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De rol van de regulator bij academische geneesmiddelontwikkeling

Marjon Pasmooij
Programmamanager Wetenschap
College ter Beoordeling van Geneesmiddelen

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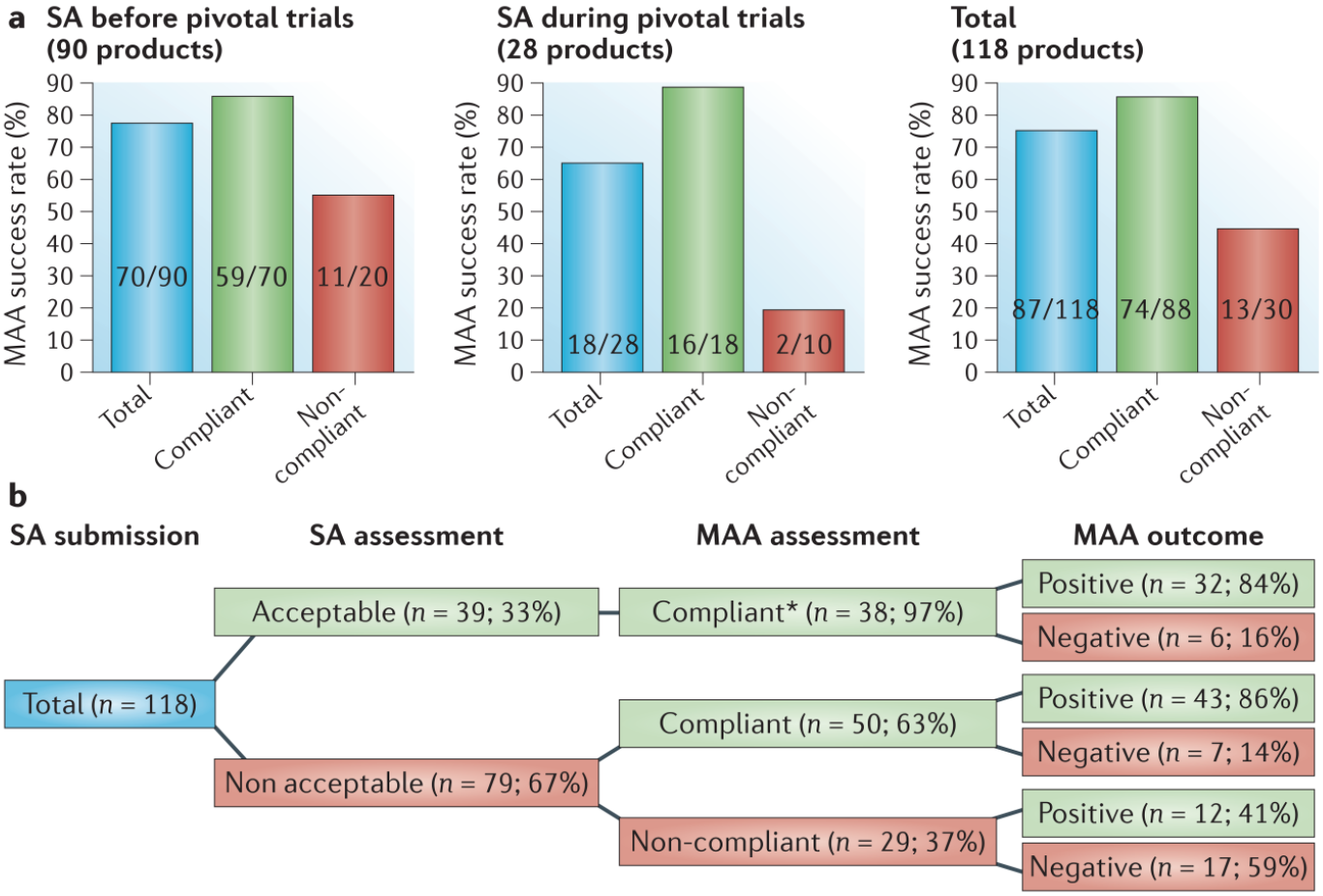
Why Scientific Advice?



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

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.

Medicines for children and rare diseases: Commission launches a public consultation

10 May 2021

The Commission has launched an Open public consultation on the revision of the legislation on medicines for children and rare diseases (medicines for special populations). This is an important step in the process of assessing the impact of possible amendments to EU rules for these medical areas which builds on the recent evaluation published in summer 2020. This evaluation showed that the regulations have stimulated research and development of medicines to treat rare diseases and of medicines for children. However, while 95% of rare diseases still have no treatment option, the evaluation also revealed shortcomings in the current system concerning in particular the development of medicines in areas of high unmet need for patients and their accessibility to all EU patients across the Member States. Interested parties, in particular health professionals, patients, doctors, academia, researchers, pharmaceutical industry and citizens are invited to share their views via a questionnaire, until 30 July, to help explore several options in view of the revision of the legislation.

