CBG MEB

COLLEGE TER BEOORDELING VAN GENEESMIDDELEN

MEB Science Day 2023

Biomarkers and Companion Diagnostics
The future of precision medicine

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COLLEGE TER BEOORDELING VAN GENEESMIDDELEN

A status update on the MEB Science Policy 2020-2024

Marjon Pasmooij

Science Department, Medicines Evaluation Board

13 April 2023

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MEB Science Policy 2020 - 2024

"To make medicines available that are both safe and effective, we need to do our job as good as possible. Regulatory Science research helps us to improve our assessment of medicines, to gather more knowledge and thus to do our work better"

Prof. Marcel Bouvy, MEB Board member

https://www.cbg-meb.nl/documenten/publicaties/2021/04/29/wetenschapsbeleid-2020-2024

MEB Science Policy 2020-2024

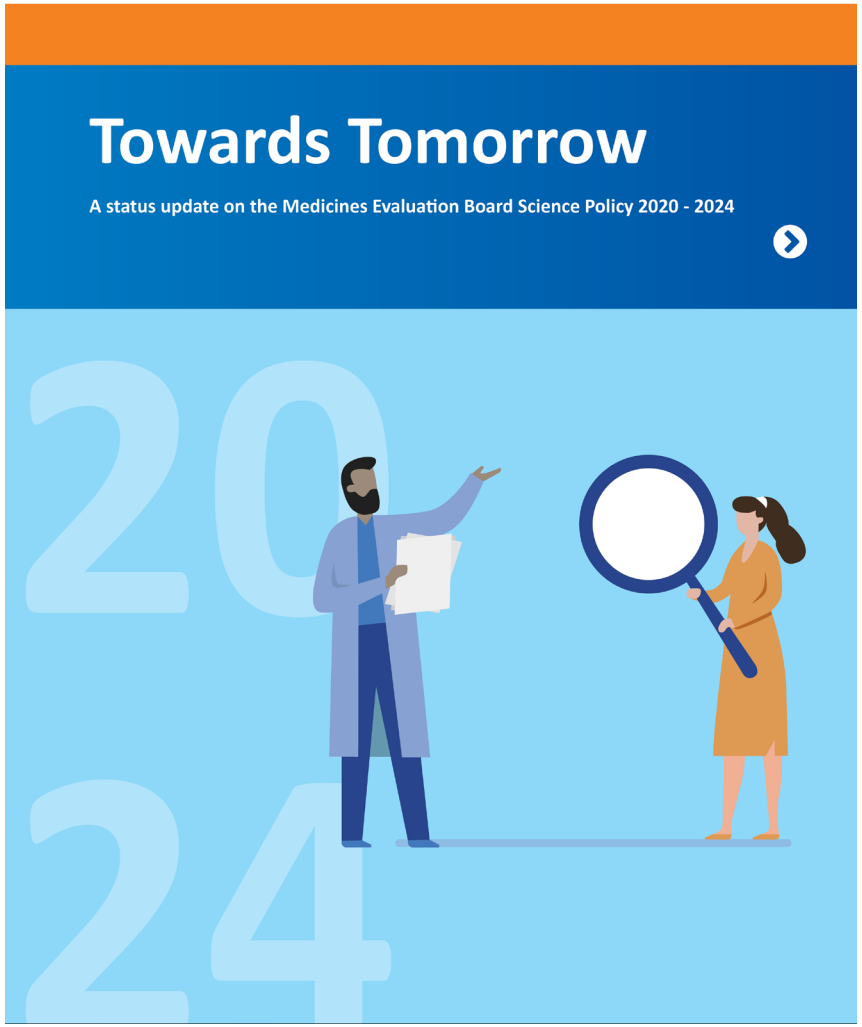
- In line with Strategic Business Plan 2020 – 2024
- Regulatory Science research connected to the assessment work, and aimed at improvement or innovation
- Research questions based on questions from own organisation
- (More) Attention to the connection of scientific research, education and the daily assessment work
- More attention for the patient perspective

