

Advanced Therapy Medicinal Products Project

Perspectives on preparedness: healthcare system readiness for adoption of disruptive innovative technologies

October 2019

The project

Having led the NHS service development work for the first two chimeric antigen receptor T-cell (CAR T) therapy products in England, I became interested in how the wider system would prepare for the next generation of Advanced Therapeutic Medicinal Products (ATMPs). I wanted to hear from partners about what they thought it would take for patients to get access to ATMPs in future. So, working as part of the Cell & Gene Therapy Catapult, I led this project to progress the agenda and my leadership as part of the Health and Care Leaders Aspiring Director Programme.

We discovered that whilst affordability is a key issue, it can crowd out other factors, reducing the adoption debate to price setting and reimbursement between companies and health service payers. Through our interviews with industry, regulators, policy makers and others, we heard about many other important issues for ATMP adoption. We asked partners about their contribution to this agenda and about their perceptions of the roles, responsibilities, actions and constraints of others. We found many points of alignment as well as significant differences in perspectives.

In 2019, the NHS Long Term Plan recognised the importance of expanding the frontiers of medical science and innovation. In September, NICE recommended access to another ATMP on the NHS. There can be no better time to build greater understanding and collaboration. Working as partners, we can ensure the right actions are taken forward at the right time. Individually and together our efforts can secure benefits for patients, healthcare, industry and the UK.

I hope you enjoy reading the report as much as I enjoyed researching it!

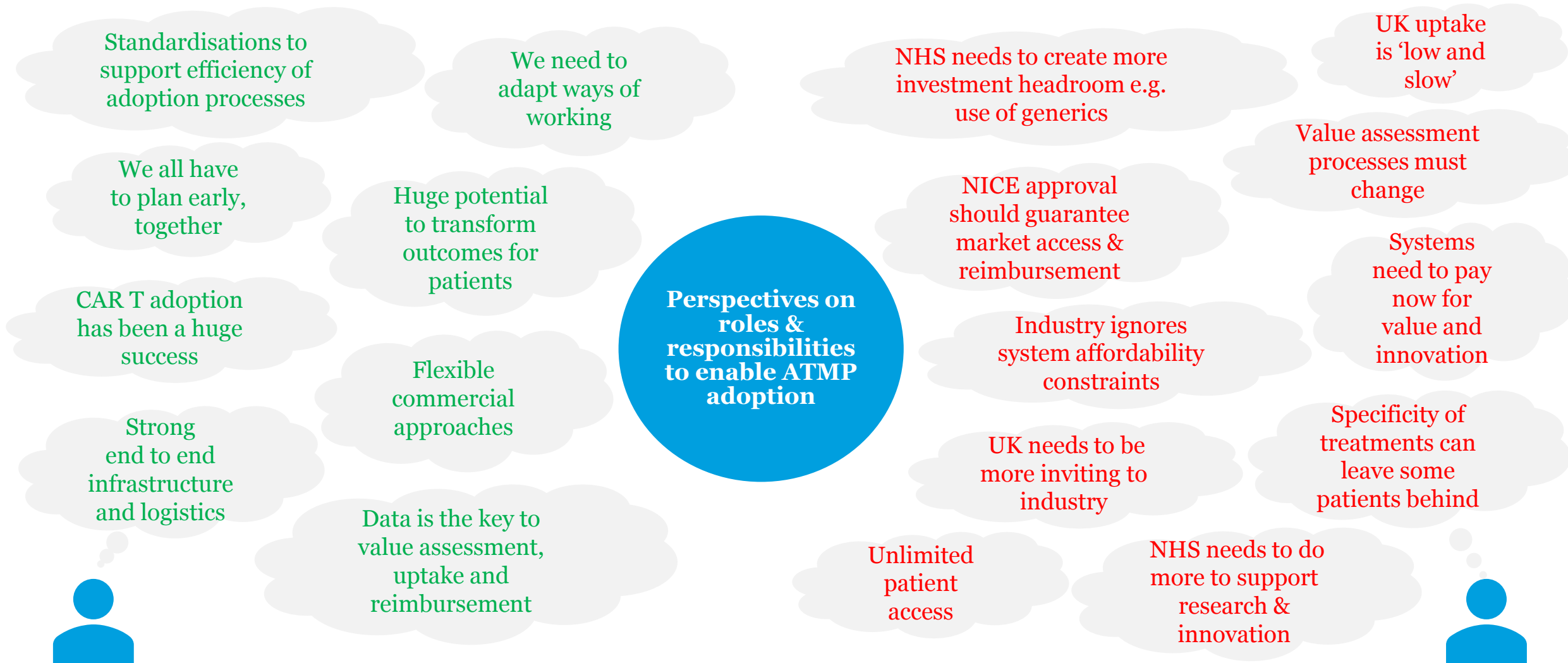


Claire Foreman

Executive summary: What we heard from partners

POINTS OF ALIGNMENT

POINTS OF DIFFERENCE



Objectives of the project and report

In this project, we set out to learn from partners what they think needs to happen to support ATMP adoption in the UK. With our focus on perspectives and partnership, we did not attempt to comprehensively assess adoptions activities in the UK or to compare arrangements with those in place internationally. Instead, we aimed to:

