

A CURE CAN'T WAIT

OUR RESEARCH STRATEGY
2015 - 2020

OUR VISION

Our vision is a world where brain tumours are defeated.

Average five year survival for malignant brain tumours is just 15%. We want a world where treatments are available so that the diagnosis of a brain tumour is no longer a death sentence.

We fight brain tumours on all fronts through research, awareness and support to save lives and improve quality of life.

- **We fund pioneering research** to find new treatments, improve understanding, increase survival rates and bring us closer to a cure.
- **We raise awareness of the symptoms** and effects of brain tumours, to reduce diagnosis times and make a difference every day to the lives of people with a brain tumour and their families.
- **We provide support and information** for anyone affected to improve quality of life.

The way we work is:

Pioneering

We are relentless and courageous in the pursuit of our vision, seeking new ways to make a difference.

Passionate

People are at the heart of our charity. We are passionate about finding a cure and ensuring that everyone affected has the highest quality of life and chance of survival.

Collaborative

We will only achieve our vision by building networks, partnerships and a strong brain tumour community. We collaborate and value everyone's contribution.

Smart

We are focused and intelligent in our approach to defeating brain tumours.

Our commitment to research

Our biggest area of investment is always in research, as this will make the greatest difference for everyone diagnosed with a brain tumour, now and in the future. Over the last five years our research spend has consistently been between 50-60% of our overall income.

We have invested over £12million and are committed to investing a further £5.8million in our current research portfolio. We have been able to leverage additional funding for brain tumour research by:

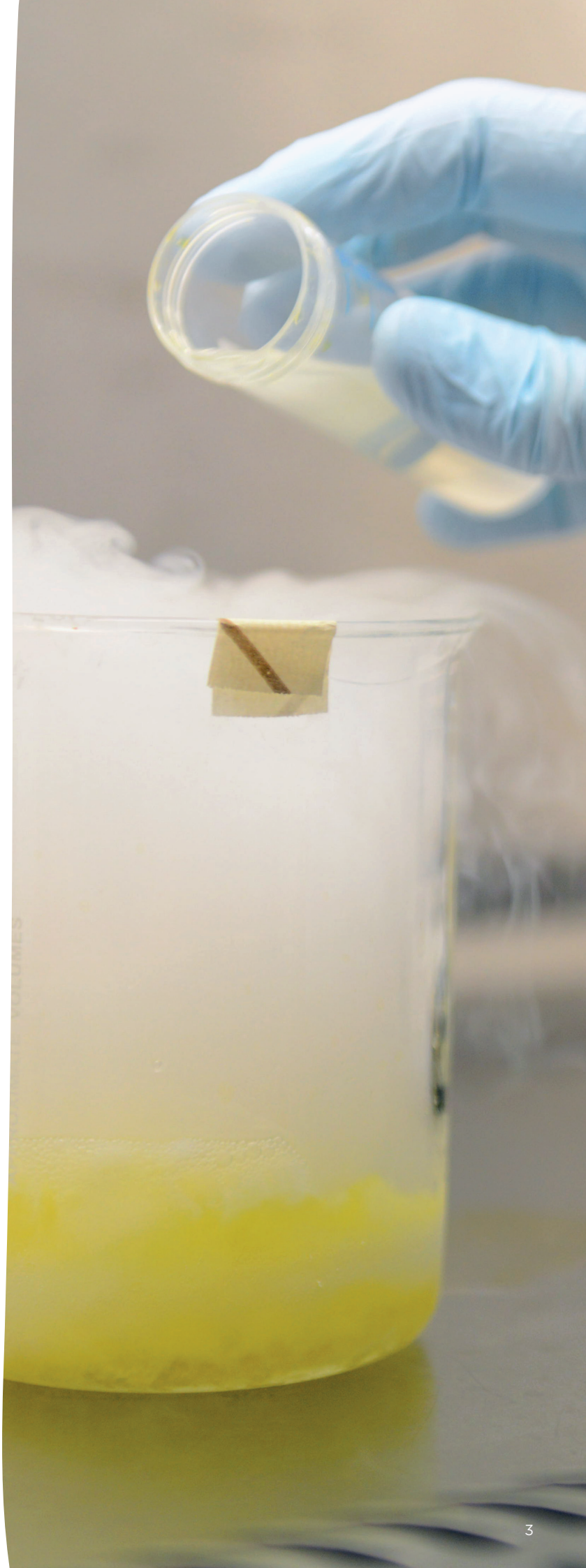
- Ensuring institutions are eligible to apply for top-up funding from the government for the grants we provide, which can be up to 28p for every £1 we invest.
- Working in partnership with other organisations, for example Cancer Research UK and the Medical Research Council, to co-fund significant programmes of research. Co-funding ensures that the funds our supporters raise are effectively doubled.
- Requiring the institutions we are funding to match our investment, again doubling the income available for research into brain tumours.

"The research we have funded to date is making progress towards better diagnostic techniques and new treatments to improve quality of life and survival rates. We could be at an historic turning point for research into brain tumours as we build on this and accelerate the pace of discovery."

Professor Colin Blakemore, Professor of Neuroscience and Philosophy, University of London, and President of The Brain Tumour Charity.

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FOREWORD

Every day, 25 people and their families in the UK are devastated by the words 'you have a brain tumour' and almost 5,000 people lose their lives every year. Brain tumours are the biggest cancer killer of the under 40s.

Despite this, unlike other cancers, survival rates for adults have improved little in over 40 years and still reduce life expectancy by an average of 20 years, the highest of any cancer. Whilst survival has improved for children, over 62% of children are left with a life-altering, long-term disability, which has a life-long, profound effect on them and their families.

Now is not the time to be timid if we want to change these shocking statistics and significantly accelerate progress. Our community wants to know why there isn't a cure and better treatment options. Now is the time to challenge the status quo, to seek answers that may lie outside of 'traditional research', to push the boundaries of human knowledge and be part of the community who not only understand the human brain but also how to control the growth that is characteristic of tumours.

'A Cure Can't Wait: Our Research Strategy' is a bold step to changing this. It is the result of listening to key opinion leaders in brain tumour research globally and those who have become experts through their own personal experiences of the disease.

Treatment for, and understanding of, brain tumours is twenty years behind other cancers. Yet science has moved on. We have built the tools and understanding that will mark the next decade as one of astonishing discoveries. The human genome has now been sequenced, we can use imaging technologies to trace the patterns of our thoughts and actions and use reactive chemicals to light up tumorous cells during brain surgery. The treatments we need lie at the centre of fields of science which are only just emerging.

We are committed to being part of a global community that will make the breakthroughs needed to understand, treat and cure brain tumours. We need patients, carers, clinicians and scientists to work together. But our community must be wider than that. Government, charities, funding bodies and everyone who cares about improving outcomes for people with brain tumours must work together to progress research in this area.

We know that, across all disease areas, funding research makes a crucial difference and requires long-term commitment. Whilst in 2013 we led a £13 million investment

programme into brain tumour research, which represented a significant step-change for this underfunded disease, we now need to focus on the future. We aim to at least double our research spending over the next five years to continue our investment in world-class, pioneering research.

