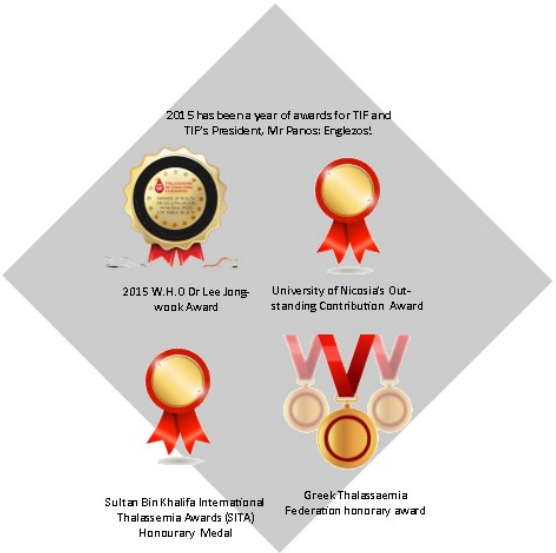
THALASSAEMIA INTERNATIONAL FEDERATION
In official relations with WHO since 1996

2016

Briefing for the Board of Directors



Briefing for the Board of Directors

by Thalassaemia International Federation

14/04/2016

February 2015-

March 2016

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Western Pacific Region (WPR)

China

Following TIF's activities in China and the previous delegation visits in the country, the collaboration between TIF and the Family Planning Bureau of China from 2010 has been fruitful with successful prevention programmes implemented in the Guangxi and Guangdong provinces.

Patient care is now TIF's main goal in the country and TIF has proceeded in signing a Strategic Agreement with the Thalassaemia Chinese Federation (TCF) on the 13th December 2015. Having signed this alliance, the two parties agreed on a plan of activities that needs to be put into place in order to achieve the aims of the cooperation, which are the promotion of the quality of health and life of thalassaemia patients.

The next step to this regard is a delegation visit which will take place between the 3rd and 8th May 2016 and which will move forward the following planned activities :

- Promotion of 5 hospital based Thalassaemia centres based on the models observed during TCF's visit in Nicosia and Torino.
- Tour of expert group to 3 hospital units for upgrading to reference centres: Delegates are: Antonio Piga, Aggie Michael and three TIF delegates (Dr Michael Angastiniotis and two other names pending).

We will keep you in the loop with more details in our next Board Member Update.

Eastern Mediterranean Region (EMR)

Pakistan

Pakistan is a high frequency and prevalence country, with 22,000 known and up to 50,000 -150,000 possible patients and up to 5,000 new cases added each year.Thalassemia constitutes a major challenge to the national healthcare system of Pakistan with its consequent medico-socio-economic repercussions, not belittling the pain and suffering to the

patients and their families.

To focus on the needs of this country, TIF realized a delegation visit between the 5th and the 6th of April 2016 in Islamabad, Pakistan.

The aim of this delegation visit was to discuss with regional stakeholders prevention and patient care matters which are of great importance for the region. The discussion also revolved around the need to promote the quality control of centres, reduce out of pocket expenses, and form private public partnerships between Thalassaemia NGOs and National Health Authorities for benefit of patients with thalassaemia.

Dr Michael Angastiniotis also met with the Director General of the Bait-Ul-Mal Foundation to discuss ways through which Thalassaemia programmes may be supported. The foundation has been very active in supporting the treatment of 7000 thalassaemia patients and has its own model Thalassaemia centre.

This delegation visit was constructive and its outcomes are anticipated to be fruitful both for the country and the region. TIF expressed its commitment to support patients with thalassaemia in the country and across the region.

South East Asian Region (SEAR)

India

Having successfully completed the capacity building activities in India and the preparation of the charter of demands of the State Task Force groups to the health authorities of 8 states (Delhi, Maharashtra, Gujarat, Punjab, Haryana, Madhya Pradesh, Utter Pradesh, Chandigarh), TIF is now keeping the momentum going by appointing a responsible person to monitor and report all developments in the 8 states.

In addition, Dr Michael Angastiniotis will visit India between the 16th and 20th of April to meet Task Force leaders and review progress in the

country. He will also take part in a series of workshops in New Delhi (16 April 2016), Kolkata (17 April 2016) and Mumbai (19 April 2016) to promote prevention, especially with regards to laboratory aspects.

We will keep you posted for any further updates in the next Board Member Update.

European Region (EUR)

Romania

A TIF delegation visit to the capital, Bucharest, took place between 24-25 March 2016 which aimed to promote TIF's activities and goals in Romania, and to find out the latest developments and policies regarding health care services for rare and chronic disorders.

Dr Michael Angastiniotis, TIF Medical Advisor and Dr Christina Stephanidou, TIF Board Member represented TIF in this delegation visit. In Bucharest, the TIF delegates attended a workshop, where patients, parents and doctors were invited to participate. After the workshop, they had the opportunity to discuss with the doctors, the patients and their families who were present at this event. In addition, the two delegates also had the opportunity to visit the local Hospitals as well as the National Institute of Hematology. Finally, TIF gave Mr Costin Ganescu from the Romanian Thalassaemia Association a donation, in recognition of the valuable work the association is carrying out.

