

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: Protein Biochemistry and Hematology Lab, at Hemopathology and Molecular Oncogenesis Laboratory, CREO (Center for Research in Hemato-Oncology), Hematology and Clinical Immunology
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Expertise and Fields of interest

Care and treatment of patients with malignant hematological diseases, particularly acute leukemias (AML and ALL); Principal Investigator and Coinvestigator in clinical trials on acute leukemias; Biochemistry of tumour-specific proteins (i.e. NPM1, EML4/ALK, BRAF-V600E); Functional genomics in onco-hematology; Target therapy in acute myeloid leukemia (AML); Clinical and translational research in AML.

• EDUCATION

- 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 POSITION

- 2016 – to date Associate Professor
Hematology/Perugia General Hospital, Perugia, Italy
- 2007 – to date Assistant Clinical Director
Hematology/Perugia General Hospital, Perugia, Italy

• PREVIOUS POSITIONS

- 2005 – 2016 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

• FELLOWSHIPS/AWARDS

- 2017 National (MIUR) Scientific Qualification for Full Professor position
- 2012 National (MIUR) Scientific Qualification for Associate Professor position
- 1998 – 2001 PhD Fellowship Award, University of Perugia, Perugia, Italy
- 2000 – 2001 NIH Fellowship Award, NHLBI, NIH, Bethesda, USA
- 1998 – 1999 Supplemental Visiting Fellow NIH Fellowship Award, NHLBI, NIH, Bethesda, USA
- 1998 – 1999 "Fondazione Istituto Pasteur-Cenci Bolognetti" 2-years Fellowship Award for PhD student activity abroad, "La Sapienza" University, Rome, Italy
- 1989 ERASMUS Project Fellowship Award, University of Perugia, for a 3-months stage at Molecular Biology Laboratory, King's College, London, UK

• TEACHING ACTIVITIES

At University of Perugia, Perugia, Italy

2014-to date Lecturer in Hematology, School of Medicine
2014-to date Lecturer in Hematology, post-graduate School of Clinical Pathology
2012-to date Lecturer in Hematology, post-graduate School of Internal Medicine
2010-to date Lecturer in Cell Therapies, School of Biotechnology
2009-to date Lecturer in Hematology, post-graduate Schools of Radiotherapy and Radiology
2008-to date Lecturer in Hematology, post-graduate School of Haematology

At University of Bari, Bari, Italy

2005-2007 Lecturer in Hematology, School of Dentistry

- **SUPERVISION OF GRADUATE STUDENTS AND POSTDOCTORAL FELLOWS**

As professor in Hematology and faculty member of the PhD school in Molecular and Clinical Medicine and Specialty School in Hematology, I had the opportunity of teaching and tutoring many either undergraduate or PhD students and postgraduate resident doctors, as well as supervising postdoctoral fellows. In particular, in the past years, I was thesis-supervisor for more than 10 students at the Biotechnology School, 12 students at the School of Medicine, and 5 PhD students, at University of Perugia.

2010 – to date 10 Postdocs/ 8 PhD Students, Hematology/Department of Medicine/University of Perugia

- **INSTITUTIONAL RESPONSIBILITIES**

At University of Perugia, Perugia, Italy

2014 – to date Researcher Board member of Department of Medicine

2013 – to date Faculty member of PhD School in Molecular Medicine, Dept. of Medicine

2013 – to date Faculty member of Post-graduate School in Internal Medicine, Dept. of Medicine

2010 – to date Faculty member of Intercourse Council of Interfaculty Biotechnology Study, Department of Experimental Medicine

2008 – to date Faculty member of Post-graduate School in Hematology, Dept. of Medicine

2008 – to date Faculty member of Post-graduate School in Radiology and School in Radiotherapy, Department of Surgery and Biomedical Science

2002 – 2013 Faculty member of PhD School in Biotechnologies in Human Bone Marrow Transplantation, Department of Clinical and Experimental Medicine

- **MEMBERSHIPS OF SCIENTIFIC SOCIETIES**

Member of national (Italian Society of Hematology, SIE; Italian Society of Experimental hematology, SIES) and international (European Hematology Association, EHA; American Society of Hematology, ASH) scientific societies.

