New Hope or False Promise?

Study shows futility of alternative Down's syndrome treatment

By KATHY FACKELMANN

A new scientific report supports previous research indicating that cell therapy, a controversial treatment unlicensed by the federal government, provides no benefit to children with Down's syndrome, a genetic disorder that afflicts about one out of every 800 infants born in the United States. Yet in spite of this and earlier findings of its ineffectiveness, some parents of Down's children remain convinced cell therapy offers their profoundly retarded offspring the promise of a better future, and so they continue to seek out the treatment.

A Swiss surgeon, Paul Niehans, developed cell therapy in the 1930s and promoted it as a cure for a wide range of human conditions, including old age. Niehans believed fetal sheep or rabbit cells released unknown chemicals that stimulated growth in aging human cells. A West German physician, Franz Schmid, then at Heidelberg University, carried that work one step further in the 1960s by giving children with Down's syndrome subcutaneous or intramuscular injections of a preparation containing freeze-dried fetal sheep or rabbit cells. Schmid, who has written several books on cell therapy, reported dramatic results in the 1960s with a regimen that includes cell therapy as well as physical and speech therapy. Now retired, Schmid continues to advocate his regimen in lectures in the United States and in Europe, claiming it boosts intelligence and improves poor motor and social skills in children with Down's syndrome.

Most infants born with Down's syndrome have 47 instead of the usual 46 chromosomes, a genetic abnormality that pervades every cell of the body and causes lifelong disabilities that include mental retardation, impaired motor ability and behavioral difficulties. In some parents, Schmid's enthusiasm inspires hopes for improving an incurable condition.

Yet most U.S. Down's syndrome authorities discount Schmid's success stories. They point to a handful of negative scientific reports, dating back over a quarter century to an Aug. 1, 1964 LANCET report. In that study, British researchers gave five children with Down's syndrome shots of animal fetal cells and compared the children with five controls. After one year, the researchers found both groups scored equally well on tests that measure intelligence and motor ability.

The LANCET study and other negative reports by European and Canadian researchers involved small numbers of patients and did not receive widespread publicity in the United States. Doctors say a small but steady number of U.S. parents continue to turn to cell therapy, despite the difficulties in getting the treatment, which is not licensed by the Food and Drug Administration. To get the unapproved therapy, patients and their parents must fly to West Germany or obtain freeze-dried fetal cell material in the mail and then find U.S. doctors willing to prepare and administer the shots.

Now, for the first time, a U.S. team of scientists has researched the subject. A report by Don C. Van Dyke of the University of Iowa Hospitals and Clinics in Iowa City and David J. Lang of the Children's Hospital of Orange County, Calif., adds weight to the scientific view that cell therapy is futile as a treatment for Down's syndrome.

Van Dyke, Lang and their colleagues studied 190 Down's syndrome patients between the ages of 2 months and 19 years who visited a clinic in the greater Los Angeles area from 1984 to 1986. The research team also interviewed parents, finding 21 (11 percent) who said their children had received cell therapy at some point in their lives. Psychologists and occupational therapists gave the 190 study participants a battery of tests to measure intelligence, motor abilities and social skills. The research team then matched the 21 children who had received cell therapy with 21 controls who had never gotten this unorthodox care by age, sex and socioeconomic level and compared test results.

The retrospective match showed no "statistically significant differences" for any of the 18 social, developmental or growth variables measured. "These findings fail to support the claims of improved functioning following cell therapy and the continuing support of this therapy by parents of persons with Down's syndrome," the team reports in the January PEDIATRICS.

The PEDIATRICS report falls short of convincing some parents. Van Dyke and Lang lumped children who had gotten just one shot in with children who had received multiple fetal cell injections, notes Janice S. Statham, who chairs the New Hope Parents Association, a Huntsville, Ala.-based group that supports cell therapy for Down's syndrome. Children must get repeated treatment to show progress, she says.

The authors acknowledge their retrospective study has disadvantages. The findings came from a larger study of Down's syndrome that wasn't designed to home in on cell therapy's efficacy, Van Dyke says. He admits some test evaluators could have known about their subjects' cell therapy status, a problem that might skew results.

