Cell and gene therapies

* 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

# Opportunities

## Clinical

* Treatments are curative, going straight to the cause not just managing the symptoms
* Treatments are minimally invasive and in some cases only one treatment session is needed. This reduces the burden on both patients and service
* Fewer side effects improves 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 additional training in delivery
* NHS infrastructure will need investment to support these and other innovative treatments

## Manufacturing

* Currently some of the most expensive curative treatments available. Price may need reduce in order to increase patients access

# How does cell and gene therapy work?

“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.”

1. A patients mother tells the story of her daughter’s cell and gene therapy treatment for leukaemia.

“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.”

1. Cells are extracted from the patient and sent to a specialist lab where defects are repaired.
2. The patient’s own cells, now repaired, are administered back to them as treatment. This may be over one or several doses.

# Outcome

Side effects are 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.”