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Online course

The Winding Road from a Brilliant Idea to Drug Approval

CSA STARS

Scientific Advice (European and National)

23-26 February 2021

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Science Programme Manager
Dutch Medicines Evaluation Board

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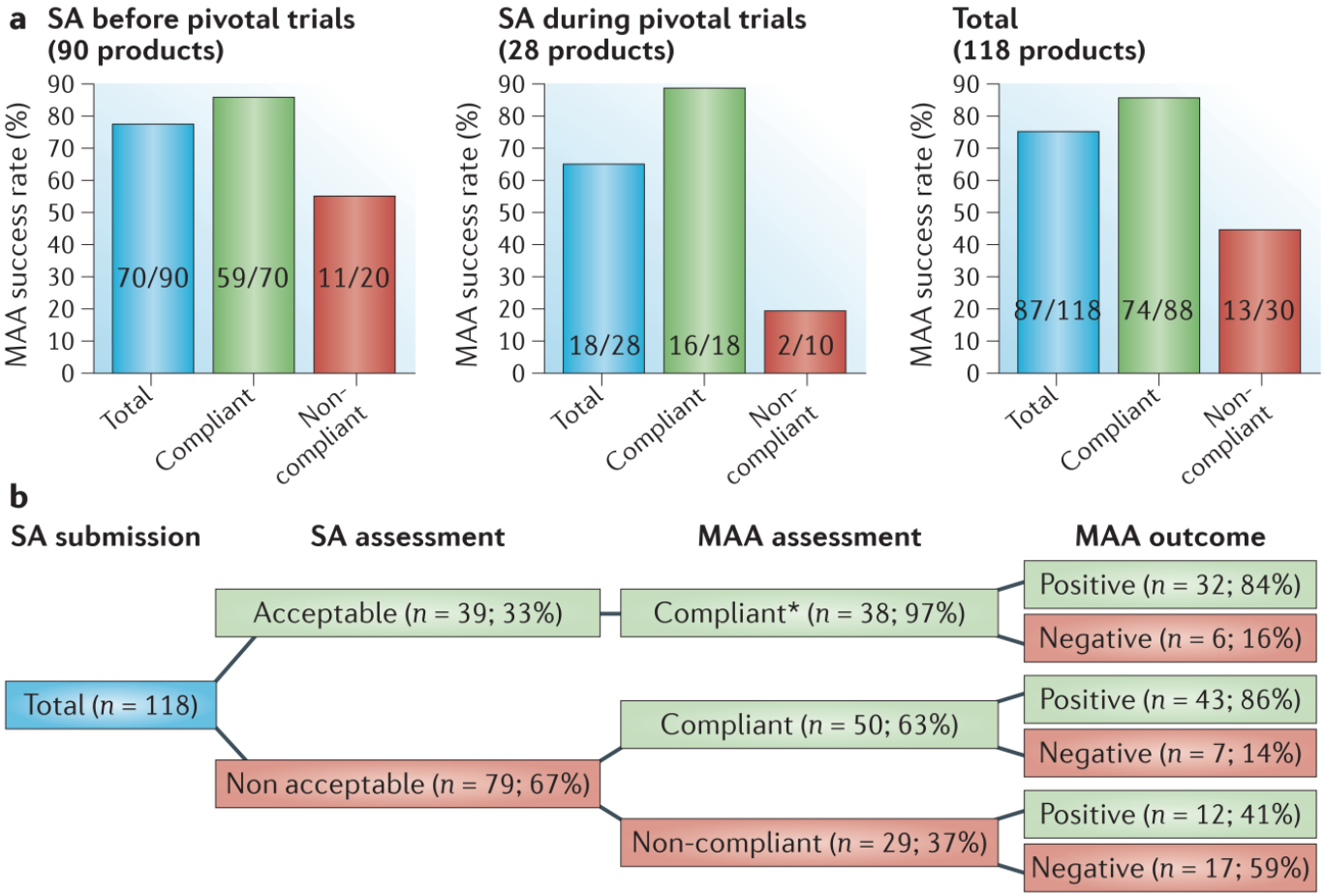
Today's program

09:00-09:40	Introduction to Day 3. How regulators work and think: the basics (Peter Mol)
09:40-09:45	Q&A
09:45-10:05	Novel regulatory tools & drug development support mechanisms (Peter Mol)
10:05-10:10	Q&A
10:10-10:25	Coffee break
10:25-10:50	Scientific advice (European & national) (Marjon Pasmooij)
10:50-10:55	Q&A
10:55-11:20	Case example – ATMP scientific advice (Viktoriia Starokozhko)
11:20-11:25	Q&A
11:25-11:40	Coffee break
11:40-12:10	Novel methodologies and Real World Evidence supporting drug regulatory decision-making
12:10-12:30	Final Q&A round (Moderator: Marjon Pasmooij)

Why Scientific Advice?