Lang concedes such flaws, but says the data from this study, taken together with past reports, indicate cell therapy doesn't work. That view is shared by most U.S. scientists working with Down's syndrome. No evidence exists that cell therapy is an effective treatment for Down's syndrome at this time, says Felix F. De La Cruz of the National Institute of Child Health and Human Development in Bethesda, Md. "I think it holds no useful promise," adds Allen C. Crocker of the Harvard Medical School in Boston.

Some parents of Down's syndrome children counter the scientific chorus of naysayers with upbeat success stories. "I'm not going to call it a 'smart pill' but naturally when a child feels better they do better in school," Statham says.

Statham's positive view seems reflective of many parents who pursue cell therapy for their children. A 1986 survey of 116 cell therapy patients by psychologist Alfred Baumeister of Vanderbilt University in Nashville found that 71 percent of the parents believed the shots improved their child's learning ability and 85 percent would recommend cell therapy to other parents. Baumeister suggests that something about cell therapy, possibly the additional attention a child receives, may provide some benefit to kids with Down's syndrome.

It seems clear that some parents will continue to pursue cell therapy, a prospect that alarms many medical professionals. "The scooping up of families for unproven treatments is something that most of us view with alarm," Crocker says.

All Down's experts agree children may

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engineers may be quite surprised, since their research journals contain almost no references to emulsion-trapped organics. Moreover, he says, the improved water solubility of organic chemicals that Chiou reports may not be restricted to water containing emulsions formed by petroleum-sulfonate surfactants. Under certain conditions, he suspects, even conventional chemical solvents may mix with oil and form similar stable, water-polluting emulsions.

The Penrose paper, by contrast, may be thought of as potentially iconoclastic, Zachara suggests, since a vocal school of water-pollution scientists "feel that colloids do not really exist in groundwater"—except as "artifacts of sampling." You can't dig a water-monitoring well without disturbing some of the soil above the groundwater, he notes. Many researchers have argued that any colloids detected in groundwater resulted either from digging the well or from removing water for testing.

At last, he says, the "Penrose paper lays this issue to rest." There's no way to explain the vast migration of the LANL radionuclides without colloids, he says.

Philip M. Gschwend agrees. An environmental organic chemist at the Massachusetts Institute of Technology in Cambridge, he says the "exciting" Penrose paper "confirms what many of us had begun to worry about: [In the disposal of low-solubility hazardous wastes,] we can no longer be sure that they will attach to large particles such as soil and stay put."

A second major implication of this paper, he says, is that traditional groundwater-sampling techniques may have to change. Currently, analysts hunting for pollutants migrating from toxic-chemical sites filter water drawn from monitoring wells to eliminate any particles too large to be transported great distances in water. But "I don't think it's possible to take out the big particles [which don't matter] and not lose some of the little ones [that do]," Gschwend says. Such filtering, therefore, risks greatly underestimating the chances that insoluble pollutants are hitching long rides with passing colloids.

"We still don't know much about how colloids form or the tendency of soils to trap and catch colloids as they're flowing by," Gschwend notes. His group began investigating both about four years ago.

Penrose's work suggests these endeavors will prove challenging for groundwater researchers. With regard to anticipating the presence and involvement of colloids, he says: "We found surface waters were boringly predictable. Once you've studied half a dozen, they start to look pretty familiar. But groundwaters are really different. Every one is a new story." □

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suffer allergic reactions to injections of cells taken from animal tissue. In addition, Crocker points out, injections of fetal sheep cells may transmit agents such as the microorganism causing scrapie, a degenerative central nervous system disease that afflicts sheep. That concern prompted the National Down Syndrome Congress, a Park Ridge, Ill.-based parent and health professionals group, to issue a 1986 statement that advises parents against the "life-threatening" treatment. However, Lang says the risk of scrapie transmission has been overblown. "We don't have any evidence at all that the slow viruses of sheep are transmissible to man," Lang says.

Even those opposed to cell therapy can understand the human motives behind it. "These parents are very much at a loss as to know what to do for their children," Lang says. "The medical profession offers them precious little."

"We do not want to be fooled," says Statham, whose group includes 1,000 parents nationwide. She notes there has never been a large, double-blind study of cell therapy's efficacy. Most scientists involved in the issue agree the therapy has not been put to an airtight test, but they say the existing data show the treatment is powerless to improve symptoms of Down's syndrome. □