

Clinical Adoption Day 2020

Implementing innovation in clinical trials and beyond; to advance institutional readiness and routine adoption of ATMPs

Workshop report



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On Thursday 26th November 2020, representatives from across the UK cell and gene therapy ecosystem came together at the third annual Clinical Adoption Day workshop to identify strategies and solutions to implementing innovation in clinical trials and beyond; to advance institutional readiness and routine adoption of Advanced Therapy Medicinal Products (ATMPs) in the UK.

This report summarises the key findings and conclusions from this year's workshop.

Organisations in attendance:

- Achilles Therapeutics
- Adaptimmune Therapeutics
- Aptus Clinical
- Association of the British Pharmaceutical Industry
- Autolus Therapeutics
- BioIndustry Association (UK)
- Birmingham Women's and Children's NHS Foundation Trust
- bluebird bio
- Bristol Myers Squibb
- Cell and Gene Therapy Catapult
- Centre for Patient Reported Outcome Research, University of Birmingham
- Cytiva
- Dignio
- Gilead Kite
- GlaxoSmithKline
- Health Research Authority
- Imperial College London
- Innovate Manchester Advanced Therapy Centre Hub (iMATCH)
- Innovate UK
- Instil Bio UK
- King's College London
- Knowledge Transfer Network
- Leeds Teaching Hospitals NHS Trust
- London Advanced Therapies
- Manchester University NHS Foundation Trust
- MedCity
- Medicines and Healthcare products Regulatory Agency
- Midlands Wales Advanced Therapy Treatment Centre (MW-ATTC)
- National Institute for Health and Care Excellence
- National Institute for Health Research
- Newcastle Advanced Therapies Newcastle University
- Newcastle Hospitals NHS Foundation Trust
- Newcastle University
- NHS Blood and Transplant
- NHS England
- NHS Greater Glasgow & Clyde
- NHS Improvement
- NHS Lothian
- NHS Scotland
- NHS Wales
- NIHR Office for Clinical Research Infrastructure
- Northern Alliance Advanced Therapies Treatment Centre (NA-ATTC)
- Nottingham University Hospitals NHS Trust
- Novartis
- OVID Health
- Pfizer
- Scottish National Blood Transfusion Service
- The Christie NHS Foundation Trust
- Thermo Fisher Scientific
- TrakCel
- Transport Genesis
- UK Research and Innovation
- University College London Hospitals NHS Trust
- University Hospitals Birmingham NHS Trust
- University Hospitals Bristol and Weston NHS Foundation Trust
- University Hospitals of Leicester NHS Trust
- University of Birmingham
- University of Edinburgh
- University of Manchester
- Welsh Blood Service
- World Courier



The UK represents **12% of global ATMP trials***, making it a **leading hub of activity** for advanced therapies. However, there remains a need to **address the challenges that are brought about by the complexity of designing and delivering ATMP trials**, including generating appropriate data required for regulatory bodies and health technology assessment (HTA).



These challenges include ensuring the UK remains a **competitive and economically viable** place to set up and deliver ATMP clinical trials, creating an environment which enables **global decision makers to prioritise the UK** and building an understanding of **how best to meet regulatory and HTA requirements**.



The Advanced Therapy Treatment Centre (ATTC) network, in collaboration with industry, the NHS and regulatory bodies is working to overcome some of these challenges. **This report outlines further proposed solutions and makes recommendations on how best to deliver these**. If taken forward, the solutions in this document would help create a better resourced and easier to navigate trial landscape for advanced therapies in the UK.

