PSI RWD SIG

Real-world data – do you know all the opportunities?

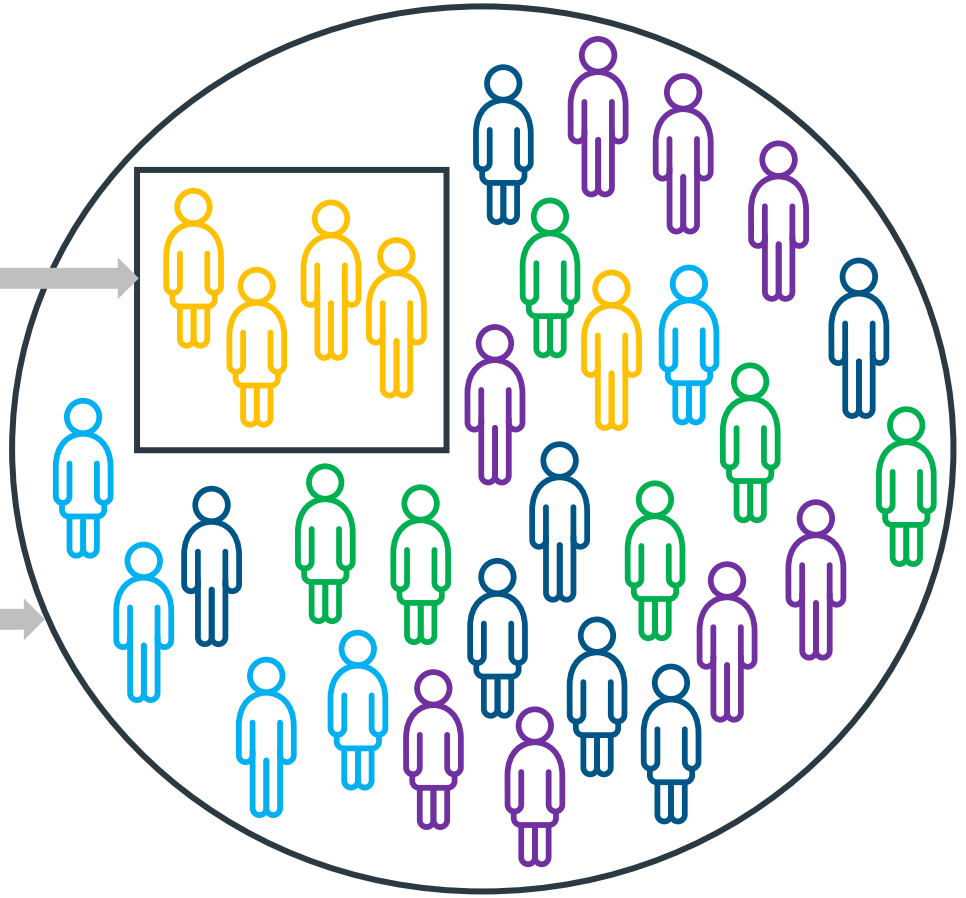
The key questions they can answer and how

