

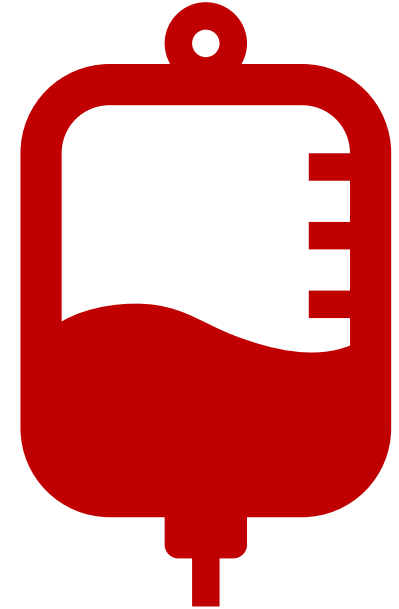
Blood & Tissue Establishments – An Expanding Role in Advanced Therapeutics

Irish Blood Transfusion Service - Research Day - 29th of November 2023

Aisling Horan

Senior Quality Scientist, Bacterial Testing

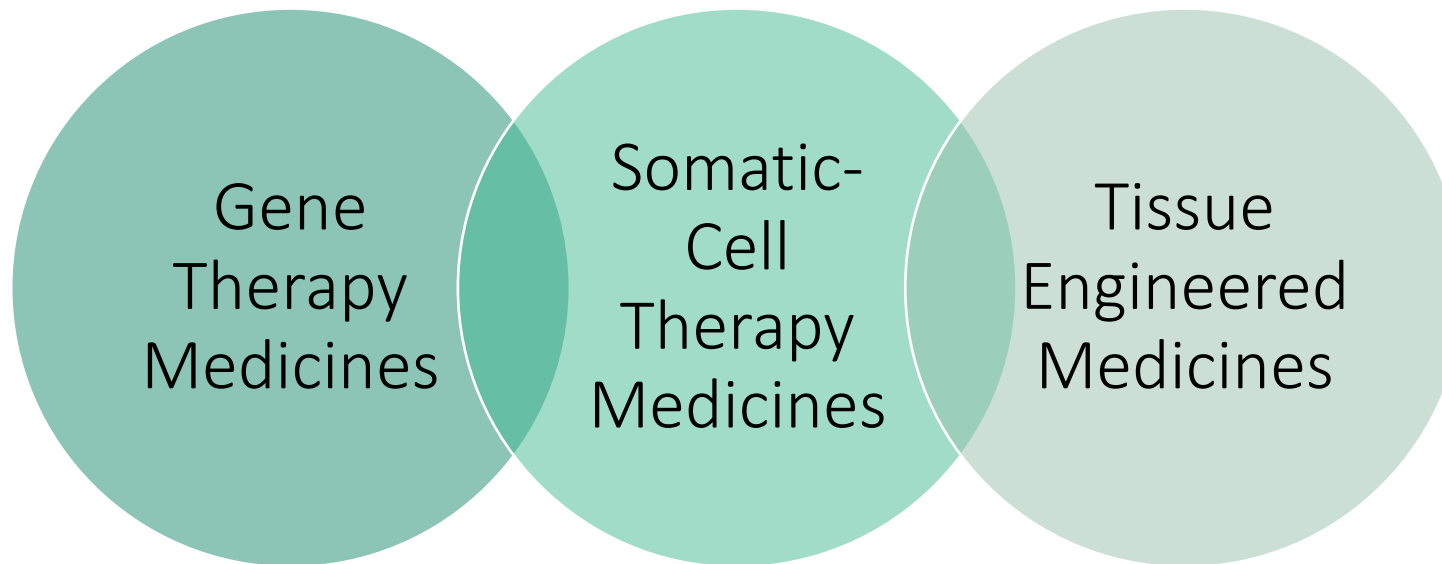
Irish Blood Transfusion Service



What are Advanced Therapeutics? ⁽¹⁾



- Advanced therapy medicinal products (ATMPs) are medicines for human use that are based on genes, tissues or cells



- Highly specialised treatments for patients with unmet medical needs for chronic & debilitating conditions

