CASE STUDY

Expanding rare disease treatment access through a strategic health technology assessment (HTA) narrative

The challenge

Expanding reimbursement to an additional rare disease subpopulation can be particularly challenging when evidence is limited and assessment criteria evolve mid-process. Our client needed a rapid NICE submission demonstrating clear value and equity for patients, for a product already reimbursed elsewhere in Europe.

Our approach

- Aligned with the client team through targeted workshops and small-group discussions to clarify priorities and evidence gaps.
- Developed a focused, subpopulation-specific HTA narrative that framed limited data within a broader story of unmet need, inequity, and meaningful quality-of-life for patients and carers.
- Reinforced the value case using European registry data, real-world outcomes, and compelling clinician and patient case studies — including sibling examples illustrating inequity in access.

How we made a difference

- We delivered a compelling NICE submission that translated limited and evolving evidence into a clear, patient-centred value story, demonstrating equity and real-world value while maintaining confidence and alignment under shifting assessment requirements.

WICKENSTONES
PARTNERS IN BUILDING VALUE