European Partnership for Action Against Cancer (EPAAC)

D4.1: European Standards of Care for Children with Cancer

January 2014
DAY 1 (Thursday 20 October 2011)

1. **Welcome**

   Professor Jerzy Kowalczyk (PL) welcomed the audience and gave a small introduction and history about the creation of the Standards of Care. He also introduced and thanked Dr Ewa Kopacz, the Polish Minister of Health, for participating in this important event as well as Mrs Sidonia Jędrzejewska (Polish MEP) who sent a video message to stress out the importance of this conference and standardising high-level care of children with cancer in all European countries.

2. **Introduction**

   ✷ Dr Ewa Kopacz (PL), Polish Minister of Health, welcomed the attendees and highlighted the significance of this event within the framework of the EU Polish Presidency; addressing inequalities in Europe and the improving child health were key priorities for the Polish government; thus the Standards of Care conference and Poland’s commitment to it was timely.

   ✷ Dr Josep Borras (ES), Work package Leader for ‘Healthcare’ in the European Partnership for Action Against Cancer project provided a brief overview about EPAAC (European Partnership for Action Against Cancer). In summary this projects aims:

   1. To identify and assess *best cancer care practices* across European health services, promoting the exchange of experiences focusing on innovative organisational approaches, including the all-important patient’s perspective
   2. To develop, review and harmonise *Clinical Guidelines* (CG)
   3. To implement a training strategy to improve *psychosocial care* and *communication skills* among health care providers

   The concept of the added-value of collaborating at a European level is at the heart of EPAAC and links well to the standards of care initiative. EPAAC and the paediatric cancer ‘Standards of Care’ project are clearly linked and it is positive for both projects that the Standards is incorporated into the EPAAC project. Several points must be considered however when forming such ‘standards’:

   ✓ A definition of standards requires a comprehensive discussion about the key components of cancer care, between all bodies involved, including scientific societies and patients.
Identify the gaps regarding best practice.
An assessment of the changes introduced after dissemination of the standards needs to be made.
Creating standards is a learning process and standards should never be ‘set in stone’ but rather are constantly changing.

Professor Ruth Ladenstein (AT), SIOPE President and ENCCA Project Coordinator provided an overview of the current challenges that the Paediatric Oncology community is facing. She focused on the major challenges and limitations for the paediatric oncology community, such as:
- Unequal access to standard treatments and care
- The struggle to run investigator-driven clinical trials within the 2001 Clinical Trials Directive
  - Definition of investigational medicinal products (IMP)
  - Currently no risk-based adjustment for paediatric academic trials
  - Insurance needs and rates

Professor Martin Schrappe (Germany), I-BFM-Chairman & SIOPE Board Member illustrated via the leukaemia I-BFM experience how to ensure the best possible care and outcome for children and young people with Cancer in Europe. The I-BFM network is international but primarily based in Europe and it sets out to provide the basis on trial design. It is not only focused on paediatric oncology only but they participate as well in joint programmes with adult oncology. In addition, the ratio of doctors versus nurses, the access to specialists, the number of beds and reimbursement systems certainly was an essential element of the high-level of treatment and thus improved outcomes provided to young leukaemia patients. Therefore the ‘standards of care’ initiative is vital to ensure high-level and equal care across Europe.

In conclusion the I-BFM experience can be summarised as follows:
- Population-based clinical trials in childhood leukaemia have provided the best available treatment as demonstrated through the outstanding success in survival of patients treated in this setting
- Clinical trials are designed as scientific instruments but more importantly, they provide quality-assured clinical care.
- Improvement in outcome may be related not only to quality of protocol but also to better (supportive) care.

