

## The Value of Gene Therapy in Spinal Muscular Atrophy

### Situation:

- In the devastating childhood disease, spinal muscular atrophy (SMA), current treatments offered modest symptomatic benefit in a minority of sufferers
- Although the client was developing a potentially curative gene therapy, they needed to translate the clinical benefit into economic terms to engage payers and health technology organizations

---

### Task: Economic modeling and Institute for Clinical and Economic Review (ICER) support

- Precision worked with the client to develop a cost-effectiveness and budget impact model for a gene therapy in SMA that overcame unique challenges of modeling an early childhood neuromuscular genetic disorder
- The resulting model overcame unique challenges of modeling an early childhood and fatal genetic disorder, including
  - Obtaining reliable utility scores
  - Deriving costs for diverse services such as in-home ventilation
  - Modeling clinical data over a lifetime horizon
  - Need for comparative data as a result of single-arm clinical trial

---

### Actions: Comparative effectiveness research for commercial success

Using the cost-effectiveness and budget impact model results, Precision supported the client's commercialization efforts with HTA bodies around the world, including ICER and NICE

- Evidence synthesis activities (systematic literature review and indirect treatment comparison) for model inputs and HTA submissions
- CE model adapted for 15 other markets

---

### Results: Thought leadership in gene therapy

- Precision's cost-effectiveness model successfully changed the prevailing mindset on how SMA progression is characterized and demonstrated the long-term value of SMA gene therapy over standard of care
- The result of the modeling helped overcome potential resistance to high price for this onetime, potentially curative therapy (e.g. ICER and Japanese Health Insurance Scheme)

“

Precision's economic model addressed unique challenges in assessing the value of a gene therapy for an early childhood neuromuscular genetic disorder.

”

---

PRECISION  
**ADVANCE** the cell & gene therapy collective™

PRECISION  
for medicine

PROJECT FARMA

PRECISION  
value & health

PRECISION ADVANCE, a collection of interconnected services and complementary teams, uniquely focuses on the complexities of clinical, regulatory, manufacturing, and commercial needs to successfully bring a cell or gene therapies to market.

---

To learn more about Precision ADVANCE,  
visit [www.precisionmedicinegrp.com/advance](http://www.precisionmedicinegrp.com/advance).