- **RESEARCH AND CLINICAL ACTIVITIES**

- Since 2005, the main research interest is represented by the genetic-molecular and proteomic approach of tumor-specific proteins in onco-hematology, and in particular the characterization and therapy of Acute Myeloid Leukemia with nucleophosmin (*NPM1*) gene mutation

- Continuous clinical-assistance activities at the Hematology Divisions of the University of Rome "La Sapienza" (2001-2004), as Assistant Clinical Director, University of Bari (2005-2007), and University of Perugia (2008) - today) as a Assistant and then Associate Professor, in general in the field of hematology and in particular in the field of diagnostics and treatment of patients with onco-hematological diseases in hospitalization, outpatient and day-hospital.

2010-2014 Professional assignment (Perugia Hospital): 'Clinic of Acute Leukemia'

2014-present Assignment of High Specialization (Perugia Hospital): 'Hematology Department Clinical Activity and Study and Application of Diagnostic and Therapeutic Protocols in Acute Leukemia'

- **SCIENTIFIC PUBLICATIONS AND OTHER ACTIVITIES**

- 86 publications in international peer-reviewed scientific journals (see PubMed), and two book chapters. **h-index** 42, **Citations**: 8006 [Google Scholar]
- Oral/poster presentations at national and international conferences [American Society of Hematology (ASH), European Hematology Association (EHA), Italian Hematology Society (SIE), Italian Experimental Hematology Society (SIES)].
- Reviewer for *Blood*, *Leukemia*, *Cancer Letter*, *Journal of Applied Hematology*, *Journal of Cancer Biology and Treatment*, *Journal of Medical Genetics and Genomics*, *Tumori*, *Infection Disorders-Drug Targets* journal
- Reviewer for grant applications to Wellcome Trust, UK (2014); Leukemia and Lymphoma Research, USA (2014); Programme de recherche translationnelle en Cancérologie (PRT-K), Call for Projects in Translational Cancer Research 2015, Institut National du Cancer – INCa, France (2015)
- 2016-to date: Panel Member of Scientific Evaluation Committee for Programme de recherche translationnelle en Cancérologie (PRT-K), Institut National du Cancer – INCa, France (2015)
- Scientific Advisor at Advisory Board for *Celgene*, *Pfizer*, *Novartis*, *Amgen*
- **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), as participant, AIRC IG 2013-14595 (3 yrs), as participant
- **Active funding for research on acute myeloid leukemia**: AIRC IG 2016 (3 yrs), as coinvestigator; European Research Council (ERC) consolidator grant 2016 (5 years-PI; 1.883.750 €) (Title: Dissecting to hit the therapeutic targets in *NPM1*-mutated AML, PI: Maria Paola Martelli); Corbel, Access to European RI, 2018

- **INVESTIGATOR IN CLINICAL TRIALS**

Co-Principal Investigator at Coordinating Center (Perugia) of the following clinical trials:

"A phase IIa study of the clinical activity and safety of actinomycin-D in patients with *NPM1*-wild type AML (other than APL) aged ≥ 70 years old and/or unfit for intensive chemotherapy, either newly diagnosed or previously treated with hypomethylating agents". EudraCT: 2017-000282-68. Code: AML-PG03 (active)

"A phase II study of the clinical activity and safety of actinomycin-D in patients with *NPM1*-mutated AML ≥ 70 years old and/or unfit for intensive chemotherapy, either newly diagnosed or previous treated with hypomethylating agents." EudraCT: 2014-003490-41. Code: AML-PG02 (active)

Responsible lead Investigator at Perugia Center of the following clinical trials:

"Ancillary Observational Study of Post-Frontline Sequential Treatment of Adult Philadelphia Chromosome-Positive (Ph+) Acute Lymphoblastic Leukemia (ALL) Patients with Dasatinib and the Bispecific Monoclonal Antibody Blinatumomab" GIMEMA LAL2317(in the activation phase)

"National Treatment Program with Sequential Chemotherapy and Blinatumomab to Improve Minimal Residual Disease Response and Survival in Philadelphia Chromosome-Negative B-Cell Precursor Adult Acute Lymphoblastic Leukemia" EudraCT number 2017-004251-23. GIMEMA LAL2217 (in the activation phase)

"A Cross-sectional Survey of Patients and Caregivers Receiving Blynicyto in Routine Clinical Practice in Europe to Evaluate the Effectiveness of Additional Risk Minimisation Measures" AMGEN Protocol number 20150228 (in the activation phase)

"An observational study of Blinatumomab safety And effectiveness, utilization, and treatment practices" AMGEN Protocol number 20150136 (in the activation phase)

"A phase III, randomized, double-blind study of induction (daunorubicin [or idarubicin]/cytarabine) and consolidation (intermediate-dose cytarabine) chemotherapy plus midostaurin (PKC412) or chemotherapy plus placebo in newly diagnosed patients with acute myeloid leukemia (AML) without FLT3 mutations". Novartis Clinical Protocol: CPKC412E230; Drug: PKC412 (Midostaurin) (in the activation phase)

"Registro italiano per la prevalenza delle mutazioni IDH1/IDH2 nei pazienti con Leucemia Mieloide Acuta". EudraCT number: 02986620. Code: AML1516 (active)

Clinical Investigator at Perugia Center of the following clinical trials:

"An open-label, multi-center, Phase IIIb study to assess the safety and efficacy of midostaurin (PKC412) in patients 18 years of age or older with newly-diagnosed FLT3-mutated Acute Myeloid Leukemia who are eligible for "7+3" or "5+2" Chemotherapy" CPKC412A2408 EUDRACT number 2016-004440-12 (in the activation phase)

D-ALBA: "Front-Line Sequential Treatment of Adult Philadelphia Chromosome Positive (Ph+) Acute Lymphoblastic Leukemia (ALL) Patients with Dasatinib and the Bispecific Monoclonal Antibody Blinatumomab" EudraCT number 2016-001083-11. GIMEMA LAL2116 (active)

"A phase II study of the clinical activity and safety of actinomycin D in patients with relapsed/refractory acute myeloid leukemia with nucleophosmin (NPM1) gene mutation" EudraCT number: 2014-000693-18, ActD-AML-PG01 (closed)

"Terapia adattata al rischio genetico/citogenetico e basata sulla determinazione della malattia minima residua per pazienti giovani (≤ 60 anni) affetti da leucemia acuta mieloide di nuova diagnosi" EudraCT number 2010-023809-36. GIMEMA AML1310 (active)

"Trattamento di prima linea della Leucemia Linfoblastica Acuta (LLA) Philadelphia positiva (Ph+)/BCR/ABL+ con un nuovo potente inibitore delle Tirosin-chinasi (TKI), AP24534 (Ponatinib). Studio multicentrico, esplorativo, di fase II in pazienti di età superiore ai 60 anni o non idonei al programma intensivo di chemioterapia e trapianto delle cellule staminali". EudraCT number 2012-002761-35. GIMEMA LAL1811 (active)

"Studio osservazionale italiano sul numero e sull'esito delle gravidanze in pazienti di sesso femminile in età fertile trattate con chemioterapia per leucemia acuta promielocitica (Studi AIDA0493 e AIDA2000)" Studio APL0511 (active)

"Qualità della vita in ex-pazienti diagnosticati di leucemia acuta promielocitica arruolati negli studi GIMEMA AIDA0493 e AIDA2000" Studio QoL-APL0512 (closed)

- **PATENTS**

Since 30 Dec 2014 Patent PCT n. PCT/EP2014/079422: Combination of an arsenic compound and at least one retinoid for treating acute myeloid leukemia

