

ASSESSING COMPLEMENTARY PRACTICE

Building consensus on appropriate research methods
Report of an independent advisory group

Chair: Professor Dame Carol Black

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Foreword

This report is the work of an independent advisory group that I had the pleasure to chair. It draws on a two-day participative conference that explored the challenges of assessing complementary practice.

During our work we reflected on the experience and the informed contributions of a wide range of academics, researchers, practitioners and funders. Our aim was to consider potential areas for consensus to guide research funders, researchers, commissioners and complementary practitioners in the task of developing and applying a robust evidence base for complementary practice.

There are four characteristics that distinguished our approach and that underpin both our analysis and our conclusions.

- We debated the relative value of different types of evidence and different methods used to generate that evidence. I hope this is apparent in our analysis.
- We acknowledged and sought to understand the technical problems in designing, implementing and interpreting research in this field. We believe that these challenges can and should be overcome.
- We engaged in the question of identifying not only the resources necessary to move forward but also the need to foster an appropriate attitude to this important task – one that is inquiring, open-minded and patient-centred.
- We put the concerns and interests of the public and the commissioners of NHS health care at the forefront of our work. We suggest that those who fund and direct research into complementary practice should also show that they have taken the views of these users of research into account.

It is our belief that the specific difficulties we grappled with reflect a more generic challenge: the challenge to use a scientific approach to understand and test those things that we, as members of the public, do to manage our health.

I hope our thinking on the specific problems of assessing complementary practice will be seen as a contribution to a wider debate – namely, how can the application of science improve our understanding of how public and health care professionals can support overall health and well-being?

When it comes to improving health and health care, politicians, health care professionals and policy-makers are increasingly recognising the need to support people in the management of long-term conditions, and the need to understand the complexities of developing and sustaining well-being as well as managing ill health.

To gain that understanding we will need to look at how we apply the scientific method to generate more rigorous knowledge of the impact of different behaviours and interventions that are embedded in the complexity of our lives, and indeed draw some of their effectiveness from the context in which they are adopted or used.

We need to match our resources and develop our methods to fit the task of testing and understanding the ways in which we sustain as well as damage our health. Part of that task is to understand more fully the therapies and complementary practices that are so commonly used. This may require new methods. It will certainly require much collaborative effort.

It is a task that researchers, funders and practitioners should feel obliged to undertake and a task that offers significant learning not only for the future of complementary practice but for our understanding about how to improve our ability to manage and sustain health and well-being.

Professor Dame Carol Black

Acknowledgements

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Summary

This report considers the difficulties of assessing the effectiveness of complementary practice. Despite the increasing popularity and use of complementary practice, the evidence base is small. Lack of research and lack of agreement on research methods has led to criticism from branches of conventional medicine.

This report sets out a way forward, suggesting how research might usefully be approached to increase the evidence base and to give complementary practitioners, commissioners and members of the public a sound basis on which to make decisions about treatment. It considers the cultural, financial and methodological challenges.

The report is based on the work of an advisory group set up by The King's Fund; the group deliberated during 2008, drawing on a conference held in 2007.

Because of its inherent characteristics, complementary practice presents researchers with a unique set of challenges:

- how to take account of the context in which the intervention is made
- how to reflect the importance attached to treating the whole person
- how to accommodate the fact that the relationship between cause and effect may not be straightforward
- how to understand 'placebo' or non-specific effects.

In order for these challenges to be met and overcome, complementary therapy practitioners need to accept the vital role that evidence plays in developing practice. There is a need for both conventional and complementary practitioners to engage in rational debate about the strengths as well as the weaknesses of that evidence.

In endeavouring to find a consensus on which to build, this report analyses contributions made to the debate under the following headings.

- Is there agreement on the need for a robust evidence base?
- What are the distinctive difficulties of research into complementary practice?
- What does it take to build confidence in a particular complementary practice?
- How can research into complementary approaches identify what works in practice?
- How should complementary practice research think about the placebo effect?
- How might research into complementary practice consider cost-effectiveness?
- How might the necessary research capacity be developed and supported?

This in turn gives rise to consideration of three broader questions and their corresponding answers.

If we know something works, do we need to know how it works?

We believe there needs to be investment in research to understand how any complementary practice works. Through such research an understanding can develop across practitioners, researchers and patients on likely and plausible cause-and-effect mechanisms triggered by the intervention. However, this research should not eclipse the need to also use robust methods to explore the clinical and cost effectiveness of specific complementary practices.

Do we need to know exactly what is working?

Rigorously controlled studies are needed to establish the clinical and cost effectiveness of an intervention but these do not need to be placebo controlled as long as they control for the possibility of the patient naturally getting better over time. Many of the non-specific, placebo or contextual effects could well be considered legitimate aspects of the complementary practice.

Should we be prepared to pay for the placebo effect?

Complementary practice often seeks to maximise the benefits of the setting and the relationship between the practitioner and the patient as part of the practice. It is our view that this effect is appropriately included in the consideration of clinical and cost effectiveness. However, there also needs to be an explanation – that commands confidence – of how the specific key physical intervention at the heart of the practice works.

If this condition is met then the placebo or non-specific effect, which is often intentionally integrated into the complementary therapy should be considered part of the treatment for which payment by an individual or the NHS may be appropriate.

All these considerations have implications for public health care commissioners as well as for individual members of the public who buy services privately. Those who pay have a right to expect evidence of, and research-backed explanations for, effectiveness. It is also vital to give therapists and conventional doctors a sound and ethical base on which to recommend specific interventions.

The report identifies five areas of consensus, which together set a framework for moving forward. These are:

- the primary importance of controlled trials to assess clinical and cost effectiveness
- the importance of understanding how an intervention works
- the value of placebo or non-specific effects
- the need for investment and collaboration in creating a sound evidence base
- the potential for whole-system evaluation to guide decision-making and subsequent research.