Brain tumours are different to other cancers – they affect the part of you that makes you, you. This is why our research strategy does not simply focus on survival but also on quality of life. It is only by taking an integrated approach with our research, influencing and advocacy activities that we will improve survival and quality of life of those affected by this devastating disease.

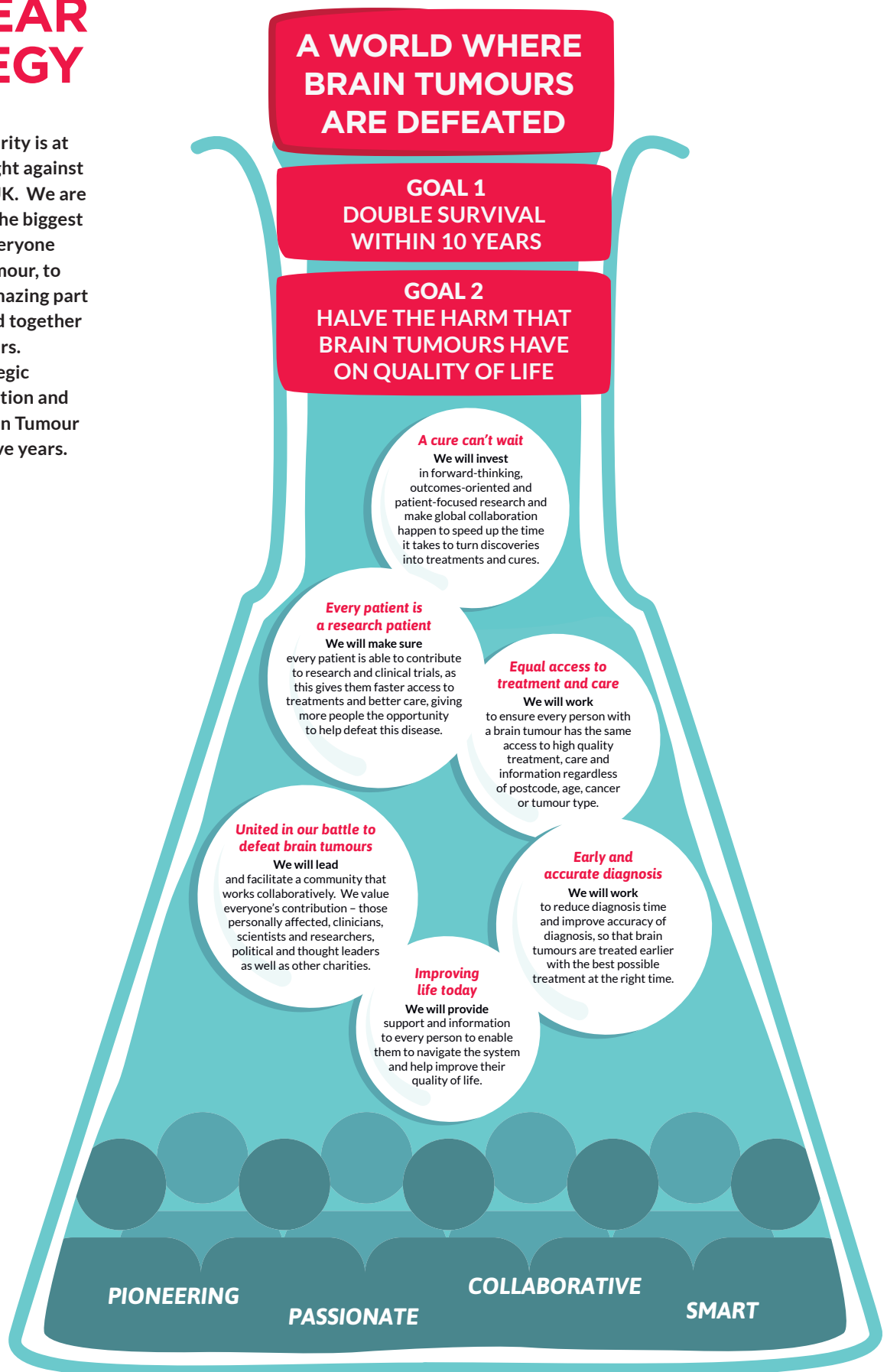
We are very grateful to all of our supporters across the UK, without whom this investment would not be possible. Yet we need to do even more to ensure we can deliver our research strategy. Together, we can revolutionise brain tumour research in the UK to lead us to the cure we are all desperately seeking.

Sarah Lindsell, CEO
December 2014

Over the past year, we have brought together researchers, clinicians and patients to answer the question: "How can we double survival and reduce the harm caused by treatments for brain tumours within the next ten years?"

OUR OVERARCHING FIVE YEAR STRATEGY

The Brain Tumour Charity is at the forefront of the fight against brain tumours in the UK. We are committed to having the biggest possible impact for everyone affected by a brain tumour, to defending the most amazing part of the human body and together defeating brain tumours. Our overarching strategic plan outlines the direction and ambitions for The Brain Tumour Charity for the next five years.



THE NEED

ADULTS

**-20
YEARS**

Brain tumours reduce life expectancy by on average, 20 years - the highest of any cancer¹



Almost half (48%) of brain tumour patients suffer from depression and anxiety⁴

ADULTS



Just 15% of adults survive for five years or more after the diagnosis of a malignant brain tumour²



Caring for someone with a brain tumour has a negative impact on the emotional wellbeing of over 80% of carers⁵

CHILDREN

130

Each year 130 children and young people (aged 0-19) in the UK lose their lives to a brain tumour³



62% of children who survive a brain tumour are left with a life-altering, long-term disability⁶

¹ Burnet, N., Jeffries, S., Benson, R., Hunt, D. and Treasure, F. (2005) 'Years of life lost (YLL) from cancer is an important measure of population burden - and should be considered when allocating research funds', British Journal of Cancer 92, 241-24

² www.ons.gov.uk/ons/publications/re-reference-tables.html?edition=tcn%3A77-239726

³ www.cancerresearchuk.org/cancer-info/cancerstats/types/brain/mortality/#age - Accessed November 2014

⁴ Quality of life in adults with brain tumors: Current knowledge and future directions. Neuro Oncol (June 2009) 11 (3): 330-339

⁵ Finding a better way? Improving the quality of life for people affected by brain tumours (2014), Report published by The Brain Tumour Charity

⁶ Macedoni-Luksic, M, Jereb, B, and Todorovski, L. Long-term sequelae in children treated for brain tumors: impairments, disability, and handicap. Pediatr Hematol Oncol. 2003; 20: 89-101



62% of adults with malignant brain tumours are diagnosed as an emergency⁷

**OVER
55,000**

people are currently living with a primary brain tumour in the UK¹⁰

< 3%

Less than 3% of brain tumour patients are on a clinical trial¹³



More than one third of people see their GP more than five times before being diagnosed⁸



95% of carers feel better informed after using our fact sheets¹¹



Adult brain tumour patients' experience of the NHS is amongst the worst of all cancer patients¹⁴

6.7 - 5

Average diagnosis time for children and young people from the first symptoms is 6.7 weeks, compared to the very best countries in the world who diagnose in under 5 weeks⁹

120

Every year we support over 120 families who have a child with a brain tumour¹²



There is currently no national study of the NHS experience for children with a brain tumour¹⁵