Since 14 Sep 2015 Provisional Patent US Application n° 62218433: DACTINOMYCIN COMPOSITIONS AND METHODS FOR THE TREATMENT OF ACUTE MYELOID LEUKEMIA

- **MAJOR COLLABORATIONS**

Prof. Torsten Haferlach, topic: Characterization of NPM1-mutated AML and BCOR mutation discovery in AML, MLL laboratory, Munchen, Germany; *Prof. Claudia Haferlach*, topic: Characterization of novel NPM1 gene translocations in AML MLL laboratory, Munchen, Germany; *Prof. Hugues de Thé*, topic: Crosstalk of NPM1 mutated protein and PML in NPM1-mutated AML, INSERM/CNRS, Paris, France; *Prof. Hans G. Drexler*, topic: Study and characterization of AML cell lines, Leibniz Institute DSMZ, Germany; *Prof Zvi Livneh*, topic: Role of NPM1 mutant in translesion DNA synthesis, Dept. of Biological Chemistry, Weizmann Institute of Science, Rehovot, Israel; *Prof. Gabriella Sozzi*, topic: EML4/ALK rearrangements in lung cancer, INT, Milano, Italy; *Prof.*

Stefano A. Pileri, topic: Histopathology studies in leukemia, University of Bologna, Bologna, Italy;
Prof. Roberto Pellicciari, topic: Drug discovery in *NPM1*-mutated AML, TES Pharma, Perugia, Italy;
GIMEMA (Italian Cooperative Group of study of hematological diseases), an ECRIN certified Data Center (2015), Italy

- **CAREER BREAKS**

Significant career breaks from 2000 to 2005 due to maternity leaves (4 children).

RECENT RELEVANT SCIENTIFIC PUBLICATIONS

Main publications of studies on NPM1-mutated AML:

1) Falini B, Brunetti L, **Martelli MP**. Dactinomycin in NPM1-Mutated Acute Myeloid Leukemia. *N Engl J Med*. 2015 Sep 17;373(12):1180-2. **IF: 55.873**

- ***This is the first report on the clinical activity of an old drug (dactinomycin) in AML, specifically in the genotype harbouring NPM1 mutations***

2) **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 May 28;125(22):3455-65. **IF: 10.452**

- ***This paper identifies ATO/ATRA as a possible "targeted therapy" approach to NPM1-mutated AML***

3) **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 Nov 11;116(19):3907-22. **IF: 10.452**

- ***This paper characterizes the leukemic stem cell in NPM1-mutated AML***

4) *Garzon R, *Garofalo M, **Martelli MP**, Briesewitz R, Wang L, Fernandez-Cymering C, Volinia S, Liu CG, Schnittger S, Haferlach T, Liso A, Diverio D, Mancini M, Meloni G, Foa R, Martelli MF, Mecucci C, Croce CM, Falini B. Distinctive microRNA signature of acute myeloid leukemia bearing cytoplasmic mutated nucleophosmin. *Proc Natl Acad Sci U S A*. 2008 Mar 11;105(10):3945-50. *Equal contribution. **IF: 9.674**

- ***This is the first report showing a distinct miRNA profiling in NPM1-mutated AML***

5) Falini B, **Martelli MP**, Bolli N, Bonasso R, Ghia E, Pallotta MT, Diverio D, Nicoletti I, Pacini R, Tabarrini A, Galletti BV, Mannucci R, Roti G, Rosati R, Specchia G, Liso A, Tiacci E, Alcalay M, Luzi L, Volorio S, Bernard L, Guarini A, Amadori S, Mandelli F, Pane F, Lo-Coco F, Saglio G, Pelicci PG, Martelli MF, Mecucci C. Immunohistochemistry predicts nucleophosmin (NPM) mutations in acute myeloid leukemia. *Blood*. 2006;108(6):1999-2005. **IF: 10.452**

