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Blood and Marrow Transplant Program
of the University of Minnesota Medical Center, Fairview

BIOCHEMICAL CORRECTION OF SEVERE EPIDERMOLYSIS BULLOSA BY ALLOGENEIC STEM CELL TRANSPLANTATION

Adult Consent For Screening

Investigator: Jakub Tolar, M.D., Ph.D.

Introduction

You have a diagnosis of a severe form of Epidermolysis Bullosa (EB) and you may be eligible to take part in a clinical research study. This consent form gives detailed information about the tests and questions involved in the research study screening process.

Clinical research tries to find better ways to diagnose and treat disease. Taking part in any clinical research involves risks and benefits. You need to understand these risks and benefits to make an informed decision about whether or not to participate. This process is known as informed consent.

Once you understand the nature of these tests and the potential risks of the screening procedures, you will be asked to sign this form if you wish to be screened. You will receive a copy of this consent form to keep as a record.

Separate consent form(s) will be presented to you by the research staff if you are eligible for the transplant protocol once the screening process is done.

Background

You have been diagnosed with a severe form of Epidermolysis Bullosa (EB), a disease that results in severe blistering throughout the body. EB is the result of a defective gene that is responsible for making a specific type of collagen, laminin, integrin or plakin. As a result, the outer layer of the skin and lining of the gastrointestinal tract are unable to anchor themselves to the body. Blood and marrow transplantation is a therapy that may help treat your disease.

However not all patients with EB are suitable candidates for transplantation. Therefore, you will be asked to undergo screening tests and procedures to determine whether or not transplantation would be a good treatment option for you.

Screening Process
You will be asked to undergo the following procedures:

- Skin biopsies to assess the amount (if any) of collagen, laminin, intergrin or plakin (specific for your disease)
- Skin fragility testing
- Photographs (of the skin lesions)
- History and physical examination
- Routine blood tests (requiring approximately 3 teaspoons of blood) to test liver, kidney and bone marrow function
- Routine evaluations of heart and lung function
- Quality of life survey (17 questions)

Side effects and potential risks
Skin biopsy. The most common side effect is slight soreness for several days at the site of the skin biopsy. It may be associated with some discomfort from the placement of the lidocaine, the anesthetic. There may be bleeding and bruising. Rarely, infection may occur. The skin biopsy will leave a small scar.

*Skin fragility test***.** There is mild if any discomfort from the continuous suction used to make the blister. However, the blister site will likely be very sensitive for several days to anything, like clothes that rub the area. The procedure is not likely to leave any scar.

Blood tests. There is a risk of local skin infection, pain and bleeding. While the risks are generally low, they may be higher in patients with severe EB.

Alternative treatments
Should you choose not to participate in the screening procedures we will not be able to determine your eligibility for bone marrow transplantation. Other options that could be considered include other investigational therapies at other institutions or continuing with the current care plan.

Voluntary nature of the study
Your participation in this study is voluntary and your decision whether or not to participate in this study will not affect your relations with the University of Minnesota Medical Center, Fairview or with the physicians here. If you decide to participate in the screening, you are free to withdraw at any time. You may participate in the screening and decide not to continue with the transplantation component of the study.

Costs
You and/or your insurance company will be responsible for all medical expenses relating to, or arising from, the screening procedures. Tests performed in the research laboratory will not be charged to you or your insurance company. Research tests will be explained to you separately.

Compensation for Research Related Injury
In the event the screening procedures result in an injury, treatment will be available, including first-aid, emergency treatment, and follow-up care as needed. Care for such injuries will be billed in the ordinary manner, to you or your insurance company. If you think that you have suffered a research related injury let the study physicians know right away.

Confidentiality
Information will be kept confidential; however, this study will use an external (outside of the University Of Minnesota) panel of experts in EB and pediatric transplant to review the study data and provide recommendations on the study's course. Since these persons are located in different parts of the world, study information on a patient level will be uploaded onto a secured website. Any information that would make it possible to identify you will be removed prior to being uploaded to the website site. Also, only the investigators involved in this study and the members of the panel will have access to this site.

Your information and your hospital records are confidential. Your name or any other information that can identify you will not be used in study reports or publications. The Food and Drug Administration or other authorized agencies may inspect your clinical records without removal of such identifying information.

Protected Health Information (PHI)
Your PHI created or received for the purposes of the screening procedures is protected under the federal regulation known as HIPAA. Refer to the attached HIPAA authorization for details concerning the use of this information.

Contacts
Please ask any questions you have; we want you to understand the treatment and the study as well as you can. If you have additional questions later concerning this study you are encouraged to call Dr. Jakub Tolar (612-626-2961), the principal investigator.

This research has been reviewed and approved by an Institutional Review Board (IRB) within the Human Research Protections Program (HRPP). To share feedback privately with the HRPP about your research experience, call the Research Participants' Advocate Line at 612-625-1650 or go to https://research.umn.edu/units/hrpp/research-participants/questions-concerns. You are encouraged to contact the HRPP if:

● Your questions, concerns, or complaints are not being answered by the research team.
● You cannot reach the research team.
● You want to talk to someone besides the research team.
● You have questions about your rights as a research participant.
● You want to get information or provide input about this research

Statement of physician/BMT staff obtaining consent
Sign this form if the research screening procedures have been fully explained to you and you agree to participate. Please read this consent form. You may request to have it translated into a language you understand if English is not your first language. Ask questions and talk it over with the doctor and nurse working with you before you are admitted for work-up or call the BMT Clinic at: (612) 626-2663. Your participation is voluntary.

You will be given a copy of this form to keep for your records

Statement of Consent

I have read the above information and had my questions answered to my satisfaction. I give consent to participate in the screening procedures.

SUBJECT'S SIGNATURE:_____DATE:_____

Printed name: _____

I have explained fully the above objective of the screening procedure, what is to be expected and the possible complications.

_____ _____
Signature of Person Obtaining Consent Date

_____
Printed name of person obtaining consent