Agenda

1. Introduction
2. Innovation Task Force
3. SME Office
4. Orphan Medicinal Products
5. Paediatric Medicines
6. Scientific Advice
7. Early Access Tools
8. Closing remarks

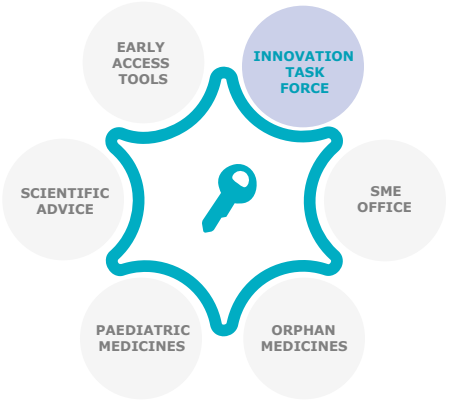
Platforms for early dialogue with regulators

ENTRANCE TO EMA

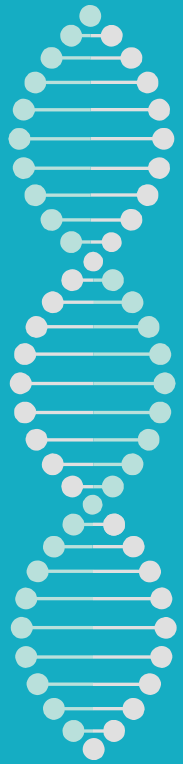
The various entry doors for regulatory dialogue

Platforms for early dialogue with regulators

INNOVATION TASK FORCE



Early dialogue - ITF



- Earliest entry door for products based on emerging science
- Multidisciplinary EMA group set up in 2001
- Focus on emerging therapies/technologies & borderline products
- A "soft landing zone"

MAIN TASKS

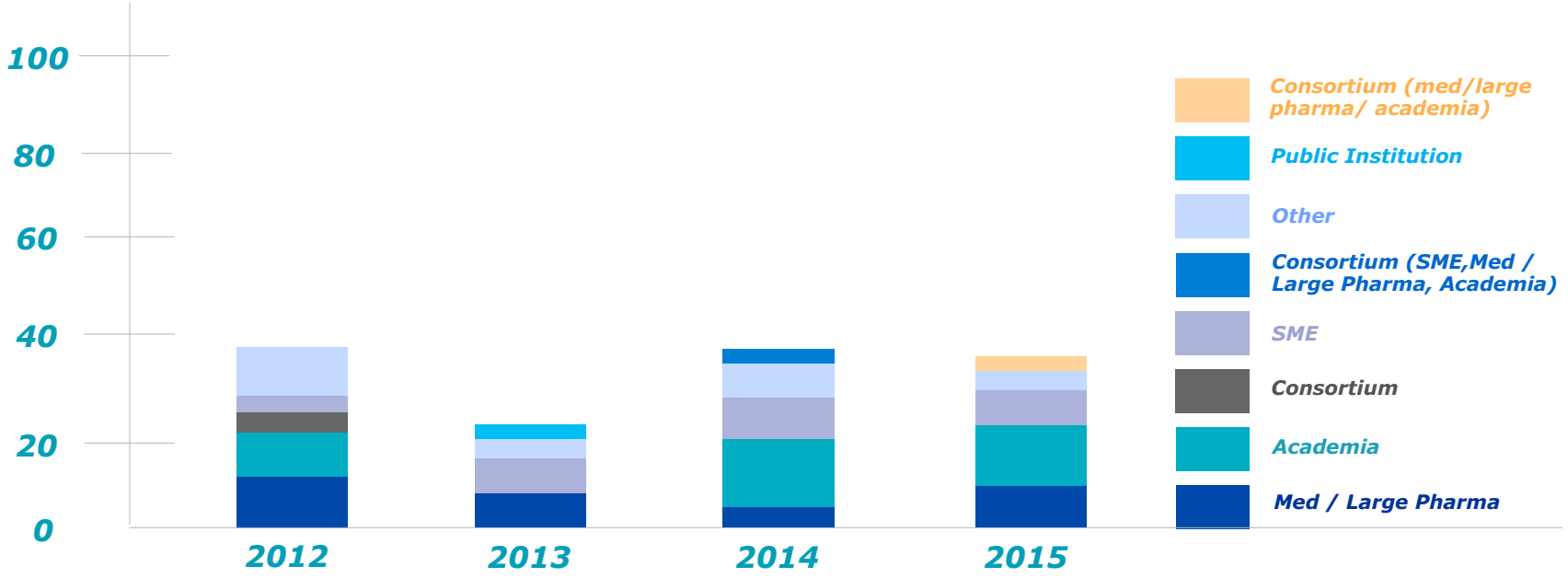
- *Briefing meetings with sponsors*
- *Regulatory advice on classification for borderline products*
- *Preparation for future formal steps*