Based on this, we are able to set out a consensus for identifying appropriate methodologies that might be used in research into complementary practice.

Research in this area will always find it difficult to compete for resources. Complementary practice may not have the same potential to save lives or change the course of an illness as some more orthodox treatments; nevertheless, it affects people's quality of life and, like much of general practice, therefore has a highly significant impact on health. In a health care system where resources are always stretched, there is always a good case for funding research into a full range of practices that may offer much to the cost-effective treatment of all types of ill health.

1 Introduction

This report outlines areas of potential consensus to guide research funders, researchers, commissioners and complementary practitioners in developing and applying a robust evidence base for complementary practice. It is the result of the work of an independent advisory group and draws on a two-day conference of researchers, academics, practitioners and funders of research into complementary practice.

Millions of people regularly use complementary practices to help them manage their health. Yet the evidence base for complementary practice is often weak, comparatively small, and subject to criticism from many in the worlds of orthodox medical practice and basic science. There is no clear agreement on where the funders of research should target their resources to generate the evidence that individuals, NHS organisations or private insurers need to make informed decisions on using or providing complementary therapies.

Researchers need to develop sound approaches to the generation of such evidence. It is important that those who fund them ensure that their investigation is likely to produce relevant, useful and valid findings. Different complementary disciplines should be backed by sound evidence to help individuals decide on therapies appropriate for them.

Where complementary therapies are offered as part of the NHS it is imperative that commissioners who are allocating public funds use agreed and transparent processes, and that evidence is central to the decision-making.

The National Institute for Health and Clinical Excellence (NICE) is central to interpreting and assessing this evidence and promulgating evidence-based practice. It is noteworthy that the evidence is now sufficiently robust for NICE to include acupuncture as a treatment for low back pain.

The advisory group and the conference

The King's Fund convened an independent advisory group with an interest in research, regulation, public involvement and complementary practices to provide an analysis of the evidence presented and to identify areas of agreement. The group was chaired by Professor Dame Carol Black.

The King's Fund also brought together researchers, academics, practitioners and funders of research to participate in a two-day consensus conference into complementary practice. The conference took place on 22–23 October 2007. The aim was to explore whether it was possible to reach a consensus to guide those who fund research, those who carry it out, those who commission and those who deliver, in the task of developing a robust evidence base.

The conference had an invited audience of more than 100 participants from a variety of backgrounds. Over the two days the advisory group and the participants were able to question and challenge speakers to identify areas of both difficulty and possible agreement. Evidence was taken from researchers and academics, a major funder of

research, practitioners and consumer representatives. Both speakers and participants were able to submit written evidence on what they regarded as appropriate research methods for assessing complementary practice. However, while participation was broad, it proved difficult to engage all the potential funders of research, and the advisory group hopes that this report will help to bring more funders into this important debate.

The key areas covered during the conference were:

- Setting the context: understanding the needs of key groups affected.
- Setting the context: understanding the needs of key funding organisations.
- What evidence counts? Matching evidence needs with research methods.
- What evidence counts? The unique interaction between patient and practitioner.
- What evidence counts? Treating muscular skeletal conditions.

The conference was conducted on the basis that participants agreed that their views could be reported but not attributed, with the exception of the advisory panel members to whom (with their permission) comments have been attributed.

Before considering the material presented and the potential for consensus, it is worth acknowledging two issues:

- the difficulties of defining both complementary practice and a range of research terms
- the importance of establishing the right culture to bring about change.

The problems of definition

Definitions of 'complementary practice' and the various terms used within different practices are imprecise. This report seeks to maintain a focus on generic issues of importance across the field of complementary practice rather than addressing the specific challenges for each individual practice. However, there are three important characteristics of complementary approaches that have significant implications for understanding methodological problems faced by research in this area.

- First, complementary practice often encompasses an intervention (physical treatment or manipulation) as well as the context for that intervention. Context in this setting means both the physical setting for the delivery of care and the therapeutic relationship between practitioner and patient.
- Second, complementary practice is often concerned with broad and individualised (or self-defined) concepts of health and well-being that refer to the importance of treating the whole person.
- Third, complementary practice often adopts a view of the world that emphasises the inter-related and complex nature of human systems where the relationship between cause and effect may not be straightforward.

It is also worth commenting on the use of the term 'placebo'. This is a word that is imbued with meaning from its use in the field of pharmacological and traditional research. In this context the placebo effect is generally seen as a non-specific effect, unrelated to the treatment, which should be 'subtracted' from the overall treatment effect in order to assess the effectiveness of the intervention under scrutiny. This is entirely valid. However, the placebo effect can also be considered in broader terms and seen rather as the contextual effect, reflecting the contribution that the context for the intervention (the physical setting and the therapeutic relationship) makes to its effect.

We need to acknowledge that much of complementary practice seeks to integrate the positive aspects of placebo and that it needs to be viewed as an integral part of the treatment rather than an aspect that should be isolated and discounted. There is much still to be learned about how such contextual effects can improve specific interventions. Indeed there is a strong case for reviewing the state of knowledge around placebo and context to devise a more systematic approach to the generation of knowledge in this important area. That said, natural human curiosity is such that many people will want to reassure themselves that a treatment is effective over and above its 'placebo' effects.

There are also significant implications of such learning for conventional practice – not least in the ethical difficulties presented by health care professionals prescribing interventions where much of the effect may be related to placebo or context.

The need for culture change

There are occasional rows in the media over the efficacy and effectiveness of complementary practices but they are rarely helpful.

We need to create an environment that encourages investment in research that will lead to a satisfactory evidence base to inform individual and public decision-making in this area. Support for any consensus will depend not just on the level of agreement but on a willingness on all sides to engage in a rational debate.

All parties need to be open to challenge. That includes the need for complementary practitioners to accept the vital role of evidence in the development of their practice and not to assume effectiveness without understanding, and in some cases developing, the research base.

The questions arising from the evidence

The analysis of material presented to the group in written submissions and at the conference led to the following questions.