⁷ NCIN Routes to Diagnosis Report, published September 2012

⁸ Finding a better way? Improving the quality of life for people affected by brain tumours (2014), Report published by The Brain Tumour Charity

⁹ The University of Nottingham, data collected by paediatric neuro-oncology centres for the HeadSmart campaign

¹⁰ www.braintrust.org.uk/news-detail.php?id=362&year=2013

¹¹ The Brain Tumour Charity impact survey, carried out by dunhumby, August 2013

¹² The Brain Tumour Charity internal statistics 2014

¹³ NIHR Clinical Research Network: Cancer, Annual Report 2013-14

¹⁴ The National Cancer Patient Experience survey, Quality Health, September 2014

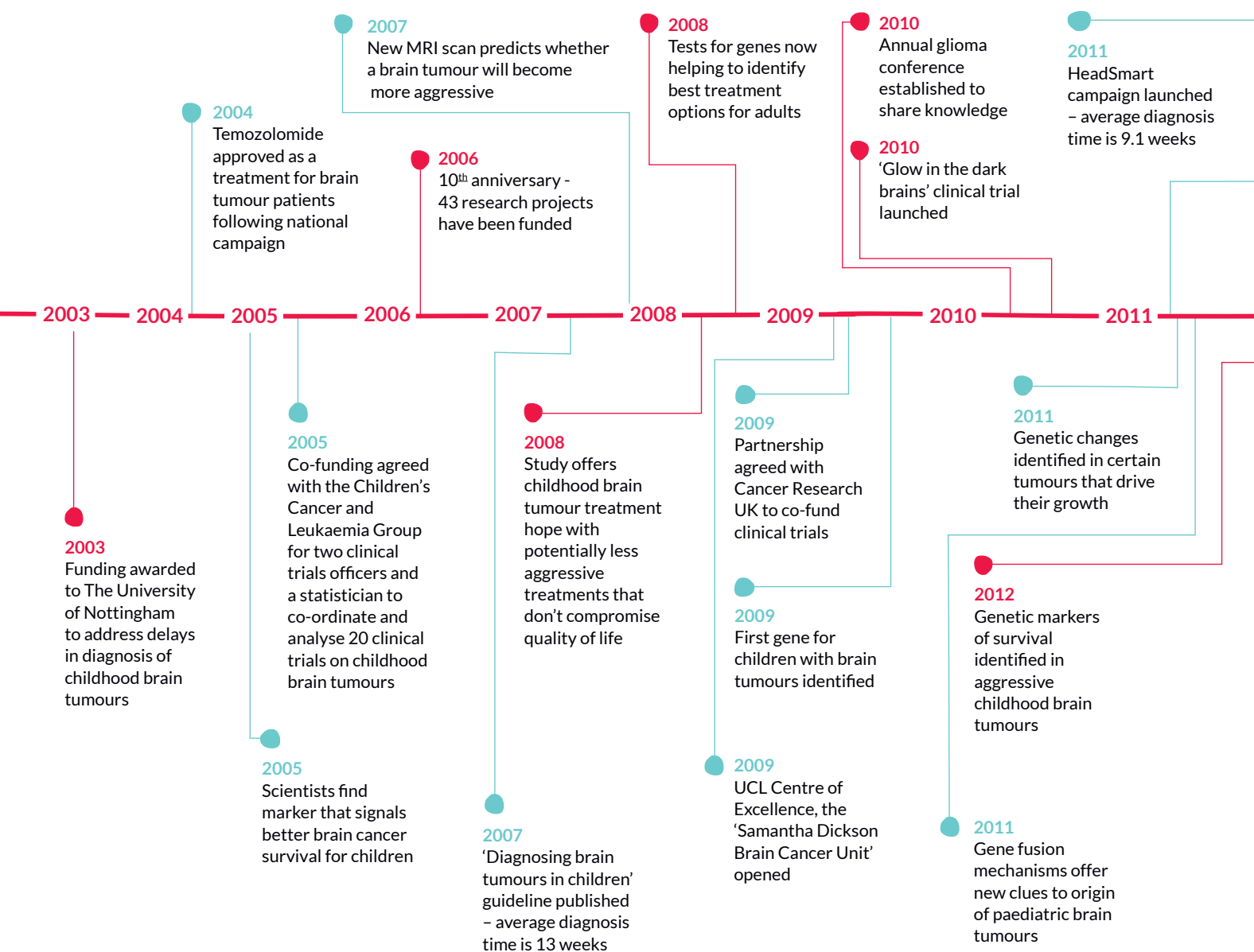
¹⁵ No data available

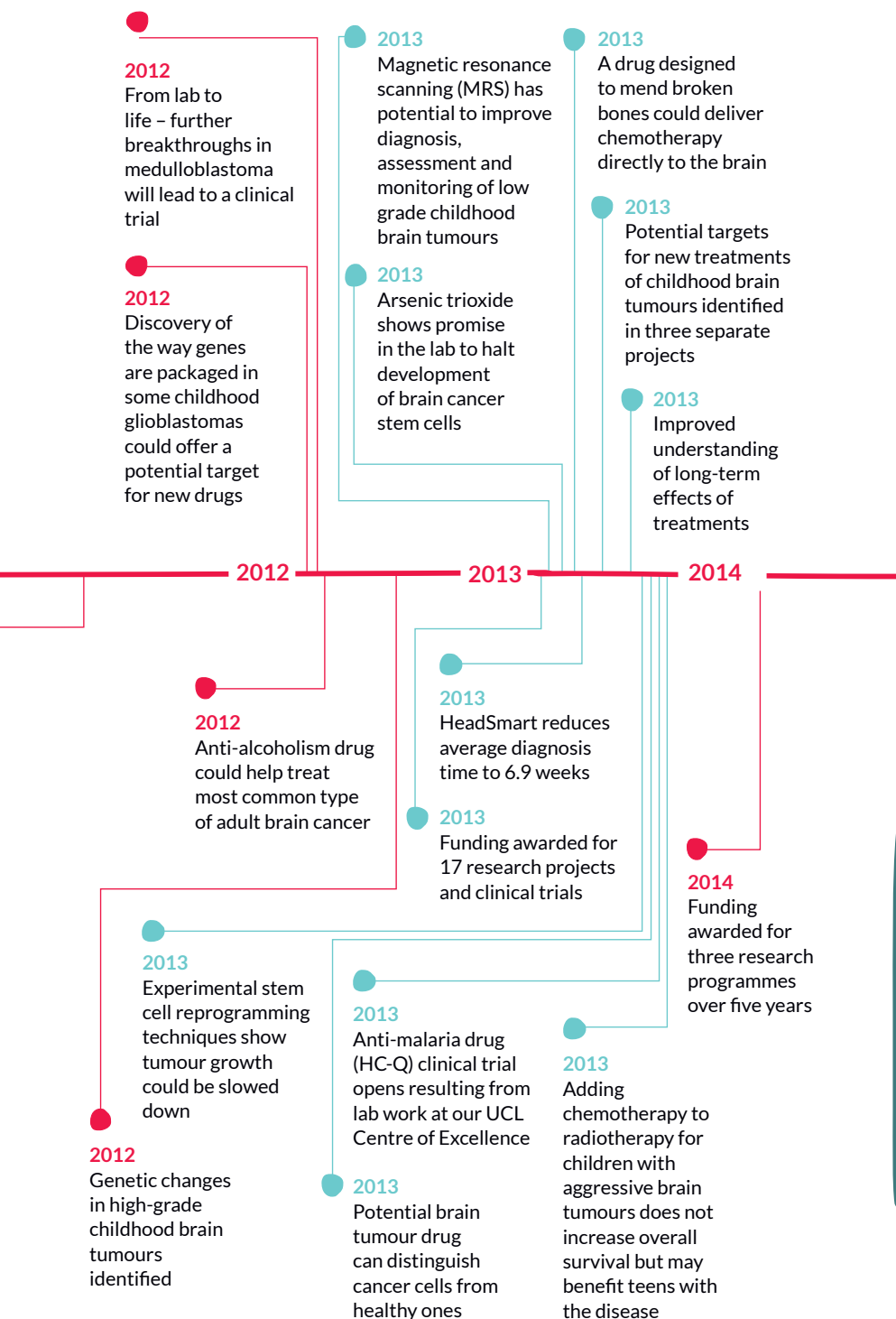
PROGRESS TO DATE

As a charity, we have undergone rapid expansion in the past two years, following the merger of Brain Tumour UK, Samantha Dickson Brain Tumour Trust and The Joseph Foote Trust.

We are now in a strong position to stimulate advances and accelerate developments. In 2014, we brought together the world's leading scientific and clinical experts at our 'Brilliant Minds International Symposium' to discuss global collaboration and feed into our research strategy. We will continue to work globally to bring about the best possible outcomes for people affected by brain tumours in the UK.

We are spearheading the drive for investment into research to discover better treatments, reduce diagnosis times, improve quality of life and ultimately find a cure, building on the experience of the last 18 years and the impacts, breakthroughs and achievements that we have funded so far.





Our funding has already resulted in real impacts for those affected by brain tumours, for example:

- Diagnosing children's brain tumours faster through research into patient pathways that led to our HeadSmart campaign.
- The discovery of new tests and procedures to detect genetic biomarkers of certain tumours in adults.
- The discovery of gene fusions in specific childhood brain tumours (astrocytomas) which has led to the development of new diagnostic tools.

Read more in our Research Impact Report: thebraintumourcharity.org/impactreport

We remain focused and committed to ensuring that the research we fund has the greatest impact.

We are now at a point where we can capitalise on our success to date and focus towards a significant change in outcomes for those affected by this devastating disease.

"These landmark discoveries are important foundations, yet the underlying cause of why our cells sometimes start growing out of control is unknown. As a community we need to push the boundaries of human knowledge, build tools that allow us to investigate and test ideas. We also need to understand the daily impact of living with a brain tumour and remember the many lives lost to brain tumours each year."

Clare Normand, who lost her son Alec to a brain tumour

EXECUTIVE SUMMARY

A CURE CAN'T WAIT: OUR RESEARCH STRATEGY

In the next five years, over 46,000 more people in the UK will have been diagnosed with a brain tumour. Almost 25,000 of these will die. We hear all too often from those affected by brain tumours that there are limited, if any, treatment options for their disease which would increase chance of survival and quality of life. **We will change this.**

'A Cure Can't Wait: Our Research Strategy' outlines our priorities contributing towards achieving our strategic goals:

1. **Double survival**
2. **Halve the harm that brain tumours have on quality of life.**

Our goal is to do this within 10 years and this strategy represents the work we need to do over the next five years to help us get there.

Critically, by integrating our research funding, influencing and advocacy activities we will be able to accelerate progress to benefit patients, as lack of research alone is not the only thing holding us back. Our influencing and advocacy work will seek to optimise the environment in which research

is conducted, thereby facilitating the rapid translation of fundamental research discoveries to benefit patients. This integrated approach to our work is reflected in this strategy.