Eleanor Ralphs, Associate Director in Real-World Biostatistics

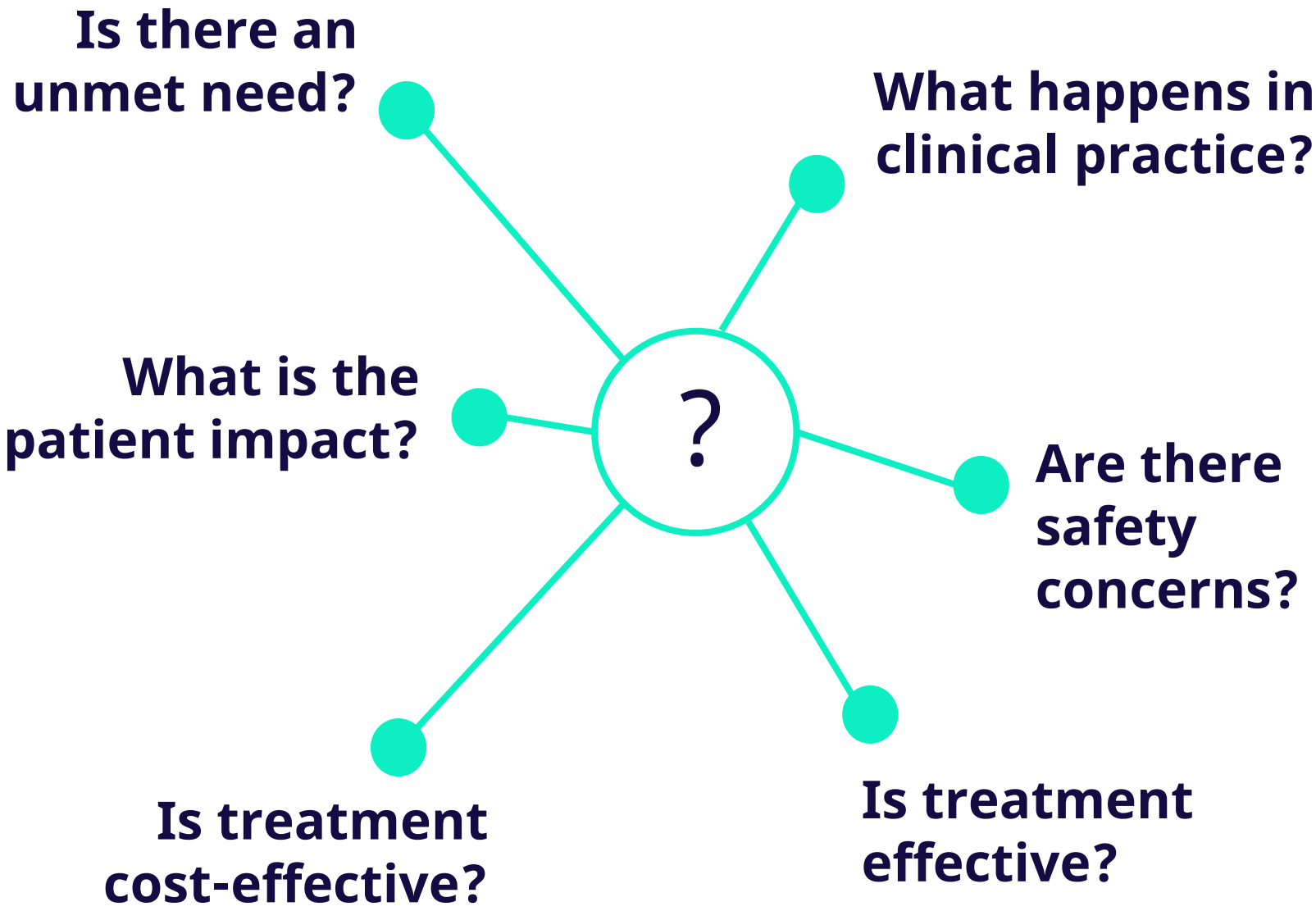
IQVIA

Evidence submitted to regulators and HTA often rely on **controlled settings**

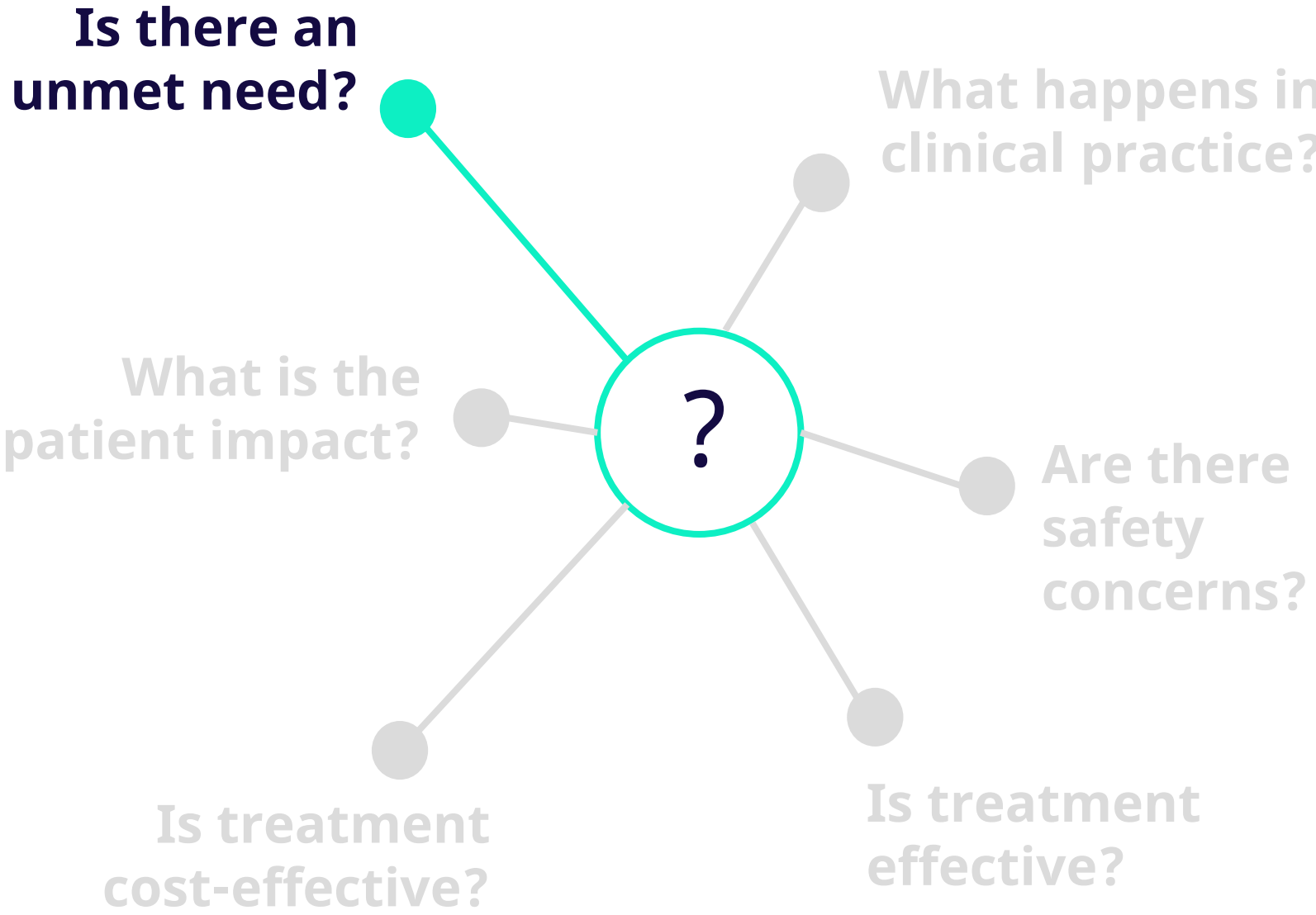
Not fully reflecting **real-world patient populations** or long-term outcomes



