Evidera | PPD

CASE STUDY

Enhancing Oncology Market Access with a Real-World Evidence EAMS Add-on

200+ IIIb/IV, XAP, registries, and large observational studies in oncology

Evidera's team of researchers understand the unique challenges associated with oncology product development and market access

EXPERIENCE WITH **100+ DATA SOURCES** ACROSS **20+ COUNTRIES**

BACKGROUND

A large pharmaceutical client needed assistance conceptualizing and implementing real-world data (RWD) collection in an Early Access to Medicines Scheme (EAMS) for an oncology therapy. The EAMS program in the UK is governed by the Medicines and Healthcare products Regulatory Agency (MHRA) and provides access to medicines that address unmet medical need but do not yet have a marketing authorization. The UK EAMS is one of a few programs of its kind to have issued guidelines for systematic collection of RWD. The MHRA guidance suggests that EAMS data collection must include patient demographics, disease characteristics, dose and duration of treatment, comorbidities, concomitant medications, adverse events, and other factors known to affect efficacy or importance. However, incorporation of additional data collection in EAMS, including long-term outcomes and patient-reported outcomes, must be agreed upon on a case-by-case basis. At the time of the client request, there was no framework for how to collect and incorporate these RWD for health technology appraisals.

KEY TAKEAWAYS

- To our knowledge, this is the first UK EAMS to successfully implement an RWD collection framework that included patient questionnaires and longer-term medical record data collection as an optional add-on
- High rates of consent for completion of quality of life questionnaires, as well as completion of baseline and follow-up data by two-thirds of patients
- Observational research that provided early insight into a real-world population with unmet need, which could be utilized in reimbursement assessment

APPROACH

Our real-world evidence experts worked to develop an RWD collection framework to be included as an add-on to the oncology EAMS. To do this, our team evaluated the existing EAMS guidance and the ethics and regulatory landscape to define procedures for observational study design, setup, and conduct. Out of this research the framework was developed which included:

- A data collection approach that minimized administrative burden and did not hinder or deter drug access
- A flexible approach to incorporating RWD so that patients and sites felt comfortable taking part to get access to the medication; participation in the RWD collection add-on was encouraged, but not required
- Ability for patients to report on their quality of life during and beyond treatment

Early stakeholder identification and engagement in setup of the observational research was essential to implementation of the add-on. Additionally, ethics approval for the study was sought in parallel to the MHRA EAMS approvals. This approach, along with the detailed framework, allowed for the collection of patient-reported outcomes and long-term medical record data to support market access and provide additional evidence for health technology appraisals.

1st successful EAMS study in the UK

RESULTS

More than 85% of patients who received treatment via the EAMS consented to longer term medical chart reviews and of these, over 75% of patients also consented to quality of life questionnaire completion. Additionally, nearly two-thirds of patients who consented to questionnaire completion returned at least one questionnaire. Collection of patient-reported outcomes was completed in March 2019. Interim data on quality of life were shared with NICE for consideration as part of a reimbursement assessment.

IMPACT

- First successful study that built data collection into an EAMS program resulting in faster and more efficient time to data/insight
- To our knowledge, the first UK EAMS to include patient questionnaires and longer-term medical record data collection as an optional add-on
- Allowed for real-world evidence generation that initiates prior to and continues after marketing authorization for the main trial outcomes in a non-trial population (24 weeks quality of life, 12 months progression-free and overall survival)
- More cost-effective implementation versus bespoke data collection

RWE can initiate **prior to and after** marketing authorization

Learn more about how we can design and execute studies to uncover real-world insights.

Our team of researchers offers leadership in the design and execution of studies to meet payer and regulator evidence requirements. We offer diverse scientific methods and high-quality project and data management expertise. Methods include analytics of secondary data sources, bespoke multi-national data collection, hybrid studies, and pragmatic studies.

For more more information, contact us at info@evidera.com or visit our website at evidera.com/what-we-do/real-world-evidence/.