To achieve our ambitious goals, we will focus our efforts on five priority areas that we believe will have the greatest impact for everyone affected by this disease. We need to develop new and more effective treatments through increased understanding of the mechanisms of tumour growth and development over time. In parallel, promising discoveries made in the laboratory need to be tested and translated as soon as possible into new treatments. Finally, we will identify ways of modifying patient management and pathways to optimise the quality of life for those affected by a brain tumour.

A CURE CAN'T WAIT

DOUBLE SURVIVAL HALVE THE HARM

ACCELERATE

Biobanking to accelerate research progress through centralised collection of, and access to, brain tumour tissue samples

DIAGNOSE

Diagnosing brain tumours earlier and more accurately for both children and adults

UNDERSTAND

Increasing understanding of the genetics and biology of tumour development to identify effective new treatments

CATALYSE

Translating laboratory discoveries into new and effective treatments that increase survival and quality of life

ENHANCE

Enhancing care and quality of life for everyone affected by a brain tumour, including patients and carers

AN INTERNATIONAL APPROACH

PIONEERING

PASSIONATE

COLLABORATIVE

SMART



As a committed member of the AMRC (Association of Medical Research Charities), we will continue to fund only the highest-quality, pioneering research selected through an open, transparent process and subject to stringent international peer review.

OUR APPROACH

To deliver our research priorities, we will pursue a smarter, impact focused and outcomes driven approach to developing and managing our grant portfolio. We will continue to fund high quality research into high and low grade tumours, both adult and paediatric, across tumour types.

Collaboration is key

- We know from the paediatric community that a collaborative approach has enabled progress to be made and brought together researchers across the country and internationally. We will work to facilitate this consortium approach for research into adult brain tumours.
- We know that we cannot achieve this alone. We will seek to develop further co-funding partnerships with research institutions, medical research charities and funding organisations, both in the UK and internationally, where synergies exist to achieve our ambitions. We will also continue to develop our current partnerships, which include Cancer Research UK, the Medical Research Council, Children with Cancer UK and Great Ormond Street Hospital Children's Charity.
- In line with our collaborative approach to funding, we will encourage and facilitate collaborations and consortia between researchers to drive forward our understanding of this disease.

Pioneering in our approach

- To achieve our goals, we will nurture innovation. We will encourage pioneering approaches and new techniques to tackle this disease. We acknowledge that new tactics are needed and that some may be high risk, but also potentially high reward.
- We will facilitate attracting new people from other disciplines and research fields into this area as well as developing the next generation of future leaders in brain tumour research, who are passionate about improving outcomes.

United as a community affected by brain tumours

- Every day we witness the impact of a diagnosis on people's lives and this makes us committed to finding a cure. We can only do this as part of a community of people affected by and working against this disease.
- We will lead and facilitate this united community that includes those personally affected by brain tumours, clinicians, researchers,

political and thought leaders as well as other charities. In particular, when those personally affected become actively involved as experts by experience, we are collectively far more effective.

Learning from every patient

- As we begin to understand the complexity of this disease, it is imperative that we harness the knowledge and learn from each and every patient to accelerate progress and change outcomes for the future. This may be through biobanking or participation in clinical trials.
- Research must continue to focus on the priorities of those affected by brain tumours and we will look for further funding opportunities to increase the involvement of our community in the design, commissioning and dissemination of research.



Rebecca
(left) with
her sister.

RESEARCH IS LETTING ME LIVE MY DREAM

Rebecca's story

In March 2013, in the middle of her studies to become a doctor, Rebecca found out she had a brain tumour.

