PUBLIC AND PATIENT INVOLVEMENT (PPI)

Resources for developers of cell and gene therapies

GENETIC ALLIANCE UK

ABOUT GENETIC ALLIANCE UK

Genetic Alliance UK is the largest alliance of organisations supporting people with genetic, rare and undiagnosed conditions in the UK. Our 200+ members and the people they support are at the heart of everything we do.

We advocate for fast and accurate diagnosis, good quality care and access to the best treatments. We actively support progress in research and engage with decision makers and the public about the challenges faced by our community.

We run two long standing projects:

Rare Disease UK, a campaign focused on making sure the new UK Rare Diseases Framework is as successful as possible, and to ensure that people and families living with rare conditions have access to a final diagnosis, coordinated care and specialist care and treatment.

SWAN UK (syndromes without a name), the only dedicated support network in the UK for families that have a child or young adult with an undiagnosed genetic condition.

This work was carried out by Genetic Alliance UK and was commissioned by the Accelerated Access Collaborative.

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Creative Works
7 Blackhorse Lane
LONDON
E17 6DS
Email: contactus@geneticalliance.org.uk
Website: geneticalliance.org.uk

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Author: Amy Hunter, Director of Research, Jennifer Jones, Research Associate, Sophie Peet, Policy Analyst, Matt Hamer, Communications Officer. In citations please list report author as Genetic Alliance UK.

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FOREWORD

Involving patients and carers in the research and development of new therapies is vital. The end product is more fit for purpose, supporting a better patient experience and outcome.
Alice Williams, NHS England and NHS Improvement.

The Early Stage programme is part of the Accelerated Access Collaborative. This programme supports potentially highly effective therapies and products through the regulatory and approvals process, and recently has had a spotlight on gene, cell and tissue therapies, often collectively known as Advanced Therapy Medicinal Products (ATMPs).

The ATMP Patient and Public Involvement and Engagement (PPIE) Working Group includes a wide range of stakeholders and patient partners focused on improving opportunities for patients and researchers to work together, and raising the profile of ATMP therapies with patient organisations. We aim to be champions of patient and public involvement in health research, especially in these pioneering fields. We are delighted to launch the directory of patient and public involvement resources for developers of ATMPs. The directory can be used to find practical guidance to enable stronger and more meaningful patient involvement to facilitate better outcomes.

We are grateful to the authors who have delivered a tremendous resource. It not only points us to valuable materials and guidance, but also highlights areas where more can be done to ensure meaningful and comprehensive involvement. While a wealth of patient and public involvement tools were identified to support health research this hasn't yet cross-pollinated to the specific environment of ATMPs.

The community represented within the ATMP PPIE Working Group still has work to do to ensure patient and public involvement becomes a core way of working to those developing ATMPs. Part of this is a familiar message underlining the benefits of early engagement with people living with health conditions in research and treatment delivery – the other part is specific to ATMP development where effective PPIE is vital.

ATMPs include medicines that are truly changing the paradigm of what treatments can achieve and how they work. The idea of chronic use of medicines, or coming off treatment, do not necessarily apply – this fundamentally changes the questions patients and their families must ask themselves when consenting to treatment. When developers genuinely involve patients throughout the development pathway, we can expect research to be truly informed by patient need, trials to be patient centred and their decisions to be properly informed.

N.Meade

Nick Meade
Joint Interim CEO, Genetic Alliance UK and Co-Chair of the ATMP PPIE Working Group

A Williams

Alice Williams
Head of Patient and Public Involvement (Innovation, Research and Life Sciences),
NHS England and NHS Improvement and Co-Chair of the ATMP PPIE Working Group

SUMMARY

There is considerable government support for innovation within the NHS, exemplified by the creation of the Accelerated Access Collaborative (AAC) which is facilitating development of advanced therapy medicinal products (ATMPs) such as gene and cell therapies.

A key commitment of the AAC is to integrate public and patient involvement in each area of its work. Such involvement aims to give a voice in research to those with lived experience – to ensure that efforts and investments are focussed on real unmet needs, that research approaches are cognisant of public and patient realities, and to foster understanding and relationships between developers and the ultimate beneficiaries.

There is a need to support ATMP developers to incorporate meaningful public and patient involvement. Despite the existence of few toolkits or guidelines aimed specifically at ATMP R&D, there is a large body of resources, publicly available, that are relevant. The AAC, through the ATMP Public and Patient Involvement and Engagement (PPIE) Working Group, commissioned Genetic Alliance UK to identify and catalogue relevant resources into a single, navigable directory.

The directory – which is available as a user-friendly interface on the EuroGCT website, and a downloadable spreadsheet for Future NHS account holders – includes information about and links to toolkits, guidelines, advice, templates and case studies. They originate from a wide variety of organisations including industry, academia, research funders, health organisations and patient groups.

The directory can be found on the EuroGCT website at www.eurogct.org/ppi-directory.

This report provides a brief guide to using the directory, outlines how it was developed and describes the gaps in available resources that became apparent.

