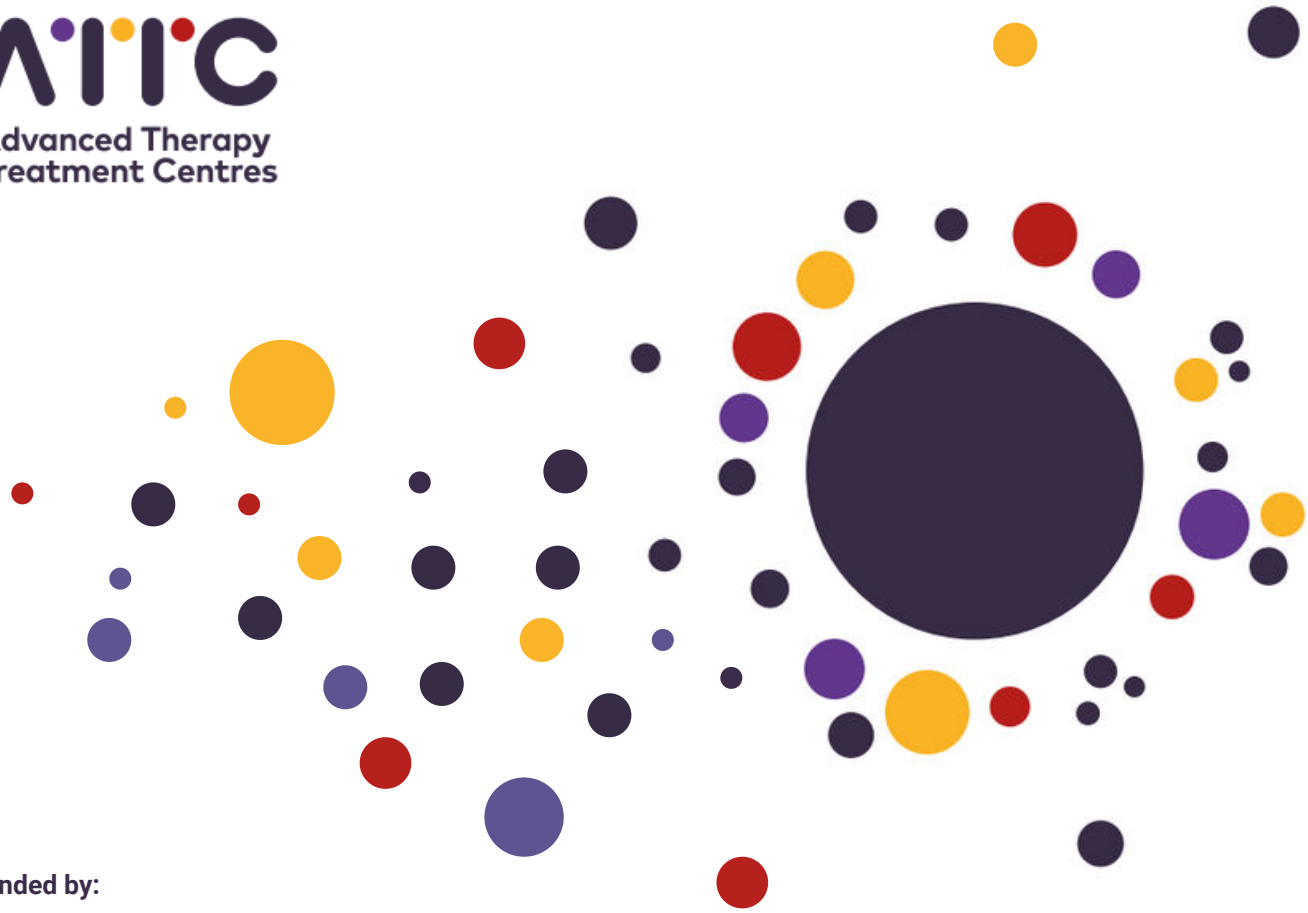
Advanced Therapy Treatment Centres



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Advanced Therapy Adoption Challenges in the United Kingdom

December 2023

Coordinated by

CATAPULT Cell and Gene Therapy

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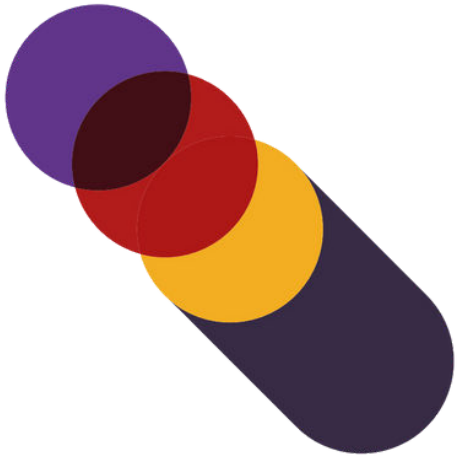
Executive summary



This report looks to capture the key discussions and findings from the Advanced Therapy Treatment Centres (ATTC) Network's October UK Advanced Therapies Adoption Challenge event. This event sought the direct input of expert speakers and attendees on three priority topics:

- The importance of a healthy clinical trials landscape.
- The need for internationally competitive value and reimbursement processes.
- Ensuring the health system is truly ready to deliver advanced therapies to the benefit of the UK population.

