

GLOBAL EPI RESEARCH CASE STUDIES



Case Study #1

Problem:

A registry platform provider sought our expertise to design research-worthy registry questionnaires and data collection tools that met the varying needs of multiple registry stakeholders (which included a rare disease patient advocacy group, two large international pharmaceutical companies, and multiple clinician KOLs)

GER's solution:

- Served as Epidemiologist KOL on an expert panel
- Collected and synthesized the research priorities and objectives from all registry stakeholders
- Organized topic areas into discrete survey modules, allowing for phased design, approval, and launch of targeted survey modules over several stages of the project
- Designed multiple survey modules and data collection tools for implementation within the registry framework
- Designed data coding and data analysis plan
- Provided recommendations to improve registry design and implementation
- Deliverables: multiple questionnaires and data collection tools; participation at expert panel meeting; presentations to stakeholders

Case Study #2

Problem:

Our client sought an estimate of the upper-bound prevalence for a genetic disorder that is rare, has a variable phenotype, lacks an ICD code, could be diagnosed by multiple types of healthcare providers, and on which there were few published studies.

GER's solution:

- Served as Epidemiologist KOL on an expert panel
- Performed comprehensive review of published and unpublished research; identified and evaluated all potential data sources and statistical approaches; obtained and analyzed disease registry data; identified sources of under-ascertainment in the available data; and identified the most likely lower-bound prevalence estimate
- Developed a ***data-driven correction factor*** to derive an upper-bound prevalence rate
- Deliverable: comprehensive report detailing methods, data sources, analytic approaches, the solution and rationale, as well as additional considerations for projecting the prevalence of the disease in the future

Case Study #3

Problem:

Our client was undertaking a patient flow modeling project for specific cancer subtypes in multiple ex-US countries. They intended to utilize country-specific data published in CI5 for their top-line incidence data, and desired validation of this approach. They also required data on the distribution of patient characteristics (sex, age, disease stage) by each subtype in each country, to be used as inputs in their model.

GER's solution:

- Evaluated the representativeness of the CI5 data and the validity of the client's analytic approach for each country
- Provided recommendations for making **evidence-based adjustments** to the data or utilizing alternative data sources that would provide **better precision and validity**
- Performed a systematic literature review for each country/region to provide country-specific distributions of the key variables required for inputs into the patient flow model.
- Deliverables: comprehensive report for each country detailing methods, data sources, analytic approaches, the solution and rationale, as well as additional considerations; provided instruction to the client on unfamiliar methodological concepts.

Case Study #4

Problem:

A client was interested in the current prevalence of a specific neurological disease in the US, but found only outdated and conflicting prevalence information in an incidence/prevalence database provided by another vendor (not GER).

GER's solution:

- Performed a systematic literature review, which included constructing prevalence rates by filling in data gaps using epidemiological and statistical techniques and by utilizing publicly available data such as historical Census data
- Adjusted prevalence estimates from published reports by a **correction factor derived from capture-recapture methods** to account for ascertainment bias
- Performed a meta-analysis to determine a summary prevalence estimate and most likely (95% confidence) range for the current US prevalence of the disease.
- Obtained access to the largest disease registry in the US to establish a minimum prevalence for validation purposes
- Analyzed historical trends in the prevalence and mortality to present a detailed discussion of factors capable of influencing future trends in disease prevalence
- Received authorization to communicate findings to the National Institute for Neurological Disorders and Stroke (NINDS), resulting in a change to the prevalence estimate used in their lay audience publications
- Deliverable: comprehensive report and client educational session

**For further information,
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