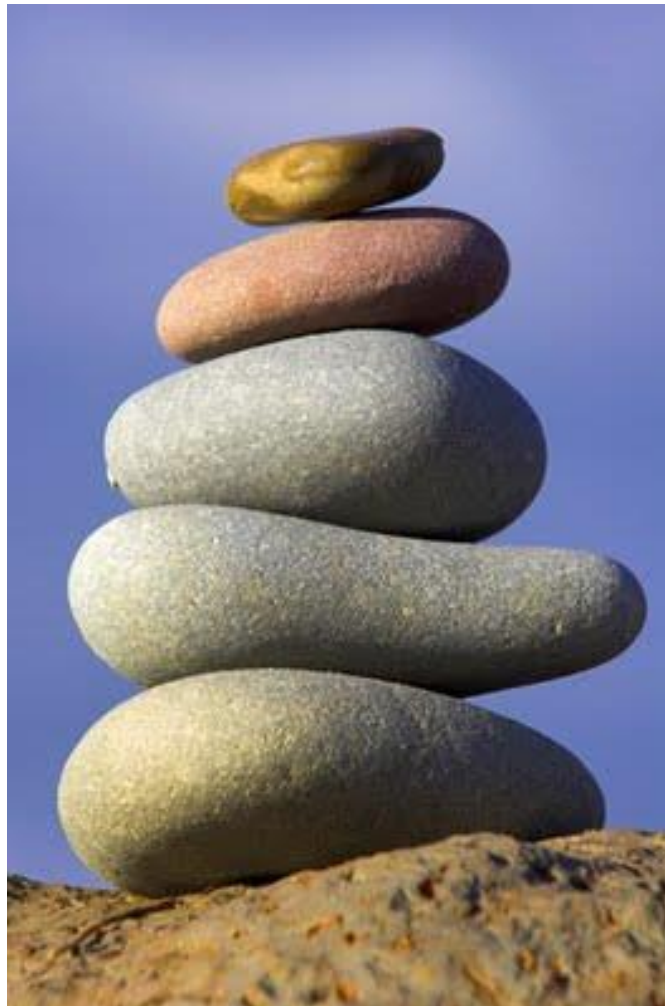


## **YOUR TREATMENT DECISION GUIDE**

**A step-by-step process to help patients think about the positive and negative effects, and the uncertainties, of treatments**



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- members of three focus groups held in 2014 in Toronto to review an early version of the Guide
- funding in 2015 from Merck & Co., Inc., through CNETS Canada, that allowed us to develop a plainer version of the guide
- plain language editor, Debra Isabel Huron.

## LEARNING ABOUT THIS GUIDE

The people who wrote this guide are patients, like you. They have a disease or health problem that affects them in ways that change everything. They have had a lot of experience learning about their disease or health problem, and about the healthcare system.

They know it can be difficult to choose from among treatment options.



This Guide is called a Treatment Decision Guide because it is based on an approach that uses the science of treatment decision-making in the health field (see Book #1 listed in Appendix 4).

This Guide will help you ask clear and focused questions of people on your healthcare team, and get answers from them.



The Guide will also help you learn more about how Canada regulates drugs and products used in medical treatments (see Appendix 3). We hope the Guide will give you a clearer picture of treatment options, no matter how experienced a patient you may be.

## ***Who will benefit most?***

Patients with a chronic, serious or life-threatening disease or condition, and the people who care for them, will benefit most from this Guide.

The Guide does not talk about any one disease or condition. It can be used by people who have any kind of chronic or life-threatening disease or health problem.

## ***How will this Guide help you?***



First, the Guide invites you to review your thinking. Steps 1 to 3 allow you to explore your values and needs as they relate to your disease or health problem.

Starting at Step 4, you will begin to look at treatment options based on personal knowledge — what has meaning to you, and reflects your own needs and values.

Steps 5 to 11 take you deeper into the factors that will play a role in the decisions you make about treatments.

The Guide will also help you think through the possible outcomes of your choices and what these could mean to you in the longer term.

## ***When should you use the Guide?***

Use it early and often!

We suggest you start using the Guide after you have

- heard the diagnosis
- had time to understand the news
- had time to adapt.

You need to be ready to think carefully about your treatment choices. This means you should have some idea of what the options are for treating the disease or health condition. Your doctor and other healthcare providers will be able to outline these for you.

If you have been living for a long time with a disease or a health problem, and changes to treatment are happening, this Guide can help you decide what to do if

- new treatments are being offered to you
- you decide it is time to rethink your treatment options.

### ***How should you use this Guide?***

Be gentle with yourself.

Take small steps, and not too many at once.

You may want to have someone you trust go through the Guide with you. They could read the questions. You could think about answers that are true for you. Talking about things can help you feel more comfortable. We suggest you take your time.

Taking a break between steps will probably help, too. Steps 5 to 11 are more complex than the earlier steps.

You will need time to digest your thoughts and feelings. If you always stay within your comfort zone, you will get the most out of this experience.





*"Parkinson's disease cut my writing days short, so I turned my creative talents back to my love of watercolor painting. I paint whenever I can and try to find the luminous color that underlies each day.*

*"My advice to others: slow down and look for rainbows in mud puddles. Think big." —Sheila Moriarty, Roseville, MN*

## ***What can you expect in each step of this Guide?***

Each step begins with a brief statement about the purpose of the step. It appears in blue text.

This is followed by main questions for you to think about. These questions appear **in bold and black.**

**Possible answers** appear next. This text invites you to think about specifics. Sometimes, we ask you more questions. The goal is for you to dig deeper into your own mind and heart. Having someone you trust and who supports you can be helpful as you move through this part of a step.

### ***Personal Stories***

*The people who worked to create this Guide are also living with a life-threatening disease or a chronic health problem. They agreed to share bits of their experiences in boxes with green text. We do not include their names, but all their stories are true.*

This kind of box contains extra information we think is useful.

It may send you to a website, or it may give you a definition or summary of information.

### **TRUE, FALSE or MAYBE?**

A box like this appears at the end of most steps. Each box contains a few statements. Your job is to decide whether to answer TRUE, FALSE or MAYBE to the statements.

Appendix 2 provides explanations for some of the answers.





# THE STEPS

## STEP 1: SETTING MY GOALS



Step 1 invites you to think about what matters most to you about your health. Being clear about this at the start will help you define what you really **need**, in contrast to what you would **like**.

**When I think about the disease or health problem that affects me, what are my main goals?**

### Possible answers

I want to:

- live as long as I can
- have good quality of life as much as I can, and “feel better”
- return to work
- be more independent in my day-to-day life.

*“All patients hope that what their doctor has prescribed will help them return to their previous life, before they received their diagnosis.”*

*“I think that one of the first thoughts that goes through anyone’s mind when they hear the words “you have cancer” is: can you cure me? A cure is, of course, ideal, but in the absence of a cure, “stable disease” (in other words, cancer that has stopped growing) is an excellent outcome.*

*“In my case, when I have stable disease, I remind myself, ‘I do not know what tomorrow will bring, but today, I can do anything that I would have done if I did not have cancer.’”*

We suggest you provide answers to these questions:

What health goals are important to me?	
If I had to choose, which goal is my most important health goal? Why?	
When I think about my other health goals, how would I list them so that the last goal on the list is the least important to me?	

**Can I break down my thinking and feelings about my disease or health problem into parts so that each one becomes a specific health goal?**



## Possible answers

The aspect of my disease or condition that bothers me most, and that I most want to reduce, get rid of, or manage is:

- how the disease grows or progresses
- symptoms of my disease that bother me the most, such as
  - pain
  - nausea or vomiting
  - problems being mobile
  - sleep problems
  - problems with memory
  - thinking problems
  - feeling tired (fatigue)



*“My rheumatologist is very concerned about any joint that is swollen and inflamed. I have lived with swollen joints for over 25 years, and this does not bother me as much as the flu-like symptoms that come when I have a disease flare-up. Even though this is incapacitating, my doctor ignores this aspect of my disease.”*

## What can I do to achieve my goals?



*“Rheumatoid arthritis can be very severe or very mild. Someone who has a very mild form may decide not to take any medication and will treat the disease, often successfully, with complementary or natural health products. Someone with a severe form of the disease may be prepared to take more risks with medications that have serious and sometimes dangerous side effects since the effects of the disease can be severe and life-threatening.”*

### Possible answers

- a) I can make an effort to learn about and understand my disease or health problem and the treatment options that may work best for me.

Sources of information on health include: family doctor; medical specialist; hospital; pharmacist; the Internet (websites such as Health Canada, Food and Drug Administration (U.S) and the European Medicines Agency); public library; social worker; community health centre; the ministry of health in my province or territory, and charities.

- b) I can use the time I have with my doctor, nurse, pharmacist and dentist by asking focused questions that will give me a clear understanding of my treatment options. I can also tell them what I think and feel so they know about my medical situation and needs.

*“Once the doctor told me I had cancer, I didn’t hear anything after that. At that time, I was living in a fog after two difficult life events in the same month. My health care team was able to help me recognize that this was an extraordinary time of stress. They helped me find other resources to help in the decision-making process.*

*“They also helped me find a counsellor to talk to so that I could find more ways to cope with both my body and my emotions. I was finally able to choose treatments based on knowing that it was the best fit for me, not just one I would have picked while living in a fog of stress.”*

- c) When I begin treatment, I can report my experiences with my treatment as it happens so that my healthcare team can adjust things to give me the best care.
- d) I can build relationships with others, such as patients in support groups; this may help me to make decisions over time.



*“Do not be afraid to get a second or even third opinion on your diagnosis and treatment options. Medicine is not a one-size-fits-all practice. Find a doctor you are comfortable working with — this will hopefully be a very long-term relationship!”*

*“A doctor gave me the best description of the modern doctor-patient relationship. He said a doctor is the captain of your ship, but you are the ship's owner.”*

*“I had never even heard of hereditary angioedema. I did not know what to expect, ask, feel, or who to turn to for help. There just seemed to be a huge maze of terminology that I did not understand. Worry consumed me at first.*

*“Then I learned that the best way to be empowered was to keep asking questions.”*

**TRUE, FALSE or MAYBE?**

- 1) I have little say in how my disease is managed.
- 2) Everyone with the same disease has the same concerns, or feels the same way, about their disease.
- 3) There may be a number of different treatment options to manage my disease.
- 4) At the physical level, everyone with the same disease or health problem responds in the same way to the same treatment.

See Appendix 2 for explanations.

## STEP 2:

# DISCOVERING MY THINKING



Who are you? Why do you think and decide the way you do?

