CBG MEB
MEDICINES EVALUATION BOARD

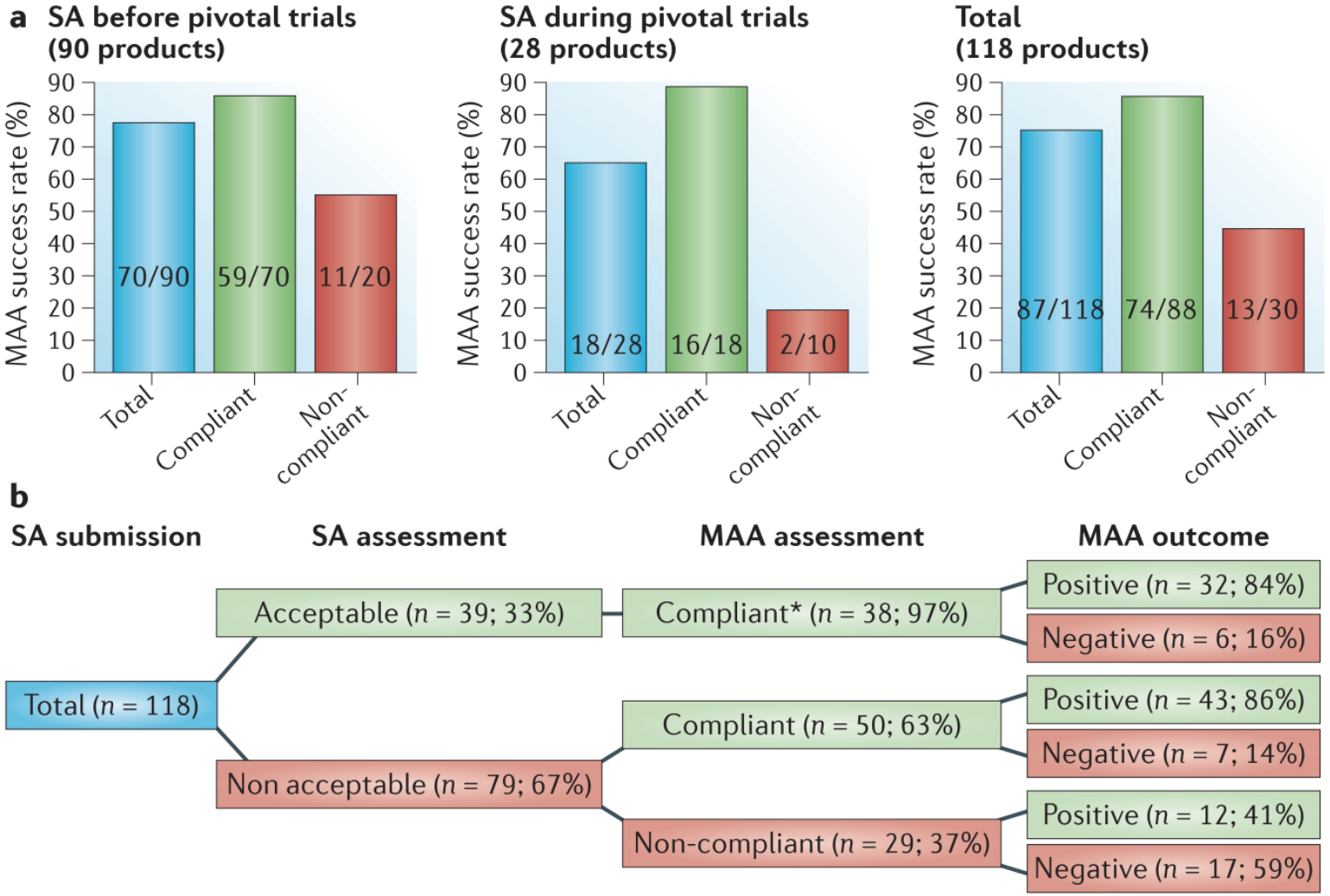
Scientific Advice (European and National): Support along the way

Dr. Marjon Pasmooij

MEB Science Day, 17 February 2022

GOOD MEDICINES USED BETTER

Why Scientific Advice?