Hofer et al., 2015, Nat Rev Drug Discov

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EMA's early development advice service

- **Innovation task force** (ITF) as discussion platform for early dialogue with sponsors
- **Scientific advice** on the appropriate tests and studies in the development of a medicine, including engagement with other decision makers
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PRIME = PRIority MEdicines; SME = micro, small and medium-sized enterprises; ATMP = Advanced therapy medicinal product

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Innovation Task Force (ITF)

- Part of the European Innovation Network
  - The **aim of the network** is to make the regulatory support for medicines developers currently available at national and EU levels more visible and attractive to innovators
- The ITF is a multidisciplinary group that includes scientific, regulatory and legal competences. It was set up to ensure coordination across the Agency and to provide a forum for early dialogue with applicants on innovative aspects in medicines development.

- https://www.ema.europa.eu/en/human-regulatory/research-development/innovation-medicines#eu-innovation-network-section
- itfsecretariat@ema.europa.eu for human medicines, or
- vet.applications@ema.europa.eu for veterinary medicines.

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ITF scope

- Establish a discussion platform for early dialogue with applicants, in particular micro, small and medium-sized enterprises (SMEs), academics and researchers, to proactively identify scientific, legal and regulatory issues of emerging therapies and technologies
- Identify the need for specialised expertise at an early stage

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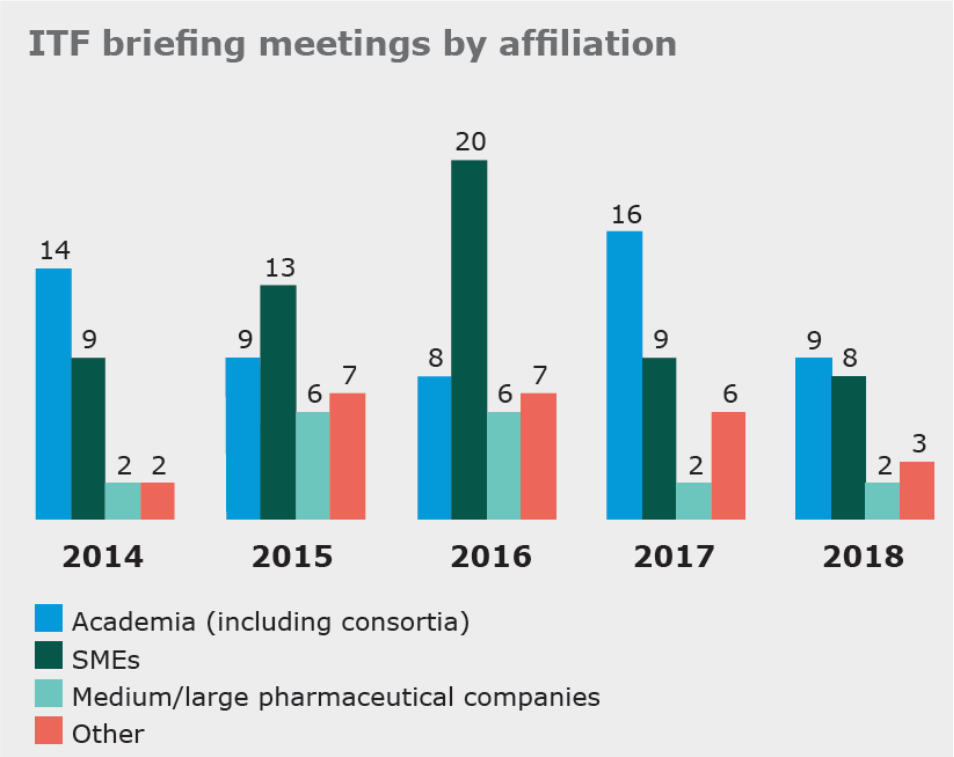
ITF in numbers

- Multidisciplinary platform for preparatory dialogue and orientation on innovative methods, technologies and medicines:
- In 2018, 58 requests leading to 22 meetings
- 77% of these meetings with academic developers and SMEs
- 32% were related to innovative methods and 13% concerned ATMPs



