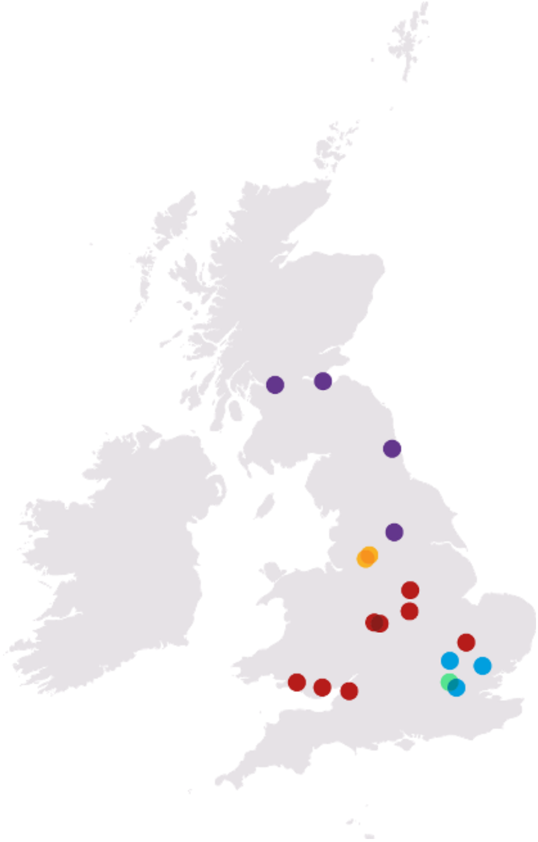
Advanced Therapy
Treatment Centres

LONDON
ADVANCED
THERAPIES

Patient and Public Engagement in the development of cell and gene therapies – why it matters and how to engage with people



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INDUSTRIAL
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Cell and Gene Therapy

Outline of today's webinar

What we will cover today

1. **Why patient and public involvement matters**
2. **Some of the unique challenges** for patient and public involvement in ATMPs
3. **Spotlight on real life:** hearing from patients and researchers
4. **Principles and ideas for high quality involvement**
5. **Practical tips** and tools to help you involve people
6. **Q&A**

This presentation has been developed by the national ATMP Patient and Public Involvement Working group.

The Working group is a network of research, provider, charity, industry, policy and other organisations, with people with lived experience as core members of the group. The working group is part of the NIHR-led national co-ordinating group for ATMPs.

Laurence Woollard Webinar Chair, Director of On The Pulse consultancy and patient partner

Alice Williams, Head of Patient and Public Involvement, Innovation, research, and life sciences team, NHS E/I

Dr Emma Lane, Lane Lab and LEARN Study Group Leader, Cardiff University

Cheney Drew, Research Fellow and Senior Clinical Trials Manager Cardiff university

Director of Research, Genetic Alliance UK

Lesley Gosden, Patient Partner

Astrid Burrell, Patient Partner

Nick Meade, Joint Interim CEO, Genetic Alliance UK

Joanna Calder, Patient partner

Sound familiar?

'As a researcher, I don't often meet with patients.'

'I have ideas to improve your trial design, some simple changes would make a huge difference to me'

'Clinicians have a huge body of expertise, but so do I. I have a lifetime of expertise of living with my condition!'

'How do we focus our research on what matters to patients?'

'I need to know that someone like me has been through this before.'