The voices of those with lived experience were also highlighted throughout the day, recognising the true real-world impact of these innovations and ensuring that input from the patient community was fully considered in all discussions.

Following on from these highly valuable and expert-driven conversations, the ATTC Network has since developed a series of actionable recommendations with one core ambition in mind – truly making the UK a world leader in advanced therapies.

These headline recommendations are as follows:

1 Leverage existing world-leading expertise to enable the UK to be the best place internationally to trial and deliver advanced therapies.

2 Realise the value of advanced therapies to ensure that UK patients are able to benefit from the latest cutting-edge health technologies.

3 Invest in supporting workforce and NHS delivery infrastructure to allow the UK to benefit from advanced therapy delivery at scale.

4 Improve the use of data system-wide to harmonise the advanced therapies ecosystem.

5 Create a government-led advanced therapy taskforce in early 2024 to implement these recommendations and cement advanced therapies as a strategic UK-wide health and life sciences policy priority.

Further detail on these strategic recommendations can be found later in the report.

It is important to note that this event and recommendations build on the findings of the National Cell and Gene Therapy Vision for the UK which was commissioned by the Cell and Gene Therapy Catapult on behalf of the ATTC Network.

Seizing a generational opportunity

Professor Neil Watson

Co-Director of the Northern Alliance ATTC

Advanced (cell and gene) therapies represent a paradigm shift in how we view healthcare. These technologies will help us do things differently, treating the root causes of disease and transforming outcomes for patients.

As many as 36 advanced therapies across 34 different indications are now expected to launch in the UK within the next three years.[i] While not all these pipeline innovations will succeed, the opportunity here is clear – not only for patients, but for the broader healthcare system and essential life sciences investment. The UK must be ready to seize this opportunity and truly become one of the best places in the world to develop, launch, and receive these innovative medicines.

The ATTC Network, as coordinated by the Cell and Gene Therapy Catapult, has already begun to cement this foundation. The network has so far been instrumental in translating the theory of advanced therapies into practice.

Early work into specific areas such as clinical trials, workforce, and advanced therapy infrastructure has helped to ensure that the pioneering 'first wave' of these technologies have already begun to transform lives.

Unfortunately, the sad reality is that this is not enough. To realise the full benefits of these innovations, we must be able to achieve all of this at scale. The delivery of advanced therapies en masse offers an entirely different wealth of opportunity – more access, more competition, lower costs, and most importantly, better health outcomes.

With such a conundrum in mind, the ATTC Network convened a full-day event in October, hosted by the Royal Society of Medicine, to gain key insights and input from expert stakeholders involved in the advanced therapy pathway. This report looks to bring attention to these highly valuable conversations and in doing so, outline priority and actionable areas for improvement within the UK innovations landscape. We have reached out to those unable to attend on the day to ensure continued collaboration.

It is acknowledged that the ATTC Network is not alone in driving these ambitions and therefore these recommendations must be considered within the full context of similar initiatives.

The UK was quick out of the blocks on advanced therapies, it would be a failure if we were to lose this lead now.

Fundamentally, no change is not acceptable.

Endorsed by:

Northern Alliance Advanced Therapies Treatment Centre | iMATCH Advanced Therapy Treatment Centre | Midlands-Wales Advanced Therapy Treatment Centre

i. Doak, B. (2023) 'NHS readiness and future resource capacity' [PowerPoint presentation]. UK Advanced Therapies Adoption Challenge.

Making progress for patients

The prospect of transforming people's lives through advanced therapies is hugely exciting. We have begun to see how these innovations can make a significant impact on diseases such as cancer and rare inherited conditions, and we may soon see the same transformative impact on more common, priority public health conditions.

I have been fortunate enough to observe the evolution of these technologies first-hand, including the system-wide challenges of funding, adoption, and implementation. With innovation often comes disruption – and this is particularly true for advanced therapies.

To truly maximise the potential of advanced therapies for patients of the future, we must work to get the environment right now. We must overcome barriers in the system and reconfigure patient pathways to ensure people benefit from the best examples of these innovations. Achieving change won't be simple but it is encouraging to see that progress is already being made.

The future looks bright. To get there we must use the vast expertise at our disposal and drive forward the most tangible, implementable solutions to establish a flexible platform for advanced therapies.