- Is there agreement on the need for a robust evidence base?
- What are the distinctive difficulties of research into complementary practice?
- What does it take to build confidence in a particular complementary practice?
- How can research into complementary approaches identify what works in practice?
- How should complementary practice research think about the placebo effect?
- How might research into complementary practice consider cost-effectiveness?
- How might the necessary research capacity be developed and supported?

These questions are addressed in the next section.

2 Seven areas of analysis

This section of the report analyses key issues that arose during the conference, presents the different views expressed and summarises the deliberations of the advisory group.

Is there agreement on the need for a robust evidence base?

I think putting more effort into research, more effort into building the science base and the methodology base of complementary practice, putting much of that into a transparent and open public debate so that the public can engage in it, will result not only in better practice and better understanding of complementary therapy but professions that are fit for regulation and in whom the public can have confidence.

Harry Cayton (advisory group member)

This captures the shared commitment among those attending the consensus conference. Although the event explored areas of disagreement, the importance of research was a common starting point. All those who contributed to the debate emphasised the importance of generating a high-quality evidence base for complementary practice. As advisory group member Professor George Lewith pointed out, ‘to starve the system of more knowledge means we will continue to make bad decisions’.

It seems to me that we should offer people treatments which are effective in meeting the objectives those people have set for themselves, and that requires an empirical test... there is no reason why we shouldn't have such empirical tests, for complementary as for other treatments.

Professor Richard Lilford (advisory group member)

There was also agreement on the need for such tests – particularly of effectiveness – to consider opportunity costs. Professor Lilford argued that, ‘For everything we do there is something else that has to be forgone. And as resources are limited... we have an obligation to the community to put our resources where they will do the most good.’

In response to the core question about the appropriate research methods for complementary practice, all speakers and participants emphasised the need to match appropriate research methods to the subject being researched rather than labelling particular methods appropriate or inappropriate.

What are the distinctive difficulties of research into complementary practice?

Much of the evidence presented during the conference focused on the characteristics that make research difficult in complementary practice. However, this builds a strong case for research investment, since each distinctive challenge resolved, through research, offers an opportunity for learning that could be applied not just in complementary practice but also in orthodox research and practice, especially in the case of complex disorders which are often treated with complex interventions.

Variability across therapists/practitioners

Complementary practice tends to emphasise the central role of the practitioner. The unique nature of their skills and the environment in which the intervention takes place can be difficult (although not impossible) to accommodate in the research design.

The deliberate integration of intervention and therapeutic context

In much of complementary practice the close alignment of the active physical intervention and the context for the practice makes it difficult to adopt the traditional research approach that seeks to separate out the effectiveness of the 'active ingredient' from the effectiveness of the whole.

The complex and dynamic nature of commonly treated conditions

The difficulty of researching effectiveness is related both to the complexity of the intervention and the complexity of the condition being treated and the difficulty of measuring intervention-related outcomes.

One speaker explained the difficulty of reaching judgements when it was hard to assume a direct relationship between the 'signal' (the intervention) and the 'noise' (the outcome). He went on to say:

Most people, most of the time, get better anyway without any treatment, particularly a lot of the chronic conditions that complementary medicine is aimed at like migraines, eczema, rheumatoid arthritis, things like that which are chronic relapsing remitting conditions. You treat someone when they are in a relapse then the chances are they are going to have a remitting condition anyway. So these temporal changes will occur whether you treat or not, so that is why we need a control group.

The different theoretical perspective that arises from treating the whole individual

There is no agreed definition of complementary practice but it could be argued that one common aspect is the way in which it focuses on whole individuals rather than isolated illnesses or dysfunctions. Hence the problem for any researcher attempting to isolate and measure the intervention or its outcome.

Is there a difference in research in complementary medicine and conventional medicine? I think there is a big difference and I think the difference is there because of the theory base of the therapies. Pharmaceutical and surgical techniques are based on biomedical theory and that is the theory that links one intervention with one disease, or biomedical change, or symptom. So there is a disease, you give the intervention and then you measure the effect on the disease before and afterwards, very roughly speaking, that sort of pattern.

Complementary therapies are based on a very different theory base. Most of them are based on alternative theories that conceptualise health and illness as a function of the whole individual within their life context and that means they are inherently individualised because their whole focus is on the individual, it is not on one disease or one symptom. That means that the interaction and the participation is very important and also that treatment effects are going to be at the whole person level, maybe at different levels: physical, emotional, mental, sometimes spiritual.

Conference speaker

It should be noted, however, that medical members of the conference did not all buy into the idea that their practice was atomised in the way the above speaker seemed to imply. An argument was made that any systematic approach to research in the area of complementary practice is inevitably different to the approach taken in the dominant pharmaceutical model – as one participant put it, when we are considering complementary practice: ‘Often these practices are used without theory or hypothesis for their mechanism of effect and we are trying to understand a little bit of what is going on beneath the practice. We are going in a very different, atypical, direction when we are looking at complementary therapy.’

The common use of un-researched complementary practice

The conference heard of a number of ways in which research can take a conventional treatment from initial idea to accepted general usage. This is typically characterised as moving from the bench to the bedside, or from the laboratory to the health professional or patient. The difficulty is that many of these approaches have little applicability to complementary practice where the therapies are already in common use.

One result of this is that research participants as well as the established research community come to the task of assessing complementary practice with established views. Finding research subjects or researchers with a balanced and open-minded view towards the research itself may be difficult.

Indeed, as one participant noted, there is also the difficulty of trial contamination given that some subjects ‘go ahead and continue to either use the intervention of the study, or multiple other outside interventions, without informing investigators’. This may dilute any quantifiable impact from the study and certainly compounds the many technical problems researchers in this area already face.

