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AS MODIFIED
Aug 07, 2017
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RESEARCH SUBJECT INFORMATION AND CONSENT FORM

TITLE: Comparison of Ideal vs. Actual Weight Base Factor Dosing

PROTOCOL NO.: None
WIRB® Protocol #20162706
STUDY00000329

SPONSOR: WA State Health Care Authority

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STUDY-RELATED PHONE NUMBER(S): Rebecca Kruse-Jarres, MD, MPH
Office Hours Phone Number: 206-689-6570
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SC IRB Number	Consent Version Date
STUDY00000530	2AUG2017

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STUDY-COORDINATOR(S): Heidi Thielmann, PhD
206-689-6234

This consent form may contain words that you do not understand. Please ask the study doctor or the study staff to explain any words or information that you do not clearly understand. You may take home an unsigned copy of this consent form to think about or discuss with family or friends before making your decision.

Patient Name: _____

Medical Record: _____

A person who takes part in a research study is called a research or study subject. If you are a parent or guardian signing on behalf of a child, you are being asked to provide consent on behalf of the study subject because the subject is not able to provide such consent. Minor subjects will be asked to provide verbal assent. The words "you" and "yours" in this consent form generally refer to the study subject, however, there may be some instances where "you" refers to the person providing consent.

You have the option to take part in a research study. The goals of this form are to give you information about what would happen in the study if you choose to take part and to help you decide if you want to be in the study.

Feel free to take notes, write questions or highlight any part of this form.

SUMMARY

Special proteins in your blood help you to make a clot and stop bleeding when you are cut or hurt. These proteins are called clotting factors. Hemophilia happens when there is a change or mutation in a gene (small piece of your DNA) that makes a clotting factor. This change in your gene can result in low levels of clotting factor, which can result in spontaneous or prolonged bleeding. The bleeding can be treated and prevented with clotting factor concentrate. Clotting factor replacement is administered intravenously. Factor dosing is currently based on a patient's actual weight.

This study will be conducted at four locations: Washington Center for Blood Disorders at Bloodworks Northwest, Oregon Health & Science University, Seattle Children's Hospital, and Providence Sacred Heart Children's Hospital. Subjects will be recruited at these centers, and it is expected that up to twenty patients will enroll. This study is a part of the Bleeding Disorder Collaborative for Care. The Bleeding Disorder Collaborative for Care is working to improve care to patients with bleeding disorders by developing evidence-based practices related to bleeding disorders.

About twenty subjects will participate in this study.

PURPOSE OF THE STUDY

We are conducting this study to see if the current recommended factor dosing strategy in overweight and obese patients may deliver too much clotting factor. This study will look at ways to prevent delivering too much factor by using a patient's ideal body weight as a new dosing strategy. It could be used to replace the dosing strategy of using a patient's actual body weight in overweight or obese patients. It may also help us find the safest dose for you.

PROCEDURES

In order to take part in this study, you must be a male, at least 12 years of age, and have hemophilia A. Your Body Mass Index, or BMI, must be classified as either overweight or obese for your age based on Centers for Disease Control and Prevention (CDC) definitions. You also must be willing and able to comply with the testing schedule.

In this study, you will have several blood samples taken over a period of time in order to determine how your body handles the factor product you are taking. This is called a pharmacokinetic (PK) study. The blood draws you will have for this study are referred to as PK tests. You will provide your own factor product for this study.

Prior to the first study visit after your screening visit, you will have to stop taking any FVIII (factor 8) products for either 48 hours if currently using a short-acting FVIII product, or 72 hours for a long acting FVIII product.

We will measure factor levels immediately before, and at multiple points after 2 different factor doses. At one visit, you will be given a dose of 50 U/kg for hemophilia A based on ideal body weight. At another visit, you will be given a dose of 50 U/kg for hemophilia A based on actual body weight. The order of these two doses will be determined by chance. The procedure will be the same for both tests.

The first blood draw occurs before your factor is taken, and it will measure the baseline level of FVIII in your blood. The second draw will occur 20-40 minutes after you take factor, and it will also measure the amount of FVIII in your blood. This is called the recovery level. At each draw, you will have 1 teaspoon of blood drawn for a maximum volume of 40 ml per PK test. The table below lists the time points you will have your blood drawn.

Blood draw time points:

Hemophilia A – regular half-life product

Baseline	Measured when you arrive at clinic
Recovery - after factor is taken	20 - 40 minutes
	5 to 7 hours
	20 to 26 hours
	44 to 50 hours

Hemophilia A – extended half-life factor

Baseline	Measured when you arrive at clinic
Recovery- after factor is taken	20 - 40 minutes
	5 to 7 hours
	20 to 26 hours
	44 to 50 hours
	69 to 75 hours
	93 to 99 hours

Blood draws to measure half-life of the factor product at the remaining time points can be drawn in other local labs. If you can't come to this center, you can go to another lab. The staff here can arrange this for you. If the 20 – 40 minute recovery draw is missed at clinic, you can still be included in the study. You can have your blood drawn again within two months of the first attempt.

