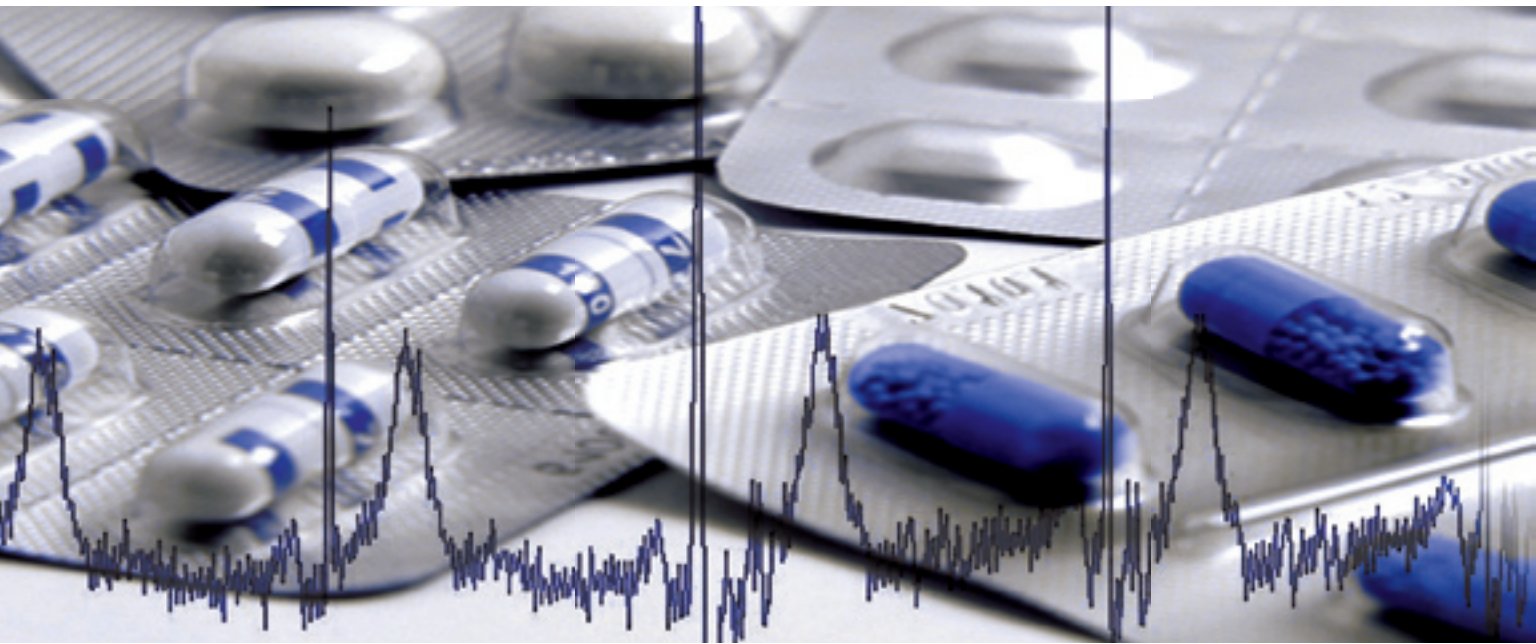


# QA

**What is  
Evidence-based  
medicine (EBM)?**

**FOR PATIENTS**





# Acknowledgements

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# What is Evidence-based medicine (EBM)?

Evidence-based medicine (EBM) is a term that has become widely used in health care settings. But, what exactly is EBM and what does it mean for you? EBM is “the conscientious, explicit, and judicious use of current best evidence in making decisions about the care of individual patients” (Haynes and Haines, 1996). The “best evidence” comes from the more than 20,000 scientific research studies that are published every year, and “patient care” involves everything from diagnosis to treatment.

EBM is not just a recipe for treatment (e.g. every 15 year-old male with depression is not automatically prescribed the same medication). It is a methodical approach to patient care that is comprised of three components: the scientific evidence, the expertise of your health provider(s), and you. EBM “integrates the best external evidence with individual clinical expertise and patients’ choice” (Haynes and Haines, 1996) to ensure that patients receive the treatment that best meets their needs. Evidence-based medicine goes beyond treatments. It also applies to how patients are assessed, diagnosed, monitored, and followed over time.

*Reference:* Haynes and Haines. 1996. Getting research findings into practice: Barriers and bridges to evidence-based clinical practice. *BMJ*. 312: 71-72.

## Evidence-based medicine and you

When we are unwell, it is often difficult to tell what has caused the problem and what can best help. Imagine that you are ill for a few days with a sore throat and cough. When your condition improves, you might wonder what made you feel better. Was it the bedrest, the cold medication, the chicken soup, or was it simply giving your body enough time to fight off the illness? And what if your friend gave you a “secret remedy” made from milk, cognac and grass? Is that why you feel better?

Before you spend time and money on a treatment, you might want the best information about whether the treatment works, how well it works compared to other treatments and what possible problems can result from the treatment. You should be aware of possible problems (such as the types of side effects that might happen, the cost of the treatment and the difficulties in taking the treatment) that may occur when you and your health care provider are deciding which treatment you should have. (Note: a health care provider could be a doctor, nurse, psychologist, social worker, etc.).

