

Real-World Data (RWD): Patient Perspectives and Applications in Healthcare

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Content

- Background: Real4Reg & Real-world data (RWD)
- Real4Reg survey: Patients' views on RWD
- Use cases of RWD

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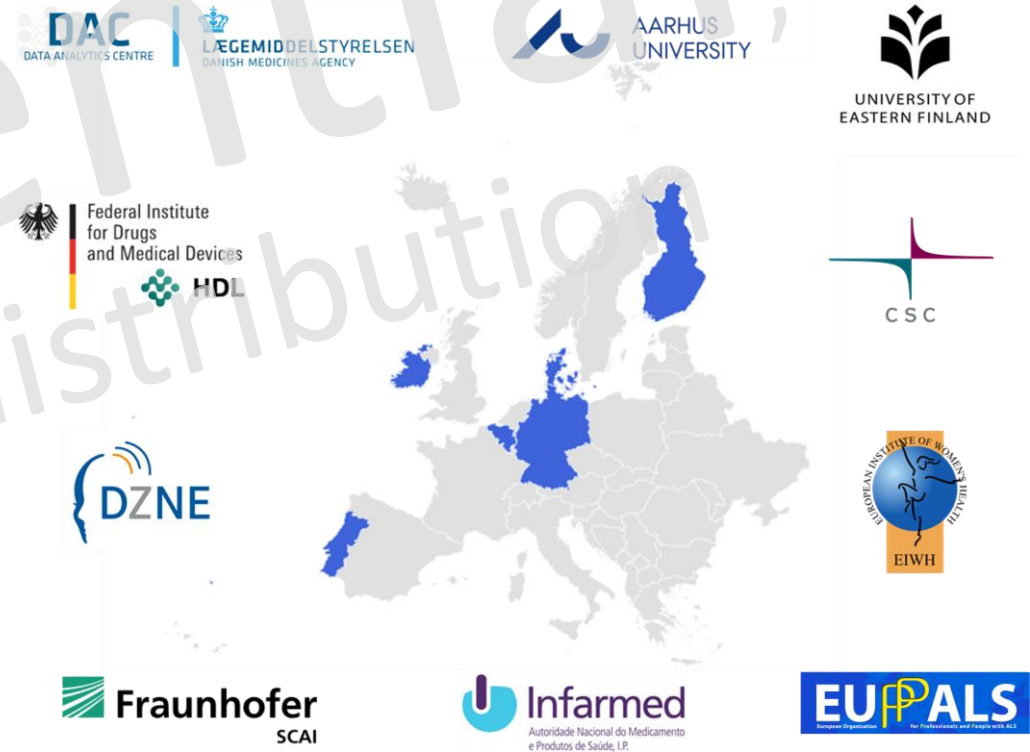
The Real4Reg project

Real4Reg: Development, Optimisation & Implementation of AI-Methods for RWD Analyses in Regulatory Decision-Making & HTA along the Product Life-Cycle

Duration: 2023-2026

Consortium: 10 Partners, 6 EU countries, Lead: BfArM

Data: registry data from Denmark, Finland and Portugal, claims data from Germany



Real-world data

Routinely collected data, outside clinical trials

Many different sources, for example:

- Disease registries
- Health insurance claims
- Electronic health records

Real-world data

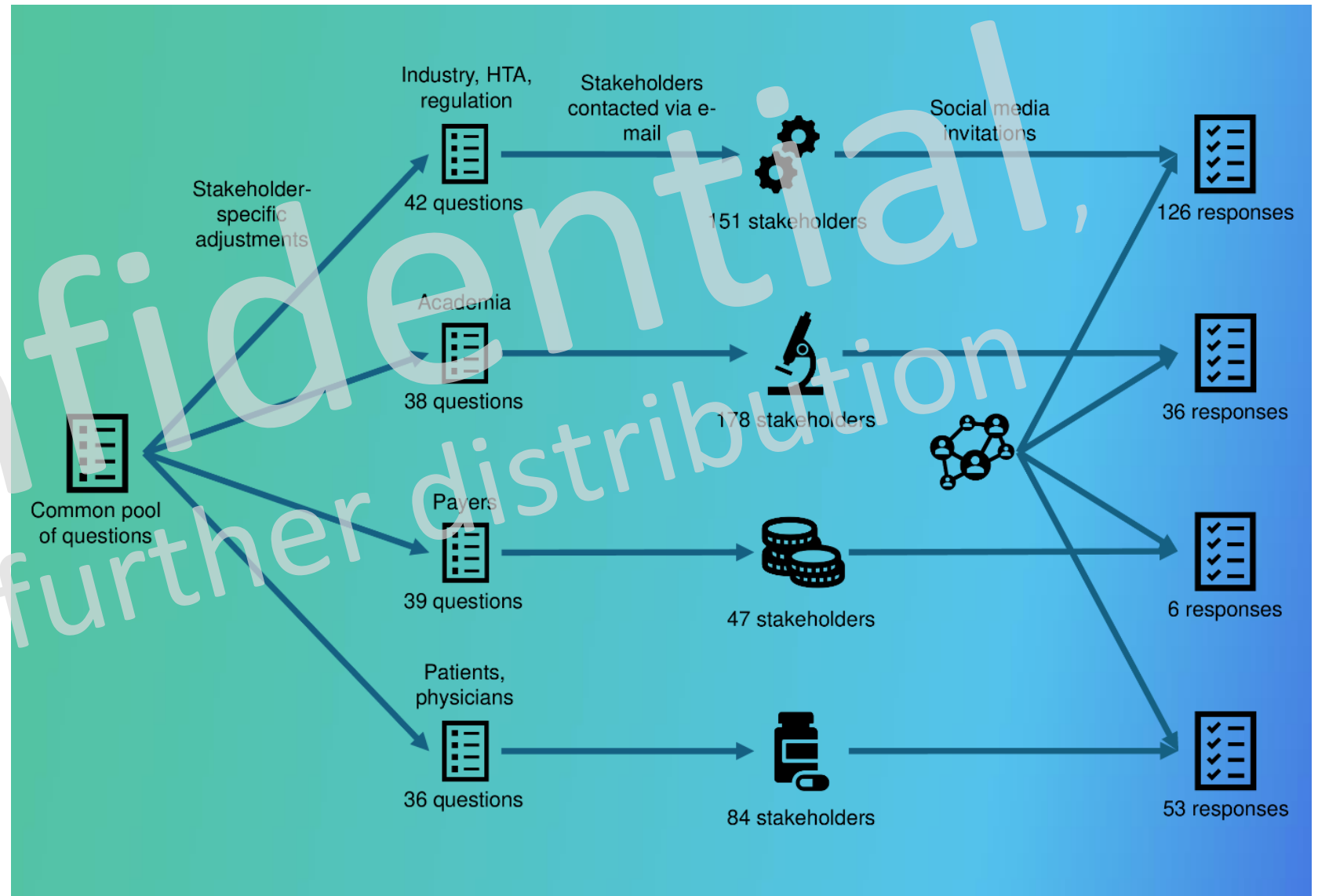
Advantages:

- Data represents actual clinical practise
- Potentially large sample sizes
- Less burden on patients & physicians
- Can deliver evidence quickly

Real4Reg survey

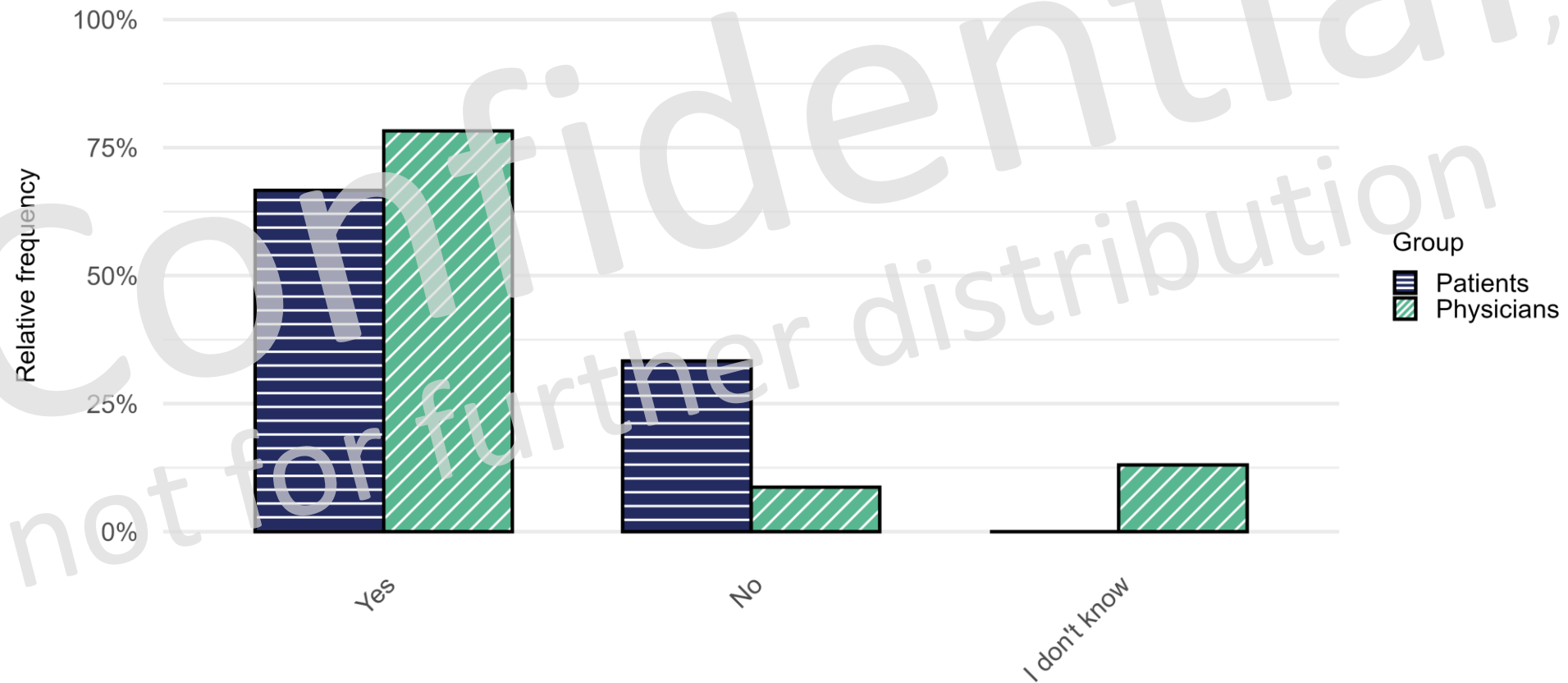
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Survey setup