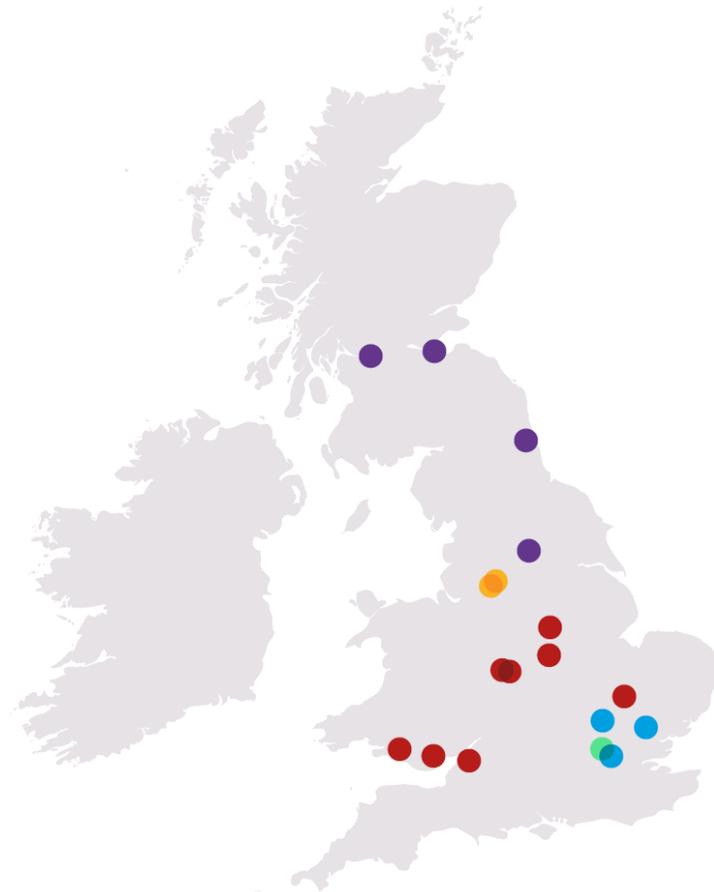
Three challenge areas were discussed to address implementing innovation in ATMP clinical trials; to advance institutional readiness and routine adoption of cell and gene therapies:

- Ensuring the UK remains a competitive and economically viable place to set up and deliver ATMP clinical trials
- Creating an environment which enables global decision makers to prioritise the UK
- Successfully leveraging available clinical data for HTA

Participants agreed on strategies to overcome these challenges and recommendations to be taken forward:

1. Clinical and regulatory stakeholders to **increase resources** to support clinicians and industry to navigate the set-up of trials, including support for rapid trial set-up.
2. All ATMP stakeholders to continue **building and maintaining the positive collaboration work** between all parties to enhance the acceptance and routine use of innovative clinical trials and ATMP adoption mechanisms.
3. Manufacturers, regulatory bodies, NICE and NHS **to work together** to implement strategies to mitigate against ATMP trial data challenges and optimise the HTA process.

The ATTC network



- Develop and deliver systems for the routine delivery of these disruptive therapies
- Disseminating the learnings and systems from the ATTC clinical centres to enable more widespread adoption of these innovative therapies across the NHS
- **64 partners** – true collaboration between NHS, industry and academia
- **200 – 250 people** working on the programme
- Working in collaboration with London Advanced Therapies

Highlights from the ATTC network in 2020



Ramping up national ATMP education and training initiatives: developed standardised e-learning modules and held education days to build a better understanding of ATMPs for both clinical and non-clinical staff, enabling them to communicate the benefit of trials to patients and their families.



Developing patient-focused resources: developed educational resources, established advisory boards and held focused events which are designed to support patients to navigate advanced therapy trials and participate in the design of innovative clinical trial protocols.



Growing institutional readiness: developed a novel assessment tool for measuring institutional readiness and a toolkit of best practice documents designed to fulfil service gaps, supporting hospitals to bolster their ability to engage with UK-wide advanced therapy trials.

