

Making advanced
therapies for
brain diseases
a reality



Advanced
Neurotherapies
Centre

Canolfan
Niwrotherapiau
Datblygiedig



Ymchwil Iechyd
a Gofal **Cymru**
Health and Care
Research **Wales**



Ariennir gan
Lywodraeth Cymru
Funded by
Welsh Government

2025 - 2026 Annual report

Advanced Neurotherapies Centre

brain.wales



Contents

Foreword	3
Our team	4
Our key aims	6
Core metrics 2025 - 26	7
Key achievements	8
Areas of impact	9
Delivering ATMPs to the human brain	10
Clinical trial portfolio	11
Supporting ATMP development for neurological disease	12
Spotlight on: traumatic brain injury	13
Using people's lived experience to improve our trials	14
Spotlight on: research nursing	15
Collaborative work	16
Conclusion	18

Foreword

I am delighted to present our first report as the Advanced Neurotherapies Centre (ANTC), marking our official transition from the BRAIN Unit and the beginning of an exciting new chapter for our team and our partners.

The ANTC stands among only a handful of centres worldwide with the specialist expertise and research excellence required to tackle one of the most complex challenges in modern medicine: the safe and effective delivery of advanced therapy medicinal products (ATMPs) to the human brain.

Difficulties in achieving reliable, well quantified delivery remain a major cause of early phase clinical trial setbacks, preventing many promising therapies from progressing to the patients who need them.

To address this, we continue to build our translational research directly around ongoing clinical trial activity.

By integrating laboratory science with real world clinical delivery, we are deepening our understanding of delivery science, refining the techniques that support safe intervention, and driving improvements in how gene therapies are administered for neurological and neurodegenerative diseases.

This joined up approach is helping to move innovative ideas more quickly and confidently toward meaningful clinical impact.

Looking back over the year's achievements, I am incredibly proud to lead this centre at such a pivotal time.

From our successful launch in June 2025, to the delivery of 10 gene therapy surgeries and expanding into new areas of discovery, we have made remarkable progress.

We are also delighted to congratulate Dr Ronak Ved (see highlight in this report on head injury research, page 14) and Dr Harsh Bhatt (working on brain tumour biology) on the award of their PhDs.

It is my pleasure to share these accomplishments—and the momentum they represent—with you in this report.

We also took the opportunity to honour the contributions and achievements of Dr Laura Bunting, our former Centre Manager, Health and Care Research Wales Infrastructure Lead, and dear friend who sadly passed away in January 2026.

Laura was instrumental in establishing the BRAIN Unit, and the impact of her dedication and leadership continues to be felt across ANTC. She is greatly missed.



A stylized, handwritten signature in black ink, likely belonging to Professor William Gray.

Professor William Gray
Director of the Advanced
Neurotherapies Centre

Our team

Senior leadership



Professor William Gray

Director



Professor Anne Rosser

Deputy Director



Dr Cheney Drew

Lead for clinical trials
methods
and public involvement



Dr Jane Davies

Senior Research Nurse



Jo-Ann Baker

Centre Manager



Clinical trials delivery and support teams

Mr Amad Khan
Suzannah Peck
Alison Johnson
Arsenia Davies
Dr Gowthaman Sarasawathy
Carol Thomas
Matt Healey
James Davies
Luke White
Robert James



ATMP development lab teams

Dr Mariah Lelos
Professor Bill Griffiths
Dr Owain Howells
Dr Tom Massey
Professor Vincent Dion
Dr Ronak Ved
Harsh Bhatt
Dr Francesco Bedogni
Dr Chloe Ormonde
Dr Sam Loveless
Narcy Villalobos
Georgia Wilson



Intracranial delivery research teams

Professor Sam Evans
Dr Dmitri Sastin
Dr Daniel Parry
Dr Sacha Chiuta
Dr Sanchita Bhatia
Professor Derek Jones
Professor Angela Mihai
Juan Hernandez Vega
Dr Bailin Deng
Dr Victor Romero Cano
Dr Marco Palombo
Dr Daniel Gallichan
Dr Leandro Beltrachini