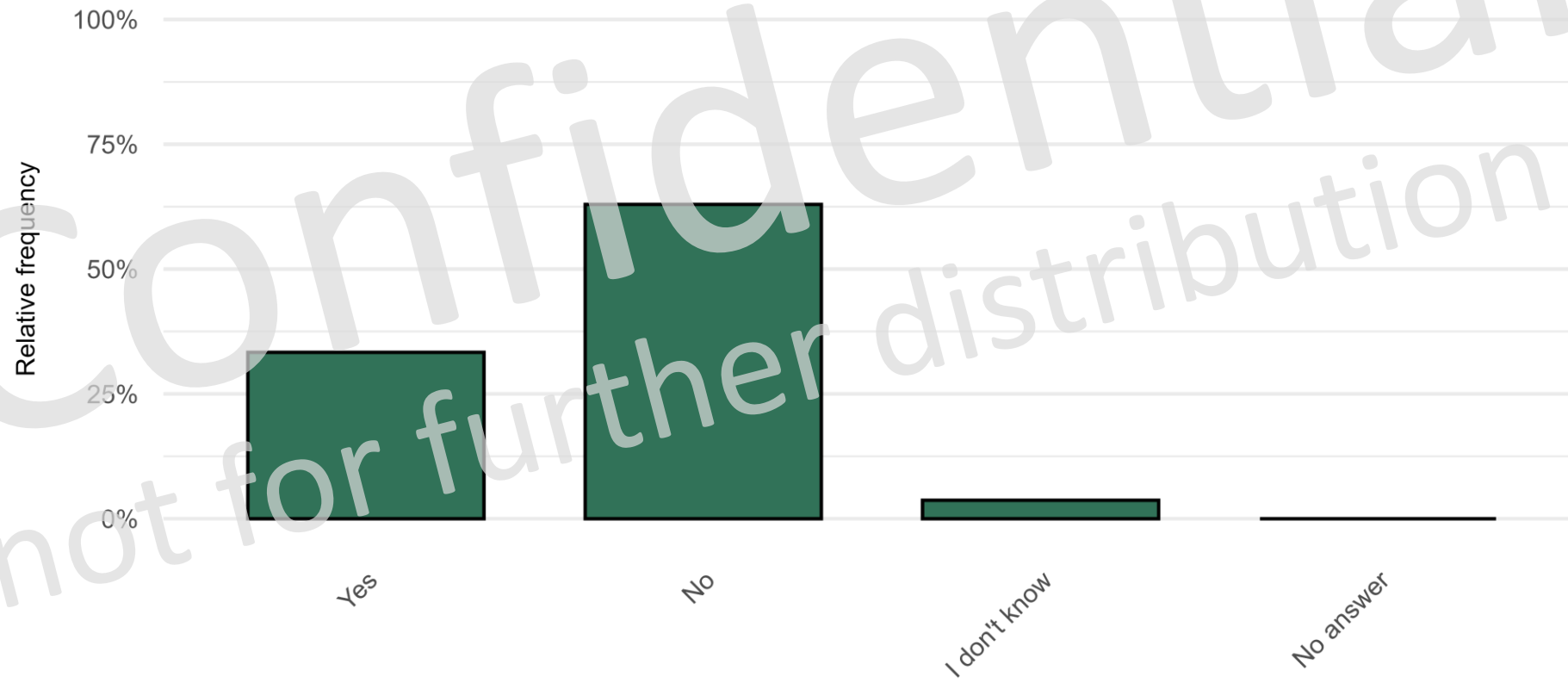


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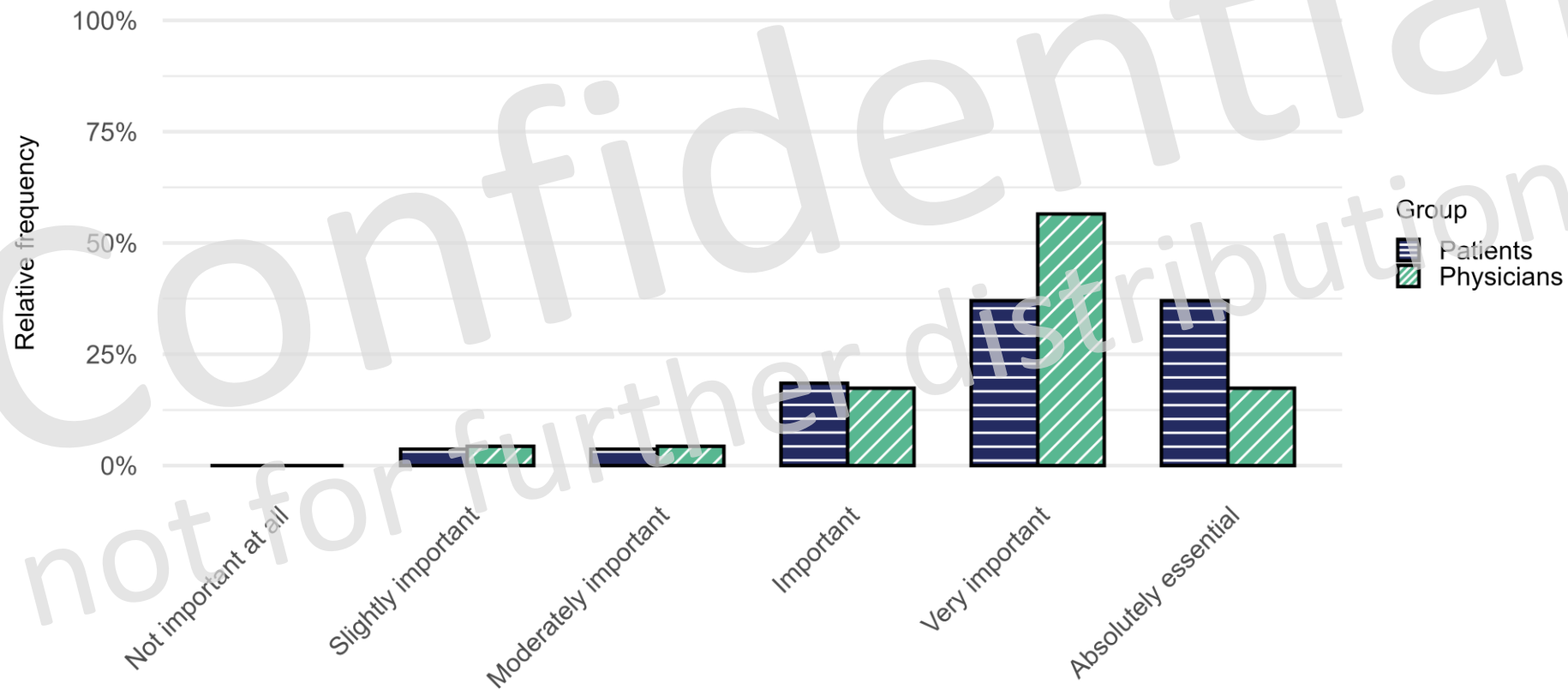
Have you heard of RWD before?