Project Background ⁽²⁾



- Advanced Therapies a core module of MSc in Pharmaceutical Quality Assurance and Regulation at Technological University Dublin
- Increasing interest in ATMPs in Ireland – Centre for Cell Manufacturing/REMEDI in Galway, Introduction of CAR T therapy at St James Hospital in 2021, Cell Therapy Facility at Takeda in 2022, Launch of ATMP.ie website in 2022.
- IBTS Strategic Plan 2021 –2025 specifies the need to develop "***A strategy to facilitate IBTS in the introduction of GMP compliant regenerative / cellular medicines.***"
- Recognised with development of 'Cellular Therapy Research Group' between St. James Hospital, Trinity College Dublin and IBTS

Research Question & Project Aims



Is there scope for the Irish Blood and Tissue establishment to extend its service to support ATMP advancement in Ireland?



Investigate the opportunities a blood establishment has to facilitate the development of novel advanced therapies



Gauge interest among ATMP stakeholders in Ireland to the concept of increased blood establishment support



Explore existing partnerships between blood establishments and ATMP development in Europe



Assess the viability of this potential role for the IBTS and determine the challenges to its implementation



Determine next steps for the IBTS to facilitate advanced therapies in Ireland

European Blood & Tissue Establishments (BTEs) & ATMPs

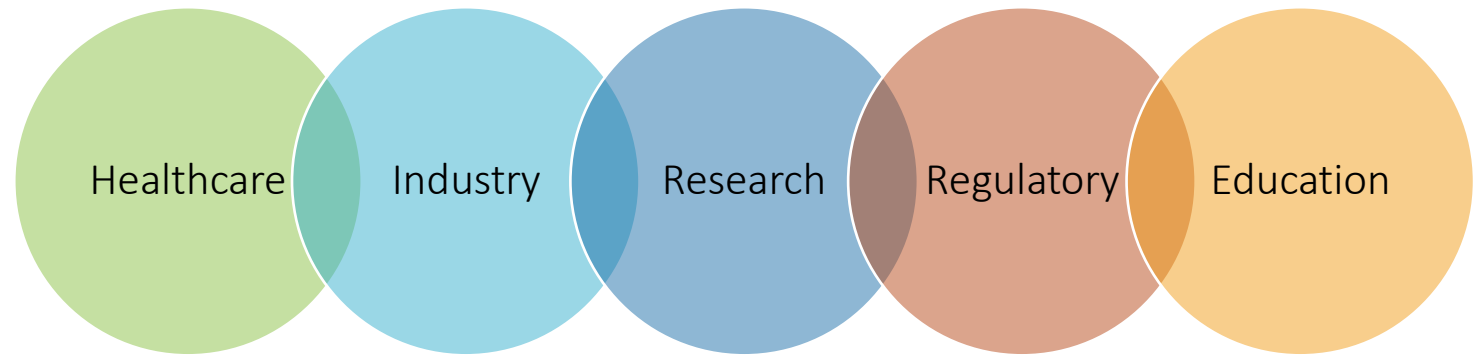
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- Potential of BTE's for supporting ATMPs has been recognised and applied by several European establishments
- Leaders include Banc I Sang de Teixitis, Barcelona, NHSBT & SNBTS, UK and Sanquin, Netherlands.

Research	Manufacturing	Logistics	Education
Academic Partnerships	Provision of Starting Materials	Cold Storage Solutions	Subject Matter Experts
Healthcare Collaborations	GMP Manufacturing	Shipping/Distribution	Training Partnerships
Clinical Trials	Process Development	Donor Management	Online ATMP resources

Survey of Irish ATMP Stakeholders



ATMP Stakeholder Information

Blood Establishments and ATMPs

Challenges associated with ATMP Advancement in Ireland

Regulatory Considerations for ATMPs

Future Prospects for Blood Establishments and ATMPs

ATMP Stakeholder Survey



85% believed Blood and Tissue Establishments have a role to play in advancement of ATMPs



67% are involved directly with research, development or supply of products for haematological conditions



61% felt they would directly benefit from increased BTE support for ATMP progression

