

**Paolo G.V. Martini, Ph.D.**

97 W. Springfield St. #1, Boston, MA 02118

Cell: 617 803-0173; home: 617 267-5322

[paolom67@hotmail.com](mailto:paolom67@hotmail.com)

**SUMMARY:**

Chief Scientific Officer and Head of Discovery Rare Diseases at Moderna, currently working on messenger RNA therapy for rare/orphan genetic diseases. From early concept, through *Proof of Concept* to IND filing and beyond.

- An accomplished scientist, with a background in molecular biology and experience in targeting Rare Genetic Diseases with different therapeutic modalities
- mRNA, gene therapy, protein/antibody engineering and purification, small molecules, siRNA. Experience with microarray technology, genes transcriptional regulation, ligand-binding interactions, reporter assays, RNA/DNA manipulation.
- Assay development for genetic diseases and oncology platforms, identifying small molecules for drug discovery targets and managed the Gene Expression Profiling and Genotyping platform for oncology and autoimmune diseases for global projects, involving international sites.

Lecturer and organizer of international meetings and presentations.

Strong written and oral communication skills; well-developed managerial skills with experience in supervising and developing junior and senior staff.

See also profile on linked-in: <http://www.linkedin.com/>

**CITIZENSHIP:** USA and Italy

**EXPERIENCES:**

**Moderna, Cambridge, MA**

**Chief Scientific Officer Rare Diseases and Head of Discovery 2015-current**

- Buiding the internal portfolio for of Rare Diseases with mRNAs as therapeutic. Focus on Liver diseases. Overseeing 25 Research projects as supervising a group of Project Leaders and Research Associates (25 FTEs currently).
  - Managing the internal portfolio and budget for Discovery, FTEs and resources for successful IND filing. Working directly with Non-Clinical, Clinical and CMC/Manufacturing and Business Development
  - **Member of the Moderna Senior leadership**
- Managing the alliance with Partners for the development of mRNA therapeutic for Rare disorders

**Shire Human Genetic Therapies, Lexington, MA**

**Senior Director Discovery Biology and Translational Research 2013-2015**

- Drug Discovery: from concept to *Proof of Concept*. Focus on Renal, Skin and Pulmonary Fibrosis, Hematology-Oncology and rare Neurodegenerative diseases. Leading 2 Research project, and overseeing 12 Research projects. Moved 2 projects into Development, 1 into IND and supported marketing for V-priv (Velaglycerase alpha) and Replagal
- Technologies and new platform: targeting rare genetic diseases with AAV mediated gene therapy, antibody and bispecific antibodies, mRNA therapy, protein therapeutics, antisense nucleotide, and small molecules

- I am collaborating and working on 2 key projects for gene therapy through our collaboration with Sangamo Biosciences for AAV mediated delivery of Zinc Fingers- like regulators
- I have identified and led the partnership with arGEN-X for implementation of the antibody technology at Shire. arGEN-X is now our partner of choice for therapeutic antibody development
- I have identified and led the acquisition of Fibrotech and their small molecules with a novel and innovative MOA for targeting kidney fibrosis. Implementing the small molecules effort to further develop next generation candidates
- Early Development project team leader: from POC through IND enabling tox studies to Clinical studies. Leading a Team for IND submission for a Chronic kidney disease therapy
- Business Development: due diligence and new acquisitions. Responsible for identification and evaluation of novel technologies and preclinical and clinical assets for Fibrosis, Hematology Oncology and Neurodegenerative diseases
- Managing 8 PhDs and 6 Research Specialists

**Director Discovery Research, Project Leader**

**2011-2012**

- Project Leader in Discovery Research focusing on proteins and antibodies as therapeutics (phage display library screening platforms and/or antibody generation and humanization) for inflammation and fibrosis
- Supporting the Business Development Unit: due diligence on new technologies, platforms and drug candidates.
  - Identified and promoted the acquisition of IgAN Biosciences for IgA Nephropathy
- Lead 2 Research Projects to Proof of Concept using multiple approaches for evaluation of the “best in class” drugs

**Director of Molecular Biology**

**2010-2011**

- **Protein Expression and Purification Research Department and Discovery Research** for target/disease identification. Enzyme Replacement therapies and antibody target therapy for small population genetic diseases. Managed Protein Expression Group for protein production in mammalian and insect cells (wave bioreactor) and E. coli (fermentation) hosts, and protein characterization.
  - Project Leader: led 1 research project into Development. Demonstrated Proof of Concept in-vivo for a Lysosomal Storage Disease and transferred the project to Process Development.
    - published data in peer reviewed journals (see list of publications)
  - Managed 3 Scientists and 5 Research Specialists
  - Supervised 10 discovery research projects focused on genetic diseases: protein expression in mammalian, insect cells and bacteria for protein replacement therapy utilizing different technologies and different approaches for expression optimization (plasmids, transfections reagents/systems, media, cells technologies optimization for improved expression)
  - Department budget responsibilities
  - Member of the Shire Research Steering Committee for projects evaluation, management and POC studies

**Associate Director**

**2008-2009**

- **Protein Expression and Purification Research Department:** Enzyme Replacement therapies for small population genetic diseases. Protein expression in mammalian, E. Coli and insect cells hosts
  - Managed 2 Scientists and 5 Research Specialists
  - Supervised 9 discovery research projects focused on genetic diseases: protein expression in mammalian, insect cells and bacteria for protein replacement therapy utilizing different technologies and different approaches for expression optimization (plasmids, transfections reagents/systems, media, cells technologies optimization for improved expression)
    - Lead 2 Projects to POC.
  - Department budget responsibilities
  - Member of the Shire Research Coordination Meeting for projects management and POC studies

