

POSITION TITLES: Distinguished Professor and Chairman, Department of Ophthalmology, University of Pittsburgh School of Medicine, The Eye and Ear Foundation Endowed Chair, Director of the UPMC and UPSOM Vision Institute; Emeritus Professor, Sorbonne University, Paris, France;

EDUCATION/TRAINING

| INSTITUTION AND LOCATION | DEGREE | COMPLETION DATE | FIELD OF STUDY |
|---|--|------------------------|--|
| Paris University Medical School, Paris, France | Medical Degree | 12/1980 | Medicine |
| Rothschild Ophthalmology Foundation, Paris, France | Residency | 10/1979 -09/1980 | Ophthalmology |
| Louis Pasteur University Hospital, Strasbourg, France | Residency | 10/1980-03/1984 | Neurology, Neurosurgery, Ophthalmology |
| French National Certifying Board | Ophthalmology | 04/1984 | Ophthalmology |
| Louis Pasteur University Hospital, Strasbourg, France | Clinical Fellow Chef de Clinique des Universités | 04/1984-03/1988 | Ophthalmology |
| Massachusetts Eye and Ear Infirmary, Harvard Medical School, Boston, Massachusetts | Research Fellow | 10/1986-09/1987 | Ophthalmology (Prof. D. M. Albert) |
| Harvard Biological Laboratories, Department of Cellular and Developmental Biology, Harvard University, Cambridge, Massachusetts | Visiting Scholar | 02/1987-09/1987 | Biology (Prof. J. E. Dowling) |

A. Personal Statement

The primary focus of my laboratory and clinical investigations is to understand the mechanisms associated with retinal degeneration in order to develop vision restoration therapies. Together with my collaborators I have been working on i) the conception, development, and evaluation of innovative treatments for retinal diseases, with a special focus on genetic rod-cone dystrophies and age-related retinal degenerations; ii) translation to the clinic of sight saving approaches, including neuroprotection, stem cells, gene therapy and optogenetics, pharmacology, and visual prostheses.

I led several first-in-man vision restoration clinical trials (Gene Therapy, Retinal Prosthesis, Optogenetics). I founded and directed (from 2008 to 2020) the Institut de la Vision in Paris, a site for translational research

on treatments for currently untreatable inherited and age-related ocular diseases that comprise 18 principal investigators and more than 300 members, and functions in synergy with the Quinze-Vingts National Eye Hospital and the Clinical Investigation Center, overseeing more than 80 clinical trials, some of them within the most advanced areas of biomedical technologies worldwide, such as retinal implants and gene therapy, including several first-in-human trials. In 2016, I was appointed Professor and Chairman of the Department of Ophthalmology at the University of Pittsburgh Medical School, and The Eye and Ear Foundation Endowed Chair. I am actively expanding the Department of Ophthalmology at the University of Pittsburgh (now more than 40 PIs) launching breakthrough trials and developing new models of care around a new translational Vision Institute (400,000 sqf, opened in May 2023). I published more than 700 peer-reviewed articles and co-authored more than 90 patents. I co-founded a dozen start-up companies, including Fovea pharmaceuticals, GenSight Biologics, Pixium Vision, Sparing Vision, SharpEye, VegaVect, Avista, Tilak, and Tenpoint.

B. Positions and Honors

University positions

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| 1987-1992 | Visiting Lecturer in Ophthalmology - Department of Ophthalmology - Harvard Medical School, Boston, Massachusetts, USA |
| 1988-2002 | University Professor of Ophthalmology – Strasbourg University (1st class since 1999) |
| 1993-1995 | Visiting Professor - Department of Ophthalmology and Visual Sciences, University of Madison/Wisconsin |
| 2001-2017 | Cumberlege Chair: Professor of Biomedical Sciences - Institute of Ophthalmology-University College London |
| 2002-2022 | University Professor of Ophthalmology – Paris (VI) Pierre and Marie Curie University Medical School, now Sorbonne Université (Exceptional Class since 2010) |
| 2012-2015 | Visiting Professor: Hebrew University of Jerusalem, Israel |
| 2015-2016 | Invited Professor at Collège de France : Chaire « Innovation Technologique» |
| Since 2016 | Professor and Chairman of the Department of Ophthalmology at the University of Pittsburgh School of Medicine, The Eye and Ear Foundation Endowed Chair (Distinguished Professorship since 2019) |
| Since 2017 | Honorary Professor, University College London |
| Since 2017 | Adjunct Professor of Robotics and Bioengineering, Carnegie Mellon University, Pittsburgh |
| Since 2019 | Adjunct Professor of Ophthalmology, Hebrew University of Jerusalem, Israel |
| Since 2023 | Emeritus Professor of Ophthalmology, Sorbonne Université |

Hospital positions

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| 1988-2001 | Hospital full time clinician, Ophthalmology-Ophthalmological Clinic–Strasbourg University Hospital |
| Since 2001 | Hospital clinician, Ophthalmology–Quinze-Vingts National Ophthalmology Hospital, Paris |
| 2001-2020 | Chairman Department of Ophthalmology–Quinze-Vingts National Ophthalmology Hospital, Paris Chairman Department of Ophthalmology and Vitreo-Retinal Diseases–Foundation A. de Rothschild |
| Since 2016 | Chairman Department of Ophthalmology/University of Pittsburgh Medical Center |

Hospital and university activities

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| 1988-2002 | Director of the Eye Pathology Laboratory -Louis Pasteur University-Strasbourg |
| 1994-2001 | Head of the Functional Unit "Retina and Oncology" at Ophthalmology Clinic – Strasbourg University Hospital |

