

Advanced Therapies in Wales: Your questions answered!

Advanced treatments like gene and cell therapies are set to revolutionise current care pathways. However, bridging the gap between research and treatment is critical in this emerging field. We asked leading stakeholders your most important questions exploring how industry and health and social care can help to translate these developments into transformative therapies for patients across Wales.



Q1

In Wales, who is the best contact for those working in the gene therapy field of Pharma for securing future partnerships with Cardiff and Vale University Health Board?

In the first instance, the [Advanced Therapies Wales](#) programme team and [Life Sciences Hub Wales](#) are happy to act as point(s) of contact. They can explore any potential future partnership opportunities and support organisations in bringing innovation to Wales.

Answered by Dr Mark Briggs

Q2

How do we attract clinical trial innovation to Wales?

The [Advanced Therapies Wales](#) programme is working with [Life Sciences Hub Wales](#), Health and Care Research Wales and clinical centres to identify the challenges of attracting and delivering clinical trials within Wales. The programme also continuously works in collaboration with other aligned initiatives such as the Midland-Wales Advanced Therapies Treat Centre, having already established one ATMP trial with several others in set up. Similarly, the programme will continue to support clinicians looking to conduct a clinical trial who already have established relationships with therapy researchers and developers.

Answered by Dr Mark Briggs

Q3

How do we encourage active clinical trial uptake in Wales?

Engagement is key for ensuring patient awareness of the existing opportunities for clinical trial participation. Additionally, it is important that companies looking to recruit patients into trials are aware of the opportunities in Wales. The clinical trial units coordinated with ATTC are both excellent facilities located in both South and North Wales.

Answered by Prof Steve Conlan

Good infrastructure is critical for delivering early phase trials, with ongoing discussions with colleagues in Cardiff and Vale University Health Board occurring. We are eager to bring trials to Wales that benefit our patients as early as possible. Covid-19 gave a short gap in programme recruitment mostly due to a pause in sending referrals, but this has restarted after a brief pause in March and early April.

Answered by Dr Ceri Jones

Q4

What are the patient recruitment initiatives?

There are standard and established processes to identify potentially eligible patients to join a clinical trial apply. However, it is recognised that recruitment into trials can be a slow process. The trial sponsor should therefore be amenable to discussing potential additional strategies outside of the norm to identify and recruit the desired number of patients in a timely manner. For example, this may draw upon sponsor experience and recruitment best practice in other locations.

Answered by Dr Mark Briggs

Q5

How can we increase international collaboration for Advanced Therapies supporting patients worldwide?

The Advanced Therapies Wales programme team is open to collaborating and learning from other organisations. [Life Sciences Hub Wales](#) is the primary point of contact, taking the lead in uniting national and international partners.

Answered by Dr Mark Briggs



Q6

Is there plans for a Regenerative Medicine programme?

'Regenerative medicine' will remain within the Advanced Therapies Wales programme.

Answered by Dr Mark Briggs

Q7

What hurdles or bottlenecks do you see for ATMPs in Wales?

We must overcome many challenges before ATMPs are broadly and equitably available to our population and similarly before Wales is able to gain maximum economic benefit. These major areas are outlined in the Statement of Intent for Advanced Therapies published by the Welsh Government.

Answered by Dr Mark Briggs

Q8

Is there a plan to ensure we have the right workforce (and skillset) in Wales to support ATMPs?

Having the right workforce available to support this sector is an essential part of the plan for Advanced Therapies Wales. This includes NHS staff and the wider industry and academic workforce. The Advanced Therapies Wales team are already working on this through the Midlands-Wales Advanced Therapy Treatment Centre (MWATTC), which will form the basis of the Wales plan.

Answered by Cath O'Brien

Q9

How are technical support services being managed?

The Advanced Therapies Wales programme will consider the full breadth and scope of the estates, resources and infrastructure required for adopting and deploying such treatments.

Answered by Dr Mark Briggs

Q10

Advanced Therapies is a broad area – what should Wales specialise in?

The development, production and delivery of Advanced Therapies requires the input, expertise, and participation of many different organisations on the journey from initial concept to routine adoption and delivery. As such, Wales should aim to provide support to such organisations where there is already a (developing) expertise, looking to strategically address any identified gaps.

Answered by Dr Mark Briggs

Q11

How do we capture the patient voice?

We want to achieve this through engaging and working with the public in numerous ways. We are keen to draw upon the experience and expertise developed within Genomic Partnership Wales, specifically from Wales Gene Park colleagues. We will also look to work with relevant third Sector organisations, as well as collaborating and co-hosting with similar activities organised by the UK Advanced Therapies Treatment Centre programme. In response to the current Covid-19 pandemic, we envisage hosting a series of virtual events and workshops over the next 12-18 months to capture patient voice. We will use work done by Genomic Partnership Wales and charity organisations as an example and guide to help achieve this.

Answered by Dr Mark Briggs

Q12

How do we ensure that we capture the patient/public voice when discussing Advanced Therapies, balancing high cost and accessibility?

Public voice and opinion importance is acknowledged and forms a major future focus area for the Advanced Therapies Wales programme. They intend to extensively engage with the public on a variety of topics around awareness and education, encompassing opinions on cost against accessibility. This process will draw upon the experience of other programmes, such as Genomics Partnership Wales, and collaboratively with national UK initiatives such as the Advanced Therapy Treatment Centres.

Answered by Dr Mark Briggs

Q13

What are the access and reimbursement challenges for commercial products?

