Survey results: Use(-fulness) of RWE in regulatory decisions

Multi-stakeholder workshop on Real World Data (RWD) quality and experience in use of Real-World Evidence (RWE) for regulatory decision-making
26-27 June 2023

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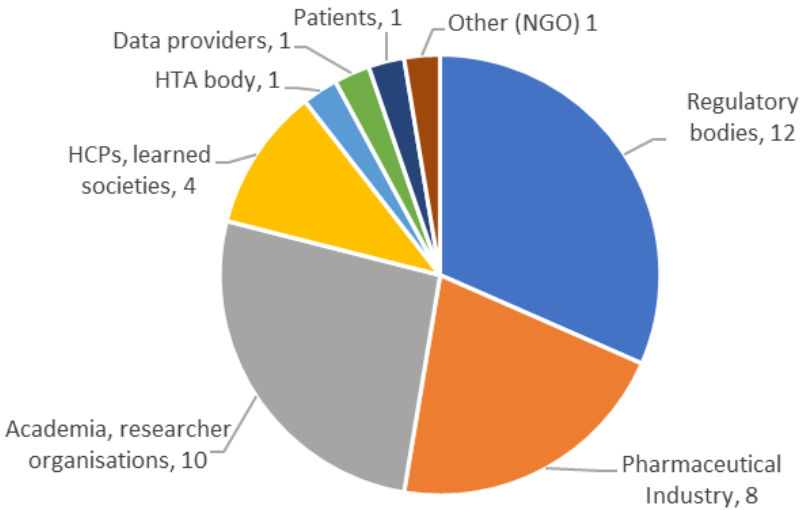
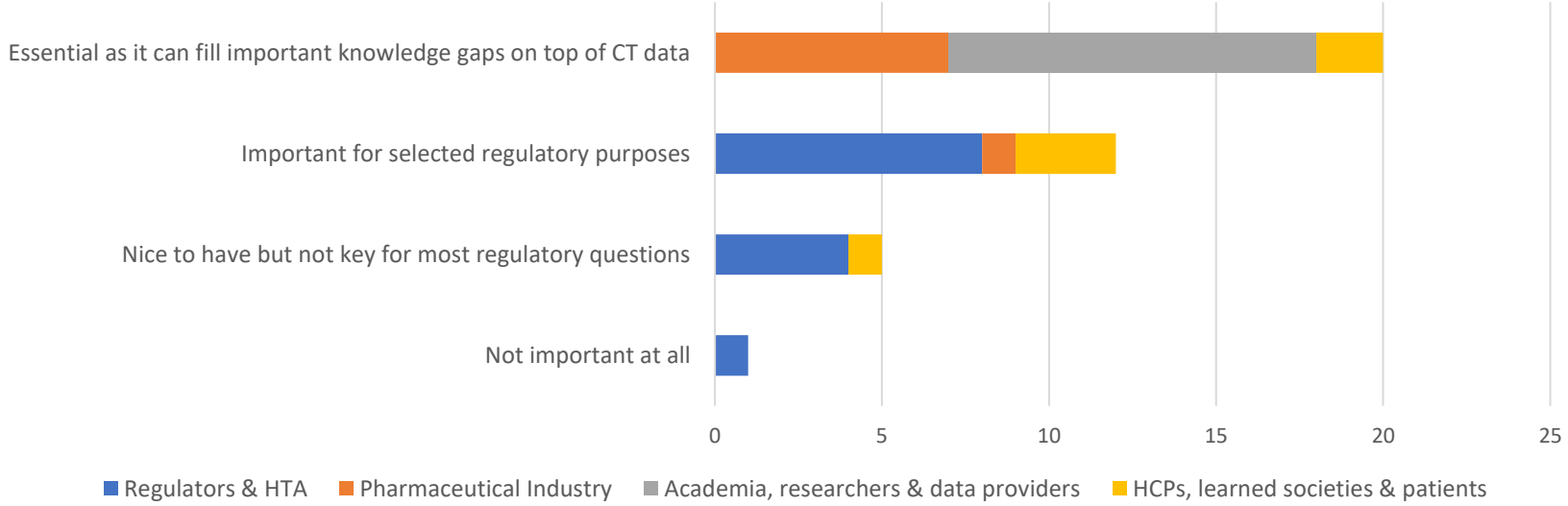
Background on the survey

