

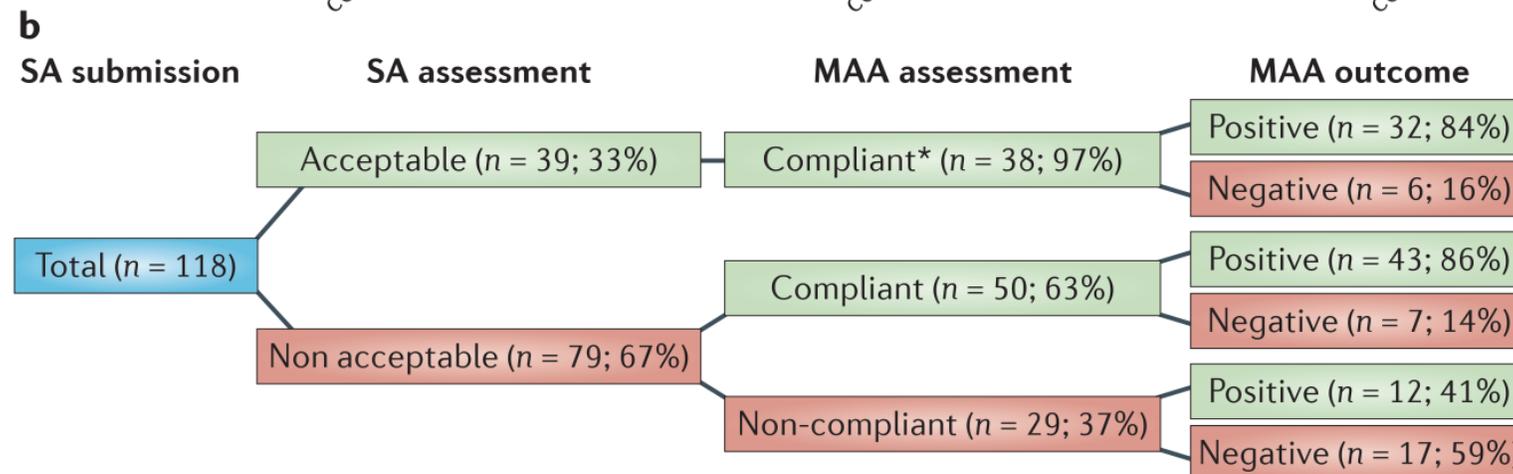
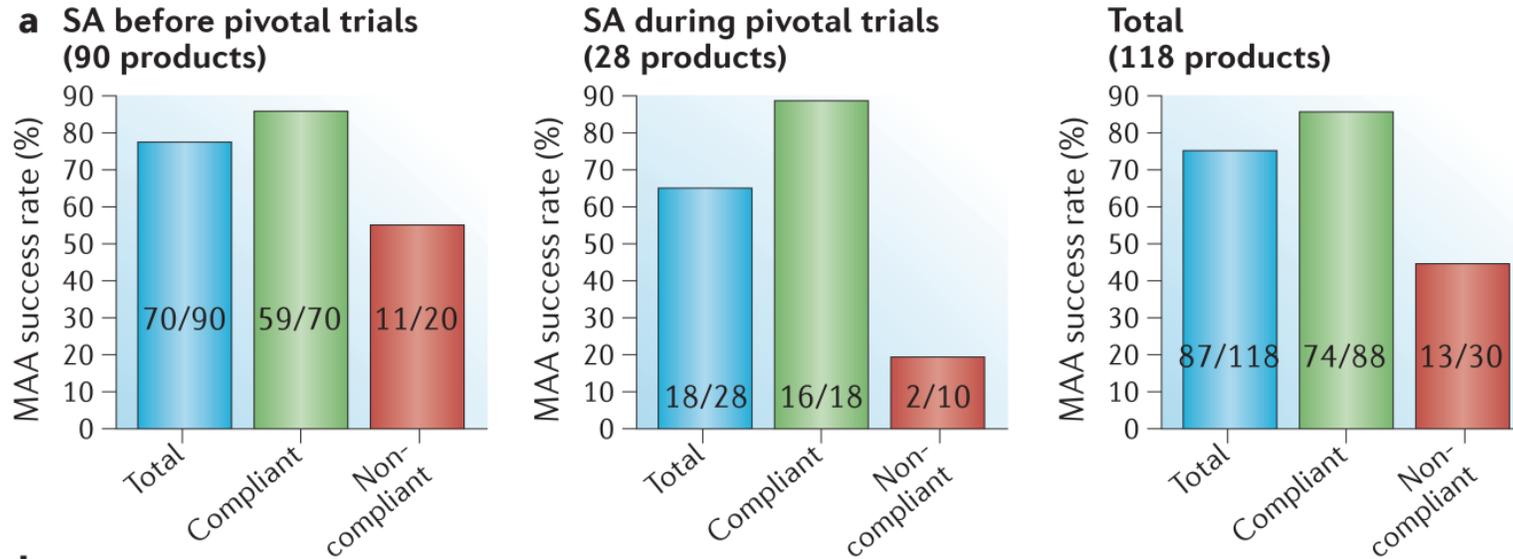
Scientific Advice (European and National): Support along the way

