

Finding the balance

Benefits and risks of medical treatment

A paper and consultation document



Association of the British Pharmaceutical Industry
Long-term Conditions Alliance



**Association of the
British Pharmaceutical Industry**

12 Whitehall
London SW1A 2DY

Telephone: +44 (0)870 890 4333

Fax: +44 (0)20 7747 1411

Web: www.abpi.org.uk



Registered Charity Number: 1057711

**LTCA
Long-term Conditions Alliance**

202 Hatton Square
16 Baldwins Gardens
London EC1N 7RJ

Telephone: +44 (0)20 7813 3637

Fax: +44 (0)20 7813 3640

Web: www.ltca.org.uk

Please send your comments on this document to

Marjorie Johnson: mjohnson@abpi.org.uk

and

Mark Platt: markplatt@ltca.org.uk

Finding the balance

Benefits and risks of medical treatment - a paper and consultation document

INTRODUCTION

Modern life involves making countless choices about benefit and risk, some more significant than others. Sometimes we make instant decisions without the need for much thought. Other decisions require information to consider and time to reflect.

Healthcare is no exception. Increasingly, as patients and carers, we are becoming full participants in decisions about our medical treatment. If we are to make informed decisions about treatments that best meet our individual needs, we need to be equipped with the skills and knowledge necessary to enable us to balance for ourselves the benefits and risks of medical treatments.

To that aim, this consultation document starts by laying out central themes that lie within the larger concept of benefit/risk in our personal health decision making. It then translates these themes into practical examples and suggests ways forward - not just through big public education programmes that are easy to propose but so hard to make effective - but through a range of more manageable initiatives involving a variety of stakeholder partnerships.

In capturing the human dimension in what is often considered solely the remit of scientists, it is hoped that these partnerships could lead to a significant improvement in our ability to make informed decisions about our medical care, and thus make a real difference to the nation's health and well-being.

We would like you to tell us what you think about these ideas, how we could develop them further, what we have missed and other ideas for manageable programmes that stakeholders (including those not specifically mentioned here) could undertake. Based on the comments that we receive, we hope to expand the report and consider how we may take these activities forward, or encourage others to do so.

Comments are always welcome but for the expanded report, please contact us, using the email addresses on the opposite page, by Friday 27 June.

The themes and many of the ideas in this document emerged over the past year, beginning with a large conference held at the end of 2006. *Finding the Balance*, run jointly by the ABPI and the LTCA, set out to address the following areas:

- a common language for benefit and risk,
- the quality of information available for decision making,
- why information isn't everything - making room for difference,
- personal responsibility vs protection from ourselves.

Finding the balance

CENTRAL THEMES

- An understanding of the nature of the benefits and risks of our medical treatment, and the impact that they may have on our lives, is crucial to our informed participation in, and our sense of ownership of, our own healthcare.
- The perception of the value of benefits and the importance of risks can differ, sometimes substantially, between different individuals - for instance, a member of the public without experience of a given condition may view risks differently from a patient living with that condition. A patient's view may change during the disease pathway. Similarly, individual carers, health professionals or health policy makers may have different perspectives.
- Patients' differing views on benefits and risks should not be seen as any less or any more valid in or of themselves. Individual life circumstances, age, gender, cultural perspectives, personal aspirations and private fears may influence the decisions patients make in favour of, or against, a specific treatment - or of leaving a condition untreated.
- A person with the heavy responsibility of making decisions on another's behalf, for a young child or elderly parent for instance, may have greater and/or different concerns and fears than they might if they were making the decision about their own care.
- Access to high quality information about one's condition and its treatment which is both medically accurate and relevant to real patient experience is crucial.

- Information needs are likely to change as a condition progresses, an individual becomes more expert in its management, new treatments are developed, old ones withdrawn or new thinking emerges.
- How benefits and risks are presented and explained can have a big impact on how we interpret their importance and in turn, on the choices we make.
- Understanding attitudes to risk, and the factors that influence those attitudes, is essential to good benefit/risk communication and a health literate population.
- The public's ability to understand and willingness to accept uncertainty needs to be better recognised.
- Conclusions about benefits and risk reached by researchers, doctors, policy makers and regulators must be informed by those who are directly affected, if those conclusions are to speak to the reality of people's lives.
- Respect for difference - and the willingness and ability to accommodate it within the healthcare system - underpins constructive health-enhancing relationships between those receiving medical care and those who develop, deliver, inform about and regulate medical treatments.

