Summary Report
International Childhood Cancer Awareness Day

20 February 2013,
European Parliament

To mark International Childhood Cancer Day, SIOPE organises an awareness-raising event at the European Parliament each February.

This year's event was timely , as currently Members of the European Parliament and national ministries are discussing the proposal by the European Commission to reverse the huge regulatory burden posed by the former EU Clinical Trials Directive (2001/20/EC) through a Clinical Trials Regulation.
The event, entitled **"The EU Clinical Trials Regulation: Improving research for children and adolescents in Europe?**" was hosted by Glenis Willmott, Member of the European Parliament (MEP) and Rapporteur of the European Commission proposal. A forward-looking and lively discussion took place, and participants included EU and national decision-makers and key experts from several paediatric disease areas (including members of the European Network for Paediatric Research at the European Medicines Agency (Enpr-EMA)).

MEP Glenis Willmott and Prof. Gilles Vassal, President of SIOPE, chaired the opening session and introduced the event. Clinical trials (CTs) for children and adolescents in Europe are mainly sponsored and conducted by academic institutions, and funded by public money and/or charity support. Their objective is treatment optimisation - using well-established drugs - and the introduction of new, safe and effective innovative medicine in standard care.
On this occasion, Prof. Vassal suggested a pragmatic approach to trial governance and highlighted the need to increase the cure rate and to improve the survivors' quality-of-life by integrating research into high-quality care. Concerning the new Regulation, SIOPE is currently asking for a fair and proportionate regulation of childhood cancer trials and for an insurance scheme for academic CTs secured through a 'national indemnity schemes' (you can read here the full list of amendments to the CTR and the position paper of SIOPE).

Prof. Helms, Chair ofEnprEMA made clear the need to revise the CTR in order to increase the availability of medicinal products authorised for use in the paediatric population. He suggested to consider the scope of off-label therapy and to have more precise and risk-adapted rules, in consultation with investigators, parents and patients. Finally, he underlined the importance of supporting paediatric research generally and also noted the lack of consideration of the paediatric population in the text of the legislation and the need to include the contribution of parents and children in clinical trials.

Providing her personal experience Ms. Patricia Blanc, parent representative and founder of the charity '*Imagine for Margo*', explained with a clear message the importance of developing new and innovative treatments for children with cancer. She explained that the only way to improve the current situation and accelerate research is to encourage regulations that can reduce red tape while ensuring safety for patients. She showed some key figures:

1 child in 440 has cancer before the age of 15 The numbers are increasing by +1.5 % each year 15,000 children and young adults are diagnosed each year in Europe 3 700 will lose their battle 10 kids a day are dying of cancer in Europe

One of the key policymakers responsible for the Clinical Trials Regulation dossier Stefan Führing from the European Commission's DG Health and Consumers, explained some key amendments to the original Directive, that can help academics carry out trials. He emphasised the Commission's commitment to supporting investigator-driven trials and suggested that any amendments made by MEPs, including those related to reporting, should take into account the limited budgets of academics already overburdened with administrative responsibilities.

Dr. Ingrid Klingmann from the European Forum for Good Clinical Practice (EFGCP) and Kathy Oliver, patient advocate and Co-Director of the International Brain Tumour Alliance, chaired the two sessions of the event. The first session focused on the **low-intervention categorisation of clinical trials.**

Prof. Pamela Kearns clearly defined what a "low-intervention clinical trial" is: a trial where all drugs are licensed and used according to standard practice, and where no more than the minimal risk is posed to the safety of the subjects. The majority of paediatric CTs comply with this definition, and should be categorised as such, and the CTR applies a shorter timeline for low-intervention CTs authorisation, with reduced labelling requirement for investigational medicinal products, proportionate safety reporting and no insurance requirement. More clarity is nevertheless required, as there is still space for an ambiguous interpretation of what a low-intervention trial is.

Prof. Kris de Boeck presented the case of cystic fibrosis (CF), a rare paediatric disease which implies a high treatment burden on patients. In the case of this disease low intervention 'comparative effectiveness trials' (to identify the best drug combination) and trials in children screened at birth for CF are needed. However the complex labelling of investigational products and safety reporting of the Regulation impedes these trials to smoothly start.

Among the other issues related to enhancing paediatric research in Europe, the topic of **national indemnity schemes** is also very important.

Prof. Ruth Ladenstein, ENCCA Project Coordinator, clearly exposed the current situation: the 2001 Directive (CTD) introduced an obligatory insurance for every CT in Europe, which substantially increased the costs and the administrative burden of conducting a CT:

Consequences of the EU Clinical Trials Directive (2001/20/EC): 25% decrease of CT applications 90% increase in the delay for launching a CT

However CTs do not always pose an additional risk to subjects compared to treatment in normal clinical practice, and a specific damage compensation for low-interventional CTs should not be needed. Current

insurance costs facing academics are extremely high and differ hugely between Member States: in rare diseases multinational trials are common and thus a disproportionate burden is placed on those conducting trials that the pharmaceutical industry has no interest in.
The CTR proposal obliges EU Member States to set up a national indemnification mechanism (NIM) for compensating damage. She welcomed therefore the NIM scheme proposal, as these mechanisms will serve the patient rights, have a potential of harmonisation across Europe and encourage fairer costs and secure future academic clinical research.

Dr. Nicola Ruperto from PRINTO illustrated the situation of research in paediatric rheumatic diseases, highly debilitating and very common in children. CTs are also not-for-profit in this disease area and some drugs (although their efficacy and safety is proven) are not approved because within the mainstream treatment they are used off-patent. Therefore, Prof. Ruperto called for a stronger recognition of the role of academia research and an insurance waiver on standard treatment studies.

At the end of the meeting, all participants were greatly impressed by the words of Anneke Gommers, a 26-old girl suffering from cystic fibrosis, who presented her personal story Her enthusiasm in participating in clinical trials in order to improve the treatment regime, the health conditions and the quality-of-life of other patients reminded everyone why all efforts need to be made to enhance optimum paediatric research.

Building on this very engaging and insightful meeting, SIOPE will continue in its concerted efforts to ensure the needs of the European paediatric oncology community are heard.
All presentations and **pictures** from this event, as well as SIOPE's **position paper** and proposed **amendments** to the CTR are now online and downloadable here.