We can turn to real-world data to help bridge evidence gaps

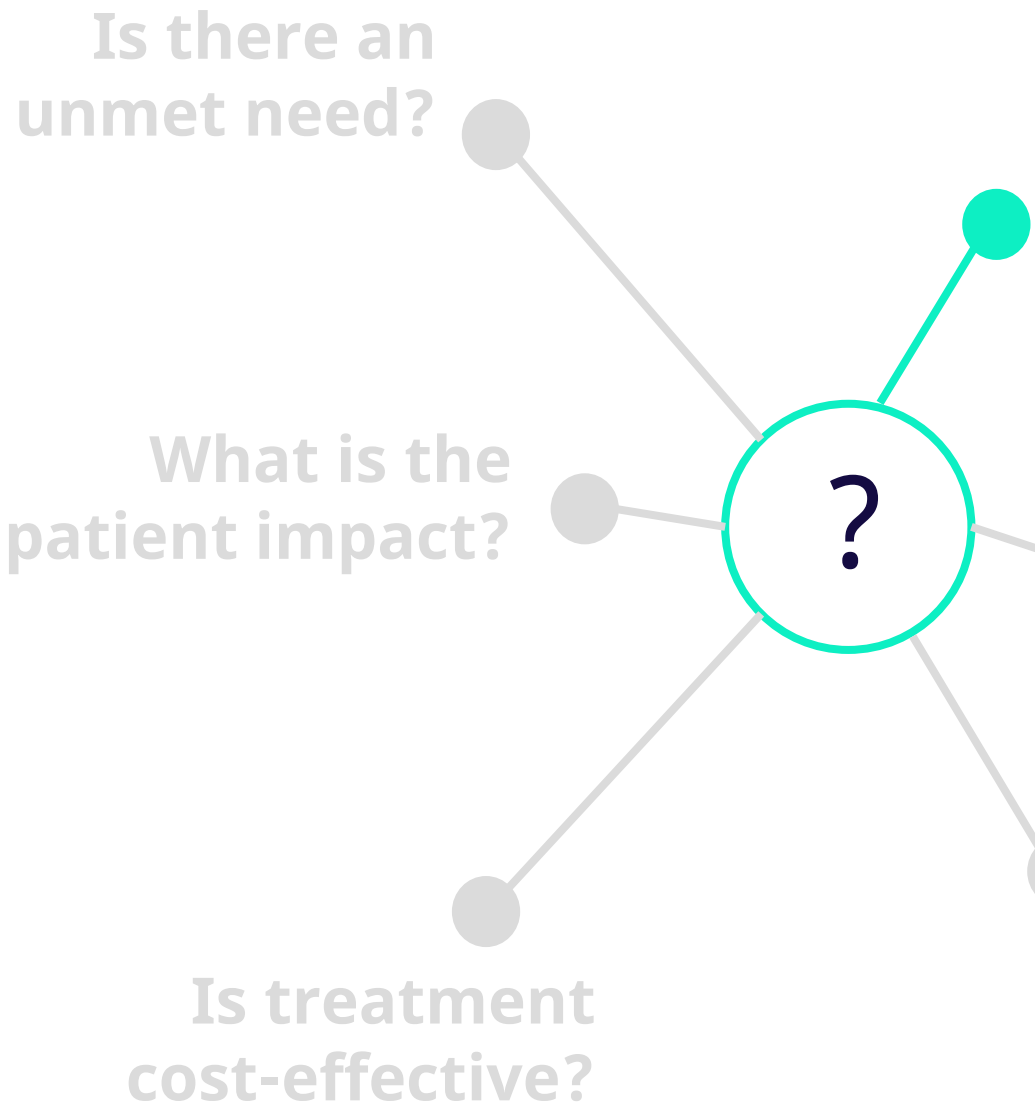


Real-world data can help answer key questions



- Informs **trial design**
- Provides outcome rates without intervention for **sample size calculations**
- Informs opportunity for **label expansion**