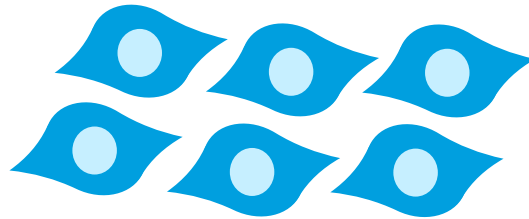
- Work across organisational boundaries to hear and understand some of the issues and perceptions of partners in relation to healthcare system readiness for adoption of treatment innovation. These are set out in the ‘deep dive’ sections.
 - Contribute to environment shaping by sharing insights, increasing understanding and enhancing engagement by producing a report of the ideas and issues identified by partners.
 - Set out areas for further exploration and collaboration to progress healthcare system readiness to accommodate technological innovation in therapeutics, particularly when the innovation is disruptive as opposed to incremental and about a class of treatments, not individual therapies.
 - Set out a range of potential partner actions to optimise the ‘ecosystem’ for adoption of cell and gene therapies, and potentially other innovative medicines development.
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ATMP can include any of the following medicinal products for human use:

- **a gene therapy medicinal product**



- **a somatic cell therapy medicinal product**



- **a tissue engineered product**



The full definition of ATMPs is found in Directive 2001/83/EC as amended by the ATMP Regulation 1394/2007.

Adopting innovation: what makes ATMPs different?

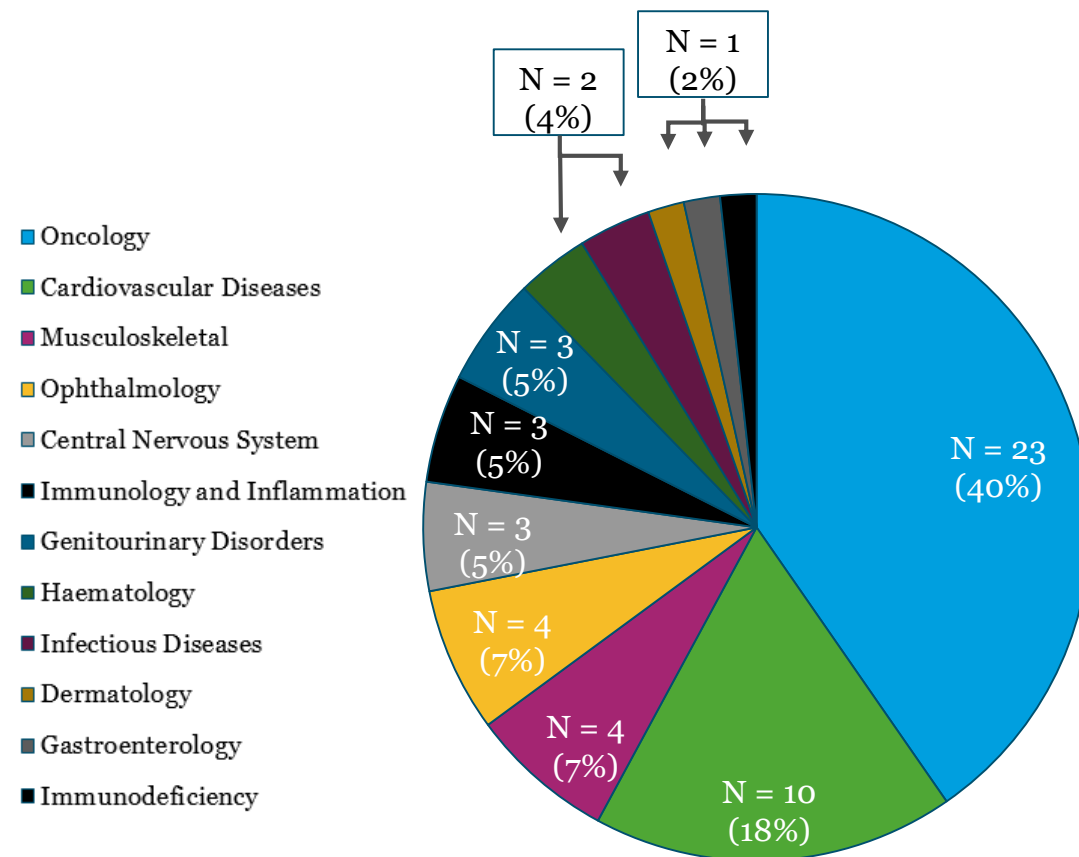
Well understood features of treatment innovations (e.g. heart transplant)			Novel features of treatment innovations (e.g. ATMPs)		
Extreme efficacy	Durable outcomes	High upfront cost	Complexity	Rate of innovation	Volume of innovation and speed of obsolescence
Disease modifying and potentially “curative”.	A single administration may lead to many years/ a lifetime of improved health.	Potentially high cost manufacture, administration and/or hospital care. High healthcare value.	ATMPs are often complex in their: <ul style="list-style-type: none"> • Biology/action • Manufacture • Administration 	Scientific advances; industry corporate agendas; progressive regulators; and extreme efficacy combine to drive shortened development times to a few years.	Huge amount of innovation taking place, encouraged by clinical results and corporate investment. Next generation products are rapidly superseding existing technologies.

