THALASSAEMIA INTERNATIONAL FEDERATION
In official relations with the World Health Organization

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68th session of the WHO Regional Committee for Europe
Rome, Italy, 17–20 September 2018

STATEMENT OF THALASSAEMIA INTERNATIONAL FEDERATION (TIF)

Agenda Item 5 (o): Action Plan to Strengthen the Use of Evidence, Information and Research for Policy-Making in the WHO European Region (resolution EUR/RC66/R12)

The Thalassaemia International Federation, is making this statement in order to support the above resolution and emphasise the need for accurate information in formulating policies for chronic and rare disease, of which the haemoglobin disorders are a major example. These disorders, mainly represented by thalassaemia and sickle cell disease, are complex lifelong haematological disorders, which are complicated over time by multi-organ involvement. Effective management requires many resources, which the health services of each country are called upon to provide, if patient survival and wellbeing are to be safeguarded. In service development, the following services must be provided and coordinated: blood adequacy and safety which involves the blood transfusion services, essential drugs, technology (e.g. MRI to assess iron overload, molecular laboratories), day care units for blood transfusions and outpatient clinics, multidisciplinary care and psychosocial support[1].

For maximum effectiveness, these elements of patient care require expertise, which necessitates more policy considerations, including accredited reference centres and networking, educational activities such as e-platforms, publications and workshops, manpower planning and a plan for monitoring and evaluation. This need for complex services makes it essential to understand the real burden of disease, which is an important issue for budgetary and public health planning. This in turn requires epidemiological studies (geomapping, micromapping and, disease surveillance). How many patients, where do they live, how many are added each year? Such information, is needed to support policy and service development. The most basic source of such information, is a patient registry, which is defined as “an organised system that collects and stores information in the form of unified data, to evaluate specified outcomes for a population defined by a particular disease”[2].

The majority of European countries do not have such registries, for haemoglobin disorders or any other rare disease. Not knowing the real burden of disease is a major obstacle to effective health planning. Registries are essential tools for gathering information, which help to define the epidemiology, clinical outcomes and the natural history of these rare conditions. Such information will help to improve quality of care and to plan services, as well as to assist in research projects including clinical trials and the recruitment of volunteer patients. Policies concerning reference centres, networking and cross-border health, make the development of registries at healthcare facility, national and international level, necessary tools to facilitate the creation and implementation of these policies. Registries can also be designed to support auditing a service, for clinical trials and other research. In this respect patient sub-groups can be identified, for more specific enquiries and research. The organisation of data electronically is a major tool in making sense of the clinical data collected over many years, and so expansion of registries to complete electronic medical records should also be considered.

These are all in agreement with the principles contained in resolution EUR/RC66/R12. TIF, reiterates that life-long and complex disorders require even more careful data collections and so urges for a stronger reference to this unmet need in all future regional documentation on the subject. TIF also urges the Regional Office to adopt a coordinating role, so that more countries adopt rare disease registries in an

interoperable system, able to serve cross-border health in a more effective way. These rare disorders can only be served in Pan-European setting.

1. Cappellini MD, Cohen A, Porter J, Taher A, Viprakasit V (Editors). Guidelines for the clinical management of transfusion dependent thalassaemia (TDT). 2014. TIF publication 20.
2. Gliklich R, Dreyer N. Registries for Evaluating Patient Outcomes: A User's Guide. Rockville, MD: Agency for Healthcare Research and Quality; 2010. AHRQ Publication No. 10-EHC049.