The ultimate goal of EBM is to help patients receive the treatment that is most appropriate for them. This means finding a balance between the scientific evidence, the patient's values and the experience of their health provider(s). Remember, people can respond differently to any given treatment, so it is impossible to know exactly how you may respond. However, at least you can know what the chances are that you might be helped or harmed by a given treatment. This can help you, your doctor and other health providers come to a better decision about what treatment is right for you.

Evidence-based patient choice is a very important part of evidence-based health-care. It is important for patients to increase their knowledge about their health-care options, and for them to understand that not all of the information available (for example: on the internet, in books or in magazines) is equally correct. It is important to know whether the information you find or are given is trustworthy, and to know the evidence for or against your treatment of choice. If you hear about a treatment being helpful or harmful, you should try to find out where the information came from and whether or not it should be trusted. Health providers can help you with this.

Remember, if you need medical advice, you should always seek the services of a competent health provider. Competent health providers can be those who are licensed by legally constituted regulatory bodies, such as doctors, nurses, psychologists, social workers, etc. It is much more difficult to determine the competencies of those persons who advertise their services outside of legally recognized regulatory bodies. Use such self-proclaimed "experts" at your own peril.

Don't assume you know everything about your problem or condition – even if you have looked up your symptoms on the internet or in a book. Diagnosing or treating yourself may turn out to cause more problems than you can predict. If you find information you think is relevant to your condition, take a copy to your appointment and discuss what you have found with your health care provider.

## **M**aking sense of media reports

Hardly a day goes by without news reports about the latest medical research. Sometimes the media presents the story exactly as it is, and faithfully reports the findings of a new study. Other times, the media sensationalizes, distorts or even misrepresents what the study actually found. Based on the news reports you have seen, it might surprise you to learn that scientific studies cannot prove anything with absolute certainty. Medical research is based on the concept of probability

(how likely it is that an event will or will not occur), and not on the concept of proof.

For example, a recent study reported that 70 per cent of people who took the treatment improved substantially. These findings give us an idea of the likelihood of improvement, but the findings cannot predict with 100 per cent accuracy what will happen to any individual who takes the treatment. If a treatment is effective for seven out of 10 people, you still don't know if it will work for you until you try it. However, if an alternative treatment works for nine out of 10 people, you may have a better chance of success with the alternative.

That being said, we're all familiar with the headlines – drug X causes cancer, food Y prevents dementia, activity Z induces weight loss. Perhaps these headlines should read “drug X might cause cancer in mice,” “food Y might help to prevent dementia in middle class Norwegians who are also taking aspirin” and “activity Z induces weight loss when combined with a healthy diet in some but not all people.”

The truth is, most health information is incredibly complex. Reporters do not necessarily have the time to read and analyze entire research papers, and instead may rely on short press releases for their information. Further, some reporters may not have the knowledge or experience that they need in order to critically evaluate complex scientific information. Deciding which media reports to trust and which to question is not easy, especially because the reports are not consistent and sometimes seem contradictory. One day the headlines might read “Antidepressants increase suicide risk in teens” and the next, they read “Antidepressants lead to fewer, not more, teen suicides.”

If you're wondering about the validity of a news report, it might help to ask yourself a few questions about the story. Is the story actually an advertisement for something? Is it sensationalized to make a better selling story? Is there any background information provided that might help you evaluate it better? Does the story give you different points of view, or just one? Does the report provide expert opinion from people who were not involved in the study? If the report is particularly one sided (whether very positive or very negative), you should start to wonder if it is accurate. And, if the report does not give the study results in context or in statistical probabilities, it is a good idea to question the information provided.

**If you want to follow up on a media report, there are a few things you can do:**

1. Discuss the report with your health provider to determine if it is a scientifically valid study and if the information that it provides is relevant to you.
2. Try to find the source of the information. Is the information based on a high quality, unbiased study published in a reputable medical journal? Or is it based on a testimonial or another form of non-scientific information?
3. Try to find out if there is other collaborative information from independent sources (to avoid making important health decisions on single bits of information).
4. Try to find out what experts in the field think of the new information. Sometimes studies can be controversial and not all research that is published is ultimately found to be correct.

## Making sense of websites

Information found on websites is similar to that found in the media – it can be difficult to tell which websites to trust, and which to question. Also, not all information on a website is necessarily of the same level of scientific validity – there may be different types of information on the same website. Remember to ask yourself who is responsible for the website, who wrote the article you are reading, and what the original source is. Think about what the authors or organizations have to gain or lose. Websites that are created and maintained by credible organizations and that provide access to the original research studies on which they base their information tend to be more trustworthy.

Be wary of blogs – they can be heavy on personal experience and light on scientific evidence. Generally, websites of not-for-profit organizations and governments contain more reliable information than blogs and commercial sites. And, be very careful of commercial sites masquerading as not-for-profit sites – trust organizations you know!