2005-2020 Director of the Clinical Investigation Centre –503 INSERM/ Quinze-Vingts National
 2005-2019 Director National Reference Centre REFERET for Inherited Retinal Dystrophies
 2019-2023 Director Institut Hospitalo-Universitaire FOReSIGHT
 2023- Director of the Vision Institute of Pittsburgh (University of Pittsburgh School of
 Medicine and Medical center)

Research activities

1999-2002 Director of INSERM EMI 99-18 – UPRES EA 24-10: Cellular and Molecular
 Physiopathology of the retina -Louis Pasteur University, Strasbourg
 2002-2008 Director of UMR INSERM-UPMC 592: Cellular and Molecular Physiopathology of
 the Retina, Paris
 2003 - Co-founder of European Vision Institute, EEIG
 2005-2007 Coordinator of the Vision Research National Program (Inserm).
 2005-2008 Scientific director of the Vision Institute project – CHNO des Quinze-Vingts
 2006- Director of the “Seeing and Hearing” Institut Carnot (Vision Institute with Pasteur
 Institute)
 2006-2018 Director of the “Seeing and Hearing” Scientific Cooperation Foundation
 2007-2017 Coordinator of the Foundation Fighting Blindness Paris Research Centre
 2009-2020 Director of the Research Centre: Vision Institute, UMR S 968 Inserm, UM80 Sorbonne
 Univ./ UMR 7210 CNRS
 2011-2019 Director of the Laboratory of Excellence "LIFESENSES: senses for a lifetime"
 (22M€ Grant)
 2019-2024 Director Institut Hospitalo-Universitaire FOReSIGHT (50M€ Grant)
 2016- Chairman of the University of Pittsburgh School of Medicine Ophthalmology
 Research Center (Vision Institute of Pittsburgh)

C. Other Experience and Professional Memberships

Director, The Foundation Fighting Blindness Retinal Degeneration Fund
 Advisor, UPMC Enterprises
 Advisor, LightStone Ventures
 Member of Conseil Présidentiel de la Science, advising Président Emmanuel Macron (2023-)
 Member Conseil de l'Ordre National du Mérite
 Member of Commission Innovation 2030
 Member of Conseil Stratégique des Industries de Santé (2021)
 Président du Conseil Scientifique : Institut de Réhabilitation Visuelle Saint-Louis, Paris
 Conseil Scientifique, Institut de la Myopie, Paris
 Board of Trustees, Institut du Cerveau de l'Enfant, Paris
 Vice-President (Strategy): Biocluster Brain and Mind, Paris
 Co-Chairman: Ocular Disease Forum 2024- Berkeley
 Co-Chairman: Vision Restoration and Rehabilitation and Outcome Measures, Collaborative Community
 of Ophthalmic Innovation (with FDA and NEI)

Journal editorial boards: Journal of Clinical Investigation; Science Translational Medicine; Progress in
 Retinal and Eye Research; Translational Vision Science and Technology (Associate Editor 2012-);
 Ophthalmology and Therapy

Journal reviewing: The Lancet; Science; Nature Medicine; Nature Biotechnology; Nature
 Communications; Science Translational Medicine; Science; Journal of Clinical Investigation; Proceedings
 of the National Academy of Sciences of the United States of America; Progress in Retinal and Eye
 Research; Investigative Ophthalmology & Visual Science; Restorative Neurology and Neuroscience;
 Graefe's Archive for Clinical and Experimental Ophthalmology; JAMA Ophthalmology; PLOS Genetics;
 PLOS ONE; Scientific Reports; Orphanet Journal of Rare Diseases; Experimental Eye Research;

Neuroscience & Behavioral Reviews; Retina, The Journal of Retinal and Vitreous Diseases; Translational Vision Science & Technology; Expert Review of Molecular Diagnostics; Ophthalmic Research; Laboratory Investigation

Extramural grant reviewing: Foundation Fighting Blindness, US; Neurosciences and Mental Health Board (NMHB), UK; Israel Ministry of Science, Technology and Space, Israel; Deutsche Forschungsgemeinschaft (DFG), Germany; Agence Nationale de la Recherche (ANR), France; Banque publique d'investissement (Bpifrance), France; Fédération des Aveugles de France; Retina France, France; European Research Council (ERC); Israel Science Foundation (ISF), Israel; Medical Research Council, UK; Fondation Recherche Médicale (FRM), France; Fondation Simone et Cino del Duca-Institut de France, France; National Institute for Health Research, UK; European Research Area Networks-Neuron; Fight for Sight, UK; Retinitis Pigmentosa Fighting Blindness, UK; Department of Defense, USA (Chair, 2024); Brain Initiative, NIH, USA