TIF reiterates its aim to further support national efforts to address the control of thalassaemia, and to identify solutions to improve thalassaemia patients' quality of life and access to quality healthcare.

TIF is currently in the process of identifying experts or doctors in order to translate the 3rd edition of the Guidelines for the Clinical Management of Transfusion Dependant Thalassaemia in Romanian.

1. COUNTRIES

Turkey

TIF was invited by medical specialists to attend the 2nd Thalassaemia and Haemoglobinopathies Congress in Antalya in Turkey between the 30th of March and the 2th of April 2016.

TIF Delegate, Dr Michael Angastiniotis delivered a presentation for participants at the congress entitled “The value of Prevention in the Care of Thalassaemia”.

He also attended a meeting with the board of the Turkish Thalassaemia Federation. Dr Angastiniotis on behalf of TIF gave the Turkish Federation an honorary plaque, as well as a donation, in recognition of the valuable work the association has accomplished in the region.

TIF is currently exerting efforts for the translation of TIF’s Guidelines for the Clinical Management of Transfusion Dependant Thalassaemia (3rd edition) and TIF’s Educational Platform into Turkish and is seeking to identify experts/doctors who can undertake to this task.

Germany

TIF, represented by Ms Lily Cannon, TIF Operations Manager, will attend a state-of-the-art symposium on benign hematology entitled the Hämatologie Heute (German for “hematology today”), which will be held between the 21st and the 23rd of April 2016, and is organized jointly by Prof Holger Cario of Ulm University and Dr Stephan Lobitz, of the Charité Universitätsmedizin, Berlin. The symposium will be held in Scharnhorststr (Karl Storz endoscopes teaching centre).

This three-day symposium is the fifth to be organized in Germany and is definitely a story of success that helped to “resuscitate” hematology in Germany but also regionally .

Topics covered during the symposium include Thalassemia, Neutropenia, Unclear Anemia, Neonatal Hemochromatosis and Pregnancy in Sickle Cell Disease. The participants will primarily consist of clinicians who are

actively treating patients with sickle cell disease and tranfusional iron overload. There will also be several patient organisations attending.

You can find the program of the conference on the following link: http://www.haematologie-heute.de/programm/.

Cyprus

TIF in collaboration with the Cyprus Alliance for Rare Diseases (CARD) have organized a Press Conference to commemorate the Rare Disease Day, celebrated every year on 29 February.

The conference was held on the 29th of February in Nicosia. The theme of this year's Rare Disease Day in accordance with the European Organisation for Rare Diseases (EURORDIS) was: "Patient Voice". The day's slogan was: "Join us in making the voice of rare diseases heard". Thalassaemia united its voice with other rare diseases to be heard and taken under consideration.

The theme of the press conference was: "Reform in health and rare diseases in Cyprus". On this day individuals who are affected by rare diseases, together with patient organizations, politicians, carers, health professionals, researchers and the pharmaceutical industry, unite their voice to inform and sensitize the society on rare diseases and the challenges that patients face.

During the Press Conference, a presentation of TIF's and CARD's activities took place, with a special emphasis on TIF's smartphone application, ThaliMe. Additionally, a film created by the members of the Cyprus Alliance for Rare Disorders was screened as well as the official video of EURORDIS.

2. TIF CONFERENCES

MARK YOUR CALENDAR!

2nd Middle East, Gulf, Maghreb and African Regions (MEGMA) Conference on Thalassaemia and Other Haemoglobinopathies

Autumn 2016

TIF is delighted to announce the organisation of the long-awaited 2nd MEGMA Conference on Thalassaemia and Other Haemoglobinopathies, covering the **Middle East (ME), Gulf (G), Maghreb (M) and African (A)** regions.

Participating Countries

Afghanistan	Jordan	Pakistan	Syria
Algeria	Kuwait	Palestine	Tunisia
Bahrain	Lebanon	Qatar	United Arab Emirates
Egypt	Libya	Saudi Arabia	Yemen
Iran	Morocco	South Africa	
Iraq	Oman	Sudan	

Who should attend?

- Patients and Patient Organisations
- Healthcare Professionals/ medical specialists

2. TIF CONFERENCES

- Academics/ researchers
- Policy-makers at national, regional, and international level
- Industry

A truly inspiring event, the two day conference aims to bring together stakeholders from 22 countries to discuss avenues of action with a particular focus on **PATIENT EMPOWERMENT** and patient-association **CAPACITY BUILDING**. Furthermore, the scientific programme will cover a broad range of topics on the clinical management and **MULTIDISCIPLINARY CARE** and most importantly novel and **INNOVATIVE TREATMENT** methods thalassaemia and other haemoglobinopathies.

Please circulate this information to all the members of your association and feel free to include it in your newsletter, website and social media profile.