Professor Janez Jazbec (SL) a member of the European Medicines Agency (EMA) Paediatric Committee, presented an overview of the development of paediatric oncology drugs and raised the challenges that are faced in this area and how this needs to be urgently addressed if we are truly seeking to provide the best possible care to young patients in Europe.
- Over 80% of drugs to treat children with cancer are “off-label”.
- Less than 15% of drugs approved for adults with cancer concerns paediatric oncology.
Less than 50% of drugs commonly used for adults with cancer concern paediatric oncology

In summary, the paediatric oncology community in Europe are facing numerous challenges in providing drug treatment to children and adolescents:

- A standard list of drugs is being daily prescribed off-patent and off-label to young cancer patients.
- The off-patent list and priority list are not connected:
  - The off-patent list, even though standard treatment and clearly improving outcome, is now able to come under the standard funding application procedure
  - The priority list is only for new developments
- The main focus is currently on substances and diseases and not on:
  - Targets
  - Pathways
  - Mechanism of action
- There needs to urgently be a strategy to develop and facilitate the development of new drugs
- Strengthening the process currently in place is a possibility (currently the PDCO at EMA understands the issues and is seeking ways to deal with these urgent problems but are over-burdened)
- Speeding up the process from approval to trials is essential
- All the regulatory and administrative burdens placed upon the paediatric oncology community as a result of the EU Clinical Trial Directive needs to be addressed.

- **Professor Jerzy Kowalczyk (PL)** presented an overview of how the European Standards of Care for Children with Cancer project commenced, including incorporating the creation of the link with EPAAC. He furthermore provided an update on the translations currently available: now translated into 6 different languages (English, Polish, Spanish, Serbian, and Portuguese & Greek), more translations are in process and expected to be published in due course. Kowalczyk also presented the next steps that should be taken to ensure this initiative moves to the next level and all the commitment, team work and efforts are not lost. Future actions to be considered include
  - Convince regulatory authorities both at EU and national level to issue national regulations on standards in paediatric oncology
  - Monitor the process of implementation of recommendations in paediatric oncology centres in each country
  - Identify a registry of centres that follow recommendations (potential partners in clinical trials)
  - Prepare the national versions of European Standards, taking into account variations in culture at country-level.

- **Mrs Anita Kienesberger (AT)**, ICCCPO Board Member and ECCO Patient Advisory Committee Member, shared the experience of ICCCPO working together with parents and survivors. ICCCPO is the only non-medical childhood cancer organisation representing families of children with cancer world-wide. ICCCPO connects parents, survivors, childhood cancer agencies and other supporting
organisations and aims to be recognised world-wide as the voice that represents families of children with cancer. By sharing information and experience, it is hoped that access to the best possible treatment and care for children with cancer everywhere in the world can be achieved.

In Europe there is no separate European branch of ICCCPO; however specific goals have been set for Europe as there are some characteristics unique to the European parent/patient experience, such as:

- Tackle EU policy issues
- Encourage close collaboration with SIOPE (which is already ongoing)
- Improve regional cooperation between parent groups all over Europe
- Motivate parent organisations to develop activities solely focussed on fundraising and instead work more deeply on advocacy issues

Kienesberger also presented the new initiative: “The International Childhood Cancer Survivors Network” which was created as a result of the increasing number of survivors. Survivors naturally have a different point-of-view to patients and need to be heard. Their voice, power and influence is very important on all levels, and in particular in their communication of political “health” issues at national, EU and international level. Long-term follow up is a key topic when discussing survivors and initiatives such as the Survivorship passport, which is an electronic record of treatment received and is being developed in the ENCCA (European Network for Cancer research in Children and Adolescents° project, is important and ICCCPO is heavily involved in its creation, development and eventual dissemination.

Movie: “The last thing to come back was his smile”, a video from Jimmyteens.tv, which is a project that aims to support young patients to talk about their disease and is targeted in particular at adolescents affected by cancer. A service provided to patients in the UK, the aim is also to educate people about the effects of cancer, not just the medical impact, but also the emotional impact patients and their friends, family and medical staff, experience before during and after treatment.
3. **Panel Discussion “National Perspective on Standards and Care for Children with Cancer”**

**Professor Riccardo Riccardi (IT)** opened this second session by highlighting the fact that there is 20% difference in outcomes between central and Eastern European countries. There also needs to be considerably more attention and awareness-building for patients with late effects. Professor Riccardi illustrated that these issues are to be covered in the ENCCA project.

