**Norfolk and Waveney Clinical Policy Development Group**

**Experimental and Unproven Treatments Policy**

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**Equality Statement**

The Norfolk & Waveney Integrated Care Board (Norfolk & Waveney ICB) and the Clinical Policy Development Group (CPDG) are committed to ensuring equality of access and non-discrimination as enshrined in the Health and Social Care act 2012. In carrying out its functions, the CPDG will have due regard to the different needs of protected equality groups, in line with the Equality Act 2010. This document is compliant with the NHS Constitution and the Human Rights Act 1998.

**Clinical Governance Statement**

It is important that the implementation of this policy is seen as an opportunity to encourage team working and cooperation between commissioners, primary and secondary care providers. Service Providers will be expected to collect and provide audit data on request as part of a professionally-led clinical review and audit cycle.

**Exceptionality**

For patients not meeting the policy criteria or where a treatment is not routinely funded, an application should be made to the Individual Funding Request (IFR) panel if the referrer considers that there are clinically exceptional circumstances. IFR policy and procedure documents can be found on Knowledge Anglia.

**Scope of Policy**

This policy applies to any patient for whom Norfolk & Waveney ICB is the responsible commissioner.

This policy deals with the funding of experimental treatments only. It does not cover primary research into novel treatments.

Treatments which are judged to be experimental or not to be of proven effectiveness will not be routinely funded.

1. **What is an Experimental Treatment**

Those funding health services seek to provide as comprehensive a healthcare service as possible across all patient groups and across the entire patient pathway, within an overriding legal obligation to stay within the financial budget allocated to them. Given that demand for healthcare will always exceed the resources available to fund treatment, it is justifiable to give the funding of experimental treatments a lower priority than funding the provision of core services and treatments of proven benefit.

Criteria for considering a treatment as experimental include:

* The treatment is still undergoing clinical trials for the indication in question.
* There are no relevant articles published in the peer-reviewed journals available on the treatment for the indication in question.
* The treatment does not have approval from the relevant government body.
* The treatment does not conform to usual clinical practice in the view of the majority of medical practitioners in the relevant field.
* The treatment is being used in a way other than that previously studied or that for which it has been granted approval by the relevant government body.
* The treatment is rarely used, novel, or unknown and there is a lack of authoritative evidence of safety and efficacy.

From the position of those funding healthcare, two other criteria can be added:

* The evidence is not yet available for public scrutiny.
* The decision maker does not have confidence in the evidence base that has been presented (which refers to the interpretation of the evidence).

1. **Excess Treatment Costs (ETCs) in research**

Funding of Excess Treatment Costs in research for National Institute for Health Research (NIHR) supported studies is managed via a national management model for England introduced by NHS England in 2018. Funding of ETCs for studies eligible for payment through this process fall outside the scope of this policy. Further information can be found at the dedicated National Institute for Health Research page: <https://www.nihr.ac.uk/researchers/collaborations-services-and-support-for-your-research/run-your-study/excess-treatment-costs.htm> .

ETCs for studies that are not NIHR supported or fall outside the scope of the national model for England will not be routinely funded by Norfolk & Waveney ICB. When there is good reason for considering requests, these will be managed through the individual funding request policy and process. (See 4.2 below)

1. **Commercially funded trials**

Norfolk & Waveney ICB position is that where a clinical trial of a treatment has been initiated and sponsored by a manufacturer of pharmaceuticals or medical devices, or by some other commercial organisation, responsibility for funding on-going access to the treatment rests with those parties.

1. **Non-commercially funded trials**

**4.1 Ongoing access to treatment following a clinical trial**

Norfolk & Waveney ICB will consider funding on-going access to the treatment given in a trial in circumstances where;

* The clinical trial is to be wholly funded by non-commercial bodies and is supported by the NIHR;
* and the request is made, and written agreement reached **before** the clinical trial commences.

Treatment will be funded only for as long as the patient’s supervising clinician agrees that the treatment is clinically appropriate, and that the treatment is meeting the identified clinical outcomes.

Norfolk & Waveney ICB expects that all research organisations planning a trial;

* define and agree the arrangements for funding the treatment after the end of the trial for those patients where the trial has shown a clinical benefit. This is in line with the ethical approval requirements of the Health Research Authority (HRA) for clinical trials <https://www.hra.nhs.uk/>
* Ensure patients participating in a trial are made fully aware of the arrangements for when the trial concludes as part of the process of giving their consent to participate in the trial. This includes making patients aware of whether or in what circumstances they can expect to continue to receive treatment after the end of the trial, in line with the HRA guidance <http://www.hra-decisiontools.org.uk/consent/content-sheet-involved.html> on the information to be provided to participants taking part in a research.