More details will be available soon

2. TIF CONFERENCES

Side-meeting to the 69th World Health Organization (WHO) General Assembly

TIF is the process of organizing a parallel side-meeting during the 69th WHO General Assembly which is to take place between the 23rd and the 28th of May 2016, in Geneva, Switzerland. The side-meeting will revolve around the risk of using substandard, spurious, falsified and counterfeit medicinal products, which falls within the scope of the agenda of the General Assembly agenda, Article No. 16.4 Substandard, spurious, falsely labelled, falsified and counterfeit (SSFFC) medical products.

Following the trend in recent years of the transformation of health care systems into more patient orientated ones, and the effort to save costs by introducing more and more the use of generic drugs, TIF has been focusing in the last 2-3 years in spreading awareness and updated and upgraded information on the use of safe and effective generic drugs whose manufacture is carried out in accordance to international acceptance standards (FDA/ EMA). In addition, TIF has placed emphasis on the role of policymakers at the national, regional and international level, as well as of the health professionals and patients, in ensuring their safety and efficacy.

Our organisation has been involved in a number of discussions and has received a number of complaints and reports on the use of drugs that under the label “generic”, are actually of unknown origin and quality of production, and as such, put our patients into great risks. TIF very much supports the increase of access of quality drugs to patients across the world and realizes that generic drugs will solve a lot of the economic and financial problems related to increased access to life-saving drugs. However, TIF also focuses on the safety and quality of the generic drugs that are offered to its patients all over the world.

The outcome / deliverables of the side-meeting will be the development of a position paper and the preparation of a booklet of information for the patients across disorders with regards to how they should ensure, based on expert knowledge, that the generic drugs they receive are truly generic and have the drug safety and efficacy expected.

3. EUROPEAN AND INTERNATIONAL COLLABORATION

For the suggested programme of the meeting, please see annexes.

European Medicines Agency (EMA)

We are delighted to inform you that TIF is in the process of renewing its eligibility status with the European Medicines Agency (EMA). TIF has already submitted its eligibility documents which are in the second phase of being evaluated and confirmed to fulfil the eligibility criteria for working with EMA.

The European Medicines Agency (EMA) engages with a network of over thirty-five eligible organisations, including TIF, ensuring that the needs and concerns of a wide range of patients and consumers are represented via direct contact with the Agency. These organisations include:

- umbrella organisations encompassing a number of smaller or national organisations;
- organisations with a focus on a specific area.

Eligible organisations receive targeted EMA communications and consultations and frequently assist in the identification of experts for product-specific matters.

For more information on the eligibility and engaging with EMA representing a patients' or consumers' organisation, see http://www.ema.europa.eu/ema/index.jsp?curl=pages/partners_and_networks/q_and_a/q_and_a_detail_000082.jsp. The result of the evaluation process will be available in the upcoming months.

Multi-Stakeholder Symposium on improving Patient Access to Rare Disease Therapies

TIF, represented by Mr Stelios Elia, TIF EU Policy Officer, has participated in the “Multi-stakeholder Symposium on Improving Patient Access to Rare Disease Therapies”, which was held in Brussels between the 24th and the 25th of February 2016.

3. EUROPEAN AND INTERNATIONAL COLLABORATION

EURORDIS brought together patient leaders, industry, academics, regulators and other players, who discussed the current state of play and how to shape a more effective way to address value determination, appraisal, pricing and reimbursement of orphan medicines. The goal of the symposium was to improve patients' access to rare disease therapies throughout Europe.

The symposium was a great success having brought together all participants to a common understanding of issues/challenges of access to rare disease therapies, and having shared existing and new processes to recognize which ones work best.

EUPATI Webinar

TIF virtually attended the EUPATI Webinar entitled Informed Consent for Vulnerable Populations on 21 March 2016.

In the current Clinical Trials Directive, the definition of conditions for inclusion of vulnerable populations is limited to clinical trials with minors and incapacitated adults who are not able to give informed legal consent. Released in April 2014, the new “EU Clinical Trial Regulation” (EU Regulation 536/2014) expands on information given for the above populations.

In this EUPATI Network Webinar the speakers presented the new conditions for informing and including the vulnerable populations in clinical trials. Participants had the opportunity to ask questions and discuss the impact of these new regulations on increasing the number of clinical trials in these populations and thus on enabling new treatments based on reliable data.

Global collaboration with Kiadis Pharma

TIF continues efforts to strengthen its new collaboration with Kiadis Pharma

3. EUROPEAN AND INTERNATIONAL COLLABORATION

N.V. (“Kiadis Pharma” or the “Company”), a clinical stage biopharmaceutical company developing innovative T-cell immunotherapy treatments for blood cancers and inherited blood disorders.

In this context, representatives from Kiadis Pharma, Mrs Susan Veenhoff (Director Public Affairs) & Dr Jeroen Rovers (Senior Vice President & Chief Medical Officer) have visited the TIF headquarters on 09 February 2016.

