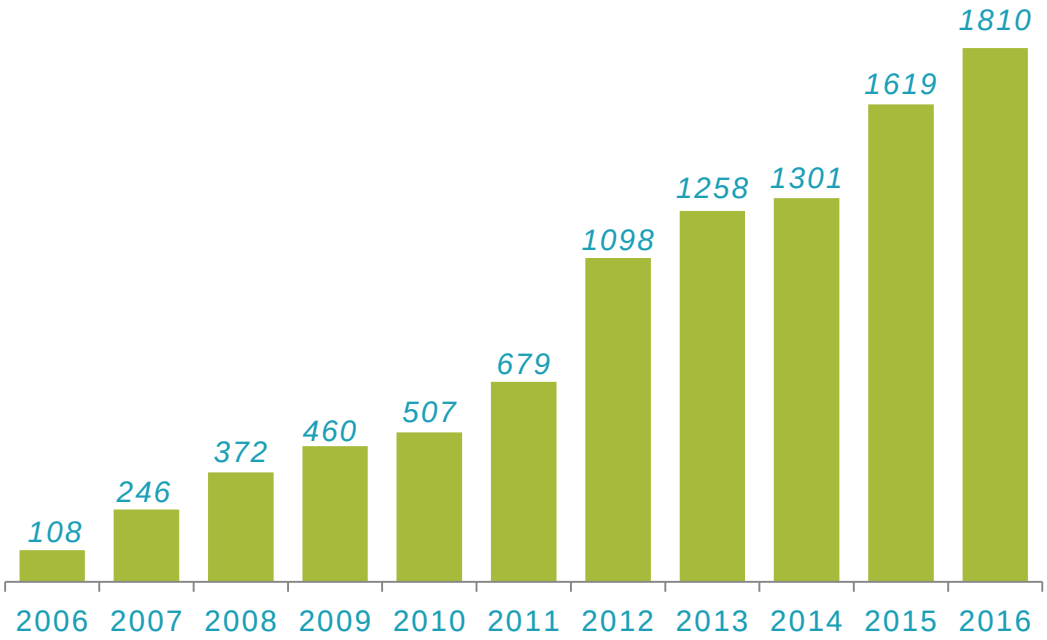
- To qualify for SME status companies must:

1) be established in the EEA

2) meet the following thresholds:

Enterprise category	Headcount: annual work units (AWU)	Annual turnover	or	Annual balance sheet total
Medium-sized	< 250	≤ EUR 50 million	or	≤ EUR 43 million
Small	< 50	≤ EUR 10 million	or	≤ EUR 10 million
Micro	< 10	≤ EUR 2 million	or	≤ EUR 2 million

