

# Long term thinking: Advanced Therapy Medicinal Products. From Clinical Trials to Licensed products.

Melvin C<sup>1</sup>, Burgess S<sup>1</sup>, Duran-Jimenez B<sup>1</sup>

Pharmacy, Manchester University NHS Foundation Trust

## Introduction

•The First Gene therapy medicines are licensed in the UK

- Chimeric antigen receptor T-cells (CAR-T) are NICE approved for NHS use.
- NHS hospitals will be required to incorporate ATMPs into routine practice.

UK hospital pharmacy departments generally lack the infrastructure to handle advanced therapy medicinal products (ATMPs) from a governance and operational point of view<sup>1</sup>. Horizon scanning predicts exponential growth in this area<sup>2</sup>

Manchester University NHS Foundation Trust (MFT) was one of the 8 (first wave) centres commissioned as an Advance Therapy Treatment Centre (ATTC) to provide CAR-T to adult and paediatric UK patients.

MFT pharmacy division created in collaboration with Clinicians, Stem Cell Lab the Governance infrastructure (policies and procedures) to comply with national regulations and guidelines, in an effort to be commissioned as an ATTC.

## Role of Pharmacy

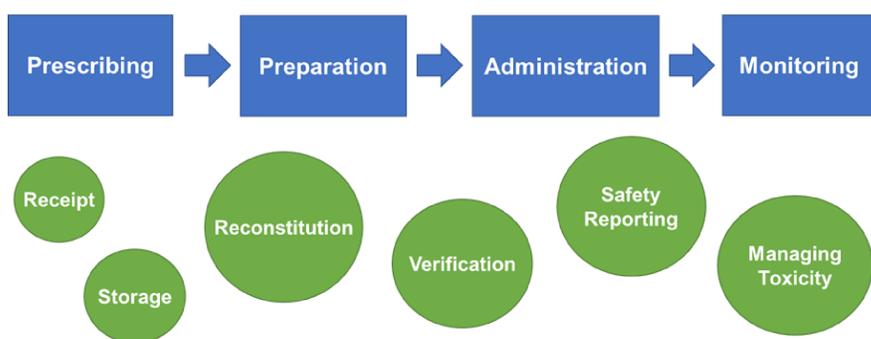


Figure 1: Role of pharmacy: Establishing Governance for a safe ATMP delivery to patients.

## Aim

This case study illustrates the processes MFT pharmacy division followed for a successful implementation of governance and operational procedures in line with current regulations, to be commissioned as an ATTC for CAR-T

## Method: Identifying actions required for commissioning

Governance, quality and safety gap analyses	<ul style="list-style-type: none"> <li>• Performed using current ATMP legislation<sup>3</sup> and national guidance.</li> </ul>
Audits	<ul style="list-style-type: none"> <li>• Of trust governance systems and processes, including clinical settings and the stem cell laboratory (SCL) prior to Joint Accreditation Committee ISCT-Europe &amp; EBMT (JACIE) accreditation.</li> </ul>
Pharmaceutical companies	<ul style="list-style-type: none"> <li>• Performed audits and delivered specific training for their products.</li> </ul>
Collaboration	<ul style="list-style-type: none"> <li>• Regular multidisciplinary meetings.</li> <li>• A task and finish group - including clinical/medicines management pharmacists and clinical trials, stem cell lab (SCL). staff.</li> </ul>

## Results

A list of Actions were identified:

- Creation and implementation of an ATMP policy.
- Oversight of the SCL for licensed products as per current clinical trial processes:
  - Audit to SCL management systems and processes performed by Pharmacy for this purpose and an audit plan was drafted.
  - Consideration to set a technical agreement between pharmacy and the stem cell lab to delegate some activities, which are currently done under section 10 exemption. Roles and responsibilities were agreed with the stem cell lab and pharmacy.
- An Advance Therapy Assurance Committee was approved to feedback to the Medicines Optimisation Board: Strategic planning priorities established.

MFT successfully received ATTC status in the first wave of commissioned centres.

First Adult Patient was treated in April

First Paediatric patient was treated in May

## Risk Assessment

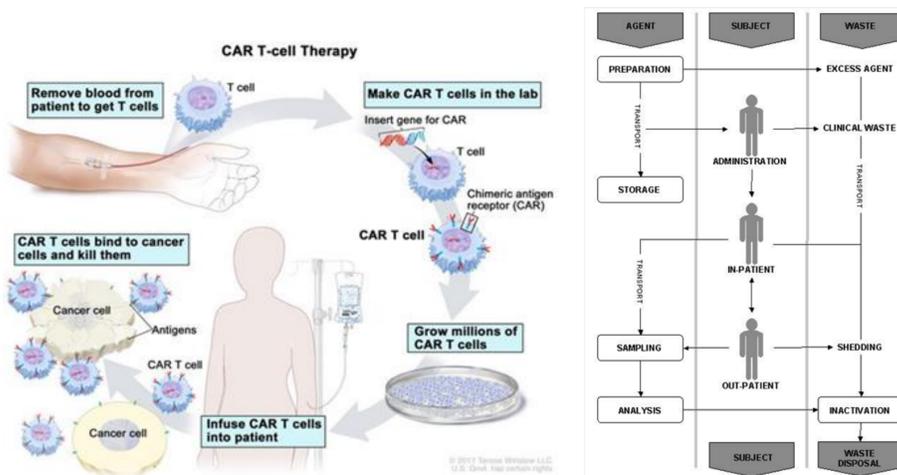


Figure 2: CAR-T therapy overview.

Ref: HSE-SACGM Compendium of guidance part 6

## Discussion

With labs and ASUs at capacity, expansion and future proofing are required.

Requirements going forward:

- Careful assessment to anticipate and identify key areas of focus to ensure compliance and patient safety.
- Streamlined and adaptable procedures applicable to all potential ATMP trials.
- Use horizon scanning to predict likely dates of ATMP implementation
- Trust advanced Therapy Assurance group formed reporting to Medicines Optimisation Board

Challenges going forward:

- Horizon Scanning: 30 new ATMPs
- Communication, equipment, data management systems: 30 years
- Capacity and contingency planning.
- Brexit.

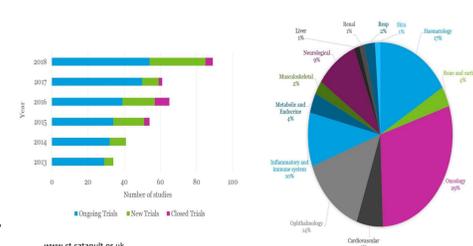


Figure 3: UK ATMP trials Growth.

## Acknowledgements

ATMP Pan-UK Pharmacy working group, iMATCH, Stem Cell Lab and Specialist Pharmacy Service (SPS)

## References

1. Stoner N. Are UK hospital pharmacy departments ready for the rise of gene therapy medicinal products? Expert Opin Biol Ther, 2018; 18 (8): 837-840.
2. Jørgensen J, Mungapen L & Kefalas P. Data collection infrastructure for patient outcomes in the UK – opportunities and challenges for cell and gene therapies launching, J Mark Access. Health Policy, 2019; 7 (1): 1573164.
3. Detela G, Lodge A. EU Regulatory Pathways for ATMPs: Standard, Accelerated and Adaptive Pathways to Marketing Authorisation, Mol Ther Methods Clin Dev, 2019; 13 (1): 205–232.