phuse·share
connect·share·advance

Single Day Event

THE ROLE OF REGISTRIES IN REAL-WORLD DATA AND THEIR IMPROTANCE IN BUILDING HEALTHCARE FUTURE

Nishanth Sungaram,
Fortrea

Agenda

- Introduction
- Types and data source of Registries
- Registry Studies vs. Submission Studies
- Impact and Investment on Registries
- Technologies involved
- Benefits, Challenges of Registries
- Case study
- Factors influencing Future of Registries

Introduction

“A registry has been defined as the organized collection of uniform observational data to evaluate specific outcomes for a defined population of persons. Registries may be developed to examine the natural history of disease, to analyze the effectiveness and safety of treatments, to measure quality of care, and other purposes”

- No restriction in time or type of collected data
- In principle, no testing of research hypothesis (i.e., cohort study)

Types of Registries

Patient

- Collect data regarding the health status of patients and the care they receive
- Evaluate outcomes, best practices, and treatment guidelines
- Established by patient foundations and pharmaceutical organizations

Specialty

- Focus on advancing care outcomes across a medical specialty or subspeciality
- Aim to develop guidelines and decision support tools and advance research
- May serve as QCDRs to allow clinicians to CMS under MIPS

Population

- Focus on entire patient population, spanning specialty care and specific disease
- Seek to capture comprehensive population-level health status data

Device

- Focus on tracking the safety and effectiveness of medical devices
- Support post-market surveillance
- Established by medical specialty organizations and medical device companies

Payer

- Focus on improving outcomes and reducing costs
- Aim to measure and enhance value
- Established by healthcare payer organizations

QCDR: Qualified Clinical Data Registry; CMS: Content Management System; MIPS: Merit-based Incentive Payment System

Data source of Registries

Primary:

- Patients
- Care Givers
- Clinicians
- Etc..

Secondary:

- Electronic health records
- Ancillary clinical information systems
- Clinical data warehouses (CDWs) or integrated data repositories (IDRs)
- Administrative (claims) databases
- Death indexes
- Aggregate/non-patient level databases
- Existing registries
- Distributed research networks

Registry Studies vs. Submission Studies

	Registries	Submission
Purpose	gather real-world data on the use and outcomes of a drug or medical device in a particular patient population	generate the data required to support regulatory approval of a new drug or medical device
Design	typically, observational in nature and may be conducted prospectively or retrospectively	randomized controlled trials (RCTs) designed to minimize bias and provide the highest level of evidence to support regulatory approval
Population	enroll a broader patient population than submission studies	typically enroll a more narrowly defined patient population that is representative of the intended use of the drug or medical device
Endpoints	may have a wide range of endpoints, including patient-reported outcomes, quality of life measures, and clinical outcomes such as mortality and morbidity	typically focus on clinically relevant endpoints such as survival, disease progression, or symptom relief

Impact and Investment on Registries

Market Statistics

Study Period	2019 - 2030
2024 Market Size	2,397.5 Million
2030 Forecast	5,638.3 Million
Growth Rate (CAGR)	15.3%
Largest Region	North America
Fastest Growing Region	Asia-Pacific
Nature of the Market	Consolidated

Market Size Comparison

Technologies involved

Some of the different technologies involved in registry studies include:

- Electronic health records (EHRs)
- Health information exchanges (HIEs)
- Data warehousing
- Data visualization tools
- Natural language processing (NLP)
- Machine learning (ML) and artificial intelligence (AI)

Benefits

Evidence Generation/Real-world data

Cost-effective

Large sample size

Long-term follow-up

Access large datasets more rapidly and less expensively

Insurance billing and claims

Rare Diseases Support

Enhanced Clinical Decision-Making

Improved patient care

Challenges

- Variable quality of data (accuracy and completeness) – sometimes necessary information may be unavailable
- Lack of active follow-up
- Often, a lack of detail in the data collected
- Missing or incomplete data may represent a greater source of bias for registries
- Data selection and quality are defined by the register and not controlled by the researcher