**Professor Thomas Klingebiel (Germany)**, Chair of the German Society for Paediatric Oncology and Haematology (GPOH), presented an overview of the structure and activities of GPOH. Created 30 years ago as a scientific project which facilitated the structuring of paediatric oncology in Germany, the GPOH currently has approx. 1000 Members (multidisciplinary including physicians, nurses and psychosocial specialists). The primary activities of the GPOH include:

- Organising/facilitating basic research and clinical studies
- Registration of all cancer patients <18 yrs (National Registry with ~ 100% recruitment)
- Close collaboration with study groups

Professor Klingebiel elaborated on the German “Vereinbarung”, an agreement of the (German) Common Federal Commission on measurements to secure the quality for the hospital care for children and adolescents with haematopoietic diseases which sets down the legislation on the daily work of the Paediatric Oncology community. The objective is to secure structure, process and outcome quality of paediatric haematopoietic oncology and to guarantee high-quality care for all children independent from their socio-economical situation and residence. In order to achieve these objectives each centre requires a multi-professional certified board consisting of physicians, nurses and other necessary professionals. This legislation was considered to be a very good example of ‘standards’ written into national law.

In conclusion, it is clear that the paediatric oncology community is facing a lot of challenges due to the current EU Clinical Trials Directive. Therefore the work of SIOPE & ENCCA is vital in this way. Strong support of parent and patient organisations is also required when addressing the national politicians about the burden of “over” regulation. Moreover, the ‘standards of care’ project is necessary and very helpful and should be well-funded and well-delivered in order to make a positive impact.

**Professor Alexander Karatchunskiy (RU)** from Research Institute of Paediatric Haematology in Moscow, presented the treatment data of childhood ALL in Russia and explained that he gained a lot of knowledge from international groups particularly Germany. This information-exchange and support on ‘lessons learned’ ensured he could achieve concrete success, while also modifying in order to fit to the national cultural context of Russia. An important facet of this initiative was to incorporate regular visitations to the participating hospitals to monitor the quality of treatment and documentation and provide support to the clinics by training in staff on the relevant
protocols and supportive care, a major challenge since the standards of many centres in Russia are quite heterogeneous.

Dr Armando Pinto (PT), Oporto IPO’s Paediatric Department, representing the Portuguese Ministry of Health, provided an overview of the national perspectives of Portugal. With 10 million inhabitants, of which 2 million are less than 18 years of age, Portugal faces 300 new cases of paediatric cancers annually.

Portugal has 4 Paediatric Cancer Units (<18 years) with paediatric subspecialties. Accommodation for parents and family members is provided and the Portuguese paediatric oncology community participates in research development programmes with international collaborators such as EORTC and SIOPEN. However Portugal still faces several challenges:

- No National Paediatric Cancer Plan
- No National Paediatric Oncology Network
- No National Childhood Cancer Registry
- Same protocols for the same diseases are required
- There needs to be a better link with our Primary Health Care System

Pinto stressed the importance of the standards of care and the need for a “European plan for children with cancer” as well as the promotion of further collaboration on EU research and research projects and the Portuguese Paediatric Oncology Community.

Mrs Alina Maria Mitroi (RO), P.A.V.E.L., presented the Romanian parent association; which is a member of ICCCPO, UICC, ECPC, EUROCHILD, IAPO, HOPE and Associate member of SIOPE. The association runs a variety of programmes in order to help young patients re-integrate into their social, educational and professional life after their complex and challenging experience with cancer.

Given the experience of more than 15 years in this field, Mitroi pointed out the numerous weaknesses of the Romanian health care system such as:

- Late diagnosis
- No national cancer registry
- Poor monitoring after hospital leave
- Lack of working standards for multidisciplinary teams
- Poor drug access
- No educational plan for young patients and no professional integration plan for professionals

Although there are several areas to be addressed in Romania, PAVEL has stepped in to help parents and patients. In the Oncology Institute of Bucharest for example, PAVEL has hired social assistants, psychologists and play therapists for young patients as amongst other social services. On a yearly basis, approximately 170 families benefit from the provided social services such as food, transport, drugs, medical investigations and medical equipment. Moreover, in ’1998 PAVEL created the patient-parent house in Bucharest that has hosted more than 570 beneficiaries (children, parents, family and others) since its creation.
PAVEL realises that there is still a long way to go in Romania to meet the European standard of care: however one of the future projects that PAVEL will be focusing on is the dissemination of the (PAVEL) association experience as well as a programme on best practice to other hospitals/organisations and expand expert teams in order to obtain better monitoring of patients and eventual better outcome.