Registered SMEs

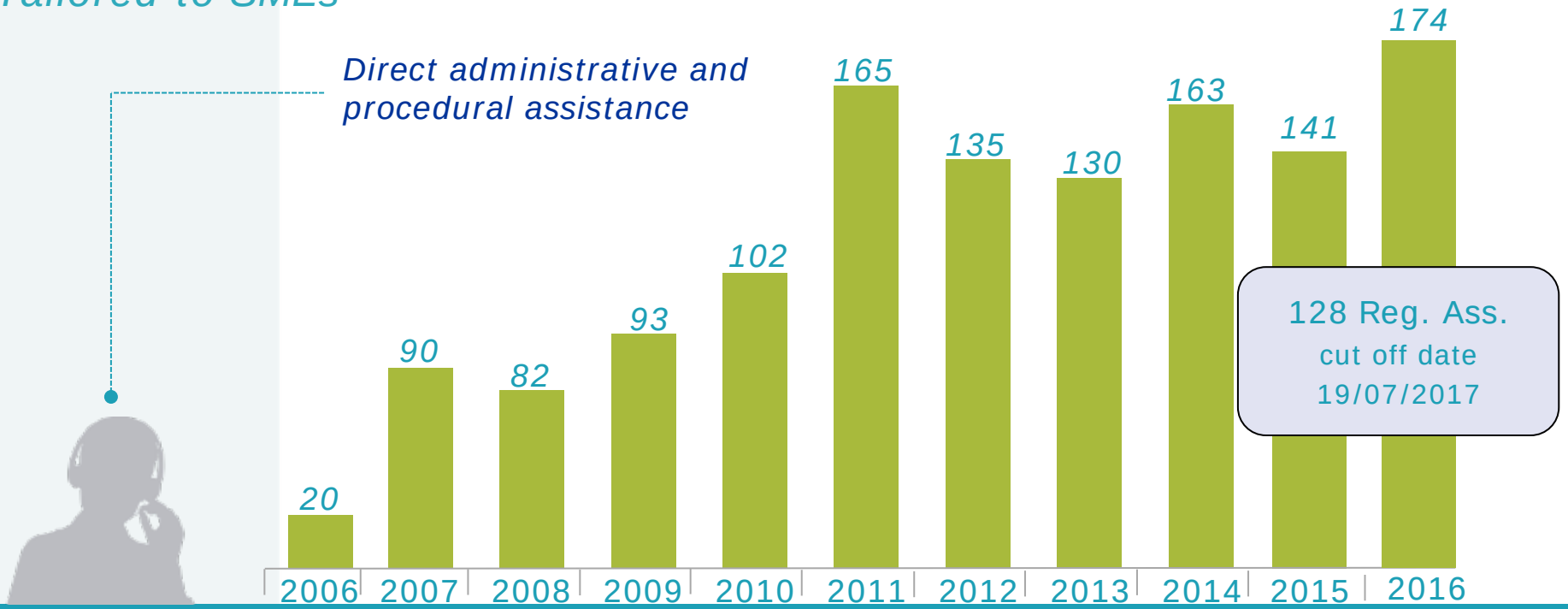


- From 28 countries across EU
- Top 3 countries : UK, Germany and France
- 41% micro, 34% small, 25% medium
- Majority human (78%), 4% vet, 5% human/vet & 13% service providers
- Information on registered companies available in the SME public register

Regulatory assistance to SMEs

Tailored to SMEs

Direct administrative and procedural assistance



SME fee incentives

- Fee reductions and exemptions for scientific advice, scientific services, inspections & establishment of maximum residue limits
- Deferrals of the fee payable for an application for marketing authorisation or related inspection
- Conditional fee exemption
- Fee reductions and exemptions for post-authorisation procedures and pharmacovigilance activities
- Waiver of the MedDRA licensing fee for micro and small companies

Full details on all fees and fee reductions are available in: Explanatory note on general fees payable to the European Medicines Agency and Explanatory note on pharmacovigilance fees payable to the European Medicines Agency

Training & awareness for SMEs

Provide training
&
ease the access to regulatory information

Info days

Annual or Biannual regulatory training course tailored for SMEs

Supporting innovative medicines' development and early access; 17/11/2017

Newsletters

Circulated quarterly; published on the EMA Website.

Announcements

Information sent by email to SMEs and stakeholders

SME User Guide

Updated regularly

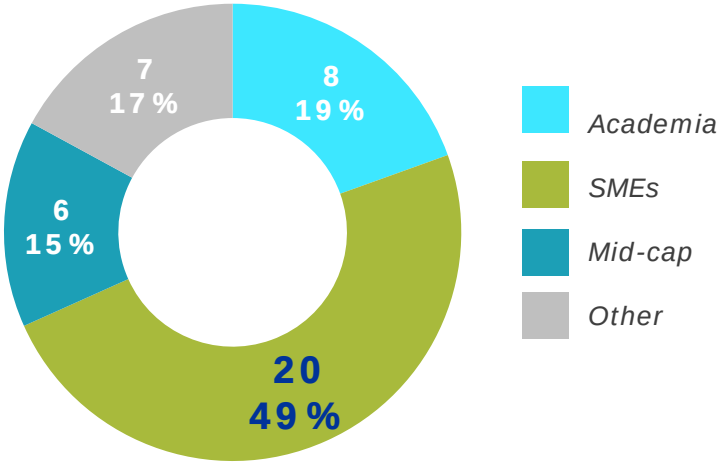
3 Support to innovation – *SME briefing meetings*

- provides a platform for early dialogue with SME to discuss regulatory strategy of medicinal product development and navigate the range of procedures and incentives available
- multidisciplinary group, co-operation with other EMA offices (scientific advice, paediatrics, orphans, regulatory affairs, etc)
- open to medicinal products for human and veterinary use
- free of charge

2005 - 2015: **65**
2016: **13**
2017 (Jan - July): **9**

Support to innovation - *Innovation Task Force & ATMPs*

ITF briefing meetings (2016)



20 out of 41 ITF meetings in 2016 were for SMEs applicants

ATMPs

- Certification of quality and non-clinical data for ATMPs intended for human use: for SMEs only

6 *ATMP certifications* ***FINALISED (till end 2016)***

Support to innovation - *Scientific Advice & Protocol Assistance*

2016

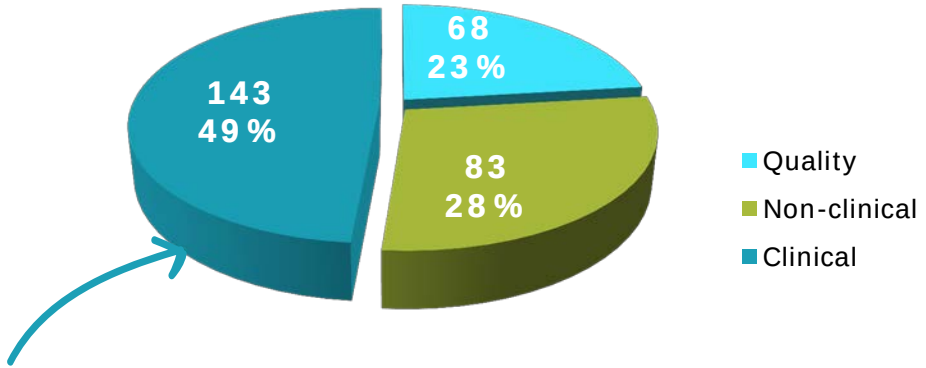
177 SA and PA submitted by SMEs

26 % ***of all SA by SMEs***

46 % ***of all PA by SMEs***

Human medicines

Scope of scientific advice - human products - SMEs (2011-2016)



