Clinical trials

The purpose of clinical trials for brain tumour patients is to advance understanding of tumours and to improve diagnosis and treatment. Some trials also seek to better control symptoms and improve quality of life for those living with a tumour. By their definition, clinical trials are experimental, and while the hope is
that they will be beneficial, there is
no guarantee.

The Brain Tumour Charity is the largest dedicated funder of brain tumour clinical trials and the only brain tumour charity to have AMRC (Association of Medical Research Charities) accreditation for its research peer review process.

# In this fact sheet:

* What is a clinical trial?
* Developing a new treatment
* Answers to some common questions that you may have about
clinical trials

## What is a clinical trial?

A clinical trial is an experiment that involves patients in a new way of managing a condition. This might include investigating a new treatment or a new way of giving an existing treatment, or a new approach to diagnosing an illness or assessing an outcome after treatment. Trials are vital to establish whether a new approach is better than the old one; if it isn’t, there is little point in persisting with it.

## Developing a new treatment

There are normally four phases to developing a new treatment. Clinical trials can therefore be a lengthy process. The phases are as follows:

### Phase 1

Phase 1 sets out to answer the question of whether the treatment is safe and, if so, what is the right dose regime to use. Drugs are tested in the laboratory before being given to people. Usually, but not always, they are tested on animals. If it seems that they could help people with brain tumours, a ‘phase 1’ (safety) study must be done. Often a very low dosage is given to the first few patients. If there are no side effects amongst this group, the next group of patients is given a slightly higher dose, and so on until side effects are experienced that indicate it would be unsafe to increase the dosage any further. This is known as the ‘maximum tolerated dose’ or MTD.

The MTD helps determine the dose for the next studies. Sometimes a researcher is happy to stop before a MTD is reached, and sometimes a drug is considered to be safe enough to progress with the study at, or near, the maximum treatment dose. Phase 1 trials tend to be carried out in specialised clinical research units rather than local hospitals and can last several months. Patients will receive optimum care and attention while in the unit. If the treatment is safe, it will be progressed to phase 2.

### Phase 2

Phase 1 tells the clinical researchers what dose of the new treatment should be given. In phase 2, the aim is to find out whether the new treatment does what is hoped:

* Is it any good?
* Does it shrink the tumour? (known as ‘response’ to the treatment)
* Does it keep the tumour away for longer? (known as ‘progression
free survival’).
* Does it make the patient feel better?

This phase uses a larger group of patients and can last for a couple of years. If the technique looks promising after phase 2 then it will proceed to phase 3.

### Phase 3

Phase 3 looks at whether the new treatment works better than the existing, ‘standard’ treatment or, sometimes, whether it produces fewer side effects. This is done by comparing two groups of patients with similar characteristics. Some of the patients receive the standard treatment and some receive the new treatment. The outcome of the two groups is compared to see whether the new treatment is better.

Which treatment (standard or new) patients receive is often decided on a random basis. That is, the treatment is allocated by chance – like tossing a coin. Neither the researcher nor the patient can influence this decision. It is the most successful way of ensuring that the results of the trial are not biased and a true comparison has been done.

In addition, a technique called ‘blinding’ may be used. In a ‘single’ blind trial the researcher, but not the patient, will know which treatment they are receiving. In a ‘double blind’ trial neither the patient nor the researcher knows the treatment allocation. Again, this technique improves the validity of the trial, for example, any effects seen can be more confidently attributed to the treatments themselves and not to other factors such as placebo effects. A placebo is a harmless, ‘dummy drug’ that is used to assist blinding so that those involved do not know whether they are receiving the experimental
drug or not.

### Phase 4

Phase 4 trials are conducted when a drug has been shown to be effective and has been licensed to treat an illness. This phase aims to find out what happens when the drug is given to thousands of people in the general community. The aim is to assess any long-term risks and benefits of the drug, and any rare side effects.

## How do I find out about clinical trials?

Every trial has a set of ‘entry criteria’ that you must fit to be able to enter. By far the best way is to speak to your clinician about trials that may be suitable for you. You can call our Information and Support Team on 0808 800 0004 or email info@thebraintumourcharity.org

The following websites list current clinical trials:

* brainstrust’s ‘Brain tumour hub’: [braintumourhub.org.uk](http://www.braintumourhub.org.uk/)
* Cancer Research UK: [cancerhelp.cancerresearchuk.org/trials/trials-search](http://cancerhelp.cancerresearchuk.org/trials/trials-search)
* National Cancer Research Network: [ncrn.co.uk](http://www.ncrn.co.uk/)

## How am I selected for a clinical trial?

Some entry criteria will be specified before you enter; others will not and require trial-specific tests, which can only be carried out once you have agreed in principle to enter the trial.

## What happens if I agree in principle to enter a clinical trial but then don't meet all of the criteria?

You will not be allowed to enter a clinical trial unless you meet all of the criteria. If you cannot enter the trial, your doctor will talk through any alternative treatments available to you or suitable clinical trials.