The ATMP pipeline

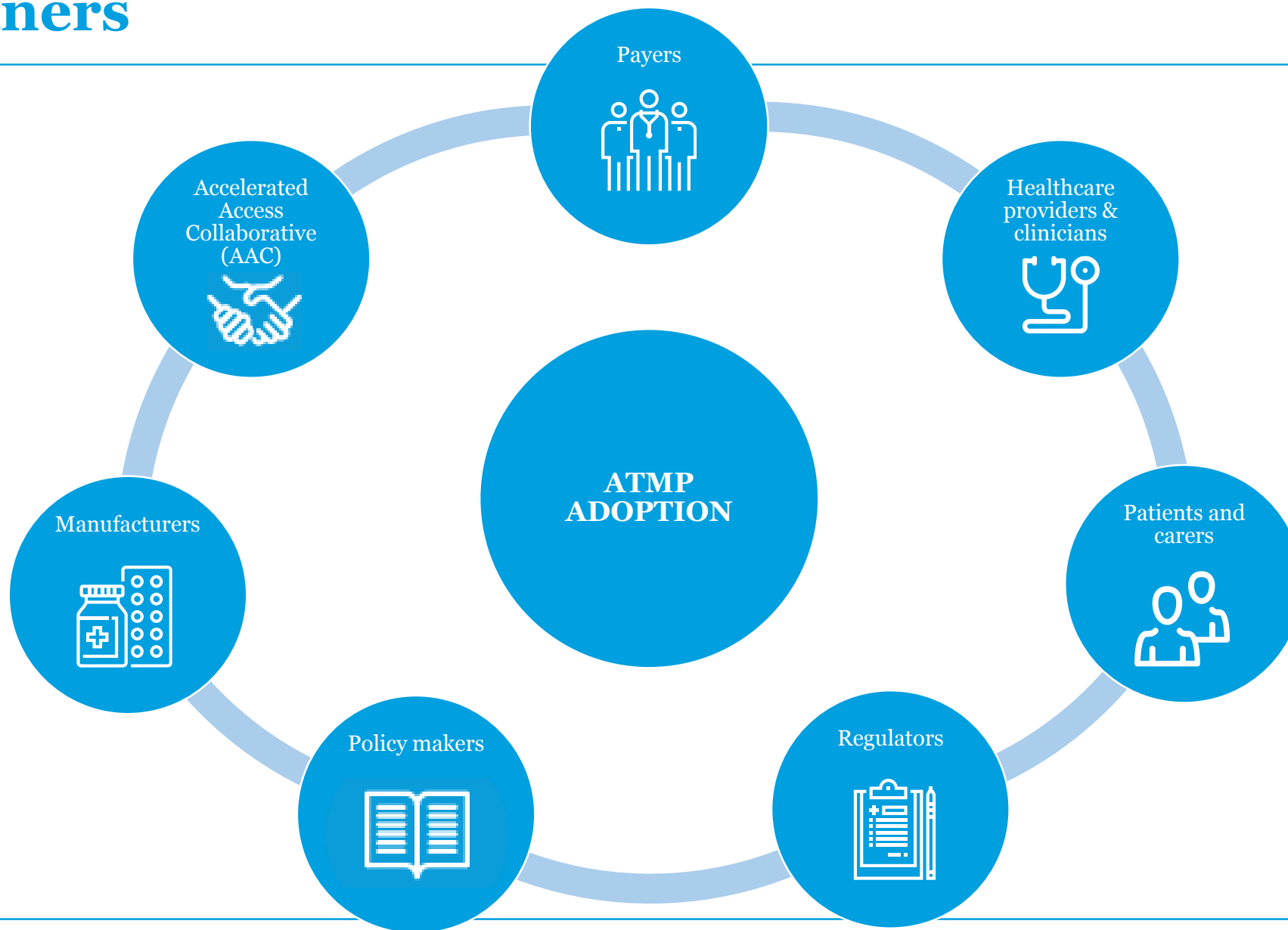
Reimbursement status of approved ATMPs in the EU

Product	Date of MAA approval	Reimbursed in Member States	Reimbursement status
Zynteglo (bluebird bio)	03/06/19	-	Pending
Luxturna (Spark/Novartis)	23/11/18	UK	Due to start January 2020
Yescarta (Kite/Gilead)	27/08/18	UK	Through the UK Cancer Drugs Fund
Kymriah (Novartis)	27/08/18	UK	Through the UK Cancer Drugs Fund
Alosifel (Takeda)	23/03/18	-	Rejected by NICE Aug 2018
Spherox (CO.DON AG)	10/07/17	Germany, UK	UK - restricted beyond its regulatory label
Zalmoxis (MolMed)	18/08/16	France, Germany, Italy	
Strimvelis (GSK)	26/05/16	Italy, UK	Use is limited in one centre in all of Europe
Imlygic (Amgen)	16/12/15	Germany, UK	UK - restricted beyond its regulatory label
Holoclax (Chiesi)	17/02/15	France, Italy, Spain, UK	Restrictions applied to the indication and/or the level of reimbursement