With the support of international thalassaemia experts, through the extensive network of TIF, Kiadis Pharma aims to develop innovative products to improve treatment of thalassaemia patients and will be entering clinical development in the first quarter of 2016. Kiadis Pharma believes that its product ATIR201™ has the potential to address the current risks and limitations connected with hematopoietic stem cell transplantation (HSCT), being graft-versus-host disease (GVHD), opportunistic infections and limited donor availability, and make HSCT a first-choice treatment of β-thalassaemia major.

This collaboration will allow TIF to further expand its international educational and awareness programs for patients and healthcare professionals, with a specific focus on hematopoietic stem cell transplantation (HSCT) as a curative treatment for thalassaemia patients.

4. PROJECTS

Thal e-Course, as a part of TIF's Expert Patients Programme

As already announced in our previous Board Member Update, a major advance in the Expert Patient programme has been the establishment of an electronic educational platform.

TIF is in the pleasant position to inform you that the platform is in the process of being reviewed by medical specialists in the field and will shortly be ready to be piloted. TIF has already gathered a group of pilots for this purpose who are expected to test the platform in the upcoming month.

As previously mentioned, the platform will utilise a critical mass of medical educational content representing various educational approaches (e.g. conventional teaching, active learning, e-learning and blended learning) to address different audiences, languages and cultures. The learner is therefore encouraged to adopt active learning, in a self-directed, personalised collaborative, learning environment. TIF wants every patient, wherever he or she may live, to be able to transform themselves into an expert advocate for their disease.

For more information, please visit: academy.thalassaemia.org.cy

More information will follow in upcoming updates.

Development of a new smartphone application!

TIF is in the process of developing a simple and user-friendly smartphone application which will inform and educate users on the prevention aspect of thalassaemia, and will be based on TIF's publication "Beta-Thalassaemia, Alpha-Thalassaemia and Sickle Cell Disease Educational Community booklet (2007) Eleftheriou A, Angastiniotis M", available here.

The mobile application will be able to run on iOS and Android devices as well as a Web Device. The iOS application will be able to run on all devices running

at least iOS 7.0 and can be made available to users via the iTunes App Store internationally. The Android application will be able to run on all Android devices running at least version 4.0 of Android and can be made available to users via the Google Play (Android app store) internationally.

Initially, our aim is to target the Indian market because the issue of the prevention of thalassaemia is very significant as indicated by the high rate of affected births, and there are over 900 million smartphone users within the country.

More information in our next update.

Living with thalassaemia: The Video Challenge competition continues for a second year!

Join the 2016 Video Challenge competition and let your imagination flow!

As you already know, TIF is holding its special "Living with Thalassemia Video Challenge" for a second consecutive year, asking members of the thalassaemia community to share videos relating to thalassaemia that will spread a positive, hopeful, or inspiring message. We are very pleased to inform you that we have received a total of **256** entries for this competition so far.

The deadline for the contest is on the 6th of May 2016, with the selection procedure taking place in mid-May. Participants are asked to upload a 2-5 minute video about their experiences living with thalassaemia and unleash their creative spirit with short films, fiction stories or animation videos about thalassaemia. The possibilities are endless!

The TIF video-sharing platform, designed with a primary focus on connecting thalassaemia patients around the world, constitutes one of TIF's most

powerful projects. The aim is to motivate patients, providing them with an incentive to upload videos in a contest that will culminate with the publication of the winning videos during May, the month of the International Thalassaemia Day!

To view entries to last year's competition, please visit: http://goo.gl/1Lh4Mh

e-ENERCA project

At this stage of the project, the registry prepared by TIF and its partners is now ready for piloting in two centres.

Preliminary data on epidemiology and other outcomes of the registry has been presented at the European School of Haematology (ESH)-ENERCA training course on diagnosis and management of rare anaemias, which was held in Lisbon, Portugal between the 29th and the 30th of January 2016.

Apart from the obvious benefits of identifying rare anaemia patients and where they are located, in support of rational policy planning for service development, the registry will also help to keep track of increases in cases due to migration currently occurring in Europe from the Middle East and Africa, which are increasing the numbers of haemoglobinopathies in European regions with historically low prevalence of such disorders, as well as other rare anaemias from countries with traditional consanguineous marriage customs. Policy adjustments may be required in European countries to accommodate the needs of congenital and chronic diseases that require specialised services.

TIF's MSc in Inherited Haemoglobin Disorders: Thalassaemia and Sickle Cell Syndromes (Blended)

TIF is delighted to announce that the work for establishing its MSc course in

4. PROJECTS

Inherited Haemoglobin Disorders, Thalassaemia and Sickle Cell Syndromes (Blended mode) in collaboration with the University of Nicosia, Cyprus is in progress and is gradually advancing. More details about this programme here.

Please stay tuned for more in upcoming Board Members' Updates.

