Summary findings from: “ATTCs, clinical infrastructure requirements and adoption of ATMPs” workshop
19 September 2018
Introduction

On 19 September 2018 members of the organisations listed here met to address in an open and unattributed way what needs to be done to facilitate the adoption of Advanced Therapy Medicinal Products (ATMPs) in the UK NHS system. This document summarises the outputs of both the morning and afternoon sessions.

Organisations represented

<table>
<thead>
<tr>
<th>ABPI</th>
<th>Horizon Discovery</th>
</tr>
</thead>
<tbody>
<tr>
<td>Anthony Nolan</td>
<td>Health Research Authority</td>
</tr>
<tr>
<td>Atelerix</td>
<td>iMATCH – Manchester Advanced Therapy Treatment Centre</td>
</tr>
<tr>
<td>Bayer</td>
<td>Innovate UK / UKRI</td>
</tr>
<tr>
<td>Bellicum Pharmaceuticals</td>
<td>Janssen-Cilag Ltd</td>
</tr>
<tr>
<td>BIA</td>
<td>Knowledge Transfer Network</td>
</tr>
<tr>
<td>BioMarin Pharmaceutical</td>
<td>London Advanced Therapy Network</td>
</tr>
<tr>
<td>bluebird bio</td>
<td>MHRA</td>
</tr>
<tr>
<td>Celgene</td>
<td>Midland &amp; Wales Advanced Therapy Treatment Centre</td>
</tr>
<tr>
<td>Cell Medica</td>
<td>Muscular Dystrophy UK</td>
</tr>
<tr>
<td>Covance</td>
<td>NHS England</td>
</tr>
<tr>
<td>Deltohn Ltd</td>
<td>NHS Scotland</td>
</tr>
<tr>
<td>Dignio</td>
<td>NHS Wales</td>
</tr>
<tr>
<td>Exmoor Pharma</td>
<td>NICE</td>
</tr>
<tr>
<td>Gilead Sciences</td>
<td>Northern Alliance Advanced Therapies Treatment Centre</td>
</tr>
<tr>
<td>Health Innovation Network</td>
<td>Novartis</td>
</tr>
<tr>
<td></td>
<td>Orchard Therapeutics</td>
</tr>
<tr>
<td></td>
<td>Pfizer</td>
</tr>
<tr>
<td></td>
<td>Sanofi</td>
</tr>
<tr>
<td></td>
<td>Scottish Medicines Consortium</td>
</tr>
<tr>
<td></td>
<td>TrakCel</td>
</tr>
<tr>
<td></td>
<td>Valid Insight</td>
</tr>
<tr>
<td></td>
<td>Videregen</td>
</tr>
<tr>
<td></td>
<td>Welsh Health Specialised Services</td>
</tr>
<tr>
<td></td>
<td>World Courier Services</td>
</tr>
</tbody>
</table>

NHS England
Morning Session:
Advanced Therapy Treatment Centres and NHS clinical infrastructure
The network of Advanced Therapy Treatment Centres will develop and deliver systems for the delivery of cutting edge cell and gene therapies.

- The network will increase the ability of the NHS to deliver disruptive medicines.
- The centres will develop systems and processes within the trusts and hospitals capable of delivering advanced therapies at scale to patients across the NHS.
- The learnings and systems from the initial centres will be rolled out to other centres in the UK.
### ATTC and ATN areas of focus

<table>
<thead>
<tr>
<th>Northern Alliance Advanced Therapies Treatment Centre</th>
<th>iMATCH – Manchester Advanced Therapy Centre Hub</th>
<th>MW-ATTC - Midlands &amp; Wales Advanced Therapy Treatment Centre</th>
<th>London Advanced Therapies Network (ATN)</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Supply chain, starting material procurement, secondary manufacturing, handling by clinicians and long term follow up, informatics infrastructure</td>
<td>• Developing a coordinated strategy to scale up advanced therapies for patients</td>
<td>• Developing a patient and public engagement strategy</td>
<td>• Connecting capabilities in advanced therapies in London – Knowledge exchange</td>
</tr>
<tr>
<td>• Stress test the system using four exemplars with different challenges particularly on the supply chain</td>
<td>• Maximising access to patients</td>
<td>• Electronic capture of patient recorded reported outcome data such as quality of life, adverse events, feedback to the clinical team</td>
<td>• Rapid resolution of IP problems within London – uniform protocols to allow third parties to interact with London in a single transaction in 14 days max</td>
</tr>
<tr>
<td>• Evaluate the modality of distributed manufacturing</td>
<td>• Delivering electronic sample traceability and tracking systems</td>
<td>• Maximising efficiency, data capture</td>
<td>• Training: development of short courses that can meet these skills gaps</td>
</tr>
<tr>
<td></td>
<td>• Maximising efficiency, data capture</td>
<td>• Clinical scaling-up activity</td>
<td></td>
</tr>
<tr>
<td></td>
<td>• Safe delivery of ATMPs with end to end training and education</td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td></td>
<td>• Development of a trial acceleration programme for cellular therapies (TAP-CT)</td>
<td></td>
</tr>
<tr>
<td></td>
<td></td>
<td>• Focus on health economics and clinical pathway mapping</td>
<td></td>
</tr>
</tbody>
</table>

**Cell and Gene Therapy Catapult coordination**
What is required to accelerate adoption

IUK ATTCs, London ATC and other centres

Establish clear pathways to gather industry feedback:
Establishing streamlined information flow (e.g. through industry partner group) to ensure requirements of industry are taken into account.

Define common framework:
Help companies navigate with harmonised procedures and practices:
e.g.: apheresis, treatment regimens, patient selection.
Work with companies and their specific protocols to produce common frameworks and systems.