Do your best to make sure the information is accurate, objective and trustworthy, but remember that this can be very difficult! Deciding what is worthy of believing and what is better ignored can be very difficult, even for experts. The information should be based on the highest quality research, and should be presented in an easy-to-understand fashion. Also, be careful that the information you are reading is up-to-date (on what date was the information added to the website?). Lastly,

beware of “health myths” – watch out for misleading words like “miracle,” “breakthrough,” and “all-natural.”

Remember, if you have any questions that you can't find answers to, don't be afraid to ask your health provider(s).

## **W**hat is meant by scientific evidence?

### **(a) Types of research paper**

The scientific evidence used in medicine comes from a pool of tens of thousands of published research studies. There are many types of studies, and the design of any given study usually depends on the question that the researchers want answered. Studies can differ considerably in the way they are designed and conducted, and can therefore differ considerably in quality. Studies that stringently adhere to scientific research principles tend to be given more credibility than studies with more relaxed methods, but all study types have their own unique advantages and disadvantages.

Scientific evidence is hierarchical in nature, with study design (the way in which a study is developed and conducted) being an essential component of where the results of a study sit on the hierarchy. Study designs near the top of the hierarchy (for example: Randomized Clinical Trials – also known as RCTs) are less likely to report misleading or incorrect findings than those lower down on the hierarchy. The following is the agreed upon hierarchy of scientific evidence from the bottom (least robust evidence) to the top (most robust evidence):

Case reports → Case series → Cross-sectional surveys → Retrospective comparative studies → Prospective comparative studies → Randomized clinical trials (RCTs) Systematic reviews of RCTs

Clinical decisions are usually based on studies at the top of the hierarchy (i.e. randomized clinical trials and systematic reviews), but the information from other types of study is not useless – it all contributes to the general knowledge on a given topic, and might inform future research that employs more stringent scientific methods.

1. A *case report* is a report about one patient. For example, a case report could describe a patient whose medical problem was successfully treated by a novel treatment.
2. A *case series* is a report on several patients. For example, a case series may report on an unexpected side effect experienced by several people taking the same medication.
3. A *cross-sectional study* looks at one or more health problems in a group of people at any one time. Health surveys are common examples of cross-sectional studies.
4. *Retrospective comparative studies* (e.g. case-control studies) look back in time to determine the association between a specific health problem and what may be causing it or helping it. There are two or more groups in these studies, the people who have the health problem and the people who don't. The researchers then try to determine what factors predict who gets and who doesn't get the health problem. For example, people who have broken hips may be more likely to have used a certain type of medication than people who don't have broken hips, suggesting that the medication may increase the risk for hip fractures.
5. *Prospective comparative studies* (e.g. cohort studies) look forward in time. Two or more groups are selected, based on their exposure to something (for example a medication, a living environment or a past illness). At a later point, the groups are evaluated for a specific health problem. For example, researchers may wonder if a specific type of sleeping pill increases the risk of falls and hip fractures. Starting with a large group of people, they can study them over a period of years to see if more people taking the medication experience falls and hip fractures than those not taking the medication.

None of the above types of studies (case report, case series, cross-sectional study, retrospective comparative study) can be used as evidence to support (or refute) a particular treatment or a particular hypothesis about what causes a specific disorder or problem. However, they can be used to create ideas about what might be an effective treatment or what might cause a specific disorder.

These ideas can then be tested by other more scientifically valid studies, which are described below.

6. *Randomized controlled trials* (RCTs) are another type of prospective comparative study, but with one important difference. People in these studies are randomly allocated to a treatment or other condition by the researchers. In cohort studies, the researchers do not intervene. They simply observe what happens under “normal” circumstances, which has its advantages and disadvantages. A major disadvantage is that it can be very hard to compare people who get a certain treatment with people who do not get that treatment, even if they have the same diagnosis. The people who did not get the treatment may have a higher risk for a problem with the treatment. For example, when studied under normal use, a medication may not appear to cause hip fractures. However, the reason for this could be that doctors avoid giving the medication to people who are unsteady on their feet and are at higher risk for hip fractures, but they do give it to people a low risk for hip fractures. So, the reason the medication appeared safe is because it was given to a group of people at low risk of hip fracture and they had the same rate of hip fractures as people at high risk. In essence, the medication changed the low risk group to a high-risk group but this study design would not be adequate for determining this.

To get around this problem, randomized controlled trials have become the best way to measure the safety and effectiveness of medications and other medical treatments. Randomization means that people are allocated by chance to one of two or more different treatment groups and they have an equal chance of being put into any one of the groups. This helps to ensure that the groups are equal at the start of the treatment (i.e., they are at the same risk of having a good or a bad outcome during treatment). If a difference in outcomes is then found across groups, it is much safer to conclude that the difference is attributable to the treatment.

7. A *systematic review* is a special type of evaluation of many selected studies that have been conducted on a certain topic. For example, a researcher might gather and evaluate all of the high quality RCTs involving treatment of anxiety with selective-serotonin-reuptake inhibitors (SSRIs). These studies are sometimes called meta-analyses because of the statistical method used to combine the selected studies.

## (b) Parts of a research paper

Although studies vary greatly in design, authors tend to stick to a fairly rigid format when publishing the results of their studies. Published papers are broken into five sections, and the content of each section is quite invariable across the board.