**Senior Scientist**

**2007-2008**

- **Protein Expression and Purification Research Department:** Enzyme Replacement therapies for small population genetic diseases  
Cloning, expression and development of proteins (mammalian, bacteria, insect cells expression) for replacement therapy and relative functional *in-vitro* assay for genetic diseases
  - Managed 1 Scientist and 3 Research Specialists
  - Supervising 10 discovery research projects focused on genetic diseases: protein expression in mammalian, insect cells and bacteria for protein replacement therapy utilizing different technologies and different approaches for expression optimization (plasmids, transfections reagents/systems, media, cells technologies optimization for improved expression)
  - Department budget responsibilities
  - Member of the Shire Research Coordination Meeting for projects management and POC studies

**Serono Research Institute (now EMD-Serono), Rockland, MA**

**2001-2006**

**Group Leader**

**2004-2006**

- **Biotherapeutic Discovery Department:** Protein Engineering for Oncology drug discovery. Development of new engineered proteins/antibodies (variable regions amplification/cloning) and relative assays for cancer therapy. Set up a platform for lentivirus delivered shRNA target identification in mammalian cells and animals.
- **Systems Biology Department:** Oncology Genomic Platform  
Breast Cancer genomic and genotyping study. Early access to Affymetrix 500K SNPs Chips for breast tumor tissues and breast cancer cell lines analysis for target and biomarkers identification. The work was presented at the 12<sup>th</sup> International Meeting on Steroid Hormones and Hormone and Cancer, in Athens, Greece. The work has been published on a peer reviewed journal.  
Use of R/Bioconductor for microarray analysis.  
Multiple Sclerosis human and mouse model comparison by gene expression: a collaborative project with San Raffaele Hospital, Center of Excellence for Multiple

Sclerosis, laboratories of Prof. Giancarlo Comi and Dr. Gianvito Martino, Milano, Italy. Three abstracts submitted to international meetings.

Supervision of 2 post-doctoral fellows and 1 research assistant: managed and developed individual projects for the post-doctoral fellows and for the research assistant. Leader in the global organization of the gene expression profiling platform coordinating four research sites' Group Leaders for data posting, analysis and presentations (Boston, Paris, Geneva, Ivrea).

- Coordination of local projects and out-sourced collaborations with San Raffaele Hospital and University of Milano: meetings, presentations, legal matters.

**Senior Principal Investigator**

**2001-2004**

- Set-up and implemented Affymetrix Microarray Technology Platform at Serono, for gene expression profiling and genotyping. Managing gene expression profiling platform from experimental design to data analysis. Set-up quality controls and validation technology by TaqMan (ABI 7900HT). Two complete studies within Serono therapeutic areas have been run under my direction and 2 targets have been selected for screening.
- Cloned and de-orphanized novel GPCRs: analyzed their physiology and action using classical molecular biology techniques (Western, Northern, Southern blot, RT-PCR, TaqMan); involved in the Knock-out mice design and characterization. Patent to be filed. A journal article has been published.
- Set-up three nuclear hormone receptors secondary screening assays (reporter based assays) for small molecules' target validation and tested lead compounds for activity and cross reactivity.

**University of Illinois Urbana-Champaign, Urbana, IL**

**1998-2001**

**Post-Doctoral Research Fellow/Instructor** (Dr. Benita S. Katzenellenbogen's lab)

- Cloned and characterized a novel estrogen receptors co-regulator with the use of the yeast two-hybrid screening, analyzed its function with the use of several molecular biology techniques and identified its chromatin remodeling activity. The work has been presented at International and national conferences and several peer reviewed articles have been published.
- Supervised 2 graduate students and 2 undergraduate students and led the students to successful graduation.

**Schering AG (now Bayer HealthCare), Berlin, Germany**

**1996-1997**

**Post-Doctoral Research Fellow** (host Dr. Ursula Habenicht)

- Cloned the human homologue of the estrogen receptor beta from a human prostate cancer cDNA library. Set-up and implemented estrogen receptor isoforms screening assays and validated selected natural derivative compounds.
- Established a new electroporation-based transfection method for mammalian suspension cells and tested interleukin antagonist in cell-based assays.

**University of Milano, Milano, Italy**

**1995-1996**

**Post-Doctoral Research Fellow** (Dr. Paola Negri-Cesi's lab)

- Analyzed the presence and function of the 5 $\alpha$ -reductases and aromatases enzyme in prostate cancer cells using either radio-enzymatic assays or basic molecular biology techniques.

**PROFESSIONAL COMPETENCIES:**

**-Discovery Research:** Direct and advance projects from proof of concept to IND filing. Collaborate with multiple disciplines to create well defined programs for target identification and validation.

**-Cell Culture:** variety of mammalian cell culture (HEK-293, HT1080, MCF-7, MDA-MB-231, CHO, PC12, CV-1, LNCaP, DU145, etc.), suspension and monolayer, primary cell culture (astrocytes and human prostate) and Schneider Drosophila cells; transient and stable transfection system (CaPO<sub>4</sub>, Lipofectamine, Electroporation); reporter gene assays (CAT, Luciferase, GFP), wave bioreactor production.