Step 2 will help you understand more about what lies behind your reactions and judgments. This may help you to think more clearly when the time comes to decide on treatments.

- **Am I willing and able to think clearly now?**
- **What exists in my situation, or in me, that affects my thinking?**
- **Are the influences that affect my thinking truly meaningful to me?**

## Possible answers

Dig a bit deeper by asking yourself these questions:

- Do I have enough energy and focus to make this effort right now, or should I ask for support?
- Are my circumstances complicated, and do I want to ask for help thinking things through?
- Who might be able to help out? Helpers may be healthcare providers, a caregiver, a patient navigator, my patient support group, or a patient advocacy group.



Generally, what tends to affect my thinking and decisions? Some **factors** may include:

- Where I am in life: my age, male or female, culture I am part of, lifestyle, family
- My emotions: fear, hope, frustration, feeling desperate
- How others around me feel — my spouse, partner, children or parents
- My own and other people’s assumptions about the situation of having a life-threatening disease or chronic health problem.

*“When I was first diagnosed with HIV I asked my doctor about joining a clinical trial, since there were no effective treatments yet available for my disease. She recommended one. I thought, wrongly, that it must be a good drug being tested or my doctor would not have recommended it. Then I spoke to a woman in my support group who knew a lot about treatments and the trial. She warned me that the drug was very toxic and better drugs were coming to trial. I am sure glad I waited.”*

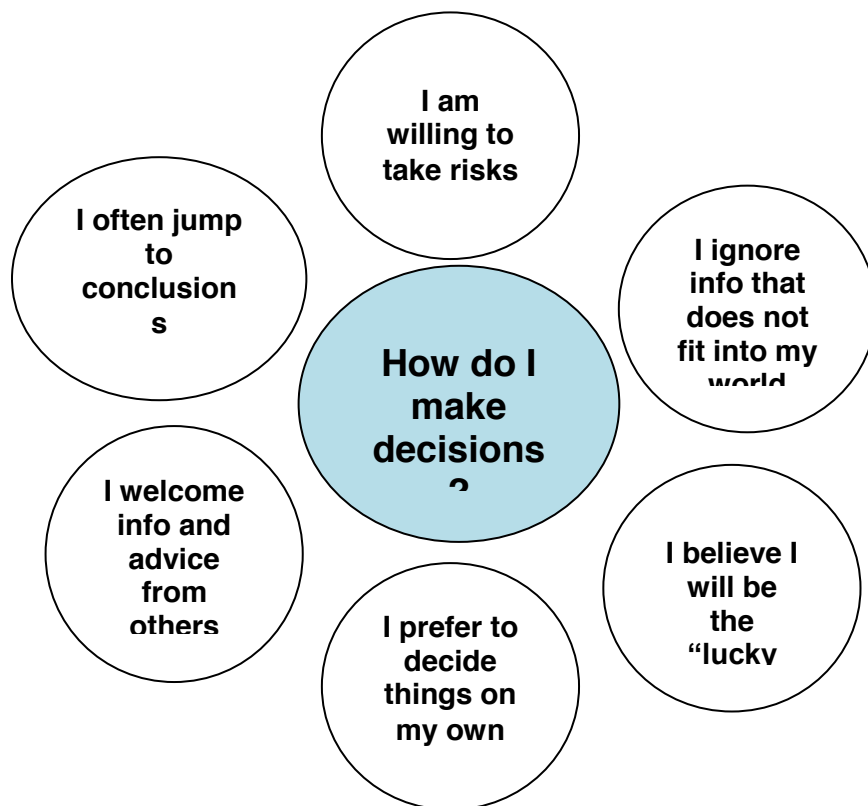


When I think about **the factors** above, which ones have the most meaning and are most important to me? Why?

*“I know I have to make some decisions about my treatment. How do I do this when no one in my family wants to have a real conversation about this? Everybody is trying to protect everybody.”*

*“With the help of the medical team we were finally able to have an open conversation about what we were each scared of. Once we could do that I was able to come up with a treatment plan that worked for me and my family. While the worry will never completely go away it helps that we all know what to expect with this treatment plan.”*

Put a check mark inside the circle or circles that describe you.



Please ask yourself a few more questions:

- Do I tend to like certain options more than others because I want to avoid regret later on?
- Do I tend to dismiss the importance of things that may happen in the future more than things that might happen now or soon?
- Generally, am I comfortable with uncertainty? Why or why not?
- Do I tend to listen to, or do I tend to ignore, my “gut feelings” or intuition?

Please take time to think about structure and numbers.

- Do I like to take a structured approach when I am making important decisions?
- Do I like or dislike numbers?
- Do I understand the numbers that explain the possible positive and negative outcomes of a certain treatment?
- Should I seek help to understand these numbers?



To test how well you understand numbers that explain the benefits and harms of a treatment, do the online **Medical Statistics Quiz** from Dartmouth Medical School (PDF will load).

LINK TO BOLD TEXT ABOVE:

[http://dartmed.dartmouth.edu/spring08/pdf/disc\\_drugs\\_we/quiz.pdf](http://dartmed.dartmouth.edu/spring08/pdf/disc_drugs_we/quiz.pdf)

Find answers to the quiz **here**.

LINK TO BOLD word "here."

[http://dartmed.dartmouth.edu/spring08/html/disc\\_drugs\\_we\\_answers.php](http://dartmed.dartmouth.edu/spring08/html/disc_drugs_we_answers.php)

Here are some final points to consider:

1. Critical thinking is a skill that people can learn. It involves deciding whether a claim is true, partially true or false. Do I enjoy critical thinking? If not, do I know someone who can do this and will help me?
2. How important is peace of mind to me?



3. Can I see and separate out my short-term goals (things I must handle now, like pain), medium-term goals (such as gaining better control of my mobility) and longer-term goals (such as being able to return to work)?

4. Can I take the long view (decide what will be in my best interests far into the future)?
5. Whose advice do I trust when I must decide something? Why? Do they understand me and have my best interests at heart?



6. Have my thinking or have my personal circumstances changed so that I need to rethink a treatment decision I made before?

*When I was single with no small children relying on me, I would not have been prepared to risk the uncertainties and the burden from possible toxic effects from an unproven treatment. But now that I have a young family, I would be more open to being part of a clinical study and maybe exposing myself to test treatment, if nothing else is available to treat the disease, in the hope that I just might be able to survive or maintain quality of life, to support my children while they are*

#### TRUE, FALSE or MAYBE?

- 1) I should not let my emotions affect decisions about my healthcare.
- 2) I have a clear understanding of how well a licensed (approved) treatment will work and how safe it is for me.
- 3) I am good at understanding the numbers that describe the potential for benefit and for harm that may come from treatments.
- 4) Knowing that I may be biased in some of my thinking, or misled by false beliefs, will help me make better decisions.
- 5) Taking time to think about my medical decisions will help me make better decisions in the end.

See Appendix 2 for explanations.

## STEP 3: SEEING MY OPPORTUNITIES



Step 3 offers you a set of guiding principles for making wise treatment decisions.

- 1) **How can I choose treatments that will help me achieve my own health goals?**
- 2) **Who can help me (or provide information) so I can make the best decisions about if, when and how to take treatments?**



*"Friendship is born at that moment when one person says to another: "What! You, too? I thought I was the only one." –C. S. Lewis, author of The Chronicles of Narnia*

## Possible answers

Take time to decide on the questions and concerns you should raise at medical appointments, and with healthcare staff.

Make it your goal to learn all you need to know about your treatment options.

It is up to you to plan ahead, so you can get the most out of your health visits.

*“Most of the time, it seems that we are pretty powerless to change things. As long as we are alone it might stay that way, but when we find others like us, together we can change our world.”*

*“Join a support group, or consider starting one!”*



*“I, as the patient and person taking the medication, know how it is affecting me and should never allow a healthcare provider to dismiss my concerns. If I feel a drug is doing something different than expected I should mention it to my provider and not allow them to ignore what is happening.”*

*“When I was first diagnosed with a rare disease it was very difficult to come to terms with the lack of knowledge about the illness and the uncertainty about my future. When we finally came together and formed a patient group, we were able to get past being angry at how the system was failing us. We began to work with the doctors and healthcare centres to come up with some pretty smart ways to get better care, not just for ourselves, but for all patients with our illness. It has been very empowering.”*

### TRUE, FALSE OR MAYBE?

- 1) Everyone with the same condition feels the same way about the possible benefits and harms of a certain treatment.
- 2) People with the same disease can have very different life circumstances or values that they need to consider when they are making choices about their health and treatment options.
- 3) Once a treatment is licensed, people tend to overestimate its possible benefits and underestimate its possible harms.

See Appendix 2 for explanations.

## STEP 4: LAYING OUT MY TREATMENT OPTIONS



Now that you know more about what you need and why, Step 4 asks you to explore treatment options. Asking your doctor — and others on your health team — is the best place to start. You may also find it helpful to talk to people who share your disease, through a patient support or advocacy group for your disease or condition.

You may wish to write down your options as you go through the rest of the Guide.

**What are the options for treatments?**

**Where can I go to find out more about the options that apply to me?**

*“After I was diagnosed I expected the doctor to direct me to the next steps, lay out the treatment plan and tell me what to do. Instead I was presented with a range of options. How was I to know which was best? I did not go to medical school!”*

## Possible answers



*“There are different ways to take medications for rheumatoid arthritis. Some are in pill form, others are self-injected, and others given by an intravenous tube (IV) at a clinic. Some patients do not like needles and prefer to take their medicine as pills. Others are comfortable injecting the medicine. It makes them feel like they have some control and are doing something to manage the illness. There are others who require the help of health professionals to manage their disease.”*

- a) There is usually more than one approach to treatment. My healthcare team may offer me medicines along with other options — surgery, exercise, diet — and different ways to receive the treatments.
- b) Treatments can be combined. Treatment approaches can also be combined. Do I know about the ways treatments may be combined? What does my healthcare team suggest?



- c) Talking with others may help me learn more about other treatment options or approaches. The people to talk to include family, friends, a patient support group, or professionals like a dietician, physiotherapist or occupational therapist.

*“One of the best actions I took to support my health was to join a support group. Sitting with the loneliness of this disease at times was worse than any symptom I was having. Even so, without being aware, I was having more symptoms.*

*When someone in my support group mentioned the same thing, I raised the issue. My medical team told me that there was no treatment to manage this symptom. But when I brought in the additional information from my support group, they explored treatment. I am now free of this symptom and feeling great!”*

- d) It can happen that I will reach a point with my disease, or my life, where I want to rethink the treatment approach. I will need to discuss this with my healthcare team.
- e) Sometimes it makes sense, too, to ask my doctor to review the medications I am taking to see if they are still doing me good.