TIF's new endeavour: Clinical Laboratory Preceptorships

The development of clinical and laboratory preceptorships (mentoring) is a new venture of TIF, which is expected to become an integral part of its educational programme for health professionals dealing with haemoglobinopathies. A series of preceptorships are planned for 2016.

Preceptorships provide opportunities for professionals to work closely for a short period of time with an expert in their field of interest, in order to benefit from their practical experience. The objective is to help raise standards, as well as to increase collaborations and networking. The first clinical and laboratory preceptorship, which will take place in the early part of the year, will be for a laboratory scientist, and will take place in Palermo, Italy under the supervision of Prof Aurelio Maggio, Director of the Department of Haematology and Oncology at the Villa Sofia-Cervello Hospital in Palermo. Expert centres in Asia have also been identified, and will host regional candidates during 2016.

The main impact of this initiative will be to increase expertise in laboratory diagnosis and research, as well as in clinical care, at centres across the world, ultimately benefiting patients in terms of better diagnosis and management.

TIF plans to make this programme a permanent part of its Educational Programme.

European Reference Networks (ERN)

TIF, as the umbrella federation of thalassaemia in Europe and worldwide, undertook the initiative to begin a line of communications with all relevant

4. PROJECTS

stakeholders throughout Europe regarding the formation of a rare haematological diseases ERN (HD-ERN).

We have worked in three main directions, namely:

i. Disseminating knowledge and information to our member base in Europe, highlighting the value of ERNs and the importance of advocating to their national health authorities for the national designation of their haemoglobinopathy centres;

ii. Sensitizing healthcare professionals (HCPs) working in haemoglobinopathy expert centres across Europe about the importance of applying and receiving national designation from the national health authorities as well as the future implications that being part of an ERN will provide for them and their Centre (e.g. EU funds for research);

iii. Working in the context of a common plan of action to enlighten both HCPs and national health authorities about ERNs in collaboration with the other haematological patient associations that are members (together with TIF) of the European Hematology Association (EHA) Patient Advocacy Group. This is a group of 10 haematological patient organisations working at the national, regional and international level representing patients with malignant and non-malignant haematological disorders, collectively forming the Patient Advocacy Group of the European Hematology Association (EHA) – the regional professional body for haematologists, providing the patient perspective to EHA's advocacy and educational programme.

Based on our communication with the above-mentioned stakeholders, we have ascertained that there is poor knowledge amongst HCPs about ERNs, and even more confusion at the level of policy-makers at the national level regarding criteria etc. However, by joining forces with the European Hematology Association (EHA) we are confident that we will reach the grass-root national levels of haematologists urging them to lobby their governments for national designation of their centres.

Additional to the aforementioned actions, TIF has also participated in five calls with the EHA Patient Advocacy Group, in the last four EHA also participated.

5. COMMUNICATIONS

March Newsletter!

Our March Newsletter will soon be available on our website.

Annual Report

TIF is in the process of compiling its Annual Report. which is anticipated to be ready and uploaded on TIF's website end of April.

6. INTERNATIONAL THALASSAEMIA DAY

We are delighted to present you the 2016 theme for the 8th of May activities which is:

"Access to Safe & Effective Drugs in Thalassaemia".

This year's theme highlights the need to support policy decisions that safeguard health and reduce inequalities in the health field. TIF's mission is to ensure equal access to quality health care for all patients, so that they receive appropriate treatment and drugs, free of charge or reimbursed by the government, and in concurrence with the guidelines and standards of international experts.

You are kindly requested to send us your plans of action for the 8th of May 2016 through completing the following **form.**
or alternatively by sending them to us at thalassaemia-lp@thalassaemia.org.cy.

International Photography Contest 2016

Photography contest
Thalassaemia International Federation (TIF) is organising a Photography competition for the year 2016, with the aim of creating awareness about thalassaemia, in the context of the International Thalassemia Day, celebrated each year on 8 May.

Participation in this contest is open for all ages, and all countries across the world.

Deadline
The deadline for the competition is Tuesday, 30 April 2016.

Subject
The subject of this photography contest is based on this year's theme for the International Thalassaemia Day

The prizes granted by Thalassaemia International Federation for this

competition are the following:

- The first winner - $ 500,
- The second winner - $ 300
- The third winner - $ 100

Submissions
Submissions should be sent to thalassaemia-lp@thalassaemia.org.cy with the name and surname of the participant as well as the title of the photography contest title in the subject of the email.

For further clarifications, please contact Thalassaemia International Federation (TIF) at thalassaemia-lp@thalassaemia.org.cy or 00357 22 319 129

Click here for the **Terms and Conditions** of this contest.
Click here for the **FAQ** section.

Anthem translated and sung in English for this year's International Thalassaemia Day!