ITF briefing meetings

- complement and reinforce existing formal regulatory procedures *(e.g. biomarker qualification, ATMP classification, ATMP certification, designation of orphan medicines, CHMP scientific advice, etc.)*
- facilitate the informal exchange of information and the provision of guidance early in the development process
- involve EU regulatory network (*Committees and Working Parties experts*)

ITF BM cover *regulatory*, *technical* and *scientific* issues arising from *innovative* medicines development, *new technologies* and *borderline* products.

*ITF Briefing meetings users – **CHANGE OVER TIME***



Entry through ITF

- Individual or company (big pharma or SME) ***within*** or ***outside*** EEA
- ***Emerging*** therapies/technologies or Borderline therapeutics
- Areas where there is ***no established EMA*** scientific, legal and regulatory ***experience***
- Request form + briefing document
- ***Free*** entry (no fee for ITF consultation)

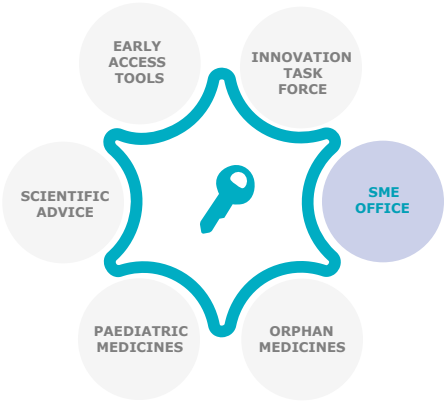
itfsecretariat@ema.europa.eu

Further information:

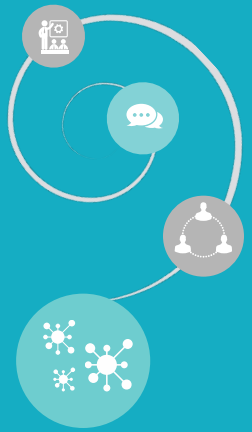
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000334.jsp

Platforms for early dialogue with regulators

SMALL & MEDIUM ENTERPRISES



*Early dialogue – **SME OFFICE***



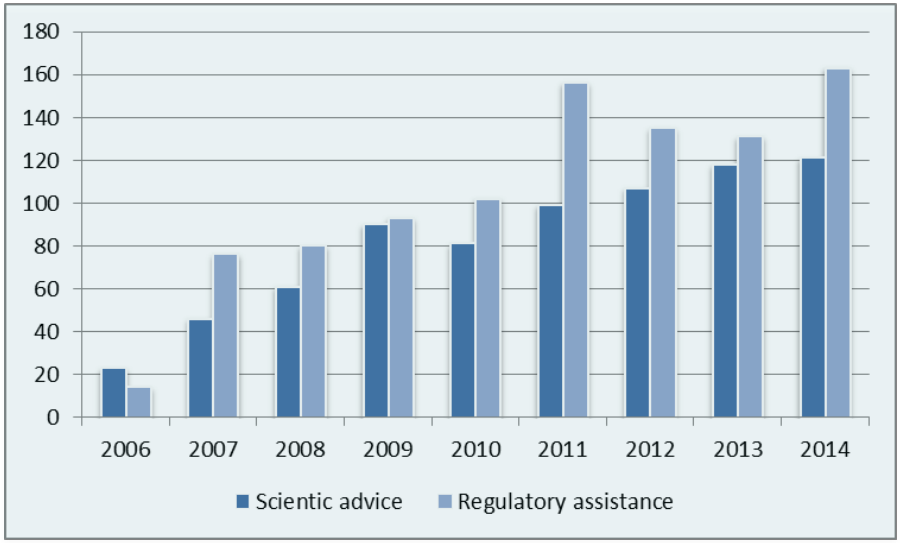
- SME office (one-stop shop)
- Regulatory Assistance
- Fee reductions and deferrals
- Certification for ATMPs
- Translation of product information
- SME User Guide / News Bulletins / Workshops

