

CATAPULT
Cell and Gene Therapy

Challenges of ATMP Development and Supply

July 2018

Dr. Michael Bennett
Director of Business Development

Where are we now

861+ regenerative medicine companies worldwide, including cell and gene therapies

464

North America

233

Europe and
Israel

64 companies
in the UK

126

Asia

15

South America

1

Africa

22

Oceania

CATAPULT
Cell and Gene Therapy

Cell and Gene Therapy Trials Worldwide

Gene and Cellular Therapies and Other Regenerative Medicine Products – Q1 2018 Clinical Trials

959

Clinical trials underway
worldwide by end of Q1 2018

Ph. I: 320

Ph. II: 549

Ph. III: 90

Number of Clinical Trials Utilizing Specific RM/AT Technology: Q1 2018

Gene Therapy

Total: 319

Ph. I: 110

Ph. II: 174

Ph. III: 35

Gene-Modified Cell Therapy

Total: 284

Ph. I: 121

Ph. II: 152

Ph. III: 11

Cell Therapy

Total: 332

Ph. I: 84

Ph. II: 211

Ph. III: 37

Tissue Engineering

Total: 24

Ph. I: 5

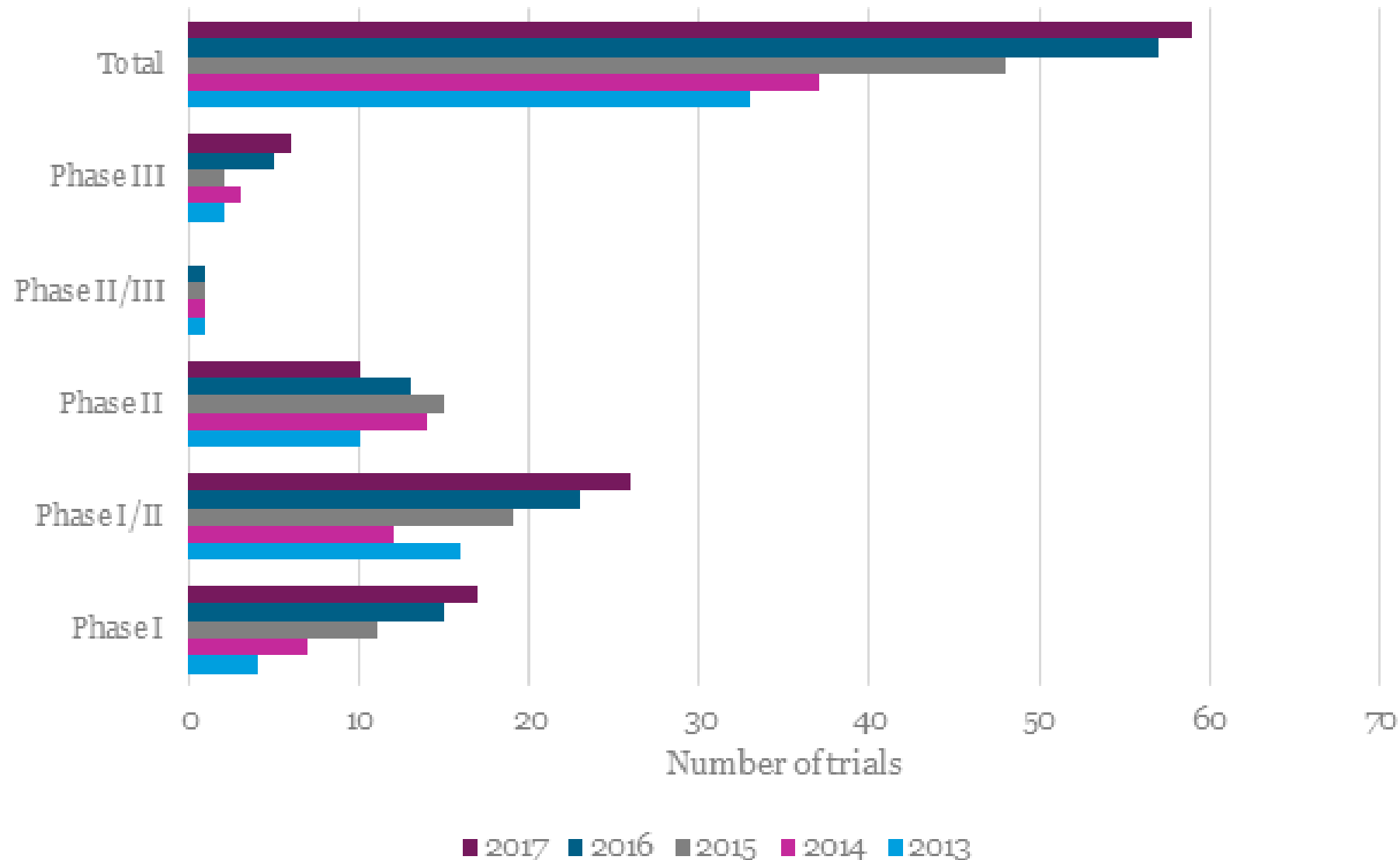
Ph. II: 12

Ph. III: 7

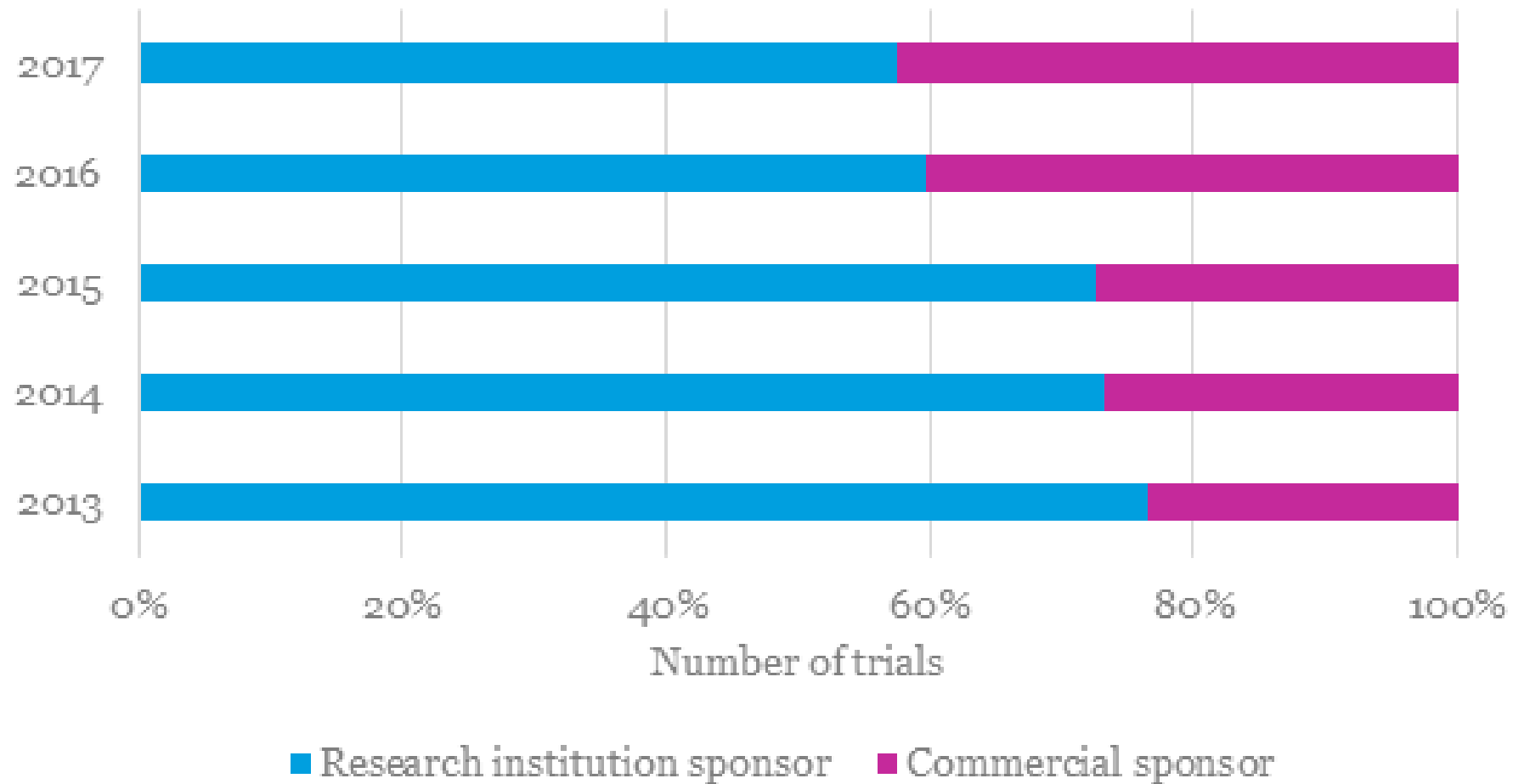
*Total number of clinical trials represents sector-wide figures; please note that products employing cell-based immunotherapy are accounted for in both the gene therapy & gene-modified cell therapy and cell therapy sectors. As a result, the total number of clinical trials does not equal the sum of the trials within the individual technology groups.