WHAT DOES THIS MEAN IN PRACTICE?

- Health service users - in particular those with long-term conditions - should be encouraged to learn about their condition, its treatment and self-care and, where appropriate, to see doing so as an integral part of their wider care plan.
- Service users should be encouraged to raise any questions/concerns about treatments with health professionals, rather than keeping such questions/concerns to themselves. Unasked questions and unexpressed concerns often lie at the root of poor health outcomes.
- Today's doctors are more likely to recognise that informed and involved patients are likely to have better outcomes, as well as use NHS resources more effectively, but day to day demands on clinicians' time tend to take precedence. Clear steps need to be devised and taken to ensure that the concept of the informed and involved patient is made a reality embedded at the heart of medical practice.
- Clear high quality benefit/risk communication tools need to be developed, with the involvement of coalitions of stakeholders. These should build confidence and trust, minimise the likelihood of misunderstanding between patients and their doctors and acknowledge that diverse approaches may be equally valid.
- Health communicators, whether from the healthcare professions, government, regulatory bodies, industry, the voluntary sector or the media should make clear to the public that a benefit/risk assessment, even when based on a wealth of high quality evidence, cannot give absolute definitive answers.
- Effective communication needs to encourage awareness that no treatment is entirely risk-free for every individual, nor can the potential benefits be guaranteed, but that inadequate treatment can have consequences and/or risks of its own.

- Health professionals need to be given practical training to help them develop the skills to work constructively with, and to maximise health benefits for, patients whose views on appropriate treatment differ from their own.
- Medicines are frequently taken incorrectly, making them less effective and less safe than they would otherwise be.
- People often do not have their prescription filled or they may cease to take a medicine when it is important that they do so, because for example:
 - Media comment about risks of treatment may cause undue alarm.
 - The patient, or parent, thinks that “less medicine is better medicine” (eg not using the asthma medicine that keeps the underlying inflammation under control because, in the short term, adequate relief is achieved from quick-acting bronchodilators).
 - The patient feels better and so thinks the medicine isn't needed anymore (eg stopping antibiotics mid-course as the inflammation abates).
 - The patient did not feel unwell in the first place (eg asymptomatic high blood pressure) so the side effects of anti-hypertensives seem unacceptable.
 - The doctor uses language that is unclear eg “the problem is under control”, which some people may think means it is not so important to have a prescription refilled, particularly if they experience some side effects.
- Regulators need to ensure that the real impact of a condition on people's diverse lives is understood when difficult decisions about the balance of benefit/risk have to be made, whether when licensing a new product, considering a new indication, putting in place new conditions of use or taking a product off the market.

SUGGESTIONS FOR ACTION

(Please note that the suggestions below focus on medicines but could be adapted to cover other types of treatments.)

In the GP practice

- **GP practices should be asked to audit their existing financial, staff and space resources and then put together a plan as to how these resources could be further deployed to enable, assist and accommodate the involved and informed patient and also to spark interest from patients who have not yet considered becoming so.**
- **In conjunction with primary care Patient Participation Groups, create a standard GP “Dear patient” and/or a more two-way version eg “Our contract with you” and a “Your medicine and you” booklet to give to new patients and to have available in surgery waiting rooms covering;**
 - Why it is important that patients understand their medical condition, the likely benefits and risks involved in its treatment and any risks involved in leaving the condition untreated.
 - That if treatment is needed, the doctor will endeavour to work with the patient to meet his or her individual needs.
 - That patients should not necessarily expect to receive a prescription when they visit the doctor and that if a medicine is prescribed, it will be for a good reason.
 - The importance of taking a medicine as directed.
 - That prescribed medicines have been through an intensive research process to demonstrate safety and effectiveness, but no medicine is entirely free of side effects for every person who may take it.
 - That most side effects are transient and mild when compared to the potential seriousness of the condition and/or the degree to which it interferes with our ability to fulfil our responsibilities and enjoy life, whilst noting that some side effects can be serious.
 - That any unexpected adverse effect should always be reported (with instructions as to how to do that), so that those who monitor the safety of medicines on the public's behalf receive the information they need as quickly as possible.
 - That patients should speak openly with a healthcare professional if they are considering not using, or stopping taking, the treatment the doctor has prescribed. Patients may have good personal reasons for doing so, but should ensure they understand why the treatment was prescribed and any impact that not taking it may have.