Professor Gillian Leng

President-Elect of the Royal Society of Medicine and Commissioner on the NHS Innovation and Life Sciences Commission

Realising the full potential of the UK life sciences powerhouse

The UK is blessed with an enviable heritage in life sciences innovation and is internationally recognised for its contributions to improving global healthcare outcomes. To this day, we remain a leader in research, generating world-class publications that are amongst the most highly cited (top 1%) globally.[ii] Unfortunately, this high standard of performance is no longer the case across the board.

The government's most recently published Life Sciences Competitiveness Indicators show that the UK's influence is waning. Our proportion of direct life sciences inward investment is down and we have been falling behind in key areas such as clinical trial competitiveness.[ii] The good news is that we can fix this if we act quickly.

My recent commercial clinical trial review has put in place a framework to return vital clinical research to its best - leveraging the UK's established foundation of expertise and building new examples of best practice. The advanced therapies landscape offers a similar opportunity to regain global leadership in this fast-growing area of biomedical science.

Annual investment into advanced therapies saw a 31%[iii] increase between 2021-2022 and the UK now represents 14% of all global commercial ATMP clinical trials.[iv] These metrics show that we can become a world leader in this field but only if we continue to invest in its success. Increasing the desirability of the UK as a competitive destination for life sciences investment is essential to achieving these ambitions.

Lord O'Shaughnessy

Partner at
Newmarket Strategy

ii. Department for Science, Innovation and Technology, Department of Health and Social Care and Office for Life Sciences (2023) Life sciences competitiveness indicators 2023. Available at: https://www.gov.uk/government/publications/life-sciences-sector-data-2023/life-sciences-competitiveness-indicators-2023 (Accessed: December 2023)
iii. Cell and Gene Therapy Catapult (2022) Annual Review 2022: Ten years of accelerating innovation in cell and gene therapies. Available at: https://cgt.ams3.cdn.digitaloceanspaces.com/Catapult-Annual-Review-2022.pdf (Accessed: December 2023)
iv. Cell and Gene Therapy Catapult (2022) UK ATMP Clinical Trials Report 2022. Available at: https://cgt.ams3.cdn.digitaloceanspaces.com/Clinical-Trials-Database-2022-Commentary.pdf (Accessed: December 2023)

The advanced therapy landscape

The event started by reflecting on the current, highly dynamic UK advanced therapy landscape. Dr Jaqueline Barry (Chief Clinical Officer at the Cell and Gene Therapy Catapult) gave an insightful presentation outlining the growth currently happening in the sector.

It is clear that the UK is continuing to make great progress in this space, with the annual investment into advanced therapies increasing year-on-year. Similarly, the UK has also seen the number of people employed in advanced therapy and bioprocessing industries double, from 3,033 in 2020 to 6,956 in 2022.[iii]

The UK is not only performing well in advanced therapy sector growth but is also, for the time being, performing comparatively well on the number of academic and commercial clinical trials being initiated. As of 2022, UK clinical trials represented 8% of all international advanced therapy trials and 14% of commercial trials.[iv] These figures are a testament to the work of organisations such as the Cell and Gene Therapy Catapult and ATTC Network to date.

Dr Barry later reflected on the large number of cell and gene therapies in development globally, highlighting the reality that while we are in the early days of realising the true potential of these technologies, a multitude of advanced therapies for a broad range of clinical conditions are on the immediate horizon.

There remain barriers to overcome to ensure that the UK can maintain its competitive edge in advanced therapies – notably including system readiness and capacity, as well as improvements to data collection, standardisation, and coordination.

Professor Eric Alton (Professor of Gene Therapy and Respiratory Medicine at Imperial College London) followed on from Dr Barry with an engaging presentation on the science and promise of advanced therapy technologies.

Professor Alton spoke on the different types of advanced therapies, outlining that while they are often referred to under this umbrella terminology, they actually represent many different and uniquely precise technologies – from gene addition and cell therapy to more recent gene editing techniques.

The science and progression of these technologies is cause for celebration and this has accelerated in recent years as more long-term clinical data has become available. We are now seeing real-world examples of these medicines changing lives - particularly in rare diseases, where patients have historically been challenged with a limited availability of treatment options.

Professor Alton also acknowledged the challenges presented by advanced therapies. While the latest generation of these innovations offer clear benefits, they do present fundamentally different hurdles when compared to historically simpler medicines. Key challenges remain around complicated manufacturing processes, and further steps could be made to accelerate preclinical advanced therapy development.

