NORTHWELL HEALTH

Cohen Children's Medical Center of NY

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New Hyde Park, N.Y. 11040

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CONSENT FORM FOR RESEARCH

TITLE: **THE EPIDEMIOLOGY AND BIOLOGY OF DIAMOND BLACKFAN ANEMIA: THE DIAMOND BLACKFAN ANEMIA REGISTRY**

Principal Investigator: Adrianna Vlachos, MD

About this research

You are being asked to participate in a research study. Scientists do research to answer important questions which might help change or improve the way we do things in the future.

Taking part in this research study is voluntary

You may choose not to take part in the study or may choose to leave the study at any time. Deciding not to participate, or deciding to leave the study later, will not result in any penalty or loss of benefits to which you are entitled and will not affect your relationship with Northwell Health.

This consent form will give you information about the study to help you decide whether you want to participate. Please read this form, and ask any questions you have, before agreeing to be in the study.

Important Information

This information gives you an overview of the research. More information about these topics may be found in the pages that follow.

Why is this research study being done?	The purpose of the study is to learn about the diagnosis, treatment and side effects of Diamond Blackfan anemia.
What will happen to me during the study?	Data will be obtained from all registered patients and their physicians via a detailed questionnaire and via collection of data from their medical records. Follow –up telephone calls will also be conducted 1-2 times per year.
How long will I participate?	Once you have consented to participate in the registry your data will be collected indefinitely or until such time that you/your child elect(s) to withdraw from the study.
Will taking part expose me to risks?	You/your child may experience slight psychological stress in responding to some of the questions. However, the questionnaire has been designed to minimize such stress.

	As this study involves the use of your identifiable, personal information, there is a chance that a loss of confidentiality will occur.
How will my data be protected?	Your/Your child's data will be stored on a confidential computer database that has been specifically designed for the DBAR.

Please review the rest of this document for details about these topics and additional things you should know before making a decision about whether to participate in this research.
You/your child are being asked to participate in a research study being conducted by Cohen Children's Medical Center of Northwell Health. You are encouraged to ask questions before deciding whether you/your child wish to participate and any time during the course of the project. Your child will be asked to re-consent for his/her participation once he/she reaches the age of 18.

Purpose of the project
You are being asked to participate in this research study because you/your child has been diagnosed by a physician to have Diamond Blackfan anemia. The purpose of the study is to learn about the diagnosis, treatment and side effects of Diamond Blackfan anemia. All information will be obtained through questionnaires, interviews and the review of medical records. The study is called the Diamond Blackfan Anemia Registry (DBAR). The DBAR will attempt to enroll all patients with DBA including children and adults, in the United States and Canada.

Diamond Blackfan Anemia (DBA) is a rare pure red cell anemia resulting from a failure of the bone marrow to produce red cells. The anemia may present in infancy or childhood. There are only about 700 cases reported in the literature. This disorder is very varied in how ill an affected person becomes and its cause is still unclear. In order to provide a database for future laboratory, clinical and epidemiologic studies (studies of how many people have the disease, and what other conditions are associated with the disease) the Diamond Blackfan Anemia Registry (DBAR) has been established. This registry serves as a valuable source of information which may ultimately lead to improved treatments.

Description of procedure

You will be asked to complete a questionnaire within approximately 1 month of receipt of the signed consent form. We will also ask permission to contact your physician by telephone about your/your child's medical condition. We will ask your/your child's physician to assist in filling out the questionnaire. We will ask for copies of your/your child's medical record. As "Registry "participants, you will be contacted periodically by telephone (approximately once or twice per year) for follow-up questions. No further contact will be made if you/your child decide to withdraw participation in the registry at any time.

The DBAR will attempt to enroll all patients with DBA including children and adults, in the United States and Canada.. These data will be stored on a confidential computer database that has been specifically designed for the DBAR. Enrollment will require the signature of the adult patients or the signature of the minor patient's parents/Legal Guardians.

We also may contact you in the future for other research endeavors in the form of other surveys and/or questionnaires.

Please let us know if you wish to be contacted. _____ Yes _____No

Possible benefits of participation
Although there may be no direct benefit to you/your child through participation in the study, patients may benefit from increased knowledge about the disorder Diamond Blackfan Anemia. In addition, participation in this study will help researchers learn more about Diamond Blackfan anemia. Results from the DBAR will be published in peer-reviewed journals and will be made available to physicians, patients and the family group (DBAF, Inc.).

Alternative available
There is no alternative except not participating in the study. By not participating, it will not impact on any other care you/your child would receive at Northwell Health.

Costs
No compensation for participation will be given. There will be no additional cost to you for participating in this study**.**

Compensation for Research-Related Injury
If you are hurt from being in the study, you will receive medical care and treatment as needed from Northwell Health. However, you will be responsible for the costs of such medical treatment, directly or through your medical insurance and/or other forms of medical coverage. No money will be given to you.

Voluntary Participation
Your participation in this project is voluntary. The quality of your/your child's medical care will be the same, whether you join, refuse to join, or decide to leave the study.

If you do not join the study you/your child will not be penalized or lose benefits to which you/they are entitled. If you join the study you/your child may withdraw at any time without prejudice to your/their future care at Northwell Health.

Confidentiality
If you agree (to allow your child) to be in this study, we will collect health information that identifies you/your child. We may collect the results of tests, questionnaires and interviews. We may collect information from your/your child's medical record. We will only collect information that is needed for the research. This information has been described in this consent form. If you sign this consent form, you are giving us permission to collect, use and share your/your child's health information. This permission is called authorization. If you do not want to provide authorization, then you cannot participate in this research study.

