

PERSONAL INFORMATION **Armando Magrelli**WORK EXPERIENCE

- October 2018- Present **Vice-Chair of Committee Orphan Medicinal Products**
European Medicine Agency (Netherlands)
- February 2018- Present **adjunct Professor**
University of Rome Tor Vergata (Italy)
adjunct Professor for European Pharmaceutical Legislation at the Faculty of Pharmacy of the University of Rome Tor Vergata
- March 2013- Present **Member of Scientific Advice Working Party**
European Medicines Agency (United Kingdom)
The Scientific Advice Working Party (SAWP) is a standing working party with the sole remit of providing [scientific advice and protocol assistance](http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000049.jsp&mid=WC0b01ac05800229b9). It was established by the [Committee for Medicinal Products for Human Use](http://www.ema.europa.eu/ema/index.jsp?curl=pages/about_us/general/general_content_000094.jsp&mid=WC0b01ac0580028c79) (CHMP). For human medicines, scientific advice and protocol assistance are given by the Committee for Medicinal Products for Human Use (CHMP) on the recommendation of the Scientific Advice Working Party (SAWP). The SAWP coordinates the provision of [scientific advice and protocol assistance](http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000049.jsp&mid=WC0b01ac05800229b9). It brings an integrated view forward to the CHMP and the COMP on:
 - quality relating to the development of medicinal products;
 - non-clinical and clinical safety and efficacy relating to the development of medicinal products;
 - the significant benefit of orphan medicinal products.
- May 2012- Present **Member of Committee Orphan Medicinal Products**
European Medicines Agency (United Kingdom)
Examine application for the designation of a medicinal product as an orphan medicinal product which is submitted to it in accordance with the Regulation. The following represent the priority tasks of the Committee: Designation Protocol Assistance Marketing Authorisation (Review of Criteria for Maintenance of Orphan Status i.e. prevalence, seriousness, significant benefit when applicable).
- July 2008- Present **Researcher**
Istituto Superiore di Sanità (Italy)
Studying the role of microRNA in rare diseases; Rare diseases Epidemiology; Hepatoblastoma; Multiple Exostosis; Hailey-Hailey; Rare Diseases and Oxidative stress; Bioinformatics;
- January 2006-June 2008 **Senior consultant in bioinformatics**
Rome Oncogenomic Center -Istituti Fisioterapici Ospitalieri Centro Ricerca Sperimentale Istituto Regina Elena Rome (Italy)

Linking transcriptome to proteome: functional oncogenomics for diagnosis and treatment of human cancers. Develop new bioinformatic approaches in microRNAs predictions and data analysis; we developed a software freely available to the scientific community (<http://www.macinolab.org/Genemir.htm>), able to merge different sources on microRNAs. We also applied this tool in our experimental approaches on CLL and other pathologies (hepatoblastomas etc.).

January 2005-January 2006

Senior Consultant in Bioinformatics and Data Analysis

University of Rome La Sapienza (Italy)

New Bioinformatic approach to study microRNAs, and their targets; System Biology of high throughput data from microRNA;

April 2004-December 2007

Computer systems analyst

Con Soluzioni Con Srl (Italy)

Main activities were on -System Administration on Windows, Macintosh and Unix environment. -Internet environment. -HTML Web Browser scripts. -LAN and WiFi networks planning and management. -Web design and web publishing. -Web Administration. -Sql Server management. -Microsoft Transact Sql 2003. -Visual Basic. -Microsoft Visual Studio Net. -Software Packages for Web publishing and editing (Macromedia, PHP, Java). -Perl, BioPerl, Perl DBI.

April 1999-April 2004

Researcher

National Council of Research (Italy)

The creation of an animal model, able to show the existence of the RNA Interference phenomena in higher organisms: in other words the presence of a post-transcriptional mechanism that can abolish gene expression of every mRNA, leaving intact the genome it derives from. Such project has been carried on in collaboration with Prof. Klaus Rajewsky, Head of EMBL Mouse Program, obtaining two transgenic mouse lines to be used as an experimental model. -The study of High Throughput Screening techniques and setup of a specific lab for genome wide analysis. To this purpose I attended an International post lauream course held by EMBL at Heidelberg (Germany), focused on microarrays and high throughput screening techniques. -The in vivo and in vitro characterization of a gene involved in the Juvenile Parkinson, with the relative production of a specific animal model for the study of this pathology

June 1996-March 1999

Senior Researcher

Idi Farmaceutici Spa (Italy)

I have been put in charge of the setup and startup of the Molecular Biology Lab. Then, since the experimental model used in the Lab was made of primary cultures of human keratinocytes able to produce functional skin, one of the main issues, and the focus of my work, was the identification of molecular markers for epidermal stem cells, with the aim to make more effective the in vitro cell culture, for a subsequent transplant on patients with different pathologies such as bad burnt. Furthermore, as human keratinocytes represent a perfect target for ex-vivo gene therapy, I was involved in a project on the gene transduction in cells derived from patient suffering for epidermolysis bollosa.