**-Assay development:** Receptor binding assays (radioactive, SPA, Fluorescence) (invasion, proliferation, migration), ELISA, EIA, RIA. Use of multiplex technologies (Luminex) Yeast and mammalian two-hybrid system (library construction, protein-protein interaction and analysis).

**-DNA/RNA manipulation:** DNA cloning, sequencing, mutagenesis, PCR, Southern analysis, antisense methodologies, Chromatin immunoprecipitation assay, Real time PCR, RNA extraction, purification, Northern analysis, RPA, siRNA in drosophila and mammalian cells. Affymetrix Microarray whole human/mouse genome analysis, SNPs chip (100/500K)

**-Protein/Antibody:** expression in mammalian, insects' cells and bacteria, and basic purification: structure and analysis, light scattering, SDS-PAGE, immunoprecipitation, Western analysis, fusion/tagged protein cloning/expression and interaction assays, IHC, ICC. Antibody engineering, variable regions identification, CDR grafting, cloning and expression.

**Softwares:** Use of different computer programs for bioinformatics analysis (genome) statistic and graphic presentation (MS Office, R/Bioconductor, Rosetta Biosoftware, Vector NTI, Sequencher etc.).

## PATENTS

- Peptide linkers for polypeptide compositions and methods for using same. US serial number 61/449,25
- Mannose Receptor C Type 1 (MRC1) codon optimized cell line and use thereof. US serial number 61/365,55
- Treatment of Sanfilippo Syndrome Type B. US2011/0318327A1
- Methods for treating Lysosomal Acid Lipase Deficiency WO/2012/112677A3 EP12746845A
- Anti-CCL2 antibodies for treatment of Scleroderma WO/2013/177254A1
- Anti-CCL2 and anti-LOXL2 combination therapy for treatment of Scleroderma WO/2014/190316A1
- Polynucleotides encoding acyl-coa dehydrogenase, very long-chain for the treatment of very long-chain acyl-coa dehydrogenase deficiency WO/2017/201332a1
- Polynucleotides encoding citrin for the treatment of Citrullinemia type 2 WO/2017/201349a1
- Polynucleotides encoding  $\alpha$ -galactosidase a for the treatment of Fabry disease WO/2017/201328a1
- Polynucleotides encoding galactose-1-phosphate uridylyltransferase for the treatment of Galactosemia type 1 wo/2017/201348a1
- Polynucleotides encoding porphobilinogen deaminase for the treatment of Acute Intermittent Porphyria WO/2017/201346a1
- Polynucleotides encoding cystic fibrosis transmembrane conductance regulator for the treatment of Cystic Fibrosis WO/2017/201347a1
- Polynucleotides encoding lipoprotein lipase for the treatment of Hyperlipidemia WO/2017/201333a1
- Polynucleotides encoding jagged1 for the treatment of Alagille syndrome WO/2017/201342a1
- Polynucleotides encoding methylmalonyl-coa mutase WO/2017/106799a1

**ACADEMIC EXPERIENCES:**

**Brandeis University**, Rabb School for Graduate Studies, Waltham, MA. **2007-2011**  
**Adjunct Faculty**

- Molecular Biology RBIO 101: molecular biology laboratory for graduate students
- Molecular and cell Development RBIO102: molecular biology for bioinformatic

**EDUCATION:**

**Ph.D.** in Metabolic Endocrinology Sciences, University of Milan, Department of Endocrinology, Milan, Italy, November 1995.

Research Advisor: Prof. Marcella Motta

**BS/MS**, Pharmaceutical Chemistry and Technologies, University of Milan, Faculty of Pharmacy, Milan, Italy, 1992.

**AWARDS:**

Shire "Making an Impact" Award, March 2010

Shire "Making a difference" Award, April 2009

Shire "Making a difference" Award, January 2009

Shire Project Team Recognition Award 2008

Shire Performance Recognition Award 2007

Breast Cancer Research Foundation Fellowship, 2000

Susan G. Komen Breast Cancer Foundation Postdoctoral Fellowship. 1998

Schering Foundation Postdoctoral Fellowship. 1996

**EDITORIAL BOARDS, Reviewer (ad-hoc):**

Keystone Symposia: Member of the Scientific Advisory Board

Oxford Global: member of the Advisory Board for the Annual Protein Congress (2011)

Journal Steroid Biochemistry and Molecular Biology

Endocrine-related Cancer

BMC Journals

MIT press

**MEMBERSHIPS:**

American Society of Hematology (ASH): since 2015

American Society of Nephrology (ASN): since 2013

The Endocrine Society, USA: since 2001

American Society for Biochemistry and Molecular Biology (ASBMB): since 2003.

American Association for Cancer Research (AACR): since 2005.

American Management Association (AMA): since 2004.

**LANGUAGES:**

Italian, English (fluent), French (basic), German (basic).