Aim to stimulate ***INNOVATION***
& ***DEVELOPMENT*** of ***NEW MEDICINES***

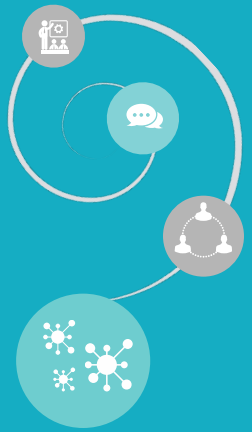
Experience with SMEs (year end 2014)

- *1450 companies assigned SME status*
- *From 27 countries across EEA*
- *45% micro, 38% small, 17% medium*
- *Majority human (72%), 5% vet, 6% human/vet & 17% service providers*
- *Public register of companies launched in 2010*

Scientific advice & Regulatory assistance



Entry through SME Office



- Enterprise ***established in EEA***
- ***EU definition*** of an SME: < 250 employees & ≤ € 50 mil in turnover or ≤ € 43 mil in balance sheet
- For medicinal ***products eligible for centralised procedure*** (except for scientific advice)
- Regulatory ***assistance*** / Regulatory ***briefing meeting***
- ***Free*** entry (no fee for SME office consultation)

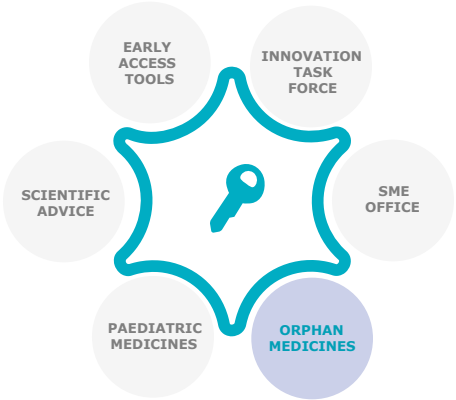
sme@ema.europa.eu

Further information:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000059.jsp&mid=WC0b01ac05800240cc

Platforms for early dialogue with regulators

ORPHAN MEDICINES



*Early dialogue – **ORPHAN MEDICINES***

EU Orphan legislation since 2000

- 10 years of Market Exclusivity
- Protocol assistance throughout development
- Fee reductions
- Access to centralised procedure

INCENTIVES

Patients affected by ***RARE DISEASES*** have the ***SAME RIGHTS*** as fellow citizens

Orphan designation criteria

Rare Condition < 5 per 10,000 or
Insufficient return on investment ?

Serious condition or life threatening?

Existence of satisfactory methods?

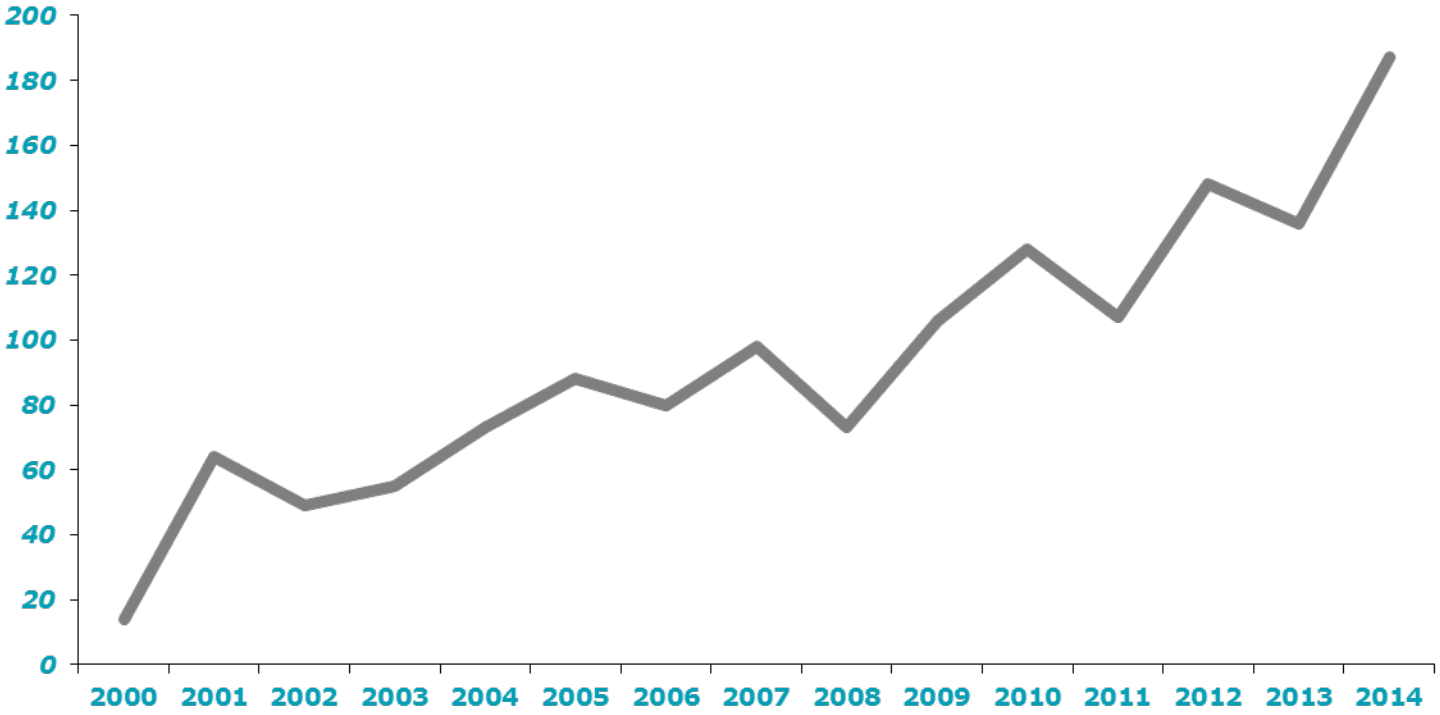
NO?