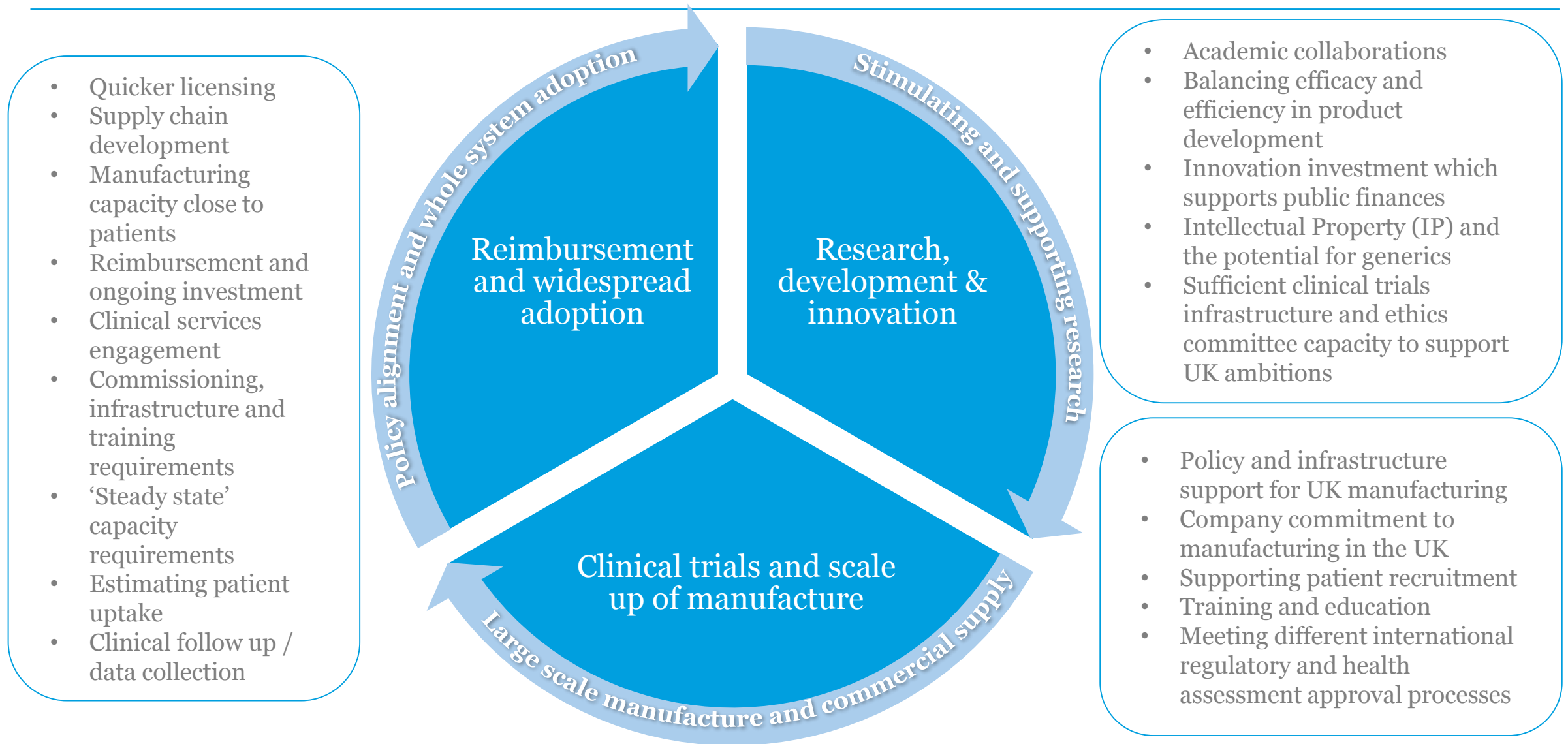
Number of cell and gene therapies in Phase 3 development (with US and/or EU trial site)



