Introduction to the use of Observational Data

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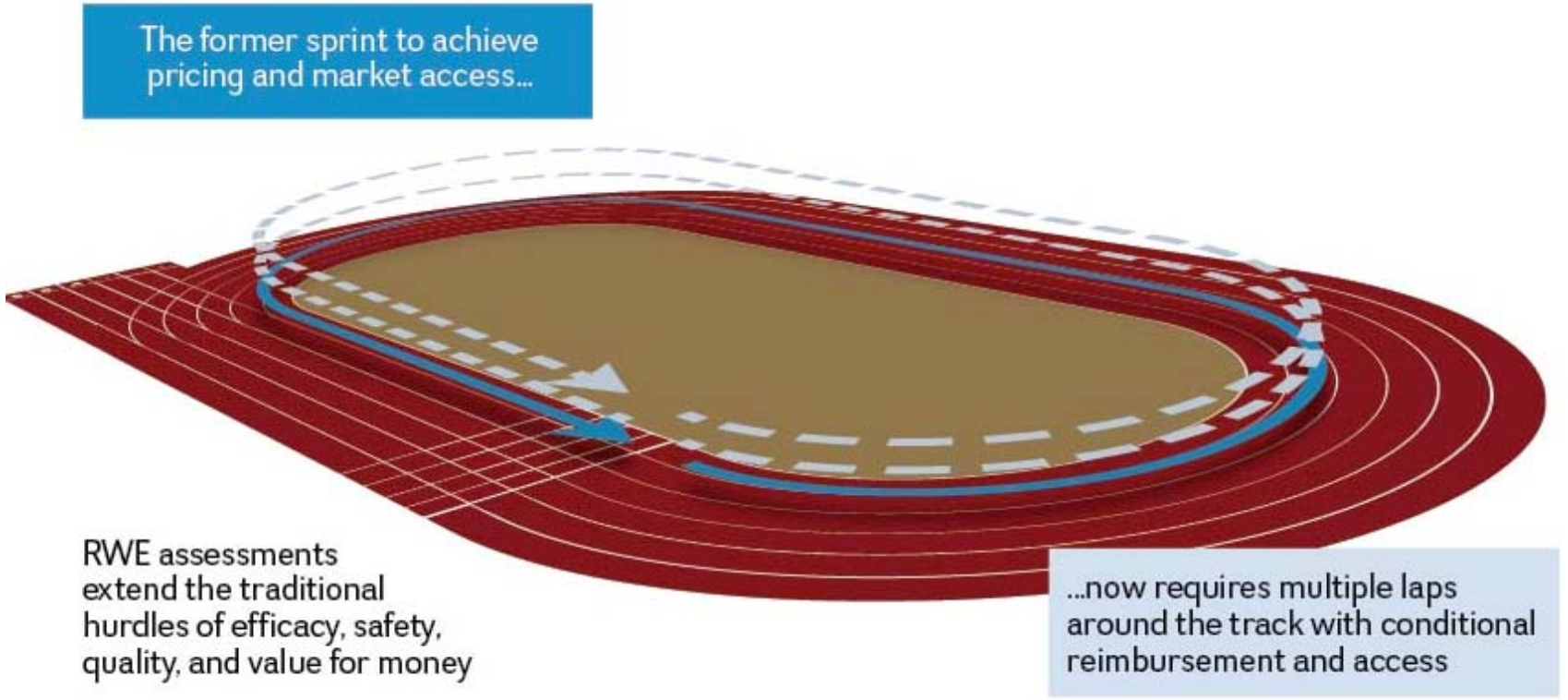
Evidence now required across the product lifecycle

New approaches to evidence generation are needed



Value demonstration more than just at launch

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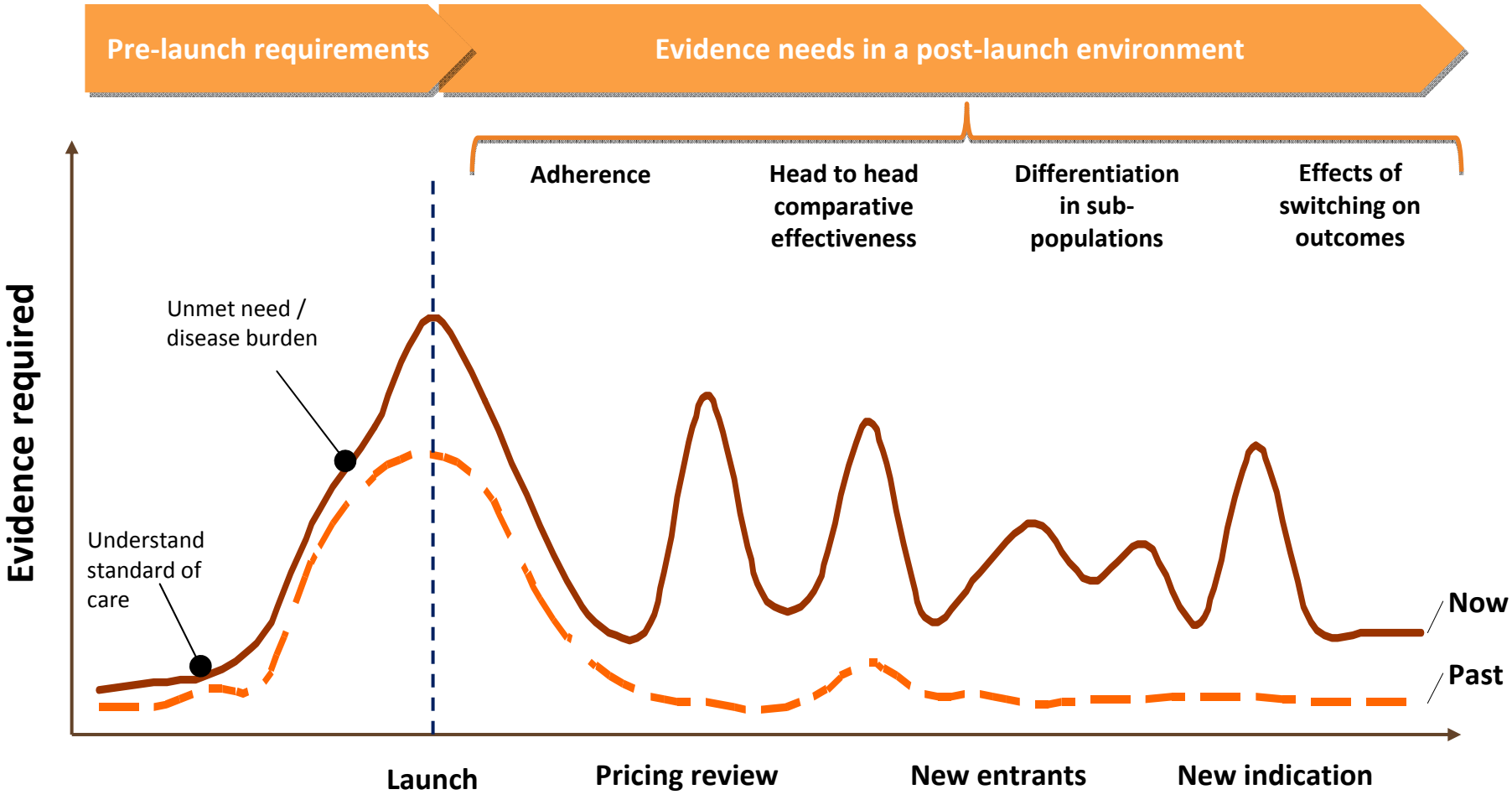
The typical registry design strategy

