***Clinical Trial in patients with a severe bone marrow disorder.***

**THE TRIAL AT A GLANCE**

Dear patient,

I recently informed you that you have a **serious bone marrow disorder**. This means that your bone marrow does not produce blood cells the way it should, and is therefore seriously disturbed. For quite a few people this will later lead to **leukaemia or blood cancer**. Maybe you already have leukaemia. I also told you that in your case we cannot use the intensive and aggressive – meaning more 'poisonous' - chemotherapy.

That is why we invite you to take part in a clinical trial (further referred to as "the trial") that is meant to evaluate an investigational medicinal product for the treatment of your disease or condition.

Before you agree to take part in this trial, we want to fully inform you about the trial and its implications in terms of organisation, and its possible risks and benefits, so you can decide for yourself if you want to take part. This process is known as giving "**informed consent**".

This chapter will already **give you an idea** of what will happen during this trial, but we nevertheless ask you to read all the pages, even if it will take you a lot of time. It is important that you read and understand all the information. If you don't do this, you will take part in the trial without knowing what you’re signing up for. So ask me all your questions.

In this trial, the **investigational medicinal product BEGREM****[[1]](#endnote-1)** will be tested. This investigational medicinal product **has not yet been approved by the Belgian authorities**. It has not yet been proven that it can cure, improve or stabilise your disease or condition. That is what we want to demonstrate with this trial, **so it is uncertain at this point whether you will benefit from it**. That is precisely what we want to find out: does the investigational medicinal product work, does it not work, or not enough.

In concrete terms, this means that during the trial you will receive the standard chemotherapy SABORDI1 via an injection under the skin or via a drip in the vein (as do all patients in your situation). If you take part in the trial, you will receive in addition the investigational medicinal product BEGREM. You will take the investigational medicinal product in the form of a tablet.

If you agree to take part in the trial, I will carry out **prior tests** to check whether you meet all the conditions to be accepted for this trial. Among other things, I will take a bone marrow biopsy, which is painful, despite the anaesthetic I will give you. Only if you meet all the conditions to take part, will we start the standard chemotherapy PLUS the investigational medicinal product.

**I cannot tell you yet how long this trial will take**. In order to tell you that, I need to know how well the investigational medicinal product works for you, and how well you tolerate the treatment. You will also have to fill in questionnaires. Even after the period of administration of the investigational medicinal product has ended, you will remain in the trial and will be followed up during the consultations and by telephone, in order to find out how you are doing.

It is also very important that you know that all medicinal products have **side effects**. These side effects can be very serious. Therefore, it is really important that you **report any side effects or any new health problem to me**.

The sponsor of this trial, the **company SPOCT1**, has taken out **insurance** for this trial.

You are **not allowed to become pregnant** or to get someone pregnant during the trial and for some time after it. I will discuss with you the appropriate method of contraception.

**SPOCT** has designed this trial and has asked me and the hospital - together with other investigators and hospitals - to conduct it, and pays us a fee for it.

This means that all treatments and examinations that you will undergo or receive in the context of the trial will be **free of charge for you**. All **other** treatments or examinations that you would have undergone anyway if you did not take part in the trial, **must be paid for by your health insurance and by yourself**. If you need to use contraception, this will be reimbursed by SPOCT.

The data collected during this trial will be **treated confidentially**.

You should also know that within the framework of this trial, a number of **biological samples** (e.g. blood, urine, bone marrow) will be taken and transferred to SPOCT. These samples will be stored for a very long period of time, and will be used by SPOCT to perform other tests as well. Me or my staff will give you clear information about this, and you will have to decide what you do or do not want. It is important to think about it carefully.

**There is one thing that I would like to emphasise very strongly: you are not obliged in any way to take part in this trial**. Even if you have already started the trial, you can stop at any time. I will fully understand your decision, and will continue to take care of you as before.

The authorities and an ethics committee have evaluated this trial. It is not because they have approved this trial that you should feel obliged to take part.

Once the trial is over, it is possible that you may be eligible to be treated further with the **investigational medicinal product via an extension trial**.

To be able to take part in this trial, you must, for your own safety, **agree that I, the investigator, inform your treating physicians** of your participation in this trial**.** You are **not allowed to take part in another clinical trial at the same time** without informing the researcher or the trial staff. We may refuse that participation to other trials for justified reasons. It is also very important that **you cooperate** and follow the instructions that the trial staff and I give you regarding the trial. You will receive an "emergency card", which says that you are taking part in a clinical trial. You must carry this card with you at all times; this is necessary for your safety should you have to undergo emergency treatment in a hospital where they don’t know you.

If you agree to take part, you will need to sign the informed consent form. I will also sign the form and thereby confirm that you have received the necessary information about the trial. You will receive a signed and dated copy of the form.

Now that you have some idea what this trial is about, please take your time to read the other pages of this document. You do not have to do that all at once. It is important that you understand what you are reading. Feel free to discuss the trial with a trusted person (for example a friend, relatives, your family doctor). My staff and I are also available to help you if there is anything that is not clear. It is our job to make sure that you understand all the information.

With my best regards

Your treating physician and investigator

Dr. Thomas Schwencke

1. The names of the trial medicines and the name of the sponsor are fictional. [↑](#endnote-ref-1)