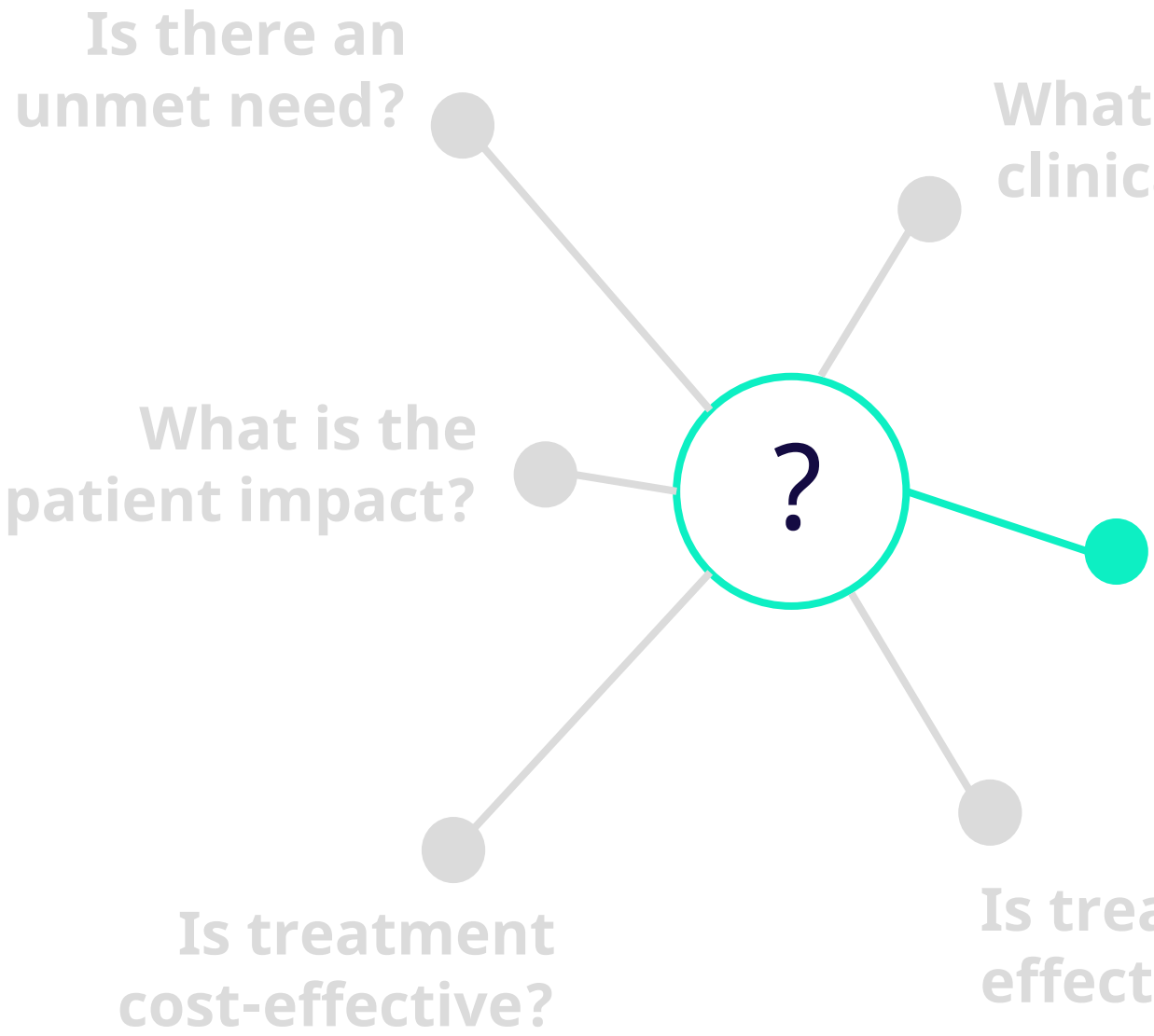
Type of RWE: **Natural history of disease studies**



What happens in clinical practice?