(Hopefully) a strategy of the past

- Basic risk aversion strategy
  - Wait until regulatory approval
  - Initiate registry
- Deluxe risk aversion strategy
  - Basic risk aversion strategy, plus...
  - Burden of illness study
- Platinum risk aversion strategy
  - Deluxe risk aversion strategy, plus...
  - Post-launch database study

New demands require new evidence solutions

Continuous evidence requirements



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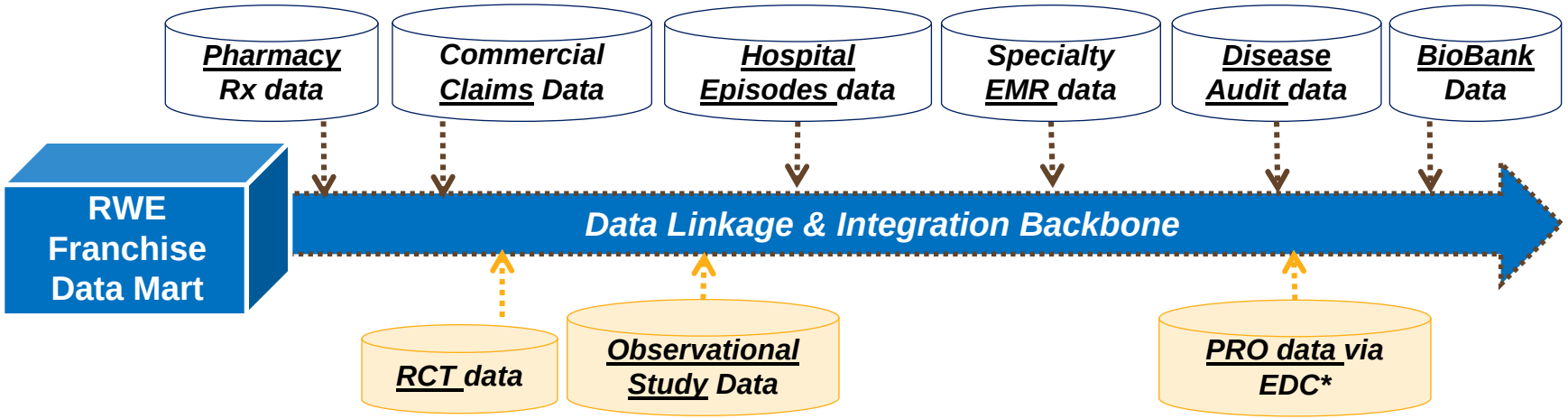
Evidence generation: start early and build over time

One such approach is the creation of franchise "data-marts"

Leverage Existing Real World Data Sources *(source, access, and integrate based on brand needs)*



Leverage internal RCT and other data *(to complement existing real world sources)*

VALUE

- Rx patterns
- Patient diagnosis, history & co-morbidities
- Comparative RCT & Real World patient cohorts
- Hospital dx, procedures, tests, LoS, ward, specialist
- PC & Hospital episodes data linked to Rx data,
- Detailed and longitudinal TA specific clinical and outcomes data across PC & SC

