

“Bubble baby” lives a normal life after gene therapy

Owen Dyer *London*

The first success for gene therapy in the United Kingdom has restored health to an infant who last year was confined to a sterile room and needed to be kept alive by a ventilator.

Rhys Evans, now 18 months old, suffered from X chromosome linked severe combined immunodeficiency disorder, popularly known as “baby in the bubble” syndrome, after the sterile plastic spheres used to protect some babies from infection. He now lives a normal life at home.

At a press conference last week his doctors pronounced themselves “ecstatic” about the boy’s recovery. “We’re very pleased to announce the first successful cure of a patient in the United Kingdom by gene therapy,” said Professor Christine Kinnon, director of the Centre for Gene Therapy of Childhood Disease at the Institute of Child



Rhys Evans, who was “skin and bones” before treatment

Health, London, who collaborated with doctors at Great Ormond Street Hospital in treating Rhys.

Dr Adrian Thrasher, consultant paediatric immunologist at Great Ormond Street Hospital, was more cautious: “I’d like to say that Rhys is cured, but we can’t say that. He will be followed up for years and years.”

Rhys’s condition appeared soon after his mother stopped breast feeding him at four months. A series of chest infec-

tions developed into severe pneumonia. “He was skin and bones, too weak to hold his head up,” said his father, Mark Evans.

The standard treatment for the condition is a bone marrow transplant, but Rhys had no obvious donors. A US donor was finally located at the same time that gene therapy became available. The parents opted for the new treatment, partly to avoid the chemotherapy that accompanies transplantation.

The treatment began with the extraction and freezing of cells from Rhys’s bone marrow. A mouse retrovirus was genetically engineered to carry a healthy copy of the gene that was defective in Rhys. A bag containing the retrovirus was connected to a bag of his bone marrow. The virus then spliced the healthy gene into the stem cells, which were then reinserted into the bone. Within a few months his lymphocyte count began to climb rapidly.

Three months ago a second, 10 month old baby underwent gene therapy for severe combined immunodeficiency disorder at Great Ormond Street. That patient shows even more promising results than did Rhys at the same stage, Dr Thrasher said. □

Medicare will now cover some treatments for Alzheimer’s disease

Fred Charatan *Florida*

The Bush administration has told Medicare contractors to stop automatically denying Medicare payments to people with Alzheimer’s disease.

Because of evidence of fraud over several years, the Centers for Medicare and Medicaid, which administers healthcare services for Americans aged over 65 and for people with low income, had cautioned its contractors to be sceptical of claims relating to patients with Alzheimer’s disease. For example, some healthcare providers were billing for treatment for patients who had deteriorated so much that no benefit would result.

To avoid paying such dubious claims, about 20 of the 50 Medicare contractors that process claims for the centres had established computer systems that automatically denied payment of claims relating to Alzheimer’s disease. In some of these cases, however, payment might eventually be won after a lengthy appeals process.

Late last year, after two years of lobbying by the Alzheimer’s Association and the American Bar Association’s Commission on Legal Problems of the Elderly, the centres notified the 50 Medicare contractors that they could no longer issue blanket denials of payments for treatment of Alzheimer’s disease.

A statement issued last week by the centres’ administrator, Tom Scully, said: “Advances in medical science are helping physicians diagnose Alzheimer’s disease in its earliest stages. Depending on a beneficiary’s medical condition, the Centers for Medicare and Medicaid Services believe that certain specific therapies can be helpful in slowing a beneficiary’s decline due to this terrible illness.”

The centres had therefore issued instructions to Medicare contractors not to install systems that would automatically deny Medicare covered services to patients with dementia. □

African Americans are less likely to see an oncologist, study shows

Scott Gottlieb *New York*

Differences between races in patients’ likelihood of surviving cancer may stem from differences in access to chemotherapy or even to the doctors who prescribe it.

Examining the medical records of patients with lung cancer, a group of Harvard University researchers led by Dr Craig Earle of the Center for Outcomes and Policy Research at the Dana-Farber Cancer Institute, Boston, found that African Americans are less likely than other races to see an oncologist (*Journal of Clinical Oncology* 2002;7:1716-8).

In an earlier study the same group of researchers used data from the US National Cancer Institute to look at the use of chemotherapy in advanced non-small cell lung cancer. They found that only about a quarter of patients with non-small cell lung

cancer who were of Medicare age received chemotherapy (*Chest* 2000;117:1239-46).

In the current study the researchers went on to ask whether the unexplained variation in the use of chemotherapy for advanced lung cancer was due to access to oncologists’ services, rather than to treatment decisions made after a visit to an oncologist. They found that 73% of patients with non-small cell lung cancer were referred to a medical oncologist, although only 26% ultimately received chemotherapy.

Of the non-medical factors, level of education, income, and race played significant roles in whether a patient with advanced non-small cell lung cancer was referred to a medical oncologist. Overall, white patients were significantly more likely than non-

white patients to be assessed by an oncologist (74% *v* 70%, $P<0.001$), and white patients in higher socioeconomic groups were more likely than other groups to be seen by an oncologist and to receive chemotherapy. Black patients were significantly less likely than patients of other races to receive chemotherapy.

“Black race, probably acting as a proxy for lower socio-economic status, was associated with both a diminished likelihood of seeing a cancer specialist and subsequently receiving chemotherapy,” the authors write.

The current study adds to research indicating that inequalities in health outcomes may be related to African Americans having less access to certain kinds of care.

“Clearly, insurance is not the only factor leading to barriers in cancer care,” write Dr Alfred Neugut and Dr Victor Grann of the Herbert Irving Comprehensive Cancer Center at Columbia University’s College of Physicians and Surgeons, New York, in an accompanying editorial. □