

# Accelerating cell and gene therapy adoption

Funded by



UK Research  
and Innovation

Coordinated by

**CATAPULT**  
Cell and Gene Therapy

**NHS**  
University Hospitals Birmingham  
NHS Foundation Trust

UNIVERSITY OF  
BIRMINGHAM

GIG  
CYMRU  
NHS  
WALES  
Ymddiriedolaeth GIG  
Prifysgol Felindre  
Velindre University  
NHS Trust

**NHS**  
Blood and Transplant

**NHS**  
Cambridge  
University Hospitals  
NHS Foundation Trust

**NHS**  
Nottingham  
University Hospitals  
NHS Foundation Trust

**NHS**  
University Hospitals  
Bristol and Weston  
NHS Foundation Trust

**NHS**  
University Hospitals  
of Leicester  
NHS Foundation Trust

**NHS**  
Birmingham Women's  
and Children's  
NHS Foundation Trust

**cytiva**

**4** FOUR  
**PLUS**

**InstilBio**

**ixaka**

**ThermoFisher**  
SCIENTIFIC



**Milenyi Biotec**

**TrakCel**

**OrbsenTherapeutics**  
Redefining Cell Therapy

**World Courier**  
AmerisourceBergen

**Aston University**  
BIRMINGHAM UK

PRIFYSGOL  
**BANGOR**  
UNIVERSITY

UNIVERSITY OF  
**OXFORD**

**Swansea University**  
Prifysgol Abertawe

The ATTC Network	4
Leadership	6
Supporting Clinical Adoption	7
Case Study: NHS Readiness	8
Education	11
Case Study: Education and Training	12
Patient and Public Involvement and Engagement (PPIE)	14
Case Study: PPIE	15
Logistics	16
ATTC Logistics Solutions	17
Case Study: Pharmacy Standardisation	18
Case Study: Simplifying Logistics	20
Electronic Solution for Hospitals	22
Trials Acceleration Programme for Cellular Therapy (TAP-CT)	24
Case Study: Accelerating Patient Access	25
Health Economics	26
Achievements and Impact	27
Commercial Partners	28



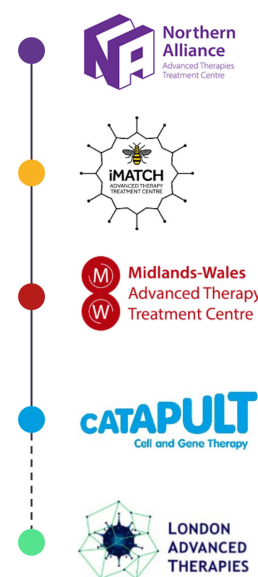
# The ATTC Network

In the last decade multiple new cell and gene therapies have been developed to treat cancers and a range of inherited diseases. These advanced therapies are different from existing treatments in terms of their ability to potentially offer curative approaches and also their disruptive impact on ways of working in the NHS.

The Advanced Therapy Treatment Centres (ATTC) are a world-first, UK system operating within the NHS framework and coordinated by the Cell and Gene Therapy Catapult (CGT Catapult) to address the unique and complex challenges of bringing pioneering advanced therapy medicinal products (ATMPs) to patients. The 3 UK centres are:

- Midlands-Wales Advanced Therapy Treatment Centre (MW-ATTC)
- Northern Alliance Advanced Therapies Treatment Centre (NA-ATTC)
- Innovate Manchester Advanced Therapy Centre Hub (iMATCH)

The network was supported by the Industrial Challenge Strategy Fund delivered by UK Research and Innovation with the aim to develop first-of-a-kind technologies for the manufacture of innovative medicines across areas including blindness, cancer, heart failure, liver disease, neurological conditions and rare paediatric diseases. The ATTCs are working together with industry partners, academic partners and the wider public sector to deliver the necessary processes, skilled staff and infrastructure at a scale that will be needed as more treatments move from clinical trials to marketed products.



## Midlands and Wales Advanced Therapy Treatment Centre

The Midlands and Wales Advanced Therapy Treatment Centre (MW-ATTC) is a regional network which initially comprised of 13 partners. We have grown in the last four years to 20 partners and our clinical reach now extends to Cambridge, Bristol, Leicester and Oxford. Industry, healthcare and university partners have worked alongside each other to create solutions to challenges identified in delivering ATMPs to patients across eight work packages.

We have created robust processes to maintain chain of identity and custody from procurement to administration for our exemplar ATMPs, including comprehensive monitoring and data management. We have tested and integrated market-leading solutions for ATMP transport and tracking using state-of-the-art IT systems. The clinical teams have established an expert network across a number of hospitals with the requisite skill needed to receive and administer ATMPs. We have also worked closely with logistics and manufacturing partners to create more efficient pathways and increase access to clinical trials.

*The aim of the MW-ATTC is to enable UK advanced therapy companies to reach the clinical market, whilst simultaneously building clinical capacity and capability regionally to deliver these breakthrough therapies to patients*



We welcome approaches from advanced therapy companies, logistics partners and NHS Boards and Trusts to join our network .





## Professor Philip Newsome

Director



Phil Newsome is Professor of Hepatology, Director of the Centre for Liver and Gastrointestinal Research at the University of Birmingham and, Consultant Hepatologist at the Queen Elizabeth Hospital in Birmingham, United Kingdom where he is the Deputy Director for the Birmingham NIHR Biomedical Research Centre. He also recently completed his term as Secretary General (President) at the European Association for the Study of the Liver.

He has extensive experience of delivering and managing large programmes, delivering cell therapy studies across the UK, internationally and working alongside industry. He runs a large laboratory group focussing on the role of

cell therapy in liver injury and has established three cutting edge clinical trials where he is the Chief Investigator. One of these, REALISTIC was the largest clinical trial of haematopoietic stem cell therapy in patients with liver cirrhosis in Europe/US. The others include the MERLIN consortium, which is a clinical trial of mesenchymal stromal cells in patients with primary sclerosing cholangitis (PSC) and the POLARISE basket trial in patients with PSC, Rheumatoid Arthritis, Crohn's disease and Lupus Nephritis.

Professor Newsome's research portfolio has led to high impact publications throughout, including both original articles (NEJM, PNAS, Gastroenterology, Hepatology, Journal of Hepatology, Gut, American Journal of Transplantation, Annals of Internal Medicine, Lancet, Nature and Nature Comms) and review articles (FASEB, Gastroenterology, Journal of Hepatology).

## Dr Mark Briggs

Co-Director



Mark Briggs is the Assistant Director for Innovation for Cardiff and Vale University Health Board and the Precision Medicine Ambassador for the Life Sciences Hub Wales. Prior to these roles he was Programme Lead for Advanced Therapies Wales and both Head of Cell and Gene Therapy for the Welsh Blood Service and Head of Strategy for Research, Development and Innovation for the Velindre Cancer Centre within the Velindre University NHS Trust.

Prior to moving to the public sector Mark spent more than 20 years within R&D in the commercial Life Sciences industry in a series of senior roles leading the development and application of enabling and disruptive technologies primarily

for drug discovery, development, safety testing. In his latter corporate years, he focussed upon the industrialisation of advanced therapies with a view to enabling safe and robust, turn-key production of both genetically and non-genetically modified cellular therapies. During this time, he was co-author on numerous peer-reviewed publications and co-inventor of several granted international patents.

## Advanced Therapies NHS Readiness Toolkit

The NHS readiness toolkit is a pan-ATTC output which provides resources for healthcare organisations that are working towards the delivery of advanced therapies for patients.

The Toolkit is intended for senior hospital management, operational managers, clinicians, pharmacists, nurses, laboratory teams and others involved in the delivery of advanced therapies.