Dr. Marjon Pasmooij

MEB Science Day, 17 February 2022



Why Scientific Advice?





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

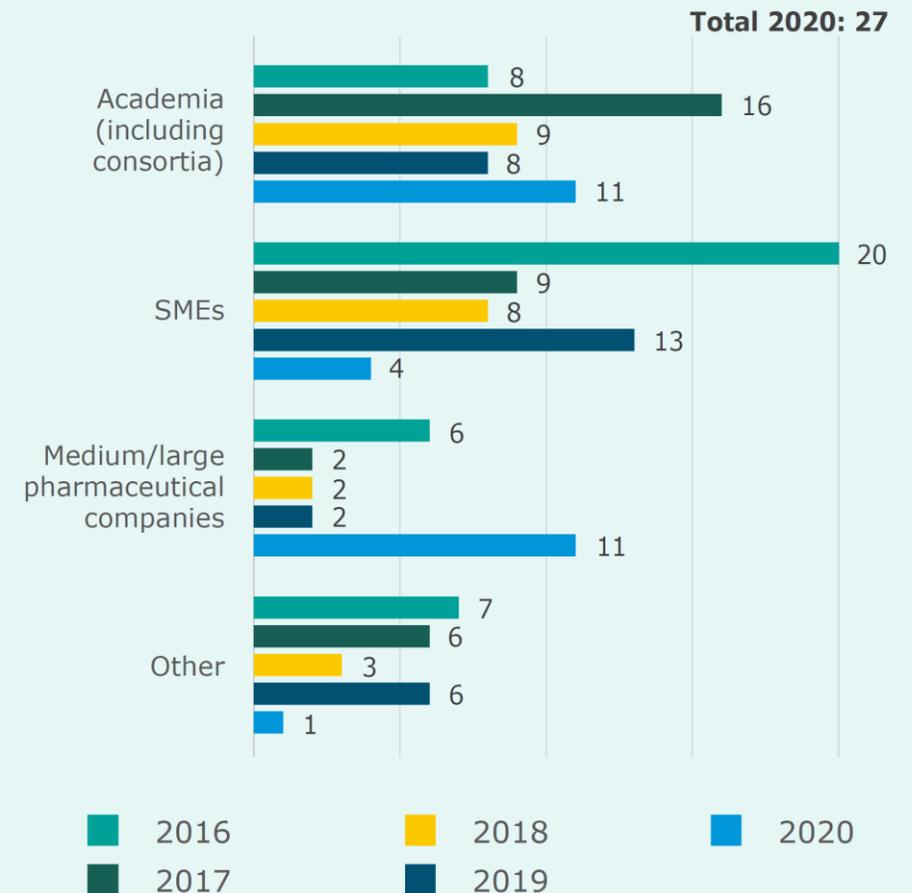
PRIME = PRIority MEdicines; SME = micro, small and medium-sized enterprises; ATMP = Advanced therapy medicinal product

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.

