

## PERSONAL INFORMATION

Paola Baiardi

## WORK EXPERIENCE

January 2013–Present

**Coordinator Scientific Direction**

Istituti Clinici Scientifici Maugeri IRCCS (Italy)

Management and supervision of scientific activities. Evaluation of research projects and proposals. Scientific and methodological support for drafting research proposals and papers. Member of the Institutional Review Board and of the local Ethical Committee.

January 2001–December 2012

**Coordinator of cooperative research projects**

Salvatore Maugeri Foundation (Italy)

Workpackage leader and coordinator of European and National projects. Planning and management of clinical trials with focus on paediatrics and rare diseases. Drafting of statistical analysis plans. Statistician in charge for paediatric trials. Realization and management of disease and drug registries (rare diseases). Others: post marketing surveillance measures, pharmacoepidemiological studies, regulatory issues

January 1992–December 2000

**Biostatistician**

Salvatore Maugeri Foundation (Italy)

Planning and management of clinical studies. Survival analysis, Meta-analysis, Psychometric Techniques. Preclinical research (toxicology and pharmacology). Decision support systems (neural networks)

April 1987–December 1991

**Research Assistant**

Salvatore Maugeri Foundation (Italy)

Experience in DBMS (DataBase Management System), planning and carrying out of clinical managerial procedures (particularly in oncology). Design and modelisation (object-oriented approach) of an clinical information system

## EDUCATION AND TRAINING

January 1997–July 2000

**Specialist in Biostatistics and Epidemiology**

University of Milan (Italy)

Biostatistics - planning and management of clinical studies - data analysis  
Epidemiology - design of clinical and drug utilisation studies - data analysis

November 1982–March 1987

**Degree in Mathematics**

University of Pavia (Italy)

Principles of mathematics - Applied mathematics - Probability Theory - Informatics

## ADDITIONAL INFORMATION

**Expertise**

As biostatistician with wide experience in healthcare, she focused on methodological aspects related to the conduction of clinical trials in small populations (rare diseases, paediatrics, population subgroups), including the implementation of innovative study designs as well as new approaches to achieve scientific evidence in these niche contexts (modeling and simulation techniques, extrapolation of efficacy data from adults to children). Her remarkable experience is also related to the planning and management of clinical trials in her fields of interest. She has also wide experience in regulatory issues at the National and European level.

Main research areas are:

- 1) Methodology for clinical trials in small populations, with special attention to trial designs aimed at increasing acceptability while limiting sample size. Applications in case of paediatrics and rare diseases: she has been in charge of the planning and management of early phase trials based on Bayesian sequential designs and of phase III trials in chronic diseases based on innovative designs (randomized-withdrawal design). Use of modelling and simulation techniques and extrapolation approaches in paediatric trials carried out within the framework of FP7 funded trials for off-patent drugs;
- 2) Planning and management of clinical trials and observational (cohort) studies. Main area of interest: oncology, nephrology, cancer rehabilitation and rheumatology. Her experience is mainly in the planning and reporting of phase II (Simon's designs) and phase III trials;
- 3) Pharmacoepidemiological evaluations aimed at assessing drug usage and safety. She has performed studies of drug utilization in children (respiratory drugs) and methodological studies aimed at collecting adverse drug reaction in the paediatric population;
- 4) Drug post marketing surveillance, mainly oriented to the realization of nationwide disease registers with focus on small populations (children and rare diseases). Thanks to her collaboration with patient associations, she has to his credit extensive knowledge and experience in thalassemia and children affected by rare diseases.

As Coordinator of the Scientific Direction of a Research Hospital she has developed skills in management of clinical research, covering all the aspects related to the identification and management of personnel and resources needed to carry out the researches as well as the supervision and monitoring of the scientific activities.

#### Publications

Author/co-author of more than 150 publications at national and international level (H-Index: 29). The most recent and/or relevant ones are listed below:

- 1) Ranucci G, Buccigrossi V, Borgia E, Piacentini D, Visentin F, Cantarutti L, Baiardi P, Felisi M, Spagnuolo MI, Zanconato S, Baraldi E, Giaquinto C, Guarino A. Galacto-Oligosaccharide/Polidextrose Enriched Formula Protects against Respiratory Infections in Infants at High Risk of Atopy: A Randomized Clinical Trial. *Nutrients*. 2018 Mar 1;10(3).
- 2) Vandermeer B, van der Tweel I, Jansen-van der Weide MC, Weinreich SS, Contopoulos-Ioannidis DG, Bassler D, Fernandes RM, Askie L, Saloojee H, Baiardi P, Ellenberg SS, van der Lee JH. Comparison of nuisance parameters in pediatric versus adult randomized trials: a meta-epidemiologic empirical evaluation. *BMC Med Res Methodol*. 2018 Jan 10;18(1):7.
- 3) Esposito V, Mazzon G, Baiardi P, Torreggiani M, Semeraro L, Catucci D, Colucci M, Mariotto A, Grosjean F, Bovio G, Esposito C. Safety and adequacy of percutaneous kidney biopsy performed by nephrology trainees. *BMC Nephrol*. 2018 Jan 15;19(1):14.
- 4) Gialanella B, Prometti P, Monguzzi V, Ferlucchi C, Baiardi P, Comini L. Determinants of functional outcome in hip fracture: the role of comorbidity. *Aging Clin Exp Res*. 2018 Jun;30(6):643-650.
- 5) Bonifazi F, Conte R, Baiardi P, Bonifazi D, Felisi M, Giordano P, Giannuzzi V, Iacono A, Padula R, Pepe A, Caterina Putti M, Ruggieri L, Carlo Del Vecchio G, Filosa A, Maggio A, Ceci A; HTA-THAL Multiregional Registry. Pattern of complications and burden of disease in patients affected by beta thalassemia major. *Curr Med Res Opin*. 2017 Aug;33(8):1525-1533.
- 6) Giannuzzi V, Conte R, Landi A, Ottomano SA, Bonifazi D, Baiardi P, Bonifazi F, Ceci A. Orphan medicinal products in Europe and United States to cover needs of patients with rare diseases: an increased common effort is to be foreseen. *Orphanet J Rare Dis*. 2017 Apr 3;12(1):64.
- 7) Balbi B, Vallese D, Chavannes N, Stallberg B, Baiardi P. General practitioners and rare lung diseases: a task force for the development of rare lung diseases educational material. *Breathe (Sheff)*. 2016 Dec;12(4):341-348.
- 8) Masnaghetti SE, Sarzi Braga S, Vaninetti R, Baiardi P, Pedretti RF. Altered fasting glycemia in cardiac patients during in-hospital rehabilitation: impact on short and long-term follow-up. *J Cardiovasc Med (Hagerstown)*. 2016 Oct 15.
- 9) Smania G, Baiardi P, Ceci A, Cella M, Magni P. Model-Based Assessment of Alternative Study Designs in Pediatric Trials. Part II: Bayesian Approaches. *CPT Pharmacometrics Syst Pharmacol*. 2016 Aug;5(8):402-10.
- 10) Raglio A, Giovanazzi E, Pain D, Baiardi P, Imbriani C, Imbriani M, Mora G. Active music therapy approach in amyotrophic lateral sclerosis: a randomized-controlled trial. *Int J Rehabil Res*. 2016 Dec;39(4):365-367.
- 11) Smania G, Baiardi P, Ceci A, Magni P, Cella M. Model-Based Assessment of Alternative Study Designs in Pediatric Trials. Part I: Frequentist Approaches. *CPT Pharmacometrics Syst Pharmacol*. 2016 Jun;5(6):305-12.

