Treatment of Cancer Associated Thrombosis: Insights beyond RCTs

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www.garfieldregistry.org

The GARFIELD-VTE registry is funded by an unrestricted research grant from Bayer AG

VTE Garfield global anticoagulant registry in the field

Disclosures

Grants and personal fees

Bayer

Personal fees

Boehringer-Ingelheim Pharma, Daiichi Sankyo Europe, Sanofi SA, Janssen Pharma, Verseon Inc.

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My talk today

- **Randomised Controlled Trial vs. Real World Evidence**
- **The Garfield VTE study**
- **Managing CAT:**
  - Patterns of practice
  - Burden of disease

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Real-life studies validate use of therapies in general population

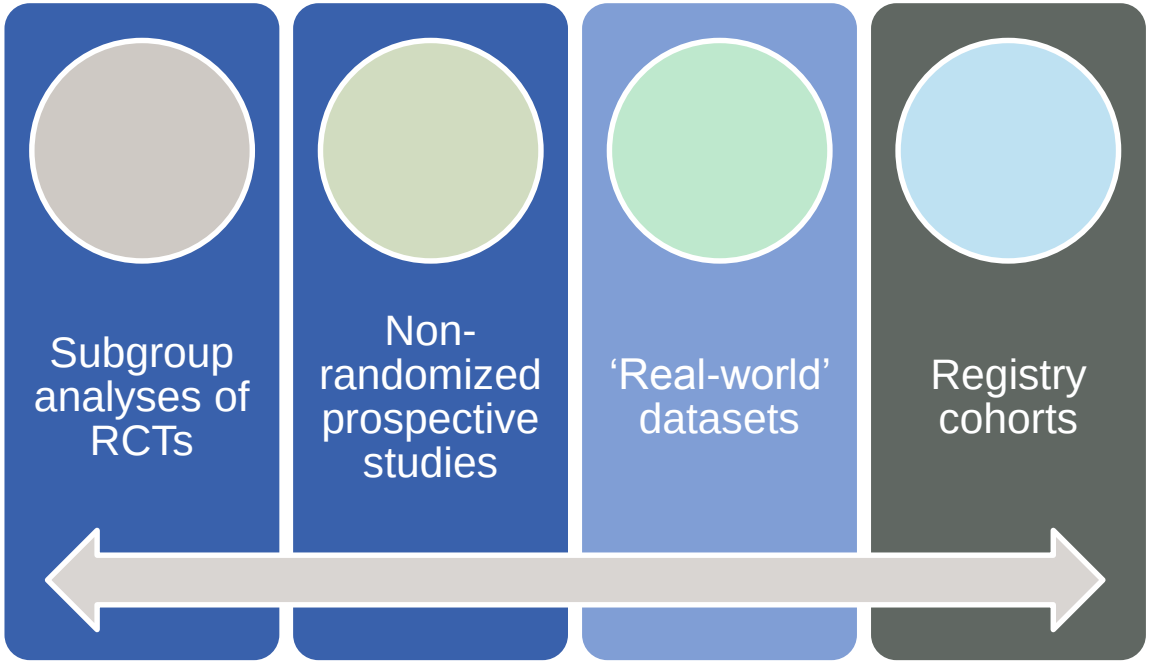
Clinical trials: Interventional, controlled

- The standard for assessing efficacy and safety of therapies and supporting marketing approval
- Randomised and powered
- Strict design to ensure well defined inclusion and exclusion criteria
- Strict protocol adherence, appropriate clinical endpoints and statistical validity
- Hypothesis driven

Real-life studies: Non-interventional, observational

- Describe practice patterns and outcomes, and their evolution
- Identify opportunities to improve quality of care
- Identify sub-optimal implementation of approved therapies
- Hypothesis generating

Data beyond the RCT



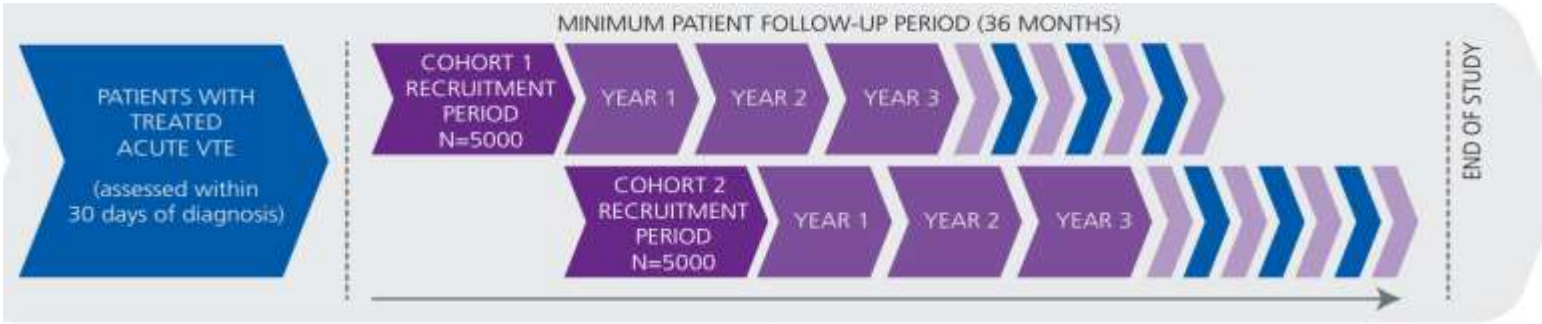
Using outcomes datasets: what is appropriate?