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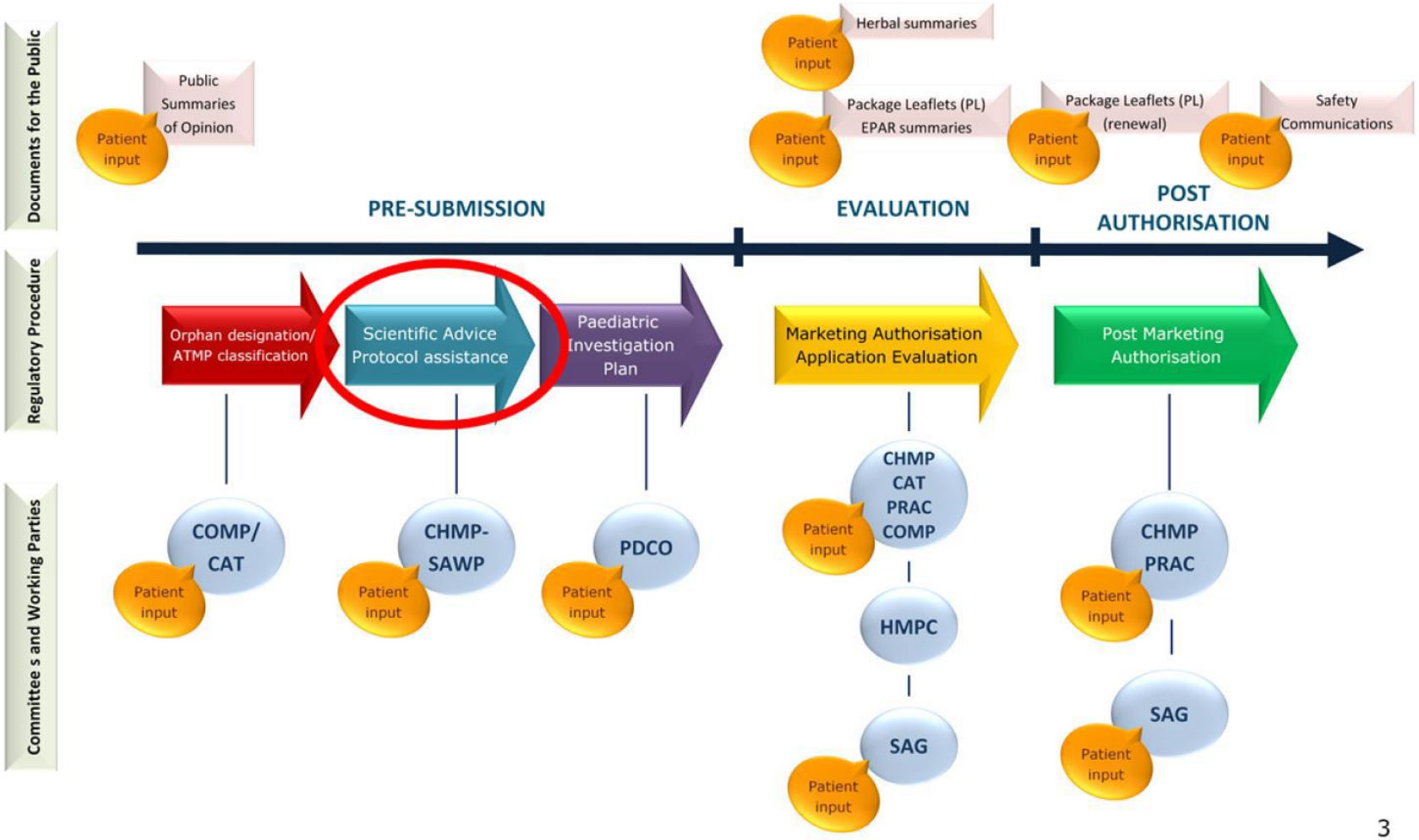
When does the SAWP come into the picture?