More information:

- Evaluation of the medicines for rare diseases and children legislation

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 a European level.

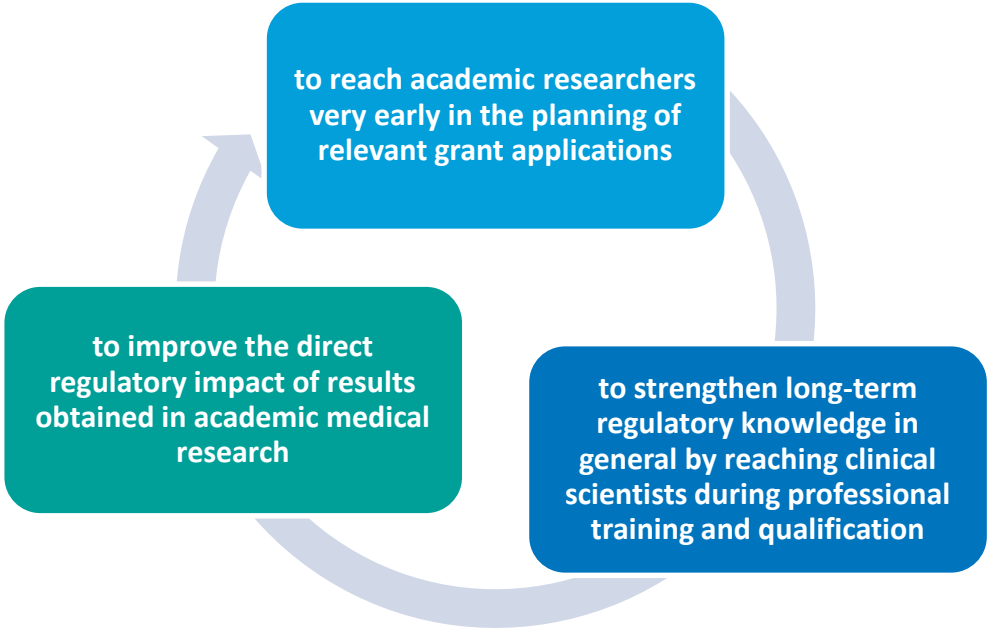
- “Advies op Maat”

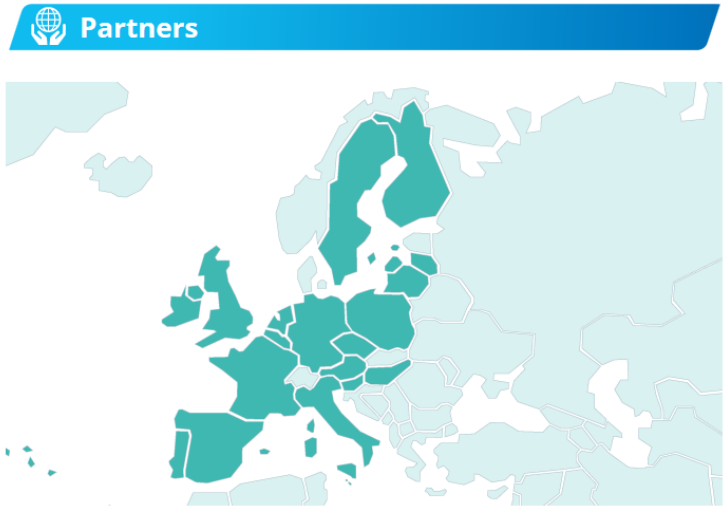
Advies bedoeld voor start-ups, kleine ondernemingen en academische groepen. Over het algemeen zal het primair betrekking hebben op de farmaceutische of pre-klinische aspecten van een geneesmiddel in de vroege ontwikkelfase, fase I klinisch onderzoek, of op de ontwikkeling en registratie van een nieuwe toepassing van een bestaand geneesmiddel. Verder kan men terecht met vragen over de 'regulatoire routekaart'. Voor advies op maat bestaat een mogelijkheid tot een verkennend vooroverleg, voorafgaand aan de eigenlijke aanvraag.

https://www.cbg-meb.nl/onderwerpen/hv-wetenschappelijk-en-regulatoir-advies

STARS: Strengthening training of academia in regulatory sciences & supporting regulatory Scientific Advice