Project management and communications

Ashlie Brown
Helen Hughes
Kate Martin
Becs Parker

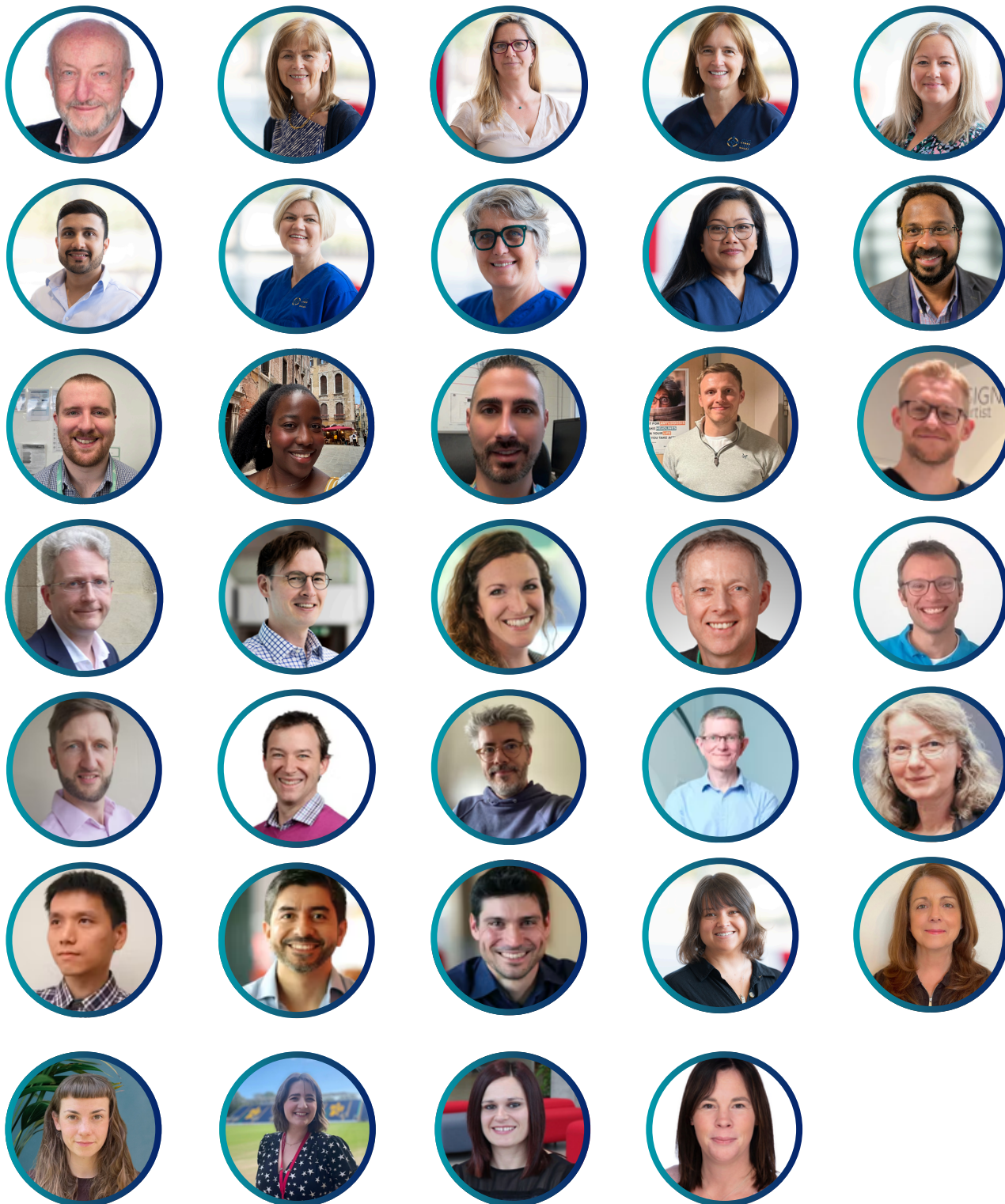
With thanks to:

- Swansea Bay MRI Physics team
- Cardiff and Vale University Health Board clinical delivery and operations teams
- St Mary's Pharmaceutical Unit, Cardiff (SMPU)

The people working to make advanced therapies for brain diseases a reality



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Our key aims

We are entering an exciting era in which disease-modifying treatments are becoming a reality for brain diseases that are currently untreatable.

This includes Alzheimer's and Huntington's diseases (HD), or limited to symptomatic approaches, such as Parkinson's disease (PD) and many forms of epilepsy.

Many of the most promising emerging treatments use advanced therapeutic medicinal products (ATMPs), which involve delivering genes, ribonucleic acid (RNA), or cells directly into the brain.

The Advanced Neurotherapies Centre is one of only a few centres worldwide with the research capacity to address the translational challenges of intracranial delivery.

We are developing and investing in "delivery science" and de-risking first in human trial transition using human tissue.

We prioritise delivery science for improving and quantifying clinical trial ATMP delivery, as the greater need and opportunity for impact.



