Evidence for mobilizing policy on hemophilia in India : I. Utility of hemophilia registry data from Maharashtra, 1989-2009

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Translating knowledge, making science work

Introduction

Public health indicators are quantitative summaries to guide public health policy and to measure the impact of interventions.[1]

There is very little data on the epidemiology of hemophilia in India and other developing countries.

Data on various categories of indicators that reflect population trends , quality of life and required interventions are important for advocating hemophilia services in developing countries.

Objective

To determine the twenty year trends of hemophilia in Maharashtra

Methodology

Study setting : Maharashtra , one of 28 States and 7 union territories of India, is the second most populous state in the country with a population of 112,372,972 Hemophilia services provided through 7 treatment centres (HTC)s

Type of study : Descriptive

Data sources : From national hemophilia registry (NHR) forms, HTC specific registration formats, treatment product usage registers

Inclusion criteria : Registered patients with residential address within Maharashtra

Exclusion criteria : Patients diagnosed but not registered at HTCs , having residential address outside of Maharashtra

Data collection tools : Structured data collection format.

Variables : (a) Demographic : Age, sex, place of residence. (b) Clinical : Type of disorder, severity (c) Family history data : affected or not, relationship with other affected members, (d) HTC service indicator : age at diagnosis, age at registration

Data cleaning and compilation : Data compilation using MS-Excel (Version 2007) and Statistical Package for Social Sciences (version 17)

Analysis :

Indicators for trends analysis :

1. Annual number of registrations 2. Registration of hemophilia A patients based on severity
3. Registrations of female patients and patients with other bleeding disorders
4. Age at diagnosis 5. Age at registration 6. Delay between diagnosis and registration
7. Registration by place of residence 8. Family history

Results

1. Number of registered and total patients (1989-2010)

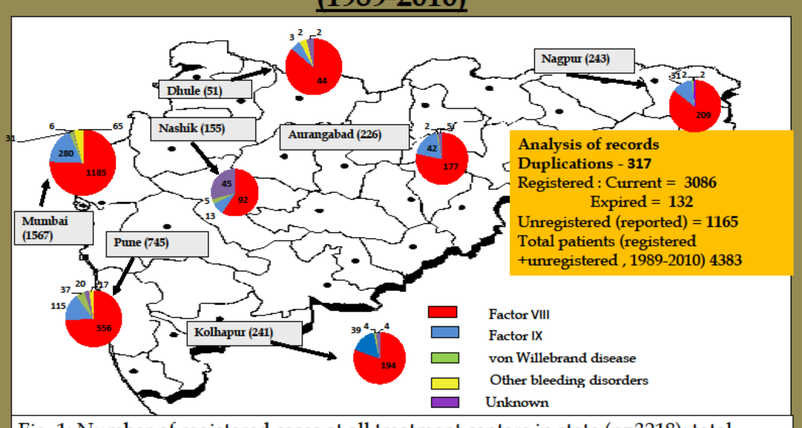


Fig. 1: Number of registered cases at all treatment centers in state (n=3218); total patients (n=4383)

2. Distribution by type of disorder and severity

Table 2. Distribution of patients by type and severity of disorders (n=3050 registered patients, type and severity unknown = 140; other bleeding disorders = 28)

Severity	Hemophilia A	Hemophilia B	von Willebrand disease
Severe	1257	222	30
Moderate	346	81	13
Mild	261	68	11
Unknown	580	150	26
Total	2449	521	80

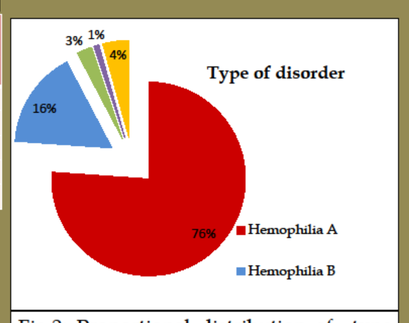


Fig.2: Proportional distribution of type of disorder (n=3218)

