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World Federation of Hemophilia Guide to Developing a National Patient Registry

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Introduction

Hemophilia affects about 400,000 people worldwide. With treatment products and proper care, people with hemophilia can live healthy lives. Without treatment, hemophilia causes crippling pain, severe joint damage, disability, and death. Today, about three quarters of the people living with hemophilia receive little or no treatment.

The World Federation of Hemophilia (WFH) is an international not-for-profit organization working to introduce, improve, and maintain care for people with hemophilia around the world. The WFH works towards these goals through its network of committed volunteers and stakeholders. National hemophilia organizations and hemophilia treatment centres are key partners in this network. They play a major role in improving care within their countries.

Starting on the path to improved care can be a daunting task. An important early step is to establish a national patient registry. A patient registry is an invaluable tool for improving the lives of people with hemophilia. It is essential for tracking the identification and diagnosis of people with hemophilia, and monitoring of their health, as well as long-term planning for hemophilia organizations, and priority setting for health care. Having a national patient registry means being able to answer fundamental questions about the hemophilia population within your country – such information is essential for lobbying government and advocating for improved care.

In a joint meeting of the World Health Organization (WHO) and WFH on Hemophilia Care in Developing Countries, held in Geneva, Switzerland in 1997, having a national registry of people with hemophilia was identified as a key priority. The report published by the WHO and WFH includes the following statement, "In order to allow for the proper planning and development of health services, the establishment of a national registry of people with hemophilia is essential. It is therefore a recommendation that priority be given to identification and diagnosis of affected people and their families and to the central registration of individuals with hemophilia and related disorders. In order to be successful, such a scheme must guarantee confidentiality and respect for human rights."

Having a national patient registry is also very useful globally. Being able to compare information among different countries can be helpful when lobbying for improved care. The WFH has collected and published data annually since 1998. This survey provides illustrations of the general state of hemophilia care on a global basis and examples of achievement with limited resources that encourage other countries to attempt the same. Having a national patient registry helps ensure that the global data collected by the WFH is accurate and meaningful.

Quantifiable results contained in national registries and the WFH Global Survey facilitate the measurement of the effectiveness of healthcare programs. Basic data, such as increases in the number of patients diagnosed or the life expectancy of people with hemophilia are useful in evaluating the worth and success of various development programs to improve care within countries.

This guide explains what a registry is, identifies the different types of registries that have been used successfully around the world, details the steps involved in setting up and maintaining an effective national patient registry, and discusses how to use the valuable data contained in a registry.

Section 1

All about national patient registries

What is a registry?

A registry is a database or a collection of records of people identified with hemophilia or inherited bleeding disorders. It includes information on personal details, such as age, sex, type of bleeding disorder, severity, type and amount of treatment received, and complications such as inhibitors, liver disease, joint disease, etc. Generally, this data is kept in a computer database. A national registry centralizes this data for an entire country to avoid duplication of names.

The benefits of a national registry

A national registry carries many benefits for the individual with hemophilia. This is the primary goal of developing such a registry – to help the individual by increasing awareness of the prevalence of the disease, delineating needs of patients in the community, identifying shortcomings in the healthcare delivery system, predicting future needs and areas of concern, and empowering the national hemophilia organization and physicians to lobby effectively on behalf of people with hemophilia.

Advocating for health care. One of the first steps in establishing an effective hemophilia program is to develop a national registry of patients. In some cases, this essential work is overlooked because other issues such as organization of care, financing of care, and need for treatment products may seem more important. However, the planning necessary to address these other issues must be based on an understanding of the number of patients being served, the level of care they receive, their location, and their unmet medical needs – all information that is supplied by a national patient registry.

When a patient organization maintains control of a registry, it has strategic power for lobbying and the ability to prove to authorities that patients exist. For example, when the Russian Hemophilia Society meets with the government authorities it can provide data from its national patient registry on more than 7,875 patients with hemophilia and other bleeding disorders in Russia. This demonstrates that the patients actually exist since there is no other registry in the country besides the patient organization registry.

All countries have a limited amount of resources to spend on health care and they allocate resources based upon what they assume is the highest priority. Often expensive chronic diseases such as hemophilia are considered of lower priority. For groups advocating for hemophilia care, it is necessary to demonstrate the needs of the patient population, and to show how the government's investment of small amounts of resources will improve survival and reduce the expensive complications that use up even more health resources.

National hemophilia organizations, when meeting with health authorities, are almost always asked "How many patients exist in the country and how much does treatment cost?" The group that can answer these questions will be in an extremely strong position to take advantage of the receptive nature of the health authorities, as have some WFH national member organizations, when obtaining increased resources to supply unmet needs in their countries. You can count on the fact that governments will allocate resources only if you have proper documentation of needs.

To be successful, advocacy for care for the hemophilia population must have public understanding and support. Groups or individuals advocating for improved care should take every opportunity possible to explain the needs and the state of health of patients with hemophilia. Data from a national registry provides the valuable tool to accomplish this task.

For example, a national patient registry of hemophilia patients in the Republic of Georgia is demonstrating the effectiveness of the simple reorganization of health-care services and that provision of some viral-inactivated clotting factor is having major effects on health outcomes for hemophilia patients in that country. This data is providing encouragement for other governments in the region to attempt similar programs.

Data from a national registry can also be used when lobbying for compensation for people with hemophilia who have acquired HIV or hepatitis C from treatment products.

Allocating resources – priority setting. Medical needs for the hemophilia population in a country will vary widely. At first, resources may only be initially available in small amounts. Those resources should be used to achieve visible results. This is important for two reasons. First, successful healthcare outcomes (such as reduced days of hospitalization or absence from school) will increase the support and enthusiasm of the affected population, and second the successes will demonstrate to the health authorities the usefulness of this program.

Data obtained from a centralized registry is invaluable in identifying the needs for resources and the highest priorities for successful outcomes. For example, when the Ministry of Health in Chile began to organize care for the patients with hemophilia, treatment centres were designated to deliver care on the basis of need and clotting factor was allocated to the locations where patients resided using data obtained from a national registry of patients. If there had been no registry or the data had been poor, resources would certainly have been wasted.

A registry contains important information which can be used for protecting, maintaining, and defending budgets. For example, in some countries, as the level of care improved, patients had fewer and fewer complications, so that some policy makers thought that expenditures for hemophilia treatment centres could be diverted to other uses and patients could receive their care from any physician in the community. In the US, the use of data from a registry of over 3,000 patients, demonstrates that the patients receiving care from hemophilia treatment centres (HTCs) have a 70% reduction in mortality and a 40% reduction in hospitalization than patients receiving care from health-care providers outside the HTC system – even though the patients in the centres are the most severely affected and both groups have equal access to treatment products. As a result, support for the hemophilia treatment centres has continued.

Monitoring trends in health. Some national registries routinely collect health outcome data to monitor changes in the state of health of patients that need prompt attention. Examples of these data include the number of infections with blood-borne viruses (HIV, HCV, etc.), the number of patients with joint disease or the degree of joint disease in the patients, the number of patients that have inhibitors, liver disease, are hospitalized, or die. This information is critical in identifying the changing needs of the patients, identifying particular problems that need to be addressed, or assessing and documenting the effect that changes in healthcare delivery have made for the population.

An example of this monitoring process is seen in Venezuela, where data on HIV and hepatitis infections acquired by patients receiving cryoprecipitate and fresh frozen plasma is routinely entered into the registry. Using these data, a successful appeal was made to the Ministry of Health to dramatically increase the purchase of viral-inactivated concentrates to treat patients with hemophilia. The use of this material has virtually stopped the transmission of the blood-borne viruses in those receiving these products. Documentation of this type is a powerful tool for showing health officials why they should continue to support these programs.

Research projects can also be planned and conducted more precisely using the data from national patient registries.

Improving the tendering process. Reliable data from a national registry can also be used during the tendering process to purchase treatment products. It helps to determine what quantities of treatment products should be purchased; it can be used to plan the distribution of the nationally purchased products; and it can be used as part of an audit of the whole tendering process.

Serving as a distribution mechanism. A registry is also an effective way to organize and monitor the distribution and use of clotting factor concentrates. Both Uruguay and Canada connect their registries to the distribution of clotting factor concentrates. Participation in the registry is increased because of the motivation to receive factor. This also ensures that everyone entered in the registry has the correct diagnosis. This technique allows for the evaluation of patterns of factor usage and excessive use of concentrates can be identified, evaluated, and addressed. A system such as this also increases the accountability of those who distribute factor concentrates. Knowing who uses factor and where they are and what their needs are increases the flexibility of the distribution system making for more efficient use of limited life-saving resources. If a particular product is recalled or identified as having some safety or quality issue, registry data can be used to track its use and to assist in the patient notification process.