"I only went to hospital as a precaution after getting a bump on the head, so when I had a CT scan and the words 'brain tumour' came out of the doctor's mouth, my life stopped. It was a rollercoaster of fear, terror, disbelief and denial. The end of my studies was in sight and suddenly I didn't know what the future would hold for me.

"I am one of the lucky ones. Thanks to research into my particular tumour type, my consultant knew how to give me the best treatment for a bright future, and I have resumed medical training. At the time of

writing, I am doing a placement at the same hospital where I had radiotherapy, treating people with cancer including brain tumours.

"However, every patient and every tumour is different. The fundamental knowledge gained through research that gave me back my life simply isn't there for too many types of brain tumour. Tragically, this disease is destroying dreams and tearing families apart every day.

"The other day my consultant told me that my brain tumour is likely to grow back. I turned to my mum and said: 'Don't worry, in ten years' time there'll be loads of new treatments.' We need that new, top quality research now to find these treatments."

ACCELERATE

Biobanking to accelerate research progress through centralised collection of, and access to, brain tumour tissue samples

A key to unlocking progress in brain tumour research is through access to centralised tissue banks containing patients' tumour samples. Despite the importance of access to tissue, we know that routine collection for research isn't a reality yet. Nevertheless, we also know that many of those affected are prepared to gift their tumour samples for research for the benefit of others in the future.

As there are many types of brain tumour, some of which are very rare, we need to ensure that we learn from every patient to accelerate the advancement of more effective treatments in the future.

Centralised biobanking

We need to radically increase the number and frequency of tissue samples banked for research to improve outcomes. This will create a comprehensive resource that will enable key questions to be answered sooner.

In paediatric cancers, the limited and disparate number of cases has already resulted in central tissue banking. Therefore, we aspire to facilitate the centralised and increased collection of adult tumour tissue. Currently, a limited number of adult tissue samples are stored in local tissue banks across the

UK, resulting in small, fragmented collections. This, together with current access arrangements, can make it challenging to get sufficient samples to study individual tumour types, especially the rarer ones. Undoubtedly, creating a centralised bank with a single point of access will accelerate research towards improving survival and quality of life.

Tracking tumours over time

Brain tumour characteristics and behaviours can also change over time. We must therefore endeavour to bank multiple samples from the same patient at different times to track changes in the tumour over the course of the disease from diagnosis and surgery, to recurrence and at autopsy. This will advance our understanding of how patients can be most appropriately and effectively treated.

Centralised adult tissue banking requires a systems and cultural change in the approach to collecting samples from across the community. It will need commitment from patients, scientists and clinicians to ensure that we learn from each and every patient diagnosed with this disease.

WE WILL:

- Initiate and fund a centralised biobank with a single point of access for collection of adult brain tumour tissue.
- Facilitate storage of tissue in this bank and ensure samples are accessible and available for high quality research projects.
- Work with the community to increase longitudinal banking of samples at diagnosis, recurrence and autopsy for both adult and paediatric tumour samples.
- Work to facilitate an international agreement of the core data set so that tumours can be compared. This is a standardised list of the essential scientific and medical information relating to tumour samples that will be common across banks.

WE WILL CALL ON:

- People affected by brain tumours to initiate conversations on gifting their tumour tissue for research.
- Healthcare professionals to take a collaborative approach, working together to create a comprehensive centralised resource that will benefit the whole research endeavour.
- Other organisations to work with us to share expertise to facilitate rapid progress.
- The Government to ensure that proposed EU data protection regulation does not adversely affect the ability of researchers to access tissue or patients to share their data for research.
- UK Departments of Health to ensure that research time is included as standard in neuro-pathologists' work plans given their essential role in banking of tissue for research.

76%

of patients who responded to a survey said they would be willing to undergo an operation to collect biopsy samples from their tumour even if this surgery was not for treatment purposes and would not benefit them directly¹⁶

¹⁶ [Internal survey of 50 people, May 2014.] Unpublished



“HE WASN’T BORN WITH DIPG, SO WE WANTED HIM TO BE BURIED WITHOUT IT”

Alfie’s story

When asked what he wanted to be when he grew up, six year old Alfie replied "I want to be a rock star." At the start of 2013, Alfie began suffering from disturbed sleep, started staring sideways when looking at people and became very clumsy and wanted to be carried everywhere as he was too tired to walk. In the space of a week, Alfie was dribbling, slurring his speech, unsteady on his feet and could not put pen to paper.

In July 2013, Alfie had a seizure at school. His parents Zoe and Danny took him to hospital and, after insisting on an MRI scan, he was diagnosed with an inoperable and incurable tumour of the brainstem, known as diffuse intrinsic pontine glioma (DIPG).

“With DIPG there’s just not enough known about it,” says Zoe. “There was nothing else anyone in the world could do.”

Zoe and Danny always had tissue donation on their minds but didn’t think of it until they heard they only had weeks left to spend with Alfie. Zoe called the consultant, Dr Shaun Wilson, and said she wanted to donate the tumour. Being one of the first donations of its type in the UK, it took Sean a week to find out the process. Zoe then met the John Radcliffe donation team, where she and Danny signed the consent form.

Sadly, Alfie died on 2 June 2014. His family had a couple of hours with him and then he was moved to another hospital to make the donation. Although it was a hard decision it is an amazing one. Researchers need to know what they are researching which is made possible through access to samples. Whole tumours allow them to see how a tumour is structured and how it grows. Zoe is surprised more patients don’t know donation is an option. Families like Alfie’s make an essential difference to drive forward research.

DIAGNOSE

Diagnosing brain tumours earlier and more accurately for both children and adults

Diagnosing a childhood brain tumour early and accurately will save lives and reduce long-term disability. There is little to suggest that this is not the case for adults. When cancer is diagnosed at an early stage, there can be more treatment options and better chances of a good recovery. This is why raising awareness of cancer symptoms is a key aim of the government's cancer strategy.²⁰

Early diagnosis

Our evidence-based, multi award-winning HeadSmart²¹ campaign aims to reduce the time it takes to diagnose childhood brain tumours by raising public and healthcare professional awareness of the symptoms. This campaign is based on research we funded to understand the route to diagnosis experienced by children with brain tumours in the UK.

We have already seen the average time for a childhood brain tumour diagnosis fall from over 14 weeks to 6.7 weeks since the publication of our guideline for healthcare professionals in 2007 and the public-facing campaign which launched in 2011. Our goal is to reduce this to five weeks or less, to be on a par with the best in the world, and we will continue to invest in Headsmart to achieve this.

Too often we hear from those affected by delays in diagnosis. Particularly for adults we hear that, despite knowing themselves that there was something wrong, long delays occur before receiving the correct diagnosis. At present we do not know the average time to diagnose an adult brain tumour in the UK nor the impact that an earlier diagnosis could have on outcomes and quality of life.

Accurate diagnosis

As well as being diagnosed early, it is also vital that patients receive an accurate tumour diagnosis, as identifying the correct tumour type means they can receive the most appropriate and effective treatment. As our understanding of the molecular mechanisms underpinning different tumour types develops, we can use this to help inform diagnostic and prognostic assessments, alongside standard pathology tests. This combined approach towards making more accurate diagnoses is likely to be included in the next revision of the World Health Organisation's (WHO) classification of central nervous system tumours due in 2015.