- That a decision not to take a medicine should always be communicated back to the practice so that medical records are accurate.
- That doctors will be respectful of individuals' healthcare decisions.

Hospital consultants and other hospital doctors, nurses and pharmacists

- People in hospital may not always be interested in the medicines that they are given, just in feeling better. Also, some people may not feel emotionally up to taking in a lot of information at the time and hence go home inadequately knowledgeable and/or prepared. As with GP practices, hospital personnel should audit how information is provided. They should also make it a policy to encourage patients to ask questions and ensure that they and/or their carers are aware of how to access further information once they leave hospital.
- Any existing research and, where needed, new research should be compiled to find out how clearly those leaving hospitals with a treatment regime to follow once at home, understand their medicines.
- Professional bodies, always including their lay representatives, should develop workshops for health professionals working in specific disease areas, led by relevant voluntary health groups with the necessary knowledge and experience to:
 - Improve recognition of the treatment issues different patients are likely to have.
 - Identify important information that does not seem to be communicated as well as it should or which is most likely to be misunderstood.
 - Help doctors and nurses work constructively with patients whose firm views about their treatment may not be considered clinically ideal.

These workshops should be accredited and the voluntary groups should be paid by the respective health departments of the devolved administrations as service providers.

Information

- **All health sectors** should actively support the Department of Health pilot project work known as the **Information Prescription**, with a view to it being taken up throughout the UK. This would mean that every patient who receives a diagnosis of a medical condition would be equipped to find quality information and local support. Issues about the Information Prescription that still need to be resolved should not be allowed to result in loss of momentum or lessening of DH commitment to the project.

All health sectors need to ensure that people with disabilities, special needs, reading and language limitations and, where applicable, their carers, have access

to the information tools they need to participate in their own day to day care and in healthcare decisions. Those involved in the direct provision of healthcare need to make sure they know where such tools are found.

- A **“READ YOUR PATIENT INFORMATION LEAFLET!”** message needs to be widely publicised, particularly at the pharmacy. (This ties in with the “Talk to me, I’m a pharmacist” message raised further on.) Progress is being made in the readability and relevance of Patient Information Leaflets (PILs) at the UK and EU regulatory level, within the pharmaceutical industry as a whole and in the individual companies working with user panels. But this work will be of limited value unless people realise **how important it is to read the leaflet** and ask questions where they need clarification or advice. Patient Information Leaflets should be provided to patients when they are hospital inpatients.
- Where quality research does not already exist, **research needs to be done to identify the reasons why a significant number of people do not take medicines** that are essential for them to maintain reasonable health and sometimes even preserve their lives (eg anti-rejection medicines for those with organ transplants). Discussions between healthcare professionals and patients as well as educational materials/activities need to be informed by the results from such research, so as to help people avoid unnecessary health crises.
- **Pharmaceutical companies** should create a dedicated section on their websites where all the information available to members of the public can be accessed easily. This would include (or provide links to) the Summaries of Product Characteristics, Patient Information Leaflets, European Public Assessments Reports (EPAR), IFPMA Clinical Trials Register, Medicines Guides, Ask about Medicines, along with the other information companies are permitted to provide. This should also include a brief explanation of the different types of information provided and, possibly, what cannot be provided by the company and why.

Language

- With the **MHRA** in the lead, patients, healthcare and health information professionals, the pharmaceutical industry and the medical press, working together, should develop and promote a common language of benefits and risks capable of translating complex concepts into straightforward, “real life” terms.
- A coalition campaign should be conducted, with the **Medical Journalists Association** in the lead, to encourage journalists to make use of this language and to do their best to ensure that headlines (often written by other staff members) properly reflect the story.