Hofer et al., 2015, Nat Rev Drug Discov

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

PRIME scheme for enhanced support of medicines targeting an unmet medical need

Qualification of novel methodologies in the context of research and development

Specific frameworks for **paediatric development** and **orphan medicines**

SME support including briefing meetings to discuss regulatory strategies as well as certification of quality and non-clinical data for ATMPs

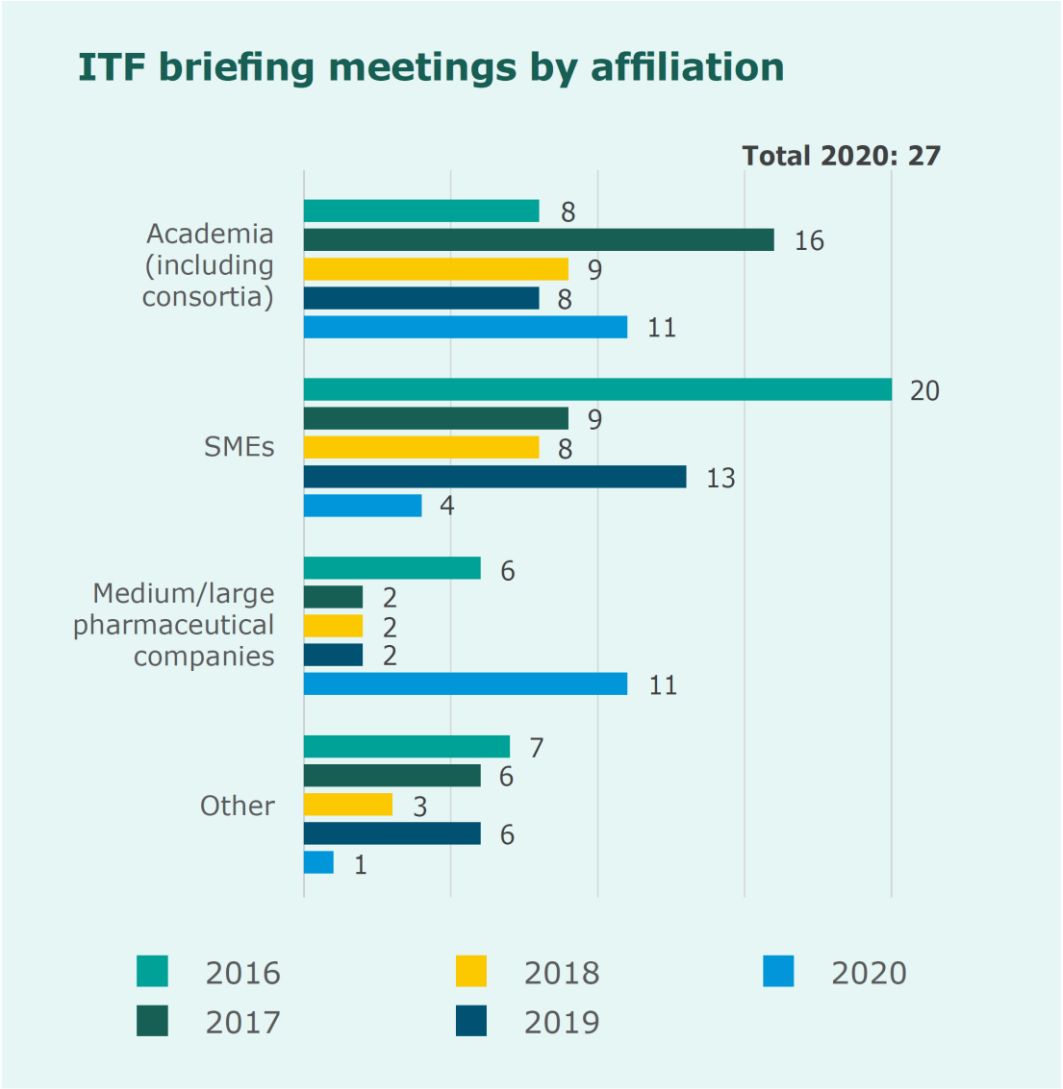
PRIME = PRIority MEdicines; SME = micro, small and medium-sized enterprises; ATMP = Advanced therapy medicinal product

ITF in numbers

Multidisciplinary platform for preparatory dialogue and orientation on innovative methods, technologies and medicines:

In 2020, 11 out of 27 meetings (41%) with academic developers

- 30% of the meetings concerned innovative methods to support the development of medicines
- 22% manufacturing technologies.



