GENE AND CELL THERAPY

Providing good information to involve and engage

GENETIC ALLIANCE UK

EURO GCT
European Consortium for Communicating Gene & Cell Therapy Information

ATTC
Advanced Therapy Treatment Centres

Coordinated by
CATAPULT
Cell and Gene Therapy

ABOUT GENETIC ALLIANCE UK

Genetic Alliance UK is the national charity working to improve the lives of patients and families affected by all types of genetic conditions. We are an alliance of over 200 patient organisations.

Rare Disease UK is a multi – stakeholder campaign run by Genetic Alliance UK, working with the rare disease community and the UK's health departments to effectively implement the UK Strategy for Rare Diseases

SWAN UK (syndromes without a name) is a patient and family support service run by Genetic Alliance UK. SWAN UK offers support and information to families of children with undiagnosed genetic conditions.

Address: Genetic Alliance UK,
Creative Works
7 Blackhorse Lane
London
E17 6DS

Telephone: 0330 124 0441

Email: contactus@geneticalliance.org.uk

Website: geneticalliance.org.uk

Facebook: GeneticAllianceUK

Twitter: @GeneticAll_UK

Instagram: geneticallianceuk

Registered charity numbers: 1114195 and SC039299

Registered company number: 05772999

INTRODUCTION

'Patient and public involvement' (PPI) in the development of therapies aims to give a voice to those with lived experience of a therapy or its target condition. Done effectively, it can improve the focus of efforts and investments on real unmet needs, ensure that the delivery of research takes account of the daily lives of the people taking part, and foster better understanding and relationships between the researchers working on new therapies and their ultimate beneficiaries.

Individuals affected by conditions, and their families and carers, need to have access to information to allow them to make informed decisions about taking part in research trials and initiating treatment in the clinic.

For success in both of these contexts, and where new technologies are concerned, there is a need for accessible and appropriate information and support. In November 2020 Genetic Alliance UK ran a series of online workshops on gene and cell therapies for 20 participants with lived experience of rare, genetic conditions. The group consisted of individuals with a rare condition, of such individuals parents, family members and informal caregivers from all over the UK. A wide range of rare conditions was represented. The aims of the workshops were to ensure that the community is better able to understand gene and cell therapies and is empowered to:

- Inform gene and cell therapy development at all stages of the process
- Engage with regulatory and health technology assessment (HTA) bodies in relation to gene and cell therapies
- Make informed choices in research and clinical settings
- Advocate more broadly for gene and cell therapies.

Building on the momentum of this and similar activities, EuroGCT[1] was launched by the European Commission to 'provide reliable and accessible information about the use of cells and genetic material to treat disease for the public, patients, and researchers.' EuroGCT brings together patient representatives and science communicators with leading researchers, ethicists, lawyers and social scientists to develop material and foster engagement with gene and cell therapy development. Their website will be continually developed over the next four years.

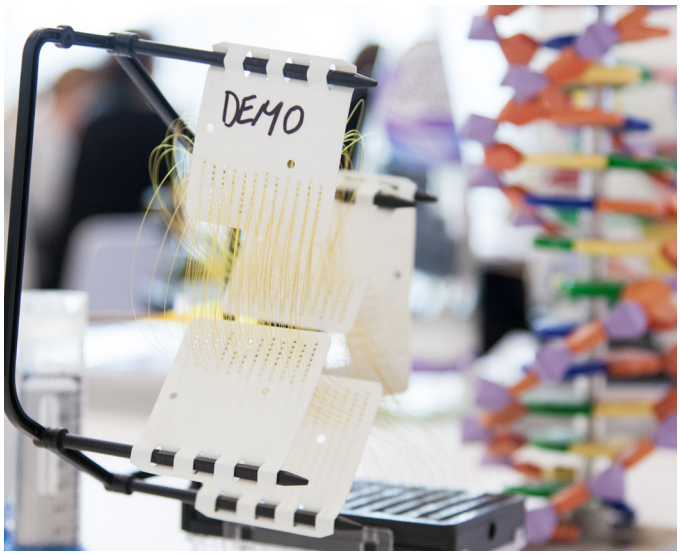
With the support of the Cell and Gene Therapy Catapult and the Advanced Therapy Treatment Centres (ATTC) Network[2], Genetic Alliance UK and EuroGCT joined forces in March 2022 to progress our understanding of the information needs of people living with rare conditions who wish to engage with gene and cell therapy progress.