Governance	Strategy, business and financial planning	Quality risk management	Operational delivery	Clinical practice	Education and training	Long term follow up
21	10	13	44	40	51	1

## Examples of published documents



# Case Study: NHS Readiness

## The challenge

There has been an exceptional growth in Advanced Therapy Medicinal Products (ATMPs) development in recent years which is creating an unprecedented challenge to the NHS. The UK healthcare system is well-equipped to deliver conventional therapeutic products, however these innovative cell and gene therapies require the development of new infrastructure, systems, processes, and skilled staff in order to treat patients safely and at scale as these ATMPs move from clinical trial to marketed products.

## The solution

In response to this challenge, three Advanced Therapy Treatment Centres (Midland Wales [MW-ATTC], Northern Alliance [NA-ATTC] and iMATCH) were established in 2018. These centres act as a network, supported by the Cell and Gene Therapy Catapult. They have developed a range of key, free-to-access, materials aimed to inform, educate and expand NHS expertise and delivery of Advanced Therapies. MW-ATTC focussed on the creation and delivery of a range of information and resources to provide and support NHS organisations with a variety of tools to enable the successful delivery of ATMPs. These wide-ranging materials include;

- Educational material, i.e. Introduction to Advanced Therapies, logistic / carrier training and competency assessments etc.
- Pharmacy governance, template protocols and checklists etc.
- Organisational guidance generic Advanced Therapy pathways, CRF matrices, workforce identification hierarchy etc.

NA-ATTC produced a number of guidance documents and toolkits which clinical sites can utilise to accelerate the adoption of ATMPs, both through clinical trials and licensed medicines, examples include:

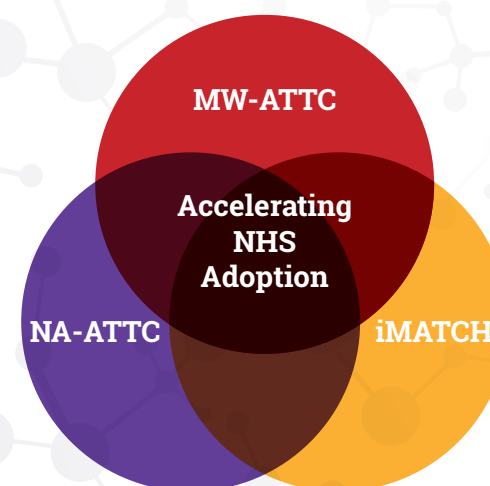
- An Institutional Readiness toolkit and questionnaire which allows NHS clinical sites to assess their own Institutional Readiness levels for the four classes of AT medicines – tissue engineered, cell-based gene, somatic cell and virus-based gene products.
- A clinical trials toolkit which includes costing guidelines.
- A suite of guidance documents and SOPs for clinical delivery and management of CAR T-Cell Therapies.
- Guidance for developing a hospital business case for ATMP service provision.
- Pharmacy governance and preparation facilities requirements for gene therapies.

iMATCH's key Institutional Readiness activities focus across education, governance and safe patient management to increase patient access to ATMPs. Materials generated include:

- Education programme to up-skill the workforce and aid delivery.
- Presentations were developed as aids e.g. JACIE readiness for immune-effector cells and ATMP Pharmacy training.
- Governance structures and terms of reference across various committees e.g. ATMP Board.
- Oncology adoptive T-cell therapy mapping – concept and complexities of the patient pathway.
- Checklists for handling ATMPs e.g. Thawing Checklist.

## The results

The NHS Readiness Toolkit, launched in April 2021, was developed to provide easy access to the portfolio of resources created by the ATTCs. These resources span 8 categories, including governance, operational delivery and clinical practice, and allow hospitals to evaluate their readiness to deliver ATMP, and demonstrate and embed best practice by preparing UK healthcare organisations to develop local structures, systems, pathways, procedures, processes and workflows.





Our ATTC has added significantly to training and education and we contribute to each of the elements below. We have also worked alongside our pan-UK training community to create new resources.



### Governance

This section is intended to assist NHS organisations to create or modify governance structures to permit the introduction of advanced therapies, encompassing all levels of governance responsibilities and reporting within hospitals. Also see: Business and financial planning, Operational delivery

[Find out more >](#)



### Business and financial planning

The introduction of advanced therapies into hospital treatment pathways requires a significant financial investment in resources and facilities. Information available here will help organisations to develop the appropriate business and financial plans as they prepare to deliver advanced therapies.

[Find out more >](#)

Examples of categories within the NHS Readiness Toolkit

## Making further impact

The NHS Readiness Toolkit was visited by over 10,000 users within the first nine months of its launch, demonstrating a clear need for these resources. Feedback from users has highlighted the impact which the Toolkit is already making, and as the ATTC programme matures and further outputs are shared, the Toolkit's reach is anticipated to extend.

**"I find the NHS Readiness Toolkit provides me with a go-to repository of guidance to support the service implementing advanced therapies into the clinical setting. Some of the resources I have found most helpful are the training, education components as well as the clinical practice section which holds useful information such as the thawing SOP and checklist."**

*Debbie Worthing, Lead Research Nurse Advanced Therapies Programme*

**"The Advanced Therapies NHS Readiness Toolkit has been a vital resource for me in coordinating ATiMP cancer trials. In particular, the Clinical Practice guidance and the tools in its Education sections were the best I have found for teaching both research-familiar and research-naïve NHS staff about the differences and complexities of ATiMP trial work, which has benefitted us when planning and managing our early-phase ATiMP cancer trials."**

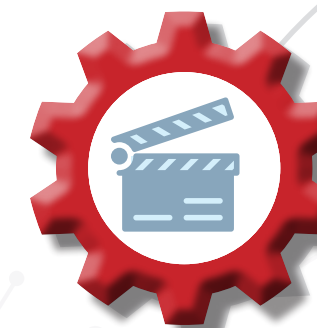
*Sam Moody, Clinical Trial Coordinator in Newcastle*

As more hospitals prepare to deliver advanced therapies, the resulting impact will be clear for the greater number of patients who are able to access these life changing medicines faster than ever before.



Access the NHS  
Readiness Toolkit here

### Training videos



Thawing and inspecting frozen cell products  
Management of Cytokine Release Syndrome  
Transporting of Advanced Therapies

### Expert led webinars

34 pan-ATTC recorded webinars  
15 of which led by MW-ATTC



### Competency assessments

Procurement, infusion, Handling,  
Medical Equipment and spillage SOPs  
Patient pathway flowsheets  
Training Matrix



### E-Learning Modules

Introduction to ATMPs  
Low temperature transport  
CAR-T cell therapy  
In Vivo gene therapy  
ATMP logistics in hospitals  
Immune effector cell therapy



### Exemplar protocols, policies & risk assessments

Storage, transfer, receipt and checklists  
Study intensity protocol  
GTMP approval  
GMO risk assessment



### Resources to support face-to-face training

Procurement, infusion, handling,  
medical equipment and spillage SOPs  
Patient pathway flowsheets  
Training Matrix