Delivering ATMPs to the human brain and innovating delivery technology in early phase clinical trials

We are:

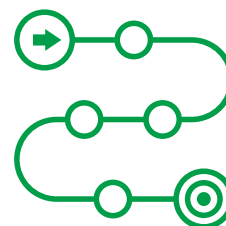
- delivering advanced therapies with NHS and industry partners
- optimising therapy delivery through research and innovation
- preventing early trial failures caused by poor delivery



Supporting ATMP development for neurological disease

We are:

- developing living human brain-tissue models to confirm drug targeting and detect early toxicity
- using adult and foetal tissue to study major brain disorders and advance a first-in-human Huntington's disease cell therapy
- advancing lipidomic, genetic and tissue-analysis methods to identify biomarkers and improve patient selection for trials



Using lived experience to improve how our trials are designed

We are:

- embedding the patient voice across all our research
- ensuring our work stays aligned with stakeholder needs in a rapidly evolving ATMP landscape
- improving clinical trial design through patient-focused research adopted by national and international groups



Core Metrics

Reporting period: 2025/2026

Health and Care
Research Wales
infrastructure award
to the group



Direct
funding
awarded

£571,261

Jobs created
through direct
funding



Grants won during reporting period

Grants won	Led by group	Group collaborating
Number	8	4
Value	£1.73m	£119k
Funding to Wales	£1.73m	£77.7k
Funding to group	£1.7m	£77.7k
Additional jobs created for Wales	14	1
Additional jobs created for group	14	1



Number of publications



Number of public
engagement events



Number of public
involvement opportunities

Key achievements

Advanced Neurotherapies Centre: year in review

Over the past year, the Advanced Neurotherapies Centre (ANTC) has consolidated its position as a national leader in early-phase neurotherapeutic research while laying firm foundations to become a Centre of Excellence for advanced therapies.

Despite operating in a rapidly evolving research and healthcare environment, the Centre has delivered major clinical, scientific and strategic progress, transforming early-phase clinical delivery.

ANTC remains **the only centre in the UK** currently able to deliver direct-to-brain gene therapies in adults.



Strengthening research and innovation pipelines

Alongside clinical delivery, ANTC advanced its research capability through expanded access to international disease imaging datasets, new interdisciplinary collaborations, and investment in high-performance computing. Together, these developments are enhancing Cardiff's capacity in imaging, data science and drug-delivery modelling, supporting more personalised and efficient trial design (see page 11).

Building national capacity and influence

Through Cardiff Health Partners, ANTC has helped drive system-wide improvements to support the delivery of advanced therapy medicinal products across Wales.

Recruitment and surgical delivery continued across multiple first-in-human and early-phase trials, strengthening Cardiff's role as a critical UK gateway for advanced neurotherapeutics (see page 11).

A major milestone was the publication of early results from the uniQure AMT-130 Huntington's disease trial, which demonstrated a substantial reduction in disease progression compared with matched controls. While early, these findings have generated international interest and renewed hope among patients and families affected by Huntington's disease.

This work has resolved key operational barriers and established a clear plan to double surgical capacity in 2026, alongside proposals for expanded staffing and upgraded facilities (see page 9).

Leadership beyond Wales

ANTC has played an active role in national policy discussions, parliamentary engagement and UK research networks, ensuring Welsh expertise helps shape the future of advanced neurotherapies.

Growing international collaborations continue to reinforce Cardiff's reputation as a centre of excellence in neurotherapeutic innovation.



These achievements mark a year of real progress. We're bringing transformative treatments closer to patients while strengthening Wales's leadership in advanced neurotherapies.

Areas of impact

Centre development and capacity

During the past year, ANTC has made significant strategic progress toward becoming an internationally recognised Centre of Excellence for advanced neurotherapies, with a strong focus on long-term sustainability, scalability and system readiness.

Through Cardiff Health Partners, ANTC has led and contributed to a series of Task and Finish Groups spanning Cardiff University, Cardiff and Vale University Health Board and Velindre University NHS Trust. These groups identified critical operational barriers to delivering ATMPs and developed coordinated, system-wide solutions.

Policy influence

In parallel, ANTC has strengthened its national policy presence.

In November 2025, Professor William Gray addressed the Huntington's Disease Association parliamentary reception at the Palace of Westminster following international interest in early AMT-130 findings.

Key outcomes include the resolution of longstanding challenges and an agreed plan to double surgical capacity from one to two gene therapy cases per month in early 2026. Further proposals to expand clinical staffing, secure dedicated clinical space and refurbish theatre infrastructure are progressing.

ANTC's designation as one of three flagship Cardiff Health Partners programmes reflects its strategic importance to Wales's ambitions across innovation, health equity and economic development. Collectively, this work provides a clear pathway toward establishing a national service for advanced neurotherapies.

Engagement with Welsh policymakers was reinforced through the visit of Stephen Doughty MP in January 2026 (pictured below).

ANTC also expanded its national profile through the UK Dementia Trial Network, an all-Wales application for early-phase trials centre accreditation, and a keynote presentation at the MRC Regional Visit in January 2026.





Delivering ATMPs to the human brain and innovating delivery technology in early phase clinical trials



Clinical trial activity at ANTC continues to go from strength to strength, reinforcing its position as the only UK centre currently delivering direct-to-brain gene therapies in adults.