1. The *abstract* is an overview or summary of the entire study (i.e. objective or purpose, methods, results, conclusions).
2. The *introduction* states the purpose or objective of the study, in the context of what is already known about the topic. It describes why the researchers are asking the question, and why the question is an important one.
3. The *methods* section describes exactly how the study was conducted, and in great detail. It includes information on the study design, participants and procedures.
4. The *results* section is a summary of the data that was collected and the statistical significance of the findings (i.e. how sure the researchers are that their results are accurate).
5. The *discussion/conclusions* section includes the authors' explanation of and interpretation of the results. This often includes whether or not the expected result was obtained, how the results fit with those of previous studies, and suggestions for future studies.

## Best evidence informs patient care

As you know, not all scientific evidence carries equal weight – some research is more likely to be valid than other research. The validity of scientific studies depends on many factors, including the type of study conducted, the statistical analyses used and the rigour of the external independent review of the results.

However, whatever the study design or statistical analysis, all therapeutic studies conducted in universities or hospitals, and all those that receive any federal funding must be approved by a research ethics board before the study begins. This process helps ensure that the participants of the study are sufficiently protected, and that the research meets the highest ethical standards.

### (a) Randomized controlled trials

The best evidence for safe and effective treatments comes from clinical trials (also called

clinical studies, controlled clinical trials, or randomized controlled trials). In the case of treatment, this research tells us whether a particular treatment is likely to work, how well it works compared to other treatments, and what the risks of the treatment are.

The study participants (often patients) are divided randomly into two or more groups. One of the groups is given the experimental treatment and the other is given either an alternative treatment (a previously established and known effective treatment) or a placebo treatment. Placebo treatments use “inert” compounds, which have no direct therapeutic effects. The key is that the participants must be divided into groups absolutely randomly, to ensure that the groups are comparable right from the start and that the only difference between the groups is the treatment.

At the end of the study, the differences in outcomes between the groups (both therapeutic outcomes and unwanted outcomes such as side effects) are statistically analyzed from group data. Predetermined acceptable levels of probable outcomes (that is, outcomes that are less likely due to chance alone) help guide the interpretation of the study results.

Although clinical trials all follow the same basic framework, the details can differ considerably between trials (e.g. length of study, number of participants enrolled in the study, etc.). Sometimes a study involving 50-100 people and spanning a week or two is sufficient, but sometimes many thousands of people need to be included and followed for many years. For long-term chronic diseases (e.g. cancer, diabetes, schizophrenia, etc.), many people need to be studied for long periods of time in order to reveal useful information about what is the best treatment approach. Studies with large numbers of participants often (but definitely not always) provide stronger evidence than studies with small numbers of participants.

## **(b) The preponderance of evidence**

Medical treatments are built on the preponderance of scientific evidence. The treatments with the highest levels of validity (the most certainty that they are effective and safe) are those on which large numbers of clinical trials have been conducted in different settings and in different types of patients and in which the results are generally similar.

Remember that not all studies necessarily come to the same conclusion. For example, eight studies of treatment “X” could show that it is safer and more effective than treatment “Y” while two more studies could show the opposite. The preponderance of evidence is then addressed using special statistical techniques (i.e. meta-analysis, described above). Meta-analyses (often provided as systematic reviews) then provide guidance on which treatment is more likely to be safe and effective.

### **(c) Systematic reviews**

This type of analysis is very important, because rarely does a single trial provide strong enough evidence to offer a reliable answer to a health care question. Unfortunately, for many reasons (too few participants, non-random distribution into groups, lack of a placebo treatment, etc.) the results of individual studies can be misleading or incorrect. Sometimes single studies are overly optimistic about a treatment, and sometimes they are overly pessimistic – in either case, the results of flawed studies invariably provide bad evidence. Accordingly, patient care decisions are rarely made based upon the results of a single study. Best evidence in medicine comes from careful consideration of the results of all trials that have been conducted on a given subject. For treatment or therapy questions, a systematic review is the highest level of evidence possible.

Systematic reviews are difficult and time-consuming to conduct, because they must be thorough and comprehensive – not only must the authors find all of the trials on the subject in which they are interested, they must then carefully evaluate and quality-assess every one of those trials (i.e. often hundreds of trials). Trials should only be included in a systematic review if they meet specific pre-defined criteria, and if they are of good quality. By considering the results of all of the high-quality trials and disregarding the others, systematic reviews yield an overview of the state of medical knowledge on the subject of interest.

Because the technique is a relatively new one, there are many topics that have not yet been systematically reviewed. And in some cases, even when a topic is systematically reviewed, there might not be enough good quality evidence to provide a solid answer to the question being asked. Older systematic reviews sometimes included studies that were not of the highest quality, so their results may have been affected by that fact. Today, systematic reviews provide the reader with more sophisticated methods that help readers evaluate the probability that certain treatments are safe and effective.

