



Pasqualina Colella

Affiliate, Pediatrics - Genetics

Bio

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Dr. Pasqualina Colella is a scientist with expertise in gene and cell therapy for inherited diseases. She is currently a Senior Research Scientist at Stanford University, where she leads translational research on hematopoietic stem cell transplantation (HSCT), microglia replacement, myeloid cell recruitment to the central nervous system, and genome editing. Her goal is to improve treatment outcomes for neurodegenerative lysosomal storage diseases.

Dr. Colella earned her PhD at TIGEM (Italy), where she developed gene therapies for inherited retinal disorders, including dual AAV approaches for large gene delivery targeting Usher syndrome type 1B and Stargardt disease. During her postdoctoral studies at Genethon (France), she advanced AAV-based gene therapy for Pompe disease, improving efficacy through transgene and promoter engineering. Her work has contributed to several ongoing clinical trials, including those for USH1B and Pompe disease.

Her research has led to more than 30 peer-reviewed publications (16 as first author, 7 as co-corresponding) and 6 patents. She has received awards including the Marie Skłodowska-Curie Fellowship, the Innovative Research Grant from the National MPS Society, and Stanford School of Medicine Shoshana Levy Award to Support Women in Science (Career Development Award). She was also a finalist for the ERC Starting Grant in 2019.