ATMP Stakeholder Survey



Key challenges associated with ATMP advancement in Ireland

Funding

Lack of ATMP Expertise

Understanding ATMP Regulatory Requirements

Government Support

ATMP Quality Assurance

Acquisition of Starting Materials

ATMP Stakeholder Survey



BTE services/skills applicable to ATMP advancement in Ireland

Expertise in Blood, Cells
and Tissue Regulation

Provision of Blood
Components for ATMP
research

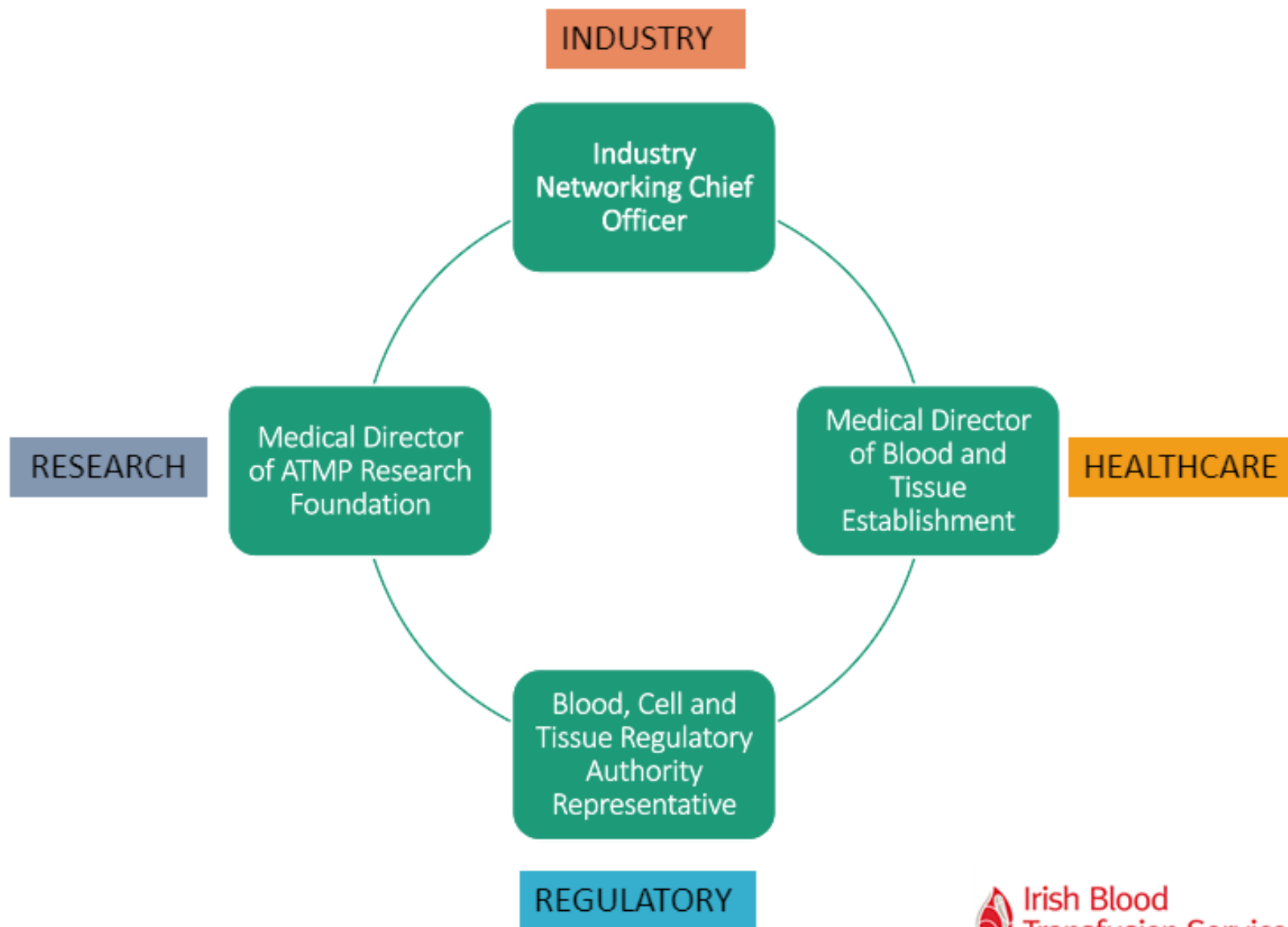
Access to Starting Materials

Expertise in Therapeutic
Apheresis

Product Logistics –
Cryopreservation, Shipping
& Distribution expertise

Research Collaborations

Subject Matter Expert Interviews



Strategic ATMP Development in Blood and Tissue Establishments



BTE Strengths	BTE Requirements
<ul style="list-style-type: none">• Primary source of substances of human origin in the country• Collection, preservation, storage transport and delivery of cell-based products• Licenced by national regulatory authority• Member of EBA• Product Screening• Quality controlled processes• Robust Traceability Policies• Collaborative clinical relationships between blood establishments and relevant healthcare personnel• Professional development training• Donor Recruitment• Upstream Manufacturing Processes• Irish Unrelated Bone Marrow Registry	<ul style="list-style-type: none">• Capital Investment• Increasing ATMP knowledge• Production of platelet lysate and cells• GMP cleanroom space• Increased GMP manufacturing capabilities• Explore private/public partnership• Development teams• Project collaboration with European BTEs• Diversity of donors• Understanding of SoHO regulation• Strategic Planning

BTC for ATMP Development And Manufacture (BADAM) model ⁽⁷⁾



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Review article

Manufacturing processes

A management model in blood, tissue and cell establishments to ensure rapid and sustainable patient access to advanced therapy medicinal products in Europe

