

Fondazione Telethon-funded Research Positions Available in the Cavazza group

The newly established Cavazza group at the Centre for Genomic Medicine and Rare Diseases at **UniMORE** (**Italy**) is looking to recruit a **post-doctoral fellow** and a **graduate fellow** starting from Spring 2025.

The group's research activities focus on designing and optimising genome editing-based strategies to treat rare diseases. Specifically, the research positions will be dedicated to the development of blood brain barrier (BBB)-crossing tools to deliver CRISPR-Cas and base editors to the brain, with the final aim to treat neurodegenerative traits of lysosomal storage disorders.

Your role:

- Characterize BBB-targeted delivery tools carrying gene and base editors
- Design and perform screening experiments in cell lines and 3D BBB-on-a-chip systems
- Assess the therapeutic efficacy of the devised gene editing platform in correcting the disease in relevant in vitro and in vivo models
- Closely collaborate with the Cavazza group situated at the UCL Great Ormond Street Institute of Child Health (London, UK)
- Contribute to the operation and maintenance of lab infrastructure and equipment
- · Mentor undergraduate students

Education and experience:

- Ph.D. in Biology or equivalent qualification (for postdoctoral fellow position); B.Sc. or M.Sc. in Biology, Botechnology or equivalent qualifications (for graduate fellow position)
- •Previous experience in cellular and molecular biology
- •Previous experience with CRISPR-Cas technology
- •Ability to work independently.
- •Team player with good communication skills (Italian and English preferred)

Lab Location:

Centre for Genomic Medicine and Rare Diseases
Department of Medical and Surgical Sciences For Children and Adults
University of Modena and Reggio Emilia
Azienda Ospedaliero Universitaria Policlinico di Modena, via del Pozzo 71, Modena, Italy



he total duration of the contract will be 2 years with the possibility of renewal. The salary will be defined according to the applicant's experience.

In order to submit your application, please send an email with **your CV and a motivation letter** to **alessia.cavazza@unimore.it**

Relevant publications:

- 1. Cavazza A*, Molina-Estévez FJ, Plaza Reyes A, Ronco V, Naseem A, Malenšek S., Pečan P, Santini A, Heredia P, A Aguilar-González, H Boulaiz, Q Ni, M Cortijo-Gutierrez, KPavlovic, I Herrera, B de la Cerda, E M Garcia-Tenorio, E Richard, S Granados-Principal, A López-Márquez, M Köber, M Stojanovic, M Vidaković, I Santos-Garcia, L Blázquez, E Haughton, D Yan, R M Sánchez-Martín, L Mazini, G Gonzalez Aseguinolaza, A Miccio, P Rio, L R. Desviat, M A.F.V. Gonçalves, L Peng, C Jiménez-Mallebrera, F Martin Molina, D Gupta, D Lainšček, Y Luo*, K Benabdellah*. Advanced Delivery Systems for Gene Editing: A Comprehensive Review from the GenE-HumDi COST Action Working group. Molecular Therapy Nucleic Acids, 2025; https://doi.org/10.1016/j.omtn.2025.102457.
- **2.** Rai R, Steinberg Z, Romito M, Zinghirino F, Hu YT, White N, Naseem A, Thrasher AJ, Turchiano G, **Cavazza A***. CRISPR/Cas9-Based Disease Modeling and Functional Correction of Interleukin 7 Receptor Alpha Severe Combined Immunodeficiency in T-Lymphocytes and Hematopoietic Stem Cells. <u>Human Gene Therapy</u>. 2024 Apr;35(7-8):269-283. doi: 10.1089/hum.2023.100. Epub 2024 Feb 29
- **3.** Rai R, Naseem A, Vetharoy W, Steinberg Z, Thrasher AJ, Santilli G, **Cavazza A***. An improved medium formulation for efficient *ex vivo* gene editing, expansion and engraftment of hematopoietic stem and progenitor cells. Molecular Therapy Methods Clin Dev. 2023 Feb 28;29:58-69. doi: 10.1016/j.omtm.2023.02.014.
- **4.** Rai R, Romito M, Rivers E, Turchiano G, Blattner G, Vetharoy W, Ladon D, Andrieux G, Zhang F, Zinicola M, Leon-Rico D, Santilli G, Thrasher AJ, **Cavazza A***. Targeted gene correction of human hematopoietic stem cells for the treatment of Wiskott Aldrich Syndrome. <u>Nature Communications</u>. 2020 Aug 12;11(1):4034.