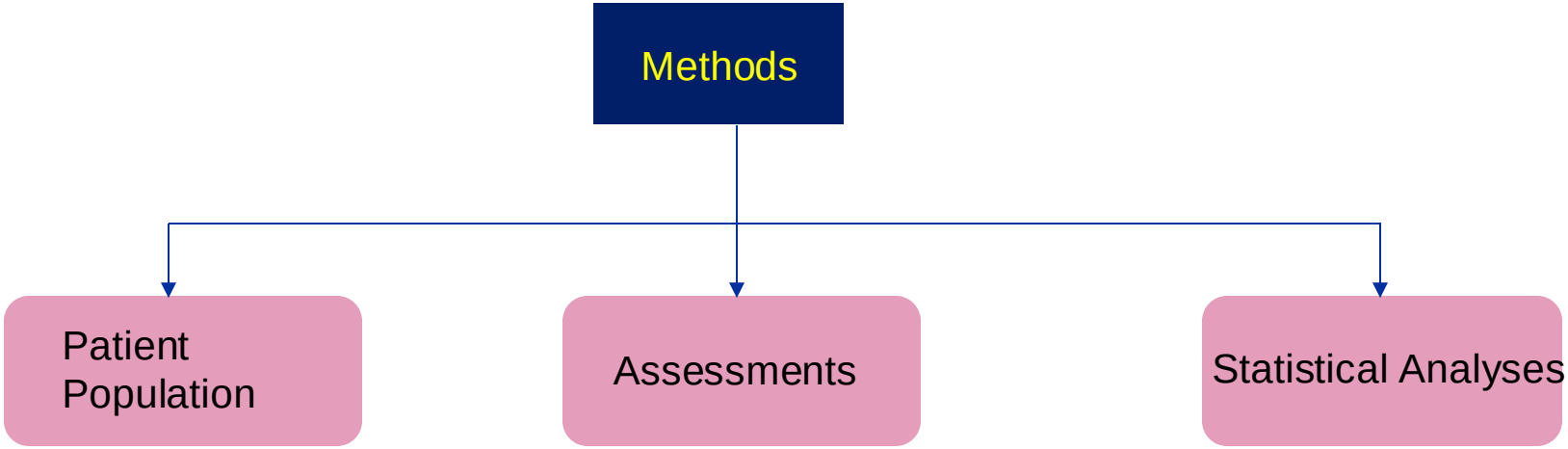
A Case study

Background: The case study considered here is a rare disease and there were no major clinical trials conducted for the rare disease medication. So, Registry studies help us to analysis the better patient outcome in different categories and analysis.

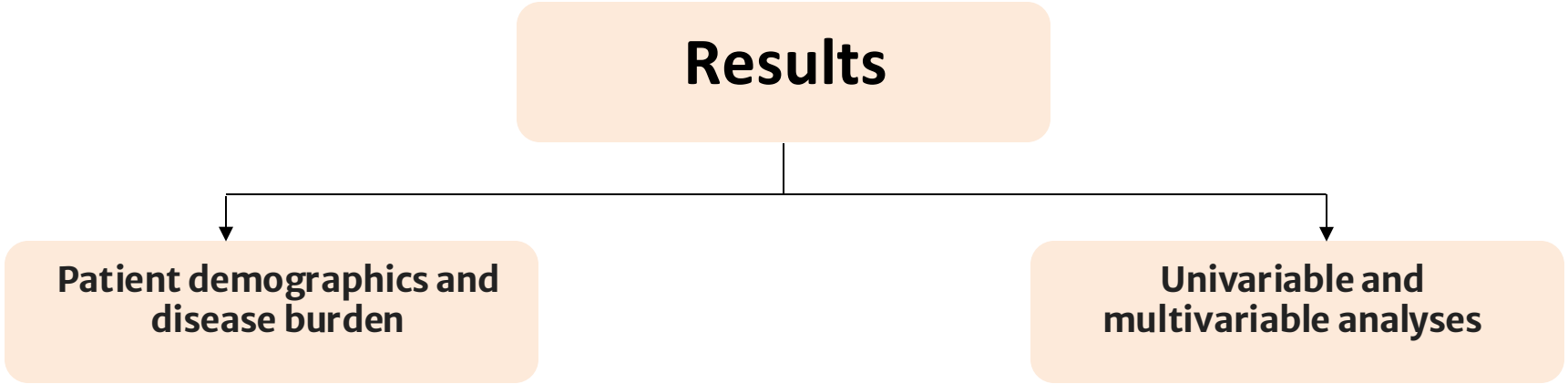
Abstract: [1]The objective of this analysis was to identify risk factors for thromboembolic events (TE) in patients with paroxysmal nocturnal hemoglobinuria (PNH) who were not treated with C5 inhibitors.