YES + Significant Benefit?

ORPHAN DESIGNATION

PRE-SUBMISSION MEETINGS
with Orphan Medicines team

Status of Orphan Designations



Entry for Orphan Medicines

- Individual or company ***established*** in ***EEA***
- For medicinal products meeting ***orphan criteria***
- Opportunity for pre-submission ***dialogue***
- ***Protocol assistance*** once designated
- ***Free*** entry (no fee for orphan designation)

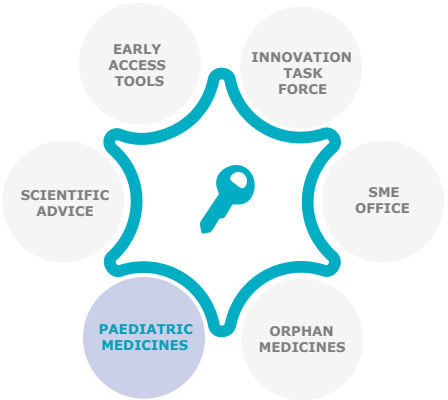
orphandrugs@ema.europa.eu

Further information:

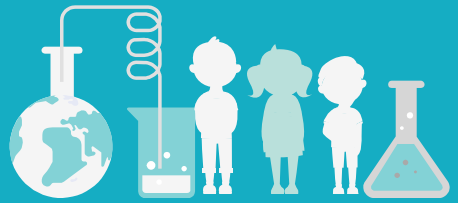
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000029.jsp&mid=WC0b01ac058002240ce