The infographic overleaf was created to promote the directory and can be downloaded at www.eurogct.org/ppi-directory.

Genetic Alliance UK would like to thank the members of the ATMP PPIE Working Group for their support and insights during the development of the directory, and EuroGCT for hosting the directory and developing the online interface.

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?

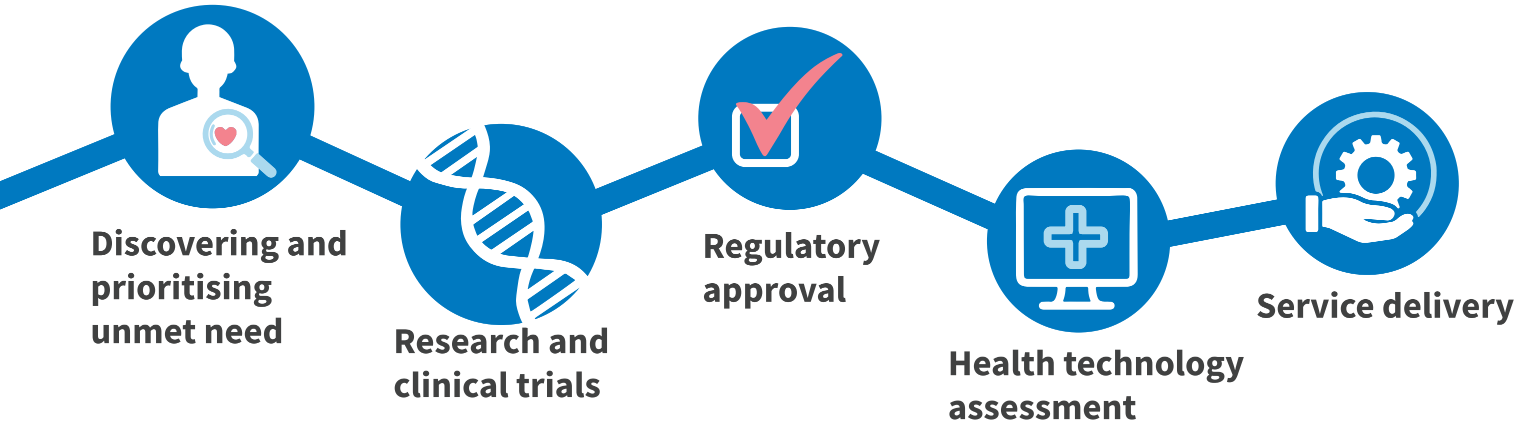
Resources are available as websites, reports, videos, slide decks, training sessions, toolkits and examples.

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



Discovering and prioritising unmet need

Research and clinical trials

Regulatory approval

Health technology assessment

Service delivery

GENETIC ALLIANCE UK

NHS

ACCELERATED ACCESS COLLABORATIVE

EURO GCT

eurogct.org/ppi-directory

TERMINOLOGY

Many resources blur ‘involvement’ (active partnerships between individuals and researchers/developers, which allows the research to be influenced by the individuals), ‘participation’ (subjects recruited into research studies), and ‘engagement’ (dissemination and championing of research to the public). There are some commonalities, for example the need to consider diversity when recruiting, but careful use of terminology is helpful due to the many distinct roles and needs associated with these different types of activities.

Our focus for populating the directory was on resources that support involvement, but given the fluid use of definitions many of the materials that are included also cover participation and engagement activities.

It is helpful to be aware that some authors are using other terms altogether, such as ‘commercial involvement’ rather than public and patient involvement. This is consistent with the general move away from use of the term ‘patient’ for people when they are not actually in hospital or otherwise receiving treatment. In the US, individuals taking part in involvement activities are sometimes simply referred to as patient advocates.

GUIDE TO USING THE DIRECTORY

The directory contains information about and links to over 150 publicly available resources – toolkits, guidelines, advice, templates and case studies. It is available through an easy-to-use online interface on the EuroGCT website, and as a downloadable spreadsheet for holders of a Future NHS account. In both cases the directory can be navigated with filters which allow users to narrow down resources to those most relevant to their needs.

For example, there are filters for: ‘elements of PPI’ (roles and responsibilities, how to recruit individuals etc); type of research (general biomedical, pharmaceutical, ATMPs etc); stage of development (prioritising patient needs, R&D, regulatory approval etc) and so on.

In addition to indexed resources, the directory contains two additional types of resource:

- Information about and links to larger ‘repositories’ of information (such as the NIHR website that includes around 170 resources including training sessions). These are searchable web resources.
- Links to academic journals that have a focus on PPI.

HOW THE DIRECTORY WAS DEVELOPED

Collection

The flow chart overleaf outlines the process that was followed to identify and select the resources in the directory.

Potential resources were found through extensive stakeholder input and structured searches. Searches focussed on the Web of Science database[1] and the Patient Experience Library[2], using combinations of the following search terms:

- Advanced Therapy Medicinal Products; ATMP
- patient; public; service user; client; PPI
- involvement; engagement; contributor
- guidelines; toolkits; resources; toolbox

The focus was on resources from the UK, but where there were specific gaps in UK-based material (e.g. for regulators), the search was widened to Europe, then North America, then the rest of the world.

