Open source workflows for HTA
insights and implications from a pharma industry survey

Rossella Belleli (Roche), Amelie Elsäßer (Boehringer Ingelheim),
Nermina Ferizović (University College London),
Anders Gorst-Rasmussen (Novo Nordisk)

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"We are a community dedicated to leading and promoting the use of statistics within the healthcare industry for the benefit of patients."

Acknowledgement

Rest of HTA ESIG Open Source subteam

- Cedric Revil (MSD)
- Ian Bridges (Amgen)
- Iain Bennett (AbbVie)

Disclaimer

Views and opinions expressed are those of the authors
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3

What will I tell you?

1   HTA Open Source needs dedicated work

2   There is a window of opportunity with EU HTA coming
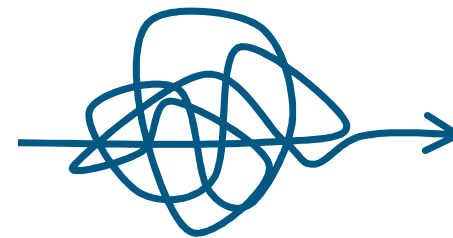
3   A concerted effort is needed to build momentum

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pharmaverse

+ More robust and well-thought-out solutions

+ Greater potential to bring unified solutions to regulators

+ Better enablement of our medicines to reach patients and society faster through the pooling of skills and talents

+ Talent attraction and increased transparency of our industry

?

Drug development → **Marketing authorization** → → **Patient access**

REGULATORY ASSESSMENT

HTA (HEALTH TECHNOLOGY ASSSESSMENT)

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Open source in HTA

HTA transparency focus aligns with the open source mindset; also, open source tools and co-creation have precedence in HTA

> "HTA is a **transparent and accountable process** that can be used by decision makers and other stakeholders to support the decision-making process in health care at the policy level by providing evidence about given technologies
> https://www.who.int/health-topics/health-technology-assessment

R routinely used in NICE Decision Support Unit Technical Support Documents

'R for HTA' academic consortium (R in cost-effectiveness models)

Collaboration set up by Duchenne UK (patient charity) to **develop tools** and evidence to support HTA, bringing together patient orgs, clinicians, academics, pharma, HTA agencies and other advisers
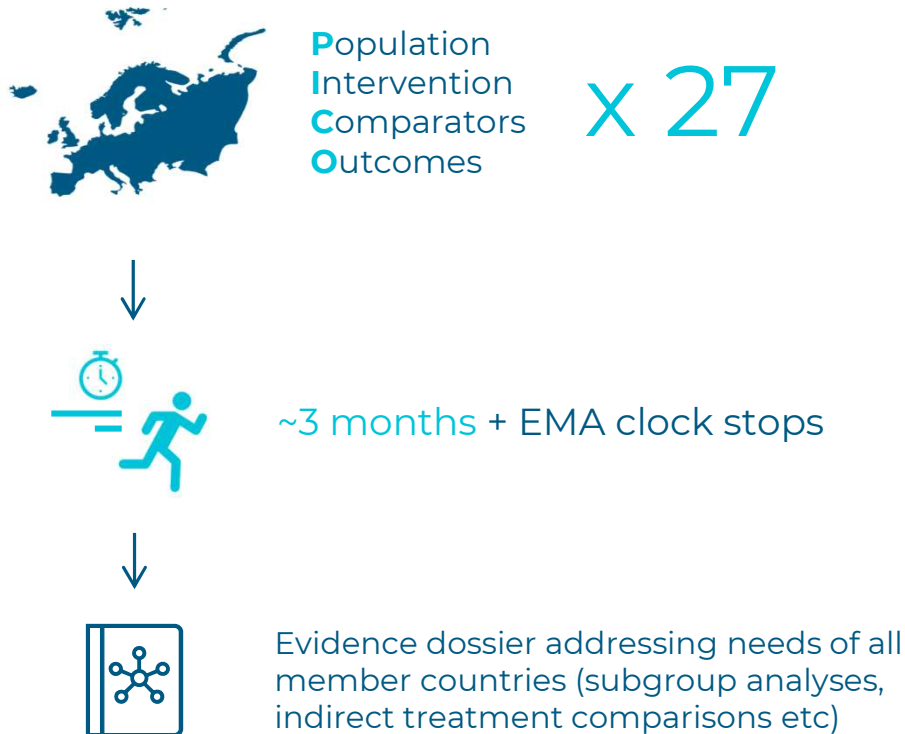
NICE Pathways Pilot: **reference health economic models** for renal cell carcinoma (model available on GitHub for stakeholders)