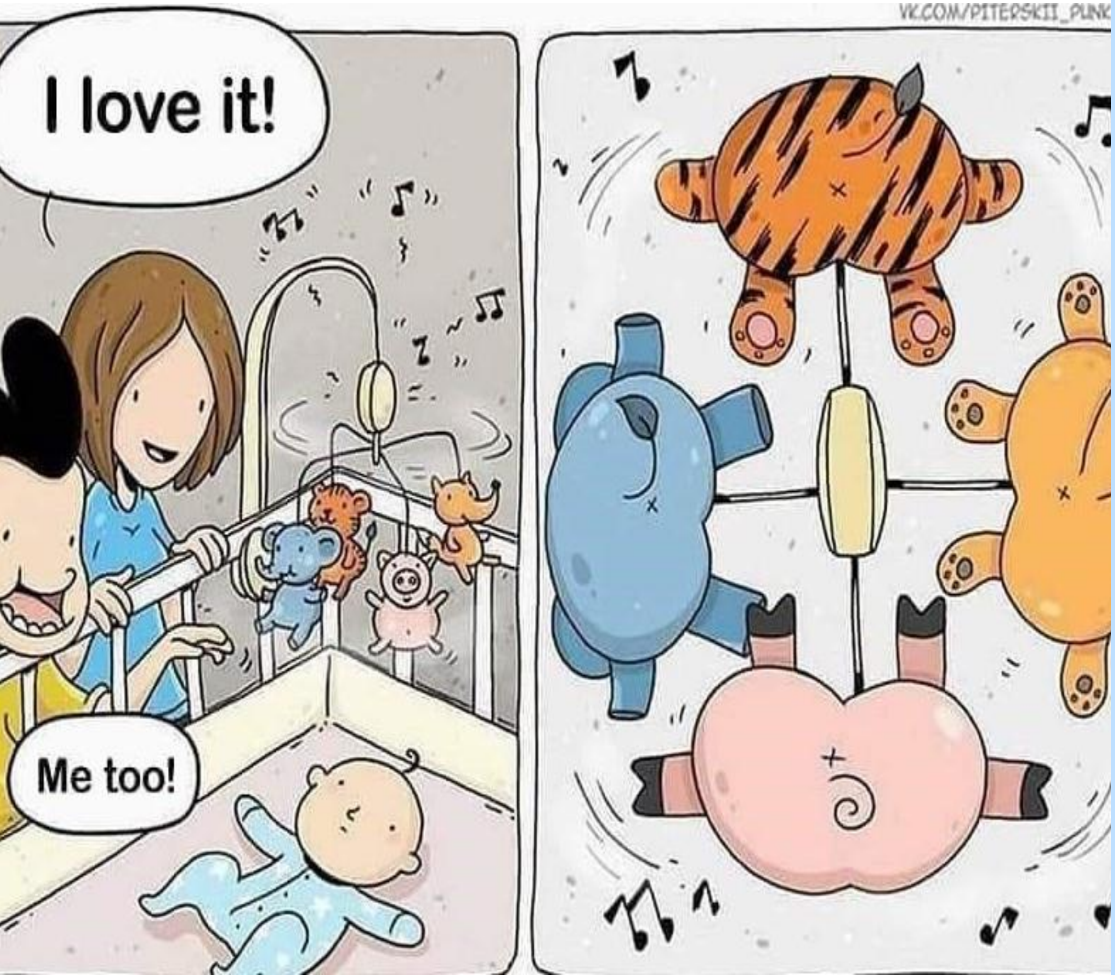
'Who can help me develop really good information for my patients?'

'I don't really know where to start to involve patients in my work'

'Will my family get the information and support they need while I am having treatment?'

'Too often trials measure what's measurable rather than what's meaningful to people living with this condition!

What do we mean by patient and public involvement?



Patient and Public Involvement is:

- a **collaborative approach** to co-creating patient-centred treatments, services and research.
- listening to, learning and **improving things together**.
- patients and the public. **People with lived experience of conditions, families and carers, patient advocacy organisations (community groups and charities).**
- '***Nothing about me, without me'.*** From bench to bedside: in research, treatment planning, commissioning, and service delivery.

Why does it matter?

