




# **TOWARDS THE FUTURE OF BRAIN RESEARCH**

## **Besta Young Researchers' International Conference**

 **Fondazione I.R.C.C.S.  
Istituto Neurologico Carlo Besta**

Sistema Socio Sanitario

 **Regione  
Lombardia**

**27<sup>th</sup> & 28<sup>th</sup> September 2018**

**Museo Nazionale della Scienza e  
della Tecnologia "Leonardo da Vinci"**

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Fabio Moda  
Alessandro Perin  
Chiara Pisciotta  
Alberto Raggi  
Cristina Rosazza

## Invited Speakers

Nadia Bolognini  
Boriana Büchner  
Michele Corbetta  
Maurizio D'Antonio  
Elisa Giorgio  
Janneke Hoeijmakers  
Cristina Lebrero-Fernández  
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Julika Pitsch  
Luis Sanchez-Perez  
Karl-Michael Schebesh  
Claudio Soto  
Michela Tinelli  
Carlo Viscomi

## Chairs and Panelists

Alexis Brice  
Stefano Cappa  
Monica Di Luca  
Gaetano Finocchiaro  
Giuseppe Lauria Pinter  
Giovanni Leonardi  
Giovanni Luigi Mancardi  
Renato Mantegazza  
Pierluigi Nicotera  
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## Scientific Secretariat

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## ■ Conceptual notes and Objectives of the International Conference

International meeting of scientific dissemination on trends and future of brain and neuroscience research and of exchange of ideas between young researchers of the IRCCS Foundation Neurological Institute Carlo Besta and colleagues from all over the world. The multidisciplinary approach guiding the Conference will show the benefits of interaction in complexity, as requested by the complexity of the brain. This approach allows the understanding of the need of translationality in research, promotes interdisciplinary collaborations and the creation of fruitful dialogues between different areas of neurosciences. This Conference responds to the requirements of international guidelines for biomedical research, i.e. promoting benefits for citizens and high standards of care, and will show the scientific ability as well as the relational and intellectual aliveness generated by Italian IRCCS institutes (scientific institute for research and care), such as the Neurological Besta of Milan is.

This Conference, in line with the new trends of the European Commission that is planning the new Framework Program of Research (Horizon Europe), is divided in 4 sessions where the state of art of neuroscience research, diagnosis and therapy for neurological conditions will be discussed. The main connection between the **4 Sessions** is the common effort of neurologists, basic neuroscientist, neurosurgeon, child neurologist to innovate and to achieve personalized medicine. Speakers and Chairs are between the best national and international representatives of their area, and those researchers that were trained in Lombardy as well as in other Italian regions, through their work at IRCCS Foundation Neurological Institute Carlo Besta will provide visibility to the educational, cultural, scientific capacity of Italian Institutions.

## ■ Razionale ed Obiettivi della Conferenza Internazionale

Incontro internazionale di divulgazione scientifica e scambio di idee tra giovani ricercatori in neuroscienze della Fondazione IRCCS Istituto Neurologico Carlo Besta e colleghi da tutto il mondo sui nuovi trend di ricerca e pratica in neuroscienze. L'approccio previsto è di tipo multidisciplinare e farà emergere il valore dell'interazione nella complessità, come richiesto dalla complessità del cervello. Questo approccio, che si caratterizza per la capacità di emersione della traslationalità della ricerca, promuoverà nuove fruttuose collaborazioni interdisciplinari e una apertura del dialogo tra esperti in discipline affini nell'ambito delle neuroscienze. L'incontro risponde alla linee guida internazionali in materia di ricerca biomedica, finalizzate a garantire al cittadino una sanità efficiente e in grado di rispondere alle necessità di assistenza. L'evento mostra la capacità scientifica e la vivacità intellettuale e relazionale che si genera in un IRCCS come quello milanese del Besta che tra l'altro intende prevenire e frenare la fuga dei cervelli e dei giovani laureati all'estero offrendo possibilità di ricerca di alto livello.

Questo meeting internazionale, in linea con i nuovi orientamenti della Commissione Europea che sta preparando il prossimo programma settennale di ricerca (Horizon Europe), è suddiviso in **4 sessioni** in cui verranno espone e discusse lo stato dell'arte e la conoscenza, la diagnostica e gli approcci terapeutici più avanzati nelle patologie neurologiche: la connessione tra le sessioni è lo sforzo di perseguire una medicina personalizzata in neurologia, neurochirurgia e neurologia pediatrica. Relatori e i moderatori sono tra i migliori esponenti del mondo scientifico nazionale ed internazionale, e i ricercatori che si sono formati in Lombardia o in altre regioni, attraverso il lavoro al Neurologico Besta daranno anche visibilità alla capacità scientifica, formativa e culturale delle istituzioni italiane.

Thursday, 27<sup>th</sup> September

08:30 – 9:00 **Registration**

09:00 – 9:30 **Welcome and introduction**

**Rappresentanti di Regione Lombardia**

**Rappresentanti della Città di Milano**

**Armando Bartolazzi** - Sottosegretario, Ministero della Salute

**Alberto Guglielmo** - Presidente Fondazione IRCCS Istituto Neurologico Carlo Besta

**Fabrizio Tagliavini** - Direttore Scientifico Fondazione IRCCS Istituto Neurologico Carlo Besta

## **SESSION I – Innovative diagnostic approaches to neurological disorders**

Pioneering thinking often fosters the development of new approaches to diagnosis. This session will focus on the innovative and promising approaches to diagnosis of some neurological disorder, including neurodegenerative diseases and brain tumors.

**Chairs:**

**Gaetano Finocchiaro** (Fondazione IRCCS Istituto Neurologico Carlo Besta, Italy)

**Stefano Cappa** (IRCCS Centro San Giovanni di Dio Fatebenefratelli, Italy)

09:30 – 10:15 **Innovative approaches for detecting disease-specific biomarkers in peripheral tissues of patients with dementia**

**Fabio Moda** - Fondazione IRCCS Istituto Neurologico Carlo Besta

**Claudio Soto** - University of Texas, USA

10:15 – 11:00 **Future of functional MRI in the neurological setting**

**Cristina Rosazza** - Fondazione IRCCS Istituto Neurologico Carlo Besta

**Michele Corbetta** - University of Padova, Italy

11:00 – 11:30 **Coffee break**

11:30 – 12:15 **Advances in fluorescein-guided techniques for brain tumor surgery**

**Morgan Aldo Broggi** - Fondazione IRCCS Istituto Neurologico Carlo Besta

**Karl-Michael Schebesch** - University Medical Center Regensburg, Germany

12:15 – 13:00 **The classification and clinical approach to pediatric brain tumors in the molecular era**

**Silvia Esposito** - Fondazione IRCCS Istituto Neurologico Carlo Besta

**Maura Massimino** - Istituto Nazionale dei Tumori, Italy

13:00 – 13:15 **Closing remarks on morning session**

13:15 – 14.00 **Lunch**

Thursday, 27<sup>th</sup> September



## SESSION II – New developments in neurological disorders

Providing care in an environment that challenges the limits of knowledge, technology and skill means the need to innovate is always present. This session will highlight some of the most significant developments in neuroscience and clinical neurology that have been achieved so far and will illustrate new approaches to the understanding and management of neurological diseases.