Where commissioning responsibility for a patient on a clinical trial transfers to Norfolk & Waveney ICB from another NHS commissioner, and there is written evidence of an agreement to fund on-going treatment costs (after completion of the trial) by the previous NHS commissioner, Norfolk & Waveney ICB will fund those commitments made by the patient’s previous NHS commissioner. This only applies to non-commercial trials supported by the NIHR.

The provider of the trial treatment and the clinician should take care to ensure that participants in a trial do not assume that Norfolk & Waveney ICB will or might fund ongoing treatment once the trial has completed, unless Norfolk & Waveney ICB has given a prior written commitment to provide such funding which would apply to that participant

All requests for on-going funding following a clinical trial shall be made via the IFR process **before** the trial commences.

**4.2 Funding Excess Treatment Costs (ETCs) for studies that are not NIHR supported or fall outside the scope of the national model for England**

NHS Treatment Costs, including Excess Treatment Costs are the responsibility of commissioners and are expected to be met through the normal commissioning process [(Attributing the cost of health and social care Research & Development (AcoRD), DHSC, 2012)](https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/351182/AcoRD_Guidance_for_publication_May_2012.pdf). In practice, for NIHR supported research, this process is managed through the NHS England national management model.

Given a high proportion of research in the UK is supported via the NIHR and will be eligible for payment of ETCs through the national system, requests to fund ETC that fall outside the scope of this system not expected to commonly arise.

When determining whether to fund ETCs it is important to establish what the status of a trial is, who has sponsored it and which bodies contribute to funding the trial.

Those commissioning health care may be asked to explicitly fund trials in two ways:

1. A request to support a trial by funding a number of patients or any qualifying patient to enter the trial. In these instances, the request should be treated as a service development. If it is a very large trial with considerable budgetary consequence it is more likely that prioritisation should be through the annual commissioning process.

*A request for a treatment should be classified as a request for a service development if there are likely to be a cohort of similar patients who are:*

* *In the same or similar clinical circumstances as the requesting patient whose clinical condition means that they could make a like request (regardless as to whether such a request has been made)*

*AND*

* *Who could reasonably be expected to benefit from the requested treatment to the same or a similar degree.*

1. A request to support a single patient to enter a trial. This request should be managed under the organisation’s individual funding request policy and process.

The most common situation in which commissioners find themselves is as the recipients of requests to fund on-going treatment once the trial has ended. This is addressed in 4.1 above

**4.3 Assessing Requests to fund**

Requests to fund ongoing treatment following a clinical trial, or to fund Excess Treatment Costs for studies that fall outside of the scope for payment through the national system will be assessed against the following criteria:

* The potential strategic importance of the treatment. This requires a judgment to be made on whether the trial will address key national priorities for the health issues for a particular patient group or programme area (e.g., cancer, cardiovascular disease).
* The status of the clinical trial including whether or not the trial is supported by the NIHR and other relevant professional and research bodies.
* The quality of the trial and whether or not it is reasonably expected to generate the sort of information needed to enable those funding healthcare to reach a view on the clinical effectiveness and cost effectiveness of the treatment. Specialist advice may need to be sought on the methodology to be adopted within any trial.
* Ownership of the data. Trials which do not guarantee that the data will be made available to public authorities and research communities for independent evaluation will not be considered for funding
* Affordability and priority when compared to competing unmet needs

In all circumstances where funding is granted, the Provider must keep a record of acceptance to ensure pick-up funding is honoured, either for an individual patient or for the trial.

**5.** **Use of an existing treatment experimentally for rare clinical circumstances (outside the context of a clinical trial)**

Norfolk & Waveney ICB will give consideration to supporting an existing treatment in an experimental context for rare clinical situations provided that the clinician making the application is able to demonstrate that running a good quality clinical trial for the treatment in the clinical situation in question is impossible.

It is important for decision-makers to distinguish between those instances where trials are either impossible or improbable and those where the research community and industry have not prioritised a trial.

This type of request will be considered under Norfolk & Waveney ICB individual funding request policy and process.

In assessing these cases Norfolk & Waveney ICB will make a decision having regard to the following factors:

* the biological plausibility of benefit based on other evidence
* the potential benefit and risks of the treatment
* an estimate of cost of the treatment and the anticipated value for money
* the priority of the patient’s needs compared to other competing needs and unfunded developments.

The clinician will be expected to provide as much information as possible about the treatment, relevant research upon which the claim for biological plausibility of the treatment is based, and costs, as well as clinically relevant information on the patient. In addition, the clinician will identify the clinical markers and clinical outcomes that will be monitored to assess treatment response.

