

Universal Data Collection (UDC) Inhibitor Pilot Project Fact Sheet for HTC Providers



WHAT IS THE UNIVERSAL DATA COLLECTION (UDC) INHIBITOR PILOT PROJECT?

The UDC Inhibitor Pilot Project began in 2005 to explore methodologies to collect the information required to accurately determine the incidence of inhibitors among people with hemophilia. A limited number of federally funded hemophilia treatment centers (HTCs) across the United States submit data to the Centers for Disease Control and Prevention (CDC), which receives and organizes the information. The pilot aims to:

- Standardize the way blood is drawn, prepared, and tested.
- Determine how best to track blood product usage.

CDC hopes that the project can be moved beyond the pilot stage and expanded so that issues of inhibitor development can be adequately explored. Specific issues to address include:

- Identifying if inhibitors develop after a change in treatment regimen.
- Determining if there is a genetic link to inhibitor development.
- Studying risk factors for inhibitor development.

WHAT IS INVOLVED IN THE UNIVERSAL DATA COLLECTION (UDC) INHIBITOR PILOT PROJECT?

▪ *What information is collected?*

Hemophilia treatment center (HTC) health care providers collect treatment product usage information and obtain a sample of blood from all participants each year and prior to treatment product changes. Blood is tested at the CDC laboratory, and test results are reported to the study participant's physician. Any results above a standard value are followed up with repeat testing. The lab at CDC also performs genetic testing to provide the study participant and his or her physician with more information about the participant's hemophilia gene and any mutations.

▪ *How is this information obtained?*

A patient must give his or her written permission to take part in the study. HTC pilot sites collect participants' blood samples, completed data forms and infusion logs and submit them to CDC. Infusion information is also obtained from pharmacy dispensing records and the patient's medical record when an overnight stay is required.

▪ *How often is information collected from participants?*

A study participant must fill out an infusion log every time he or she administers clotting factor. The HTC data coordinator develops a plan to collect accurate records of treatment product use from each participant. A sample of blood is collected at the beginning of the study, every year during the regular UDC visit, every time a participant plans to switch products while in the study, or if an inhibitor is suspected.

WHAT IS THE PURPOSE OF THE UNIVERSAL DATA COLLECTION (UDC) INHIBITOR PILOT PROJECT?

▪ *Why is the information collected?*

The development of an antibody (inhibitor) to clotting factor is considered an important blood safety issue for people with hemophilia. It is anticipated that an understanding of the causes of inhibitors may lead to decreased incidence of inhibitors, decreased health care costs, and safer and more effective treatment products for people with hemophilia.



WHO CAN PARTICIPATE IN THE UNIVERSAL DATA COLLECTION (UDC) INHIBITOR PILOT PROJECT?

A person can take part in the Universal Data Collection Inhibitor Pilot Project if he or she is enrolled in the regular UDC or Baby UDC and is seen in one of the HTC pilot sites.

HOW IS UNIVERSAL DATA COLLECTION (UDC) INHIBITOR PILOT PROJECT INFORMATION KEPT CONFIDENTIAL?

The identity of individual participants is known only to the staff of their HTC. Information from individuals is recorded on a standard form and sent to the CDC using a code number (no original medical records are sent) instead of a person's name. Participants' information is also protected by HIPAA (Healthcare Insurance Portability and Accountability Act) regulations. Further, the project is covered by a certificate of confidentiality which prevents the patient's identifying information from being disclosed even if ordered by a court of law. Finally, no individual data is released in reports or analyses; results are reported so that no particular individual can be identified.

WHO PROVIDES INPUT TO WHAT DATA IS COLLECTED AND ANALYZED?

An Inhibitor Working Group was developed in 2005 to assist in the development and implementation of the Inhibitor Pilot Project. The working group is comprised of HTC physicians and nurses, representatives of federal partners such as the National Institutes of Health (NIH) and the Food and Drug Administration (FDA), genomic experts, and industry representatives who work for pharmaceutical companies.

WHAT ARE THE SIGNIFICANT FINDINGS OF THE UNIVERSAL DATA COLLECTION (UDC) INHIBITOR PILOT PROJECT?

Although the UDC Inhibitor Pilot Project is still in the data collection phase, easier ways for HTCs to prepare and ship blood samples have been identified, and methodologies for detecting and measuring inhibitors are being compared. In addition, analyses on the association between genetics and inhibitor development are currently underway.

WHAT PUBLICATIONS HAVE BEEN GENERATED AS A RESULT OF THE UNIVERSAL DATA COLLECTION (UDC) INHIBITOR PILOT PROJECT?

The following abstracts have been produced:

- Creary M, Soucie JM, Miller C, Hooper WC, Abshire T, Brettler D, et al. U.S. Inhibitor Pilot Project: Participant Characteristics and Infusion Log Adherence. Poster presented at American Society of Hematology; December 2007; Atlanta, GA.
- Soucie JM, Miller C, Creary M, Hooper WC, Abshire T, Brettler D, et al.). U.S. Inhibitor Pilot Project: Study Design and Methods Validation. Poster presented at International Society on Thrombosis and Haemostasis; July 2007; Geneva, Switzerland.
- Miller CH, Platt SJ, Abshire T, Brettler D, Brockenstedt P, DiPaola J, et al. Experience with a modified Nijmegen-Bethesda Method for measurement of inhibitors in hemophilia patients: the CDC Inhibitor Surveillance Pilot Project. Poster presented at American Society of Hematology meeting; December 2007; Atlanta, GA.

WHAT FUTURE PUBLICATIONS OR ANALYSES ARE PLANNED FOR DATA FROM THE UNIVERSAL DATA COLLECTION (UDC) INHIBITOR PILOT PROJECT?

- Miller CH, Hooper C, Abshire TC, Bockenstedt PL, Brettler DB, Di Paola J, et al. Factor VIII and Factor IX mutation analysis in 600 U.S. hemophilia patients: correlation of mutation type with history of inhibitor. Abstract submitted to American Society of Hematology meeting, December 2008. A manuscript is planned.