- Disease burden
- Patterns of practice
- (Comparative) effectiveness and safety

Garfield-VTE: A Prospective global disease registry

Design
- Independent academic research initiative
- 10 878 newly diagnosed VTE patients in 28 countries
- Randomised selection of sites representative of national VTE care settings
- Unselected prospective patients enrolled consecutively
- Long-term follow-up (minimum of 3 years)
- Two sequential cohorts of 5000 patients

Audit requirements
- 10% of all CRFs monitored against source documentation
- Electronic audit trail for all data modifications
- Critical variables subjected to additional audit
- Compliant with Declaration of Helsinki



N.B. Striped area indicates possible follow-up for up to 2 years after the initial 36-month follow-up period

ClinicalTrials.gov identifier: NCT02155491

Weitz JI et al, Thromb Haemost 2016;116:1172–1179

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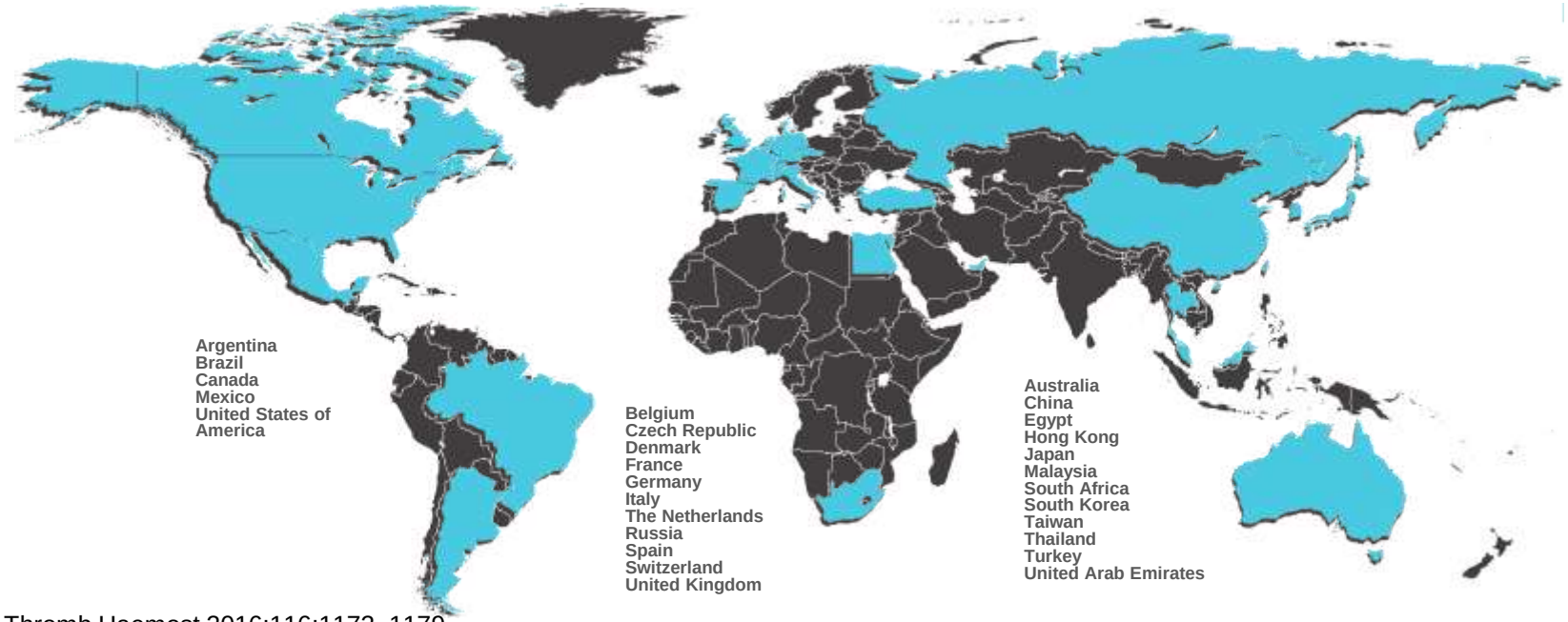
Participating countries