Contract standardisation:
To ensure timely implementation and adoption, contracts need to be standardised.

Clinical scale up requirements:
Understanding what is required at organisational level e.g. number of apheresis units, ICU beds.

Speed of access:
Streamline both procurement of starting material and delivery to product into health care system. Define courier best practice.

Patient data collection and sharing
Bring data together, capture number of interventions post treatment, capture data in real time, agree where data should be held, connect with Digital Innovation hubs, development of Registries to meet both regulatory and reimbursement requirements.

Patient monitoring:
Development of companion diagnostics, validated analytical methods and patient monitoring, staff training for safe patient management will need to take place.

Patient engagement:
Engaging the patients, patient groups and charitable associations further.

Access to expert knowledge by industry:
Need for experts, education and physicians to help trial design, formulating a database of knowledge network.
The ATTC network will work together with its partners to address the identified priorities to ensure they provide easily-run and ready to use systems and solutions that can then be rolled out to other NHS centres, leading to enhanced patient access to ATMPs.
Afternoon session:
Affordability, payment and reimbursement of cell and gene therapies
Recurrent themes

There were many points raised, but a group of issues which came up repeatedly:

- The high degree of novelty and the early stage creates an even greater need and opportunity for **patient involvement**.
- There are many areas where there is a need to invest in new ways of **gathering**, **handling**, and **interpreting data**.
- There is an opportunity to adopt new ways of following **patient progress**, including taking advantage of new technology.
- There is an increased need to plan ahead for the investment in the **infrastructure** to accommodate novel ways of operating.
- The scale of expected growth and change is not well understood; there is a need for information and forecasts for **planning** purposes.
- There is a need for development of novel, workable and mutually beneficial payment process, such as **outcomes-based payments**.
- There is need to ensure **political support** through understanding of the potential of ATMPs to be transformative, at all levels.
Where individual companies can lead:

- Converge practices: find ways to use existing or common SOPs and protocols, keeping a global perspective
- Collaborate with other stakeholders and each other, and share the investment burden
- Keep in mind "what is best for the patient and what is best for the healthcare system"
- Develop and offer models for risk sharing
- Incorporate companion diagnostics and other patient selection tools to reduce product performance risk
Where National Health Services (NHS) can lead:

- Work with industry, ahead of need, to prepare for adoption
- Work with industry and the intermediary organisations to establish globally compatible data and information systems
- Resolving the practical barriers, such as budgetary system, to allow novel risk sharing payment models, e.g. Annuity
- Prepare for the increased demands on commissioners and the commissioning systems
- Coordinate across the NHS regional bodies
- Prepare to enable risk sharing mechanisms, including maintaining confidentiality
Where “Regulators and Market Access stakeholders” can lead:

- Increase capacity to conduct health technology appraisals and ensure there is sufficient capacity to collaborate and work on solutions
- Examine ways to overcome the “ultra-orphan” cost disincentive to develop and to use ATMPs
- Celebrate their roles in the adoption of high value therapeutics in areas of high unmet need
- Create trust by allowing/facilitating upward movement in price following data gathering
- Examine, clarify and communicate the role of novel payment mechanisms, funding options and the PPRS in facilitating adoption
- Establish processes and practices for pre-launch stakeholder engagement, including addressing NHS infrastructure needs
- Examine the full value of “curative” medicines both at the patient level and the national/macro economic level and how this can be incorporated into HTA methodology
Where political decision makers can lead:

- Support appropriate mechanisms to achieve the benefits of the Cancer Drugs Fund in high unmet need areas outside of oncology: e.g. rare diseases and diabetes
- Hear and take time to understand the message about benefits that come with ATMPs and where they can be transformative
- Take note of the impending demands on the NHS bodies
- Enable investment, in advance, by NHS Bodies in ATMPs and the associated infrastructure
- Allow and enable any legislative change required to implement novel pricing and reimbursement mechanisms, such as for outcomes based pricing models or annuity payments
Where “intermediary” organisations can lead:

- Develop the capacity within the ATTCs and elsewhere to facilitate and manage change in the healthcare practice associated with ATMPs
- Foster the creation of new technological approaches, e.g. social media and patient tracking
- Break-up the “Affordability” question: budget, value, cost, risk, micro, macro etc.
- Identify what infrastructure is needed for the operation of novel pricing and reimbursement mechanisms including outcomes-based pricing, seeking simplicity
- Build the case for and communicate the full value of ATMPs at the patient and national/macro-economic level
- Provide information that helps the NHS bodies plan for the development of the ATMP industry

**ATTCs, CGT, Catapult, ABPI, BIA and UKRI**
Where patients and patient organisations can lead:

- Work with NHS Bodies and ATTCs to develop effective patient tracking and data systems.
- Collaborate with others to lobby for *Cancer Drugs Fund* type benefits outside of oncology.
- Promote political support for greater investment in emerging ATMP field.
- Collaborate to develop clinical outcome measures to support novel valuation, pricing and reimbursement mechanisms.
What next?

Individual organisations can and will create their own agendas going forward. Nonetheless, it is necessary for all the different stakeholders to come together to tackle these issues, as it is too big task for any one group to drive, and it is in everyone’s interest to resolve.

Any organisation seeking help or input in coordinating a further action or collaboration in this area should feel free to contact the Cell and Gene Therapy Catapult.

The Cell and Gene Therapy Catapult, through its coordinating role in the ATTC network and its “Proof of Adoption” strategic priority, will monitor progress over the coming months.

Regular consultation is anticipated to measure progression and impact.

This is just a starting point for a series of actions. It is hoped that, through this workshop, its outputs and the subsequent discussions, no critical area will be neglected or forgotten in the complementary activities that follow.

For more information contact us at comms@ct.catapult.org.uk