6) Falini B, *Bolli N, *Shan J, **Martelli MP**, Liso A, Pucciarini A, Bigerna B, Pasqualucci L, Mannucci R, Rosati R, Gorello P, Diverio D, Roti G, Tiacci E, Cazzaniga G, Biondi A, Schnittger S, Haferlach T, Hiddemann W, Martelli MF, Gu W, Mecucci C, Nicoletti I. Both carboxy-terminus NES motif and mutated tryptophan(s) are crucial for aberrant nuclear export of nucleophosmin leukemic mutants in NPMc+ AML. *Blood*. 2006;107(11):4514-23. *Equal contribution. **IF: 10.452**

- ***This paper clarifies for the first time the molecular basis of altered nucleo-cytoplasmic traffic of nucleophosmin in NPM1-mutated AML***

Main publications of studies on other tumor-associated genetic lesions:

7) Grossmann V, Tiacci E, Holmes AB, Kohlmann A, **Martelli MP**, Kern W, Spanhol-Rosseto A, Klein HU, Dugas M, Schindela S, Trifonov V, Schnittger S, Haferlach C, Bassan R, Wells VA, Spinelli O, Chan J, Rossi R, Baldoni S, De Carolis L, Goetze K, Serve H, Peceny R, Kreuzer KA, Oruzio D, Specchia G, Di Raimondo F, Fabbiano F, Sborgia M, Liso A, Farinelli L, Rambaldi A, Pasqualucci L, Rabadan R, Haferlach T, Falini B. Whole-exome sequencing identifies mutations of BCOR in acute myeloid leukemia with normal karyotype. *Blood*. 2011;118(23):6153-63. **IF: 10.452**

- ***This paper describes the discovery of BCOR mutations in AML with normal karyotype***

8) **Martelli MP**, Sozzi G, Hernandez L, Pettirossi V, Navarro A, Conte D, Gasparini P, Perrone F, Modena P, Pastorino U, Carbone A, Fabbri A, Sidoni A, Nakamura S, Gambacorta M, Fernández PL,

Ramirez J, Chan JKC, Grigioni WF, Campo E, Pileri SA, Falini B. *EML4-ALK* rearrangement in non-small-cell lung cancer and non-tumor lung tissues. *Am J Path*, 2009 Feb;174(2):661-70. **IF: 4.591**

9) Tiacci E, Trifonov V, Schiavoni G, Holmes A, Kern W, **Martelli MP**, Pucciarini A, Bigerna B, Pacini R, Wells VA, Sportoletti P, Pettirossi V, Mannucci R, Elliott O, Liso A, Ambrosetti A, Pulsoni A, Forconi F, Trentin L, Semenzato G, Inghirami G, Capponi M, Di Raimondo F, Patti C, Arcaini L, Musto P, Pileri S, Haferlach C, Schnittger S, Pizzolo G, Foà R, Farinelli L, Haferlach T, Pasqualucci L, Rabadan R, Falini B. BRAF mutations in hairy-cell leukemia. *N Engl J Med*. 2011;364:2305-12 **IF: 55.873**; *Total Citations*: 341

10) Tiacci E, Park JH, De Carolis L, Chung SS, Broccoli A, Scott S, Zaja F, Devlin S, Pulsoni A, Chung YR, Cimminiello M, Kim E, Rossi D, Stone RM, Motta G, Saven A, Varettoni M, Altman JK, Anastasia A, Grever MR, Ambrosetti A, Rai KR, Fraticelli V, Lacouture ME, Carella AM, Levine RL, Leoni P, Rambaldi A, Falzetti F, Ascani S, Capponi M, **Martelli MP**, Park CY, Pileri SA, Rosen N, Foà R, Berger MF, Zinzani PL, Abdel-Wahab O, Falini B, Tallman MS. Targeting Mutant BRAF in Relapsed or Refractory Hairy-Cell Leukemia. *N Engl J Med*. 2015 Oct 29;373(18):1733-47. **IF: 55.873**

- The last two publications describe the discovery (2011) of BRAFV600E mutation in hairy cell leukemia (HCL) and its translation in clinics (2015)

Perugia, 15-9-2018

Maria Paola Martelli, MD, PhD

Last ten-year publications.