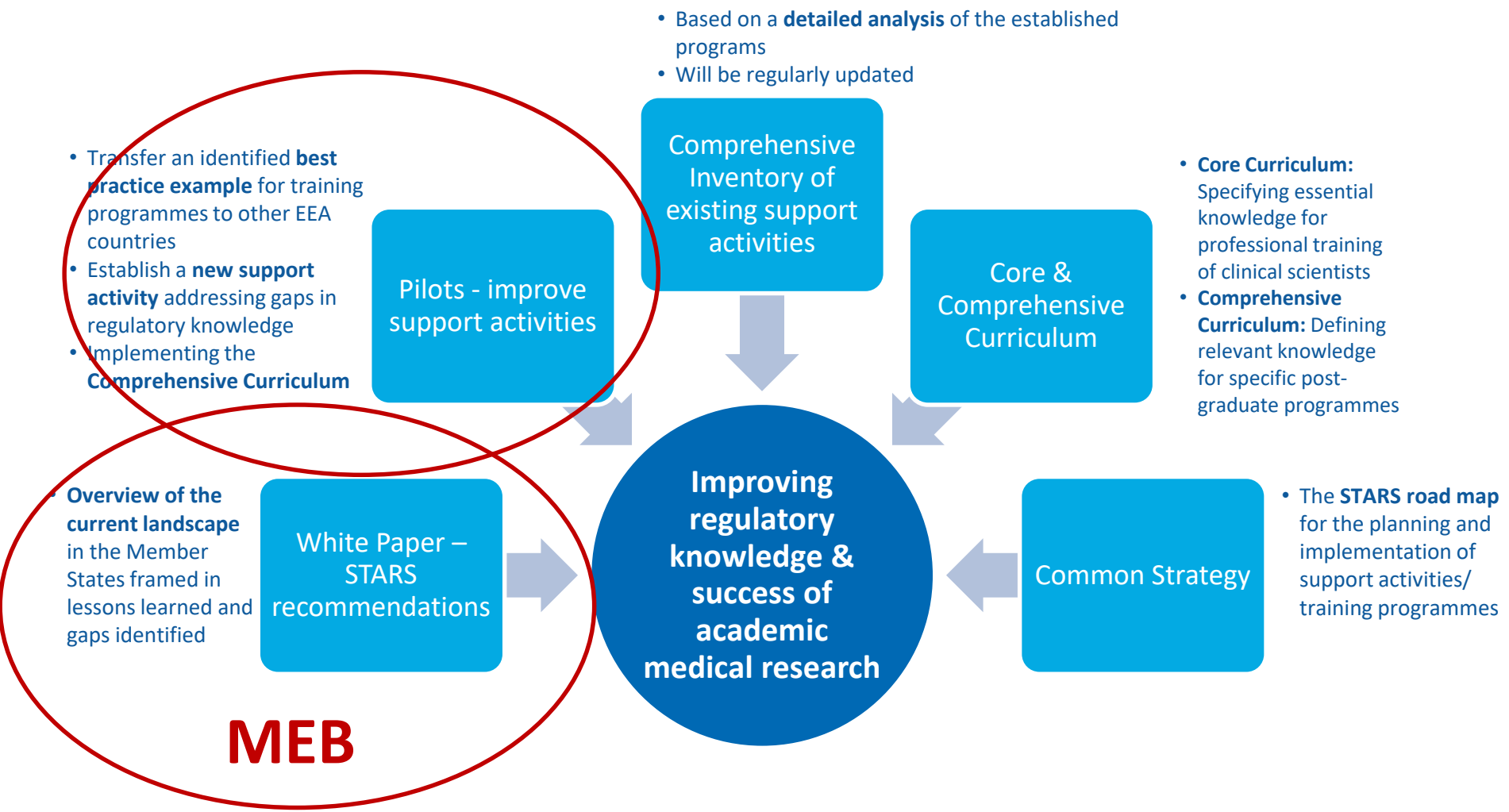
- Consortium of 18 European countries represented via their NCA
- 4 associate countries
- EMA