3. Distribution by age and residence

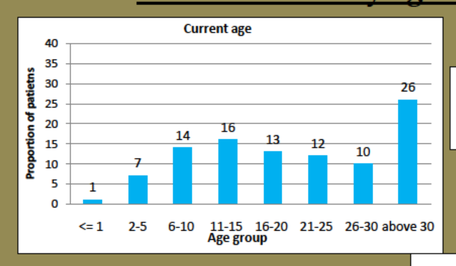
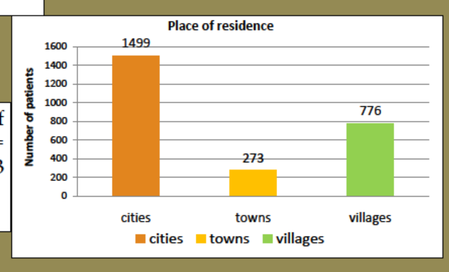


Fig. 3:Proportionate distribution of patients by calculated current age (n=2494)

Fig.4: Distribution by place of residence (n=2688) cities = 1499 (58%), towns = 273 (10%) and rural = 776 (29%)



4. Distribution by age at diagnosis and registration

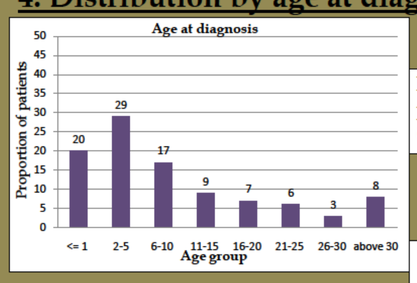


Fig.5: Proportionate distribution of patients as per age at diagnosis (n=1401)

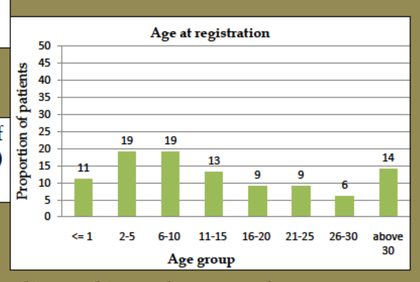


Fig.6: Proportionate distribution of patients as per age at registration (n=2860) .

5. Trend analysis :annual registrations

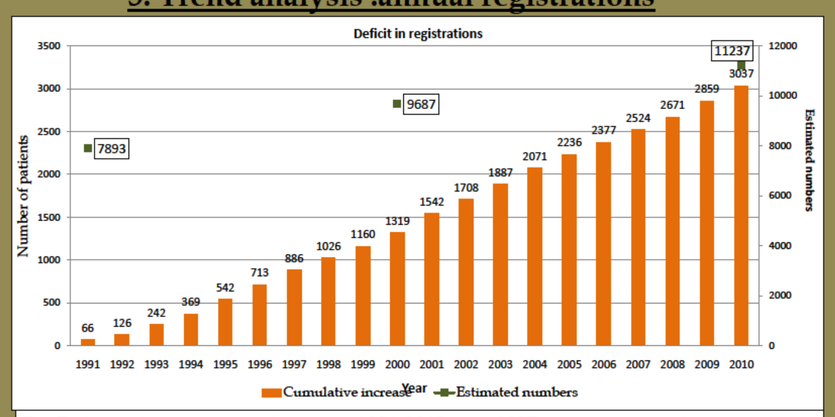


Fig.7: Annual registrations between 1991-2010. (n= 3037; average annual registrations from 1991- 2010 = 152 (± 38). From 1991-2000 the average annual registrations = 131 (± 41) and average annual registrations from 2001-2010 = 172 (± 24).