### Chairs:

**Giuseppe Lauria Pinter** (Fondazione IRCCS Istituto Neurologico Carlo Besta and Università degli Studi di Milano, Italy)

**Giovanni Luigi Mancardi** (IRCCS Ospedale Policlinico San Martino, Italy)

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|---------------|---|
| 14:00 – 14:45 | <b>New developments in Charcot-Marie Tooth disease</b><br><b>Chiara Pisciotta</b> – Fondazione IRCCS Istituto Neurologico Carlo Besta<br><b>Maurizio D’Antonio</b> – IRCCS Ospedale San Raffaele, Italy                               |
| 14:45 – 15:30 | <b>New developments in EEG measurements in epilepsy (human and animal models)</b><br><b>Vadym Gnatkovsky</b> – Fondazione IRCCS Istituto Neurologico Carlo Besta<br><b>Julika Pitsch</b> – University of Bonn Medical Center, Germany |
| 15:30 – 15:45 | <b>Coffee break</b>   |
| 15:45 – 16:30 | <b>Pain genetics</b><br><b>Daniele Cazzato</b> – Fondazione IRCCS Istituto Neurologico Carlo Besta<br><b>Janneke Hoeijmakers</b> – Maastricht University Medical Center, Netherland   |
| 16:30 – 17:15 | <b>HIGHLIGHTS: Education &amp; training in neurosurgery up to new horizons</b><br><b>Alessandro Perin</b> – Fondazione IRCCS Istituto Neurologico Carlo Besta<br><b>Christian Matula</b> – Medical University of Vienna, Austria      |
| 17:15 – 17:30 | <b>Closing remarks on afternoon session</b>   |
|               | <b>Social Event</b>   |
| 17:30 – 19.00 | Best of Museo Nazionale della Scienza e della Tecnologia “Leonardo da Vinci” and Visit to the Museum Temporary Exhibition   |
| 19.00 – 21.00 | <b>Centenario Cocktail</b> Sala delle Colonne (by invitation only)  |

Friday, 28<sup>th</sup> September

## SESSION III – Innovative therapeutic approaches to neurological disorders - I

Treatment and prevention of neurological disorders is challenging for pharmaceutical industry, but also for public and private institutions, physicians, patients, and their families. This session will illustrate the most innovative advances in the field of genetic, motor and mitochondrial disorders.

### Chairs:

**Massimo Zeviani** (Medical Research Council, UK)

**Alexis Brice** (ICM Brain & Spine Institute, France)

- |               |  |
|---------------|--|
| 9:00 – 9:45   | <p>Allele-specific silencing as therapeutic strategy for disorders due to gene duplication: a proof of principle in Autosomal Dominant LeukoDystrophy (ADLD)</p> <p><b>Stefania Magri</b> – Fondazione IRCCS Istituto Neurologico Carlo Besta</p> <p><b>Elisa Giorgio</b> – University of Turin, Italy</p> |
| 9:45– 10:30   | <p>Advanced experimental models for new potential therapeutic approaches in the treatment of mitochondrial diseases</p> <p><b>Andrea Legati</b> – Fondazione IRCCS Istituto Neurologico Carlo Besta</p> <p><b>Carlo Viscomi</b> – Medical Research Council, UK</p>   |
| 10:30 – 11:00 | <p><b>Coffee break</b></p>   |
| 11:00 – 11:45 | <p>Establishment of a global patient registry for mitochondrial disorders</p> <p><b>Anna Ardisson</b> – Fondazione IRCCS Istituto Neurologico Carlo Besta</p> <p><b>Boriana Büchner</b> – Friedrich-Baur-Institute, Ludwig-Maximilians University, Germany</p>   |
| 11:45 – 12:30 | <p>Non-invasive brain modulation in rehabilitation of childhood motor disorders</p> <p><b>Riccardo Masson</b> – Fondazione IRCCS Istituto Neurologico Carlo Besta</p> <p><b>Nadia Bolognini</b> – University of Milano-Bicocca, Italy</p>  |
| 12:30 – 12:45 | <p><b>Closing remarks on morning session</b></p>   |
| 12:45 – 13:30 | <p><b>Lunch</b></p>  |

Friday, 28<sup>th</sup> September

## SESSION IV – Innovative therapeutic approaches to neurological disorders - II

### Chairs:

**Renato Mantegazza** (Fondazione IRCCS Istituto Neurologico Carlo Besta, Italy)

**Monica Di Luca** (Università degli Studi di Milano, Italy)

- 13:30 – 14:15 **Molecular and cellular immuno-mediated mechanisms and novel therapeutic approaches in preventing autoimmune diseases progression**  
**Alessandra Consonni** – Fondazione IRCCS Istituto Neurologico Carlo Besta  
**Cristina Lebrero-Fernández** – University of Gothenburg, Sweden
- 14:15 – 15:00 **Brain cancer immunotherapy**  
**Cristina Corbetta** – Fondazione IRCCS Istituto Neurologico Carlo Besta  
**Luis Sanchez-Perez** – Duke University Medical Center, USA
- 15:00 – 15:30 **Coffee break**
- 15:30 – 16:15 **HIGHLIGHTS: Brain disorders: from the evaluation of cost to the recognition of the value of treatment**  
**Alberto Raggi** – Fondazione IRCCS Istituto Neurologico Carlo Besta  
**Michela Tinelli** – The London School of Economics & Political Sciences, UK
- 16:15 – 17:30 **Panel discussion – Towards the next century: driving the steps of young researchers of the brain**  
**Chair:**  
**Gianluca Vago** (Università degli Studi di Milano, Italy)  
*Alexis Brice, Stefano Cappa, Monica Di Luca, Giovanni Leonardi, Giovanni Luigi Mancardi, Fabrizio Tagliavini, Massimo Zeviani*
- 17:30 – 17:45 **Conference Closing Remarks and Opening of Researchers' Night**  
**Fabrizio Sala**, Assessore alla Ricerca e Innovazione Regione Lombardia (TBC)

## From 18:00 - LA NOTTE DEI RICERCATORI al Museo della Scienza e della Tecnologia

- 19:00 – 19:30 Meeting with the astronaut **Maurizio Cheli**: “Back to the future: Brain in the Space”. Conference in streaming with 26 IRCCS of the Italian Neuroscience and Neurorehabilitation Network.

# ABSTRACTS

## **1. Innovative approaches for detecting disease-specific biomarkers in peripheral tissues of patients with dementia (Moda – Soto)**

Diagnosis of neurodegenerative diseases such as prion diseases, Alzheimer's disease, Parkinson's disease and Frontotemporal Dementia can be difficult, especially in early stages where symptoms might overlap. These diseases are characterized by the deposition of  $\beta$ -sheet rich protein aggregates in the brain, whose shape, size, distribution and composition are specific for each disease. Therefore, they are considered disease-specific biomarkers (DSB) and definitive diagnosis relies on their post-mortem detection within the brain. By taking advantage of innovative techniques we will demonstrate the possibility of detecting DSB in peripheral tissues (urine, blood and cerebrospinal fluids) of patients with dementia, thus setting the basis for a non-invasive and early diagnosis when brain damages are still not severe and irreversible.

