Cell and Gene therapy – the future of medicine?

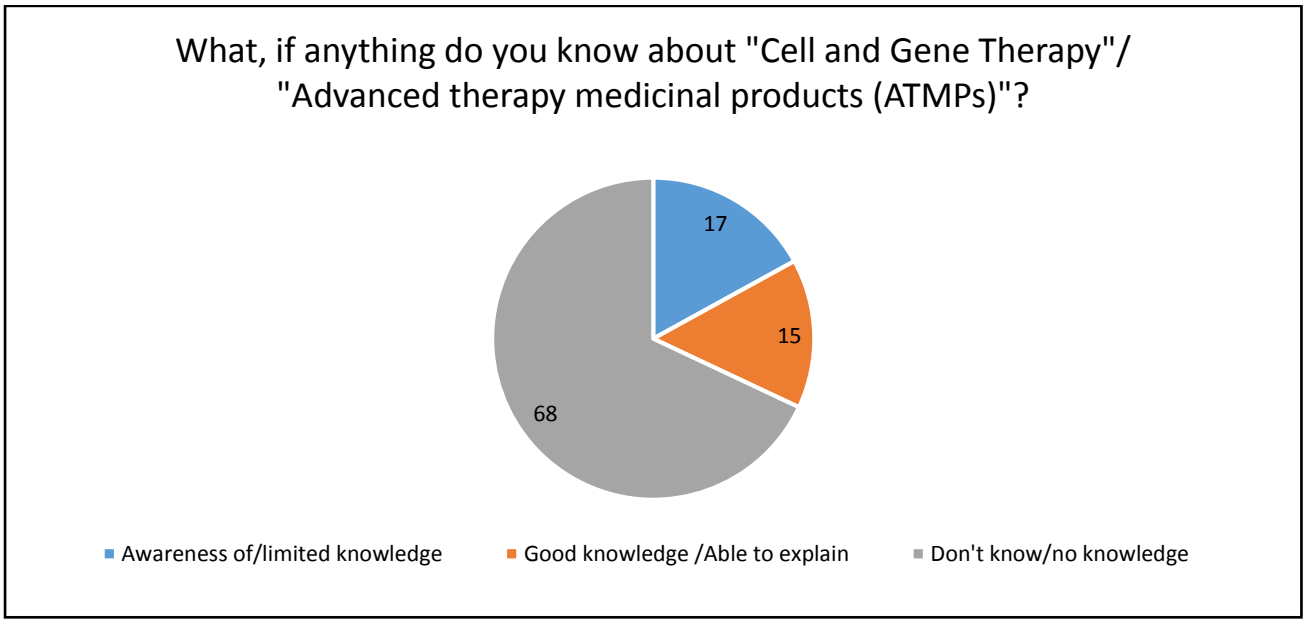
Cell and Gene therapies are a transformative new generation of treatments that are set to revolutionise the way we think about and treat disease. Cell and Gene therapies use the body's own cells or genes to target the cause of disease. This is different to current drugs which often target the symptoms of a disease. Cell and Gene therapies can have much longer effects than traditional treatments. The UK is home to world leading research and manufacturing capabilities for Cell and Gene therapies.

On Wednesday the 28th of July 2021, Daniel Zeichner hosted a Policy Connect roundtable to raise awareness of the barriers and opportunities associated with these new treatments.

Speakers included:

- Prof Emma Morris, Professor of Clinical Cell and Gene Therapy at UCL
- Fiona Marley, Head of Highly Specialised Commissioning at NHS England and NHS Improvement
- Dr Jacqueline Barry, Chief Clinical Officer at the Cell and Gene therapy Catapult
- Sian Laidouni a patient representative

They were joined by a wide range of stakeholders including charities, academics, life sciences industry figures, clinicians and wider health care professionals. The session was opened with some data showing that only 15% of MPs had a good understanding of these treatments (see graph below). There was, however, broad interest in learning more about these therapies and the associated issues.



Cell and Gene therapies – a different kind of treatment

"*Any cell or gene therapy is potentially curative*" Prof Emma Morris, UCL

Cell and Gene therapies utilise the body's own genes or cells to fight disease. They include techniques that correct genetic mistakes or introduce new genes to immune cells improving their function. They are a form of personalised medicine and as such are more expensive than "off the shelf" treatments. Speakers highlighted that the manufacturing and administration of these treatments is more complex than traditional drugs. However, these treatments can last much longer and have far less side effects than other treatments. Cell and Gene therapies can therefore improve the quality of life of the patient receiving them.

