Understanding Treatment Goals and Evidence to Support Decision-Making

A Discussion Guide for Patients and Their Doctors

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- This tool is for people diagnosed with metastatic breast cancer (mBC) who are talking about treatment options with their doctor
- Your voice matters. While your doctor is the mBC expert, you are the expert on yourself. It is important that you help your doctor understand your treatment goals and priorities
- To do this, it is important that you have a clear picture of the treatment options available to you, including how they have been studied
- The evidence supporting the use of available treatment options may come from clinical trials (required for a treatment to be approved for its use in patients) and from real-world evidence (that complements the data from clinical trials by providing information on how the treatment works in everyday life)
- Understanding these types of studies can help you to make your treatment decision. This tool will explain these concepts in more detail
- It’s important you share what’s on your mind. There are spaces throughout this document for you to write down any questions or useful information that you may want to talk about with your doctor
- You may wish to use this tool to explain the information available for your treatment options to your family and friends

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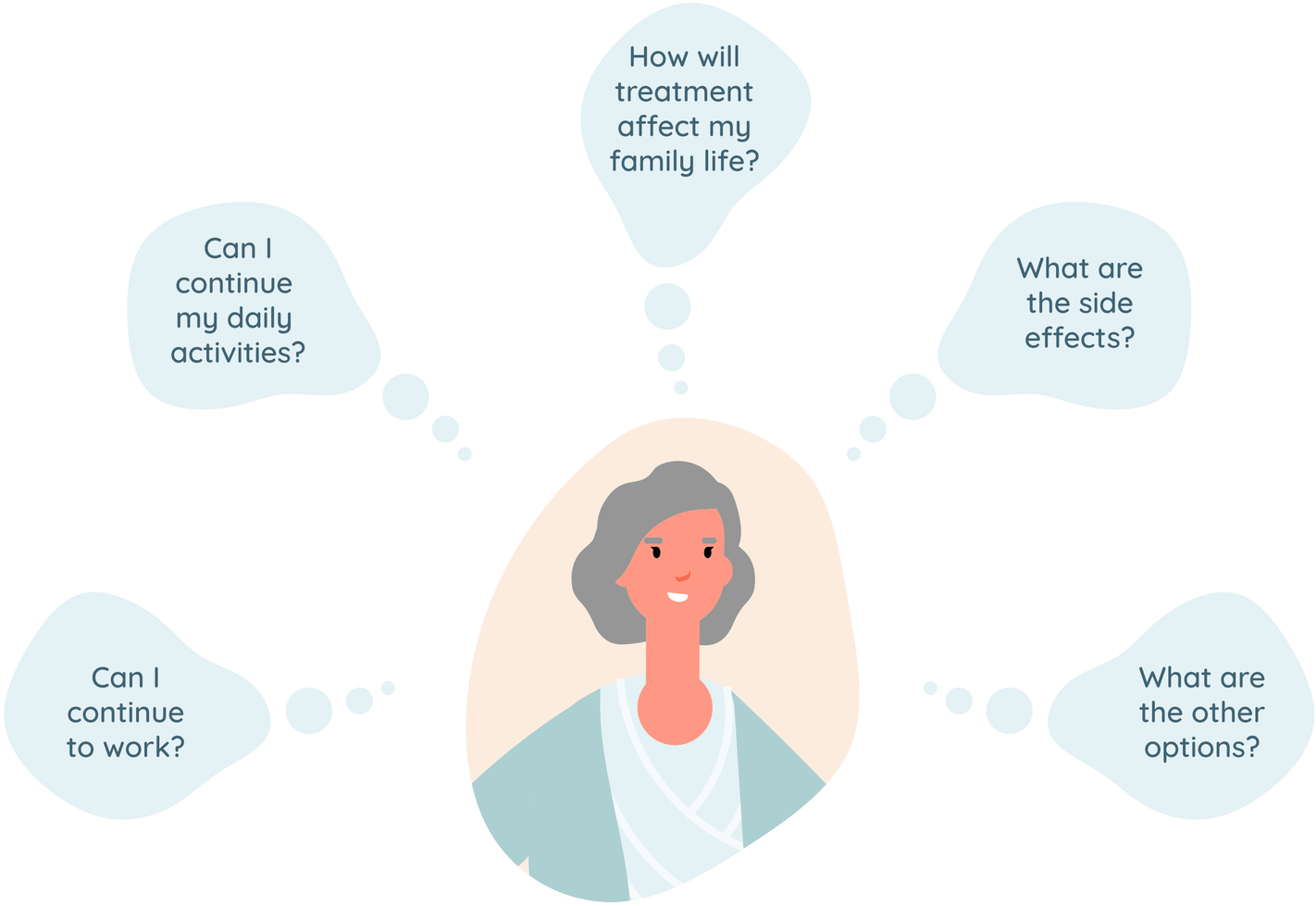
Understanding Your Treatment Goals