ITF briefing meetings by affiliation





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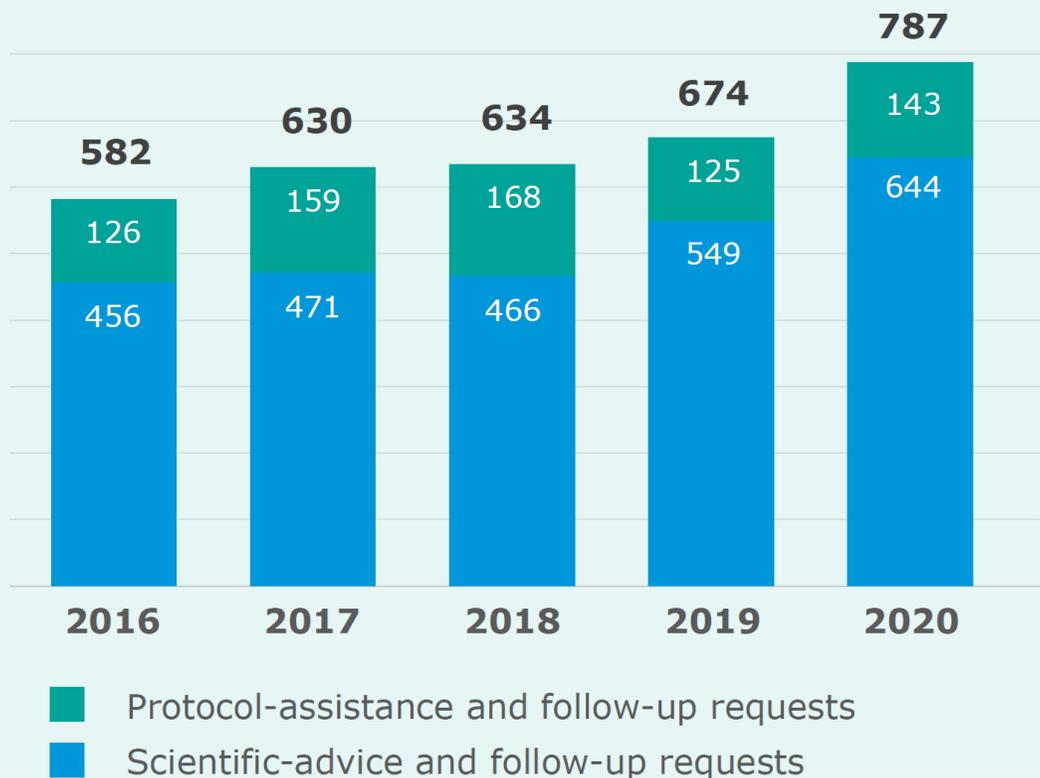
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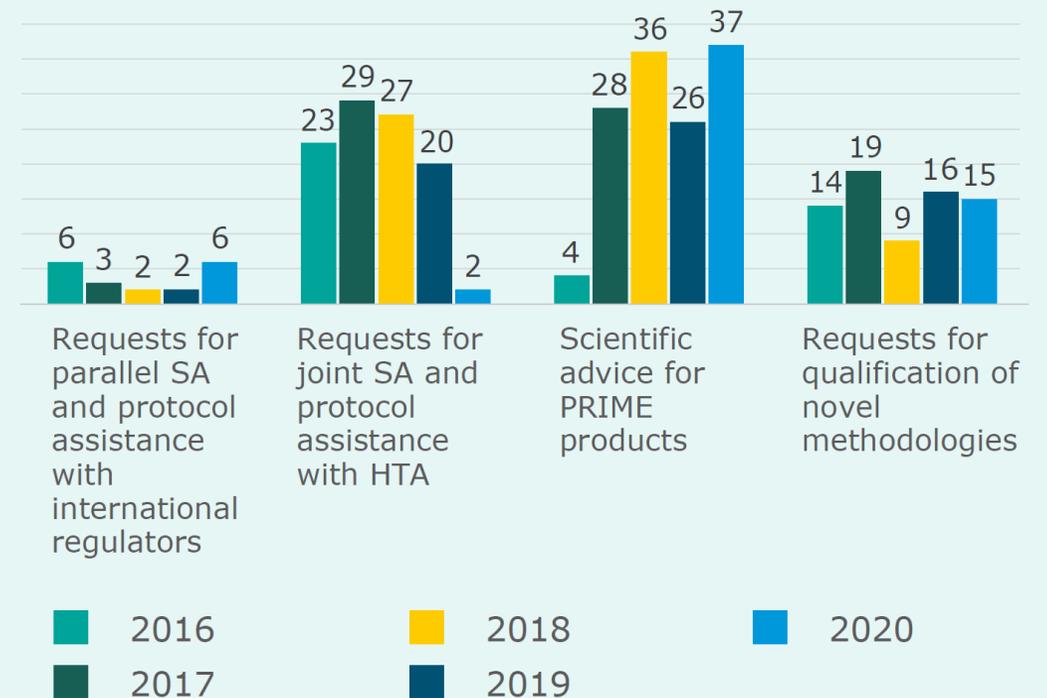
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Scientific Advice, PRIME and Qualification of novel methodologies in numbers

