CML Horizons 2016: Learn. Share. Grow.

6-8 May 2016, Ljubljana, Slovenia

From 6-8 May 2016, the CML Advocates Network welcomed 132 delegates (among them almost 30 newcomers) supporting patients and families affected by Chronic Myeloid Leukemia (CML) to its annual conference CML Horizons that this year was held in Ljubljana (Slovenia). Delegates from 65 countries worldwide (Europe, North America, Asia, Africa, Middle East and Latin America) came to Ljubljana to learn from medical experts, share best practice in patient advocacy and grow their organization's capacity. Besides enlightening medical sessions, interesting panel discussions an interactive poster session was held.

CML Horizons is a fully community-run and multi-sponsored conference, which has evolved from the "New Horizons" conferences 2002-2011. The annual conference is run by the CML Advocates Network, hosted by the non-profit, Swiss-based and patient-driven "Leukemia Patient Advocates Foundation".

DAY 1: Friday 6 May

CML 201 – Overview of CML Horizons 2016 and preparation for the medical sessions
(Pat Garcia-Gonzalez)

The first day of the meeting started with CML201, a pre-session to the conference chaired by **Pat Garcia-Gonzalez** (USA). Pat gave a brief overview of the history of CML Horizons and thanked all sponsors for making the conference possible. Since 2011, CML Horizons has been organized by CML Advocates Network. The goals of the network include promoting collaboration and sharing best practices between its member organizations as well as providing educational materials on CML.

This year's conference was attended by patient advocates, pharma representatives and speakers from 65 countries. On the agenda were 4 medical sessions, 5 advocacy sessions as well as a competitive poster session. There was ample opportunity for networking with other advocates, industry representatives and physicians.

Pre-session CML201 was held to refresh some medical concepts in context with CML, to introduce the doctors speaking at the meeting and to help delegates set goals for themselves for the conference. The pre-session was run as an interactive quiz: two competing teams were asked to define or explain terms and concepts in context with CML, such as tyrosine kinase inhibitors (TKIs), treatment free remission (TFR), polymerase chain reaction (PCR), BCR-ABL, generic drugs, approved label, difference between first line treatment versus first generation therapy, or between resistance and intolerance. A medical expert panel composed of Dr. Daniela Žáčková (Czech Republic), Prof. Gianantonio Rosti (Italy) and Prof. Steve O'Brien (UK) assessed which team gave the better answer. Where applicable, the doctors expanded on the answers and added further information, thereby providing advocates with a solid background of understanding for the sessions to follow.

Giora Sharf (Israel) then welcomed all participants and thanked the Steering Committee, especially Jelena Čugurović and Jan Geissler, for their hard work during the last nine months in organizing the meeting. Giora also thanked the sponsors: due to their generous financial support nearly 50 requests for travel grants had been approved. Another big thank you went to the Liberty teams from Serbia and Slovenia for their logistic support, and to the speakers for making themselves available despite their busy schedules.

Welcome to Ljubljana
(Milena Remic, Slovenian Lymphoma and Leukemia Patient Association)

Milena Remic welcomed the participants with video impressions of the Slovenian capital Ljubljana, named European Green Capital 2016. Milena then introduced the Slovenian Lymphoma and Leukemia Patient Association: for the last nine years the organization has been collaborating closely with medical professionals to assure early diagnosis, best treatment, support and information for Slovenian patients with diseases of the blood. This year's activities focus on holistic care from nutrition to therapeutic exercises and psychological support.
Slovenia has a population of 2 million people, 200 of whom are CML patients. All of them have access to modern treatment and therefore a good perspective to live for many years or even decades. Milena stressed that goal number 1 of all CML patient organizations should be to ensure access to modern CML treatment without delay for all patients in the world. Her vision is that with courage, collaboration and the voice of CML advocates, CML will be eradicated one day.

Video message (Tanja Fajon, Slovenian Lymphoma and Leukemia Patient Association)

Tanja Fajon, Slovenian delegate to the EU parliament in Brussels, greeted the conference participants with a pre-recorded video message. Tanja described how CML patients are in a permanent battle for life and for best access to medical care, medication and information. Her own experience with the disease told her that life is unpredictable. It also told her that with every year there is a better chance for treatment. In Tanja's view, the disease is a self-awareness test: it makes patients appreciate how precious life is and to learn to fight for their goals. Tanja encouraged CML advocates to stand together as survivors and strengthen forces by exchanging best practices to support other patients on a day to day basis. The joint mission is to improve quality of life and survival chances for leukemia patients.

Update on the CML Advocates Network (Jan Geissler)

Before beginning with his update on the activities of CML Advocates Network, **Jan Geissler** thanked Giora for his amazing work over the last months to organize CML Horizons.

Jan explained that the considerable growth of the network had been driven mainly by the network itself, CML Horizons and the Max Foundation who were encouraging organizations in emerging countries to join. Today, CML Advocates Network is a powerful network with 107 member organizations in 82 countries. About one third of them also deal with other rare cancers or other types of leukemia. Hardly any other network in the cancer community compares in size. CML Advocates Network might even be the largest single-disease patient network worldwide. Projects vary greatly and would not be possible without the tremendous dedication and work of all the volunteers involved.

One of the successful activities in the last year was the launch of a mobile app for iOS and Android users to track medication intake and PCR results. The app had been developed in response to the results of the adherence survey. The results had revealed that many patients were looking for a mobile tool to assist them in their everyday lives. The app is available in 7 languages and is not restricted to any specific drugs, treatment regimens or manufacturers. It is set up in a way that member organization can easily adapt it to their local needs and languages.

Further projects included the preparation and submission of a manuscript on the adherence study to the journal *Blood* for publication, the CML trials database or the patient-friendly summary of the European Leukemia Net (ELN) CML treatment recommendations. In addition, the book "Faces of Courage and Hope" with stories of 16 CML patients was published in 2015 and shipped to 52 countries. Many copies are still available and can be provided at production cost to any organization interested in distributing them.

A new initiative presented was the CML Community Advisory Board (CAB). The first pilot meeting organized by the CML Horizon Steering Committee was scheduled for 9 May 2016. The meeting will

address matters of particular interest to patients such as adherence issues or access to treatment or trials. Nine members of the Steering Committee were invited plus six further member representatives as well as five representatives from pharmaceutical companies. The objective is to provide input from patients into pharmaceutical development. A similar Advisory Board Meeting might be held with investigators at a later date.

One of the next steps will be to re-activate the CML Advocates Research Group and combine it with the CML Community Advisory Board. Ongoing projects will be continued including the CML trials database, the Network's website; the fellowship program for the European Hematology Association (EHA) meeting in June and, not least, CML Horizons 2017.