[1]EuroGCT.org
[2]theattcnetwork.co.uk

GENE AND CELL THERAPY WORKSHOP MARCH 2022

Seven people took part in a lively discussion via Zoom following presentations by representatives of the Cell and Gene Therapy Catapult and EuroGCT. Lee Coney[3] presented an overview of gene and cell therapies and their complexities relative to 'standard' therapeutics. Amanda Waite[4] and Jennifer Lorigan[5] talked about the approach EuroGCT is taking in developing and presenting its materials, such as co-creation with intended audiences and gathering feedback to ensure their materials are well signposted from key places and easy for individuals to navigate.

Information about the participants' characteristics is given in annex 1.



DISCUSSION POINTS

Where do people have least understanding?

Participants made it clear that information about the technology, how it works and what it might achieve or what the limits are, is important. They felt least informed about questions such as what type of condition might be a target for which therapies, and whether and why some conditions are not suitable for gene and cell therapies. Information is needed around risks associated with gene and cell therapies, whether their delivery is restricted to specific organs and what effect they might have on the rest of the body.

As with any new technology, the use of technical language can be a barrier to PPI and people considering joining a trial or starting a treatment. Workshop participants flagged that, for example, clarity is needed around the relationship between common terms such as ATMPs[6] and 'gene and cell therapies', and gene editing versus gene therapies.

Underpinning the efforts to support people to engage with these new therapies is the need to ensure access to good information: participants reported being unsure about how to find the best sources of information, especially when they are not taking part in clinical trials.

What are the key questions people want to know the answers to?

Our participants are keen to understand the expected outcomes of gene and cell therapies in terms of both general expectations/hopes and what impact they will have on patients. The discussion demonstrated that individuals will have a lot of technical questions, such as understanding what neutralising antibodies are and their effect, why interpatient variability is seen, and how the therapies are made and delivered.

In addition, there is an appetite for very clear information in clinical trials listings to include whether the trial is for a gene or cell therapy, with links to further technical information such as how the therapy works. It was suggested that information about companies developing cell and gene therapies is also of interest to people living with rare conditions.

[3]Head of Non-clinical Safety at Cell and Gene Therapy Catapult
[4]Project Manager – Information and Networks at EuroGCT
[5]Information Officer – Gene and Cell Therapy at EuroGCT
[6]Advanced Therapy Medicinal Products – a legislative term referring to tissue-based therapies, such as gene and cell therapies, which involve substantial changes being made to those tissues (the term excludes e.g. organ transplants and blood transfusions).

How can the information be delivered?

Participants said they would like their questions - about the therapies, and what is coming in the pipeline – to be answered by their healthcare professionals and on the NHS website. Their experience suggests there is work to do to improve this route to support. Continued conversations with, and information developed by, patient organisations will also be valuable. A pre-workshop survey, along with discussion during the workshop, uncovered a mixed bag of experiences in terms of sources of information about participants' conditions: patient advocacy groups, scientific articles and healthcare professionals scored highly, and NHS and international websites, and message boards, scored lowest. Participants also noted that commercial therapy developers can be useful sources of information.

It was suggested that media training for scientists and healthcare professionals delivering information on a wider stage could be useful. The existence of patient and healthcare professional 'influencers' and bloggers on social media was flagged.

The benefits of delivering information and communicating about gene and cell therapies through both digital and in-person routes were acknowledged. Similarly, a wide range of formats was proposed to suit the preferences of different people: animations, short films and podcasts, written leaflets and glossaries, and lists of frequently-asked-questions with answers in plain English. This reflected responses to a question in the pre-workshop survey that indicated individual differences in preference for learning about something through reading, watching videos or 'being told'.

A key consideration in developing an approach to delivering information in the most useful way is to identify the intended audience: participants felt that this might extend beyond patients and their families to the general public and children (via schools).

If you have previously searched for information to do with your condition, what search terms have you used?

We suggested some types of search term that might be used by someone searching for information about their condition and therapies: most participants agreed they would use terms related to their specific condition and symptoms but no-one said that they would use a broader umbrella term for related conditions, or the word 'therapies'. One commented that 'most people wouldn't know to include the words gene or cell therapies'.