**FEATURE ARTICLES:**

Private Ambition. Virginia Li, BioCentury Weekly February 5, 2018

RNA replaces Enzymes. How Moderna replaced ERT with RNA for rare metabolic diseases. Michael Leviten, BioCentury Innovation, January 4, 2018

Can a multibillion-dollar biotech prove its RNA drugs are safe for a rare disease?  
Kelly Serwick, Science Dec 19, 2017

<http://www.sciencemag.org/news/2017/12/can-multibillion-dollar-biotech-prove-its-rna-drugs-are-safe-rare-disease>

Protein Production Continues to Post Gains, Morrow K. J. Jr., Genetic Engineering and Biotechnology News, Oct 1 2010 (Vol. 30, No. 17)

## BOOK CHAPTERS

Complement Systems: Methods and Protocols. Methods in Molecular Biology, Vol. 1100, Gadjeva, Mihaela (Ed. 2014), Humana Press. Norton A.W., **Martini P.G.V.**, Cook L.C., Alderucci S., Lundberg D.M., Fish S.M., Bedard C., Gill J., Tzianabos A.O., Concino M.F.. Expression and Purification Methods for the Production of Recombinant Human Complement Component C2

## SELECTED PUBLICATIONS:

An D., Schneller J.L., Frassetto A., Liang S., Zhu X., Park JS., Theisen M., Hong SJ., Zhou J., Rajendran R., Levy B, Howell R., Besin G., Presnyak V., Sabnis S., Murphy-Benenato K.E., Kumarasinghe E.S., Salerno T., Mihai C., Lukacs C.M., Chandler R.J., Guey L.T., Venditti C.P., **Martini P.G.V.** Systemic messenger RNA therapy as a treatment for methylmalonic acidemia. Cell Reports, 21: 3548–3558, 2017

Patton C., Farr III G.H., An J., **Martini P.G.V.**, Maves L Lipid nanoparticle packaging is an effective and non-toxic mRNA delivery platform in embryonic zebrafish. Zebrafish, *in-press*

Lechner S.M., Abbad L., Boedec E., Papista C., Le Stang MB., Moal C., Maillard J., Jamin A., Bex-Coudrat J., Wang Y., Li A., **Martini P.G.V.**, Monteiro R.C., Berthelot L. IgA1 Protease Treatment Reverses Mesangial Deposits and Hematuria in a Humanized Mouse Model of IgA Nephropathy. J. Am. Soc. Nephrol, 27(9): 2622-9, 2016

Moretti R.M., Montagnani-Marelli M., Taylor D.M., **Martini P.G.V.**, Marzagalli M., Limonta P. Gonadotropin-Releasing Hormone Agonists Sensitize, and Resensitize, Prostate Cancer Cells to Docetaxel in a p53-Dependent Manner. PLOS One, 9(4): e93713. doi:10.1371/journal.pone.0093713, 2014

**Martini P.G.V.**, Cook L.C., Alderucci S., Norton A.W., Lundberg D.M., Fish S.M., Langsetmo K., Jönsson G., Lood C., Gullstrand B., Zaleski K.J., Savioli N., Lottherand J., Bedard C., Gill J., Concino M.F., Heartlein M.W., Truedsson L., Powell J.L., Tzianabos A.O. Recombinant human complement component C2 produced in a human cell line restores the classical complement pathway activity *in-vitro*: an alternative treatment for C2 deficiency diseases. BMC Immunology, 11: 43, 2010

Peterson S., Iskenderian A., Cook L., Romashko A., Tobin K., Norton A., Gómez-Yafal A., Heartlein M.W., Concino M.F., Liaw L.L., **Martini P.G.V.** Human Sulfatase 2 inhibits in vivo tumor growth of MDA-MB-231 in human breast cancer xenografts. BMC Cancer, 10: 427, 2010.

Moretti R.M., Mai S., Montagnani Marelli M., Bani M.R., Ghilardi C., Giavazzi R, Taylor D.M., **Martini P.G.V.**, and Limonta P. Dual Targeting of Tumor and Endothelial Cells by Gonadotropin-Releasing Hormone Agonists to Reduce Melanoma Angiogenesis. Endocrinology, 151 (10): 4643-4653, 2010.

**Martini P.G.V.**, Bienkowska J., Taylor D., Jackson J., McAllister G., Keilhack H., Campbell R.K. Comparative expression analysis of four breast cancer subtypes versus matched normal tissue from the same patients. J. Steroid Biochem. Mol. Biol., 109 (3-5):207-11, 2008.

O'Lone R., Knorr K., Jaffe I., Schaffer M., **Martini P.G.V.**, Karas R.H., Bienkowska J., Mendelsohn M.E., Hansen U. Estrogen receptors alpha and beta mediate distinctive pathways of vascular gene expression: mitochondrial electron transport and reactive oxygen species. Mol. Endo., 21 (6):1281-1296, 2007.

A. Pravettoni, O. Mornati, **P.G.V. Martini**, A. Colciago, F. Celotti, M. Motta, P. Negri-Cesi. The endogenous estrogen receptor beta (ER-beta) modulates the proliferation of the androgen-independent prostate cancer cell line DU145. Mol. Cell. Endocr., 263: 46-54, 2007.

M. Muda, C. He, **P.G.V. Martini**, T. Ferraro, S. Layfield, D. Taylor, C. Chevrier, R. Schweickhardt, C. Kelton, P. L. Ryan and R. A.D. Bathgate. Splice variants of the relaxin and INSL3 receptors reveal unanticipated molecular complexity, Mol. Human Reprod., 11(8): 591-600, 2005.

Guerini V., Sau D., Scaccianoce E., Rusmini P., Ciana P., Maggi A., **Martini PGV.**, Katzenellenbogen B., Martini L., Motta M., Poletti A. The Androgen Derivative 5alpha-androstane-3beta,17beta-diol (3beta-diol) Inhibits Prostate Cancer Cell Migration through Activation of the Estrogen Receptor beta Isoform. Cancer Res., 12 (65): 5445:5453, 2005.

