'Cutting edge' CAR T cell immunotherapy approved in England. But is the NHS ready?

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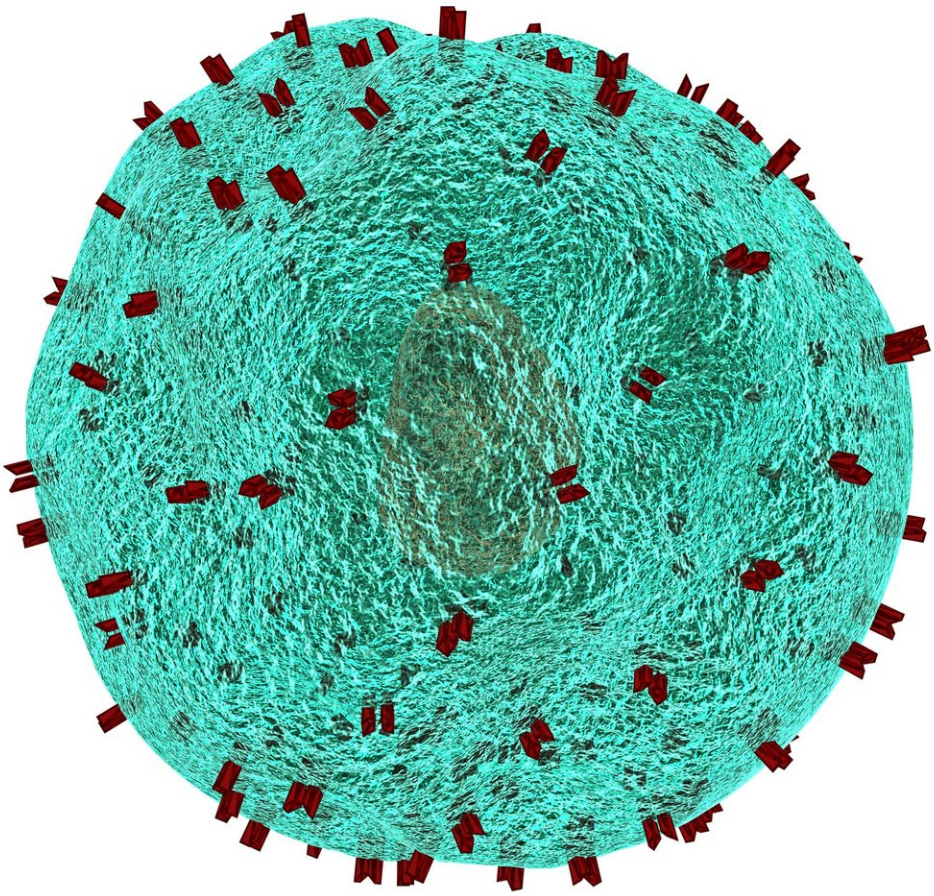

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Today, a new blood cancer treatment was approved for use on the NHS in England. And in early September, NHS England announced another, similar treatment will be available to some children and young people with a different type of blood cancer.

As with any new drug approval, these announcements are great news for the patients who may benefit. But these are no ordinary drugs.

The two treatments – called axicabtagene ciloleucel (Yescarta) and tisagenlecleucel (Kymriah) respectively – have attracted attention because of the ground-breaking way in which they're made and work.

This makes their approval a fascinating live case study for an NHS that's attempting to position itself as a world-leader in cancer treatment.

What is CAR T cell therapy?

Yescarta and Kymriah are two of a new line of immune-boosting treatments called CAR T cell therapies.

They involve extracting specialised immune cells, called T cells, from a patient's blood and altering them in the lab so they recognise and fight cancer cells. The modified T cells are multiplied a thousand-fold in the lab and then put back into the patient's bloodstream.

The results from small trials have been promising, as the cellular engineering turns patients' own immune systems into precise anti-cancer weapons. And unlike standard chemotherapy, the treatment only has to be given once.

CAR T cells are "the first truly personalised therapy, because the cells come from the patient," says Professor Karl Peggs, a Cancer Research UK-funded immunotherapy expert. He says this makes it a game-changer "in the small niche in which it is being licensed and used".