## What happens if I agree to enter a trial but then change my mind?

You are free to leave a trial at any time without obligation to explain why.

## How long do I stay on the trial?

The trial will go on until either of the following:

* The trial comes to an end (as defined it its ‘protocol’, a document detailing the design and implementation of the trial).
* In phase 1, when the maximum tolerated dose is reached.
* If the treatment is clearly failing, the trial will be stopped.
* If your doctors believe it is in your best interest to take you off the trial, they will do so as they have a duty of care to you.
* You decide to withdraw. It is your right to leave the trial at any time you wish without obligation to give a reason.

## How can I get onto a trial if my hospital does not offer it?

This may not be easy. Individuals in clinical trials often require close surveillance, which means having easy access to the trial site. It can mean travelling and staying near the site. Sometimes a trial is geared to taking patients from other regions. The best thing is to discuss your wishes with your doctors to see if particular arrangements can be made.

## Will I need to fund travel costs myself?

Travelling backwards and forwards to the hospital can be time consuming and costly. You may wish to ask your doctor about the expected number and frequency of visits before you agree to enter. This said, you should be able to claim travel costs. Whilst it would be unusual to remunerate patients for taking part in oncology trials, it is also very unusual to expect you to be out of pocket with respect to travel costs.

## What are the benefits of taking part?

The clinical trial is running because there is belief that the new treatment may be better than the guarantee that it is. If the trial treatment is an improvement you may be one of the first patients to benefit from it. In addition, some patients report that they are pleased to be helping advance science, even if they do not benefit directly. Without trialling a new treatment, no further progress could ever be made.

An indirect consequence is that whilst you are taking part in a trial, you are often even more carefully monitored. This means that any changes to your health – even if they are not related to the new drug, might be picked up and dealt with quickly. On the other hand it must be remembered that new treatments may carry additional risks and trial participation can be inconvenient and time consuming. All this must be weighed up before agreeing to take part.

## What are the risks of taking part?

The clinical trial is going ahead because the researchers have good reason to believe it may be better than the standard treatment. Nonetheless, clinical trials are experimental by nature and there is a chance that the new drug will be no better, or not as good even, as the standard drug. Unexpected side effects are also a possibility, although the researchers will monitor you closely whilst you are in the trial and make every effort to keep these to a minimum.

## What reassurance can you give me that it's safe for me to enter a clinical trial?

The UK exercises some of the most rigorous patient protection practices in the world, including (but not limited to):

* **Medicines and Healthcare Products Regulatory Agency (MHRA)**
A body that ensures the trial products meet international standards of good practice.
* **Research Ethics Committee**
A board that ensures patients’ well-being and rights are maintained. They also ensure that information given to patients tells them everything they need to know and is easy to understand.
* **Clinical trial committees**
All trials are scrutinised at national or local level (often both) to ensure their design and implementation are appropriate and scientifically sound.

# What if I have further questions?

If you require further information, any clarification of information, or wish to discuss any concerns, please contact our Support and Information Team.

* Call 0808 800 0004 (free from landlines and most mobiles including 3, O2, Orange, T-mobile, EE, Virgin and Vodafone)
* Email support@thebraintumourcharity.org
* Join our online forums at [www.thebraintumourcharity.org/forums](http://www.thebraintumourcharity.org/forums)

# About us

The Brain Tumour Charity makes every effort to ensure that we provide accurate, up-to-date and unbiased facts about brain tumours. We hope that these will add to the medical advice you have already been given.

Please do continue to talk to your doctor if you are worried about any medical issues. We are the UK’s leading brain tumour charity. We fund scientific and clinical research into brain tumours and offer information and support to those affected, whilst raising awareness and influencing policy.

We rely 100% on charitable donations to fund our vital work. If you would
like to make a donation, or want to find out about other ways to support us including fundraising, leaving a gift in your will or giving in memory, please visit us at [www.thebraintumourcharity.org](http://www.thebraintumourcharity.org) or call 01252 749043.

# About this fact sheet

This fact sheet has been written and edited by The Brain Tumour Charity’s Support and Information Team. The accuracy of medical information has been verified by a leading neuro-oncologist. Our fact sheets have been produced with the assistance of patient and carer representatives and up-to-date, reliable sources of evidence. If you would like a list of references for any of the fact sheets, or would like more information about how we produce them, please contact us.

# Clinical trials

# Your notes



Hartshead House

61-65 Victoria Road

Farnborough

Hampshire

GU14 7PA

01252 749990

enquiries@thebraintumourcharity.org

<www.thebraintumourcharity.org>

© The Brain Tumour Charity 2014. Registered Charity Number 1150054 (England and Wales) and SC042096 (Scotland).

Version 1 (clear print), first produced in standard print format April 2012. Review date, by April 2014.