Over the past year, the Centre supported recruitment and surgical delivery across two major gene therapy trials, with all UK neurosurgical procedures led by Professor William Gray.

Key milestones included publication of early results from the uniQure AMT-130 Huntington's disease trial, demonstrating a marked reduction in disease progression compared with matched patient groups.

While preliminary, this data has generated significant hope within the Huntington's disease community and attracted widespread international attention.

During the year, **seven** REGENERATE PD and **three** ASPIRE FTD surgeries were successfully completed.

ANTC also expanded its non-surgical portfolio, becoming one of only three global recruitment sites for Alnylam's first-in-human phase 1 RNA interference therapy for Huntington's disease (PI: Dr Tom Massey).



Image shows: Professor Gray and colleagues beginning the stereotactic surgery to deliver a gene therapy.

Additional early-phase studies in myotonic dystrophy further broadened patient access to cutting-edge therapeutics.

This clinical momentum has been matched by progress in imaging and computational innovation.

Access to the CHDI Foundation's global Huntington's disease imaging datasets is enabling refinement of disease-progression models with potential to inform future trial design and personalised treatment strategies.

To support this work, high-performance computing infrastructure is being installed at Cardiff University Brain Research Imaging Centre (CUBRIC), alongside strengthened collaborations with the Schools of Computer Science and Mathematics, including development of a multiphase biomechanics model to advance infusion-based drug delivery.



Image shows: a gene therapy is being infused directly into a trial participant's brain under general anaesthesia in our dedicated surgical MRI scanner.



Our clinical trials portfolio

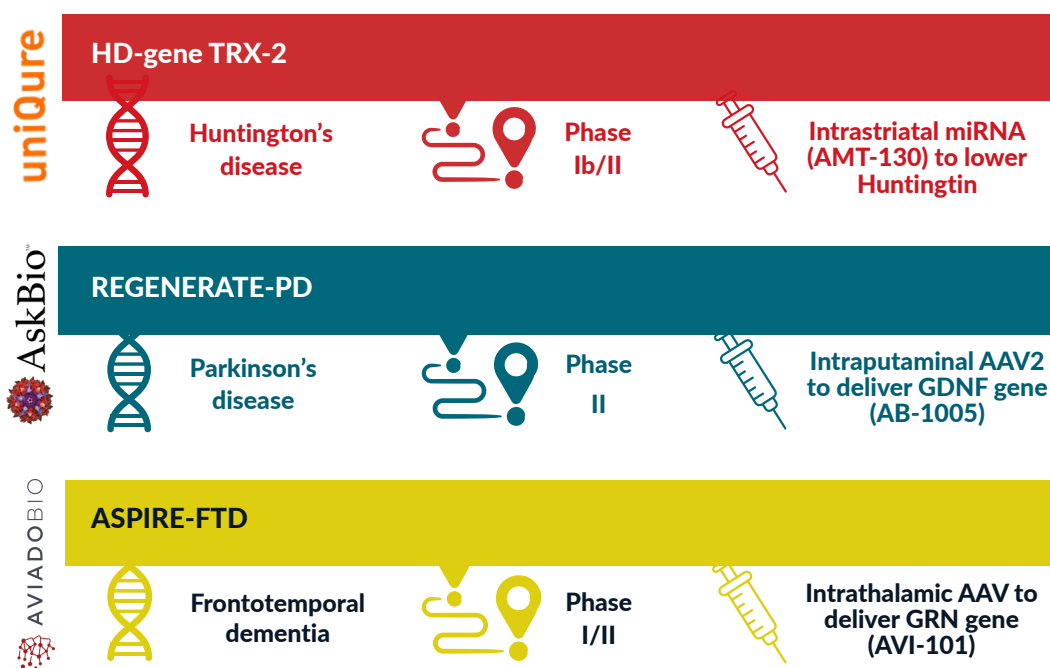
In this reporting period, the ANTC has delivered advanced therapies to **12** clinical trial participants.