Facilitating the establishment of a hemophilia communication network. A national hemophilia association forms an important communication network for distribution of information, education materials, and other notices important to the health and well-being of hemophilia patients. The existence of a registry, by its very nature of identifying patients with hemophilia, provides those patients with the opportunity to voluntarily join the national hemophilia organization and have access to important educational materials and notices provided by that association.

A patient registry empowers the national hemophilia society of a country. It is very useful to the patient organization because of the strategic demographic information it contains about the hemophilia population it represents. The national hemophilia association can access the exact location of people with hemophilia in cases of emergency.

Ensuring better global data. Data collected by the WFH's Global Survey has been extremely useful for the WFH and others in advocating for improving care for the world's hemophilia population. Documentation of the effectiveness of reorganization of health services and provision of even small amounts of safe clotting factor on health outcomes for patients with hemophilia has been successful in convincing Ministries of Health to treat hemophilia as a priority and to start national programs to improve care for hemophilia patients. The WFH began collecting information on people with hemophilia in member countries in 1998. The WFH Global Survey gathers basic demographic information such as hemophilia types and age distribution, data on the prevalence of infectious diseases such as HIV and HCV in the population, access to care, and the use of treatment products from more than 80 countries.

Obtaining quality data is most important for building the integrity of and respect for this information, because that respect is absolutely necessary for the data to have any impact on governmental officials. National registries greatly improve the quality of data by ensuring that it is collected in a systematic way, is reviewed by interested parties before it is given to WFH, and is supplied in a routine and easy manner.

Types of national registries

A national registry may be organized and managed by the national patient organization, as in India, Venezuela, Russia, and Mexico; by physicians as in the United Kingdom, Canada, the Philippines, and Thailand; by the Ministry of Health, as the United States, Chile, Uruguay, or Egypt; or a mixed system as in Iran and Georgia. Each type of registry has advantages and disadvantages.

Patient organization registries

A patient organization registry is set up and maintained by the patient organization. It may be as simple as a database of patient names, addresses, ages, and diagnoses. Registries organized by patient groups provide mostly demographic data and create an active, effective communication network. A patient-run registry can act as a catalyst to trigger interest and motivation for doctors and Ministries of Health. Much of the data collected for a patient-run registry can be complementary with the data found in a medical registry. A patient-run registry can be an outreach tool – building a registry helps to build the patient community. It can help to determine needs of members and it can map out their geographical distribution.

A patient registry provides the patient organization with a network for quick distribution of educational material and notices, but will not usually have access to health outcomes or treatment data. In addition, the registry may be incomplete because of the reluctance of some patients to make their condition publicly known. Finding the expertise necessary to manage such registries can be a challenge, for example because of lack of access to computer expertise.

Medical registries

A national medical registry is a database created by a network of treatment centres, often bringing together information they already collect individually. As well as demographic data, it will likely contain more detailed clinical information including in patient descriptions, factor levels, treatment products used, and complications of hemophilia. Medical professionals generate a lot of information when they see their patients. By standardizing how they record and store this information, they can build very useful registries within their clinics. By coordinating between clinics, doctors and other medical professionals can create patient registries with good clinical data on the health status of people with hemophilia within a country. Since physicians have access to much more information, physician managed registries frequently have more detailed data, useful for managing the patient's health care or useful for conducting studies about aspects of either the disease processes or patient management. On the other hand, these registries are often restricted to the practices of a limited number of physicians, excluding significant proportions of the patient population. In addition, because patients may be seen by more than one physician or clinic, they may be included in a registry more than once. Purging the registry of patients who have moved, died, or are no longer seen by the clinic may not be a top priority. Likewise, if the motivation behind the registries is to conduct research rather than to use the data for public health management, access to data from these registries may be limited to a few investigators.

An effective national medical registry does require standardization of data collection from a network of HTCs, a single coordinating centre, and a high degree of cooperation between physicians.

Health Ministry registries

A Ministry of Health registry is one that has been mandated by the government. It will likely contain all of the information that patient and medical registries contain. National registries run by the Ministry of Health must do so in coordination with the physicians, but will definitely have a broad public health focus. In most cases, the health officials need to provide resources to obtain data from hospitals, physicians' offices, and clinics. Ministries of Health usually need data of a national focus and strive to ensure that the registry achieves that goal. A decree from the Ministry of Health can help to ensure that each HTC submits its data. Proper management of hemophilia on a national level depends on obtaining data that is credible and reflects an accurate picture of the national needs and resource utilization for hemophilia patients. Registries managed by groups other than the Ministry of Health may be satisfied with data obtained from only a sample of the whole population, and miss the national picture. Government-funded national registries are generally under the direction of one person. This benefits the registry by ensuring a unity of focus and the ability to adapt the registry to changing needs. Government support for the registry is vital in helping to motivate participation.

Impediments to establishing a Ministry of Health registry include bureaucratic inertia, resistance to making the necessary investment, and political motivations.

Mixed systems

Some national patient registries are mixed systems combining aspects of these three types in ways designed to fit local needs and conditions. Often a patient group or one clinic can start the process by building a simple registry; when the

benefits of good data collection are observed by the community, there can be considerable incentive and motivation to set up more sophisticated and even nationally mandated registries. Mixed systems have several benefits – they involve many stakeholders in a common purpose; they can create understanding of the registry among patients, physicians, and government; they generate feedback; they can address the interests and concerns of each group of stakeholders, and they can benefit from the skill-sets of the various participants.

The form of registry selected by a country must be one that will best fit the local needs. All types (including mixed systems) have been instituted in the past and each type has served the purposes of the hemophilia population well.

Section 2

Basic principles of data collection

Amount and type of data collected. All data collection is a compromise and it is impossible to collect all the data that everyone wishes to have. Data collection imposes a work burden on the people who collect it, so it must be collected as efficiently as possible. Every bit of data that is collected but not used wastes the collector's time repeatedly with every patient entered into the registry. Similarly the more data collected, the greater the fatigue for the collector, and the greater the chance for error. With very long or complex collection forms, it is difficult to maintain good motivation on the part of the collector. Uses for the data must therefore be determined before starting to collect the data. Not a single piece of data should be collected without first knowing how the data will be analysed and what question it will answer.

In other words, the most important questions for any registry are "What do we want to know?" and "Why do we want to know it?" If you cannot answer those two questions for each piece of data collected, don't collect it.

The goals of the registry should be accuracy, simplicity, and completeness.

- **Accuracy**: as mentioned above, accuracy is important because policy decisions will be made on data. Bad data yields bad policy decisions and lack of credibility.

- **Simplicity** is important to reduce the number of errors and fatigue on those collecting the data.

- **Completeness**: a registry needs all the data – missing data reduces the accuracy and the quality of the data. Therefore there is a need for a high response or inclusion rate of patients.

Confidentiality and ethical issues. These issues can often decide the success of the registry. Two primary issues for participants often override all others, namely, "Who owns the data?" and "How do you maintain patient confidentiality?" National laws, customs, and the registry design and use will often dictate the answers to these questions, and to whether patient consent must be obtained. However, policies concerning these issues must be established in the beginning, understood, and accepted by all parties. In reality, the data belongs to the individuals of the patient community. Its use in a national registry is justified because it benefits the entire community of patients including the individual. When patients understand this principle they strongly support this application of their data, especially if they know that their data can be made anonymous.

Healthcare providers sometimes feel a proprietary ownership of the data because of the work they have done to collect it. In addition, they often have concerns that they may be violating patients' privacy rights by relinquishing control of the data to a third party registry. Providers in university medical school settings sometimes have an underlying wish to publish the data themselves, even though most of the

data placed in the registry is of a public health interest rather than that used in research studies. All these concerns can be met during the design phase of the registry. Data can be stripped of personal identifiers so that the data is anonymous and the patients' identities remain available to only those who they trust, for example, their healthcare provider. This can be achieved through the creation of a personal identification number to replace the patient's name when registry data is shared. For example, in the US when a new patient is entered into the registry, software is used to generate a unique 12 digit personal ID number ensuring that only that patient's own HTC can identify him. When an identifying number is used, it allows the registry data to be shared while confidentiality is ensured.