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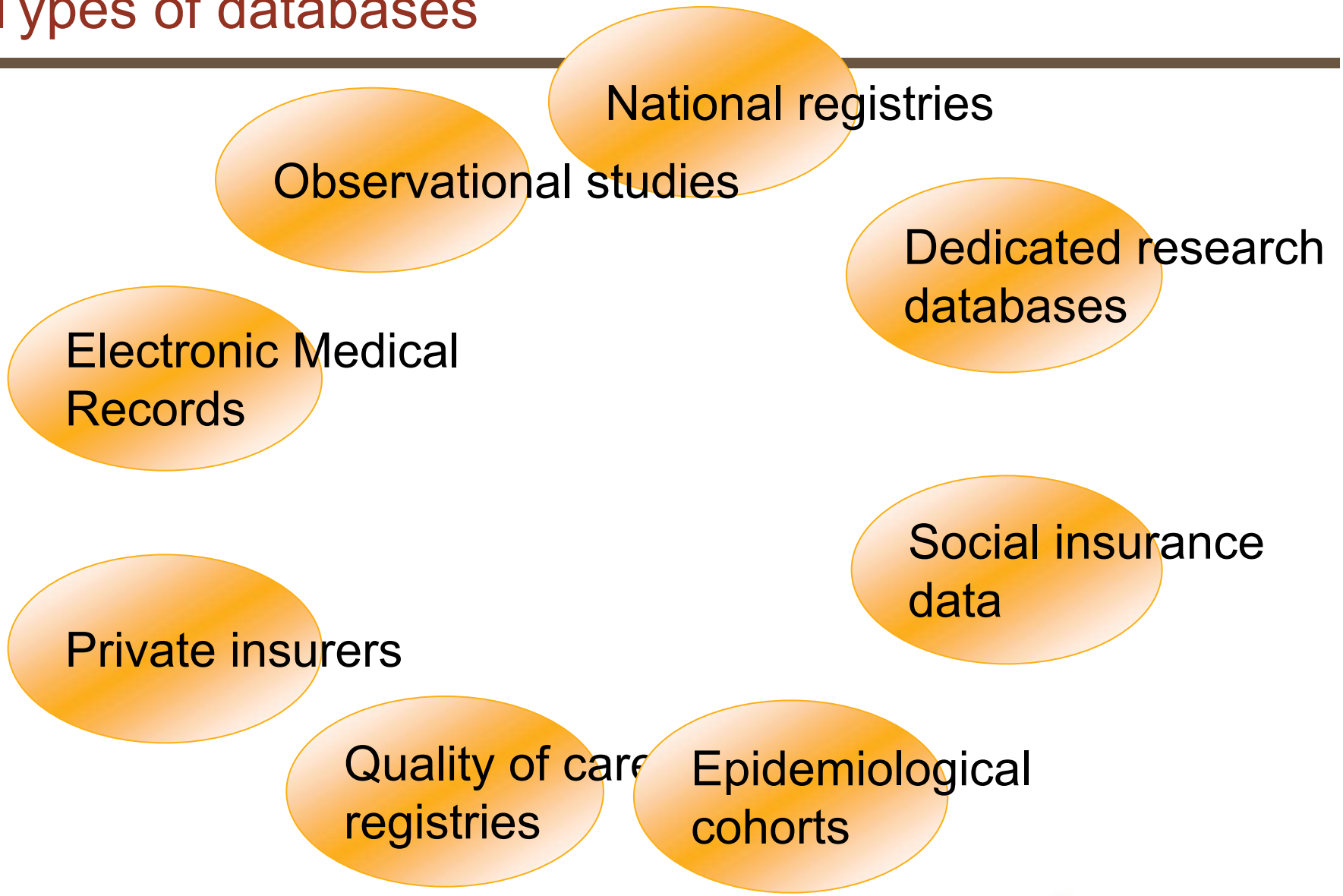
Introduction to Database Research

Types of databases



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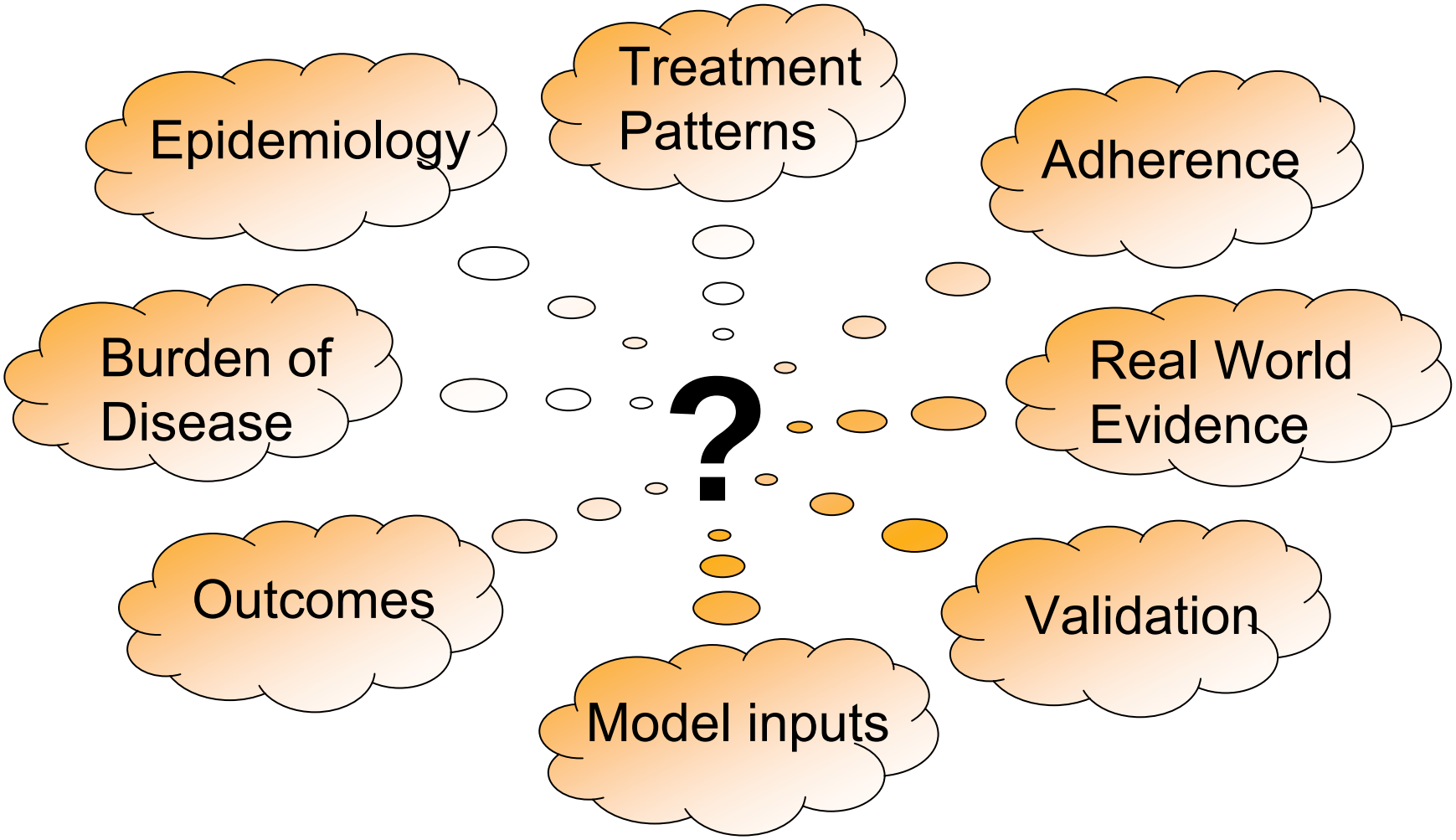
How can we use them in HE&OR?