Medicines lifecycle



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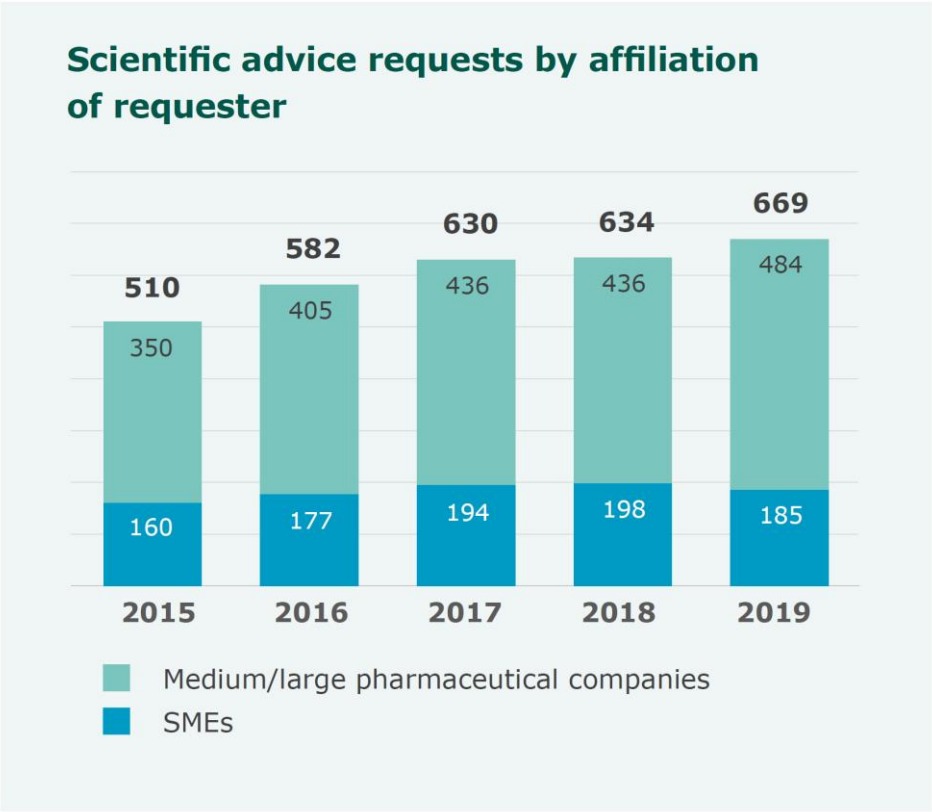
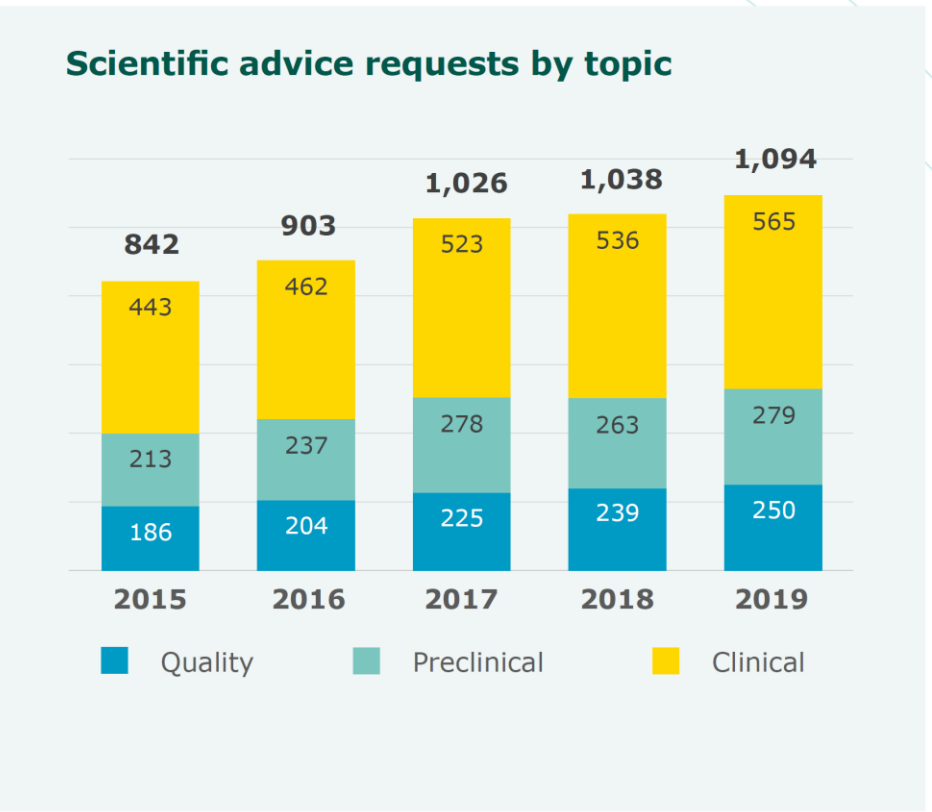