Current guidance is that all products that meet the necessary criteria to become available to the population in Wales, such as receiving a positive recommendation from the All Wales Medicines Strategy Group (AWMSG) or the National Institute for Health and Care Excellence (NICE). Providing local access to all such treatments may not be possible. Therefore, some may need to be commissioned outside of Wales that could, for example, draw upon the New Treatment Fund provision by the Welsh Government. Similarly, the significant cost and limited clinical evidence base for some of the advanced therapies may prove challenging when considering current reimbursement models. This is globally evident, with payors and the industry therefore exploring alternative reimbursement models such as ones based upon positive outcomes.

Answered by Dr Mark Briggs

Q14

We estimated with Welsh Health Specialised Services Committee (WHSSC) that £1 in every £200 spent on medicines in Wales (2019/20) were on ATMPs for 20 patients. Any comments?

There is already significant cost associated with the delivery and care pathways for patients receiving some of the first approved advanced therapies. We are working with academic and other interested organisations at a national and UK level to explore the validity of current appraisal and assessment models as well as new ones, such as outcomes-based reimbursement within a Value-Based Health Care context.

Answered by Dr Mark Briggs

Q15

Can you comment on funding for patients travelling to England for CAR-T cell therapy?

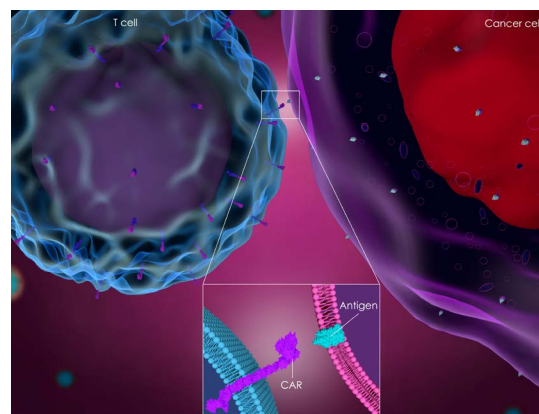
Deciding where to commission relevant approved treatments is currently made by the Welsh Health Specialised Services Team. It is dependent on the ability to deliver the service within Wales and other UK arrangements. As such, some Welsh patients may be referred to and treated at centres outside of Wales.

Answered by Dr Mark Briggs

Q16

CAR-T therapies are very complex and expensive. How does the financial model work, i.e., will future approved CAR-T approaches be available to cancer patients in Wales?

Future CAR-T and other advanced therapies will continue to be assessed and appraised by organisations such as the National Institute for Health and Care Excellence (NICE). Where there is a recommendation that such treatments should become available to individuals meeting the eligibility criteria then these will be appropriately commissioned by the Welsh Health Specialised Services Team. Information gathered through horizon scanning activities, by various organisations including the specialised commissioners, is used to forecast budgetary requirements submitted to Welsh Government for standard financial planning cycles. However, as more advanced therapies become available and commercial competition increases, as well as reductions in manufacturing cost due to increases in volume and other efficiency improvements, it is anticipated that the per treatment cost will significantly reduce.



Answered by Dr Mark Briggs

Q17

Is there possibility to understand genetic predisposition to support our knowledge of how people will react favourably or unfavourably to therapies such as CAR-T?

Considering factors such as genetic predisposition (telomere length / telomerase activity) is important. However, it is likely to be one of several important influential factors, such as the quality of T-cells present. We are currently developing partnerships with academic colleagues to consider and investigate these factors.

Answered by Dr Ceri Jones

Q18

Is there a relationship between T-cell quality, such as presence of exhaustion markers, telomere length measured during patient selection and apheresis, a therapy's probability of success?

Research has identified a correlation between proliferation markers and patients responding to therapy. We are lucky to have Prof Duncan Baird and Dr Stephen Man in Cardiff, who are world experts in telomere analysis and T-cell immunophenotyping. They are currently involved in a pilot study to answer these questions. There is hope that following initial data development, this could be opened up to a wider international research group.

Answered by Dr Ceri Jones

Q19

Guidance on ACRE approval for deliberate release of genetically modified organisms (GMOs) is lacking. Are the ATTC addressing this for Advanced Therapy Investigational Medicinal Products (ATIMPs) that are GMOs?

The Advisory Committee to Releases to the Environment is primarily focussed on providing statutory advice to the UK Government on the risks to human health and the environment from the release of genetically modified organisms (GMOs) associated with 'engineered' plants and animals. Unlike these GMOs that could survive, grow and spread if released into the general environment, ATMPs would not neither persist nor proliferate and pose more of a standard biological risk if humans encountered them. All genetic modified ATMPs and gene modifying materials used in their manufacture are handled under rigorously controlled production and administration conditions that are closely regulated and managed. Any excess material or 'contaminated' consumables are destroyed as part of routine processing of laboratory and clinical waste.

Answered by Dr Mark Briggs

Q20

Has the COVID-19 pandemic adversely impacted on the manufacturing of ATMPs?

Globally and alongside many industries, the pandemic significantly impacted ATMP manufacture. However, this has primarily affected the ability to administer the treatments to patients when healthcare and clinical resources have been in such high demand and stretched. The impact upon academic and commercial researchers, developers and producers has been significantly less.

Answered by Dr Mark Briggs



Life Sciences Hub Wales is here to help accelerate the development of Advanced Therapies in Wales. To get involved, or find out more, email us today hello@lshubwales.com



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