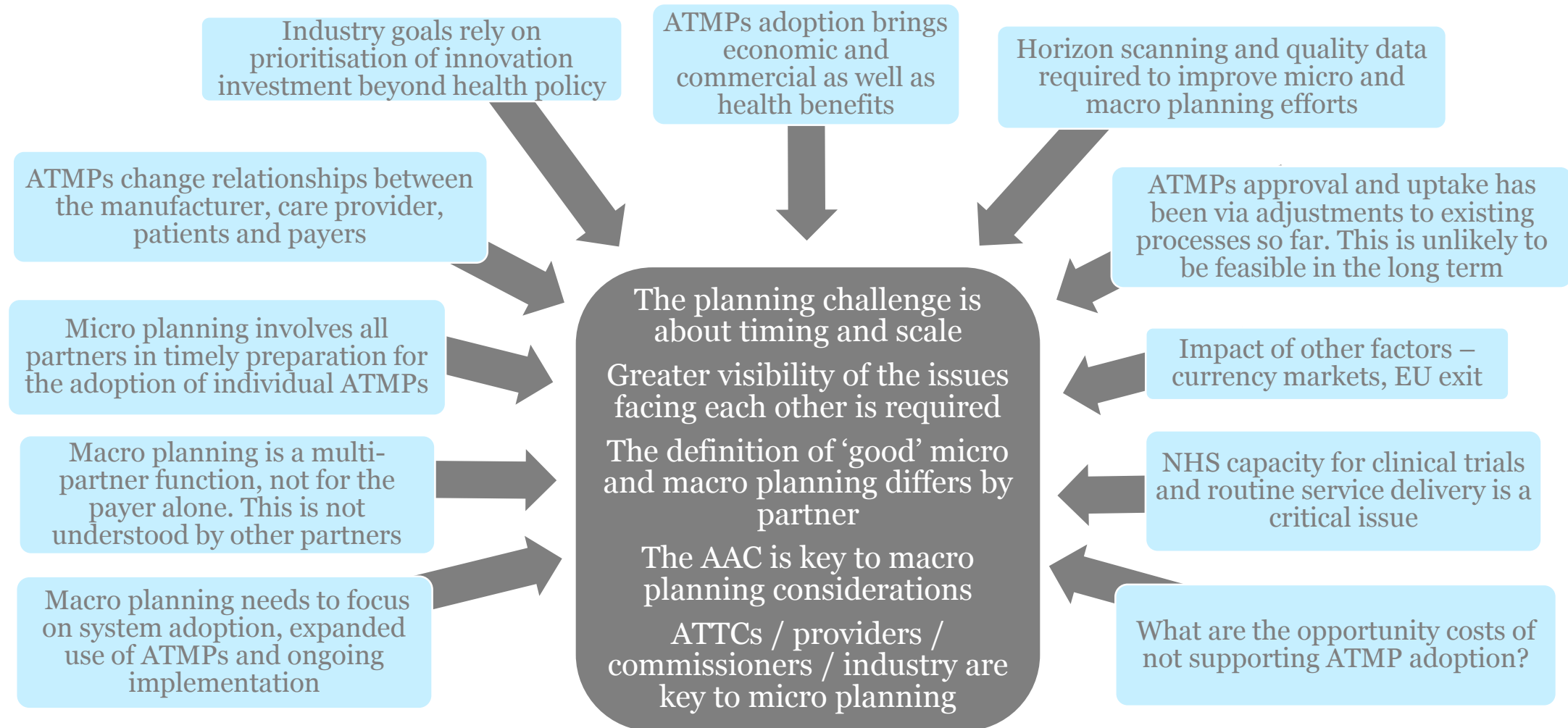
The Partners



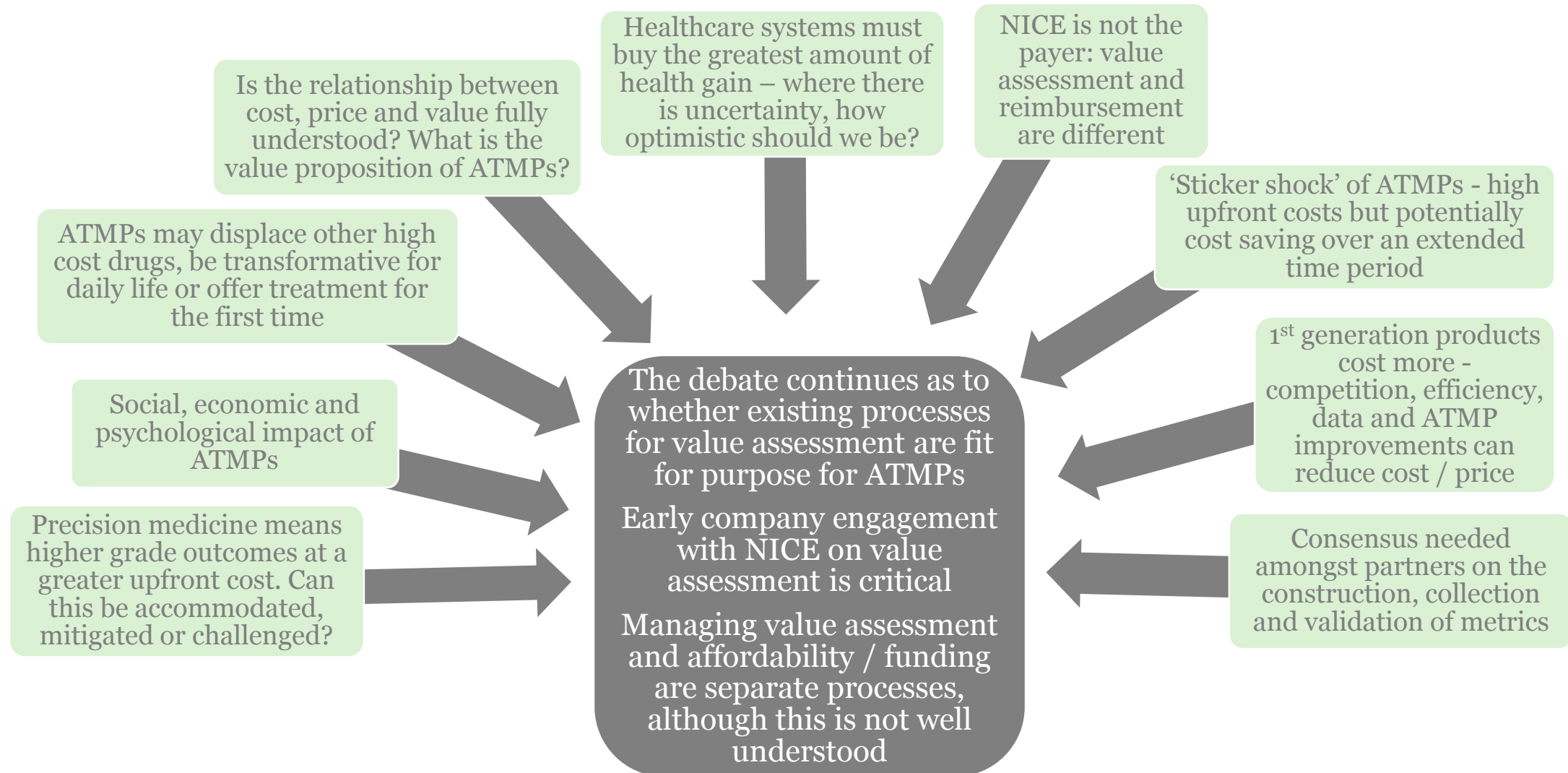
ATMPs: Headline issues raised by partners