- Growing evidence shows benefits of involving people in **clinical research**;
  - increased quality (design, protocols, information)
  - real world relevance of research,
  - improved recruitment and retention,
  - Stronger funding applications
  - Increased credibility
  - Greater public awareness of the research field
- Similarly for **mainstreamed treatment pathways**:
  - Improved delivery and real world 'fit'
  - Improved patient and family understanding and engagement
  - Better education and information resources

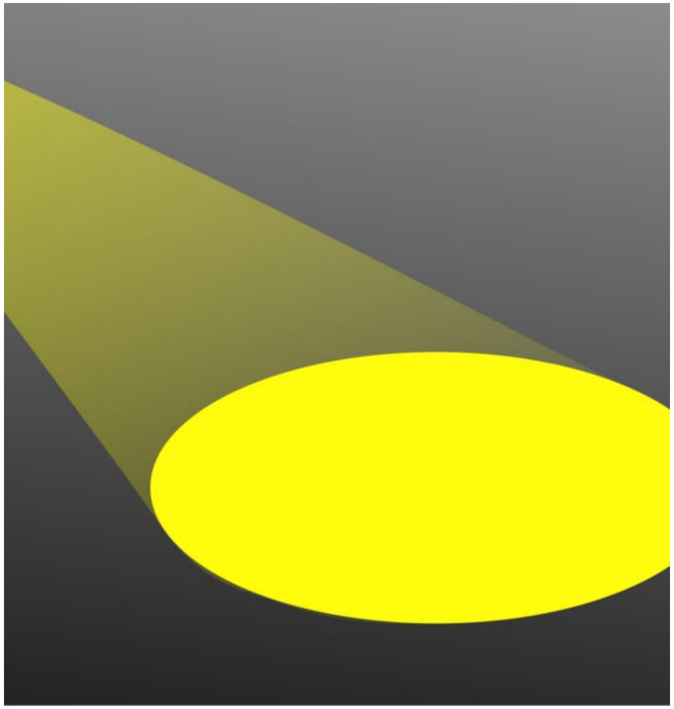
- The uptake of these therapies requires appropriate patient and public engagement and buy-in. There is *a wide gap in knowledge and awareness about cell and gene medicine* (ARM Foundation).
- **Public opinion matters** (accurate or not).
- It shapes policy and political responses, which in turn impact funding, regulation and delivery bringing specific risks where the science is advancing faster than public opinion.

Additional considerations for cell and gene therapies

- Novel therapies
- Terminology
- Significant long term implications (including on family/carers)
- Complexity of informed decision making
- Equity of access (to research participation as well as involvement)
- Science is moving quickly; information and discussion with patient communities is vital, not just for informed communities, but also to avoid/ address myths
- Variation in involvement and tolerance of risk across conditions and patient communities
- Transparency of implications for participation in future trials