Objective: Get a **snapshot** of the different views on **opportunities and current challenges to fully integrate RWD/RWE in regulatory decisions**

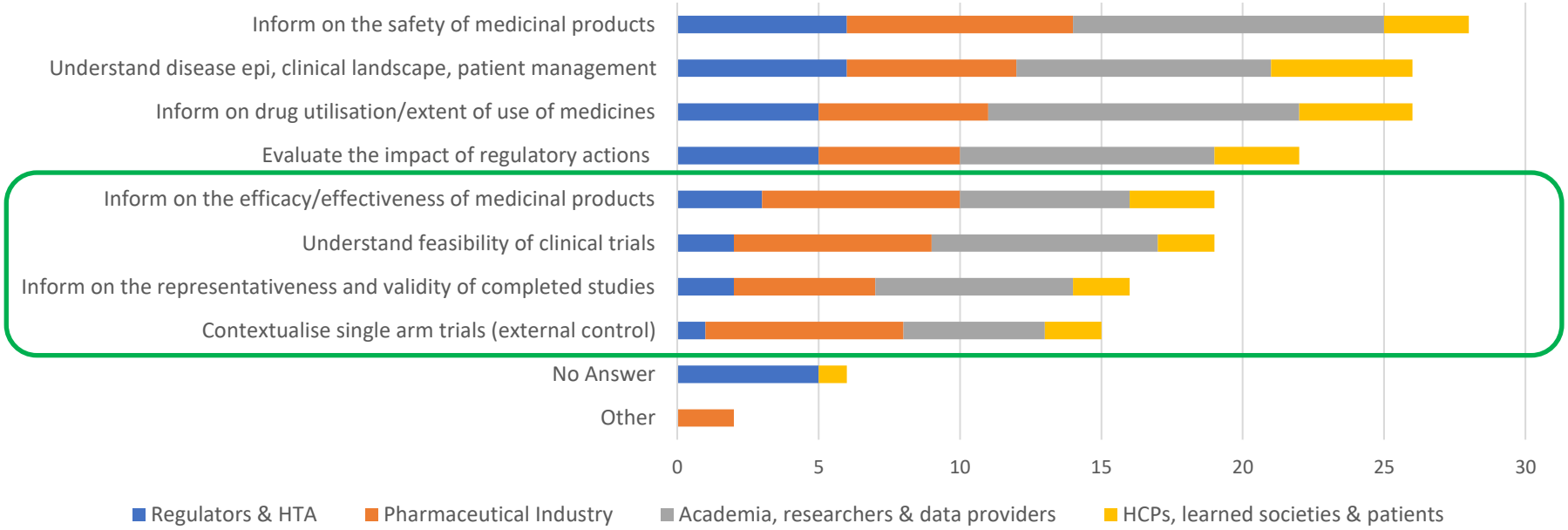
- Survey sent to participants of the workshop (up until closing date 19/06)
- **Number of responses: 38**
- Results presented for 4 responder groups:
  - Regulators + HTA bodies
  - Pharmaceutical industry
  - HCPs, learned societies, patients & NGOs
  - Academia, researchers, data providers



1. **How important** would you currently rate the **use of RWD** to generate evidence for regulatory decision-making?



