SCE 0813 Public Evidence Appendix

Pediatrics

Literature review date: September 13, 2026

Controlling principle. Pediatric cell, gene, cord-blood, MSC, and extracellular-vesicle evidence must remain product-, indication-, age-, developmental-stage-, route-, endpoint-, and follow-up-specific. Evidence from an established hematopoietic or gene-modified therapy cannot be transferred to unrelated investigational products.

Point-by-point evidence framework

Controlling Point	Evidence focus	Interpretive boundary
Cerebral palsy	Cord-blood and cord-tissue products, motor outcomes, neuroinflammatory and repair hypotheses, and rehabilitation context.	Pooled motor-function signals are clinically interesting but heterogeneous; they do not establish class-wide efficacy or replacement of rehabilitation and established multidisciplinary care.
Hypoxic-ischemic injury	Neonatal HIE, therapeutic hypothermia, cord-derived cell adjuncts, survival, and neurodevelopment.	Early favorable neurodevelopmental signals come from small heterogeneous studies; investigational cell products are adjuncts to, not substitutes for, established neonatal care.
Autism claims	Cord-blood and other cellular studies, behavioral and social-response endpoints, immune-selected subgroups, and trial heterogeneity.	Results are mixed. A positive result in a small selected subgroup does not establish general efficacy for autism or justify broad stem-cell treatment claims.
Genetic diseases	Established hematopoietic stem-cell transplantation and licensed gene-modified hematopoietic-cell therapies versus investigational MSC/EV approaches.	Disease-specific established therapies must not be used as evidence for unrelated stromal-cell or EV treatment of genetic disease.
Pediatric consent and assent	Parental permission, developmentally appropriate child assent, risk communication, therapeutic misconception, and vulnerability.	Parental permission does not eliminate the child's interests; assent should be sought when developmentally appropriate, and research uncertainty must be explicit.
Long-term developmental surveillance	Growth, neurologic development, cognition, behavior, endocrine and immune function, delayed adverse effects, quality of life, and independence.	Short follow-up can miss benefits and harms that emerge with maturation; pediatric interventions may require surveillance into adolescence or adulthood.

Evidence-handling rules

Positive, negative, null, mixed, and inconclusive findings are weighted by design quality, directness, product identity, developmental context, and duration of follow-up rather than by whether they favor the intervention.

Mechanistic evidence, development stage, clinical efficacy, safety, regulatory status, guideline position, manufacturing quality, consent and assent, and long-term developmental effects remain separate analytical dimensions.

Established pediatric neurologic, neonatal, genetic, rehabilitation, developmental, behavioral, and multidisciplinary care remains the appropriate comparator where indicated.

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