The opportunity for valuable learning

All these characteristics are distinctive but not unique to complementary practice. This is not a reason to hold up our hands but rather to roll up our sleeves – tackling the challenges to research in this field offers us the opportunity to take forward the work that we can do across health care in general. As Professor Lilford noted, ‘All the problems that you find in complementary medicine you will encounter in some other kind of treatment ... when we stop and think about it... how different is it to any branch of health care – the answer to emerge from our debates is that it may only be a matter of degree.’

What does it take to build confidence in a particular complementary practice?

There is a paradox at the heart of the noble human endeavour to inquire rigorously into how everything works. On the one hand, those of a scientific disposition are driven to challenge every hypothesis and view every account or piece of evidence, as partial. On the other hand everyone needs confidence in some common accounts of how things work (even when they are incomplete) so that we do not deny ourselves the undoubted benefits of treatments or technologies, even when our knowledge of their exact mechanism of effect might not be comprehensive.

The degree of confidence is related to our interpretation of the current evidence and it is important to acknowledge that this is only ever a judgement of the facts as we know them at the time. As one participant put it, ‘Instead of thinking of numbers needed to treat we should think of numbers needed to convince.’

It might be surprising to you to know that although I am a pharmacologist by background I don't necessarily need to know the mechanism of action of a treatment when I give it to a patient. In fact to be honest with you there are very few things I really really understand about how they work at a molecular level.

Conference speaker

More to the point, there is often a lack of rigorously proven theory about the mechanisms that deliver improved health. This is true for many conventional interventions. Yet, where commissioners, practitioners and public have a justifiable confidence in some of the likely causes and effects and their proven effectiveness, an incomplete understanding of the 'mechanisms of action' does not necessarily stop the health system from providing a range of treatments.

Nevertheless, a set of potential hypotheses about the cause and effect that lies at the heart of the physical intervention is important. We develop a confidence (or uncertainty) about the way in which a particular intervention might be beneficial based on the plausibility of these hypotheses and evidence from trials that deliberately set out to understand and test these mechanisms.

This confidence is important. We should always consider the possibility that any positive findings of effectiveness may be due to other factors such as the 'non-specific' aspects of the treatment. Those aspects of the experience are not about the physical intervention, but rather about the context in which it is provided, such as: our expectations, the health care setting, or the therapeutic relationship (to name but a few). Indeed, we have to consider the possibility that the positive effects of these aspects may be masking a physical intervention that is in itself ineffective or even detrimental.

One speaker powerfully challenged participants to consider if it is possible to 'bypass efficacy by doing effectiveness studies' and asked whether there 'can be effectiveness without efficacy?' Most participants and the advisory group agreed that issues of efficacy (the question of whether the specific intervention works – and how) cannot simply be eclipsed by evidence of effectiveness (the question of whether the whole intervention including all the non-specific effects generates positive outcomes). However, by the same token many felt that the degree of confidence needed about the exact mechanisms that enable a practice to work may well be influenced by the robustness and strength of the findings of effectiveness.

One of the advisory group was asked what evidence would be required to convince him that a particular complementary approach worked.

Scientific evidence that makes the theory more plausible, or direct empirical evidence that is just so strong that even somebody who starts off very sceptical changes their mind... the two would be complementary. I would not form a view that it worked, purely on the basis of scientific evidence. Just like I try not to let myself form a view, or too strong a view anyway, on what works in clinical medicine just on the basis of its apparent theoretical probability. And many is the time when I have been very surprised by the results of direct comparative studies.

Professor Richard Lilford (advisory group member)

How can research into complementary approaches identify what works in practice?

The demonstration of clinical effectiveness is essential. There is no way that one can reasonably take interventions to the public and offer them to patients without demonstration of clinical effectiveness and it is impossible to estimate cost-effectiveness without some good evidence of clinical effectiveness.

Conference speaker

This position goes directly to why understanding the appropriate matching of research questions and research method is so important to advance scientific inquiry into complementary practice. One of the central problems is the need to recognise that most of the conditions commonly treated by complementary practice are such that it may be difficult to attribute changes in health to the complementary intervention. Uncontrolled studies are therefore not likely to be convincing.

Studies need to be controlled although not necessarily by a placebo control, in order to tackle the problem of assuring real connection between health changes and intervention. Two particular problems need to be carefully considered, first the danger of ‘regression to the mean’. This is a statistical phenomenon that can make natural variation in repeated data look like real change. It happens when large or small measurements are followed by measurements that are closer to the mean. This can confound the ability to attribute health change to an actual intervention and can be alleviated through good study design and the use of suitable statistical methods. Second, the need to be wary of ‘temporal effects’ whereby natural changes in the course of the disease over time mean that measurements after a period of severity in the illness (which may have brought the person into a research trial) are quite naturally followed by less severe outcomes (regardless of the intervention). Again the ability of the research to control adequately for these potential effects is important in ensuring high-quality evidence.

However, implementing a sound controlled approach to effectiveness brings difficulties. Where researchers might usually test ‘usual treatment’ against ‘complementary practice’ we can come unstuck, since many (although not all) complementary approaches are a combination of usual and complementary practice. When this is the case, the test can become one of ‘usual treatment’ against ‘usual treatment plus complementary practice’. This is methodologically difficult since it is intuitively possible that people tend to value ‘usual treatment plus complementary practice’ more if for no reason that they are getting additional treatment.

However, narrower tests of ‘sham’ complementary practice against ‘real’ complementary practice are equally fraught with difficulty. Giving a realistic ‘sham’ treatment may be technically difficult. There is also the profound question of drawing a hard and fast boundary around the ‘active’ element of the intervention when many aspects of the context – including the therapeutic relationship – are deliberately and consciously integrated into the intervention itself as a deliberate part of the approach.

When it comes to demonstrating a plausible effect arising from the core physical intervention the approach taken in the field of acupuncture provides a valuable example. Here some researchers have compared the effects of real and sham acupuncture. However, while this approach is useful in developing confidence in efficacy, the real test of effectiveness would require a more appropriate comparator to be the usual non-acupuncture treatment (in a trial suitably controlled to account for possible ‘regression to the mean’ and ‘temporal effects’). Furthermore, it may be appropriate in this case not to control for so-called placebo effect.