Registry management. The most direct way to manage confidentiality and ethical issues is to involve all parties in the design and management of the national registry. The stakeholders concerned with hemophilia care (i.e., patient representatives, healthcare providers, and government representatives) all have different perspectives and concerns about a registry. A registry committee comprised of representatives from these different groups can often agree upon the design of the registry that addresses everyone's concerns and thus builds support for participation in the proposed registry. Individuals, respected and trusted to be representatives by their individual groups, are particularly helpful as members of this committee. Possible members of the registry committee might include respected representatives from the hemophilia association, physicians from hemophilia treatment centres, nurses, physical therapists, and social workers who work with patients with hemophilia, and a representative of government.

Experience has shown that many people will want access to the registry data – government, patient organizations, researchers, drug companies, other hemophilia organizations, and the WFH. This is good, because the data becomes more important with use and as a result, the registry receives more support for its continuation. As we will see in the following paragraphs, the registry committee is critical in determining how and in what form the data will be managed and circulated.

System of data collection. The method of data collection will depend to a great extent on the location of the data. In general, data is most efficiently collected at the site of medical care as part of routine clinical practice, because the data here is usually the most accurate and up to date. However, depending on the nature of the registry, other methods may be more suited. For example, in some countries, patient identification (outreach) campaigns are conducted during which visits are made to different locations in the country, patients suspected of having a bleeding disorder are seen by clinical personnel, blood samples are obtained and tested for diagnosis, and patients are entered into the patient registry.

Registry maintenance. A national registry is a dynamic tool for hemophilia care. It must be continually updated in order to remain accurate and useful. There will be a tendency to place the results on the shelf after the set-up work is completed. This must be avoided at all costs and the following are important elements preventing that from happening.

1. Maintaining the registry must be an **assigned responsibility**. The person receiving that assignment must understand the nature of the registry, its elements, and uses, and understand that it is an important tool for patient care.

2. The registry committee is an important element in keeping the registry active and fulfilling the desired needs. **Periodic review of the data** and suggestions for deletions or additions of elements as the needs of the community change are important responsibilities of the committee. In addition, suggestions for types of analysis and uses for the data will enhance the usefulness of the committee and provide ongoing motivation and support concerning the need to keep the registry current.

3. There should be **standard procedures** for routinely registering new patients, recording deaths, and updating other elements of the registry, so that they are automatic. A common error occurs when a patient is entered into the registry twice. Standard procedures for entering and updating data will help to avoid duplication and increase the accuracy of the registry.

4. **Share the compiled data** and keep it visible. Communications with health-care providers, patients, and health authorities should be routine, so that all understand the usefulness of the registry and see the benefits of keeping it current.

5. Keep the registry **user-friendly** to ensure that its data can be maintained, updated, and exploited for the benefit of people with hemophilia.

Section 3

Steps to establishing a national patient registry

There are nine basic steps in developing a national patient registry for hemophilia. Depending on the nature of the registry, the steps may vary in importance and consideration, however, all should be addressed to some degree with even the simplest registry, for time spent at this point will save many hours of problem solving later. The steps are (1) organizing a registry committee; (2) establishing goals; (3) preparing an action plan; (4) selecting the system of data collection ; (5) determining the data content; (6) designing the data collection form; (7) collecting the data; (8) analysing the data; and (9) reviewing the registry.

Step 1: Organize a registry committee

Who will benefit from a patient registry? These are the persons or groups who will be most interested in establishing and maintaining a registry. The most likely groups include the patients, the healthcare providers, and the health authorities. It is extremely important that representatives of these groups be asked to participate in a registry committee so they will learn the value of and support the patient registry. This is a critical step, for the clear understanding of the needs and uses of the data will directly affect the success of the registry. In addition, the level of motivation will be directly proportional to the ability to achieve a common direction.

The registry committee should meet on a regular basis, either by conference call or in person. In the early stages of the registry formation, meetings will be more frequent for the committee and will focus on achieving consensus and making decisions concerning the design of the registry. In addition, this committee should address other important policy decisions that confront the registry, such as ownership of data and access to the registry.

Step 2: Establish goals of the registry

The registry committee formed in Step 1 should set the goals of the registry. Motivation will remain high if this group feels that its goals are being met.

It is important to determine how the data is going to be used before beginning the system. Data collection requires time, so every bit of data collected must have a purpose to prevent wasted energy. Consequently, the goals of the registry will dictate every bit of data collected. If you want eggs; you must design a system to produce eggs, not one for making sausages. Start the process by saying, "we are going to use the registry data to 1)… 2)… and 3)…" If you cannot do this, the registry is most certain to be a failure.

For instance, if the data is to be used to measure the prevalence of HIV infection to allocate resources for HIV care and prevention, why collect data on the number of persons with inhibitors? Collect data for specific needs and uses as defined by these goals. Know the purposes for which the data will be used and state these clearly. Possible goals might include:

- Determine the prevalence and incidence of hemophilia and its complications (e.g., disease manifestation, demographics, nature/type of complications, source of medical care).
- Study the occurrence of complications and the use of healthcare resources over time.
- Develop population-based data on the social and economic impact of hemophilia and its complications (e.g., medical care, hospitalizations, lost days of work).
- Detect emerging infectious disease situations that require intervention.
- Understand the demographics of HIV/AIDS and HCV.

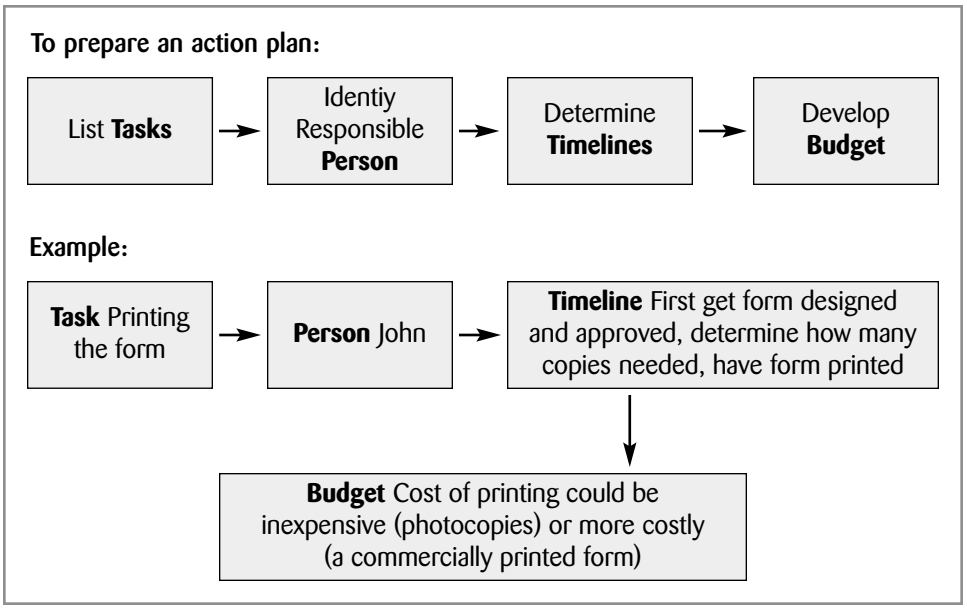
Step 3: Prepare your action plan

Once you have organized your committee and defined the goals of the registry, it is essential to take the time to plan what has to be done, by whom and when. Preparing a detailed action plan together with the members of the committee should include:

- **Tasks** – detail the tasks that have to be accomplished
- **People involved** – list who will work on which task and what their role will be
- **Timelines** – detail by what time the tasks must be completed
- **Budget** – identify sources of income and expenses

Planning the details of the registry comprises specific tasks. Decisions have to be made whether personnel will be volunteers or salaried. Responsibility for making sure the data is complete, free of errors, and consistent must be determined; responsibility for data analysis must be assigned and the communication channels determined.

Additional tasks include determining the methods of data entry, printing of data forms, identifying resources for postage, and methods of dissemination of results, for example, by mail, newsletter, publication, or internet web site. Depending on the complexity of the registry, technical assistance may need to be provided concerning filling out the forms, and personnel involved in data collection and management may need training. These are a few of the examples where preplanning can reduce greatly the questions needed to be answered before the sixth step, developing the data collection form.