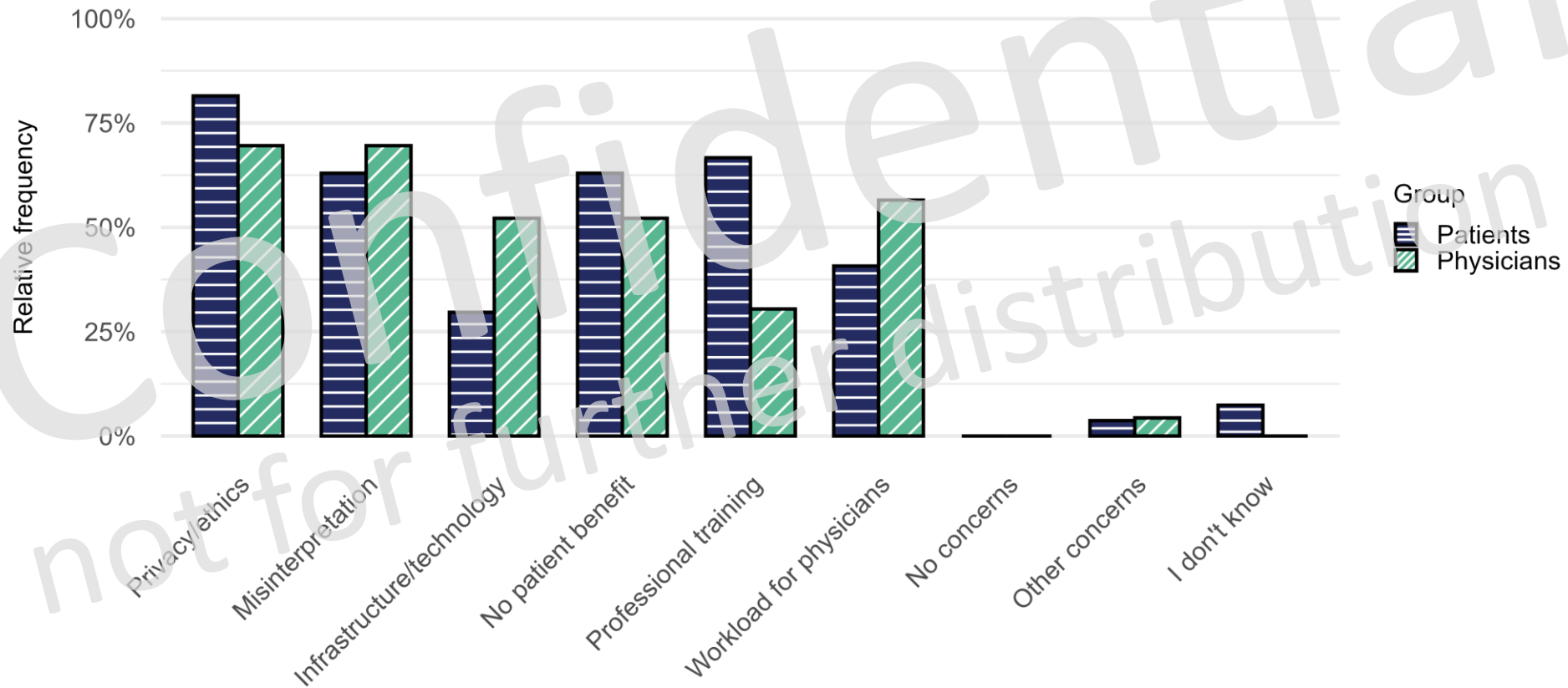
Were you ever educated about RWD use?



