




Francesco Puzzo

Postdoctoral Scholar, Human Gene Therapy

 NIH Biosketch available Online

 Curriculum Vitae available Online

Bio

PROFESSIONAL EDUCATION

- Doctor of Philosophy, Universita Degli Studi Di Ferrara (2017)

Publications

PUBLICATIONS

- **Aptamer-programmable adeno-associated viral vectors as a novel platform for cell-specific gene transfer.** *Molecular therapy. Nucleic acids*
Puzzo, F., Zhang, C., Powell Gray, B., Zhang, F., Sullenger, B. A., Kay, M. A.
2023; 31: 383-397
- **Loops as Determinant of AAV Integration by Homologous Recombination**
Puzzo, F., Zhang, F., Pekrun, K., Kay, M. A.
CELL PRESS.2022: 90-91
- **Programmable Adeno-Associated Viral Vectors for Cell Specific Targeting**
Puzzo, F., Zhang, C., Zhang, F., Kay, M. A.
CELL PRESS.2022: 66
- **Promoterless AAV Vectors with Homology Arms Can Integrate and Express from Transcriptionally Active Sites in Non-Targeted Loci**
Stephens, C. J., Pekrun, K., Xu, J., Tsuji, S., Jing, Y., Puzzo, F., Zhang, F., Kay, M. A.
CELL PRESS.2022: 391-392
- **Improved Genome Editing through Inhibition of FANCM and Members of the BTR Dissolvase Complex.** *Molecular therapy : the journal of the American Society of Gene Therapy*
de Alencastro, G. n., Puzzo, F. n., Pavel-Dinu, M. n., Zhang, F. n., Pillay, S. n., Majzoub, K. n., Tiffany, M. n., Jang, H. n., Sheikali, A. n., Cromer, M. K., Meetei, R. n., Carette, J. E., Porteus, et al
2021; 29 (3): 1016-27
- **Rescue of Advanced Pompe Disease in Mice with Hepatic Expression of Secretable Acid α -Glucosidase.** *Molecular therapy : the journal of the American Society of Gene Therapy*
Cagin, U., Puzzo, F., Gomez, M. J., Moya-Nilges, M., Sellier, P., Abad, C., Van Wittenberghe, L., Daniele, N., Guerchet, N., Gjata, B., Collaud, F., Charles, S., Sola, et al
2020; 28 (9): 2056-2072
- **Proteins Complex of the Fanconi Anemia Pathway as Determinant of AAV-Mediated Genomic Targeted Integration**
Puzzo, F., de Alencastro, G., Pavel-Dinu, M., Zhang, F., Pillay, S., Majzoub, K., Tiffany, M., Jang, H., Sheikali, A., Cromer, K. M., Meetei, R., Carette, J. E., Porteus, et al
CELL PRESS.2020: 459
- **Exploiting the Regenerative Capacity of Liver for Nuclease-Free Genome Editing**
Puzzo, F., De Alencastro, G., Patijn, G., Zhang, F., Pekrun, K., Kay, M.

CELL PRESS.2019: 463

- **AAV Gene Transfer with Tandem Promoter Design Prevents Anti-transgene Immunity and Provides Persistent Efficacy in Neonate Pompe Mice** *MOLECULAR THERAPY-METHODS & CLINICAL DEVELOPMENT*
Colella, P., Sellier, P., Verdera, H., Puzzo, F., van Wittenberghe, L., Guerchet, N., Daniele, N., Gjata, B., Marmier, S., Charles, S., Sola, M., Ragone, I., Leborgne, et al
2019; 12: 85–101
- **Targeting Mitochondrial Defects to Increase Longevity in Animal Models of Neurodegenerative Diseases** *REVIEWS ON BIOMARKER STUDIES OF METABOLIC AND METABOLISM-RELATED DISORDERS*
Casajus Pelegay, E., Puzzo, F., Yilmazer, A., Cagin, U., Guest, P. C.
2019; 1134: 89–110
- **Targeting Mitochondrial Defects to Increase Longevity in Animal Models of Neurodegenerative Diseases.** *Advances in experimental medicine and biology*
Casajus Pelegay, E., Puzzo, F., Yilmazer, A., Cagin, U.
2019; 1134: 89–110
- **Rescue of GSDIII Phenotype with Gene Transfer Requires Liver- and Muscle-Targeted GDE Expression** *MOLECULAR THERAPY*
Vidal, P., Pagliarani, S., Colella, P., Verdera, H., Jauze, L., Gjorgjieva, M., Puzzo, F., Marmier, S., Collaud, F., Sola, M., Charles, S., Lucchiari, S., van Wittenberghe, et al
2018; 26 (3): 890–901
- **Rescue of Pompe disease in mice by AAV-mediated liver delivery of secretable acid alpha-glucosidase** *SCIENCE TRANSLATIONAL MEDICINE*
Puzzo, F., Colella, P., Biferi, M. G., Bali, D., Paulk, N. K., Vidal, P., Collaud, F., Simon-Sola, M., Charles, S., Hardet, R., Leborgne, C., Meliani, A., Cohen-Tannoudji, et al
2017; 9 (418)
- **Autophagy determines efficiency of liver-directed gene therapy with adeno-associated viral vectors.** *Hepatology (Baltimore, Md.)*
Hösel, M., Huber, A., Bohlen, S., Lucifora, J., Ronzitti, G., Puzzo, F., Boisgerault, F., Hacker, U. T., Kwanten, W. J., Klötting, N., Blüher, M., Gluschko, A., Schramm, et al
2017; 66 (1): 252–265
- **Liver-Mediated Gene Therapy with an Engineered Secretable GAA Transgene Results in Whole-Body Correction of Pompe Disease**
Ronzitti, G., Colella, P., Puzzo, F., Biferi, M., Bali, D., Paulk, N. K., Vidal, P., Collaud, F., Sola, M., Charles, S., Hardet, R., Leborgne, C., Meliani, et al
CELL PRESS.2017: 244
- **Long-term exposure to Myozyme results in a decrease of anti-drug antibodies in late-onset Pompe disease patients.** *Scientific reports*
Masat, E., Laforêt, P., De Antonio, M., Corre, G., Perniconi, B., Taouagh, N., Mariampillai, K., Amelin, D., Mauhin, W., Hogrel, J. Y., Caillaud, C., Ronzitti, G., Puzzo, et al
2016; 6: 36182
- **The carboxyl-terminal region is NOT essential for secreted and functional levels of coagulation factor X.** *Journal of thrombosis and haemostasis : JTH*
Branchini, A., Baroni, M., Burini, F., Puzzo, F., Nicolosi, F., Mari, R., Gemmati, D., Bernardi, F., Pinotti, M.
2015; 13 (8): 1468–74