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# Advantage Australia: a stepping stone to Asian and global development of cellular therapies

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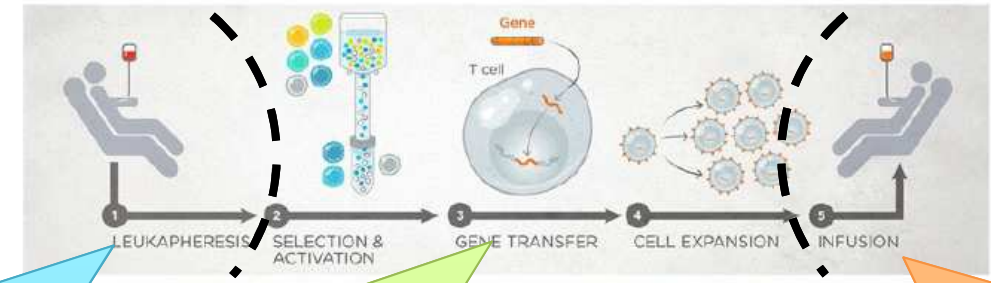
# Summary

- Australia has all the necessary elements to reduce risks associated with scale development of cellular therapies
- The Australian regulatory environment enables globalisation via Australia to occur rapidly at any stage of development

# Significant challenges remain for high quality, rapid deployment




**Clinical site: collections**

- Limited experience in specialised, non-mobilised patient apheresis
- Inconsistent quality systems and analytical capability
- Need to maximise collection data
- Cryopreservation capability?
- Patient screening requirements
- Impact on production failure rates

**Manufacturing**

- Single global hubs cannot respond to patient demand
- Limited ex-US experience in cGMP cellular immunotherapy production (especially Japan): comparability, consistency risks
- Turning on a network takes time and major effort
- COGS, consistency, scalability

**Clinical site: patient access and care**

- Competition for trial patients, especially in US
- Limited on-site product storage, dispensing
- Patient scheduling and conditioning

***Building clinical centres of excellence integrated with regional manufacturing hubs can de-risk and accelerate global deployment***



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# Australian cellular therapy eco-system

- **>28 clinical trials open**
- No approved products (several applications, autologous exemption)
- First industry sponsored CAR-T patient treated outside N America
- Rapid recruitment; ethnically diverse

Clinical trials and products

- **>15 companies** focussed on product development or infrastructure support

Companies

- **>30 research centres**
- Several stem cell and regenerative medicine focussed centres
- World class research hubs and precincts integrating basic and clinical research and translation

Research

- **Rapid (14 day) Clinical Trial Notification** for first in human and IND approved trials
- Biologics framework since 2011
- Mutual recognition of GMP with EU
- No local study requirement for BLA
- **GMP manufacturing licenses, includes collection**

Regulatory

- **R&D tax incentive** (40-45% tax refund or offset)
- Co-operative Research Centre and Industry Growth Centre programs
- Austrade regen medicine missions