*“Living with rheumatoid arthritis means I have had to take many medications daily for the past 30 years. Without these drugs, I know I would be living in a long-term care facility instead of being able to care for my daily needs mostly on my own.*

*“When I was waiting for a hip replacement, pain was so bad I could not sleep at night. A family member suggested I try acupuncture and I decided to take their advice, even though I had very little faith that it would work. After about the third treatment I was able to sleep through the night.*

*“Another time, a joint in my lower back became inflamed and caused severe sciatica. My doctor suggested that I try physiotherapy. Two weeks after starting the treatment, which involved ultrasound, heat and a set of exercises, the inflammation went down and the pain medicine was again able to control my pain levels. I continue to do these exercises two years later and the sciatica has not come back.”*

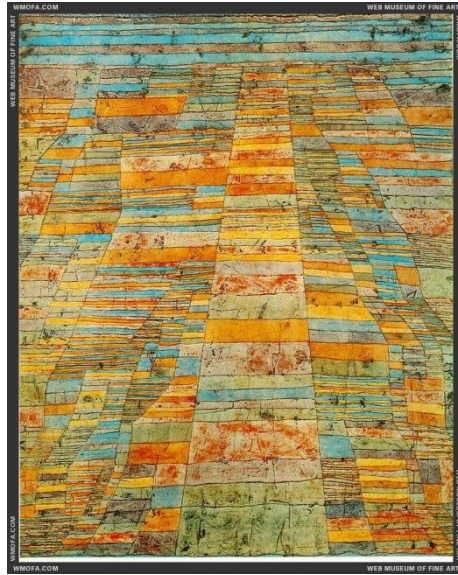
#### TRUE, FALSE OR MAYBE?

1. There may be a number of treatments, and treatment approaches, to help me manage my disease.

See Appendix 2 for explanations.



# STEP 5: LEARNING ALL I CAN ABOUT EACH OF MY TREATMENT OPTIONS



Now that you are aware of your treatment choices, this step allows you to dive deeper into those options. In this step, you will work to collect the best and most reliable information you can find.

## Understanding the positive effects, negative toxic effects, and troublesome effects of treatment

**Positive effects:** A positive effect of treatment is also called a **benefit**. Three kinds of positive effects exist:

1. A positive effect of treatment can **modify disease** by slowing it down or even reversing it. This kind of treatment effect may also provide relief from symptoms of the disease. Two examples of treatments that modify disease are antibiotics and surgery.
2. A positive effect of treatment can **manage disease**. The treatment does not change the fact that the disease exists. Examples of this are hormone treatments, such as those used for Type 1 diabetes and clotting factors, such as those used for bleeding disorders.
3. A positive effect of treatment can **manage symptoms**. The treatment does not change the fact that the disease exists. Instead, its goal is to reduce or control symptoms of the disease, such as pain, stiffness or swelling.

**Negative toxic effects:** A toxic effect of treatment is also called a **harm**. It is negative and serious. A toxic effect can cause a severe reaction, birth defects, drug dependence or death. Patients must report toxic effects to their doctor right away, so that the effects do not progress. The same treatment may cause toxic effects in one person but not in another.

**Troublesome effects:** This kind of negative effect (or harm) is less serious than a toxic effect of treatment because it is not life-threatening. Even so, it can be severe, such as a bad headache. A troublesome effect may last only a short time, or it may be present as long as the treatment continues. It can affect whether you decide to continue a treatment. The same treatment may cause troublesome effects in one person but not in another.

*"I have learned  
can about my  
research on wh  
though my onc*



- **Are there licensed treatments for my condition? What are they? How recent was the licensing? See Appendix 3 to learn about drug and product licensing in Canada**
- **Was the treatment turned down for licensing, or removed from markets anywhere in the world? If so, why?**
- **If a treatment is on the market in Canada, is it licensed with full authorization (Notice of Compliance, or NOC) or with conditional authorization (Notice of Compliance with Conditions, or NOC/C)? See Appendix 3 to learn more about authorizations.**



- **What is known about the positive and negative effects of treatment?**

- **What do I need to know that is still not known about the positive and negative effects of treatment?**

- Is funding for this treatment covered by any source, such as a provincial government, the company that manufactures it, or a private insurance company? If not, why?
- Are there treatments under investigation for my disease or health condition? What are they?

*“As someone with Stage 4 breast cancer, the best advice I can give someone who's just been told they have cancer is, ‘Do not Google it.’ I had to wait to see an oncologist, and was terrified by what I found on the Internet. Then I saw my oncologist, who told me my cancer could probably be managed for years, and that most Internet cancer information is out-of-date, a sales pitch, or just plain wrong. He gave me hope — and also the links to websites that have reliable, up-to-date information.”*

### Sources of information about treatments

- Your doctor, pharmacist and others on your healthcare team are good sources. Other ways to learn about treatments include patient advocacy and support groups for your disease. You can connect with them in person where you live, or online (forums where people share their experiences).
- For details on licensed drugs, products, and devices, visit government websites such Health Canada, the Food and Drug Administration (in the U.S.) and the European Medicines Agency. See Appendix 3 for information about access to drugs and medical products in Canada.
- Health or cancer agencies funded by a province or territory often provide useful information about treatments on their websites. So does the National Health Service in Britain.
- Visit **this website** for information on clinical trials. Companies that make drugs (manufacturers) may also provide online information about clinical trials. LINK TO: <https://clinicaltrials.gov/>
- Look for information on drugs and medical products at the company’s website.



## Possible answers

For each treatment option, you will need to seek answers to the questions above. We also suggest you assess the positive, negative, and troublesome effects of each treatment option. The section that follows guides you through questions to ask about each treatment you are looking at.

<b>Assess the positive effects</b>	
<p>What are the known possible benefits of this treatment for my disease or health problem?</p> <p><b>Benefits</b> are the <b>positive effects</b> of treatment that are desired.</p>	<p>Does the treatment change the way the disease progresses, or does it mostly provide relief from symptoms?</p> <p>How much benefit, and what kinds of benefit, can I expect from the treatment?</p> <p>How many patients with the disease are expected to achieve benefits?</p> <p>How long does it take before people feel the benefits of the treatment?</p> <p>How long does treatment benefit last, if I take the treatment as recommended?</p>
<p>What did studies show about this treatment?</p> <p>Does my doctor believe that the studies were designed well, and big enough to allow the experts to be confident in the benefit findings?</p> <p>If there was more than one study, did the results of the studies agree with each other?</p> <p>Do I know what the lowest dose is that may provide benefit?</p> <p>Have the benefit findings from the studies done before licensing been updated? In other words, do new studies exist that deal with the treatment’s benefits in the general population, after licensing?</p> <p>See Appendix 3 for details on licensing.</p>	
<p>Making it personal</p> <p><b>People like me:</b> In a scientific study or clinical trial, <b>people like me</b> refers to patients who were part of the study and who share characteristics that you have, such as</p> <ul style="list-style-type: none"> <li>• Same gender (male or female)</li> <li>• Same disease or health problem</li> <li>• Same sub-type of disease or health problem</li> </ul>	<p>Can I predict if I will benefit?</p> <p>What is the chance of benefit from this treatment to patients most like me?</p> <p>Were <b>people like me</b> part of the clinical studies for this treatment? (See definition of <b>people like me</b> on left.)</p> <p>What is the lowest dose that might benefit a patient like me? Has this been studied?</p>

<ul style="list-style-type: none"> <li>• Same severity of disease</li> <li>• Same type of “other” problems, such as kidney or liver malfunction, heart problems, or lifestyle factors, such as smoking, being overweight, etc.</li> </ul> <p>Why does this matter? If the study did not contain <b>people like me</b>, the treatment may not be one that will work well or be safe for you.</p>	<p>How long will I have to take the treatment before I start to feel benefits?</p> <p>Is it known whether short-term benefits from the treatment continue in the longer term, or do these positive effects change over time?</p> <p>Is this treatment designed or expected to work on aspects of the disease that are most important to me?</p>
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*“Since each person responds differently to medications, it often takes trying a few drugs to find one that works well to control symptoms of rheumatoid arthritis.”*

<p align="center"><b>Assess the negative (toxic) effects</b></p>	
<p>How safe is this treatment option for me?</p> <p>A <b>toxic effect</b> from treatment is one that causes serious harm, such as</p> <ul style="list-style-type: none"> <li>• death</li> <li>• a serious reaction</li> <li>• birth defects</li> <li>• drug dependence.</li> </ul>	<p>Can the serious and possible toxic effects of the treatment be managed — by reducing their potential and how severe they are — in my particular case?</p>
<p>How safe is this treatment option, generally?</p>	<p>Did studies done before licensing ask specific questions about negative effects? If so, which ones did the studies deal with?</p>

	<p>Were the studies conducted well enough to allow experts to be confident in the findings about the negative effects?</p> <p>Were the studies big enough (have enough patients in the studies) to allow experts to be confident in the findings about negative effects?</p> <p>If there was more than one study, did the results of the studies agree with each other about the negative effects?</p> <p>Is there other information, beyond studies, that showed or helped experts to understand the negative effects?</p> <p>What do I know about the lowest dose that may reduce harm while still giving me benefits?</p> <p>Are there any studies happening now, or planned, or any patient registries of the treatment that are exploring this treatment's negative effects?</p>
<p>What do I need to know about the toxic effects of this treatment?</p>	<p>What are the known toxic effects of the treatment?</p> <p>What did studies and other information reveal about possible toxic effects of this treatment?</p> <p>How often did these occur?</p> <p>Was any particular group of patients more likely to be affected by these toxicities than other groups?</p> <p>At what dose do the toxic effects tend to occur?</p>

	<p>Is the appearance of any toxic effects delayed? If so, by how long? Am I likely to experience treatment benefit before toxic effects occur?</p> <p>How do the toxic effects progress?</p> <p>How long do toxic effects last?</p> <p>Can my doctor manage the toxic effects? How?</p> <p>Are the toxic effects reversible?</p> <p>Do the toxic effects continue if the treatment is stopped? For how long? Based on studies, did they always stop?</p>
Making it personal	<p>Are any of the treatment's toxic effects more or less likely to happen to me? Why?</p> <p>What would the physical and practical effects be if I were to experience any toxic effects?</p> <p>How would my doctor manage these toxic effects, if I were to have them?</p>