For which **regulatory purpose** do you consider RWD most suitable/relevant? *(multiple choice)*



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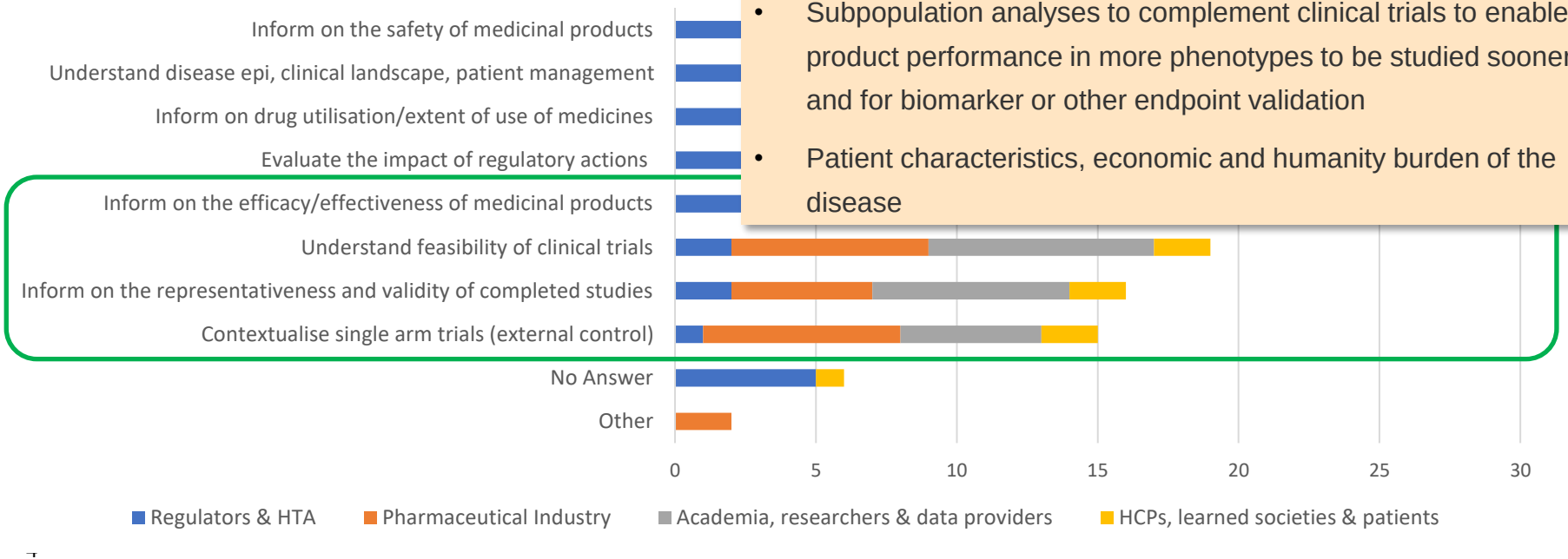
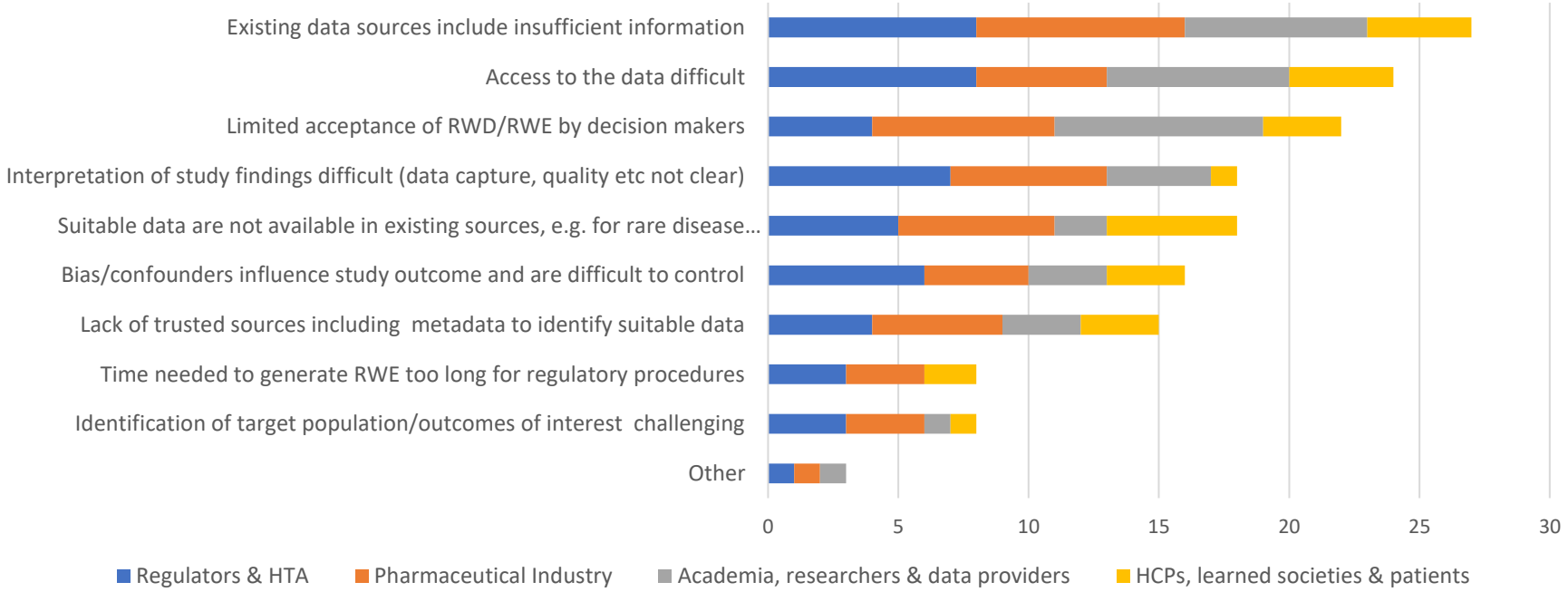