How should complementary practice research think about the placebo effect?

If there is such a thing as placebo effect we should pay for it. People who improve after treatment improve for one of four reasons: first, regression to the mean; second, there is temporal effect; third, there may be a placebo effect; and finally there may actually be treatment efficacy. The last two we should pay for. The other two are going to happen anyway whether you treat or not, so we shouldn't pay for those.

Conference speaker

This is a strong position with implications for appropriate research methods.

Not only complementary therapies but a wide range of conventional therapies need to be evaluated using pragmatic ('real world') randomised control trials, because this allows us to evaluate effectiveness including any placebo effect, while eliminating regression to the mean effect and temporal change.

Conference speaker

The advisory group believes there are good reasons for including placebo effects in cost-effective assessments of complementary practice. The rationale is that complementary practice often includes a deliberate attempt to fashion an intervention that incorporates and, indeed consciously integrates, such effects into a more holistic and individualised treatment package. In such cases trying to identify and isolate the specific active ingredient may divert the research endeavour away from a true assessment of the treatment. Indeed, it is evident that such a true assessment should respect the underlying theoretical integrity of the treatment.

As one speaker put it: 'I can see how scientists would want to take it apart and look at each individual thing, but if they are all intimately connected then it becomes meaningless to do so because you destroy what you are doing, so in that sense it is not really a meaningful choice.' This is not the case for the typical pharmacological intervention.

However, there is a balance in such judgements and the apparent counterview also clearly expressed at the conference – should be acknowledged.

The notion that because things are complex that this somehow enables us to say it doesn't fit the scientific model is absolute nonsense. It is because things are complex that we have evolved the scientific model.

Conference participant

Although these may seem mutually exclusive positions, this is not necessarily the case. It is possible to accept the argument that there is less reason to adopt a particular scientific approach that distinguishes between 'active' and 'non-specific effect' – trying to account for the former and isolate and subtract the latter – in much of complementary practice. Indeed, a more scientific approach might be to consider how to assess the effectiveness of the whole while using our research expertise and creativity to control for the distortions that may effect any appropriate measurement or conclusion.

How might research into complementary practice consider cost-effectiveness?

The approach to identifying potential consensus has been to acknowledge that there are many legitimate responses that the research community may adopt to the difficulties of generating high-quality research in this area. This report aims to mark out the most productive terrain to explore and to encourage that exploration. Having established

some of these boundaries in relation to the assessment of effectiveness in complementary practice, there are many approaches to considering cost-effectiveness.

A conventional route might stress taking forward findings of effectiveness into a subsequent comparative exercise, looking at the findings and the relative costs for different treatments. However, it is possible to consider cost-effectiveness as a starting, rather than a finishing, point. Looking at the issue this way would enable the research community to adopt approaches such as health service system-wide trials of new care pathways that included complementary practice with the primary intention of testing the potential for different patterns of service to show a positive potential to reduce costs and deliver health outcomes.

Such work could easily precede and inform the prioritisation of more detailed studies of efficacy and effectiveness. As advisory group member Professor Adrian Eddleston put it, such system-wide cost-effectiveness studies could help us.

We shouldn't just be interested in the number like it is some dial on a dashboard. We should be interested also in what it means. And to that we might need to include, in our outcomes, things that help us understand the meaning of what we are doing. I am interested in service delivery trials, so I argue for the inclusion of lots of end points in those studies to help understand the result that you have got. So in the studies of an intervention one would like to look at the biochemical, or neurophysiological, or some things that might help you understand why you got the result that you got.

Professor Adrian Eddleston (advisory group member)

How might the necessary research capacity be developed and supported?

Throughout the conference many participants, across the different communities of research, practice and academia, identified the positive effect that the larger funding organisations can have in helping them to face research challenges. Many called for funding organisations to do more to help build a stronger research capacity in this area, systematically taking forward a research agenda that is technically difficult, professionally challenging and controversial. Participants cited examples of how funding organisations had in the past helped to lead such initiatives by inviting participation in collaborative research endeavours. Many participants referred to the research capacity-building programme managed by the NIHR Coordinating Centre for Research Capacity Development (NIHR CCRC) and discussed the need to develop support in this field on an ongoing basis.

There was also some discussion around the difficulties of engaging a critical mass of serious researchers. As one participant commented: 'We have had considerable trouble getting the clinical trial units interested in working with us and that has really inhibited our work.'

Many saw the answer as needing better and sustained research funding alongside a collaborative effort to connect the scientific, research and complementary practice communities. As a representative from one funding organisation noted, 'It is about pulling together as a network of different people with different expertise, pulling together the skills and the knowledge and, using that collaborative effort to generate high-quality applications.'

3 Discussion and conclusions

Conference participants reached a consensus in a number of areas.

- The public needs more robust evidence to make informed decisions about the use of complementary practice.
- Commissioners of public health care need more robust evidence on which to base decisions about expenditure of public money on complementary practice.
- There are no straightforward or right or wrong research methods for complementary practice.
- There are differences of opinion about the important questions that need to be addressed and the appropriate methods required to answer those questions.
- An attempt to map out a potential consensus response to these differences of opinion could be useful.

Mapping potential consensus

In order to map out a consensus, the advisory group believes it is helpful to consider three broad categories of question:

- whether something works
- how something works
- how much something works.

Broadly speaking, the first category of question is concerned with understanding the effect of the intervention without necessarily seeking to understand the mechanisms that lead to that effect. The second category of question seeks an increasingly refined and robust understanding of the mechanisms by which a specific action or practice achieves a particular effect in the recipient. The final category of questions is concerned with measurement and often with a comparative measurement. It is particularly important in addressing issues of cost-effectiveness.