"*it's better … for these children to be able to live their lives without so many toxic treatments*" - Sian Laidoni, Patient representative.

It was highlighted that the UK is at the forefront of global Cell and Gene Therapy research; home to 12% of global clinical trials (compared to 3% for other research areas). Attendees underlined that this is not by accident and cited government investment as a factor. Examples included a manufacturing centre in Stevenage and upskilling via an apprenticeship programme. In addition, the innovative regulation on offer in the UK makes it an attractive place for industry. It was agreed that we need to ensure the UK maintains this world leading position in the future.

Evidencing value

Currently the system used by regulators for approving new treatments for use in the NHS involves evaluating the treatment benefits in comparison to the costs. The cost-benefit analysis is usually carried out on an annual basis and considers physical health. Attendees suggested quantifying value in other ways, for example considering the broader impact of chronic diseases on family and society. Other benefits not currently assessed included the patient's quality of life and the ease of administration of CAR T therapy (a type of cell therapy) as opposed to other treatments.

"*A switch was flicked, and she was back to normal*" - Sian Laidoni, Patient representative.

Some attendees suggested that the current system of evaluating value by annual impact of a treatment was agreed to be inappropriate for cell and gene therapies as well as other personalised medicines and treatments for rare diseases. The need to determine the benefit of treatments over years rather than months was also discussed by attendees, using a new framework.

Furthermore, there was some agreement that there can be too many routes to funding which makes it confusing and a barrier:

"*…there are many laudable routes for these solutions, but it's quite confusing for somebody to work out which pot to dip into and as a consequence some really promising solutions simply don't get through*" - Anne Marie Morris MP

These problems are not new or unique to cell and gene therapies. To address these points a NICE methods review examining how independent committees look at evidence for new

technologies is underway. Attendees suggested the use of transferrable findings across treatments to show benefit. The Innovative Licensing and Access Pathway (ILAP)[1] was referenced as a way of regulators looking at the different data developers are producing to build confidence and understanding. Attendees suggested being more willing to accept risk or real-world evidence when large scale trial data is lacking as has been done during the COVID-19 pandemic.

A pathway to adoption

The UK is in a good place for Cell and Gene therapy adoption. It is seen as a good place to base both the research into and the manufacturing of Cell and Gene therapies. Attendees discussed the many reasons for this, such as the strength of UK research on the global stage along with strong international collaborations. The commercial medicines directorate[2] and the UK's good record for working with the pharmaceutical industry were seen as competitive advantages. Other strengths include the success of schemes like EAMS (easy access to medicines)[3] which has treated over 2,000 patients. The UK is working towards making Cell and Gene more accessible, which will be helped by the NICE methods review and continual stakeholder engagement.

"*There's no point making a drug available if you haven't got the service to deliver it*"- Fiona Marley, NHS England.

Importantly, ensuring the NHS is ready for delivering Cell and Gene therapy was highlighted. To expedite the process the Advanced Therapy Treatment Centre (ATTC) network[4] has developed an NHS readiness toolkit[5] for hospitals to use. This Network is embedding centres of excellence and best practice across the UK. Ensuring that resources like these are used and that Cell and Gene therapy continues to be embedded across all regions of the UK is essential. The need for efficient use of data and the importance of patient identification was also highlighted:

"*Use the amazing data we have in the NHS – collect it once and use if often*" - Jacqueline Barry, Cell and Gene therapy Catapult.

Patient representative Sian Laidoni concluded the session by thanking all those involved: "[When] *you realise the UK are at the forefront of this for leukaemia you feel so proud and so overwhelmed that all of you people are working so hard to save children like mine."*

[1] The Innovative Licensing and Access Pathway (ILAP): https://www.gov.uk/guidance/innovative-licensing-and-access-pathway
[2] Commercial Medicines: https://www.england.nhs.uk/medicines-2/commercial-medicines/
[3] Early Access to Medicines Scheme: https://www.gov.uk/government/publications/early-access-to-medicines-scheme-eams-how-the-scheme-works
[4] The Advanced Therapy Treatment Centre network: https://www.theattcnetwork.co.uk/
[5] The NHS readiness toolkit: https://www.theattcnetwork.co.uk/advanced-therapies-nhs-readiness-toolkit