*“When I had a toxic reaction to my drugs after years of success, there were only a few options available to me. Luckily, my doctor and I were able to develop new system. We used some very old drugs and some very new drugs. I am still using this system 8 years later, and it has been very successful for me.”*

<b>Assess the troublesome effects</b>	
What do studies of this treatment say about troublesome	What did the studies show are the possible troublesome

<p>effects?</p> <p><b>Troublesome effects</b> of treatment are minor negative effects that are</p> <ul style="list-style-type: none"> <li>• unpleasant or</li> <li>• inconvenient.</li> </ul> <p>A troublesome effect is not a serious or toxic effect of treatment.</p>	<p>effects?</p> <p>How often did the troublesome effects occur in the studies?</p> <p>Was any particular group of patients more likely to experience these troublesome effects than other groups?</p> <p>In the studies:</p> <ul style="list-style-type: none"> <li>• How severe are the troublesome effects?</li> <li>• At what dose does the troublesome effect occur?</li> <li>• How long do the troublesome effects last?</li> <li>• Can a doctor manage the troublesome effects? If so, how?</li> <li>• Are they reversible?</li> </ul> <p>Do any troublesome effects appear if the treatment is stopped? If so, what are they and how long do they last? In the studies, did they always stop?</p>
<p>What else do I know about the troublesome effects of this treatment?</p>	
<p>Making it personal</p>	<p>Are any of these troublesome effects more or less likely to happen to me? Why?</p> <p>What would the physical and practical effects be if I were to experience any troublesome effects?</p> <p>How would my doctor manage troublesome effects, if I were to have them?</p>

*“I began to have allergic reactions to different antibiotics that I had never had problems taking. I was concerned because I needed dental work that required antibiotics. My doctor got me connected to a hospital's drug allergy clinic and they did a full day of testing me for drug allergies. Now we know what drugs are safe and which to avoid. I was able to get the dental work done and have no worries about my need to use antibiotics in the future.”*





### Other things to consider about this treatment

<p>Is this a new or an older treatment?</p>	<p>If this is an older treatment, have any issues emerged among patients who have used the treatment, since it was licensed? What were the positive or negative effects on patients? Have these issues been resolved?</p>
<p>Is my doctor suggesting this treatment because it is a common one for my condition? Or is it an off-label drug or treatment?</p> <p>An <b>off-label drug or treatment</b> is one that</p> <ul style="list-style-type: none"> <li>• is licensed in Canada but is being used for a disease or for people that are not covered by the licensing, or</li> <li>• is not licensed in Canada but may be used in special cases, if a doctor believes it may benefit a patient.</li> </ul>	<p>If the treatment is off-label for my disease or health problem, why does my doctor think the treatment will be safe, work well, and provide benefits to me?</p> <p>How much is known about the possible positive and negative effects of the treatment if it is used off-label?</p>
<p>Is this an expensive treatment?</p>	<p>Will this treatment be paid for by someone other than me? If so, by whom? If not, why?</p>
<p>Are there other, or different treatment approaches, for my disease?</p>	
<p>To answer the questions above, do I have access to good quality and relevant information?</p>	
<p>What information should I ask my doctor to provide? My pharmacist? Clinic nurses? Other people on my healthcare team? Do I wish to consult a patient advocacy group that works with people who have my disease or health problem?</p>	
<p>Have I asked my healthcare provider for the patient package insert that is part of the Product Monograph, for this treatment?</p>	
<p>How can I contact other patients to ask questions (through a patient advocacy group, or a disease association)?</p>	
<p>Do I need, or want, other information from my healthcare team, after considering what is in this Guide and from other sources? What information do I need?</p>	



A Product Monograph is a document that provides important details on a drug, treatment or product that is on the market in Canada. Every drug or product licensed by Health Canada must have a Product Monograph. It is written by the company that makes the product, using a standard format set by Health Canada. It must be approved by Health Canada.

The consumer information section of the Product Monograph helps patients learn more about what the product is, how to use it, and what its possible negative effects are.

Appendix 3 provides more information about Product Monographs and tells you how to search online for them.

***“A new medication may have possible serious side effects that are not yet known because the drug has not been on the market very long. During clinical trials, it is tested in a very limited number of people. This does not reflect what happens when it is on the market and available to the public. Older drugs have been on the market for a long time so more is known about whether they are safe and work well.”***

Some systems exist in Canada to help pay for drugs and medical treatments. They include

- Funding by federal, provincial, and territorial governments
- Insurance from private companies
- Insurance through your job or workplace

***“As a group leader working with patients, I am always shocked when I hear a doctor say they will not even tell the patient about one of our treatment options because they feel it is too expensive. This is not the doctor’s decision to make.”***

**Checklist to ensure you can trust online information.** (Adapted from Reference 10, listed in Appendix 4)

- What organization or person hosts and adds content to the website? You can trust a well-known and highly regarded medical institution, a government health department or agency, and credible not-for-profit health organizations.
- For whom is the site intended? Some websites are for the general public. Others are for healthcare providers. Does the website state its privacy policy (how it collects and shares information about you, and what it does with your email address)?
- What is the purpose of the site? Look for a mission statement. Check to see if the site is for educational purposes or if it promotes a certain product or service. Look for a statement saying that information on the site is meant to add to but not replace any information or advice from a healthcare provider.
- Is there advertising on the site that relates to or affects information the site provides? Find out who sponsors the website. Is it a health institution or a company that wants to sell a product?

**Protect yourself!**

- Beware of a website that describes products as “a miracle” or tells you there is a “secret ingredient.”
- Find out how current the information on the website is. Look for dates that tell you when it was updated or reviewed.
- It should be easy to contact the people who are responsible for the website. Look for a valid email address or a contact form you can fill out.

*“As someone with a rare disease, I have come to learn how lucky I am that I actually have treatment **OPTIONS**. Many people with a rare disease do not. Unfortunately, there aren’t enough patients with my disease to do proper clinical trials so it is difficult to access some of these treatments. Getting involved with the patient group helped me find a unique way to get the treatment option that was best for me.”*

**TRUE, FALSE or MAYBE?**

- 1) People tend to overestimate how much is known about the positive and negative effects of licensed treatments on individual patients.
- 2) New treatments are as good as, or better than, older ones.
- 3) I can only be prescribed a treatment if it is approved for my disease or health problem.
- 4) Information in medical and scientific journals is the best source of trusted information about treatments.

See Appendix 2 for explanations.

# STEP 6: EXPLORING THE UNCERTAINTIES OF EACH TREATMENT OPTION

There may be uncertainty about how a treatment will work for you. In Step 5, you may have dealt with this when you thought about whether *people like me* are likely to have positive or negative effects from a treatment.

The purpose of Step 6 is to understand more about uncertainties.



As adults, we know that life holds uncertainties. This is also true of medical treatments. Why? Because science is complex and must take into account all aspects of a situation. The government bodies that regulate drugs and medical products know this. That is why they do not simply say, “Here is a drug that will work for everyone.” Instead, they let the public know that uncertainties exist, so that patients will not have false expectations.



## Uncertainties

In the world of medicine, this term refers to anything that suggests some level of doubt, or lack of confidence, about a treatment’s positive and negative effects.

It can also refer to doubt about

- when it is best to use the treatment
- who will benefit, or
- results of studies done to assess the treatment.

**What do I or feel when I positive and negative effects of treatment?**

**still not know unclear about consider the**

**Are there any other uncertainties about the treatment that I should consider?**

## Can I use the uncertainties to help me ask clearer questions about whether this treatment will work for me?

*“Having a rare disease is not easy. It means you have to take a lot more responsibility for your health. You cannot rely on the experts to advise you. The truth is no one knows what the best treatment options are. There are a lot of uncertainties.”*

### Possible answers

We suggest you think about uncertainties based on whether a treatment is licensed or unlicensed in Canada.

<b>For licensed treatments</b>	
A licensed treatment is one that Health Canada has assessed and then approved for use in this country. (See Appendix 3 to learn more.)	
<b>If there are uncertainties about the treatment’s possible benefits and harms, how are the experts dealing with this?</b>	<p>Is there a patient registry that you can consult?</p> <p>Are further studies planned?</p> <p>Why are these studies being done?</p> <p>Has a government (in Canada or elsewhere) ordered the studies? If so, can I join a study?</p> <p>Should I join a study?</p>
What is still <b>unknown</b> about the possible benefits and harms of the treatment after licensing?	
What is still <b>not clearly understood</b> about the possible benefits and harms of the treatment after licensing?	
Since the treatment was licensed have issues emerged that relate to positive or negative effects on patients? How uncertain are the experts in their judgments about these issues?	
Is the information that I have considered consistent when it comes to the possible benefits and harms of the treatment? Do the experts and the people on my health care team agree with each other on this?	
Has the experts’ understanding of the treatment’s possible benefits and harms changed recently? If so, why?	



*“I love my doctor. She is so careful and is always honest with me about what is known about possible treatments and what is not known. She is never afraid to admit uncertainty — even her own. That is one of the reasons that I respect and trust her so much.”*



#### **For “off-label” treatments**

An “off-label” drug or product may be licensed in Canada yet still called “off-label,” which means it may be used for a disease or for people that are not covered by the licensing.

An “off-label” drug or product may NOT be licensed in Canada yet it may be used in special cases, if a doctor believes it may be of benefit to a patient. (See Appendix 3)

**Should I consider being part of a clinical trial for an “off-label” treatment?**

You should find out

- how far along the test treatment is
- what proof exists about the drug’s positive or negative effects
- what is still unknown about these effects.



*“Personally, I would not want to take a drug until it is in phase 3 clinical trial. That’s when I believe the potential benefits begin to outweigh the potential harms.*”

*“For people with rheumatoid arthritis, 15 years ago we may have been willing to take a bit more risk [with off-label drugs] since many of us were completely out of treatment options and had been for some time.”*

#### TRUE, FALSE or MAYBE?

- 1) Information about a treatment's potential benefits and harms is complete at the time of licensing.
- 2) Before they are licensed, treatments are not usually studied with actual patients like me.
- 3) It is not easy to collect reliable information on a treatment's possible benefits and harms once it is on the market.
- 4) There can be differences of opinion among the experts about the possible positive and negative effects of a treatment, both before and after it is licensed.
- 5) It is clear what the most effective dose is for someone like me (the dose that will provide the best chance of experiencing the treatment’s benefits while reducing its negative effects).
- 6) The best practice of science results in answers and truths that everyone can understand and agree on.

See Appendix 2 for explanations.