However, the science indicates that investment into overcoming these unique issues is worthwhile. The potential for widespread patient benefits is incredibly promising, with advanced therapies offering an opportunity for us to entirely reconsider how we view care today.

iii. Cell and Gene Therapy Catapult (2022) Annual Review 2022: Ten years of accelerating innovation in cell and gene therapies. Available at: Cell and Gene Therapy Catapult (2022) Annual Review 2022. (Accessed: December 2023)
iv. Cell and Gene Therapy Catapult (2022) UK ATMP Clinical Trials Report 2022. Available at: https://cgt.ams3.cdn.digitaloceanspaces.com/Clinical-Trials-Database-2022-Commentary.pdf (Accessed: December 2023)

Clinical trials

Professor Fiona Thistlethwaite (Director at the iMATCH ATTC) and Chris Vann (Chief Operating Officer at Autolus and Treasurer of the Alliance of Regenerative Medicine [ARM]) led a panel discussion on the importance of a healthy clinical trial ecosystem in order to drive world-leading advanced therapy research in the UK.

Mr Vann spoke to his experience with both Autolus and ARM, noting that while the UK remains a leader within Europe on advanced therapy clinical trials, Europe itself is now being "left behind" on the number of new trials being initiated when compared to both North America and Asia. This presents a challenge – does the UK want to be a true world leader in advanced therapies, or just amongst the best in the continent?

Mr Vann later argued for the UK to distinguish itself further as a priority advanced therapy research destination, improvements to the clinical trial ecosystem should be made. Investments into reinforcing the UK's already highly skilled research workforce and expediting trial approvals were outlined as key priorities. While seemingly tangential, the importance of comprehensive value and reimbursement processes was recognised as a tertiary point. For manufacturers to invest in UK advanced therapy trials, successful reimbursement must be considered a plausible outcome of successful clinical trials.

Professor Thistlethwaite reflected separately on the quickly evolving advanced therapy trial landscape. The complex nature of these technologies, necessarily, translates into complex processes adapting these processes to the clinical setting. The work of the ATTC Network has, to date, been instrumental in making these processes simpler – creating ready-to-use solutions that can be more widely rolled out to the NHS, improving institutional readiness, and sharing key learnings across UK hospitals. By building a UK ecosystem to deliver these disruptive innovations, it is hoped that patient access to potentially life-saving medicines can be improved.

Professor Thistlethwaite highlighted that she believed this spirit of collaboration, alongside the UK's strong research track record and active biotech sector, make the UK "uniquely placed" to deliver on the promise of advanced therapies, with clinical trials being "a key stepping stone to future adoption".

Lord O'Shaughnessy hosted a separate keynote later in the day, drawing attention to the headline findings of his commercial clinical trials review.

UK research is of fundamental importance to the broader life sciences sector, providing direct benefits to both patient outcomes and GDP. From 2018 to 2019, the NHS received an estimated income of £355 million from life science companies delivering research in the UK and saved an estimated total of £28.6 million from pharmaceutical cost-saving, where a trial drug replaced the standard of care treatment.[v] These figures highlight the true cost of UK R&D underperformance.

Unfortunately, as highlighted by Lord O'Shaughnessy, the UK is currently experiencing an acute period of exactly this. The total number of commercial clinical trials (across all medicinal product types) initiated in the UK per year fell by 41% between 2017 and 2021, and the number of Phase III commercial trials initiated in the UK fell by 48% within the same period.[vi] It is clear that the UK cannot achieve its ambitions of being a "life sciences superpower" without fixing the root causes of these issues.

Lord O'Shaughnessy's recommendations to address these issues are comprehensive. Establishing examples of clinical trial excellence is essential and the UK needs to learn from international examples of best practice where possible. However, fixing these issues in their totality, all at once, is not realistic - we need to fix these problems for a small number of areas first. Advanced therapies offer an ideal opportunity to do just this.

v. Department for Science, Innovation and Technology, Department of Health and Social Care and Office for Life Sciences (2023) Commercial clinical trials in the UK: the Lord O'Shaughnessy review - final report. Available at: https://www.gov.uk/government/publications/commercial-clinical-trials-in-the-uk-the-lord-oshaughnessy-review/commercial-clinical-trials-in-the-uk-the-lord-oshaughnessy-review-final-report (Accessed: December 2023)

vi.Association of the British Pharmaceutical Industry (2023) NHS patients losing access to innovative treatments as UK industry clinical trials face collapse. Available at: https://www.abpi.org.uk/media/news/2022/october/nhs-patients-losing-access-to-innovative-treatments-as-uk-industry-clinical-trials-face-collapse/ (Accessed: December 2023)

Value and reimbursement

Professor Mark Sculpher (Director of the Centre for Health Economics at the University of York), Emma Clifton-Brown (Head of Health and Value at Pfizer), and Matthew Durdy (Chief Executive Officer at the Cell and Gene Therapy Catapult) held a compelling panel discussion on the key challenges facing the assessment of advanced therapies through current UK health technology appraisal (HTA) processes. Professor Gillian Leng chaired and facilitated the panel.

Professor Sculpher initiated discussions by highlighting the "significant constraints" that UK payers must consider when assessing new innovative health technologies. Not every innovative therapy can be funded; therefore, adequate processes for transparently determining instances of best system value are essential. NHS resources are finite and stretched, meaning that properly recognising this value in the health system is a must.