Advocacy Session #1: Patient Advocacy in Research

Chairs: Jan Geissler, Kristina Modic

- **Patient involvement in medicine R&D and PRO** (Bettina Ryll)
- **Patient-generated data: how can advocacy groups generate and publish results – a practical example: the CML Adherence Survey** (Giora Sharf)
- **How to partner with medical writers** (Marion Alzer)

In the first advocacy session **Bettina Ryll** addressed two key questions: why patient advocates should care about drug development and where and how they should get involved. Bettina argued that patients should care because they carry the ultimate risk to their lives by testing new drugs in clinical trials or by having no or only delayed access to therapies. The speaker urged that patients should be engaged in clinical trial design at an early stage. As a matter of fact, however, patient advocates are typically not asked for their input unless recruitment problems occur at a late stage of the process. In general, such problems can be attributed to poor trial design. This is why it would be much more efficient if patients were involved much earlier, helped design trials that are attractive to patients and avoid errors that cannot be corrected later on. After all, according to Declaration of Helsinki, clinical research should always be in the interest of the patient and all other interests should be secondary.

Bettina stressed that patient advocates can maximize their impact by turning opinions into data. Instead of projecting their own individual perceptions onto a wider community, they need to show that their opinions are based on data from the group they are representing. To illustrate her point, Bettina presented a project conducted jointly by the European Medicines Agency (EMA) and Myeloma Patient Network Europe (MPNE). They investigated how willing patients were to accept risks in a stage IV melanoma setting. Not surprisingly, patients were willing to accept higher risk than regulators. Interestingly, regulators were more open to risk than patient advocates. This showed convincingly that the patients themselves should be involved in benefit and risk assessment of new therapies.

Bettina concluded that patient input needs to be strategic for maximum impact. Social media provide patient networks with direct access to patient communities, which provides both an opportunity and an obligation for advocates to reach out to patients. Advocates should learn how to capture, measure and document patients' interests and preferences, and how to turn opinions into data. Such evidence-based advocacy regarding patients' needs can have a real impact for the benefit of patients.

Giora Sharf presented the CML adherence study as a practical example of patient-generated data, illustrating how patient advocates can perform and lead research for patients and make the results available to a wide audience.

The study was conducted because earlier studies had identified non-adherence as a key problem in CML. The study investigated patient behavior with the objectives to help identify the true issues behind non-adherence, explore cultural influence and support development of physician and patient tools to improve patient outcomes. A pilot study sponsored by a pharmaceutical company was

conducted in 2011-2012 to test the hypothesis and design. In 2013, CML Advocates Network in collaboration with CML experts and with support by a logistics agency then conducted an independent, multi-sponsored scientific study on adherence. Validated online and paper-based tools were used to collect data from 2546 respondents in 79 countries in 12 languages. Analysis of the data revealed huge differences between individual countries in terms of accidentally and intentionally missed doses. Differences were also observed between drugs and administration regimens as well as between the results from the pilot study and the full study. In addition, the survey showed that adherence is a joint responsibility of doctor and patient and is strongly influenced by their relationship.

Giora stated proudly that they had been the first patient group to present scientific data during an oral session at the European Hematology Association (EHA) congress 2013. In addition, a poster was presented at the American Society of Hematology (ASH) meeting 2013. A manuscript on the results was prepared with support by a medical writer and submitted to *Blood*.

Giora concluded that patient-led projects to generate data consume considerable time and resources. Organizers should seek professional support by a reliable agency and medical writers from the very beginning. This is costly but will save a lot of time.

Insights into how patient organizations can partner with medical writers were provided by **Marion Alzer** who also presented practical examples of collaboration projects.

Marion explained that medical writers can help patient organizations communicate their messages effectively and timely. The know-how and expertise of medical writers is based on their scientific background and specific communication skills. This enables them to collect and present complex information about medicine and health in clear language and in a manner that is appropriate for the purpose and target audience. Professional services they provide include writing, editing, translation and consultancy.

Marion recommended that patient advocates considering collaboration with a medical writer should determine their needs and expectations, outline their project in detail, and clearly define responsibilities from the very beginning of a project. Medical writers can be found in global and local business networks, through word-of-mouth recommendations or by searching freelance directories of professional medical writers' associations. Project budgeting should take into account that every project is unique and that costs vary depending on many factors.

One example of successful collaboration with a medical writer was the patient-friendly summary of the European LeukemiaNET (ELN) treatment recommendations in CML. The European workgroup of the Network contracted a medical writer to summarize the ELN guidelines in English lay language. The draft document was reviewed by patient advocates and CML experts. Translations by professional or community translators followed. The translations were then reviewed against the English text for scientific accuracy and completeness by local hematologists. The patient-friendly summary is available for download in 20 languages. It is easily accessible and provides highly relevant information to patients in a language they can understand. The English version alone has been accessed nearly 70,000 times.

Another example of collaboration is the CML Clinical Trials Database. In this project, the medical writer is the main contact for collecting and processing information, and updating the database on an ongoing basis. Currently 29 trials are listed in the English-language database and 26 trials in parallel in a separate database for German-speaking regions. Marion encouraged the audience to forward any relevant information to her to keep the database up to date.

Medical Session #1: CML State of Play and Future

Chairs: Sarunas Narbutas, Lisa Machado

- **ELN Recommendations on treatment choice and response** (Gianantonio Rosti)
- **Imatinib and Ponatinib - two ends of the spectrum in 2016's reality?** (Steve O'Brien)
- **Switching treatments in suboptimal response and intolerance: What to take when?** (Daniela Žáčková)
- **Debate on state of play 2016 in CML therapy** (Gianantonio Rosti, Daniela Žáčková, Steve O'Brien)
- **Future of CML: ABL001 and others treatments in the pipeline** (Gianantonio Rosti)

Prof. Gianantonio Rosti (Italy) gave an overview of the ELN recommendations from 2013 on treatment choice and response in CML. The recommendations include milestones for the response to first line treatment: optimal response, warning and failure. These three milestones form the basis for treatment decisions and are equally applicable to the three drugs available for first line use in many parts of the world: imatinib, nilotinib and dasatinib.

Randomized clinical trials (ENESTnd and Dasision) revealed very similar 5-year survival results for second generation TKIs nilotinib and dasatinib versus the first generation TKI imatinib in early chronic phase CML. Therefore, Prof. Rosti sees no reason to prefer second generation TKI over first generation imatinib for efficacy. However, many other variables also influence the choice of first line treatment, such as risk, comorbidities, drug interaction or compliance.

CML is a chronic disease. Prevention of disease progression is therefore important. ENESTnd showed that nilotinib induced major molecular response (MMR) more rapidly than imatinib, thereby preventing disease progression. For some patients, prognosis is linked to the rapidity at which disease progression is stopped and for them nilotinib is the right choice.

As for the factor risk, a higher probability of disease progression was seen in patients defined as high risk according to the Sokal Score. Prof. Rosti therefore views high risk as a clear indication for second generation TKIs as first line treatment.

Looking at discontinuation as an endpoint, some data suggests that first line use of second generation TKIs increases the chance of achieving deep molecular response and consequently hope for future discontinuation. This needs to be investigated further in clinical trials.