Purpose: Thromboembolic events (TEs) are the leading cause of death in patients with PNH, accounting for 40% to 67% of deaths before the complement inhibition era, with a 5-year mortality rate of approximately 30%. The risk of incident thrombosis increases over time if targeted therapy is not initiated.

A Case study - Methods



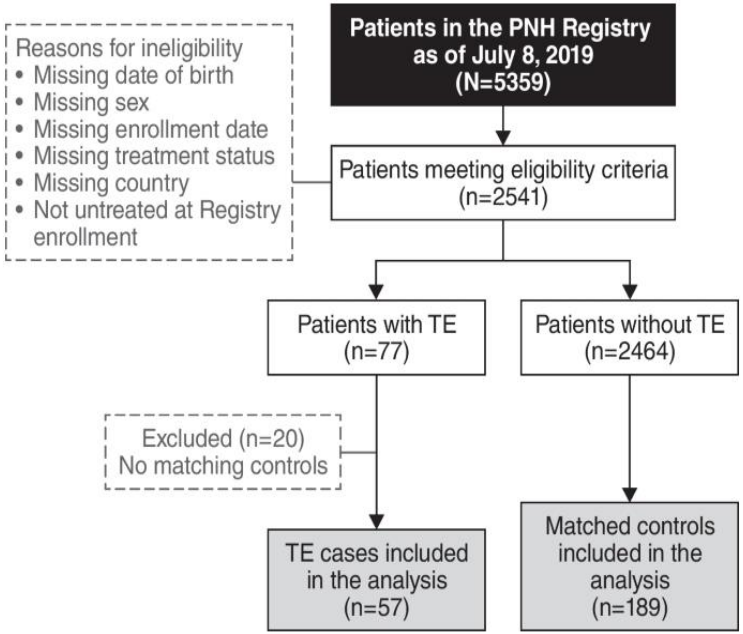
A Case study - Results



A Case study (Cont.)

