

Methods for Estimating Incidence and Prevalence for Orphan Medical Device Designation Using Electronic Health Records: Use Case Transcatheter Pulmonary Valve Implementation

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FACTS & FIGURES

Start date:	1 st May 2025
End date:	30 th April 2030
IHI funding:	€13,298,375
Industry contribution:	€13,551,747
Other:	€590,000
Total Cost:	€27,440,122
Project website:	https://ihi-greg.eu/
Social media:	https://youtube.com/@IHIGREG

CHALLENGE

In the European Union, orphan medical device designation requires evidence that a device is intended for a disease or condition affecting no more than 12,000 individuals per year, together with evidence of unmet need or expected clinical benefit over available alternatives. For many rare device indications, however, published literature and routine statistics are insufficient to determine whether this threshold is met.

This project evaluates how routinely collected health data sources can support orphan device designation by identifying and quantifying relevant patient populations.

APPROACH & METHODOLOGY

We used transcatheter or percutaneous pulmonary valve implantation as a case study to develop a reproducible approach to estimating rare device-relevant populations using routinely collected healthcare data from the United Kingdom (Clinical Practice Research Datalink GP Online Database, CPRD-GOLD) and Aragon, Spain (BIGAN).

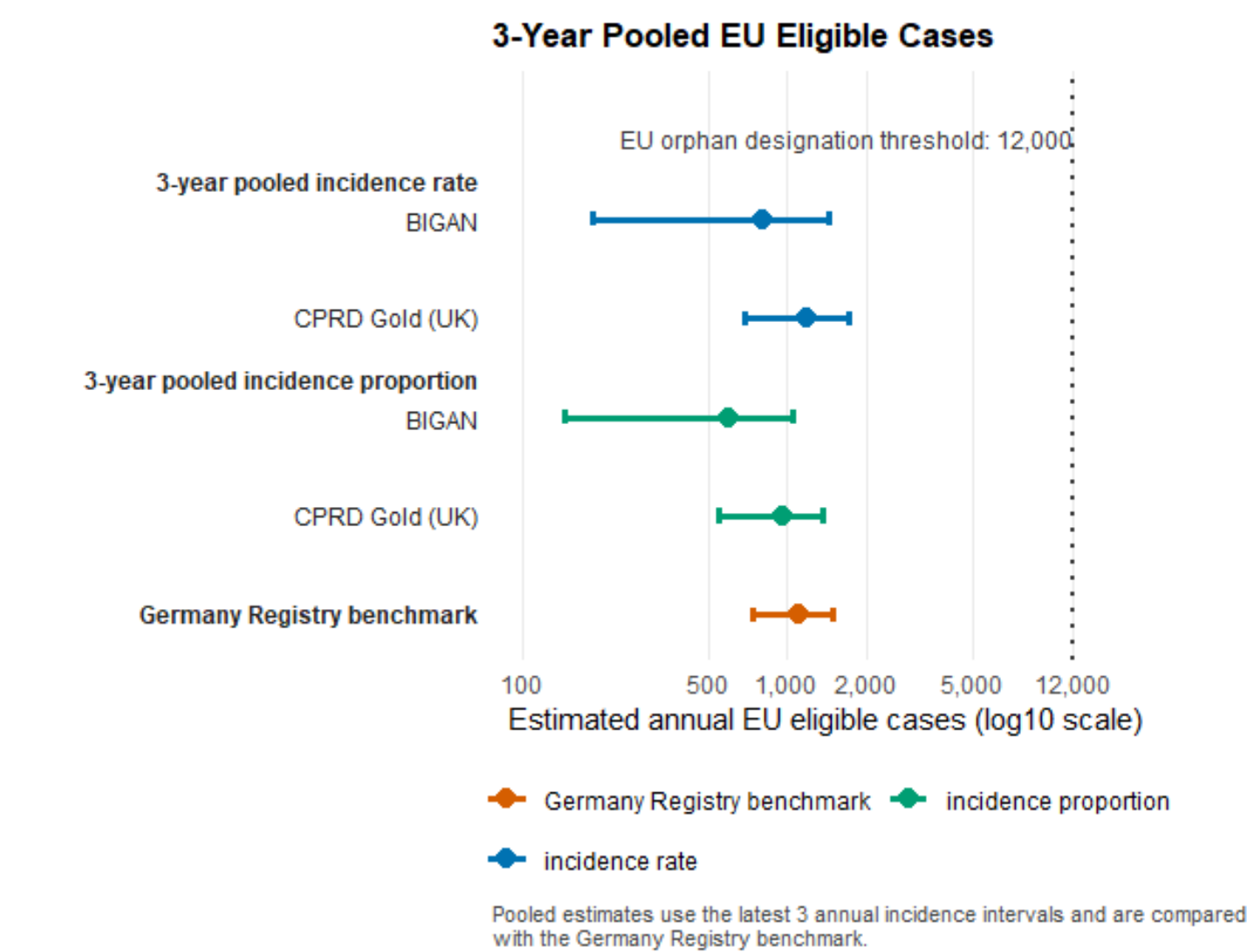
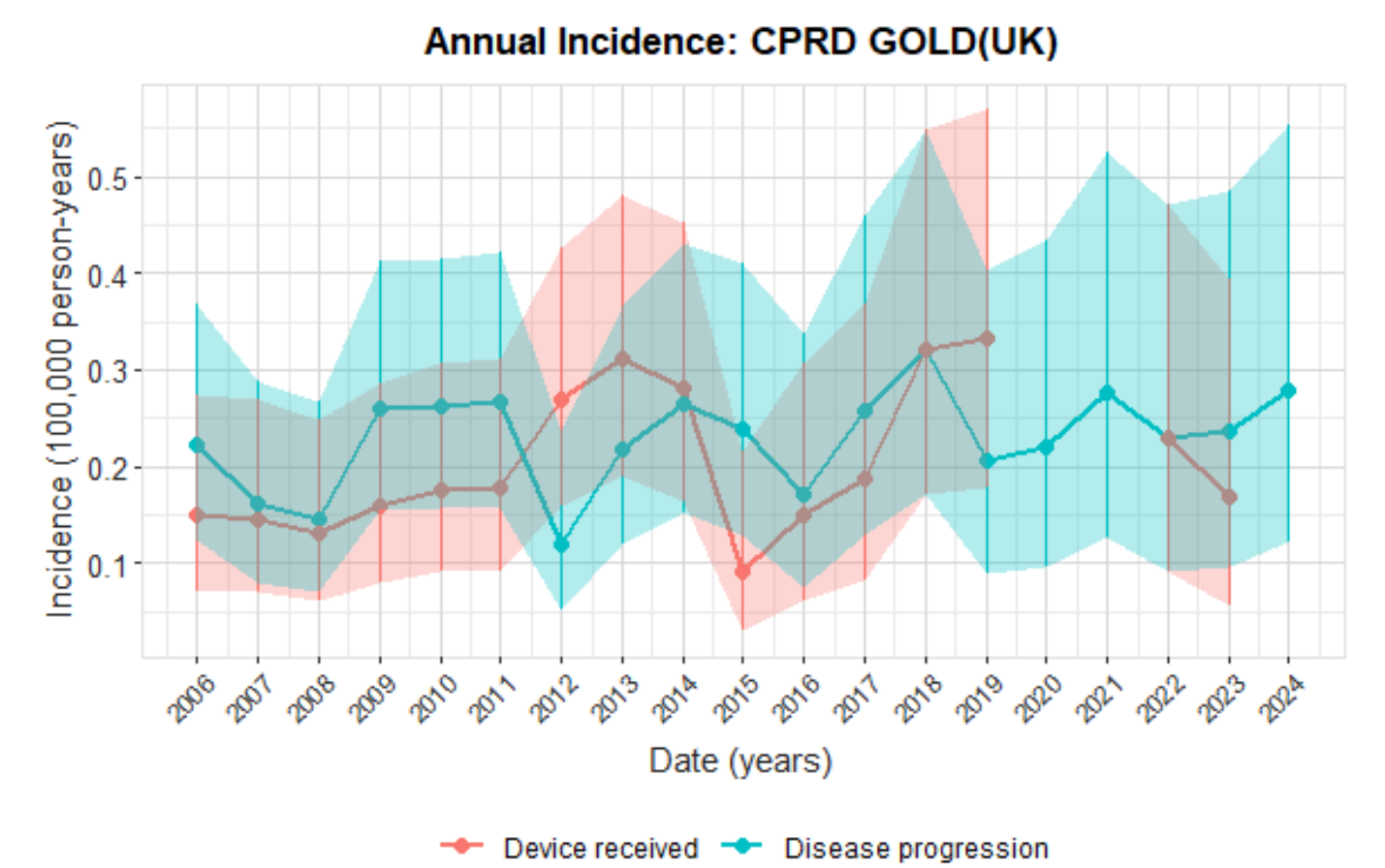
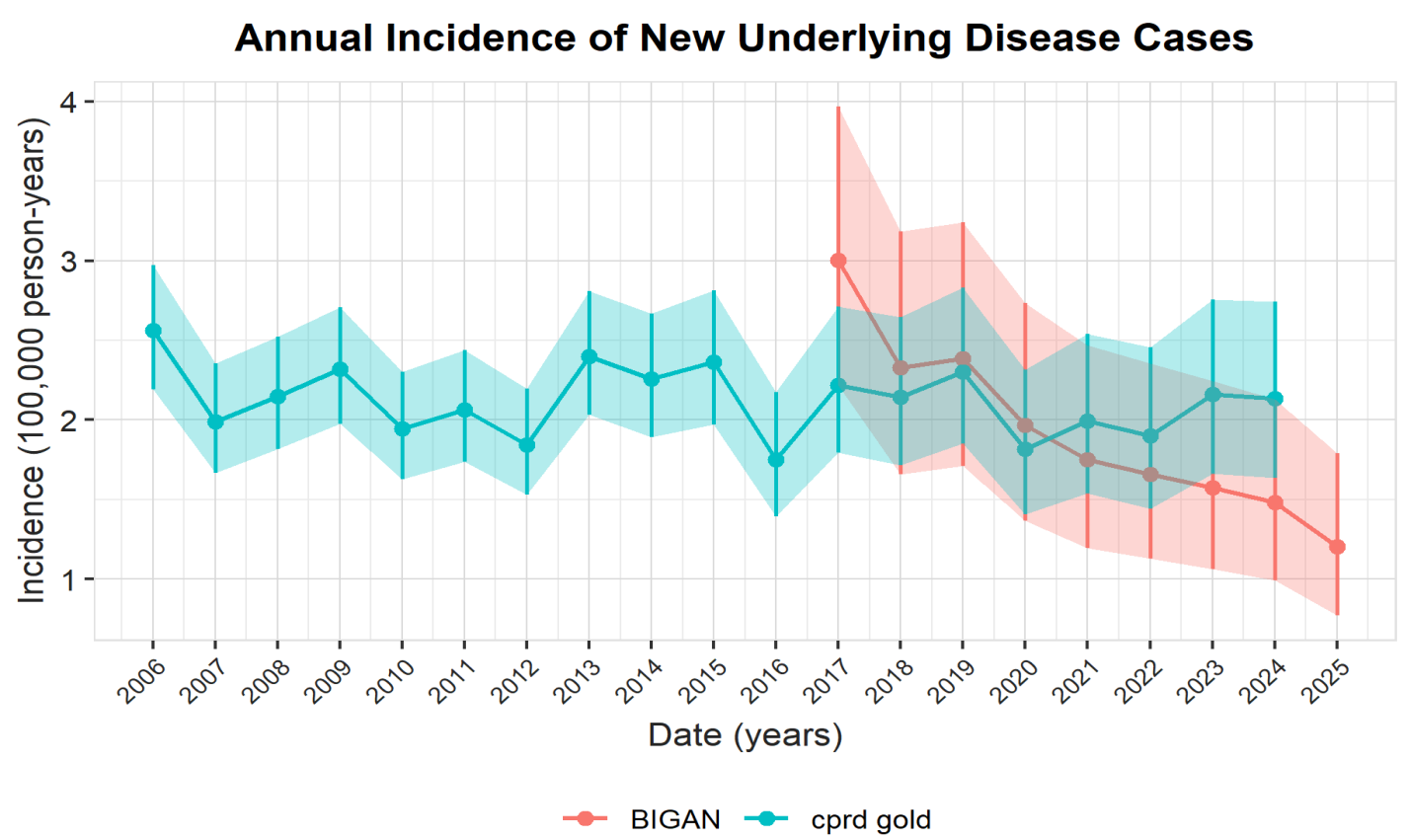
We identified patients with underlying congenital heart disease, disease progression indicating potential device eligibility, and pulmonary valve replacement procedures. We estimated incidence, standardised by age and sex, and extrapolated to the European population. Sensitivity analyses tested alternative population definitions and incidence measures, and we compared results with published and registry-based evidence to assess robustness.



CAREER IMPACT

Being part of this collaboration has strengthened my skills in multidisciplinary working, cross-sector communication, and translating methodological research into evidence that can support regulatory decision-making. It has also broadened my understanding of how academic research can interact with industry and regulatory priorities.

RESULTS



VALUE OF IHI COLLABORATION

This project brought together academic researchers, clinical experts, regulatory perspectives and pharmaceutical-industry partners. This enabled cross-disciplinary collaboration that would have been difficult to establish within a single organisation. The project combined expertise from different sectors to ensure that the work was both scientifically robust and relevant to future orphan medical device designation in Europe.

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