October 1992-October 1993

Professional Consultant

University of Rome La Sapienza (Italy)

Involved in the National Research Program on Advanced Biotechnologies. Regarding signal transduction of blue light in fungi.

EDUCATION AND TRAINING

November 2015-November 2015

Invited Speaker

Istituto Ortopedico Rizzoli (Italy)

- February 2014-February 2014 **Invited Speaker**
Uniamo Federazione Italiana Malattie Rare ()
- November 2012-November 2012 **Invited Speaker**
Ospedale Pediatrico Bambino Gesù IRCCS (Italy)
- May 2012-May 2012 **Invited Speaker**
Associazione Amici di Magù Diversamente Genitori Onlus (Italy)
- October 2009-October 2009 **Participant**
Istituto Superiore di Sanità (Italy)
- February 2009-February 2009 **Participant**
Istituto Superiore di Sanità (Italy)
- July 2008-November 2008 **Participant**
Società Italiana di Genetica Umana (Italy)
- October 2008-October 2008 **Participant**
Istituto Superiore di Sanità (Italy)
- October 2008-October 2008 **Invited Speaker**
Istituto Superiore di Sanità (Italy)
- December 2005-July 2008 **training in biotechnological Patents**
Studio Ferrario (Italy)
- November 2006-November 2006 **Bioinformatic Instructor**
Rome Oncogenomic Center (Italy)
- October 2005-October 2005 **Programming in Perl**
CINECA (Italy)
- July 2005-August 2005 **Visiting Scientist**
New York University (United States)
- March 2005-March 2005 **Invited Speaker**
Centro Ricerca Sperimentale, Istituto Regina Elena (Italy)

March 2004-April 2004	Certificate of attendance Con Soluzioni Con Srl (Italy)
February 2004-March 2004	Certificate of attendance Con Soluzioni Con Srl (Italy)
June 2003-June 2003	Certificate of attendance Con Soluzioni Con Srl (Italy)
June 2003-June 2003	Certificate of attendance Con Soluzioni Con Srl (Italy)
April 2001-May 2001	Visiting Scientist European Molecular Biology Laboratory (Germany)
November 2000-December 2000	Certificate of attendance European Molecular Biology Laboratory (Germany)
June 2000-June 2000	First Certificate in English University of Cambridge ()
April 1999-April 1999	Certificate of attendance International Centre for Genetic Engineering and Biotechnology (Italy)
October 1993-June 1997	PhD in Molecular Biology Max-Planck-Institute for Plant Breeding Research (Germany) Involved in a research project on generation of plants with dwarf phenotype following the infection with Agrobacterium rhizogenes. I achieved knowledge about plants genetic and molecular biology applied to transgenesis and chimerism. In the same period I took the chance to specialize, working at the local Data Elaboration Office, on the management and maintenance of UNIX based networks
December 1989-October 1992	Graduated M.Sc. in Biology University of Rome La Sapienza (Italy)

ADDITIONAL INFORMATION

Expertise

Publications

A.Magrelli, K.Langenkemper, K. Dehio, J.Schell and A. Spena. "Splicing of rol A transcript of Agrobacterium rhizogenes in Arabidopsis;" (1994) Science, 266, 1986-1988.

P. Ballario, P. Vittorioso, A. Magrelli, C. Talora, A. Cabibbo and G. Macino. "White collar-1, a central regulator of blue light responses in Neurospora, is a zinc finger protein." (1996) EMBO-Journal.

15(7),1650-1657.

D. Marazziti, E. Golini, A. Magrelli, R. Matteoni and G.P. Tocchini-Valentini. "Genomic Analysis of GPR37 and Related Orphan G-Protein Coupled Receptor Genes Highly Expressed in the Mammalian Brain". (2001) *Current Genomics*, 2 (3), 253-260 .