Coordinated by

CATAPULT
Cell and Gene Therapy

ATTC network priorities for 2021-2022



Logistics/Supply Chain



Digital and Data



Clinical Trial Acceleration



Standardisation and Best Practices



Health Economics



Pharmacy and Cell Labs



Education



Institutional Readiness



Public Involvement and Engagement



Regulatory



Patient Reported Outcome Research



Rapid Alert – Adverse Events

Advancing institutional readiness and routine adoption of ATMPs by implementing innovation in clinical trials

Priorities and recommendations

Priorities identified to drive future progress and support further scale-up of ATMP clinical trials to maintain the UK's leadership:

1

Ensuring the UK remains a competitive and economically viable place to set up and deliver ATMP clinical trials

2

Creating an environment which enables global decision makers to prioritise the UK

3

Successfully leveraging clinical data for HTA

1

Ensuring the UK remains a competitive and economically viable place to set up and deliver ATMP clinical trials

Regulatory



Piloted joint MHRA and HRA submission and approval process should become standard.

MHRA are open to innovative trial designs using nonstandard data sets.

Developers can be guided by the **Innovative Licensing and Access Programme (ILAP)** which brings together key stakeholders and provides a toolkit to facilitate early discussion on trial design.

HRA are to roll out a **pilot to trial accelerated approval** in 15 days instead of 30.

MHRA to roll out an ATMP clinical site accreditation scheme



Clinical trial sites



Better allocation of resources, including access to and education of staff, to support the management of trials.



Solution: Increase resources to support clinicians and industry to navigate the set-up of trials, including support for rapid trial set-up.



NHS Trusts



Reduce time to approval through local R&D teams

Standardised financial processes and contracts, under development by the NIHR and the ATTC Network, will making contracting smoother.

2

Creating an environment which enables global decision makers to prioritise the UK

NHS-driven collaboration



Leveraging the strength of the NHS to work with industry partners to

1. Design trials which are innovative but practical
2. Have systems in place which allow for the rapid and seamless adoption of ATMPs post-licensure



Full ecosystem collaboration

Using the **lessons learned during the COVID-19 pandemic**, where the NHS, regulators and industry demonstrated how collaborative working and joint risk-taking can accelerate clinical trials and subsequent routine adoption and supply.



Solution: Continue building and maintaining the positive collaboration work between all UK ATMP stakeholders to enhance the acceptance and routine use of innovative clinical trials and ATMP adoption mechanisms.



Regulator supported collaboration

Optimising the potential of the end-to-end approach in the ILAP and other initiatives, to drive risk appetite and joint working.



Key considerations for HTA in ATMP clinical trials

Clinical trial data from randomised controlled trials and long-term patient follow-up is not always available for HTA. ATMP-specific considerations include:



Targeting **small and specific patient populations** due to rare disease or precision medicine.



Randomised comparator arms may not be **feasible or ethical**, but external data sets lack exchangeability



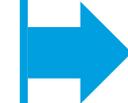
The use of **surrogate outcomes is common** due to the difficulty of capturing rare or delayed patient-relevant clinical events during the trial timeframe.



Short-term trial data required to justify long-term claims of efficacy and safety, despite not having the longevity to provide certainty in these areas.



Limited experience of the healthcare resource required to deliver the therapies, preventing accurate cost estimates.