STARS partner countries © DLR

STARS – Expected outcomes

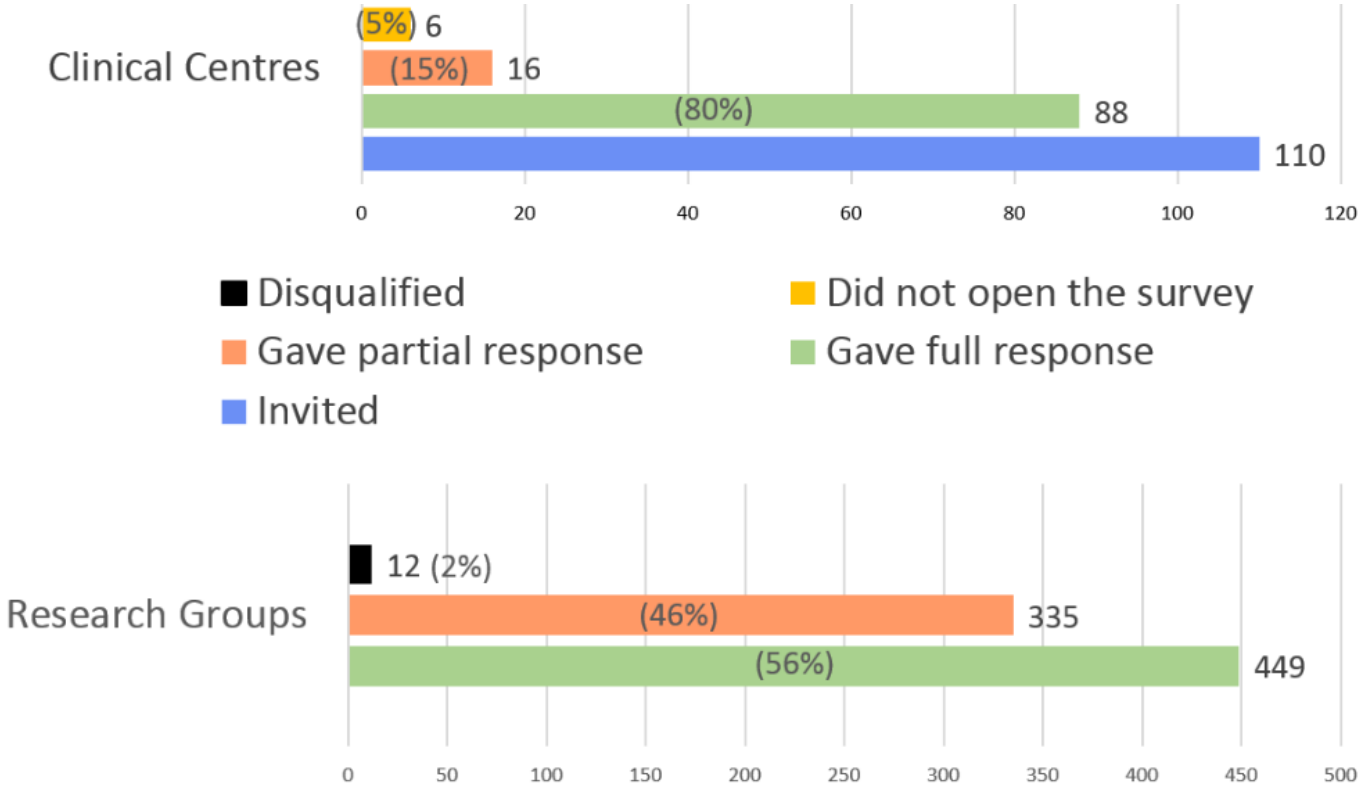


Surveys on the status quo in regulatory training activities in Europe

Clinical research centers & academic research groups

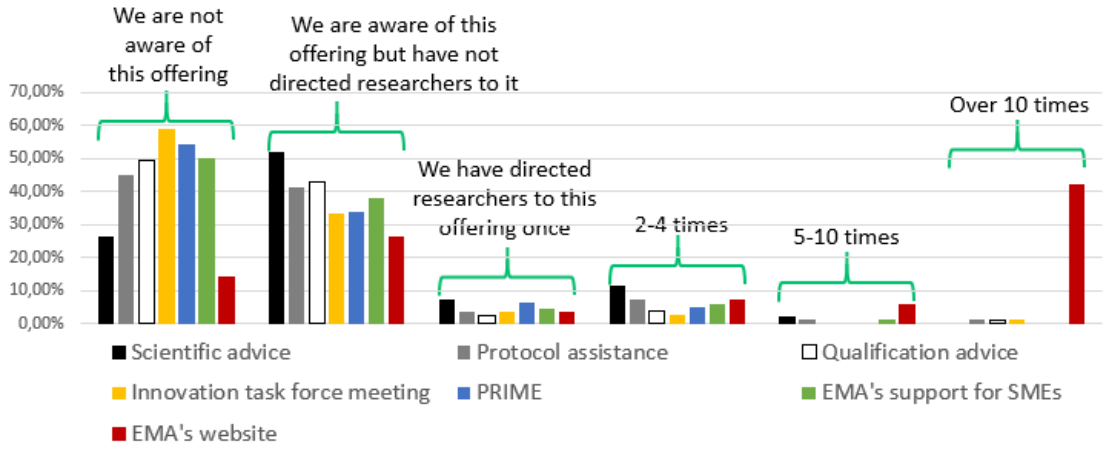
- Awareness/knowledge about sources of regulatory guidance and support and their usage
- Challenges in regulatory matters for academic researchers
- Training needs