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Strength and Weaknesses of Database Research

Strengths	Weaknesses
Registries reflect real life practice	No Randomization – selection bias when comparing groups
Broader enrollment criteria	Can't decide what data to collect
Less protocol driven	Poor reporting/coding
Cost effective use of data that is already collected	No impact on data points collected
Time efficient	Data is not exclusive
Allows for large sample sizes	Data is often not for sale

Insurer Claims Database: MarketScan

- **Contributors are Thomson Reuters clients**
  - **Payers, e.g., self-insured employers and Medicaid states**
    - **Over 150 employers in the most recent 3 years**
    - **Payers direct their carriers (~200 in most recent 3 years, from national to local plans) to submit data**
  - **Health Plans**
    - **Plans are submitting directly to MarketScan**
    - **Typically regional plans, e.g., BCBS**
    - **Approximately 20 plans submit, ~25% of MarketScan claims populations**
- **Contributors release data to MarketScan in exchange for benchmark reports**

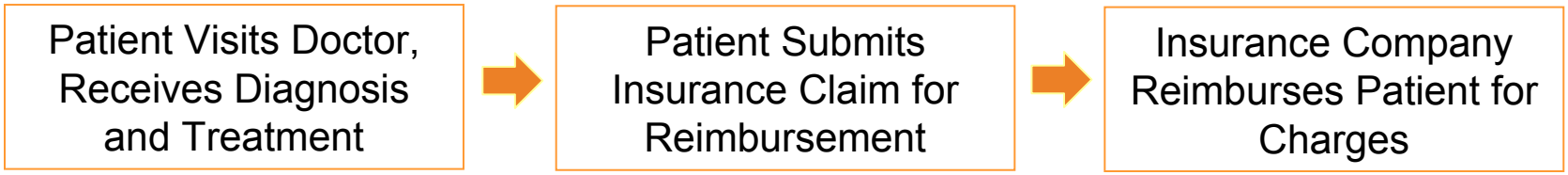
Electronic Medical Record: GPRD

Coverage

- Vision is used by many GP practices
- UK-wide
  - England
  - Scotland
  - Wales
  - Northern Ireland
- We collect from ~7% of the UK
- GPs are paid based on the number of patients

Examples

Lucentis: Prevalence, incidence and cost of DME using insurance health claims data



- Medical Encounter Data from all available healthcare sites, e.g.
  - Inpatient Hospital
  - Outpatient Department
  - Emergency Room
  - Physician Office
  - Surgery Center
  - Etc.
- Pharmacy Claims
  - All Outpatient Pharmaceutical Purchases
- Insurance Plan Enrollment Records
  - Demographic Data
  - Benefit Eligibility Data

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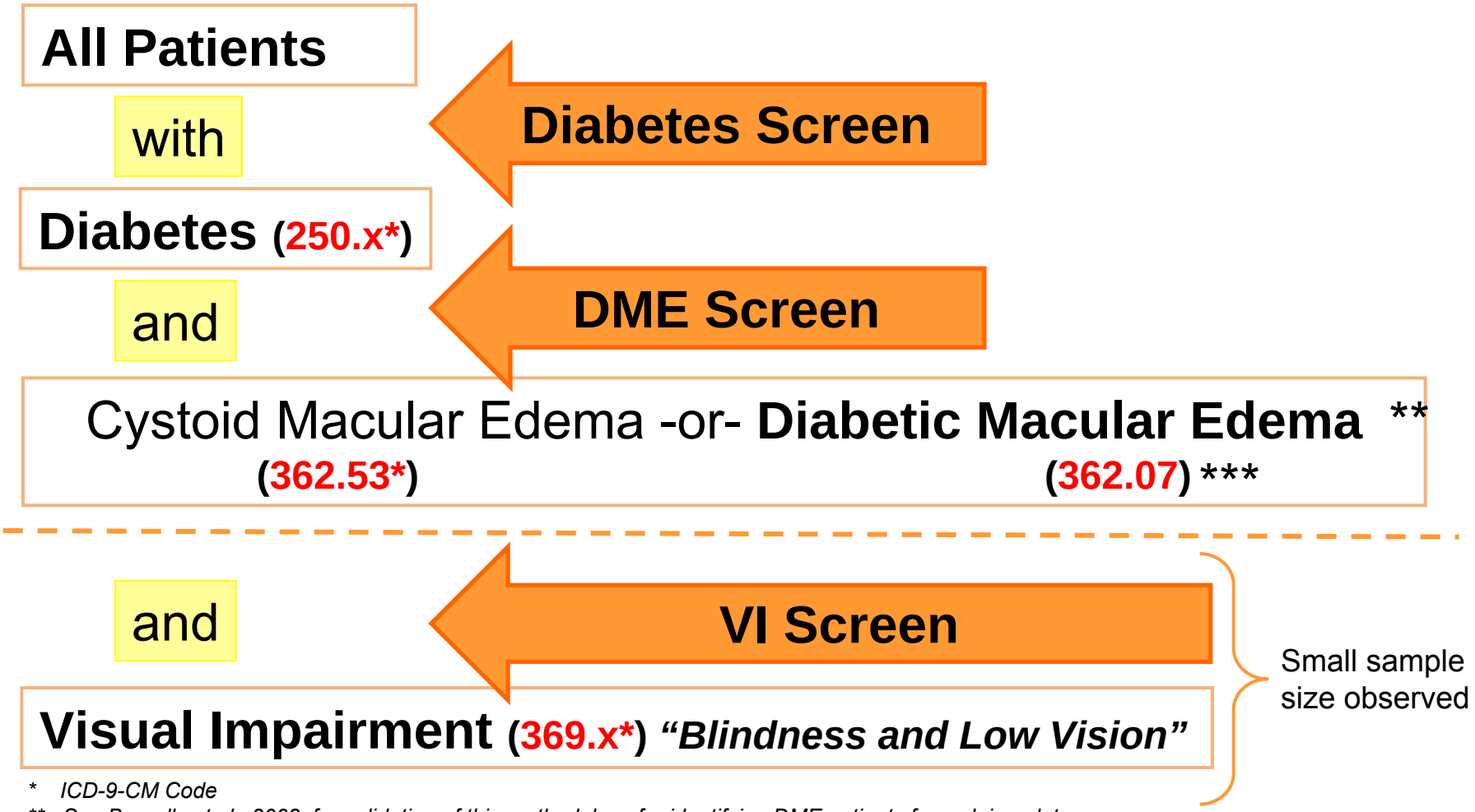
Sample Selection Methodology Schematic



