CURRICULUM VITAE (updated 2023)

PERSONAL DATA

Name: Mario Amendola
Nationality: Italian
E-mail: mamendola@genethon.fr

WORKING EXPERIENCE

Nov 2022-pres	**Associate Professor** of Molecular Biology, University of Foggia, Italy
Dec 2016-pres	**Chargé de Recherche de 1ère classe (CR1) INSERM,** UMR_S951, Evry, FR.
July 2015-pres	**Team Leader** of the 'Therapeutic genome editing' group, Genethon, Evry, FR.
May 2010-Jan 2015	**Postdoctoral fellow,** The Netherlands Cancer Institute, Amsterdam, NL (Supervisor Prof. Bas van Steensel). Topic: 'Role of Lamins and Nuclear Envelope Proteins in **Genome Organization and Gene Regulation**'.
Sep 2007-May 2010	**Postdoctoral fellow**, HSR-TIGET, Milan, IT (Supervisor Prof. Luigi Naldini). Topic: 'Development of a **Lentiviral Platform** for Delivery of **microRNA** and microRNA-based **siRNA** into Primary Cells' and 'Exploiting endogenous **microRNA regulation** for **hematopoietic stem cell** specific transgene expression'
Aug 2005-Nov 2005	**Ph.D.** visiting, Salk Institute, San Diego, USA (Supervisor Prof. Fred H. Gage). Topic: 'Hippocampal Neural **Stem Cells**'.
Jan 2003-Sep 2007	**Ph.D.** student, HSR-TIGET, Milan, IT (Supervisor Prof. Luigi Naldini). Topic: '**Gene Transfer and Therapy** of the Central Nervous System'.
Sep 1999-Dec 2002	**M.Sc.** student, Cancer Institute, Candiolo, IT (Supervisor Prof. Luigi Naldini). Topic: 'Development of **bidirectional promoter**'.

EDUCATION

12/12/2018	**HDR** habilitation to lead research (Habilitation à diriger des recherches): Université Evry Val-d'Essonne, Université Paris-Saclay
17/09/2007	**Ph.D.** in **Molecular Medicine** (top-ranking candidate), University Vita e Salute, San Raffaele Hospital, Milan, IT. Dissertation on "Development of New Lentiviral Vectors for Coordinate or Self-Regulated Gene Expression and Application to CNS Delivery".
July 2002	**MSc**. (5-year course) in **Medical Biotechnology,** University of Turin, Turin, IT. 1st Class Honors (full marks and exceptional honors), Dissertation on "Lentiviral Vectors for the Coordinate and Regulated Expression of More Transgenes".

ADDITIONAL COURSES

- 2020: "Animal experimentation course for project leaders", 7 days (INSERM, Paris, Fr)

Management:
- 2021 INSERM (coherences) Managing a team, 30 hours (https://www.coherences.net/)
- 2018 Ecole POP, la formation projet, 2 days
- 2016 EMBO Laboratory Management Course for Group Leaders, 4 days (http://lab-management.embo.org/course-overview#researchleadership)

Bioinformatics and statistics courses:
- 2015: 'Getting Cleaning and Data', 4 weeks (www.coursera.org/course/getdata)
- 2014: 'An Introduction to Python', 9 weeks (www.coursera.org/course/interactivepython)
- 2013: 'Data Analysis', 8 weeks (www.coursera.org/course/dataanalysis).
- 2012: 'Computing for Data Analysis', 4 weeks (www.coursera.org/course/compdata).
- 2012: 'Statistic One', 8 weeks (www.coursera.org/course/stats1).

- 2010: 'R programming', 2 weeks (at the Netherlands Cancer Institute, Amsterdam, NL).

AWARDS & HONORS

- 2022: **Travel grant** (PI), American Society of Gene and Cell Therapy Meeting, Washington, USA
- 2020: **Travel grant** (PI), American Society of Gene and Cell Therapy Meeting, Boston, USA
- 2019: **Travel grant** (PI), American Society of Gene and Cell Therapy Meeting, Washington, USA
- 2019: **Excellence in Research Award** (PI), American Society of Gene and Cell Therapy Meeting, Washington, USA
- 2018: French **Qualification as Research Director (HDR)**
- 2017: Italian National Academic **Qualification as Associate Professor**
- 2016: **Chargé de Recherche de 1ère classe (CR1)** INSERM (France)
- 2011-2013: **EMBO Long Term Fellowship,** postdoctoral fellowship
- 2009: **Excellence in Research Award**, American Society of Gene Therapy Meeting, San Diego
- 2009: **Travel grant**, American Society of Gene Therapy Meeting, San Diego, USA
- 2008: **Travel grant**, American Society of Gene Therapy Meeting, Boston, USA
- 2006: **Travel grant**, American Society of Gene Therapy Meeting, Baltimore, USA
- 2003: **Travel grant**, American Society of Gene Therapy Meeting, Washington, USA
- 2003: **PhD fellowship,** San Raffaele Vita-Salute University (top candidate), Milan, IT
- 2003: **"Premio Luigi Casati"**, top three scientific master thesis, Perugia, IT
- 1998 - 2002: O.N.A.O.S.I. **Fellowship**, for proficient study progress. Yearly for 5 years