Study records that identify you/your child will be kept private. All participants will receive a unique patient identification number (UPIN). You/your child will not be identified in study records or publications disclosed outside Northwell Health.

The following reviewers may access your study and medical records to make sure that this study is being done properly:

- Representatives from federal and state government oversight agencies such as the Department of Health and Human Services
- Representatives from the National Institute of Health/NHGRI
- Representatives from the Northwell Health Institutional Review Board (IRB - the committee that reviews research at this institution)

If your/your child's research record is reviewed by any of these groups, they may also need to see your/your child's entire medical record.

We will do our best to protect the privacy of your records but it is possible that once information is shared with people listed on this form, it may be released to others. If this happens, your information may no longer be protected by the federal law.

Data from this study may be used in medical publications or presentations. The information will be de-identified so that individual subjects cannot be recognized and the information will no longer be considered PHI.

If the researchers learn about potential serious harm to you or someone else or other public health concerns, it will be shared with the appropriate authorities.

Will you be able to access your records?
If your/your child's research records are used for decisions related to your/your child's clinical care, then you have the right to review this information and request changes. This is limited to information about your treatment, and does not include information related to procedures or tests that are for research purposes only. You may access this information only after the study analysis is complete. You have the right to know who has and who will see your records. To request this information, please call the Human Research Protection Program at 516-465-1910

How long will your health information be kept?
The information that is collected for research will be analyzed for many years and it is not possible to know how long this analysis and follow-up will take. Therefore, you are allowing access to this information indefinitely.

Can you change your mind?
If you change your mind about being in the study, you may withdraw at any time. If you want us to stop collecting your health information, you need to send a letter to the researcher at the following address:

Dr. Adrianna Vlachos
Feinstein Institutes for Medical Research
350 Community Drive
Manhasset, NY 11030

Your letter needs to say that you have changed your mind and do not want the researcher to collect and share your/your child's health information. You/your child may also need to leave the research study if we cannot collect any more health information. We may still use the information we have already collected. We need to know what happens to everyone who starts a research study, not just those people who stay in it.

Certificate of Confidentiality

To help us protect your privacy, this research is covered by a Certificate of Confidentiality from the US Department of Health and Human Services (DHHS). The Certificate of Confidentiality means that researchers cannot be forced to identify you, even under a court subpoena. The Certificate does not mean the Secretary of DHHS approves or disapproves of the project. It adds special protection for the research information about you. You should know, however, that researchers may provide information to appropriate individuals or agencies if harm to you, harm to others or child abuse becomes a concern. A Certificate of Confidentiality does not prevent you or a member of your family from voluntarily releasing information about yourself or your involvement in this research. However, if an insurer, employer or other person learns about your participation and gets your consent to receive research information, then the researchers will have to provide your information.

Will my information be used for research in the future?

Information for this research may be used for future research studies or shared with other researchers for future research. If this happens, information which could identify you will be removed before any information that is shared. Since identifying information will be removed, there will not be an additional consent for future research. By consenting to participate in this study you are agreeing to allow your de-identified data to be used by future researchers without additional consent.

Who can answer your questions about this study?

If you have any questions about the study, you may call Dr. Adrianna Vlachos, MDat516-562-1506. If you have questions about side effects or injury caused by research you should call Dr. Adrianna Vlachos, MDat 516-562-1506. If you need emergency care, dial 911 or go to the nearest Emergency Room. If you have questions about your rights as a research subject, or concerns about being in the study, you may contact the Office of the Institutional Review Board (the committee that oversees research at this institution) at (516) 465-1910. A signed copy of this consent form will be given to you.

Signature Page Follows

Summation/Signature

You have read the above description of the research study. You have been told of the risks and benefits involved and all your questions have been answered to your satisfaction. A member of the research team will answer any future questions you may have. You voluntarily agree to join/have your child join this study and know that you/your child can withdraw from the study at any time without penalty. By signing this form, you have not given up any of your/your child's legal rights. You will be given a copy of this consent form.

_____ _____
Subject's Printed Name Age

If subject is 18 years of age and older:

_____ _____
Subject's Signature Date

_____ _____ _____
Witness' Printed Name Signature Date
(Preferably someone with no connection to this research project; witness is not required if consent is obtained by mail.)

If subject is younger than 18 years of age:

_____ _____ _____
Parent/Legal Guardian's Printed Name Signature Date

Relation: ☐ Mother ☐ Father ☐ Legal Guardian

_____ _____ _____
Witness' Printed Name Signature Date
(Preferably someone with no connection to this research project; witness is not required if consent is obtained by mail.)

Investigator's Signature

I have offered an opportunity for further explanation of the risks and discomforts which are, or may be associated with this study and to answer any further questions relating to it.

_____ _____ _____
Investigator's Printed Name Signature Date

ASSENT BY MINOR SUBJECT NINE YEARS OF AGE OR OLDER

You are being asked to agree to participate in this research study. You have the right to find out what is involved for you if you participate, and to tell your parent(s)/legal guardian whether you do or do not want to participate.

Your parents will also be asked to give permission for you to participate in this study.

Dr. _____ and your parent(s)/legal guardian have explained to you the procedures that are involved.
Dr. _____ and your parent(s)/legal guardian have also explained the possible discomforts, risks and inconveniences that may be involved if you participate.

You have asked any questions you have, and all your questions have been answered.

Check one:

_____ I agree to participate in this study.

_____ I do not agree to participate in this study.

Subject's name_____ Subject's age_____

Subject's signature_____ Date_____

Witness signature_____ Date_____

Relationship of Witness to Subject_____

All procedures, risks and discomforts have been explained to the subject.

Investigator's Signature_____ Date_____