~£800k income to Wales through delivery of this portfolio



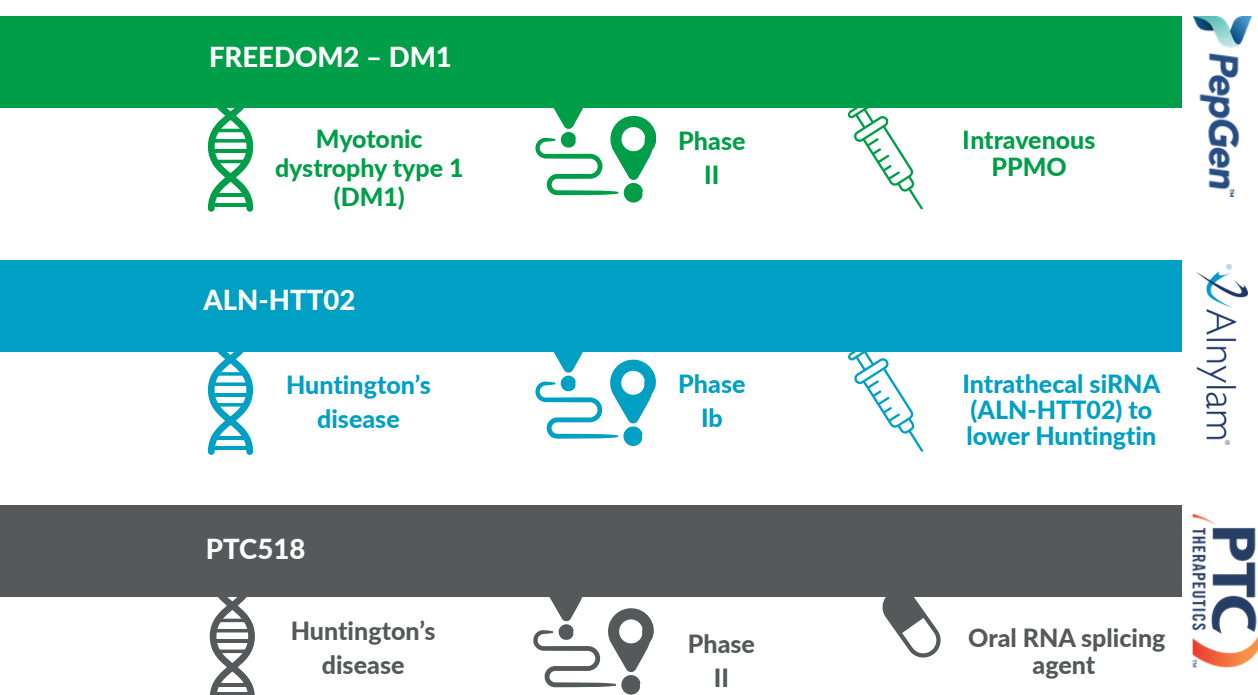
Neurosurgical

Delivers therapies directly to specific areas of the brain using surgically placed devices while the patient is in an MRI scanner, to see exactly where the therapy is going in real-time.



Intrathecal (IT) and intravenous (IV) and oral

Delivers therapies to the brain through the blood, via a cannula inserted into a vein. We use this for ATMPs that we know will cross the blood-brain barrier.





Supporting ATMP development for neurological disease

Professor Anne Rosser and Dr Mariah Lelos are developing a novel stem cell-derived cell therapy product to treat people with Huntington's disease.

Funded by a five-year MRC Programme Grant and an MRC Gap Fund grant, and supported by ANTC infrastructure, over the last year the group has worked to characterise the gene expression profiles of both their cell therapy product, and the final in vivo graft, by single cell/nuclear RNAseq.

These high-resolution data further confirm that they are able to generate the required medium spiny neuron progenitor cells, which are able to survive, integrate into the brain and improve function in models of Huntington's disease.



The group completed a gap analysis and risk assessment with the UK Cell and Gene Therapy Catapult, which confirmed that the preclinical package is comprehensive and the team are ready to complete a safety and toxicity study, the final hurdle before clinical translation.

Professor Rosser and Dr Lelos also engaged with the Medicines and Healthcare products Regulatory Agency through a Scientific Advice meeting and were able to agree the design of the safety and toxicity study.

The team are now seeking external funding to progress the cell therapy product through this regulatory step, with the vision of establishing a first-in-person clinical trial thereafter.