Professor Iskra Russeva Cristozova (BG) presented the Bulgarian current situation with nearly 150 new cases per year. Currently there are 3 specialised centres where children with cancer can be treated: 1 in Sofia, 1 in Varna and 1 in Plovdiv as well 1 boarding house containing 18 beds in 6 double rooms to accommodate the parents and family of the young patients.

**Conclusion**
Professor Ruth Ladenstein expressed at the end of this session the need and importance of education amongst the different EU countries. Even EU Member States are facing significant disparities in the services available and the level of services provided to children with cancer. It is essential that high-level, experienced organisations and networks in paediatric oncology share their experience and expertise: therefore more twinning/partnering) programmes and knowledge-sharing amongst the different countries should be encouraged, such as the successful partnership between Germany and Russia for ALL patients, and this sort of scheme can benefit both parties (for example more patients in a clinical trial versus the sharing of knowledge. It is that the High-level experienced groups link with other European countries to share expertise.
DAY 2 (Friday 21 October 2011)

1. **Panel Discussion “Inequalities in Accessibility to Optima Treatment and Care for Childhood Cancer Patients in Europe”**

   - **Professor Martin Schrappe (DE)**, I-BFM-Chairman and SIOPE Board Member opened the second day of the conference.

   - **Dr Marjetka Jelenc (SL)** from the Institute of Public Health in Slovenia, illustrated how the European Partnership for action against cancer (EPAAC), aims to reduce inequalities across Europe. There is comprehensive evidence that significant EU health variability exists in both the delivery of services and the outcomes of care. Therefore EPAAC has as a general objective to contribute to the reduction of cancer burden in EU by actions in several areas, such as health promotion, screening and early diagnosis, cancer-related health care, coordination of cancer research and cancer information and data.

   In the health care area, which incorporates the European Standards of Care for Children with Cancer project, EPAAC aims to promote the exchange of experience and best practices in health services across Europe, such as standards of care for children as well the development, review, and harmonisation of clinical guidelines (on rare cancers and nutritional care guidelines for example) and the implementation of a training strategy to improve psychosocial and communication skills among health care providers.

   In addition to this, EPAAC also involves national governments (EU Member States, Norway and Iceland) who share experiences on National Cancer Plans. Work on this area and contributions from the national ministries have to date been very successful. It is clear that there is huge potential for partners to learn from each other, both for those Member States who do not already have plans and for those that do to update them.

   - **Professor Walentyna Balwierz (PL)**, is a paediatric oncologist based in Poland and provided a testimony on her experience of attempting to provide non-commercial clinical trials to children with cancer a standard of care.

   Balwierz gave a brief overview on the history of conducting clinical trials in Poland. Until 2006, only an approval from the Ethics Committee was required in order to conduct a clinical trial in Poland. However, since 2006 several additional requirements have been imposed by the EU Clinical Trial Directive to conduct trials in European countries. As a result, since 2006 the EuroNet-PHL-C1 protocol is the only non-commercial trial which has been registered in Poland and the reason for the few registrations is because of the administrative burden linked to the sponsor of the trial and the contract related to the national sponsor of the study and the involved clinical trial study centres. For this study, only 14 of the 17 centres in Poland confirmed they would participate; however the three remaining centres refused because of the
considerable responsibility and legal and financial implications linked to the contract. In the meantime the situation in Poland got more complicated because of a new pharmaceutical regulation, introduced at national level in April 2011 that also had an impact on the clinical trials regulations. As a result, the Polish paediatric community presented a petition on this matter to the Polish Ministry of Health.

Prof. Balwierz underlined that policymakers need to realise the importance of this major issue affecting children with cancer.

