Navigating Real-World Data Collection From Early Access Programs in the UK: An Underutilized Opportunity

RWD30

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INTRODUCTION

Regulatory authorities and payers expect a range of data sources to inform decision-making. Accordingly, adoption of real-world data (RWD) across the product lifecycle continues to accelerate. Advances in data availability and analytical methods increase this potential for real-world evidence (RWE) to inform clinical prescribing and broader market access.

Well-designed clinical trials are the cornerstone of explanatory evidence-based medicine. Conventional use cases involving RWD might describe therapy area standards of care (including comparator use), resource utilization, or natural history of conditions.[1] Pre-approval, opportunities to gather early real-world insights on innovative treatments are underutilized.

Early Access Programs (EAP) offer a potential lifeline for patients suffering from a serious or life-threatening disease or condition, who have exhausted all available therapeutic options and are ineligible or unable to enter clinical trials.[2] The purpose of EAPs remains treatment, not research. However, sponsors, regulators and payers are progressively open to EAP data collection and utilization.[3] Most examples relate to rare disease, hematology or oncology, where EAPs provide global access for patients with substantial unmet need.

Operating within its specific regulatory structure, the UK Early Access to Medicines Scheme (EAMS) provides a platform for suitable patients to access innovative treatments 12 to 18 months pre-marketing authorization.[4] Outside the EAMS process, significant EAP provision in the UK consists of *physician-initiated* (unsolicited) treatment requests for individual patient supply.

OBJECTIVE

EAMS creates a supporting framework for the systematic collection of optional RWD. It includes the opportunity for early engagement between the sponsor and National Institute for Health and Care Excellence (NICE) to shape the data collection plans to support health technology appraisal. Additional benefits include faster adoption following the EAMS period and reimbursement decision.[4]

For physician-initiated treatment requests, data collection may be an afterthought or overlooked entirely. The lack of a clear framework, perceived complexity and stretched local resources further deter advancement in data collection from these heterogeneous populations. Identifying a compliant, low-burden pathway presents a valuable opportunity to gather country-level data.

Supported by the conducive UK NHS research ecosystem, this case outlines how an independent observational study was operationalized to efficiently capture valuable insights relating to treatment experience for requests made for individual patient supply.

Table 1: EAMS & Individual Patient Data Collection Key Features

	Early Access to Medicines Scheme RWD	Individual Patient Supply Requests RWD
Data Collection Pathway	Supporting framework, defined opportunity for broad data collection	No RWD framework, opportunity to utilize NHS research ecosystem
Ethics & Approval Requirements	Streamlined within the EAMS framework; specific EAMS-RWD provision	Usual ethics-HRA review, proportionate to the proposed data collection
Resource Support Infrastructure	Early engagement with key partners, including MHRA, NICE & NHS England	NIHR Study Support Service, RDNs & Costs/contract support (NCVR)
Insights Utility	Yields versatile datasets, Market Access capability	Answers a defined research question