Professor Sculpher expanded on this point, noting that accounting for this can be particularly challenging in reimbursement decisions for advanced therapies, given the intrinsic long-term uncertainty associated with the technologies. By providing better real-world data, designing more complete trials, and offering more pricing flexibility to appropriately share risk between manufacturers and the health system, he argued that the value of these innovations can be more widely recognised.

Ms Clifton-Brown added to the conversation, stating that the UK can look to address these issues "from a position of strength". The UK has demonstrated positive examples of reimbursement success for advanced therapies, but there have also been instances of significant challenge – with both providing opportunities to learn and optimise processes for future benefit. Existing systems such as managed access are already world-leading in their ability to manage clinical uncertainty. Patient experience shows why getting these processes right matters so much, not just for improved outcomes but for the broader health system value and ecosystem investment.

Ms Clifton-Brown highlighted distinct challenges with existing HTA processes and the limitations they can present, having not been designed with specific consideration for advanced therapies. The impact of data uncertainty remains considerable and "disproportionately" affects long-term benefit therapies with higher up-front costs. Ms Clifton-Brown further stressed that "the balance between commercial viability and affordability is the key to unlocking the benefits that come from these therapies" and realising the "massive opportunity" they offer.

Mr Durdy considered a more holistic perspective, arguing that the conversation surrounding the value of advanced therapies must evolve from an HTA discussion to a broader system discussion. Advanced therapies offer an opportunity to transform healthcare, and as such should be considered at that level.

Mr Durdy argued that achieving advanced therapy delivery at scale is essential to realising the true value of these innovations. By ensuring patient access at volume, the health system is able to "justify" its investments into advanced therapy infrastructure and truly reap its benefits. Volume importantly translates into increased competition, advancements in manufacturing, significant reductions in costs, and, ultimately, better patient access. Recognition of advanced therapy value beyond just direct patient benefit is needed to achieve this.

The sentiment here was clear. By investing more in these technologies now and considering the total value of the opportunity provided by these innovations, the UK will be able to fully unlock the potential of advanced therapies.

NHS readiness

Professor Phillip Newsome (Director at the Midlands and Wales ATTC) facilitated the final panel of the day. Ben Doak (National Senior Programme of Care Manager for Innovative Treatments at NHS England), Glyn Wood (Strategy Manager at Manchester University NHS Foundation Trust), and Dr Beatriz Duran Jimenez (representing the Pan-UK Pharmacy Working Group for ATMPs on behalf of Anne Black) all discussed the ongoing barriers to realising the wider system uptake of advanced therapies, key infrastructure demands, and ongoing workforce requirements.

Mr Doak spoke on NHS England's role in the adoption of commissioned advanced therapies. Currently, service commissioning relies on a three-year horizon scanning process based on intelligence gathering through company, clinical, and patient engagement. To date, this has translated into UK hospitals delivering advanced therapies to hundreds of patients a year, with CAR-T therapies representing the 'lion's share' of treatments delivered.

Once again, the unique nature of advanced therapies presents unique challenges here. Mr Doak reflected on the specific challenges of setting up often complex services for advanced therapies as close to HTA decisions as possible. As such, NHS England has begun to take a strategic approach to these technologies by building expertise through disease-specific centres and upskilling providers in anticipation of growing advanced therapy pipelines.

Despite this, it is clear hurdles still remain. Service commissioning largely remains structured on a topic-by-topic basis, demand for providers and specific services (such as intensive care and apheresis) is increasing, and the workforce demands of delivering advanced therapies continue to grow.

Mr Wood echoed these sentiments, particularly noting the difficulties of building bespoke patient pathways product-by-product within post-reimbursement statutory windows.

He argued that this was a key challenge given that HTA mechanisms only assess the value of new medicines and not the operational feasibility of their introduction into the broader health system. Greater standardisation of advanced therapy delivery mechanisms would, therefore, create more efficiency within the system – driving cost-savings and inevitable patient benefits.

Dr Duran Jimenez provided additional support to these statements. From her point of view, moving towards a more collaborative commissioning process that better considers regional-level input would allow the NHS to evolve its approach to advanced therapy delivery.

Dr Duran Jimenez also outlined the "frustration" of workforce capacity being a limitation to realising innovation. Investment into essential infrastructure, such aseptic services, as well as growing and maintaining specialist headcount, must be seen as a priority to prevent the loss of "historical knowledge" and to build the capacity to deliver excellence faster.

It was further recommended that in order to allow for growth in research and licensed ATMPs in the UK, all of the above should be included within regional ICS operational strategies.

