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Transplantation shown to be highly effective in treating immune deficiency in children

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Babies who are born with severe combined immunodeficiency (SCID) can be successfully treated with a transplant of blood-forming stem cells, according to experts led by Memorial Sloan Kettering's Richard J. O'Reilly, MD, a world-renowned pioneer in the development of transplant protocols. Their review will be published in the July 30 issue of the *New England Journal of Medicine*.

SCID is a group of inherited disorders that cause the immune system to severely malfunction. When this breakdown occurs, babies no longer have the ability to fight off routine infections because their natural, built-in defense system has been damaged. If undiagnosed or left untreated, SCID is almost always fatal within the first year of life.

A review of more than 240 patient cases found transplants to be quite effective, especially when performed early in life. Of those children receiving transplants within three and a half months after birth, 94 percent were alive five years later. The best results, not surprisingly, were seen after transplant from "matched sibling" donors. But among patients who didn't have a matched sibling, overall five-year survival rates were quite high—77 to 93 percent—if they were transplanted in the first three and a half months of life.

"This confirms that transplants for SCID work well in very young children, but it also shows that any child with this disease can be treated with a high likelihood of a cure with a transplant from a parent or unrelated donor, not just a matched brother or sister," explained Dr.

O'Reilly, Chair of MSK's Pediatrics Department and Chief of the Pediatric Bone Marrow Transplant Service. "Irrespective of the transplant approach used, if the child is transplanted early—without infection—you will have an extraordinarily good result."

But a critical factor continues to be ensuring that the child has no infection at the time of transplant. "This really illustrates the importance of more widespread screening for SCID so that doctors can intervene immediately," said Dr. O'Reilly. Currently, only 21 states have implemented testing as part of routine newborn screening, including New York, California, and Florida. "By the time symptoms of infection appear, the circumstances for transplant are often less favorable."

MSK has played a pioneering role in advancing stem cell transplantation. The first successful transplant of stem cells from an unrelated donor was performed at MSK in 1973 specifically to treat a child with SCID. In 1981, MSK introduced T cell depletion, a groundbreaking procedure for improving the success of unmatched transplants. Most of the important benchmarks in the development of transplantation and cell therapies have come from the studies of these relatively rare cases involving children who have a lethal genetic immune system disorder, said Dr. O'Reilly.

"Great strides have been made in a relatively short time, allowing children with SCID not only to survive but also to lead normal lives," Dr. O'Reilly added. "Paul Simon had that song [The Boy in the Bubble] where he says, 'These are the days of miracles and wonder.'…. Our research provided a way to cure babies in the bubble. We don't need the bubble anymore."

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