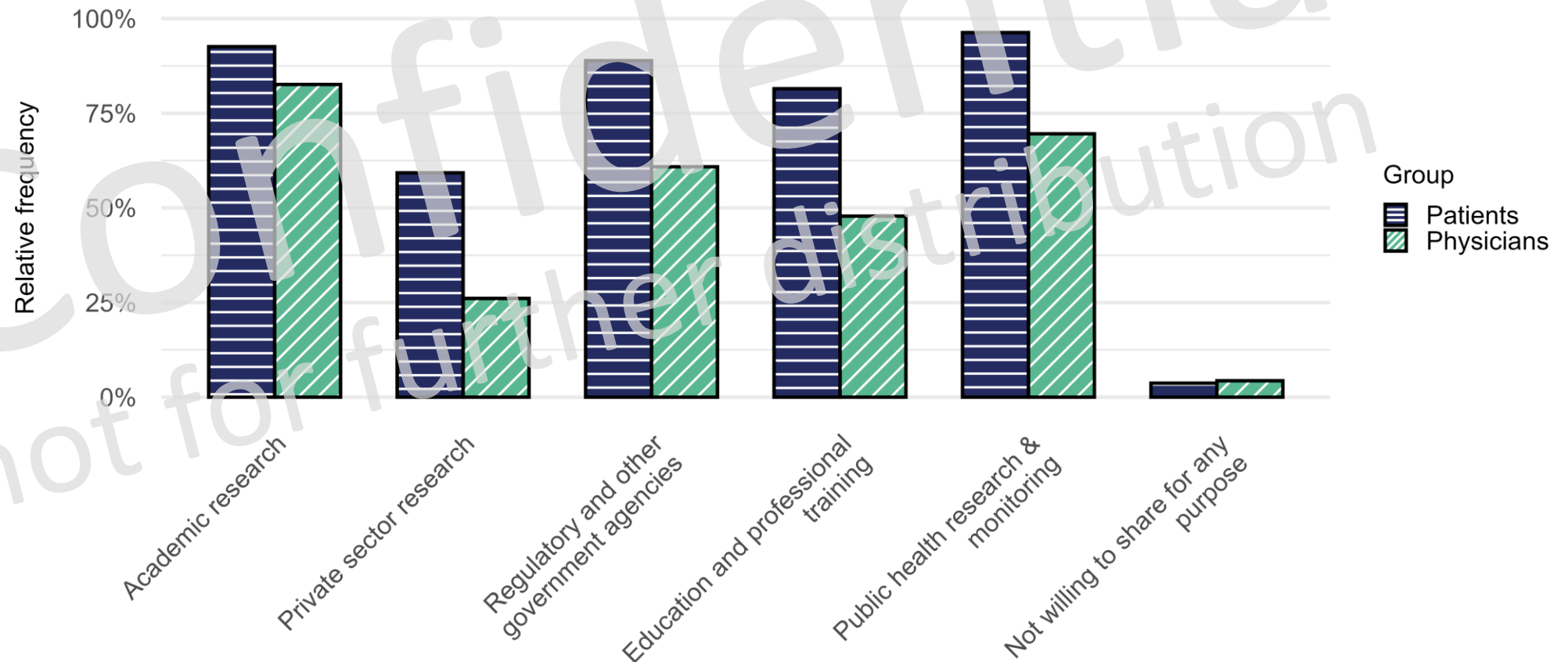
How important will RWD be in the future?



What concerns do you have regarding RWD?



For what purposes would you be willing to share your personal health-related data?



More information & results

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Key Stakeholders' Knowledge, Opinions, and Interests on Real-World Evidence in the Regulatory Process—Results of an EU-Wide Survey

[Frank Lucas Depner](#), [Martin Russek](#), [Christoph Röthlein](#), [Cornelia Becker](#), [Jonas Peltner](#), [Kerstin Pfeifer](#), [Evy Reviers](#), [Dirk De Valck](#), [Julia Wicherski](#), [Sirpa Hartikainen](#), [Anna-Maija Tolppanen](#), [Britta Haenisch](#) ✉

First published: 20 December 2025 | <https://doi.org/10.1111/cts.70454>

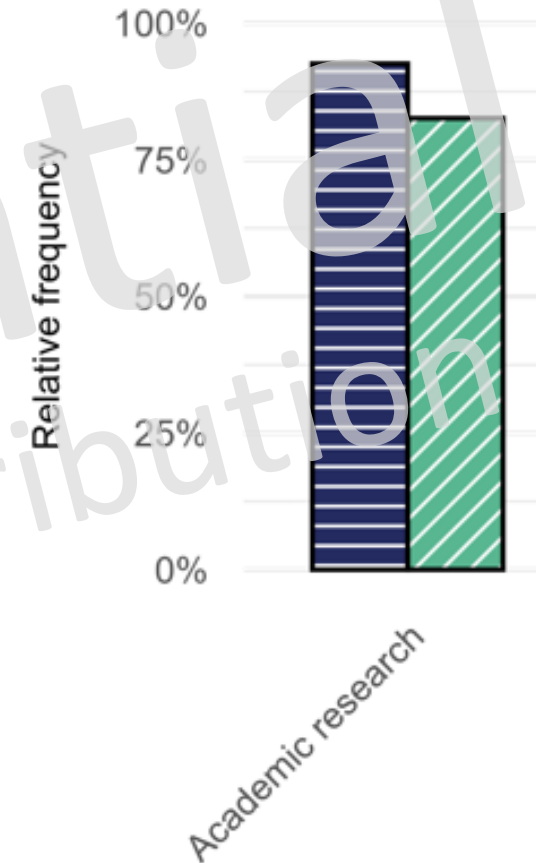


Use of RWD in healthcare

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Academic research

- Main goal: advancing scientific knowledge
- Development of new methods
- Identification of side effects
- Estimating effectiveness of drugs
- Exploring drug use patterns



Academic research: Paracetamol/Tylenol

Political claims:
acetaminophen causes
autism

Effect: reduced use by
pregnant people

Research: data do not
support the hypothesis

Acetaminophen Use During Pregnancy and Children's Risk of Autism, ADHD, and Intellectual Disability

Viktor H. Ahlqvist, PhD¹; Hugo Sjöqvist, MSc¹; Christina Dalman, MD, PhD¹;

Håkan Karlsson, PhD²; Olof Stephansson, MD, PhD^{3,4}; Stefan Johansson, MD, PhD^{3,5}; Cecilia Magnusson, MD, PhD^{1,6}; Renee M. Gardner, PhD¹; Brian K. Lee, PhD^{1,7,8}

