

PERSONAL INFORMATION

Name: Anna Kajaste-Rudnitski, PhD

Email: anna.kajaste@unipv.it

Date of birth: 20/12/1980

Nationality: Finnish

Unique identifiers: <http://orcid.org/0000-0003-1549-2426>

<https://www.scopus.com/authid/detail.uri?authorId=8344219300>

EDUCATION

- 2006 Ph.D. in Biochemistry and Molecular Biology at the Université Pierre-et-Marie Curie, Paris VI (Paris, France) under the direction of Dr. Philippe Desprès.
- 2004 M.Sc. in Microbiology/Virology at Université Pierre-et-Marie Curie, Paris VI / Ecole Normale Supérieure (Paris, France) under the direction of Dr. Philippe Desprès.
- 1999 Baccalaureate (Scientific Program) at the Lycée Franco-Finlandais d’Helsinki, Helsinki, Finland.

CURRENT POSITION

From Oct 1st, 2023:

Full Professor of Molecular Biology, Department of Biology and Biotechnologies, University of Pavia, Pavia, Italy

PREVIOUS POSITIONS

- 2016 – 2023 Group Leader, Retrovirus-Host Interactions and Innate Immunity to Gene Transfer
San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)
Ospedale San Raffaele (OSR), Milan, Italy
- 2012 – 2016 Project Leader, Retrovirus-Host Interactions and Innate Immunity to Gene Transfer
San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)
Ospedale San Raffaele (OSR), Milan, Italy
- 2006 – 2012 Postdoctoral fellow in the Unit of Dr. Elisa Vicenzi at the Division of Immunology, Transplantation and Infectious Diseases, OSR, Milan, Italy

FELLOWSHIPS AND AWARDS

- 2004 Three-year PhD fellowship from the French Ministry of Education
- 2007 One-year "INGENIO" fellowship for Young Researchers awarded by the Regione Lombardia, Italy.
- 2019 Winner of the Outstanding New Investigator Award of the American Society of Gene and Cell Therapy (ASGCT)

ABILITAZIONE SCIENTIFICA NAZIONALE

In 2019-2022, AKR obtained the “Abilitazione Scientifica Nazionale di Seconda Fascia” in the following disciplinary sections: SETTORE CONCORSUALE 05/E2, BIOLOGIA MOLECOLARE; SETTORE CONCORSUALE 06/A2, PATOLOGIA GENERALE E PATOLOGIA CLINICA; SETTORE CONCORSUALE 06/N1, SCIENZE DELLE PROFESSIONI SANITARIE E DELLE TECNOLOGIE MEDICHE APPLICATE; SETTORE CONCORSUALE 05/I2 – MICROBIOLOGIA, and the “Abilitazione Scientifica Nazionale di Prima Fascia” in the following disciplinary sections: SETTORE CONCORSUALE 05/E2, BIOLOGIA MOLECOLARE; SETTORE CONCORSUALE 05/I2 – MICROBIOLOGIA and SETTORE CONCORSUALE 06/A2, PATOLOGIA GENERALE E

PATOLOGIA CLINICA.

ACADEMIC SUPERVISION

- 2006 – 2012 During her post-doctoral period, AKR helped supervise 4 M.Sc. research fellows, one M.Sc. student and a Ph.D student
- 2012 – present Since AKR joined SR-Tiget, she has supervised 7 M.Sc. students, 5 PhD students and 4 post-doctoral fellows.