* *ICD-9-CM Code*

** *See Bearelly et al., 2008, for validation of this methodology for identifying DME patients from claims data.*

*** *Code available from 2005 on*

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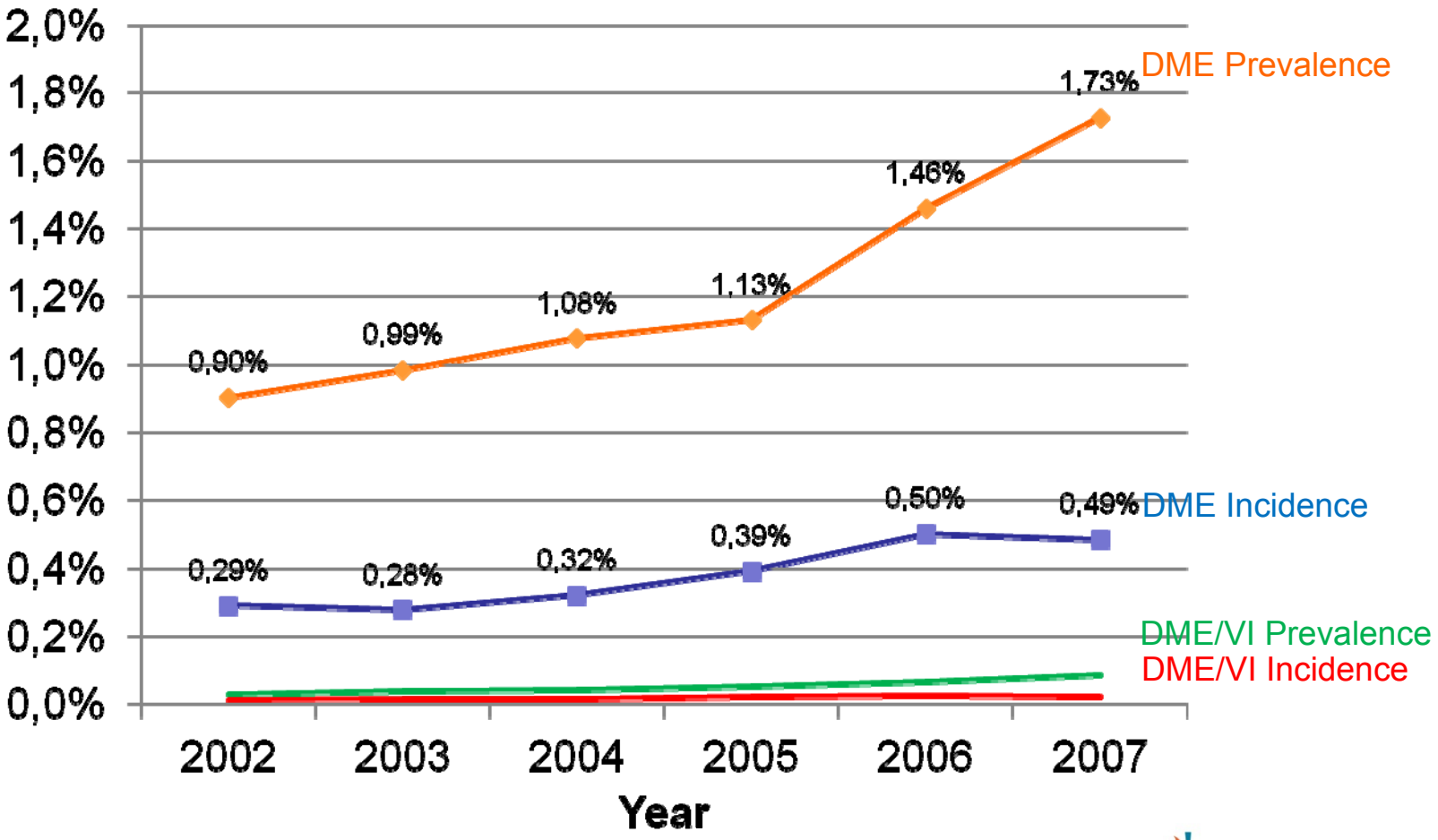
US Prevalence and Incidence of DME and DME/VI, 2002-7 Among Diabetic Population



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Retrospective Cohort Study of the Effects of Early vs. Late Treatment of Insomnia in Patients Initiating Anti-Depressant Medications

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[2]Novartis Pharmaceuticals, East Hanover, NJ, USA.

