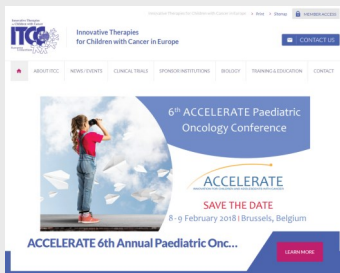


// NEW WEBSITE

Since December 1st, 2017 the new ITCC website has been on-line. Replacing our previous one which was launched at the time of ITCC creation in 2003. Featuring a new map of the centres and a full description of our structure, our new website intends to be a useful tool for parents and physicians giving an overview of all ongoing trials within the consortium.

www.itcc-consortium.org



// ITCC & ANZCHOG

During the ITCC Annual Meeting in November 2017 in Paris a memorandum of understanding was signed between ITCC and ANZCHOG in the presence of ACCT's CEO Dr. Geoffrey McCowage. This provides a framework to facilitate consultation and cooperation in order to enable access to innovative therapies for Australian and New Zealand children.



// EDITO

This is the first time ever that ITCC has sent a newsletter to all its partners, from Industry, Academia, Regulatory Agencies, Parents and Patients Advocacy groups. The New Year was indeed a good opportunity to improve communications and share with you our news and projects. In 2018, ITCC will continue to **enlarge the portfolio of therapeutic innovations** for children and adolescents, reinforcing the biology and preclinical work to drive forward the development of our ambitious plans through scientific endeavor. The **outstanding collaboration between the clinical and research teams within ITCC** is essential and of major value to improve and accelerate drug development in partnership with the pharmaceutical industry. Through the implementation of the recommendations made in the European Joint Action on Rare Cancers, ITCC will **improve access to innovation** for children across Europe. ITCC will launch its Fellowship programme to **train the key opinion leaders of tomorrow** in the field of precision medicine and therapeutic innovation. Running the **ACCELERATE** multistakeholder platform with SIOP-Europe, ITCC will continue to propose and develop new concepts and resources such as the Pediatric Strategy Forums in partnership with the European Medicine Agency,

// ITCC IN 2017

During the year 2017, ITCC kept improving and developing continual commitment in terms of clinical trials. 290 patients with solid tumors or leukemias were included in 21 open trials in 2017. Among those 21 trials, 13 were industry-sponsored and 8 were academic-sponsored.

22 molecules have been developed. Here are the pharmaceutical companies which developed most of them:

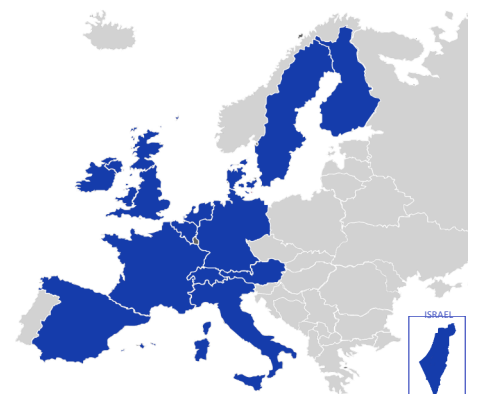
Novartis: 5; Roche: 4; AstraZeneca: 3

Since the creation of ITCC, more than 2 300 children and adolescents with cancer have had access to innovation through the ITCC Consortium.

the **ITCC-P4** pediatric preclinical proof of concept platform within an IMI2 project and the **ITCC BigData program** aggregating clinical, molecular and immunology data from more than 2000 children and adolescents at relapse. It is now recognized at the highest European levels that the regulatory environment needs to be improved in order to accelerate innovation for children with cancer: prioritizing the hundreds of anticancer drugs developed in adults more effectively to meet the needs of children, and incentivizing the development of specific pediatric anticancer drugs and the repurposing of existing drugs. ITCC will work with SIOP-Europe and parents from Unite2cure to **make new proposals for future European legislative programmes**. ITCC wants to strengthen its collaboration with pharmaceutical companies and to propose **privileged partnership** arrangements to those willing to increase their R&D activities in pediatric oncology.