Step 4: Select the system of data collection

Choices here are dictated by the goals identified in Step 2 combined with the need for an efficient data collection system. The general principle is the simpler the system of collection, the better. Considerations for selection of a system will include the location of the data, the barriers to collecting the data, the cost to obtain it, the completeness of the data, and the validity and suitability of the data available. Every system will be different.

First, you must determine whether needed information can be obtained from physicians, from hemophilia treatment centres, or whether it must come from the patients themselves. Confidentiality issues need to be considered when designing the form and manner which data is collected. In addition, you must determine if permission is required to obtain the data and if so, who can grant the permission (i.e., the patient or other authority).

Second, you need to determine the cost of obtaining the data and the levels of expertise and technical knowledge required. These issues may force you to modify your registry design to meet available resources. If you want to analyse the data over time, then the frequency of data collection will have to be determined. Selection of software may also be necessary and dependent on the complexity of the registry. (Software such as Microsoft Excel, Microsoft Access, Filemaker Pro or MySQL may be selected. Many computers come with database software pre-installed that can be used to create a simple registry database.)

	A	B	C	D	E	F	G	H
1	**1. Name**	**2. Date of birth**	**3. Sex**	**4. Address, City, Region**	**5. Place of Birth**	**6. Treatment Status**	**7. Another person?**	**8. Factor deficiency type**
2	Smith, Joe	9/9/89	male	123 First Avenue, Smallville	Capital City	transfer to SHTC	no	VIII
3	Morales, Juan	2/5/79	male	890 Second Street, Capital	Mexico	transfer to CCHC	yes - uncle OMJ	VIII
4	Brown, Mary	6/8/90	female	39567 Long Street, Capital	Capital City	new at CCHC	unknown	vWD
5	Ng, Peter	12/30/76	male	874-66 River Street, Capital City	Capital City	established CCHC	unknown	XI
6	Hassan, Ali	5/23/92	male	54 Canal Avenue, Capital City	Capital City	established	yes - brother not on file	IX
7	Gonzalez, Pedro	11/3/70	male	2234 Queen Street, Capital City	Capital City	established CCHC	yes - brother RG	VIII
8	Gonzalez, Roberto	11/3/70	male	1555 Third Avenue, Smallville	Smallville	established	yes - brother PG	VIII
9	Cliquot, Jean	8/15/85	male	University Residence, Capital City University	Smallville	transfer toCCHC	unknown	VIII
10	Smith, Joe	3/28/95	male	459 Main Street, Capital City	Australia	new at CCHC	yes - cousin in Australia	IX
11	Smith, Jane	6/16/93	female	65 Park Street, Smallville	South Africa	new at SHTC	unknown	XI

Sample data entered into a computer spreadsheet.

The registry committee will usually select a registry design that will be a compromise of these and other issues based on the needs of the particular country. For this task, it is often helpful for the registry committee to establish a priority list of issues to consider while making the final decision. For example, a country that wants a relatively extensive registry system could use the following weighted criteria to select a registry design:

1. Cost/ease of data collection
2. Relevance in healthcare setting
3. Confidentiality requirements
4. Usefulness over time
5. Adaptability to making changes
6. Confidence in accuracy of data collected by this method
7. History of successful application elsewhere
8. Meets the needs of HTCs, national patient organizations, and Health Ministry.

Step 5: Determine the data content

This step will take the most time and discussion.

a. List the agreed upon **goals** of the registry as identified in Step 2. These goals should be the driving force for determining what data is to be collected.

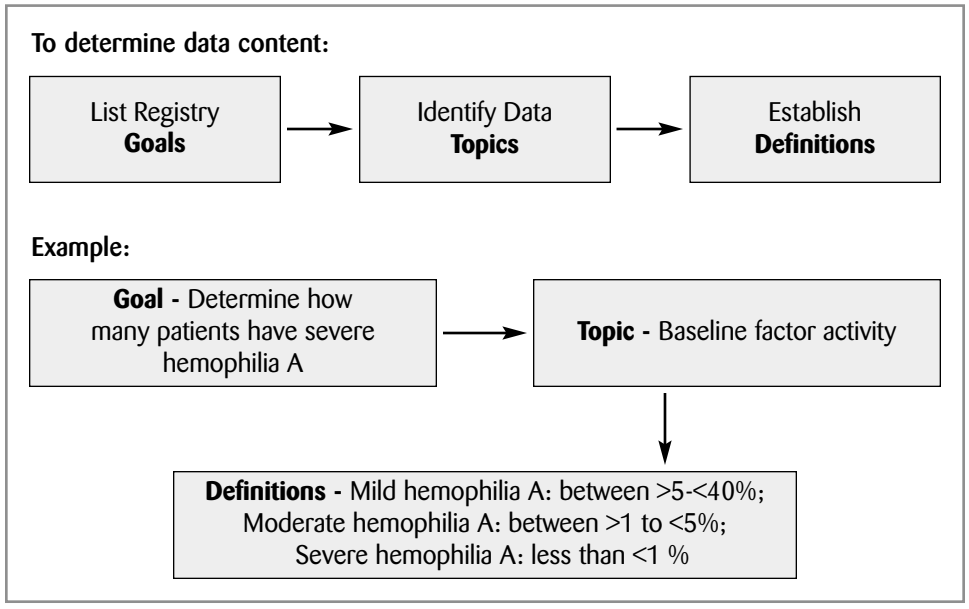
b. Identify the specific **data topics** needed to meet the stated goals. Limit the data topics to the minimum required to answer the questions. The data system is not a medical chart; its success will depend upon its simplicity. Possible data topics might include the prevalence of: (1) hemophilia A and hemophilia B, (2) HIV infection, (3) AIDS syndrome, (4) chronic hepatitis, (5) joint disease, or (6) inhibitors. Other possible topics are the incidence of certain diseases or the annual use of healthcare resources. Each topic should be discussed in detail to gain a thorough understanding of the topic and its value to achieving the goals of the registry.

   For example, if a goal of the registry is to determine the prevalence of various bleeding disorders, the registry committee should determine a data topic which relates to this goal. The level of available detail on the topic should be sufficient to give meaningful conclusions. For example, if the data available lists patients as having either hemophilia or

other bleeding disorders, meaningful conclusions concerning the number of von Willebrand patients will not be obtained.

c. Establish **definitions** for each topic. These definitions will determine what data will be collected and the minimum amount of data necessary and will ensure that the data is uniform and well understood by everyone. Consider the example of the diagnosis of hemophilia. A patient is diagnosed as having hemophilia, but the diagnosis can be more specific – hemophilia A or B? Is it mild, moderate, or severe? How was this diagnosis confirmed? What is important is that everyone completing the questionnaire gives responses which are comparable and based on the same understanding of common terms and diagnoses.

   The committee needs to determine the definitions. The definitions must be precise, and must consider if the diagnosis of a particular condition or complication will be uniform across regions, and if specialized tests are available in only certain parts of the country. If the data is representative for only a segment of the population, it may not be valid for the whole population. This became an important issue early in the HIV epidemic in patients with hemophilia, because the prevalence of HIV infection identified by hemophilia treatment centres was much higher than that in the general hemophilia population, because the patients seen in hemophilia treatment centres were, in general, those with the most severe hemophilia and received the most exposure to clotting factor concentrates.



Step 6: Design the data collection form

This step is a critical step in establishing the registry. All the planning to this point can be wasted if the data collection form is designed in such a way that no one will use it, or if it collects data that yields poor or unreliable analysis. Since it can be a labour intensive process, the following approach is recommended.

You agreed on **goals** in Step 2, **topics** and **definitions** in Step 5 – keep this in mind as you design the data collection form.

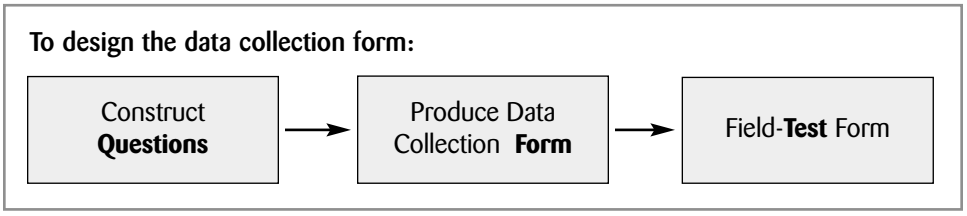
a. Construct **questions** for the data collection form. Avoid "open-ended" questions that require interpretation. For example, don't ask questions such as, "How did your joints feel last month?" You could receive answers such as any of the following: "good, well, bad, horrible, almost good, fair, sometimes alright, sometimes bad, etc." Analysing this data or even placing it in the registry database becomes an impossible task. It is better to ask, "How many days did you stay home from school because of bleeding in your joints?" The answer is a number, which has meaning as to the functioning of the individual, and requires no interpretation to place in the registry database or analyse.