Awards: Thesis medal (1980), IPSEN Foundation International Prize (1985), Fondation pour la recherche thérapeutique (1986), Research To Prevent Blindness International Scholar Award (1990), Research Prize of the French Society of Ophthalmology (1990), Prix de parrainage de la Fondation Alsace (1994), Mérite Typhlophile Gold Medal (Fight against Blindness) (2001), Fondation Bettencourt Schueller 'Coups d'élan pour la Recherche Française' Prize (2002), Knight of the National Order of Merit (2004), Foundation Fighting Blindness (FFB) Trustee Award (2005), Emilia Valori Grand Prix of the French Academy of Sciences (2005), The Institut de France's Foundation NRJ Grand Prix scientifique (2006), Alcon Research Institute Award for Excellence in Vision Research (2006), Elected to the European Academy of Ophthalmology (2006), The Altran Foundation Innovation Award (2007), Quebec Vision Research Network's Prize (Canada) (2007), Elected to the Academia Ophthalmologia Internationalis (2007), Elected to the Academy of Sciences-Institut de France (2007), Gold Medal of the University Pierre et Marie Curie (2008), Knight of the National Order of Legion of Honor (2008), Honoris Causa Doctorate from the University of Geneva (2010), Coscas Medal, Retina (2012), Rome, Jules Gonin lecture, Prize of the of the Retina Research Foundation, Reykjavik (2012), CNRS Medal of Innovation (2012), ARVO Silver Fellow (2013), Prix La Recherche (2013), Officer of the National Order of Merit (France) (2014), Prix Inter-Optiques de la Filière de la Vision (2014), Prix Chaptal of the French Industry (2014), Special Recognition Award Retina International (2014), Elected to the German National Academy of Sciences Leopoldina (2014), Foundation Fighting Blindness (FFB) Llura Liggett Gund Lifetime Achievement Award (2015), Prix Opecst-Inserm (2015), Prix Alfred Monnier (2015), Elected to the National Academy of Technologies of France (2015), Ladies Hospital Aid Society (LHAS) doctor of distinction (2017), Elected Member, The Association of American Physicians (2018), Officer of the National Order of the Legion of Honor (2018), Distinguished Professor at the University of Pittsburgh (2019), Prix Charpak-Duboussset-Académie Nationale de Médecine (with Serge Picaud) (2019), Médaille Grand Vermeil de la Ville de Paris (2019), Dastgheib Pioneer Award in Ocular Innovation-Duke University (2020), Gold Fellow ARVO (2020), Médaille Ambroise Paré-Académie Nationale de Chirurgie (2020), Elected to the National Academy of Surgery of France (2020), Breakthrough of the Year in the Life Sciences category-Falling Walls Foundation, Berlin, Germany (2021), The Chica and Heinz Schaller Foundation Award in Translational Neuroscience (FENS) (2022), Peter Watson Medal-Cambridge University (2022), Elected member of the American Ophthalmology Society (2021, induction in 2022), Commandeur Ordre National du Mérite (2023), Ambroise Paré Medal Award, the National Academy of Surgery (2023), The American Foundation for the Blind Corinne Kirchner Award (2023), The International Prize in Translational Neuroscience-The Reemtsma Stiftung- Max Planck Gesellschaft (with Botond Roska) (2023), Elected Fellow of the National Academy of Inventors (2024), The EURORDIS-Rare Diseases Europe Black Pearl Scientific Award (2024), The Michaelson Award & Lecture, The Macula Society (2024), The Fishman Award and Lecture (2024), The 2024 Wolf Prize in Medicine, The Wolf Foundation (with Botond Roska) (2024), Future Vision Laureate Award (2025).

D. Contributions to Science

1) Together with my team, I worked on the conception, development, and evaluation of innovative treatments for retinal diseases -with a special focus on genetic rod-cone dystrophies- including neuroprotection, stem cells, gene therapy, pharmacology, and artificial retina. I was the first to hypothesize and demonstrate that rod photoreceptors produce a protein that rescues cone photoreceptors, thereby maintaining light-adapted and high-resolution vision. We (with S. Mohand-Said and Thierry Leveillard) identified the underlying signal: Rod-derived Cone Viability Factor (RdCVF) and determined its mechanisms of action (stimulation of aerobic glycolysis, and antioxidant).

- a. Mohand-Said S, Deudon-Combe A, Hicks D, Simonutti M, Forster V, Fintz AC, Léveillard T, Dreyfus H, **Sahel JA**. Normal retina releases a diffusible factor stimulating cone survival in the retinal degeneration mouse. *Proc Natl Acad Sci U S A*. 1998 Jul 7;95(14):8357-62
- b. Mohand-Said S, Hicks D, Léveillard T, Picaud S, Porto F, **Sahel JA**. Rod-cone interactions: development and clinical significance. *Prog Retin Eye Res* 2001; 20: 451-67.
- c. Léveillard T, Mohand-Said S, Lorentz O, Hicks D, Fintz AC, Clérin E, Simonutti M, Forster V, Cavusoglu N, Chalmel F, Dollé P, Poch O, Lambrou G, **Sahel JA**. Identification and characterization of rod-derived cone viability factor. *Nat Genet*. 2004 Jul;36(7):755-9.
- d. Aït-Ali N, Fridlich R, Millet-Puel G, Clérin E, Delalande F, Jaillard C, Blond F, Perrocheau L, Reichman S, Byrne LC, Olivier-Bandini A, Bellalou J, Moyse E, Bouillaud F, Nicol X, Dalkara D, van Dorsselaer A, **Sahel JA**, Léveillard T. Rod-derived Cone Viability Factor acts by stimulating aerobic glycolysis. *Cell*, 2015 May 7;161(4):817-32.

2) RdCVF use is now in translation as a possible therapeutic agent to save cones and treat a spectrum of retinal degenerative diseases as a mutation independent strategy.

- a. Yang Y, Mohand-Said S, Danan A, Simonutti M, Fontaine V, Clerin E, Picaud S, Léveillard T, **Sahel JA**. Functional cone rescue by RdCVF protein in a dominant model of retinitis pigmentosa. *Mol Ther*. 2009 May;17(5):787-95.
- b. Léveillard T, **Sahel JA**. Rod-derived cone viability factor for treating blinding diseases: from clinic to redox signaling. *Science Translational Medicine*. 2010 Apr 7;2(26):26ps16.
- c. Byrne LC, Dalkara D, Luna G, Fisher SK, Clérin E, **Sahel JA**, Léveillard T, Flannery JG. Viral-mediated RdCVF and RdCVFL expression protects cone and rod photoreceptors in retinal degeneration. *J Clin Invest*. 2015;125(1):105-16.
- d. **Sahel JA**, Bennett J, Roska B. Depicting brighter possibilities for treating blindness. *Sci Transl Med*. 2019 May 29;11(494). pii: eaax2324.

The IND for the clinical trial has been granted in 2022 and the trial started in 2023. The first three cohorts were enrolled by the end of 2024, and the study, as validated by the DSMB, will continue at US sites, including Pittsburgh.