Platforms for early dialogue with regulators

PAEDIATRIC MEDICINES



Early dialogue – PAEDIATRIC MEDICINES



Obligation

- To study new medicinal products or new indications in children

Reward

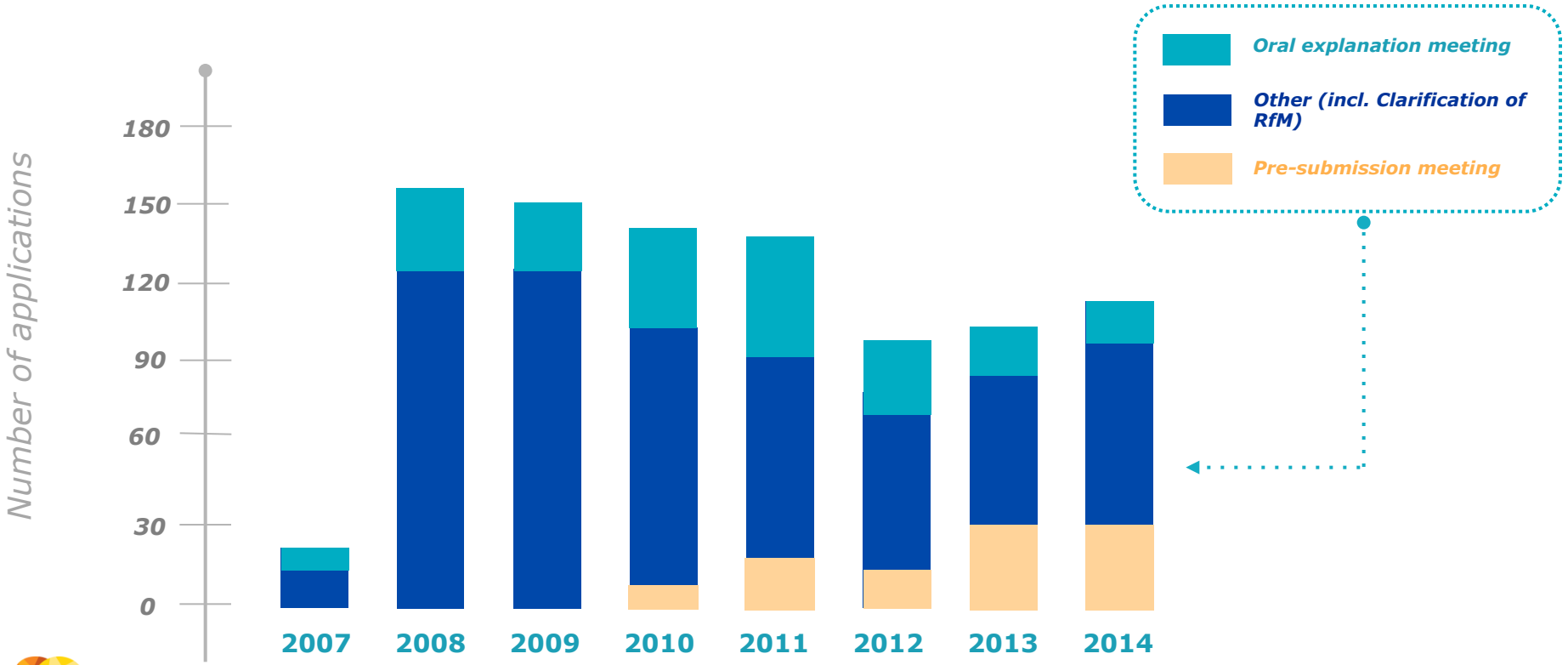
- Extension of supplementary patent certificate
- Extra market exclusivity for orphan (2 years)

Agree on Paediatric Invistigation Plan

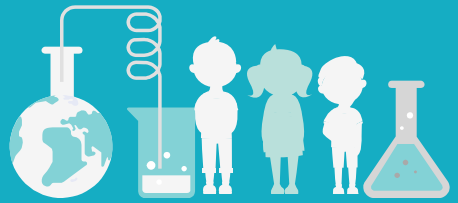
- PIP outlines timing & measures to be undertaken
- Agreed by Paediatric Committee (PDCO)
- Deferral or Waiver, if applicable
- Compliance check at time of marketing application

To increase research into, availability of, and information on
MEDICINES FOR CHILDREN

Paediatric Medicines – Total Number of company interactions



Entry for Paediatric Medicines



- ***Individual*** or ***company*** within or outside the EEA
- Opportunity for ***early paediatric interaction meeting*** (pilot)
- Pre-submission ***dialogue*** with paediatric team
- ***Scientific Advice*** (free of charge) for paediatric indications
- ***Free*** entry (no fee for PIP, or waiver)

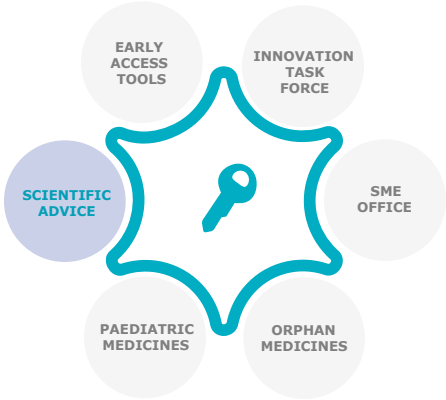
paediatrics@ema.europa.eu

Further information:

http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000023.jsp&mid=WC0b01ac058002240cd