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Pan-European HTA from 2025

will bring challenges in terms of volume and urgency of statistical work – but also opportunities for working smarter

Population
Intervention
Comparators
Outcomes

x 27

↓

~3 months + EMA clock stops

↓

Evidence dossier addressing needs of all member countries (subgroup analyses, indirect treatment comparisons etc)

'WORK SMARTER' QUESTIONS
that HTA Open Source could help to address

- How can we build robust **automation/ standardization** to help deliver required analyses with short timelines?

- How can industry contribute proactively yet 'at arms length' to **advocate and shape best practice** around statistical analyses for EU HTA?

- How can we shape analytics approaches which best enable **mobility and agility** within and across companies?

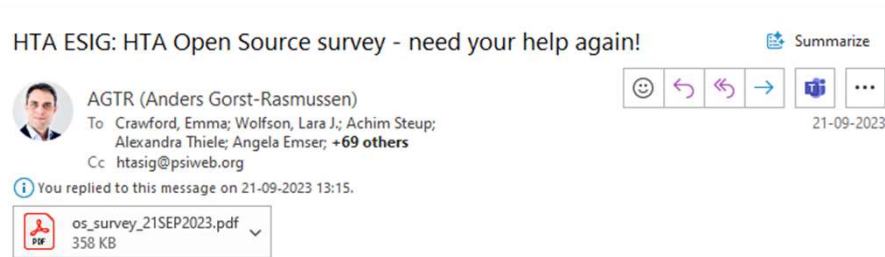- How can we use tools and software to **cultivate best practice** within and across organizations?

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HTA ESIG Open Source survey (autumn 2023)

Small anonymous survey within the ESIG (1 response per company), aiming to understand how organizations are currently operating/expect to operate

HTA ESIG: HTA Open Source survey - need your help again!   📇 Summarize

AGTR (Anders Gorst-Rasmussen)
To   Crawford, Emma; Wolfson, Lara J.; Achim Steup;
      Alexandra Thiele; Angela Emser; **+69 others**
Cc   htasig@psiweb.org
ⓘ You replied to this message on 21-09-2023 13:15.   21-09-2023

📄 os_survey_21SEP2023.pdf ⌄
   358 KB

Dear members of the HTA ESIG,

Thanks a lot to the all of you who answered the survey about pre-specification. We received 14 responses and will report back to the full SIG on the results later in the autumn.

HOWEVER: we need your help again with another survey!

This one pertaining to open source solutions (tools and reporting frameworks) in HTA

https://forms.office.com/e/7Ur93R1VJv

16 responses

87% pharma, **13%** contract research organization
37% use primarily R

Medium (**31%**) to high (**69%**) opportunity seen for automation and standardization for further HTA work

63% considering development of new tools for the EU HTA Joint Clinical Assessment procedure

Key areas for tool development: Indirect Treatment Comparisons/Network Meta Analysis and standardization/automation

Main barriers: resources, lack of experience/upskilling, and IT infrastructure

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8

Barriers for open source in HTA

Discussions in the HTA ESIG Open Source subteam

Do we have sufficiently specific, shared cross-company objectives in HTA?

To gain momentum and requires clear shared objectives to drive broad adoption (think: **mmrm** package for R)

Standardizing vs templating

The more heterogeneous and dynamic nature of HTA statistical work may require different tactics compared to the more standardized regulatory statistical setting

Is now the right time?