>> Professor Ruth Ladenstein commented that we need to “teach our politicians” on what a study is. Communication to policymakers is key and being able to speak the same language and explain to them the seriousness of the situation and the concern that it is raising amongst the paediatric community in Poland needs to be done in partnership with patients and their families. It was also pointed out that, as a country with a large population in comparison to many of its European neighbours, Poland’s active participation in trials truly compliments multinational trials due to the increase in the number of trials. Due to the over-regulation, multinational trials are also being negatively affected; i.e. policymakers need to be informed when that it comes to paediatric oncology trials, the consequences of not participating does not only affect Polish patients and their ability to receive the best available treatment but in fact also affects patients in other countries.

Dr Gerlind Bode (DE), member of the ICCCPO Board and the European CanCer Organisation (ECCO) Patient Advisory Committee, explained the importance of the Standards of care initiative from a patient/parent perspective. ICCCPO represents 59 parent groups from 29 different countries in Europe. Experience has demonstrated that the quality of medical, supportive and psychosocial care of children with cancer often depends on the situation in the individual hospital and/or the economic, social and political situation of the country. However, each child has the right to access the best possible treatment within Europe and partnership between SIOPE and ICCCPO on the Standards of Care project is important.

This initiative if successful, help to combat the major inequalities in terms of access and care standard at national, regional and institutional levels and in addition combat the current treatment tourism, where parents are desperate to attain the best and most appropriate treatment despite the ordeal of travelling as well as the expense.

>> Parent groups can play a major role in attempting to turn around the current inequities by lobbying

✓ At local level with hospital authorities
✓ At national government level
✓ At European level

✓ Mrs Elzbieta Pomaska (PL) from the Communication without Barriers Foundation is a parent of a child survivor of cancer. She shared the outcome of a questionnaire on the achievements and challenges of parent
organisations in Poland. Poland has 9 parent organisations, all of whom completed the questionnaire:

- All organisations share the same mission and have similar objectives
- They are not informed about the activities of other similar organisations in Poland
- They have no access to information regarding sources and method to obtain financial support
- They have no ability to develop effective programmes to promote their organisation, setup business plans and organisational and administrative models
- They have no little or no support from local authorities/governments
- They have no information or access to materials issued by other organisations

In summary it was concluded that while all organisations faced the same issues, collaboration is not currently taking place and there is a real need to do so. Forming a national network to support each other and work closely on common goals could ensure that there is a powerful voice
Professor Marzena Samardakiewicz (PL) of the SIOP Psychosocial Working Group Committee, illustrated the importance of psycho-social care for children with cancer and their families, a key element of the European Standards of Care for Children with Cancer project. Since the creation of IPOS, the International Psycho-Oncology Society in 1984, great progress has been made on promoting this issue. A particularly significant milestone took place in 2010 when “Distress” was recognised as the sixth vital sign.

Psychosocial issues in paediatric oncology is a particularly complicated process as there are so many factors involved such as the medical decision, treatment strategies, family and many others. Therefore it is important to:

- Train staff in basic psychological skills
- Provide support and supervision to staff
- Provide access to core mental health staff
- Integrate psychosocial staff into multi-disciplinary teams
- Use and develop screening tools (distress, QoL, adaptation)
- Educate and share multidisciplinary knowledge

Mrs Katie Rizvi (RO) founder and Vice-President of the organisation, ‘Little People’ explained that despite the challenges in Romania, her organisation was able to attract private funding for their activities. Significant progress has been made in Romania in relation to child and adolescent cancer:

- Since 2009 a national registry has been created
- Since 2011 paediatric oncology has became a subspecialty, a major achievement
- And currently there are discussions on how to standardise the approach of psychological interventions (such as the ‘Little People’ campaign of the ‘brave lion’, with a pre and post intervention test of which the results will be published in 2012).

>> An Open Letter has also been sent by the ‘Little People’ organisation to the new Minister of Health in Romania (who is in fact a paediatric haematologist), on the importance of the European Standards of Care initiative.

2. Panel Discussion “Recommendations on implementation of European Standards of Care for Children with Cancer”

During this last panel discussion experts provided their input/recommendations on how to implement the European Standards of Care for Children with Cancer.