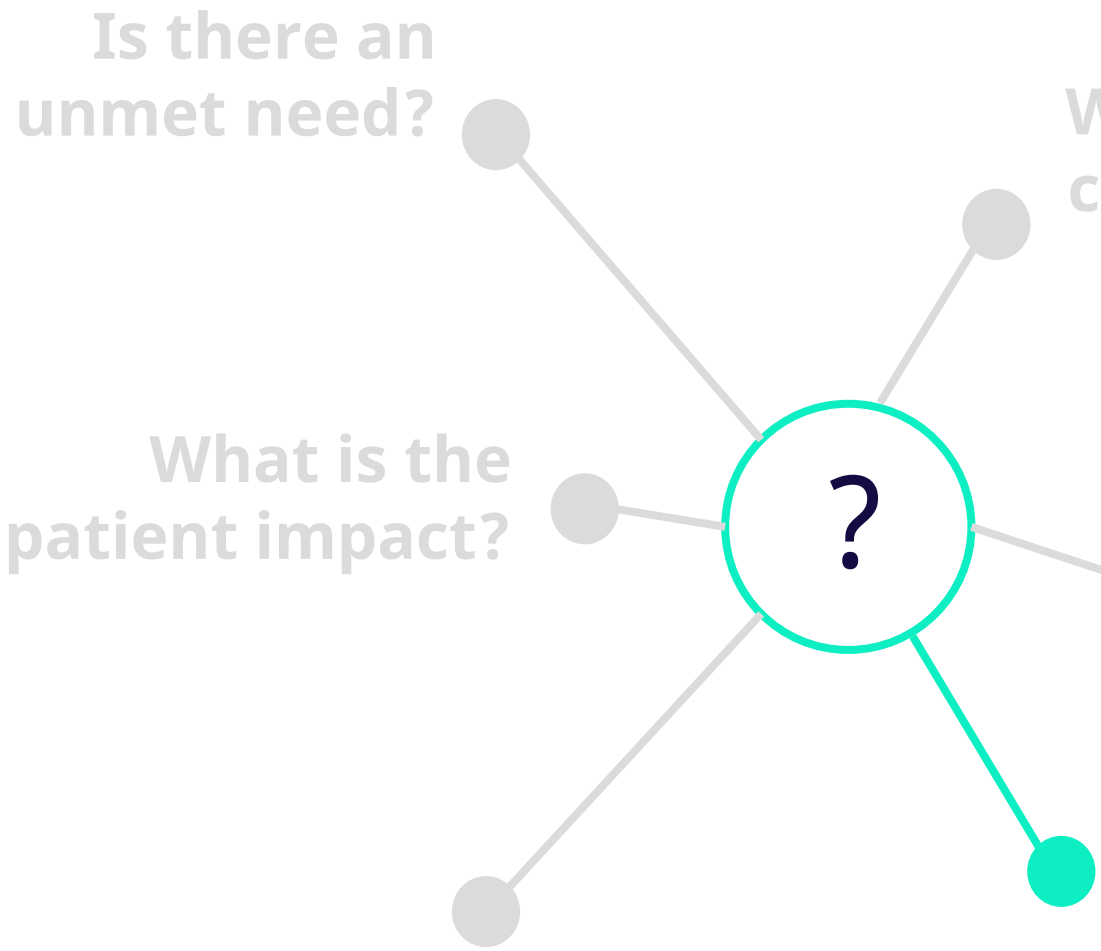
- Informs control groups
- Extended **follow-up** insights
- Provides **adherence** assessment

Type of RWE:
Treatment patterns study (patient journey study)



