

Business Situation

- A healthcare equity investment firm wanted to assess KOL & payer receptivity to new emerging therapies for Spinal Muscular Atrophy (SMA); The client had rights to global sales for a in-line brand for SMA and wanted to understand the future market landscape, specifically the impact of gene therapy

Approach & Methodology

- Development of all qualitative research materials, recruitment, and fielding, as well as summation of all key stakeholder findings and create a supporting epidemiology-based forecast
- Specific KOL objectives included current drivers and barriers to treatment, perspectives on new SMA therapies, particularly the impact of gene therapy, the evolution of treatment algorithm as new treatments become available and adoption of new therapies
- Specific payer objectives included management of new gene therapies (e.g. restrictions, reimbursement eligibility), willingness to pay, and contracting expectations for SMA treatments

Deliverables & Business Outcomes

- Designed & developed an intuitive epidemiology-based forecast model with robust scenarios to illustrate the potential impact of new product launches on the in-line SMA brand of interest

Product A Perspectives & Experience
KOL Experience

KOLs perceived Product A to be an effective treatment for SMA & appropriate for Type 1, 2 and 3 patients

KOL Experience with Product A

- All KOLs currently had patients treated with Product A, and perceived it to be an effective treatment for SMA
- KOLs noted that it took time for their institutions to adopt the product (approximately 6 months to 1 year)
- All mentioned the significant insurance hurdles with PA requirements
 - Initial authorization was perceived to be more challenging than subsequent reauthorizations

Product A Eligible Patients

- KOLs perceived Product A to be appropriate for Type 1, 2 and 3 SMA
- 1 KOL stated they would not recommend Product A to patients who have had a brain injury or if they did not have meaningful eye movement
- 1 KOL stated they felt Product A was most appropriate for patients under the age of 18 and perceived little benefit in older Type 3 patients