The options for consideration by Norfolk & Waveney ICB in these instances are, in principle;

* Not to Fund
* Fund on the condition that the patient enters a properly conducted *‘n of 1’* trial. In practice this would be challenging due to the need for expert advice and support on design, set-up, management and analysis of such a trial, as well as additional funding considerations.
* Fund a trial of treatment but make ongoing treatment subject to the demonstration of clinical benefit for the individual patient using criteria agreed in advance with the clinical team.
* Fund with no evaluation requirements, although an outcomes report should be requested from the clinician

In all instances, contribution to any relevant clinical database or population registry which is operating will be an additional condition before Norfolk & Waveney ICB gives approval to funding for the treatment

**6.** **Funding potentially important treatments but where there is minimal evidence and / or concerns remain about the value of the treatment**

**6.1 Treatments for which there is minimal evidence of effectiveness and no current research options**

Very rarely those funding healthcare services may consider an experimental treatment so important that they wish to see a publicly funded trial established. In the first instance, advice should be sought from the National Institute of Health Research (<https://www.nihr.ac.uk/partners-and-industry/charities/identify-research-needs.htm>) whereby topics of interest for research can be raised. Norfolk & Waveney ICB may however, consider initiating (and possibly funding) the whole trial themselves. Expert advice on design and development of trials, and applying for funding will, in this instance need to be sought.

**6.2 Treatments for which there are adequate trials, and which have demonstrated effectiveness but for which concerns remain over the true value of the treatment**

It is possible to have a situation where a treatment is supported by reasonably good trials, but important questions remain about the treatment and how best to implement. In these instances, the requirement for ongoing evaluation is legitimate.

Issues that might result in Norfolk & Waveney ICB feeling that a treatment should only be made available if there is ongoing evaluation include but are not limited to:

* Where concerns remain about the nature of the benefit and/or risks
* Where a treatment’s true place in management has yet to be established
* Where there is potential for significant variation in clinical practice (which might otherwise be difficult to control).
* Where it is not known how best to deliver the treatment (e.g. dose, frequency, sequencing, concurrent treatment, duration of treatment)
* Where there is a good chance that real-life effects and/or costs may differ from those seen in clinical trials because of difference in context, patient mix, treatment delivery, service provision etc

Decision-makers should, therefore, be able to apply conditions when funding treatments in this category.

