

Regenerative Medicine Business Models

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23 May 2017



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WP 3 Objectives

- Business Models
- Value Chains
- Innovation Ecosystems
- 10 Case studies in the UK
 - Private Firms (7)
 - Public Organisations (1)
 - University Spin Offs (1)
 - Research and Development Consortia (1)
- Case studies at various development and commercialisation stages
- None have marketing authorisation
- 1 used specials/unlicensed medicines during clinical trials for reimbursement



Regenerative Organisations Case Studies

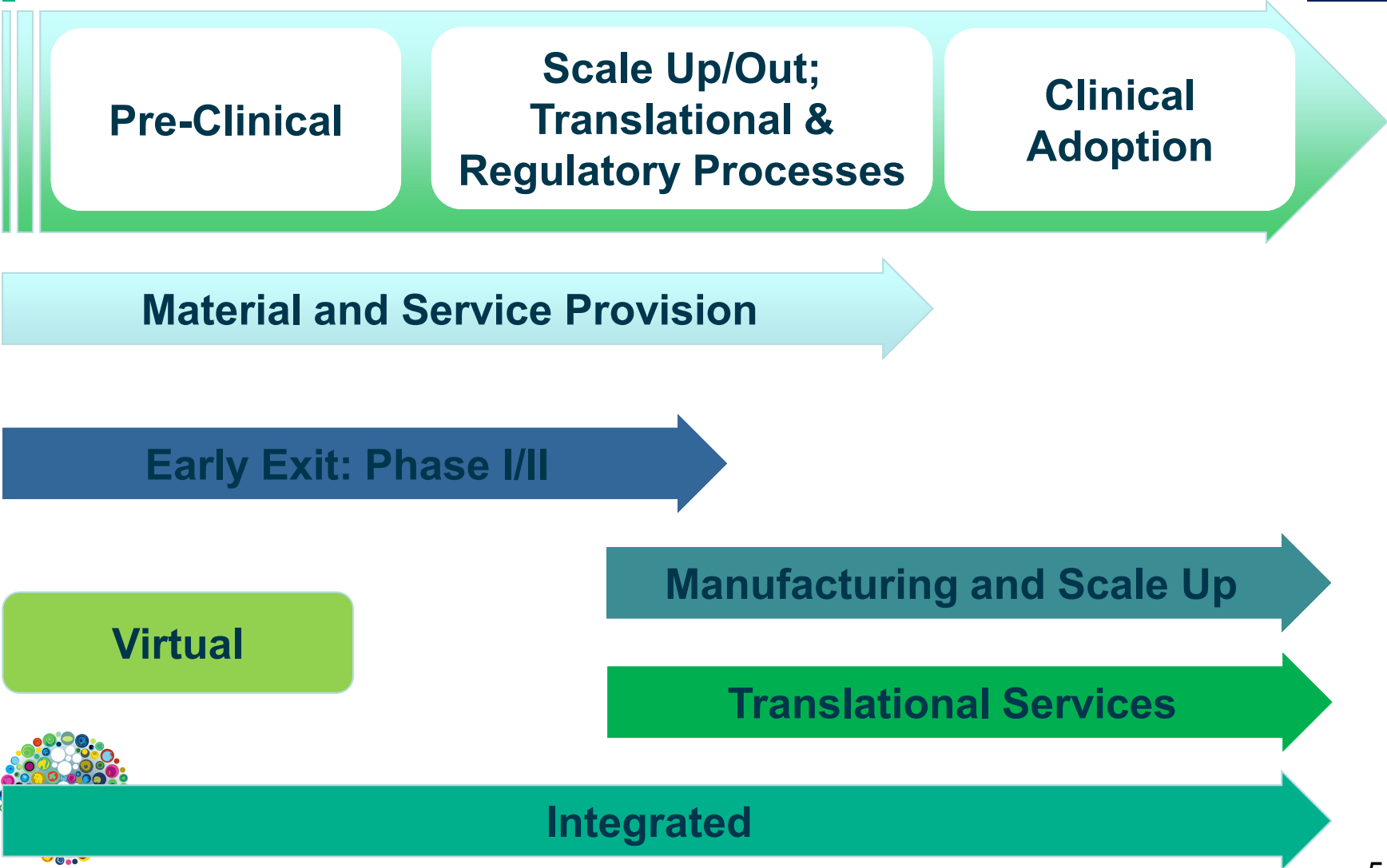
Type of Organisation and Therapy Area	Funding Sources	Therapy Stage
[Firm A]: <i>autologous</i> - Immunotherapy	Grants + PE > £70m	Clinical Trials
[Firm B]: <i>autologous</i> - Immunotherapy	Public + PE + Financial Institutions ~ >£5m	Clinical Trials
[Firm C]: <i>autologous</i> - Cell therapy + Surgery	Grants and PE	Clinical Trials?
[Firm D]: <i>autologous</i> – Tissue engineering + Surgery	Grants and PE > £8m anticipating Euro 7m	Clinical Trials/ Compassionate Use
[University Spinoff] - Cell therapy	Grants	Animal Studies
[Firm E]: <i>drug discovery</i>	Grant + Others	CMO
[Firm F]: <i>allogeneic</i> - Cell therapy	PE + Grants >£40 m	Clinical Trials
[Public Organisation]: <i>allogeneic</i> – Cell and organs	Public	CMO
[Firm G]: <i>allogeneic</i> - Cell therapy	PE + Public funds (£52 m). Burn rate £1 m p.m.	Clinical Trials – Specials / Unlicensed
[Research Consortium]: <i>allogeneic</i> - Cell therapy	Grants: ~ £15m over 8 yrs	Pre-Clinical