As some of you may already know, an anthem for thalassaemia has been created in Greek by Mr. George Theofanous, a world renowned composer, musician, and lyricist. The music for this anthem was composed by him and the lyrics were written by Mr. Stavros Stavrou. This composition was sung by the children of his Musical Workshop and it was presented for the first time during TIF's formal Musical Evening and Gala Dinner, entitled Precious Rubies, held at the Presidential Palace on 6 May 2015 to honour last year's International Thalassaemia Day.
We are delighted to share with you that this anthem has now been translated and sung in English! You can listen to it by clicking the following link:

We invite you to translate them into your language, so that we can spread the joyous and hopeful message conveyed by the anthem to all those affected by thalassaemia for the International Thalassaemia Day 2016.
Closing, we would like to express a special thank you to Mr George Theophanous, our Musical Ambassador for his support and contribution to advocating the rights of thalassaemia patients.

Medical Journalism at the forefront: through TIF's interactive workshop in Cyprus!

16 May 2016, Journalist House, Nicosia

TIF is planning to organise a workshop on medical journalism, on the 16th of May 2016, from 10:00 a.m. to 13:00 p.m. at the Journalist House in Nicosia.

This workshop will be organized in collaboration with the Cyprus Union Journalists. The Cyprus News Agency acted as the Communication sponsor.

The aim of the workshop will be to discuss and exchange views, experiences, and knowledge on how media should cover health issues. Renowned BBC Journalists from the UK have been invited as speakers for this workshop

More information in our next Board Member's Update

Toolkit available!

Click here for the poster.
Click here for the banner.

International Thalassaemia Day website!

We have devoted a website for our members to share their plans for the 8th of May and keep posted on the latest news of the International Thalassaemia Day 2016! You are encouraged to visit it at:

http://tif-8-may-2016.weebly.com/

7. TIF PUBLICATIONS

OUT NOW!

Publications

- Guidelines for the Management of Transfusion Dependent Thalassaemia (TDT), 3rd Edition (2014), English

Translations of Publications

- A Guide for the Haemoglobinopathy Nurse (2013), Greek

COMING SOON

Publications

- Shorter Version of the Guidelines for the Management of TDT (2014), English, Turkish
- Shorter Version of the Guidelines for the Management of Non Transfusion Dependent Thalassaemia (NTDT) (2013), English, Turkish
- Shorter Version of the Prevention of Thalassaemias and other Haemoglobin Disorders, Vol 1, and 2, 2nd Edition (2013)
- Update of the Guidelines for the Management of NTDT (2013), English

Translations of Publications

- A Guide for the Haemoglobinopathy Nurse (2013),Farsi, Myanmar
- Guidelines for the Management of TDT 3rd Edition (2014),French, Turkish, Greek, Arabic, Chinese
- Sickle Cell Disease (2008), Arabic
- Guidelines for the Clinical Management of Non-Transfusion Dependent Thalassaemias(2013) in French

Translations of Videos

- All about Thalassaemia Cartoon Animation (2013) is being translated into Bahasa Indonesian, Malaysian, Chinese, Hindi, Urdu

POSITION PAPERS

It is TIF's policy to present its position on key issues of concern to patient groups, health authorities, professionals and society as a whole. Position papers are curently being prepared on the following topics:

7. TIF PUBLICATIONS

Table 1.1 Translation and Distributions of TIF publications

- Liver disease in thalassaemia - liver disease is emerging as a major cause of morbidity and mortality in thalassaemia patients. TIF therefore maintains that its prevention, monitoring and case management must be a priority for thalassaemia services.

- Effective monitoring of patients - TIF is concerned that few centres follow full monitoring protocols, with the result that only a minority of patients are benefiting from early identification of and protection from potential threats to their health.
- Adherence to treatment – the need for strict adherence to chelation and other aspects of the thalassaemia treatment protocol, as per international guidelines, is a subject that must be revisited, especially in view of new therapies that may lead to a final cure, but from which patients may only benefit if they maintain good overall health The first of these position papers will be released before the upcoming months..

7. TIF PUBLICATIONS

PUBLICATION	YEAR	LAN-GUAGES	COPIES/ DISTRIBUTION
Blood Safety Kit Manual for patients and parents	1999	1	6,000 /25 countries
Guidelines for the clinical management of Thalassaemia – 1st Edition	2000	10	50,000 /60 countries
Compliance to Iron Chelation Therapy with Desferrioxamine	2001	12	41,000 /45 countries
About thalassaemia	2003 & 2007	23	34,000/45 countries
Prevention of Thalassaemias and other haemoglobinopathies – Vol. I	2003 & 2013	2 1	5,000 /32 countries 2,000/ ongoing
Prevention of Thalassaemias and other haemoglobinopathies – Vol. II	2005 & 2012	1 1	5,000 /32 countries 1,000/ ongoing
Guidelines for the clinical management of thalassaemia – 2nd Edition & 2nd Revised Edition	2007 & 2008	9	12,000 /30 countries
Patients' Rights	2007	6	10,000 /25 countries
A guide to establishing a non-profit patient support group	2007	6	8,000 /20 countries
Set of Educational booklets: 1. β-thalassaemia; 2. α-thalassaemia; 3. sickle cell	2007 & 2013	12 1	15,000 /25 countries 5,000/on-going (Cyprus & Greece)
Sickle Cell disease – Booklet for patients, parents and the community	2008	4	6,200 /15 countries
All About thalassaemia – Cartoon Booklet	2010	9	17,000 /15 countries
Guidelines for the management of Non-Transfusion Dependent Thalassaemia (NTDT)	2013	1	7,200/ongoing
A Guide for the Haemoglobinopathy Nurse	2012	2	3,600/ongoing
Emergency Management of Thalassaemia	2012	2	5,600/ongoing
Guidelines for the Management of Transfusion Dependent Thalassaemia	2014	1	11,000/ongoing
Haemoglobin disorders	2014	1	2,600/ongoing