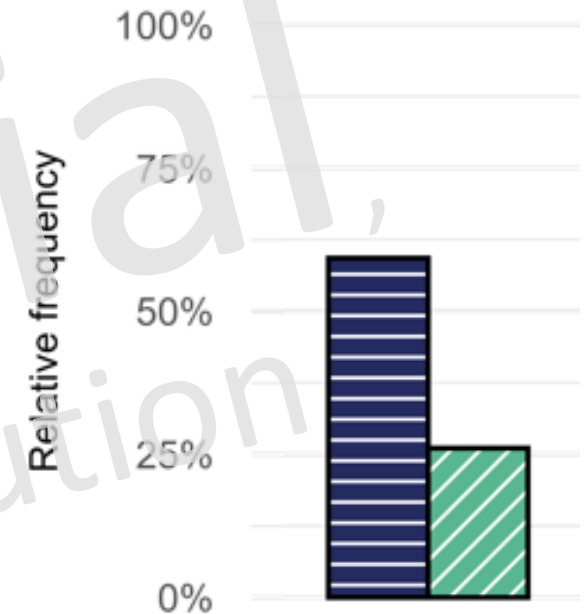
Findings In this population-based study, models without sibling controls identified marginally increased risks of autism and attention-deficit/hyperactivity disorder (ADHD) associated with acetaminophen use during pregnancy. However, analyses of matched full sibling pairs found no evidence of increased risk of autism (hazard ratio, 0.98), ADHD (hazard ratio, 0.98), or intellectual disability (hazard ratio, 1.01) associated with acetaminophen use.

Meaning Acetaminophen use during pregnancy was not associated with children's risk of autism, ADHD, or intellectual disability in sibling control analyses. This suggests that associations observed in other models may have been attributable to confounding.

Ahlqvist VH, Sjöqvist H, Dalman C, et al. Acetaminophen Use During Pregnancy and Children's Risk of Autism, ADHD, and Intellectual Disability. *JAMA*. 2024;331(14):1205–1214. doi:10.1001/jama.2024.3172

Private sector research

- Main goal: development of new medicines
- Identification of drug targets
- Effectiveness and safety studies for authorization
- Effectiveness, safety and cost-effectiveness studies for health technology assessment
- Patient preferences



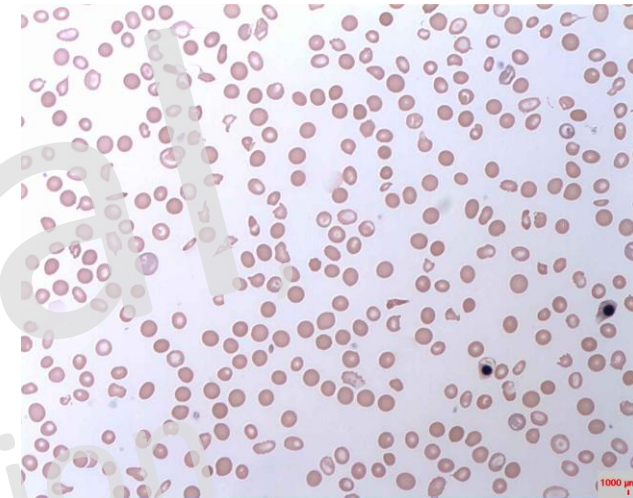
Private sector research: Soliris

Paroxysmal nocturnal hemoglobinuria (PNH): rare, life-threatening blood disorder

Soliris: drug that reduces need for blood transfusion in PNH patients – for patients already on transfusions

Study using PNH registry: better laboratory values, less fatigue, better quality of life for patients not yet on transfusions

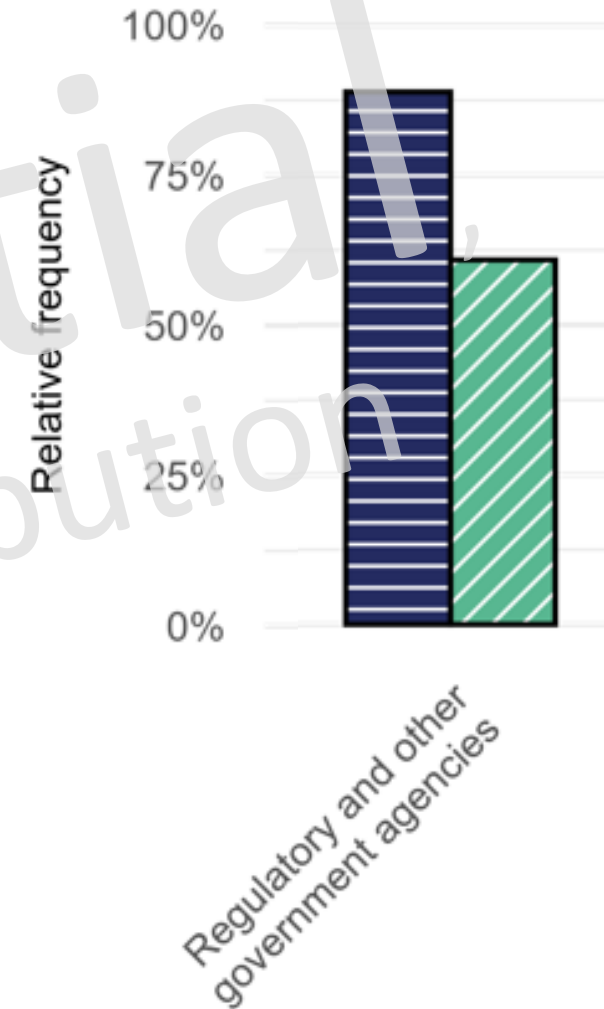
-> After regulatory assessment, treatment also available for patients without blood transfusion



File:Haemolytic Anaemia.jpg. (2025, February 13). *Wikimedia Commons*. Retrieved April 11, 2026, from <https://commons.wikimedia.org/w/index.php?title=File:Haemolytic Anaemia.jpg&oldid=998168905>.

