Measuring the quality of haemophilia care across different settings: a set of performance indicators derived from demographics data

Mark Brooker[1], Alfonso Iorio[2], Jeffrey S. Stonebraker[3], J. Michael Soucie[4]
(1) World Federation of Hemophilia
(2) Department of Clinical Epidemiology and Biostatistics, McMaster University, Hamilton, ON, Canada
(3) Poole College of Management, North Carolina State University, Raleigh, NC, USA
(4) Division of Blood Disorders, Center for Birth Defects and Developmental Disabilities, Centers for Disease Control and Prevention, Atlanta, GA, USA

Background: Haemophilia is a rare disease for which quality of care varies largely around the world. Our aim was to propose evidence-based indicators as surrogate measures for provision of haemophilia care across countries and over time.

Material and Methods: Guiding criteria for possible indicators were feasibility and prompt applicability to countries with a wide range of data collection capacity. General population epidemiological data and haemophilia A population data from the WFH Annual Global Survey (AGS)[1] were used to calculate the indicators. Data collected for the years 2013 and 2010 in a sample of eleven countries were used for this pilot exercise.

Results: Three indicators were identified: 1) the *percent difference between the observed and the expected haemophilia A incidence,* which would be close to null when all of the people with haemophilia (PWH) theoretically expected in a country would be known and reported to the AGS; 2) *the percentage of the total number of PWHA with severe disease*; and 3) *the ratio of adults to children PWHA standardized to the ratio of adults to children for males in the general population,* which would be close to one if the survival of PWH is equal to that of the general population. Country specific values have been calculated for the 11 countries.

When calculating the *percentage difference of observed from expected incidence of haemophilia A* in the eleven countries considered in this pilot exercise, estimates ranged from 54.2% less than expected incidence of haemophilia A in South Africa to 111.1% more than expected in Ireland. Two countries (Georgia and United States) had an observed incidence that was within 3% of the expected.

Calculations for the *percentage of PWHA with severe haemophilia* for the eleven countries showed the percentage of patients with severe haemophilia ranged from 23% in the United Kingdom to 70% in the Republic of Korea. The mean and median values for the 11 countries were 41% and 34%, respectively.

For the *standardized ratio of adult to children PWHA to the ratio of adult to children in the general population* the ratios ranged from 0.56 in US to 1.58 in Poland. The ratio averaged 0.82 for the pilot countries and two countries (the Republic of Korea and South Africa) had ratios very close to 1.0.

The strengths and limitations of this approach are primarily determined by the quality and completeness of the data available in each country. Each WFH Report on the Annual Global Survey includes a discussion and list of caveats regarding the published data (see, for example, pages 1-2 of the 2014 report). All of those issues apply to any use of the data such as in this exercise.

Conclusions: We have identified and tested three promising indicators of quality of care in haemophilia. Further testing on a wider set of data from the AGS will be needed to confirm their value and further explore their measurement properties.

Table 1. *Percentage difference between the observed and the expected* incidence of *haemophilia A in eleven countries*, 2013 (Indicator 1)

Country	Estimated incidence	Percent difference from expected incidence
Australia	21.19	38.5
Canada	24.90	62.8
Georgia	15.62	2.1
Ireland	32.30	111.1
Korea, Republic of	9.29	-39.3
Malaysia	7.44	-51.4
Poland	10.23	-33.1
South Africa	7.00	-54.2
Turkey	16.18	5.8
United Kingdom	23.37	52.7
United States	15.72	2.8

This indicator assumes an expected incidence of people with haemophilia A in the population of 15.3 x 100,000 males, estimated from Soucie et al. 1998 [2].

Table 2. *Severe haemophilia A patients as percentage of total haemophilia A patients in eleven countries*, 2013 (Indicator 2)

Country	Total haemophilia A	Severe haemophilia A	% severe haemophilia A
Australia	2,071	713	34
Canada	3,006	869	29
Georgia	232	64	28
Ireland	575	197	34
Korea, Republic of	1,602	1,116	70
Malaysia	1,109	384	35
Poland	2,280	1,216	53
South Africa	1,741	1,045	60
Turkey	4,369	1,476	34
United Kingdom	5,651	1,291	23
United States	12,957	6,841	53

Table 3. *Ratio of adult to children haemophilia A cases standardized to the ratio of adult to children in the general population of eleven countries*, 2013 (Indicator 3)

	Ratio (19-44)/(5-13)		Rate Ratio
	PWH	Population	PWH/Pop
Australia	2.788	3.396	0.82
Canada	2.947	3.668	0.80
Georgia	2.775	3.540	0.78
Ireland	2.255	3.127	0.72
Korea, Rep. of	4.179	4.298	0.97
Malaysia	0.387	2.256	0.17
Poland	7.112	4.509	1.58
South Africa	2.617	2.625	1.00
Turkey	2.070	2.573	0.80
United Kingdom	2.916	3.417	0.85
United States	1.652	2.945	0.56

0.17 for Malaysia is an outlier due to the high number of patients with age reported as unknown in the global survey.

[1] World Federation of Hemophilia. World Federation of Hemophilia Report on the ANNUAL GLOBAL SURVEY 2014 [Internet]. World Fed. Hemoph. 2015. p. 54. Available from: http://www1.wfh.org/publications/files/pdf-1627.pdf

[2] Soucie JM, Evatt B, Jackson D. Occurrence of hemophilia in the United States. The Hemophilia Surveillance System Project Investigators. Am J Hematol 1998; 59: 288–94.

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Mark Brooker
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