CELL & GENE COLLECTIVE

PATIENTS ARE GEARING UP FOR CELL AND GENE THERAPIES – **ARE YOU?**

JUNE 2021

The insights shared in this report stem from the Cell & Gene Collective's Patient Advocacy Summit, exclusive polling and secondary research.

66%

of people surveyed in the UK would **feel comfortable** receiving a cell or gene therapy.[1]

45%

of people surveyed in the UK believe **investment** into cell and gene therapy should be a **Government priority.**[1]

Cell and gene therapies are no longer distant scientific explorations – they are becoming a reality for patients and the NHS, with a strong pipeline and many new treatments expected to come to market in the coming years. It is thought that roughly 2,500 patients will benefit in 2021, rising to over 10,000 by 2029.[2]

We believe this is important for a wide range of patients, spanning oncology, rare and ultra-rare conditions.[3] But, the patient and health system journeys for cell and gene therapies are fundamentally different than traditional medicines. For example, some cell and gene therapies are being investigated to improve outcomes for patients after a single administration,[4] in contrast to conventional treatments administered on a regular basis.

Our exclusive polling revealed growing optimism for cell and gene therapies among the UK general public, with fewer than one in ten thinking this type of treatment is bad, and the majority saying they would be comfortable receiving a cell or gene therapy if they had a life threatening condition.[1] Likewise, our Patient Advocacy Summit demonstrated that patients are becoming more knowledgeable about treatment options, and their associated patient organisations are mobilised.

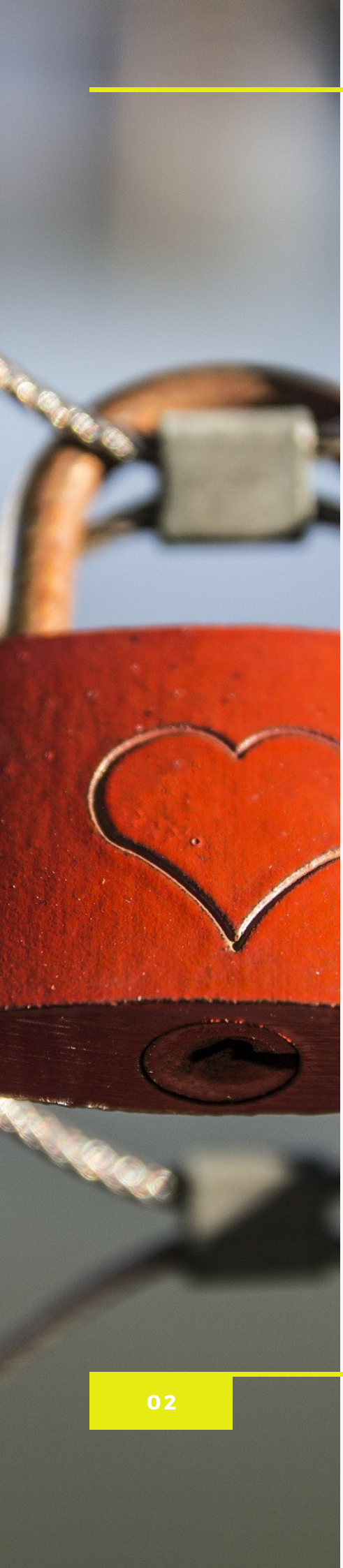
If we are to retain the UK's world leadership in cell and gene therapy and help convert it into tangible patient benefit in the NHS, the patient voice is crucial. Together with industry, patient groups are ready to use this pivotal moment to make sure the next wave of clinical trials and medicines can be accessed smoothly and without delay.

Patients are not passive recipients in this journey, they want to be part of the pioneering journey with informed consent. **How do we make this happen?**

1 Developing a common **language**

Cell and gene therapies are complex, and patients wishing to become well informed about their treatment options call for consistent language to be used to enable them to understand what a specific treatment option will mean for them. Patients don't want language to be 'dumbed' down; instead, patients want to understand the basic science and its terminology, and this in turn will help to manage expectations and help inform decision making.

> **"We need to have a commonality of language, using different terms for the same thing can become incredibly confusing for patients. There is one common message from patients – don't dumb it down, use the correct medical terminology and help us to understand it."**
>
> **Debra Morgan, The Haemophilia Society**



2 Access to good quality **information**

Even for patient groups who have a long history in cell and gene therapy, the level of understanding of these medicines in their patient communities remains a challenge. For some patient communities, this is hampered by scepticism and a loss of trust in the health and care system stemming from experiences such as the contaminated blood scandal in the UK. Providing patient communities with good quality, disease specific information will raise the level of understanding and will empower patients to make informed decisions.

3 Improved access & design of **clinical trials**

Cell and gene therapy trials continue to prove limited in capacity for some disease areas, and as a result create a "competitive" nature for families. We heard from patient groups that swift and seamless access to clinical trials for patients through increased trial capacity and access to diagnostic testing for rare conditions is essential, as is addressing the issues surrounding trial participation through transparent consent forms. There is also a need for evidence generation in real world settings to overcome uncertainty around clinical outcomes. Supporting patients to access clinical trials will help more patients to benefit from approved treatments in the future, and will ensure the UK retains its position as a leader in the development of innovative cell and gene therapies.

4 Developing innovative methods for assessing **value & commercial decisions**

Through the NICE Methods and Process Review and the NHS Commercial Framework, there is a push in the health and care system to provide quicker and easier access for patients to approved, innovative therapies. Yet when it comes to cell and gene therapies, patient groups expressed concern at the level of progress being made, and fear that patients may have difficulty in accessing approved cell and gene therapies at scale in the future. Policy makers must set their expectations high with regards to the potential of cell and gene therapies and take steps to address commercial challenges now to ensure patients can benefit in the future.

A National Action Plan for cell and gene therapy can help the health and care system consider all of these pieces to deliver the change needed for cell and gene therapy patients in the NHS at scale and at pace.

[1]Deltapoll interviewed 1,552 British adults online in September 2020 to determine how much individuals know and understand about cell and gene therapy, how they feel about it and how they value it. The data have been weighted to be representative of the British adult population as a whole. Data on file. Last accessed April 2021.
[2]The Cell & Gene Therapy Catapult, Advanced Therapy Treatment Centres, Patient and public perspectives on cell and gene therapies presentation, 24th March 2021.
[3] The Cell and Gene Therapy Catapult UK clinical trials database.
[4] Mendell JR, Al-Zaidy S, Shell R, et al. Single-Dose Gene-Replacement Therapy for Spinal Muscular Atrophy. The New England Journal of Medicine. 2017 Nov;377(18):1713-1722. DOI: 10.1056/nejmoa1706198

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