3) Utilizing a variety of molecular and functional genomics approaches, my group aims to identify novel disease causing genes for retinal degenerations. Besides research on developmental biology, functional genomics, physiology and therapeutics, my team (with M.Paques, S. Mohand-Said and I. Audo) conducts research on genotype-phenotype correlations with high resolution in vivo non-invasive high-resolution retinal imaging techniques (optical coherence tomography and adaptive optics) aiming at identifying patients eligible for clinical application of innovative therapies. Together with M. Fink (Institut Langevin), I led a very competitive, large, ERC-Synergy grant (2014-2021) aiming at developing novel technologies for morpho-functional imaging of the visual system.

- a. Roska B, **Sahel JA**. Restoring vision. *Nature*. 2018 May;557(7705):359-367
- b. **Sahel JA**, Grieve K, Pagot C, Authié C, Mohand-Said S, Paques M, Audo I, Becker K, Chaumet-Riffaud AE, Azoulay L, Gutman E, Léveillard T, Zeitz C, Picaud S, Dalkara D, Marazova K. Assessing photoreceptor status in retinal dystrophies: from high resolution imaging to functional vision. *Am J Ophthalmol*. 2021 : S0002-9394(21)00212-9

- c. Verschueren A., Boucherit L., Ferrari U., Fouquet S., Nouvel-Jaillard C., Paques M., Picaud S., **Sahel JA**. Planar polarity in primate cone photoreceptors: a potential role in Stiles Crawford effect phototropism, *Commun Biol* 5, 89 (2022)
- d. Authié CN, Poujade M, Talebi A, Defer A, Zenouda A, Coen C, Mohand-Said S, Chaumet-Riffaud P, Audo I, **Sahel JA**. Development and Validation of a Novel Mobility Test for Rod-Cone Dystrophies: From Reality to Virtual Reality. *Am J Ophthalmol*. 2024 Feb;258:43-54. doi: 10.1016/j.ajo.2023.06.028. Epub 2023 Jul 16. PMID: 37437832.

4) We have developed a number of regenerative therapeutic approaches to restore vision such as gene therapy (including the first advanced trials on mitochondrial diseases), leading to the first demonstration in several large scale randomized trials of the efficacy of Gene Therapy in Leber Hereditary Optic Neuropathy.

- a. Vignal C, Uretsky S, Fitoussi S, Galy A, Blouin L, Girmens JF, Bidot S, Thomasson N, Bouquet C, Valero S, Meunier S, Combal JP, Gilly B, Katz B, **Sahel JA**. Safety of rAAV2/2-ND4 Gene Therapy for Leber Hereditary Optic Neuropathy. *Ophthalmology*. 2018 Jun;125(6):945-947.
- b. Newman NJ, Yu-Wai-Man P, Carelli V, Moster ML, Biousse V, Vignal-Clermont C, Sergott RC, Klopstock T, Sadun AA, Barboni P, DeBusk AA, Girmens JF, Rudolph G, Karanja R, Tiel M, Blouin L, Smits G, Katz B, **Sahel JA**. Efficacy and Safety of Intravitreal Gene Therapy for Leber Hereditary Optic Neuropathy Treated within 6 Months of Disease Onset. LHON Study Group. *Ophthalmology*. 2021 May;128(5):649-660.
- c. Yu-Wai-Man P, Newman NJ, Carelli V, Moster ML, Biousse V, Sadun AA, Klopstock T, Vignal-Clermont C, Sergott RC, Rudolph G, La Morgia C, Karanja R, Tiel M, Blouin L, Burguière P, Smits G, Chevalier C, Masonson H, Salermo Y, Katz B, Picaud S, Calkins DJ, **Sahel JA**. Bilateral Visual Improvement with Unilateral Gene Therapy Injection for Leber Hereditary Optic Neuropathy. *Science Translational Medicine* Dec 9; 12 (573) 2021: eaaz7423.
- d. Nancy J Newman, Patrick Yu-Wai-Man, Prem S Subramanian, Mark L Moster, An-Guor Wang, Sean P Donahue, Bart P Leroy, Valerio Carelli, Valerie Biousse, Catherine Vignal-Clermont, Robert C Sergott, Alfredo A Sadun, Gema Rebolleda Fernández, Bart K Chwalisz, Rudrani Banik, Fabienne Bazin, Michel Roux, Eric D Cox, Magali Tiel, **José-Alain Sahel**, the LHON REFLECT Study Group, Randomized trial of bilateral gene therapy injection for m.11778G>A *MT-ND4* Leber optic neuropathy, *Brain*, 2022;, awac421, <https://doi.org/10.1093/brain/awac421>
- e. Yu-Wai-Man P, Newman NJ, Biousse V, Carelli V, Moster ML, Vignal-Clermont C, Klopstock T, Sadun AA, Sergott RC, Hage R, Degli Esposti S, La Morgia C, Priglinger C, Karanja R, Tiel M, **Sahel JA**. Five-Year Outcomes of Lenadogene Nolparvovec Gene Therapy in Leber Hereditary Optic Neuropathy. *JAMA Ophthalmol*. 2024 Dec 19. doi: 10.1001/jamaophthalmol.2024.5375. PMID: 39699886.

5) With B. Roska at the Institute of Ophthalmology of Basel, S.Picaud and Deniz Dalkara at Institut de la Vision in Paris, we demonstrated that different retinal cell types such as "dormant cones" and Ganglion Cells can be converted into "artificial photoreceptors" by targeting the expression of genetically encoded light sensors enabling mice with retinal degeneration to perform visually guided behaviors. I led the first demonstration of the efficacy of Optogenetics in partial function restoration in humans. I also led (with Dr. Palanker) the first in-human wireless artificial retina trial.