**Martini P.G.V.**, Katzenellenbogen B.S. Modulation of the estrogen receptor activity by selective coregulators. J. Steroid Biochem. Mol. Biol., 85(2-5): 117-122, 2003.

Rajendran R.R., Nye A.C., Frasor J., Balsara R.D., **Martini P.G.V.**, Katzenellenbogen B.S. Regulation of nuclear receptor transcriptional activity by a novel DEADbox RNA helicase (DP97). J. Biol. Chem., 278:4628-4638, 2003.

**Martini P.G.V.**, Katzenellenbogen B.S. Regulation of prothymosin alpha gene expression by estrogen in estrogen receptor-containing breast cancer cells via upstream half-palindromic estrogen response element motifs. Endocrinology, 142(8):3493-3501, 2001.

Delage-Mourroux R., **Martini P.G.V.**, Choi I., Kraichely D.M., Hoeksema J., Katzenellenbogen B.S. Analysis of estrogen receptor interaction with a repressor of estrogen receptor activity (REA) and the regulation of estrogen receptor transcriptional activity by REA. J. Biol. Chem., 275:35848-35856, 2000.

**Martini P.G.V.**, Delage-Mourroux R., Kraichely D.M., Katzenellenbogen B.S. Prothymosin alpha selectively enhances estrogen receptor transcriptional activity by



interacting with a repressor of estrogen receptor activity (REA). Mol. Cell. Biol., 20:6224-6232, 2000.

Katzenellenbogen B.S., Choi I., Delage-Mourroux R., Ediger T.R., **Martini P.G.V.**, Montano M.M., Sun J., Weis K., Katzenellenbogen J.A. Nobel Symposium on Estrogens. Molecular mechanism of estrogen action: selective ligands and receptor pharmacology. J. Steroid Biochem. Molec. Biol., 74(5):279-285, 2000.

Katzenellenbogen B.S., Montano M.M., Ediger T.R., Sun J., Ekena K., Lazennec G., **Martini P.G.V.**, McInerney E.M., Delage-Mourroux R., Weis K., Katzenellenbogen J.A. Estrogen receptors: selective ligands, partners and distinctive pharmacology. Recent Progress in Hormone Research, 55:163-195, 2000.

Gee A.C., Carlson K.E., **Martini P.G.V.**, Katzenellenbogen B.S., Katzenellenbogen J.A. Coactivator peptides have a differential stabilizing effect on the binding of agonists and antagonists with the estrogen receptor. Mol. Endocrinol., 13:1912-1923, 1999.

Montano M.M., Ekena K., Delage-Mourroux R., Chang W., **Martini P.G.V.**, Katzenellenbogen B.S. An estrogen receptor-selective coregulator that potentiates the effectiveness of antiestrogen and represses the activity of estrogens. Proc. Natl. Acad. Sci., USA, 96:6947-6952, 1999.

Negri-Cesi P., Poletti A., Colciago A., Magni P., **Martini P.G.V.**, Motta M. Presence of 5 $\alpha$ -reductase isozymes and aromatase in human prostate cancer cells and human benign hyperplastic tissue. Prostate, 34:283-291, 1998.

#### **SEMINARS AND SYMPOSIA, INVITED SPEAKER:**

**Martini P.G.V.**, Messenger RNA as a software of life. Industry-Academia Alliances Conference, Doha, Qatar, February 2018

**Martini P.G.V.**, Rachlin E. mRNA therapy for rare genetic diseases. King Abdullah Medical City, Riyadh, Saudi Arabia, November 2017

**Martini P.G.V.** messenger RNA therapy for monogenic disorder, Keystone Symposia, Non-coding RNAs: From Disease to Targeted Therapeutics, Banff, Ab, Canada, February 2017

**Martini P.G.V.** mRNA therapy: a novel approach for the treatment of rare diseases. Murdoch Children's Research Institute, David Danks Speaker series, Melbourne, Vic, Australia, October 2016

**Martini P.G.V.** mRNA therapy: a novel approach for the treatment of rare diseases. Seattle Children's Research Institute, Speaker series, Seattle, WA, USA, June 2016

**Martini P.G.V.** Drug Discovery in Rare Diseases: From Concept to Proof of Concept and Beyond. Drug Discovery USA Congress Oxford Global, Boston, MA, USA, October 2014

**Martini P.G.V.**, Alderucci S., Holmes K.D., Molina S., Zhang B., Iskenderian A., Cook L.C., Norton A.W., Lundberg D., Langsetmo K., Concino M.F. Expression and

Production of Secreted Proteins: Plasmid Engineering and Transfection Strategies for Protein Expression Optimization, 3<sup>rd</sup> Annual Protein Congress, Berlin, Germany, October 2010.

**Martini P.G.V.**, Peterson S., Iskenderian A., Cook L., Romashko A., Tobin K., Norton A., Gómez-Yafal A., Heartlein M.W., Concino M.F., Liaw L.L., Human Sulfatase 2 inhibits in vivo tumor growth of MDA-MB-231 human breast cancer xenografts. Faculty of Pharmacy, University of Milan, Milan, Italy, October 2010.

**Martini P.G.V.**, Concino M., Tzianabos A., Onderdonk A., Robinson G. Differential in-vitro responses in inflammatory and immune cytokine production elicited by enzyme replacement therapies for Gaucher disease, 8th European Working Group on Gaucher Disease (EWGGD) Meeting, Budapest, Hungary, June 2008.