If you have a bleeding episode before coming to clinic, testing will be delayed until the bleed stops. If you have a bleed after any of the recovery draws, then you will need to start that PK test series again. You should treat with your regular factor regimen if a bleed occurs.

The experimental parts of this study are:

- being randomly assigned to receive your factor VIII by idealized weight or actual weight in a particular order,
- taking a dose based on idealized weight one time instead of taking your usual dose, and
- the additional blood draws.

RISKS AND DISCOMFORTS

Physical Risks

When you give a blood sample, you might feel a little pain from the needle stick. You might feel light-headed or faint. Later, you might have a bruise, and there is a small risk of infection.

It is not known if the lower dose based on ideal body weight is enough to prevent bleeding. Therefore you could be at a potentially higher risk for bleeding when taking the lower dosing at ideal body weight. If you have a bleed, treat with your usual regimen.

Privacy Risks

We do not think that there will be risks to your privacy and confidentiality by sharing your test results with the investigators, but absolute confidentiality cannot be guaranteed.

NEW INFORMATION

You will be told about any new information that might change your decision to be in this study. You may be asked to sign a new consent form if this occurs.

BENEFITS

You may not receive direct benefit by being part of this study. During the study, you will gain information about factor levels in your body based on different factor dosing strategies. Information learned from this study may help others in the future.

COSTS

If you join the study, costs to you would include your usual insurance deductibles and co-payments. All of your insurance company's usual rules would apply.

You will be asked to provide your own factor product for this study. Any blood draws performed for research purposes only will be covered by the study.

PAYMENT FOR PARTICIPATION

The IRS has certain rules about paying people who take part in research studies. If you took part in this study, we would ask you to provide your name, mailing address, and social security number so we could pay you.

You can be in this study even if you do not give us this information. If you decide not to give us this information, you would receive no payment.

The payments you would receive for being in this study might be taxable. Seattle Children's is required to report to the IRS study payments of $600 or more made to anyone in any year.

You may receive up to $600 for participating in the research blood draws as outlined in the below table. You will receive either a reloadable debit/gift card called a ClinCard or a check. It is your choice. We prefer to pay you with a ClinCard, but a check can be used instead. You will need to provide your Social Security number to receive payment. The study staff will provide you with additional information about how the ClinCard works. It is important that you do not lose the ClinCard. Costs for replacing a lost or stolen ClinCard will be your responsibility. The cost to replace the ClinCard is $7. The ClinCard will be given to you at one of your clinic visits. It will be given to you within two weeks of completing the extra blood draws. The check will be mailed directly to your home.

	Compensation
PK1: baseline visit, labs at baseline, immediately after and at 5-7 hours	$100
PK1: follow-up labs at 20-26 hrs	$50
PK1: Follow-up labs at 44-50 hrs	$50
PK1: labs at 44-50 hrs [EHL only]*	[$50]
PK1: labs at 69-75 hrs [EHL only]*	[$50]
PK2: baseline visit, labs at baseline, immediately after and at 5-7 hours	$100
PK2: follow-up labs at 20-26 hrs	$50
PK2: Follow-up labs at 44-50 hrs	$50
PK2: labs at 44-50 hrs [EHL only]*	[$50]
PK2: labs at 69-75 hrs [EHL only]*	[$50]

*EXTENDED HALF-LIFE PRODUCT

ALTERNATIVE TREATMENT

The alternative is not to join the study. You will continue to receive your factor VIII regardless of your participation in this study.

CONFIDENTIALITY

If you take part, we will make every effort to keep your information confidential.

We will store all of your research records in locked cabinets and secure computer files. We will not put your name on any research data. Instead, we will label your information with a study number. The master list that links a person's name to their study number is stored in a locked cabinet or on a secure computer file.

If results of this research are published, we would not use information that identifies you.

We would only use your information for research. These are some reasons that we may need to share the information you give us with others:

- If it's required by law.
- If we think you or someone else could be harmed.
- Sponsors, government agencies, such as the FDA or research staff sometimes look at forms like this and other study records. They do this to make sure the research is done safely and legally. Anyone who reviews study records would keep your information confidential.
    - Agencies or sponsors that may look at study records include:
        - WA State Health Care Authority, Bloodworks Northwest, Seattle Children's, Oregon Health & Science University, Providence Sacred Heart Children's Hospital, University of Washington and its affiliates
        - Western Institutional Review Board, Seattle Children's Institutional Review Board

If you join this study, we would put information about this study in your medical record. We do this because the research study involves patient care.