6. Trend analysis :annual registrations of hemophilia A patients by severity

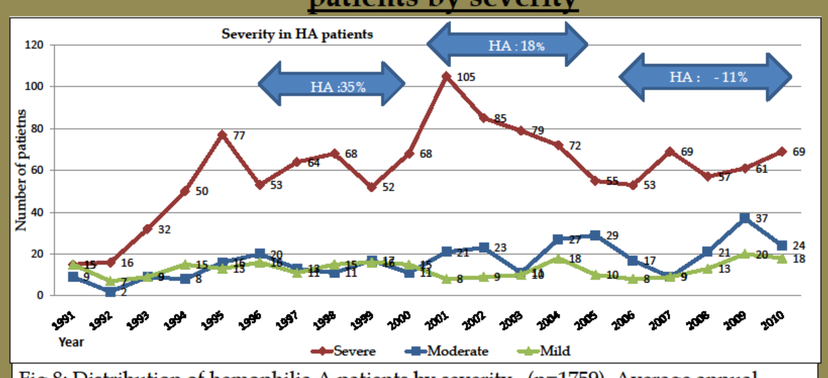


Fig.8: Distribution of hemophilia A patients by severity (n=1759). Average annual registrations of severe HA patients (n=1200) is 60 (± 21), average annual registration of moderate HA patients (n= 335) is 17 (± 8) and average annual registrations of mild HA patients (n=255) is 13 (± 4)

7. Trend analysis :other bleeding disorders and women with bleeding disorders

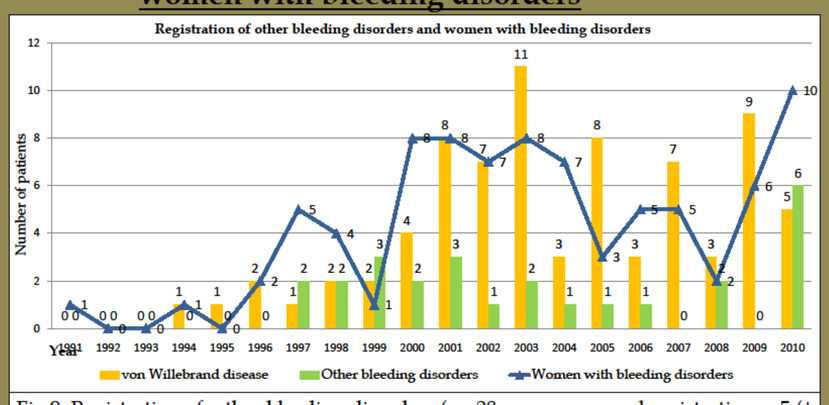


Fig.9: Registration of other bleeding disorders (n= 28; average annual registrations =5 (± 3) and female patients (n= 88); average annual registrations =4 (± 3)) .

8. Trend analysis: Age at diagnosis and registration

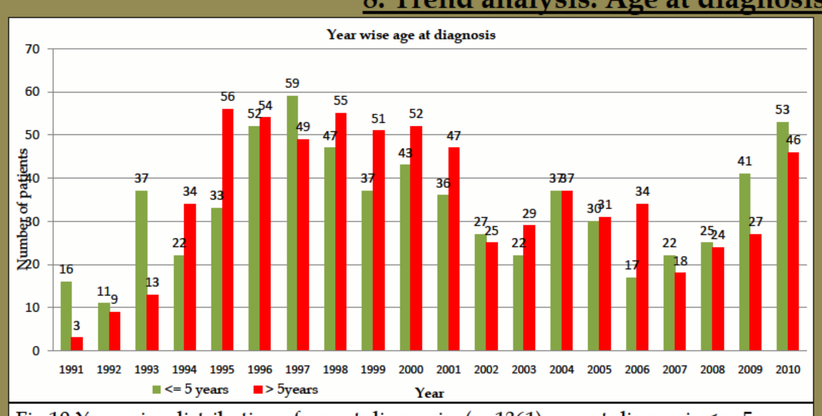


Fig.10 Year wise distribution of age at diagnosis (n=1361); age at diagnosis <= 5 years (n=667) and age diagnosis > 5 years (n=694). Average annual registrations of patients with age at diagnosis<=5 is 33 (± 13)and average annual registrations of patients with age at diagnosis > 5 years is 35 (± 16).