## **2. Future of functional MRI in the neurological setting (Rosazza – Corbetta)**

The brain remains one of the major frontiers in science. Using neuroimaging techniques it is possible to study how different regions of the brain interact and exchange information to mediate behavior in health and neurological disorders. It is important to translate this cutting-edge research into clinical setting possibly for the individual patient.

## **3. Advances in fluorescein-guided techniques for brain tumor surgery (Broggi – Schebesch)**

The treatment of most brain tumors (BT) include surgical resection and adjunctive therapies such as chemo and radiotherapy. It has been shown that extent of resection (EOR) impacts on the outcome of patients affected by malignant BT. However, surgical removal of the whole tumor mass is sometimes difficult to achieve, due to intraoperative difficulties in tumor tissue recognition at the margins of the resection. Several fluorescent agents have been employed to better visualize the neoplastic area and therefore increase the EOR. Of these, the use of sodium fluorescein is spreading worldwide; low toxicity, high sensitivity and specificity for tumor tissue and low costs represent the main advantages of fluorescein.

## **4. The classification and clinical approach to pediatric brain tumors in the molecular era (Esposito – Massimino)**

The 2016 edition of the World Health Organization Classification of Tumors of the Central Nervous System integrates both morphologic and molecular features for diagnostic purposes. In the field of pediatric neuro-oncology this has generated a great deal of diagnostic changes especially for patients with medulloblastoma, high-grade glioma and other embryonal tumors, but



the impact on therapy is a modulation between still important clinical features of patients at different ages and these new acquisitions. An improved understanding of the molecular genetics, epigenetics, and cellular biology underpinning childhood brain tumors will potentially enable more effective and less toxic treatment strategies. This could spare children from the severely detrimental consequences associated with conventional treatment protocols and improve the outlook for patients with currently incurable disease.

## **5. New developments in Charcot-Marie-Tooth disease (Pisciotta – D’Antonio)**

Charcot-Marie-Tooth (CMT) disease is the most common neuromuscular disorder and is associated with mutations in several genes, encoding both myelin and axonal proteins. Axonal degeneration is the final common pathway of all forms and a major reason for disability. Derangement of axon-glia interaction, which is critical for nerve functioning and an important determinant of axonal degeneration, appears to be the basis of many types of CMT. The node-paranode structure seems to be affected first. Our projects will shed light on the mechanisms of axonal degeneration secondary to axon-glia dysfunction. The concept of node-paranodopathy is novel and emerging for inherited disorders, and different therapeutic approaches may be tested, on the basis of our results.

## **6. New development in EEG measurements in epilepsy (human and animal models) (Gnatkovsky – Pitsch)**

A major challenge in understanding focal seizure initiation is the contribution of network mechanisms. Computer-assisted EEG analysis is used to characterize spectral seizure profiles and it enabled showing that seizure pattern from patients with focal epilepsy and in different animal models of epilepsy are very similar. These complementary approaches in focal epilepsies can give us new insights into the understanding of epilepsy. We performed long-term EEG analyses to study seizure dynamics and distribution in several in vivo epilepsy animal models and addressed whether EEG-dynamics reflect developmental functional stages of a structural seizure focus after a transient epileptogenic event such as status epilepticus. We expect our findings to be critical in understanding chronically hyperexcitable neuronal networks after transient brain insults and in deriving biomarkers of epileptogenesis. We aim to deepen our collaborative efforts further in the future with respect to extrapolating on different rodent models of epileptogenesis.

## **7. Pain genetics (Cazzato – Hoeijmakers)**

Neuropathic pain is a common feature of peripheral neuropathies causing a significant impact on patients’ quality of life and health care costs. However, some patients with peripheral neuropathy complain severe pain, whereas others experience only mild symptoms or have a painless form, regardless the underlying etiology. Recent studies suggested a genetic substrate for neuropathic pain focusing in particular on the role of ion channels. Gain-of-function variants in voltage-gated sodium channels  $\alpha$ -subunits expressed in sensory neurons have been identified in about 10% of patients with idiopathic painful small fiber neuropathy. Moreover, different variants have been found to cluster in painful and painless diabetic neuropathy. These findings strengthen

the hypothesis of a genetic background underlying individual pain phenotypes, which could identify protecting or predisposing pain profiles.

## **8. Education and training in neurosurgery up to new horizons (Perin – Matula)**

It is a matter of fact that education and training represent one of the most important tasks and needs in Neurosurgery. The purpose of academic-focused neurosurgical education is to teach the clinical knowledge, the surgical skills necessary to become a well-trained neurosurgeon, but also to share the principles of the scientific working. However, increasing expectations from the “learners” but also teacher side, the phenomenon of working hour reduction, new curricular mandates, but also new teaching possibilities have significantly influenced training programs all around the world to ensure adequate education and training in less time, limited resident independence and -not to forget- limited resources. So-called “milestones” concepts including defined educational outcomes have been established, virtual training simulation programs have come up, and several e-learning tools have sometimes even replaced traditional training concepts. At the same time, health care systems have undergone rapid changes, which also must be considered in modern education and training programs. Different survey conducted worldwide found that graduating residents felt inadequately prepared for their future in Neurosurgery. No doubt, there is a global need for harmonizing education and training in Neurosurgery and it’s time to embark for new horizons.

## **9. Allele-specific silencing as therapeutic strategy for disorders due to gene duplication: a proof of principle in Autosomal Dominant LeukoDystrophy (ADLD) (Magri – Giorgio)**

Allele-Specific silencing by RNA Interference (ASP-RNAi) holds promise as a therapeutic strategy for downregulating a single mutant allele with minimal suppression of the corresponding wt allele. This approach has been effectively used to target autosomal dominant mutations and SNPs linked with aberrantly expanded trinucleotide repeats. Here, we propose ASP-siRNA as a suitable choice to target duplicated disease genes, avoiding potentially harmful excessive downregulation. As a proof-of-concept, we studied Autosomal Dominant adult-onset demyelinating LeukoDystrophy (ADLD) due to lamin B1 (LMNB1) duplication, a hereditary, progressive and fatal disorder affecting myelin in the central nervous system. ADLD patients have three, equally expressed, LMNB1 alleles. We chose to target the non-duplicated allele by ASP-siRNA, exploiting a frequent coding SNP. We identified three siRNAs able to efficiently restore physiological mRNA and protein levels in patients’ fibroblast and to improve ADLD-specific cellular alterations, corroborating ASP-siRNA therapeutic potential in ADLD. Our work opens new therapeutic possibilities for all Mendelian and syndromic disorders associated with gene(s) duplication.

## **10. Advanced experimental models for new potential therapeutic approaches in the treatment of mitochondrial diseases (Legati – Viscomi)**

Mitochondrial Disorders form a group of rare, genetically and clinically heterogeneous conditions characterized by impairment of the mitochondrial oxidative phosphorylation system

(OXPHOS). For this reason tissues and organs with high-energy demand based on efficient OXPHOS are preferentially involved in mitochondrial disease. The minimum prevalence of isolated or combined, genetically defined OXPHOS defects is approximately 1:4300 live births. The extreme genetic, phenotypic and clinical course variability represent a challenge to the development of effective diagnostic and therapeutic strategies. However, new possibilities have recently been emerged from studies in model organisms and are awaiting verification in humans.

