PERSONAL INFORMATION

Name: Anna Kajaste-Rudnitski, PhD
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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

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)

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 became independent, she has supervised 8 M.Sc. students, 6 PhD students and 5 post-doctoral fellows.

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 in the past three years

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); EMBO Workshop on Pathogen Immunity and Signalling, San Servolo, IT (2024); 31st ESGCT Annual Congress, Rome, IT (2024).

ACTIVE 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.
- 2019-2025 (extended): 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.

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.

SELECTED PUBLICATIONS *(h-index 22, Scopus)* For complete list please visit:

https://pubmed.ncbi.nlm.nih.gov/?term=kajaste-rudnitski+a&sort=pubdate

1. Valeri E, Breggion S, Barzaghi F, Abou Alezz M, Crivicich G, Pagani I, Forneris F, Sartirana C, Costantini M, Costi S, Marino A, Chiarotto E, Colavito D, Cimaz R, Merelli I, Vicenzi E, Aiuti A, Kajaste-Rudnitski A. A novel STING variant triggers endothelial toxicity and SAVI disease. *J Exp Med*. In press.
2. 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.
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. 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.
5. 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.
6. 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.
7. Soldi M, Sergi 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.
8. 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.
9. 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.
10. Petrillo C, Calabria A, Piras F, Capotondo A, Spinozzi 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.
11. 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. *Recommended in F1000Prime as being of special significance in its field.*
12. 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.

13. 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.

Pavia, 03/06/2024