## Practice guidelines

It is difficult for physicians to search the literature and critically evaluate the studies that they find every time they need to make a treatment decision. For this reason, groups of experts compile the evidence so that physicians do not have to. Expert committees review the literature, decide how it should influence patient care, and then distribute recommendations in the form of evidence-based practice guidelines. Recommended interventions are often categorized as “Best-supported (Well-established) interventions” or “Promising (Probably efficacious) interventions” based on the strength of the evidence.

## Where can I find research papers?

### (a) Online databases

Online databases allow individuals to search through millions of research articles quickly. However, because many journals are not available free of charge, you might not always be able to access the full-text article through these databases. One way to solve this problem is to go to a public library, hospital library or university library. Many libraries have print and/or online subscriptions to journals, and this might allow you to access articles that you cannot access from home. However, even when the full-text version is not available, you will usually be able to read a free summary (abstract) of the article.

#### *PubMed and PubMed Central*

*www.pubmed.gov and www.pubmedcentral.nih.gov*

These databases (from the US National Institutes of Health) provide free abstracts from thousands of journals, as well as links to some free full-text articles.

#### *Cochrane Database*

*www.cochrane.org*

The Cochrane Collaboration conducts systematic reviews of the effects of healthcare interventions. Abstracts and plain language summaries are available.

#### *HighWire Press*

*http://highwire.stanford.edu*

This database (a division of the Stanford University Libraries)

includes almost five million articles, two million of which are provided free of charge.

### *MedlinePlus*

[www.nlm.nih.gov/medlineplus](http://www.nlm.nih.gov/medlineplus)

MedlinePlus provides authoritative information from the US National Library of Medicine and the US National Institutes of Health. It provides easy access to journal articles, information about drugs, a medical encyclopedia, patient tutorials and the latest health news.

## **(b) Open-access journals**

Some research papers are available free of charge in journals called open-access journals. These journals are peer-reviewed like other journals, but are available online and free of charge.

### *Open Medicine*

[www.openmedicine.ca](http://www.openmedicine.ca)

Open Medicine is a Canadian independent, peer-reviewed, open-access journal. Full articles are provided free of charge to the general public.

### *PLoS Medicine*

<http://medicine.plosjournals.org>

PLoS Medicine is a peer-reviewed, open-access journal (from the Public Library of Science). Full articles are provided free of charge to the general public.

## **Q**uestions to ask

When you have found a study, ask yourself these questions to determine whether or not the results of the study might apply to you or your family members.

### **1. What kind of study is it?**

What is the study design? Where does it fit on the evidence hierarchy? Does it provide strong evidence or weak evidence? Remember, for clinical decisions, randomized clinical trials and systematic reviews provide the best evidence.

### **2. Was the study conducted on animals or humans?**

Research conducted on humans is more likely to apply to you than research conducted on animals.

### **3. Did the study include people like you?**

Check to see if the study participants were similar to you in age, sex,

educational level, ethnocultural background, diagnosis and health concerns. The results of studies that were conducted on people who are very different from you might not apply to you.

**4. Is the study published in a peer-reviewed journal?**

In peer-reviewed journals, an independent panel of experts reviews every article before it can be accepted for publication. For this reason, research that is published on a website or in a brochure is less believable than research that is published in a peer-reviewed medical journal.

**5. Who funded the study?**

Knowing who paid for the study and what stood to be lost or gained from the results is very important. People or organizations that fund studies sometimes stand to make or lose money from positive or negative study results (e.g. if a study was funded by a company who make and sell the study treatment, a positive result would benefit the company financially). Studies funded by independent agencies may be less biased.

**6. How many people participated in the study?**

Generally, the larger the number of participants in a well-designed and well-conducted study, the more confidence we can have in the results (but, this is not always true). Also check how many people dropped out of the study (“withdrew” were “lost to follow up,” etc.) - high drop out rates can weaken the results.

**7. Do the authors discuss clinical significance?**

The authors of research studies always discuss statistical significance – that is, whether the observed result is probably real or whether it could have occurred by chance. However, clinical significance is another concept altogether. Clinical significance refers to whether the observed result will make any real difference in the lives of patients. For example, a given drug could decrease blood pressure by two points (and this result could be statistically significant), but that two-point difference might not noticeably improve a patient’s condition.

**8. Who conducted the study and where?**

Check to see that at least one of the authors is an expert in the field, and

that the authors are affiliated with reputable universities, hospitals or research institutions. Be careful of authors without any clear affiliations.

**9. Is there more than one side to the story?**

Find out whether or not the results have been replicated by other researchers. A new study that is consistent with previous high-quality studies is more convincing than one that is not. If the new study is the first of its kind, more research will be needed to confirm the result. Further, when authors report on controversial topics, they should include a discussion of the “other side” of the argument in their paper.

## Communicating with your healthcare provider

In the past, doctors gave orders and patients followed them; now, the patient-doctor relationship is more of a partnership. Patients and their families work as a team with physicians and other health care professionals to alleviate problems and to ensure the best care possible. What’s your role in this partnership? Whether the patient is you, a friend or a family member, your role is to be informed, ask questions and express any concerns that you may have. Be informed about the illness and available treatments, ask questions if your healthcare provider’s explanations are unclear, and express your concerns (even if you are not explicitly asked about them).