Understanding your treatment goals can help you make the best treatment decision for you. It is important that you talk to your doctor about your goals and priorities so they can support you in making this decision.



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While your doctor is the expert in mBC, you are the expert on yourself. Everyone's priorities differ, and only you know what matters most to you. Knowing your needs, feelings, and goals can help your doctor work with you to make informed decisions about your treatment.

For many people diagnosed with mBC, treatment discussions may focus on how long a treatment will work to control their disease.

It is also important to consider how you will feel as you live your life while on treatment for mBC. Consider the example questions below. Are there any that you particularly relate to when thinking about how you want to live your life while being treated for mBC? There may be other questions that you think of that are not listed. Feel free to write these in the space below to talk about with your doctor.

Can I carry on working?

Will I be limited in doing daily activities at home or outside?

How will my treatment affect my family life?

What are the side effects associated with this treatment, and are they easy to manage?

Can the dose of my treatment be reduced if I get side effects – and will it still work equally well?

What is other patients' experience with controlling their disease?

How will my doctor and my medical team help me during my treatment?

Are there other treatment options which might meet my personal preferences and goals?

For more information on setting short- and long-term treatment goals and discussing these with your doctor, please visit 'A Goal Agreement for Patients and HCPs', available from the Useful Resources section.

Use the space below to write your thoughts on your goals and priorities for treatment. Think about the time of treatment, use in daily life and side effect management

It is important to understand the evidence supporting treatment options to help you make the best treatment decision for you. The next sections explain these types of evidence in more detail.

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Different Types of Evidence to Support Treatment Options

Learning about the treatment options available to you, how they have been studied, and the **data** available from these studies can help you when discussing options with your doctor.

There are different types of studies, but two main types are clinical trials and real-world studies. Both give information on the **efficacy** and **safety** of a treatment and how patients feel while taking it, but how they are studied is different:

- **Clinical trials – give information about the efficacy and safety of a treatment in a specifically defined group of patients**[1]
- **Real-world studies – give information on how well the treatment works in everyday clinical practice**[2]

Data are specific pieces of information collected from studies[3]

Safety is how well a treatment is tolerated and the side effects it may cause[4]