Eight themes

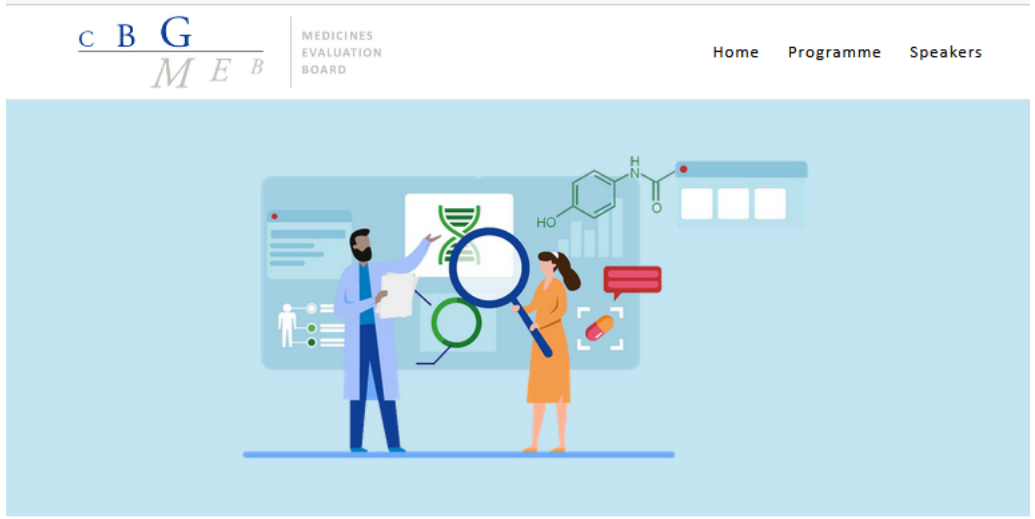
1. Replacement, reduction and refinement of animal tests (3Rs)
2. Advanced Therapy Medicinal Products (ATMPs)
3. Data-driven assessment
4. Personalised medicine & biomarkers
5. Medical devices
6. Generics
7. Medicines used better
8. Safety and effectiveness after authorisation

Status Update MEB Science Policy 2020 - 2024

Today published…



Status Update MEB Science Policy 2020 - 2024



MEB SCIENCE DAY 2023

Thursday 13 April 2023
Time: 11.45 – 18.00
Location: Auditorium Mediaplaza, Jaarbeurs Utrecht