# STEP 7:

## ANTICIPATING OTHER KINDS OF DECISIONS THAT FLOW FROM TREATMENT

Each decision we make may set us up for having to make other decisions later on.

The purpose of this step is to anticipate what some of those later decisions may be, based on the treatment(s) you choose.



**What other kinds of decisions may I need to make if I choose this treatment?**

**What might be the impacts of my treatment choice on me?**

### Possible answers

Here are some examples of the other kinds of decisions people may face:

- Visits to the clinic (or hospital) and the impact this has on my daily routine
- Need to travel away from home
- Need for home care
- Changes to or limits on other treatments I take
- Need for other treatments to manage negative effects of this treatment (new medicines, or starting other therapies, such as physiotherapy)



- Choice to join a clinical trial and the commitment it entails
- Effects on finances (having to reduce spending elsewhere so I can afford the treatment)
- Effects on daily activities or life choices (making changes to meal times, what foods you eat, or birth control due to the treatment)

*“When I was diagnosed with cancer, the health care team had a treatment plan all mapped out before I even met with them. As soon as they told me the plan I said, ‘I will not be able to walk my daughter down the aisle at her wedding next month.’ The team told me I needed to start the treatment plan the next week. They did not understand how important the wedding was for me. In the end, I delayed my treatment. I sleep well at night and I am confident that the benefit of being there on that amazing day for my daughter was far greater than the risk of waiting to begin treatment.”*



## STEP 8: IMAGINING MY POSSIBLE OUTCOMES

The purpose of Step 8 is to explore how your body and mind feel after looking closely (in Steps 6 and 7) at the positive and negative effects of treatment, and the other kinds of effects treatment may have on your life. The goal of this step is to help you understand how comfortable you are with each of your treatment options.

**What could the positive and negative impacts from taking a certain treatment feel like to me?**

**Now that I am aware of the uncertainties that come with the treatment, how do I feel about taking this treatment?**



### Possible answers

Imagine how you would feel if you experienced the effects listed below:

- What would it feel like if the treatment reduced or cured my disease or health problem?
- How would I feel if the treatment did not work as I hoped it would?
- What would the more serious side effects, or toxic effects, feel like?
- What would the troublesome, but not dangerous, negative effects feel like?

Take a moment to imagine these possible treatment effects:

- What would the physical and practical impacts be if I were to experience the benefits of this treatment?
- What would the physical and practical impacts be if I were to experience the toxic effects of this treatment?
- What would the physical and practical impacts be if I were to experience the troublesome effects of this treatment?



Use the empty space below to write your answers to four more questions:

1. How would the possible effects of this treatment affect my life and the people and routines that matter to me? Effects are things like making and eating meals; going to the bathroom; and spending time in clinic or hospital.
2. What would the impacts be if I had to have more or different treatments to manage the toxic or troublesome effects of this treatment?
3. How do I feel about perhaps having to deal with delayed negative effects from this treatment?



4. Could I tolerate living every day with the possible negative effects, if I choose this treatment?



*“When I think about enrolling in clinical trials for experimental treatments, I realize I would not feel able to deal with the higher uncertainties about positive and negative effects, compared to licensed treatments. I am still working full time while living with my chronic disease. It is not a time in my life when I can take days off work to recover from a negative treatment reaction that might occur.”*



Finally, please consider these two questions:

1. What do I imagine the best-case scenario of my taking the treatment could be?
2. What do I imagine the worst-case scenario of my taking the treatment could be?

**TRUE, FALSE or MAYBE?**

- 1) Licensed treatments work for everyone.
- 2) Licensed treatments are highly effective.
- 3) Licensed treatments are safe.
- 4) Licensed treatments carry very few chances of negative effects.
- 5) Other and different treatments are sometimes needed to manage the negative effects of treatments used for the primary disease.

See Appendix 2 for explanations.

# STEP 9: WEIGHING THE POSITIVE EFFECTS, NEGATIVE EFFECTS AND UNCERTAINTIES OF MY TREATMENT OPTIONS

In this step, you will consider how you feel, so far, about each of the positive effects, negative effects and uncertainties about treatments. Why do you feel as you do? The goal is to make a general list of what you are feeling, not to create a neat, numbered list.



**For each treatment:**

**What is the value or importance to me of the treatment's benefits, harms and uncertainties?**

**How do I feel about the practical effects that may be part of the treatment?**

**Possible answers**

In this section, you have time to think about

- Positive treatment effects
- Negative treatment effects
- Uncertainties around this treatment

Then, you will look at your own situation.

### Positive treatment effects

Decide on two or three positive effects that are most important to you. Use the table below to list these positive effects from the most important to least important for each treatment option.

Treatment option #1	Treatment option #2
Best positive effect	Best positive effect
Next best positive effect	Next best positive effect
Least important positive effect	Least important positive effect
Treatment option #3	Treatment option #4
Best positive effect	Best positive effect
Next best positive effect	Next best positive effect
Least important positive effect	Least important positive effect

Now, consider these questions: What treatment options can I exclude, if any? Why?

What treatment option(s) are most likely to meet my main health goals?

What treatment option(s) do I feel most confident about?



## Negative treatment effects

Decide on two or three negative effects that are most important to you. Use the table below to list these negative effects from the most important to least important for each treatment option.

Treatment option #1	Treatment option #2
Worst negative effect	Worst negative effect
Next worse positive effect	Next worse positive effect
Least important negative effect	Least important negative effect
Treatment option #3	Treatment option #4
Worst negative effect	Worst negative effect
Next worse positive effect	Next worse positive effect
Least important negative effect	Least important negative effect

Now, consider these questions: What treatment options can I exclude, if any? Why?

What treatment option(s) are most likely to meet my main health goals?

What treatment option(s) do I feel most confident about?

## Uncertainty around this treatment

1. Am I likely to experience the possible positive effects before the negative ones, or will it be the opposite? How do I feel about this?



2. How do I feel about the possible need to manage the toxic effects of each treatment option? What about the toxic effects that may not appear for a long time? What about the rare toxic effects?



3. How do I feel about other kinds of decisions I may have to make if I take this treatment? What are these other important decisions? Can I list them in order, from most to least important?

*“With my illness, one treatment option was to learn how to start my own intravenous (IV) so that I could infuse my medication at home. I chose this option because I thought it would give me more flexibility and control over my life. Unfortunately, I have small veins that are hard to access, so I often end up in the emergency room anyway.*

*“Some people I have talked to say that treating themselves at home feels like the illness is everywhere they turn. They prefer to have fixed appointments to get treatment so they can deal with their illness at specific times and then put it aside and get on with their lives.”*

4. How do I feel about uncertainties in what experts say about the treatment? How does this affect my comfort level with this treatment?
5. Can I identify which treatment options I feel most confident about and why?
6. What uncertainties are most important to me? Can I list these in order, from most to least important?



## Your own situation

Take some time to think about your life circumstances and values, and the people most important to you, that will help decide on the treatment options that are best for you. Use these questions as a guide:

- Does my age make a difference to my treatment options?
- Does being male or female make a difference to my treatment options?
- Do I have any other health problems that could affect the type of treatment that may be best for me?
- Do I have medical coverage to pay for the treatment?
- Could my daily routines — related to family, work, where I live — affect whether I am able to take the treatment as recommended?
- Are there important people in my life that could or should influence my decision? If so, who are they and what is their role?

*“I once had a bad cold that went to my chest. Being on a biologic I have to be very careful not to get an infection. I went to my family doctor asking for an antibiotic. He gave me the prescription but asked me to wait one more day before filling it. The next day I woke up feeling better and never filled the prescription.”*

### TRUE, FALSE or MAYBE?

- 1) **People with the same disease can have very different life circumstances that may affect how they see and feel about the positive and negative effects of a certain treatment.**
- 2) **People with the same disease can have very different personal or cultural values that may affect how they see and feel about the positive and negative effects of a certain treatment.**

See Appendix 2 for explanations.

## STEP 10: IDENTIFYING TRADE-OFFS I AM WILLING TO MAKE AND THOSE I AM NOT



Making trade-offs is part of many decisions. When we decide to save money, we tell ourselves that the long-term goal of money in the bank outweighs the short-term pleasure we may get from a new pair of shoes.

Your disease is a heavy burden you want to reduce. In Steps 6 and 7, you learned that certain treatments may add to your burden (from the side effects and life effects that you may experience). On the other hand, the treatment may reduce your burden, if the positive effects of treatment occur for you. As you work through this Guide, you are deciding on a treatment that, as far as you can tell, offers the best way to reduce your overall health burden.

In Step 10, you have time to assess the trade-offs you are willing to make, and those you are not.





### Who is likely to benefit? Who may suffer harm?

Experts think a lot about who will benefit from a treatment and who will be more likely to suffer harm from it.

Even when a treatment is licensed, the answers to these questions may be unknown. This is because studies done before the drug or medical device was licensed may not have looked closely at the subgroups likely to benefit and the subgroups likely to experience harm.

It can take time and new studies to find answers. In the meantime, patients need to assess how they feel about the positive and negative effects of a treatment, and the uncertainties about those effects.

**In order to gain possible benefits from taking the treatment, what trade-offs of possible harms or uncertainties do I need to make?**

**How much possible harm (from negative effects of the treatment) am I willing to endure, if I can get benefits from the treatment?**

**Are there some possible harms from this treatment that I am willing to accept and others I am not?**

**What is my tolerance with uncertainties? This means, how much uncertainty about positive and negative effects am I willing to accept?**

### Possible answers

The first step in being clearer about trade-offs is for you to identify them.

- Do I know what the most important trade-off decisions are that I need to make when it comes to a treatment's possible positive and negative effects?
- Have I asked my healthcare team or people I trust to suggest other possible trade-offs that I need to consider?

Trade-offs that relate to benefits and harms from a treatment	
Questions to consider	Write your answers here
If I really could get the benefits promised by the treatment, what toxic effects would I be willing to endure?	
What troublesome effects would I be willing to endure in exchange for possible benefits promised by the treatment?	
Are the practical effects on my life (see Step 7) from taking this treatment worth it, if I can get the possible benefits?	
How do my feelings about my disease or health problem affect <b>how willing I am</b> to endure possible negative effects from the treatment?	
How do the feelings of <b>those I care about</b> affect how willing I am to endure possible negative effects from treatment?	