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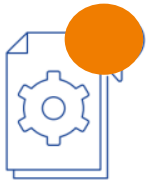

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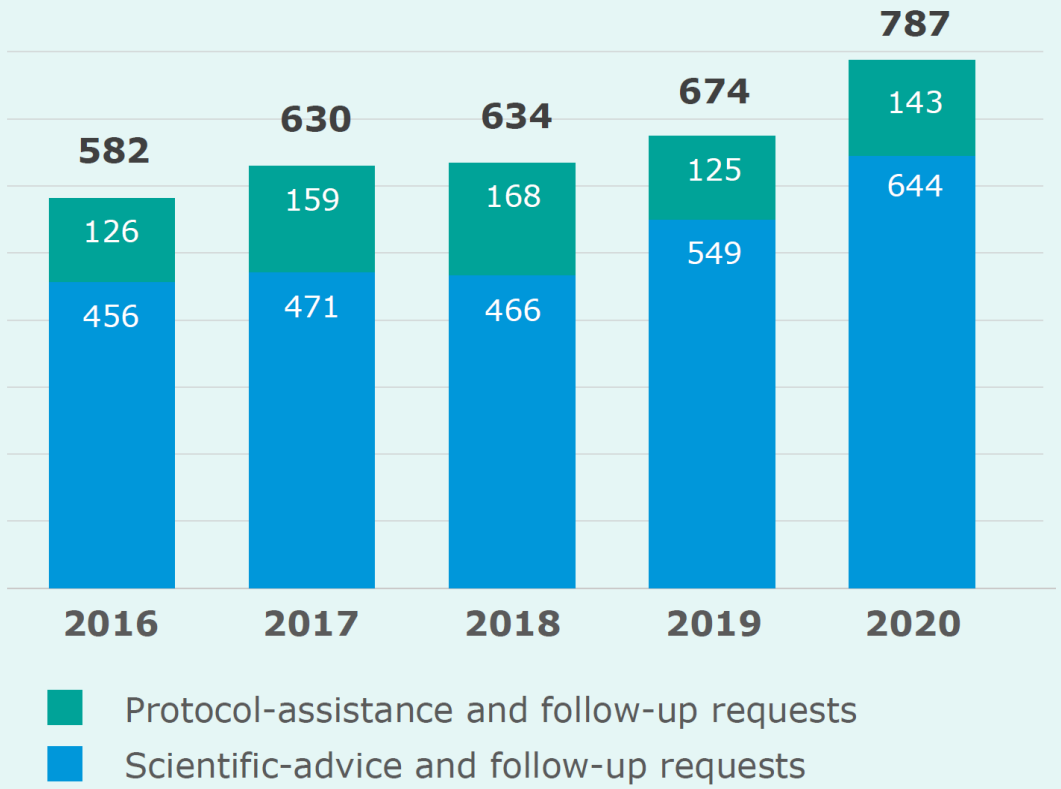
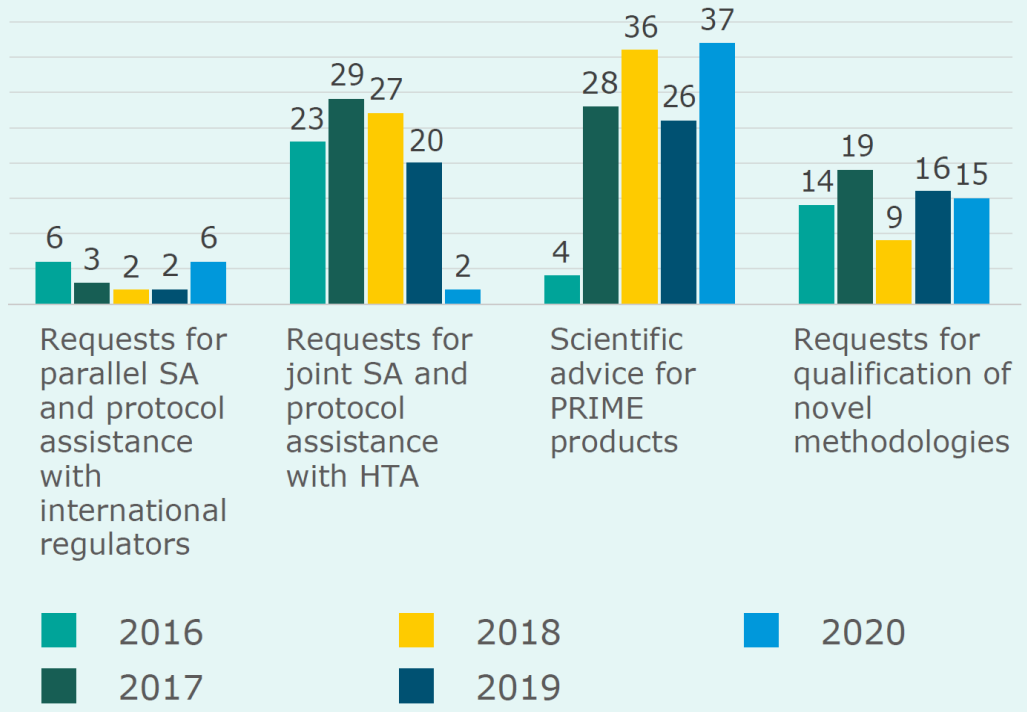
Scientific Advice, PRIME and Qualification of novel methodologies in numbers

Scientific advice and protocol-assistance requests received - total



Scientific advice and protocol-assistance requests received - special programmes



Regulatory Scientific Guidelines

For each step in the procedure: guidelines!