Guidance around EU HTA is being finalized; companies are still deciding how to strategically adopt it in workflows

Are external incentives too limited?

While regulatory authorities such as FDA are increasingly interested in open source, there is comparatively less interest from HTA bodies

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Your Opinion?

Go to questions!

Question 1 – most promising collaboration targets?
Rank the options below from top to bottom

Tools for end-to-end HTA analysis and reporting

Tools for indirect treatment comparison analyses e.g. NMA, MAIC, ML-NMR etc

Other specialized HTA statistical analyses
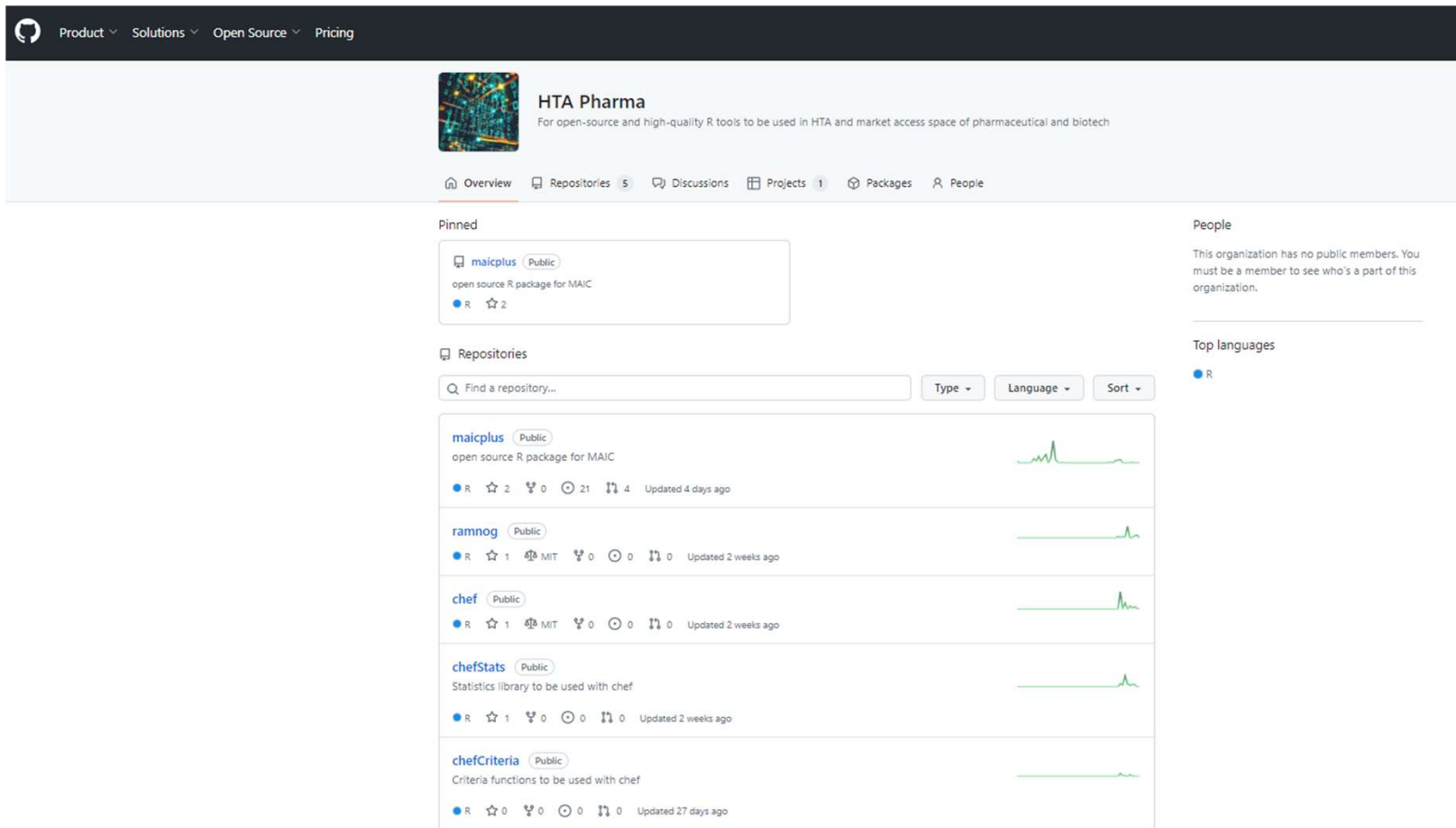
Question 2 – biggest barriers?
Rank the options below from top to bottom

