

CURRICULUM VITAE

Family name, First name: **Martelli, Maria Paola**

Researcher ID (ORCID): 0000-0001-9139-1729

Date of Birth: 18-7-68

Place of Birth: Perugia, Italy

Nationality: Italian

Family Status: married, with four children

Work address: CREO (Center for Research in Hemato-Oncology), Hematology and Clinical Immunology Section, Department of Medicine and Surgery, University of Perugia, P.le Menghini 8/9, 06132 Perugia, IT

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Expertise and Fields of interest

Diagnosis, care and treatment paths of malignant hematological diseases, particularly acute leukemias; Principal Investigator and Coinvestigator in clinical trials on acute leukemias; Phase-1 clinical experimentation and diagnostic laboratories; Clinical and translational research in AML; Biochemistry and functions of tumour-specific proteins (i.e. mutant NPM1, IDH1/IDH2, DNMT3A and FLT3; EML4/ALK; BRAFV600E); Pathology and functional genomics in AML; Drug development/targeted therapy in AML.

• EDUCATION AND KEY QUALIFICATIONS

- 1998 – 2001 - PhD in Biotechnologies in Human Bone Marrow Transplantation/Hematology
University of Perugia, Perugia, Italy/NHLBI, NIH, Bethesda, USA
PhD Supervisor: Dr. Barbara E. Bierer (NHLBI, NIH, Bethesda, USA)
- 1993 – 1997 - Specialty in Hematology (Honours Commendation)
“La Sapienza” University, Rome, Italy
- 1987 – 1993 - M.D. (Honours Commendation) University of Perugia, Italy

• CURRENT POSITIONS

- Since 1/10/24 - Director of Hematology Clinical Department, Hospital of Perugia
- Since 1/11/23 - Head of the Hematology and Clinical Immunology Section
Department of Medicine and Surgery, University of Perugia
- Coordinator of the PhD Course in ‘Clinical and Molecular Medicine’
Department of Medicine and Surgery, University of Perugia
- Director of Specialty School in Hematology, University of Perugia
- 2016 – to date - Associate Professor in Hematology
Department of Medicine and Surgery, University of Perugia

• PREVIOUS POSITIONS

- 2023 – 2024 - *Acting* Director of Hematology Clinical Department, Hospital of Perugia
- 2007 – 2023 - Assistant Clinical Director
- Assignment of High Specialization (Perugia Hospital): 'Hematology Department Clinical Activity and Study and Application of Diagnostic and Therapeutic Protocols in Acute Leukemia', Hematology/Perugia General Hospital, Perugia, Italy
- 2007 – 2016 - Assistant Professor/Researcher
Hematology/University of Perugia, Perugia, Italy
- 2005 – 2007 - Assistant Professor/Researcher
Hematology/University of Bari, Bari, Italy
- 2005 – 2007 - Assistant Clinical Director
Hematology/Bari General Hospital, Bari, Italy
- 2002 – 2004 - Assistant Clinical Director
Hematology/Umberto I General Hospital, Rome, Italy
- 1998 – 2001 - NIH Research Fellow, NIH, Bethesda, USA

RESEARCH ACHIEVEMENTS AND PEER RECOGNITION

The PI, a clinical scientist and researcher, has worked as a leader in a large research group, alongside prof. B. Falini, and has distinguished herself for her ability to conduct innovative translational research and provide new insights into methodology and concepts in AML, particularly in AML with *NPM1* mutations, since its discovery in 2005, having contributed to defining the biological, molecular and clinical features of this nosological entity. She has published **153 articles**, and two book chapters (**H-index 49**, [Scopus]), and has consistently proven attractive for external research funding. In 2016, she was awarded an **ERC Consolidator grant**, followed by an **ERC-PoC** grant in 2023. Contribution to science stems from the PI's interdisciplinary approach to understand the mechanisms that regulate leukemia development, identify unmet medical needs and influence patient management and treatment. This has made the PI a key leader in the Italian hematology network and an internationally recognized leader in translational and clinical research in AML. As academic with different leadership responsibilities, she has mentored multiple students, postdoctoral fellows at different levels, as well as contributed to the strategic leadership of her research and clinical center.