Appointments with your health care provider are a wonderful opportunity to ask questions and clarify issues, however, feeling rushed or stressed can make it easy to forget some of the topics you wanted to cover. Preparing for your appointment can help you get the most out of the experience.

Arrive early so that you can spend as much time as possible with your healthcare provider. Bring a list of concerns and questions, and a list of all the prescription and over-the-counter medications, herbals and vitamins that you are taking. Bring a pen and a piece of paper to take notes during the appointment, and make sure to copy down your healthcare provider’s instructions. If you think it would be helpful, bring a friend or family member along to take notes and ask questions on your behalf. Most importantly, keep asking questions until you understand your diagnosis and what you need to do to get well.

Asking very specific questions might help your healthcare provider break complicated information into easy to understand chunks, and might help you and your healthcare provider communicate better.

## Diagnosis:

- What do you think my diagnosis is?
- What else could it be?
- What does this mean for my day-to-day life? For my future?
- What do I need to do to get well?
- Where can I get more information about my condition?

## Treatment options:

- What are my treatment choices?
- What kinds of studies have been done on these treatments?
- What are the benefits and risks of each treatment?
- How likely is each treatment to help me?

## Medication:

- What does this medicine do to my body?
- What are the alternative medicines and why are you recommending this one over the others?
- What kinds of studies have been done with this medication?
- What other medicines or treatments has it been compared to?
- How likely is this medicine to help a person like me?
- What are the risks and benefits of this medication?
- Will the medicine interact with anything I am already taking?
- What are the potential side effects? Will they fade over time?
- When can I expect to see results?
- How will I know if this medication is working for me?
- How long will I have to take the medicine?
- What will happen if this medication does not work for me?
- What is the number needed to treat (NNT) of this drug? What is the number needed to harm (NNH) of this drug? (See the glossary for the definitions of these terms)

## Psychological treatments (therapy):

- How will this therapy help me?
- What are the alternative therapies and why are you recommending this one over the others?

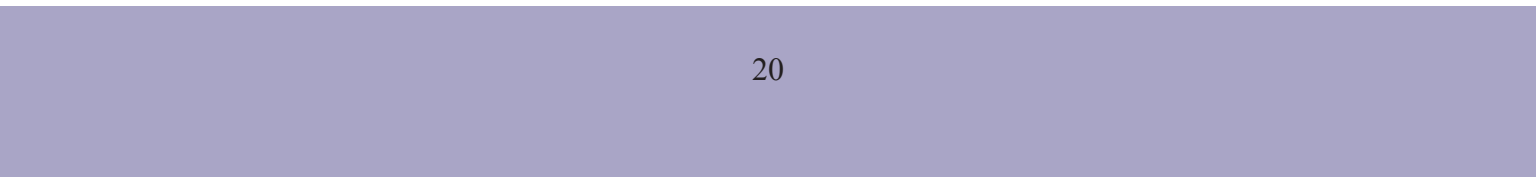
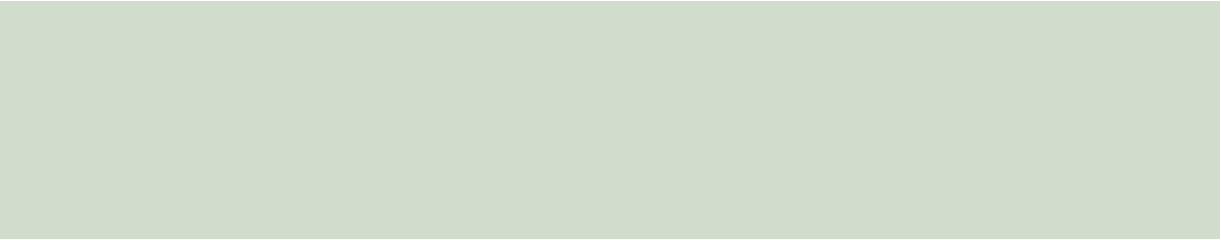
- What kinds of studies have been done with this therapy?
- What other treatments has this therapy been compared to?
- How likely is this therapy to help a person like me?
- What are the risks and benefits of this therapy?
- What are the potential side effects of this therapy? Have studies assessed possible side effects?
- When can I expect to see results?
- How will I know if this therapy is working for me?
- How long will I need to continue the therapy?
- What will happen if this treatment does not work for me?
- What is the number needed to treat (NNT) of this treatment? What is the number needed to harm (NNH) of this treatment? (See the glossary for the definitions of these terms)

## **C**hild and adolescent mental health

Evidence-based medicine is extremely important in the treatment of mental illness in general, and is particularly important in the treatment of mental illness in children and adolescents. Children and adolescents often present with a complexity of problems that should be addressed when planning treatment (e.g. youth who present with mental disorders such as Attention Deficit Disorder may also demonstrate learning disabilities or behavior problems).

Treatment decisions should be based on the appropriateness of the treatment for a specific mental disorder, the appropriateness for specific symptoms, the appropriateness when compared to other treatments, and the youth/family's acceptance of the treatment. Fortunately, the treatments available for children and adolescents with mental disorders are many and varied. Unfortunately, very few have been adequately evaluated for efficacy in this population. There are many gaps in the scientific literature, and the research that does exist varies greatly in quality.