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Chantal Lechanteur⁶, Etienne Baudoux⁶, Nicola Gardiner⁷, John De Vos⁸, Joaquim Vives^{1,9,10,**}

All-Ireland Integrated Innovation Hub



- **Centre for Advanced Precision Cellular Therapeutics**
- **A recent proposal from Trinity College Dublin to form a collaborative network for therapy development in Ireland**
- **Aim to promote personalised cell therapies as first line treatments**
- **SME's assigned to working groups in Design, Manufacturing, Evaluation, Translation, Training**
- **Opportunity for expanding IBTS partnerships & building ATMP knowledge**

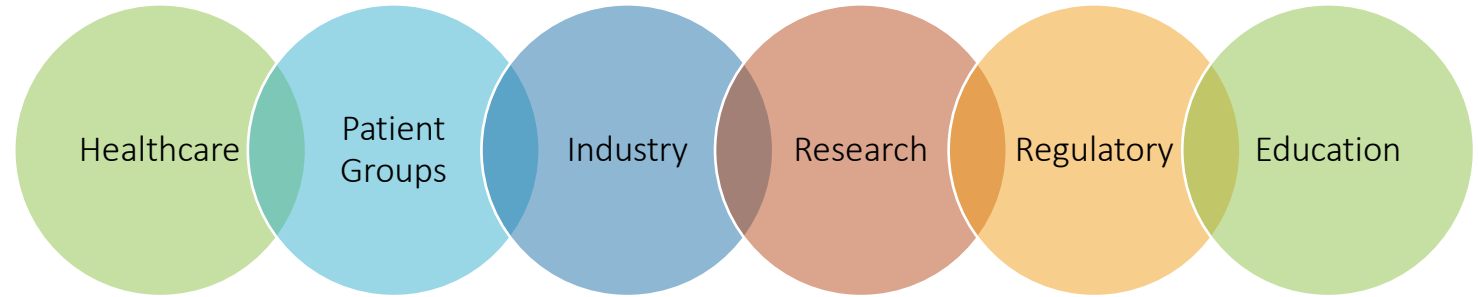
Recommendations & Next Steps ⁽⁷⁾



- *Appoint a Multidisciplinary Team*
- *Build ATMP expertise*
- *Foster ATMP relationships*
- *Collaborate with European Blood Establishments*
- *Assess Current IBTS Strengths and Requirements*

- *Horan et al, Manuscript in Progress. The Expanding Role of Blood and Tissue Establishments in the Development of Advanced Therapy Medicinal Products – An Irish Perspective*

Conclusions



- **Blood and Tissue Establishments globally are supporting the development of ATMPs**
- **There is significant interest from ATMP developers, researchers, clinicians and supporting services for the IBTS to become more involved with the Irish ATMP industry**
- **Current BTEs services and partnerships can be leveraged to support the development of advanced therapeutics in Ireland**
- **Key to the expansion of service is the maintenance of current protocol to ensure products are of high quality, safe and effective for patients**

Thank you for joining!

Acknowledgments:

Survey Respondents

Subject Matter Experts

Shada Warreth, NIBRT

Allison Waters, IBTS

Elaine Harris, TU Dublin

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