All three questions are important. Although they may be connected, there is no particular order in which the questions should be addressed. Each of the three broad areas of inquiry should be pursued and could be pursued simultaneously.

Each question stimulated a specific area of dispute. These disputes are captured in the response to the following questions and it is on these questions that the advisory group has focused its efforts to map out a potential consensus that could take us forward.

- If we know something works, do we need to know how it works?
- When thinking about how something works, do we need to know exactly what is working – showing its effect separately from any difference made by the context in which the care is delivered?

- When addressing the question of how much something works, should we be prepared to pay for the so-called placebo effect?

If we know something works, do we need to know how it works?

Although exchanges over this question were heated it is possible to outline a potential consensus on the issue. Broadly speaking, the answer to the question is a qualified yes.

However, research into how an intervention works rarely provides complete or conclusive evidence. Instead, the findings usually change the degree of confidence or uncertainty about a chain of cause and effect that may explain how the essence of the intervention is working.

No strategy for improving the evidence base for complementary practice would be complete without investment in work to address these questions – in short, research evidence to help us understand the mechanism of effect. Such work is at the heart of progress in science and is crucial to the fundamental human desire to extend our knowledge and continue to question, challenge and learn about the world around us.

As an aside, we would also encourage further research to better understand the mechanism of effect for the common non-specific aspects of treatment. This should be conducted alongside specific inquiry into the different physical interventions used across the spectrum of complementary practice.

However, given the incomplete nature of the evidence that often emerges from such research inquiries the group does not believe that it would be right to set a hard and fast level of evidence that has to be generated from such work before progressing with other important inquiries into measuring the effect of the intervention in practice. Indeed, we believe it is the combination of evidence across efficacy and mechanisms of action, clinical and cost effectiveness (depending on the complexity of the treatment and condition treated) that is important. It is a judgement of this combined evidence that should ultimately influence the decision-making of individuals and the commissioners of public health care.

Demonstrating a plausible chain of cause and effect that may be judged sufficient to give us confidence that the practice works is an important part of that judgement. However, the threshold used should vary depending on other evidence of clinical and cost effectiveness in practice.

Do we need to know exactly what is working?

This is an important area where there is a need to challenge the various positions presented at the conference. The commonly experienced high ‘signal’ to ‘noise’ ratio in complementary practice justifies a focus on rigorously controlled studies. This would tell us whether or not interventions were effective having taken account of possible temporal effects and potential regression to the mean – ie, changes that may have occurred anyway.

Such control does not necessarily mean designing placebo-controlled trials. However, it is important to have enough evidence of a plausible mechanism to discount the possibility of non-specific effects completely masking an ineffective physical intervention (*see above*). The justification for this rests on the inappropriateness of attempting to control for placebo, in order to subtract it from the effect, when the practice itself is consciously attempting to integrate and harness such effects. Indeed, attempting this integration is often a clear part of the underlying theory informing the practice.

Should we be prepared to pay for the so-called placebo effect?

The view of the advisory group is that it is appropriate to pay for true placebo (rather than regression to the mean or temporal effects). This is an effective and often distinctive element of the complementary practice (giving benefit over and above an orthodox approach). So when it comes to research design there may be good reasons for valuing these effects.

It is worth emphasising that complementary practices are a broader intervention where much of the context of delivery should legitimately be considered part of the package being assessed for overall effectiveness (subject to sufficient confidence over the mechanisms of action of the physical intervention).

Reflections on the position of commissioners

The advisory group reflected on the implications of these questions and answers for those who commission public health care. Their decisions involve some particularly difficult judgements. Before public money is spent on providing complementary practice, the advisory group felt the following should be demonstrated:

- evidence for effectiveness, suitably robust to control for potential distortions in the findings (from temporal or regression effects) but not necessary controlling for the placebo effect
- a rational articulation of plausible potential explanations for the impact of the physical intervention – backed up by research – accepting the commonly partial and contestable nature of such findings. For example, this could be achieved by rigorous research on mechanisms of action that develops a potentially plausible understanding of how the practice works.

There are other criteria, particularly concerning cost-effectiveness. But without these prior conditions being met a public health care system cannot reach a sound judgement that a complementary practice should be provided at taxpayers' expense.

The alternatives to this position are not acceptable. The public health care system should not sanction an intervention without a demonstrative mechanism for direct health benefit in which there is a degree of common and expert confidence (although this may not always be unanimous).

Although, having said this, we believe that commissioners can also play a valuable role by considering the commissioning of complementary treatments in ways that enable an assessment not only of cost-effectiveness for the NHS, but also, through good design, evidence of clinical effectiveness.

Reflections on the position of clinical professionals

The advisory group considered the difficult position of clinical professionals, particularly in the case of an intervention where research into the mechanisms of action were casting significant doubt over a plausible mechanism to explain how the physical treatment at the heart of the intervention was working and where no placebo controls had been included in the evaluation. Even if effectiveness findings were positive (comparing a complementary approach against standard care) the health professional would be put in an invidious position as these positive findings could relate solely to non-specific (placebo) or contextual factors. Could a health professional recommend such a treatment? And if they did would they be obliged to be clear about the possibility that the

actual physical intervention was ineffective – even if such a statement undermined the potential for harnessing or maximising a positive placebo effect?

As one of our participants put it: ‘The conundrum, presumably, if you are going to be honest with a patient you have to say, this treatment... is in fact mostly or 100 per cent placebo, but I am going to give it to you.’ There are profound implications of such a position. As another participant said in reply: ‘Do we tell them that the placebo is a placebo? Are we completely honest within our own paradigm? For what it is worth, my values suggest that yes, we do. We are constrained in our particular profession by that and it is fine.’ It is understandable that doctors may not wish, or may even feel that it is unethical, to facilitate a predominantly placebo treatment. Indeed professional status may in the advisory group’s view require such an ethical stance. However, we also recognise that within the placebo effect there may well be a number of active and effective therapeutic actions that we are not yet able to fully describe, recognise and apply in practice.