   The type of data collected should be based on the ability to select simple choices, for example, checking boxes labelled as yes, no, or unknown is easy for the data entry person, and will yield more consistent answers from location to location. Alternatively checking elements of a multiple choice list, for example, a list of treatment products is also simple and easily standardized. Numerical data, such as age, birth date, or the number of units of factor VIII used are also acceptable. All these examples are well-suited for computer analysis, and can be easily managed without interpretation by either the person entering or analysing the data.

b. Produce/prepare the data collection **form**. Simple, easy-to-use, well-designed forms will greatly enhance the willingness of workers to supply the data. The data form could be on paper (paper forms are easy to distribute and fill out, and cheap since multiple copies can be made and left wherever they are needed) or it could be on the computer screen using software such as Access or Filemaker. (An onscreen data form removes the step of having to enter data that has already been written on paper.) Identifying a person specifically responsible for the data entry and man agement is critical to obtaining good data. In addition, once obtained, the data should be checked for inconsistencies and these clarified as soon as possible. For example, if a data form is obtained that lists the age of the patient as 89 and a birth year of 1989, this needs to be clarified before the data is entered into the registry.

c. **Field-test** the data collection form. This means having it filled in by some of the people who will actually use the form to see if they have any problems. This step is crucial to determine the feasibility of collecting the needed data. Field-testing, which should be done by the participating centres, will often identify problems with missing data, definitions, and interpretation of results. After the field test, the definitions and data collection instrument should again be modified to correct the problems encountered.

The order of these procedures has been established for good reason. If this process isn't taken in order, much time will be wasted retracing the same ground to achieve consensus. The temptation to go directly to the questions to be included on the questionnaire or instrument must be resisted.

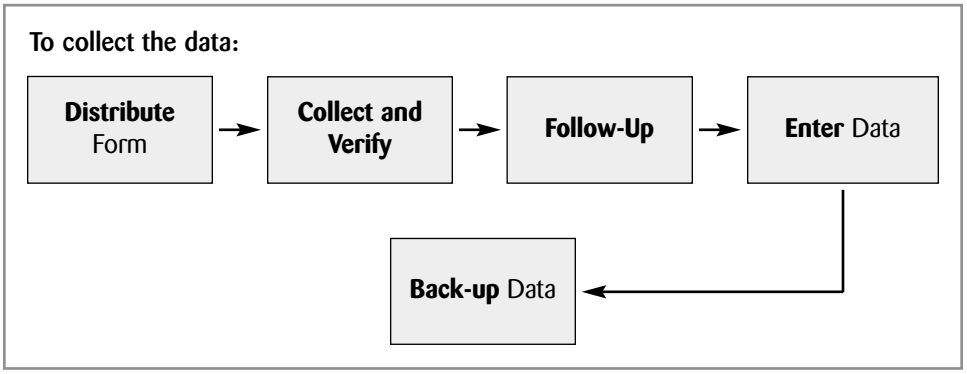


Step 7: Collect the data

Once you have field-tested the data collection form, the next step is to collect your data. This is achieved by first **distributing the form** to participating centres or directly to patients in the case of a patient organization registry. There should be a clear deadline for returning the completed forms so that you can ensure that timelines are met. The next stage is to **collect** the completed forms and follow-up the ones that have not been returned. It is also important to **verify** the information on each form as there is always some information missing or inconsistencies in some of the replies. In these cases, it is important to follow-up and contact the centre or the patient to double-check the information and/or obtain the missing information. This ensures that your data is complete.

Once all information is gathered, the next important step is to **enter the data** into a database. This is a labour intensive task which depending on the complexity of the registry may require technical assistance. It is always preferable to enter the information rapidly so that it can then be analysed and the results disseminated to as many stakeholders as possible.

It is essential to **back-up the registry data**. Valuable data can be lost forever in the event of computer breakdowns, software failures, and theft. A regular and tested back-up system will help to protect the crucial information contained in the registry.



Step 8: Analyse the data

Analyse the data and distribute the results. Quick distribution of the results will be greatly appreciated by the participants, ensuring their continued efforts to keep the registry up to date.

Many of the goals set by the registry committee will require some analysis. Some analysis can be done easily by simply adding up the data, but it is also possible for an expert to use registry data in many ways to do much more with the data. Analyses of your registry data could include:

- Summary numbers - basic demographics. How many people have been identified with hemophilia A and B?
- Geographic distribution analysis. Where patients live, where services (such as clinics) are needed.
- Utilization of services and resources.
- Estimation of factor concentrates needed based on what is currently used.
- Analysis of number of patients undiagnosed.
- Analysis over time.

Example 1: Estimate how many people with hemophilia have not been included in the registry. Take the total number of patients identified with hemophilia A and/or B; calculate predicted number of patients using accepted prevalence number and national population figures. Compare these two numbers to determine how many patients are unidentified nationally.

Example 2: Evaluate future lab-testing needs. Laboratory analysis is the best way to diagnose patients. You can use registry data to determine how many patients have been confirmed by lab testing and then estimate how many of the identified population still need to be lab-tested. It is then possible to determine how many people on the registry will need to undergo laboratory testing, and to estimate the cost of this testing.

Step 9: Review the registry system

The ongoing role of the registry committee is to periodically review the system, its results, and problems. The system can be modified to meet the changing needs of the participants. Problems with confusing or vague data can be dealt with by making questions more clear or possible answers more obvious. New questions can be added when new types of information become desirable.

If the goals are realistic and the data collection system meets these goals, then the system will flourish. Ideally, hemophilia data will be used to reduce the human and financial burden of bleeding disorders by focusing the national emphasis away from rehabilitation programs towards prevention and early intervention.

Section 4

Maintaining and using a national registry

The registry committee should oversee the maintenance of the registry. One of their priorities should simply be to keep the registry up to date, to make sure that the community is aware of its value and of the importance of providing new information when it becomes available.

It is essential to inform the population of your results. This will demonstrate that the work to gather the data leads to positive outcomes and helps members of the community to feel they are contributing to improving hemophilia care. If the data is used to influence policy, or in scientific studies, share that information with the community. Help build a sense of ownership as well as reinforce the value of the data.

Once the numbers have been accumulated, they can be put to several uses.

- **Develop policy**: Once a registry is in use, it can help to evaluate the state of hemophilia care and be used as a tool to identify which areas need attention, which can be most easily improved. With numbers to back you up, you can make clear, specific proposals to your Ministry of Health. "This will help 300 people and will cost $24,000."
- **Develop resources**: It is possible to identify what resources are lacking for hemophilia care and to target the needs. As well, existing resources can be maximized, eliminating waste. Planning is much easier when you have data to work from.
- **Manage resources**: "There is a need here…" Using geographical information in the registry it is possible to identify regions or centres which are underserved. It is also possible to identify the most efficient ways to use resources. The resources that are available for hemophilia should be used as effectively and efficiently as possible. A registry help to measure effectiveness as well as identify ways to improve efficiency.
- **Define needs**: Detailed information about the health status of people with hemophilia can help to identify needs such as vaccinations for common local diseases (i.e., hep A vaccination).
- **Monitor blood safety**: An up-to-date registry can be used to investigate suspected outbreaks of blood-borne diseases, identify new outbreaks, as well as to communicate with the patient community about emerging problems.
- **Manage treatment**: Over time it is possible to study more complex issues: inhibitors with different products; joint disease in children; ways to improve care; benefits of physical therapy.

Keep your national patient registry up to date, adaptable to changing needs, continually used and remember that the registry review committee is essential.

Conclusion

A national patient registry is a critical tool for monitoring the identification and diagnosis of people with hemophilia, and evaluating their health. As well it serves as an essential tool for long-term planning for hemophilia organizations, and priority setting for health-care resource allocation. When a patient organization has access to the data in a registry, it has strategic power for lobbying and the ability to prove to authorities that patients exist. It also allows the organization to provide quantifiable data on the number of individuals with the condition, their associated health problems, a use of treatments. Having a national patient registry means being able to answer fundamental questions about the hemophilia population within your country – such information is invaluable for improving the lives of people with hemophilia.