1. https://www.webofscience.com
2. https://www.patientlibrary.net/cgi-bin/library.cgi

The process that was followed to identify and select the resources in the directory.

Stakeholder input: NHSE/I ATMP PPIE working group

Literature/web search

Representatives of: NHSE/I; NHS Wales; Cell and Gene Therapy Catapult; NHS Foundation Trusts; NIHR; Health Innovation Network; health charities/patient groups; universities; industry.

Authored by: regulatory bodies; health organisations; professional societies and colleges; other independent associations; education and patient advocacy organisations; academic institutions; funding bodies; pharmaceutical companies; health charities/patient groups.

LONG LIST OF TARGET WEBSITES AND ONLINE DOCUMENTS

Search terms applied within websites/ documents

Further websites/ documents referenced in target sites added to long list

Comb websites for resources and ATMP-specific information

SHORT LIST OF RESOURCES

Exclude: publication earlier than 2010; not publicly available; solely about 'engagement' not 'involvement'

FINAL LIST OF 150+ RESOURCES

Separate out: peer-reviewed articles and large online repositories

Logged and indexed

Logged as separate resource types

Indexing

Analysis of the content of the first tranche of resources collected gave rise to a set of tags for indexing. The tags were organised into six groups and this framework was reviewed by stakeholders on the ATMP PPIE working group. The resources in the directory were then indexed using the agreed framework, which is detailed in the table below/overleaf.

Topic	Index tag	Notes
PPI elements	– What is PPI & benefits – Standards for PPI – Strategies / statements / policies on PPI – How / who to recruit[1] – PPI pools / lists of organisations – Roles and responsibilities[2] – Budgeting / compensation – Project planning – running activities[3] – Case studies, examples of good practice – Maintaining / supporting PPI – Training[4] – Knowledge building – setting the context[5] – Communication / plain language / glossaries – Using technology – Feedback to / from PPI – Evaluating / recording PPI impact	1. Expressions of interest forms, application forms, interview questions, involvement agreements, codes of conduct, rules of involvement, complaints procedures, adverts, addressing Equality, Diversity and Inclusion (EDI), guidance on eligibility, conflicts of interest. 2. Includes job descriptions. 3. Includes methods for engaging individuals, how to run panels etc. 4. Includes generic training for participants and specific training on e.g. analysis, commenting on research proposals, speaking up in committee meetings. 5. Includes partnership working, capacity building (e.g. plain English explanations of processes / how systems work).
Type of research aimed at	– Research in general (including health and social care) – Biomedical / lab research in general – Pharmaceutical – ATMP / gene therapy – Other field	
Organisation of origin	– Funder – Academia – Healthcare organisation – Regulatory body – Health Technology Assessment organisations – Industry – Third sector / charity / patient advocacy / education – Other organisation	

Development pipeline stage	– Identifying patient need / prioritising – Research and clinical development – Regulatory approval – Health technology assessment – Service delivery post authorisation	
Target audience	– Researchers / developers – Public / patients – Charity sector – Industry – Other group	
Country of origin	– UK – Europe – North America – Other country	

RESOURCE GAP ANALYSIS

The numbers of resources collected for the directory were compared across the indexing framework to identify gaps where no or few resources are available. The outcome is summarised in the table below.

Topic	Gaps
PPI elements	– The vast majority are non-ATMP-specific resources, but they are often transferable. However, resources with a tight focus on involvement (distinct from engagement and participation) for ATMP developers are lacking. – Gaps exist in specific training resources for PPI participants to build knowledge about how different stages of development work (especially regulatory approval, HTA and service delivery research) and how they can contribute. – Gaps exist around how to collect information from PPI participants about their knowledge, training and support needs.
Type of research aimed at	– There are fewer resources aimed at basic biomedical research, pharmaceutical development, ATMP development and delivery.
Organisation of origin	– There are fewer resources from regulatory bodies, HTA bodies and industry. Transparent industry case studies would be useful as examples where PPI has worked well.
Development pipeline stage	– There are fewer resources targeted at the following stages: regulatory approval, HTA and ATMP-specific service delivery research.
Target audience	– There are fewer resources aimed at industry. Transparent industry case studies would be useful as examples where PPI has worked well.

SUGGESTED ACTIONS FOR READERS

With over 150 indexed resources, this directory is a valuable tool for researchers and developers wherever they are on their journey to embed meaningful patient and public involvement in their work. Stakeholders rightly expect a research culture that involves patients at each stage and as standard. To help this become a reality we encourage readers to:

- Use the filters in the directory and find something that works for you.
- Celebrate your involvement work!
- Share this resource widely – it has applicability across the research and development pathway and can support many areas of health research.
- If you work in ATMP development – consider how your own practice might bring insight to others, and how you could share your knowledge – there is a real need for practical ATMP-specific resources.

To find out more about the ATMP PPIE Working Group, email england.aacppi@nhs.net