TEACHING ACTIVITIES

- 2023-present: Principal Lecturer of the BSc Course of Molecular biology, of the MSc course of Advance Molecular Biology and of the MSc course of Molecular Biology of the Cell at the University of Pavia, Pavia, Italy
- 2022-2023: Principal Lecturer of the Course of Emerging Biotechnologies: innate Immunity and Nucleic Acid Sensing in Human Health and disease, Vita-Salute San Raffaele University, Milan, Italy
- 2019-present: Lecturer at the Course of Regenerative Medicine and Bioethics, Vita-Salute San Raffaele University, Milan, Italy
- 2020-2021: Invited Lecturer at the Doctorate Course in Molecular and Cellular Biology Department of Biosciences – University of Milan, Italy
- 2019-2020: Invited Lecturer at the Course of Molecular Virology for the “Laurea Magistrale in Biotechnologie del Farmaco” at the Department of Biosciences – University of Milan, Italy
- 2018-2019: Principal Lecturer of the Course of Molecular Medicine and Bioethics, Vita-Salute San Raffaele University, Milan, Italy
- 2016-2017: Lecturer at the Course of Genome Editing: technology, applications and ethical implications, PhD Program in Molecular Medicine, Vita-Salute San Raffaele University, Milan, Italy
- 2014-present: Lecturer at the Course of Gene Therapy, Vita-Salute San Raffaele University, Milan, Italy
- 2010-2011: Lecturer at the Course of HIV Immunopathogenesis, Vita-Salute San Raffaele University, Milan, Italy
- 2009-2010: Lecturer at the Course of Infectious Diseases, Vita-Salute San Raffaele University, Milan, Italy
- 2009-2013: Lecturer at the Course of Biotechnological Applications and Bioreactors, Politecnico di Milano, Milan, Italy

PROFESSIONAL ACTIVITIES AND COMMISSIONS OF TRUST

- Since 2020, member of the Editorial Board of *Gene Therapy*
- Since 2016, member of the Board of the PhD Program in Cellular and Molecular Physiopathology, Vita-Salute San Raffaele University, Milan, Italy
- Since 2012, External examiner during Master Thesis defences, Vita-Salute San Raffaele University, Milan, Italy
- Since 2012, Member of the American and European Societies for Gene and Cell Therapy
- Since 2019, Member of the Italian Society of Biophysics and Molecular Biology (SIBBM)
- Ad hoc reviewer for PLoS One, Journal of Virology, Virology, Current HIV Research, Cell Reports, Cell Stem Cell, Journal of Experimental Medicine
- Grant reviewer for the French National Research Agency (ANR) and the European Research Council (ERC)

- 2022, invited member of the SAB of the Genethon Institute, Paris, France
- Since 2022, Nominated member of the ASGCT Committee on Immune Responses to Gene & Cell Therapy
- Since 2022, consultant for AIFA, the Italian Medicines Agency, during examination of medicinal product approval dossiers.
- Since 2022, Chair of the ESGCT Immune responses to Gene Therapy Committee
- Co-organizer of the EMBO workshop on “Pathogen Immunity and Signaling”, 08-12 April 2024, San Servolo, Venice, Italy

INVITED LECTURES

CIEMAT/CIBERER/Fundación Jiménez Díaz, Madrid, Spain; Institute of Molecular Virology, Ulm University Medical Center, Ulm, Germany (2015); 9th Stem Cell Clonality and Genome Stability Retreat, Florence, Italy (2016); 8th Annual International Fanconi Anemia Gene Therapy Working Group, Heidelberg, Germany (2017); 10th Blizard HIV symposium, Queen Mary University of London, UK (2018); Fanconi Anemia Scientific Symposium, Los Angeles, CA, USA (2018); Groupe Reflexion SIDA, Institut Pasteur, Paris, France (2019); Fanconi Anemia Scientific Symposium, Chicago, USA (2019); Retreat of the Division of Genetics and Cell Biology, OSR, Brescia, Italy (2019); 22nd ASGCT Annual Meeting, Washington DC, USA (2019); 27th ESGCT Annual Congress, Barcelona, Spain (2019); Cell Symposium on Gene- and Cell-Based Therapies: CRISPR, Stem Cells, and Beyond, Mission Bay Conference Center at UCSF, CA, USA (2020); 23rd ASGCT Annual Meeting, Boston, USA (2020); 24th ASGCT Annual Meeting, Washington DC, USA (2021, virtual); 25th ASGCT Annual Meeting, Washington DC, USA (2022); the XIII SIICA national meeting, Naples, Italy (2022); 2022 FASEB Science Research Conference on Genome Engineering, Lisbon, Portugal (2022); 5th International Conference on CRISPR Technologies, Berkeley, California, USA (2022); 4th Annual Gene Therapy for Neurological Disorders, Boston, MA, USA (2022); InnaSCo-FrontInnov symposium, Lyon, FR (2023); National ABDC meeting, Paestum, IT (2023); ESGCT Stem Cell Clonality and Genome Stability Retreat, Brussels, BE (2023).