Heterogeneity of HTA statistical work (compared to regulatory statistical work)

Lack of familiarity with open source

Unclear business case and/or lack of resources

https://github.com/hta-pharma/

Case I: maicplus

Cross-company collaboration on a new package for
Matching **A**djusted **I**ndirect **T**reatment **C**omparison

Aim: collaborate on implementing a high quality open-source MAIC for HTA statisticians

Achievements
- ✓ Implements comprehensive analysis and visualization tools, including anchored and unanchored for time-to-event and binary outcomes
- ✓ Open source available on GitHub and standard software engineering checks performed
- ✓ Combines good practices and use cases of MAIC from each company

Learnings from challenges
- ✓ Requires project management skills to coordinate effective collaboration of contributors
- ✓ Onboarding new developers takes time to adapt to best practices
- ✓ Conflicts may arise due to differences in expected use and software design across companies
- ✓ Progress depends on contributor's overall workload, continued motivation and allocated time

Chen, G., Seo, M., Antoniou, M., Belleli, R., Kalyvas, C. and Gravestock, I., 2023. SA83 {Maicplus}: An R Package to Support Analysis and Reporting of Matching Adjusted Indirect Treatment Comparisons (MAIC) for HTA Dossiers. Value in Health, 26(12), p.S558.

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Case II: chef

An open source framework to build modular analysis pipelines for HTA, originally built within German HTA submissions (AMNOG) in mind

HTA analyses requests

Biostat

Analyses
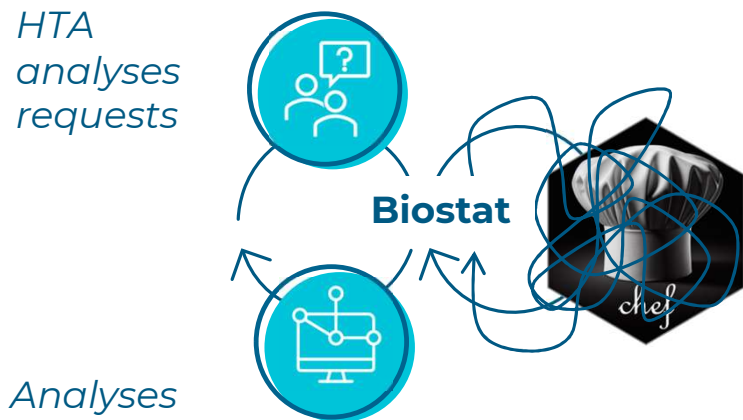
Curious to learn more about **chef**?
Visit the poster session later today!

From Novo Nordisk internal framework to open source

Building a great technical solution is one thing – getting it out there *and* leveraging that it's out there is a very different thing!

- Business case and legal considerations for open sourcing a functional, internally developed setup
- Choice of license (how restricted vs open)?
- Contributor identity (personal vs company)?
- Hosting options?

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Concluding remarks

1   HTA Open Source needs dedicated work

2   There is a window of opportunity with EU HTA coming

3   A concerted effort is needed to build momentum

Mini-survey – results

Want to get involved?

Join the HTA ESIG today!

htasig@psiweb.org

14

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Your Opinion?

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Other specialized HTA statistical analyses

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Question 1 – most promising collaboration targets?
Rank the options below from top to bottom

Question 2 – biggest barriers?
Rank the options below from top to bottom

1

2

3