### Formal courses

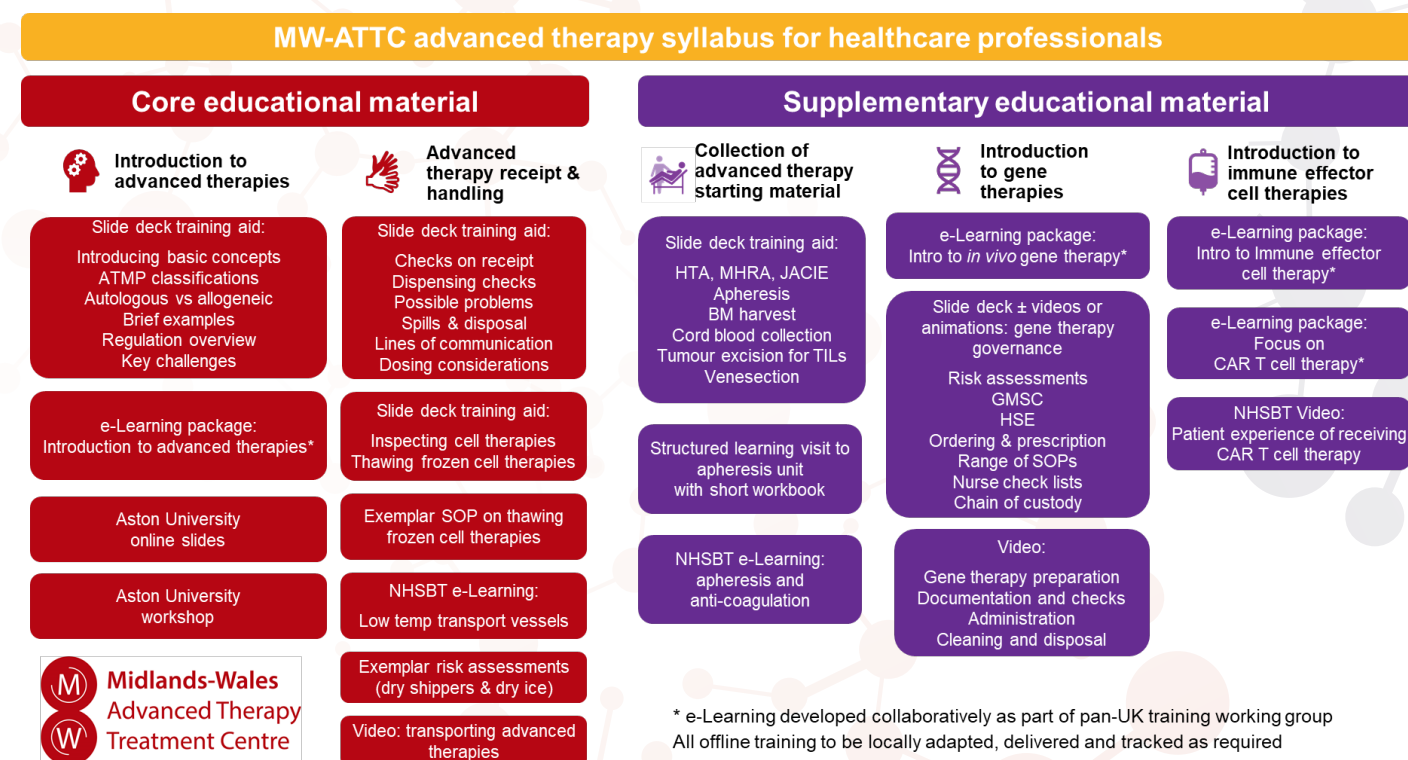
# Case Study: Education and Training

## The challenge

Advanced therapy medicinal products (ATMPs) have the potential to address significant and growing unmet healthcare needs. These treatments offer exciting therapeutic and potentially curative options for diseases which cannot be addressed adequately by existing pharmaceuticals. The UK is at the leading edge of this disruptive field and there is an opportunity to build a large-scale industry delivering health and wealth to the country. Currently in the UK, there is no coordinated education and training programme on advanced therapies available to existing NHS staff, despite the rapid pace with which new therapies are becoming available. ATMPs are complex treatments, requiring innovative ways of working, and new knowledge and skills, for the many staff groups involved in their clinical delivery. These therapies also present urgent learning needs for the wider healthcare community and for teams responsible for clinical education and training.

## The solution

Contributions from a network of subject matter experts have guided the development of a structured syllabus intended to provide healthcare professionals with the required underpinning knowledge and awareness of ATMPs to supplement further product-specific and practical training in the workplace.



## The results

Training packages produced by NHSBT are two of the most frequently downloaded resources on the ATTC network website and have been implemented at several face-to-face training sessions, generating positive feedback.

- An animated video on transporting advanced therapies was produced by NHSBT in collaboration with World Courier. The video is primarily aimed at logistics personnel but is accessible for anyone requiring a brief introduction to the advanced therapy supply chain. This video has also been widely used across the ATTC network.
- Findings from a training needs analysis survey were summarised in a report co-written by NHSBT and the Cell and Gene Therapy Catapult.
- An introductory training workshop was successfully piloted by Aston University in conjunction with MW-ATTC and future courses and training packages are planned.
- A short video has been produced to communicate a patient's experience of receiving CAR-T cell therapy.
- Training is being created for research nurses to explain processes involved in collecting starting material for the manufacture of advanced therapies. This will be supplemented by a workbook to provide structured learning objectives for visiting an apheresis unit.
- An e-learning package and exemplar risk assessments are being developed on the safe use of dry shippers and dry ice.
- A competency assessment framework is being developed to record and measure successful staff training.

## Making further impact

The Cell and Gene Therapy Catapult are coordinating the development of education and training across the ATTCs and London Advanced Therapies network. A series of e-learning modules are being developed by a team working collaboratively across the network, with an in-built programme of review and testing by clinical experts and end users to ensure quality assurance of training content. The outputs of this joint approach will be made widely accessible to the UK health and social care workforce via Health Education England's e-Learning for Healthcare hub. This learning platform is an authoritative and trusted source, supporting our standardised, national approach that will equip NHS staff with theoretical knowledge and awareness to complement their practical training in the workplace.



# Patient and Public Involvement & Engagement



## 3 Patient experience videos

**Nitya's Story - What is it like to receive CAR T cell therapy?**  
22nd July 2020 | Midlands - Wales News

The first video was produced by NHSBT in collaboration with University Hospitals Bristol and Western NHS Foundation Trust.

Nitya, a University student from Gloucestershire, was the first patient to undergo CAR T cell therapy at the Trust in February 2019.

The next two videos are of Anne and Sophie, both patients who have also received CAR T therapy at University Hospitals Birmingham NHS Foundation Trust explain how they met by chance outside the Pharmacy department and struck up a friendship. They go on to describe their experience of the treatment.

**ADVANCED  
THERAPIES** 2021

## An interactive series introducing cell and gene therapies.



Introducing cell and gene therapies



Ask the experts: Liver disease and rheumatoid arthritis



Ask the experts: Cancer



Pharmacy, logistics and a look to the future

## 3 Publications

### 1 Patient and Public group

A scoping review of patient and public perspectives on cell and gene therapies

Patient and public perspectives on cell and gene therapies: a systematic review

Production of a patient toolkit for manufacturers and Sponsors

# Case Study: PPIE

## The challenge

Over the last decade, new advanced therapy medicinal products (ATMPs) have been developed to treat various cancers, inherited diseases and chronic conditions. Although they offer ground-breaking new opportunities for the treatment of disease and injury, the uptake of these therapies requires appropriate patient and public engagement and buy-in. There is therefore a need to offer patients and their families educational resources which provide accurate, relevant and valued information about ATMPs in order to increase their knowledge and understanding and empower them in making decisions about these therapies, which may also potentially improve patient recruitment for ATMP trials.

## The solution

In order to gain an initial understanding of patient perspectives of ATMPs, a comprehensive systematic review of published literature on the subject was recently conducted within the MW-ATTC (Aiyegbusi et al 2020, Nature Communications). The high-impact review included 35 publications and summarised findings on patient concerns, expectations and information needs relating to ATMPs.

## The results

The systematic review revealed a number of misconceptions about ATMPs among patients and the general public. The review also highlighted a lack of accurate information and clarity about the potential benefits and risks of these novel therapies. These issues have led to over-optimism in some patients and a lower level of acceptance in others. Acceptance of cell and gene therapies varied among patients but generally increased after the provision of information.