INTELLECTUAL PROPERTY RIGHTS

1. Title: "Lysosomal acid lipase variants and use thereof"
   Inventors: **M. Amendola,** M. Laurent (EP20306524.8; 09/12/2020)
2. Title: "Enhancing utrophin expression in the cell by inducing mutations within the regulatory elements and therapeutics use thereof"
   Inventors: **M. Amendola,** S. Guiraud (EP20306112.2; 29/09/2020)
3. Title: "Bifunctional lentiviral vectors allowing the bs-globin silencing and expression of an anti-sickling HBB and uses thereof for gene therapy of sickle cell disease"
   Inventors: M. Brusson, F. Mavilio, M. Cavazzana, **M. Amendola**., A. Miccio. (EP20306075.1; 22/09/2020)
4. Title: "Precise integration using nuclease targeted IDLV"
   Inventors: **M. Amendola,** A. Sakkal, G. Pavani (EP19305967.2; 22/07/2019)
5. Title: "Correction of beta-thalassemia phenotype by genetically engineered hematopoietic stem cells "
   Inventors: **M. Amendola,** G. Pavani,. (EP19305484.8, 15/04/2019)
6. Title: "Bifunctional vectors allowing Bcl11A silencing and expression of an anti-sickling HBB and uses thereof for gene therapy of B-hemoglobinopathies"
   Inventors: M. Brusson, F. Mavilio, M. Cavazzana, **M. Amendola**., A. Miccio. (EP19305356.8; March 22, 2019)
7. Title: "Recombinant vectors suitable for the treatment of IPEX syndrome"
   Inventors: M. Cavazzana, **M. Amendola**, E. Six, I. André (EP193051331; 05/02/2019)
8. Title: "Recombinant vectors suitable for the treatment of IPEX syndrome"
   Inventors: M. Cavazzana, **M. Amendola**, E. Six, I. André (EP18306526.7; 20/11/2018)
9. Title: "Genetically engineered hematopoietic stem cell as a platform for systemic protein expression"
   Inventors: **M. Amendola,** G. Pavani. (EP18305026.9, 12/01/2018)
10. Title: "Lentiviral vectors carrying synthetic bi-directional promoters and uses thereof"
    Inventors: L. Naldini, **M. Amendola**, E. Vigna. (WO2004094642, 04/09/2004)

FUNDING

Competitive grants:

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- 2023-2027: Horizon EU Grants, co-PI and WP Leader
- 2022: DIM Gene Therapy, SafeSCD, instrument funding, PI
- 2023-2025: ANR-PRC, PEMGeT, partner
- 2022-2027: Horizon EU Grants (101057659 - GAP-101057659), partner
- 2022-2024: Paris Region PhD 2021, PI
- 2022-2024: ANR-PRC, (ANR-21-CE14-0063 IRIS), partner
- 2021-2023: ANR-PRC, (ANR-20-CE17-0016-01 HemoLen), project coordinator
- 2020-2022: Postdoctoral fellowship Genopole, Supervisor
- 2016-2020: Fondagen Grant, PI, start-up grant
- 2017-2020: ANR-PRC (ANR-16-CE18 STaHR), partner
- 2017-2020: BHAP, Bayer, supervisor
- 2019-2020: Marie Curie postdoctoral fellowship, supervisor
- 2011-2013: EMBO Long Term postdoctoral Fellowship, PI

Support:
- INSERM
- AFM-Telethon

UNIVERSITY TEACHING RESPONSIBILITIES

2023-: Board of the XXXIX PhD cycle in "Basic and Clinical Neuroscience", University of Foggia

Italian National Academic Qualification as Associate Professor in:
- Genetics (06/02/2023-06/02/2033): (Bio/8) 05/I1 prima fascia
- Histology (24/12/2019-24/12/2028): (Bio/17) 05/H2 seconda fascia
- Molecular biology (07/12/2017-07/12/2023): (Bio/11) 05/E2 seconda fascia
- Genetics (05/12/2017-05/12/2023): (Bio/8) 05/I1 seconda fascia
- Applied Biology (18/09/2018-18/09/2024): (Bio/13) 05/F1 seconda fascia
- Applied medical technologies (15/10/2018-15/10/2024): (Med/46) 06/N1 seconda fascia

Habilitation à diriger des recherches (HDR): Université Evry Val-d'Essonne, Université Paris-Saclay 12/12/2018

Member of the jury:
- HDR: Sonia Albini, Université Evry Val-d'Essonne, Université Paris-Saclay 08/04/2021 (rapporteur)
- HDR: Giuseppe Ronzitti, Université Evry Val-d'Essonne, Université Paris-Saclay 10/07/2020

- PhD: Giulia Hardouin, hematologie oncogenese et biotherapies, Université de Paris, France (05/12/2022)
- PhD: Juliette Rosier, Ecole Doctorale des Sciences de la Vie et de la Sante, Université de Bordeaux, France (rapporteur 12/12/2022)
- PhD: Federica Cascino, PhD course in Molecular Medicine, Universita Vita-Saluta San Raffaele, Milan, Italy (rapporteur 15/12/2022)
- PhD: Patrice Vidal, Ecole Doctorale «Complexité du vivant», Sorbonne Université, Paris, France (rapporteur 12/12/2018);
- PhD: Alessia De Caneva, PhD course in Biology and Biotechnology, Universita degli Studi di Ferrara, Ferrara, Italy (22/03/2018)
- PhD: Aurelien Jacob, PhD in translational and molecular medicine, Universita degli studi di Milano-Bicocca, Italia (2020)
- PhD: Antoine Merien, Université Evry Val-d'Essonne, Université Paris-Saclay (2020)

External PhD surpervisor:

- Halegua Thibaut, Ecole Doctorale Biologie Moléculaire Intégrative et Cellulaire, Université de Lyon, France
- Giulia Hardouin, Ecole Doctorale hematologie oncogenese et biotherapies, Université de Paris, France
- Panagiotis Antoniou, Ecole Doctorale hematologie oncogenese et biotherapies, Université de Paris, France
- Anne Chalumeau, Ecole Docorale hematologie oncogenese et biotherapies, Université de Paris, France
- Chiara Simoni, San Raffaele Ph.D. program

Teaching:

- Academic years: 2022/2023
  Level: undergraduate students
  Course: Master Clinical and Experimental Biology (coordinator Pr. Claudia PICCOLI)
  University: Universita degli Studi di Foggia, Italy
  Duration: 48h
  Course title: Advanced methods in molecular biology

- Academic years: 2019/2020; 2020/2021
  Level: undergraduate students
  Course: Master BME/MCB (coordinator Pr. HACEIN-BEY Salima)
  University: Faculté de Pharmacie de Paris, France
  Duration: 3h
  Course title: Genome editing: general concepts and tools

- Academic years: 2020/2021
  Level: undergraduate students
  Course: Master in Biosante (coordinator Pr. Prats)
  University: l'Université de Toulouse, Francia
  Duration: 3h
  Course title: Genome editing: general concepts and CRISPR

- Academic years: 2018/2019, 2019/2020
  Level: undergraduate students
  Course: Cellular and Molecular Biotherapies (Faculté de Pharmacie)
  University: Université de Paris Descartes, France
  Duration: 2h
  Course title: Therapeutic genome editing

- Academic years: 2017/2018; 2018/2019, 2019/2020, 2020/2021
  Level: undergraduate students
  Course: BTCG Master (coordinator Pr. Perea/Pr Baldeschi)
  University: l'Université d'Evry-Val-d'Essone, France
  Duration: 2-3h
  Course title: Genome editing

- Academic years: 2003/2004, 2004/2005, 2005/2006
  Level: undergraduate students
  Course: molecular and cellular medical biotechnology, Master (coordinator Pr. Naldini)
  University: University Vita-Salute San Raffaele, Milano, Italy
  Duration: 4 hours
  Course title: Gene Therapy

SCIENTIFIC PRESENTATIONS

Chairmen:

- 18/05/2022: Annual congress of American Society of Gene and Cell Therapy (ASGCT) 2022

- 16/05/2021: Annual congress of the American Society of Gene and Cell Therapy (ASGCT) 2021
- 21/10/2021: Annual congress of the European Society of Gene and Cell Therapy (ESGCT) 2021
- 19/03/2020: Séminaire Institut des Biothérapies, Paris, France
- 29/11/2019: Symposium BioTherAlliance, Evry, France

Invited Speaker:

- 12/06/2023: French society for inborn errors of metabolism
  "Gene therapy for lysosomal acid lipase deficiency"
- 22/11/2022: University of Foggia, Italy
  "Editing hematopoietic stem cells for treating genetic disease"
- 24/11/2022: Cité des sciences et de l'industrie museum, Paris, France
  "Réparer les gènes : du concept au médicament"
- 31/03/2022: CRISPR and Translational Medicine congress, Bordeaux, France
- 20/05/2021: Young Researchers of Auvergne Rhône Alpes Region Congress, France
  Round table on "CRISPR-Cas9/OGM and genetic manipulation in humans"
- 20/05/2021: Cercle des Pharmaciens et Dirigeants des Industries de Santé
  "Thérapie génique et CRISPR CAS9 : du concept génétique au médicament!"
- 25/01/2021: Institute de Myologie, Paris, France
  "Ex vivo editing of human hematopoietic stem cells for erythroid expression of therapeutic proteins"
- 08/10/2020: Fete de la science 2020
  «Les ciseaux génétiques, une arme pour guérir demain ? »
- 25-27/03/2020: Inserm workshop, Bordeaux, France
  "CRISPR-Cas9: yet more breakthroughs and challenges"
- 27/01/2020: Imagine Institute, Paris, France
  "Ex vivo editing of hematopoietic stem cells for erythroid-specific expression of therapeutic proteins"
- 19/03/2020: Séminaire Institut des Biothérapies, Paris, France
- 29/11/2019: Symposium BioTherAlliance, Evry, France
- 13/11/2019: Sanofi CRISPR Symposium, Vitry-sur-Seine, France
- 01/07/2019: International Center for Genetic Engineering and Biotechnology (ICGEB), Trieste, Italy
- 05-07/06/2019: Instituto de Investigação e Inovação da Universidade do Porto, Porto, Portugal
  Workshop on "Genome editing using CRISPR-Cas9"
- 24/05/2019-24/05/2019: 3 Edition Femmes, Fibromes et Fertilité; Évian-les-Bains, France
  "Édition du génome humain : quand la science- fiction devient réalité"
- 14/05/2019: UCL, Institute of Child Health; London, UK
  "Ex vivo editing of hematopoietic stem cells for erythroid-specific expression of therapeutic proteins"
- 30/01/2019: Université de Nantes, Nantes, France
  "Ex vivo editing of hematopoietic stem cells for erythroid-specific expression of therapeutic proteins"
- 2017: Imagine Institute, Paris, France
  "Empowering hematopoietic stem cells for protein replacement therapy"
- 2015: Genethon, Evry, France
  "Nuclear Lamina-Chromatin interactions"
- 2009: Institute of Molecular Biotechnology, Vienna, Austria
  "New Lentiviral Vectors for Co-ordinate Transgene Expression and miRNA/siRNA Delivery"

Selected oral presentations (as first name):