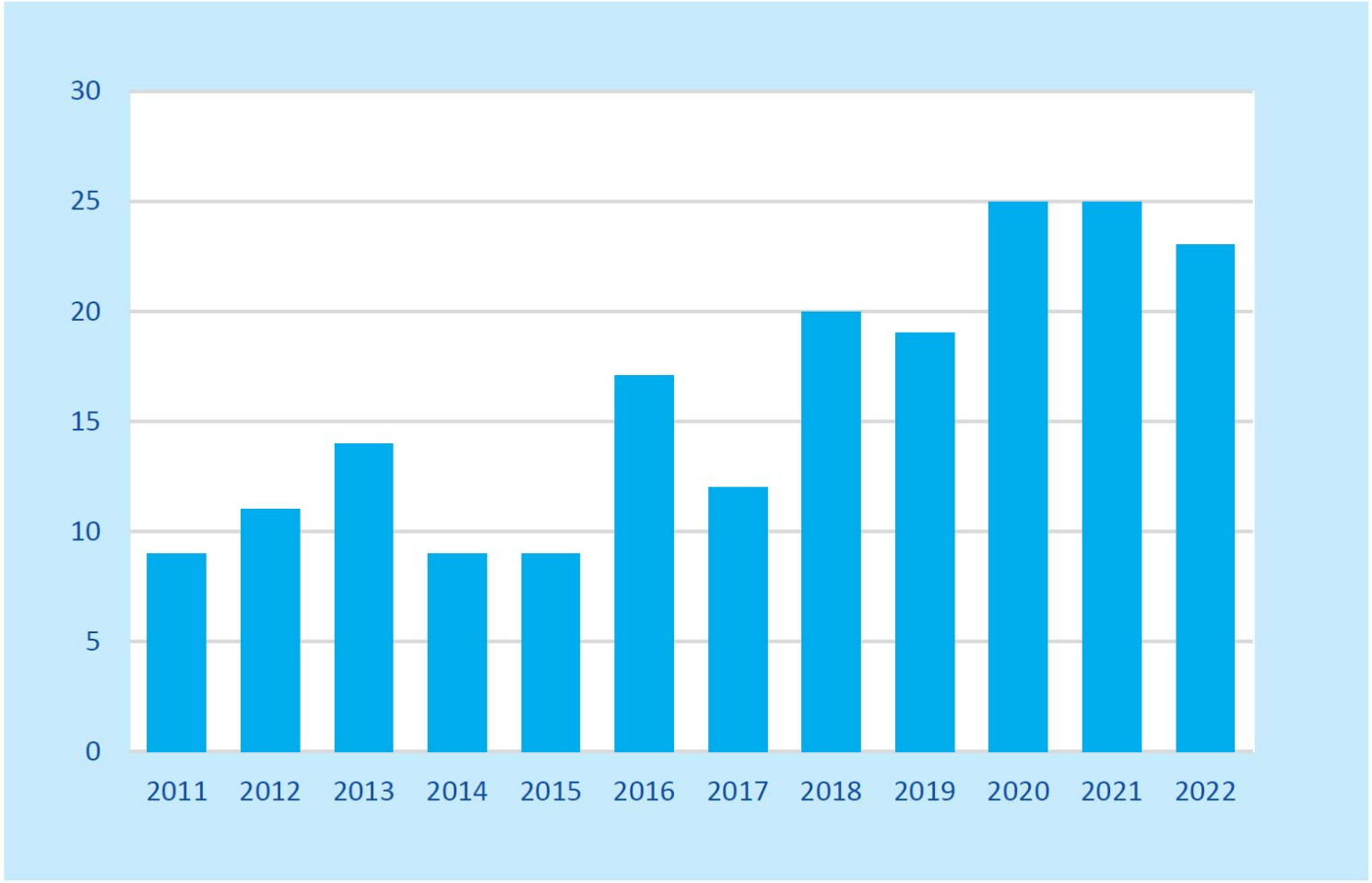
Towards Tomorrow

A status update on the Medicines Evaluation Board Science Policy 2020 – 2024

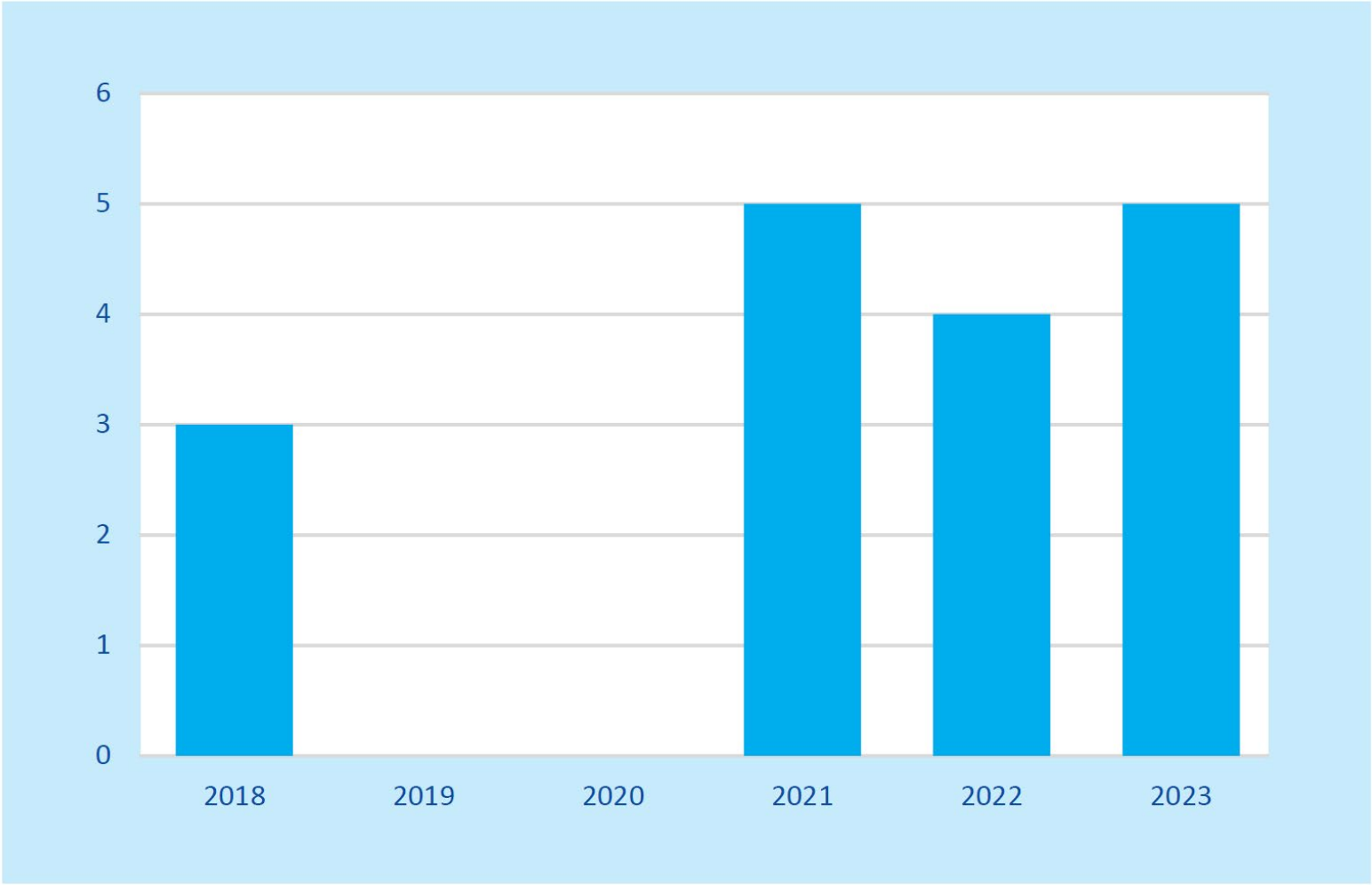
The MEB adopted eight main themes within the Science Policy 2020–2024 "Regulating with the Knowledge of Tomorrow". Many of the themes are relevant for developing medicines for humans and animals. In this brochure, the MEB zooms in on the progress we have made for each of these themes. Furthermore, based on developments in the last few years, we describe progress in other areas, such as male/female differences and sustainability. Lastly, we highlight two specific projects: STARS (Strengthening Training of Regulatory Science in Academia) and the European Medicines Regulatory Database. Many scientific activities were performed in collaboration with bachelor's, master's and PhD students. This brochure also includes interviews with all PhD students.

Update MEB Science Policy 2020-2024

Bachelor's and master's students per year



PhD defences per year



PhD defences in 2023

Remy Francisca (Erasmus MC)