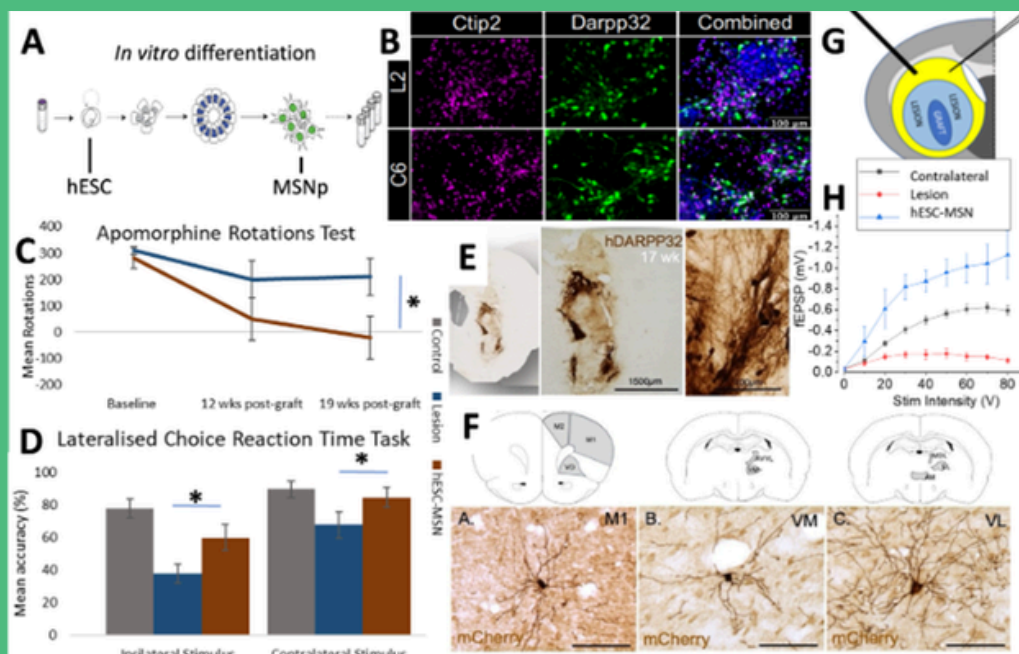
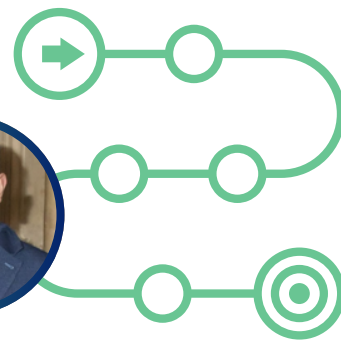


Figure 1: Key summary data generated for hPSC-MSNp product RHD-001.

- A)** Summary of the protocol to make the cell product.
- B)** Evidence of cells expressing key markers CTIP2 and DARPP32.
- C-D)** Post-transplant, the cell product induces motor and cognitive improvements in an animal model of Huntington's disease.
- E)** Grafts express mature MSN markers (DARPP32 shown).
- F-H)** Behavioural recovery is underpinned by restoration of innate circuitry validated by (F) monosynaptic rabies tracing and (G-H) slice electrophysiology post-synapse potentials.

Spotlight on

Dr Ronak Ved - MRC Clinical Research Fellow



Developing new treatments for traumatic brain injury

“ In my role as a neurosurgery registrar based at the University Hospital of Wales, I regularly care for patients who have suffered a traumatic brain injury (TBI).

TBI is a leading cause of death and disability in Wales, the UK, and worldwide.

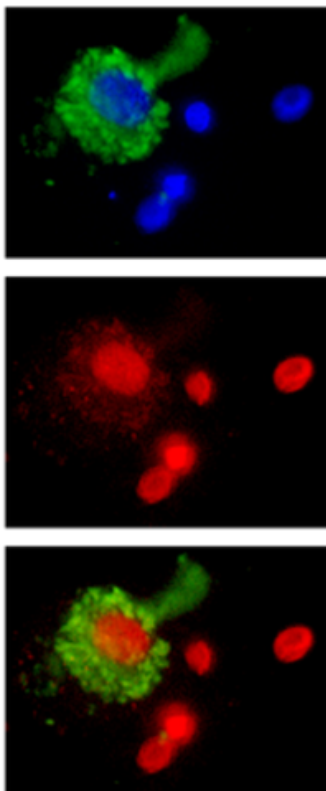
Since the pathophysiology of TBI is still not fully understood, treatment options for these patients are limited.

This is, in part, also due to a prior reliance upon animal models of head injury to study TBI, which whilst important, come with significant limitations and ethical concerns.

This work, performed at and supported by the ANTC and the Rhiannon Jade Smith Memorial Trust, could help to progress our search for more effective treatments for brain trauma patients, and streamline pathways for drug development and subsequent progress to human clinical trials.

It may also help to reduce some of the previous reliance upon ethically challenging, and more expensive, animal testing within the research field of TBI.

We’d like to congratulate Ronak on successfully defending his viva based on the work above in April 2026.



Dr Ved’s method



I utilised a novel weight-drop, human brain tissue, petri-dish based model of TBI, developed by our human tissue team at the ANTC, to assess the impacts of trauma upon human brain cells.

I used resources and tissue at the ANTC, alongside support I obtained from a Medical Research Council Clinical Research Fellowship Award, to design a series of experiments using this model to investigate human brain cell responses to a blunt force injury.

I then identified a specific cell signalling pathway which, when manipulated, markedly improved human brain cell survival after traumatic injury.



Image shows: microscopic image of a human brain immune cell (Microglial cell: Green periphery, purple centre) expressing the neuroinflammatory protein, HMGB1 (Red) after being exposed to a weight drop injury in the lab



Using people's lived experience to improve how our trials are designed

Over the past year, the ANTC has further strengthened its commitment to placing patients and the public at the heart of its research, clinical activity and strategic direction.