Daniela Marazziti, Elisabetta Golini, Silvia Mandillo, Armando Magrelli, Walter Witke, Rafaele Matteoni, and Glauco P. Tocchini-Valentini Altered dopamine signaling and MPTP resistance in mice lacking the Parkinson's disease-associated GPR37/parkin-associated endothelin-like receptor *PNAS* 2004 101: 10189-10194.

Fulci V, Chiaretti S, Goldoni M, Azzalin G, Carucci N, Tavolaro S, Castellano L, Magrelli A, Citarella F, Messina M, Maggio R, Peragine N, Santangelo S, Mauro FR, Landgraf P, Tuschl T, Weir DB, Chien M, Russo JJ, Ju J, Sheridan R, Sander C, Zavolan M, Guarini A, Foa R, Macino G. Quantitative technologies establish a novel microRNA profile of chronic lymphocytic leukemia. *Blood*. 2007 Feb 27;

Armando Magrelli, Gianluca Azzalin, Marco Salvatore, Mara Viganotti, Fabrizio Tosto, Teresa Colombo, Rita Devito, Alessandra Di Masi, Antonio Antoccia, Stefano Lorenzetti, Francesca Maranghi, Alberto Mantovani, Caterina Tanzarella, Giuseppe Macino, Domenica Taruscio . Altered microRNA Expression Patterns in Hepatoblastoma Patients. *Transl Oncol* 2009 Aug 18;2(3):157-63.

Elisabetta Caprini, Cristina Cristofolletti, Diego Arcelli, Paolo Fadda, Mauro Helmer Citterich, Francesca Sampogna, Armando Magrelli, Federica Censi, Paola Torreri, Marina Frontani, Enrico Scala, Maria Cristina Picchio, Paola Temperani, Alessandro Monopoli, Giuseppe Alfonso Lombardo, Domenica Taruscio, Maria Grazia Narducci, Giandomenico Russo (2009) Identification of key regions and genes important in the pathogenesis of Sezary syndrome by combining genomic and expression microarrays. *Cancer Res*. 2009 Nov 1;69(21):8438-46.

Cialfi, Oliviero, Ceccarelli, Marchese, Barbieri, Biolcati, Uccelletti, Palleschi, Barboni, De Bernardo, Grammatico, Magrelli, Salvatore, Taruscio, Frati, Gulino, Screpanti, Talora Complex multipathways alterations and oxidative stress are associated with Hailey-Hailey disease. *Br J Dermatol*. 2010 Mar;162(3):518-26.

Maria Grazia Di Certo, Nicoletta Corbi, Georgios Strimpakos, Annalisa Onori, Siro Luvisetto, Cinzia Severini, Angelo Guglielmotti, Enrico Maria Batassa, Cinzia Pisani, Aristide Floridi, Barbara Benassi, Maurizio Fanciulli, Armando Magrelli, Elisabetta Mattei, Claudio Passananti. The artificial gene *Jazz*, a transcriptional regulator of *Utrophin*, corrects the dystrophic pathology in *mdx* mice. *Hum Mol Genet*. 2010 Mar 1;19(5):752-60.

Maranghi F, Lorenzetti S, Tassinari R, Moracci G, Tassinari V, Marcoccia D, Di Virgilio A, Eusepi A, Romeo A, Magrelli A, Salvatore M, Tosto F, Viganotti M, Antoccia A, Di Masi A, Azzalin G, Tanzarella C, Macino G, Taruscio D, Mantovani A. In utero exposure to di-(2-ethylhexyl) phthalate affects liver morphology and metabolism in post-natal CD-1 mice. *Reprod Toxicol*. 2010 Jul;29(4):427-32.

Zuntini M, Salvatore M, Pedrini E, Parra A, Sgariglia F, Magrelli A, Taruscio D, Sangiorgi L. MicroRNA profiling of multiple osteochondromas: identification of disease-specific and normal cartilage signatures. *Clin Genet*. 2010 Jun 23.

A. Di Masi, M. Viganotti, A. Antoccia, A. Magrelli, M. Salvatore, et al. Characterization of HuH6, Hep3B, HepG2 and HLE liver cancer cell lines by beta-catenin pathway, microRNA expression and protein expression profile. *Cell Mol Biol*, 2010 10:1299-317

Salvatore M, Magrelli A, Taruscio D. The Role of microRNAs in the Biology of Rare Diseases. *Int J Mol Sci*. 2011;12(10):6733-42.