• PUBLICATIONS

PI's contribution to science with focus on *NPM1*-mutated AML is highlighted by the following representative publications, in all of which she had key role in the original conception, study design, principal experimental activities, writing, and editing:

1) Falini B, **Martelli MP**. Comparison of the International Consensus and 5th WHO edition classifications of adult myelodysplastic syndromes and acute myeloid leukemia. *Am J Hematol*. 2023;98(3):481-492 [IF: 9.9][Cit: 36] - **This paper highlights the PI's expertise in the field of myeloid neoplasms, particularly AML, in terms of definition of entities and classifications.**

2) Falini B, Brunetti L, Sportoletti P, **Martelli MP**. *NPM1*-mutated acute myeloid leukemia: from bench to bedside. *Blood* 2020;136:1707 [IF: 23.1][Cit: 229] - **This paper highlights the key contribution of the PI in research and clinical achievements in *NPM1*-mutated AML.**

3) **Martelli MP**, Rossi R, Venanzi A, et al. Martelli MP, Rossi R, Venanzi A, Meggendorfer M, Perriello VM, Martino G, Spinelli O, Ciurnelli R, Varasano E, Brunetti L, Ascani S, Quadalti C, Cardinali V, Mezzasoma F, Gionfriddo I, Milano F, Pacini R, Tabarrini A, Bigerna B, Albano F, Specchia G, Vetro C, Di Raimondo F, Annibali O, Avvisati G, Rambaldi A, Falzetti F, Tiacci E, Sportoletti P, Haferlach T, Haferlach C[^], Falini B[^]. (^co-last). Novel *NPM1* exon 5 mutations and gene fusions leading to aberrant cytoplasmic nucleophosmin in AML. *Blood* 2021;138:2696 [IF: 23.1][Cit: 42] - **This paper comes from the strategy of application of integrated diagnostic approaches to AML by the PI, leading to discovery of novel genetic alterations with clinical impact on diagnosis and prognostic stratification of AML patients. It stresses also the role of pathology and immunohistochemistry in diagnosis and biology understanding of AML with *NPM1* mutations (Falini et al. *Blood* 2006;108:1999; Falini et al. *Am J Hematol*. 2023;98:481).**

4) Falini B, Brunetti L, **Martelli MP**. Dactinomycin in *NPM1*-Mutated Acute Myeloid Leukemia. *N Engl J Med*. 2015;373:1180 [IF: 78.5][Cit: 63] - **This is the pivotal report on the clinical activity of an old drug (dactinomycin) that, based on biological novel concepts on *NPM1* mutant functions, we have 'repurposed' in AML, specifically in the genotype harbouring *NPM1* mutations, and then further investigated in clinical and mechanisms of action studies (leading to a patent and other relevant publications: WO2017046108A1; Gionfriddo et al. *Leukemia*. 2021;35(9):2552-2562; Wu et al. *Cancer Discov*. 2021;11(12):3198-3213)**

5) **Martelli MP**, Gionfriddo I, Mezzasoma F, Milano F, Pierangeli S, Mulas F, Pacini R, Tabarrini A, Pettirossi V, Rossi R, Vetro C, Brunetti L, Sportoletti P, Tiacci E, Di Raimondo F, Falini B. Arsenic trioxide and all-trans retinoic acid target *NPM1* mutant oncoprotein levels and induce apoptosis in *NPM1*-mutated AML cells. *Blood* 2015;125:3455-65 [IF: 23.1][Cit: 128] - **This paper identifies the drug-induced *NPM1* mutant degradation as potential strategy for a 'targeted-therapy' in *NPM1*-mutated AML, inspiring PI's further studies on the topic (leading to patents, grants award and other publications: US20160317579A1; IT202100025976A1; AIRC-IG to MPMartelli, id. 21419; AIRC-fellowship to R. Ranieri, id. 25347; Spinozzi et al. *Biomolecules*. 2022;12:904; Ranieri et al. *Leukemia*. 2022;36:2351).**