- **Patient organisations**, with the necessary external support, should develop advice to doctors, practice nurses, and pharmacists regarding specific conditions ie:
 - What information patients are likely to take in (or not take in) about benefit and risk,
 - Common misapprehensions and misunderstandings about a condition and/or its treatment,
 - How life circumstances/lifestyle factors and cultural perspectives may contribute to people using the treatment incorrectly or not at all.

And with more broad-based health information and communication bodies on

- Commonly used medical language that may mean different things to different people or which may not adequately convey the importance of the information being provided.
- **“I'm a pharmacist - talk to me” campaign**

The public is still largely unaware of the intensive specialised training in medicines that pharmacists undergo - more training about medicines than any other group of health professionals.

Pharmacists have a crucial role to play in helping people to understand their medicines and thus use them safely and effectively. Good work is already being done to encourage the public to talk to their pharmacist. But without an appreciation of the extent of pharmacists' expertise, people are less likely to seek their advice or express their concerns.

The Royal Pharmaceutical Society of Great Britain, along with other pharmacy organisations, in conjunction with industry and other health bodies should develop a high profile campaign to raise awareness of the enormous information resource pharmacists can provide.

Industry and clinical trials

The pharmaceutical industry needs to look closely at every aspect of its work to ensure meaningful patient involvement.

This is particularly so in the area of clinical research where trials designed mainly for regulatory purposes, usually without patient involvement in their development, may bear little relation to the use of the medicine in the real world.

MHRA/EMEA communication with the public

Regulators should ensure that systems are in place so that decisions and public announcements about medicines are always informed by those who will be directly affected by them. In addition, how and why different groups of people may react to particular public announcements needs to be considered from the outset and built into communication plans.

Much good practice is already in place (patient and public involvement process and risk management plans to allow useful but potentially risky medicines to be used by the people who need them), but there is still much to be done to have a good understanding between regulators and the public about how benefit/risk decisions are made.

Given that occasionally new safety information may make it necessary for very rapid decisions to be made, a formal but fast response consultation process could help ensure that such decisions are clearly communicated, reflect patients' needs and, where likely, avoid panic.

MHRA has agreed these Key Messages about medicines (developed as part of the work of the Long-term Leadership Strategy communications group)

Overall balance between risk and benefit

- There are risks associated with every medicine; no medicine is risk free.
- Nobody wants to have to take a medicine. However when we have to, we need to be satisfied that the benefit outweighs the risk.
- Regulators have to decide whether the benefits outweigh the risks before giving a licence to a medicine. The regulators base this decision on reliable information and rigorous testing.
- Once a medicine is available to patients, they have the final say. They decide for themselves whether the benefits outweigh the risks. This is because everyone is different and preferences vary. The health professional treating them can help them to reach this decision.
- Both the known benefits and the risks of a medicine change over its lifetime. This is because the more it is used, the more we know about it.

Testing of medicines

- Medicines have to be licensed by the Government before they can be prescribed. To get a licence all new medicines have to go through a testing process which is strict and detailed.
- The testing process takes a long time and is expensive. On average, it takes about 10 years to develop and test a new medicine.

Laboratory testing

- Before a medicine is tested in humans, it is tested in the laboratory and/or on animals. This is called **pre-clinical testing**, and gives us greater confidence in its likely safety in humans.
- The vast majority of medicines that are tested in this pre-clinical testing go no further. This is because it is found that they are likely not to be safe and effective in humans.

Testing in humans

- If the pre-clinical testing suggests that the medicine is safe, it is then tested on humans. These tests are called clinical trials and they have an excellent safety record.
- However, no process is without risk. This means that there will always be some risk when a new medicine is given to people for the first time.
- Every clinical trial is therefore independently checked before it is about to go ahead. This makes sure that any risks are as low as possible.