Regulatory agencies

- Main goal: ensure only safe, effective medicines reach market
- Benefit-risk assessment of new medicines
- Monitor safety & effectiveness after authorization
- Identify unmet medical needs of patients



Regulatory agencies: HPV vaccination



First HPV vaccination to prevent cervical cancer authorized in EU in 2007

Initial studies maximum of 4 years

DARWIN-EU study: estimation of effectiveness after up to 15 years

Findings consistent with those from early RCTs, confirming effectiveness

The effectiveness of HPV vaccination against invasive cervical cancer and related precancerous lesions: a multinational target trial emulation study

Marta Alcalde-Herraiz BSc^{a*}, Mike Du PhD^{a*}, Aina Sanchez-Parada BSc^b, Talita Duarte-Salles PhD^{c,d}, Anna Palomar-Cros PhD^d, Agustina Giuliadori PhD^d, Antonella Delmestri PhD^a, Prof Hedvig Marie Egeland Nordeng PhD^{e,f}, Nhung T H Trinh PhD^e, Saeed Hayati BSc^e, Prof Daniel Prieto-Alhambra MD^{a,c}  , Marti Catala PhD^{a†}, Albert Prats-Urbe PhD MD^{a†}

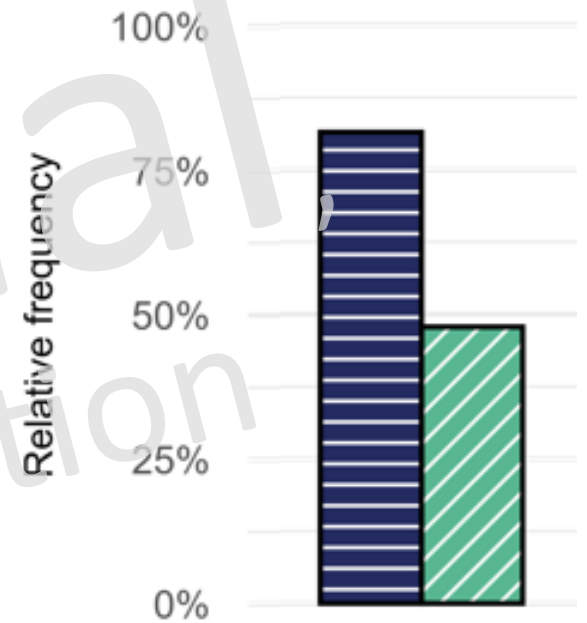
Findings

After two-step matching, our analysis included 81 863 vaccinated and 46 357 unvaccinated women from the UK; 148 214 vaccinated and 39 952 unvaccinated from Spain; and 14 885 vaccinated and 4073 unvaccinated from Norway. Fewer than five cervical cancers were observed per cohort, precluding vaccine effectiveness estimation for this outcome. Meta-analytic vaccine effectiveness at 15 years was 42% (95% CI 6–64) against CIN2+ and 58% (6–82) against conisation.

Alcalde-Herraiz, Marta, et al. "The effectiveness of HPV vaccination against invasive cervical cancer and related precancerous lesions: a multinational target trial emulation study." *The Lancet Primary Care* (2026).

Education & professional training

- Main goal: give students and professionals realistic experiences
- Learn complexities of real data sets
- Learn to handle big data sources
- Get realistic output