- a. Busskamp V, Duebel J, Balya D, Fradot M, Viney TJ, Siebert S, Groner AC, Cabuy E, Forster V, Seeliger M, Biel M, Humphries P, Paques M, Mohand-Said S, Trono D, Deisseroth K, **Sahel JA**, Picaud S, Roska B. Genetic Reactivation of Cone Photoreceptors Restores Visual Responses in Retinitis pigmentosa. *Science*. 2010 329(5990):413-7.
- b. Palanker D, Le Mer Y, Mohand-Said S, Muqit M, **Sahel JA**. Photovoltaic Restoration of Central Vision in Atrophic Age-Related Macular Degeneration. *Ophthalmology*. 2020 Feb 25. pii: S0161-6420(20)30189-5.
- c. **Sahel JA**, Boulanger-Scemama E, Pagot C, Arleo A, Galuppi F, Martel JN, Degli Esposti S, Delaux A, de Saint Aubert JB, de Montleau C, Gutman E, Audo I, Duebel J, Picaud S, Dalkara D, Blouin L, Tiel

M, Roska B. Partial recovery of visual function in a blind patient after optogenetic therapy. *Nature Medicine* 2021 Jul;27(7):1223-1229.

- d. Palanker D, Le Mer Y, Mohand-Said S, Sahel JA. Simultaneous Perception of Prosthetic and Natural Vision in AMD Patients, *Nature Communications*, (2022) 13:513

E. Additional Information: Research Support and/or Scholastic Performance

Ongoing research support

- 2024-2028 ARPA-H: THEA Eye Transplantation : Sub No. 63619542-341318 |around 9M\$
- 2022–2026 NIH/NEI R01 EY033049-01A1: “Retinal-adhesive thermoresponsive gel for AAV-mediated gene delivery to the outer retina” \$2,857,940
- 2022–2026 Research to Prevent Blindness: Unrestricted Grant (Support research at the University of Pittsburgh Department of Ophthalmology) \$575,000
- 2020–2025 NEI/NIH: R01EY030517 “Distinguishing normal aging from age-related macular degeneration at the level of single cells in the living human eye” \$2.4M
- 2019-2024 Director of the University-Hospital Institute “FOReSIGHT”, 50M€, role: PI; goals: creation of unique university-hospital environment of multidisciplinary and collaborative research allowing a rapid and highly efficient translation of scientific discoveries to the clinic and advanced education of researchers and clinicians.
- 2021-2025 Grant from the Hillman Foundation (Access to care, innovative vision restoration therapies-\$20M)
- 2022-2024 Grants from the Beckwith Institute, UPMC/the Richard King Mellon for a clinical trial on Cortical Vision (\$1M)
- 2023-2025 Bespoke GCT Program (The NIH Foundation) : new AAV Vectors (co-I; Leah Byrne PI). : \$500K
- 2022-2027 UNIRARE Natural History Study in Retinal Degenerations (FFB, 40 centers) Coordinator, \$6M
- 2022-2024 DOD-MTEC Grant (selected for funding 2022-2024) : MTEC-22-02-MPAI-001, PI, “Towards High Resolution Vision Restoration by Optogenetic Therapy” \$3M
- 2022-2026 NEI RO1: PI EY033049 : Targeting Central Retina in Gene Therapy, \$2.9M
- 2023-2025 Foundation for the National Institutes of Health (FNIH): “Quantification of AAV dose-response with single cell resolution” \$575,000
- 2019–2024 Foundation Fighting Blindness PPA-0819-0772-INSERM: Next Generation Optogenetics for Vision Restoration: Development of an in vivo toolkit for assessing the remaining retinal cells in RCD retinas with novel imaging techniques” \$361,976
- 2023-2024 Eye and Ear Foundation/RK Mellon Foundation “Clinical Trial to Test the Efficacy of a Neuroprosthetic to Treat and Cure Blindness” \$300,000
- 2022-2028 JAEB Center for Health Research Foundation/Foundation Fighting Blindness Consortium # 2870/Uni-Rare “Universal Rare Gene Study: A Registry and Natural History Study of Retinal Dystrophies Associated with Rare Disease-Causing Genetic Variants” \$212,816
- 2019-2024 NIH/Oregon Health Sciences University: R01HD095968 “The Natural History of LCHAD Retinopathy” \$132,714

Completed research support

- 2005-2009 Coordinator of the Integrated project Functional GENOMIC of the RETina-EVI-GENORET, the largest Vision Project funded by EU (FP6- 512036) 10M€; goals: genetic and genomic characterization of retinal diseases; establishment of therapeutic targets for novel strategies to fight blindness
- 2007-2017 Coordinator of the Foundation Fighting Blindness Paris Research Centre (C-CL-0912-0600-INSERM01; C-GE-0912-0601-INSERM02) 670 000\$; 1,8M\$; goals: characterization of patients with rare retinal diseases, creation of cohorts of patients with genetic and age-related retinal dystrophies

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| 2014-2019 | Coordinator of the project “SIGHT AGAIN” (ANR-BPI France) appr. 6M€, role: PI; goals: development of optogenetic and prosthetic approaches for vision restoration |
| 2017-2018 | DARPA Grant on CORTICAL SIGHT \$6M, role: PI; goals: studies of neuronal circuitry of visual processing and development of therapeutic strategies for vision restoration at cortical level |
| 2017-2019 | Thome Foundation Research Grant on Age-related Macular Degeneration \$500k, role: PI with Ethan Rossi; goals: High resolution imaging and deep phenotyping in age-related macular degeneration. |
| 2014-2020 | Coordinator of the European Research Council Synergy Grant “HELMHOLTZ” 13M€, role: PI; goals: developing and implementing in the clinic of high resolution/cell-resolution in vivo non-invasive retinal imaging technologies |
| 2015-2021 | Coordinator of the Recherche Hospitalo-Universitaire Program “LIGHT4DEAF”: USHER Syndrome 9.5M€, PI |
| 2011-2022 | Director Laboratory of Excellence “LIFESENSES: Senses for a Lifetime”, Investments in the Future initiative of the French government (ANR-10-LABX-65) 22M €, role: PI; goals: deciphering fundamental mechanisms of vision and hearing loss, development of therapeutic strategies to preserve and rescue vision and audition |
| 2006-2022 | Director of Carnot Institute funding (National Agency for Research) 300-800k€/y, role: PI; goals: development of academia-industry partnerships for promoting translational research |
| 2019-2022 | Grant from the Richard King Mellon Foundation to the Cortical Sight Project \$6M |
| 2022-2023 | Grant from the Jewish Healthcare and Pittsburgh Foundations: a quality process based on Digital Twins. \$400k |