## Making further impact

Utilising the results of the systematic review, particularly the gaps in educational resources which were highlighted, the MW-ATTC are developing a series of informational webinars aimed at patients and the public which will be delivered virtually in the summer of 2021. Additionally, patient and public partners in MW-ATTC focus groups will contribute to the shaping and creation of new materials within the centre which will address topics identified in the review.



View all of our resources for patients and the public here

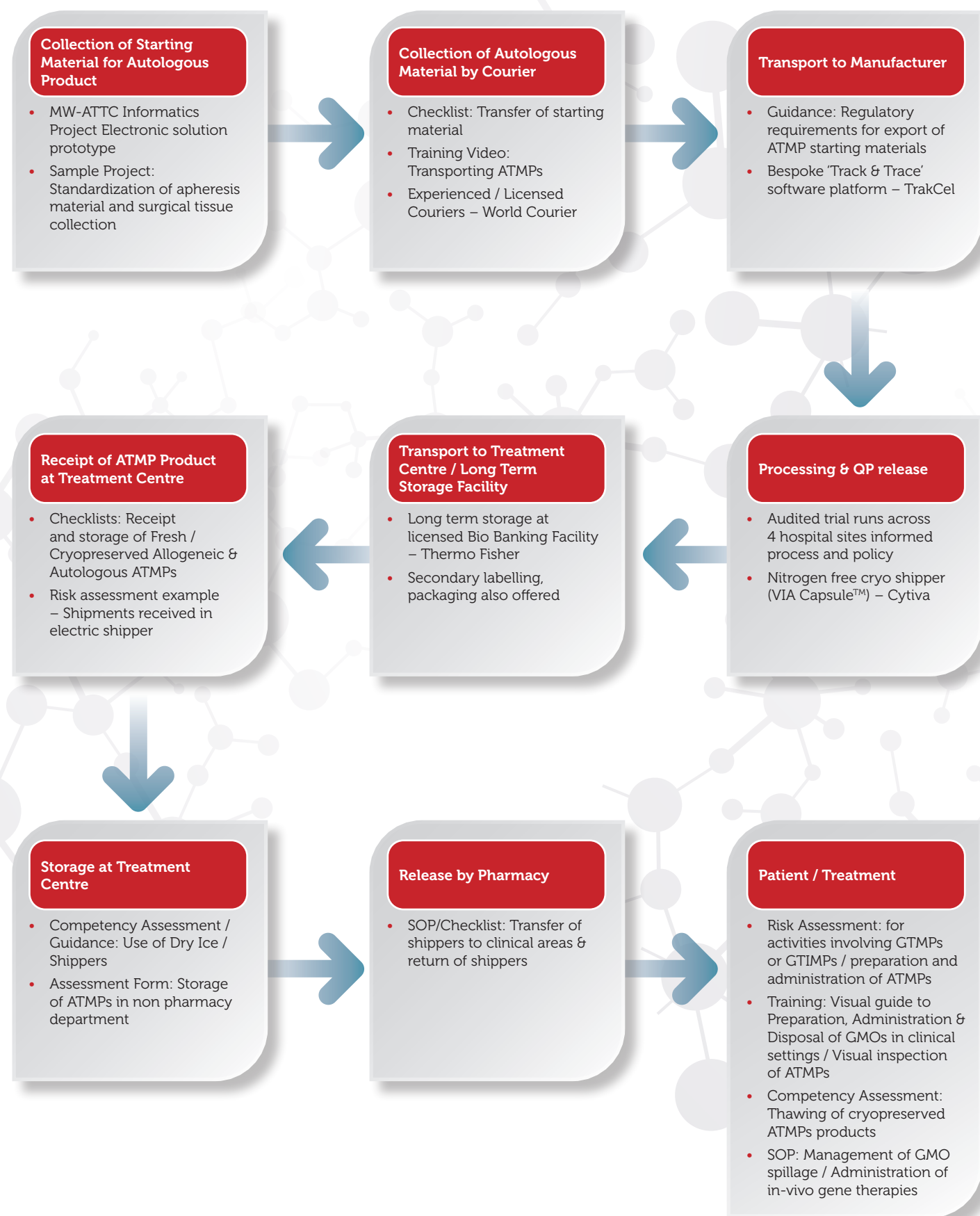
**ATTC**  
Advanced Therapy  
Treatment Centres

**MW** Midlands-Wales  
Advanced Therapy  
Treatment Centre



## Logistics and Orchestration

Identifying the potential issues & developing solutions for clinical sites delivering ATMPs



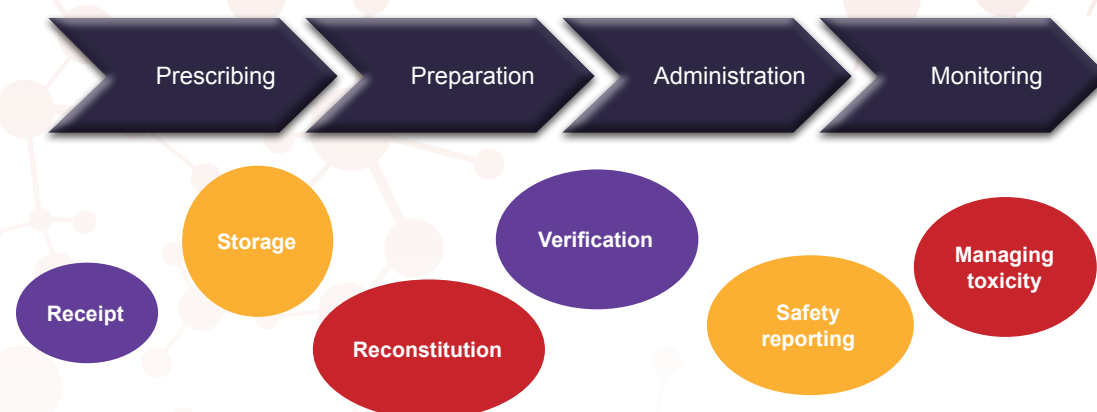
# Case Study: Pharmacy Standardisation

## The challenge

Advanced therapy medicinal products (ATMPs) have substantially different pathways through patient care compared to conventional treatments. These products may be manufactured for specific patients following harvest of a patient's blood or bone marrow and may be only suitable for that particular patient (autologous). Other ATMPs are made on a larger scale, allowing many patients to be treated from the same manufacturing batch (allogeneic).

All ATMPs have specific storage, handling and dispensing instructions that must be followed to ensure that the quality and safety of products are maintained. Pharmacists are responsible for ensuring that the ordering, storage, reconstitution and dispensing of ATMPs are in line with their product specifications, patient clinical needs and protocols when the products are offered as part of a clinical trial.