Therapies and products covered in the case studies

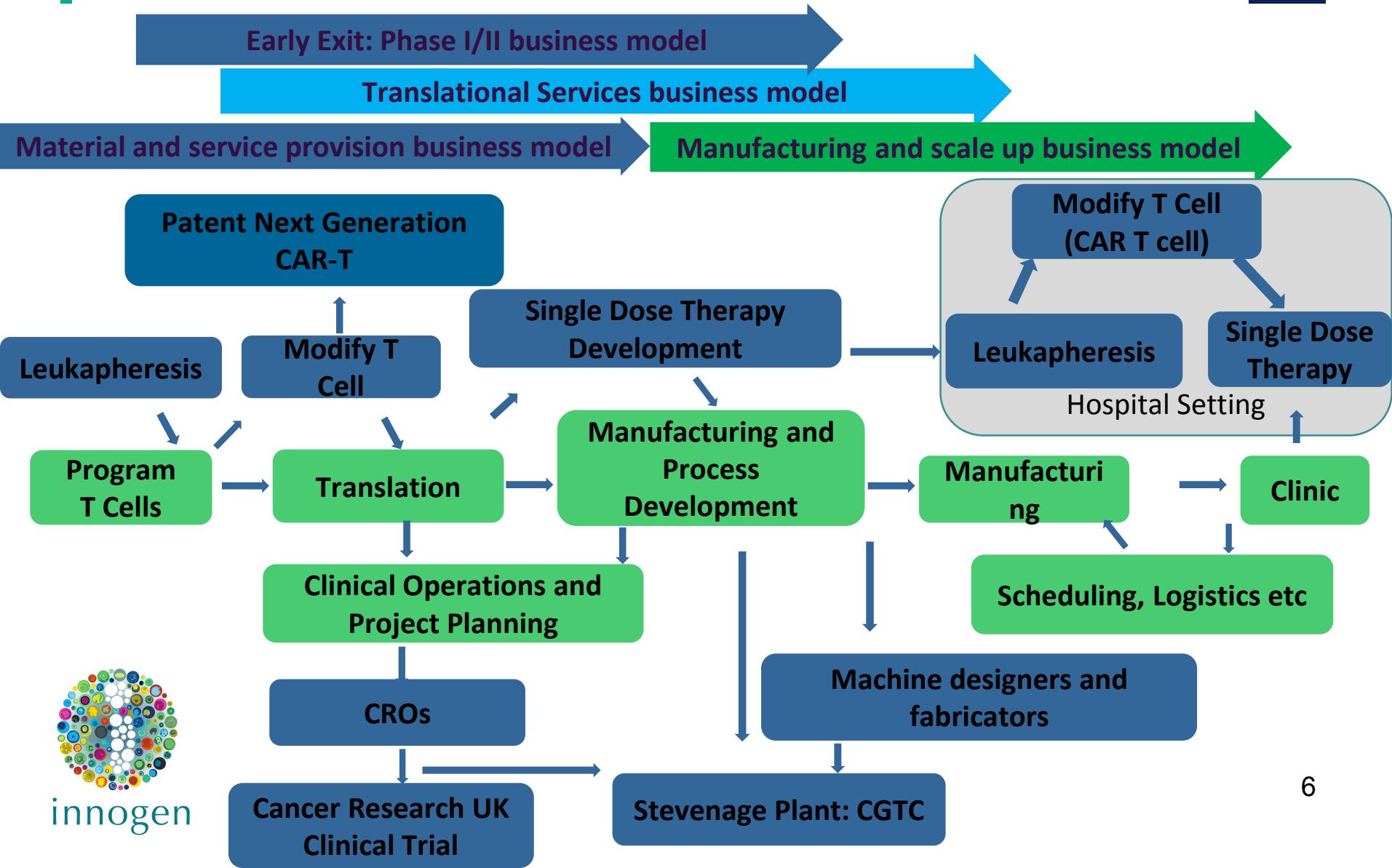
- Ophthalmology
- iPSC (induced Pluripotent Stem Cells)
- Chimeric Antigen Receptor Technology
- Autologous Immunotherapies
- Solid Tumours
- Blood Cancer (myeloma)
- Epidermolysis Bullosa
- Meniscal tear repair
- Beta Thalassemia
- Osteoarthritis
- Parkinson's and brain treatment
- Stem cells for drug discovery
- Organ generation
- Severe Influenza and Ebola
- HIV
- Pancreatic islets transplant
- Alzheimer's