Organization of International Conferences and Symposia

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| April 2002 | Consensus Conference (Foundation Fighting Blindness, Inserm, AFM) “Clinical Trials Orphan Retinal Degenerations”, Paris, France |
| April 2005 | “Ophthalmic Pan-European Clinical Trials and the Impact of EU Directive” Royal Soc. of Med., London, |
| July 2007 | “Hearing and seeing: Fighting sensory disabilities” Collège de France, Paris, France |
| Dec 2008 | Fondation Singer-Polignac/“Seeing and Hearing” Foundation/Institute of Ophthalmology UCL/Institut de la Vision Symposium “Fighting blinding diseases: towards a joint endeavour” Paris, France |
| Nov 2009 | ESF-UB Conference in Biomedicine in association with Eurovisionnet and Fondation Voir et Entendre “Rare Diseases II: Hearing and sight loss”, Sant Feliu de Guixols, Spain |
| April 2014 | Symposium “Stem Cell and Gene Therapy”, World Ophthalmology Congress, Tokyo, Japan |
| April 2014 | First French-Japanese Conference “Innovation in Ophthalmology”, Tokyo, Japan |
| June 2014 | Symposium “Colors and Art” celebrating Prof. Philippe Lanthony's career, Paris, France |
| July 2014 | Symposium "Cell and Gene Therapy Approaches in Retinal Degenerations", FENS Forum, Milan Italy |
| Febr 2015 | Vision-Innovation Congress, UNESCO Paris, France |
| April 2016 | Vision Research Symposium, Institut de la Vision - Nuffield Laboratory of Ophthalmology, Univ. of Oxford |
| June 2016 | International Symposium « Cortical plasticity in auditory and visual impairment », Collège de France, Paris, |
| June 2016 | International Symposium « Vision restoration: New strategies, new challenges », Collège de France, Paris, |
| Sept 2016 | Second French-Japanese Conference “Innovation in Ophthalmology”, Tokyo, Japan |
| April 2018 | French-Japanese Scientific Seminar "Regenerative Medicine for Vision Restoration”, Kobe, Japan |
| May 2019 | French American Symposium on Inherited Retinal Dystrophies, Los Angeles, US |
| Nov 2019 | French-Japanese Scientific Seminar "Vision restoration: emerging therapies", Paris, France |
| Nov 2019 | International Conference “10 years of fighting blindness”, Paris, France |

- Nov 2017-2024 Optic nerve Regeneration, The Fox Center annual meeting, Pittsburgh, PA, USA
 Sept 2021 Workshop on Usher 1b Syndrome, Foundation Fighting Blindness
 Oct 2021 International Symposium on Usher Syndrome
 Sept 2022 The Cambridge Ophthalmology Symposium: The Eye and the Brain, Cambridge, UK
 Oct 2022 Joint Symposium University of Pittsburgh-FDA on Outcomes in Bioengineered Implants for the Visual System
 Nov 2022 Joint French-Japanese Scientific Seminar 2022 - Vision Restoration: Emerging Therapeutic Approaches, Paris, France
 Febr 2024 The Macula Society Meeting The Michaelson Lecture “Gene-Independent Strategies in Retinal Dystrophies: The Translational Pathways” Palm Springs, CA USA

Recent Lectures

- Feb 2023 Glaucoma Research Foundation, Glaucoma 360 12th Annual New Horizons Forum “The future in vision restoration and neuroprotection”, San Francisco, CA, USA
 Mar 2023 Pittsburgh Ophthalmology Society, 58th Annual Meeting, “Gene therapy for retinal degenerations and ARMD: promises and challenges”, Pittsburgh, PA, USA
 Mar 2023 International Rare Diseases Research Consortium Conference (IRDiRC) and RE(ACT) Congress 2023 “Developing Gene-Independent Approaches for Retinal Dystrophies”, Berlin, Germany
 Apr 2023 The Association for Research in Vision and Ophthalmology (ARVO) 2023 Annual Meeting “Bringing gene-independent strategies to the clinic: focus on Optogenetics”, New Orleans, LA, USA
 June 2023 Académie Nationale de Chirurgie (The National Academy of Surgery) “Gene therapy and hereditary retinal dystrophy”, Paris, France
 Aug 2023 Association for Ocular Pharmacology and Therapeutics XVI Biennial Meeting 2023 (AOPT) Keynote “Gene-independent Strategies in Retinal Degenerations”, Indianapolis, IN, USA
 Sept 2023 Byrne Lab Science, 2023 Pittsburgh Gene Therapy Bootcamp Lecture Series invited speaker, “Optogenetics in the clinic”, UPMC Vision Institute, Pittsburgh, PA, USA
 Sept 2023 Vanderbilt Eye Institute’s (VEI) Distinguished Lecture Series “Gene-independent strategies in retinal degenerations”
 Oct 2023 European Vision & Eye Research (EVER) Board Congress 2023 – the De Laey EVER Keynote Lecture “Cone-directed strategies in retinal degenerations”, Valencia, Spain
 Nov 2023 France Science Summit, invited panelist and guest speaker, “Overview of Franco-American Partnerships, highlighting key cooperation tools”, The Embassy of France, Washington, DC, USA
 Nov 2023 The University of Pittsburgh School of Medicine, Physician Scientist Training Program (PSTP) Symposium keynote speaker, Pittsburgh, PA, USA
 Jan 2024 Monaciano III Symposium, “Stabilizing the IRD therapy business model”, Rome, Italy
 Febr 2024 47th Annual Macula Society Meeting – I.C. Michaelson Symposium “Gene-Independent Strategies in Retinal Dystrophies: The Translational Pathways”, Palm Springs, CA, USA
 May 2024 2024 Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting invited symposium speaker and moderator, “Neuro-ophthalmology and genetics”, Seattle, WA, USA
 May 2024 Kresge Eye Institute – Robert and Gerry Ligon Lecture “Developing and Accessing Vision Restoration Strategies”

