RWD50

Data Linkage to Support Real World Evidence in Rare Disease

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Introduction

- There is limited real-world evidence (RWE) on healthcare resource utilisation (HCRU) in rare diseases, particularly when stratified by disease severity, making regulatory and reimbursement submissions challenging.[1]
- A pharmaceutical company commissioned the assessment of secondary care HCRU and costs for a rare progressive disease.

Objectives

- To assess feasibility in the generation of timely, high-quality RWE in rare diseases and estimate monthly secondary care HCRU and costs (i.e., three years pre-diagnosis and five years post-diagnosis), stratified by World Health Organisation (WHO) Functional Class (FC), risk status, and treatment regimen[2] within the National Health Service (NHS) England.

Method

- A retrospective, non-interventional cohort study was conducted using pseudonymised patient-level data from a national rare disease registry linked to NHS Hospital Episode Statistics (HES).[3]
- Research Ethics Committee (REC), Health Research Authority (HRA), and Data Access Request Service (DARS) approvals were obtained before linkage[4] of HES records of additional rare disease patients over an expanded time period.
- NHS Digital linkage rules were applied to join registry and HES records using pseudonymised identifiers.[3,5]
- Three-years pre-diagnosis and 5-years post-diagnosis HCRU were analysed and calculated unit costs using the NHS National Tariff Payment System.[6]

Results

- **Approach 1**: Developed a linkage methodology to join the National Institute of Health Research data with HES records to capture real-world patient secondary care activities without altering current diagnostic or treatment journeys to generate interim results (Figure 2 and 3).[3] Average monthly HCRU costs were calculated pre- and post diagnosis.[2]
- Distinct patterns of healthcare utilisation were observed before and after diagnosis. Interim findings supported a health technology assessment submission to the National Institute for Health and Care Excellence of a new therapy in a rare condition.
- **Approach 2**: Linked NHS numbers from National Condition Registry (NCR) data with HES activity allowed the generation of enhanced final results (data not presented).
- Analysis competed 60% faster than the typical timelines (i.e., 10 months vs. 24 months for similar RWE studies).[7]

Conclusion

This study demonstrates how linked national datasets can be used to generate timely, high-quality RWE in rare diseases. Our approach enabled stratified cost estimation to inform regulatory decision-making and offers a scalable framework to address evidence gaps in complex conditions.[1] The analysis was leveraged by the client to support their submission to obtain NICE approval for a new medicine to treat this rare disease.[8]

Figure 1: Diagnostic methodology used in the academic literature over our HES datasets to estimate the healthcare resource utilisation for rare disease patients

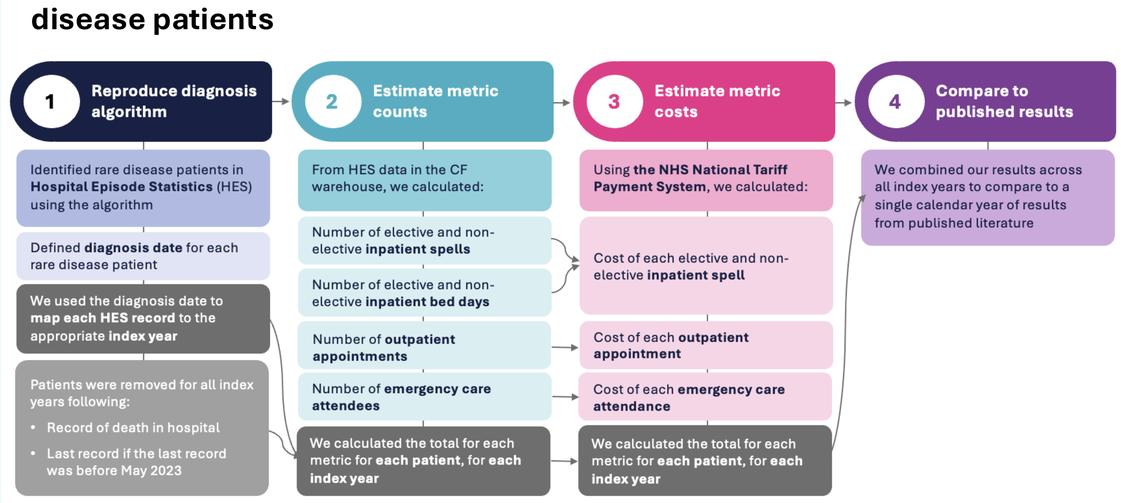
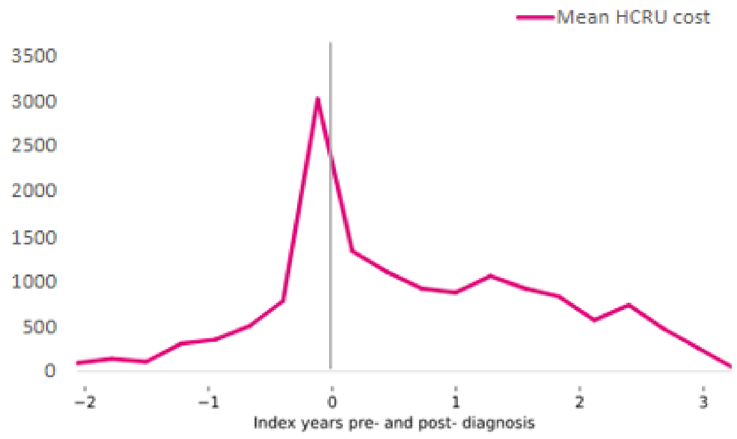


Figure 2: Mean cost of healthcare resource utilisation

Mean cause of all-cause HCRU per rare disease patient (£) per quarter

Apr. 2020 – Oct. 2023, stratified by death within study period

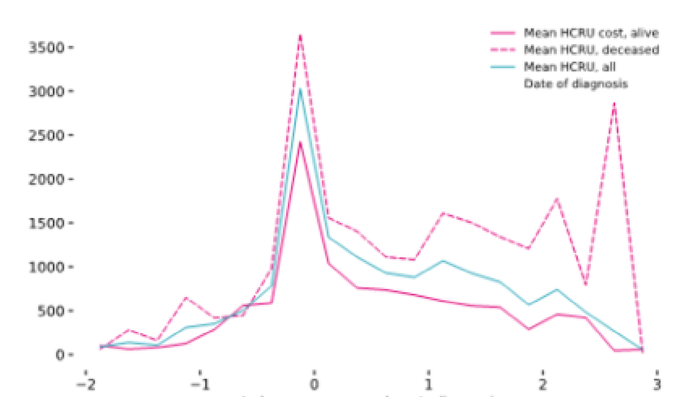


- The overall cost of all-cause HCRU for rare disease patients peaks in the 3 months preceding a rare disease diagnosis, and the 6 months following
- The cost of HCRU declines somewhat steadily, almost reaching zero by 3 years after diagnosis

Figure 3: Costs of HCRU is higher for patients who died during of the study prior, a proxy for higher disease severity, than for patients still alive throughout the study period

Mean cost of all-cause HCRU per rare disease patient per quarter (£)

Index years pre- and post- diagnosis



- Patients who died over time period, here used as a proxy for disease severity, cost more than those alive throughout the duration of the end of the study period
- This supports the hypothesis that patients with more acute rare disease have a greater HCRU

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Experts in health

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