First Results - Surveys

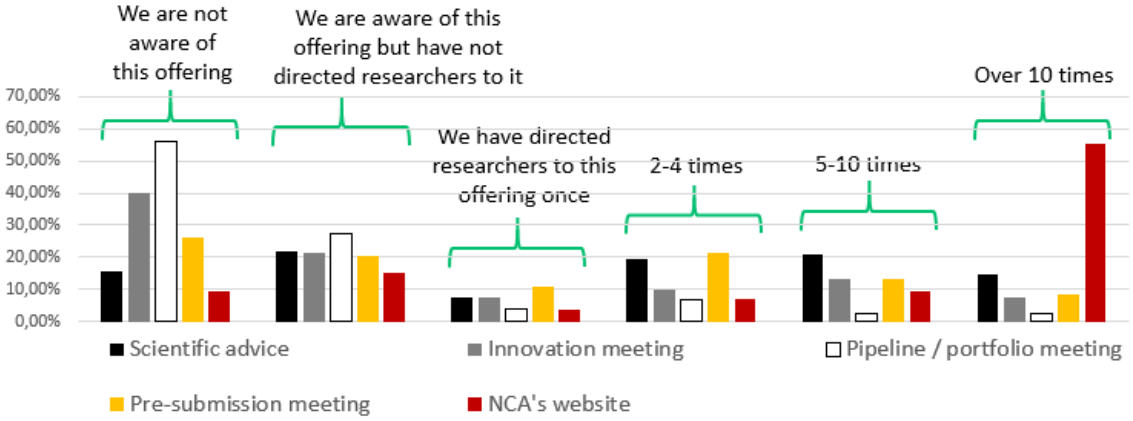


Awareness and utility of EMA's and NCA's support 2014-2018 (Survey for Clinical Centres)

EMA's support offering

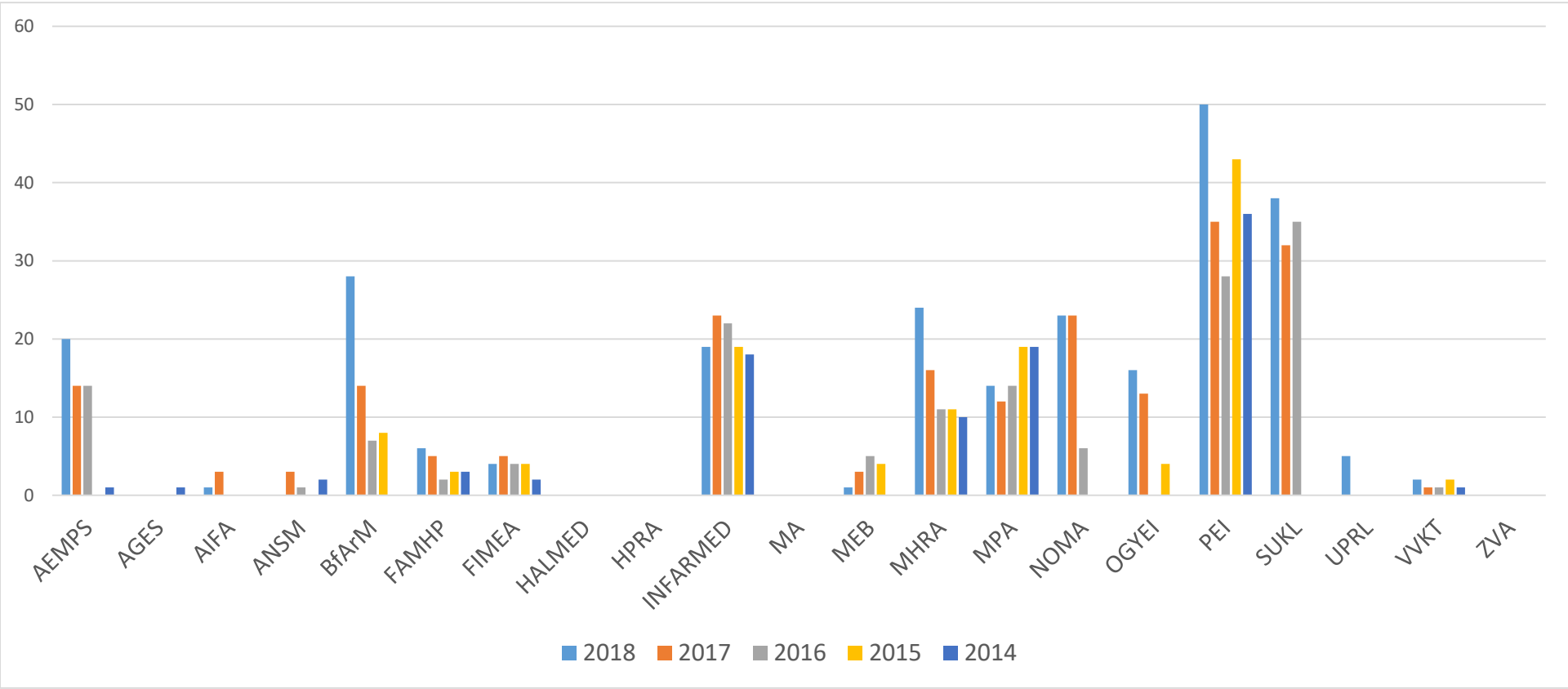


NCA's support offering



- The best known and most used support offerings of NCAs and EMA are the web-based services.
- Many centres are not aware of the other offerings (e.g ITF and pipeline meetings).
- Recommendation to use EMA's and NCAs' support services such as SA and pre-submission meetings is at modest level (in average, the local support units have directed one of their customers to NCA-SA per a year).