Some basic info on what the SAWP does





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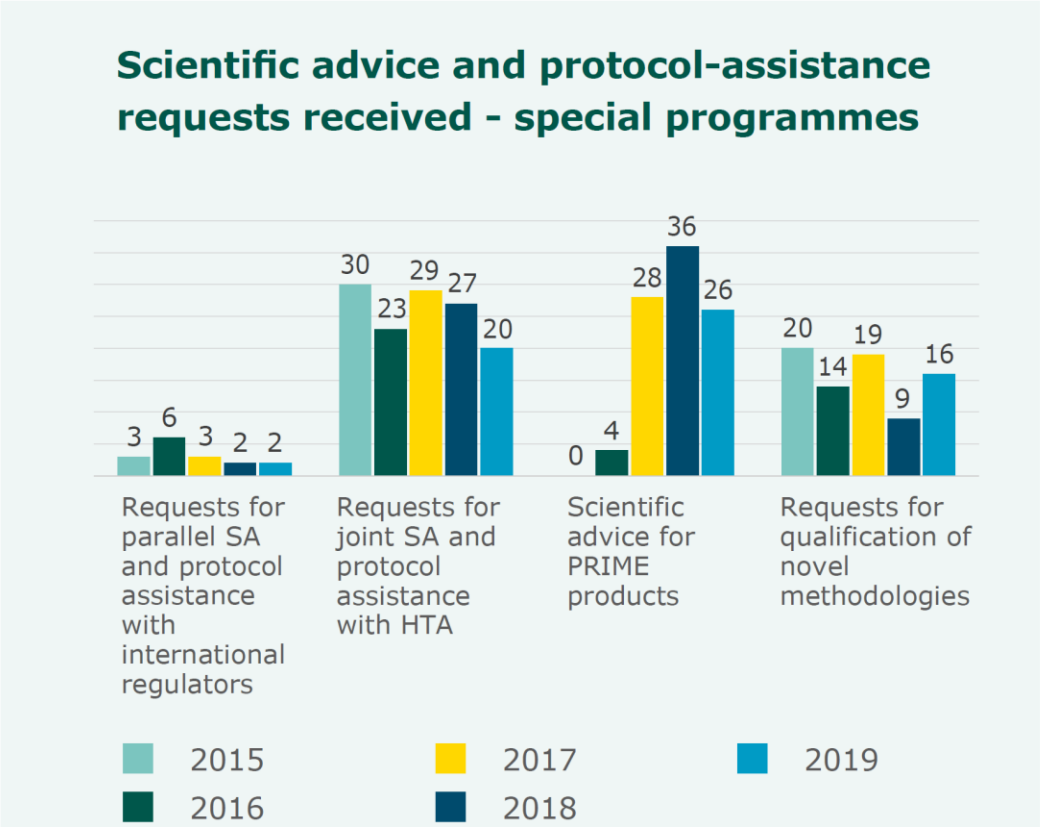