ALLIANCE
for
Regenerative Medicine

In the UK the majority of therapies are in early phase trials



Growing numbers of UK clinical trials are sponsored by a commercial organisation



Approved cell and gene products in Europe

	Product	Target	Year	Note
1	ChondroCelect	Cartilage repair	2009	withdrawn from use
2	MACI	Cartilage repair	2012	suspended for use
	Glybera	Treatment of LPL deficiency	2013	Exceptional circumstances [License expires 25 October 2017, will not renew]
3	Provenge	Treatment of advanced prostate cancer	2013	withdrawn from use
4	Holoclar	Treatment of limbal stem cell deficiency	2015	conditional
5	Imlygic	Treatment of advanced melanoma	2015	Standard, Reimbursed in the UK
6	Strimvelis	Treatment of immunodeficiency due to adenosine deaminase deficiency (ADA-SCID)	2016	conditional
7	Zalmoxis	Adjunctive treatment in (HSCT) of adult patients with high-risk haematological malignancies	2016	
8	Spherox	Cartilage repair	2017	

Look at the price tags!



Strimvelis

£525,000

Ex-vivo stem cell gene therapy to treat a very rare disease called ADA-SCID (Severe Combined Immunodeficiency due to Adenosine Deaminase deficiency)

Estimated to occur in ~ 15 patients per year in Europe



KYMRIAH™

£350,000

An ex-vivo CD19 immunotherapy approved in the United States for the treatment of patients up to 25 years of age with B-cell precursor ALL that is refractory or in second or later relapse.

Expected to treat a few hundred children per year



YESCARTA™

£265,000

A CD19-directed genetically modified autologous T cell immunotherapy indicated for the treatment of adult patients with relapsed or refractory large B-cell lymphoma after two or more lines of systemic therapy



LUXTURNA™

£600,000

AAV gene therapy to treat biallelic RPE65 mutation-associated retinal dystrophy that leads to vision loss and may cause complete blindness in certain patients.

ATMP manufacturing and supply challenge



Cells taken from patient

Manufacture

Supply chain

Arrival at hospital

Delivery to patient

Payment

Challenges

- Approved facility
- Trained staff
- Tracking

Challenges

- Cost of goods
- Analytics
- Product release
- Scale

Challenges

- Temperature
- Time restrictions
- Patient specific

Challenges

- Thawing
- Registry

Challenges

- Pharmacy
- Tracking
- Training

- **Accelerate** the commercialisation of innovations from research
- **Complement** industry and academia with **unique technical facilities and expertise**
- **Innovate** in collaboration with academic and industry network
- **Facilitate** operating in UK as a global centre; working with Government, the NHS and international regulators

Development laboratories

- 1200m² purpose built centre
- Analytical characterisation
- Process development
- Viral vector



GMP manufacturing development centre

- 7200m² manufacturing centre designed specifically for cell and gene therapies
- 12 segregated large modules
- Secure supported collaboration model
- Centre of a CGT industry cluster



Cell and gene therapy specialists (>170)

Industrialisation

- Process development
- Analytical development
- Manufacturing systems
- Supply chain