Number of Scientific Advice meetings for Academic/no profit applicants in year 2014-2018



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Are Academic Applicants applying at EMA for SA?

- **In the years 2014-2018 no academic applicant applied for SA**
- **However, academic entities are present at EMA**
  - **But involved in large multinational consortia**
  - **Often combined with industry (e.g. EFPIA)**

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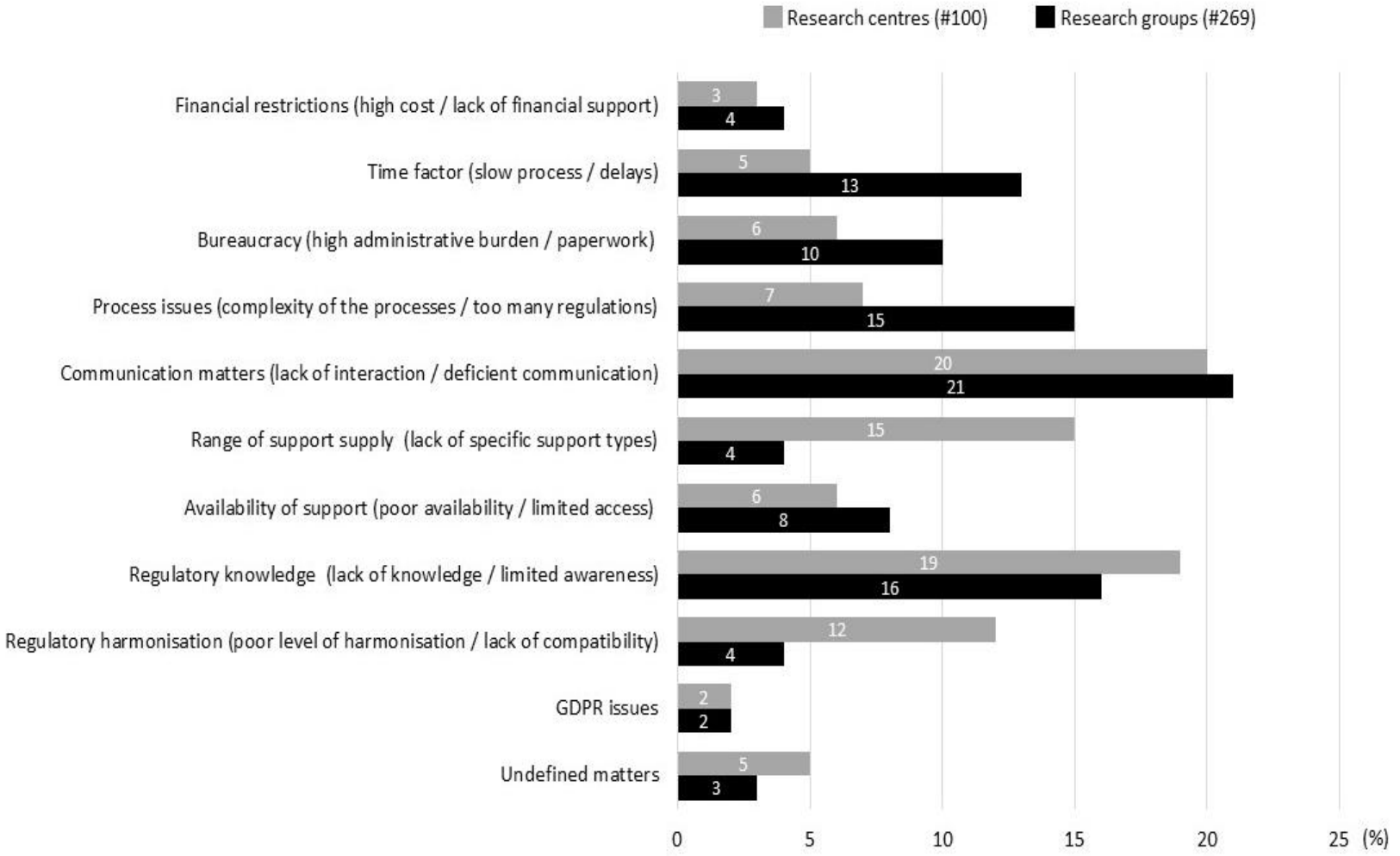
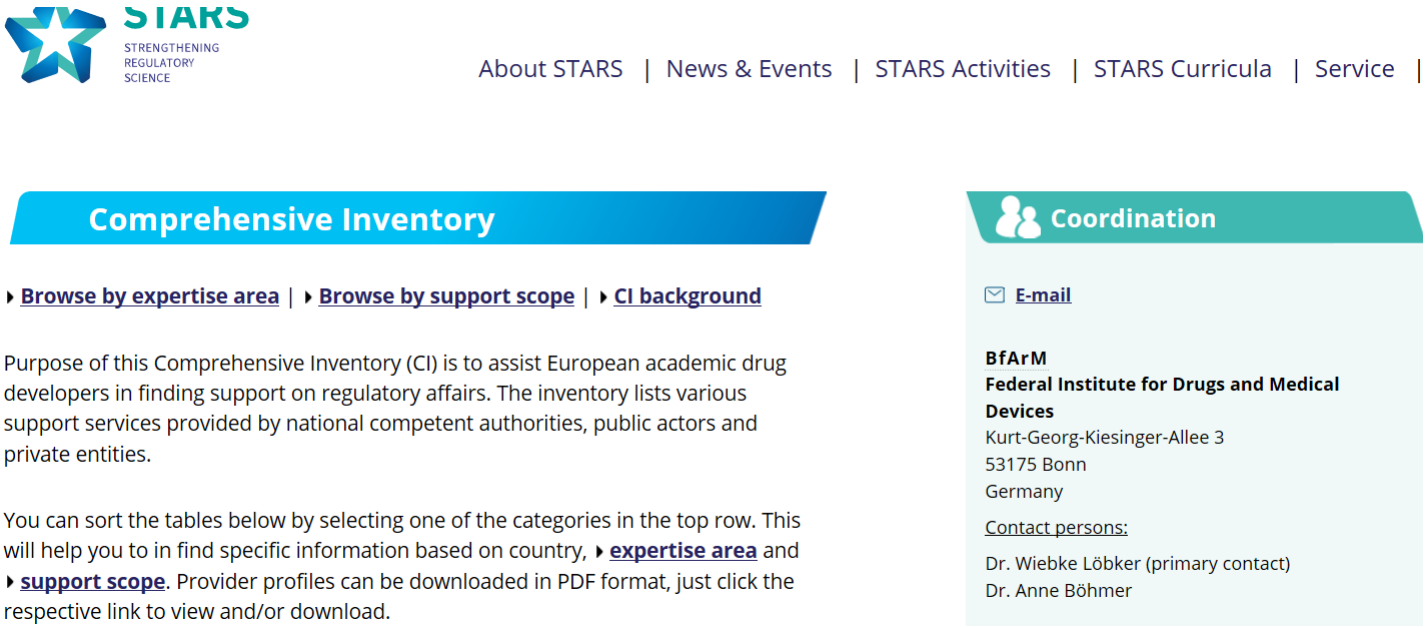
Distribution of open-ended answers to the question “what is the most critical gap or deficiency in the current regulatory system?”