### The role of pharmacy

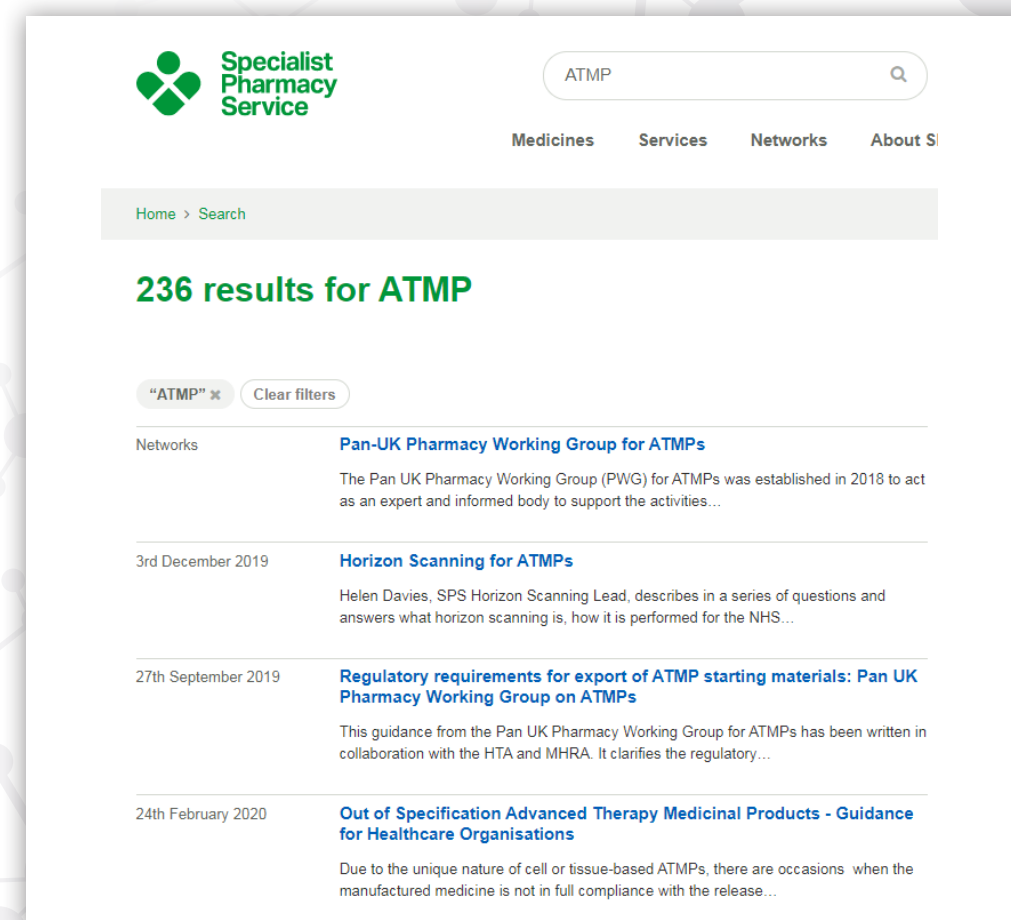


## The solution

The pan-UK Pharmacy Working Group (PWG) is acting as an expert and informed body to support the activities of the three ATTCs and, indeed, hospitals around the UK in the administration of ATMPs. The group consists of pharmacists from across the UK that specialise in the governance, clinical trials, prescribing, administration and monitoring of ATMPs and is an excellent example of collaboration across the NHS. The aims of the group are to promote good practice, identify and resolve pharmacy issues to maximise the effectiveness and development of services for hospitals to administer advanced therapies.

## The results

The group has developed guidance and checklist documents in the implementation of ATMPs to provide consistency in governance, clinical and operational aspects for pharmacies across the country. The PWG is now formally a Specialised Pharmacy Service Network and is becoming an important partner to the NHS in the administration and commissioning of ATMPs and will continue to develop best practice and guidance for this class of medication<sup>1</sup>.



Specialist  
Pharmacy  
Service

## Making further impact

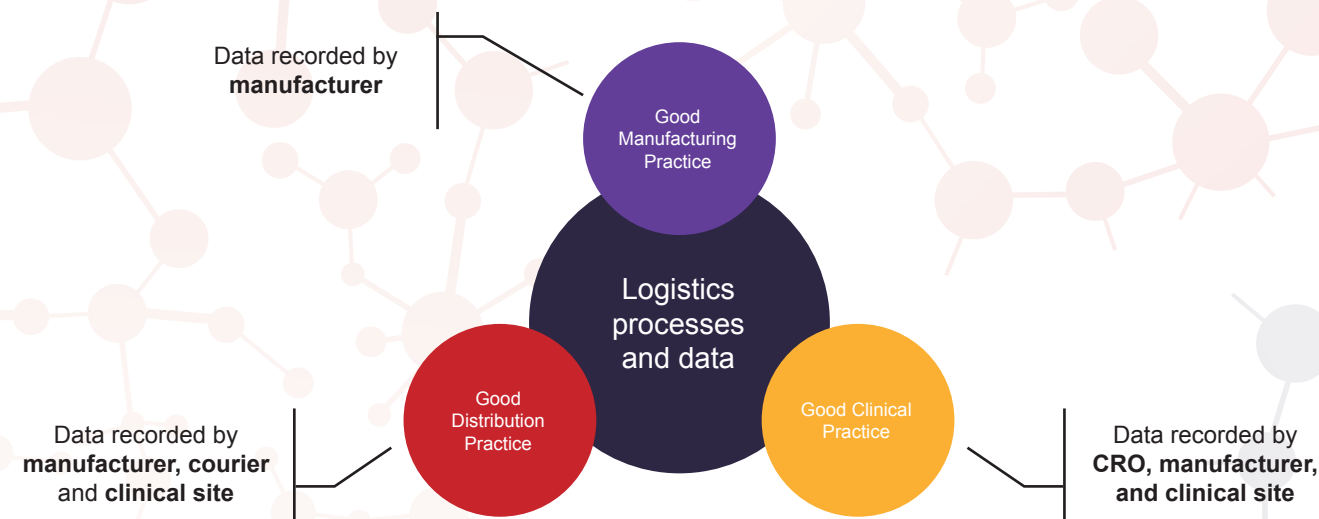
As new hospitals begin to use ATMPs in the clinic, there are procedures and checklists available to support clinical staff. Commercial ATMP developers also benefit due to the more consistent approach at different hospitals, making set up at different sites a smoother process.



# Case Study: Simplifying Logistics

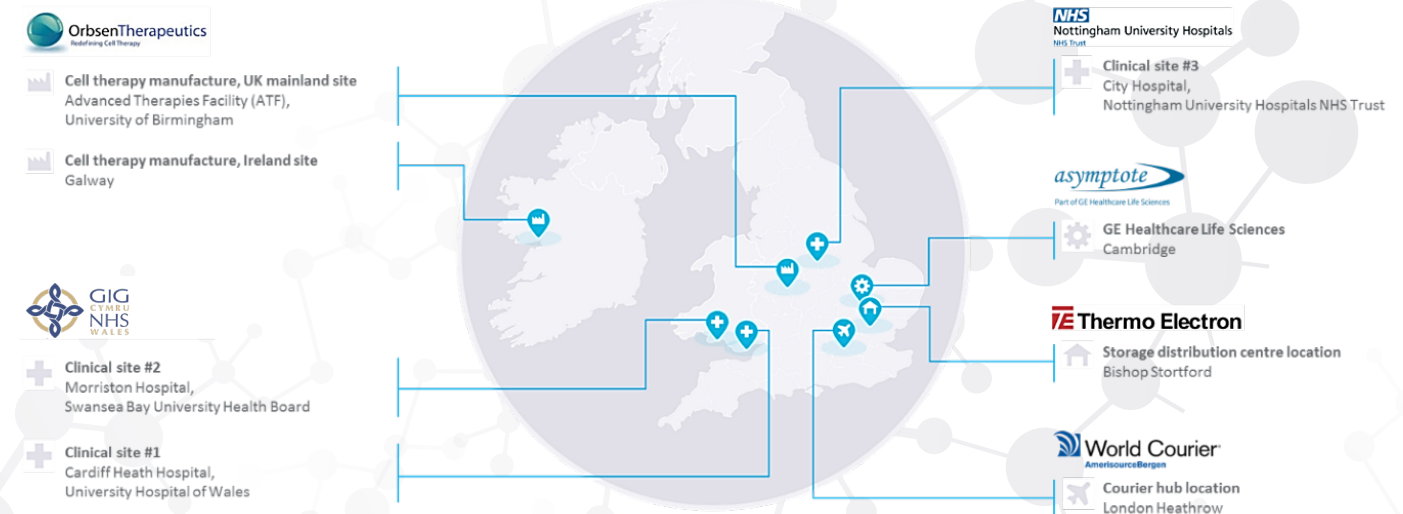
## The challenge

Cell and gene therapies are expensive and complex treatments to manufacture. These treatments need to be accurately monitored to maintain the viability of the product once it reaches the patient. The issue with the data for the logistics of cell and gene therapies is that data collection is recorded by multiple parties; the manufacturer, courier, CRO and clinical site.



