

ICER vs. Infants

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The National Coalition for Infant Health is a collaborative of more than 180 professional, clinical, community health, and family support organizations focused on improving the lives of premature infants through age two and their families. NCfIH's mission is to promote lifelong clinical, health, education, and supportive services needed by premature infants and their families. NCfIH prioritizes safety of this vulnerable population and access to approved therapies.

An incomplete and poorly conceived report from the controversial Institute for Clinical and Economic Review (ICER) has set its sights on a most unbecoming target: infants with a degenerative, life-threatening neurological condition. (1)

Once dubbed “floppy baby syndrome,” spinal muscular atrophy occurs when a genetic mutation disrupts the body’s supply of motor neurons. (2) Affected infants slowly lose muscle tone and movement. They miss milestones like sitting up or rolling over. As

“But several research organizations are investigating the use of human milk for the health of infants and children – from improving the efficiency of vaccines to providing the cellular foundation of new medicines.”

their movement and mobility wane, many are bound to strollers or wheelchairs. Some require mechanical ventilation to breathe and feeding tubes for nutrition as they lose the ability to swallow.

While many rare diseases have no effective treatment, the families of spinal muscular atrophy patients will soon be fortunate enough to have two. One is FDA approved; the other is in clinical trials with FDA review anticipated this spring.

But perhaps two treatment options are too many for ICER.

In its latest evidence report, the group sets out to determine whether the two spinal muscular atrophy treatments are cost-effective. As with many of ICER’s previous reports, the exercise is premature. (3) Neither therapy has long-term data. Moreover, the newer therapy does not yet have a price. However, that does not stop ICER.

ICER concludes that neither the existing therapy, called “nusinersen,” nor a new gene therapy meets ICER’s cost-effectiveness threshold. (4,5) However, ICER also effectively pits the two drugs against each other, suggesting that the new gene therapy could at

least be more cost-effective than existing treatment – if it assumes the price point concocted by ICER.

Comparing the two drugs is hardly apples to apples. Even ICER admits it is “naïve.”

The existing treatment, nusinersen, is a periodic spinal injection that boosts the body’s production of a vital protein. It has treated thousands of patients since first approved in 2017.

AfPA member Safi Shareef, MD, a pediatric neurologist, recalled seeing a baby girl with spinal muscular atrophy who began treatment with nusinersen. “Suddenly, she started to meet her milestones. She was starting to sit up, starting to stand up,” Dr. Shareef recalled, calling the progress “miraculous.”

The newer treatment is a gene therapy, a one-time infusion that replaces the defective gene by inserting an inactivated virus into the body. The data used by ICER is from the drug’s Phase I clinical trial, conducted on 15 patients. Early results show promise, and the possibility of treatment by a single infusion, as opposed to periodic injections, is an attractive possibility.

However, neither the disease nor its treatment is one-size-fits-all. Many families would welcome a single-infusion treatment, Dr. Shareef hypothesized, though others may choose differently. “Families may elect not to do gene therapy if, for example, their child is prone to severe complications from viral illness,” Dr. Shareef explained.

The decision between treatments rightfully belongs to a patient’s family and their health care provider. Families may not have a say in the matter if insurers listen to ICER. In the past, the organization’s findings have influenced both Medicaid and commercial health plan coverage, with CVS Health announcing last year that its plan formularies could exclude drugs that did not meet ICER’s threshold. (6)

Facing a devastating diagnosis, families of infants with spinal muscular atrophy do not need ICER economists streamlining their already sparse treatment options. Moreover, they do not need deeply personal medical decisions made for them based on what is cost-effective for the system.

They need options, and they need access..

References:

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NCFIH National Coalition for Infant Health

Protecting Access for Premature Infants through Age Two

A collaborative of professional, clinical, community health, and family support organizations improving the lives of premature infants and their families through education and advocacy.

National Coalition for Infant Health Values (SANE)

Safety. Premature infants are born vulnerable. Products, treatments and related public policies should prioritize these fragile infants' safety.

Access. Budget-driven health care policies should not preclude premature infants' access to preventative or necessary therapies.

Nutrition. Proper nutrition and full access to health care keep premature infants healthy after discharge from the NICU.

Equality. Prematurity and related vulnerabilities disproportionately impact minority and economically disadvantaged families. Restrictions on care and treatment should not worsen inherent disparities.



The National Coalition for Infant Health advocates for:

- **Access to an exclusive human milk diet** for premature infants
- **Increased emotional support resources** for parents and caregivers suffering from PTSD/PPD
- **Access to RSV preventive treatment** for all premature infants as indicated on the FDA label
- **Clear, science-based nutrition guidelines** for pregnant and breastfeeding mothers
- **Safe, accurate medical devices** and products designed for the special needs of NICU patients

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