Provides **regulators** and **patients** with safety evidence

Type of RWE: **Post-authorization safety studies**, e.g. pregnancy studies

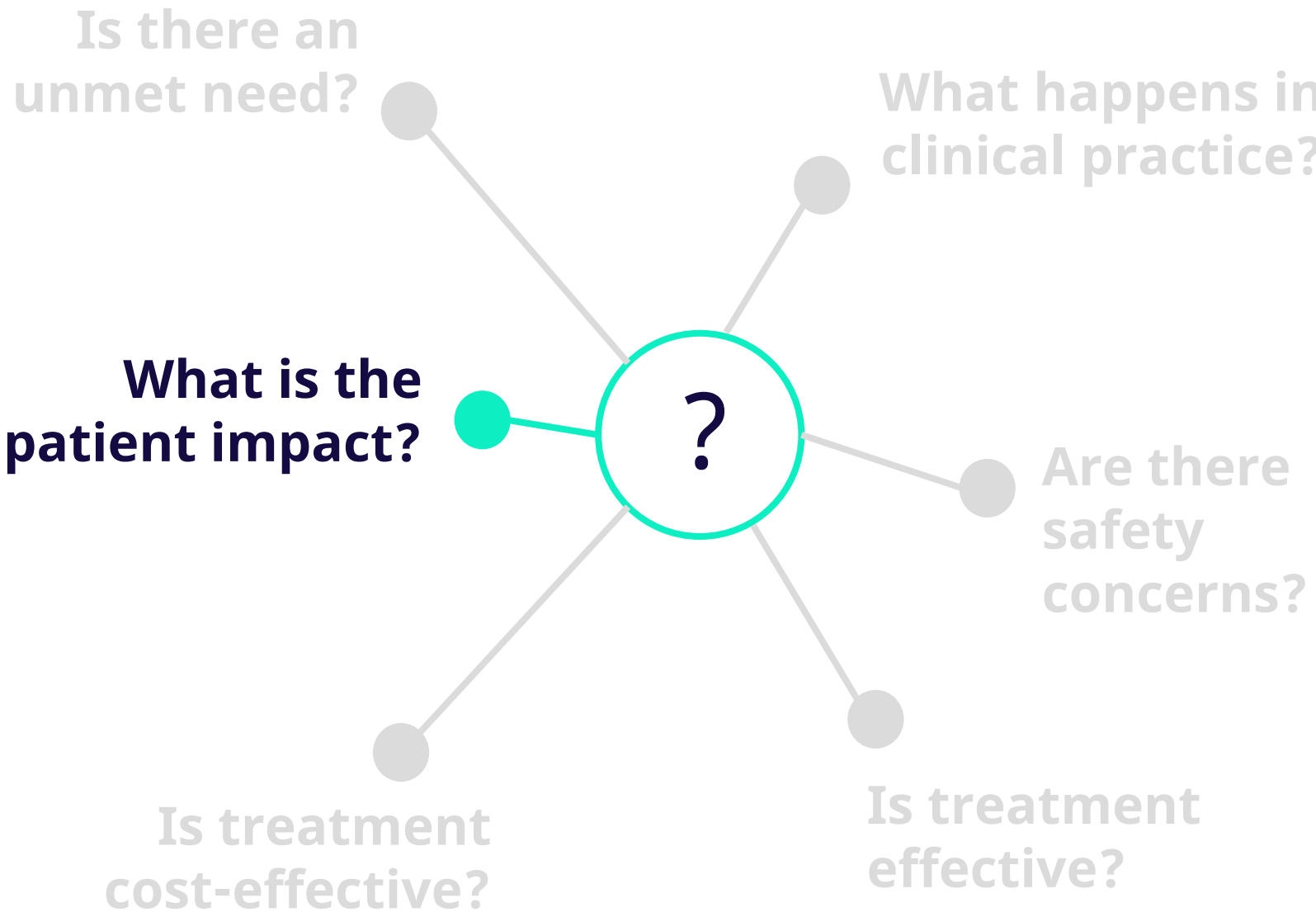


- Informs choice of trial **endpoints**
- Informs on expected **effect size of control** group
- Provides **reg/HTA evidence**
- Provides **clinicians** with effectiveness

Type of RWE:
comparative studies,
e.g. external comparators

- Informs clinicians on the **burden** in the healthcare setting
- Informs health **economic evaluations** (e.g. CEMs)

Type of RWE: Health care **resource utilisation** (HCRU) study



- Informs health **economic evaluations** (e.g. CEMs)
- Informs clinicians and patient treatment choice with evidence of wider **impact on living**

Type of RWE: Health-related **quality of life** (HR-QoL) studies

Case study 1

Is there an unmet need?

Setting the scene

Endometrial cancer diagnosis 20 per 100,000 women
age-standardized incidence

18% recur
within first 2 years

Unmet need

Critical evidence gaps in high-risk disease
Lack of knowledge of clinical outcomes

Solution

RWE study was conducted to bridge the gap

Design: Retrospective population-based cohort study

Setting: England, NHS clinical practice

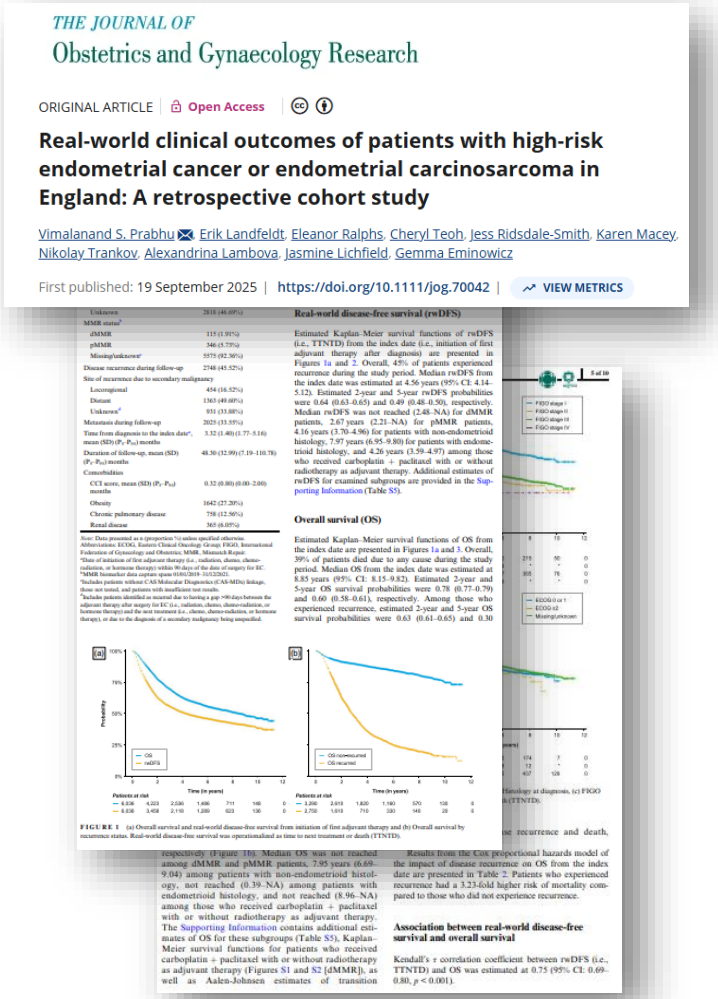
Data source: National Cancer Registration and Analysis Service (NCRAS), Public Health England