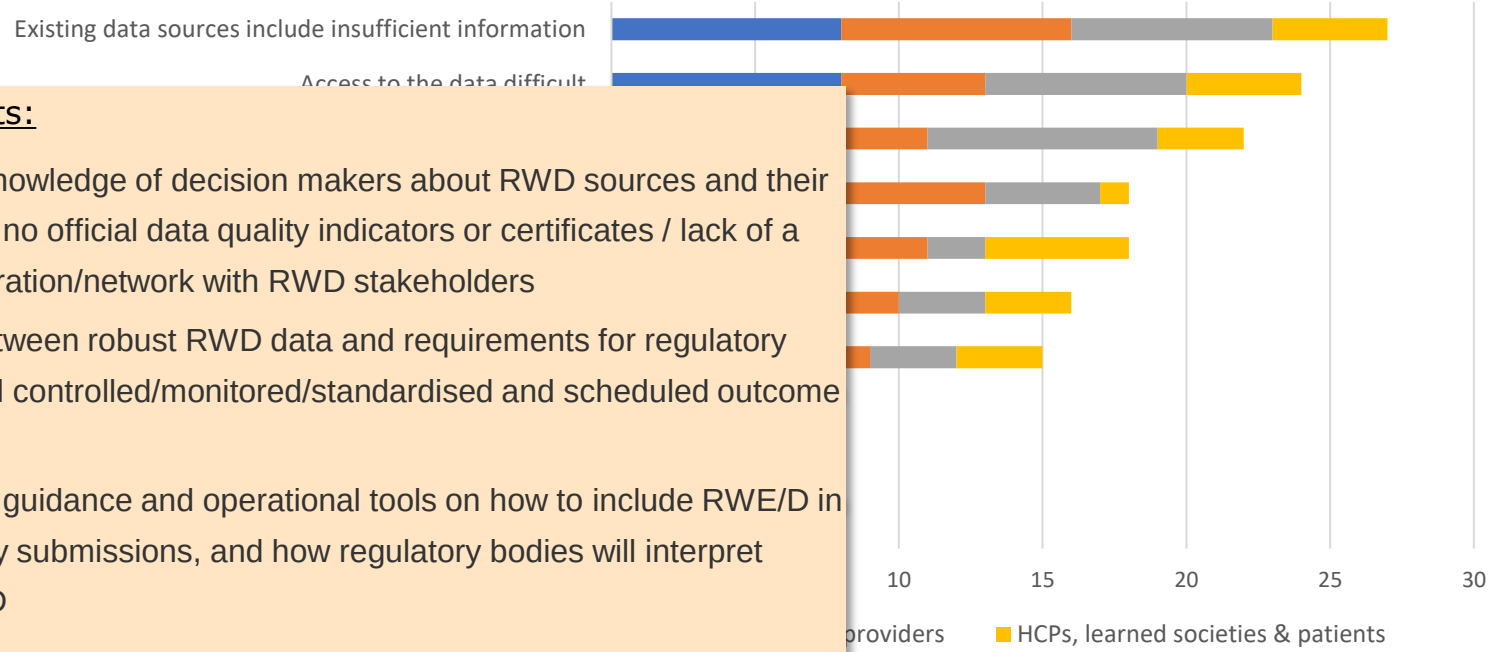
Other comments:

- Subpopulation analyses to complement clinical trials to enable product performance in more phenotypes to be studied sooner, and for biomarker or other endpoint validation
- Patient characteristics, economic and humanity burden of the disease

2. Where do you currently see the **main challenges** to fully integrate RWD/RWE in regulatory decision-making? *(multiple choice)*



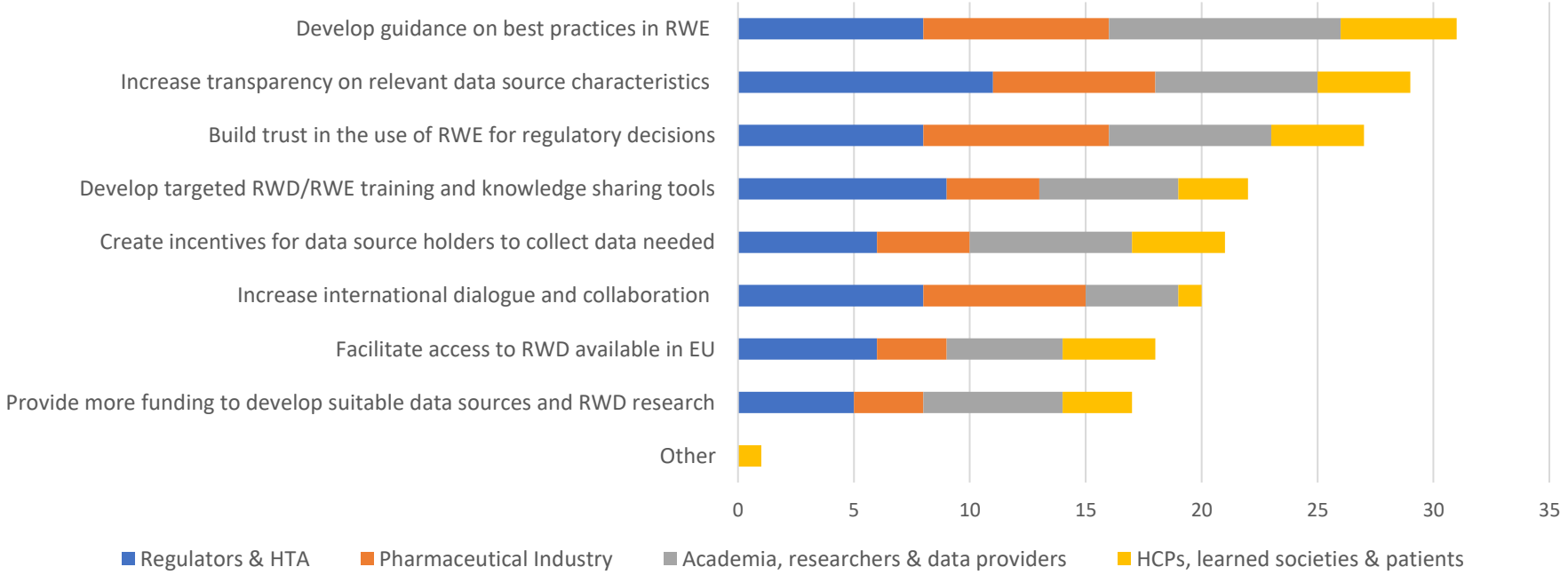
2. Where do you <u>currently</u> see the **main challenges** to fully integrate RWD/RWE in regulatory decision-making? *(multiple choice)*