June 2024 Retina International World Congress 2024, joint Retina International and ERN-EYE meeting, 2nd Multi-Stakeholders Meeting on Clinical Trials for Inherited Retinal Diseases – “Challenges for Innovative Treatments: LHON, the challenges of Control groups”, Dublin, Ireland

June 2024 From the Eye to the Brain, Nature Conference “Neuroprotection of Cone Photoreceptors as a Gene-Independent therapeutic strategy in Inherited retinal diseases” Rome, Italy

June 2024 Sculpted Light in the Brain Conference “Sculpting the Future of Vision Restoration” Paris, France

June 2024 Institute of Molecular and Clinical Ophthalmology Basel (IOB) “Translational studies in neuroprotection and vision restoration” Basel, Switzerland

July 2024 Gilbert Family Foundation, Vision Restoration Institute Annual Meeting panelist, “NF1-OPG – Setting preclinical evidence standards for initiating clinical trials”

Aug 2024 33rd Biennial Symposium of the Center for Visual Science Keynote Opening Lecture “Developing Gene-Independent Approaches for Retinal Dystrophies” Rochester, NY USA

Aug 2024 Byrne Lab Science, 2024 Pittsburgh Gene Therapy Bootcamp Lecture Series invited speaker, “Gene Therapy Clinical Trial Endpoints”, UPMC Vision Institute, Pittsburgh, PA, USA

Sept 2024 Military Operational Medicine Research Program Neurosensory Injury Prevention and Treatment IPR Virtual Meeting, “Towards High Resolution Vision Restoration by Optogenetic Therapy”

Sept 2024 Center for Connected Medicine & UPMC Enterprises, Top of Mind Summit: Life Sciences 2024 panelist, “Getting Close & Personal: Strategies for Efficient Trials and Enhanced Patient Engagement”

Sept 2024 2024 i2Eye Conference, “Optogenetics, gene therapy, & vision restoration”, UPMC Vision Institute, Pittsburgh, PA, USA

Oct 2024 2024 Society for Neuroscience Meeting invited speaker and moderator “Miracles Can Happen: Recent Advances in the Restoration of Vision, Hearing, and Touch”, Chicago, IL, USA

Oct 2024 Inserm, “International Joint Labs: a new Inserm initiative to enhance collaborations” The Embassy of France, Washington, DC, USA

Oct 2024 It’s Possible at Pitt: Life Sciences Series, “Life Sciences Economic Development Approach”, the University of Pittsburgh School of Medicine, Pittsburgh, PA

Nov 2024 The Fichman Lecture: Cone Rescue in Retinal Dystrophies, Jerusalem- Hadassah Hospital and Hebrew University

Dec 2024 Bascom Palmer Eye Institute (BPEI) James V. Bastek, MD Inaugural Endowed Speaker Series on Hereditary Retinal Disease Research; and “Gene Therapy for Leber Hereditary Optic Neuropathy”, / “Vision Restoration: Prosthetics and Optogenetics”, / “Visual Outcomes in Retinal Degeneration Trials”, Miami, FL, USA

Dec 2024 Optogenetics Symposium – From Scientific Discovery to Medical Application to Societal Impact, “Translational Pathway for Optogenetic Vision restoration” Charité Hospital-Leopoldina Academy, Berlin, Germany

Dec 2024 Humboldt University, (ECN) Optogenetics Speaker Series, “Challenges in Vision Restoration Research”, Berlin, Germany

Dec 2024 Urania-City of Berlin Public Lecture “Is the human dream of restoring sight to blind people coming true?”, Berlin, Germany

- Jan 2025 Collaborative Community on Ophthalmic Imaging (CCOI) 2025 Virtual Conference invited speaker, moderator and group leader “From Retinal Prostheses to Optogenetics”
- Jan 2025 Collaborative Community on Ophthalmic Imaging (CCOI) 2025 Virtual Conference invited speaker, moderator, group leader and session chair “Vision Assessment and Outcome Measures”
- Jan 2025 Keynote Lecture: Gene Therapy for LHON, Optic Nerve Network “Optic Nerve Diseases”, Milan, Italy

Recent Webinars/Podcasts

- June 2023 Association for Research in Vision and Ophthalmology (ARVO) SIG invited speaker and panelist, “PRPH2-related retinal disease: A discussion of current clinical observations and basic science towards the development of effective therapies”
- Feb 2024 Foundation Fighting Blindness (FFB), Eye on the Cure Podcast – Episode 61
- June 2024 The Eye & Ear Foundation of Pittsburgh, Sight + Sound Bites: Seeing the Future of Gene Therapy panelist, “Genetics and Gene Therapy Options”
- Sept 2024 Del Monte Institute for Neuroscience Podcast, “Neuroscience Prospectives: How One Doctor is Revolutionizing Vision Loss”, the University of Rochester, Rochester, NY, USA
- Nov 2024 FOReSIGHT Academy: when Research and Clinical meet The Gene Therapy Revolution, “Gene-independent gene therapies: optogenetics and RdCVF”