Study period: 2014–2023

Population: Adults with high-risk endometrial cancer who received adjuvant therapy within 90 days following surgery

Index: The date of initiation of first adjuvant therapy

This real-world study demonstrates a substantial unmet clinical need in high-risk endometrial cancer



THE JOURNAL OF
Obstetrics and Gynaecology Research

ORIGINAL ARTICLE | Open Access

Real-world clinical outcomes of patients with high-risk endometrial cancer or endometrial carcinosarcoma in England: A retrospective cohort study

Vimalanand S. Prabhu, Erik Landfeldt, Eleanor Ralphs, Cheryl Teoh, Jess Ridsdale-Smith, Karen Macey, Nikolay Trankov, Alexandrina Lambova, Jasmine Lichfield, Gemma Eminowicz

First published: 19 September 2025 | https://doi.org/10.1111/jog.70042 | VIEW METRICS

Ongoing burden at a population level (n=6,036)

Unmet need is systemic, not confined to select centres or trial-eligible patients

High recurrence rates (45%) under existing treatment strategies

Unmet need for more effective adjuvant or maintenance strategies

Substantial mortality (39%), median overall survival of 8.85 years

Unmet need for long-term disease control

Prabhu, V.S., Landfeldt, E., Ralphs, E., Teoh, C., Ridsdale-Smith, J., Macey, K., Trankov, N., Lambova, A., Lichfield, J. and Eminowicz, G., 2025. Real-world clinical outcomes of patients with high-risk endometrial cancer or endometrial carcinosarcoma in England: A retrospective cohort study. Journal of Obstetrics and Gynaecology Research, 51(9), p.e70042.

Running a real-world study was beneficial

Large, representative population	Ongoing burden at a population level (n=6,036)	Unmet need is systemic, not confined to select centres or trial-eligible patients
Reflected real treatment pathways and outcomes	High recurrence rates (45%) under existing treatment strategies	Unmet need for more effective adjuvant or maintenance strategies
Long follow-up	Substantial mortality (39%), median overall survival of 8.85 years	Unmet need for long-term disease control

Case study 2

What is happening in clinical practice?

Setting the scene

Non-small cell lung cancer (NSCLC) accounts for most lung cancer diagnoses

Management of non-metastatic NSCLC is complex and stage-dependent - Rapid advances in recent years

Unmet need

Limited contemporary evidence of routine clinical practice

Solution

RWE study was conducted to bridge the gap

Design: Retrospective population-based cohort study

Study period: 2010-2020

Setting: Canada, England, Germany

Population: Adult patients diagnosed with stage I–IIIC non-metastatic NSCLC

Data sources:

- Regional Canadian oncology database
- National English cancer registry
- Regional German cancer registries

Index: The date of initial NSCLC diagnosis

Data sources: Canadian oncology database = Oncology Outcomes (O2); English cancer registry = National Cancer Registration and Analysis Service (NCRAS); Four regional German cancer registries = VONKOdb

Real-world evidence shows stage-dependent treatment gaps and poor outcomes in NSCLC, particularly in advanced disease

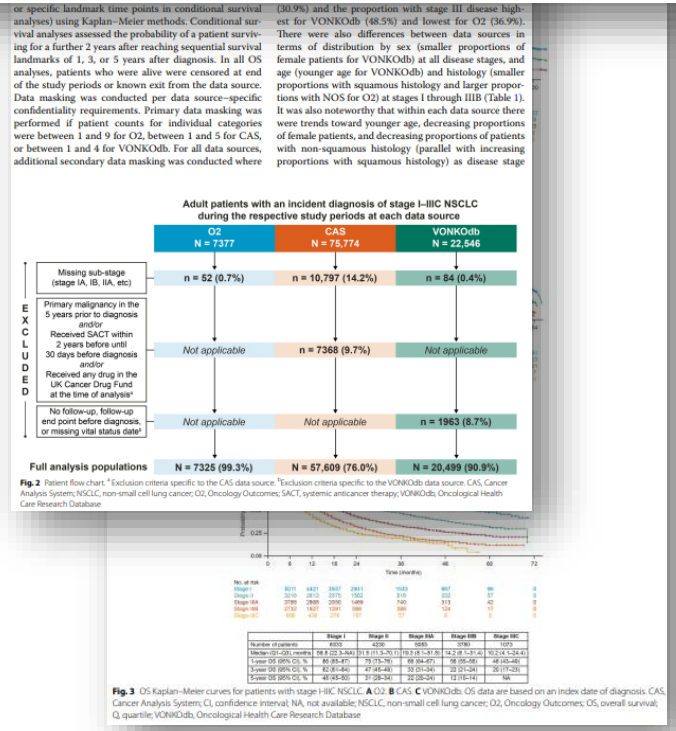
Greystoke et al. BMC Pulmonary Medicine (2025) 25:265
https://doi.org/10.1186/s12890-025-03715-9