Scientific advice and protocol-assistance requests received - total



Scientific advice and protocol-assistance requests received - special programmes



For each step in the
procedure: guidelines!
> 450 guidelines



The screenshot shows a web browser window with the URL <https://www.ema.europa.eu/en/human-regulatory/research-development/scientific-guidelines>. The page title is "Scientific guidelines" with a "Share" button. A dark blue sidebar on the left contains a list of menu items: Adaptive pathways, Advanced therapies, Clinical trials, Compassionate use, Compliance, Data on medicines (ISO IDMP standards), Ethical use of animals, Innovation in medicines, Medicines for older people, Orphan designation, Paediatric medicines, Pharmacovigilance, and PRIME: priority medicines. The main content area features a bold introductory paragraph, a paragraph explaining the Agency's encouragement of guidelines and the need to justify deviations, a list of complementary documents, and a section titled "Compilation of European Commission and Agency guidelines" which includes a paragraph and a list of guideline categories.

Adaptive pathways

Advanced therapies

Clinical trials

Compassionate use

Compliance

Data on medicines (ISO IDMP standards)

Ethical use of animals

Innovation in medicines

Medicines for older people

Orphan designation

Paediatric medicines

Pharmacovigilance

PRIME: priority medicines

Scientific guidelines [Share](#)

The European Medicines Agency's Committee for Medicinal Products for Human Use prepares scientific guidelines in consultation with regulatory authorities in the European Union (EU) Member States, to help applicants prepare marketing authorisation applications for human medicines. Guidelines reflect a harmonised approach of the EU Member States and the Agency on how to interpret and apply the requirements for the demonstration of quality, safety and efficacy set out in the Community directives.

The Agency strongly encourages applicants and marketing authorisation holders to follow these guidelines. Applicants need to justify **deviations from guidelines** fully in their applications at the time of submission. Before that, they should seek [scientific advice](#), to discuss any proposed deviations during medicine development.

The guidelines are complementary to European Pharmacopoeia monographs and chapters:

- [Status of European Medicines Agency scientific guidelines and European Pharmacopoeia monographs and chapters in the regulatory framework applicable to medicinal products](#)

Compilation of European Commission and Agency guidelines

This section of the website updates and replaces the previous [volume 3 of the rules governing medicinal products in the European Union \(EudraLex\)](#), published by the European Commission. It contains:

- all valid guidelines originally published in volume 3;
- all valid guidelines published by the Agency since 1995;



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- 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.

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.



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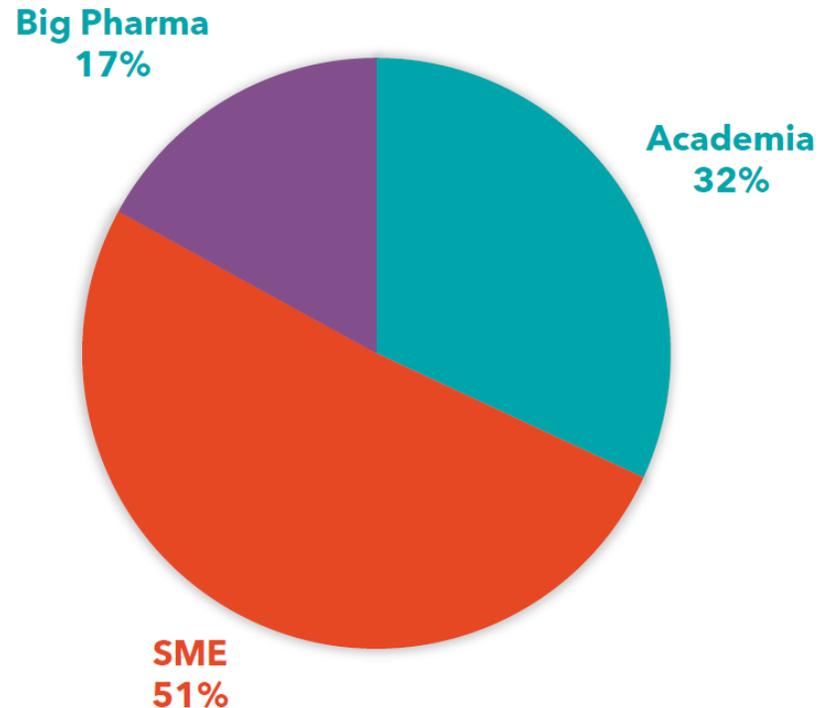
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- National advice is an important first contact point, and can be a first step for subsequently asking for advice on a European level.
- Advantage on national level, face-to-face meeting, whereas at a European level a selection is made for which scientific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