Spotlight on real life

Astrid Burrell, Lesley Gosden, Emma Lane and Cheney Drew



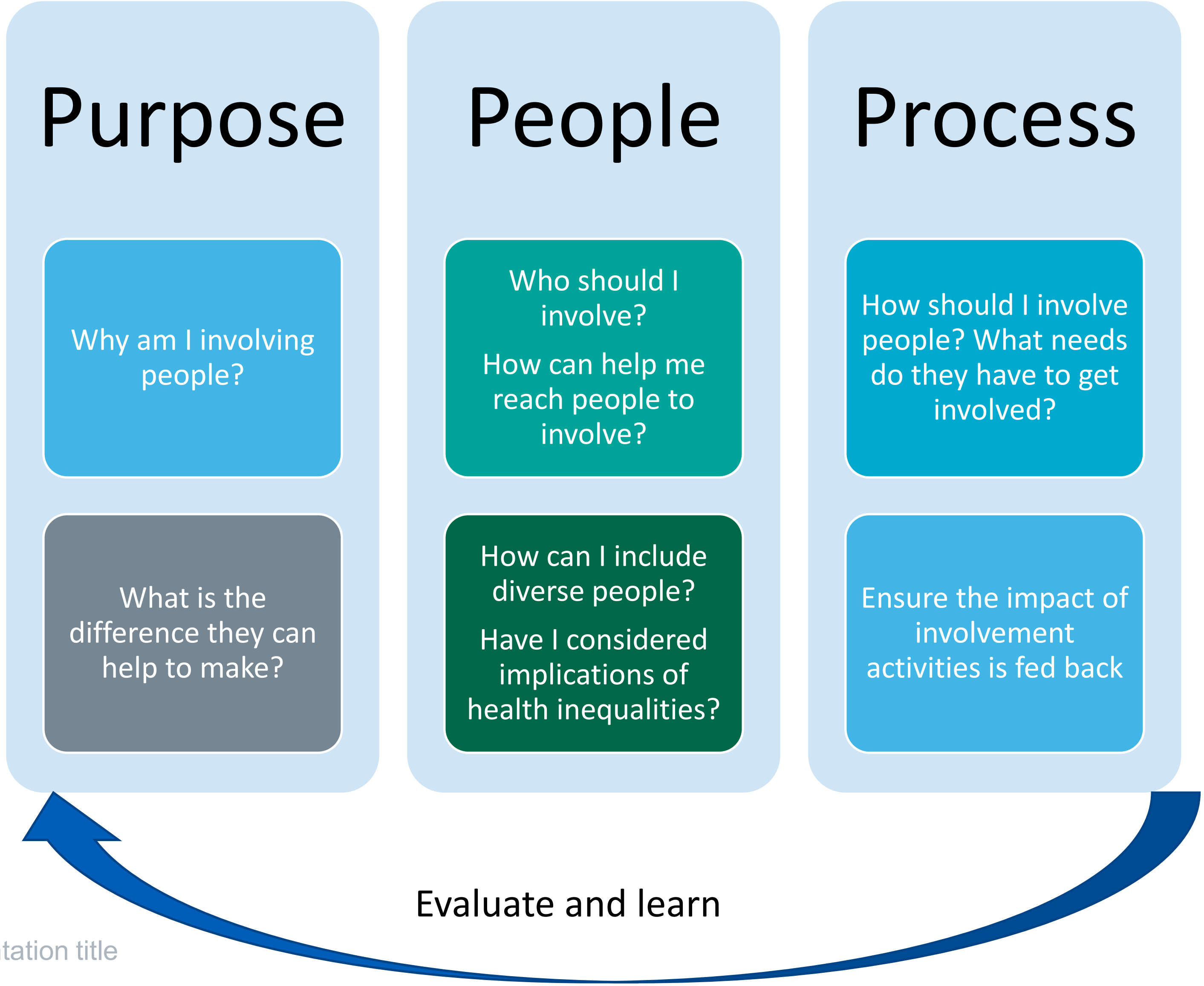
video

Principles for high quality involvement

- **It starts with people** (how would you or your family members like to be treated if you were to get involved with developing new treatments?)
- **Be honest**, transparent about the scope of the work and what involvement is expected to impact.
- Help people to **get involved effectively** – meet access needs and think about inclusivity.
- **One is not enough**. Involve a diversity of people. Include carers and families.
- **Value people's input** (recognise their contributions, be clear about what support or remuneration is on offer to enable their involvement).
- **Listen and act.** Or be honest about why something won't be acted on. Ensure people know how they have made an impact
- **Grow;** learn and improve

BEWARE: Doing this poorly can sometimes be worse than not doing it at all – it risks people becoming really disillusioned; and **sharing their experience across often small patient communities**, making people more reluctant to get involved.

Where to start?



Purpose

Why am I involving people?

What is the difference they can help to make?

People

Who should I involve?

How can help me reach people to involve?

How can I include diverse people?

Have I considered implications of health inequalities?

Process

How should I involve people? What needs do they have to get involved?