6) **Martelli MP**, Pettirossi V, Thiede C, Bonifacio E, Mezzasoma F, Cecchini D, Pacini R, Tabarrini A, Ciurnelli R, Gionfriddo I, Manes N, Rossi R, Giunchi L, Oelschlägel U, Brunetti L, Gemei M, Delia M, Specchia G, Liso A, Di Ianni M, Di Raimondo F, Falzetti F, Del Vecchio L, Martelli MF, Falini B. CD34+ cells from AML with mutated *NPM1* harbor cytoplasmic mutated nucleophosmin and generate leukemia in immunocompromised mice. *Blood* 2010;116:3907 [IF: 23.1][Cit: 102] - **This seminal paper characterizes the leukemic stem cell at the top of the leukemic hematopoiesis hierarchy in *NPM1*-mutated AML, and as potential target of immunotherapeutic approaches (leading to grants award and other publications: two AIRC-**

fellowship, V. Ciaurro, id. 20856, and C. Quadalti, id. 20868; Perriello et al. *Cancers*. 2021;13:496).

- **n=3 PATENTS**

1) *US20160317579A1*: Combination of an arsenic compound and at least one retinoid for treating acute myeloid leukemia [**Inventors**: H. de The, A. Bazarbachi, H. El Hajj, M.P. Martelli, B. Falini]; 2) *WO2017046108A1*: Dactinomycin compositions and methods for the treatment of acute myeloid leukemia [**Inventors**: B. Falini, M.P. Martelli]; 3) *IT202100025976A1*: Combinazione di principi attivi per il trattamento della leucemia acuta mieloide (LAM) con mutazione della nucleofosmina (*NPM1*) [**Inventor**: M.P. Martelli].

- **n=1 SPIN-OFF**

In 2021, the PI found and is Chief Executive Officer (CEO) of *toClinics* srls, *spin-off* of the University of Perugia and *women-led* company (co-founders and members of the BoD: Dr. Ilaria Gionfriddo, PhD and Dr. Francesca Milano, PhD) with focus on preclinical, in mice, *in vivo* pharmacological research in oncology. *toClinics* is part of the HAMU (Abruzzo, Marche, Umbria, center of Italy regions) hub, which promotes paths of innovative entrepreneurial growth and network of the territory.

- **The latest show how the PI is also keen to support technology transfer, industrial research in the academy and entrepreneurial activities.**

- **RESEARCH GRANTS AWARD**

Previous funding for research on acute myeloid leukemia from *Italian Ministry of Health, Bando Ricerca Finalizzata* 2008 (2 yrs- coordinator of research unit; 140.000 €) and *Bando Cellule Staminali* 2008 (2 yrs- PI; 210.000 €); from *Cassa di Risparmio di Perugia' Foundation, Bando Ricerca di Base* 2010, for a one-year Research fellowship (PI); from AIRC (Associazione Italiana Ricerca contro il Cancro): *AIRC IG 2006* (3 yrs), *AIRC IG 2010* (3yrs), *AIRC IG 2013* (3 yrs), *AIRC IG 2016* (3 yrs) (to Brunangelo Falini), as coinvestigator; *European Research Council (ERC) consolidator grant* 2016 (5 years, 1.883.750 €) (PI, Title: Dissecting to hit the therapeutic targets in *NPM1*-mutated AML) (<http://erc.martellilab.eu/>); *Corbel* 2018, European Union's Horizon 2020 research and innovation programme under grant agreement No 654248. (PI, Title: High-throughput compound screening approach to identify drugs targeting the acute myeloid leukemia (AML) entity harbouring *NPM1* gene mutation); *AIRC IG 2019* (3+2 yrs) (to Brunangelo Falini), as coinvestigator; *AIRC IG 2018 Id. 21419* (5 years-PI; 1.775.000 €) (PI, Title: A novel chemo-free approach to *NPM1*-mutated AML: from bench to bedside); *PRIN PNRR* 2022 (24 months) (Title: Clonal evolution in newly defined high-risk acute myeloid leukemia and role of leukemic stem cells as disease reservoir); *PRIN* 2022 (24months) (Title: Investigating DNA methyltransferase 3A (DNMT3A) gene mutation for its essentiality in acute myeloid leukemia (AML) maintenance, associated-pharmacogenomic profile and potentiality in human AML development)