All our ITCC members from research labs and clinical teams (now more than 1 000 people!) and the ITCC Office are eager and committed to make life-changing breakthroughs for children and adolescents. **Working TOGETHER** with all our partners is the best way forward!

Prof. Gilles VASSAL
ITCC President



// THE ITCC P4

Innovative Therapies for Children with Cancer Paediatric Preclinical Proof-of-concept Platform (ITCC-P4) is a newly formed public-private partnership supported by the European consortium 'Innovative Medicines Initiative' (IMI). The consortium with currently 21 partners from 8 countries aims to establish 400 new patient-derived preclinical models of high-risk pediatric solid tumors which will be fully characterized (molecularly, immunologically, pharmacologically and clinically well-annotated) and build a sustainable comprehensive platform to use these models for drug testing. It brings together many of Europe's most distinguished academic and clinical research institutions, well established Small-to-Medium sized Enterprises (SMEs), members of the European Biopharmaceutical Enterprises (EBE) and the members of the European Federation of Pharmaceutical Industries and Associations (EFPIA), thus providing a unique setting to improve patient outcomes by introducing new and effective medicines in standard of care of young people still dying of these rare cancers.



For further information, please visit: www.itccp4.eu

// IMPROVING REGULATORY ENVIRONMENT

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Review

From class waivers to precision medicine in paediatric oncology

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// THE ITCC FELLOWSHIPS CURRICULUM

In 2018 and within the frame of the Education & Training Committee, ITCC will anticipate launching training fellowships which will allow a doctor in training to spend time in one of our larger ITCC Centres gaining valuable clinical experience in managing patients undergoing early phase trials.

The fellowships will be linked to an educational curriculum covering all aspects of drug development. Within the next 2 years we are planning to extend our fellowship programme to scientists wishing to develop specialised postdoctoral research programmes.

// NDDS 2.0

The Second Neuroblastoma New Drug Development Strategy (NDDS) Meeting was held at Clare College Cambridge on 26th and 27th September 2017. The objective of the meeting was to enhance collaboration and identify ways to target ATRX, TERT and MYCN, and evaluate and prioritise other targets for drug development for neuroblastoma. The focus was on molecularly targeted drugs and epigenetic modifiers, although combinations with immunotherapy were discussed. The meeting brought together European and US experts in neuroblastoma biology and pre-clinical and clinical drug development, representatives from pharma, patient representatives and members of the PDCA as observers. Strategies to target MYCN and a potential strategy to target telomerase activation were identified. Early phase paediatric trials which should be opened with high priority and combinations which could be evaluated in first relapse studies were highlighted. Further preclinical research and strategies to drugs TERT, telomerase activation, ATRX and BRP1 are required. Furthermore, this meeting will be an initiator of regular European - US communication, dialogue and planning.

// OUR SPECIAL PARTNERS

They financially support scientific research, and bring together the drive, inspiration and knowledge of everyone involved, from researchers, doctors and parents to politicians and pharmaceutical companies.



They want to make sure all children with cancer are cured, and better cured. By supporting scientific research, removing obstacles and connecting people.

ITCC established a partnership with KICKCANCER in 2017, a public interest foundation registered in Belgium whose goal is to support and fund research projects in order to improve the outcome of children and adolescents with cancer. 2 out of 9 European projects have been selected for funding:

- The project of Dr Marie-Anne Debily from Gustave Roussy/CNRS (France) about DNA methylation;
- The project of Prof Janet Shipley from the ICR (The UK) about rhabdomyosarcoma.



The association « Imagine for Margo – Children without Cancer », created on November 26th, 2011,

is a recognized public interest organization intended to lead awareness and fund raise activities for European research in support of specific, innovative and more effective treatments for childhood cancer. In addition, it provides support to affected families and contributes to the well-being of hospitalized children.

ITCC and Imagine for Margo have established a partnership and since 2012, Imagine for Margo has co-funded 7 projects:

- VINILO in low-grade glioma
- BEACON in neuroblastoma
- BIOMEDE in diffuse intrinsic pontine glioma
- AcSé ESMART in all cancers and leukemias in relapse
- PARC in all cancers and leukemias in relapse
- MICCHADO in high-risk tumors
- MAPPYACTS in all cancers and leukemias in relapse



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