38%

of people living with a brain tumour visited their GP more than five times before being diagnosed¹⁷

62%

of all malignant brain tumours are diagnosed through an emergency presentation - higher than any other cancer analysed^{18 19}

WE WILL:

- Invest in research to investigate the diagnostic pathway for adults diagnosed with a brain tumour in the UK. This research will provide an evidence base to understand the impact that an earlier diagnosis could have on outcomes.

WE WILL CALL ON:

- NHS England, NHS Wales, NHS Health Scotland and NHS Northern Ireland to ensure that there is equal access to molecular diagnostic services for brain tumour patients across the UK.
- NHS England, NHS Wales, NHS Health Scotland and NHS Northern Ireland to ensure that molecular diagnostic tests included in the next revision of the World Health Organisation's (WHO) classification of CNS tumours are subsequently included in the National Cancer Intelligence Network's (NCIN) Cancer Outcomes and Services Dataset.

"Brain tumours really do change personalities. My ex-partner had a grade four glioblastoma and the transformation in him was shocking. Sadly our relationship broke down before we realised what was going on and sadly after the tumour being removed it returned and he has now passed away. More awareness is needed as we were fobbed off with it being stress and non-epileptic attack disorder despite seizures occurring daily. It took us four months to get a scan and it was only then that we knew what we were dealing with."

Hayley

¹⁷ Finding a better way? Improving the quality of life for people affected by brain tumours (2014) Report published by The Brain Tumour Charity

¹⁸ Compared to an average of 23% across all cancers

¹⁹ NCIN Routes to Diagnosis Report, published September 2012

²⁰ Improving outcomes: a strategy for cancer, published in 2011

²¹ HeadSmart is endorsed and supported by the Royal College of Paediatrics and Child Health, the Royal College of GPs, the Royal College of Ophthalmologists, the College of Emergency Medicine, the Royal College of Radiologists, the General Optical Council and the Children's Cancer and Leukaemia Group.



HEADSMART HELPED A WORRIED MUM PUSH FOR A SCAN

Luke's story

After Fay and Steve's six-year-old son, Luke, started being sick and having frequent headaches, they took him to the GP. The GP suggested he might be worried about school when his tests didn't flag up any concerns. When Luke was sick again, Fay began searching online.

"I very quickly came across the HeadSmart website, which lists all the main signs and symptoms of children's brain tumours."

Fay then remembered all of Luke's other niggles over the previous few weeks, such as mood swings and problems with co-ordination.

"The HeadSmart information crystallised everything. It gave me what I needed to go back to the GP and say: 'I'm really worried'."

This time, Luke was sent for a scan which confirmed that he had a brain tumour. Neurosurgeons were able to remove the tumour completely and Luke will be monitored closely until he is an adult.

"Luke is a real inspiration," says Fay. "He takes everything in his stride and is well on the road to recovery."

UNDERSTAND

Increasing understanding of the genetics and biology of tumour development to identify effective new treatments

Conventional therapies for brain tumours have had limited efficacy over several decades resulting in consistently poor survival rates alongside severe side effects. Therefore, there remains a high unmet clinical need to develop new treatments.

Furthermore, there are over 140 different types of brain tumours²², each with differing biological characteristics. The underlying molecular mechanisms and pathways involved in tumour growth are complex and poorly understood for many of these tumour types.

Developing new and targeted treatments

To develop new treatments, we need to understand about the changes occurring within tumour cells. This will provide information on how tumours start and grow, how cells within tumours differ from one another and why many tumours are resistant to treatment.

Through advances in genomics we can now gain a more detailed understanding of the biological make-up and characteristics of tumour types to develop molecular profiles. This can then be used to develop targeted treatments as well as aiding diagnosis and treatment stratification. By understanding a patient's individual tumour and giving more precise and potentially kinder treatments, this could reduce their side effects and dramatically improve quality of life.

Tumour progression

The characteristics of brain tumours can also change over the course of the disease. In some cases tumours can progress from low grade to high grade disease, but the processes responsible for this change in behaviour are not well understood. Furthermore, some tumour cells are able to invade new areas of the brain, but the changes in cellular processes that instigate this and their interaction with healthy tissue is not clear.

Detailed analysis of the abnormalities within tumour cells will improve our understanding of how tumours progress. By collecting and banking tumour samples from patients we have the potential to achieve this and see real advances.

Research case study

Our funded research at Newcastle University found a biomarker for a sub-type of medulloblastoma. The survival rate for children with this marker was over 90% and only 65% for those without it²³. Together we found a test that could diagnose the different types of tumour within thirty days. This work has led to a pan-European clinical trial to give more targeted treatment, meaning those with a higher survival rate can receive less aggressive treatment, so that their tumour is stopped with fewer side effects.

WE WILL:

- Invest in research that advances understanding of the underpinning genetics and biology of brain tumours which will enable us to identify new targeted treatments.
- Fund research to identify and distinguish between tumour sub-types to enable better prognosis and treatment decision making.

WE CALL ON:

- The Government to ensure that funding is maintained for the Charity Research Support Fund in England and Research Excellence Grant in Scotland, which currently provides up to 28p extra for every pound that we spend on research.
- The Business, Innovation and Skills Department to ensure that they continue to support capital and large infrastructure investment in research institutions as part of the new science and innovation strategy.
- The Business, Innovation and Skills Department to ensure that investment in science funding is maintained through the Research Councils, such as the Medical Research Council.

²² Louis DN, Ohgaki H, Wiestler OD, Cavenee WK, Burger PC, Jouvet A, Scheithauer BW, Kleihues P. The 2007 WHO Classification of Tumours of the Central Nervous System. *Acta Neuropathol.* 2007; 114:97-109

²³ Ellison DW, Onilude OE, Lindsey JC, et al. B-Catenin status predicts a favorable outcome in childhood medulloblastoma: the United Kingdom Children's Cancer Study Group Brain Tumour Committee. *J Clin Oncol.* 2005;23(31):7951-7957



“SIX LITTLE CHARACTERS THAT I HOPE WILL MAKE A LIFETIME OF DIFFERENCE”

Gideon's story

Gideon was diagnosed with a brain tumour in 2012 at the age of just 34. He'd been suffering from small 'out of mind' experiences, each lasting just a few seconds. A keen cyclist, Gideon suffered one of these experiences whilst out on a ride, which forced him to stop. At this point he visited his GP and, following an MRI scan, learnt the crushing news that he had a brain tumour. The 'out of mind' experiences were in fact epileptic seizures. Gideon and his wife have two young children.

"It might look like a collection of characters, but the little code 1p/19q is life changing for me.

"I have an incurable and in my case inoperable brain tumour. When I had a biopsy to discover exactly what type of brain tumour I had, there were two things I was hoping for.

"First, was hoping that my tumour would turn out to be an oligodendroglioma. The chances were slim - it's one of the rarest types of my kind of tumour, but life expectancy with

an 'oligo' is better than the alternatives. Second was that, if my tumour was an 'oligo' that the genetic make-up of the tumour would have a certain set of genes deleted: 1p/19q. Radiotherapy on oligos with those genes deleted is far more effective.