4 Potential areas for consensus

The advisory group believes there is the possibility of creating a new consensus on the role of different research methods, their applicability and their limitation in assessing complementary practice. This view is informed by both hearing and questioning the opinions of many others in open debate. We offer it in good faith as a position that we believe is sound and amenable to agreement by all parties. We hope that it will guide researchers and the organisations that fund research and help to generate the kind of agreement about methods that might enable a burgeoning of much needed rigorous research in this area.

A summary of our position

The potential areas for consensus can be summarised in **five statements** where the advisory group believes there is the possibility of a helpful and liberating consensus that might enable a concerted effort to be taken to delivering the high-quality evidence that is needed.

Controlled trials of effectiveness and cost-effectiveness are of primary importance

We recognise that it is the assessment of *effectiveness* that is of primary importance in reaching a judgement of different practices. Producing robust evidence that something works in practice – that it is effective – should not be held up by the inevitably partial findings and challenged interpretations arising from inquiries into how the intervention works.

Research into the impact of complementary practice needs to be undertaken in a way that controls for temporal changes in health and the potential for measurements in health to regress to a mean – the patient getting better over time regardless of any intervention.

However, such trials do not necessarily always need to be placebo controlled. Having controlled for temporal effects and regression to the mean, many of the other so-called non-specific, placebo or contextual effects that might have an impact on the health of the recipient could well be legitimate aspects of the complementary practice. As such they should be considered part of the effect of that practice. This does not mean that placebo-controlled trials are always unnecessary as the placebo component may itself be ephemeral and many people will be interested in isolating the placebo component.

It is important to understand how an intervention works

Research into how complementary practice might work is extremely worthwhile. It seeks to understand more about the mechanism of action for complementary practice. Through such an understanding we can develop confidence across practitioners, researchers and patients on likely and plausible cause-and-effect mechanisms triggered

by the intervention. This is significant and valuable if the use of public funds on complementary practice is to be justified.

We recognise that where the mechanism of action is not known (a situation that applies to many orthodox as well as complementary practices) adoption of the intervention might be warranted by robust clinical and cost-effectiveness data.

Placebo or non-specific effects are of value

In much of health care the effect of the physical intervention can be enhanced by the therapeutic relationship within which the treatment is given as well as the context within which care is received, such as the expectations of the patient and the setting in which the care is provided. This applies in orthodox treatment and there may be much that could be learned from complementary practice on how to harness and enhance such positive aspects of care.

In complementary practice much is done to incorporate these effects into the intervention. This should be recognised. As long as findings from research can provide confidence in the positive effect of the physical intervention at the heart of the treatment, then any added benefit brought by the therapeutic relationship and the context for treatment should count as part of the treatment effect. For complementary therapies such a holistic approach to effectiveness should be adopted by bodies such as the National Institute for Health and Clinical Excellence (NICE), when comparing cost-effectiveness across a range of treatments.

There is a need for strategic investment and the development of collaborative effort

The challenges in generating robust evidence for effectiveness, efficacy and comparative cost-effectiveness are substantial. Those who seek to achieve this need to engage with a wider community of scientists to develop new methods. Researchers need access to expertise and guidance in the formulation of fertile research questions and rigorous means of addressing them.

There is a case for those who fund such research to adapt their approach and work alongside those already involved in this field. At the same time they need to encourage a new cadre of high-quality researchers to work in this area as well as building on existing capacity. All this must be driven by a commitment to achieve the highest levels of rigour.

This might include initiatives such as working with researchers and practitioners to improve the quality of applications for research funding and developing cross-funder strategies for building research capacity in complementary practice. In addition, work should be undertaken to develop robust metrics for the evaluation of the impact of services in practice.

Commissioning complementary practice as part of a range of services should depend on the strength of the evidence. Given the limitations of the evidence base, commissioners might want to only consider commissioning complementary practice alongside a robust evaluation or research trial to assess effectiveness.

Indeed, those responsible for funding research should engage commissioners to ensure that their views are taken into account when setting research priorities. Research funders are also well placed to engage the public in a dialogue about the public understanding of science in this field and to help people understand how to use future evidence in their own decision-making.

There is potential for whole-system evaluation

The advisory group believes there is merit in considering research into cost-effectiveness as a starting point, rather than an approach that is adopted much later in the process of research inquiry. This would mean adopting approaches such as system-wide trials of care pathways that included complementary practice, where possible in comparison with established pathways. Such studies would test the potential for different patterns of service to show reduced costs and/or deliver better health outcomes. Such work could precede and inform more detailed studies of effectiveness and mechanisms of action. Indeed, funding for complementary practice could be conditional on it being part of such comparative research.

A consensus on methodology

We appreciate that the task of selecting research for funding requires a proper assessment of the quality and importance of applications. Our contention is that the assessment of quality in this area is complex and that a dialogue is required between those who design and implement research and those who fund it. This should lead to a better understanding of the distinctive characteristics of methodology required to undertake sound research in this area.

Having considered all the informed contributions presented to it, the advisory group has established a set of consensus position statements for developing methodology and evidence in this area of health care (*see below*). We offer it to all parties in the hope that our recommendation for ongoing dialogue might be helped by this opening contribution.

A summary of a potential consensus on the place of appropriate methodology in research into complementary practice

All methods, if correctly matched to the research question and used correctly, can answer appropriate questions and add to our understanding.

The sole use of any one method is unlikely to be sufficient to establish a robust evidence base for the use of complementary practice.

Studies into how an intervention works are necessary, but alone are not sufficient to establish a robust evidence base for the use of complementary practice.

Studies that assess both how interventions work and their clinical effectiveness may be attractive, if rarely achievable. However, such work may risk missing out a substantial part of the treatment if it discounts all but the specific aspects of the intervention.

Studies into effectiveness are the most valuable but need to be controlled for temporal and regression effects and considered alongside evidence of the plausibility for the intervention. Patient satisfaction alone is insufficient evidence.