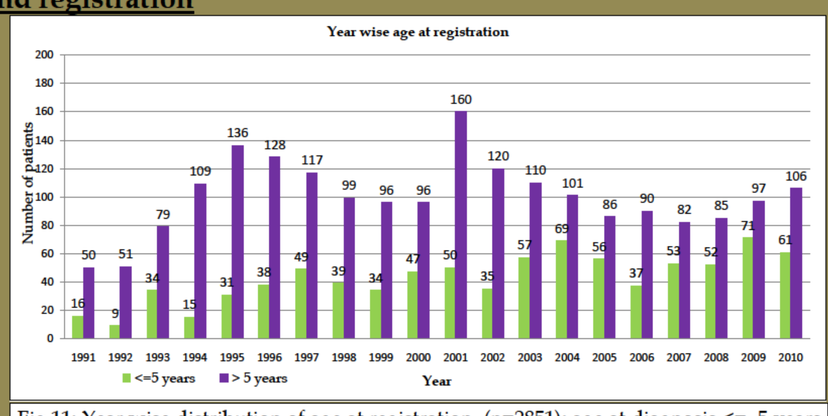


Fig.11: Year wise distribution of age at registration (n=2851); age at diagnosis <= 5 years (n=853) and age diagnosis > 5 years (n=1998). Average annual registrations of patients with age at registration <=5 is 43 (± 17)and average annual registrations of patients with age at registration > 5 years is 99 (± 25).

9. Trends in delay between diagnosis and registration

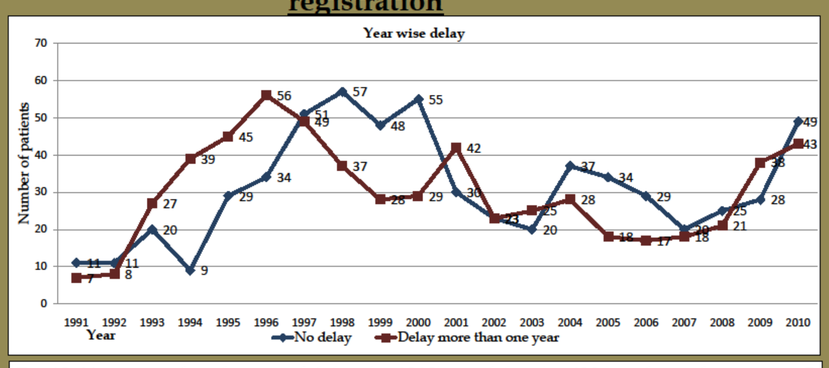


Fig.12: Year wise distribution of delay (n=1218); no delay (n=620) and age diagnosis > 5 years (n=598). Average annual registrations of patients with no delay = 31 (± 15)and average annual registrations of patients with delay more than one year = 30 (± 13).

10. Trend analysis : place of residence

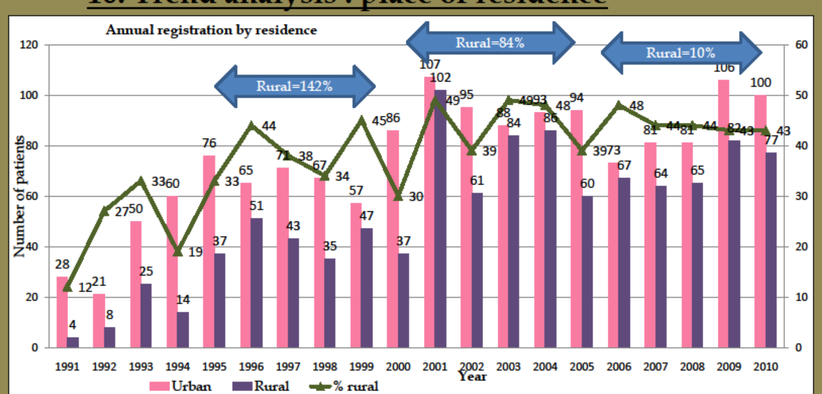


Fig.13: Year wise registration of patients by place of residence (n= 2548, urban (n= 1499) rural (n=1409). Average annual urban registration = 75 (± -23) and average annual rural registration = 52 (± 27