"It turns out I'm one of the lucky ones. My biopsy did reveal I had an oligodendroglioma and that I have the 1p/19q co-deletion. Just knowing these things has meant I can look further into the future than I once feared I could. It's given me hope that brain tumour research will 'catch up' with my condition, and that one day soon research will find a way to control or better treat my brain tumour.

"Finding out the detail isn't everyone's cup of tea. But testing for my genetic make-up gave me peace of mind, armed me with knowledge and gave me just that little extra hope. Six little characters that I hope will make a lifetime of difference to me and my family."

CATALYSE

Translating laboratory discoveries into new and effective treatments that increase survival and quality of life

Currently, it can take 15 years to get new treatments from the laboratory to the clinic. There can be delays in translating research from the laboratory to clinical trials and further delays getting new medicine through regulatory approval. During this period and due to the time lags inherent in the system, those affected by a brain tumour are missing out on promising new treatments. We want to change this. We want to get effective treatments to patients faster.

Emerging technologies and novel approaches

There are unique challenges to developing treatments for brain tumours, such as the blood-brain barrier preventing drugs reaching the tumour site and the inter- and intra-heterogeneity of many tumour types. We therefore need to adopt novel approaches to treatments and drug delivery, by bringing together researchers from other fields, such as physics and chemistry, to tackle these barriers which are limiting progress in brain tumour research.

We also want to ensure that advances in current treatments, such as surgery and radiotherapy, as well as technological developments, such as improved imaging, are applied to improve outcomes for brain tumour patients.

Increasing the number of clinical trials

Clinical trials are essential for assessing and improving treatments. Therefore, more clinical trials will save more lives. Currently, less than 3% of adult brain tumour patients take part in a clinical trial testing a new treatment in the UK, compared to an average of 7.5% across all cancers²⁴. This has to change.

We will continue to support early phase feasibility studies which pilot the safety and efficacy of novel treatments and treatment delivery approaches. Whilst we are not in a position to fully fund large late phase clinical trials, we want to support researchers in the development and design of trials. We will work to bring together partners, industry and patients to help facilitate the progression of treatments to the clinic.

Exploring new avenues

In addition to new drug discoveries, there is increasing interest in repurposing – testing drugs already used to treat other cancer sites or diseases and exploring their potential to treat brain tumours. These drugs may have the ability to treat brain tumours if we discover they are able to overcome challenges, such as crossing the blood-brain barrier and targeting biological characteristics that are key for brain tumour growth. As these drugs are already known to be safe and well-tolerated, they can potentially be accelerated through to clinical trials.

Research case study

From our funded research, we know that hydroxychloroquine, a drug originally designed for use against malaria, may help to make radiotherapy more effective in some glioma patients. This drug is now being investigated in a clinical trial to assess its effectiveness against brain tumours, having moved from lab to clinical trial in an unprecedented time of two and a half years²⁵.

WE WILL:

- Increase our investment into translational and early-phase clinical trials and continue to co-fund feasibility studies.
- Develop partnerships with the pharmaceutical industry and bring together researchers, clinicians and industry leaders to facilitate clinical research and accelerate access to potential new drugs.
- Identify the regulatory barriers to allow for more flexible clinical trial design.
- Work collaboratively with international partners to influence regulators nationally and internationally.

WE WILL CALL ON:

- Healthcare professionals to ensure that all newly diagnosed patients are given information about suitable clinical trials.
- The National Cancer Research Institute Brain Tumour Clinical Studies Group to explore ways to develop the clinical trial portfolio.
- The Government to invest in initiatives, such as the Early Access to Medicines scheme, so that new drugs can get to patients faster.
- The Government to support adaptive licencing of drugs – an initiative which is being considered by the European Medicines Agency (EMA).
- Other partners to share the cost of clinical trials with us, helping our research funding to reach even further and have a bigger impact.

²⁴ NIHR Clinical Research Network: Cancer, Annual Report 2013-14

²⁵ HC-Q trial: public.ukcrn.org.uk/search/StudyDetail.aspx?StudyID=13134 – Accessed November 2014



SEEKING HOPE AT ANY COST: WHY WAS NO CLINICAL TRIAL AVAILABLE?

Richard's story

Richard Hall was diagnosed with a glioblastoma – the most aggressive type of brain tumour – in March 2012.

From that moment on, he was willing to try any treatment that his oncology team or researchers could offer.

“He used to say: ‘I’ll take whatever they can give me,’” recalls his sister, Mel. “His view was that every extra day they could keep him alive was a day nearer to a cure.”

But Richard’s quest to join a clinical trial was thwarted at every turn.

“All of the research we could find was only for a very limited number of people with a very particular type of tumour,” says Mel. “Study after study after study was ‘not suitable’ for Richard.”

The former police officer, who died in January 2013 just two days before his 51st birthday, did find one project in which he could participate. “It would have involved giving blood samples for screening,” says Mel.

But, having indicated his willingness to participate in the trial, Richard was never contacted about it and so missed out.

“He just felt so frustrated. The tumour was going to kill him anyway – he was going to die within months – so why didn’t they just try something?

“Even if it was too late to save Richard’s life, it might have helped them understand a bit more about what he had and how it could be treated in someone else.”

81%

of people think that
everyone diagnosed with
cancer should be offered
the opportunity to take part
in a clinical trial²⁶

²⁶ Charity Awareness Monitor, July 14, nfpSynergy

ENHANCE

Enhancing care and quality of life for everyone affected by a brain tumour, including patients and carers

Being diagnosed with a brain tumour is a life-changing event and there are currently over 55,000 people living with a brain tumour in the UK²⁹. The symptoms are profound, ranging from headaches and problems with co-ordination, to changes in personality and the ability to communicate. Treatments can also be toxic with significant side effects. All of this can have an impact on social, physical, functional and emotional wellbeing. Treating and managing the effects of both the tumour and the treatment is a difficult process that presents questions for researchers.

There are currently limited interventions available to enhance quality of life for those affected by a brain tumour. Most people with a brain tumour are not being monitored to assess the impact of the long-term effects of the different types of brain tumours, their symptoms and treatments on their quality of life.

For children, brain tumours occur as a developmental condition and have long-term outcomes that vary from full recovery to profound and multiple learning difficulties and severe physical disability. We want to change this, so it is necessary to assess the differences in outcomes.

Improving quality of life

Research has traditionally focused on increasing overall survival and on stopping the growth of the tumour with little regard for the side effects. This has meant that the quality of life for those who haven't long to live has been ignored, as have the long-term side effects for those who survive. We need a better understanding of the broad effects of the different tumours and their treatments to be able to make improvements in treatments and management of care.

Ensuring equal treatment and care for all

We want every person affected by a brain tumour to have access to the very best care and treatments available. Unfortunately, there are significant variations across the country. For example, half of Clinical Commissioning Groups in England do not allow GPs direct access to scans to detect brain cancer³⁰, meaning that patient experience and access to treatments varies depending on where you live. We want to change this.