> 450 guidelines

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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.

Orphan medicines

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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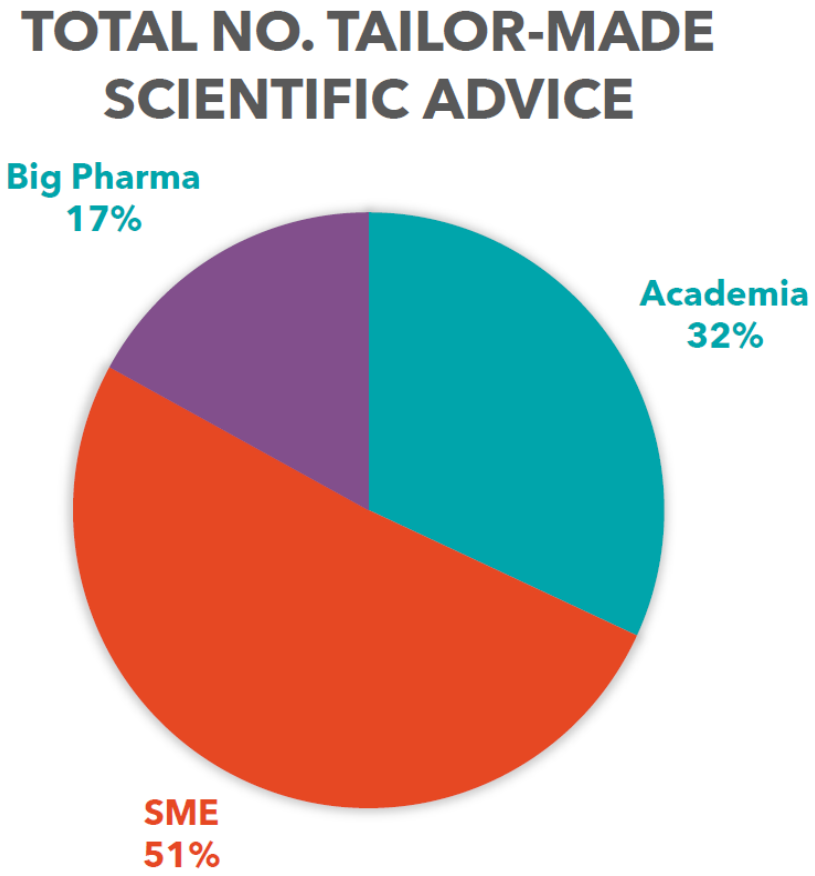
National Scientific Advice

- National advice is an important first contact point, and can be a first step for subsequently asking for advice on a European level.
- Advantage on national level, face-to-face meeting, whereas at a European level a selection is made for which scientific advices a discussion meeting is held.
- Tailor-made Scientific Advice (“Advies op Maat”)
- https://www.cbg-meb.nl/onderwerpen/hv-wetenschappelijk-en-regulatoir-advies
  nationalscientificadvice@cbg-meb.nl