Prof. Steve O'Brien (UK) presented imatinib and ponatinib as the two ends of the spectrum of CML therapy in 2016. The development of therapies in CML which have greatly improved survival rates has been a great success story. Current issues with these therapies are related less to achieving even better efficacy, but focus more on benefits and risks. Risk-benefit considerations and cost-effectiveness with generic imatinib will drive the choice of treatment increasingly.

In the UK, the spectrum of drugs available for the treatment of CML includes imatinib, dasatinib, nilotinib, bosutinib and ponatinib. Imatinib will go off patent in 2016 and will then be available as a generic drug. Imatinib has been available as one of the most successful cancer medicines in at least the last two decades. It is safe and effective and, once off patent, should also be cost effective. Second and third generation TKIs have not been able to follow the success of imatinib.

Prof. O'Brien presented data from clinical trials (SPIRIT 2, Dasision, ENESTnd) to illustrate some benefit and safety considerations regarding imatinib vs. second generation drugs. Dasatinib and nilotinib achieved a clearly superior PCR response than imatinib. This might give patients hope for a potentially higher chance of achieving treatment free remission. However, a quarter of patients on dasatinib are known to develop pleural effusion as a side effect. No difference was seen in terms of overall survival and CML-related deaths. The difference in progression free survival was marginal.

Ponatinib was developed as a so-called third generation TKI. The drug is able to target T315I mutation that can cause resistance. In the PACE trial, ponatinib proved very effective in the early chronic phase of CML and also good at salvaging patients with resistance or intolerance. Ponatinib might seem to be the most effective drug but there might be risks – as with all CML drugs. The key concern with ponatinib has been cardiovascular risk.

It is known that achievement of an early good response is a predictor of longer-term outcome. Taking this into account, research now addresses the issue of risk and benefit to help select the right drug at the right dose for the patient concerned. A new clinical study SPIRIT 3 in the UK will investigate the course of patients switched to ponatinib after poor response to imatinib, nilotinib or dasatinib. The trial aims at reducing cardiovascular events by lowering the ponatinib dose, thereby optimizing safety. It is hoped that discontinuation of treatment might be possible after a few years.

Daniela Žáčková (Czech Republic) shared the latest updates on switching treatments in suboptimal response and intolerance, and explained what to take when. Dr. Žáčková initially mentioned that the category suboptimal response as defined in the ELN treatment recommendations from 2009 no longer exists. The criteria of the category were incorporated into the category failure in the ELN recommendations from 2013. Now, the only intermediate category between optimal response and failure is warning. An important objective of response definitions is to guide treatment decisions. Optimal response means that there in no indication for switch whereas failure requires a switch to a more potent drug to prevent risk of progression or death. Warning implies that a patient should continue their therapy but needs to be monitored closely.

Dr. Žáčková reflected whether warning response had any prognostic value. Studies showed that the absence of an early molecular warning response at 3 months was associated with significantly worse outcome. Nevertheless, the ELN treatment guidelines do not recommended a switch with warning response as a single measurement because it is not deemed sufficient to justify such a huge step. Outcome can still be favorable at 6 months. Furthermore, controlled trials did not show any benefit of early versus late switch. Therefore, monitoring is crucial to the decision whether to change treatment.

Switch to other treatments is only recommended in case of treatment failure or in the event of non manageable or serious toxicity.

Factors to be considered when choosing TKI for a subsequent line include: availability of drugs, treatment line, cost, efficacy, mutational status, comorbidities, and toxicity profiles of TKIs. Allogeneic stem cell transplantation is still an option as third line treatment. Limited data revealed that outcome in third line therapy was excellent.

During the following debate many questions focused on the issue of risk. The physicians stated that their appreciation and acceptance of risk might be very different to that of the patient. Therefore, risk should always be discussed with the patient and the patient should be actively involved in the decision making process. Some clinics have their own internal recommendations of which score to adopt to assess risk. Treatment decisions may also be influenced by the reimbursement status in the respective country. Advocates criticized that there are no straightforward criteria how to define or categorize risk in CML. This scenario is difficult to handle for many physicians, especially those who have limited experience with treating CML patients.

With regard to patient input in clinical research, regulators and funders in the UK require that patients are engaged in trial design. This cooperative thinking is regarded as very useful and adds valuable aspects to trials which would otherwise be missed. Nevertheless, there is still room for improvement.

Further questions and discussion touched on switching and discontinuing treatment, achieving and losing deep molecular response, overall survival as well as limited access to treatment, and diagnostic tests. Voices from the audience expressed concern that treatment decisions in certain countries are

guided by cost considerations rather than clinical treatment guidelines, and patients are even expected to have stem cell transplantation as second line treatment although this procedure may not be medically justified.

In his second presentation, **Gianantonio Rosti** voiced that the dream of every hematologist was to cure CML. Unfortunately, this dream cannot come true with TKIs because sleeping leukemic stem cells are insensitive to the action of TKI. Nevertheless, many patients can achieve a stable molecular response and continue their lives without any treatment for many years. The question arises which treatment objectives will be followed in the future: to kill even the last remaining leukemic stem cells and cure CML or to increase the number of patients who are able to stop treatment?

Prof. Rosti explained that many different drugs are known to target CML hematopoietic stem cells. These drugs include IFN-alpha as well as many investigational drugs. IFN is potent and low-cost but also quite toxic whereas the investigational drugs have not yet been proven to be efficacious in CML and their long term side effects are unknown. Interferon may mediate immunosuppression and Philadelphia-positive cells are potentially sensitive to such suppression. Interferon and TKI can act in a synergistic manner and hit different targets. Although there may be some long term benefit, adding another therapy to a TKI also means adding risk. Several clinical trials combining IFN and imatinib have resulted in higher rates of patients achieving deep response earlier but they did not show any other significant benefit. IFN might prove useful in CML in the very long term. Long term effects of nilotinib plus pegylated IFN are currently being investigated in the CML V (TIGER) study in Germany. The TIGER trial uses TKI in combination with low-dose pegylated IFN which is associated with lower toxicity than regular IFN. Still this combination cannot be promoted outside of clinical trials. Prof. Rosti does not anticipate seeing IFN prescribed in routine clinical practice. Other approaches using immuno-adoptive therapy to eradicate Ph-positive stem cells have been unsuccessful.

Another potential strategy to target leukemic stem cells includes the combination of TKIs with JAK2-inhibitors. Like IFN, they act in a synergistic manner with TKI but are associated with the risk of aplasia. Their effect on sleeping CML stem cells is unknown.

A potentially very promising drug is ABL001, a potent specific inhibitor of BCR-ABL with a distinct mechanism of action. ABL001 was well tolerated in heavily treated CML patients resistant or intolerant to other TKIs. Given in combination with a TKI, resistance to therapy can be prevented. A phase 2 trial exploring efficacy and safety of ABL001 is planned in the third quarter of 2016.

Generally speaking, patients with a high response rate may be reluctant to add another drug to their TKI therapy in the face of increased toxicity without knowing the potential benefit. Patients evaluate side effects differently than their doctors. Therefore, Prof. Rosti suggested that patients should participate in clinical trials and assess their own side effects.