62%

of children who survive a brain tumour will be left with a life-altering, long term disability²⁷

49%

of high grade glioma patients suffer from neurocognitive deficits²⁸

²⁷ Macedoni-Luksic, M, Jereb, B, and Todorovski, L. Long-term sequelae in children treated for brain tumors: impairments, disability, and handicap. *Pediatr Hematol Oncol*. 2003; 20: 89–101

²⁸ Quality of life in adults with brain tumors: Current knowledge and future directions. *Neuro Oncol* (June 2009) 11 (3): 330-339

²⁹ www.braintrust.org.uk/news-detail.php?id=362&year=201312

³⁰ GP magazine, 21 November 2014, report on responses from 182 Clinical Commission Groups to a GP freedom of information request.

WE WILL:

- Invest in research on approaches such as epilepsy and fatigue management to enhance the quality of life of those affected by brain tumours.
- Invest in a longitudinal study to collect data on the long-term and late effects of a brain tumour, to ensure that future treatment decision making helps to improve quality of life.
- Identify and analyse long-term survivors to see what we can learn from the biology of their tumours and response to treatment as well as other environmental impacts, such as diet and lifestyle.
- Invest in pilot studies designed to transform the NHS experience through testing different models of service delivery.

WE WILL CALL ON:

- Researchers to take toxicity and effects on quality of life into account when new treatments are tested rather than just focusing on survival.
- Regulators and the NHS to take the impact on quality of life into account when considering the value of adopting new treatments/drugs.
- Other funders and coalitions that work in similar areas, to seek co-funding opportunities and influence further funding.
- NICE to appraise the evidence we develop and update national guidelines to improve the NHS standards of care for people with brain tumours.



“LIFE IS STILL TO BE ENJOYED, EVEN IF LIFE IS CHALLENGING AND A LOVED ONE IS UNWELL”

Sara and Neal's story

Neal was diagnosed with a glioblastoma in 2002. Six months later he was given the all clear. Whilst in remission a year later, Neal and Sara got engaged but shortly after discovered the tumour had come back. They brought forward their wedding after being told that he may only have a short time to live.

Neal had five years of chemotherapy and six weeks of radiotherapy and his new wife Sara became his carer. At first her caring role included driving Neal to the hospital each week to have chemotherapy, being with him to see the doctor and have his treatment and then driving him home and making sure he was okay.

Sara says: “As you can imagine, my caring role has changed significantly and life has become increasingly difficult, not only physically but emotionally. Watching my loved one suffer and his quality of life deplete has been unbearable, and caring for Neal has been the hardest thing I have done and will probably ever do.”

Neal has had a number of brain tumours, affecting his balance and speech. He had a stroke over two years ago which means he is now nursed at home requiring round the clock care. Although they make the most of every opportunity and have some carers who come to help, his quality of life is poor. Neal now lives in a hospital bed in the front room and is hoisted for a few hours a day into a wheelchair. Apart from a few words a week, he's sadly unable to speak as his swallowing and speaking have also deteriorated.

“I noticed I had stopped doing the things I enjoy, mainly because of lack of energy and feeling constantly drained. For 12 years I have been running twice a week and really enjoy it but I had become too tired to exercise. I had also stopped going out and seeing friends like I used to. Today I give myself time and space to do the things I enjoy. It's not always easy! But in doing so, I am happier and stronger and am able to keep caring for Neal and lift his spirits. It's easy to forget why you are here and that life is still to be enjoyed, even if life is challenging and a loved one is unwell.”



EVALUATING OUR IMPACT

It is of paramount importance that we regularly monitor the progress, outcomes and impact of the research we fund and provide evidence of how the funds raised by our supporters are helping to defeat brain tumours.

We require grantholders to submit annual progress reports and we only continue to support the project if satisfactory progress has been made. Where required, we seek advice from our expert Grant Review and Monitoring Committee (GRAM) and Trustees.

We track and evaluate the outcomes of the research we fund using an online research outcomes and impact system called ResearchFish. Through this system our researchers report their outputs to us, from the discovery of new drugs and scientific publications to health policy change and infrastructure development. This helps us capture any outputs arising from our research even after projects have finished and truly evaluate the impact they've had in defeating brain tumours and the role they have had in advancing the field.

Our research strategy will also be evaluated against our two key goals, doubling survival and halving the harm, which is reliant on data collection by third party agencies.



TIMESCALES

We will start the implementation of 'A Cure Can't Wait' in December 2014 which will represent an investment of a minimum of £20million.

The initial calls for research applications will be launched as follows:

December 2014	Adult brain tumour diagnostic pathway call
January 2015	Biobanking call
February 2015	Paediatric initiative
March 2015	Collaborative initiative on tumour biology Feasibility study project grants
June 2015	Quality of life initiative
September 2015	Translation Innovation Fund

All of our research calls are open, fair and transparent and subject to international peer review, followed by a review by our independent Grant Review and Monitoring Committee before final decisions are made by our Board of Trustees.

Further details on the scope and remit of these schemes along with other future calls will be made available from January 2015.



About us

The Brain Tumour Charity is at the forefront of the fight to defeat brain tumours and is the only national charity making a difference every day to the lives of people with a brain tumour and their families.

We fund pioneering research to increase survival and improve treatment options. We raise awareness of the symptoms and effects of brain tumours to get earlier diagnosis and to help families cope with everything that the diagnosis of a brain tumour brings. We provide support for everyone affected so that they can live as full a life as possible, with the best quality of life.

We are committed to having the biggest possible impact for every person affected by a brain tumour, to defending the most amazing part of the human body, so that getting the diagnosis of a brain tumour no longer means a death sentence.

We believe that fighting brain tumours on all fronts through research, awareness and support is the only way to save lives, reduce long-term disabilities and help everyone with a brain tumour and their families.

We are members of the Association of Medical Research Charities, The Information Standard, The Helplines Partnership and the Fundraising Standards Board.

Acknowledgements

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We would also like to thank everyone who supports our research portfolio and processes, including our Research Sub-Committee of Trustees, our Grant Review and Monitoring Committee and our international peer reviewers.



THANK YOU

We are 100% reliant on voluntary donations to fund our work. We thank every Supporter Group, fundraiser, donor, corporate partner and trust for their ongoing commitment to help us find a cure.



“BRING IT ON!”

Bhavna's story

I was diagnosed with a glioblastoma in September 2013. Since receiving the devastating news of my diagnosis, my attitude has been 'bring it on'. I still think the same way now – which has been helped by going to the gym and boxing twice a week!

Early in my treatment programme, I was advised by my clinical nurse specialist and oncologist to make sure I had access to the best support and information services by contacting The Brain Tumour Charity. In November 2014 I went to their London Information Day, which taught me so much and left me feeling more able to ask questions and take control.

When my hair loss started due to treatment I was very lucky that I didn't lose much, but I still wanted to cover my head. I tried a wig, which I didn't like, and various scarves. I thought it was really cool to be able to wear The Brain Tumour Charity's bandana on Bandanas for Brain Tumours Day. The company I worked for, HC-One care homes, got involved too. Staff and residents all joined in and we raised over £3,500.

Raising money for research is crucial as it's so underfunded. Brain tumours are not as well-known as other cancers and they don't get talked about as much. In fact, I think a lot of people don't even realise children can get brain tumours. We have to do everything we can to fund research as it's the only way we can find a cure.

Taking part in The Twilight Walk Windsor in the pouring rain with my family showed me the power of the united brain tumour community. The next five years will bring challenges but also incredible opportunities if we're ready to stand together and take them. Bring it on!

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