In 2016, ***54 %*** *of clinical questions in SA were related to confirmatory trials*

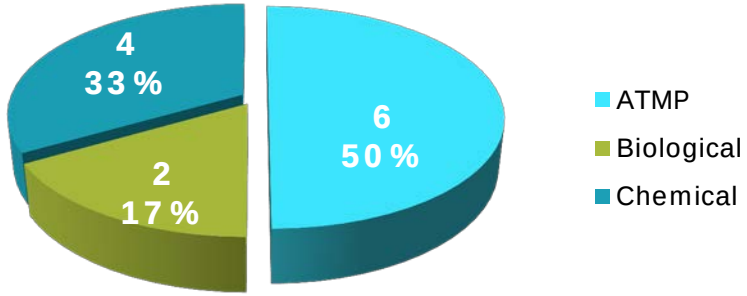
6 ***Parallel HTA Advice by SMEs***

Support to innovation - *PRIME (PRIority MEdicines)*

Key figures (2016-July 2017)

- 121 applications received up to July 2017 (half by SMEs)
- 28 medicines granted PRIME of which **12** are SMEs
- 1 SME early entrance (SMEs can apply earlier on the basis of compelling non-clinical data and tolerability data from initial clinical trials)
- additional SME fee reductions for scientific advice

Human products
SMEs (2016 – July 2017)



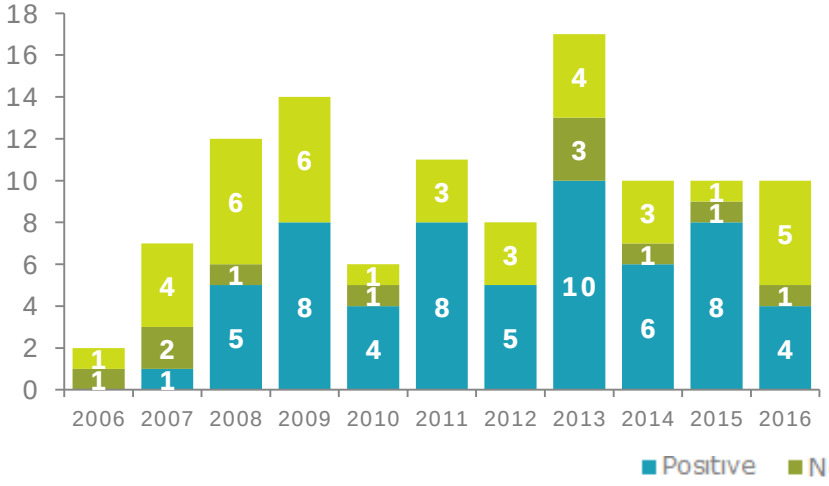
Therapeutic areas:

- Oncology
- Immunology
- Haematology
- Gastroenterology
- Neurology
- Infections diseases

4 Marketing authorisations

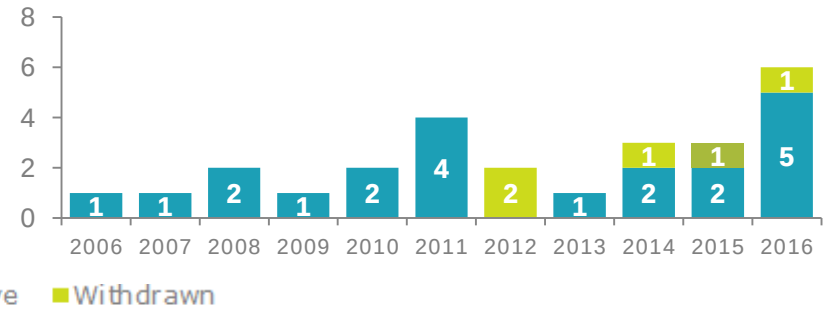
SME Applicants – MAA outcome by year (2006-2016)

Human medicines



27 MAA submitted by SMEs in 2016

Veterinary medicines



9 MAA submitted by SMEs in 2016

Take home messages

- SMEs are a source of pharmaceutical innovation
- The EMA remains committed to fostering an environment which provides incentives to SMEs: awareness of the EMA SME initiative, training and education, supporting innovative medicines' developments, and further engaging with SMEs, partners and stakeholders

Further information

See: supporting SMEs

Contact us at: sme@ema.europa.eu