Active funding for research on acute myeloid leukemia:

- *GIMEMA-AIL* 2022 (Title: TRacKing neutrotrophin tyrosine kinases receptor (NTRK) genes and exploring their leukemogenesis role in *NPM1*-mutated AML)
- *ERC – European Research Council – ERC PoC* (18 months-PI) (Title: Re-envisioning risk biomarkers in acute myeloid leukemia (AML) at single-cell level)
- *AIRC 5xmille 'Actionable targets in clonal progression and systemic spreading of myeloid neoplasms'* PI – Unit Perugia
- *PROGETTO PNC-E3-2022-23683269 PNC-HLS-TA*, PI – Unit Perugia
- *PNRR-TRI* (24 months) (Title: IMPATHY-AML: Integrating Molecular and digital PATHology to track new response biomarkers in high-risk Acute Myeloid Leukemia during treatment)
- *AIRC IG 2024* (60 months) (Title: *NPM1*-mutated AML and its microenvironment: dissecting mutations and drug conditioning for new therapeutic options)

- **INVITED SPEAKER CONFERENCES**

The PI frequently presented results of her research and perspectives in AML in national and international meetings and invited speaker conferences: e.g. **2017**, Italian Society of Hematology (*SIE*), *Educational* lecture; **2018**, Italian Society of Experimental Hematology (*SIES*), lecture; **2019-20-21**, international *Society of Hemato-Oncology (SOHO) for Italy*, *Educational* lecture; **2021-22**, *1st and 2nd SOHO Italy International School of AML*, *Educational* lecture; **2021**, *Euro-BioImaging The Virtual Pub* (DE), *Key* lecture; **2021**, “*Novel translational approaches in hematological malignancies: from bench to bedside*” Catania, *Key* lecture; **2022**, *SIES*, *Educational* lecture; **2022**, ‘*Single-Cell Multi-omics Europe Symposium: Heme Malignancies*’, Josep Carreras Leukemia Research Center, Barcelona (Spain), *Expert* lecture; **2024**, *POST- SAN DIEGO 2023*, Post-ASH meeting, Verona, Italy, *Expert* lecture.

- **COMMISSIONS OF TRUST AND PEER REVIEWER**

As **member of scientific committee and working groups**, the PI is involved in deciding scientific and strategic directions for the scientific societies and working groups, as well in fostering research programmes and development and valorization of innovative research in health sector: **2021**-to date, Member of the *Scientific Committee* of the *Biomedical Cluster of Umbria* (agreement between 34 Umbrian healthcare companies, the University of Perugia, local research centers and public and private healthcare facilities); **2021**-to date, Member of the *Acute Myeloid Leukemia (AML) Scientific Working Group (SWG)* of the *European Hematology Association (EHA)*; **2023**-to date, elected Member of the *Board of Directors* and nominated *Vice-President* of the *Italian Society of Experimental Hematology (SIES)*.

The PI has also contributed extensively to clinical research ethics oversight through **membership on Ethics Committee**: **2021**-to date, Member of the *Ethics Committee Review Board*, Regione Umbria and Perugia Hospital, Perugia, Italy.

As **member of research funding bodies panel (2016-2018)**, Panel Member of the *Scientific Committee* for *Programme de Recherche Translationnelle en Cancérologie (PRT-K)*, *Institut National du Cancer – INCa, France*; **2021**-to date, Panel Member of the *Scientific Committee* for *Associazione Italiana per la Ricerca sul Cancro – AIRC, Italy*) or **external expert reviewer (2014, Wellcome Trust, UK; 2014, Leukemia and Lymphoma Research, USA; 2015, Institut National du Cancer – INCa, France; 2025, Cancer Research UK – CRUK)**, the PI evaluated multiple grant proposals promoting high quality research.