This evidence of plausibility will be revealed by studies into the mechanisms of effect. These are much needed to discount the possibility of an intervention consisting of a 100 per cent placebo effect which would place the practitioner under an unacceptable ethical dilemma.

Studies that seek to control for all aspects of placebo risk not establishing a real value for the effect of the whole intervention. Discounting the placebo-related

aspects of an intervention may reduce its assessed value when compared with other less integrated interventions.

Nevertheless, placebo effects may be ephemeral and there is a place for placebo-controlled studies – including three-way studies looking at active treatment, placebo (or sham treatment) and ‘treatment as usual’ – to take forward our understanding of placebo.

Studies based on whole systems or care pathways offer a good way of reaching a better understanding of the impact and cost-effectiveness of complementary practice. The opportunity for such work to inform and help set priorities for future research questions should be further developed.

The advisory group acknowledges that many interventions in complementary practice do not have the same potential to save lives or change the course of an illness as some more orthodox treatments. This makes it difficult for research in this area to compete effectively for limited resources.

However, the group also believes there is a need for greater clarity about the criteria that research proposals need to satisfy to be serious contenders. There is merit in ensuring a balance in research investment across different branches of health care including complementary practice. Complementary practice affects quality of life, like much of general practice, and therefore has a highly significant impact on health. We also believe there is limited value in deciding what should be funded by testing the relative benefits of different funding decisions against a single scale of potential impact. Again we recommend dialogue and a consequent clarification of criteria for funding that acknowledges the distinctive features and challenges of research in this area.

We also recognise the importance of the need for trust and confidence in the relationships between funding organisation, researcher and practitioner. Of course this must be built on the quality of work, but we also acknowledge the importance of researchers demonstrating their commitment to concepts such as equipoise. Researchers and practitioners in this field need to demonstrate that they are prepared to work towards the application of research findings and be able to show how research is guiding future practice. Indeed, we suggest that the community of complementary practice researchers and complementary practitioners need to show that research findings are being consolidated and adopted in the systems that subsequently determine professional practice and professional regulation.

Advisory group members and conference speakers

Advisory group members

Harry Cayton, Chief Executive, Council for Healthcare Regulatory Excellence
Professor Adrian Eddleston, then Vice-Chairman, The King's Fund
Professor George Lewith, Professor of Health Research, Complementary and Integrated Medicine Research Unit, University of Southampton
Professor Stephen Holgate, MRC Clinical Professor of Immunopharmacology, University of Southampton
Professor Richard Lilford, Head of School of Health and Population Sciences, University of Birmingham

Conference speakers included:

Dr Alan Breen, Professor, Anglo-European Chiropractic College
Professor Nicky Britten, Professor of Applied Health Care Research and Deputy Director of the Institute of Clinical Education, Peninsula Medical School
Professor Edzard Ernst, Chair in Complementary Medicine, Exeter University
Professor Linda Franck, Chair of Children's Nursing, Great Ormond Street Hospital for Children NHS Trust
Professor Stephen Holgate, MRC Clinical Professor of Immunopharmacology, University of Southampton
Dr Janine Leach, Senior Research Fellow for Osteopathy, National Council for Osteopathic Research
Dr Hugh MacPherson, Senior Research Fellow, Department of Health Sciences, York University
Professor Ann Moore, Director of the Clinical Research Centre for Health Professions, University of Brighton
Dr Charlotte Moore, Cancer Research UK
Dr Charlotte Paterson, Senior Research Fellow, Peninsula Medical School
Sir Michael Rawlins, Chair, NICE
Dr Wendy Smith, National Institute for Health, USA
Professor David Torgerson, Department of Health Sciences, University of York
Dr Adrian White, Clinical Research Fellow, Primary Care Research, Peninsula Medical School; Editor, *Acupuncture in Medicine*

Appendix B

Use of language in the report

When we use the term *efficacy* we are referring to work that considers whether the specific intervention works. Often this work also focuses on testing what the underlying mechanisms of action for the intervention might be. These mechanisms of action are chains of cause and effect that might explain the positive (or negative) impact of the physical intervention at the heart of the practice.

Although we accept that there are good reasons for also looking at the ‘mechanism of action’ for the non-specific aspects of the intervention, such as the physical context for the practice or the therapeutic relationship within which the intervention is provided, we predominantly consider this work in relation to the physical intervention at the heart of the practice (unless otherwise explicitly stated).

When we talk about the *effectiveness* of complementary practice we refer to the health outcome delivered by the intervention and unless otherwise stated we include the non-specific aspects of care in the intervention. We make the case that research into the clinical effectiveness of complementary practice should control for temporal and regression effects but not necessarily seek to control for the positive aspects of the context in which the practice is delivered or the therapeutic relationship that the practice seeks to create (although we accept there are methodological issues concerning the consistency of these effects).

For us, effectiveness studies do not necessarily require an explanation of the mechanisms of action although they may assist us in generating hypotheses for subsequent testing. We argue that judgements over the value of complementary practice need to consider the findings from work testing mechanisms of action and clinical effectiveness (and cost-effectiveness) together.

We argue that any judgement over the interpretation of effectiveness should encompass questions over the plausibility of the mechanism of action for the physical intervention at the heart of the practice. However, we also acknowledge that the weight to be given to the results of effectiveness studies may differ depending on the strength of findings in relation to studies into the mechanisms of action. This is important to avoid judging an intervention as a whole to be positive when the physical intervention at its heart may be potentially ineffective and could be masked by the more positive impact of surrounding non-specific effects.

When we talk about *placebo effects* we are limiting ourselves to those non-specific aspects of the intervention that are outwith the specific physical intervention, specifically we consider the therapeutic relationship and the context in which care is given although we accept that there may be other facets of the non-specific yet active elements of the practice (such as the belief systems of the care-giver and cared for) that we have not considered in detail.