Medical Corner (from TIF's medical sources)

Trial of Merganser's beta thalassemia drug candidate begins

A clinical trial is underway to evaluate M012, which is being developed as a treatment candidate for conditions such as beta thalassemia and some subtypes of myelodysplasia.

Merganser Biotech Inc. has initiated the first clinical trial of its lead new drug candidate under development to treat rare hematological and iron overload diseases.

The King of Prussia biopharmaceutical company is studying M012 as a potential treatment for conditions including beta thalassemia, a blood disorder that reduces the production of hemoglobin, and certain subtypes of myelodysplasia, a bone marrow disorder in which the bone marrow does not produce enough healthy blood cells.

"Beta thalassemia is a devastating disease that affects children and adults throughout the world," said Dr. Brian MacDonald, CEO of Merganser Biotech. "Our goal is to develop novel medicines to safely improve health outcomes for people suffering from this disease. The initiation of clinical trials with M012 represents an important step towards this goal." (AABB 25/2/2016)

Read more here

Patients enrolled in Phase III trial of sickle cell disease drug

Patient recruitment has been completed for a late-stage trial of Mast Therapeutics' vepoloxamer as a treatment of vaso-occlusive crisis in patients with sickle cell disease. The primary endpoint of the 388-patient study is reduced length of vaso-occlusive crisis. (AABB 22/2/2016)

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Read more here

Towards better prevention and management of chronic diseases

by Martin Seychell, Deputy Director General of the Directorate for Health and Food Safety, European Commission

The burden of chronic disease is staggering: 86% of all deaths, or 4 million per year, are related to chronic diseases in Europe.

Chronic diseases develop slowly, are long-lasting and often incurable. They have caused great human suffering and placed an enormous burden on health systems as well. 70% to 80% of all healthcare costs in the EU - an estimated €700 billion - are currently spent on chronic diseases. In addition, chronic diseases completely stop many people from being able to work and nearly a quarter of those who do work - 23.5% - suffer from a chronic condition. Disease-related absenteeism, as a result, costs the EU an estimated 2.5% of GDP annually.

But although the diagnosis is bleak, the prognosis need not be. Many chronic diseases, like cardiovascular diseases and type 2 diabetes, could be prevented by healthy lifestyle choices, and others illnesses, like multiple sclerosis or dementia, can be managed to help patients feel their best and remain active for longer. In short, we can greatly reduce their burden by working together toward better prevention and management.

But how can we go about this? What approach could we take? To present its ideas and open up discussion on this theme, the European Commission is inviting Member States, international organisations and stakeholders to its Chronic Diseases Conference on 21 April in Brussels.

This event, designed to encourage greater cooperation, will also be the right

time to launch the EU Health Policy Platform. It will be a powerful tool for communicating and working collaboratively between the European Commission and EU and national health stakeholders but also amongst health stakeholders themselves.

For all stakeholders, including those working in the field of chronic diseases, this platform will be a better way of advancing their work via online and face-to-face discussions, sharing of best practices, drafting of joint statements and promotion of events, and it gives access to a library of relevant materials.

The challenge is huge but so are the possibilities (EC Newsletter, 28/03/2016).

Read more here

Rare Disease Day 2016

29 February 2016 marks the ninth international Rare Disease Day, coordinated by EURORDIS, aiming to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives. On and around this day hundreds of patient organisations from countries and regions all over the world will hold awareness-raising activities based on the slogan *Join us in making the voice of rare diseases heard*. The theme of this year's World Rare Disease Day "***the voice of the patient***" highlights that patients must express their needs for the change that will improve their lives, the lives of their families, as well as those who take care of them. (EURORDIS, 29/02/2016)

Read more here

AbbVie's hepatitis C drug obtains EMA panel backing

AbbVie gained a positive opinion from the European Medicines Agency's Committee for Medicinal Products for Human Use for the combination of

Viekirax, or ombitasvir, paritaprevir and ritonavir, and Exviera, or dasabuvir, without ribavirin for the treatment of chronic hepatitis C patients with genotype 1b and compensated cirrhosis. The opinion was backed by Phase IIIb trial data showing a 100% sustained virologic response rate 12 weeks following treatment (AABB 29/02/206).