## **11. Establishment of a global patient registry for mitochondrial disorders (Ardissone – Büchner)**

Much progress has been achieved in recent years by the implementation of national patient registries (mitoNET, MITOCON, NAMDC) and biomaterial banks that enable natural history studies, treatment trials, and large-scale NGS diagnostics. In 2014, GENOMIT was funded as a network of eight partners in Germany, Austria, Italy, France, and USA which act in close collaboration with Mitochondrial Patient organizations to improve the diagnosis and care for patients with mitochondrial disease. GENOMIT partners are established national hubs for the biochemical and genetic diagnosis and care of patients with mitochondriopathies. They represent the existing national patient registries and have access to the largest collection of mitochondriopathy-related NGS data in Europe. Each partner has also developed unique expertise that will be shared synergistically within the network. GENOMIT will thus create the critical mass to expand knowledge on the natural history, and genotype-phenotype correlation of mitochondrial disease, and gain insight into pathophysiologic mechanisms and feasibility of novel therapeutic approaches. The involved researchers and patient organizations world-wide agree, however, that the time is ripe now for the next step: to harmonize existing national resources and integrate them into a global registry, linked to a repository of the respective local and national biomaterial banks.

## **12. Non-invasive brain modulation in the rehabilitation of childhood motor disorders (Masson – Bolognini)**

Childhood hemiplegia is the most frequent form of cerebral palsy, with lifelong effects on the upper and lower limb function of the affected side. An acquired, unilateral brain injury early in development can alter the physiological maturation of corticospinal tracts, leading to a prevalent ipsilateral control of the affected side by the healthy hemisphere. To contrast this process, motor rehabilitation tries to restore the balance between the activity of the two hemispheres. Recent studies suggest that non-invasive techniques, such as transcranial direct current stimulation (tDCS), can modulate brain activity helping motor learning and strengthening the efficacy of rehabilitation in improving motor outcome.

### **13. Molecular and cellular immunomediated mechanisms and novel therapeutical approaches in preventing autoimmune diseases progression (Consonni – Lebrero Fernandez)**

Current therapies for Myasthenia Gravis (MG) range from symptomatic to disease modifying treatments, often accompanied by severe side-effects. Novel immunomodulatory approaches, possibly antigen-specific, are requested to re-enforce tolerance of the autoaggressive T cells that drive autoantibody production. We evaluated the clinical efficacy of an antigen-specific recombinant fusion protein, nasal administered, in the mouse EAMG model. The recombinant fusion protein contains a cholera toxin A1 subunit with the specific R9K mutation (CTA1R9K), the immunodominant epitope 146-162 of Torpedo AChR alpha subunit, and a dimer of the IgG-binding D region of *S. aureus* protein A (DD). Nasal treatments were associated with EAMG amelioration, reduction of anti-mouse AChR antibody levels, preservation of muscle AChR content, down-regulation of IFN $\gamma$  and IL17 pro-inflammatory mRNA, and upregulation of TGF $\beta$ , IL10, FoxP3 transcripts in lymph nodes and spleens. This preclinical study provides strong evidence on the efficacy of the fusion protein in mice EAMG, suggesting its use in clinical trial.

### **14. Brain Cancer Immunotherapy (Corbetta – Sanchez-Perez)**

Glioblastoma (GBM), the most aggressive primary brain tumor and one of the most lethal adult cancer worldwide, remains incurable despite conventional Stupp therapy including surgery, radiotherapy and chemotherapy with the alkylating agent temozolomide. Immunotherapy has emerged as the breakthrough against cancer, showing great clinical impact and, it is an attractive option also for GBM. Active immunotherapy has been proven effective in prolonging survival of GBM patients, but the tumor volume at the time of vaccine, the potential anergy and low frequency of tumor-specific T cells could limit its efficacy. To overcome these constraints, adoptive cell therapy, based on tumor infiltrating lymphocytes (TIL) and chimeric antigen receptor T cells, is being investigating as a promising alternative for GBM. In particular, TIL-based therapy may represent an attractive approach to optimize a personalized adoptive cell therapy design, and it may rapidly progress also for such a cold cancer as GBM in synergy with other therapies including radiotherapy as an adjuvant for enhancing both local and systemic immunotherapy.

### **15. Brain disorders: from the evaluation of cost to the recognition of the value of treatment (Raggi – Tinelli)**

Brain disorders are complex and interlinked with hundreds of specific diagnoses, resulting in fragmented research and practice. They are increasingly prevalent due to population ageing: the overall cost for Europe was estimated at 798 billion € in 2010, and they account for 29% of Years Lived with a Disability. The Value of Treatment project is aimed to examine health gains and socio-economic impacts resulting from best health interventions (pharmacological and psychosocial) in comparison with current care through the identification of the major unmet needs, treatment gaps and, thus, priorities for policymakers.

# SPEAKERS BIOSKETCH

## Claudio Soto



Dr. Soto is the Huffington Distinguished University Chair, Professor of Neurology and Director of George and Cynthia Mitchell Center for Alzheimer's disease and related Brain Disorders at the University of Texas Medical School in Houston. He received his PhD in biochemistry and molecular biology from the University of Chile in 1993 and was a postdoctoral fellow at the Catholic University of Chile and at the New York University School of Medicine. Between 2003 and 2008, he served as Director of the George and Cynthia Mitchell Center for Neurodegenerative Diseases and Professor on the Departments of Neurology, Neuroscience & Cell Biology and Biochemistry & Molecular Biology at the University of Texas Medical Branch in Galveston. His research interest deals with the understanding of the molecular basis of neurodegenerative diseases associated with the misfolding and brain accumulation of proteins, particularly focusing in Alzheimer's, Parkinson's and prion-related disorders. His work has led to a better understanding of these diseases and to the development of novel strategies for diagnosis and therapeutic intervention. Dr Soto's innovative method for biochemical diagnosis is a non-invasive and high sensitive test for various brain diseases. Also, the novel therapies coming from his work are currently being tested in patients affected by neurodegenerative diseases. Dr Soto has been invited speaker in more than 200 International scientific meetings worldwide. He has been awarded many grants from the Federal government, and private foundations for a total funding of over 30 million dollars. He is author of 185 publications in peer-reviewed journals (H-index: 63).

## Fabio Moda



Dr. Moda works as senior researcher at FINCB Neuropathology Unit since 2003. He graduated in medical biotechnology in 2007 and obtained his PhD in Translational and Molecular Medicine in 2010. In 2011 he moved to the University of Texas Health Science Center at Houston (USA), under the supervision of prof. Soto, for a 2 years post-doc experience. During his professional career, he has been involved in several projects aimed at detecting peripheral biomarkers associated with distinct degenerative dementias, including Alzheimer's disease, Parkinson's disease, atypical parkinsonisms, frontotemporal dementia and prion diseases. His research activities are mainly focused on the study of the molecular basis associated with protein misfolding and neurodegeneration, with the aim of developing strategies for early diagnosis and therapeutic treatments. Dr. Moda is PI of several national (Italian Ministry of Health, Ricerca Finalizzata) and transnational projects (Alzheimer's Association, Michael J Fox Foundation, EuronanomedIII) for a total funding of about 800.000€. He is author of 32 publications in peer-reviewed journals (H-index: 10).

