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Bio

PROFESSIONAL EDUCATION

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

Publications

PUBLICATIONS

- **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*
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- **Proteins Complex of the Fanconi Anemia Pathway as Determinant of AAV-Mediated Genomic Targeted Integration**
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- **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. n., Puzzo, F. n., Gomez, M. J., Moya-Nilges, M. n., Sellier, P. n., Abad, C. n., Van Wittenberghe, L. n., Daniele, N. n., Guerchet, N. n., Gjata, B. n., Collaud, F. n., Charles, S. n., Sola, et al
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- **Exploiting the Regenerative Capacity of Liver for Nuclease-Free Genome Editing**
Puzzo, F., De Alencastro, G., Patijn, G., Zhang, F., Pekrun, K., Kay, M.
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- **AAV Gene Transfer with Tandem Promoter Design Prevents Anti-transgene Immunity and Provides Persistent Efficacy in Neonate Pompe Mice** *MOLECULAR THERAPY-METHODS & CLINICAL DEVELOPMENT*
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- **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.
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- **Targeting Mitochondrial Defects to Increase Longevity in Animal Models of Neurodegenerative Diseases.** *Advances in experimental medicine and biology*
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- **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*
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- **Long-term exposure to Myozyme results in a decrease of anti-drug antibodies in late-onset Pompe disease patients.** *Scientific reports*
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