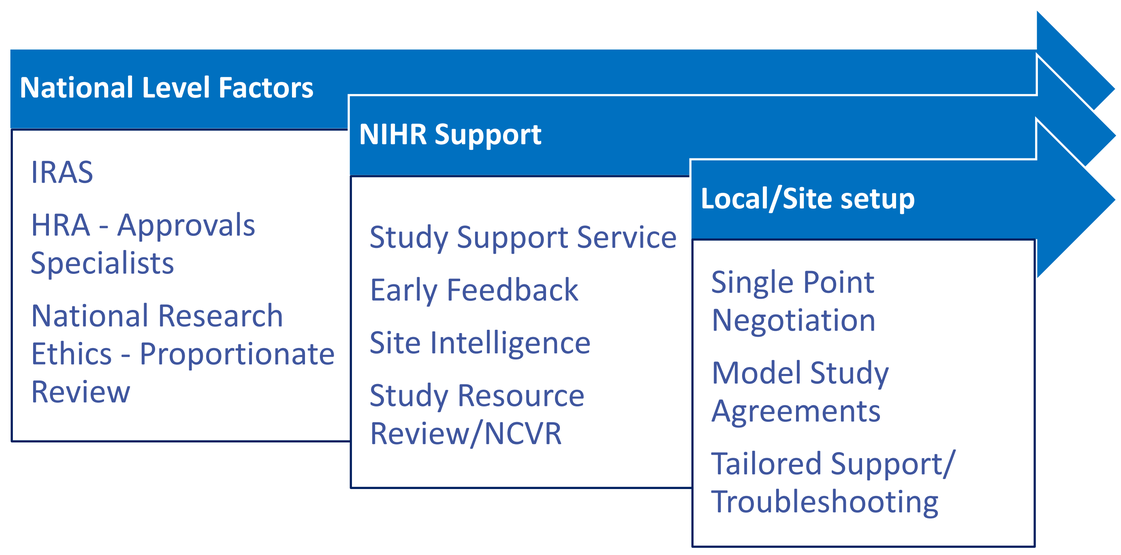
METHODS

A retrospective observational study in a rare, hereditary disease was designed to operationalize data collection on UK patients accessing EAP treatment via a non-EAMS, individual patient arrangement.

Design was refined utilizing Health Research Authority (HRA) feedback and National Institute for Health and Care Research (NIHR) Study Support Services – guided by IRAS (Integrated Research Application System) for appropriate study classification. The National Contract Value Review (NCVR) and model agreements provided a standardized site negotiation approach, contributing to an efficient study setup.

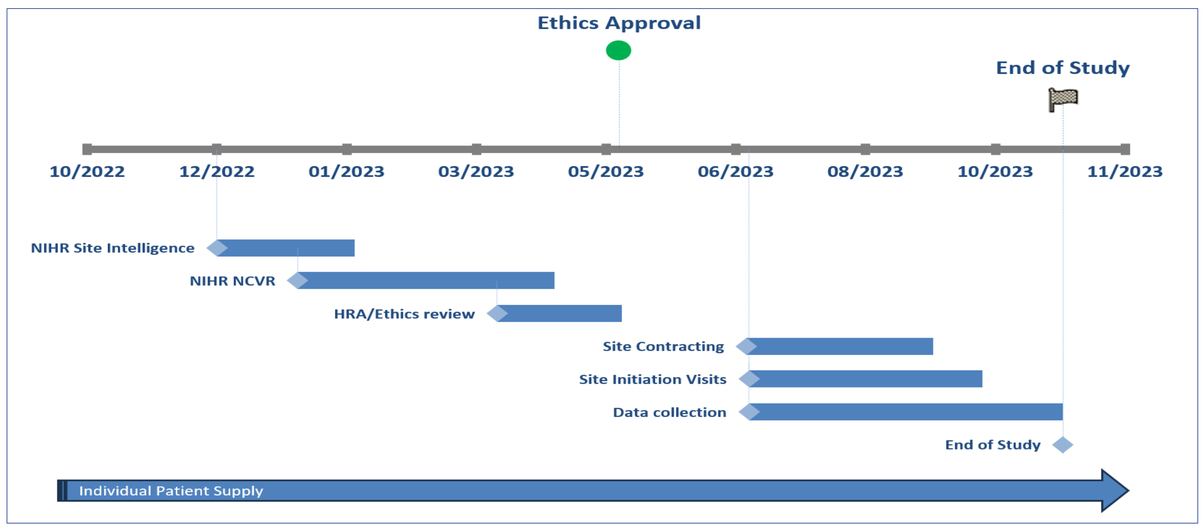
Following collaborative review, Research Ethics Committee (REC) gave their positive opinion. Research Delivery Networks (RDN) liaised with sites, helping to assemble site teams and troubleshooting to ensure study performance remained on track.

Figure 1: Factors supporting study design & setup in the UK



The study went live in June 2023, completing by October 2023. Provided with an opportunity to opt out, all 142 EAP patients participated in the data collection. This reflected patients' trust and confidence in their care teams. Baseline clinical characteristics and longitudinal data on treatment response were collected from medical records, including, where available, patient-reported outcome measures. Findings have been shared with the scientific community at medical congresses, with a publication plan in place.

Figure 2: Study timeline utilizing NHS research environment



CONCLUSION

- The EAMS framework for data collection provides multiple benefits where timelines and resources permit.
- Where the supply route is through individual physician-initiated requests, opportunity exists to broaden insights gained from EAPs.
- The UK NHS research ecosystem supports data collection activities, including Proportionate Review, NCVR, NIHR and RDNs.
- Although less common, an independent observational study approach to gathering insights from Individual Patient Supply Requests was effective, well-executed and accepted by EAP participants.

References

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