- 2009: American Society of Gene Therapy, 12th Annual Meeting
  Travel grant + Excellence in research award
- 2008: American Society of Gene Therapy, 11th Annual Meeting
  Travel grant
- 2006: American Society of Gene Therapy, 9th Annual Meeting
  Travel grant
- 2004: American Society of Gene Therapy, 7th Annual Meeting

Travel grant
- 2003: American Society of Gene Therapy, 6th Annual Meeting

Selected oral presentations (as last name):
- 2022: American Society of Gene and Cell Therapy, 12th Annual Meeting
  Travel grant
- 2021: French society of cytometry, 24th Annual meeting
- 2021: European Society of Gene and Cell Therapy, 28th Annual Meeting
- 2020: American Society of Gene and Cell Therapy, 13th Annual Meeting
  Travel grant
- 2019: American Society of Gene and Cell Therapy, 12th Annual Meeting
  Travel grant + Excellence in research award
- 2019: American Society of Gene and Cell Therapy, 11th Annual Meeting
  Travel grant

Selected international poster presentation (as first name):
- 2013: EMBO Nuclear Structure and Dynamics meeting

SCIENTIFIC COMMITMENTS:

- 2023: **Member** of the viral vector committee to identify speakers for the Brussels ESCGT meeting
- 2023-pres: **Scientific leader** of Genethon Bioinformatic platform
- 2022-pres: **Associate Editor** in "Genome Editing in Human Health and Disease" (Frontiers in Genome Editing)
- 2022-pres: Management **committee board** representing France for European Cooperation in Science and Technology (COST) action CA21113 (Genome Editing to Treat Human Diseases)
- 2022-pres: elected **Board Member** of the French society of Cell and Gene Therapy
- 2022: invited by the comité des médicaments de thérapie innovante (CAT), 'Agence nationale de sécurité du médicament et des produits de santé (ANSM)
- 2022-pres: **Member** of the INSERM French National Specialized Scientific Commission n 7 (CSS7), "Technologies for Health".
- 2022-pres: **Review Editor** in "Molecular Diagnostics and Therapeutics" (Frontiers in Molecular Biosciences)
- 2022-pres: Genome editing **expert** for the Technological Research Accelerator in Genomic Therapy (ART-TG; INSERM US35)
- 2021: **Member** of the committee for the selection of ingegnieur de recherche N°2 BAP A INSERM
- 2021-pres: **Member** of Think Thanks Gene & Cell Therapy Institute
- 2020: **Reviewer** of the abstracts in the 'RNA Virus Vectors' category for the American Society of Gene and Cell Therapy Meeting
- 2020-pres: **Member** of the Association for Responsible Research and Innovation in Genome Editing" (ARRIGE)
- 25/03/2021-2022: **Associate Editor** in "Genome Editing in Blood Disorders" (Frontiers in Genome Editing)
- 28/10/2019-25/03/2021: **Review Editor** in "Genome Editing in Blood Disorders" (Frontiers in Genome Editing)
- 21/04/2017|-pres: **Review Editor** in "Gene and Cell Therapies" (Frontiers in Medicine)
- **Journal reviewer** for: Trends in Biotechnology (1), BMC genomics (1), Nucleic Acids Research (1), Nature Biotechnology (1), Nature Medicine (1), Nature Communication (2), Science Translational Medicine (1), Journal of clinical investigation (2), Blood (1), Molecular Therapy (12), Molecular Therapy Nucleic Acid (1), Molecular Therapy: Methods & Clinical Development (1), Gene Therapy (1), Human Gene Therapy (14), Journal of Virology (1), Scientific Reports (5), Frontiers in Medicine (2), Frontiers in Genome Editing, Methods (2), Stem Cells (2), Comp Struct Biotech (1), iScience (1).
- **Grant reviewer** for: ERC (1), ANR (2), Italian Science Fund (FIS), MRC (2), GOSH Children's Charity (4), Wellcome Trust (1), IFCAH (1), Cyprus RPF (5), Rosetrees Trust (1), AFM-Telethon (1), Agence de la biomédecine (1)
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- 2016-pres: Organizer of the Lecture Series at Genethon
- 2016-pres: Member of the Genethon postdoctoral program committee
- 2013: Volunteer for the 5th EMBO Meeting 2013, Amsterdam, NL
- 2012: Organization of Chromatin Dynamics lab retreat, Netherlands Cancer Institute, Amsterdam, NL
- 2000: Assistant tutor EMBO Practical Course "Lentiviral Vectors for Gene Transfer", Istituto di Candiolo, Turin, IT

Interviews and press release:

- 2023: Interview for Le Monde: « Thérapies géniques : le ciseau moléculaire Crispr livre ses premiers »
- 2022 : Interview for the Genethon website on the Horizon-RIA grant (https://www.genethon.com/sickle-cell-anemia-a-project-involving-the-gene-editing-team-has-just-secured-european-funding/)
- 2022: Preparation of educational materials (fact sheets) on gene therapy for Duchenne Muscular Dystrophy and Spinal Muscular Atrophy and in the frame of the initiative of the "Education" group of the Gene and Cell therapy Institute (https://www.gcti.fr/).
- 2022: The Washington Post, "With CRISPR gene editing, unique treatments begin to take off for rare diseases" by Meeri Kim; https://www.washingtonpost.com/health/2022/02/05/crispr-rare-diseases/
- 2022: Le Parisien, "Altos Labs, la start-up à 3milliards de dollars qui se lance dans la quête de l'éternité" by Damien Licata Caruso ; https://www.leparisien.fr/high-tech/altos-labs-la-start-up-a-3-milliards-de-dollars-qui-se-lance-dans-la-quete-de-leternite-06-02-2022-SKLCKSON25G2NIKOPO6MTG2A7I.php
- 2021: Press release/interview for News on a gene therapy approach developed in our lab (Pavani 2019 and 2020 "CRISPR Enables Plug-and-Play Expression of Therapeutic Transgenes in Blood Cells" by Gorm Palmgren (https://crisprmedicinenews.com/news/crispr-enables-plug-and-play-expression-of-therapeutic-transgenes-in-blood-cells/)
- 2021: Invited blog article for Nature Bioengineering community on a gene therapy approach developed in our lab (Pavani, 2020) https://bioengineeringcommunity.nature.com/posts/redit-engineering-erythroid-cells-for-therapeutic-applications
- 2020: Press release/interview for INSERM on a gene therapy approach developed in our lab (Pavani, 2020) https://presse.inserm.fr/en/a-novel-genome-editing-tool-for-rare-hereditary-diseases/40579/
- 2018: Youtube video "Fondagen participe au financement des recherches de Mario Amendola sur la drépanocytose" by Genopole
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MEMBERSHIP

