 Times 5.9.2018

Game-changing NHS treatment to save children with leukaemia

Chris Smyth Health Editor

Children suffering leukaemia will become the first to be cured by routine NHS use of a "revolutionary" treatment that re-engineers the immune system to fight cancer.

The head of the NHS said that a new front had been opened against cancer after the first of a new class of therapies was approved in record time, paving the way for living drugs to personalise treatment for thousands of patients.

The one-off treatment appears to offer a permanent cure for some people and the NHS has accepted the need to pay for it. It costs £282,000 per patient at full price.

Chimeric antigen receptor T-cell therapy, known as CAR-T, involves harvesting a patient's immune cells and genetically engineering them so that they produce a synthetic molecule which attacks cancer cells. The re-engineered T-cells are then infused back into the body where they can multiply and wipe out the disease.

Simon Stevens, head of NHS England, says in a speech to be given today that "CAR-T therapy is a true game changer and NHS cancer patients are now going to be amongst the first in the world to benefit". He adds: "We are just at the beginning of a new era in personalised medicine and so we expect to see many more treatments like this over the next five to ten years."

Kymriah, a type of CAR-T that was developed at the University of Pennsylvania and approved by European regulators only ten days ago, will be used in NHS hospitals in London, Manchester and Newcastle within weeks. Mr Stevens argues that CAR-T is "one of the most innovative treatments that has ever been offered on the NHS" and that it "is leading from the front on innovative new treatments" after a confidential discount was agreed.

Initially Kymriah will be used each year on about 30 children with B cell acute lymphoblastic leukaemia (ALL) who do not respond to current leukaemia treatments or have relapsed.

Alasdair Rankin, director of research at the blood cancer charity Bloodwise, said: "CAR-T cell therapy offers the genuine chance of a long-term cure for children who otherwise would have no other hope." Dr Rankin said that he believed many types of blood cancer would ultimately be treated by CAR-T as results were amassed and prices fell. "The results we've seen so far have been very, very impressive," he said. "Theoretically the benefit of CAR-T is it is a living therapy which can stick around, monitor the cancer and theoretically wipe it out."

A trial found that 80 per cent of patients with ALL given the drug saw their cancer vanish completely, although it can have severe side-effects.

Mr Stevens has taken a tough line in price negotiations with the pharmaceutical industry in an effort to reduce costs and ensure that spending on medicines does not "crowd out" money for staff and equipment.

Last week a type of CAR-T therapy for adults with blood cancer, Yescarta, was rejected as too expensive. In comments likely to be seen as targeted at Gilead, which makes Yescarta, Mr Stevens will tell the NHS Expo conference in Manchester that the deal with Novartis for Kymriah "shows how responsible and flexible life sciences companies can succeed — in partnership with the NHS — to make revolutionary treatments available to patients".

Analysis

The technique that sounds like science fiction — a "living drug" — will become routine on the NHS with no limit yet set on its potential (Chris Smythe writes). CAR-T involves taking blood to separate out T-cells, which marshal the immune system's response to attackers. These cells are genetically engineered to produce synthetic proteins not found in nature which allow the T-cells to attach to cancer cells.

These "chimera" cells are then multiplied in the laboratory and put back in the body, where they proliferate and attack cancer.

Raj Chopra, of the Institute of Cancer Research in London, said that the method gave doctors a new weapon. "In blood cancer the question is can you give it earlier? We may be able to cure people at a much earlier stage," Professor Chopra said. "For solid tumours it is going to be a bit longer, maybe five or ten years."

Research is under way to produce "off the shelf" therapies that do not need patients' own immune cells, which could reduce eye-watering prices. The hope is that in future modifying the immune system to fight cancer will be as routine as targeted medicine is today.