FUNDING

- 2023-2025: PI of a **Foundation for the National Institutes of Health (FNIH)** grant. Title: “Investigating Innate Sensing and Antiviral Restriction of AAV vectors in the Human Central Nervous System”. 575,000 USD.
- 2022-2024: PI of a **Core Grant from the Italian Telethon Foundation (TELE22-AK)**. Title: “Dissecting Innate Immunity and Nucleic Acid Sensing in Gene Therapy and Disease”. 180,000 €/year.
- 2021-2023: PI of a **Sponsored Research Agreement with Spark Therapeutics** to study gene therapy vector sensing and restriction in relevant human target tissues: 300,000 €/year.
- 2021-2024: co-PI of “**Giovani Ricercatori**” Grant funded by the Italian Ministry of Health (GR-2019-12369357). Title: “Enhancement of expression, bioavailability and cross-correction of chimeric GALC enzyme to refine gene therapy approaches for Globoid cell leukodystrophy”. 150,000 €/3 years.
- 2020-2021: PI of a “Telethon Rare Diseases and COVID-19 Special Grant” by the **Italian Telethon Foundation Grant (GSP20006_Covid037)**. Title: “Investigating and harnessing cell intrinsic innate immunity against SARS-CoV-2 – Insight from the Aicardi-Goutières Syndrome”. 50,000 €/year.
- 2020-2023: PI of a “**Giovani Ricercatori**” Grant funded by the Italian Ministry of Health (GR-2018-12366006). Title: “Dissecting innate immunity in hematopoietic stem cells for improved gene therapy”. 150,000 €/year
- 2019-2024: PI of an **ERC Consolidator Grant (819815-ImmunoStem)**. Title: “Dissecting and Overcoming Innate Immune Barriers for Therapeutically Efficient Hematopoietic Stem Cell Gene Engineering”.

398,875 €/year.

- 2016-2019: PI of a **Core Grant from the Italian Telethon Foundation** (TELE16C03). Title: “Mechanisms of enhanced HSC transduction and nucleic acid sensing”. 164,420 €/year.
- 2016-2019: PI of a **Core Grant from the Italian Telethon Foundation** (TELE16D03). Title: “The Aicardi-Goutières Syndrome – From nucleic acid sensing to disease modelling”. 123,767 €/year.
- 2016-2018: PI of a **Fanconi Anemia Research Fund** Grant. Title: “Investigating the Impact of Lentiviral Transduction on Fanconi Anemia Hematopoietic Stem Cells for Improved Gene Therapy”. 87,500 USD/year.
- 2014-2016: PI of an **Italian Telethon Foundation Grant** (TGT11D04). Title: “Dissecting lentivirus-host interactions to improve gene transfer”. 150,000 €/year.
- 2011-2014: PI of a **“Giovani Ricercatori”** Grant funded by the Italian Ministry of Health (GR-2009-1471693). Title: “TRIM22-mediated inhibition of HIV-1 and other human retroviral pathogens: investigation of the molecular interactions for novel therapeutic approaches”. 145,800 €/year.

PATENTS

I am the main inventor in four International Patents (US10391201B2/ WO2015162594A3; WO2017198868, WO2018193118 and PCT/EP2022/061114) for improved HSC transduction and preservation during ex vivo gene transfer and editing.

