CASE STUDY

Overcoming lack of robust data in rare and variable condition

The challenge

Data limitations in rare diseases can restrict the ability to quantify disease burden and understand the experience of patients and caregivers. Our client sought to conduct a large-scale, international study to ensure that the patient and caregiver lived-experience is appropriately captured.

Our approach

- Formation and management of an expert steering group (including clinicians and patient groups).
- Design, development and fielding of a large-scale, global survey (2,988 patients and caregivers): https://oife.org/what-we-do/the-impact-survey/
- Analysis and publication of data (including multiple peer-reviewed manuscripts and conference presentations).
- Ongoing management of dataset – including managing access requests in line with a data management agreement.

How we made a difference

- Robust evidence – to the standards of regulatory and health technology assessment agencies – which deepens understanding of the burden of disease, natural history, lived experience of patients and caregivers, and associated economic and humanistic outcomes.

WICKENSTONES
PARTNERS IN BUILDING VALUE