

# KEY ISSUES WITH ICER'S REPORT ON SPINAL MUSCULAR ATROPHY



The Institute for Clinical and Economic Review (ICER) assessed two treatments for spinal muscular atrophy (SMA), a rare and deadly genetic condition impacting 1 in 11,000 newborns each year. The final report was disappointing in a number of ways:

## Ignores the Strain SMA Puts on Families

ICER determined that caregiver burden was not within the scope of their assessment of SMA treatment. ICER omitted caregiver burden from its final value calculation in spite of numerous calls from stakeholders to include it. One comment letter to ICER from an SMA parent emphasized the burden of missing work for doctor's appointments and to support her child at school.

## Disregards the Spectrum of Patient Experiences

ICER's methods put patients into three buckets (1) sitting and walking, (2) need for permanent ventilation, and (3) death. SMA is a complex illness, and this overly simplistic categorization does not capture the experiences and health gains of all patients.

## Makes Incorrect Assumptions

ICER's model is heavily laden with assumptions regarding longer-term outcomes, like the assumption that repeated lumbar punctures (which are necessary to administer the treatment) are problematic for patients, despite being tolerated during trials.

## Fails to Listen to the SMA Community

ICER only gives credit to treatments if patients achieve certain milestones, like sitting or walking. This contradicts the recognition of both patients and the FDA, who value any gain in abilities that allow independence in daily living. As one parent noted to ICER, "until you live with SMA, you do not realize how every little bit of strength... improves quality of life."

**"Improvements in mobility, however small they may be deemed, often represent major improvements in quality of life and the value of these gains cannot be discounted."**

– Muscular Dystrophy Association

**With such consequences for children and their families, it is alarming to see ICER ignore patients and provide such flawed assessments of breakthrough therapies.**