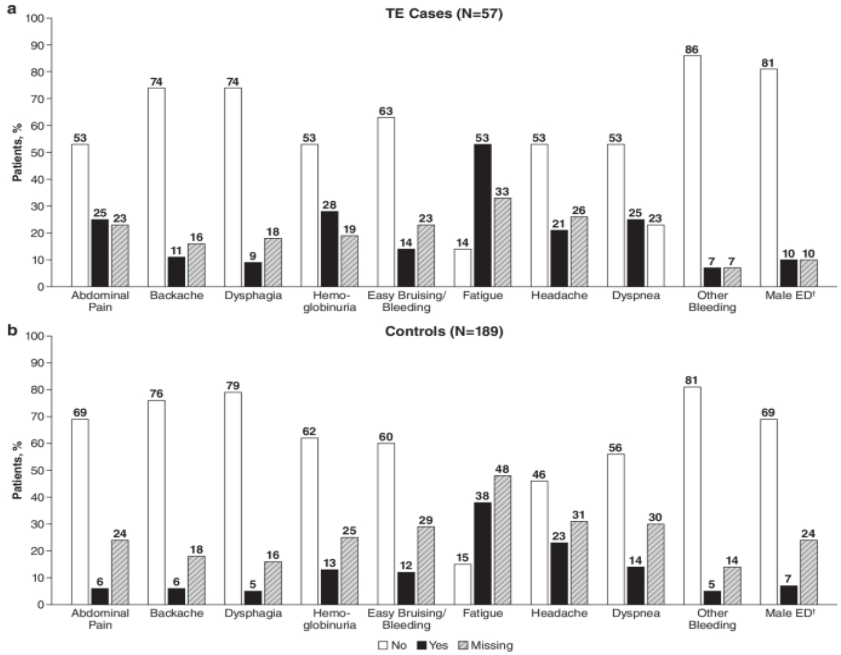
Patient demographics

Fig. 1



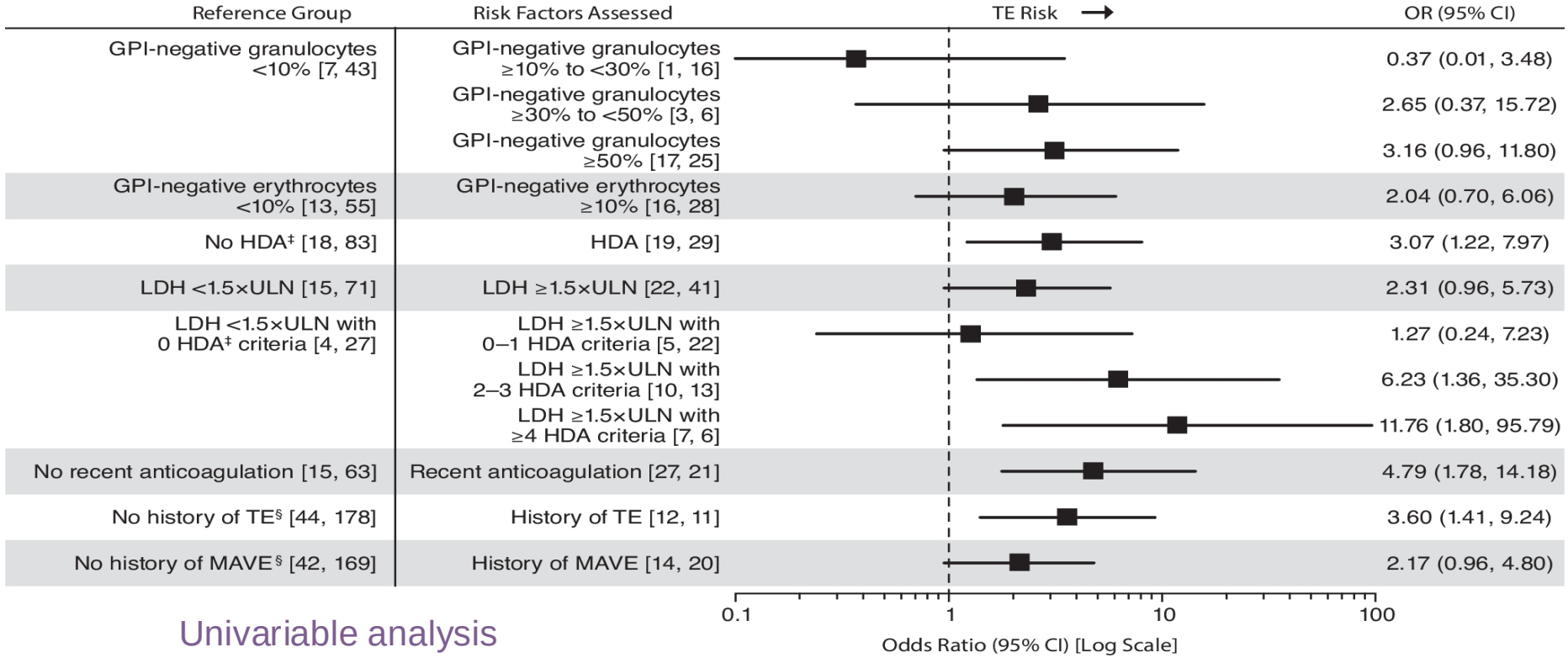
TE case and control selection from the International PNH Registry. PNH, paroxysmal nocturnal hemoglobinuria; TE, thromboembolic event

Fig. 2



Physician-reported symptoms within 6 months before the index date. (**a**) TE cases. (**b**) Controls. †TE cases (n = 31), controls (n = 103). ED, erectile dysfunction; TE, thromboembolic event