Case Study: Venezuela

A patient organization registry

The first registry in Venezuela began in 1963 as a medical registry. It was a list based on the medical histories of patients assessed at the National Hemophilia Centre (NHC) in Caracas. It contained the date of the patient's first diagnosis, detailed information on treatment and injuries and placed particular emphasis on the family name and family tree. However, many patients came only once for initial diagnosis and never returned because they lived far away in other parts of the country. Often, the initial information collected was no longer accurate because people moved frequently.

It was not until 1990 that the Venezuelan Association of Hemophilia (VAH) decided to ask, "How many patients are there and where are these patients?" In the struggle to obtain treatment for all the people with hemophilia, the VAH first had to locate the patients, seek their support, and prove to the state that they existed.

When the registry project began, one of the most important goals was to know the exact number of people with hemophilia in Venezuela. At that time 495 patients had been identified. This information was necessary because the first question any government will ask is the population size and location. Having this information enables governments to accurately calculate how much treatment is needed and how much it will cost. Since this would be a patient-run registry, there was no need to have all the detailed medical data that appeared in the NHC registry.

The Venezuelan registry includes:

Full name: having the first and last name helps not only with identification but also with tracing the patient's family. There may be relatives with hemophilia.

Date of birth: the patient's age and date of birth can also give an idea of what type of treatment the patient has received to date.

National identification number: there are people with the same name belonging to the same families, but they at least each have a different identification number. The ID number is a more certain form of confirmation of the patient's existence.

Updated address: this allows care delivery services to meet their needs. Also patients can be put in contact with each other for support.

Updated telephone number(s): this allows for quick contact and communication of information.

Type and severity of factor deficiency: it is of utmost importance to know what type of factor deficiency the patient has and the degree of severity to ensure the administration of proper treatment.

One of the younger members of the society who was an expert with computers designed a simple data collection form and created a registry. The form is simple to fill out with some boxes that make it easier to input the data in a spreadsheet. All the forms are entered into the database, creating a listing of patients by states/provinces as well as by type of hemophilia and by age group. The forms

are distributed at the NHC during patient clinics and also to the different regional chapters of the VAH. Every three months a new listing is produced and shared with the medical staff.

At the National Hemophilia Centre, or on visits made by the NHC and the VAH to various cities of the country along with volunteer staff of the area, registry forms are distributed even when the patient or his relative points out that he has already filled it out previously. On nearly all these visits campaigns for diagnosing new patients are carried out and the forms of the new people from whom blood samples are taken are kept aside until the medical team provides the results. This way the information is completed and immediately included in the registry. The doctor includes the clinical data of all the patients in alphabetic order in the national medical registry, with their corresponding diagnosis, complications, date of birth, home state, and medical history number. Prior to this, the doctor has checked these data with the medical and paramedical teams.

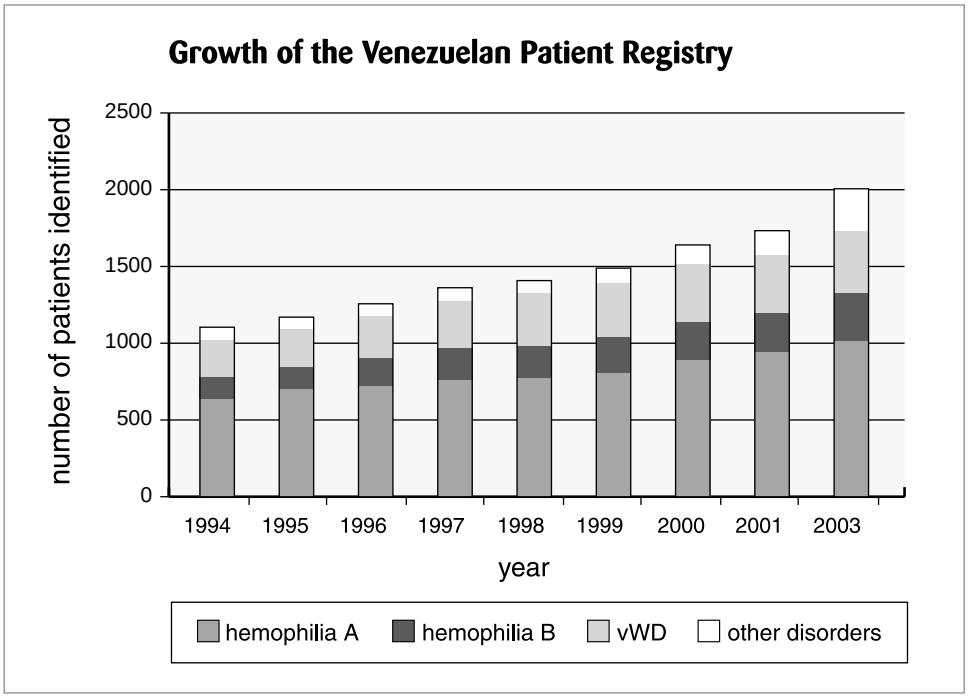
Parallel to this, the new forms received during the month and the new ones gathered from the patients awaiting diagnosis are cross-checked and a list of the patients who were misdiagnosed is compiled; this is corrected and the patients are notified. If the patient is from the interior of the country, his treating physician and the VAH chapter are notified in writing, to avoid further mistakes in treatment. Every three months the lists are checked as a routine part of data verification and control. The VAH does not have an assigned yearly budget for this management. This activity is part of the global volunteer work of both the HTC and the VAH.

State laws protect doctor-patient confidentiality, and consequently not everybody can access the medical histories. This made it necessary to start a parallel VAH registry which provides the list of patients and their contact information. Therefore the VAH registry was started precisely because the VAH could not access the data from the NHC (medical registry) due to confidentiality laws in the country. However, over time the medical doctors did allow the VAH coordinator to have access to the medical registry in order to initiate the VAH registry and in order to cross-check the information between the two registries.

It is the VAH registry that has been provided to the Health Ministry of Venezuela, to indicate the total number of people with hemophilia who are receiving factor concentrates purchased by the government and distributed by the VAH. The data supplied to the MOH only included the names, ages, type of clotting disorder, and home state. In fact, health officials are mostly interested in the percentage of patients per state and per region. The VAH is only interested in the demographic data while the HTC is mostly interested in the medical data and the details of any complications. Whenever the VAH receives an emergency call, it immediately looks up the patient on the registry list, and with the data it can then notify the corresponding medical team.

Year	FVIII	FIX	vWd	Other	Total
1994	626	160	258	63	1107
1995	680	185	258	68	1191
1996	719	196	295	73	1283
1997	759	215	328	83	1385
1998	786	219	350	93	1448
1999	814	237	367	103	1521
2000	905	262	393	119	1679
2001	940	279	415	141	1775
2003	1035	312	457	273	2077

These figures from Venezuela can be used to graph the progress of the NMO in expanding the registry.



Venezuela's patient registry has grown steadily since 1994.

In 2003, there were 2077 people with bleeding disorders identified, registered and diagnosed in Venezuela out of a population of 24 million. In a formal agreement (the National Hemophilia Program) between the health authorities, the NHC, and the VAH, all Venezuelan people with hemophilia receive an average of 27,000 units of factor concentrates per patient per year. The VAH was able to present a proposal to the authorities due to the fact that it had all the patients accurately registered and located.

In Venezuela, the hemophilia association is the only health-based non-government organizations with national data that is complete, and accurate, and this provides credibility in lobbying arguments.

Case Study: Canada

A medical registry

The patient registry in Canada is physician-run, and has been designed to serve the interests of patients while overcoming the challenges of sharing data over great distances and protecting patient privacy. It has been used to identify the patient population, plan research projects, identify the scope of viral infections and the cause of deaths, lobby governments for resources, and has aided in the fight for compensation for patients with transfusion-transmitted viral infections. The Canadian Hemophilia Registry (CHR) is operated by the Association of Hemophilia Clinic Directors of Canada (AHCDC) with the strong support of the Canadian Association of Nurses in Hemophilia Care (CANHC) and the Canadian Hemophilia Society (CHS). It was started in 1988 to count the number of patients with hemophilia A and B; later data was added including hepatitis C and HIV antibody status, the von Willebrand disease registry and, most recently, patients with rare bleeding disorders.