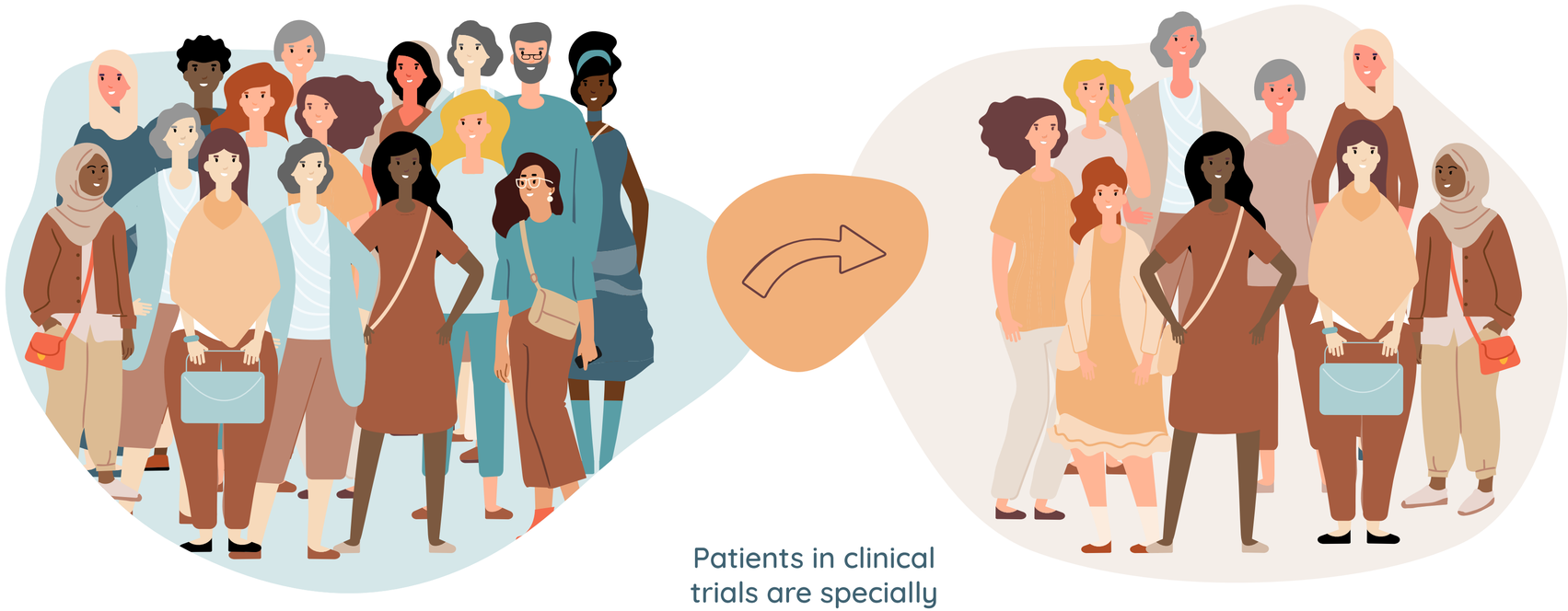
Efficacy is a measurement of the benefits of a treatment[5]

In order to help you make your treatment decisions it is helpful to know about these two different types of evidence. In order the understand real world studies, it is important that you first understand clinical trials. The next section looks at these in more detail.

Clinical Trials

Clinical trials are tightly-controlled studies that assess a treatment before it is approved by the government for use in routine clinical practice.[5]

Clinical trials are the gold standard for testing new ways to prevent, improve, or treat diseases.[1]
They assess a treatment's **efficacy** and **safety** under strictly controlled conditions.[5]



Patients in clinical trials are specially selected from the population of patients with mBC

Safety is how well a treatment is tolerated and the side effects it may cause[4]

Efficacy is a measurement of the benefits of a treatment[5]

Real-World Studies

Real-world studies give information about how a treatment already available for use in patients is used and how it performs in everyday care outside of clinical trials.[6]

Real-world studies collect data from patients living with the disease and receiving treatment. This is known as real-world data. This data is analysed according to a plan to result in real-world evidence (RWE). This evidence provides more information into how a treatment works, and its side effects from people using it in everyday clinical practice.

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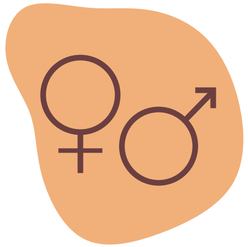
Real-world evidence (RWE) can be an important source of information when deciding on treatment with your doctor.[7]

Participants in real-world studies are patients who are taking the treatment and can look like the everyday patients treated for the disease.

This means there are many people with different features, such as:

Different ethnic backgrounds



Different genders

Large age ranges including elderly patients

Patients from rural communities

Patients with other medical conditions in addition to their cancer such as heart disease, arthritis or diabetes

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Real-world evidence can help you answer questions about treatment that are specific to you

Real-world evidence (RWE) can give information on the experience patients have with treatment after after it is approved by the government and it is being used in a wide population.[8]
The treatment is being used in everyday clinical practice, outside a clinical trial setting.