Platforms for early dialogue with regulators

SCIENTIFIC ADVICE



*Early dialogue – **SA & PA***

Scientific Advice & Protocol Assistance

- Scientific Advice can be provided on ANY scientific question
  - → Quality, non-clinical and clinical
- At any time point of the development
  - → Early advice with subsequent follow-up is recommended
- Broad advice, conditional approval/exceptional circumstances
- Parallel advice with HTAs
- Qualification of biomarkers and other novel methodologies

EU view on scientific issues not covered by or deviating from existing guidance

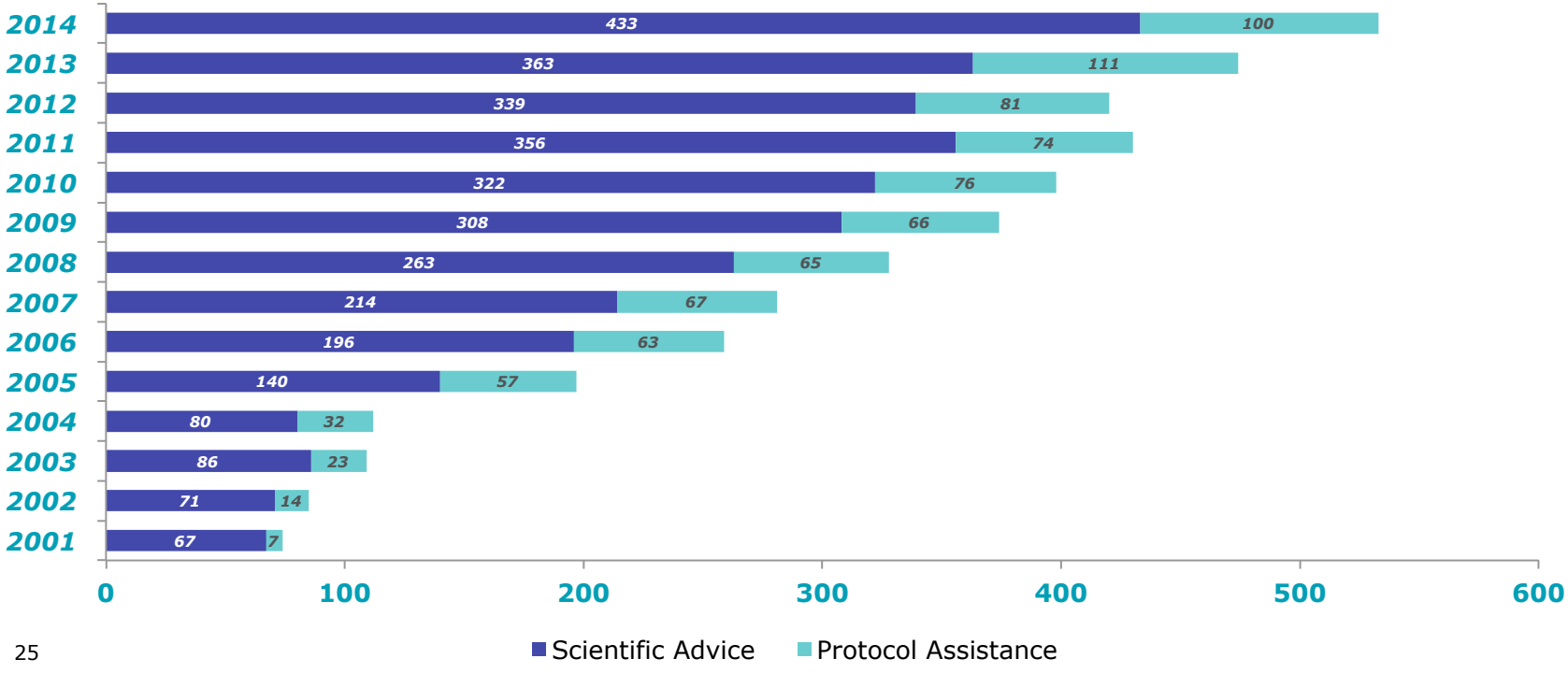
Advice on development & agreement of future strategy

Working party of CHMP

Scientific advice

- ***Voluntary***, not mandatory procedure
- ***Pre-submission meetings*** available
- Companies ***ask questions***
- ***Responses*** are ***prepared*** and discussed
- In ***50%*** of the cases a ***face-to-face meeting*** with the company is organised
- ***Written responses***, adopted by the licensing committee, sent to the company: scientific advice letter
- ***Short process***: 40 days or 70 days