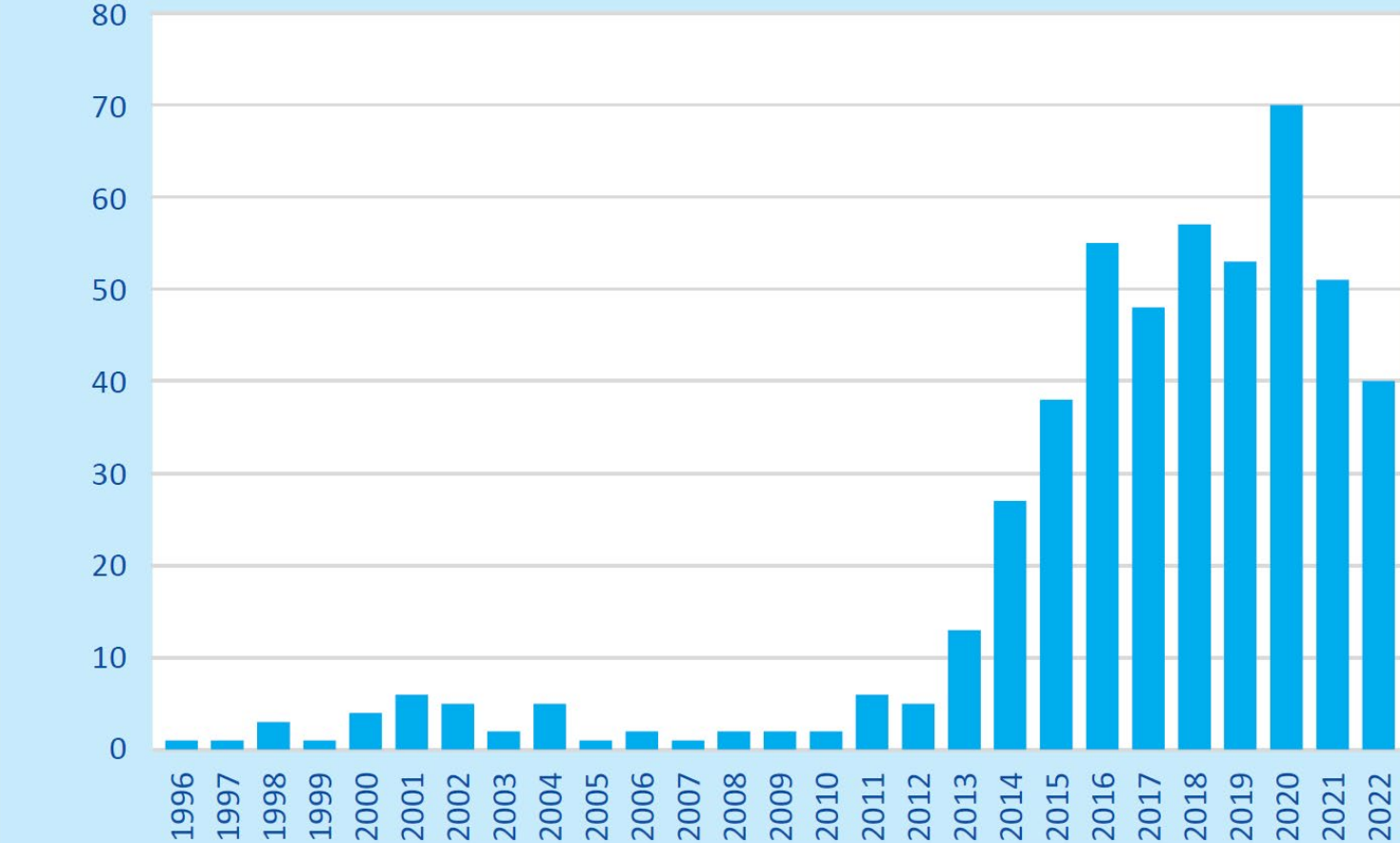
Pieter Glerum (UM)

Désirée Veening-Griffioen (UU)

Nafise Ghalandari (Erasmus MC)

Jorn Mulder (UU)

Publications per year with MEB affiliation



Eight themes

Personalised medicine and biomarkers

4.4 Personalised medicine and biomarkers

“Precision medicine” has become a topic of key interest in the scientific, regulatory and public domains. Due to rapid advances in science and specifically understanding disease pathophysiology, underlying biological pathways and specific molecular targets, a more targeted approach to diagnostics and treatment is possible. The concept of precision medicine aims to identify early-in-development investigational medicinal products with a promising treatment effect along with the patients who would respond to treatment.

In addition, precision medicine refers to subjects who may not respond to treatment but suffer from severe or rare adverse events. Finally, dose-finding can be facilitated by the current understanding of molecular targets and pharmacogenomics. Regulatory flexibility is required to stimulate the development and expedite the authorisation of promising medicinal products intended to treat life-threatening diseases.