Ensure the impact of involvement activities is fed back

Evaluate and learn

Involvement approaches to consider

Raising patient & public awareness

Governance roles and critical friend

A joint strategic vision

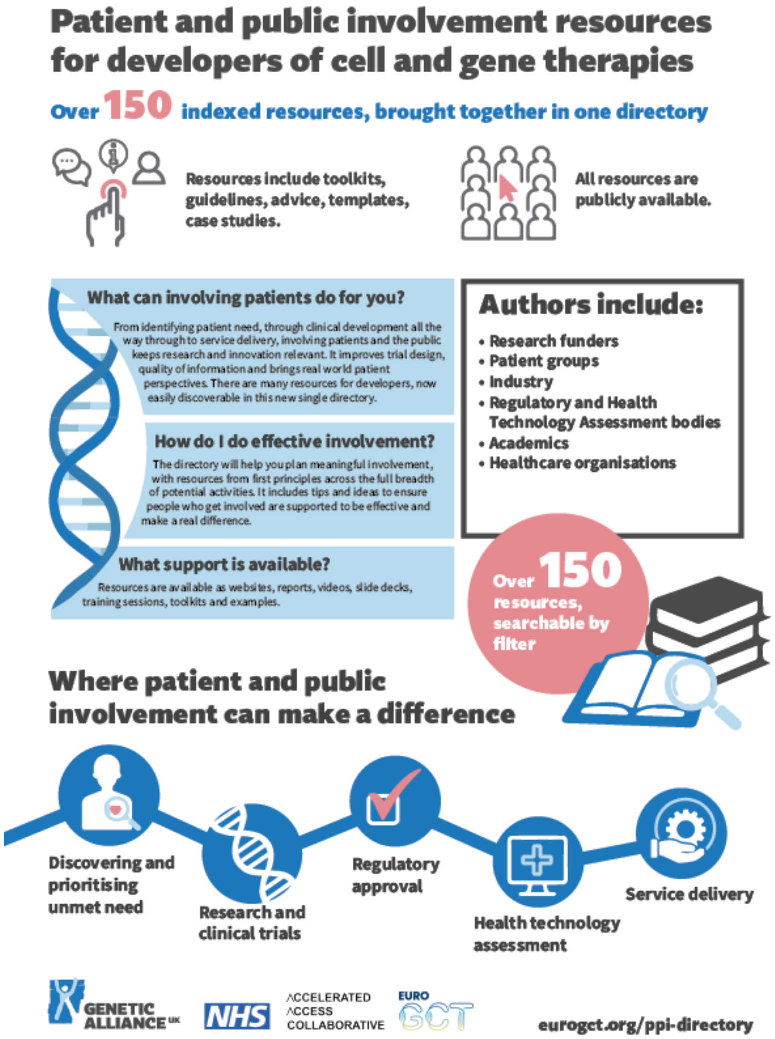
Expert through experience ideas

Real world testimony

- Involving people in oversight/policy/ formal working groups,
- Involving people in specific project work e.g design groups, funding bids, development of patient information
- What insight exists? What are people saying? What needs are they identifying? Explore social media ('social listening' exercise)
- Holding focus groups / workshops / surveys to explore ideas / experiences / need
- Hold a range of individual conversations with people with lived experience, include families and carers
- Identify the impact of health inequalities – reach out to specific groups
- Develop a patient advisory group
- Build links with relevant patient advocate organisations (charities, community groups)
- Build relationships and networks with people
- **ONE SIZE DOES NOT FIT ALL – use a range of approaches appropriate to the work.**

Practical advice and tips

- Recently collected directory of over 150 relevant and publicly available resources
- Resources include guidelines, advice, toolkits, templates and case studies...
- …covering the whole development pathway from identifying unmet need, to research design to technology appraisals to frontline delivery.
- The resources are from academia, industry, research funders, health organisations, charities, patient groups and others.
- Ultimately: use of these resources will ensure that therapies are informed by real world needs; better able to meet patient and family needs.
- The directory will be live at www.eurogct.org/ppi-directory in a few weeks.



Patient and public involvement resources for developers of cell and gene therapies

Over 150 indexed resources, brought together in one directory

Resources include toolkits, guidelines, advice, templates, case studies.

All resources are publicly available.

What can involving patients do for you?

From identifying patient need, through clinical development all the way through to service delivery, involving patients and the public keeps research and innovation relevant. It improves trial design, quality of information and brings real world patient perspectives. There are many resources for developers, now easily discoverable in this new single directory.