<u>Other comments:</u>

- Insufficient knowledge of decision makers about RWD sources and their data quality / no official data quality indicators or certificates / lack of a stable cooperation/network with RWD stakeholders
- Mismatch between robust RWD data and requirements for regulatory decision (well controlled/monitored/standardised and scheduled outcome assessment)
- Lack of clear guidance and operational tools on how to include RWE/D in the regulatory submissions, and how regulatory bodies will interpret these RWE/D

3. What needs to be done in order to **fully enable the use** of RWE in regulatory decision-making? *(multiple choice)*



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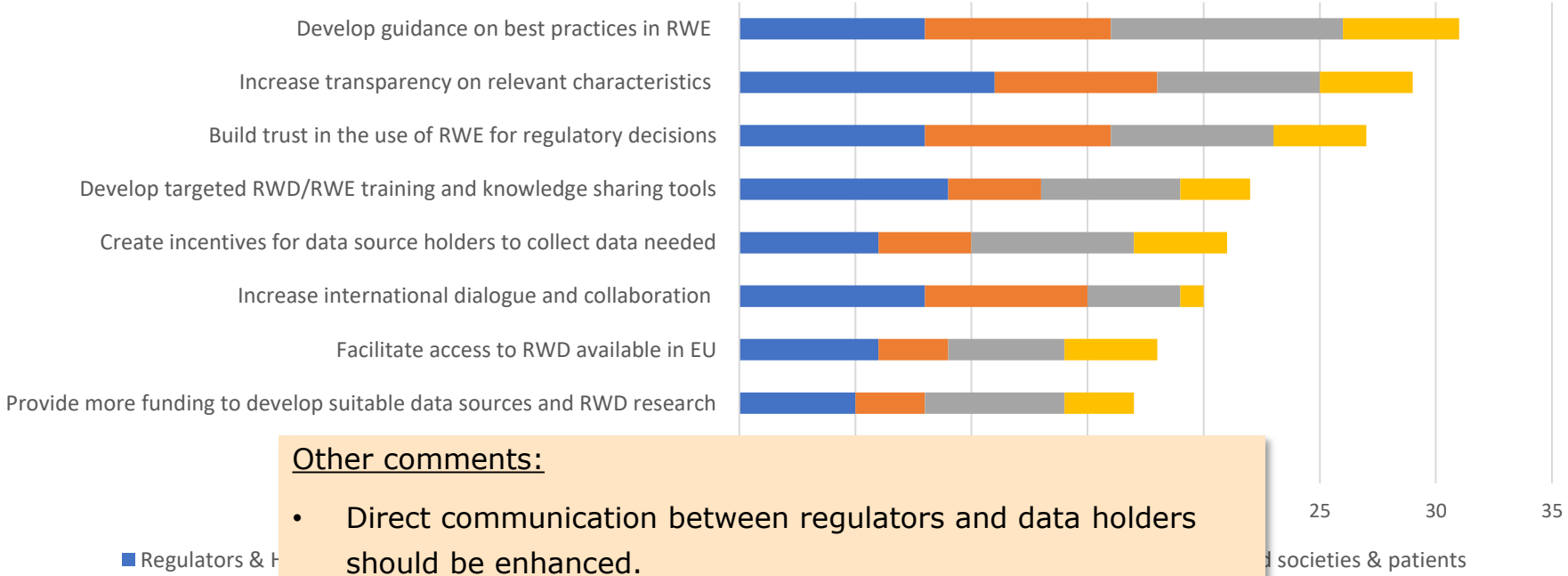
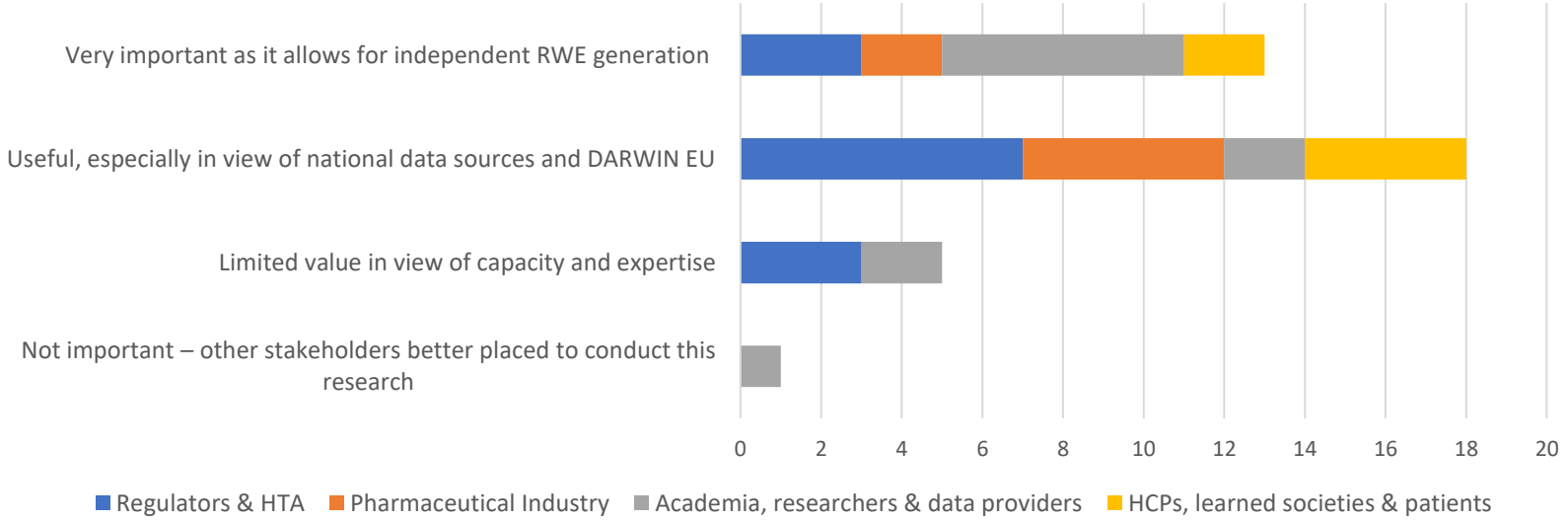