BMC Pulmonary Medicine

RESEARCH Open Access

Real-world treatment patterns and outcomes for patients with non-metastatic non-small cell lung cancer: retrospective analyses in Canada, England, and Germany

Alastair Greystoke[1,2*†], Melinda J. Daumont[3], Caroline Rault[4], Hannah Baltus[5†], Philip Q. Ding[6], Gabrielle Emanuel[7], Stefano Lucherini[8], Lien Vo[9], Valeria M. Saglimbene[10], Eleanor Ralphs[11], Cátia Leal[12], Minouk J. Schoemaker[13], Alexander Katalinic[5], Annika Waldmann[5†] and Winson Y. Cheung[6†]



Adult patients with an incident diagnosis of stage I–IIIC NSCLC during the respective study periods at each data source

	O2 N = 7377	CAS N = 75,774	VONKOdb N = 22,546
Missing sub-stage (stage IA, IB, IIA, etc)	n = 52 (0.7%)	n = 10,797 (14.2%)	n = 84 (0.4%)
EXCLUDED: Primary malignancy in the 5 years prior to diagnosis and/or Received SACT within 2 years before until 30 days before diagnosis and/or Received any drug in the UK Cancer Drug Fund at the time of analysis[a]	Not applicable	n = 7368 (9.7%)	Not applicable
EXCLUDED: No follow-up, follow-up end point before diagnosis, or missing vital status date[b]	Not applicable	Not applicable	n = 1963 (8.7%)
Full analysis populations	N = 7325 (99.3%)	N = 57,609 (76.0%)	N = 20,499 (90.9%)

Fig. 2 Patient flow chart. [a]Exclusion criteria specific to the CAS data source. [b]Exclusion criteria specific to the VONKOdb data source. CAS, Cancer Analysis System; NSCLC, non-small cell lung cancer; O2, Oncology Outcomes; SACT, systemic anticancer therapy; VONKOdb, Oncological Health Care Research Database

Fig. 3 OS Kaplan–Meier curves for patients with stage I-IIIC NSCLC. A O2. B CAS. C VONKOdb. OS data are based on an index date of diagnosis. CAS, Cancer Analysis System; CI, confidence interval; NA, not available; NSCLC, non-small cell lung cancer; O2, Oncology Outcomes; OS, overall survival; Q, quartile; VONKOdb, Oncological Health Care Research Database

Large, population-level dataset across multiple countries (n=85,433)

→ Provides robust RW baseline reflecting routine clinical practice, not trial-selected populations

Curative treatments decline sharply with stage

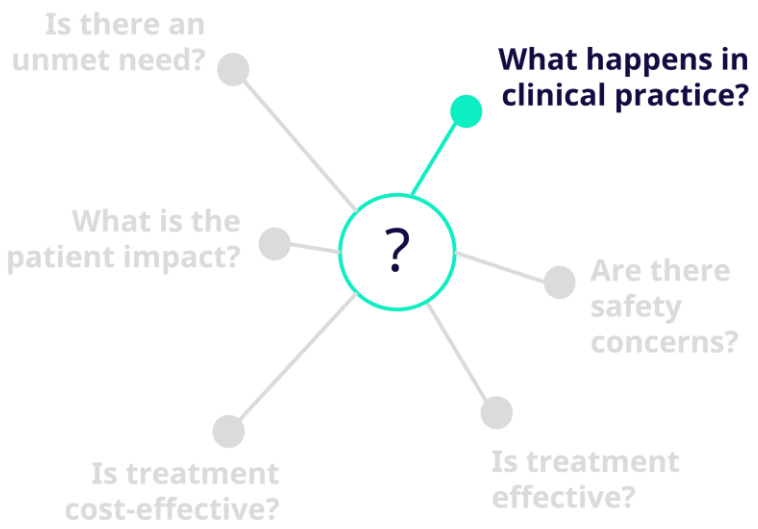
→ Limited options in later stages; need earlier detection & perioperative strategies

Poor survival in later stages (median OS ~15 months at stage III)

→ Unmet need for therapies that improve long-term survival

Greystoke, A., Daumont, M.J., Rault, C., Baltus, H., Ding, P.Q., Emanuel, G., Lucherini, S., Vo, L., Saglimbene, V.M., Ralphs, E. and Leal, C., 2025. Real-world treatment patterns and outcomes for patients with non-metastatic non-small cell lung cancer: retrospective analyses in Canada, England, and Germany. BMC Pulmonary Medicine, 25(1), p.265.

Information gained from the real-world study was useful



Large, population-level dataset across multiple countries (n=85,433)

Provides robust RW baseline reflecting routine clinical practice, not trial-selected populations

Curative treatments decline sharply with stage

Limited options in later stages; need earlier detection & perioperative strategies

Poor survival in later stages (median OS ~5–21 months)

Unmet need for therapies that improve long-term survival

- Informs control groups
- Provides **adherence** assessment
- Extended **follow-up** insights

Take home message…

Real-world data can help answer key questions