[3]University of Texas Southwestern Medical School, Dallas, TX, USA

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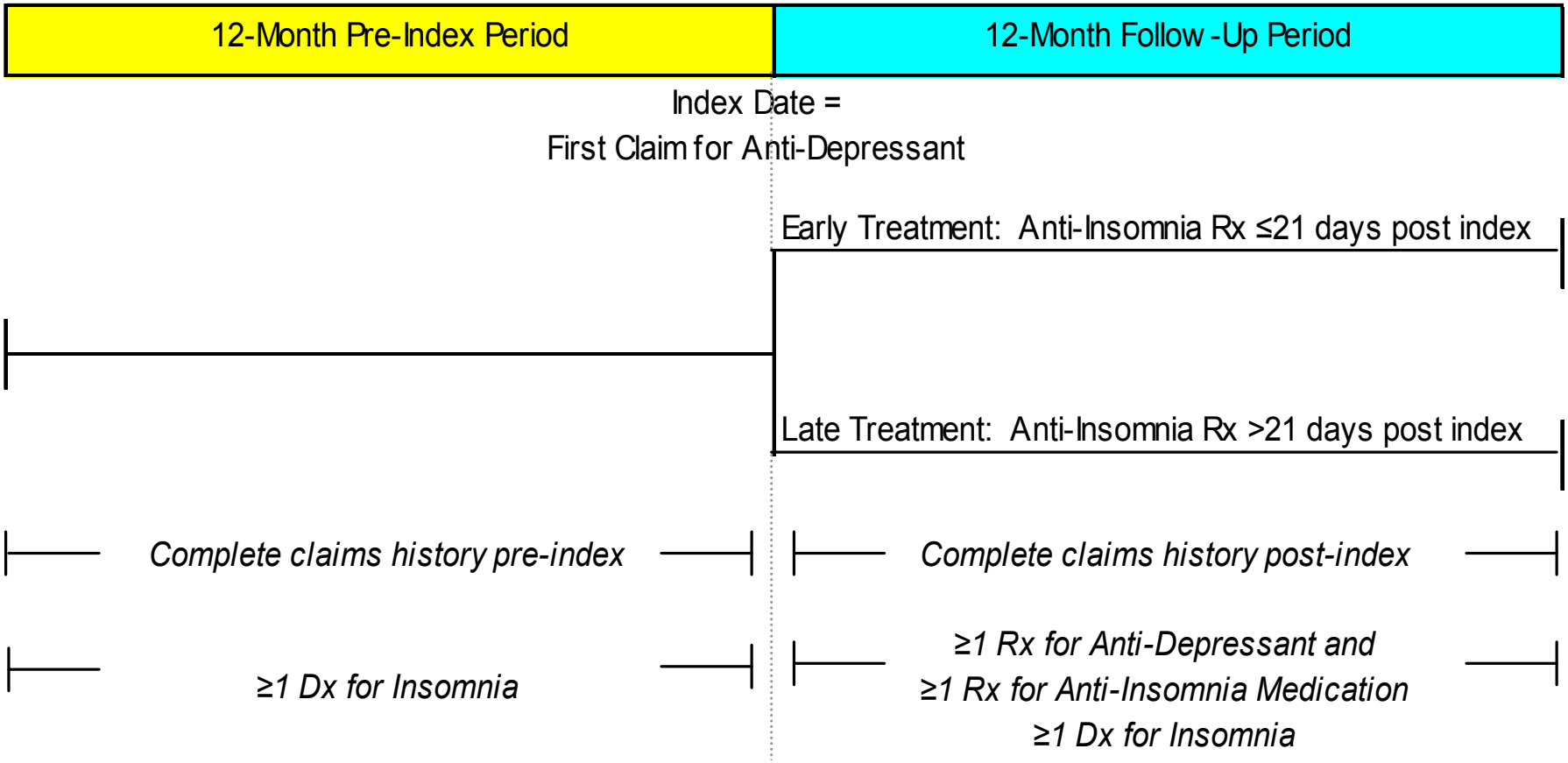
Study Design

- **Approach:** Retrospective matched cohort study
- **Data source:** MedStat MarketScan® Research Databases, including
  - Commercial Claims and Encounters (CCAE) database
  - Medicare and Coordination of Benefits (MDCR) database
  - Health and Productivity Management (HPM) database
- **Study population:** Depressed patients who initiate anti-depressants (AD) who received either:
  - **Early treatment** with anti-insomnia medications (≤3 wks post initiation of AD)
  - **Late treatment** with anti-insomnia medications(>3 wks post initiation of AD)
- **Matching**: Patients receiving early vs.. late treatment matched based on propensity scores and other patient characteristics at index date*
- **Follow-up:** 12 months post-index date
- **Outcome Measures:** Compliance with AD, switching of AD, AD and all-cause healthcare utilization and costs, employer costs of paid absences during follow-up

*Index date=Date of initiation of AD.

Study Design



Patients receiving early treatment with anti-insomnia medications matched to those receiving late treatment based on propensity scores and other characteristics at index date

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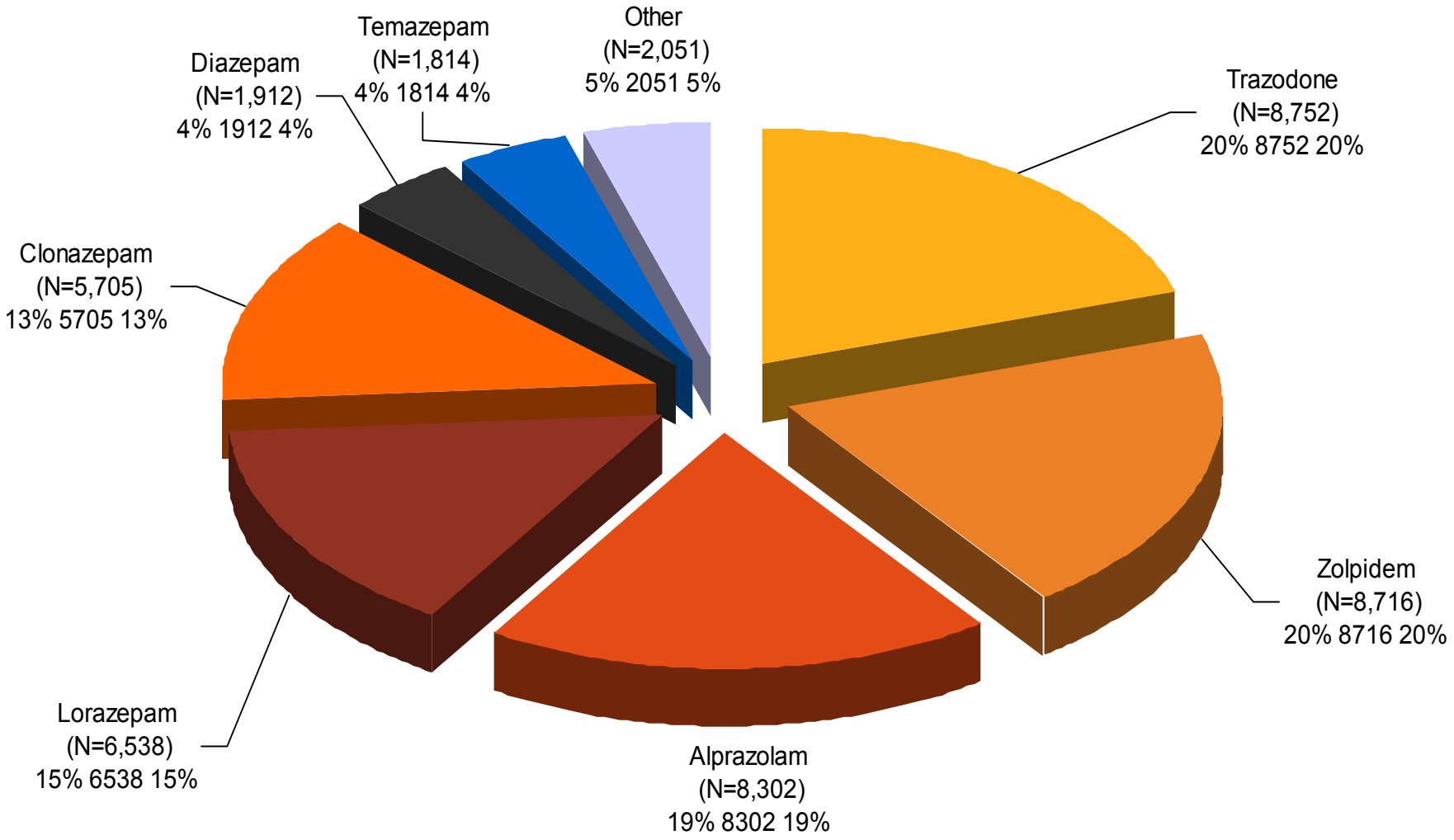
Matching