**Martini P.G.V.**, Brini E., G. McAllister, Martino G., Zaratini P. Multiple Sclerosis: a neurodegenerative autoimmune disease. Neurodegenerative Diseases Course, Harvard Medical School, Boston, MA, March 2008.

**Martini P.G.V.**, Brini E., Martino G., Zaratini P. Multiple Sclerosis: an autoimmune disease overview. Neurodegenerative Diseases Course, Harvard Medical School, Boston, MA, November 2006.

**Martini P.G.V.**, Bienkowska J., Taylor D., Jackson J., McAllister G., Keilhack H., Campbell R.K. Comparative expression analysis of four breast cancer subtypes versus matched normal tissue from the same patients. 12<sup>th</sup> International Congress on Hormonal Steroids and Hormone and Cancer, Meet the Experts, Athens, Greece, September 2006.

**Martini P.G.V.** From Academia to Industry: a new world of scientific discoveries. University of Minnesota, Minneapolis, July 2005.

**Martini P.G.V.** Ant-proliferative and anti-metastatic effect of Estrogen receptor beta isoform in human prostate cancer cell lines DU145. Seminar, University of Milan, Milan, Italy, January 2005.

**Martini P.G.V.**, Katzenellenbogen B.S. ER-alpha, ER-beta: two receptors, one ligand, multiple functions. New Frontiers in Neuroendocrinology Meeting. Symposium, University of Milan, Milan, Italy, March 2003.

**Martini P.G.V.**, Katzenellenbogen B.S. Modulation of the estrogen receptor activity by selective coregulators. 11<sup>th</sup> International Congress on Hormonal Steroids and Hormones and Cancer, Fukuoka, Japan, Symposium (9), October 2002.

**Martini P.G.V.**, Katzenellenbogen B.S. Activity and mechanism of the estrogen receptors: selective ligands and coregulators of gene transcription. VIII Congresso Nazionale, Societa' Italiana di Fisiopatologia della Riproduzione (S.I.F.R.), Napoli, Italy, Seminar, December 2001.

**Martini P.G.V.**, Katzenellenbogen B.S. Estrogen receptors: coregulators, ligands and gene transcription. University of Milan, Italy, Seminar, April 2001.

**Martini P.G.V.**, Katzenellenbogen B.S. Coregulators and estrogen receptor control of gene expression. University of Illinois Urbana-Champaign, Reproductive Biology Training Grant Seminar, February 2001.

**Martini P.G.V.**, Kraichely D.M., Katzenellenbogen B.S. Prothymosin alpha selectively enhances transcriptional activation of the estrogen receptor: involvement of the coregulator REA. Keystone Symposium, Nuclear Receptor 2000, Steamboat Springs, CO, Oral Session, March 2000.

**Martini P.G.V.**, Katzenellenbogen B.S. Prothymosin alpha selectively enhances estrogen receptor transcriptional activity by interacting with a repressor of estrogen receptor activity (REA). University of Illinois Urbana-Champaign, Reproductive Biology Training Grant Seminar, October 1999.

**Martini P.G.V.**, Ekena K., Delage-Mourroux R., Montano M.M., Harrington W., Katzenellenbogen B.S. A repressor of estrogen receptor activity (REA) functions as an anticoactivator. Oral Session OR1-2 p. 63, 81<sup>st</sup> Annual Meeting of the Endocrine Society, San Diego, CA, June 1999.

**Martini P.G.V.**, Toschi L., Negri-Cesi P., Magni P., Motta M., Schleuning W-D. Studies on the role of estrogens and estrogen receptor in solid tumor biopsies and cultured cells. Seminar at Karolinska Institute, Stockholm, Sweden. Host: Prof. Jan-Ake Gustafsson.

#### **POSTERS and ABSTRACTS:**

An J., Frassetto A., Schneller J., Liang S., Zhu X., Park JS., Hong SJ., Zhuo J., Rajendran R., Presnyak., Sabnis S., Lukacs C., Chandler RJ., Venditti CP., Guey L., **Martini PGV.**, Messenger RNA Therapy for Methylmalonic Acidemia (MMA): Preliminary *in-vitro* and *in-vivo* Efficacy. Lysosomal Disease Network (LDN) 13<sup>th</sup> Annual World Symposium on Rare Diseases, San Diego, CA, February 2017

Du H., Pescatore B., Romashko A., Meiyappan M., **Martini P.G.V.**, Charnas L., Alicia Gomez-Yafal A., Xu Y.H., Westmoreland O., Stanton L., and Grabowski G.A. Enzyme Replacement Therapy Corrects Lysosomal Acid Lipase Deficiency (LALD) in a Mouse Model of Wolman Disease/Cholesteryl Ester Storage Disease. Lysosomal Disease Network (LDN) WORLD Symposium, Las Vegas, NV, Molecular Genetics and Metabolism 102:S19. February, 2011.

Limonta P., Mai S., Montagnani-Marelli M., Ceruti S., **Martini P.G.V.**, Motta M., Moretti R.M. Gonadotropin-Releasing Hormone Agonists Sensitize Prostate Cancer Cells to Drug-Induced Apoptosis. 10th International Symposium on GnRH, Salzburg, Austria, February 2011.