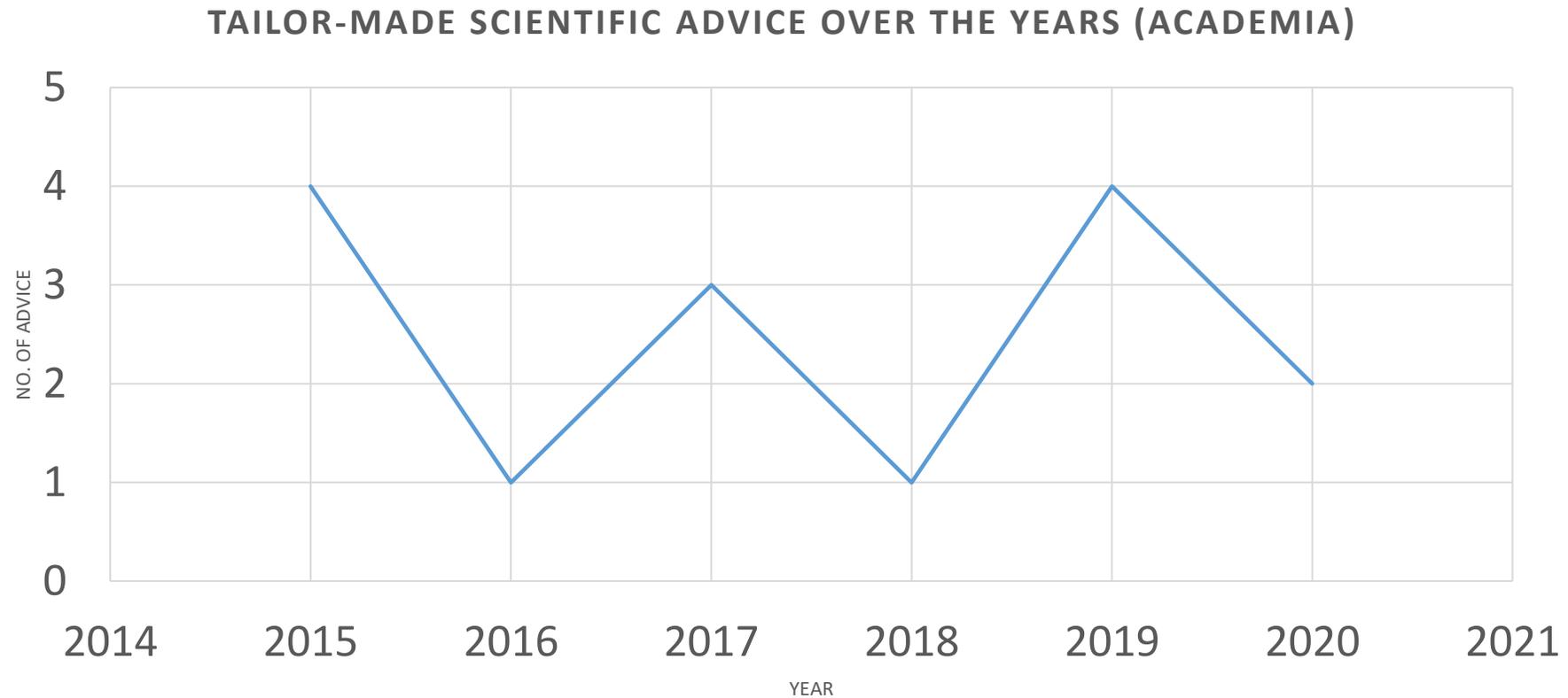
TOTAL NO. TAILOR-MADE SCIENTIFIC ADVICE



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

- 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-authorized-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

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...

$\frac{C \ B \ G}{M \ E \ B}$

- 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

$\frac{c \ B \ G}{M \ E \ B}$



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



**GOOD
MEDICINES
USED
BETTER**