Deliverables of the STARS project

First Results – Comprehensive Inventory

- **The CI offers academics list of support activities; search function – expertise area / support scope**
- **The CI was launched end of July: https://www.csa-stars.eu/Inventory-1721.html**



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About STARS | News & Events | STARS Activities | STARS Curricula | Service |

Comprehensive Inventory

▸ Browse by expertise area | ▸ Browse by support scope | ▸ CI background

Purpose of this Comprehensive Inventory (CI) is to assist European academic drug developers in finding support on regulatory affairs. The inventory lists various support services provided by national competent authorities, public actors and private entities.

You can sort the tables below by selecting one of the categories in the top row. This will help you to in find specific information based on country, ▸ expertise area and ▸ support scope. Provider profiles can be downloaded in PDF format, just click the respective link to view and/or download.

Coordination

E-mail

BfArM
Federal Institute for Drugs and Medical Devices
Kurt-Georg-Kiesinger-Allee 3
53175 Bonn
Germany

Contact persons:

Dr. Wiebke Löbker (primary contact)
Dr. Anne Böhmer

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feature

Strengthening regulatory science in academia: STARS, an EU initiative to bridge the translational gap

Viktoriia Starokozhko[1,2], Marko Kallio[3], Åsa Kumlin Howell[4], Anna Mäkinen Salmi[4], Gunilla Andrew-Nielsen[4], M. Goldammer[5], Manja Burggraf[6], Wiebke Löbker[7], Anne Böhmer[7], Eleonora Agricola[8], Corinne S. de Vries[9], Anna M.G. Pasmooij[1] and Peter G.M. Mol[1,2]
, p.mol@cbg-meb.nl, p.g.m.mol@umcg.nl on behalf of the STARS consortium