- 2015-pres: European Society of Gene and Cell Therapy
- 2003-pres: American Society of Gene and Cell Therapy

SCIENTIFIC PRODUCTION

- 33 publications in peer-reviewed journals: 28 original papers and 4 reviews; (6 first author; 3 corresponding author),
- 10 patents
- Cumulative citations index: 7191;
- H index: 25

(source google scholar)

PUBLICATIONS

1. Novel cytometry based characterization of lysosomal storage disease affected patient's cells
   Laurent M, Cosette J, Pavani G, Bayol S, Stockholm D, and **Amendola M**.
   In preparation

2. Brusson M, Chalumeau A, Martinucci P, Romano O, Poletti V, Scaramuzza S, Ramadier S, Masson C, Ferrari G, Mavilio F, Cavazzana M, **Amendola M**, and Miccio A.
   Novel lentiviral vectors for gene therapy of sickle cell disease combining gene addition and gene silencing strategies
   Mol Ther Nucleic Acids. 2023 Mar 22;32:229-246

3. Antoniou P, Hardouin G, Martinucci P, Frati G, Felix T, Chalumeau A, Fontana L, Martin J, Masson C, Brusson M, Maule G , Rosello M, Abramowski V, de Villartay JP, Concordet JP, Del Bene F, El Nemer W, **Amendola M**, Cavazzana M, Anna Cereseto, Romano O, Miccio A.
   Base editing-mediated dissection 1 of a γ-globin cis-regulatory element for reactivation of therapeutic fetal hemoglobin
   Nat. Commun. 2022 Nov 4;13(1):6618.

4. **Amendola M,** Brusson M, Miccio A.
   CRISPRthripsis: the risk of CRISPR/Cas9-induced chromothripsis in gene therapy
   Stem Cells Transl. Med. 2022 Oct 21;11(10):1003-1009.

5. Ramadier S, Chalumeau A, Felix T, Othman N, Aknoun S, Casini A, Maule G, Masson C, De Cian A, Frati G, Brusson M, Concordet JP, Cavazzana M, Cereseto A, El Nemer W, **Amendola M**, Wattellier B, Meneghini V, Miccio A.
   Combination of lentiviral and genome editing technologies for the treatment of sickle cell disease.
   Mol Ther. 2022 Jan 5;30(1):145-163.

6. **Amendola M**, Bedel A, Buj-Bello A, Carrara M, Concordet JP, Frati G, Gilot D, Giovannangeli C, Gutierrez-Guerrero A, Laurent M, Miccio A, Moreau-Gaudry F, Sourd C, Valton J, Verhoeyen E.
   Recent Progress in Genome Editing for Gene Therapy Applications: The French Perspective.
   Hum Gene Ther. 2021 Oct;32(19-20):1059-1075.

7. Pavani G., Fabiano A., Laurent M., Amor F., Cantelli E., Chalumeau A., Concordet J.P., Mavilio F., Ferrari G., Miccio A., **Amendola M.**
   Correction of β-thalassemia by CRISPR/Cas9 editing of the α-globin locus in human hematopoietic stem cells
   Blood Advances, 2021 Mar 9;5(5):1137-1153

8. Delville M, Bellier F, Leon J, Klifa R, Lizot S, Vinçon H, Sobrino S, Thouenon R, Marchal A, Garrigue A, Olivre J, Charbonnier S, Lagresle-Peyrou C, **Amendola M**, Schambach A, Gross DA, Lamarthée B, Benoist C, Zuber J, André I, Cavazzana M, Six E.
   A combination of cyclophosphamide, interleukin-2 allow CD4+ T cells converted to Tregs to control scurfy syndrome.
   Blood, 2021 Apr 29;137(17):2326-2336

9. Pavani G., **Amendola M.**
   Targeted gene delivery: where to land
   Front. Genome Ed., 2021 Jan 20;2:609650

10. Pavani G., Laurent M., Fabiano A., Cantelli E., Sakkal A., Corre G., Lenting P.J., Concordet J.P., Toueille M., Miccio A., **Amendola M.**
    Ex vivo editing of human hematopoietic stem cells for erythroid expression of therapeutic proteins
    Nat Commun. 2020 Aug 13;11(1):4146
    Among the best INSERM publications in 2020
    https://presse.inserm.fr/en/a-novel-genome-editing-tool-for-rare-hereditary-diseases/40579/

11. Romano O., Petiti L., Felix T., Meneghini V., Portafax M., Antoniani C., **Amendola M**., Bicciato S., Peano C., Miccio A. GATA factor-mediated gene regulation in human erythropoiesis.