ATTC Network post-event recommendations

These recommendations have been strategically informed by the valued input from attendees at the ATTC Network UK Advanced Therapies Adoption Challenge October 2023 event. As such, the following considers a mixed perspective of key expert input – from those with lived experience and clinical expertise to those with broader system delivery and life sciences viewpoints. Key organisational absences must however be acknowledged – particularly those from UK HTA bodies such as the National Institute for Health and Care Excellence (NICE) and Scottish Medicines Consortium (SMC) who were unfortunately unable to attend the event on the day.

While these recommendations have been informed by this multi-stakeholder input, they are ultimately the priority asks of the ATTC Network. They should be considered in support of, and in conjunction with, the recommendations of other organisations engaging in the advanced therapy landscape.

The timely implementation of the following is essential to seizing the generational opportunity offered by advanced therapies to both the health service and UK life sciences.

Recommendation Structure

Pillar 1

Clinical Trials

Leveraging existing world-leading expertise will enable the UK to be the best place internationally to trial and deliver advanced therapies.

Pillar 2

Value and Reimbursement

Realising the value of advanced therapies will ensure that UK patients are able to benefit from the latest cutting-edge health technologies.

Pillar 3

NHS Readiness

Investing into supporting workforce and NHS delivery infrastructure will allow the UK to benefit from advanced therapy delivery at scale.

The foundation

Data

Improving the use of data system-wide is essential to harmonising the advanced therapies ecosystem.

Implementation

Taskforce

Creating a government-led advanced therapy taskforce in early 2024 will cement advanced therapies as a strategic UK-wide health policy priority.

Pillar 1: Leveraging existing world-leading expertise will enable the UK to be the best place internationally to trial and deliver advanced therapies

The UK has long been a hotbed for world-class innovation and scientific research. The work of existing initiatives has helped the UK get a head start on advanced therapies – making it an attractive market to develop these transformative innovations. Unfortunately, countries like Spain and Australia have begun to outperform us on the number of new (total) clinical trials being initiated, as well as on the speed of new trial set-up.[vii]

There is now a need to act swiftly to build on existing system expertise to ensure that advanced therapies remain an exception to these negative trends – strengthening the UK's R&D credentials by making it one of the best places in the world to trial these innovations at scale.

Taking an innovation-first approach to clinical trials

The UK governments should take steps to recognise the critical strategic interest of advanced therapies to the health system holistically and the wider UK economy. To incentivise clinical trial investment, particular attention must be given to continually improving the speed of new trial approval and domestic advanced therapy expertise.

Earlier this year, Lord O'Shaughnessy's Commercial Clinical Trial Review provided a series of recommendations to make UK trials better, faster, and more internationally competitive. Within these recommendations, the introduction of a small series of Clinical Trial Acceleration Networks (CTANs) was proposed. These networks would look to establish a new 'enhanced service' for clinical trial activity by providing access to additional resources and expedited approval processes. This programme would be underpinned by private, public, and academic partnerships - providing clear examples of 'best-in-class' performance.

The implementation of this initiative has since been supported by the Department of Health and Social Care in England with a commitment of £20 million over two years to establish a small number of CTANs.

Recommendation

Advanced therapies, through the ATTC Network, offer an opportune platform to continue to implement a 'CTAN-like' model and to deliver on these ambitions.

To meaningfully implement this, steps to expand the ATTC footprint should be considered within government clinical trial funding commitments – allowing the UK to capitalise on existing system expertise and empowering the network to develop and implement innovative, equitable approaches to advanced therapy clinical trial delivery.

vii. Association of the British Pharmaceutical Industry (2023) Global rankings - Number of industry clinical trials initiated in 2021, by country, by phase. Available at: https://www.abpi.org.uk/facts-figures-and-industry-data/clinical-trials/global-data/global-rankings-number-of-industry-clinical-trials-initiated-in-2021-by-country-by-phase (Accessed: December 2023)

Pillar 2: Realising the value of advanced therapies will ensure that UK patients are able to benefit from the latest cutting-edge health technologies

A competitive medicines access environment is essential for attracting advanced therapy investment into the UK. Unfortunately, there are ongoing instances of strong misalignment between UK HTA bodies and advanced therapy manufacturers over the longer-term value provided by advanced therapies to patients.

Current UK HTA processes face specific challenges accounting for the uncertainty associated with the long-term effectiveness of these technologies. Inversely, manufacturers often seek to launch these medicines with limited longer-term clinical data – so as not to delay patient access to these innovations. This mismatch in expectations and the resultant impact on any determination of value have been the primary drivers of 'differences of opinion' in the assessment of these medicines to date.

While the clinical benefit of advanced therapies at the individual product level may vary, it must be noted that on aggregate the uncertainty around these innovations is low. Scientific rationale supports their efficacy and there is a high probability that many products will deliver very significant and lasting benefits to patients.