1. Brunetti L, Gundry MC, Sorcini D, Guzman AG, Huang YH, Ramabadran R, Gionfriddo I, Mezzasoma F, Milano F, Nabet B, Buckley DL, Kornblau SM, Lin CY, Sportoletti P, Martelli MP, Falini B, Goodell MA. Mutant NPM1 maintains the leukemic state through HOX expression. *Cancer Cell*. 2018 Sep 10;34(3):499-512.e9. doi: 10.1016/j.ccell.2018.08.005.
2. Tiacci E, Venanzi A, Ascani S, Marra A, Cardinali V, Martino G, Codoni V, Schiavoni G, Martelli MP* and Falini B* [*co-last authors]. High-risk clonal hematopoiesis as the origin of AITL and NPM1-mutated AML. *N Engl J Med*. 2018 Sep 6;379(10):981-984. doi: 10.1056/NEJMc1806413.
3. Gu X, Ebrahim Q, Mahfouz RZ, Hasipek M, Enane F, Radivoyevitch T, Rapin N, Przychodzen B, Hu Z, Balusu R, Cotta CV, Wald D, Argueta C, Landesman Y, Martelli MP, Falini B, Carraway H, Porse BT, Maciejewski JP, Jha BK, Sauntharajah Y. Leukemogenic nucleophosmin mutation disrupts the transcription factor hub regulating granulo-monocytic fates. *J Clin Invest*. 2018 Jul 17. pii: 97117. doi: 10.1172/JCI97117. [Epub ahead of print]
4. Chiaretti S, Messina M, Grammatico S, Piciocchi A, Fedullo AL, Di Giacomo F, Peragine N, Gianfelici V, Lauretti A, Bareja R, Martelli MP, Vignetti M, Apicella V, Vitale A, Li LS, Salek C, Elemento O, Inghirami G, Weinstock DM, Guarini A, Foà R. Rapid identification of BCR/ABL1-like acute lymphoblastic leukaemia patients using a predictive statistical model based on quantitative real time-polymerase chain reaction: clinical, prognostic and therapeutic implications. *Br J Haematol*. 2018 Jun;181(5):642-652. doi: 10.1111/bjh.15251. Epub 2018 Apr 19.
5. De Clara E, Gourvest M, Ma H, Vergez F, Tosolini M, Dejean S, Demur C, Delabesse E, Recher C, Touriol C, Martelli MP, Falini B, Brousset P, Bousquet M. Long non-coding RNA expression profile in cytogenetically normal acute myeloid leukemia identifies a distinct signature and a new biomarker in NPM1-mutated patients. *Haematologica*. 2017 Oct;102(10):1718-1726.
6. Tripodo C, Burocchi A, Piccaluga PP, Chiodoni C, Portararo P, Cappetti B, Botti L, Gulino A, Isidori A, Liso A, Visani G, Martelli MP, Falini B, Pandolfi PP, Colombo MP, Sangaletti S. Persistent immune stimulation exacerbates genetically-driven myeloproliferative disorders via stromal remodeling. *Cancer Res*. 2017 May 23. pii: canres.1098.2017. doi: 10.1158/0008-5472.CAN-17-1098. [Epub ahead of print]
7. Brunetti L, Di Battista V, Venanzi A, Schiavoni G, Martelli MP, Ascani S, Mecucci C, Tiacci E, Falini B. Blastic plasmacytoid dendritic cell neoplasm and chronic myelomonocytic leukemia: a shared clonal origin. *Leukemia*. 2017 May;31(5):1238-1240.
8. Forghieri F, Bigliardi S, Quadrelli C, Morselli M, Potenza L, Paolini A, Colaci E, Barozzi P, Zucchini P, Riva G, Vallerini D, Lagreca I, Marasca R, Narni F, Venditti A, Martelli MP, Falini B, Lo Coco F, Amadori S, Luppi M. All-trans retinoic acid (ATRA) in non-promyelocytic acute myeloid leukemia (AML): results of combination of ATRA with low-dose Ara-C in three elderly patients with NPM1-mutated AML unfit for intensive chemotherapy and review of the literature. *Clin Case Rep*. 2016 Oct 24;4(12):1138-1146.
9. Falini B, Martelli MP, Tiacci E. BRAF-V600E mutation in hairy cell leukemia: from bench to bedside. *Blood*. 2016 Aug 23. pii: blood-2016-07-418434. [Epub ahead of print]
10. Falini B, Martelli MP. Impact of genomics in the clinical management of patients with cytogenetically normal acute myeloid leukemia. *Best Pract Res Clin Haematol*. 2015 Jun-Sep;28(2-3):90-7. doi: 10.1016/j.beha.2015.10.005.
11. Falini B, Brunetti L, Martelli MP. Dactinomycin in NPM1-Mutated Acute Myeloid Leukemia. *N Engl J Med*. 2015 Sep 17;373(12):1180-2. doi: 10.1056/NEJMc1509584
12. Tiacci E, Park JH, De Carolis L, Chung SS, Broccoli A, Scott S, Zaja F, Devlin S, Pulsoni A, Chung YR, Cimminiello M, Kim E, Rossi D, Stone RM, Motta G, Saven A, Varettoni M, Altman JK, Anastasia A, Grever MR, Ambrosetti A, Rai KR, Fraticelli V, Lacouture ME, Carella AM, Levine RL, Leoni P, Rambaldi A, Falzetti F, Ascani S, Capponi M, Martelli MP, Park CY, Pileri SA, Rosen N, Foà R, Berger MF, Zinzani PL, Abdel-Wahab O, Falini B, Tallman MS. Targeting Mutant BRAF in Relapsed or Refractory Hairy-Cell Leukemia. *N Engl J Med*. 2015 Oct 29;373(18):1733-47. doi: 10.1056/NEJMoa1506583. Epub 2015 Sep 9.