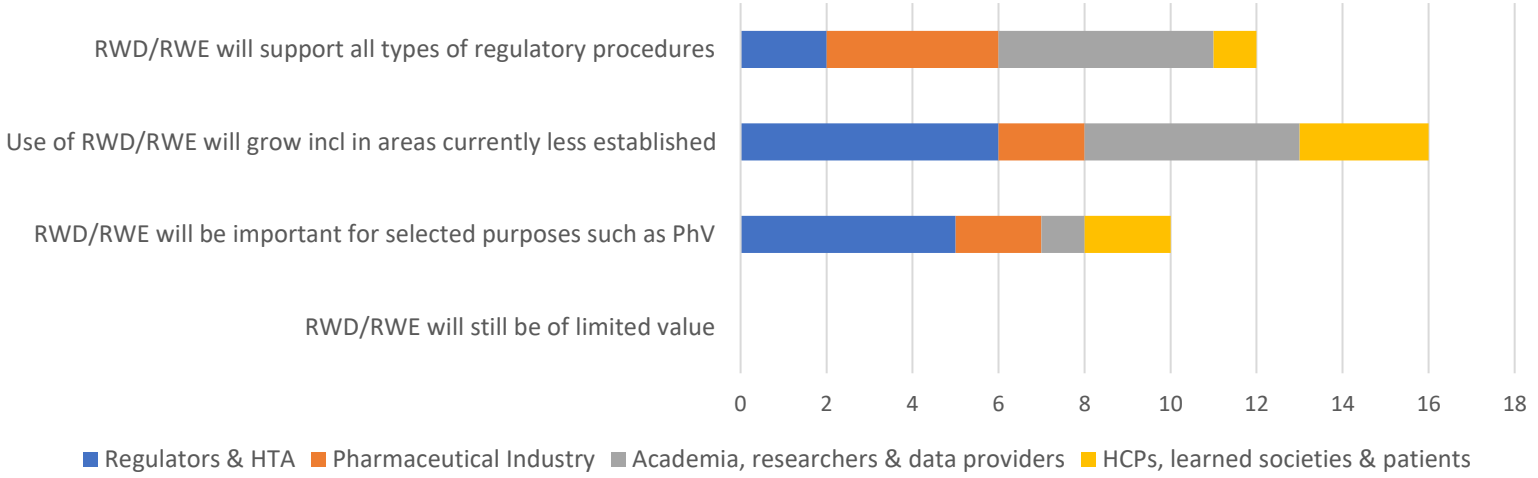
Other comments:

- Direct communication between regulators and data holders should be enhanced.

4. How do you see the **role of regulators in generating RWE**?



5. How do you see the **use of RWD/RWE** for regulatory purposes evolving in the next 5 years?



5. Any other comments? (abbrev.)

RCTs presents bias in selection of individuals (gender, age, comorbidities) if compare with RWE/RWD. Eudravigilance website should be improved and should be streamlined otherwise physicians will never use it, and it will continue as now with a clear underestimation of any clinical mild/serìous/life-threatening phenomenon.

The emergence of artificial intelligence in the regulatory setting makes the uses of real-world data and evidence ever more critical.

Much effort has been made by EU regulators to advance the use of RWE in decision making. However, quality, transparency of data sources needs to be further increased and access to data sources by all relevant stakeholders is key to generate mutual trust in RWE and regulatory decision making on data sets which include RWE.

Clarification of the role of stakeholders and the conflict of interest - which is not necessarily about funding - is key in the successful use of RWE in the future of medicines and health technology evaluation.

Presenting papers without quality appraisal (assessing risk of bias - counfounding, immortal time bias, etc), information about methodology selection of studies has been the case in regulatory submissions. Transparency and trust is key. RCT rules should be followed when submitting observational data for regulatory purposes incl pre-registration of protocols (including SAP), database feasibility assessments, justification of methodology choice a priori.

5. Any other comments? (continued, abbrev.)

RWD might also be used as part of registry based randomized clinical trials. The utilization of RCTs based on and/or including structured RWD from registries and/or EHR and eventually extracted by AI-methodology might substantially change the generation of evidence both for regulatory approval and treatment recommendations in the near future.

Promote prospective planning of RWD collection already early in development, categorise robust endpoints and quality indicators for data, first focus on areas for RWD where clinical trials are especially limited (long-term patient trajectories; risk stratification; neglected populations in clinical trials)

Critically important to build trust between regulators and the RWD/RWE community. RWD are almost never perfect for any use case, but that should not stop the community from using it. It is easy to criticize RWD in comparison to a clinical trial, but that is a false comparison because clinical trials are not feasible to answer every important public health question. The appropriate comparison is RWD compared to no information at all. We have to stop covering our eyes demanding perfection and accept that RWD has a critical place in supporting public health.

Although I think the use of RWD/E will increase over the next five years, it will likely still have more of a role in informing study planning/filling gaps for decision making on effectiveness rather than being the main source of evidence. To fully enable use of RWE for decisions about effectiveness, better data capture, more proof of concept, better methods, etc. is needed.

It would be beneficial if authorities incentivize the industry to generate and utilize RWD/RWE in MAA processes: generate more and robust data also to understand the applicability in "real" life outside of a trial setting.

Any questions?

Further information

[Insert relevant information sources or contact details as applicable.]

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