Recommendation

Performance-based reimbursement mechanisms would help address the long-term uncertainty on sustainability of effect at the time of launch. Such mechanisms would ensure that therapies that deliver long-term patient and healthcare system value are not penalised, while healthcare systems are insulated from the risk of overpaying for therapies that fail to deliver on these aspirations.

Recent steps taken within the newly announced 2024 Voluntary Scheme for Branded Medicines Pricing, Access and Growth (VPAG) undeniably represent a real positive step towards achieving this. The wider consideration of innovative payment models that help underwrite the potential risk of introducing these innovations within the NHS is a key step to 'bridging the gap' between manufacturer and health system expectations.

Recommendations

The transparent, timely, and comprehensive delivery of innovative payment model pilots is a priority. To ensure that these commitments can be delivered to the fullest effect, it is also important that existing Treasury and NHS contracting rules are reconsidered so that potential outcomes-based schemes can be delivered over five years or more.

Building upon this and to help overcome further barriers to access at the individual product level, the ATTC Network recommends that the UK governments commission an independent, formal review on the appropriate mechanisms to assess the full value of advanced therapies.

The findings of this report will allow all relevant decision-makers to consider wider value definitions of advanced therapies and to determine the merits of assessing them through potentially fundamentally different appraisal pathways.

A thorough analysis of the most recurrent HTA and NHS commissioning challenges facing advanced therapies is a vital step to ensuring relevant methods remain up-to-date and are able to sufficiently recognise where the best examples of these emerging technologies offer value now, and in the longer term. Such an analysis should also more broadly consider the benefits of alternative mechanisms for the assessment of advanced therapies and the potential for bespoke pathways to prioritise faster patient access and provide more defined frameworks for longer-term data collection.

Further considerations should be given to the 'innovation value' of advanced therapy investment to both the broader healthcare system and the UK life sciences ecosystem – with an assessment being made on the economic benefits of the UK becoming a true world leader in access to pioneering health technologies.

The development of this formal report and any resultant recommendations must lean on the full range of advanced therapy expertise within the UK and be underpinned by a productive dialogue between UK HTA bodies, key industry representation (the ABPI, BIA, and EMIG), as well as input from leading clinical voices and those with lived experience.

Pillar 3: Investing into supporting workforce and NHS delivery infrastructure will allow the UK to benefit from advanced therapy delivery at scale

Achieving scale is a fundamental hurdle to maximising the true value offered by advanced therapies system-wide. The widespread implementation of these technologies will help to establish an innovation foundation fuelled by competition, investments into the UK market, and, most notably, increased access for those who can benefit clinically.

Tactical, targeted investment into both the workforce and advanced therapy infrastructure is essential to address current bottlenecks to scale and to 'futureproof' the health system for a rapidly expanding pipeline of these innovations. Current investment and planning here is incremental and driven by short-term system requirements.

There is now a need for a strategic, longer-term approach to be adopted instead that considers both direct national and regional challenges.

Addressing short-term system bottlenecks

Workforce:

The NHS and accompanying bodies are facing continued challenges across their workforces that are not only limiting ability to deliver care but also, more specifically, capacity to deliver innovation.

Recommendation

Broader workforce issues such as those pertaining to limitations around career progression should be improved upon, with the ongoing NHS Long-Term Workforce Plan providing the necessary scope to achieving this.

In a similar light, challenges in MHRA resourcing have until very recently driven significant downstream impacts – slowing down vital clinical trial approvals. Recent improvements have meant that the MHRA is now approving trials in compliance with statutory timelines – a development that must be applauded.

Recommendations

These processes should now remain under ongoing review to help support the regulator to continue to deliver timely approvals.

To mitigate similar pressures in the future, further measures should be considered to prioritise the training, recruitment, and retention of those in key regulatory roles. These same principles should also be applied to advanced therapy practitioners more generally.

Infrastructure:

The often highly personalised nature of advanced therapies can present unique challenges such as short-product shelf-life. Advanced therapies require advanced infrastructure to meet the requirements of 'just-in-time' sample procurement and therapeutic product delivery.

Recommendations

To ensure that the UK can be a world-leading provider of advanced therapies now, targeted investment is needed at a larger number of NHS Trusts into:

A. Dedicated facilities for 'starting material' handling and patient sample processing for use in the advanced therapy manufacturing/delivery process.

-AND-

B. State-of-the-art pharmacy aseptic preparation facilities for gene therapies alongside access to cutting-edge facilities for cell and tissue therapies. These prospective facilities must be supported by rigorous oversight and quality systems - ensuring medicines of optimal quality can be administered to patients.

The ATTC Network is already positioned as a perfect test case exemplar of these vital investments and would be able to oversee the rollout of additional resources in a phased approach according to infrastructure demands.

Recommendation

To further support this, pharmaceutical companies with prospective cell and gene therapy pipelines should be encouraged to begin standardising the 'delivery logistics' of these technologies. At a UK level, the Cell and Gene Therapy Catapult and pharmaceutical industry representation should look to further vital cross-company discussions on this topic. Input from 'on the ground' clinical commissioners and providers should also be considered in such conversations.