Scientific Advice main activity so far -
scientific advice and protocol assistance for orphan drugs



Entry through Scientific Advice

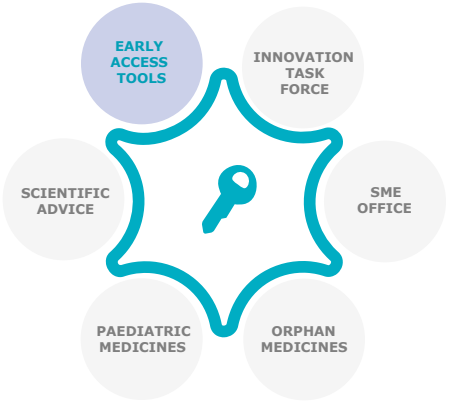
- Individual or company ***within*** or ***outside*** the ***EEA***
- For all ***medicinal products*** (*irrespective of eligibility for centralised procedure*)
- ***Briefing document*** outlining questions on development/ future strategy
- For advice on, or qualification of, ***novel methodologies***
- Fee ***payable*** (fee reductions for ATMPs, orphans, paediatrics, SMEs)

scientificadvice@ema.europa.eu

Further information:
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000049.jsp&mid=WC0b01ac05800229b9

New opportunities for dialogue

EARLY ACCESS TOOLS



Early access tools: *new opportunities for dialogue*

PRIME

New scheme for Dedicated and reinforced support

Major public health interest, unmet medical need

Relevant Q&A, scientific guidance and templates under development for launch

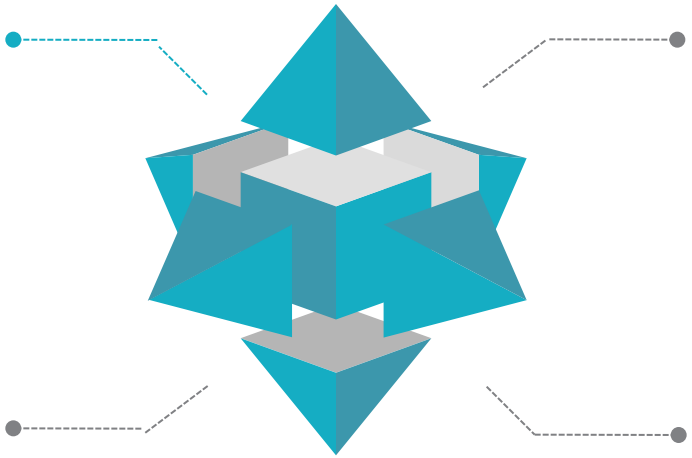
Accelerated Assessment

Ongoing revision of the guideline.

More detailed guidance on justification.

Optimisation of the assessment timetable.

Emphasis on the importance of early dialogue.



Adaptive Pathways

Pilot ongoing

ADAPT-SMART

Scientific concept of development and data generation.

Iterative development with use of real-life data.

Conditional MA

Ongoing revision of the guideline.

Emphasis on importance of prospective planning early dialogue.

Platforms for early dialogue with regulators

CLOSING REMARKS

Closing remarks

- Define regulatory strategy early in development
- Consider contact through ITF, SME, Orphan, if applicable
- Plan for PIP early in development
- Utilise available early access tools, where applicable
- Seek (multidisciplinary) scientific advice early on and seek follow-up advice as development proceeds

Thank you for your attention

Further information

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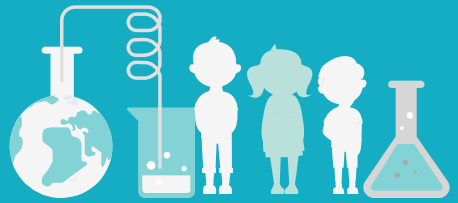
*Early dialogue – **PAEDIATRIC MEDICINES***



Paediatric investigation plan – a main tool of the regulation

- Research and development programme
- Basis for development and for authorisation of a medicinal product for children
- Generates paediatric-specific data necessary to demonstrate: Quality, Efficacy, Safety
- Includes details of the measures and their time lines
- To be agreed by the PDCO
- Binding on company (compliance check, submission validation)
- Deferral, if it is acceptable that data for children come after adults