iScience. 2020 Mar 30;23(4):101018

12. Weber L, Frati G, Felix T, Hardouin G, Casini A, Wollenschlaeger C, Meneghini V, Masson C, De Cian A, Chalumeau A, Mavilio F, **Amendola M**, Andre-Schmutz I, Cereseto A, El Nemer W, Concordet JP, Giovannangeli C, Cavazzana M, Miccio A.
Editing a γ-globin repressor binding site restores fetal hemoglobin synthesis and corrects the sickle cell disease
Sci Adv. 2020 Feb 12;6(7):eaay9392

13. Lattanzi A., Meneghini V., Pavani G., Amor F., Antoniani C., Felix T., Lee C., Porteus M.H., Bao G., **Amendola M.**, Mavilio F., Miccio A.
Optimization of CRISPR/Cas9 Delivery to Human Hematopoietic Stem and Progenitor Cells for Therapeutic Genomic Rearrangements.
Mol Ther. 2019 Jan 2;27(1):137-150

14. Antoniani C., Meneghini V., Lattanzi A., Pavani G., Felix T., Amor F., Romano O., Magrin E., Weber L., Cradick T.J., Lundberg A.S., Porteus M, **Amendola M**., Cavazzana M., Mavilio F., Miccio A.
Induction of fetal hemoglobin synthesis by CRISPR/Cas9-mediated disruption of the β-globin locus architecture.
Blood. 2018 Apr 26;131(17):1960-1973.
F1000 *Prime recommended*
Comment by Montbleau KE and Sankaran VG. ***Blood****. 2018 Apr 26;131(17):1884-1885.*

15. Haarhuis J.H.I., van der Weide R.H., Blomen V.A., Yáñez-Cuna J.O., **Amendola M**., van Ruiten M.S., Krijger P.H.L., Teunissen H., van Ruiten M.S., Medema R.H., van Steensel B, Brummelkamp T.R., de Wit, Rowland B.D.
The cohesin release factor WAPL restricts chromatin loop extension.
Cell 2017 May 4;169(4)
F1000 *Prime recommended*

16. Kind J., Pagie L., de Vries S.S., Nahidiazar L., Dey S.S., Bienko M., Zhan Y., Lajoie B., de Graaf C.A., **Amendola M**., Fudenberg G., Imakaev M., Mirny L.A., Jalink K., Dekker J., van Oudenaarden A., van Steensel B.
Genome-wide Maps of Nuclear Lamina Interactions in Single Human Cells.
Cell 2015 Sep 24;163(1):134-47
F1000 *Prime recommended*

17. **Amendola M**., van Steensel B.
Nuclear Lamins are Not Required for Genome Organization in Mouse Embryonic Stem Cells.
Embo Rep. 2015 May; 16(5):610-7.
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CONGRESS

Oral presentations
Congress Reims

1. *Ex vivo* editing of hematopoietic stem cells for erythroid expression of therapeutic proteins in vivo for LAL-D therapy.
   Marine Laurent, Giulia Pavani, Christine Jenny, Anna Fabiano, Mario Amendola
   Travel grant award, ASGCT 2022
2. ASGCT 2022Multi-modal differentiation of lysosomal acid lipase deficient and gene corrected cells: from flow cytometry to image cytometry
   Marine Laurent, Giulia Pavani, Sarah Bayol, Daniel Stockholm, Jeremie Cosette and Mario Amendola
   Congrès de la Société Française d'Immunologie et de l'Association Française de Cytométrie 2021
   Finaliste pour le prix jeune cytométriste
3. *Ex vivo* editing of hematopoietic stem cells for erythroid-specific expression of therapeutic proteins
   Giulia Pavani, Marine Laurent, Erika Cantelli, Aboud Sakkal, Guillaume Corre, Peter Lenting, Jean-Paul Concordet, Magali Toueille, Annarita Miccio, Mario Amendola
   ASGCT 2020
   Travel grant award
4. Correction of β-thalassemia phenotype by CRISPR/Cas9 editing of the human α globin locus
   Giulia Pavani, Erika Cantelli, Marine Laurent, Sophie Ramadier, Anne Chalumeau, Anna Fabiano, Jean-Paul Concordet, Fulvio Mavilio, Annarita Miccio, Mario Amendola
   ASGCT 2019
   Travel grant award
5. *Ex vivo* editing of hematopoietic stem cells for erythroid-specific expression of therapeutic proteins

Giulia Pavani, Marine Laurent, Erika Cantelli, Aboud Sakkal, Guillaume Corre, Peter Lenting, Jean-Paul Concordet, Magali Toueille, Annarita Miccio, Mario Amendola
ASGCT 2019
Travel grant award and Excellence in research award