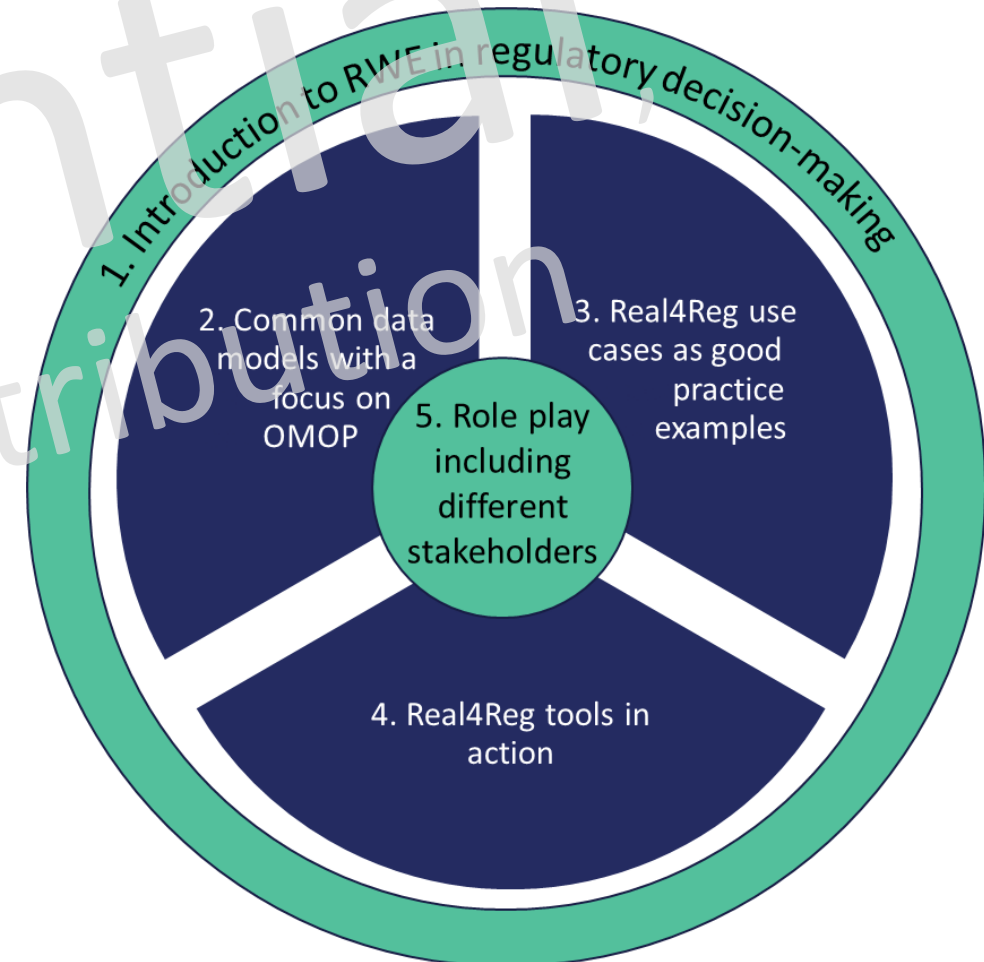


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Education & professional training: Real4Reg

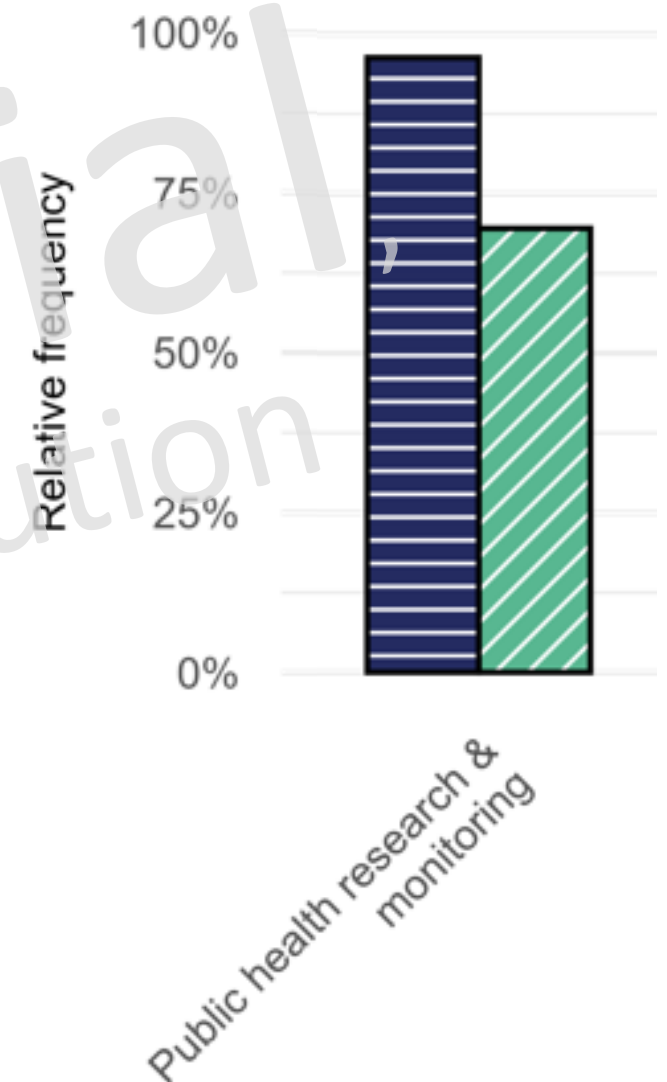
Hands-on training for professionals and interested people: using advanced algorithms to analyse RWD

Use of artificial RWD based on real RWD sources: realistic learning experiences while maintaining patient privacy



Public health monitoring

- Main goal: surveillance & interventions for public health emergencies
- Monitor occurrence of diseases
- Prepare for emergencies
- Plan & evaluate prevention programs
- Plan & evaluate outbreak intervention strategies







Public health monitoring

Common discourse on vaccine safety:
are aluminum-based vaccines unsafe?
Long-term study using Danish registry
data:

- >1 million children born in Denmark 1997 – 2020
- Investigating risk of 50 different disorders by cumulative amount of aluminum received through early childhood vaccination
- No evidence of large risk of aluminum in vaccines

Aluminum-Adsorbed Vaccines and Chronic Diseases in Childhood: A Nationwide Cohort Study FREE

✓ This article has been corrected. [VIEW CORRECTION](#)

Authors: Niklas Worm Andersson, MD, PhD  , Ingrid Bech Svalgaard, MSc, Stine Skovbo Hoffmann, MD, PhD , and Anders Hviid, MSc, DMSc  | [AUTHOR, ARTICLE, & DISCLOSURE INFORMATION](#)

Publication: Annals of Internal Medicine • Volume 178, Number 10 • <https://doi.org/10.7326/ANNALS-25-00997>

Results:

Cumulative aluminum exposure from vaccination during the first 2 years of life was not associated with increased rates of any of the 50 disorders assessed. For groups of combined outcomes, adjusted hazard ratios per 1-mg increase in aluminum exposure were 0.98 (95% CI, 0.94 to 1.02) for any autoimmune disorder, 0.99 (CI, 0.98 to 1.01) for any atopic or allergic disorder, and 0.93 (CI, 0.90 to 0.97) for any neurodevelopmental disorder. For most individually analyzed outcomes, the upper bounds of the 95% CIs were incompatible with relative increases greater than 10% or 30%.

Niklas Worm Andersson, Ingrid Bech Svalgaard, Stine Skovbo Hoffmann, et al. [Aluminum-Adsorbed Vaccines and Chronic Diseases in Childhood: A Nationwide Cohort Study](#). Ann Intern Med.2025;178:1369-1377. [Epub 15 July 2025]. doi:[10.7326/ANNALS-25-00997](https://doi.org/10.7326/ANNALS-25-00997)

Thank you!

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