

Network of Rare Blood Disorder Organizations

Review of the Recommendations from the 2006 NRBDO Conference

Silvia Marchesin, Past-President, Aplastic Anemia and
Myelodysplasia Association of Canada

Toronto, November 14, 2009



Context

The Network of Rare Blood Disorder Organizations (NRBDO) was created in April 2004.

It's a coalition of national patient groups, formed to share the best practices in health care delivery for people with rare blood disorders.

They work, advocate and lobby together in a coordinated fashion on key issues to secure and maintain patient access to comprehensive care while empowering patients who have rare blood disorders

Its secretariat is managed by the Canadian Hemophilia Society.



Context

The Network identified comprehensive care as the focus for 2005-2006, and the 2006 conference as the key activity.

The Network was originally funded through a PHAC grant for two years.

It has raised funds to continue, and obtained sponsors for this 2009 conference. The members felt it would be appropriate to reflect on what progress has been made in comprehensive care since the 2006 conference, and to focus a day on patient registries.

Detailed network objectives can be found on web:

www.hemophilia.ca – NRBDO logo.



Participating associations (2009)

Aplastic Anemia and Myelodysplasia Association of Canada
Canadian Hemophilia Society
Canadian Hereditary Angioedema Network
Canadian Immunodeficiencies Patient Organization
Canadian Organization for Rare Disorders
Canadian Association for Porphyria
Canadian Neuropathy Association
Canadian Sickle Cell Society
Quebec Sickle Cell Anemia Association
Sickle Cell Association of Ontario
Sickle Cell Disease Parents' Support Group
Thalassemia Foundation of Canada



2006 Conference

Over 140 clinicians, researchers, nurses, other health professionals, patient organization leaders, government and industry representatives

Discuss and develop the vision of comprehensive care

Three motions



Network of Rare Blood Disorder Organizations

Our Vision of Comprehensive Care for Rare Blood Disorders

Silvia Marchesin, Past-President, Aplastic Anemia and
Myelodysplasia Association of Canada

David Page, Director of Programs and Communications,
Canadian Hemophilia Society

Toronto, February 3 , 2006



Acknowledgments

Our vision of comprehensive care is broadly based on the integrated service delivery model developed since 1970 for the treatment of hemophilia and other inherited bleeding disorders in Canada and abroad.

We wish to acknowledge the health care professionals, patients and families who contributed to the development of this successful model of care through difficult times.



Our focus

Hemophilia and other rare inherited bleeding disorders

Primary immune deficiency

Hereditary angioedema

The hemoglobinopathies, notably thalassemia and sickle cell disease

Aplastic anemia, myelodysplasia and PNH



Essential characteristics of comprehensive care



Provincial/territorial designation

All provinces must designate provincial programmes with dedicated budgets which mandate specialized centres (ex: hemophilia care in Quebec, Manitoba, Saskatchewan and B.C.).

These blood disorders are too rare to be decentralized for management by regional or local health authorities! This is a recipe for unequal and sub-optimal quality of care.



National patient registries

National, anonymous patient registries must be created to...

- promote high-quality, evidence-based medicine through the collection of data
- support research into patient outcomes and improved therapies.

Model: the Canadian Hemophilia Registry



Self/family administration of therapeutics

Comprehensive care centres must be set up so as to provide training to patients and families in administration of therapies:

- clotting factor concentrates
- immune globulins
- C1 inhibitor
- iron chelation
- etc.



Self/family administration of therapeutics

Hemophilia was in the vanguard of the *self-infusion revolution*, the movement away from hospitals to self care. As new therapies become available for other disorders, these patients must also benefit.



Patients, family and association involvement

The patient (or family member) is at the core of the comprehensive care team.

Likewise, the association representing patients must be able to provide input and recommendations on service delivery.



Education of patients, families and health care providers

Education is a continuous process

Education of patients and families is key to improved care, improved health

Education of health care providers

Educational guidelines to maintain same level of education across Canada



Standards of care and portability

Patients and their families must be free to move from place to place and from province to province and have access to consistent high-quality comprehensive care.

Therefore, national standards must be developed for all rare blood disorders.



Decentralization through outreach

People with rare blood disorders are not confined to the largest cities.

Treatment and services need to be provided in the home community; only highly specialized services requiring unique facilities should be centralized.

A key function of the comprehensive care centre is therefore outreach.