10 878 patients enrolled in 28 countries



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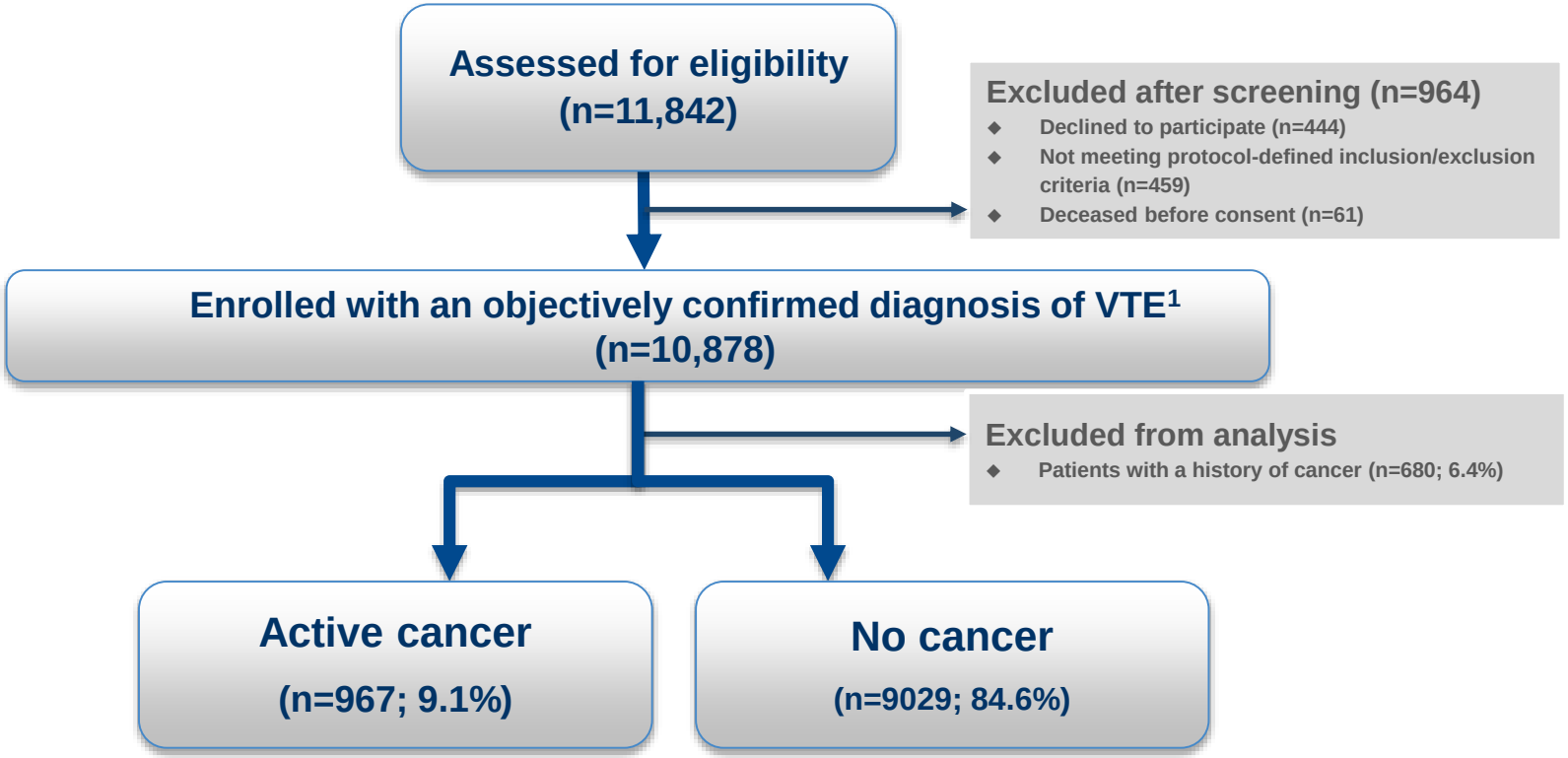
Patient population



[1] As defined by Bates et al. Chest 2012; 141(Suppl): e351S–e418S

Data cut: November 2017

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AI and machine learning will transform research and care

- **Healthcare has consistently been the top industry for investment in AI**
  - Ahead of cyber security, commerce and finance
  - Healthcare incumbents including providers, pharmaceuticals and insurers are incorporating AI into their existing services
  - Established leaders in the technology industry are adding healthcare to their core business, and start-ups are raising capital at record rates
- **AI & ML promise enormous gains across a wide range of health applications**
  - Drug discovery, risk prediction, imaging and diagnostics, virtual assistance and remote monitoring.
- **ML algorithms are already being applied in cardiovascular disease**
  - Calculate risk
  - Predict diabetes
  - Diagnose atrial fibrillation

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Conclusions

- Interest in data beyond the RCT setting
- Alternative methodological approaches
- Management of CAT in ’real world’ differs from RCT and guideline practice
- CAT carries a substantial burden of disease