More than 90 patents

Publications (More than 750 peer-reviewed articles)

<https://www.ncbi.nlm.nih.gov/myncbi/1BUauHP0OUhAh/bibliography/public/>

Selected 10:

1. Mohand-Said S, Deudon-Combe A, Hicks D, Simonutti M, Forster V, Fintz AC, Léveillard T, Dreyfus H, **Sahel JA**. Normal retina releases a diffusible factor stimulating cone survival in the retinal degeneration mouse. *Proc Natl Acad Sci U S A*. 1998 Jul 7;95(14):8357-62. doi: 10.1073/pnas.95.14.8357. PMID: 9653191; PMCID: PMC20980. <https://pubmed.ncbi.nlm.nih.gov/9653191/>
2. Léveillard T, Mohand-Said S, Lorentz O, Hicks D, Fintz AC, Clérin E, Simonutti M, Forster V, Cavusoglu N, Chalmel F, Dollé P, Poch O, Lambrou G, **Sahel JA**. Identification and characterization of rod-derived cone viability factor. *Nat Genet*. 2004 Jul;36(7):755-9. doi: 10.1038/ng1386. Epub 2004 Jun 27. PMID: 15220920. <https://pubmed.ncbi.nlm.nih.gov/15220920/>
3. Aït-Ali N, Fridlich R, Millet-Puel G, Clérin E, Delalande F, Jaillard C, Blond F, Perrocheau L, Reichman S, Byrne LC, Olivier-Bandini A, Bellalou J, Moyses E, Bouillaud F, Nicol X, Dalkara D, van Dorsselaer A, **Sahel JA**, Léveillard T. Rod-derived cone viability factor promotes cone survival by stimulating aerobic glycolysis. *Cell*. 2015 May 7;161(4):817-32. doi: 10.1016/j.cell.2015.03.023. PMID: 25957687. <https://pubmed.ncbi.nlm.nih.gov/25957687/>
4. Roska B, **Sahel JA**. Restoring vision. *Nature*. 2018 May;557(7705):359-367. doi: 10.1038/s41586-018-0076-4. Epub 2018 May 16. PMID: 29769667. <https://pubmed.ncbi.nlm.nih.gov/29769667/>

5. **Sahel JA**, Bennett J, Roska B. Depicting brighter possibilities for treating blindness. *Sci Transl Med*. 2019 May 29;11(494):eaax2324. doi: 10.1126/scitranslmed.aax2324. PMID: 31142676. <https://pubmed.ncbi.nlm.nih.gov/31142676/>
6. Yu-Wai-Man P, Newman NJ, Carelli V, Moster ML, Biousse V, Sadun AA, Klopstock T, Vignal-Clermont C, Sergott RC, Rudolph G, La Morgia C, Karanjia R, Tiel M, Blouin L, Burguière P, Smits G, Chevalier C, Masonson H, Salermo Y, Katz B, Picaud S, Calkins DJ, **Sahel JA**. Bilateral visual improvement with unilateral gene therapy injection for Leber hereditary optic neuropathy. *Sci Transl Med*. 2020 Dec 9;12(573):eaaz7423. doi: 10.1126/scitranslmed.aaz7423. PMID: 33298565. <https://pubmed.ncbi.nlm.nih.gov/33298565/>
7. **Sahel JA**, Boulanger-Scemama E, Pagot C, Arleo A, Galluppi F, Martel JN, Esposti SD, Delaux A, de Saint Aubert JB, de Montleau C, Gutman E, Audo I, Duebel J, Picaud S, Dalkara D, Blouin L, Tiel M, Roska B. Partial recovery of visual function in a blind patient after optogenetic therapy. *Nat Med*. 2021 Jul;27(7):1223-1229. doi: 10.1038/s41591-021-01351-4. Epub 2021 May 24. PMID: 34031601. <https://pubmed.ncbi.nlm.nih.gov/34031601/>
8. Nancy J Newman, Patrick Yu-Wai-Man, Prem S Subramanian, Mark L Moster, An-Guor Wang, Sean P Donahue, Bart P Leroy, Valerio Carelli, Valerie Biousse, Catherine Vignal-Clermont, Robert C Sergott, Alfredo A Sadun, Gema Rebolleda Fernández, Bart K Chwalisz, Rudrani Banik, Fabienne Bazin, Michel Roux, Eric D Cox, Magali Tiel, **José-Alain Sahel**, the LHON REFLECT Study Group, Randomized trial of bilateral gene therapy injection for m.11778G>A *MT-ND4* Leber optic neuropathy, *Brain*, 2022;, awac421, <https://doi.org/10.1093/brain/awac421>
9. Palanker D, Le Mer Y, Mohand-Said S, **Sahel JA**. Simultaneous perception of prosthetic and natural vision in AMD patients. *Nat Commun*. 2022 Jan 26;13(1):513. doi: 10.1038/s41467-022-28125-x. PMID: 35082313; PMCID: PMC8792035. <https://pubmed.ncbi.nlm.nih.gov/35082313/>
10. Authié CN, Poujade M, Talebi A, Defer A, Zenouda A, Coen C, Mohand-Said S, Chaumet-Riffaud P, Audo I, **Sahel JA**. Development and Validation of a Novel Mobility Test for Rod-Cone Dystrophies: From Reality to Virtual Reality. *Am J Ophthalmol*. 2024 Feb;258:43-54. doi: 10.1016/j.ajo.2023.06.028. Epub 2023 Jul 16. PMID: 37437832. <https://pubmed.ncbi.nlm.nih.gov/37437832/>