PERSONAL STATEMENT

My training and education are in biochemistry, molecular biology, virology and innate immunity. I started my scientific career as an undergraduate and then PhD fellow at the Pasteur Institute, Paris, France. During this time, I uncovered the molecular basis of IFN-induced OAS proteins in mediating resistance to West Nile virus infection (Kajaste-Rudnitski et al., JBC 2006) and characterized the molecular mechanism of action of DC-SIGN surface lectin polymorphisms that associate with severe Dengue haemorrhagic fever (Sakuntabhai et al., Nature Genetics, 2005). I continued my post-doctoral training in molecular virology at the San Raffaele Scientific Institute, OSR, Milan, Italy, from 2006 to 2012. During this time, I set-up several new projects and international collaborative networks within the lab, taking advantage of my previous experience and contacts at the Pasteur Institute. These efforts led me to identify immune correlates of protection to influenza vaccines (Kajaste-Rudnitski et al., AIDS 2013) and to discover novel innate immune mechanisms of restriction against HIV-1 and Influenza A (Kajaste-Rudnitski et al., JVI 2011; Di Pietro, Kajaste-Rudnitski et al., JVI 2013).

In April 2012, I joined the San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), OSR, in Milan as Project Leader, promoted Group Leader in July 2016. **My research is focused on the molecular mechanisms of host-vector interplay and innate immunity in the context gene therapy applications and autoinflammatory diseases.** In this setting, my work has been at the crossroad between basic molecular virology and translational research, and I have successfully merged my background in virology and innate immune responses with acquired expertise the fields of hematopoietic stem cell gene therapy and autoinflammatory disease modelling to better understand the complex interactions occurring between gene therapy vectors, the target cells and how these pathways intersect with immunopathologies.

I was promoted Full Professor of Molecular Biology at the University of Pavia, starting from October 2023. In this context I will pursue my studies on nucleic acid immunity in the context of gene therapies but expanding also to auto-inflammatory and infectious diseases. **My long-term goal is to provide insight into how pathogen recognition and consequent innate immune signalling may affect efficacy and safety of gene therapy approaches in clinically relevant target cells as well as to shed light on how these same pathways may contribute to autoimmune and inflammatory pathologies.**

I have contributed to the field of gene therapy through the development of improved HSC transduction protocols (Petrillo et al., *Molecular Therapy* 2015; Petrillo et al., *Cell Stem Cell* 2018; Petrillo et al., *Human Gene Ther.*, 2019) and molecular understanding of the vector/virus-host interactions in HSC (Piras et al., *EMBO Molecular Medicine* 2017; Ferrari et al., *Nature Biotechnol.* 2020, Ferrari et al., *Cell Stem Cell* 2022, Unali et al., *EMBO J* 2023). These studies provide critical information for the development of safer and more efficient gene therapy approaches and have led to the filing of several international patent applications, with expressed interest on behalf of several industrial partners. The gene transfer protocols and molecular virology research tools that I have set-up have also enabled efficient manipulation of difficult to transduce primary human myeloid cells and the molecular characterization of mechanisms of improved HSC transduction in the context of several collaborative efforts (Chiriaco et al., *Molecular Therapy* 2014; Escobar et al., *Science Translational Medicine* 2014; Zonari et al., *Stem Cell Reports* 2017; Comi et al., *Front Immunol.* 2020, Kerzel et al., *Cancer Cell* 2023).

Moreover, besides potentially impacting HSPC gene therapy, deregulation of innate immune responses, and of nucleic acid sensing is emerging as a potential driver of several autoinflammatory and autoimmune diseases including the Aicardi-Goutières Syndrome (AGS), characterized by aberrant type I interferon responses in the central nervous system. In this setting, I am interested in understanding how deregulated or compromised endogenous nucleic acid sensing pathways could contribute to AGS. In this context, we have recently uncovered DNA damage as a driver of neurotoxic inflammation in human iPSC derived AGS astrocytes (Giordano et al., *J Exp Med* 2022), paving the way towards the development of more targeted interventions to prevent neuroinflammation and immunopathology in the context of AGS.