Business Models



Innovation Pathway- Immunotherapy



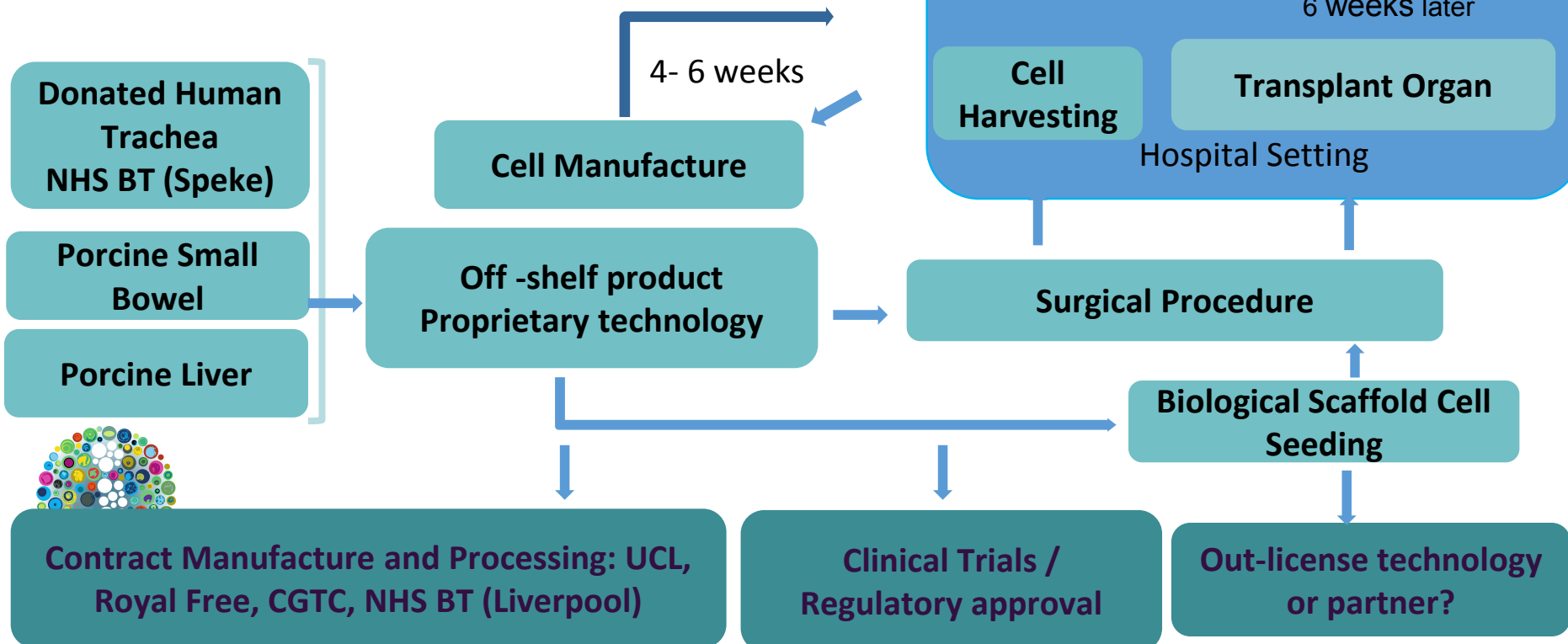
Innovation Pathway- Tissue Regeneration

Early Exit: Phase I/II Business Model

Translational services Business Model

Material and service providers Business Model

Manufacturing and scale up Business Model



Why the Virtual Business Model?

- Survival Strategy
- Keep cash burn rate low

You need small amounts
of money coming in
regularly

What burns cash?	Actors Supporting the Virtual Business Model
Process Development	CMOs and Universities
Clinical Trial Design and Management	CROs
Clinical Trial Data Management	
Regulatory Advice	Translational Services Providers
Manufacturing for Phases 1 & 2	Academic CMOs
Manufacturing for Phase 3	CGTC Stevenage Plant



National Innovation System Support

*It [CGTC] really is **a perfect model** because ... it **de-risks the manufacturing** for us. We don't have to invest in building our own building, **so we don't have that cost**. ...it also means **we don't have to give away, or trust someone else to do it [manufacturing] for us**. We **own the manufacturing and control it and also learn it**, ...when the time is right and we've proven that the medicine works and it looks like we're ready to commercialise it then we **build our own facility...** So you **push the risk**, the point at which you have to **invest your own money** in a building, further down the development pathway*

- The catapult was good for UK Plc [innovation infrastructure] as it was created to be internationally competitive and attract companies to the UK
- The NHS [clinical] and NHS Blood Transfusion has provided infrastructure for innovation
- Universities featured prominently in all 10 case studies and critical for training and retaining skills in niche areas



innogen

National Innovation System Support

- NHS
 - Funders:
 - Research Councils
 - Regional Regeneration Funds
 - Charity Organisations and others*