- Propensity scores were calculated for all subjects by estimating logistic regression model with early or late treatment as dependent variable and all pretreatment characteristics as independent variables (22,23)
- The propensity score for each member was defined as predicted probability (range: 0 - 1) of receiving early treatment conditional on observed values of other characteristics
- Matched pairs of early and late treatment subjects were identified using greedy matching technique (maximum difference in propensity score between matched pairs=0.01) (24)
- Patient characteristics that remained significantly different across pairs after propensity score matching were added to matching algorithm in stepwise fashion based on Ps until there were no significant differences between groups in patient characteristics
- Matching process repeated for subsample of patients with data on paid absences

Sleep Medications Received During Follow-up



N=number of pharmacy claims

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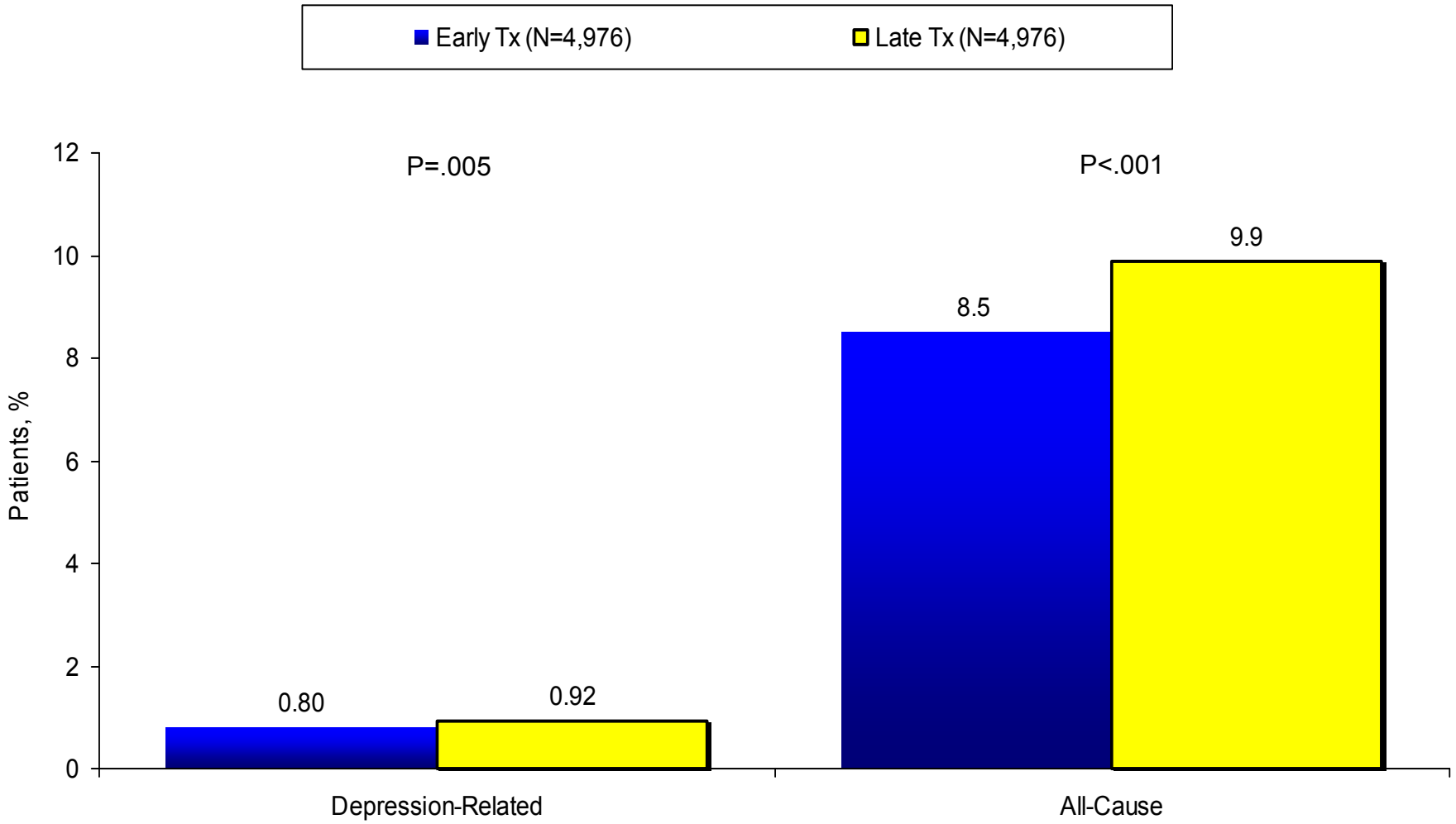
Mean Number of Outpatient/Office Visits During Follow-Up: Matched Patients