It is important for young people and their parents to know which treatments have the best evidence to support their use. As a "rule of thumb", it is usually better to use a treatment that has evidence from randomized controlled trials to support it over trials that have less rigorous evidence to support them. However, it is also important to remember that "absence of evidence is not evidence of absence." It could be that the best treatment for you or your child may not have yet been subjected to the highest standards of scientific evaluation. This is another reason why it is essential to have an informed discussion with your healthcare provider – to find out not only what the best evidence is, but to jointly decide on what the best treatment for you might be.



# Glossary

## **Adverse reaction/effect**

- a harmful or unpleasant reaction resulting from the use of a treatment
- adverse reactions/effects predict risk upon future administration, and warrant withdrawal or alteration of the treatment

## **Adverse effect**

- a harmful or unpleasant reaction resulting from any intervention (not just medication)
- predicts risk upon future administration and warrants withdrawal of the treatment or alteration of the dosage regimen

## **Bias**

- the presence of systematic error in a study (error that does not occur by chance)
- bias can occur at any point in a study (study design, data collection, data analysis, etc.), and is the result of researcher error
- bias undermines the validity of the study results

## **Blinding**

- a method used to minimize bias (i.e. systematic error) in research
- when blinding is used, information about which group participants have been assigned to (i.e. treatment group or placebo group) is concealed from participants and/or the researchers
- single blind: information is concealed from the participants
- double blind: information is concealed from the participants and the researchers

## **Case-control study**

- a type of study that looks backward in time (i.e. retrospectively) at participants' exposure to a potential risk factor
- these studies compare the exposure of participants who have a disease (cases) to the exposure of non-diseased participants (controls)

## **Case report**

- a report on a single patient with an outcome of interest

## **Case series**

- a report on a series of patients with an outcome of interest

### **Clinical trial**

- a research study that tests a treatment (drug or other therapy) for effectiveness, safety and tolerability
- these studies involve at least two groups of participants – one group receives the experimental treatment, and the other group receives either a previously established effective treatment, a placebo treatment or no treatment at all
- clinical trials are also called controlled clinical trials (CCTs) or randomized controlled trials (when patients are randomly assigned to treatment and comparison groups)

### **Clinical practice guideline**

- a systematically developed, evidence-based statement that is designed to advise the decisions of health care professionals and patients about appropriate health care

### **Cohort study**

- a type of study that looks forward in time (i.e. prospectively) for participants exposure to potential risk factors and for an outcome of interest
- these studies compare the outcomes of participants who were exposed to a potential risk factor to those of participants who were not exposed

### **Conflict of interest**

- occurs when the researchers involved in a study have financial, professional or other interests that could affect the design, conduct or reporting of the research
- includes “actual” and “potential” conflicts, and arises when the outcome of the research can benefit or harm the researchers in some way
- remember to check the “conflict of interest” statements in the research papers you read – researchers should disclose any actual or potential conflict of interest

### **Control Group**

- one of the groups of participants in a study
- this group may receive either a standard (or previously demonstrated effective) treatment or a placebo treatment (an inactive substitute)

- this group is compared to the experimental group (which receives a new treatment), to measure the effectiveness of the treatment

### **Crossover study design**

- a type of study in which more than one treatment is administered to the same group of participants in a successive fashion
- two problems can occur: carry-over effects (the effect of one treatment carries over into the second treatment period) and order effects (the order in which the treatments are administered may affect the outcome)

### **Cross-sectional study**

- a type of study in which a population is observed over a specific time interval or at a single point in time (often referred to as a “snapshot” study)
- the participants’ exposure to potential risk factors and development of the outcome of interest are determined simultaneously

### **Double blind**

- studies in which neither the participants nor the researchers know the group (control or experimental) to which the participants were allocated (until the end of the study)

### **Effect size**

- the estimate of the magnitude of the observed effect in a study
- generally, the larger the effect size, the greater the effect of the treatment

### **Epidemiologic Studies**

- studies that look at the percentage of people who are affected by a particular outcome of interest (e.g. disease, disorder, occurrence, etc.)

### **Epidemiology**

- the study of the distribution, determinants and deterrents of morbidity (illness) and mortality (death)
- that is, the study of who gets sick or dies, where they get sick or die, and when they get sick or die (distribution), what causes people to get sick or die (determinants) and what prevents these outcomes (deterrents)

### **Experimental group**

- one of the groups of participants in a study
- this group receives the new treatment that is being studied
- this group is compared to the control group (which receives a placebo or a previously established effective treatment) in order to measure the effectiveness of the new treatment

### **Gold standard**

- a method, procedure or measurement that is widely accepted as being the best available
- other methods, procedures or measurements are often compared to the gold standard

### **Incidence**

- the number of new cases of a disease/disorder that arise during a specified time period
- incidence can be expressed as an incidence rate (i.e. the number of new cases that arise in a defined population over a specified time period), or as an incidence proportion (i.e. the proportion of the people “at risk” in a defined population who will become new cases over a specified time period)