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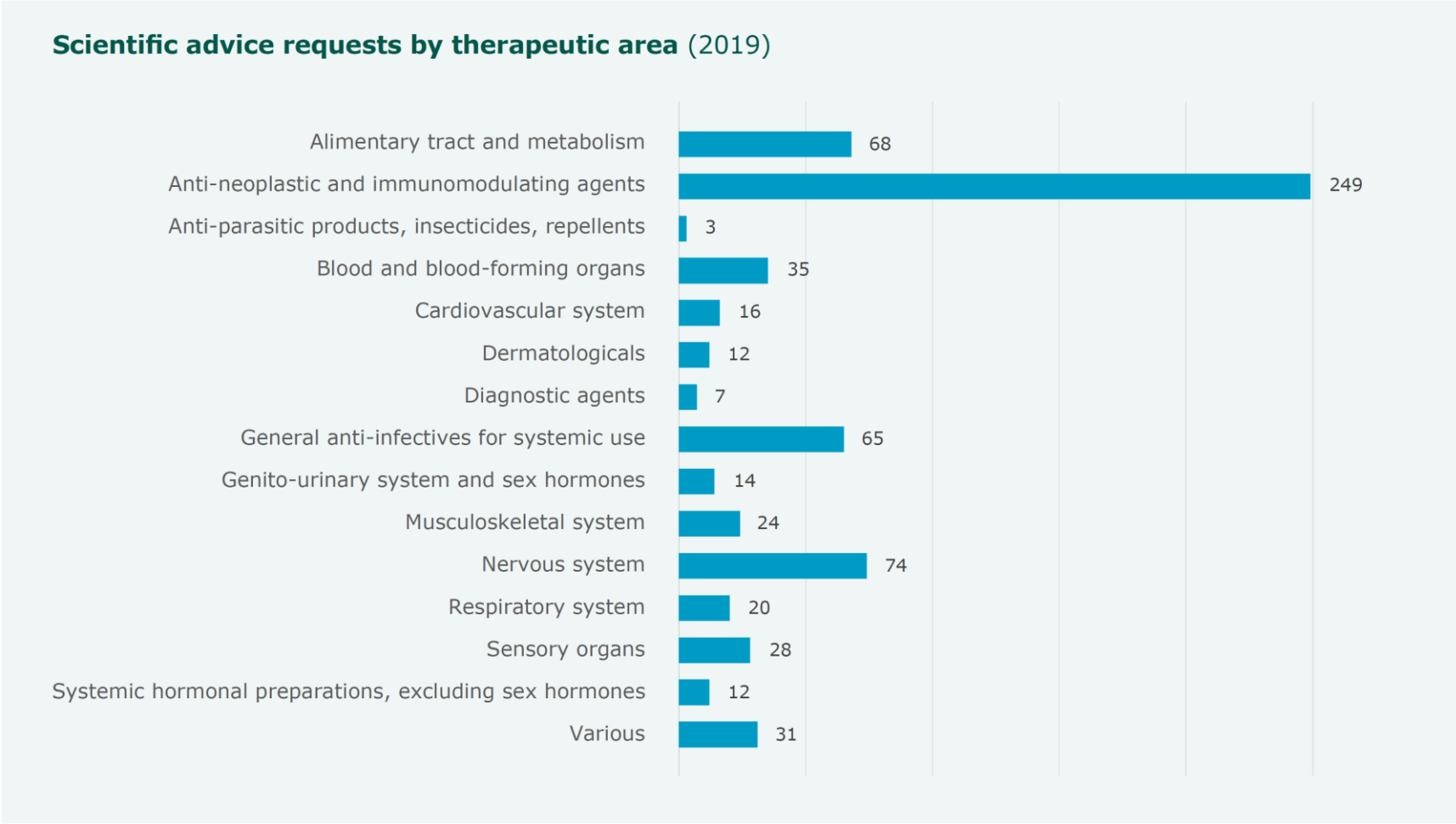
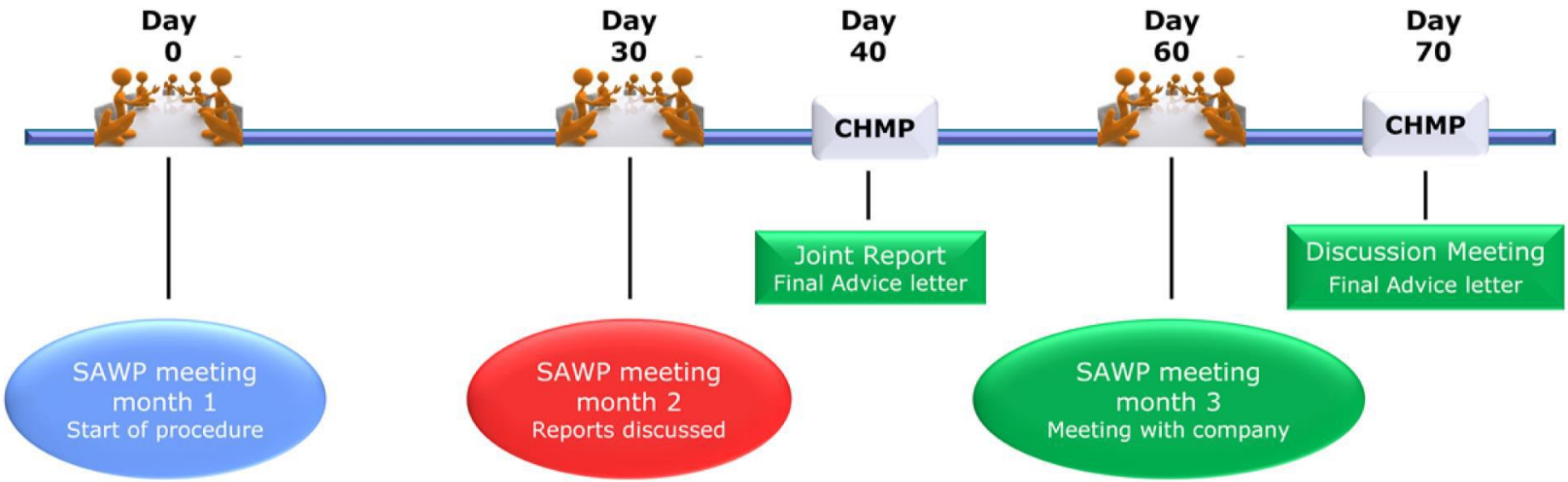