Building on the strong foundations of the former BRAIN Involve group, we refreshed our patient and public involvement and engagement (PPIE) structures to ensure that lived experience continues to shape our work in meaningful and sustainable ways.

Several experienced members have remained involved, providing valuable continuity, while new members with lived experience of neurodegenerative conditions are being recruited.

This group will play a central role in shaping grant applications, contributing to key events, and informing our public-facing communications.

Our PPIE work focuses on two key priorities: addressing inequalities in access to advanced therapy clinical trials, and increasing public understanding of emerging cell and gene therapies.

Dr Cheney Drew



Dr Cheney Drew was commissioned by the Midlands and Wales Advanced Therapy Treatment Centre (MW-ATTC) to lead public involvement and engagement across the UK-wide Advanced Therapy Treatment Centre Network, working under the governance of Cell and Gene Catapult.

As part of this national leadership role, Dr Drew facilitated a high-level round-table discussion at the ATTC Network's 2025 national conference in London, exploring how meaningful patient involvement can be embedded within research, clinical and commercial strategy.

The insights generated are helping to inform best practice across the sector.

Alongside this, Dr Drew is supporting the development of a new, PPIE-driven strategy to tackle long-standing barriers to participation in advanced therapy research.

Co-developed with patient groups, NHS partners and industry, this work will outline clear, practical actions in 2026.

In parallel, we are co-producing a series of short 'explainer' videos with members of the public to make the science behind advanced therapies more accessible.



Spotlight on

Dr Jane Davies and Suzannah Peck



Research nursing

Our research nurses are a crucial part of our clinical team, and are the familiar faces supporting patients throughout their clinical trial journey.



As Lead Research Nurse for Advanced Neurotherapies, I see research nursing as a vital bridge between clinical care, research delivery and innovation.

Within the ANTC, my colleagues and I are often the consistent, familiar presence for patients, supporting them throughout what can be a complex and sometimes daunting clinical trial journey.

Our role goes far beyond traditional nursing. It brings together advanced clinical practice, research expertise, coordination and leadership, all underpinned by a strong commitment to patient-centred care.

Working closely with multidisciplinary teams, including neurosurgery, radiology, anaesthetics and pharmacy, we coordinate highly complex early-phase trials, ensuring that treatments are delivered safely and effectively within the pressures of the NHS.

Colleagues such as Suzannah Peck, Senior Research Nurse, are central to this work. Together, we manage the day-to-day delivery of advanced therapy trials, maintaining high standards of clinical care, data integrity and regulatory compliance.

Attention to detail, organisation and adaptability are essential, as we align multiple teams, schedules and procedures to ensure everything is in place for each patient.

However, the most important aspect of our role is supporting participants and their families. Participation in these trials often involves major decisions, including experimental neurosurgery.

We provide continuity throughout the pathway, helping participants understand their options, navigate the process and feel confident in their decisions.

Over time, we often become a key point of contact, building trust and advocating for patients to ensure their needs remain central.

We also contribute to improving how research is delivered. By seeing first-hand how protocols affect patients, we can help refine processes, reduce burden and enhance the overall experience.



Ultimately, research nursing at ANTC is about balancing complexity with compassion, enabling innovative therapies to be delivered safely while ensuring patients remain at the heart of everything we do.

Dr Jane Davies



Collaborative work

Over the past year, the ANTC has played a leading role in a wide range of high-impact collaborations across the UK and Europe.

Leading international workshops on advanced therapies

What?

Our team led the Surgical Delivery Task Force in Venice, and contributed to a series of high-profile international workshops focused on advanced therapies in Huntington's disease, covering therapy evaluation, clinical trial design, biomarkers, and the global challenges of delivering advanced therapies to the brain. These workshops took place in Venice, Amsterdam, California, and across the UK.

Who?

The workshops were led and chaired by members of ANTC's senior leadership team, working with leading international experts.

Key collaborators included Professors Sarah Tabrizi (UCL), Cristina Sampaio (CHDI Foundation), ATW, the European Huntington Disease Network SDTF and multidisciplinary experts from the UK, Europe, and the United States.



What difference did it make?

Together, these workshops built international consensus on evaluation frameworks, trial methodology, outcomes, and biomarker strategy for advanced therapies in neurodegenerative diseases.

Outcomes include formal reports to the EHDN Executive Committee, white papers and peer-reviewed publications, and the identification of shared bottlenecks in advanced therapy delivery.

The work strengthened global partnerships, catalysed joint funding applications, shaped future policy and clinical trial design, and reinforced leadership in advanced therapy strategy and translational research worldwide.



Launch of the Neuro Multi-Disciplinary Research Group (MDRG)

What?

Establishment of a new Neuro MDRG as part of the Advanced Therapies Wales programme, with two meetings held in July and November 2025.