Advocacy Session #2: Best Practice in Advocacy

Chairs: Gail Sperling, Bahija Gouimi

- **Access issues as generic imatinib arrives in USA** (Joannie and Jerry Clemens, Greg Stephens, USA)
- **Advocating for access in CML/GIST therapy** (Param Puthen, India)
- **Fight for continuity of care – is it feasible?** (Tamie Kimmelmann, Israel)
- **Little by little the bird makes its nest: access to CML diagnostics** (Abdoul Nasser, Niger)
- **Exempting Goods and Services Tax (GST) on CML drugs** (Abu Hurairah Bahari, Malaysia)
- **TKI access: a road less travelled** (Ken Choy, Hong Kong)

Advocates presented inspiring initiatives from around the world. Encouraging case stories highlighted the role of CML Advocates and in particular the Max Foundation in supporting advocacy groups and finding solutions in seemingly desperate situations, thereby making the world a better place.

Joannie and **Jerry Clemens** and **Greg Stephens** discussed challenges for CML patients as generic imatinib arrives in the USA.

New and expensive treatments are available in the USA and are saving lives. This includes TKIs which are currently taken by an estimated 125,000 CML patients in the USA. Although TKIs are available, access is cost prohibitive for the average CML patient in the USA where the cost of medication is 10-40 times higher than the same or similar drugs elsewhere in the world. Patients had hoped that with the arrival of generic imatinib, treatment would become more affordable. In reality, the cost of the generic drug is still 91% of the branded product. Moreover, many insurance providers will now only reimburse the cost of the generic medication even if the doctor prescribes another treatment. Also, insurance companies and pharmacies can switch patients from second generation TKIs to generic imatinib without the doctor's or patient's approval. Transition protocols are not yet in place for patients changing from branded to generic imatinib. Patients have reported new and different side effects with generic imatinib over branded.

The advocates showed best practice examples of past efforts of CML Busters and the National CML Society (NCMLS) that addressed some of these issues. In 2014, over 400 patients, medical professionals, social workers and caregivers visited congressional officers in Washington in person. They shared their individual stories and voiced their opinion how important it was to have access to affordable life saving therapies. Although this advocacy effort did not effect a change in cost sharing laws it did achieve additional cancer research funding worth 2.4 billion US dollars in 2015. As for switching therapy to generic imatinib, CML Busters and NCMLS are working on getting transition protocols established in the USA.

Param Puthen demonstrated how the Max Foundation and Friends of Max in India worked together to change a government decision on withdrawing exemption of customs duty from the life saving cancer drug imatinib.

In January 2016, the Indian Ministry of Revenue decided to impose customs duty on imported imatinib. Before then, the medication had been exempt from customs for 14 years as part of an access program. The new policy placed 18,500 poor patients at risk who could not afford to pay for their life long medication. Together with the Max Foundation, Friends of India started an advocacy campaign and petition on all available platforms to create public awareness and demand withdrawal of the decision. They asked every CML or Gastrointestinal Stromal Tumor (GIST) patient and caregiver in India to sign a petition letter in their local language and also involved key opinion leaders and engaged with policy makers. Eventually with great persistency, they were granted a patient hearing by government officials. The Ministry of Health decided to support their cause and urged the Ministry of Revenue to change their decision. Confirmation that the Ministry of Revenue will continue to exempt imatinib from custom duty is awaited shortly.

Another example of best practices of patient advocacy was shared by **Tamie Kimmelmann**. Tamie described the successful fight of the Israeli CML Patients Organization for continuity of care after arrival of generic imatinib.

Israel has a well developed national health system. Four out of the 5 available TKIs are approved and reimbursed. The arrival of generic imatinib in 2015, however, prompted serious concerns in the CML community. Patients were worried that they might lose their response, experience new or recurring side effects, be required to change generic drugs at short intervals or might not receive adequate PCR testing. Patient advocates therefore started a petition for continuing care in CML. They met with all stakeholders including hematologists, government officials and a patients' rights association to coordinate their fight. They also created a file outlining the reasons behind their demands. Following tough talks with medical directors and heads of pharmacy of the national Health Maintenance Organizations, some of the objectives were achieved.

Tamie concluded that advocacy groups cannot and should not resist the introduction of generics. They should set achievable goals and prepare solid arguments for their cause. Importantly, they should collaborate with all stakeholders and be prepared to settle for partial achievements.

A story with a completely different focus was told by **Abdoul Nasser**. Abdoul reported how his patient advocacy group "Association de Lute contre les leucemies au Niger" with the support of the Max Foundation overcame structural health barriers. They managed to provide access to the first PCR test ever done in his home country Niger.

Niger is one of the poorest countries in the world. 86% of the population earn less than one dollar a day per person and fall below the poverty line. The estimated population of 18 million includes 1 hematologist, 32 confirmed CML patients under treatment and 250 suspected cases with no means for confirmatory tests. In the past, blood samples were shipped to France for PCR testing at a cost of USD 665 per test. Needless to say, patients had to wait months for the results.

The advocacy group's first participation in CML Horizons in 2015 prompted them to fight for PCR test access in Niger. During a meeting with the Health Minister they found out that GeneXpert machines were available in the country and could be used for PCR testing. With financial help from the Max Foundation and logistic support by the manufacturer Cepheid, they managed to import BCR-ABL test kits and overcome training issues. The first PCR test was done locally in March 2016 at only about one tenth of the original cost. This has inspired the advocates to continue their fight for affordable access to best care for all Nigerians.

The initiative of the Max Family Society Malaysia presented by **Abu Hurairah Bahari** focused on advocating exemption of CML drugs from Good and Services Tax (GST). The organization engaged successfully with policy makers and hematologists, thereby ensuring continued access to CML therapy.

In April 2015, the Malaysian government implemented GST on medication. This prevented drug supplies from being cleared at customs and resulted in medication shortages at hospitals. Hematologists and patient representatives were worried that CML drugs would no longer be accessible and affordable for patients and this would impact on patient compliance and disease control. The Max Family engaged with relevant parties and approached policy makers, raised their awareness of CML and requested exemption of CML medication from GST. Their request was eventually granted and in January 2016, all CML drugs and also all other cancer drugs were waived from GST. This shows that patient voices are heard and have significant impact on making a difference to the benefit of patients.

Perhaps the most remarkable contribution to this advocacy session was made by **Ken Choy** who gave a vivid account of issues relating to access to TKI in Hong Kong. Ken referred to CML patients as refugees of war. Pharmaceutical companies are giving them hope for survival in the form of life saving drugs. However, not all patients have access to these therapies. Instead, some patients have to fight at two fronts to survive: against cancer and against corruption and greed.

Campaigns and milestones in terms of achieving access appear similar to those in other parts of the world. Nevertheless, there are a lot more obstacles and hurdles in Hong Kong. While TKIs are saving patients' lives, their outrageous price is destroying patients' financial future. Ken illustrated real life cases of patients trying to fight their fate. He introduced the concept of financial MMR, meaning minimal monetary resources to the point of being undetectable, which patients reach before they reach clinical MMR.