Peer reviewer for scientific journals, including *Blood, Leukemia, Leukemia Research, Annals of Hematology-Oncology, Haematologica, Cancers, Frontiers, Frontiers Oncology*.

- **SCIENTIFIC ADVISORY BOARD AND SPEAKER BUREAU**

Abbvie, Amgen, Astellas, Be-One, BMS, Delbert, Gilead, Janssen, Jazz Pharmaceuticals, Novartis, Pfizer, Roche, Servier (topic: targeted therapy in AML).

- **FELLOWSHIPS/AWARDS**

1989, ERASMUS Project Fellowship Award, University of Perugia, for a 3-months stage at Molecular Biology Laboratory, King’s College, London, UK; **1998-1999**, “Fondazione Istituto Pasteur-Cenci Bolognetti” 2-years Fellowship Award for PhD student activity abroad, “La Sapienza” University, Rome, Italy; **1998-1999**, Supplemental Visiting Fellow NIH Fellowship Award, NIH, Bethesda, USA; **1998-2001**, PhD Fellowship Award, University of Perugia, Perugia, Italy; **2000-2001**, NIH Research Fellowship Award, NIH, Bethesda, USA; **2017**, National (MUR) Scientific Qualification for Full Professor position; **2022**, America Innovation Prize (Fondazione Italia-USA) – Start-up; **2023**, ‘UMBRIA IN ROSA’ award (Provincia of Perugia) – Start-up section.

ADDITIONAL INFORMATION

Other contributions to the research community

- **TEACHING AND MENTORING**

In the Perugia University academic environment, as professor in Hematology at the School of Medicine, and School of Hematology and faculty member, and then since 2023 coordinator, of the PhD School in Molecular and Clinical Medicine, the PI had the opportunity of teaching and tutoring many undergraduate, Hematology residents, PhD students and Masters students, as well as supervising postdocs:

2014-to date: *Lecturer in Hematology*, School of Medicine

2008-to date: *Lecturer in Hematology*, post-graduate School of Hematology

2011-to date: 16 *Hematology Residents*; 15 *PhD students* (7 supported with PI’s grants); 16 *Post-docs*

2017-2021: *SIES Postgraduate Scholarship for Abroad*–Dr Roberta Ranieri, FMP, Berlin, DE; “*Christian Boulin Fellowship*”, Dr Roberta Ranieri, Advanced Light Microscopy Facility, EMBL, Heidelberg, DE; *ERASMUS Fellowship*, Martina De Vizio, ERASMUS MC, Rotterdam University, Prof. Ruud Delwel; *BST bursary* for The 36th Annual Scientific Meeting of the BSTP–Pathology of mice with human immune systems, Dr Serenella Silvestri; *Leukemia Trainee Program Fellowship*, Dr Valerio Ciaurro, MDACC, Texas University, USA, Prof. Marina Konopleva; **for collaborative research projects abroad**

2017-2025: four *AIRC Fellowship for Italy* (Dr Valerio Ciaurro, 1yr; Dr Corinne Quadalti, 2 yrs; Dr Roberta Ranieri, 3 yrs; Dr. Eleonora Padiglioni, 1 yr), one *SIE ‘Francesco Lo Coco’ AIL Fellowship* (Dr Marcella Sabino, 1yr); **for research projects at the PI’s lab**

2025: tutor for second-level master's degree in Preclinical and Clinical Research at the University of Parma (Dr. Giulia Pallozzi)

- **MEMBERSHIPS OF SCIENTIFIC SOCIETIES AND ASSOCIATIONS**

Member of national (*Italian Society of Hematology, SIE; Italian Society of Experimental Hematology, SIES, Italian Society of Anatomy Pathology, SIAPeC-IAP*) and international (*European Hematology Association, EHA; European Association for Cancer Research, EACR*) scientific societies. Member of *ERC in Italy* (a non-profit association of ERC awardees); Member of the *Association of ERC Grantees*.

Career breaks and major life events

- *From 2000 to 2005, due to maternity leaves (4 children)*

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Perugia, 04/05/2026

Maria Paola Martelli