- 12) Vitacca M, Paneroni M, Baiardi P, De Carolis V, Zampogna E, Belli S, Carone M, Spanevello A, Balbi B, Bertolotti G. Development of a Barthel Index based on dyspnea for patients with respiratory diseases. *Int J Chron Obstruct Pulmon Dis*. 2016 Jun 7;11:1199-206.
- 13) Mazzanti A, Maragna R, Faragli A, Monteforte N, Bloise R, Memmi M, Novelli V, Baiardi P, Bagnardi V, Etheridge SP, Napolitano C, Priori SG. Gene-Specific Therapy With Mexiletine Reduces Arrhythmic Events in Patients With Long QT Syndrome Type 3. *J Am Coll Cardiol*. 2016 Mar 8;67(9):1053-8.
- 14) Pasini E, Aquilani R, Testa C, Baiardi P, Angioletti S, Boschi F, Verri M, Dioguardi F. Pathogenic Gut Flora in Patients With Chronic Heart Failure. *JACC Heart Fail*. 2016 Mar;4(3):220-7.
- 15) Carone M, Antoniu S, Baiardi P, Digilio VS, Jones PW, Bertolotti G; QuESS Group. Predictors of Mortality in Patients with COPD and Chronic Respiratory Failure: The Quality-of-Life Evaluation and Survival Study (QuESS): A Three-Year Study. *COPD*. 2016;13(2):130-8.
- 16) Aquilani R, Emilio B, Dossena M, Baiardi P, Testa A, Boschi F, Viglio S, Iadarola P, Pasini E, Verri M. Correlation of deglutition in subacute ischemic stroke patients with peripheral blood adaptive immunity: Essential amino acid improvement. *Int J Immunopathol Pharmacol*. 2015 Dec; 28(4):576-83.
- 17) Raglio A, Bellandi D, Baiardi P, Gianotti M, Ubezio MC, Zancchi E, Granieri E, Imbriani M, Stramba-Badiale M. Effect of Active Music Therapy and Individualized Listening to Music on dementia: A Multicenter Randomized Controlled Trial. *J Am Geriatr Soc*. 2015 Aug; 63(8):1534-9.
- 18) Ruggieri L, Giannuzzi V, Baiardi P, Bonifazi F, Davies EH, Giaquinto C, Bonifazi D, Felisi M, Chiron C, Pressler R, Rabe H, Whitaker MJ, Neubert A, Jacqz-Aigrain E, Eichler I, Turner MA, Ceci A; GRiP Consortium. Successful private-public funding of paediatric medicines research: lessons from the EU programme to fund research into off-patent medicines. *Eur J Pediatr*. 2015 Apr;174(4):481-91.
- 19) Denegri M, Bongianino R, Lodola F, Boncompagni S, De Giusti VC, Avelino-Cruz JE, Liu N, Persampieri S, Curcio A, Esposito F, Pietrangelo L, Marty I, Villani L, Moyaho A, Baiardi P, Auricchio A, Protasi F, Napolitano C, Priori SG. Single Delivery of an Adeno-Associated Viral Construct to Transfer the CASQ2 Gene to Knock-In Mice Affected by Catecholaminergic Polymorphic Ventricular Tachycardia Is Able to Cure the Disease From Birth to Advanced Age. *Circulation*. 2014 Jun 24;129(25):2673-81.
- 20) Saviola G, Abdi-Ali L, Camprostrini L, Sacco S, Baiardi P, Manfredi M, Benucci M, Bucci M, Cirino G. Clarithromycin in rheumatoid arthritis: the addition to methotrexate and low-dose methylprednisolone induces a significant additive value—a 24-month single-blind pilot study. *Rheumatol Int*. 2013 Nov; 33(11):2833-8.
- 21) Catalano O, Moro G, Perotti M, Frascaroli M, Ceresa M, Antonaci S, Baiardi P, Napolitano C, Baldi M, Priori SG. Late gadolinium enhancement by cardiovascular magnetic resonance is complementary to left ventricle ejection fraction in predicting prognosis of patients with stable coronary artery disease. *J Cardiovasc Magn Reson*. 2012 May 19;14:29.
- 22) Bianchi C, Baiardi P, Khirani S, Cantarella G. Cough peak flow as a predictor of pulmonary morbidity in patients with dysphagia. *Am J Phys Med Rehabil*. 2012 Sep;91(9):783-8.
- 23) Monticone M, Baiardi P, Bonetti F, Ferrari S, Foti C, Pillastrini P, Rocca B, Vanti C, Zanoli G. The Italian version of the Fear-Avoidance Beliefs Questionnaire (FABQ-I): cross-cultural adaptation, factor analysis, reliability, validity, and sensitivity to change. *Spine (Phila Pa 1976)*. 2012 Mar 15;37(6):E374-80.
- 24) Fregoni V, Regolo L, Da Prada GA, Zambelli A, Baiardi P, Zanini V, Villani L, Pavesi L, Riccardi A. No correlation between plasma D-dimer levels and lymph node involvement in operable breast cancer. *Breast*. 2012 Apr;21(2):220.
- 25) Monticone M, Baiardi P, Vanti C, Ferrari S, Pillastrini P, Mugnai R, Foti C. Responsiveness of the Oswestry Disability Index and the Roland Morris Disability Questionnaire in Italian subjects with subacute and chronic low back pain. *Eur Spine J*. 2012 Jan;21(1):122-9.
- 26) Altavilla A, Manfredi C, Baiardi P, Dehlinger-Kremer M, Galletti P, Pozuelo AA, Chaplin J, Ceci A. Impact of the new European paediatric regulatory framework on ethics committees: overview and perspectives. *Acta Paediatr*. 2012 Jan;101(1).
- 27) Corda L, Medicina D, La Piana GE, Bertella E, Moretti G, Bianchi L, Pinelli V, Savoldi G, Baiardi P, Facchetti F, Gatta N, Annesi-Maesano I, Balbi B. Population genetic screening for alpha1-antitrypsin deficiency in a high-prevalence area. *Respiration*. 2011;82(5):418-25.
- 28) Baiardi P, Giaquinto C, Giroto S, Manfredi C, Ceci A; TEDDY Network of Excellence. Innovative study design for paediatric clinical trials. *Eur J Clin Pharmacol*. 2011 May;67 Suppl 1:109-15.
- 29) Poggi G, Pozzi E, Riccardi A, Tonini S, Montagna B, Quaretti P, Tagliaferri B, Sottotetti F, Baiardi P, Pagella C, Minoia C, Bernardo G. Complications of image-guided transcatheter hepatic

chemoembolization of primary and secondary tumours of the liver. Anticancer Res. 2010

30) Baiardi P, Ceci A, Felisi M, Cantarutti L, Giroto S, Sturkenboom M, Baraldi E. In-label and off-label use of respiratory drugs in the Italian paediatric population. Acta Paediatr. 2010 Apr;99(4):544-9.