Ken criticized the government for not providing appropriate healthcare budgets and lacking commitment to reimburse effective TKI therapies. As for pharmaceutical companies, Ken sees it as a big mistake that they deny lower drug prices to Hong Kong. The country has neither a reimbursement program, nor a universal health insurance and patients simply cannot sustain paying lifelong medical bills. The mistake is based on Hong Kong's stigma of a high-income economy. Here, pharmaceutical companies charge the highest prices just like in the USA or Switzerland whereas they give the drug away for free to very poor countries in Africa. Pharmaceutical companies do not recognize that lifesaving drugs are a luxury for patients in Hong Kong. Ken acknowledged that Pharma do provide early access programs to free imatinib and they donate test kits.

With a call for action Ken encouraged the audience to share their success stories on achieving TKI access and funding in their countries. Finally, he expressed his gratitude to Novartis and imatinib for being alive in a very moving and courageous manner by singing a song.

The lively discussions of the day then resumed informally during the dinner at the hotel.

DAY 2: Saturday 7 May

Medical Session #2: Stopping Treatment

Chairs: Giora Sharf, Felice Bombaci

- **Oxford Style debate on stopping CML treatment** (Gianantonio Rosti, Steve O'Brien)
- **Discussion with the audience** (Daniela Žáčková, Gianantonio Rosti, Steve O'Brien)

The morning of Day 2 started with a lively Oxford Style debate on stopping CML treatment. **Prof. Rosti** acted as a lawyer presenting arguments against stopping treatment unless within clinical trials or in expert centers where frequent best quality PCR is available. **Prof. O'Brien**, in contrast, took on the role of advocating for stopping CML treatment. He argued that it can be done safely outside clinical trials, and in centers where frequent, best quality PCR is not available. Each speaker tried to persuade the audience to agree to their respective points of view.

Prof. Rosti argued that overall only a small minority of patients benefits from stopping treatment. To start with, as little as 5% of all newly diagnosed CML patients are potentially interested in a treatment stop. Patients can be considered for discontinuation if they have been in sustained deep molecular response for at least 2 years. Prof. Rosti assumes that only about 25 to 40% of patients achieve this milestone. In addition, discontinuation should preferably be attempted only in patients with low-risk at baseline. This reduces the number of patients eligible for treatment discontinuation further and therefore also lowers rates of patients in treatment free remission after 12 months. In summary, only one tenth of the originally interested 5% of all newly diagnosed patients discontinue treatment successfully.

Factors impacting on the decision to discontinue treatment include the need for life long frequent follow up as very late relapses cannot be excluded. This uncertainty can place a lot of mental stress on patients. In addition, many patients are not willing to stop treatment because logistic hurdles make it difficult to return for regular PCR tests.

Prof. O'Brien, in turn, stated good reasons for discontinuing CML treatment. He reported that many patients in the UK have stopped treatment successfully for many years. With over 1,000 patients in the literature, there is enough evidence over the last 10 years to support treatment discontinuation.

Clinical evidence suggests that the best time to stop is after patients have received TKIs for 8 years and they have been in deep molecular remission for over 5 years. Patients then have a 40% chance of staying in treatment free remission. They need to be monitored frequently in the first year and this can be done in any center. Limited data is currently available on discontinuing second generation TKI.

In terms of risk, patients may develop musculoskeletal pain as withdrawal symptoms after stopping imatinib. Nevertheless, many patients feel better after discontinuing treatment. Patients who do experience a relapse can restart treatment with their previous TKI and usually respond well. The risk of progression is very low.

Treatment discontinuation also provides cost benefits to the health system and frees resources for other purposes. In Prof. O'Brien's view, the benefits of stopping outweigh the risks.

During the following discussion, the doctors addressed many issues raised by the audience.

Prof. O'Brien emphasized that stopping without monitoring was not a good idea. He advocated monthly PCR testing for the first 6 months and thereafter every 3 months. An acceptable quality of the test is important. In Europe, a standardization process is in place to ensure good quality of PCR testing.

Prof. Rosti confirmed that nearly all patients who relapsed responded positively to retreatment with their previous treatment. He admitted that the possibility of a sudden blast crisis exists after treatment

discontinuation, but it is extremely low. This is one of the reasons why he advocated for strict adherence to the 30-day monitoring interval and for using only medical centers with relevant expertise.

Prof. O'Brien added that blast crisis is often linked to the biology of the disease and is not necessarily related to stopping treatment. He also commented that the risk of comorbidities has to be addressed before discontinuing therapy, and he advised strongly against stopping treatment without medical supervision. If doctors are reluctant to stop CML therapy, then patients can provide evidence supporting discontinuation. Patients were cautioned not to rush into stopping treatment, in particular if they have a good quality of life and are not suffering from any TKI-related side effects.

Dr. Žáčková pointed out that recommendations in some countries allow discontinuation only within clinical trials. From an economic point, frequent PCR is only reimbursed if these recommendations are followed. Having said that, medication costs are a lot higher than monthly PCR testing. Therefore, doctors need to convince payers of the cost-saving aspects of stopping treatment.

Prof. O'Brien shared a practical solution to overcome the logistics problem with frequent PCR testing. He hands out blood containers with due dates to his patients. They can then have their blood drawn locally and send the samples in these containers to Prof. O'Brien's lab for testing without having to appear in person.

Research into why certain patients can stay off treatment indicates that the duration of treatment and deepness of response seem to be decisive factors. Presence and levels of certain types of immune cells apparently can also determine whether patients will be able to stay off treatment. However, this is not yet fully understood.

Clinical trials suggest that about 1 in 4 patients develops withdrawal symptoms. Studies in the UK are investigating whether this problem can be overcome by gradually reducing the dose rather than stopping abruptly. Prof. Rosti confirmed that withdrawal symptoms seem to occur more often than reported in the literature. He believed that this problem might be underestimated.

Giora concluded that there is still a huge lack of patient-friendly information on treatment free remission. Patient advocates have a key role in educating their doctors and fellow patients.

At the beginning and at the end of this session, the audience was asked to vote for or against not stopping treatment unless within clinical trials or in expert centers where frequent best quality PCR is available. The great majority was in favor of not stopping therapy unless under the named conditions. It turned out that the arguments presented in the debate did not significantly change the audience's views on this matter.

Advocacy Session #3: Financial management

Chairs: Jan de Jong, Rod Padua

- **Fundraising in non-pharma** (Kris Rogers)
- **Writing grant requests** (Kris Rogers)
- **Show your muscles: Reporting in-kind contributions in financial reports of patient organizations** (Patrice Régnier)

The first presentation in this session focused on fundraising in non-pharma. This is a hot topic for CML patient organizations not only from the point of credibility, but also because of cuts in pharma funding due to the expiry of patents.