6. Fetal hemoglobin de-repression following CRISPR-Cas9-mediated targeting of the γ-globin promoters
Leslie Weber, Vasco Meneghini, Tristan Felix, Carine Giovannangeli, Mario Amendola, Jean-Paul Concordet, Marina Cavazzana, and Annarita Miccio
ASGCT 2017
7. Induction of fetal hemoglobin synthesis by CRISPR/Cas9-mediated disruption of the β-globin locus architecture
Chiara Antoniani, Vasco Meneghini, Annalisa Lattanzi, Giulia Pavani, Tristan Felix, Fatima Amor, Oriana Romano, Elisa Magrin, Leslie Weber, Thomas J Cradick, Ante S Lundberg, Matthew Porteus, Mario Amendola, Marina Cavazzana, Fulvio Mavilio, and Annarita Miccio.
ASH 2016
8. A Distinguishing Gene Signature of Proangiogenic Tie2-Expressing Monocytes (TEMs) Uncovers Potential New Targets of Anticancer Therapy
Ferdinando Pucci, Mary Anna Venneri, Mario Amendola, Daniela Biziato, Roberta Mazzieri, Clelia Di Serio, Luigi Naldini, Michele De Palma
ASGCT 2009
9. Regulated and Multiple miRNA and siRNA Delivery into Primary Cells by a Lentiviral Platform
Mario Amendola, Laura Passerini, Ferdinando Pucci, Bernhard, Gentner, Rosa Bacchetta, Luigi Naldini.
ASGCT 2009
Travel grant award and Excellence in research award
10. Direct Intra-Brain LV-Mediated Gene Delivery Leads to Widespread Transgene Distribution and High-Level Enzymatic Correction in the Whole CNS of Twitcher Mice
Annalisa Lattanzi, Claudio Maderna, Mario Amendola, Lucia Sergi Sergi, Daniele Conti, Sabata Martino, Luigi Naldini,
Angela Gritti
ASGCT 2009
11. Stable Knock-Down of Endogenous microRNA In Vivo by Overexpressing miRNA Target Sequences by Lentiviral Vectors
Bernhard Gentner, Giulia Schira, Alessio Cantore, Mario Amendola, Luigi Naldini.
2008 ASGCT
12. A Robust and Efficient Lentiviral Platform for Delivery of MicroRNA and MicroRNA-Based siRNA into Primary Cells: Validation by Targeting FOXP3 in Human Regulatory T Cells
Mario Amendola, Laura Passerini, Grazia Andolfi, Lucia Sergi Sergi, Rosi Bacchetta, Maria Grazia Rondarolo, Luigi
Naldini.
2008 ASGCT
Travel grant award
13. LV Expressing MR Reporter Genes Allows *In Vivo* Monitoring of Stem Cell Gene Therapy
Mario Amendola, Letterio S. Politi, Marcello Cadioli, Rossella Galli, Elena Binda, Andrea Falini, Sonia Levi, Giuseppe Scotti, Alessandra Biffi and Luigi Naldini
2006 ASGCT
Travel grant award
14. New Synthetic Bidirectional Promoters in Lentiviral Vector for Efficient and Coordinated Expression of Two Genes within the Same Cell.
M. Amendola, M. A. Venneri, R. Loew, E. Vigna and L. Naldini
2004 ASGCT
15. New Lentiviral Vectors for Efficient and Coordinated Expression of Multiple Genes within the Same Cell.
M. Amendola, E. Vigna, R. Loew and L. Naldini

2003 ASGCT
Travel grant award

Posters

1. *In vivo* gene therapy for lysosomal acid lipase deficiency
   Marine Laurent, Christine Jenny, Julie Oustelandt, Anaïs Brassier, Francesca Landini, Consiglia Pacelli, Giuseppe Ronzitti and Mario Amendola
   ASGCT 2022
2. Characterization and quantification of CRISPR/Cas9-induced genomic alterations and AAV targeted integration in human HSPC.
   Alexandra Tachtsidi, Guillaume Corre, Laurie Lacombe, Mario Amendola.
   ASGCT 2022
3. Novel cytometry based characterization of lysosomal disease affected and gene corrected patient's cells
   Marine Laurent, Giulia Pavani, Sarah Bayol, Daniel Stockholm, Jérémie Cosette and Mario Amendola
   ASGCT 2022
4. Development of a utrophin modulation CRISPR-Cas9 strategy for Duchenne Muscular Dystrophy
   Simon Guiraud, Clara Fauveau, Fetta Mazed, Sumitava Dastidar, Fatima Amor, Giuseppe Ronzitti, Francesco Saverio Tedesco, Mario Amendola
   ESGCT 2021
5. *Ex vivo* editing of human hematopoietic stem cells for LAL-D therapy.
   Marine Laurent, Giulia Pavani, Christine Jenny, Anna Fabiano, Mario Amendola
   ESGCT 2021
6. MMEJ-mediated IDLV knock-in via CRISPR/Cas9 in human hematopoietic stem/progenitor cells
   Aboud Sakkal, Giulia Pavani, Anne Galy, Amendola Mario
   ESGCT 2021
7. *Ex vivo* editing of human hematopoietic stem cells for erythroid expression of therapeutic proteins
   Giulia Pavani, Marine Lauren, Erika Cantelli, Anna Fabiano, Aboud Sakkal, Guillaume Corre, Peter Lenting, Jean-Paul Concordet, Magali Toueille, Annarita Miccio, Mario Amendola
   CSHL 2021
8. Novel lentiviral vectors for gene therapy of sickle cell disease combining gene addition and gene silencing strategies
   Mégane Brusson, Anne Chalumeau, Pierre Martinucci, Fulvio Mavilio, Marina Cavazzana, Mario Amendola, Annarita Miccio
   ASGCT 2021
9. A Novel Lentiviral Vector for Gene Therapy of β-Hemoglobinopathies: Co-Expression of a Potent Anti-Sickling Transgene and a microRNA Downregulating BCL11A
   Mégane Brusson, Anne Chalumeau, Pierre Martinucci, Luiz Guilherme Darrigo, Fulvio Mavilio, Marina Cavazzana, Mario Amendola, Annarita Miccio
   ASGCT 2020
10. Correction of β-Thalassemia Phenotype by Editing the Human α Globin Locus to Modulate α and β Globin Expression
    Giulia Pavani, Anna Fabiano, Marine Laurent, Fatima Amor, Erika Cantelli, Anne Chalumeau, Jean-Paul Concordet, Fulvio Mavilio, Giuliana Ferrari, Annarita Miccio, Mario Amendola
    ASGCT 2020
11. Scurfy CD4 T Cells Converted to Regulatory T Cells by FOXP3 Gene Transfer Rescue Scurfy Mice after the Onset of the Disease
    Marianne Delville, Florence Bellier, Roman Klifa,Sabrina Lizot, David Zemmour, Juliette Leon, Steicy Sobrino, Romane Thouenon, Armance Marchal, Alexandrine Garrigue, Soeli Charbonnier, Juliette Olivré, Axel Schambach, Mario Amendola, Chantal Lagresle, David Gross, Baptiste Lamarthée, Christophe Benoist, Julien Zuber, Marina Cavazzana, Isabelle André, Emmanuelle Sic
    ASGCT 2020

