

Using RWD to support orphan drug designation (ODD) application

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To facilitate the development of medicines for rare disease, an application for orphan drug designation was submitted to regulatory bodies. To demonstrate the rarity of the disease, prevalence and incidence estimates to support the application were calculated using a combination of real-world data (RWD) and literature.

Analyses were conducted using the RWD in UK, France, Japan and United States. Within the United States, data from MarketScan CCAE/MDCR, Optum DOD Claims and IQVIA PharMetrics Plus were used. For Japan, JMDC data was used. For UK and France, data from CPRD Gold and IQVIA France were used, respectively.

The prevalence and incidence estimates were comparable across countries and showed the rarity of the disease meets the prevalence criteria for orphan drug designation in both the US and EU. Existing literatures were compared against the estimate produced from RWD to ensure external validity.



Literature Review

Review published prevalence/incidence estimates. Published estimates are older and may not reflect recent trends. RWD can be used to update estimates from the literature and can provide more comprehensive results by specified subgroups.

UK

CPRD-Gold database

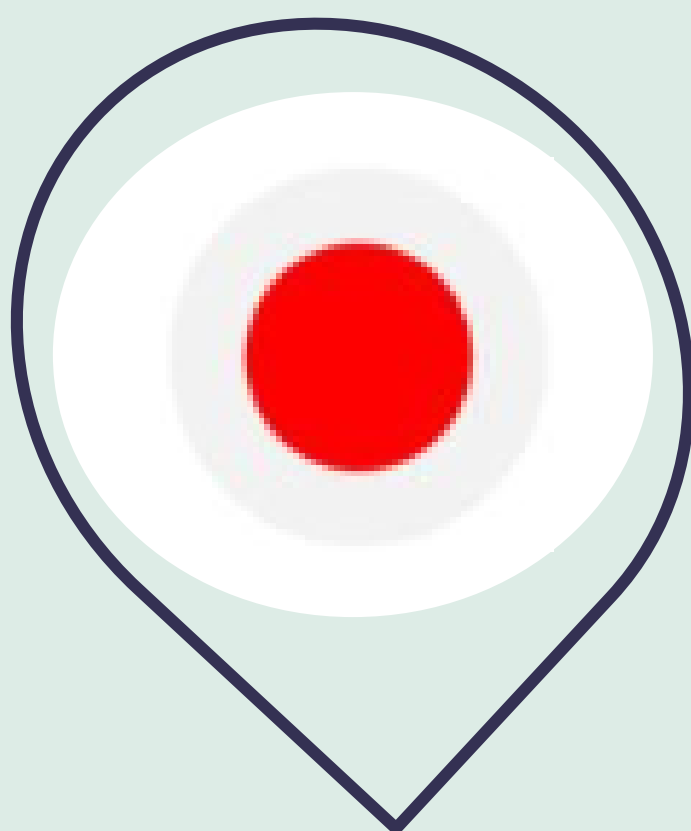
Anonymized patient data from a network of GP practices across the UK



Japan

JMDC Claims Database

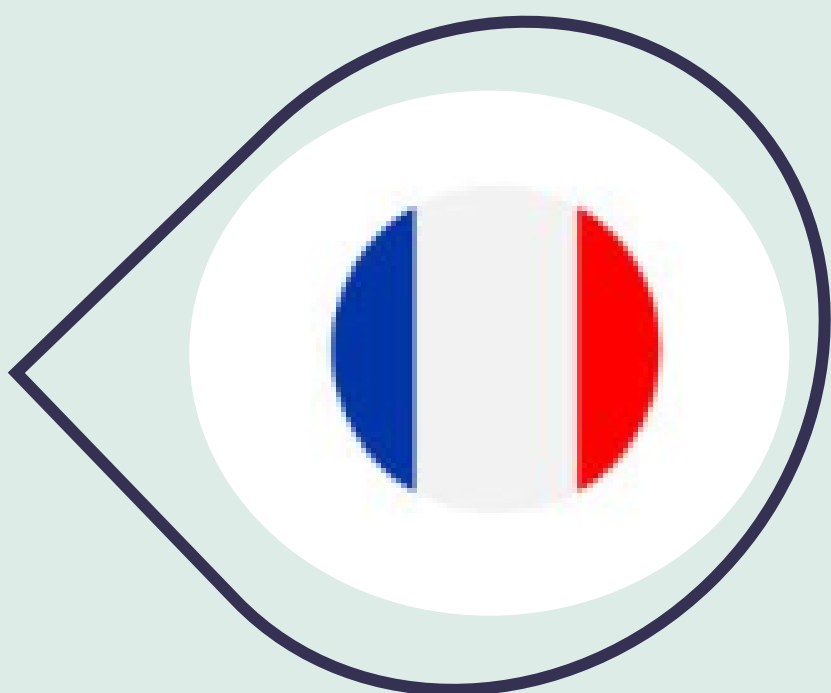
Inpatient, outpatient, dispensing, and medical examination data received from multiple health insurance associations since 2005



France

IQVIA France Disease Analyzer OMOP

Anonymized outpatient records collected from Patient Management software used by GPs and selected specialists during an office visit to document patients' clinical records.



Diagnosis code list

Use to identify patients with diseases of interest. Collaborate with medical expert to develop code list that's best represents the patient population.

Prevalence Denominator

All patients in the database who had adequate enrollment/activity during the study period and met age criteria

Prevalence Numerator

Among the denominator, patients with 1+ record with diagnosis code of interest during the study period. Analysis with 2+ records were also performed.

Incidence

Excluding patients with diagnosis in the prior year from the numerator and denominator

Reporting

Average annual prevalence/incidence per 10,000 person was reported.

US

MarketScan CCAE/MDCR

Commercial - healthcare experience of privately insured individuals sourced from large self-insured employers

Medicare - linked longitudinal claims data from Medicare Advantage and Medicare Supplemental health insurance plans



Optum DOD Claims

Closed system of administrative claims, including patient enrollment; physician, facility, and pharmacy claims; and lab results from the largest health insurer in the U.S.

IQVIA PharMetrics Plus

A closed database of adjudicated medical and pharmacy claims since 2006, data contributors are largely commercial health plans and are representative of the commercially insured population for patients under 65 years of age

ODD application

Contact information

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