> Biomarkers are biological markers indicating that someone is sick, which can predict how serious an illness will be or show whether a treatment is working. One example of this is PDL-1 protein expression for immunotherapy in the context of oncology.

Alignment of "Precision Medicine" Drug Development Trajectories With Regulatory Decision-Making Needs

Lysbeth Bakker[1]
Promotors: Prof Peter G. M. Mol[1,2]*, Prof Hiddo J. H. Lambers Heerspink*[1]
Co-promotor: Dr Viktoriia Y. Starokozhko[1,2]

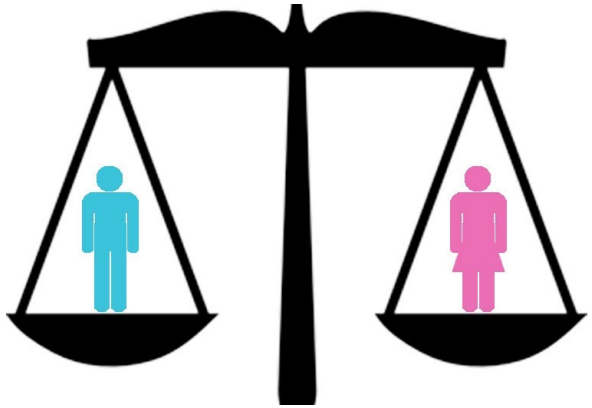
1. *Department of Clinical Pharmacy and Pharmacology, University Medical Centre Groningen, University of Groningen, Groningen, The Netherlands*
2. *Medicines Evaluation Board (MEB), Utrecht, The Netherlands*

September 2019 - June 2023

Lysbeth Bakker studied at the University of Groningen. She started with a Bachelor of Life Science and Technology, finishing in the biomedical sciences and behaviour and neuroscience tracks. During her bachelor's, she minored in pharmacy, which caused her to continue in the direction of medicines. After obtaining her bachelor's degree, she started a Master in Medical Pharmaceutical Sciences with a Pharmacoepidemiology track. In the second year of her master's, she interned at the MEB, mostly in the office in Groningen, under the supervision of Peter Mol. In September 2019, Lysbeth started a PhD under Peter Mol's supervision in regulatory science. She worked closely with MEB and EMA colleagues on several of the projects.

What is the topic of your PhD?

Other developments



Male / female differences

STARS

STRENGTHENING
REGULATORY
SCIENCE

STARS project

Sustainability

European Medicines Regulatory Database (EMRD)

EMRD

Universiteit Utrecht

CBG MEB

European Medicines Regulatory Database

Dashboard Tour

To explore all the features of this dashboard, take a guided tour around the website by clicking the button. This tour will not take long and familiarize you with all the core functionalities that this dashboard has to offer. You can end the tour at any time by clicking 'end'. When you reach the end of the tour, the tour will automatically finish.

Start Tour

Tools

This dashboard provides tools for looking up, filtering, and visualizing data points regarding pharmaceutical policy and regulation. All functionality is split up into two pages: *Data* and *Visualize*.