## Maurizio Corbetta



Prof. Corbetta is the Chair of Neurology at the University of Padova, Director of the Padova Neuroscience Center ([www.pnc.unipd.it](http://www.pnc.unipd.it)), and Professor of Neurology, Radiology, and Neuroscience at Washington University School of Medicine. He moved back to Italy in 2016 after spending nearly 30 years in the US. He has pioneered functional brain imaging studies of cognitive functions in the late 1980's. Currently his interests include the functional and structural organization of the brain, and the modifications induced by neurological disorders, specifically stroke. His research has been funded by the NIH for more than 20 years, and he is one of the Highly Cited Researcher from Thompson Reuters in Neuroscience/Behavior. He is author of 196 publications in peer-reviewed journals (H-index: 68).

## Cristina Rosazza



Dr. Rosazza is a researcher working at FINCB Neuroradiology Department of since 2008. She graduated in Psychology in Milano and did her PhD in Cognitive Neuroscience at SISSA in Trieste and a Marie Curie Post-Doc at the CNRS in Lyon. Her research activity is primarily focused on the study of brain structure and function, using mainly Magnetic Resonance Imaging (MRI). She is interested in cognitive functions such as language and reading, memory and motor functions, and in the intrinsic brain activity observed at rest. She is also interested in disorders of consciousness, in the study of the residual cerebral functioning of patients. She was awarded a fellowship from CIMEC (University of Trento) and more recently a 'Giovani Ricercatori' grant from the Ministry of Health. She is also interested in communicating science to young people and adults. She is author of 26 publications on peer-reviewed journals (H-index 11).

## Karl-Michael Schebesch



Dr. Schebesch is a Neurosurgeon, Medical Director and Senior Managing Physician at the Department of Neurosurgery, University Regensburg Medical Center. He graduated from the Medical School of the Friedrich-Alexander University Erlangen-Nürnberg in 2001 and took the doctoral degree in 2002. He started his residency at the University Hospital Regensburg in 2001 and finished the neurosurgical training program in 2008. In 2009, Dr. Schebesch became senior staff member, in 2012 he received the postdoctoral qualification and in 2013 he was appointed the Senior Managing Physician of the Department of Neurosurgery. Finally, in 2017, Dr. Schebesch nominated as 'Professor' for Neurosurgery. During his career, he did many abroad fellowships, including in Miami, Zürich and Helsinki. The main subjects of specialization are skull base surgery and vascular surgery. Since 2011, Dr. Schebesch and his team are very much involved in fluorescence-guided surgery, and together with Besta Institute Neurosurgery-II Division, some edge-cutting papers on this topic have been issued in high-level journals. He is author of 70 publications in peer-reviewed journals (H-index: 14).



## Morgan Broggi



Dr. Broggi is a neurosurgeon at the FINCB Department of Neurosurgery since 2015. After graduation (2005), he completed the 5-years residency program in neurosurgery at the University of Milan (2010). He, then started working under annual research contracts at the Department of Neurosurgery of FINCB until 2014. He also completed his PhD (2014) at the University of Milan; the field of research was the use of fluorescence-guided technique in neurosurgery. Dr. Broggi focused his clinical and research interests mainly on micro-neurosurgery, endoscopic neurosurgery, minimally invasive image-guided vascular and oncological neurosurgery, the surgical treatment of hydrocephalus, the analysis and classification of complications in neurosurgery and the application of fluorescent techniques in neurosurgery. From 2006 to 2010, he attended several times the Center of Anatomy and Cellular Biology of the Medical University of Vienna, where he trained in cadaveric dissections and surgical approaches. He completed a one-year fellowship (2009) at the Center for Minimally Invasive Neurosurgery of the Prince of Wales Private Hospital, Sydney, Australia, directed by Dr. Charles Teo. He performed more than 500 neurosurgical procedures as first operator and 1500 surgical operations as assistant. He is author of 63 publications in peer-reviewed journals (H-index: 11).

## Maura Massimino



Maura Massimino graduated at Milan University in 1987 with laude. She achieved Hematology Board in 1991 and Pediatrics Board in 1995 at Milan University. From 1987 onwards she worked at the Pediatric Oncology Unit of the Fondazione IRCCS Istituto Nazionale dei Tumori of Milan. From July 2010 onwards, she holds the position of Pediatric Oncology Unit Director. From 2000 onwards she is responsible of a Functional Unit for pediatric neurooncology activities. Her clinical research interests are on brain tumors of childhood, lymphomas and thyroid cancer, including endocrinological and neuro-cognitive late evaluations. She is affiliated to several scientific associations, including the International Society of Pediatric Oncology, the American Society of Clinical Oncology, and the European Association of Neurooncology. She has been PI or co-PI of several grant from different agencies. She is author of 263 publications in peer-reviewed journals (H-index: 34).

## Silvia Esposito



Dr. Esposito has worked as a consultant Child Neurologist at FINCB Neuropsychiatry Division since 2013. She graduated at University of Milan in 2007, obtained a post-degree specialization in Child Neurology and Psychiatry in 2013 and a PhD in Biomedical Science-Neuroscience at the University of Pavia in 2017. Her clinical and research interest primarily focus on Neurofibromatosis and genetic syndromes predisposing to nervous system tumors; she currently follows pediatric Neurofibromatosis (outpatients and inpatients), evaluating children affected with a suspected or confirmed neurocutaneous syndrome, planning genetic testing and counselling, as well as clinical and instrumental follow-up of neurological complications. She is author of 13 publications on peer-reviewed journals (H-index: 5).

## Maurizio D'Antonio



Dr. D'Antonio graduated in Biological Science at the Università degli Studi of Milan in 1998 and completed his PhD in Cellular and Molecular Biology at the University College London in 2005. In 2004 he was awarded a MRC-UK fellowship and, in 2009, the Asbury A. K. Award by the Peripheral Nerve Society. In 2011 and in 2014 he received the 'Giovani Ricercatori' grant by the Italian Ministry of Health. He is employed at the Department of Genetics and Cell Biology of the San Raffaele Scientific Institute, where he holds the position of group leader of Biology of myelin Unit. His research interest are on the molecular mechanisms underlying hereditary neuropathies, collectively known as Charcot-Marie-Tooth (CMT) disease, with the goal of developing effective therapeutic strategies. He is author of 28 publications in peer-reviewed journals (H-index: 13).

## Chiara Pisciotta



Dr. Pisciotta has been working as a neurologist at FINCB Rare Neurodegenerative and Neurometabolic Diseases Unit since 2015. She graduated in Medicine at the University "Federico II" of Naples in 2006, where she also completed her residency in Neurology in 2012 and obtained a PhD in Neuroscience in 2015. During the PhD period, she spent almost 2 years as a research fellow in a top-level lab for inherited neuropathies (Dr. Michael Shy's lab in Iowa City), adding to her clinical skills the knowledge of laboratory techniques. Her main field of interest is inherited neuropathies especially Charcot-Marie-Tooth disease (CMT), its pathomechanisms, natural history and development of outcome measures. She is the local PI in a 'Giovani Ricercatori' grant by the Minister of Health: "Upper limb impairment in Charcot-Marie-Tooth patients: evaluation of motor strategies and effectiveness of rehabilitation treatments by means of an innovative instrumental protocol". She is involved in other four projects granted by AFM-Telethon and Telethon-Italy aimed to study the mechanisms of axonal loss in late-onset CMT, disability and novel outcome measures for genetic neuropathies. She is member of the editorial board of the Journal of Peripheral Nervous System. She was recently awarded the "AMICHE PER LA VITA ONLUS" award on research in CMT. She is author of 40 publications in peer-reviewed journals (H-index: 10).