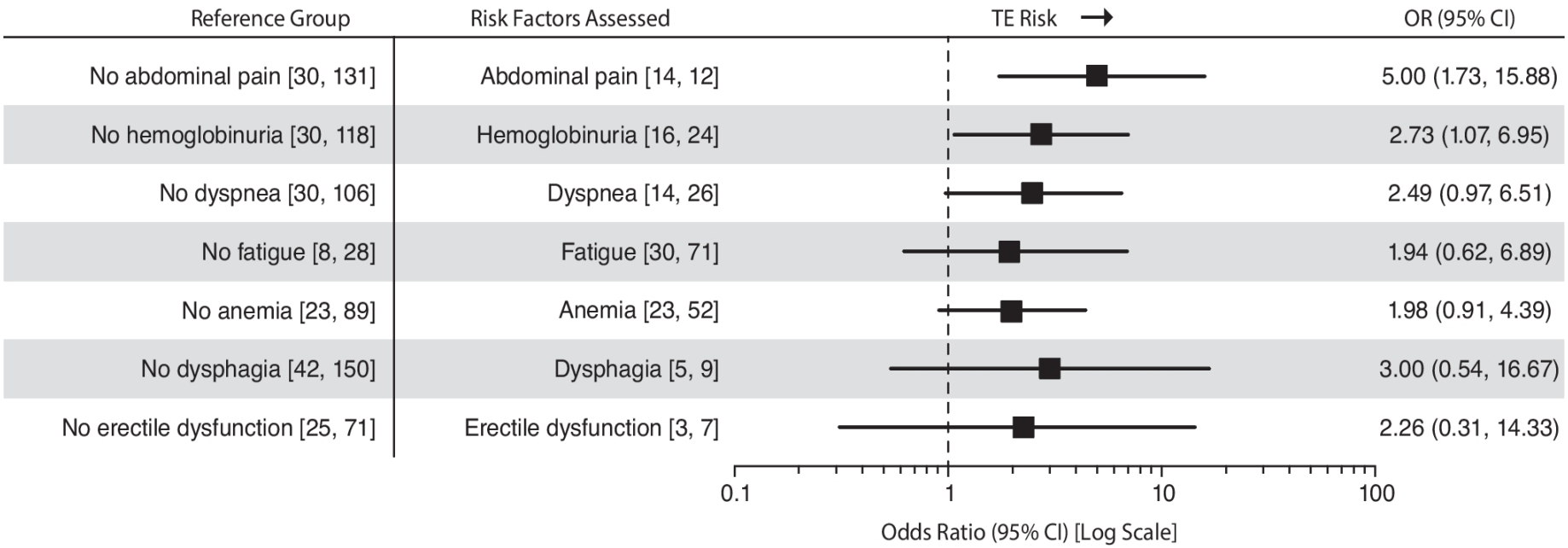
A Case study (Cont.)