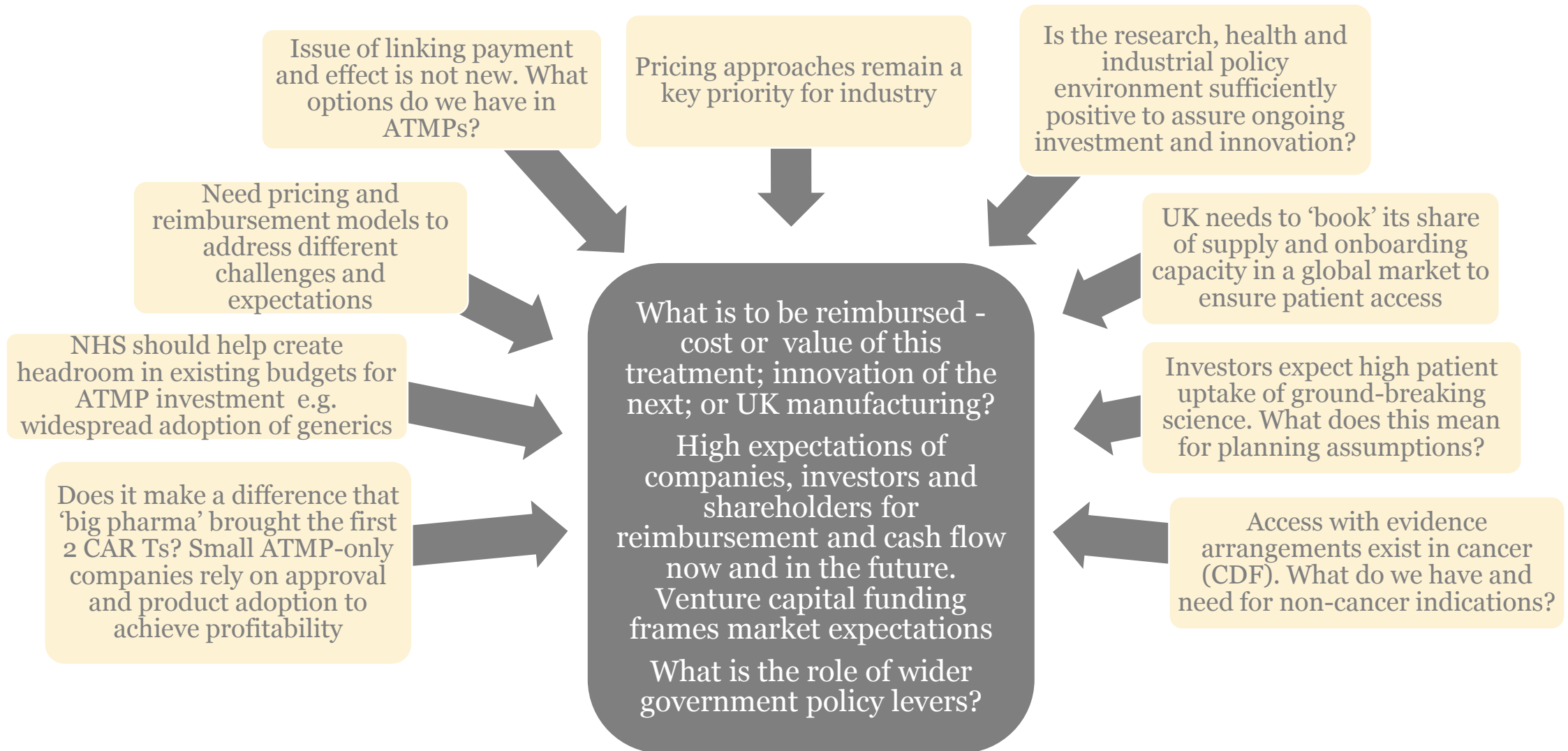
Partner perspectives deep dive: Macro (policy) & micro (product) level planning