*“To control my rheumatoid arthritis, I was willing to take an older drug that gave me very unpleasant side effects, in a lower dosage, along with a newer, more risky drug.”*

*“As someone who has rheumatoid arthritis, I feel that my health team has addressed my pain and inflammation adequately. My main concern is FATIGUE. Someone suggested that I approach each day with an imaginary bag of “energy coins” to spend. In spite of trying to exercise and pacing my activities, I still find that on many days, the energy coins are gone before the day is half over. Surely some investigation into relief for this symptom is warranted.”*

*“Most people believe that if a drug is approved by Health Canada it is safe to use. They do not realize that there are risks associated with treatment and that they can decide whether the risk of not treating their rheumatoid arthritis is equal to the possible benefit of taking the drug.*”

*“When you have a life-threatening condition like HIV, you are more likely to accept risks. Studies have shown that HIV drugs increase the risk of toxic effects, such as bone loss, heart disease, kidney and liver disease and some cancers. But what choice do I have? I have to take my HIV medications, so I do. I get tested for known toxicities and manage them as well as I can.”*

*“I’m so grateful to my specialist for telling me she could not, in good conscience, prescribe the “easy” treatment option (an oral medication) because she felt the risks of side effects were too great. With that knowledge, I chose a treatment that was much harder to take (an intravenous infusion). Even though my treatment plan is much more difficult day-to-day, I have a much better quality of life overall.”*

Trade-offs that relate to uncertainties from a treatment	
Question to consider	Write your answers here
How comfortable do I feel about taking this treatment, if the experts are unsure about (or do not agree on) whether or how well it works? How safe it is?	
Would I rather wait to take this treatment when doctors understand more about if, or how well it works? How safe it is?	
When I think about how serious, severe or urgent it is to manage my disease or condition, how does this affect my willingness to accept uncertainties that come with the treatment?	
How much uncertainty, and what kinds of uncertainties about the possible positive and negative effects of treatments am I willing to accept?	
Do my feelings about my disease or health condition affect how much uncertainty I am willing to accept? Have I thought seriously about how I feel about this?	
Are there other available treatments, and if so, how does this affect how much uncertainty I am willing to accept with this treatment?	



*“When I learned I had HIV, only one drug was available. I was told it could extend my life for up to two years. At first, I took it, because I wanted to live more than anything. After two months of experiencing so much fatigue that I could not do anything, I stopped taking the drug. I told my doctor I would rather live two years with good quality of life than four years in misery.”*

*“It was frustrating that no immediate answers were available to me. I came to learn that what I was looking for was the “science” part of medicine. But there is also the great “art” of medicine. It is this “art” part that creates a delicate balance between great uncertainty and great promise.”*

*“After I was diagnosed, I was very open with friends, family and colleagues about everything. I found support in the most unexpected places. Many opened up to me about their own experiences. I received many compliments, such as, “You are one tough cookie,” “You are amazing for the way you are living your life,” and “If ANYONE can beat this cancer, it will be you,” I often reflect on the wonderful things people have said to me when I am having a bad day. It lifts my spirits. I have not felt alone one step of the way, even though I have a rare cancer.”*



#### TRUE, FALSE or MAYBE?

- 1) I cannot have it all: get the possible benefits without exposing myself to possible harm.
- 2) I need to think about how much harm, discomfort, pain or other negative effects I am willing to experience in exchange for how much benefit I might experience.
- 3) Everyone has the same level of tolerance for possible harm from treatments.
- 4) Everyone has the same level of tolerance for uncertainties that relate to a treatment’s effects.

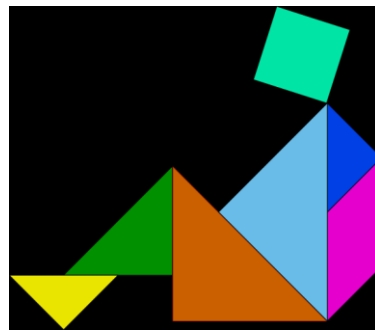
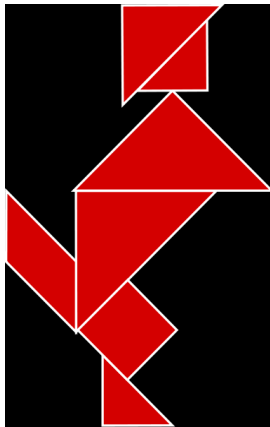
See Appendix 2 for explanations.

# STEP 11:

## JUDGING MY TREATMENT OPTIONS BASED ON THEIR ULTIMATE VALUE TO ME

The goal in this step is to apply the knowledge you have gathered about each of your treatment options and to rank the options.

You may not be able to create a clear order, or ranking, for your options. Sometimes, the impacts of the options are so much alike, you may think you are comparing a green apple to a red apple. Again, talking with others may help you sort things out. On the other hand, do not be surprised if you find differences of opinion!

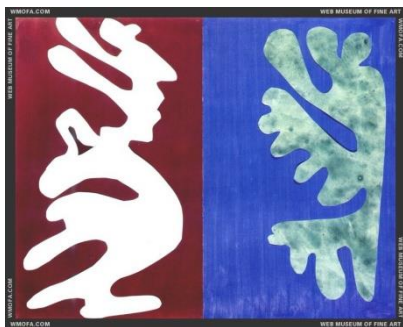


**From what I now know about each of my treatment options, AND how I feel about their positive and negative features AND how I feel about the trade-offs I am willing, or not willing, to make:**

- **which treatment(s) may help me achieve my own health goals?**
- **which treatment(s) do I have most confidence in? Why?**
- **which treatment(s) best suit my own values and circumstances? Why?**
- **which treatment(s) do I have least confidence in? Why?**
- **which treatment(s) least suit my personal goals, values and circumstances?**



## Why?



### Possible answers

- a) I need to consider what treatments, or types of treatment, will provide the most improvement in the most important aspects of my disease.
- b) I need to assess how well my treatment options will allow me to manage pain, fatigue and other kinds of discomfort or problems linked to my disease.
- c) I need to decide what treatments, or types of treatment, are most likely to work for me.
- d) I must assess which treatments I am most confident will allow me to avoid, manage, or tolerate negative effects.
- e) I need to consider which treatments are least likely to disrupt my way of life and daily activities.

*“I had such a sense of relief when I was finally diagnosed. Then, the medical team offered me only one treatment option. I had done my research and knew that it seemed to be working well for many with my disease. However, due to the nature of the treatment and its side effects, I knew it was not a fit for me. In order to take it, I would need to stop working, not be able to care for my family and have a very limited quality of life. I pushed for a second opinion, which led me to a medical team that was able to get another treatment through the Special Access Program. While I do have some severe side effects sometimes, my day-to-day quality of life is so much better and I am so grateful for that!”*

g) I need to determine what treatments fit best with my values.

*“I have decided not to take extra pain medication. Instead I opted to receive treatment from a physiotherapist. It required more work and more time on my part to reduce my pain but it has worked just as well as the pain medication.”*



*“My rare disorder is described by doctors as episodic. But there is nothing episodic about living with this disease. I have to think about it and live with it every moment of every day. I must adjust my goals, plans, and dreams every day.*

*“Once I became more open with my medical team about how this disease affects me, it became easier to come up with a treatment plan that actually worked for me. We now have a treatment plan that my doctors feel is realistic. I still have attacks sometimes but I also can hold on to my dreams and live a full life.”*

## FINAL STEP: RE-CHECKING MY THINKING



Before you make a final decision, this step will take you back to some of the questions we started with in Step 2. Why? Because your thinking and perceptions may have changed over time.

**At the start, did I recognize things about the situation, in general, that may have made it hard for me to be ready or able to think about treatment options?**

**At the start, did I recognize specific things about my situation, or about myself, that may have affected my thinking (made it less objective)? Were these things truly reasonable and meaningful to me?**

*“Do not let your disease rule your life. It is part of you, but the disease is not all there is!*

*“I am always mindful of the fact that no matter how bad things get for me, there are so many who are worse off than I. Taking stock of the many, many blessings in my life brings me peace and perspective.”*

## Possible answers

In this step, all the possible answers are more questions. You owe it to yourself to take time to answer them.

### **From then to now**

Do I need to review the goals I set when I began this Guide to see if my thinking has changed since then?

### **Factors that affect thinking**

What else — rightly or wrongly — might have affected my thinking and decisions at the start? For example:

- My emotions: fear, hope, feeling frustrated or desperate
- The way people I care about were feeling?
- My assumptions — or other people's assumptions — about treatments or other things that relate to the disease or health problem. Were those assumptions reasonable? Are they still valid based on what I know now about treatment option?

Did any of these factors affect how I saw or felt about possible benefits, harms and uncertainties?

Do these factors still have meaning; are they important to me? Why?

### **Choices about what I considered**

If I found some issue difficult to think through, did I simply choose to ignore it?

Did I focus on **some of the information** about a treatment to make it look the way I wanted it to look?  
Did I not see it as it really is?

Did I let other people's stories weigh too heavily (or not enough) in my thinking?

Did I lean towards certain option(s) more than others because I want to avoid regret later on?

Did I focus on certain information or peoples' views because they agree with my own views?

Did I ignore certain information or views because they threaten my world view?

### **Peace of mind**

How important is peace of mind to me?

### **Numbers**

Did I really understand the numbers? The probabilities? Do I need help to get a clear understanding of these?

### **Now and the future**

Did I lower the importance of things that may happen in the future compared to things that might happen now?

Did I set out (distinguish) my short-term, medium-term and long-term goals to see how they are affecting my decision in the present?

Was I able to take the long view, in my best interests?

### **Advice from others**

Was I able to figure out which treatment options had the best overall fit for me? If not, did I look for support from people I trust to help me do this?

Whose advice did I trust in making my decision? Why?

### **Your second time using the Guide**

Has any of the information on treatment choices changed since I chose a treatment? Do I need to reconsider that first choice?

Has my thinking or my personal situation changed? Do I need to reconsider the treatment decision I made before?

## LASTLY, PLEASE REMEMBER...

For your health, and the good of other patients, it is very important that you tell your health care team about **all of your experiences** with your treatment.

Let them know if:

- you respond well to the treatment
- you do not respond to the treatment
- the treatment starts not working for you
- you experience any negative effects from the treatment.

### TRUE, FALSE or MAYBE?

1. **It is important to give my healthcare team a complete picture of my experiences with treatment, both positive and negative, in order to help me and other patients get the best care possible, in the end.**

See Appendix 2 for explanations.

## APPENDIX 1: Definitions of important terms

The list on this page contains important terms that appear in the Guide. We provide them in alphabetical order. The page number(s) listed for a term tells you where to find a definition or explanation of the term.