## Julika Pitsch



Dr. Pitsch is a Principal Investigator at the Department of Neuropathology at Bonn Medical Center, Bonn University. She is also the leader of the Transgenic mouse platform with permanent EEG/video Monitoring Unit. She studied Veterinary Medicine at JLU Gießen, Germany and did her PhD (JLU Gießen) with the topic: "Functional role of expression changes and distribution of ion channels and neurotransmitter receptors in focal epilepsy in transgenic animal models". As a postdoctoral Researcher she worked in the laboratory of Prof. Albert Becker, Department of Neuropathology, Bonn Medical Center, focusing on the analysis of different transgenic and induced epilepsy animal models. Recently, she successfully obtained funding for the project "Characterization and functional analyses of human autoantibodies in limbic encephalitis mouse models". She collaborated with other researchers, and produced multiple peer-reviewed publications resulting from several projects also in cooperation with Dr. Gnatkovsky and De



Curtis, Unit of Epileptology and Experimental Neurophysiology of FINCB. Her research interests are related to molecular neuroscience and experimental neuropathology. Her expertise includes analyzing epilepsy animal models, *in vivo* models for limbic encephalitis, EEG measurements, histology, and additional molecular and functional analyses. She is author of 19 publications in peer-reviewed journals (H-index: 9).

## Vadym Gnatkovsky



Dr. Gnatkovsky is a Senior Researcher at FINCB Epileptology Department. He studied Medicine from 1992 to 1998 at the Odessa State Medical University, Ukraine. In 2002, Dr. Gnatkovsky received his PhD in Pathological Physiology followed by postdoctoral training under the supervision of Dr. Marco de Curtis of FINCB. His scientific interest addressed limbic system network connectivity, EEG rhythms, basic mechanisms underlying epileptic seizure development in experimental and human epilepsy as well as the identification and quantification of EEG biomarkers of the epileptogenic zone. Ongoing studies compare EEG data from epilepsy animal models (chronic and acute) and potential mechanisms of seizure generation with human intracranial SEEG recordings to develop and propose improved diagnostic approaches for epileptogenic network study with possible therapeutic directions. Aim of this work is studying intrinsic mechanisms of seizure generation, development and termination. Dr. Gnatkovsky has been a member of the AES/ILAE's Translational Task Force, which recently proposed standards for data acquisition and software-based analysis of in-vivo EEG recordings from animals as part of a larger effort to harmonize video-EEG interpretation and analysis methods across studies using in-vivo and in-vitro seizure and epilepsy models (Moyer et al., 2017). Currently he is an invited member of the ILAE (International League against Epilepsy) Neurobiology Commission. He was a PI in two 'Giovani Ricercatori' grants by the Italian Ministry of Health (2011, 2014). He is author of 33 publications in peer-reviewed journals (H-index: 14).

## Janneke G.J. Hoeijmakers



Dr. Hoeijmakers is a neurologist with the focus on neuromuscular disorders at the Maastricht University Medical Center+, Maastricht, the Netherlands. In February 2014 she defended her PhD thesis 'Small fiber neuropathy and sodium channels: a paradigm shift' under co-supervision of prof. C.G. Faber and dr. I.S.J. Merkies in close collaboration with prof. Waxman, Yale University, US and prof. Lauria, FINCB. The Annals of Neurology paper 'Gain of function NaV1.7 mutations in idiopathic small fiber neuropathy' was awarded with the Prinses Beatrix Spierfonds year prize and has been granted with the 2013 Annals of Neurology Prize 'for a distinguished contribution to clinical neuroscience'. For the complete thesis she received the Amsterdamsche Neurologenvereniging incentive award. To date, she is supervising PhD students on projects about the diagnostics, etiology, pathophysiology and treatment of painful neuropathies. She is involved as co-supervisor in the H2020 MSCA PAIN-Net project (Grant n°721841). She is medical advisor of the Dutch patient organization for neuromuscular disorders (Spierziekten Nederland) for the focus group 'Small fiber neuropathy' and is a committee member of the annually 'Boerhaave symposium for neuromuscular diseases'. She is author of 38 publications in peer-reviewed journals (H-index: 18).

## Daniele Cazzato



Daniele Cazzato is a neurologist and neurophysiologist with focus on clinical neurophysiology in the field of neuromuscular diseases and neuropathic pain at FINCB. He earned his master's degree in Medicine at the University of Ferrara in 2010, obtained the degree in Neurology in 2016 at the University of Modena and Reggio Emilia. He also obtained the Board Certificate in Clinical Neurophysiology by the Italian Society of Clinical Neurophysiology. He is currently a PhD student in Translational Neuroscience and Neurotechnologies at the University of Ferrara. He is involved as investigator in

clinical trials in the context of neuromuscular disorders, inflammatory peripheral neuropathies and neuropathic pain. His research interest focuses on clinical, neurophysiological, genetic and skin biopsy characterization of painful neuropathies with particular reference to small fiber neuropathy. He is author of 13 publications in peer-reviewed journals (H-index: 6).

## Christian Matula



Prof. Matula serves as Professor of Neurosurgery at the Neurosurgical Department, Medical University of Vienna, Austria. He is Vice President of the Foundation Board of Global Neuro, an independent foundation aiming to improve quality of life for patients suffering from neurosurgical disorders, and he is member of the Educational Committee of the World Federation of Neurological Surgery (WFNS). Dr. Matula received his MD degree in 1986 from the University of Vienna, completed his PhD in Neuroendoscopy in 1996 and has been appointed as Professor of Neurosurgery in 1997 at the same

University. His main research interest are on Endoscopic Skull Base Surgery, Neuroendoscopy and in the area of Neurotrauma and, more in general, in new surgical techniques and their clinical implementation. He has organized more than 120 workshop and courses worldwide and has given more than 400 invited lectures as visiting professor all over the world. He is author of 92 publications on peer-reviewed journals (H-index: 25).

## Alessandro Perin



Dr. Perin is a neurosurgeon at FINCB Division of Neurosurgery-I. His activity focuses mainly on the surgical treatment of patients with benign and malignant brain and spinal cord lesions. Before starting his surgical career he obtained a PhD in neuroscience at the University of Trieste, Italy and he completed his clinical fellowship at the Neurosurgery Department at McGill University under the supervision of Prof Rolando Del Maestro and Prof Le Blanc. In the field of training and education he is adjunct professor in Neuro-Oncology at the University of Trieste and he is the Scientific Director

of the Besta NeuroSim Center since its creation in 2015. Here he has been co-relator for several student theses regarding a variety of projects that combine the use of advanced technologies in both clinical and non-clinical scenarios. He is author of 64 publications on peer-reviewed journals (H-index: 15).

