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In Pediatrics, a Lesson in Making Use of Experimental Procedures

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If there is a model for an effective way to handle experimental procedures, it is the world of pediatric cancer.

In that arena, most doctors refuse to offer such treatments outside clinical trials, insurance companies usually pick up the tab and experimental procedures are tested quickly, before they reach the marketplace. The result is that the procedures that work are adopted widely; those that do not are quickly abandoned.

Why the difference from adult medicine? The credit, in large part, goes to pediatric oncologists. They have organized themselves into two major consortiums that enroll virtually every child in a clinical trial.

The two groups agreed to sponsor only credible studies that truly advanced knowledge of the field -- quashing the "me-too" phase 2 research that proliferates in other medical specialties. With such quality control in place, the pediatricians were able to successfully lobby health insurance companies to pay for medical care in clinical trials. The companies generally refuse to pay for experimental procedures offered outside trials.

The pediatricians have good reason to unite behind clinical trials. With comparatively few children getting cancer, the number of available test subjects was already severely limited. Doctors convinced each other that it was better for the children to test treatments promptly than to provide them, untested, to every comer.

The results are heartening. While just 1 percent of adult cancer patients enroll in clinical trials, 60 percent of pediatric cancer patients do. Cancer specialists maintain that the consequences are clear: Progress is much faster in pediatric cancer than it is in cancer that strikes adults.

"I can't imagine why adult oncologists don't follow the same route," said Dr. Kenneth Lazarus, a pediatric cancer specialist in San Antonio. "If you take all the cancers that were incurable in the late 1960s, nearly all are curable now. It's dramatic what's happened."

In the pediatric world, even bone marrow transplants were tested promptly. In the late 1980s and early 1990s, at the very time the procedure was being widely sold for adult cancer patients, pediatric oncologists began giving it to children with neuroblastoma, a cancer of the nervous system. The cancer appears in just 500 children each year. But its effects can be devastating, striking toddlers from 2 1/2 to 4 years old and often spreading to the bones, where it can cause excruciating pain before killing the child.

As soon as transplants appeared as a possibility, the Children's Cancer Group, one of the national consortiums of pediatric cancer specialists, began a randomized clinical trial. The group began the trial in January 1991; within five years, it had reached the enrollment goal of 545 children. And even though there was a belief among some pediatric cancer specialists that bone marrow transplants would be more effective than standard chemotherapy for these children, there was virtually no market for the procedure outside the clinical trial.

"We didn't have trouble finding children to enroll," said Dr. Archie Bleyer, the chairman of the Children's Cancer Group. In fact, he said, "the trial was actually completed ahead of schedule."

It is now finished, and the results show what the doctors had hoped -- that bone marrow transplants do improve survival for children with neuroblastoma.

The extraordinary success of clinical trials in pediatric cancer would not have been possible without the cooperation of health insurance companies, cancer specialists say. Unlike the situation in adult cancer, where companies have refused to pay for patients receiving experimental procedures in clinical trials, the same companies routinely pay for children in clinical trials.

Susan Radtky, stem cell transplant coordinator at Riley Hospital for Children in Indianapolis, said that the insurance companies were usually cooperative. "In five years and 200 patients, by and large, I have had 5 percent really problem cases, with insurance companies denying it," she said.

Some insurance companies even go as far as to use the reimbursement structure to push children into studies.

"I have seen contracts that said that if it is not a qualified trial being offered at a qualified center, then it is not covered," said Dr. Andrew Kelahan, the chief operating officer of the Coalition of National Cancer Cooperative Groups, which represents the major groups of medical institutions that conduct cancer clinical trials.

One way to encourage the testing of experimental procedures for adults might be for insurance companies to institute a similar system, some academic doctors say.

But insurance companies alone are unlikely to change the system of marketing untested procedures to adults.

Last fall, working with the Coalition of National Cooperative Groups, United Healthcare, which provides health insurance for 13 million Americans, worked out an arrangement to nudge adults into clinical trials. Dr. Lee Newcomer, a senior vice president for health policy at United Healthcare, explained: "If there was a new treatment out, we would pay for it under the trial scenario. But if someone said they wanted to give it off of the trial we would say no."

But by a year later, nothing had changed. "It's not working," Newcomer said. So far, only a dozen patients have entered clinical trials. Moreover, he added, most of those patients were never even told by their doctors that a clinical trial was an option. They found out about the trials and enrolled despite, not because of, their doctor's advice, he said.

Now, Newcomer said, "the onus is on the physician community." Doctors, he said, may say they believe in putting patients in trials, but when offered an incentive and an opportunity, "it doesn't happen."

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