Procedural timelines



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PRIME: in brief

Medicines eligible for PRIME must address an unmet medical need.

Preliminary data must be available showing the potential to address this need and bring a major therapeutic advantage to patients.

Special support for SMEs and academia

Micro-, small- and medium-sized enterprises (SMEs) and applicants from the academic sector can apply for PRIME at an earlier stage of development when they have compelling non-clinical data and tolerability data from initial clinical trials. They may also request a fee waiver for scientific advice.

SMEs and academia can particularly benefit from earlier scientific and regulatory support since they often lack experience with the regulatory framework.

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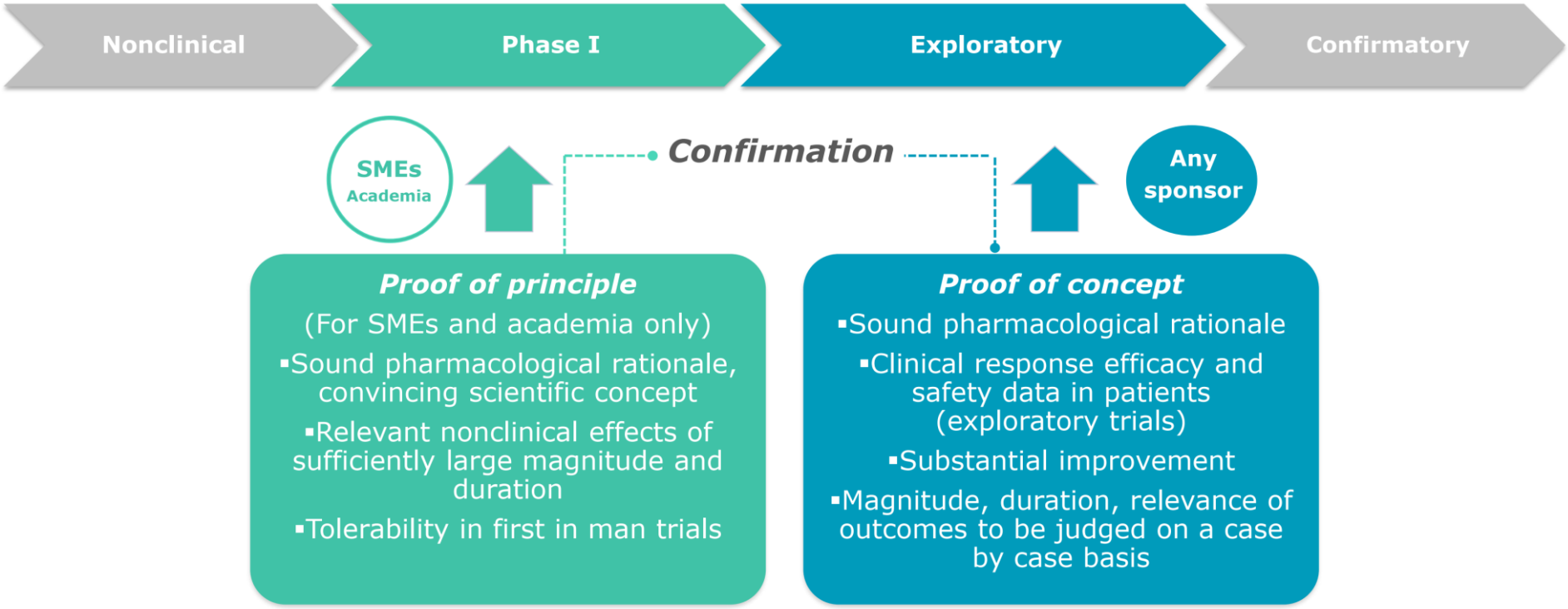
PRIME (PRIorityMEdicines)



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Orphan drugs, why?

- Rare diseases -> developing and marketing cost would not be recovered by the expected sales
- Persons suffering from rare conditions deserve same quality of treatment as other patients
- Pharmaceutical industry does not develop medicines for rare diseases under normal market conditions

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Orphan drugs

- Regulation (EC) No 141/2000 of the European Parliament and of the Council on Orphan Medicinal Products of 16 December 1999

- Criteria for designation
- Committee (COMP)
- Incentives
- Orphan Drug Development Guide Book (https://irdirc.org/orphan-drug-development-guidebook-materials/)

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Academia developing medicines for rare diseases to receive free EMA scientific advice

Share

News 23/06/2020

To further encourage the development of treatments for rare diseases, EMA will waive all fees for scientific advice for academia developing orphan medicines.

The academic sector plays an important role in the development of innovative medicines. Their scientific research is often at the source of novel methodologies and innovative medicines with a potential to benefit patients with rare diseases.

Early interaction with EU regulators is important for academia to understand the regulatory requirements and allow the generation of robust evidence needed to establish the medicines' benefits and risks. This helps them to navigate the regulatory process and ultimately to translate their discoveries into authorised, patient-focused medicines.

Gonzalez-Quevedo et al., 2020, Nat Rev Drug Discov

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Paediatric development

- Applicants can request scientific advice from EMA in preparation of a Paediatric Investigation Plan (PIP), which is aimed at ensuring that the necessary data are obtained through studies in children, to support the authorisation of a medicine for children.
- Free of charge for questions relating to the development of paediatric medicines. They can also follow up a PIP with scientific advice, for example on combined adult and paediatric development in light of the PIP requirements.

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The “SME office” at EMA

- Provide a platform for early dialogue with SMEs to discuss a regulatory strategy of medicinal product development and navigate the range of procedures and incentives available
  - Multidisciplinary group, co-operation with other EMA offices
  - Human/veterinary medicines; Free of charge

- Examples of topics :
  - Guidance on procedure and dossier content for Scientific advice, PRIME, ATMP certification, Paediatric aspects and Orphan designation
  - Data protection and market exclusivity
  - Eligibility to the centralized procedure, legal basis for submission of dossier, conditional and exceptional circumstances marketing authorisation

- https://www.ema.europa.eu/en/human-regulatory/overview/supporting-smes
- https://www.ema.europa.eu/en/partners-networks/academia