Manca S, Magrelli A, Cialfi S, Lefort K, Ambra R, Alimandi M, Biolcati G, Uccelletti D, Palleschi C, Screpanti I, Candi E, Melino G, Salvatore M, Taruscio D, Talora C. Oxidative stress activation of miR-125b is part of the molecular switch for Hailey-Hailey disease manifestation. *Exp Dermatol*. 2011 Nov;20(11):932-7.

Domenica Taruscio , Marco Salvatore , Armando Magrelli , Rosella Tomanin Predictive medicine and biomarkers: the case of rare diseases *Personalized Medicine*, March 2012, Vol. 9, No. 2, Pages 143-146.

D Taruscio, L Agresta, A Amato, G Bernardo, L Bernardo, F Braguti, et al. The Italian National Centre for Rare Diseases: where research and public health translate into action. *Blood transfusion* 12 (Suppl 3), s591-605.

Valeria De Arcangelis, Georgios Strimpakos, Francesca Gabanella, Nicoletta Corbi, Siro Luvisetto, Armando Magrelli et al. "Pathways Implicated in Tadalafil Amelioration of Duchenne Muscular Dystrophy" *Journal of cellular physiology*, 2015 DOI: 10.1002/jcp.25075

Marco Salvatore, Armando Magrelli, Valentini Flamini, Andrea Brunati, Eleonora Basso, Renato Romagnoli, Ezio David and Domenica Taruscio. "Hepatoblastoma and microRNA-483 Two Forms and One Outcome". *J Genet Syndr Gene Ther* 2016, 7: 278

Fregonese L, Greene L, Hofer M, Magrelli A, Naumann-Winter F, Larsson K, Sheean M, Stoyanova-Beninska V, Tsigkos S, Westermark K, Sepodes B.

Demonstrating significant benefit of orphan medicines: analysis of 15 years of experience in Europe. *Drug Discov Today*. 2017 Oct 9. pii: S1359-6446(17)30159-9. doi: 10.1016/j.drudis.2017.09.010.

Sheean ME, Stoyanova-Beninska V, Capovilla G, Duarte D, Hofer MP, Hoffmann M, Magrelli A, Mariz S, Tsigkos S, Shaili E, Polsinelli B, Ricciardi M, Bonelli M, Balabanov P, Larsson K, Sepodes B.

Nonclinical data supporting orphan medicinal product designations: lessons from rare neurological conditions. *Drug Discov Today*. 2017 Oct 4. pii: S1359-6446(17)30289-1. doi: 10.1016/j.drudis.2017.09.015.

Defining orphan conditions in the context of the European orphan regulation: challenges and evolution

DJ OConnor, ME Sheean, MP Hofer, S Tsigkos, S Mariz, L Fregonese, ...

Nature Reviews Drug Discovery 18 (7), 479-480

European regulators views on a wearable-derived performance measurement of ambulation for Duchenne muscular dystrophy regulatory trials

M Haberkamp, J Moseley, D Athanasiou, F de Andres-Trelles, A Elferink, et al.

Neuromuscular Disorders 29 (7), 514-516

Nonclinical data supporting orphan medicinal product designations in the area of rare infectious diseases ME Sheean, E Malikova, D Duarte, G Capovilla, L Fregonese, M Hofer, et al *Drug discovery today* <https://doi.org/10.1016/j.drudis.2019.10.015>

Using a stated preference discrete choice experiment to assess societal value from the perspective of patients with rare diseases in Italy J López-Bastida, JM Ramos-Goñi, I Aranda-Reneo, D Taruscio, et al. *Orphanet journal of rare diseases* 14 (1), 154
<https://doi.org/10.1186/s13023-019-1126-1>

Projects

Memberships

- Member of the Significant Benefit working group of the COMP at EMA (2015)- Member of the Pre-clinical working group of the COMP at EMA (2015)- Member of the working group COMP and PDCO of the EMA (2013)
- Member of the working group for the EU presidency for the organization of the Joint informal meeting PDCO and COMP Organised by Italian Medicine Agency and Ospedale Pediatrico Bambino Gesù di Roma (Italy) 2012
- Member of the Working Group for the preparation and management of the Italian Presidency of the European Union 2014. For issues relating to medicinal products for human use.- Member of The Science Advisory Board since 2010 (http://www.scienceboard.net/profile/86107)

Other Relevant Information