Data is submitted anonymously by the 24 Canadian hemophilia treatment centres (HTCs) and updated annually. Each patient is identified by factor deficiency, date of birth and an "Extra Identifier" (four letters derived from the name for example: John Smith, who has hemophilia A and was born August 9, 1990, would have the number A090890SMIT). CHR assigns this number which remains with the person if transferred to another clinic. The CHR number is used by the clinics to identify blood samples and data used in research projects. The CHR number is not used when individual data is sent to outside agencies, instead a second code, Unique Identifier (UI number) is used, assigned by clinic computers. The clinic-based computer system used for tracking factor usage and reporting adverse events, Canadian Hemophilia Assessment and Resource Management System (CHARMS), has been programmed in parallel and harmonized with CHR.

In Canada, each province has its own laws regarding the confidentiality of medical records so the participating clinics have had to make sure they are in compliance with the appropriate legal requirements for their jurisdiction. This has meant that clinics have adopted slightly different procedures regarding consent for sharing anonymous data. The CHR includes information on Canadians with hemophilia and vWD and other bleeding disorders.

Case Study: Egypt

A government registry

In Egypt, the first hemophilia patient registry began in 1971 at the Central Health Laboratories (CHL) of the Ministry of Health (MOH) in Cairo. The CHL acted as a referral unit for the diagnosis of hereditary bleeding disorders. At the time, the registry was compiled manually by adding every new diagnosed patient to the registry. It included basic information such as the name and address of the patient, birth date, sex, diagnosis, family history, and a patient identification number. To collect the patient data and history, a short questionnaire was completed. The registry data was shared with the Egyptian Society of Hemophilia (ESH) based in the hemophilia treatment centre at the Egyptian Red Crescent. Each referred patient had a record containing demographic information and the type of bleeding disorder identified.

Over the years it became apparent that there was a need to update the existing patient data (not just to continue adding new cases to the registry) and to include more data on the diagnosed patients. By the year 2000, the need for having a computerized national patient registry was critical, mainly to have the actual number of patients and to monitor treatment outcomes. Of particular importance were the problems of viral infection such as HCV due to repeated blood transfusions. So the hematology department of the CHL began to collaborate with the virology department for the diagnosis of viral infections in all patients who had a history of receiving fresh blood or blood components. Also any patients referred by the HTC were also tested for viral infections. This new data was then added to the medical registry. The patient questionnaire was updated to include more clinical information as well as past history of blood transfusion and type of components used (fresh frozen plasma, cryoprecipitate, or factor concentrates). This made it easier to better determine the need of the patients and monitor the occurrence of complications. Identification of the geographical location of patients also encouraged the establishment of regional HTCs.

By 2001, through the WFH Centre Twinning program that was established between the hemophilia treatment centre in Knoxville, Tennessee, USA, and the HTC in Cairo, the upgrade of the CHL patient registry was identified as one of the main objectives. The Tennessee centre twin donated computer software to the CHL in 2003 to be distributed to each HTC across the country so that all information and data could be centralized. This would ensure avoiding the duplication of registration of patients in different parts of the country. It was hoped that it would also result in accurate calculation of patient treatment needs and cost. To succeed in this task, the CHL communicated with all the HTCs to standardize the ID number for the patients and to verify that all cases diagnosed locally were systematically referred to the CHL for final confirmation.

Today the national hemophilia patient registry in Egypt is still managed centrally by the Ministry of Health through the head of the hematology department of the CHL. Information gathered includes demographic data (name, birth date, sex, address and telephone number, blood group), family history, consanguinity, viral profile, first site of bleeding, blood products received and number of transfusions, history of inhibitors, and the confirmed diagnosis. Specialized CHL staff is responsible for the data entry. Access to this database is

strictly limited and confidentiality is respected. Only the directors of each designated HTC in addition to the administrators at the CHL can access their own fields. The registry team at the CHL acts like a registry committee as it meets regularly with the heads of the HTCs and the ESH. It covers the task of communicating with all treatment centres and the head of the ESH to guarantee the registration of all patients.

Today, a national patient ID card is given to each patient in the country on the condition that they first obtain a confirmed diagnosis at the CHL; the patient is then referred to the Society (ESH) to obtain the patient ID card which gives them many privileges such as free treatment (based on availability) and transportation subsidies. The motivation is high since patients all want to obtain the benefits of the ID card. As a result, most of the diagnosed patients from different centres in the country are referred to the CHL and then to the ESH. This is an interesting example of collaborative work between all key players: the Ministry of Health, the ESH and the HTCs.

When a national registry is managed by the central government it can facilitate the process of collecting data from the HTCs as they must respect instructions from their Ministry of Health. It usually ensures more comprehensive data from all over the country. Having a national diagnostic reference centre that also manages the national registry can be an effective way to start and maintain a reliable registry.

Case Study: Iran

A combined patient organization / medical registry

The patient registry in Iran has a long history. Almost 40 years ago after the establishment of the first hemophilia treatment centre at Imam Khomaini Hospital, each referred patient received a record containing demographic information and the type of bleeding disorder identified. This system is still working. However, the main drawback of this system is that all patients suspected of having a bleeding disorder have a record. Therefore, it is impossible to retrieve the actual number of patients. Many attempts to computerize these records have been made but all have failed. This is partially due to the lack of appropriate management of a registry, including a database system and an assigned responsible person.

The Iranian Hemophilia Society (IHS) also has a registry. This patient organization registry has been completed with the referral of patients to the IHS chapters. Therefore, there are many patients included several times with referrals to different chapters, or some patients not included if not referred to any local chapter. There is no Ministry of Health registry in Iran.

After the opening of the first comprehensive hemophilia care centre (CHCC) in Tehran, the need for having a computerized patient registry increased, mainly to have the actual number of patients and to monitor treatment outcomes.

Over the past two years, a database has been established at the CHCC which contains demographic data and medical data. Patients are entered in this database in collaboration with local chapters in an effort to have all patients throughout the country included in this registry. It was concluded that a surprising number of patients were included more than once. While verifying records to filter the data, 900 repeated names were found and deleted. Afterwards printouts were made for each local chapter for follow-up and rechecking of the data. The stage of verifying the data will be four times yearly in order to eventually obtain accurate data.

At the moment the CHCC is responsible for managing the registry. A designated staff member, the administrator, enters the data collected from all chapters into the database. Information on treatment; bleeding history, coagulation laboratory investigations, radiographic images, infectious diseases data, family trees, and genetic analysis is entered by the specialists of each department.

There is no defined registry committee and this database has been developed voluntarily by the IHS. However, expertise is sought from specialists at the centre to add accuracy, simplicity, and completeness. Access to this database is strictly limited and confidentiality is respected. Only specialists and the administrator can alter the data fields.

Until now US$40,000 has been spent on this database which has been funded by charitable donations. The ultimate goal is to make this database web-based, in order to facilitate data entry/retrieve for all HTCs through out the country. It is important for any registry committee to put their goals in perspective in order to be able to expand their registry in future.

Sample registry form

About the sample registry form

The **sample registry** form on the next page is based on forms designed by the Centers for Disease Control. It contains sections for various kinds of data: demographic data, clinical information, and details about infectious disease and joint disease. The information in the box is essential for all registries, including Patient Organization Registries. The following sections are examples of data that is often collected in Medical or Ministry of Health registries.

PLEASE NOTE: This sample registry form is intended only as an example. It should be modified to fit national requirements.

1. **Names & 2. Date of birth**: This should be standardized so that all names and dates are entered in the same format.

3. **Sex**: Check male or female.

4. **Address**: Enter the current address.

5. **Telephone**: Enter the telephone number and area code.

6. **Place of Birth**: Enter the place of birth.

7. **Is another person with a bleeding disorder living in the same household as the patient?** Check one box only. Enter name of other person if known.

8. **Factor deficiency type**: Check known factor deficiency.

9. **Diagnostic technique**: Check all that apply.

10. **Baseline factor activity**: If known, enter the baseline factor activity as a percentage.

11. **von Willebrand disease**: If known check the type of vWD.

12. **Reason for diagnostic testing**: Check all that apply.

13. **Age bleeding disorder first diagnosed**: Enter age if known.

14a. **Has patient ever had a bleed?** Enter if known.

14b. **If yes, age at first bleed**: Enter age if known.

14c. **If yes, site of first bleed**: Enter location on the body of first bleed if known.

15. **Treatment type**

Episodic care: If patient receives treatment only in response to bleeding episodes.

Immune tolerance: If patient is undergoing immune tolerance therapy.

Prophylaxis: If patient is receiving continuous treatment lasting more than 28 days.

16. **Current inhibitor titer**: Enter the highest inhibitor number at or since last visit.