**Projects** She has been involved in several research projects within the European Community and the Italian Ministry of Health. In details:

1) Coordinator of the EU project “EuOrphan - Service for the support of the European Orphan medicine market”, an e-TEN project granted by DG Information Society (2005-2006) aimed at realising a web-based database of designated and commercialised orphan drugs worldwide.

2) Participation (WP leader) in the TEDDY (Task-Force in Europe for Drug Development for the Young) project, a FP6 Network of Excellence (2005-2010) aimed at better structuring European research in paediatrics by supporting the rational use of drugs in children and developing collaborations among the different stakeholders. She coordinated the activities of the following workpackages: a) “Paediatric Clinical Studies”, dealing with methodological issues in paediatric research ; b) “European Pediatric Medicines Database” for the collection of studies (registrative and spontaneous) supporting the use of drugs in children.

3) Participation (WP leader) in the GRIP (Global Research In Paediatrics) project, FP7 Network of Excellence (2011-2016). GRIP is a methodological research network that encompasses researchers, regulatory agencies (EMA, FDA, Japan) and WHO whose aim is to optimize all scientific and regulatory processes for paediatric drug development. GRIP has also a strong educational mission on paediatric matter. She is in charge of the workpackage “Methodology for Clinical Trials in Paediatrics” including 5 working groups (Modelling & Simulation, Extrapolation, Innovative Design, Sample Size, Comparator). She is also a teacher of the Module “Experimental Drug Evaluation in Paediatrics” for the aspects related to paediatric research methodology.

4) Participation (WP leader) in the FP7 DEEP (DEferiprone Evaluation in Paediatrics) project (2011-2014), aimed at completing the development of deferiprone chelator for its use in children in accordance with the 2009 PDCO Priority List for off-patent drugs. She is the leader of the workpackage for the planning and management of the efficacy and safety study of deferiprone in children affected by hereditary haemoglobinopathies.

At the national level she has been WP leader (2007-2011) within the project “Safety of psychotropic drugs in the Italian paediatric population”, granted by the Italian Medicines Agency (AIFA) where she coordinated an observational prospective cohort study aimed at assessing the safety of psychotropic drugs in Italy based on prescription databases.

Moreover, she participated (2007-2011) into the project “HTA-Thal: HTA evaluations of iron overloaded patients” granted by the Italian Ministry of Health, ex art.12, where she was in charge of the planning and realization of the Italian Registry of thalassaemic patients.

Finally, she is currently exploiting her expertise in the supervision of the research projects, clinical trials and other scientific activities of her Institute.

**Memberships** She is currently a member of the Paediatric Committee at the EMA (position of alternate) as representative of patient associations (Leonardo Giambone Foundation) where she is actively contributing to the activities of the Committee.

She has been (2008-2012) a member of the Paediatric Working Group at the Italian Medicines Agency (AIFA) where she provided her contribution both at the scientific and regulatory level. In details she acted as: 1) Assessor named by AIFA for CMDh Worksharing Procedures in compliance with Art. 45/46 of the Paediatric Regulation (EC) No 1901/2006 and amendments; 2) Internal reference expert for evaluation of paediatric drugs used off-label in Italy finalized to their inclusion in the Italian list of special use of drugs under the Act 648/96.

Other current memberships are:

- 1) Member ex-officio of the Ethic Committee of the Istituti Clinici Scientifici Maugeri IRCCS
- 2) Member of the Scientific Committee of Gianni Benzi Foundation
- 3) Member of the Scientific Committee of the Italian Foundation “Leonardo Giambone” for the Cure of Thalassemia

**Other Relevant Information** Teaching experience:

- 1) Contract Professor at Postgraduate School in Regulatory Disciplines, University of Pavia, Italy (years 2001 - present)

- 2) Contract Professor at Master I level in Pharmaceutical Management and Health Policy, University of Bologna, Italy (years 2003-2007)
- 3) Contract Professor at Postgraduate School in Health Psychology, University of Torino, Italy (course: Methodology for Psychosocial Research) (years 1998 - 2002)
- 4) Contract Professor at Postgraduate School in Medical Pharmacology, University of Pavia, Italy (course: Pharmacological Experimental Design) (year 1999)