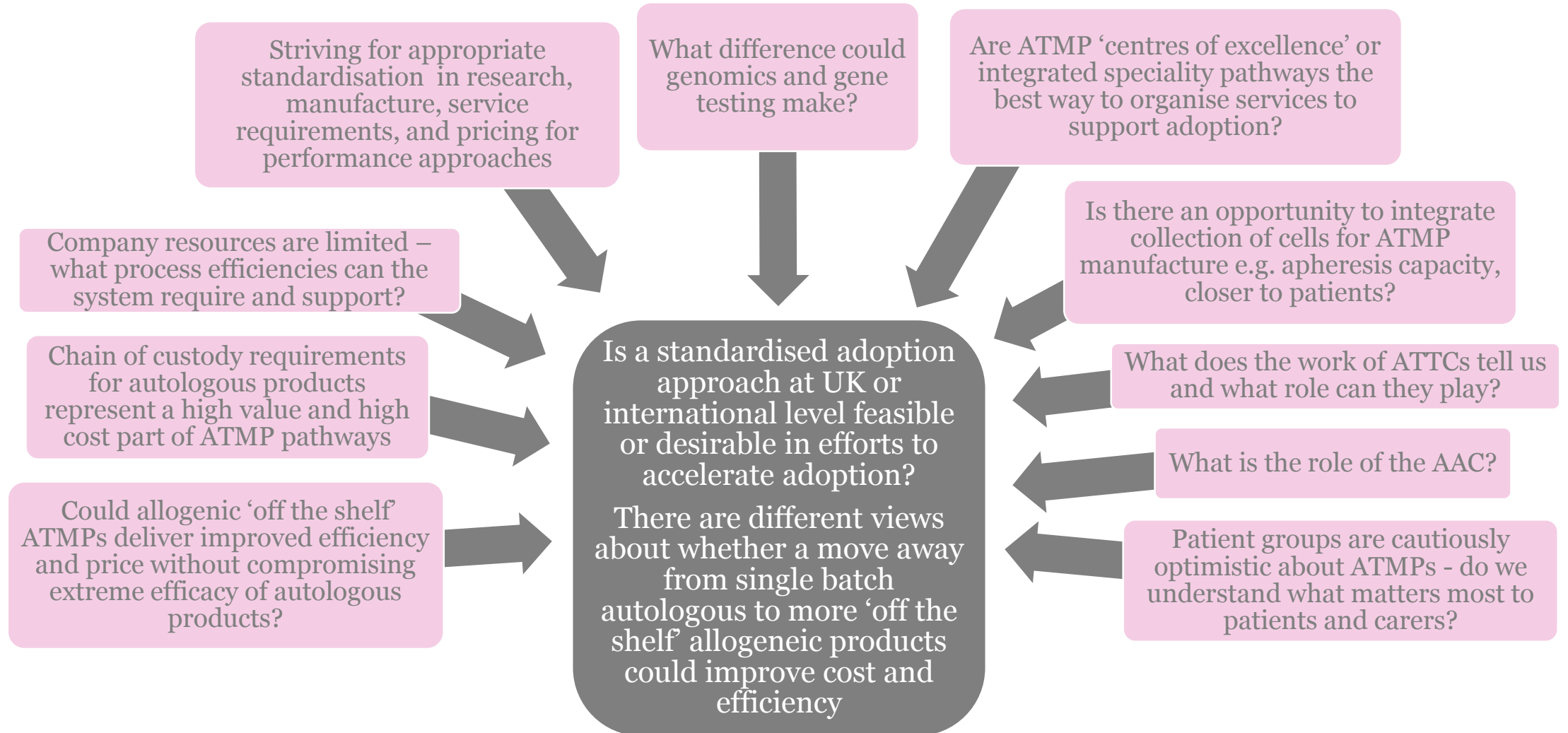
Partner perspectives deep dive: Clinical and cost effectiveness



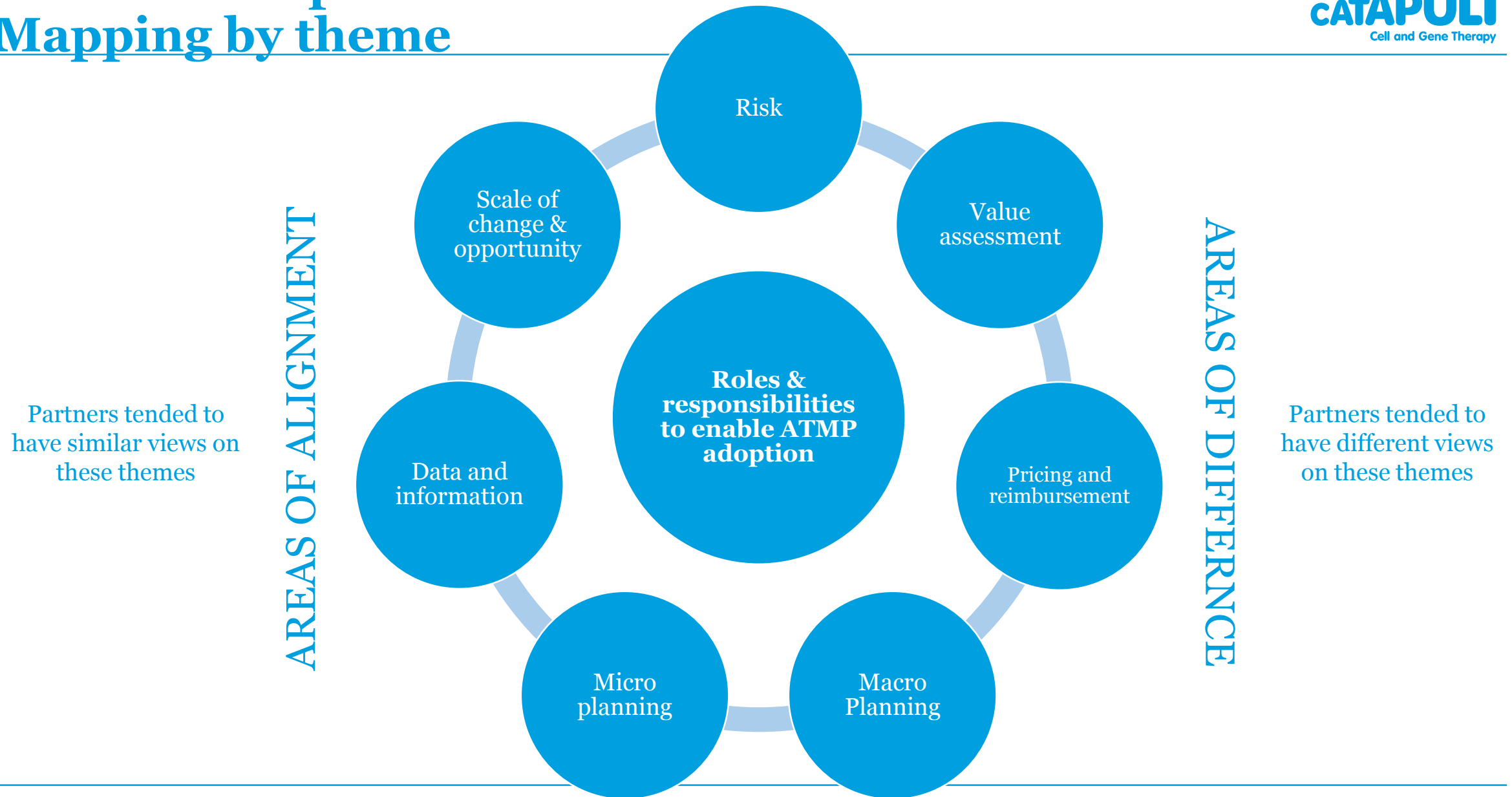
Partner perspectives deep dive: Investment, price and reimbursement