17. **Bleeding episodes in the last 12 months**: Enter the number of bleeds for each location.

18. **Treatment products used**: Check any treatment products used. Enter product name for Clotting Factor Concentrate.

INFECTIOUS DISEASE

19a. **HCV**: Enter tested or not, and status, if available. Enter date if available.

19b. **HIV**: Enter tested or not, and status, if available. Enter date if available.

19c. **Vaccination**: Check if patient has been vaccinated.

JOINT DISEASE

20a. **How often in the past year did the patient use crutches?** Enter the number of days in the past that year the patient used crutches.

20b. **How often in the last year did the patient use a wheelchair?** Enter the number of days in the past year that the patient used a wheelchair.

20c. **How does the patient describe his activity level?** Check the statement that best describes the patient's view of his activity level.

Unrestricted: Patient has few or no signs of joint disease.

Some limitation: Patient has independent mobility but some restrictions due to joint disease.

Very limited: Patient can move around independently but with difficulty.

Assistance required: Patient needs a wheelchair or help from others to move around.

Data entered by: Enter name of person creating/modifying the file.

Date created: Enter when file is first created.

Date updated: Enter every time the file is modified.

Patient ID number: Enter ID number when file is first created.

Sample registry form

DEMOGRAPHIC DATA

1. **Family Name** _____
   Given Name _____
2. **Date of birth** _____
3. **Sex** ☐ Male ☐ Female
4. **Address** _____
   City _____
   Region _____
   Postal Code _____
5. **Telephone number** _____
6. **Place of Birth** _____
7. **Is another person with a bleeding disorder living in the same household as the patient?**
   ☐ Yes Name: _____
   ☐ No
   ☐ Unknown
8. **Factor deficiency type**
   ☐ VIII
   ☐ IX
   ☐ Other describe: _____
   ☐ vWD

CLINICAL INFORMATION

9. **Diagnostic technique**
   ☐ Clinical symptoms only
   ☐ Clotting screening tests only (e.g. PT or APTT)
   ☐ Specific factor assays for factor VIII or IX
   ☐ Other describe: _____
   ☐ Not known
10. **Baseline factor activity** _____ %
    ☐ Mild hemophilia: between >5-<40%
    ☐ Moderate hemophilia: between >1 to <5 %
    ☐ Severe hemophilia: less than <1 %
    ☐ Not known
11. **von Willebrand disease**
    ☐ Type 1 ☐ Type 2a ☐ Type 2b
    ☐ Type 3 ☐ Unknown ☐ Other
12. **Reason for diagnostic testing**
    ☐ Mother known carrier
    ☐ Other family history
    ☐ Bleeding symptom
    ☐ Unknown
    ☐ Other _____
13. **Age bleeding disorder first diagnosed** _____
14a. **Has patient ever had a bleed requiring medical attention?**
    ☐ Yes ☐ No
14b. **If yes, age at first bleed** _____ ☐ age unknown
14c. **If yes, site of first bleed** _____
15. **Treatment type**
    ☐ Episodic care ☐ Immune tolerance
    ☐ Prophylaxis
16. **Current inhibitor titer** _____
17. **Bleeding episodes in the last 12 months**
    __ Joint bleeds __ Muscle bleeds
    __ Other bleeds __ Intracranial hemorrhage
18. **Treatment products used**
    ☐ Fresh Frozen Plasma
    ☐ Cryoprecipitate
    ☐ Clotting Factor Concentrate _____

INFECTIOUS DISEASE

19a. **HCV**
    ☐ Tested (date tested _____)
    ☐ Not tested
    ☐ Positive (date diagnosed _____)
    ☐ Negative
19b. **HIV**
    ☐ Tested (date tested _____)
    ☐ Not tested
    ☐ Positive (date diagnosed _____)
    ☐ Negative
19c. **Vaccination**
    ☐ Hepatitis A ☐ Hepatitis B

JOINT DISEASE

20a. **How often in the past year did the patient use crutches?** __
20b. **How often in the last year did the patient use a wheelchair?** __
20c. **What is the patient's activity level?**
    ☐ Unrestricted ☐ Some limitation
    ☐ Very limited ☐ Assistance required

FILE HISTORY

Data entered by _____

Date created _____

Date updated _____

Patient ID number _____

Glossary

CFC: Clotting factor concentrate.

Cryoprecipitate: A fraction of human blood prepared from fresh plasma. Cryoprecipitate is rich in factor VIII, von Willebrand factor, and fibrinogen (factor I). It does not contain factor IX.

Demographic data: Basic information such as age, gender and location.

Desmopressin (DDAVP): A synthetic hormone used to treat most cases of von Willebrand disease and mild hemophilia A. It is administered intravenously by subcutaneous injection or by intranasal spray.

Factor concentrates: These are fractionated, freeze-dried preparations of individual clotting factors or groups of factors derived from donated blood.

Hemophilia A: A condition resulting from factor VIII deficiency, also known as classical hemophilia.

Hemophilia B: A condition resulting from factor IX deficiency, also known as Christmas disease.

Hemophilia treatment centre: A specialized medical centre that provides diagnosis, treatment, and care for people with hemophilia and other inherited bleeding disorders.

HCV: Hepatitis C virus.

HIV: Human immunodeficiency virus. The virus that causes AIDS.

Home care: The patient administers his/her treatment product himself/herself in his/her own home.

HTC: A hemophilia treatment centre.

ID: Identification.

Identified person: A living person known to have hemophilia, von Willebrand disease, or another bleeding disorder.

Incidence: The number of new cases of a disease in a population over a period of time.

Inhibitors: Inhibitors are antibodies to factor VIII or factor IX that attack and destroy the factor VIII and IX proteins in clotting factor concentrates, making treatment ineffective.

International Unit (IU): A standardized measurement of the amount of factor VIII or IX contained in a vial. Usually marked on vials as 250 IU, 500 IU, or 1000 IU.

Mild hemophilia: Condition resulting from a level of factor VIII or factor IX clotting activity between 6 to 24% of normal activity in the bloodstream.

Moderate hemophilia: Condition resulting from a level of factor VIII or factor IX clotting activity between 1 to 5 % of normal activity in the bloodstream.

MOH: Ministry of Health. The branch of government responsible for health care.

NMO: A National Member Organization of the WFH. A national hemophilia patient organization.

Plasma-derived products: Factor concentrates that contain factor VIII or IX that have been fractionated from human blood.

Prevalence: The total number of cases of a disease in a given population at a specific time.

PWH: Person with hemophilia.

Recombinant products: Factor concentrates that contain factor VIII or IX that have been artificially produced and are, therefore, not derived from human blood.

Registry: A database or record of identified people with hemophilia or inherited bleeding disorders. A registry includes information on personal details, diagnosis, treatment and complications.

Severe hemophilia: Condition resulting from a level of factor VIII or factor IX clotting activity of less than 1% in the bloodstream.

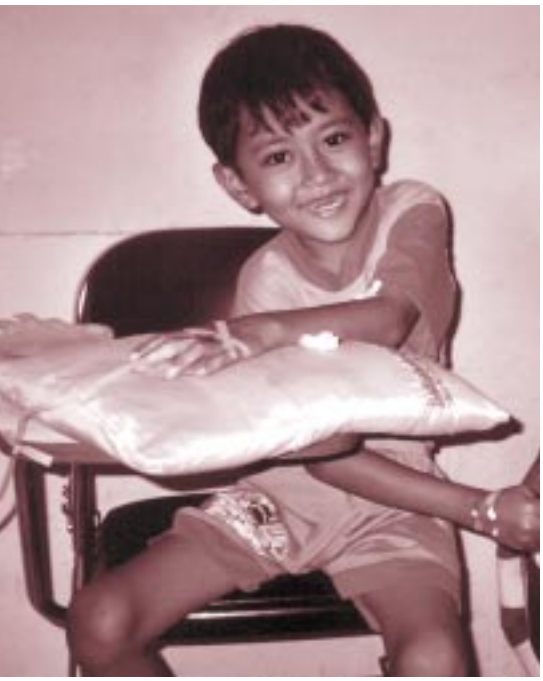
Spreadsheet: A computer or accounting program that displays data in rows and columns.

Stakeholder: Any person or organization with an interest in a topic.

von Willebrand disease: An inherited bleeding disorder resulting from a defect or deficiency of von Willebrand factor.

vWD: von Willebrand disease.

WHO: World Health Organization.



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