I have been actively involved in the educational activities and academic teaching within OSR and the San Raffaele University and now as Full Professor at the University of Pavia. My first important teaching experience was in 2018/2019 as the Principal Lecturer of the “Corso di Laurea in Medicina molecolare e bioetica” at the Università Vita-Salute San Raffaele that got very good evaluations on behalf of the students. I was an on-contract professor at the “Corso di Laurea Medicina rigenerativa e molecolare, e bioetica della ricerca (Corso di Laurea in Ricerca biotecnologica in medicina)” and the Principal Lecturer of the course “Emerging Biotechnological Technologies: innate Immunity and Nucleic Acid Sensing in Human Health and Disease (Corso di Laurea in Biotechnology and Medical Biology)” at the Università Vita-Salute San Raffaele and a member of the Board of the PhD Program in Cellular and Molecular Physiopathology and serve as examiner to Master Thesis defences as well as the PhD selection committees. At the University of Pavia, I am responsible for Molecular Biology courses for BSc and MSc students both in Italian and in English.

My research is currently funded by the Italian Telethon Foundation, the Italian Ministry of Health, the European Research Council (ERC Consolidator grant) and the FNIH. The efforts of my team have allowed me to be invited to speak at more than 10 international congresses in the past five years and the increasing impact of our work was recognized by the American Society of Cell and Gene therapy (ASGCT) that awarded me with **The ASGCT Outstanding Achievement Award in 2019**. This award is the Society's highest honour recognizing an ASGCT Member who has achieved a pioneering research success in the field of gene and cell therapy. Overall, my studies have significantly contributed to the emerging field of innate immune hurdles to gene therapy (Piras and Kajaste-Rudnitski, *Gene Therapy* 2020; Costa-Verdera et al., *Human Gene therapy* 2023) and provide novel insight for the development of innovative cell and gene therapies and to fight infectious and autoimmune diseases in the future.

PUBLICATIONS (*h-index 22, Scopus*)

1. Valeri E, Unali G, Piras F, Abou-Alezz M, Pais G, Benedicenti F, Lidonnici MR, Cuccovillo I, Castiglioni I, Arévalo S, Spinozzi G, Merelli I, Behrendt R, Oo A, Kim B, Landau NR, Ferrari G, Montini E, Kajaste-Rudnitski A. Removal of innate immune barriers allows efficient transduction of quiescent human hematopoietic stem cells. *Mol Ther.* 2024 Jan 3;32(1):124-139.