<u>Benefit (also called a positive effect of treatment)</u>	<u>Step 5, blue box entitled <i>Understanding the positive effects, negative toxic effects, and troublesome effects of treatment</i></u>
<u>Harm (also called a negative effect of treatment)</u>	<u>Step 5, blue box cited above</u>
<u>Licensed treatment</u>	<u>Appendix 3</u>
<u>Negative effects (of treatment)</u>	<u>Step 5, blue box entitled <i>Understanding the positive effects, etc.</i></u>
<u>Off-label treatment</u>	<u>Step 5, table entitled <i>Other things to consider about this treatment</i></u> <u>Step 6, table entitled <i>For Off-label Treatments</i></u>
<u>People like me</u>	<u>Step 5, table entitled <i>Assess the Positive Effects</i></u>
<u>Positive effects (of treatment)</u>	<u>Step 5, blue box entitled <i>Understanding the positive effects, etc.</i></u>
<u>Product Monograph</u>	<u>Step 5, blue box near end of Step 5</u> <u>Appendix 3</u>
<u>Toxic effects (of treatment)</u>	<u>Step 5, blue box entitled <i>Understanding the positive effects, etc.</i></u>
<u>Troublesome effects (of treatment)</u>	<u>Step 5, blue box entitled <i>Understanding the positive effects, etc.</i></u>
<u>Uncertainties</u>	<u>Step 6, blue box near beginning of step 6</u>
<u>Unlicensed treatments</u>	<u>Step 6, Table entitled for unlicensed or “off-label” treatments</u>

## APPENDIX 2: True, False or Maybe explanations

We only explain answers that we think need a detailed description.

### STEP 1: SETTING MY GOALS

- 1) **I have little say in how my disease is managed.**

**ANSWER: FALSE**

As a patient living with disease every day, you must learn to cope because the healthcare system cannot always give you the help you need. These days, patients living with serious and chronic health problems must learn to do self-care and be firm about getting the treatments they require.

- 2) **Everyone with the same disease has the same concerns, or feels the same way, about their disease.**

**ANSWER: MAYBE**

Each person is unique. For example, a woman with rheumatoid arthritis who has small children may not feel the same about the disease as a woman with grown children. Also, many patients go into denial when they learn they have a chronic condition they will have to manage for the rest of their lives.

- 3) **There may be a number of different treatment options to manage my disease.**

**ANSWER: TRUE**

It is possible to manage the same disease well with more than one kind of treatment. What works best may be a mix of treatments or treatment approaches. Some examples are: surgery with drugs; changes to lifestyle, with drugs; use of a medical device, plus drugs; physiotherapy with changes to lifestyle.

- 4) **At the physical level, everyone with the same disease or health problem responds in the same way to the same treatment.**

**ANSWER: FALSE**

Each person responds in their own way to treatment. The reasons for this include family (genetic) background, age, being male or female, life circumstances, and whether the person has other medical conditions.



## STEP 2: DISCOVERING MY THINKING

- 1) **I should not let my emotions affect decisions about my healthcare.**

**ANSWER: MAYBE**

How we feel is an important part of being human. It can help, however, to be aware of our emotions and to see how they may affect our decisions. It is good to recognize what we are feeling and see how our feelings are affecting the way we see the whole situation. Emotions are linked to our “gut feelings” or intuitions as we go through the mental process of making a decision.

- 2) **I have a clear understanding of how well a licensed (approved) treatment will work and how safe it is for me.**

**ANSWER: MAYBE**

- 3) **I am good at understanding the numbers that describe the potential for benefits and for harm that may come from treatments.**

**ANSWER: MAYBE**

- 4) **Knowing that I may be biased in some of my thinking, or misled by false beliefs, will help me make better decisions.**

**ANSWER: TRUE**

- 5) **Taking time to think about my medical decisions will help me make better decisions in the end.**

**ANSWER: TRUE**

## STEP 3: SEEING MY OPPORTUNITIES

- 1) **Everyone with the same condition feels the same way about the possible benefits and harms of a certain treatment.**

**ANSWER: MAYBE**

In general, people can define and see this differently. For example, some people want their pain to go away completely, while others are willing to accept a certain level of pain that allows some quality of life. How people feel about the benefits of a treatment may also depend on what aspect of their disease or condition bothers them the most or least.

People also differ in how they feel about negative effects, or risks of a treatment. This often relates to whether the person is alone or has others to think about.

- 2) **People with the same disease can have very different life circumstances or values that they need to consider when they are making choices about their health and treatment options.**

**ANSWER: TRUE**

- 3) **Once a treatment is licensed, people tend to focus more on its possible benefits and underestimate its possible harms.**

**ANSWER: TRUE**

It is almost impossible to guarantee what a treatment's effects will be. This is because:

- medical knowledge about the disease may not be complete
- the science used to develop a treatment is often complex
- positive and negative effects on each patient are almost impossible to predict.

When you are choosing a treatment, you may want to understand how the federal government decides to license drugs and medical products in Canada. Appendix 3 provides details.

When it licenses drugs and medical devices for sale and use, Health Canada must have confidence that:

- 1) patients who take the treatment correctly will receive the positive effects promised by the drug
- 2) possible benefits from the treatment will outweigh possible negative effects from the treatment
- 3) the combined results of possible positive and negative effects on patients will improve patients' overall health.

After doing its best to assess possible positive and negative effects, Health Canada may still have doubts about the outcomes for patients. These uncertainties mean that Health Canada may decide not to issue a license or that it will require that the sponsor of the product do more studies before it will issue a license.

You may wish to read this report about uncertainties in the licensing of drugs:

*Communicating uncertainties about prescription drugs to the public* by L.M. Schwartz and S. Woloshin.

#### STEP 4: LAYING OUT MY TREATMENT OPTIONS

- 1) **There may be a number of treatments, and treatment approaches, to help me manage my disease.**

**ANSWER: TRUE**

#### STEP 5: LEARNING ALL I CAN ABOUT EACH OF MY TREATMENT OPTIONS

- 1) **People tend to overestimate how much is known about the positive and negative effects of licensed treatments on individual patients.**

**ANSWER: TRUE**

During studies on groups of patients, the results of looking at positive and negative effects are averaged for the group. This means your response to a treatment may be different from this average. There are many reasons for this, such as a person's racial or genetic background, whether they are male or female, their age, and whether they have other medical problems. It is impossible to guarantee that an individual patient will respond well to any treatment.

- 2) **New treatments are as good as, or better than, older ones.**

**Answer: MAYBE**

When they apply to have a treatment licensed, companies do not need to show that their treatment performs better than treatments that already on the market. Instead, they need to show that the possible benefits of their treatment outweigh the possible harms, and that questions about this have been resolved.

Two benefits of treatments licensed for a longer time is that

- Doctors and others in health care have more experience using the drug or product to treat patients, and
- Companies and the government have more information about how the drug or treatment works on patients from outside the clinical trial.

In contrast, experts cannot be as certain about the effects of new treatments since these have not been used in larger numbers of patients.

- 3) **I can only be prescribed a treatment if it is approved for my disease or health problem.**

**Answer: FALSE**

Once a treatment is licensed by Health Canada, doctors can prescribe it as they wish. They must take into account the conditions of use set out in the Product Monograph, which states what disease or health problem it is designed to treat. Patients need to be aware that if a treatment is not licensed for a certain use, government health plans may not pay for it.

- 4) Information in medical and scientific journals is the best source of trusted information about treatments.**

**Answer: FALSE**

In general, negative or doubtful results of treatment studies are not published in scientific and medical journals.

We believe that the consumer information section of Health Canada's Product Monograph for a drug or treatment can be a reliable source of information for patients. The monograph sets out the possible benefits and harms of the treatment and describes the types of patients most likely to experience the benefits of treatment. It also provides details on how long to use the treatment and how to reduce the potential for harm. Other government bodies outside Canada also make information available about licensed products. See Appendix 3 for details on this.

#### **STEP 6: EXPLORING THE UNCERTAINTIES OF EACH TREATMENT OPTION**

- 1) Information about a treatment's possible benefits and harms is complete at the time of licensing.**

**ANSWER: FALSE**

To learn more, go to Statement 3 in Section 3's True, False or Maybe.

- 2) Before they are licensed, treatments are not usually studied with actual patients like me.**

**ANSWER: TRUE**

- 3) It is not easy to collect reliable information on a treatment's possible benefits and harms once it is on the market.**

**ANSWER: TRUE**

The licensing and release of a new treatment marks a high point in collecting treatment information. Information gathered before licensing is usually of high quality. It is also "narrow" because of the tightly controlled conditions that exist when studies are done. This is because pre-licensing studies aim to show whether a treatment *can* work under the best conditions, rather than whether it *will* work in real world use. When it comes to negative effects, only the

most common ones are seen in the studies because the number of people in the studies is often low.

After licensing, most of the focus turns to **negative effects**. Why? Because a good picture of these effects is more likely to emerge after large numbers of people are exposed to the treatment.

Getting information about **the benefits** of treatments after licensing is a complex problem. It involves the need for clinical studies when the desire of patients to be part of such studies is low since the drug is already on the market and easy to access.

Here is an example of how this has manifested in recent years: in cases of serious life-threatening disease where there is no available treatment on the market, Health Canada can allow licensing before the treatment has been clearly proven to provide direct benefit to patients.

The disappointing truth is that many experimental treatments have failed to pass the critical test of whether they really can work and are safe enough.

There are other impacts on patients if there is any doubt about the benefits and harms of treatments. These include

- problems with the quality of information
- lack of funding for the treatment.

If government and private organizations do not fund the treatment, patients are not likely to have access to it because the treatment — especially new ones — may be too expensive.

Some patient groups have turned to advocacy (lobbying) of Health Canada to get up-to-date information about the *positive effects* of licensed treatments.

- 4) **There can be differences of opinions among the experts about the possible positive and negative effects of a treatment, both before and after it is licensed.**

**ANSWER: TRUE**

In the end, it comes down to human judgement. Even though scientific studies use the same objective data, people come to different conclusions about what the data mean.

- 5) **It is clear what the most effective dose is for someone like me (the dose that will provide the best chance of experiencing the treatment's benefits while reducing its negative effects).**

**ANSWER: MAYBE**

A person may experience the possible negative effects of treatment when they receive higher doses of a treatment. This is why it makes sense for patients to receive the lowest dose that will still provide enough benefit. A doctor cannot know what the best dose is for you ahead of time. You may need to try different doses of a treatment before finding the best one.

- 6) **The best practice of science results in answers and truths that everyone can understand and agree on.**

**ANSWER: FALSE**

For a discussion about science and uncertainties, please read this (PDF will load).