## The solution

As a part of the Midlands-Wales Advanced Therapy Treatment Centre (MW-ATTC) project, an integrated cell therapy logistics network is being developed and tested to ensure a robust process is in place for the delivery of ATMPs into the clinical setting.



## The results

Throughout the process, Cytiva's Chronicle™ product was used to capture data generated at different points, standardising the process and managing documentation

- Chronicle™ eSOP tool captures delivery process and reads barcodes
- Dashboard monitors shipper location and condition
- Integrates with World Courier system to record documentation and events
- Managing chain of custody and identity

## Making further impact

Chronicle™ is being further tested and developed as a part of the MW-ATTC programme. The system is being built to unite manufacturing and logistics data, accurately recording and tracking products from manufacture to thawing at clinical sites to speed up and simplify the running of these complex trials.

**ATTC**  
Advanced Therapy  
Treatment Centres

**MW** Midlands-Wales  
Advanced Therapy  
Treatment Centre

**asymptote**  
Part of GE Healthcare Life Sciences

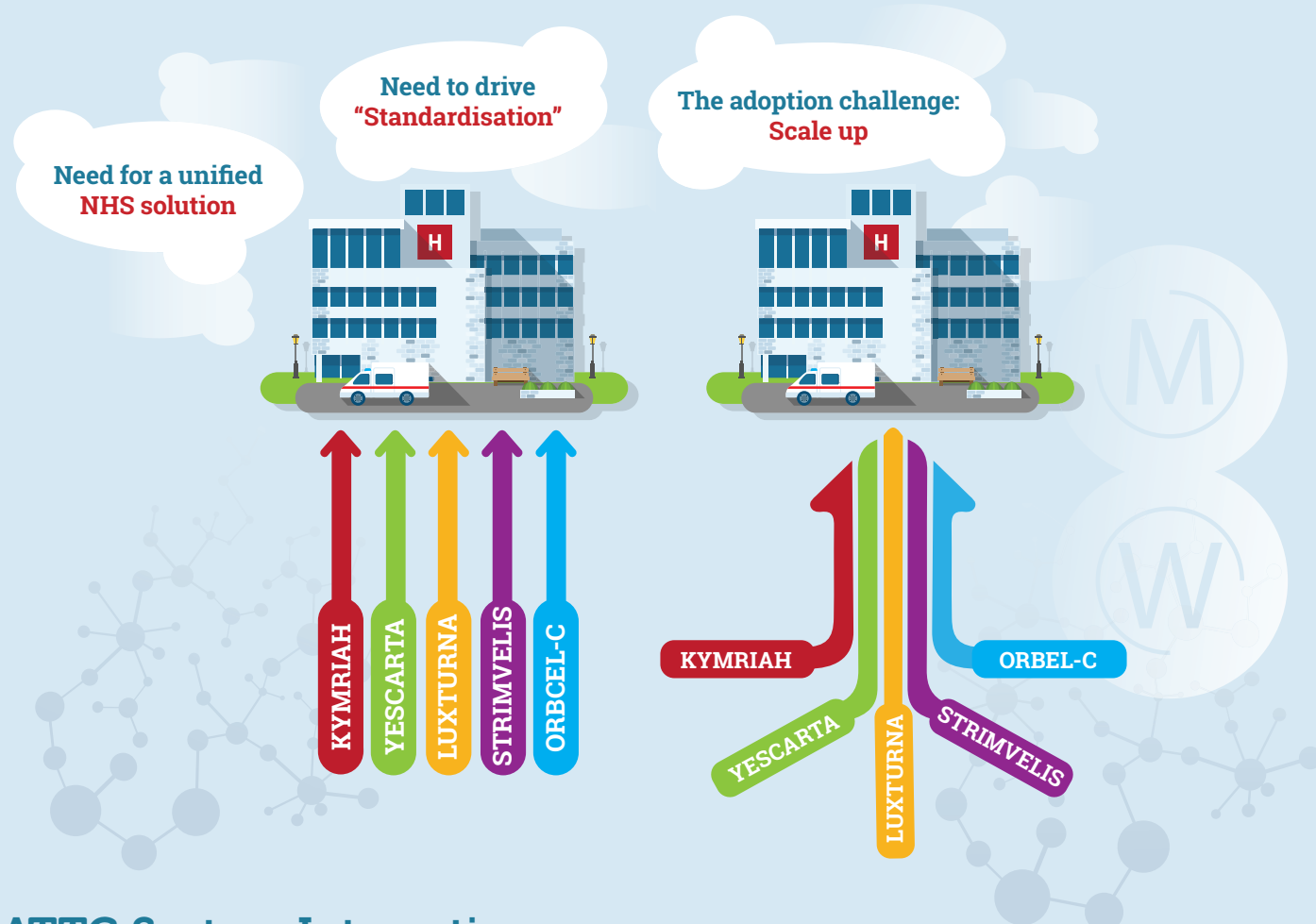
**World Courier**  
AmerisourceBergen

**ThermoFisher**  
SCIENTIFIC

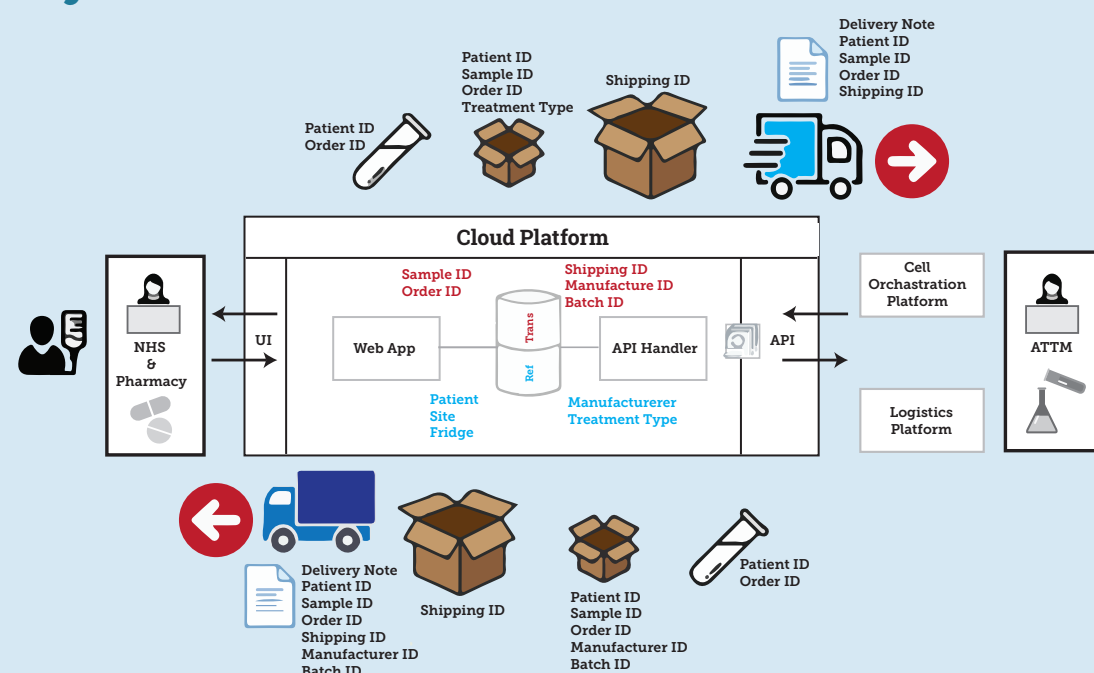
**OrbsenTherapeutics**

# Electronic Solution for Hospitals

Advanced Therapy Medicinal Products are moving at pace from clinical trials into routine clinical care. Due to the logistical complexity, currently each therapy provider is setting up an individual track and trace solution resulting in a multiplicity of ATMPs systems and extra burden for the NHS.



