Cell and Gene Therapies or Treatments for Rare Diseases

Three Key Drivers (Pain Points) to Prioritize in Your Pricing and Reimbursement Strategy for the UK at Launch

Comparative Effectiveness: Alternative Ways to Demonstrate Value vs SOC

Begin early in the clinical development process to collaborate with regulatory and payer stakeholders to plan external controls for non-RCT trials

Cost Effectiveness: Alternative CE Models and Improving Certainty

Explore alternative models that can capture novel value elements beyond QALYs (GRACE, EVL, evLYG, and HYT models)

Enhance the reliability of survival extrapolation (ML, statistical modelling, RWE and sensitivity analyses)

Managed Access Contracts: Continue to Innovate with P&R Models

Most ATMPs have been granted approval by NICE, usually with some form of managed access agreement due to substantial upfront costs and uncertain future benefits

Innovate P&R models (AI, other industry models, private equity)

Key issues noted in NICE appraisals related to limited data due to small, single arm trials; limited follow up, immature survival data; uncertain curative potential and quality of life values lacking.