- Polish Ministry of health was represented by Mrs Dagmara Korbasinska (PL)
- Spanish Ministry of health was represented by Dr Josep Borras (ES)
- The Pancare SurFup project was represented by Prof Lars Hjorth (SE)
- The ENCCA project was represented by Prof Ruth Ladenstein (AT)
- EUROCHIP project was represented by Prof Magdalena Bielska-Lasota (PL)
Mrs Dagmara Korbasinska (PL) of the Polish Ministry announced that the standards of care will be reviewed by a team of experts in order to make such standards legally binding; thus it will be obligatory for all clinics in Poland treating young patients with cancer to meet such standards of care. Moreover, as it is legally binding and will be enforced, patients and their families will be informed of their rights.

According to Korbasinska, the Polish Ministry of Health recognise the complexity when treating young people with this disease and the alarming pressure the recent regulatory issues have put on the paediatric oncology community, as well as patients. The message is clear that progress needs to be made on dealing with academic and commercial trials, as well as the off-label use issue. Currently the Polish Ministries of Health and Science plan to collaborate to tackle together these challenging issues which were discussed in detail at this conference.

Dr Josep Borras (ES) represented the Spanish Ministry of Health and outlined the characteristic decentralised nature of Spain and treating children with cancer. Currently Spain has 45 paediatric oncology units for a total population of 45 million inhabitants.

A new cancer strategy is being implemented in Spain and Borras considers that Standards of Care that have been developed can help as a guiding document working on paediatric oncology.

Spain has begun to improve the level of psycho-oncology integration as well as the teaching resources available. He also noted that Spanish patients with paediatric cancer have an average survival rate compared to the rest of Europe and thus there is room for improvement in order to meet the survival rate other European countries have achieved.

>> A Spanish paediatrician participating pointed out the need to recognise paediatric oncology as a subspecialty; She also asked for attention to be given to the definition of IMP (Investigational Medicinal Product) and a clinical trial as currently it is unclear and thus can cause significant bureaucratic issues.

Professor Lars Hjorth (SE), the Coordinator of the EU-funded Seventh Framework Programme (FP7) project, PanCare SurFup, provided an overview of this initiative, a sister project to ENCCA which will help in providing every EU childhood cancer survivor with better access to care and improved long-term health. The project stems from the PanCare initiative, a multidisciplinary pan-European network of professionals, survivors and their families from 26 different European countries.

Professor Hjorth stressed out again that childhood cancer is a rare disease and that there are significant differences in survival, follow-up and long-term effects throughout Europe. He emphasises the fact that standards of care is a very wide concept that ranges from basic science to early detection to long-term follow-up; which is a daunting task. Therefore the following points should be taken into account:
Communication between countries and projects/groups is of vital importance; the European Partnership for Action against Cancer (EPAAC) is a promising venture but a major challenge and dissemination of its activities is crucial, especially for those not directly involved in the project(s) but possessing similar goals (e.g. ENCCA and PanCareSurFup)

- EU and national rules and regulations need to be harmonised and simplified
- Academia, parents and financial support must continue to drive the development, but hopefully with greater assistance from society

Prof Magdalena Bielska-Lasota (Poland) from EUROCHIP initiative presented the activity conducted by the EUORCHIP project on the issue of costs in ALL. The EUORCHIP project has as objective to re-think cancer management on the level of reducing costs, while maintaining or improving cancer outcomes. The 'standards of care' project is a very important step as it has realised to encourage researchers clinicians and health policymakers and aims to encourage strategic changes. The standards pinpoint what areas to invest in to ensure children and adolescents can access the best possible treatment and care available.

**Conclusion and ‘take home message’**

Significant progress has been made since the last conference in 2009 which catapulted this project and partnership between paediatric oncologists, patients, parents, nurses, psychologists, counsellors, play therapists and physiotherapists. At the 1st meeting there were no policymakers and when looking to today’s event, at least 15 representatives of national ministries were represented, thanks to the kind invitation by the Polish Ministry of Health. There is still a long way to go however but improvements are being made, although slower than anticipated. As more translations of the standards are made, further lobbying activities at local and national level can take place.