Medicines approval - getting a licence

- In the UK medicines have to be licensed by a government body called the Medicines and Healthcare products Regulatory Agency (MHRA). They use a detailed process to find out whether new medicines work and are acceptably safe. You can tell a medicine is licensed by its Product Licence No. This looks like this “PL/...../.....”.
- The approval process takes the form of an independent review by the MHRA. This includes looking at all the information gathered in the laboratory and clinical trials. It also involves an inspection of the factory where the medicine will be made.
- For this detailed approval process to be effective it has to take some time. This is why there appears to be a long delay before new medicines get through the approval process and can be used by patients.
- The monitoring of a medicine does not stop when it is given a licence. A drug is on trial throughout its life. The benefit and risk balance is always being looked at.

After getting a licence

- We do not know **everything** about the risks and benefits of a medicine when it is first licensed. It is possible that rare and unexpected reactions may come to light only once the medicine is used in more people.
- A well established scheme - **the yellow card scheme** - is used to find out about side-effects in medicines after they have been approved. Both health professionals and patients can report side-effects using the yellow card scheme.
- Patients can add to the information known about the medicine by making sure that they report any problems they have while taking the medicine. They can do this by filling in a yellow card, which they can get from the doctors or a pharmacy or through the website **www.mhra.gov.uk**.
- Of course, patients should also see the healthcare professional treating them if they think they are having a side-effect to a medicine.
- The Regulator and the pharmaceutical companies keep monitoring a medicine as it is used in more and more patients. They do not just react to events.

Restricting or withdrawing a medicine

- It is rare for medicines to be withdrawn for safety reasons - there is only about one withdrawal per year on these grounds. There are so few withdrawals because the approval process is so strong.
- When restriction or withdrawal of a medicine is being considered by the Regulatory Authority, there is a step-by-step process. This includes getting independent advice or publicly consulting where appropriate and where time allows.
- Before restricting the use of a medicine or ordering its withdrawal, the Regulatory Authority needs to weigh the options and take a careful decision on benefit and risk. On the one hand, harm must be minimised. On the other, the impact of the decision on society and on patients who may be benefiting from the medicine has to be taken into account. The Regulatory Authority's role is to protect public health. It can only make its decisions on an assessment of evidence in a group of patients, rather than on the experience of an individual.
- In such circumstances the Regulatory Authority must explain its decisions so that people can understand it. It must support its decisions with open, honest handling of data and clear reasons for its actions.

Switches from prescription only to over the counter status

- Switches from prescription only to over-the-counter status for a medicine are only made when the Regulator is very confident that a medicine is safe. This is based on how the medicine has performed over the time as a prescription medicine.
- A decision is made only after consultation with the wider public and with health professionals.

CONCLUSION

Balancing the benefits and the risks of medical treatment is not just about scientific evidence and doctors' practical experience. If the outcome is to be relevant for real people's lives, it needs to be seen in the light of the individual patient's needs, wishes, priorities, responsibilities, work, culture, social life and lifestyle - factors which health professionals are rarely in a position to take fully into account.

Any of these factors may influence the trade-off between benefit and risk that people are prepared to make. These factors also influence how motivated a person will be to follow, accurately and effectively, a particular treatment regime.

Patient involvement in these decisions should not be seen as being merely about catering to personal preferences. It is about whether a treatment is really suitable for a particular person, whether people are to have the opportunity to make their own decisions on what they feel are acceptable risks, given the impact of their condition on their lives. It is also about whether treatments are used safely and effectively, preventable medical crises avoided and NHS resources used wisely.

Yet the importance of the informed and involved patient, with the ability and interest to participate fully in balancing benefit and risk, remains under-appreciated. The tools needed to help people become informed and involved often go unidentified, unexplored, insufficiently promoted and, where they are available, under-utilised.

There is a wide variety of partnership activities and campaigns that stakeholders could put in place. We, the ABPI and the LTCA, have tried to capture them in this paper, but no doubt there are many more that we have missed. We look forward to further suggestions.



**Association of the
British Pharmaceutical Industry**

12 Whitehall
London SW1A 2DY

Telephone: +44 (0)870 890 4333

Fax: +44 (0)20 7747 1411

Web: www.abpi.org.uk



Registered Charity Number: 1057711

**LTCA
Long-term Conditions Alliance**

202 Hatton Square
16 Baldwins Gardens
London EC1N 7RJ

Telephone: +44 (0)20 7813 3637

Fax: +44 (0)20 7813 3640

Web: www.ltca.org.uk