### **STEP 8: IMAGINING MY POSSIBLE OUTCOMES**

- 1) **Licensed treatments work for everyone.**

**ANSWER: FALSE**

- 2) **Licensed treatments are highly effective.**

**ANSWER: MAYBE**

When they apply to have a treatment licensed, companies do not need to show that their treatment performs better than treatments that are already on the market. Instead, they need to show that for the target population

- the possible benefits of their treatment outweigh the possible harms, and
- critical questions about this have been resolved.

As well, because each person is different, people do not respond the same way to the same treatment. Some people may experience great benefit while others do not. Some people may have negative effects while others do not. It is good when more than one treatment option exists for a certain disease or condition. Then, if one treatment does not work, another can be tried.

- 3) **Licensed treatments are safe.**

**ANSWER: MAYBE**

- 4) **Licensed treatments carry very few chances of negative effects.**

**ANSWER: MAYBE**

- 5) **Other and different treatments are sometimes needed to manage the negative**

effects of treatments used for the primary disease.

**ANSWER: TRUE**

**STEP 9: WEIGHING THE POSITIVE EFFECTS, NEGATIVE EFFECTS AND UNCERTAINTIES OF MY TREATMENT OPTIONS**

- 1) People with the same disease can have very different life circumstances that may affect how they see and feel about the positive and negative effects of a certain treatment.

**ANSWER: TRUE**

- 2) People with the same disease can have very different personal values that may affect how they see and feel about the positive and negative effects of a certain treatment.

**ANSWER: TRUE**

**STEP 10: IDENTIFYING TRADE-OFFS I AM WILLING TO MAKE THOSE I AM NOT**

- 1) I cannot have it all: get the possible benefits without exposing myself to possible harm.

**ANSWER: TRUE**

- 2) I need to think about how much harm, discomfort, pain or other negative effects I am willing to experience in exchange for how much benefit I might experience.

**ANSWER: TRUE**

- 3) Everyone has the same level of tolerance for possible harm from treatments.

**ANSWER: MAYBE**

- 4) Everyone has the same level of tolerance for uncertainties that relate to a treatment's effects.

**ANSWER: MAYBE**

**LASTLY, PLEASE REMEMBER...**

- 1) It is important to give my healthcare team a complete picture of my experiences with

**treatment, both positive and negative, in order to help me and other patients get the best care possible, in the end.**

**ANSWER: TRUE**

You should report when the treatment is

- not working for you,
- not working well enough
- giving you troublesome effects
- giving you toxic effects.

When doctors and others on your healthcare team report this to Health Canada or to the company that makes the drug or product, experts have an updated understanding about the treatment. This helps to reduce uncertainties about it.



# APPENDIX 3: A short guide to drug regulation and access to drugs in Canada

## 1. How prescription drugs and products are regulated

Health Canada regulates prescription drugs and biologic therapies and products (such as radiation, hormones, vaccines or blood products). Companies cannot market their drugs or products unless Health Canada approves them for use and sale in this country.

The Health Products and Food Branch (HPFB) of Health Canada implements the *Food and Drugs Act* and its *Regulations*. This work begins before a drug enters the market and continues while it is on the market.

Visit Health Canada’s website to learn more about these parts of the department:

- Health Products and Food Branch
- Therapeutic Products Directorate
- Biologic and Genetic Therapies Directorate

### Health Canada authorizes clinical trials

Companies and research groups (known as sponsors) conduct clinical trials in Canada.

As a patient, you may be asked to be part of a clinical trial. You may say yes or no to this request.

The purpose of a clinical trial is to gather information on a product’s safety and how well it works, by testing it on humans. Health Canada must approve all clinical trials that occur in Canada. It is involved because the goal of a clinical trial is to find out if a drug, or product, is safe and effective for people in Canada.

Before a clinical trial begins, Health Canada reviews the sponsor’s proposal to make sure that

- the clinical trial will follow the proper rules
- patients will not be exposed to too much harm
- the patient consent form allows for informed consent.

Health Canada’s review at this stage also looks at any studies the sponsor has already done on animals and on humans.

Health Canada also inspects clinical trials while they are happening. The goal is to see if the clinical trial is following proper rules. As well, if someone complains to Health Canada’s HPFB, it will determine if there are problems with the clinical trial.

## Health Canada licenses drugs for sale in Canada

New drugs or medical products can be sold in Canada only after Health Canada determines their safety, quality and how well they work. Companies and research groups (known as sponsors) must submit a New Drug Submission (NDS) to the Therapeutic Products Directorate, which does the assessment.

When Health Canada approves a drug or product, the drug or product is licensed for use and sale in Canada. This means Health Canada has decided that its therapeutic benefits are greater than the potential for harm, when the drug is used as proposed. The HPFB at Health Canada then issues a Drug Identification number (DIN) for the drug or product.

### Health Canada issues a Product Monograph

As part of approving (licensing) a new drug, Health Canada requires the sponsor (company) to provide details to the public about the drug or product's characteristics and how it should be used. This document is called a Product Monograph. Health Canada must approve the Product Monograph, which appears as a printed leaflet inside the box that the product comes in.

You can find electronic copies of Product Monographs, and other important information on authorized drugs, online using Health Canada's Drug Product Database. The database allows you to search for a drug or product using its DIN.

### Health Canada issues Notices of Compliance, with or without conditions

HPFB also has other powers. It can

- reject a New Drug Submission if the product does not meet the standards of proof (evidence) required by Health Canada
- change the status of a product (approve it), if the sponsor provides proof of its benefits
- approve a drug without conditions (called a Notice of Compliance, NOC)
- approve a drug with conditions (called a Notice of Compliance with Conditions, NOC/C).

The NOC/C may occur when there is "extraordinary patient need" and where positive effects from the drug may exist but have not been proved. In this case, the company (sponsor) must continue studies to confirm that the product can provide benefits and also continue to collect evidence of any negative effects. This website provides details on products that Health Canada has granted a NOC/C.

### Health Canada can authorize changes to products already on the market

#### Other government bodies in other parts of the world

Product Monographs and information on drugs and treatments licensed outside Canada are available from:

- The United States Food and Drug Administration
- The European Medicines Agency of the European Union.

HPFB can change a drug or product's status, and even remove it from the market. It can also change its conditions of use due to "bad news." This may occur if safety problems arise.

Changes may also reflect "good news" based on new information. For example, changes to drugs or product on the market may mean they are approved

- for more patients (or for fewer)
- for people with more or less severe versions of a disease
- for people with a different disease
- in higher or lower doses
- for shorter or longer periods of time.

Changes to drugs already on the market will require the company to issue a new Product Monograph. Patients may check for a new Product Monograph at the same database mentioned earlier.

Health Canada informs the public and health care workers of problems with products on the market through its online Warnings and Advisories.

## **2. What you need to know about access to drugs**

As a patient, your access to drugs and biologic products depends on decisions along the way that are designed to protect health and finances.

- When Health Canada authorizes a drug or product, one of two bodies to decides whether the product's benefits justify its cost, based on the price the company has set. These bodies are CADTH (national) and INESSS (in Quebec).
- Access to prescription drugs requires an appointment with a doctor or other healthcare provider and, of course, a prescription.
- The Special Access Programme (SAP) of Health Canada provides compassionate access to unlicensed drugs and products in special cases. SAP allows a doctor to prescribe an unlicensed product:
  - for emergency use
  - if other treatments have failed,
  - If other treatments are not suitable or not available
  - if a patient cannot be part of a clinical trial of the drug or product.

Sometimes, a treatment that is not licensed in Canada is licensed in the United States or Europe. One of SAP's drawbacks is that the patient's experience with the drug or product is not measured or captured. This means the results are mostly lost. SAP does not help experts determine whether a treatment works well and is safe.

## APPENDIX 4: References and suggested reading

- 1) ***Smart Choices. A Practical Guide to Making Better Decisions***  
John S. Hammond, Ralph L. Keeney, Howard Raiffa. 1999. Broadway Books, New York
- 2) ***Risk. Why We Fear the Things We Shouldn't — and Put Ourselves in Greater Danger***  
Dan Gardner. 2008. Emblem, McClelland and Stewart, Toronto.
- 3) ***Thinking, Fast and Slow***  
Daniel Kahneman. 2011. Farrar, Straus and Giroux, New York.
- 4) ***Calculated Risks. How to Know When Numbers Deceive You***  
Gerd Gigerenzer. 2002. Simon and Schuster, New York.
- 5) ***Gut Feelings. The Intelligence of the Unconscious***  
Gerd Gigerenzer. 2007. Penguin, London.
- 6) ***Communicating Uncertainties About Prescription Drugs to the Public***  
Schwartz, L.M., Woloshin, S. Arch Intern Med. 2011; 171(16): 1463-1468  
Accessible at: <http://archinte.jamanetwork.com/article.aspx?articleid=1105910>
- 7) **Dartmouth Medical School Medical Statistics Quiz (Schwartz, L., Woloshin, S., Welch, G.):**  
[http://dartmed.dartmouth.edu/spring08/pdf/disc\\_drugs\\_we/quiz.pdf](http://dartmed.dartmouth.edu/spring08/pdf/disc_drugs_we/quiz.pdf)  
  
[Click here for answers to the quiz](#)  
[http://dartmed.dartmouth.edu/spring08/html/disc\\_drugs\\_we\\_answers.php](http://dartmed.dartmouth.edu/spring08/html/disc_drugs_we_answers.php)
- 8) ***The Pills of Last Resort***  
Darshak M. Sanghavi. In: The New York Times, November 3, 2013.  
Accessible at:  
[http://www.nytimes.com/2013/11/03/magazine/how-dying-patients-get-access-to-experimental-drugs.html?pagewanted=1&\\_r=1](http://www.nytimes.com/2013/11/03/magazine/how-dying-patients-get-access-to-experimental-drugs.html?pagewanted=1&_r=1)
- 9) ***Be Lucky***  
Zeitler, K. In: Pulse — voices from the heart of medicine. November 29, 2013.  
Accessible at: <http://pulsevoices.org/archive/stories/328-be-lucky>
- 10) ***Getting Good Information from the Internet***  
This guide was developed at the First Invitational International Forum on Cross- Border Reproductive Care, Jan. 14–16 ???, Ottawa, Canada. It is based on information from the Health on the Net Foundation's website at:  
[www.healthonthenet.org](http://www.healthonthenet.org)
- 11) ***Making Sense of Uncertainty. Why Uncertainty is Part of Science***  
From the website, Sense about science  
Accessible at: <http://www.senseaboutscience.org/resources.php/127/>