 - CGTC
 - Regulatory and translational service advice
 - CMO – Stevenage Plant
- Mostly Public Funds
- Muscling out of the smaller service for a fee SMEs
 - What happens when the state withdraws its support later on?
 - How fast can SMEs step in to fill the innovation infrastructure/support system gap?



*I think we were going to ...make more money selling shovels [instead of prospecting for gold] And it was **working...it was beginning to work**. We were already cash neutral and we were beginning to work on interesting products. ...And then the Cell Therapy Catapult came along...So it **pulled the rug completely from underneath us...** they put 100 million in to it...We couldn't compete with that...*

Quick Takeaways

- Small indications attractive to small firms but not attractive to big pharma (not block-buster) especially for early exit [unmet need]
- Small indications come with clinical trial recruitment challenges
- Materials and service provision; CMOs and Translational Service: Only players with cash inflows from business activities
- Players in development activities are all in “cash-burn” activities
- University based players prefer the virtual business model to limit cash burn rate



Conclusion

- Field dominated by Small to Medium Enterprises (SMEs)
- Only 2 business models generating revenues
 - Material and service provision
 - Manufacturing and scale up
- CGTC – key innovation infrastructure supporting resource limited firms
- NHS - clinical and transfusion services key innovation infrastructure
- Skills development and retention critical for the sector
- There is a mix of Grant, Private Equity funding: ranging from £5 million to £70 million - most funding is for development work and clinical trials
- At some point **re-imburement** needs to be addressed to act as an innovation puller [Innovative Procurement]



REGenableMed Project



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