



Potentially curative treatments for acute and chronic diseases

Use the body's own cells and genes to target the cause of disease

Personalised medicines tailored to the individual

The UK is home to world leading manufacture and clinical research

# CELL & GENE THERAPIES

## OPPORTUNITIES

### Clinical

- Treatments are intended to cure, going straight to the cause not just managing the symptoms
- Treatments are minimally invasive and in some cases only one treatment session is needed. In the long term, this reduces the burden on papers, carers and services
- Fewer, and predictable side effects can be well managed and improve quality of life for patients

### Manufacturing

- The UK's investment in manufacturing, research and innovative market access programmes create a leading environment for cell and gene innovation
- 12% of global clinical trials take place in this country with multiple sites pioneering research and access to treatment

## CHALLENGES

### Clinical

- Workforce will need to grow and expand to meet demand and to receive additional training in delivery of cell and gene
- NHS infrastructure will need investment to support these and other innovative treatments

### Manufacturing

- Currently some of the most expensive curative treatments available

*I hope that one day cell and gene therapies will be the standard treatment for leukaemia as the impact on the child and the family during treatment and long term is far lower.*

## How does cell and gene therapy work?

A patient's mother tells the story of her daughter's cell and gene therapy treatment for leukaemia.



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The patient's own cells, now repaired, are administered back to them as a treatment. This may be over one or several doses.

*For our daughter the process of her treatment was a breeze. As her parents it was a bit of a rollercoaster, waiting for approval, timing the cell collection, but all in all we were very well supported by the team and ultimately the process was smooth.*

1 Patient is diagnosed with disease.

*The pain and fear you feel when your child is diagnosed with cancer is unimaginable*



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Cells are extracted from the patient and sent to a specialist lab where modifications are made.

### Outcome

Cell and gene therapies have known side effects, however these are often less frequent and severe compared to other treatments for the same disease.

Reduced time in hospital with ongoing patient follow up.

*Our daughter experienced side effects for a few days which required intensive care. However within days she was completely back to normal and back in school full time within a few weeks! It was by far the easiest treatment she has had.*

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