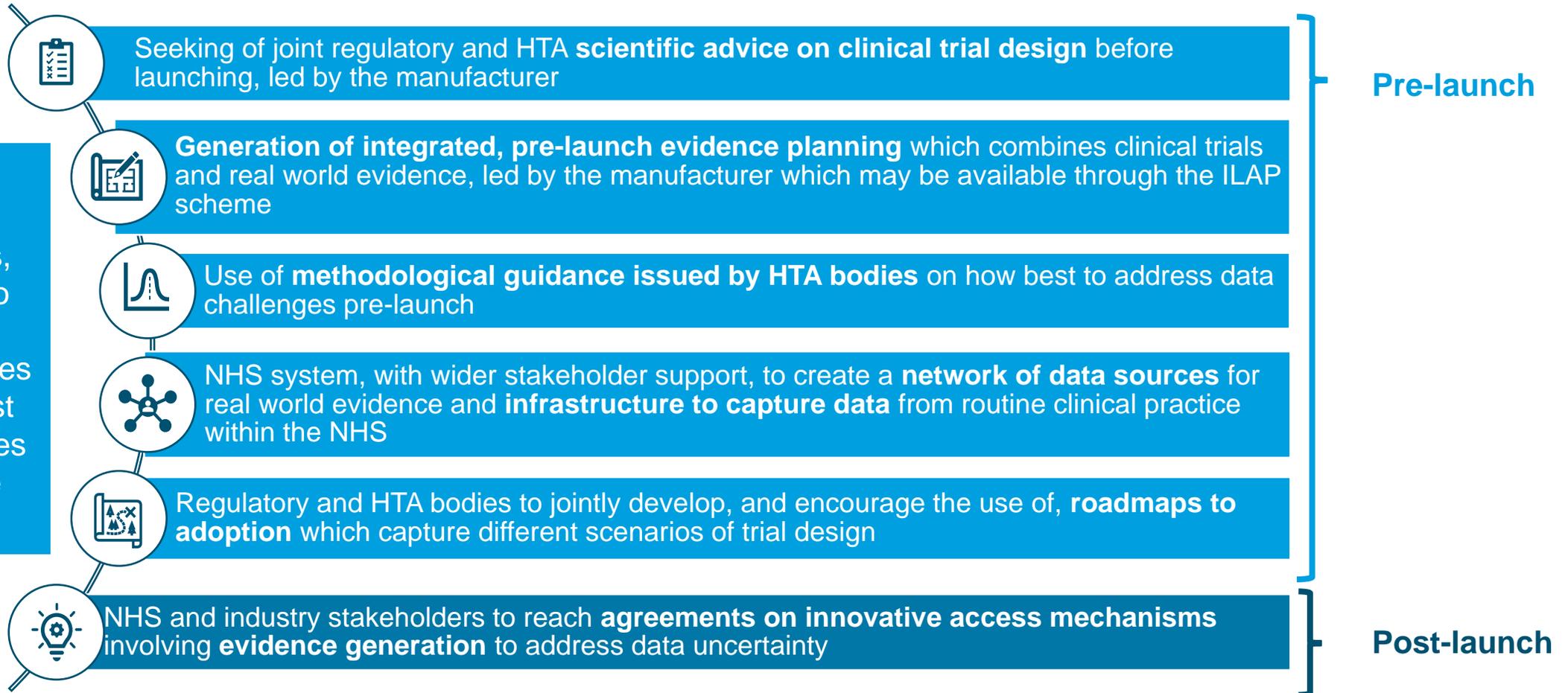
Solution:
See next slide

Working together to optimise the HTA process for ATMPs

3

Successfully leveraging clinical data for HTA

To address the key ATMP-specific considerations for HTA, the following actions should be considered:



Solution:
Manufacturers, regulatory bodies, NICE and NHS to work together to implement strategies to mitigate against trial data challenges and optimise the HTA process.

Recommended actions

In order to advance the institutional readiness and routine adoption of ATMPs in the UK, participants agreed that the following actions should be taken:



GOAL: Ensure the UK remains a global leader in delivering ATMP clinical trials and supporting their future adoption

1 Ensuring the UK remains a competitive and economically viable place to set up and deliver ATMP clinical trials

ACTION: Clinical and regulatory stakeholders to **increase resources** to support clinicians and industry to navigate the set-up of trials, including support for rapid trial set-up.

2 Creating an environment which enables global decision-makers to prioritise the UK

ACTION: All ATMP stakeholders to continue **building and maintaining the positive collaboration work** between all parties to enhance the acceptance and routine use of innovative clinical trials and ATMP adoption mechanisms.

3 Successfully leveraging available clinical data for HTA

ACTION: Manufacturers, regulatory bodies, NICE and NHS to **work together** to implement strategies to mitigate against trial data challenges and optimise the HTA process.

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