In other words, while the technology is exciting, at the moment it will only reach a relatively small number of people, and that's unlikely to change any time soon.

In fact, Peggs cautions against overplaying the potential for CAR T to turn blood cancer treatment on its head. For childhood acute lymphoblastic leukaemia – one of the cancer types CAR T can now be used in – he says only a tiny fraction of patients will benefit from the new treatment because "most patients are cured with conventional therapy".

But for the few patients in whom chemotherapy hasn't worked, the approval of CAR T offers a vital new option.

How effective is CAR T?

Impressive results have been reported in clinical trials for some types of blood cancer where there are few other treatment options available. In one trial, around 8 in 10 patients saw their disease respond to the treatment, and for around half of those patients their cancer hadn't come back a year later.

But, while the treatment has shown great promise, it doesn't work for everyone. According to Peggs, it's unknown why only some patients' cancers respond initially, and why others see their cancer come back later while some remain disease free.

More research will help answer these questions, and the terms of the

treatments' approval on the NHS state that data on their effectiveness can be collected along the way. This, again, is positive news.

The potency of unleashing the immune system also brings with it the risk of serious side effects that need expert care. In one clinical trial testing Yescarta, 94 percent of patients experienced a side effect called cytokine release syndrome, and 1 in 7 of them needed treatment in intensive care.

Despite the challenges, these treatments have attracted a lot of interest. The head of the NHS in England, Simon Stevens, has described CAR T therapy as "cutting edge" and "one of the most innovative treatments ever offered on the NHS".

But the complexity of these treatments means they pose more of a challenge to the NHS than most new drugs.

Is the NHS ready for CAR T therapies?

"In many ways, this treatment is more akin to a stem cell transplant than traditional chemotherapy," says Peggs. NHS England has selected hospitals where staff have experience of procedures like stem cell or bone marrow transplants as the first locations to offer the treatment. The NHS is already in a strong position, as it has world-class facilities for carrying out these procedures.

But those hospitals will also have to plan for patients' supportive care. To help monitor side effects, people having CAR T therapy will have to stay in hospital afterwards, and be within easy travelling distance for several weeks after they leave.

This means the treatment will initially only be given to a few patients at a time across England, in a limited number of hospitals, to make sure the

NHS can look after them properly.

It's not yet clear whether patients in Wales and Northern Ireland will need to travel to hospitals in England to be treated. And in Scotland, on top of this uncertainty, these treatments would also have to be separately approved by the Scottish Medicines Consortium (SMC) before they can receive NHS funding. The SMC is due to decide on Yescarta in November, and on Kymriah in early 2019.

Complexity aside, one of the major debates surrounding CAR T cell therapies has been their cost. The treatments are expensive, at around £280,000 per patient, though the NHS has negotiated significant discounts for both Yescarta and Kymriah. Both the NHS and the companies that develop these treatments, Gilead and Novartis, deserve huge credit for agreeing deals for these drugs.

CAR T therapy is an extreme example of a general trend for the newest cancer drugs, which are more personalised and complex, but pricier as a result. This trend makes it vital the NHS explores new ways of paying for cancer drugs, which could make it easier to approve exciting new treatments in future so that patients can access them more quickly.

We're working with partners in Greater Manchester to explore one approach called outcomes-based pricing, where the NHS pays a different price for the drug, based on the benefits it gives patients.

What does this all mean for patients and the NHS?

The NHS approvals of Kymriah and Yescarta are a huge leap forward for those patients in England with the very specific blood cancers they are approved to treat.

But even for these patients, there are still unanswered questions about

the drugs' long-term effectiveness, which patients are most likely to respond, and how to manage and reduce side effects. We, and others, are hoping to answer these questions through research.

The NHS can rightly be proud that a small number of patients in England will be the first in Europe to have routine access to this highly complex technology. But the challenges presented by exciting new treatments like CAR T won't go away. Today's good news should be just the start, not the end, of the NHS's preparations for this shift.

Provided by Cancer Research UK