11. Trend analysis : Family history

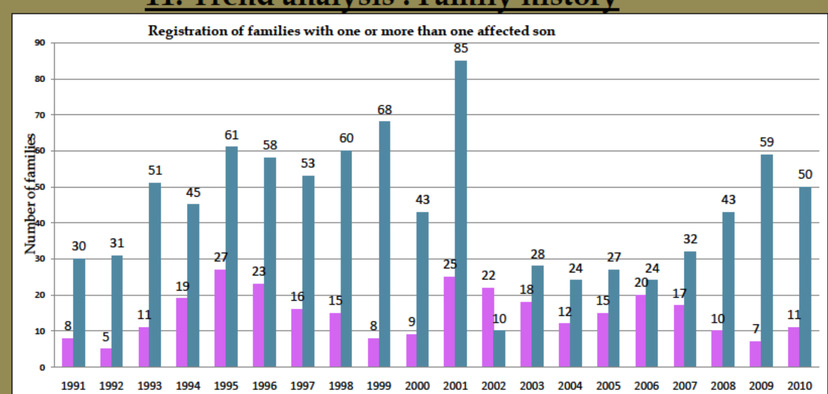


Fig. 14: Distribution of families with more than one affected son and (n = 298) and families with one affected son (n = 882).

Conclusions

1.The hemophilia registry is an example of a low cost method of rare disease surveillance as it is volunteer managed. The surveillance data can be used to determine treatment needs and longitudinal trends of the disorder. The hemophilia registry provides a model for a national rare disease registry for surveillance of rare diseases and disorders

2. Currently there are 22% patients in the first decade of life, 29% in the second decade of life and 22% in the third decade of life. 26% of the patients are currently above the age of 30 years. The proportion of younger patients remain high and thus indicates an urgent need to develop low cost management strategies to prevent disability and ensure better quality of life. The activities should focus on mainstreaming these patients from the point of view of education and occupation which is attainable through intense advocacy . Formal counseling services at the treatment centers will help these young patients manage their life with the disorder.

3. About 152 patients are added each year with 53% of them being severe and moderate hemophilia A patients with the average annual registration of severe hemophilia A patients in state being 60 (± 21). An estimate of number of hemophilia patients calculated at 1991, 2001 and 2011 indicates a deficit, a concern as to loss of opportunity in diagnosing patients.

4. 30% of the registered patients currently reside in rural areas and the numbers have increased along the 20 year period. Affordability and accessibility to clotting factor concentrate amongst these patients when the treatment centers are located at the urban level is a major factor that will affect utilization especially in case of emergencies.

Across the 20 years there has been an increase in the registrations of women with bleeding disorders and thus the treatment centers have to play a newer and important role of addressing the needs of women with bleeding disorders in the current socio-cultural background.

6. 49% of the patients were diagnosed before five years of age but only 30 % registered for utilization of clotting factor concentrate before five years of age indicating a slight delay. In the last five years more patients are diagnosed before the age of five years . However in case of registration to initiate clotting factor use, the registrations still occur after five years of age.

7. Family history was available in case of 53% of the patients and amongst the later , 48% reported history of hemophilia. The number of families with more than one affected son has shown a decrease in the last 5 years. whether this decrease is due to genetic counseling needs to be ascertained to firmly establish the credentials of genetic counseling and a common methodology to deliver it at all the treatment centers.

8. Delay in diagnosis and registration of the patients suggests that delay more than one year has reduced in the last two five year periods as compared to the years before 2000, suggesting a trend of increased access to treatment centers post diagnosis in search of clotting factor concentrate.

Recommendations

1. Wide advocacy of results of trend analysis from the hemophilia registry data
2. Mobilize funding for support of the surveillance activity and other outreach activities of the treatment centers
3. Analyzed data can be used to estimate units of clotting factor concentrate
4. Initiate age specific comprehensive care modules that cater to the specific needs of patients in various age groups to limit disability, provide psycho-social support system to improve the quality of life of patients
5. Data management, data sharing and inter linking treatment centers for better service provision and extension of outreach activities
6. **a)** Long term trends and comparison with trends in the past is of utmost importance to understand the impact of activities and interventions carried out through the treatment centers.

b) The data provides useful public health indicators that can be tools in guiding the care policy for hemophilia; a policy for best available care and management of the disorder in a low income

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