## ATTC System Interactions

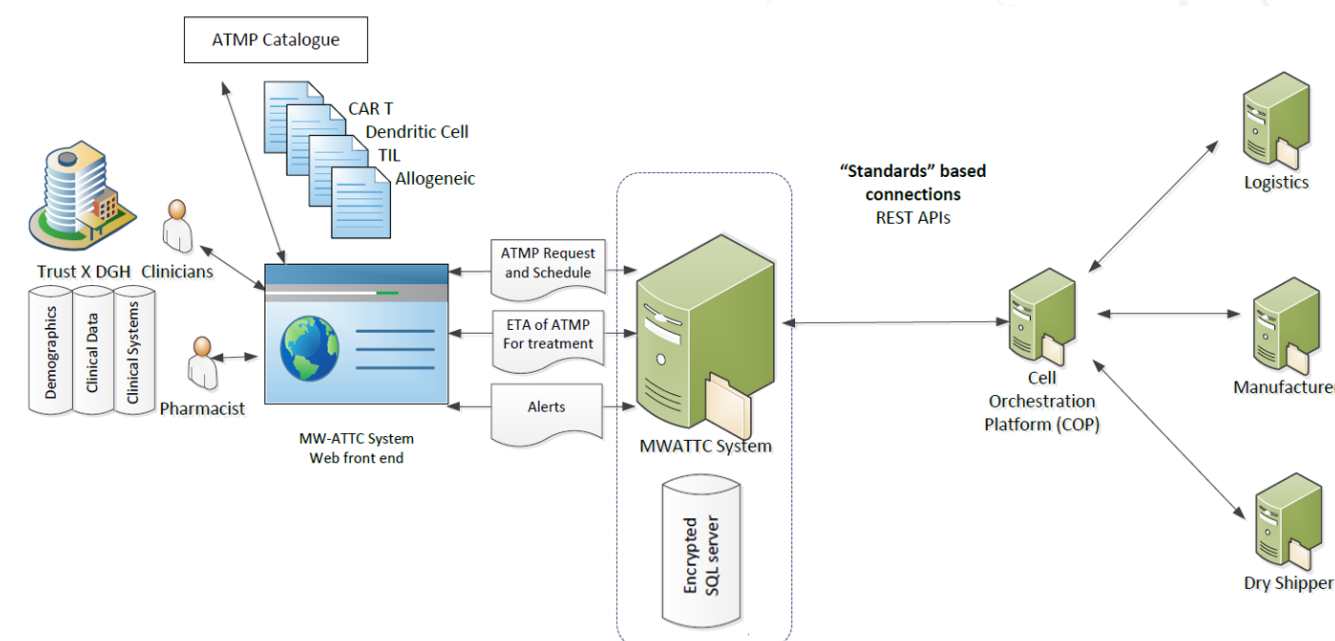


This was achieved by UHB Informatics in collaboration with our industry partners providing manufacturing insights, building upon existing MW-ATTC Cell Orchestration Platform (COP) infrastructure to further increase the robustness of the scheduling and maximising the utilisation of expensive manufacturing resources provided by the University of Birmingham.

For the NHS a web based prototype system was built on a Cloud based platform, to standardise the Ordering and Scheduling of ATMP Treatments for the NHS, in collaboration with industry partners and manufacturers, to deliver trackable 'needle-to-needle' ATMP products for ATMP Patients. The prototype allows at least one ATMP to be ordered and scheduled via a catalogue from an NHS Trust that delivers ATMP treatments. This system should be able to interface with one or more Cell Orchestration Platforms and one or more Advanced Therapy manufacturers.

Our design approach was based on a standard Ordering system as shown below.

## The system



## The Benefits

- Removal of administrative burden that makes current processes difficult and time consuming
- To allow the NHS to be aware of where their treatment is in manufacturing and delivery processes
- Helps NHS with treatment scheduling & a single UI for NHS Staff to familiarise themselves with
- Moves NHS towards mainstream ordering of ATMPs
- Provides data points to assist payer mechanism
- Provides "Standards" based APIs for COP and Advanced Therapy services suppliers integration
- All the above will speed-up the adoption and reduce implementation and operational costs



Watch our webinar to find out more

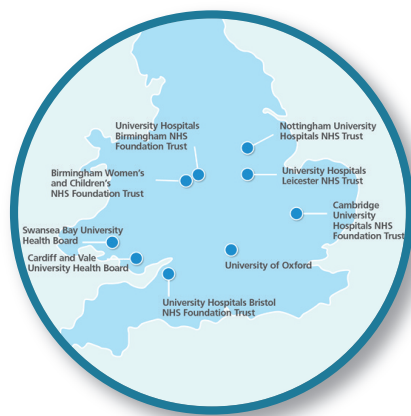


# Trials Acceleration Programme in Cellular Therapy (TAP-CT)

Advanced Therapy Medicinal Products (ATMPs) are novel and potentially life-changing therapies for a range of diseases including rare conditions with no current effective treatment. Accelerated delivery of pivotal clinical trial is essential if patient benefit is to be maximised. However, set up processes are still slow and patient recruitment to complex therapies remains challenging. The ATTC network has almost doubled the number of ATMP trials and now runs 6% of all global ATMP trials.

However, to meet the ambitious target of >10,000 patients accessing cell therapies by 2029, we must develop innovative ways of accelerating patients onto trials to receive pioneering treatments.

Modelled on existing trials acceleration programmes IMPACT and Cure Leukaemia TAP, we have set up a network of nine sites staffed with dedicated research nurses with expertise in cellular therapy trials and ATMPs in order to fast-track opening of critical trials and widen patient access to these life-changing treatments.



## Who are we?

The network is coordinated by the University of Birmingham, and each site has funded resource to support ATMP trial set up and opening. We work with both commercial and academic sponsors to facilitate accelerated set up and opening of exciting cell and gene therapy trials across a network of clinical sites spanning a large geographical area.

## What we offer:

- A large and diverse patient population across the Midlands, South Wales, Bristol, Oxford and Cambridge of more than 20 million, which will help improve meeting recruitment targets for complex trials, in particular those in rare diseases
- Dedicated nursing or clinical trials coordination resource to navigate the set-up process and to support recruitment and training of research nurses
- Resource and information to conduct rapid feasibility for Sponsors
- Access to key opinion leaders across the network in a number of clinical areas, including:

Cardiology; Covid-19; Diabetes; Gastrointestinal Disease; Haematology and Haemato-oncology; Immunology; Liver Disease; Metabolic and Endocrine Disease; Neurological Disease; Oncology; Renal disease; Respiratory Disease; Urogenital Disease

CAR-T

MSC

T-CELL

DC  
VACCINE

CMV  
VACCINE

STEM  
CELLS

AND  
MORE...

# Case Study: Accelerating Patient Access

## The challenge

When the Birmingham Children's Hospital (Birmingham Women's and Children's NHS Foundation Trust) were approached by a company to run a haematology ATMP clinical trial, the Trust, seeing the promise of the treatment, were keen to support the trial. However, having no prior experience of advanced therapies, they needed support to enable the creation of the required governance structures and procedures to rapidly set up this clinical trial.