https://www.sciencedirect.com/science/article/pii/S1359644620304347

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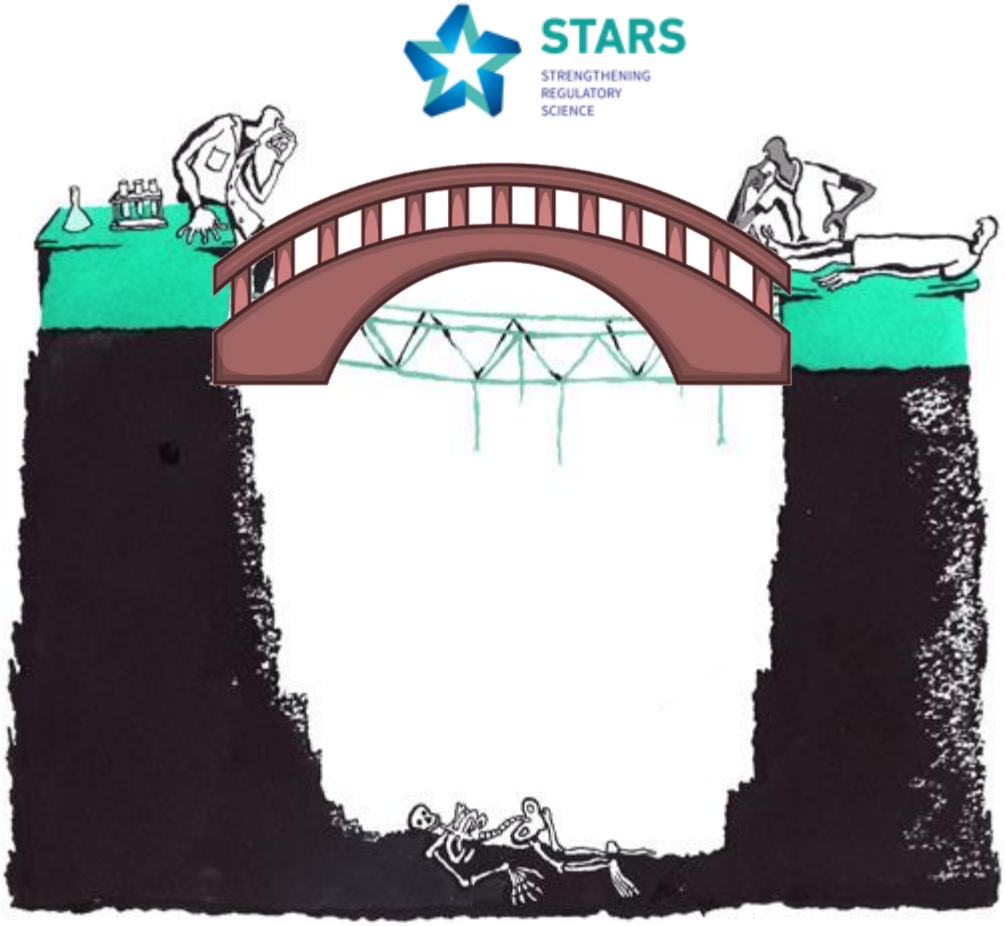
	Further steps
Academia	- Implementation of regulatory science in educational programs of medical, biomedical and pharmaceutical professionals - Implementation of regulatory science in the translational research plan by planning an early dialogue with regulators - Proactive communication with regulatory authorities and funding bodies throughout the development - Timely attention to the translation of findings into clinical practice or next development stage
Funding Bodies	- Proactive communication with regulatory authorities when taking funding decisions - Scrutinize grant proposals in the area of applied and translational research for adoption of best practices - Actively encourage dialogue between academics and regulators to help ensure maximum impact of the research - Interest in a more defined public impact of funded research projects - Reward projects that are of high public impact independently of their novelty (i.e. projects that involve paediatric formulations, new dosing regiments or new treatment combinations)
Regulatory agencies	- Active dialogue *versus* one-way communication - Open-minded communication - Continuous learning about upcoming innovative therapies through knowledge exchange with academia, i.e. invited lectures, conference attendance - Timely alignment of the regulatory requirements with the evolving developments - Involve academia in guideline development

- **Pilot I - Transfer of best-practice**
  - Topic for Pilot I: Short term training in essential regulatory knowledge
  - Transfer from NCA (**NL**, CZ) to NCA (HU, IT, AT)
  - 23-26 February 2021
  - https://www.csa-stars.eu/Results-Pilot-I-Best-Practice-Transfer-1754.html

1st European Stakeholder Workshop of STARS on November 3rd/4th, 2020, entitled: ”Towards an Improved Strategy for Regulatory Support for Academia”

Update on 1st European Stakeholder Workshop

- academic researchers, representatives of national health care systems and health ministries, patient representatives, pharmaceutical industry, leading academic societies and national and European funding agencies
- view exchange: identify gaps and write preliminary recommendations
- 100 participants
- https://www.csa-stars.eu/First-European-Stakeholder-Workshop-1739.html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2nd European Stakeholder Workshop of STARS on November 17th/18th, 2021, entitled: ”Towards an Improved Strategy for Regulatory Support for Academia”

The objectives of this second workshop are:

- to present the development and draft of the STARS Common Strategy
- to discuss the document and the recommendations that have been developed so far based on STARS activities and the partners expertise
- and to further sharpen the Common Strategy based on Stakeholder’s input during the workshop

The workshop is by invitation. In case of interest, please send an e-mail to science@cbg-meb.nl.

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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.

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Any questions?

am.pasmooij@cbg-meb.nl