## Elisa Giorgio



Dr. Giorgio graduated in Biotechnology at the University of Pavia in 2008 and obtained a PhD in Human Genetics in 2015 at the University of Torino. She has been working in the field of genetics of neurodegenerative disorders and pharmacogenomics at the University of Genova and at the Galliera Hospital, and later on moved at the Department of Medical Science of the Turin University, where she holds the position of Autosomal Dominant Leukodystrophy team leader. Her research interest deal with genetics of neurodegenerative disorders, exome sequencing, position effects in Mendelian disorders, allele-specific silencing as therapeutic option for genetic diseases. She received two national and two international awards for outstanding in science. She is author of 29 publications on peer-reviewed journals (H-index: 8).

## Stefania Magri



Dr. Magri graduated in Neurobiology at the University of Pavia in 2008, completed her PhD in Translational and Molecular Medicine at the University of Milan-Bicocca in 2012 and the Specialization in Medical Genetics, University of Milan in 2017. She's employed at FINCB since 2009, at present with a Postdoctoral Research Fellow, in the Unit of Medical Genetics and Neurogenetics. Her main research interest includes: genetics of neurological disorders (spinocerebellar ataxias, spastic paraplegias, peripheral neuropathies, and epileptic encephalopathies); NGS-based molecular analysis of genetically heterogeneous neurological disorders; functional characterization of novel variants and candidate disease-genes. She has been awarded two international awards for outstanding in science, and a grant as collaborator in a 'Giovani Ricercatori' grant by the Italian Ministry of Health (Epileptogenic encephalopathies and complex generalized epilepsies of infancy: NGS analysis and functional characterization of novel causative variants for an efficient diagnosis and a personalized treatment). She is author of 11 publications on peer-reviewed journals (H-index: 5).

## Carlo Viscomi



Dr. Viscomi graduated in Biological Sciences in 1999 at the University of Milan, where he also received his PhD in Physiology in 2002. Between 2002 and 2004 he held a post-doc position at the University of Milan, and then moved to the Neurological Institute C. Besta, where he held the position of research scientist up to 2013. Since 2013 on, he holds the position of Senior Investigator Scientist at MRC - Mitochondrial Biology Unit, University of Cambridge, UK. His main research interest are on the process of mitochondrial neurodegeneration, and he was awarded a 'Giovani Ricercatori' grant by the Italian Ministry of health (2013-106) and served as co-investigator for two research project funded by the Centres of Excellence in Neurodegeneration and by ERC. He received the Kelsey Wright Award for Excellence in Mitochondrial Medicine given by the United Mitochondrial Disease Foundation (Scottsdale, AZ, 2010). He is author of 54 publications on peer-reviewed journals (H-index: 25).

## Andrea Legati



Dr. Legati is currently a researcher at the Medical Genetics and Neurogenetics Unit of FINCB. He graduated in Biotechnology at the University of Padua in 2008 and obtained a PhD degree in Medical Genetics at the University of Brescia in 2012 with a project focused on translational genomics and neurogenetics of cerebellar ataxias. After the PhD he moved as a post-doctoral research scholar at the Semel Institute for Neuroscience and Human Behavior (University of California, Los Angeles), where he focused his research activity on genetics of neurodegenerative disorders, such as Alzheimer's disease, ALS, Parkinson's disease and Primary Familial Brain Calcification. Since 2014 he works at the FINCB Medical Genetics – Neurogenetics Unit, and is specialized in molecular genetics of mitochondrial diseases. His research interest is focused on disease gene discovery, based on combined approaches of next generation sequencing techniques and bioinformatics analysis performed on both genomic and mitochondrial DNA. He is author of 24 publications on peer-reviewed journals (H-index: 10).

## Boriana Büchner



Boriana Büchner graduated in Medicine at the University of Bonn in 2005. She completed a training in Medical Informatics at the mibeg Institute of Cologne and obtained a Dr. med. degree at the University of Cologne in 2010. Her main research interests are in rare mitochondrial disorders and, in particular, with the build-up, organization and harmonization of national and international registries on rare neurological disorders. She has been Chief operating manager of mitoNET – The German network of mitochondrial disorders since 2010, Chief operating manager of TIRCON - Treat Iron-Related Child-Onset Neurodegeneration since 2012 and, since 2017, she is responsible for the harmonization of the national data cohorts of patients with mitochondrial disorders in Germany (mitoNET), UK (Welcome Trust Centre for Mitochondrial Research), Italy (Mitocon) and USA (NAMDC) within the EU-E-Rare-funded project GENOMIT. She is author of 8 publications on peer-reviewed journals (H-index: 6).

## Anna Ardisson



Anna Ardisson is a child neurologist, employed at FINCB Child Neurology Unit since 2006. Her field of interest is focused on mitochondrial disorders, lysosomal and neuromuscular diseases. She is PI of research projects FINCB (“Infantile Mitochondrial diseases: definition of new phenotype” and “Phenotype-genotype correlations in mitochondrial diseases related to PDH deficiency”), co-PI of research project “Phenylbutyrate Therapy in Mitochondrial Diseases with lactic acidosis: an open label clinical trial in MELAS and PDH deficiency patients (Ministry of Education, Universities and Research 2016). She is also coinvestigator in observational studies in Niemann-Pick C disease (NPC Disease Registry; An international rare diseases registry for NP type A, B and C; a Prospective Non-therapeutic Study in Patients Diagnosed With NPC). She is partner of Italian Collaborative Network of Mitochondrial Diseases, Italian study group Niemann Pick C and GM1 diseases, Member of the Scientific Advisory Committee of the Italian Association Niemann Pick Onlus. She is author of 36 publications on peer-reviewed journals (H-index: 11).

## Nadia Bolognini



Prof. Bolognini graduated in 2001 in Experimental Psychology at the University of Bologna, and in 2005 obtained her PhD in Experimental & Clinical Psychology at the University of Bologna. She is Associate Professor in Psychobiology & Physiological Psychology at the Department of Psychology (University of Milano-Bicocca) and Researcher at the Neuropsychology Lab, IRCCS Istituto Auxologico Italiano. She is member of the Milan Centre for Neuroscience and Associate Editor of BMC Neuroscience. In 2012 she was awarded the Editor's Choice Award from the

Organization for Human Brain Mapping for the best article published in the journal Human Brain Mapping in 2011; in 2014 she was awarded the "SIPF Early Career Award" from Italian Society of Psychophysiology; in 2015 she received the National Prize "Nottola-Mario Luzi", awarded for the paper "Motor and parietal cortex stimulation for phantom limb pain and sensations" (Pain, 2013). Her research interests include multisensory processing, neurorehabilitation through non-invasive brain stimulation, applied forensic neuroscience. Her approach is the study of patients affected by stroke or other neurological diseases, by means of behavioral and neurophysiological methods. She is author of 92 papers on peer-reviewed journals (H-index: 25).