Read more here

Sickle cell disease among children in Africa

An article published in International Journal of Africa Nursing Sciences provides an integrative review of 63 references related sickle cell disease among children in Africa, focussing on the incidence, prevalence, morbidity, and mortality; current practices and challenges related to screening, diagnosis, and treatment. From this data the authors also provide recommendations for practice, policy, and research to improve health outcomes of children with sickle cell disease in Africa (ITHANET, 29/02/2016).

Read more here

IRDiRC publishes State of Play of Research in the Field of Rare Diseases: 2014-2015

The International Rare Diseases Research Consortium (IRDiRC) has uploaded an enlightening report which includes extensive information on the major developments across the globe in the field of rare diseases. The content of this report will be useful for any stakeholder whether it is to support decisions of policy makers and research funders, or educate the rare diseases community at large of the achievements and of observed trends which shape the future of research and development for rare diseases.

The report has been compiled by means of a systematic survey of published articles, between July 2014 and June 2015, in scientific journals and press

releases. It identifies several major policy initiatives that were taken during this period notably, the funding provided by the Canadian Institutes of Health Research and Genome Canada to study models and molecular mechanisms of rare diseases, the joint proposal of the EMA and the FDA on research for new Gaucher disease medication, among others. The report highlights the guidelines and recommendations that are likely to benefit rare disease research. The report also published the outcomes of previous major initiatives such as FORGE, Deciphering Developmental Disorders, FDA's Orphan Products Grants Program and EMA's adaptive licensing pilot project. The report also describes useful databases such as Linked2Safety, ClinRegs and ClinGen. It highlights trends that will significantly impact rare disease patients such as involving patients at the EMA for discussions on benefit/risk assessment, importance of patient reported outcomes, among others.

Read more here.

RD-ACTION: the new European Rare Disease Joint Action

RD-ACTION is a new Joint Action consisting of the member states of the European Union for rare diseases, following the two previous Joint Actions - Orphanet Joint Action and EUCERD -, and represents renewed support of the European Commission (EC) to rare diseases, through its Directorate General for Health (DG SANTE).

RD-ACTION has three main objectives:

- to contribute to the implementation, by member states, the recommendations of the EC Panel in relation to policies on these diseases,
- to support the development of Orphanet and make it sustainable,
- to help Member States to introduce the ORPHA code in their health systems to make rare diseases visible.

With a global budget of €8,344,079, this work will last three years (until June 2018), following the logic of coherence and continuity vis-à-vis the previous

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actions, but aims to go further in terms of concrete implementation and consolidation policies.

More information: European Commission press release, Orphanews

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TIF focuses on gathering news from the following sources:

1. EPHA Website/Newsletter
2. EPPOSI Website/Newsletter
3. HON Website/Newsletter
4. ECDC – Eurosurveillance Website/Newsletter
5. New Europe Daily Update
6. IAPO | International Alliance of Patients' Organizations Website/ Newsletter
7. Health-EU e-newsletter - European Commission Website/Newsletter
8. AABB SmartBrief
9. PA.S.PA.MA Website/Newsletter
10. ENERCA: European Network for Rare and Congenital Anaemias Website/Newsletter
11. European Medicines Agency (EMA) Website/Newsletter
12. European Parliament Website/Newsletter
13. European Health Commission (EHCI) Website/Newsletter
14. The Cyprus Institute of Neurology & Genetics (CING) Website/ Newsletter
15. EFN – European Federation of Nurses Associations Website/ Newsletter
16. European Hematology Association - (EHA) Website/Newsletter
17. Cyprus Medical Association (CyMA) Website/Newsletter
18. EURORDIS - The Voice of Rare Disease Patients in Europe Website/ Newsletter
19. EURORDIS - The Voice of Rare Disease Patients in Europe Therapeutic Report
20. IPE Website/Newsletter
21. IGA Website/Newsletter
22. Sickle Cell Website/Newsletter
23. OrphaNews Europe : the newsletter of the European Union Website/ Newsletter
24. EUPATI, the European Patients' Academy on Therapeutic Innovation Website/Newsletter
25. Hepatitis B and C' – Public Policy Association Website/Newsletter
26. EGAN - Patients Network for Medical Research and Health Website/ Newsletter
27. ENVI News - Europa Environment, Public Health and Food Safety Website/Newsletter
28. Heamatologica Newsletter
29. WHO Bulletin Newsletter

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The annexes referred to in the previous pages are not included in this Board Member Update. If anyone wishes to see any of these Annexes as well as more detailed information on any of the topics discussed or presented in the update, the office will be more than happy to provide them to you.

The content of this update is strictly confidential for the Board Members. It may contain information that should not be extended publicly as it may insult or may cause contradiction, dissatisfaction by our member countries or national health authorities of other countries different parts of the world. You are kindly advised to use this information for your own updating on what the TIF office is doing in the context of the policies and direction it receives after each board meeting and based on the objectives and mission of the TIF.

Stay motivated! Send us your updates at thalassaemia-lp@thalassaemia.org.cy