**7. GLOSSARY**

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| **TERM** | **DEFINITION** |
| **Annual commissioning round** | The *annual commissioning round* is the process by which major funding decisions are taken, including the allocation of new money coming into the NHS. This involves a complex process of prioritisation which involves a series of decisions. This process occurs during the months of October to March for the following financial year. |
| **Clinical Effectiveness** | C*linical effectiveness* is a measure of how well a healthcare intervention achieves the pre-defined clinical outcomes of interest in a real life population under real life conditions. |
| **Clinical Trial** | *A clinical trial* is any research study that prospectively assigns human participants or groups of humans to one or more health-related interventions to evaluate the effects on health outcomes. Interventions include but are not restricted to drugs, cells and other biological products, surgical procedures, radiological procedures, devices, behavioral treatments, process-of-care changes, preventive care, etc*.*  The ethical framework for conducting trials of medicinal products is set out in the Medicines for Human Use (Clinical Trials) Regulations 2004 (as amended). It includes, but does not refer exclusively to, randomised control trials. All research in the NHS, including Clinical Trials must adhere to the UK Policy Framework for Health and Social Care Research, 2017 DHSC <https://www.hra.nhs.uk/planning-and-improving-research/policies-standards-legislation/uk-policy-framework-health-social-care-research/> |
| **Cost Effectiveness** | *Cost effectiveness analysis* is a method for assessing or measuring the reasonably anticipated benefits and clinical effectiveness of a particular expenditure. In the health setting this will be the cost of a particular healthcare intervention together with any other costs of delivering the healthcare intervention. Cost effectiveness analysis requires an examination of expenditure to determine whether the money spent could have been used more effectively (and ideally - whether the resulting benefits could have been attained through less financial outlay). |
| **Effectiveness - General** | *Effectiveness* means the degree to which pre-defined objectives are achieved and the extent to which targeted problems are resolved. |
| **Effectiveness - Clinical** | C*linical effectiveness* is a measure of the extent to which a treatment achieves pre- defined clinical outcomes in a target patient population. |
| **Efficacious** | A treatment is *efficacious* where it has been shown to have an effect in a carefully controlled and optimal environment. However, it is not always possible to have confidence that data from trials which suggest that treatments will be efficacious will translate into clinically meaningful health gain and more specifically the health gain of interest. This is the difference between disease-oriented outcomes and patient oriented outcomes. For example, a treatment might have demonstrated a change in some physiological factor which is used as a proxy measure for increased life expectancy but this relationship might not be borne out in reality. |
| **Experimental and unproven treatments** | *Experimental and unproven treatments* are medical treatments or proposed treatments where there is no established body of evidence to show that the treatments are clinically effective. The reasons may include the following:   * The treatment is still undergoing clinical trials for the indication in question. * The evidence is not available for public scrutiny. * The treatment does not have approval from the relevant government body. * The treatment does not conform to an established clinical practice in the view of the majority of medical practitioners in the relevant field. * The treatment is being used in a way other than that previously studied or for which it has been granted approval by the relevant government body. * The treatment is rarely used, novel, or unknown and there is a lack of evidence of safety and efficacy. * There is some evidence to support a case for clinical effectiveness but the overall quantity and quality of that evidence is such that Norfolk & Waveney ICB does not have confidence in the evidence base and/or there is too great a measure of uncertainty over whether the claims made for a treatment can be justified. |
| **Healthcare intervention** | A *healthcare intervention* means any form of healthcare treatment which is applied to meet a healthcare need. |
| **NHS commissioned care** | *NHS commissioned care* is healthcare which is routinely funded by the patient’s responsible commissioner. Norfolk & Waveney ICB has policies which define the elements of healthcare it is and is not prepared to commission for defined groups of patients. |
| **NICE** | National Institution for Health Care and Excellence |
| **NICE’s Guidance on Interventional Procedures** | *NICE’s Guidance on Interventional Procedures* are a form of NHS Guidance. They aim to provide information about the safety of new interventional procedures. They are not covered by NHS Directions. |
| **Priority setting** | *Priority setting* is the task of determining the priority to be assigned to a service, a service development, a policy variation or an individual patient at a given point in time. Prioritisation is needed because the need and demands for healthcare are greater than the resources available. |
| **Service Development** | A *Service Development* is a proposal to Norfolk & Waveney ICB to provide a particular healthcare intervention to be routinely funded by the ICB for a defined group of patients.  A service development is any aspect of healthcare which the Norfolk & Waveney ICB has not historically agreed to fund, and which will require additional and predictable recurrent funding.  It also encompasses other types of investment that existing services might need, such as pump-priming to establish new models of care, training to meet anticipated manpower shortages and implementing legal reforms. Equitable priority setting dictates that potential service developments should be assessed and prioritised against each other within the annual commissioning round.  However, where investment is made outside of the annual commissioning round, such investment is referred to as an *in-year service development*.  An *in-year service development* is any aspect of healthcare, other than one which is the subject of a successful individual funding request, which Norfolk & Waveney ICB agrees to fund outside of the annual commissioning round. Unplanned investment decisions should only be made in exceptional circumstances because, unless they can be funded through disinvestment, they will have to be funded as a result of either delaying or aborting other planned developments |
| **Statutory Guidance** | *Statutory Guidance* is written Guidance which is issued by the Secretary of State or a body authorised by the Secretary of State (or by another part of government which is directly relevant for the relevant decision-making process). NHS bodies are required to have regard to statutory guidance in their decision making. Statutory Guidance is intended to assist public authorities in the exercise of their statutory duties. It suggests steps which might be taken; factors which could be taken into account and procedures which could be followed to deliver specified steps of administration, or policy delivery. NHS bodies are entitled to depart from statutory guidance if they have a good reason to do so. However:   * **The NHS body should always record that it has considered the statutory guidance as part of its decision making processes, and**   The NHS body should always record the reason or reasons why it has departed from the course of action recommended in the Guidance. |
| **Treatment** | *Treatment* means any form of healthcare intervention which has been proposed by a clinician and is proposed to be administered as part of NHS commissioned and funded healthcare |
| **Treatment – Costs** | *Treatment costs*, in the context of clinical trials, are the patient care costs which would continue to be incurred by the NHS if the service in question continued to be provided after the clinical trial had ceased. |
| **Treatment Costs – Excess** | *Excess treatment costs* are incurred where patient care is provided which differs from the standard treatment, in that it is either an experimental treatment or a service in a different location from where it would normally be delivered. The difference between the total Treatment Costs and the cost of the standard treatment (if any) constitutes the *excess treatment costs* |
| **Trial of Treatment** | A t*rial of treatment* refers to a situation where a clinician has exposed a patient to a treatment for the purpose of assessing whether or not the patient is likely to benefit from longer term treatment |

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Collaborative Commissioning Policy: On-going access to treatment following the completion of a trial explicitly funded by the Clinical Commissioning Group.

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