## The solution

Through local networks, the team at BCH were made aware of the Midlands and Wales ATTC and introduced to Prof. Phil Newsome. The MW-ATTC have since been able to provide advice and support to the team around the complex regulatory framework. Pharmacists have been linked in to the MW-ATTC network and local individuals who have been able to provide specialist support, to ensure that the team has the required working knowledge in place to safely work with ATMP's and that regulatory requirements are met.

## The results

The introduction to the MW-ATTC galvanised the actions required, providing both a road map of the requisite next steps and access to necessary training and knowledge. The availability of multiple resources, including relevant policies and procedures which can be locally adapted has drastically compressed the time taken to progress from initial contact to trial set up.



**The MW-ATTC have really helped move things faster at BWC with access to their expertise and teaching materials.**

- Dr Fiona Reynolds, Medical Director of Birmingham Children's Hospital



## Making further impact

As a result of the introduction to MW-ATTC and facilitation to this essential knowledge regarding ATMP clinical trials, access to ATMP clinical trials in the Birmingham Children's Hospital has been accelerated and BCH are now able to offer a new service. Although the timeframe for their first gene therapy trial was delayed by the COVID-19 pandemic, they plan to open a GMO trial in Spring 2021 and have already been approached by additional companies looking to partner on future ATMP trials.





**A microanalysis costing toolkit for ATMPs**



Download the toolkit and access our handy "how to" guide here

**An assessment of the cost benefits of utilising an ATMP versus the cost of standard of care treatment**  
**The production of an economic evaluation of an identified ATMP**

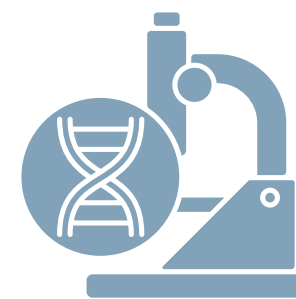


**White Paper, 'The Methods of Economic Evaluation of Advanced Therapy Medicinal Products'**

**Economic analysis of tumour storage for a Tumour Infiltrating Lymphocyte**



**Coming soon: Development of the UK's first Family Reported Outcome Measures Survey for ATMPs**



**All of our 9 clinical sites are ATMP ready**

**Contributed 43**

**original documents to the NHS Readiness Toolkit**

Delivery of MW-ATTC clinical readiness programme included the development of a suite of materials ranging from SOPs, guidance documents and training for NHS Trusts.



**We're really excited that the program has been a success. It has allowed us to build partnerships, build collaborations and expertise; not just within Birmingham, but in the wider UK cell therapy community.**

- Dr. Steve Elliman, Chief Scientific Officer



**Patient & Public Involvement & Engagement**



**Reached over 500**

patients via 4 different webinars empowering patients and public to engage with ATMP research.



**Our informatics solution is a step forward in making a standardised NHS IT system to facilitate ATMP treatments and a NHS IT system to link to cell orchestration platforms. This will allow ATMP treatments to become more mainstream.**

- Prof. Phil Newsome, Director of Midlands-Wales ATTC, University of Birmingham





# Commercial Partners



**Cytiva** is a global life sciences leader with over 8000 associates across 40 sites who are dedicated to our vision to improve access to life-changing therapies that transform human health. As a trusted partner to customers that range in scale and scope, Cytiva brings efficiencies to research and manufacturing workflows, ensuring the development, manufacture and delivery of transformative medicines to patients.



**FourPlus** are an immersive technology company offering end-to-end software solutions for the life sciences sector. The recent emergence of advanced therapies creates novel workforce and manufacturing challenges. Immersive technology such as Virtual and Augmented Reality (VR & AR) is transforming business practice across a range of major sectors, optimising tech transfer and offering innovative visualisation tools across the operational spectrum. FourPlus combine expertise in life sciences and immersive technology to build solutions that will bring about a step change in the manufacturing of advanced therapies. Our approach unlocks the potential of revolutionary medicines and helps accelerate their delivery to the patients who need them.



**Instil Bio, Inc** is a global, clinical-stage cell therapy company developing tumor infiltrating lymphocytes (TIL) for the treatment of cancer. We are building on the decades-long foundation of TIL efficacy in treating solid tumors, applying our cell therapy experience and TIL manufacturing platform to bring the promise of TIL therapy to patients in need. Instil Bio has research and cell therapy manufacturing facilities in Los Angeles, CA and Manchester, UK, with corporate offices in Dallas, TX.



**Ixaka** is a cell and gene therapy company focused on the natural power of the body to cure disease. Our proprietary technologies enhance the inherent therapeutic power of cells by targeting curative cells to the site of disease, and by directly modifying cells within the body to improve their therapeutic action. Ixaka's technologies – concentrated multi-cell therapies and nanoparticle therapeutics – demonstrate potential for the treatment of a broad range of serious diseases across oncology, cardiovascular, neurological and ocular diseases, and genetic disorders.



**Miltenyi Biotec**

**Miltenyi Biotec** is a global provider of products and services that advance biomedical research and cellular therapy. Our innovative tools support research at every level, from basic research to translational research to clinical application. Used by scientists and clinicians around the world, our technologies cover techniques of sample preparation, cell isolation, cell sorting, flow cytometry, and cell culture. Our 30 years of expertise spans research areas including immunology, stem cell biology, neuroscience, and cancer. Today, Miltenyi Biotec has 2,500 employees in 28 countries – all dedicated to helping researchers and clinicians make a greater impact on science and health.



**Orbsen Therapeutics** is a regenerative medicine company based in Galway, Ireland. Using proprietary technology, we are developing ground-breaking stromal cell treatments to address some of today's most challenging diseases. Stromal cells hold great promise as an alternative to drugs and surgical procedures for treating a wide range of medical conditions including heart disease, arterial disease of the limbs, diabetes complications, arthritis and other inflammatory conditions. The treatment potential is linked to the cells' natural capacity to dampen inflammation and promote healing, repair and regeneration of damaged tissues. Orbsen has discovered and patented a unique method for purifying these rare, therapeutic cells and developing them for clinical use. The cells can be selected from a single donor, expanded and frozen to generate many doses of a high-margin, "off-the-shelf" therapeutic product.



**Thermo Fisher Scientific** is positioned with resources and global expertise to support customers on the path towards commercialisation. Our global infrastructure enables customers to conduct clinical trials across multiple geographies while providing patients with access to life changing therapies. Cryogenic storage and logistics allow us to configure each site to meet specific requirements of individual clinical trial. We can help navigate many of the unforeseen challenges associated with cell therapy clinical development and commercialisation.



**TrakCel** is a cloud-based Cellular Orchestration Platform (COP) that enables the streamlined delivery of advanced therapies to patients. The platform can be configured to processes defined by the cell therapy manufacturer and records all critical data across the supply chain in a FDA 21 CFR Part 11 and EU Annex 11 compliant manner. TrakCel is a software platform that delivers:

- End-to-end Chain of Identity (COI) management
- Full collection to administration visibility and Chain of Custody (COC) reporting
- Automated scheduling of activities between patient facing clinical and manufacturing sites
- Integration capabilities with ERP, CRM, and other similar systems
- Workflow-driven consistent processing
- Role-based functionality and information access for physicians, nurses, & supply chain partners
- Cloud hosted multilingual platform



**World Courier** is a global specialty logistics company that designs world-class logistics and supply chain programmes in complete alignment with our customers' business goals. Pharmaceutical companies rely on us because they value the peace of mind that comes with our unsurpassed knowledge, global reach and flawless supply chain execution. Each trusted partnership we form with a customer is deeply rooted in our shared vision of improving global health. With 2,000+ associates in more than 140 offices across the globe, we offer solutions that instil confidence in the on-time, on-temperature delivery of critical products. When trust is absolutely essential, there's only one choice: World Courier.





Visit the MW-ATTC website

Funded by



UK Research  
and Innovation

Coordinated by

**CATAPULT**  
Cell and Gene Therapy