Who?

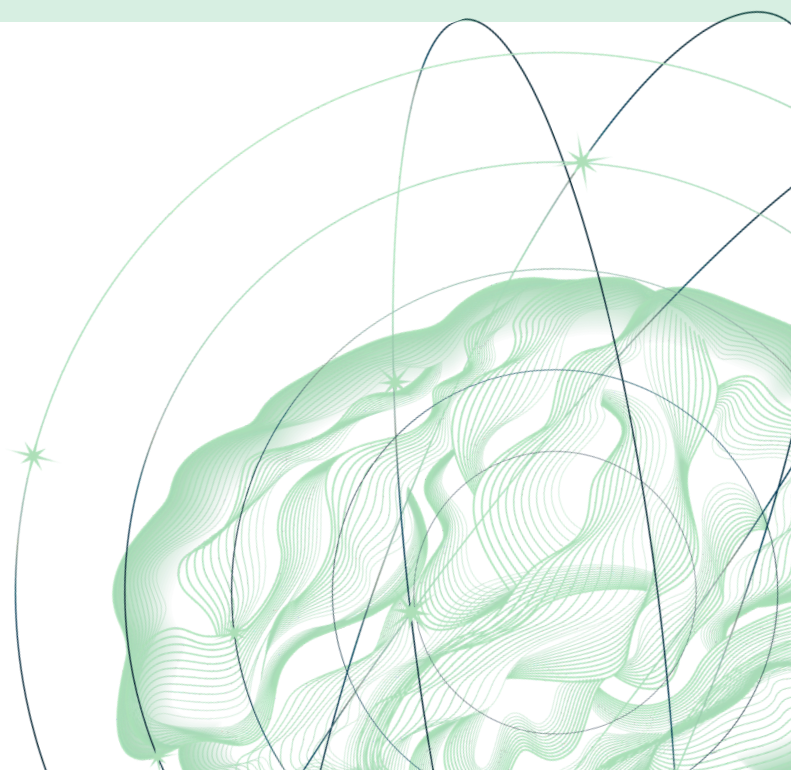
Co-chaired by Professor William Gray and Dr Tom Massey, bringing together 37 researchers from across Cardiff University, NHS partners and related disciplines.

What difference did it make?

The meetings created structured opportunities for cross-disciplinary collaboration, directly leading to new partnerships across Computer Science, Mathematics and Psychology.



They also supported the development of teams and ideas for future large-scale grant applications, strengthening Wales's neurotherapeutics research pipeline.



Conclusion

As I reflect on the year captured in this report, I am struck by how much has been achieved in a relatively short period of time.

The transition from the BRAIN Unit to the Advanced Neurotherapies Centre has been more than a change in name—it has marked a step change in ambition, scope and collective confidence.

This year has laid firm foundations for ANTC as a centre defined not only by scientific excellence, but by our ability to bring complex advanced therapies closer to patients through safe, thoughtful and innovative delivery.

What makes this progress especially meaningful is the people behind it.

The work described throughout this report has been driven by a truly multidisciplinary team—clinicians, scientists, research nurses, engineers, data scientists and public partners with lived experience—working together to solve some of the most challenging problems in modern medicine.

I am deeply grateful for their commitment, creativity and determination, and for the trust placed in us by patients and families who participate in early-phase trials. Their willingness to engage with uncertainty reinforces our responsibility to get delivery right.

Looking ahead, delivery science will be an important part of how ANTC continues to build capability and impact over the next few years.

To support this, we are excited to explore this area in greater depth through an expanded team and new collaborations across Cardiff University's Schools, particularly in Computer Science, Mathematics and Engineering.

These partnerships will allow us to better integrate experimental, clinical and computational approaches to understand how therapies behave once they enter the human brain.

The recruitment of new research staff in Advanced Computational Modelling is a key step in this journey, strengthening our capability to develop predictive tools that directly inform trial design and therapeutic strategy.

As Director, I am particularly proud of the way ANTC has grown alongside its partners.

Working with local, national and international stakeholders—including the NHS, universities, industry, funders, policymakers and patient organisations—has been essential to our progress and will remain central to our future.



Together, we will continue to build ANTC as a sustainable Centre of Excellence for advanced neurotherapies: one that combines innovation with rigour, ambition with responsibility, and scientific progress with real-world impact for patients in Wales and beyond.

Professor William Gray

Director of the
Advanced Neurotherapies Centre

We are hosted by Cardiff University, with our clinical activity supported by Cardiff and Vale University Health Board at University Hospital of Wales.

We receive core funding from Welsh Government through Health and Care Research Wales, Advanced Therapies Wales and Midland Wales Advanced Therapies Treatment Centre.



Interested in getting involved?

Help influence research by joining our public and patient involvement group.

Please contact us for more information:



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Stay in touch

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