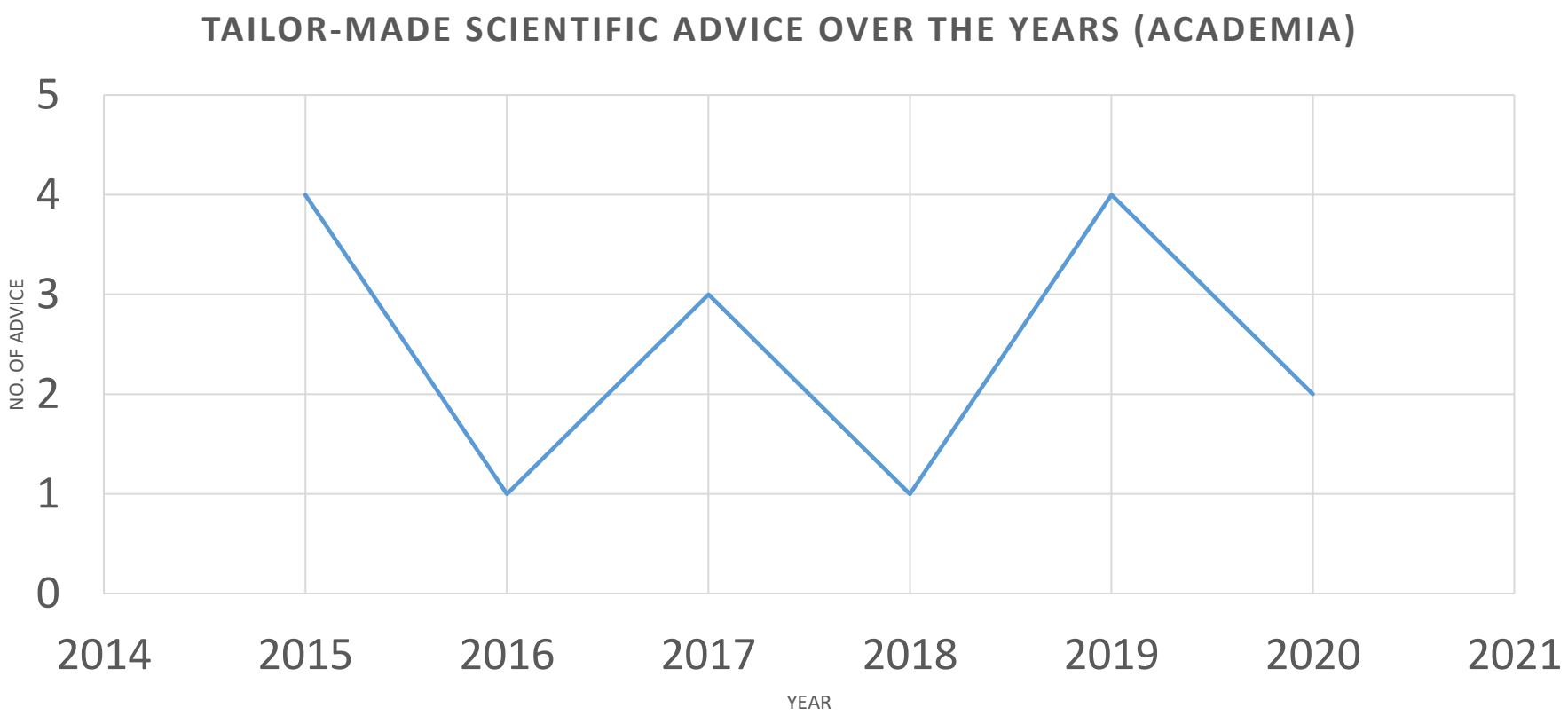
Tailor-made Scientific Advice at MEB



Total of **47** accepted requests for tailor-made scientific advice (2015-2020):

- **15** requests by academia
- **24** requests by SME
- **8** requests by big pharma

Tailor-made Scientific Advice at MEB



8 out of 15 (53%) drug rediscovery products

Repurposing of authorised medicines: Pilot

- To support not-for-profit organisations and academia to gather or generate sufficient evidence on the use of an established medicine in a new indication with the view to have this new use formally authorised by a regulatory authority.

- https://www.ema.europa.eu/en/news/repurposing-authorised-medicines-pilot-support-not-profit-organisations-academia

- Sponsors wishing to seek EMA scientific advice should complete the File drug repurposing submission form and submit it to sarepurposing@ema.europa.eu by **28 February 2022**.

- EMA Webinar “Repurposing of medicines pilot project”, 17.00 – 18.30

Academia – FAQ

Quality

Mostly specific questions of product itself

E.g.: is our GMP chemistry strategy for the production of [product name] a suitable strategy?

Pre-clinical

- For existing medicines, is it necessary to reproduce toxicity studies or can existing data be used?
- What should be known before a clinical trial is started and what is necessary for marketing registration?

Clinical

- Does the Agency agree to: study population, study design, study endpoints, placebo, statistical analysis, etc.?

Regulatory

- What are the options for registration and which authorization procedure will be advised?
- Would it be possible to request orphan designation?
- Is it feasible and preferable to obtain marketing authorization by the academia (the applicant, being an endorsed centre of expertise)?

ZIN

- How do you make reimbursement possible by health insurance companies in the Netherlands?
- Registration of a new indication is costly, the number of patients for the indication is limited in the Netherlands. If all costs should be paid by selling the drugs it would increase the price of [product name] substantially. Can this be avoided?

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.

Consortium for education & training of (bio-)medical professionals

Medicines Evaluation Board of the Netherlands

Dutch Association for Pharmaceutical Medicine

Central Committee on Research Involving Human Subjects

Center for Human Drug Research

Dutch Association of Clinical Pharmacology and Biopharmacy

LUMC/Paul Janssen Futurelab Leiden

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