13. Falini B, Sportoletti P, Brunetti L, Martelli MP. Perspectives for therapeutic targeting of gene mutations in acute myeloid leukaemia with normal cytogenetics. *Br J Haematol*. 2015 Aug;170(3):305-22. doi: 10.1111/bjh.13409.
14. 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 May 28;125(22):3455-65.
15. Pettirossi V, Santi A, Imperi E, Russo G, Pucciarini A, Bigerna B, Schiavoni G, Fortini E, Spanhol-Rosseto A, Sportoletti P, Mannucci R, Martelli MP, Klein-Hitpass L, Falini B, Tiacci E. BRAF inhibitors reverse the unique molecular signature and phenotype of hairy cell leukemia and exert potent anti-leukemic activity. *Blood*. 2015 Feb 19;125(8):1207-16.
16. Ziv O, Zeisel A, Mirlas-Neisberg N, Swain U, Nevo R, Ben-Chetrit N, Martelli MP, Rossi R, Schiesser S, Canman CE, Carell T, Geacintov NE, Falini B, Domany E, Livneh Z. Identification of novel DNA-damage tolerance genes reveals regulation of translesion DNA synthesis by nucleophosmin. *Nat Commun*. 2014 Nov 25;5:5437.
17. Sportoletti P, Varasano E, Rossi R, Mupo A, Tiacci E, Vassiliou G, Martelli MP, Falini B. Mouse models of NPM1-mutated acute myeloid leukemia: biological and clinical implications. *Leukemia*. 2015 Feb;29(2):269-78.
18. Pozzesi N, Fierabracci A, Thuy TT, Martelli MP, Liberati AM, Ayroldi E, Riccardi C, Delfino DV. Pharmacological modulation of caspase-8 in thymus-related medical conditions. *J Pharmacol Exp Ther*. 2014 Oct;351(1):18-24.
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