

Introduction to the USIDNET Immunodeficiency Patient Registry

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- Protection of the interests of research subjects
- Protection of the interests of submitting physicians / researchers

Introduction to the Registry

The Primary Immunodeficiency Disorders are uncommon and therefore understanding of these disorders in the general medical community is unfortunately often quite limited. Improvement in our understanding of these disorders and their frequency is being achieved through a Registry of patients with these disorders. This Registry combines the experiences of thousands of patients and the observations of many physicians from across the United States.

A Registry of patients with Chronic Granulomatous Disease was initially established in 1992 by NIAID thru a contract with the Immune Deficiency Foundation with the primary goal of determining the relative frequency of this disease in the United States. This initial Registry proved so successful that in 1998, an expanded renewed contract was awarded to cover more primary immunodeficiency disorders. By 2003 this expanded Registry had enrolled over 1400 patients with 7 distinct diseases including SCID, XLA, CGD, DiGeorge, Wiskott-Aldrich, CVID and Hyper IgM syndrome.

In 2003 the NIAID established a contract with the United States Immunodeficiency Research Consortium (dba USIDNET) that included upgrading the existing Registry to include all disease states now recognized by the International Union of Immunological Societies (IUIS). Now known as the USIDNET Primary Immunodeficiency Disease Registry, it houses the data from the original 1400 patients as well as newly enrolled patients.

This patient registry is designed to obtain longitudinal data on a large number of patients with the rare primary immunodeficiency diseases in order to:

- Learn more about the phenotypic variations seen in a large number of individual patients with the same molecular diagnosis
- Determine the natural history of these genetic disorders of immunity and establish genotype-phenotype correlations
- Learn effects of various treatment protocols used in these patients including unexpected side effects that may be unique to a particular diagnostic group
- Evaluate measures of quality of life in patients with these disorders and correlate these with genotype and treatment history
- Identify a larger pool of potential research subjects than would be available to individual investigators at their own institutions
- Promote collaborative research among investigators through the identification of patients with a specific diagnosis for potential participation in multi-institutional clinical trials
- Provide a minimum estimate of the prevalence of each disorder
- Act as a resource for clinical and laboratory research

Registry Operations

The success of the Registry is totally dependent upon the willingness of patients to participate and upon the cooperation of physicians in enrolling their patients with primary immunodeficiency. Therefore the operating philosophy of the Registry protects the interests of both of these critically important groups.

Protection of the interests of the patients involves adherence to the regulations established under the Federal Health Insurance Portability and Accountability ACT of 1996 (HIPAA) and the incorporation of review and approval of the Registry research protocol and related informed consent documents by Institutional Review Boards (IRBs). The USIDNET Registry has prepared Clinical Protocol and Consent documents that have been reviewed and approved by the Western IRB (WIRB). These can be used by any physician who does not have a local IRB to review his/her participation in the Registry. The WIRB has also provided a waiver of consent that permits inclusion of the data from deceased patients without the need to obtain consent from the next of kin. Copies of these documents can be found on the Registry website (www.usidnet.org) along with copies of IRB approved protocols from participating academic institutions that can be used as references for your own application.

Patients may be told about the results of research that uses Registry information. The USIDNET Registry Steering Committee may permit information about new tests, treatments, or research studies to be sent to a patient's doctor and with prior consent to share this information with some patients.

To request a report on information contained in the Registry, simply submit a Request for Query to Query@USIDNET.org. Your report will be sent to you once your request has been approved. The identity of the patients will not be revealed. Reports generated as a result of a request to the Registry will be published on the Registry website. Academic investigators may be granted a 6 month embargo of their results to permit them to prepare and publish a manuscript. Investigators from commercial organizations may also apply to the Registry for data reports. Embargos will not be granted to commercial organizations.

Any request by an investigator seeking additional information concerning a specific patient will be directed to the enrolling physician. If a collaboration can be arranged the enrolling physician may then provide additional data or contact the patient for additional studies.