How do I do effective involvement?

The directory will help you plan meaningful involvement, with resources from first principles across the full breadth of potential activities. It includes tips and ideas to ensure people who get involved are supported to be effective and make a real difference.

What support is available?

Resources are available as websites, reports, videos, slide decks, training sessions, toolkits and examples.

Authors include:

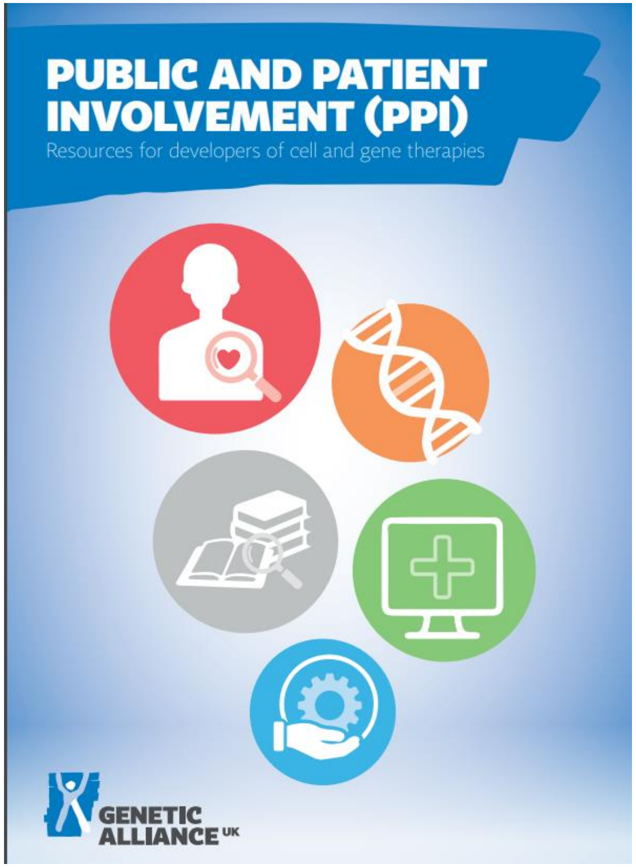
- Research funders
- Patient groups
- Industry
- Regulatory and Health Technology Assessment bodies
- Academics
- Healthcare organisations

Over 150 resources, searchable by filter

Where patient and public involvement can make a difference

GENETIC ALLIANCE UK · NHS · ACCELERATED ACCESS COLLABORATIVE · EURO GCT

eurogct.org/ppi-directory



PUBLIC AND PATIENT INVOLVEMENT (PPI)

Resources for developers of cell and gene therapies

GENETIC ALLIANCE UK

Useful links

Patient and public involvement links	Cell and Gene therapy /research material
• EuroGCT.org/PPI-Directory – this link is not yet live • Moving beyond box-ticking and lip service – why patient involvement matters in a new era of ATMPs for rare diseases • NIHR Resources for involving people in research programmes • UK Standards for Public Involvement in research • The Participation toolkit – health Improvement Scotland • NHS England and Improvement's get Involved resources • Quality engagement | NCCPE (publicengagement.ac.uk) • 4Pi Involvement Standards - NSUN website • Why does it matter? Healthtalk videos • How can PPI help your study – videos from Parkinsons UK • Public involvement in health research: what does 'good' look like in practice?	• Patient and public perspectives on cell and gene therapies: a systematic review • Improving informed consent for haemophilia gene therapy: a case for change • Advanced therapy Treatment Centre network webinar series • BSGCT webinar series • Involving patients in research, we can and should (esgct.eu) • Advanced Therapy Medicinal Products (ATMPs) Roadmap (abpi.org.uk)

Thank you for listening.

Any questions?

Working Group members

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NHS

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Cell and Gene Therapy

Cystic Fibrosis Trust

Johnson & Johnson

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Welsh Blood Service

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Midlands-Wales Advanced Therapy Treatment Centre

British Society for Gene & Cell Therapy

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