We may keep your results for up to twenty-five years, which could be through 12/31/2041.

COMPENSATION FOR INJURY

If you are injured as a direct result of this research study, Seattle Children's Hospital will provide treatment, or will refer you for treatment if needed. Neither you nor your insurance company will be billed for this treatment. This is the only compensation offered for study-related injuries. It is important that you tell the study doctor, Dr. Amanda Blair, if you think that you have been injured as a result of taking part in this study. You can call the study doctor at 206-987-2106.

VOLUNTARY PARTICIPATION AND WITHDRAWAL

Your participation in this research study is **voluntary**. You may decide not to participate or you may decide to leave the study at any time. If you choose to leave the study, it will not affect your care at Seattle Children's. You will not lose any benefits or be penalized if you choose to leave the study.

If you decide to be in the study, you can change your mind at any time. We ask that you tell Dr. Amanda Blair. You can contact her by calling (206) 987-2106.

The study doctor or the sponsor may also stop your participation in the study without your consent at any time for any reason. Possible reasons may include:

- It is in your best interest.
- You do not consent to continue in the study after being told of changes in the research that may affect you.

A description of this clinical trial will be available on http://www.ClinicalTrials.gov, as required by U.S. Law. This Web site will not include information that can identify you. At most, the Web site will include a summary of the results. You can search this Web site at any time.

SOURCE OF FUNDING FOR THE STUDY

Funding for this study comes from the general fund for Washington State, appropriation funding for fiscal years 2016 and 2017 as well as the Washington State Health Care Authority's administrative account.

QUESTIONS

If I have questions or would like to know about …	**You can call …**	**At …**
• **Emergencies** • **General study questions** • **Research-related injuries** • **Any research concerns or complaints**	**Dr. Amanda Blair**	**Phone: 206-987-2106**
• **Emergencies** • **General study questions** • **Research-related injuries** • **Any research concerns or complaints**	Cancer and Blood Disorders Center Ask for the Oncologist on call	**Phone:** 206-987-2106 **Phone:** 206-987-2000 Emergency Number (Nights/Weekends)

If you have questions about your rights as a research subject or if you have questions, concerns,

input, or complaints about the research, you may contact:

Western Institutional Review Board® (WIRB®)
1019 39th Avenue SE Suite 120
Puyallup, Washington 98374-2115
Telephone: 1-800-562-4789 or 360-252-2500
E-mail: Help@wirb.com

WIRB is a group of people who perform independent review of research.

WIRB will not be able to answer some study-specific questions, such as questions about appointment times. However, you may contact WIRB if the research staff cannot be reached or if you wish to talk to someone other than the research staff.

Do not sign this consent form unless you have had a chance to ask questions and have gotten satisfactory answers.

If you agree to be in this study, you will receive a signed and dated copy of this consent form for your records.

SIGNATURE LINES

Your signature on this form would mean:

- The research study was explained to you.
- You had a chance to ask all the questions you have at this time. All your questions have been answered in a way that is clear.
- You understand that the persons listed on this form will answer any other questions you may have about the study or your rights as a research study participant.
- **You have rights as a research participant. We will tell you about new information or changes to the study that may affect your health or your willingness to stay in the study.**
- By signing this consent form, you do not give up any of your legal rights. The researcher(s) or sponsor(s) are not relieved of any liability they may have.

Choose appropriate bulleted statement(s) below:

- o You agree to take part in the research study.
- o If the person reading this form is a parent/guardian, you agree to have your child take part in this research study.

Please Note: If the person taking part in this research study is a foster child or a ward of the state, then please tell the researcher or their staff.

Printed Name of Research Participant

Signature of Research Participant (required if 13 years or older)

Date *Time*

Printed Name of Parent or Guardian

Signature of Parent or Guardian

Date *Time*

Printed Name of Parent or Guardian

Signature of Parent or Guardian

Date *Time*

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For study team use only:

If signature of second parent not obtained, indicate why: (select one)

- ❑ The IRB determined that the permission of one parent is sufficient.
- ❑ Second parent is deceased, unknown, incompetent or not reasonably available
- ❑ Only one parent has legal responsibility for the care and custody of the child

Researcher's Signature

I have fully explained the research study described by this form. I have answered the participant and/or parent/guardians questions and will answer any future questions to the best of my ability. I will tell the family and/or the person taking part in this research of any changes in the procedures or in the possible harms/possible benefits of the study that may affect their health or their willingness to stay in the study.

_____

Printed Name of Researcher Obtaining Parental Permission or Consent

_____

Signature of Researcher Obtaining Parental Permission or Consent

_____ _____

Date *Time*

Original form to:
Research Team File

Copies to:
Participant
Parents/Guardian (if applicable)
Medical Records