Iskenderian A., A. Romashko, Nguyen D., Cook L., Norton A., Gómez-Yafal A., Heartlein M.W., Concino M.F., **Martini P.G.V.** Purified Human Recombinant Sulfatase 2 Inhibits Human Breast Cancer Cell Proliferation and Invasion *In-vitro*. 100<sup>th</sup> Annual Meeting of the American Association for Cancer Research, Denver, CO, April 2009.

**Martini P.G.V.**, Concino M., Tzianabos A., Onderdonk A., Robinson G. Differential *in-vitro* responses in inflammatory and immune cytokine production elicited by enzyme

replacement therapies for Gaucher disease, 8th European Working Group on Gaucher Disease (EWGGD) Meeting, Budapest, Hungary, June 2008.

Montagnani Marelli M., Moretti R.M., Mai S., **Martini P.G.V.**, Taylor D.M., Januszkiewicz-Caulier J., Limonta P. Molecular mechanisms underlying the antimetastatic activity of Gonadotropin-Releasing Hormone in tumors. 11<sup>th</sup> World Congress on Advances in Oncology and 9<sup>th</sup> International Symposium on Molecular Medicine, Crete, Greece, October 2006.

Moretti R.M., Montagnani Marelli M., **Martini P.G.V.**, Januszkiewicz-Caulier J., Taraboletti G., Bani M.R., Giavazzi R., Limonta P. Gonadotropin Releasing Hormone receptors in human malignant melanomas: gene expression profile associated with their inhibitory activity on cell migration/invasion. 48<sup>th</sup> National Congress of the Italian Society of Cancerology, P49, Bari, Italy, October 2006.

Moretti R.M., Montagnani Marelli M., Mai S., **Martini P.G.V.**, Limonta P. Gonadotropin Releasing Hormone inhibits the metastatic properties of hormone related and hormone unrelated tumors. P186, 12<sup>th</sup> International Congress on Hormonal Steroids and Hormone and Cancer, Athens, Greece, September 2006.

C. Ferrandi, V. Muzio, **Martini P.G.V.**, R. Furlan, G. Martino, P. Tavano, MC. Magnone, B. Greco, J-P. Gotteland, T. Wells, P.F. Zaratini. JNK inhibition as a potential treatment for MS. 22<sup>nd</sup> Congress of the European Committee for Treatment and Research of Multiple Sclerosis, Madrid, Spain, September 2006.

E. Brini, **Martini P.G.V.**, V. Barbié, R. Furlan, C Ferrandi, G. Comi, T. Wells, M. Mariani, G. Martino, P.F. Zaratini. Common transcriptional signature in the clinical forms of MS: the role of downregulation of apoptotic genes. 22<sup>nd</sup> Congress of the European Committee for Treatment and Research of Multiple Sclerosis, Madrid, Spain, September 2006.

M.C. Magnone, B. Gréco, F. Richard, C. Ogier, H. Asnagli, V. Muzio, E. Brini, R.Furlan, G.V. Martino, **Martini P.G.V.**, J.Y. Gillon, T. Wells, P.F. Zaratini. Involvement of MMP-12 in an experimental autoimmune encephalomyelitis murine model of Multiple Sclerosis. Society for Neuroscience Meeting, Atlanta, GA, October 2006.

Pravettoni A., Mornati O., **Martini P.G.V.**, Colciago A., Celotti F., Motta M., Negri Cesi P. Effect of an estrogen receptor beta selective agonist on the prostate carcinoma cell line proliferation. 79<sup>th</sup> National Meeting of the Italian Society of Urology, Bologna, Italy, June 2006.

Limonta P., Montagnani Marelli M., **Martini P.G.V.**, Taylor D.M., Januszkiewicz J., Taraboletti G., Bani R.M., Giavazzi R., Moretti R.M. Expression of Gonadotropin-Releasing Hormone receptors in human malignant melanomas and regulation of gene expression associated with their antitumor activity. 97<sup>th</sup> Annual Meeting of the American Association for Cancer Research, Washington D.C, April 2006.

Grund E., **Martini P.G.V.**, Palmer S., Miller K., Scott R., de Matos D.G. Linear Amplification of RNA in the Picogram Range Present in a Single Human Blastomere for Microarray. Poster/Oral presentation, Annual Meeting of the American Society for Reproductive Medicine, Montreal, Quebec, Canada, October 2005.

Bathgate, R., He, C., **Martini, P.G.V.**, Ferraro, T., Layfield, S., Chevrier, C., Ryan, P., Muda, M. Identification and characterization of four novel splice variants of the relaxin (LGR7) and INSL3 (LGR8) receptors. W726, 38<sup>th</sup> Annual Meeting of The Society for the Study of Reproduction, Quebec City, Canada, July 2005.

Warren P., Bienkowska J., **Martini P.G.V.**, Jackson J., Taylor D. PANP - a New Method for Gene Detection for Oligonucleotide Expression Arrays. Late Breaking Poster Session E-10. Annual Meeting of the International Society for Computational Biology, Detroit, MI, June 2005.

**Martini P.G.V.**, Taylor D., McAllister G., Jackson J., Bienkowska J., Campbell R.K. Gene Expression Profiling of Rheumatoid Arthritis Tissue Reveals Signature of Disease Process and Progression. Late Breaking Poster Session C-11. Annual Meeting of the International Society for Computational Biology, Detroit, MI, June 2005.