2. Kerzel T, Giacca G, Beretta S, Bresesti C, Notaro M, Scotti MG, Balestrieri C, Canu T, Redegalli M, Pedica F, Genua M, Ostuni R, Kajaste-Rudnitski A, Oshima M, Tonon G, Merelli I, Aldrighetti L, Dellabona P, Coltella N, Doglioni C, Rancoita PMV, Sanvito F, Naldini L, Squadrito LM. In vivo macrophage engineering reshapes the tumor microenvironment leading to eradication of liver metastases. *Cancer Cell*, in press.
3. Costa-Verdera H, Unzu C, Valeri E, Adriouch S, Gonzalez-Aseguinolaza G, Mingozzi F, Kajaste-Rudnitski A. Understanding and tackling immune responses to AAV vectors. *Hum Gene Ther*. 2023 Sep 6. doi: 10.1089/hum.2023.119.
4. Colantuoni M, Jofra Hernandez R, Pettinato E, Basso-Ricci L, Magnani L, Andolfi G, Rigamonti C, Finardi A, Romeo V, Soldi M, Sergi L, Rocchi M, Scala S, Hoffman HM, Gregori S, Kajaste-Rudnitski A, Sanvito F, Muzio L, Naldini L, Aiuti A, Mortellaro A. Constitutive IL-1RA production by modified immune cells protects against IL-1-mediated inflammatory disorders. *Sci Transl Med*. 2023 May 31;15(698):eade3856.
5. Ferrari S, Valeri E, Conti A, Scala S, Aprile A, Di Micco R, Kajaste-Rudnitski A, Montini E, Ferrari G, Aiuti A, Naldini L. Genetic engineering meets hematopoietic stem cell biology for next-generation gene therapy. *Cell Stem Cell*. 2023 May 4;30(5):549-570.
6. Unali G, Crivicich G, Pagani I, Abou-Alezz M, Folchini F, Valeri E, Matafora V, Reisz JA, Giordano AMS, Cuccovillo I, Butta GM, Donnici L, D'Alessandro A, De Francesco R, Manganaro L, Cittaro D, Merelli I, Petrillo C, Bachi A, Vicenzi E, Kajaste-Rudnitski A. Interferon-inducible phospholipids govern IFITM3-dependent endosomal antiviral immunity. *EMBO J*. 2023 Mar 27:e112234.
7. Giordano AMS, Abou Alezz M, Merelli I, Kajaste-Rudnitski A. Protocol to differentiate monolayer human induced pluripotent stem cells into inflammatory responsive astrocytes. *STAR Protoc*. 2023 Mar 6;4(1):102142.
8. Kajaste-Rudnitski A and Aiuti A. Towards improved, yet regulated gene therapy for X-CGD. *Blood*. 2023 Mar 2;141(9):966-967.
9. Ferrari S, Jacob A, Cesana D, Laugel M, Beretta S, Varesi A, Unali G, Conti A, Canarutto D, Albano L, Calabria A, Vavassori V, Cipriani C, Castiello MC, Esposito S, Brombin C, Cugnata F, Adjla O, Ayuso E, Merelli I, Villa A, Di Micco R, Kajaste-Rudnitski A, Montini E, Penaud-Budloo M, Naldini L. Choice of template delivery mitigates the genotoxic risk and adverse impact of editing in human hematopoietic stem cells. *Cell Stem Cell* 2022 Oct 6;29(10):1428-1444.e9.
10. Giordano AMS, Luciani M, Gatto F, Abou Alezz M, Beghè C, della Volpe L, Migliara A, Valsoni S, Genua M, Dzieciatkowska M, Frati G, Tahraoui-Bories J, Giliani S, Orcesi S, Fazzi E, Ostuni R, D'Alessandro A, Di Micco R, Merelli I, Lombardo A, Reijns MAM, Gromak N, Gritti A, Kajaste-Rudnitski A*. DNA damage contributes to neurotoxic inflammation in Aicardi-Goutières Syndrome astrocytes. *J Exp Med*. 2022 Apr 4;219(4):e20211121. * Senior and corresponding author.
11. Molteni R, Biavasco R, Stefanoni D, Nemkov T, Domínguez-Andrés J, Arts RJ, Merelli I, Mazza D, Zambrano S, Panigada M, Cantoni E, Tengesdal I, Maksud P, Piras F, Cesana D, Cassina L, Distefano G, Loffreda A, Gnani D, De Luca G, Tomelleri A, Campochiaro C, Joosten LAB, Dinarello CA, Kajaste-Rudnitski A, Haroche J, Cardaci S, Cenci S, Dagna L, Doglioni C, Ferrarini M, Ferrero E, Boletta A, D'Alessandro A, Montini E, Netea M, Giulio Cavalli. Oncogene-induced maladaptive activation of trained immunity in the pathogenesis and treatment of Erdheim-Chester disease. *Blood*. 2021 Oct 28;138(17):1554-1569.
12. Unali G, Giordano AMS, Cuccovillo I, Abou Alezz M, Apolonia L, Merelli I, Malim MH, C. Petrillo C, Kajaste-Rudnitski A*. The lysine-rich intracellular loop and cell type-specific co-factors are required for IFITM3 antiviral immunity in hematopoietic stem cells. *bioRxiv* 2021.04.06.438585. * Senior and corresponding author.
13. Torretta S, Scagliola A, Ricci L, Mainini F, Di Marco S, Cuccovillo I, Kajaste-Rudnitski A, Sumpton D, Ryan KM, Cardaci S. D-mannose suppresses macrophage IL-1 β production. *Nat Commun*. 2020 Dec 11;11(1):6343.