The foundation: Improving the use of data system-wide is essential to harmonising the advanced therapies ecosystem

Data needs to be better recorded, better structured, and become more widely available to support system-wide improvements to the advanced therapies ecosystem.

Data is arguably the most powerful tool in supporting the development and launch of advanced therapies. It underpins key decisions made throughout the advanced therapy lifecycle and is fundamental to improving patient outcomes and system utilisation of these technologies.

Recommendations

System-wide structured, standardised, and widely accessible data must be better collated and managed to:

- Improve the collective understanding of current standards of care (including the lived experience of those with relevant conditions).
- Provide deeper insights on clinical trial eligibility and recruitment.
- Support robust clinical trials and long-term real-world treatment outcomes.
- Guide key reimbursement and investment decisions.

Implementation: Creating a government-led advanced therapy taskforce will cement advanced therapies as a strategic UK-wide health policy priority

Recommendation

To deliver on the outlined recommendations and reaffirm the UK's position as a priority innovations market, UK governments should consider the implementation of a multi-stakeholder, cross-department advanced therapies taskforce in early 2024.

This expert team could provide direct ministerial oversight over an advanced therapies 'growth strategy' with a view of exhibiting the UK's global life sciences credentials.

A primary role of this group would be to position advanced therapies as a 'first order' policy priority and maintain its strategic policy importance.

The need to plan ahead

To effectively prepare for delivery at scale, UK governments need to take a longer-term holistic view of UK advanced therapy investment.

Recommendation

Considering this, a prospective advanced therapies taskforce should look to commission a long-term economic impact assessment that considers the broad value and opportunity provided by these technologies to: UK GDP, life sciences investment, workforce opportunities, and patient outcomes.

These findings will provide the economic basis for a multi-year advanced therapies investment programme – outlining a deliverable ATMP workforce and infrastructure roadmap that prioritises targeted support for key advanced therapy-specific roles and direct investment for critical infrastructure.

Membership built on impact and expertise

Any prospective membership of this group must include dedicated representation from: relevant health and business departments, treasuries, NHS, HTA body, and MHRA leadership, as well as direct input from lived experience voices.

It is essential that existing, established system expertise from the ATTC Network and the Cell and Gene Therapy Catapult is leveraged as a guiding component of the work conducted by such a group.

To achieve maximum impact, this expertise must be supplemented by leading clinical input such as that from the relevant medical colleges and associations.

By making a positive impact across access, clinical trials, workforce, infrastructure, and data, this bespoke taskforce will hold the keys to unleashing the true potential of advanced therapy innovation to patients, the health system, and the broader UK life sciences industry.

Organisational attendees

Association of the British Pharmaceutical Industry
Adaptimmune
Advanced Therapies Wales
Alex TLC
All Wales Medicine Strategy Group
Alliance for Regenerative Medicine
Anthony Nolan
Autolus Therapueutics
Bristol-Myers Squibb
Cardiff and Vale University Health Board
Cell and Gene Therapy Catapult
CSL Behring
Debra UK
Department for Health and Social Care
DI Rees Ltd
Diabetes UK
Duchenne UK
Ethical Medicines Industry Group
Galen Biomed Ltd
Gene People
Genes and Health
Genetic Alliance UK
Genomics England
Gilead Sciences
Great Ormond Street Hospital
Guy's and St Thomas' NHS Foundation Trust
iMATCH
Immunodeficiency UK
Imperial College London
Innovate UK
Instil Bio
J&J Innovative Medicine
Kings College Hospital
Krystal Biotech
Leeds Teaching Hospitals NHS Trust
LSD Collaborative
Manchester University NHS Foundation Trust
MAP Patient Access
Metabolic Support UK
Medicine and Healthcare products Regulatory Agency
Midlands-Wales ATTC
Newcastle University
Newcastle upon Tyne Hospitals NHS Foundation Trust
Newmarket Strategy
NHS Blood and Transplant
NHS England
NHS Greater Glasgow & Clyde
NHS National Services Scotland
NJ Redfern Ltd
Northern Alliance ATTC
Northern Health Science Alliance
Orchard Therapeutics
Oxygen Strategy
Pfizer
Resolution Therapeutics
Royal Society of Medicine
Sheffield Teaching Hospitals NHS Foundation Trust
Sickle Cell Society
Scottish National Blood Transfusion Service
Society for Mucopolysaccharide Diseases and the Rare Disease Research Partnership
Syncona
The Christie NHS Foundation Trust
The Royal Marsden
Unique
University College London
University College London Hospitals
University of Birmingham
University of Edinburgh
University of Sheffield
University of York
Vertex
Welsh Health Specialised Services Committee