Kris Rogers (Malaysia) welcomed the audience to the world of technology. He referred to the internet as technology at its best. It brings together people that share a common interest. Looking at fund-raising, advocates should focus on the fact that people are naturally social and like to interact.

Advocates should also be aware that the world is truly a digital community and that advocating sharing is crucial on the way to fund-raising.

To build an on-/off-line community, it is important to have an online presence and to use social platforms. Closed Facebook groups can create engaged communities. The main currency on Facebook is “sharing”. Many “Call to Action” tools are available to ask for donations or advertise events. Facebook and Google are good stepping stones for non-profit organizations to raise funds.

Fundraising outside of pharma should take into account local sources. Nowadays, social impact is rated high on corporate agendas. Corporations and foundations will support patient groups, especially if their support has a positive and local footprint. Kris advised that patient organizations identify their social impact and make it measurable. As an example, he mentioned the many lives saved by the work of CML advocacy groups.

Any fundraising strategy should be built on three components: searching for people with common interests using, for instance, Google Alert, connecting with like-minded people or companies that might connect to the advocacy’s cause, and engaging with those opportunities. The most effective advocates are those who share. At the end, Kris shared valuable sources on fundraising networks.

In the next session **Kris Rogers** provided insights into writing grant requests that work. Careful research within and outside one’s own network and diligent preparation of the grant proposal are fundamental to the outcome. Successful grants are often connected to people we know so it is worth engaging with them. Applicants should stick to guidelines and formats, get to the point quickly and focus on how they can deliver on the grant. They also need to show measurable outcome and return.

Grant applications should include the social impact of the proposed project. The social impact of a non-profit organization can be measured in terms of providing services, helping the community, achieving benefits and introducing innovation. Importantly, any grant application has to have a convincing story at its heart.

Kris also raised some potential issues with grants and presented tips and tricks how to avoid pitfalls. He emphasized that winning a grant is one thing, delivering is the next. Grants can sometimes risk objectivity. Kris referred to Fidelity Group as a foundation of incredible funds and that they are giving anonymously. Useful sources for further information on grant writing were also presented.

Patrice Régnier (France) demonstrated how to report in in-kind contributions in financial reports. The role of a financial report is to provide a true and fair view of the financial situation of an organization. In context of advocacy it also makes potential conflicts of interest transparent when in-kind contributions come from the pharmaceutical industry. This reflects whether an organization is independent or not, which can be very important when applying for grants.

Practical examples illustrated that the actual resources of patient organizations consist not only of funding but also of endless volunteer hours and services or goods provided to the organization free of charge. Organizations were encouraged to show their financial muscles: financial strength conveys confidence in the organization’s capacity to deliver and makes it more attractive to grant-giving institutions.

Patrice presented a solid methodology and criteria for reporting in-kind contributions. Advocacy groups were advised to keep track on how they calculate and report the volume of volunteer hours, hourly rates, overheads, office space and services. Inclusion of in-kind contributions in financial reports should be verified by an auditor and published on the website of the organization for transparency reasons.

Medical Session #3: CML at both ends of life

Chairs: Mei Ching Ong, Cornelia Borowczak

- **CML in elderly: The challenge of co-morbidities and cardiovascular predispositions on choice of therapy** (Daniela Žáčková)
- **CML in young adults: Special challenges, adherence, fertility** (Gianantonio Rosti)

Dr. Daniela Žáčková discussed the challenges of CML in elderly. Old age is commonly defined as an age of 65 years and above. Although the incidence of CML increases with age, older patients are often excluded from clinical trials. Consequently, they are underrepresented in most published studies which form the basis for treatment recommendations. According to current recommendations, TKIs should be given for life. Thanks to the efficacy of TKIs, the prevalence of older people living with CML is growing. All these are good reasons to be concerned about CML in elderly.

Prior to the arrival of TKIs, age was considered a factor for poor prognosis. TKIs have eliminated the negative effect of age on the outcome. The management of CML in elderly should not be limited due to age. However, older patients have more comorbidities and take more concomitant drugs. Therefore, treatment decisions should take into account drug interactions, risk of adverse events, treatment interruptions or dose reductions which will have an impact on efficacy. It is important to assess elderly patients for their functional, cognitive and psychological status and social support before starting TKI therapy. This will help predict patient compliance and chances of therapeutic success. The management of CML in the elderly follows the same rules as in younger patients. It should not be based on age but on indicators of frailty. The choice of the TKI should consider many factors, above all safety profiles and comorbidities.

Prof. Gianantonio Rosti turned to the opposite end of the patient spectrum and looked at special challenges, adherence and fertility issues in young adults.

Patients below the age of 29 years are a strict minority in Europe. This is different in other parts of the world where the median age is a lot younger. Clinical studies in Italy and the USA demonstrated that patients between 18 and 29 years had lower response rates and a higher probability of progression. The ENEST1st trial with nilotinib as first line therapy showed that young patients achieve similar response rates at 18 months as very elderly patients. To Prof. Rosti's knowledge this is the only situation in oncology or hematology where younger patients do not reach a better response than the very elderly.

Interestingly, younger patients tend to be less adherent than older patients. This applies to taking medication as directed as well as to continuing treatment until directed otherwise. A young patient may be more motivated to continue treatment if they understand that they may have a chance to stop therapy after a specific milestone has been reached after a few years. Adherence is strongly influenced by the relationship between patient and physician. Patients are more adherent if their doctor is approachable. Tools to increase adherence include reminders from family members and pill dispensers.

Changing over to the topic of pregnancy, Prof. Rosti pointed out that CML is not a mutational agent. There are no problems associated with fathering children. The story is completely different for female CML patients. TKIs cause damage to the fetus in about 5 to 10% of pregnancies or increase the rates of post-implantation loss. Treatment of patients in deep molecular response can be stopped before a planned pregnancy or during an unplanned pregnancy. Low-dose interferon can be given if the patient relapses. The disease should be monitored with monthly PCR testing. For patients with a good response but still detectable levels of BCR-ABL, alternative experimental treatments are possible. The next update of the ELN treatment recommendations will address the issue of treatment free remission and pregnancy.

Prof. Rosti added some brief thoughts on allogeneic stem cell transplantation. According to the ELN recommendations, it is reserved for patients in whom three lines of TKI failed.

Advocacy Session #4: "World CML Day" – Training and Best Practices

Rotating sessions:

- **Training Session: Social Media use for World CML Day** (Sofia Sá Cardoso)
- **Training Session: Posters and event planning** (Jelena Čugurović)
- **Best practice examples on the use of the World CML Day Kit in 2015** (Erin Lindsay Schneider)

Jelena Čugurović (Serbia) introduced the World CML Day toolkit with the key message "Today, Together" to the audience. The toolkit includes sub-messages and actions for different audiences, artwork for posters and flyers, for print and online use, and guidelines how to use these tools. Practical tips and specific instructions on event planning are also provided. The purpose of these tools is to support CML patient organizations in raising awareness and make people talk and care about CML. Organizations can adapt the tools according to their local needs, markets and resources. They can also use them again in 2017 and thereafter.