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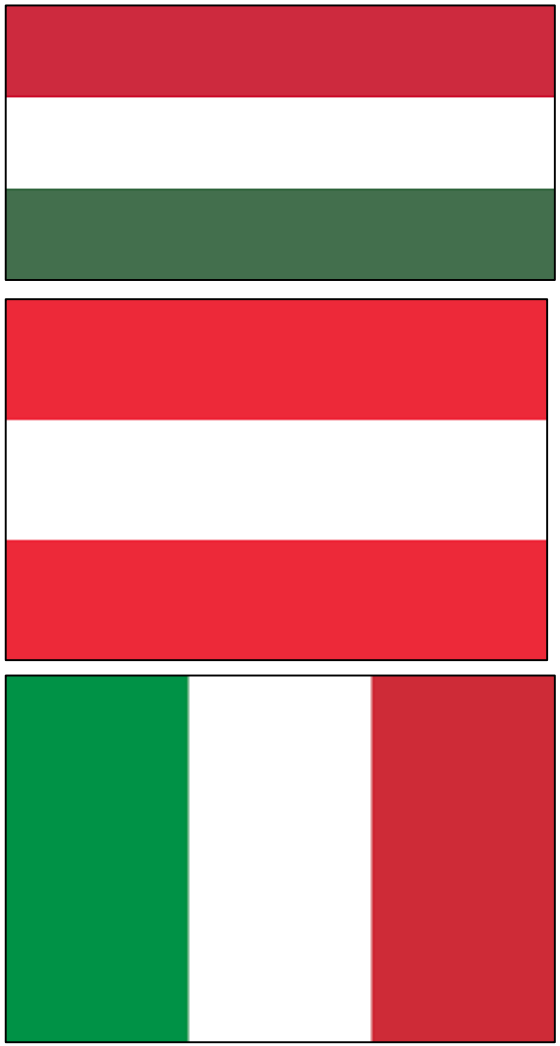
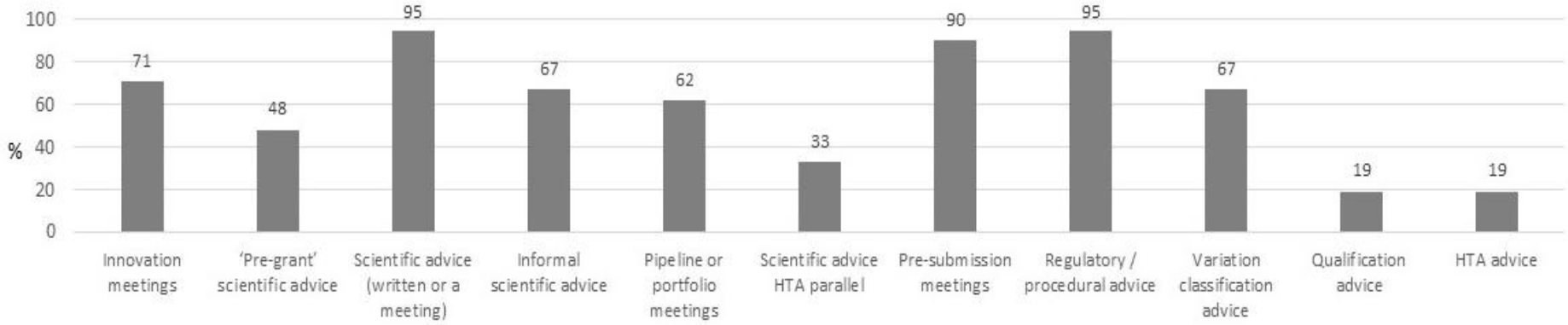
Switch from European to National



National Advice



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Scientific Advice (European vs National)

- National advice is an important first contact point, and can be a first step for subsequently asking for advice on an European level.
- Advice regarding CTA is not offered by the EMA. Many of the national medicines agencies offer both regulatory and CTA advice. In a few cases, these are separate institutions.
- Asking for national advice you can select the agency/agencies. At SAWP, two coordinators are assigned.
- National advice you can ask for a (virtual) face-to-face meeting, whereas on European level a selection is made in the high number of advices that will have a discussion meeting.

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National Advice

HU (OGYEI)

- Contact: tanacsadas@ogyei.gov.hu
- https://www.ogyei.gov.hu/szakmai_tanacsadas (Hungarian, but soon also in English)
- Face-to-face meetings (with Teams)

AT (BASG/AGES)

- Contact: scientificadvice@basg.gv.at
- Homepage: https://www.basg.gv.at/
  Link scientific advice (NASA= **NA**tional **S**cientific **A**dvice) department:
  https://www.basg.gv.at/fuer-unternehmen/arzneimittel-informationen/beratung-durch-das-basg
- Face-to-face meetings (with Webex).

IT (AIFA)

- Scientific advice suspended, only SNSA when request is related to innovation.
- https://www.aifa.gov.it/scientific-advice

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Last but not least…

- Make use of the possibilities to go for Scientific Advice for academia. Make also use of other support offices at your university/center, such as Technology Transfer Offices.

- Difference in “language”. Don’t let it stop you. Important to have the dialogue.

- Education is key to success.

  - Drug development starts in the Lab, not when a product gets the interest of Big Pharma.
  - It is not out of reach for academics and SME’s to bring a product to the market.

Any questions?

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