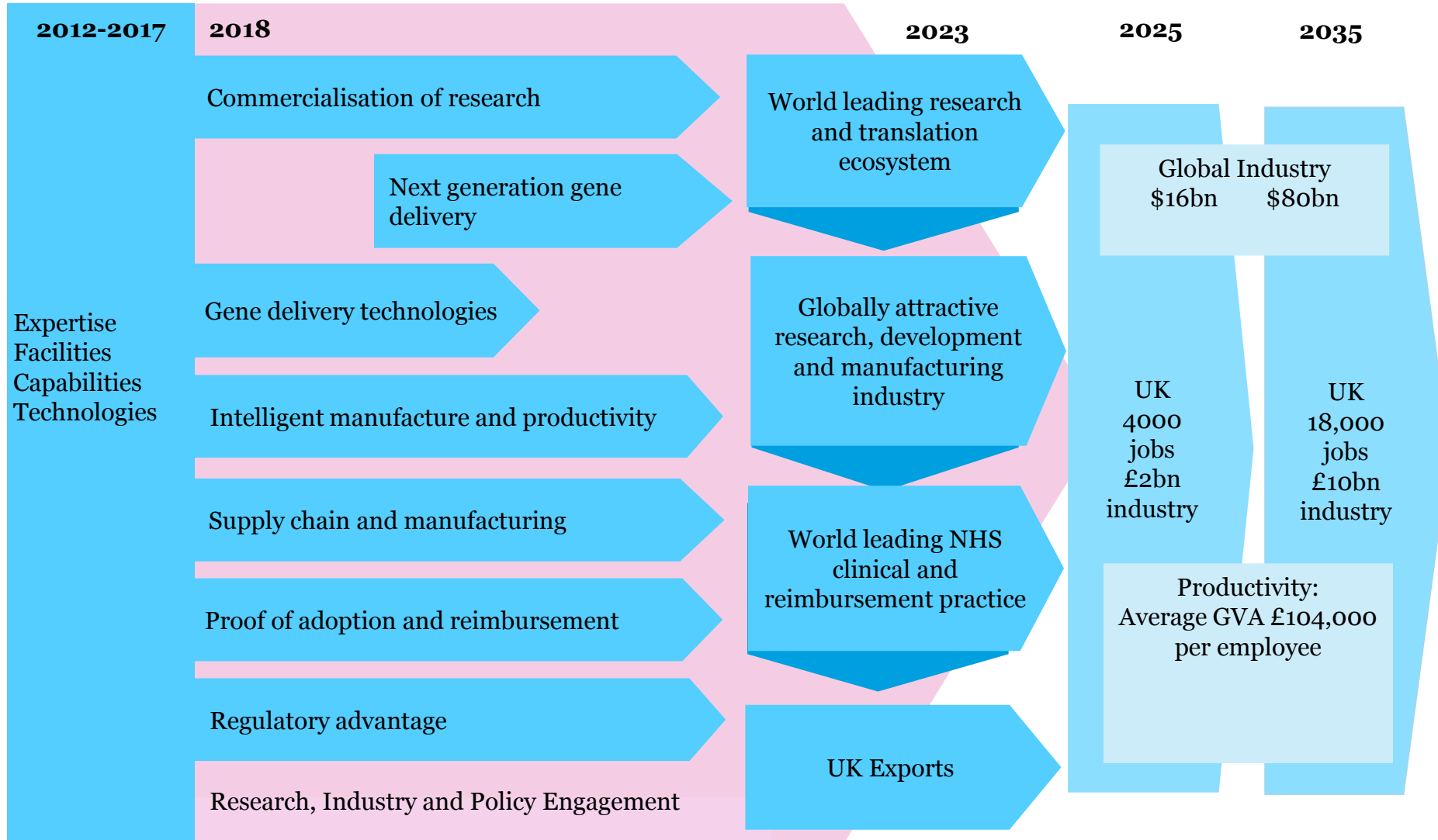
Regulatory and clinical development

- Regulatory
- Non clinical safety
- Clinical programmes
- Programme management

Engagement

- Collaboration formation
- Intellectual property and patent
- Health economics
- Reimbursement

What is CGT doing?



ISCF funded Advanced Therapy Treatment Centres



Northern Alliance Advanced Therapies Treatment Centre

iMATCH - Manchester Advanced Therapy Centre Hub

MW-ATTC - Midlands & Wales Advanced Therapy Treatment Centre

CGT Manufacturing centre



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