CML patient organizations should select one of the following four target groups and address them with the corresponding simple but clear message: "Today we live, together we fight" for the general public, "Today we talk, together we live" for patients and relatives, "Today we listen, together we help" for health care professionals, or "Today we ask, together we care" for policy makers and governments. Jelena stressed the importance of selecting the organizing team carefully. It is crucial to define roles and responsibilities, and that everyone in the team works according to the same timetable and deadlines.

Sofia Sá Cardoso (Portugal) then presented the World CML Day toolkit focusing on Social Media. The toolkit provides steps how to organize a World CML Day event and suggestions how to maximize social media presence during and leading up to World CML Day. Social media increase the visibility and impact of communication, help build long term relationships and allow interaction with the audience. When choosing social media channels, four aspects must be kept in mind: audience, goals, timings, and resources.

Sofia advised using Facebook and Twitter to communicate with the general public. Both reach a broad audience and are good for engaging with users. Facebook is particularly suitable for campaigning. Sofia described step by step how to set up and run a Facebook campaign for World CML Day. Many practical examples illustrated how such a campaign can be made attractive and popular. Twitter is good for engaging with influential individuals, especially media and policy makers, and for monitoring and joining conversations around CML. Twitter allows direct communication in real time.

Expanding on the previous two presentations, Erin Lindsay Schneider, Bahija Gouimi and Param Puthen shared best practice examples in utilizing the CML Toolkits for World CML Day 2015.

Erin Lindsay Schneider (USA) showed that advocacy groups in Spain and Russia had identified patients and families as their target groups whereas in Colombia, patients and families as well as policy makers and government were chosen. Advocacy groups not only adapted posters and flyers from the toolkit to local needs and conducted a social media campaign, but also implemented new ideas. Spain and Colombia offered an educational seminar for CML patients while Russia organized a concert event.

In Morocco, patient advocates used the event planning tools from the toolkit to organize a patient summit, a radio broadcast, a press conference and a "Dance for Life" video, showing that patients can have a normal life with cancer. **Bahija Gouimi** added that social media were also used to increase communication.

Param Puthen reported that the toolkit had been very easy to use. It had helped open doors in hospitals in India and enabled outreach to healthcare professionals, patients and caregivers in their local languages and across the country. Well organized events and promotions led to increased awareness about CML. Patients showed their solidarity by coming “Today, Together” to unite and speak together in one voice.

DAY 3: Sunday 8 May

Medical Session #4: Diagnostics and Monitoring

Chairs: Ferdinand Mwangura, Jelena Cugurovic

- **Importance of monitoring response** (Steve O'Brien)
- **State of the art testing in high-resource countries** (Gianantonio Rosti)
- **Testing and monitoring in low-resource countries** (Pat Garcia-Gonzalez)

Day 3 started with a focus on the importance of monitoring response. **Prof. O'Brien** talked about why, how and when to monitor.

The main reasons for monitoring CML are to optimize treatment and to detect side effects.

Methods of CML monitoring include blood count, bone marrow cytogenetics, blood PCR and potentially bone marrow PCR as well as mutation testing. Bone marrow examinations seem to be going out of fashion and will hopefully be replaced by newer technologies which are more comfortable for patients. **Prof. O'Brien** explained that cytogenetic testing and PCR testing are totally different technologies. Nevertheless, both tests produce results which are indicative of survival. The achievement of a complete cytogenetic response (CCyR) as a landmark of survival corresponds roughly to a PCR result of about 1-2% BCR-ABL/ABL ratio. The speaker then described the difference between qualitative and quantitative PCR, explained key terminology related to PCR and presented some pictures of older and modern PCR machines. Modern PCR technology is not accessible in all parts of the world. Prof. O' Brien showed an amazing example of how he managed to overcome this problem with fairly simple means in Malawi.

Mutation testing is a type of monitoring which is used in cases where patients do not respond well. Based on the results of mutation tests in test tubes, guidelines have been developed on treatment options for patients with mutations. Caution should be exercised as these guidelines are not based on the results of clinical trials.

Monitoring is recommended at specific intervals: at diagnosis, monthly for 3 months or possibly 6 months if the disease has been stable for years. Patients who stop treatment need more frequent monitoring.

Prof. Rosti started his presentation by re-emphasizing the importance of regular monitoring at the recommended intervals. Recommendations for molecular monitoring have been issued by the ELN and the National Comprehensive Cancer Network (NCCN) and should be followed strictly. The SIMPLICITY trial conducted in Europe and the USA revealed however that the frequency and timing of monitoring in real life did not comply with the published guidelines. Monitoring rates were low particularly at 3 months, suggesting that many physicians were not aware of how to monitor or manage CML properly. Generally speaking, poor monitoring is linked to poor outcome.

The type of monitoring depends on the level of sensitivity required to determine the treatment endpoint. Obviously, assessing major molecular response requires a different technology than determining complete cytogenetic response. Results and their implications for clinical decisions can change significantly when International Scale (IS) conversion factors are applied. Conversion factors allow laboratories to compare a patient's results over time and permit comparisons between other laboratories with validated conversion factors.

Prof. Rosti demonstrated how a network of laboratories with three national reference laboratories was set up in Italy in an effort to standardize molecular test results in CML. Any physician in Italy can send in samples and will receive centrally harmonized results. Efforts are continuing to increase the level of sensitivity that can be measured in the laboratories.

Pat Garcia-Gonzalez looked at access to molecular diagnostics in low and middle-income countries. Pat talked about the progress that has been made, discussed current challenges and gave an outlook into the future.

The main priorities of the global CML community – namely safely stopping treatment, ensuring safety when switching to generic imatinib and becoming involved in the development of clinical trials and beyond – do not apply to patients in poorer parts of the world. There, many patients do not have access to modern diagnostics or monitoring. This is why a lot of effort has been made in recent years to improve the situation in these countries.

Looking back in time, imatinib has been available in 80 low and middle-income countries since 2002. Nevertheless, even years later some 50 countries did not have access to molecular monitoring, including all countries in Sub-Saharan Africa and many countries in Asia and Central America. Patients were forced to ship their blood samples to other countries for diagnosis at a cost of USD 600. The only monitoring possible locally was via blood counts.

At the end of 2009 the Max Foundation was made aware of GeneXpert machines. These modern machines use quantitative PCR to produce accurate BCR-ABL test results, typically within a couple of hours. The technology is easy to use and allows sensitive testing that is standardized to International Scale. The cost per test was reduced to USD 50. The technology was originally developed to test for anthrax but can be used to diagnose many other diseases, including CML. More than 10,000 GeneXpert machines have been placed in countries all over the world, mainly to test for tuberculosis. Pat advised that advocates who still do not have access to monitoring in their country should find out whether any GeneXpert machines are available locally and then ask if they might be also used to test for BCR-ABL. An agreement has been reached with the Cepheid to supply the machines to 68 countries at a cost of USD 18,000 instead of USD 80,000. Since 2012, the Max Foundation has brought molecular diagnostics to 20 countries.

