Winning Entry:

Patient Evidence Collection to Support Access to Treatment

Winners’ Statement
“We are honoured to receive this award in recognition of our work to support children with a severe, ultra-rare disease in gaining access to a treatment that brings hope to them and their families.”

Executive Summary
When NICE evaluated the first treatment for metachromatic leukodystrophy, an ultra-rare disease, the patient community needed to collect robust evidence that demonstrated the true impacts of disease and its consequences on the quality of life of those affected and their families.

We conducted a collaborative study involving patients, parents and caregivers, patient organisations and clinical specialists to develop an evidence package that provided context to the clinical outcomes data under consideration and ensured the patient voice was comprehensively represented. NICE made a positive recommendation and gene therapy is now available for patients with early onset disease.