Partner perspectives deep dive: Individualisation vs standardisation



Partner Perspectives: Mapping by theme



What did we learn?

- There is huge passion and excitement amongst partners about UK-led academic and commercial ATMP innovation. The UK already boasts great science, flagship companies, a GMP manufacturing centre, ATTC test beds, positive health technology assessments, healthcare adoption and market access.
 - More is required to support ATMP adoption and all partners have views about their role and the role of others. This is not always aligned, often due to misconceptions about the responsibility, imperatives, beliefs or behaviours of others.
 - No one partner can solve the challenge of ecosystem preparedness and adoption. It relies on multiple partners, not on the healthcare payer alone. Creativity and leadership will be required by all partners.
 - So far, adoption has occurred with adaption of existing processes. It is unlikely that this will be sufficient to optimise ATMP adoption in future.
 - Despite a growing understanding of ATMPs, these are early days and the potential impact of ATMPs for individuals, healthcare, industry and the economy is not yet fully understood. This is an immature ecosystem, learning as we go.
 - To learn from experience so far, build on best practice and avoid duplication, we need data and tools to help organise how we consider, plan and act for ATMP adoption.
 - It remains unclear which critical factors – research and clinical trials; company profiles and investment; health technology appraisal; pricing and reimbursement; manufacturing; supply chain and logistics; clinical service capacity and readiness; patient uptake – will be the key to unlocking ATMP adoption.
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The healthcare opportunity

- Harness the power of the NHS and The Long Term Plan to make UK the best in the world at ATMP adoption
- Enhancing research capacity and capability to promote clinically and cost effective treatment innovations
- Step change in outcomes, experience and healthcare for patients
- Systematic service readiness for innovation uptake

The industrial opportunity

- Industrial growth for the sector and the UK role in therapeutic innovation
- Translate excellence in innovation to excellence in manufacturing
- Enhanced GMP UK manufacturing supported by the Cell and Gene Therapy Catapult
- Developing industry - NHS interface and relationships

The ecosystem opportunity

- Harness actions of all partners through the AAC
- Collaborative action to map out opportunities for greater alignment on strategic planning, including horizon scanning
- Alignment of regulatory processes to support adoption at approval
- Communication and patient engagement

Project team



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About the project

Between January 2019 to August 2019 over 20 meetings were held with companies, regulators, policy makers and patient groups to explore issues, ideas and perceptions. Thematic analysis was undertaken to identify areas of alignment, areas of difference and areas for further engagement and action. This report summarises the outputs of Claire's 'connecting' leadership project.

- Co-sponsored by NHS England and Catapult Directors.
- 'Connecting experience' project to build leadership as part of the Health and Care Leaders Aspiring Director Programme - <https://www.leadershipacademy.nhs.uk/programmes/health-care-leaders-scheme/>
- Qualitative project, focusing on views and insights of senior leaders in a range of sectors and organisations to deepen understanding.
- Identifying gaps and emergent themes, as well as well known issues.
- Signalling the potential direction for further organisational and system exploration and action.
- The outputs of this project are being shared with participants, partners and the Accelerated Access Collaborative to help current and future work on ATMP adoption.

Participating organisations

Achilles
Alliance for Regenerative Medicine
Autolus
Bellicum Pharmaceuticals
Bloodwise
Blue Bird Bio
Cell and Gene Therapy Catapult
Cell Medica
Gilead
HM Treasury
NHS England
NICE
Oxford Biomedica
Office of Life Sciences
Pfizer
Retina UK
RNIB
Syncona