Pat then weighed up pros and cons of currently available monitoring solutions in low-resource settings. She also shared insights into recent advances in developing alternatives for BCR-ABL monitoring. These include inexpensive DNA-based tests on dry blood on paper that can be shipped from anywhere in the world as well a first PCR tests that can be done at home.

Some of the projects of the Max Foundation in the very near future include preparing a PCR advocacy toolkit and finding funding to scale up the dry blood sample shipping. Pat mentioned and showed a picture of a very compact, personal-friendly version of the GenExpert which could be available soon.

At the end of her presentation Pat advocated radical innovation to ensure that people all over the world have access to good quality PCR. This can be reached by skipping less efficient, more expensive solutions and move directly to more advanced ones.

Advocacy Session #5: Psychosocial issues

Chairs: Jana Pelouchova, Rita Christiansen

- **How to train advocates to handle newly diagnosed patients and complex situations** (Guy Tavori, Cristián Neves)
- **Caring for the caregivers** (Cristián Neves)
- **Cancer at the workplace** (Ward Rommel)

Speaking from his own experience as a leukemia patient and a social worker in a hematology department, **Guy Tavori** (Israel) addressed the importance of dealing with and managing the psycho-social aspects of cancer. To better understand what cancer patients are going through, Guy

introduced the Kübler-Ross grief cycle. This model represents the five phases that cancer patients typically experience: denial, anger, depression, bargaining and acceptance of the disease.

Cristián Neves (Chile) then addressed the social support usually provided by friends and family, advocates or the community. There are different types of social support: emotional, instrumental, informational and appraisal. To get better outcomes, social support should be given in a strategic fashion. Cristián described 11 communication strategies and techniques that can be used in complex situations.

To put theory into practice, two teams of volunteers acted in role plays as a newly diagnosed CML patient and a patient advocate. The audience was asked to identify the emotional stage of each of the two patients and the type of social support they needed.

Guy concluded that a patient-centered, non-judgmental attitude of an advocate should always comprise certain elements. They include empathy, open questions, listening, providing information and resources as well as an open door policy.

In his second presentation, **Cristián Neves** focused on caregivers. He explored the meaning of caregiving and defined the term in the context of CML as “walking along with somebody who has CML”. Caregiving needs change as patients go through the different phases of the disease. Patients’ needs for support are also shaped by the personality of the patient. Caregivers can better identify needs if they are aware of how patients are coping with their diagnosis. Patients reacting emotionally for example need to be addressed differently than patients who react in a more rational manner.

Cristián named typical issues that CML patient caregivers may be confronted with and made suggestions how to handle such challenges. Caregivers who do not manage to cope with such difficulties frequently develop health problems. They should be aware that they need to take care of themselves. It is crucial that they learn to say NO and ask for help when they need it.

Ward Rommel (Belgium) looked at psychosocial challenges related to chronic cancer at the workplace and showed ways forward.

Half of the patients diagnosed with cancer in the EU are of working age. Work is important for their quality of life for personal, social or financial reasons. However, many factors can be hurdles for sustaining work or returning to work. These factors range from type of diagnosis and side effects of treatment to lack of support from managers or heavy physical demands. A recent study revealed that patients with CML have a 69% chance to return to work. Nevertheless, their risk of permanently reduced work capacity is also high. In general, people with chronic diseases in the EU have a higher risk of unemployment and fewer job opportunities. A study by the Organization for Economic Co-operation and Development (OECD) investigated disability policies in its member states from 1990 to 2007. It showed a moderate shift of focus towards stricter rules on providing compensation and more importance on integration.

Ward named several examples of equality legislation, policies and initiatives to ensure and promote integration in the EU or OECD countries, specifically the EU “Employment Directive”. Most of these documents mention disability as a ground for discrimination. However, most of them do not define disability or refer to people with chronic diseases. The Equality Act in the UK, in contrast, is an inspiring example of equality legislation. It explicitly covers cancer as a disability.

Governments in many countries have started initiatives to improve integration at the workplace. Nevertheless, employers are reluctant to recruit people with a chronic disease. Therefore, a good return-to-work policy is only feasible if advice and financial support in the event of productivity loss is available to employers. Ward showed examples of successful return-to-work activities undertaken by cancer organizations, addressing both employers and employees.

The speaker suggested that patient organizations should advocate for policies to keep people with chronic diseases at work and allow them to work in a way that takes their disabilities into account.

Best poster, closing session and farewell

At the end of the conference, **Jan Geissler** expressed that CML Horizons is one of the highlights of his advocacy work. Jan summarized the key takeaways of CML Horizons 2016:

- The World CML Day Toolkit created in 2015 has been taken to the next level. Advocates have been trained on how to utilize the tools and can now implement this knowledge in their organizations.
- The CML trials database and the patient-friendly summary of the ELN treatment recommendations are lighthouse projects. They started small but have enormous potential to be developed further. Patient communities can benefit from them on a local level.
- The CML Community Advisory Board has been set up to interact more effectively with stakeholders. It is a pilot project and will hopefully demonstrate that patient input is essential.

Before closing the conference, **Giora Sharf** awarded the diploma for the best poster to Web-forum CML-Stop from Russia.

Giora thanked the Steering Committee for their hard work in organizing the conference, the sponsors for their collaboration and support that made CML Horizons 2016 possible, and the Liberty team for managing the logistics of the meeting. He thanked all delegates for coming and whished them a safe return home.

Sponsorship Acknowledgement

We would like to thank the following organizations for providing unconditional educational funding. Without their support, this conference would not have been possible:

- Novartis Oncology (Initiating Platinum Sponsor)
- Bristol-Myers Squibb (Gold Sponsor)
- Pfizer Oncology (Gold Sponsor)
- Ariad (Gold Sponsor)
- The Leukemia & Lymphoma Society (Bronze Sponsor)
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For Additional Information

- CML Advocates Network website: http://www.cmladvocates.net/
- Web streams and PDF presentations: http://www.cmladvocates.net/cmlhorizons

About CML Horizons 2016 and this report

The program has been governed by a global steering committee consisting of CML patient advocates from North America, Latin America, Asia, Europe, Middle East and Africa. The CML Steering Committee 2016-2017 is:

- Gail Sperling (USA, elected representative region North America)
- Bahija Gouimi (Morocco, elected representative region Africa & Middle East)
- Rita O. Christensen (Denmark, elected representative region Western Europe)
- Jelena Čugurović (Serbia, elected representative region Central and Eastern Europe & West Asia)
- Giora Sharf (Israel, co-founder & permanent member of the Steering Committee)
- Jan Geissler (Germany, co-founder & permanent member of the Steering Committee)
- Jana Pelouchová (Czech Republic, co-founder & permanent member of the Steering Committee)
- Pat Garcia-Gonzalez (USA, elected representative region Latin America)
- Rod Padua (Philippines, elected representative region Asia-Pacific)

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Published by: Leukemia Patient Advocates Foundation
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