Other participants described sophisticated search methods, focussing on terms relating to the target of therapy (e.g. addressing the root cause or reducing disease progression), and 'drilling down': starting with a Google search, through Wikipedia to peer reviewed scientific literature, followed by consulting further articles referenced in the literature.

It was clear that lacking a diagnosis, and having to rely on broad symptomatic terms (such as 'global developmental delay') was a barrier to finding information. Similarly, participants described problems finding good search terms due to the 'fuzziness' of some syndrome definitions and areas of overlap with other conditions.

CONCLUSIONS AND NEXT STEPS

Genetic Alliance UK, EuroGCT and all those wishing to support individuals to become informed and better able to engage with gene and cell therapy development, should consider the following conclusions and questions arising from the workshop discussion.

Resources should be aimed at defined audiences, in terms of content, format and tone, and there are nuances that need to be taken into account such as difficulties in digesting complex information (e.g. for people with fatigue or learning difficulties), and the stage people are at (e.g. newly diagnosed or settled into 'living with a condition'). Co-production with target audiences is advised.

- How can those who are less engaged (i.e. not part of a support group or clinical trial) be reached?
- Resource providers need to be clear about whether they are simply providing resources, or actively 'upskilling' certain stakeholders.
- Prioritising which specific therapies are covered by information providers such as EuroGCT will need to be pragmatic – there are over 6,000 known rare conditions – and driven by what is in developer pipelines.
- Conversely, information providers need to be clear that they are not providing a database of all available clinical trials, but have a role to help people ask the 'right questions' of trial coordinators.
- Do providers such as EuroGCT have a role in actively standardising the language and terminology used around gene and cell therapies?
- When providing background information about rare conditions, information providers should consider whether to develop new material with partner organisations or simply signpost to them. A case-by-case approach may be necessary.
- There are too many 'kitemark' schemes for a single scheme to provide sufficient endorsement of the quality of gene and cell therapy information. Organisations such as EuroGCT need to consider how to become the trusted source and build brand recognition and credibility, for example through developing relationships with partner organisations such as the NHS in the UK, and support groups/patient advocacy organisations.
- Information providers need to adopt a strategy that harnesses existing expertise but avoids duplicating what others are doing.

Following the workshop, EuroGCT has undertaken to implement the following actions:

- Following on from the development and update of the EuroGCT website's core reference material, EuroGCT will develop additional 'plain language' formats of priority content, taking into account the recommendations on accessibility and defined audiences. Where possible, this will involve co-creation with target audience members and the incorporation of patient stories.
- EuroGCT will build links with healthcare providers and organisations that are directly in contact with people affected by conditions, alongside our ongoing work to ensure reliable information is findable by people searching online for information about their condition. They will continue to seek opportunities to reach less engaged and under-represented audiences.

- EuroGCT's remit is information provision, but they will partner with others who are in a position to upskill stakeholders, including via ATMP Engage[7]. They will aim to build capacity in this process by sharing best practice.
- EuroGCT will be responsive to developments in the therapy pipeline in its prioritisation of content. Where information about development for a specific indication may have future relevance for a wider range of conditions, they will aim to direct people to this.
- EuroGCT will continue to develop its resources to enable informed decision-making in relation to trials, also taking into account accessibility of language and formats. While they are not in a position to improve equity of access to trials, they can aim to disseminate information beyond trial hub centres.
- EuroGCT will aim to use consistent language with clear definitions in its content, and also to explain other terminology that may be used in different contexts.
- EuroGCT will continue to map and signpost existing relevant information.
- EuroGCT will continue to develop relationships with trusted sources of information, including NHS and support groups/patient advocacy organisations, with the aim to build trust in the EuroGCT website.
- EuroGCT will aim to avoid duplication through ongoing mapping and networking activities, and will signpost outputs of other relevant organisations, groups and projects to bring resources together in a single information hub.

ANNEX 1

Workshop participant characteristics

Sex:	Five female; two male
Age:	Two were 25-34; 3 were 45-54; one was 55-64; one was 65-74
Ethnicity:	Six white English; one other white background
Region:	All lived in England
Experience:	Three had a health condition; four were parents/carers of someone with a health condition; in addition, three worked for charities representing people with a health condition
Condition type:	All participants' experience was with a diagnosed, rare genetic condition; three had experience of more than one condition
Education:	One was educated to A level or equivalent; four to graduate level (e.g. BA); one to post-graduate level; one preferred not to say

[7]eurogct.org/atmp-engage