14. Soldi M, Sergi L, Unali G, Kerzel T, Cuccovillo I, Capasso P, Annoni A, Biffi M, Rancoita PMV, Cantore A, Lombardo A, Naldini L, Squadrito ML, Kajaste-Rudnitski A. Laboratory-Scale Lentiviral Vector Production and Purification for Enhanced Ex Vivo and In Vivo Genetic Engineering. *Mol Ther Methods Clin Dev.* 2020 Oct 20;19:411-425. * Senior and corresponding author.
15. Piras F and Kajaste-Rudnitski A*. Antiviral immunity and nucleic acid sensing in haematopoietic stem cell gene engineering. *Gene Ther.* 2020 Jul 13:1-13. * Senior and corresponding author.
16. Comi M, Amodio G, Passeri L, Fortunato M, Santoni De Sio F, Andolfi G, Kajaste-Rudnitski A, Russo F, Cesana L, Gregori S. Generation of powerful human tolerogenic dendritic cells by lentiviral-mediated IL-10 gene transfer. *Front Immunol.* 2020 Jun 30;11:1260.
17. Ferrari S, Jacob A, Beretta S, Unali G, Albano L, Vavassori V, Cittaro D, Lazarevic D, Brombin C, Cugnata F, Kajaste-Rudnitski A, Merelli I, Genovese P, Naldini L. Efficient gene editing of human long-term hematopoietic stem cells validated by clonal tracking. *Nat Biotechnol.* 2020 Jun 29.
18. Petrillo C, Calabria A, Piras F, Capotondo A, Spinazzi G, Cuccovillo I, Benedicenti F, Naldini L, Montini E, Biffi A, Gentner B, Kajaste-Rudnitski A*. Assessing the impact of Cyclosporine A on lentiviral transduction and preservation of human hematopoietic stem cells in clinically relevant ex-vivo gene therapy settings. *Hum Gene Ther.* 2019 Apr 30. * Senior and corresponding author.
19. Petrillo C, Thorne LG, Unali G, Schiroli G, Giordano AMS, Piras F, Cuccovillo I, Petit SJ, Ahsan F, Noursadeghi M, Clare BS, Genovese P, Gentner B, Cittaro D, Naldini L, Towers GJ, Kajaste-Rudnitski A*. Cyclosporine H Overcomes Innate Immune Restrictions to Improve Lentiviral Transduction and Gene Editing In Human Hematopoietic Stem Cells. *Cell Stem Cell.* 2018 Dec 6;23(6):820-832.e9. * Senior and corresponding author. **Recommended in F1000Prime as being of special significance in its field.**
20. Piras F, Riba M, Petrillo C, Lazarevic D, Cuccovillo I, Bartolaccini S, Stupka E, Gentner B, Cittaro D, Naldini L, Kajaste-Rudnitski A*. Lentiviral Vectors Escape Innate Sensing but Trigger p53 In Human Hematopoietic Stem and Progenitor Cells. *EMBO Mol Med.* Sep;9(9):1198-1211. 2017. * Senior and corresponding author
21. Zonari E, Desantis G, Petrillo C, Boccalatte FE, Lidonnici MR, Kajaste-Rudnitski A, Aiuti A, Ferrari G, Naldini L, Gentner B. Efficient Ex Vivo Engineering and Expansion of Highly Purified Human Hematopoietic Stem and Progenitor Cell Populations for Gene Therapy. *Stem Cell Reports.* Apr 11;8(4):977-990. 2017.
22. Turrini F, Marelli S, Kajaste-Rudnitski A, Lusic M, Van Lint C, Das AT, Harwig A, Berkhout B, Vicenzi E. HIV-1 transcriptional silencing caused by TRIM22 inhibition of Sp1 binding to the viral promoter. *Retrovirology.* Dec 18;12(1):104. 2015.
23. Kajaste-Rudnitski A*. and Naldini L., Cellular innate immunity and restriction of viral infection - implications for lentiviral gene therapy in human hematopoietic cells. *Hum Gene Ther.* Apr;26(4):201-9. 2015. * Senior and corresponding author
24. Petrillo C, Cesana D, Piras F, Bartolaccini S, Naldini L, Montini E, Kajaste-Rudnitski A*. Cyclosporin A and Rapamycin relieve distinct lentiviral restriction blocks in hematopoietic stem and progenitor cells. *Mol Ther.*, Feb. 23. 2015. * Senior and corresponding author
25. Chiriaco M, Farinelli G, Capo V, Zonari E, Scaramuzza S, Di Matteo G, Sergi LS, Migliavacca M, Hernandez RJ, Bombelli F, Giorda E, Kajaste-Rudnitski A, Trono D, Grez M, Rossi P, Finocchi A, Naldini L, Gentner B, Aiuti A. Dual-Regulated Lentiviral Vector for Gene Therapy of X-linked Chronic Granulomatosis. *Mol Ther.*, May 29. 2014.
26. Escobar G., Moi D., Ranghetti A., Ozkal-Baydin P., Squadrito ML., Kajaste-Rudnitski A., Bondanza A., B. Gentner B., De Palma M., Mazzieri R. and Naldini L. Genetic Engineering of Hematopoiesis for Targeted IFN- α Delivery Inhibits Breast Cancer Progression. *Sci Transl Med.*, Jan 1;6(217):217. 2014.
27. Ghezzi S, Galli L, Kajaste-Rudnitski A, Turrini F, Marelli S, Toniolo D, Casoli C, Riva A, Poli G, Castagna A and Vicenzi E. Identification of TRIM22 Single Nucleotide Polymorphisms Associated with Loss of Inhibition of HIV-1 Transcription and Advanced HIV-1 Disease. *AIDS.*, Sep 24;27(15):2335-44. 2013.

