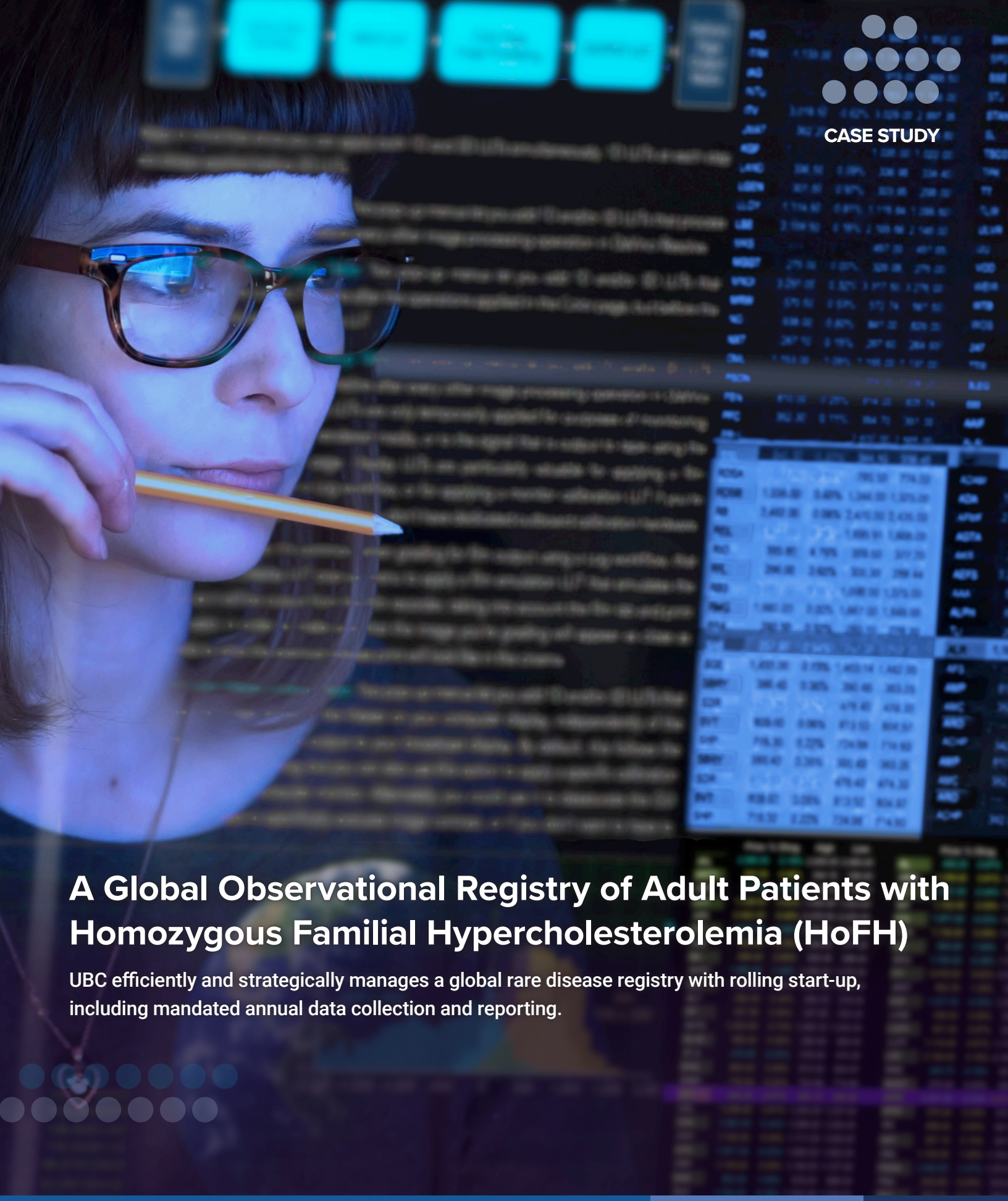

CASE STUDY

A Global Observational Registry of Adult Patients with Homozygous Familial Hypercholesterolemia (HoFH)

UBC efficiently and strategically manages a global rare disease registry with rolling start-up, including mandated annual data collection and reporting.

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SITUATION

For more than 6 years, UBC has been conducting a long-term global product exposure registry for patients with homozygous familial hypercholesterolemia (HoFH). Our responsibilities include registry design, program management, site regulatory approval (central and local), site management, data collection and management, statistical analysis, and medical writing with annual reporting to regulatory agencies. Countries are selected and initiated into the program based on commercial availability of the drug. The registry currently involves more than 75 sites in 11 countries. The regulators have specified that the registry remain open indefinitely, rather than agreeing to a finite study period based on length of patient follow-up and sample size. In order to obtain a sufficient sample size, prevalent cases are included which necessitates retrospective data collection.

CHALLENGES

Delays with the reimbursement process in several countries had a measurable impact on site selection, overall recruitment, site engagement, and costs. Interested sites often had to be put on hold. Data collection is based on medical record review at specified intervals, rather than at each patient's usual care visits. Managing the annual interim analysis and reporting to FDA/EMA necessitates that each site complete all data abstraction in a timely manner. Given the rare disease indication, many sites treat only a few patients; therefore, the study results are of great interest in providing a more robust picture of real world patient treatment and outcomes. However, keeping this study top of mind so that sites are diligent in obtaining information indefinitely requires continual reminders.

SOLUTIONS

UBC addressed these challenges in many ways:

Timing of Site Initiation

- Country-specific study launch plans were created based on estimated drug reimbursement timing
- Sites were informed of expected reimbursement timelines

Medical history and retrospective data collection

- Clear, well-defined guidelines and timelines for retrospective data entry were created
- A plan to aggressively engage sites to ensure current and complete data entry on all specified data points
- Sites were offered support staff to provide on-site assistance with chart abstraction and data entry

Sources of case identification

- Collaborations were established with other functional areas such as the REMS team and the drug coordinating center, and with the sponsor to proactively manage and enroll new patients who have been prescribed the drug

Creative site engagement

- Country-specific study-branded Investigator Kits, including all program materials necessary for a site to participate in the registry were developed to reduce site burden
- UBC staff were paired with each site to enable continuity in site contact calls and correspondence, thereby developing positive relationships with sites

RESULTS

UBC's strong, consistent support services proactively engage sites by developing robust CRA site management and establishing strong relationships with the Sponsor Medical Science Liaisons. This in turn has ensured, year after year, that study milestones are met to support the mandated FDA/EMA annual analyses.