12. Ex Vivo Editing of Human Hematopoietic Stem Cells for Erythroid-Specific Expression of Therapeutic Proteins
    Giulia Pavani, Marine Laurent, Erika Cantelli, Anna Fabiano, Aboud Sakkal, Guillaume Corre, Peter Lenting, Jean-Paul Concordet, Magali Toueille, Annarita Miccio, Mario Amendola
    ASGCT 2020
13. Unwanted DNA Methylation and Transcriptomic Changes Can be Induced in CD34+ Cells by Vectors During the Ex-Vivo Gene-Modification Process
    Guillaume Corre, Mirella Mormin, Giulia Pavani, Mario Amendola, Anne Galy
    ASGCT 2020
14. Fetal Hemoglobin De-Repression Following CRISPR/Cas9 Mediated Targeting of the γ-Globin Promoters as a Therapeutic Strategy for β-Hemoglobinopathies
    Giacomo Frati, Leslie Weber, Tristan Felix, Antonio Casini, Clara Wollenschlaeger, Vasco Meneghini, Mario Amendola, Anna Cereseto, Wassim El-Nemer, Jean-Paul Concordet, Carine Giovannangeli, Marina Cavazzana, Annarita Miccio
15. Targeting the hemoglobin loci in hematopoietic stem cells for systemic protein expression
    Giulia Pavani, Erika Cantelli, Aboud Sakkal, Peter Lenting, Jean-Paul Concordet, Annarita Miccio, Fulvio Mavilio, Mario Amendola
    ASGCT 2018
16. Viral and non-viral delivery of the CRISPR-Cas9 system in human hematopoietic stem and progenitor cells
    Annalisa Lattanzi, Vasco Meneghini, Giulia Pavani, Fatima Amor, Chiara Antoniani, Ciaran Lee, Matthew Porteus, Thomas J Cradick, Ante S Lundberg, Gang Bao, Mario Amendola, Fulvio Mavilio, Annarita Miccio
    ASGCT 2017
17. Optimization of lentiviral vectors for Factor VIII erythroid-specific expression
    Pavani Giulia, Erika Cantelli, Sabine Charrier, Samia Martin, Miccio Annarita, Mavilio Fulvio, Amendola Mario
    Gordon conference Red cells 2017
18. Induction of fetal hemoglobin synthesis in human erythroblasts by CRISPR/Cas9-mediated editing of the β-globin locus
    Chiara Antoniani, Vasco Meneghini, Annalisa Lattanzi, Tristan Felix, Oriana Romano, Elisa Magrin, Leslie Weber, Giulia Pavani, Thomas J Cradick, Ante S Lundberg, Mario Amendola, Wassim El-Nemer, Marina Cavazzana, Fulvio Mavilio, and Annarita Miccio.
    ASGCT 2017
19. Viral and non-viral delivery of CRISPR/Cas9 in human Hematopoietic Stem and Progenitor Cells (HSPC)
    Annalisa Lattanzi, Chiara Antoniani, Ciaran Lee, Giulia Pavani, Fatima Amor, Matthew Porteus, Gang Bao, Mario Amendola, Fulvio Mavilio, Annarita Miccio
    ESGCT 2016
20. Optimization of dual-gRNA lentiviral vectors for targeted genomic deletions
    Pavani Giulia, Lattanzi Annalisa, Amor Fatima, Antoniani Chiara, Mavilio Fulvio, Miccio Annarita, Amendola Mario
    CSHL Genome Editing 2016
21. Induction of fetal hemoglobin in adult erythroblasts by genome editing of the β-globin locus
    Chiara Antoniani, Annalisa Lattanzi, Giulia Pavani, Vasco Meneghini, Elisa Magrin, Mario Amendola, Matthew Porteus, Marina Cavazzana, Fulvio Mavilio and Annarita Miccio
    ASGCT 2016
22. Optimization of dual-gRNA lentiviral vectors for targeted genomic deletions
    Pavani Giulia, Lattanzi Annalisa, Amor Fatima, Antoniani Chiara, Mavilio Fulvio, Miccio Annarita, Amendola Mario
    ASGCT 2016
23. Novel approach for investigating long non coding RNA-genomic DNA interactions

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24. Development of New MR-Reporter Genes for Stem Cell Therapies
    Sara Pizzi, Mario Amendola, Anna Cozzi, Andrea Falini, Angela Gritti, Sonia Levi, Giuseppe Scotti, Alessandra Biffi, Luigi Naldini, Letterio S. Politi.
    2008 ASGCT
25. Efficient Tet-Dependent Expression of Human Factor IX In Vivo by a New Self-Regulated Lentiviral Vector
    Elisa Vigna, Mario Amendola, Fabrizio Benedicenti, Antonia Follenzi, Luigi Naldini.
    2003 ASGCT