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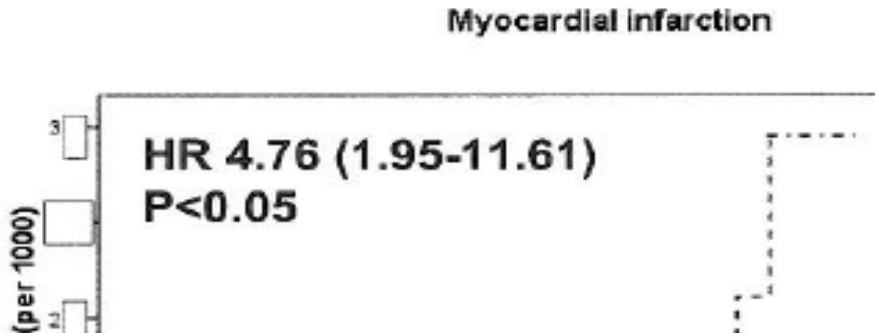
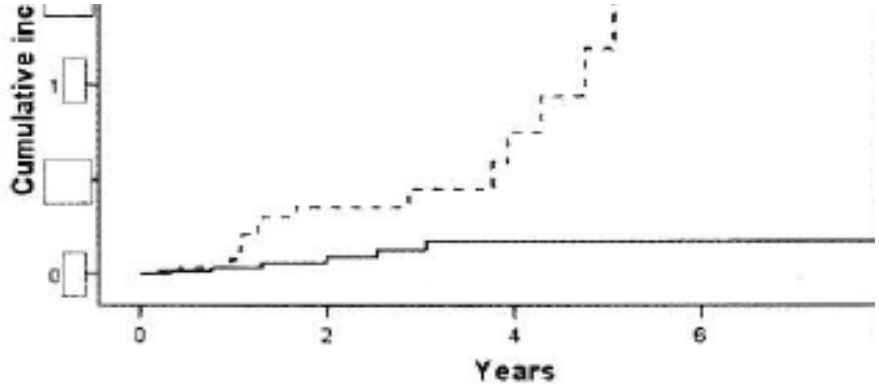
Caution!!!

Preliminary analysis: from KOL



Oral bisphosphonate trt

5-fold, significant increased risk of MI due to oral BPs!!



No treatment

Consequence for oral BPs: Death of class

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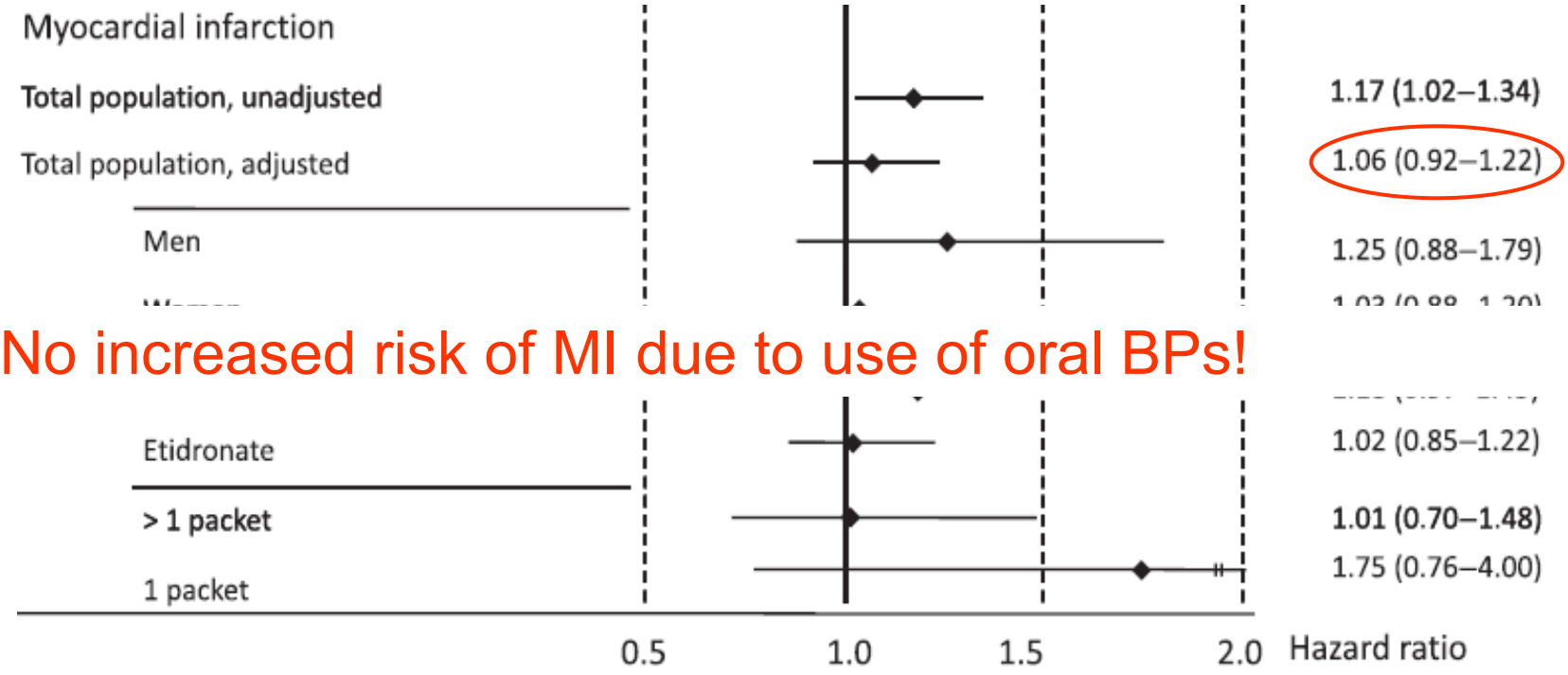
Final analysis: collaboration NVS and KOL



No increased risk of MI due to use of oral BPs!

Consequences for oral BPs: None

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