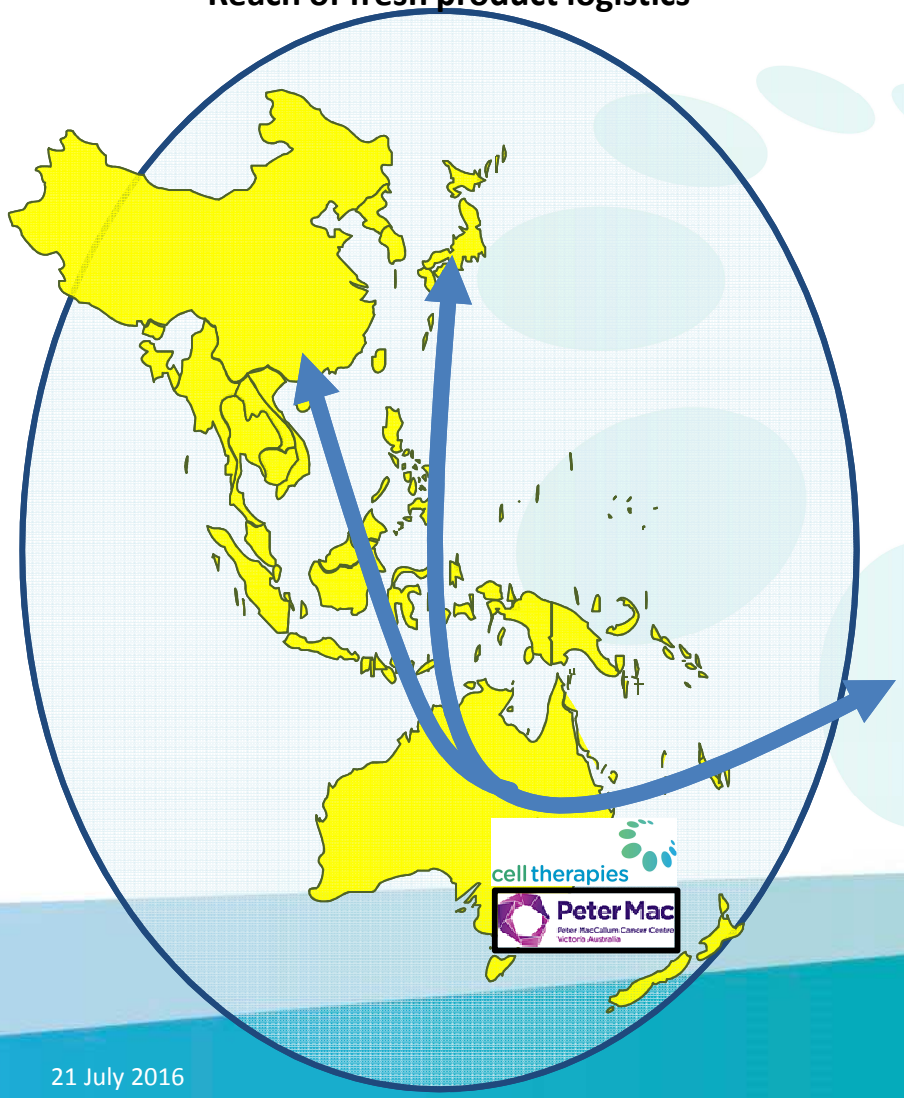
Government support

# Efficient CTN clinical trial approval process

| | Clinical Trial Notification (CTN) | Clinical Trial eXemption (CTX) |
|---|---|---|
| Process | • Ethics review + 14 days for TGA to issue CTN number<br>• Limited CMC required | • Ethics review + TGA review (9-12 months)<br>• Full CMC required (as for IND) |
| Applicable to | • First in human studies (except gene modified)<br>• Studies with US IND or EU CTD approval | • Any product where ethics committee feels unqualified to evaluate<br>• Gene modified products |
| Opportunities | • Fastest route to clinic anywhere<br>• Generate clinical data for IND<br>• Rapid extension/expansion of multi-centre clinical trials | • Procures GMP certification of manufacturing that is often required in Asian jursidictions<br>• <10% of all Australian clinical trials |

# Australia: a gateway to and from Asia for clinical and commercial scale cellular therapy manufacturing and deployment

**Reach of fresh product logistics**




**Opportunities**

1. *World class Australian technologies*
- Rapid generation of clinical data to support regulatory filings in global markets
- All the infrastructure to support ongoing development and optimisation

2. *Western products*
- Rapid expansion of clinical trials/BLA's
- Familiar system – but able to reach Asia logistically ($850^{+}$m patients)
- Ongoing process development
- Controlled test environment for scale deployment

3. *Asian products*
- Translate to western GCP and GMP environment in same time zone

4. *Common benefits*
- Cost effective
- Quality

# Case studies

## Australian mesenchymal technology: outbound

- First generation autologous product: GMP process development and Phase 1 trial
- Data supported US allogeneic IND, minimising additional clinical data needed

## Australian dendritic cell vaccine: outbound

- GMP process development and CTX
- Cell collection network in EU, Korea
- Technology transfer to EU and US CMO's for global multicentre study under US IND

## US gene-modified immunotherapy: inbound

- Asian node of global autologous network
- R&D tax credit
- Global pivotal study under US IND: CTN in Australia
- First ex-North America patient; highest recruitment

## US gene-modified stem cell therapy: inbound

- US start-up – lab scale
- Process development/ optimisation in Australia
- R&D tax refund opportunity
- FIH data potential on unmodified vehicle for IND submission

## US gene therapy: global deployment

- CTPL/Invetech partnership to design global deployment system (production + supply chain) for US technology

thank you

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