Where Does Real-World Evidence Come From?

Real-world studies collect data according to strict patient privacy laws, from multiple sources such as patient files, patient-reported data (like questionnaires), digital devices and apps, and information from pharmacies and insurance.

This means the evidence can give important extra information to what is gathered in clinical trials, from a wide variety of patients. Real-world evidence (RWE) is becoming an increasingly important source of information on how effective a treatment is when it is used in everyday clinical practice.
It complements the robust data collected from clinical trials and can help you to answer the question, **“will this treatment work for me?”**.

Patient registries

Patient questionnaires

Patient health records

Electronic devices and digital health applications

Information from insurance and pharmacies

What is the Difference between Clinical Trials and Real-World Studies?

Provides information on the efficacy and safety of a treatment in a controlled clinical trial setting

Restricted patient population

Provides information on a drug often before it is approved for use in the wider population

Provides information on the efficacy and safety of a treatment in everyday clinical practice

Broad patient population

Provides information on a drug after it has been made available to the wider population

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Real-world evidence (RWE) cannot be directly compared to clinical trials.

Clinical trials give us important information on the efficacy and safety of the treatment. They are used to determine if a treatment should be approved for use in the general population. To be able to do that, they need a strictly controlled setting, and patients are selected based on specific and strict rules. This means that some patients, such as those who are elderly or have many other medical conditions are not included, which can lead to the underrepresentation of certain patients but increases the quality and conclusiveness of data.[9]

Real-world evidence (RWE) offers information about a treatment being used in everyday clinical practice. When a patient takes a medication, the results and sometimes their experiences are recorded.[10] This means the data comes from everyday clinical practice and from a diverse range of patients – the data is more representative of the 'real-world'. It also means that real-world evidence (RWE) may provide more information on groups that have been less represented in clinical trials.[9]

Clinical trials are the standard for researching new treatments. Real-world evidence (RWE) can give important extra information about how well a drug works in everyday clinical practice. This information complements each other and can help you understand the treatment options for mBC and how they work.

Safety is how well a treatment is tolerated and the side effects it may cause[4]

Efficacy is a measurement of the benefits of a treatment[5]

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How Can This Information Help Your Treatment Decisions?

Understanding what matters most to you and learning about a treatment, including the evidence supporting it, can guide treatment discussions between you and your doctor.

Real-world evidence (RWE) should not replace the data from clinical trials but can be another useful source of information to look at when deciding on a treatment. Real-world evidence (RWE) can be collected from a diverse range of patients and look at many aspects of a treatment. It gives you information about how a treatment is doing in 'everyday clinical practice' – in the 'real-world'.[6]

Understanding the totality of evidence, from both clinical trials and real-world evidence (RWE), can help you to think about how a treatment might help you personally and align with your individual goals and priorities.[7] It can help you answer the question – "what does it mean for me?".

Use the space below to write down your goals and priorities, and how they can fit into your real-world.

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What's Next? Staying Involved in Your Treatment Decisions

Take some time to go through the information in this tool in more detail. You may wish to share this information with family and friends. You can talk about how real-world evidence, clinical trials and treatment goals can help your treatment decisions. More information is in the 'Useful Resources' section below.

It is important you keep thinking about your goals and priorities – only you know what matters most. This should be considered when making decisions about your treatment with your doctor. By staying well-informed and sharing your treatment priorities and goals, you can help to make sure your treatment is best suited for you and your well-being.

Use the space below to write down 5 top questions or comments you want to talk to your doctor about at your next appointment.

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Useful Resources

Explore the links below for more information on some of the concepts introduced in this tool and to help with setting goals and making the most out of your conversations with your doctor.

- Shared decision-making: A Conversation Toolkit for Patients with mBC (https://www.breastcancervision.com/patient-toolkit)
- Real-world evidence: About Real-World Evidence (https://www.ispor.org/strategic-initiatives/real-world-evidence/about-real-world-evidence)

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