28. Pezzoli D., Kajaste-Rudnitski A., Chiesa R., Candiani G., Lipid-based nanoparticles as non-viral gene delivery vectors. *Methods Mol Biol.*, 1025:269-79. 2013.
29. Cassetta L, Kajaste-Rudnitski A, Coradin T, Della Chiara G, Barbagallo M, Saba S, Alfano M, Cassol E, Vicenzi E and Guido Poli. M1 Polarization of Human Monocyte-Derived Macrophages Restricts Pre- and Post-Integration Steps of HIV-1 Replication. *AIDS*. Jul 31;27(12):1847-56. 2013.
30. Di Pietro A.*, Kajaste-Rudnitski A.*, Oteiza A., Nicora L., Towers GJ., Mechti N., Vicenzi E. TRIM22 Inhibits Influenza A Virus Infection by Targeting the Viral Nucleoprotein for Degradation. *J Virol.*, Apr;87(8):4523-33. 2013. *shared first authorship
31. Mascagni P, Vicenzi E, Kajaste-Rudnitski A, Pellicciotta G, Monti A, Cervi C, Vitalucci R, Toffoletto F. Assessment of efficacy and safety of pandemic A/H1N1/2009 influenza vaccine in a group of health care workers. *Med. Lav.*, May 103(3): 220-29. 2012.
32. Mariani SA, Brigida I, Kajaste-Rudnitski A, Ghezzi S, Rocchi A, Plebani A, Vicenzi E, Aiuti A & Guido Poli G. HIV-1 Envelope Dependent Restriction of CXCR4-Using Viruses in Children But Not Adult Untransformed CD4+ T Lymphocyte Lines. *Blood.*, Mar 1;119(9):2013-23. 2012.
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