## Riccardo Masson



Riccardo Masson is a clinical and research fellow at FINCB Developmental Neurology Unit, section of Motor Development Disorders, since 2016. He graduated in Medicine in 2010 and he completed his residency in Child Neurology in 2016 at University of Verona. He spent one year at Stella Maris Institute working on early motor development and rehabilitation in children with genetic disorders or acquired brain lesions, with a special focus on perinatal stroke and the role of quantitative EEG in detecting subtle asymmetries

of electrical activity as prognostic factors for motor outcome. He is now working on rehabilitation approaches in developmental motor disorders following brain injuries (cerebral palsy), with a main interest in pharmacological treatment of dystonic cerebral palsy, definition of outcome measures and new techniques for improving motor recover in congenital hemiplegia. He is also involved as investigator in several pharmacological trials (phase 1, 2 and 3) on new drugs for the treatment of neuromuscular disorders of infancy (spinal muscular atrophy and Duchenne muscular dystrophy) and in the study of the relationship between nutritional aspects and motor outcome in these disorders. He is author of 5 publications on peer-reviewed journals (H-index: 1).

## Cristina Lebrero-Fernández



Dr. Lebrero is a Postdoctoral Researcher at the Department of Microbiology and Immunology at the University of Gothenburg (Sweden). She received a B.S. degree in Biotechnology from the University of León (Spain) in 2011, participating in the Erasmus programme at the University of Gothenburg in 2010-2011. She obtained the PhD in Medical Science from the University of Gothenburg in 2016. During her PhD studies, she explored the butyrophilin and butyrophilin-like family, its role in epithelial cell-

intraepithelial T lymphocyte cross-talk and its contribution to pathophysiology. She joined the Nils Lycke's



laboratory in 2016, where her current research involves study of the mechanisms and implications of the CTA1-X-DD and CTA1R7K-X-DD immuno-modulating fusion proteins in mucosal immunity and tolerance, respectively. During these years, she has taught on several immunology courses, she has attended many international meetings and congresses, she has been involved in the supervision of one bachelor's and two master's thesis. She is author of 5 publications on peer-reviewed journals (H-index 1).

## Alessandra Consonni



Dr. Consonni graduated in Medical, Molecular and Cellular Biotechnologies at the Vita-Salute San Raffaele University in 2007, and she obtained a PhD in Neurobiology in 2013 at the University of Insubria (Varese). She currently holds a Postdoc position at the FINCB Neuroimmunology and Neuromuscular diseases laboratory. Her research interest deal with neuroimmunology and neuromuscular diseases, with particular interest in autoimmunity and animal models of Multiple Sclerosis (MS) and Myasthenia Gravis (MG).

The focuses of her research activities are mainly based on: the development of new drugs and cellular therapies for MS/MG in the animal model; the identification of methods to counteract the breaking of self-tolerance (fusion proteins of immunogenic epitopes); the analysis of the composition of the gut microbioma of patients with MG and MS through NGS methodologies. She is author of 8 publications on peer-reviewed journals (H-index: 4).

## Luis Sanchez-Perez



Prof. Sanchez-Perez currently holds the position of Assistant Professor at the Duke University Medical Center, Department of Neurosurgery. He graduated in Industrial Biotechnology in 2000 and obtained a PhD in Immunology at the Mayo Clinic College of Medicine in 2005. His research interest deal with tumor immunotherapy with specific training on T cell biology. His work has largely focused on manipulating murine and human T cells to potentiate T cell trafficking into tumors, tumor recognition and

enhanced antitumor activity. His expertise includes the in vitro and in vivo evaluation of murine T cell phenotype, function, trafficking and antitumor efficacy against intracranial brain tumors. He has extensively worked to develop effective vaccination and T cell therapy strategies for the treatment of malignant glioma tumors. For these research activities, he received three different awards from the National Institute of Health. He is author of 53 publications on peer-reviewed journals (H-index: 21).

## Cristina Corbetta



Cristina Corbetta is a PhD student in Translational and Molecular Medicine (DIMET) at the University of Milano – Bicocca, working at FINCB in the Laboratory of Immunotherapy of Brain Tumor, Unit of Molecular Neuro-Oncology. She graduated in Biology at University of Milano – Bicocca in 2014 and, in the same year, she started her post-lauream scientific training at the Unit of Molecular Neuro-Oncology (FINCB), by attending the AmadeoLab.

She collaborated to the in vivo experiments, in particular to set up a protocol for peptide-based vaccinations in a murine model of IDH1 mutated glioma. In 2014, she was awarded an annual AIRC fellowship, focused on understanding of the mechanism involved in glutamate release by glioblastoma

(GBM). Her research is primarily concerned with brain cancer immunotherapy, particularly she is working on characterizing the immunological response of GBM patients enrolled in clinical studies based on dendritic cell (DC)-based immunotherapy, in order to evaluate whether this treatment is able to activate specific immune effector cells involved in a long-term response and in a clinical benefit. Moreover, she is also investigating alternative strategies of immunotherapy based on chimeric antigen receptor T cells and tumor infiltrating lymphocytes derived from GBM specimens. She is author of 4 publication on peer-reviewed journals (H-index: 2).

## Michela Tinelli



Dr. Tinelli is currently Assistant Professorial Research Fellow at the London School of Economics, Department of Health Policy, Personal Social Services Research Unit and Senior Associate Researcher at the LSE Consulting. She acts also as academic expert for the European Brain Council and she is member of the Scientific Committee of the International Institute for Compassionate Care where she leads the health economics and cost-effectiveness research stream. Her research is primarily concerned with the socioeconomic burden of chronic diseases (including brain disorders), the development and investigation of the benefits of person centered approaches in social and health care. She is also interested in developing person-relevant outcome measures (PROMs) and their use to inform policy and practice decision making. She has conducted research on various social and health care issues relating to cross-border care in Europe and person-centered care services for chronic disease management in UK and internationally. As quantitative researcher, she has specialist skills in discrete choice experiments and other benefit evaluations in health policy developments, including clinical outcomes, health related quality of life, and patient satisfaction, which she has applied in benefit and economic evaluations. She has also expertise in survey development and design. She is author of 24 publication on peer-reviewed journals (H-index: 9).

## Alberto Raggi



Dr. Raggi is employed as senior researcher at FINCB Neurology, Public Health and Disability Unit since 2004. He graduated in Psychology at the Catholic University of Milan in 2003, obtained a post-degree specialization in Systemic-Relational Psychotherapy in 2010 and a PhD in Psychology, Health and Statistical Sciences at the University of Pavia in 2017. He is CO-PI in a recent MoH research grant and was awarded the “Informa Best Review Paper” in 2011 for the review “Systematic literature review on ICF from 2001 to 2009: its use, implementation and operationalization. *Disabil Rehabil.* 2011;33:281-309”. He is Member of the editorial board of the following: *International Journal of Rehabilitation Research*; *Biomed Research International*; *Scientific World Journal*. Member of the Besta Institute committee on the evaluation of clinical trials quality. His research interest deal with the evaluation of research outcomes with patient-reported outcome measures, including disability, quality of life and impact on work-related activities, development of outcome measures, evaluation of diseases’ burden and cost. He is author of 145 publication on peer-reviewed journals (H-index: 22).

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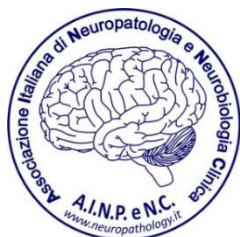


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