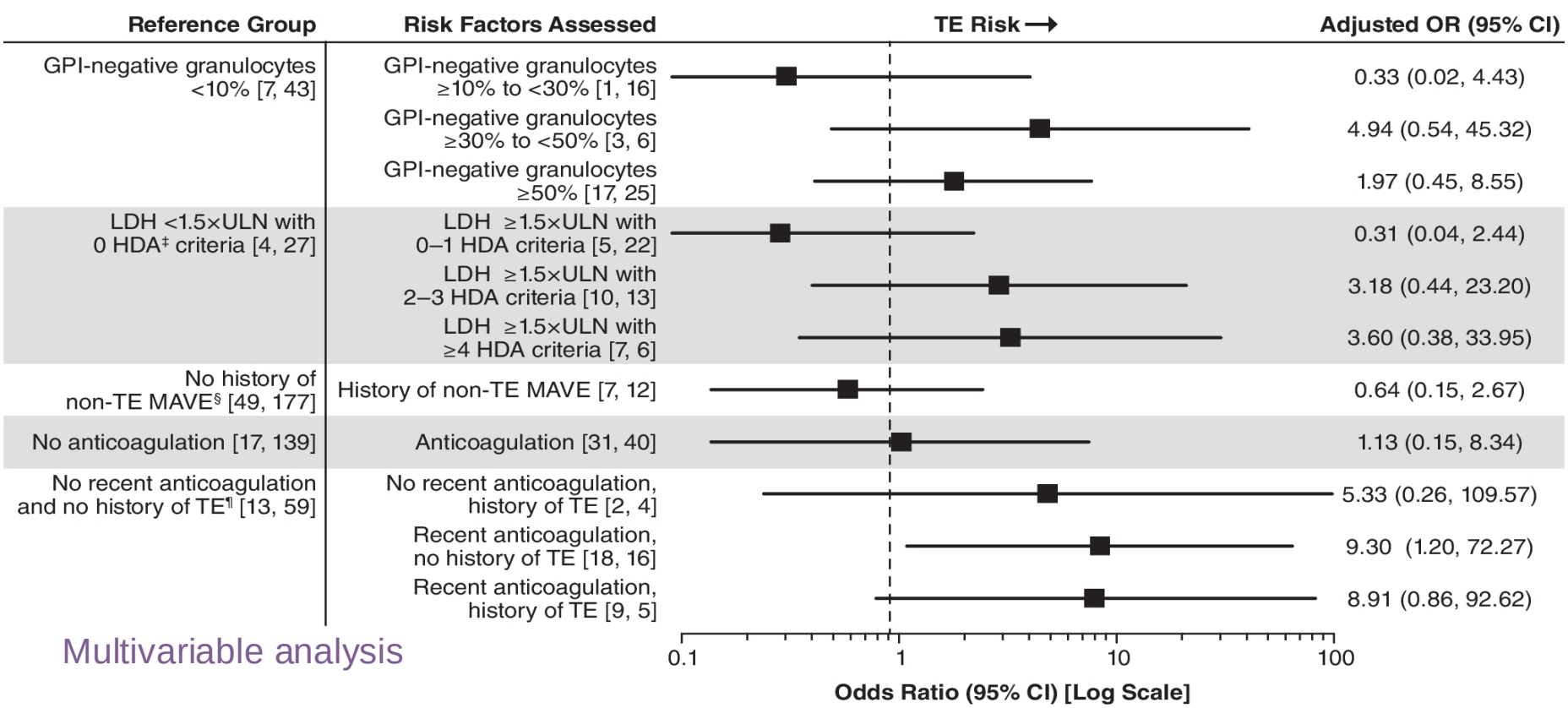
Univariable analysis

A Case study (Cont.)

Univariable analysis



A Case study (Cont.)



Multivariable analysis

A Case study - Conclusion

Discussion & Conclusion of a case study:

- This analysis found that increased TE risk was also associated with a set of physician-documented symptoms, including abdominal pain, and dysphagia, which may be symptomatic markers of hemolysis or nitric oxide sequestration.
- Risk of TE increased with intravascular hemolysis, abdominal pain, chest pain, dyspnea, and hemoglobinuria, but not anemia.
- Patients receiving primary prophylaxis who commence complement inhibition may be able to stop anticoagulation after intravascular hemolysis is controlled
- The findings of this analysis were based on data derived from a broad global database and confirm the results of previous studies.
- Further study to identify and monitor risk factors for TE in patients with PNH is warranted to better inform treatment decisions and improve patient outcomes.

Factors influencing Future of Registries

Emerging trends or technologies that may influence the future of registries-

- **Increasing popularity of data coordinating centers** – it collaborates with teams to enhance data system usability, function, and compliance
- Increasing **adoption of electronic health records (EHRs) and interoperability standards** -- This will enable seamless data sharing between different registries and healthcare systems or organizations. -- richer and more comprehensive datasets, leading to more robust research and improved patient care

Factors influencing Future of Registries

- Growing use of **machine learning** algorithms may allow for more sophisticated data analysis and predictive modeling within registries
- Collaboration between different stakeholders, including researchers, healthcare providers, and patient advocacy groups, may enable more patient-centered and meaningful research
- Innovative strategies and systems that will allow for collection, curation and analysis of the data needed to deliver great care has great scope

References

- [1]https://clinicaltrials.gov/study/NCT01374360
- https://doi.org/10.1007/s00277-023-05402-3
- https://alexion.com/our-research/research-and-development
- https://www.navitaslifesciences.com/the-rising-popularity-of-clinical-patient-registries-and-their-importance-in-building-healthcare#:~:text=Clinical%20registry%20studies%20are%20based,also%20include%20data%20from%20caregivers
- https://yoursay.plos.org/2024/03/best-practices-for-clinical-registries-while-leveraging-real-world-evidence/
- https://www.psmarketresearch.com/market-analysis/patient-registry-software-market-report
- https://www.pnhinterestgroup.org/
- https://pro.carenity.com/library/patient-registries/
- https://www.arbormetrix.com/blog/clinical-data-registry-basics/

Thank You!

The Global Healthcare Data Science Community

Contact Channels
UK +44 1843 609600
office@phuse.global
phuse.global

Social Media
@phusetwitta
/phusebook
/phusetube
/company/phuse