### **Informed consent**

- participants are informed about all aspects of the study (purpose, procedure, risks, benefits, etc.) before deciding whether or not participate in the study

### **Longitudinal study**

- a type of study in which a population is observed at more than one point in time, so that the researchers follow the participants forward in time

### **Meta-analysis**

- a type of study that combines the results of individual studies into one result to get an overall view of the effectiveness of a treatment
- assuming the methodology is sound, they provide very good evidence for or against a given treatment

### **Number needed to treat (NNT)**

- the number of patients who would need to be treated with the experimental treatment in order to achieve the desired outcome in one patient

### **Number needed to harm (NNH)**

- the number of patients who would need to be treated with the experimental treatment in order to achieve a harmful outcome in one patient
- the definition of harmful outcome depends on the treatment, and can range in severity from an unwanted side effect to death

**Observational study**

- a study in which participants are observed (or outcomes are measured) without any intervention by the researchers
- no attempt is made to affect the outcome (no treatment or intervention is given to participants)
- changes or differences in one variable (e.g. whether someone smoked) are studied in relation to changes or differences in another (e.g. whether they died)

**Odds**

- using odds is one way to express the likelihood of an event (another way to express likelihood is using probability)
- the odds of an event are calculated by comparing the probability that an event will occur to the probability that the event will not occur
- for example, if four out of five patients achieve complete remission of symptoms after the administration of a given medication, the odds of full remission would be four to one
- therefore, the odds of an event that is certain to happen are infinity and the odds of an impossible event are zero

**P-value**

- expresses the probability that the results of a study (or results even more extreme) could have occurred by chance
- p-values range from zero to one
- the p-value helps determine whether the results are statistically significant or not (the convention is that if  $p \leq 0.05$  the result is statistically significant, but sometimes a more stringent criterion of  $p \geq 0.01$  is used)

**Placebo**

- a sham, fake, or inactive treatment/intervention received by the participants in the control group
- indistinguishable from the active treatment or intervention received by participants in the experimental group

**Practice guideline**

- a systematically developed statement designed to help clinicians and patients make decisions about appropriate health care for specific clinical situations
- expert committees review the scientific literature, decide how it should influence patient care, and then distribute recommendations in the form of evidence-based practice guidelines

**Prevalence**

- the total number of existing cases of a disease/disorder in a defined

- population over a specified time period
- can be expressed as a prevalence rate (proportion of a defined population with a disease over a specified time period), or as point prevalence (proportion of a defined population known to have the disease/disorder of interest at a particular point in time)

### **Probability**

- using probability is one way to express the likelihood of an event (another way to express likelihood is using odds)
- probability is the chance of an event occurring, and is expressed as the the number of actual occurrences divided by the total number of possible occurrences
- probability is expressed as a decimal between 0 and 1
- for example, if four out of five patients achieve complete remission of symptoms after the administration of a given medication, the probability of full remission would be four divided by five, or 0.8

### **Prospective study**

- a type of study that follows participants forward in time
- participants are exposed (or not exposed) to an intervention of interest, and then observed over time to determine how effective (or safe) the intervention is

### **Randomization**

- when a randomization procedure is employed, the participants in a study are assigned to groups randomly
- this means that every participant has an equal chance of being in the treatment group or in the control group
- this procedure assures that any differences in outcomes between the groups are due to the treatments alone and not to other factors

### **Randomized controlled clinical trial**

- a study in which participants are randomly assigned into at least two groups, an experimental group and a control group
- the experimental group receives the new treatment, and the control group receives the standard treatment (a previously established effective treatment) or a placebo treatment
- these studies demonstrate whether the treatment works, its risks and how well it works compared to other treatments

**Retrospective study**

- a type of study that looks backward in time in order to determine whether there is an association between an outcome of interest and an exposure (which may be causing or helping the outcome)
- these studies include two or more groups of participants – the people who have the outcome of interest (often a health issue) and the people who do not
- using these studies, researchers try to determine what factors predict who gets and who does not get the outcome of interest

**Risk factor**

- anything that increases a person's chances of getting an illness (can be aspects of a person's health, lifestyle or environment)
- remember, risk factors *increase a person's chances of getting* an illness – they do not *cause* the illness

**Safety**

- the potential of a treatment or therapy to cause serious adverse effects

**Systematic review**

- a systematic evaluation of many highly selected studies that have been conducted on a certain topic
- is sometimes called a meta-analysis because of the statistical method used to combine the selected studies

**Single blind**

- studies in which the participants do not know the group (control or experimental) to which they have been allocated (until the end of the study)

**Tolerability**

- the potential of a treatment or therapy to cause unpleasant adverse effects
- these effects are not medical emergencies, but can affect quality of life and willingness to continue the treatment

**Variable**

- a measure that can vary within a study
- can refer to participant characteristics (age, sex, etc.) or to the main variables of the study (i.e. those that are controlled/manipulated by the researchers like medication type or dose, and those that are measured by the researchers to try to gauge the effect of the treatment, like resolution of symptoms or appearance of side effects)





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