Guerini V., Sau D., Rusmini P., Ciana P., Maggi A., **Martini P.G.V.**, Katzenellenbogen B., Motta M., Poletti A.. The Androgen Derivative 5alpha-androstane-3beta,17beta-diol (3beta-diol) inhibits migration of prostate cancer cell by activation of the estrogen receptor beta. P-297, 96<sup>th</sup> Annual Meeting of the America Association for Cancer Research, Anaheim, CA, April 2005.

**Martini P.G.V.**, He C., Chevrier C., Taylor D., Kelton C., Schweickhardt R., Muda M. Identification of novel splice variants of the relaxin receptors LGR7 and LGR8. P3-58, 86<sup>th</sup> Annual Meeting of the Endocrine Society, New Orleans, LA, June 2004.

Negri-Cesi P., **Martini P.G.V.**, Pravettoni A., Colciago A., Mornati O., Motta M. Possible involvement of the estrogen receptor beta in the modulation of DU145 prostate cancer cell proliferation. Abstract P2-411. 85<sup>th</sup> Annual Meeting of the Endocrine Society, Philadelphia, PA, June 2003

Rajendran R.R., Nye A.C., **Martini P.G.V.**, Katzenellenbogen B.S. A novel DEAD box RNA helicase that acts as a nuclear receptor coregulator. Symposium Abstract P3-614, 83<sup>rd</sup> Annual Meeting of the Endocrine Society, Denver, CO, June 2001.

**Martini P.G.V.**, Katzenellenbogen B.S. Prothymosin alpha gene expression regulation by estrogen in estrogen receptor-containing breast cancer cells via upstream half-palindromic estrogen response element motifs. Abstract P3-434. 83<sup>rd</sup> Annual Meeting of the Endocrine Society, Denver, CO, June 2001.

**Martini P.G.V.**, Katzenellenbogen B.S. Regulation of prothymosin alpha (Pt $\alpha$ ) gene expression by estrogen receptor in estrogen receptor-containing breast cancer cell and analysis of the Pt $\alpha$  gene regulatory regions. Hormones and Cancer 2000, Port Douglas, Australia, November 2000.

**Martini P.G.V.**, Delage-Mourroux R., Kraichely D.M., Katzenellenbogen B.S. Mechanisms of selective regulation of estrogen receptor activity by REA, a coregulator of estrogen receptor activity, and prothymosin alpha. ICE 2000, Sydney, Australia, October 2000.

Delage-Mourroux R., **Martini P.G.V.**, Kraichely D.M., Hoeksema J., Katzenellenbogen B.S. Analysis of estrogen receptor interaction with a repressor of estrogen receptor activity (REA) and the regulation of estrogen receptor transcriptional activity by REA. Keystone Symposium, Nuclear Receptor 2000, Steamboat Springs, CO, March 2000.

Katzenellenbogen B.S., **Martini P.G.V.**, Rajendran R.R., Delage Mourroux R., Sun J., Choi I., Kraichely D.M., Katzenellenbogen J.A. Estrogen Receptor alpha and beta: selective ligands and coregulator modulation of receptor activity. Keystone Symposium, Nuclear Receptor 2000, Steamboat Springs, CO, March 2000.

**Martini P.G.V.**, Katzenellenbogen B.S. Development of tissue-selective antiestrogens and identification of coregulators that enhances the effectiveness of antiestrogens. Susan G. Komen Breast Cancer Foundation Symposium, Dallas, TX, October 1999.

Katzenellenbogen B.S., Choi I., Delage Mourroux R., Ediger T.R., **Martini P.G.V.**, Montano M.M., Sun J., Weis K., Katzenellenbogen J.A. Molecular mechanisms of estrogen action: selective ligands and receptor pharmacology. Nobel Symposium on Estrogen and Women's Health: Benefit or Threat? Karlskoga, Sweden, July 1999.

Katzenellenbogen B.S., Ediger T.R., Sun J., Choi I., Weis K., **Martini P.G.V.**, Delage-Mourroux R., Katzenellenbogen J.A. Molecular mechanisms of estrogen action: receptor partners and cell biology. Frontiers in Estrogen Action, Los Angeles, CA, April 1999.

Katzenellenbogen B.S., Ediger T.R., Choi I., Sun J., Montano M.M., **Martini P.G.V.**, Delage-Mourroux R., Rajendran R.R. Transcriptional regulation by estrogen receptor and insights into the actions of antiestrogens. Proceedings Amer. Assoc. for Cancer Research 40: 752-753. April 1999

Katzenellenbogen B.S., Ediger T.R., Ekena K., Sun J., Choi I., Weis K., **Martini P.G.V.**, Montano M.M., Delage-Mourroux R. Molecular mechanisms of estrogen and antiestrogen action via estrogen receptor alpha and beta. Keystone Symposium on Molecular Pathogenesis of Bone Diseases, Lake Tahoe, CA, March 1999.

**Martini P.G.V.** and Katzenellenbogen B.S. Expedited development of more tissue-selective antiestrogen for breast cancer treatment and prevention. Reeves Symposium, Susan G. Komen Breast Cancer Foundation, Dallas, TX, October 1998.

Negri-Cesi P., Poletti A., Colciago A., Magni P., **Martini P.**, Motta M. The 5 $\alpha$ -reductase type 1 in the human prostate cancer cell line LNCaP: expression and possible control mechanisms. International Symposium on Biology of Prostate Growth, Washington DC, March 28-31, 1996.