Data

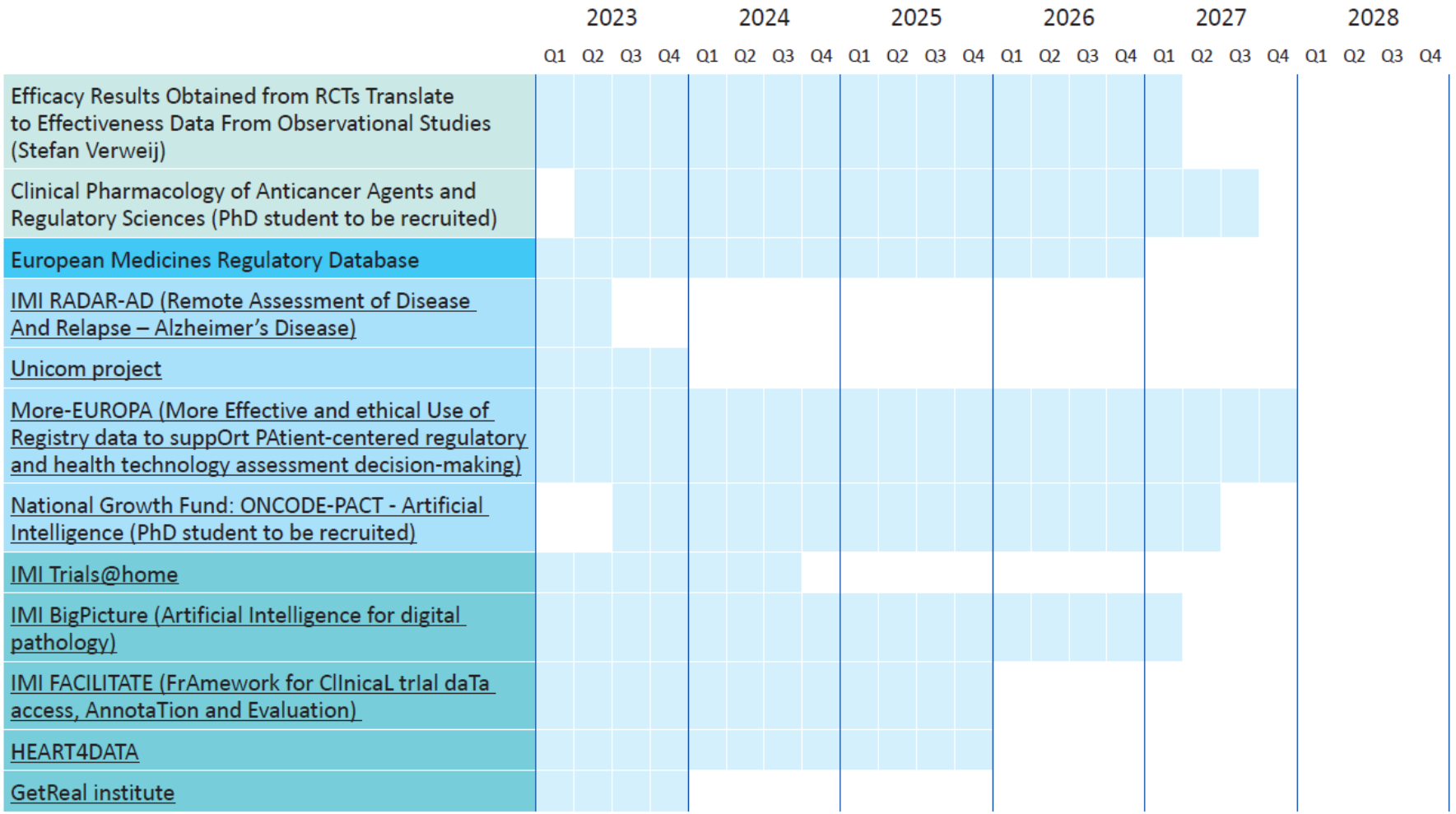
The data page is optimized to find data points quickly. Searching can be done based on keywords and letter combinations, but filters can also be applied to ensure that only data points that meet specific (parameter) requirements appear. The data points can be sorted by a parameter of your choice. Finally, a selection can be made manually from the remaining data points, which can be used for visualization or exported to a local file if desired.

Visualize

The data points selected on the data page can be displayed graphically on the visualization page. An unlimited amount of visualizations can be added, each with its own title. There are four types of charts to choose from: a bar chart, a line chart, a pie chart, and a histogram. For each of these chart types, it can be determined which variables are shown on the axes and whether labels or a legend should be shown. For these variables, you can choose which values/categories are shown or skipped in the diagram. For bar charts, you can also choose to display the chart horizontally and to stack the bars ("Stacked") so that fewer bars need to be shown. If the latter is chosen, you can also choose to make all bars the same length ("Fully stacked") so that the graph is expressed in relative quantities. For line charts and histograms, it is possible to zoom in on a subset of the diagram. For any diagram, all quantities

Overview of Regulatory Science Projects

Theme 3: Data-driven assessment



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