Multi-disciplinary care

People with rare blood disorders need more than drug therapy. Emphasis must be on:

- aggressive maintenance, early intervention and rehabilitation to reduce the burden of disease
- anticipation and prevention of physical, social, vocational and psychosocial problems
- assistance and encouragement to the patient to lead as independent and normal a life as possible.



Defined core services delivered by a comprehensive care team

- Diagnostic services
- Education
- Therapeutic services
- Nursing evaluation
- Genetic counselling
- Psychosocial evaluation and services
- Laboratory evaluation
- Provision and monitoring of blood products and supplies, adverse reactions



Defined core services

- Home/self infusion
- Women's issues, gynaecology and obstetrics
- Allied medical specialist services
- Outreach
- Referral services
- Case management
- Program coordination and management
- Research
- Coordinated hospital and ER care



A comprehensive care team

- Patient / family
- Physician
- Nurse coordinator
- Social worker
- Clerical staff
- Specialized professional services, as needed

Note: In hemophilia, 95% of the cost of care goes to blood products. The other 5% supports comprehensive care clinics. Doesn't it make sense to invest in these clinics and ensure qualified, well-organized comprehensive care teams, optimize benefits from therapies and demonstrate outcomes?



Programme evaluation and accreditation

Standards without evaluation and accountability are unproductive. Evaluation and accreditation should...

- be conducted by qualified peers
- be based on measurable standards
- be conducted regularly
- provide remediation
- entail consequences.



National collaboration among health professionals and patient organizations

We believe we are too small and rare to act in isolation or opposition. We need...

- strong links between patient organizations and health care professionals
- strong links on a national level among teams of health care professionals, notably physicians and nurse coordinators, to facilitate standards of care, patient registries and research collaboration
- strong links among patient organizations such as the NRBDO.



Post-market surveillance including blood-borne pathogen surveillance

Blood-borne pathogens are not limited to HIV, HBV and HCV. In the last 30 years, we have seen the emergence of an average of one blood-borne agent per year, including...

- vCJD
- West Nile Virus
- SEN-V
- hepatitis E, G
- etc.



A key role of a comprehensive care centre is to monitor for adverse reactions and conduct systematic blood-borne pathogen surveillance. No one else is doing this!

Collaborative Research



To measure health outcomes and conduct research into improved therapies, a comprehensive care centre linked into a national network, with an integrated information system, is essential.

Flexibility in organization

The organization and delivery of comprehensive care for rare blood disorders must be flexible to reflect local realities:

- comprehensive care centres for single diseases in very large cities vs. centres for a combination of similar diseases in smaller cities
- a network of comprehensive care centres in populous provinces vs. a single comprehensive care centre in a smaller province vs. a single centre for a grouping of provinces (e.g., the Maritimes) vs. a designated national programme.



Motion 1: Our principles

- **Motion 1:** The Network of Rare Blood Disorder Organizations, collectively, and its member organizations, individually, will advocate with provincial/territorial governments for comprehensive care for rare blood disorders. The Network recommends that the following components, in principle, be considered essential to comprehensive care:
 - Provincial/territorial designation
 - National patient registries
 - Self/family administration of therapeutics
 - Patient, family and association involvement in care
 - Education of patients, families and health care providers
 - Standards of care and portability
 - Decentralization through outreach
 - Multi-disciplinary care
 - Defined core services delivered by a comprehensive care team
 - Programme evaluation and accreditation
 - National collaboration among health care professionals and patient organizations
 - Post-marketing surveillance including blood-borne pathogen surveillance
 - Collaborative research
 - Flexibility in organization

Adopted unanimously by all participants



Motion 2: Continue NRBDO

Motion 2

The Network of Rare Blood Disorder Organizations recommends that:

Health Canada/PHAC, CIHR, Provincial and Territorial Health Ministries continue to support the work of the Network of Rare Blood Disorder Organizations.

Adopted unanimously by all participants.



Motion 3: National Working Group

- **Motion 3**

The Network of Rare Blood Disorder Organizations recommends that:

The Public Health Agency of Canada establish a national working group to coordinate and support the development of national data base registries, including quality of life measures, for the rare blood disorder disease groups including, but not limited to:

- Primary immune deficiency
- Hereditary angioedema
- Rare blood disorders
- Hemoglobinopathies
- Bone marrow disorders
- Porphyria
- Hemophilia/bleeding disorders

... and that Dr. Tom Bowen be mandated to represent the Network of Rare Blood Disorder Organizations for this initiative.

Adopted unanimously by all participants



Network of Rare Blood Disorder Organizations

I wish you an excellent conference!

