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Publications

PUBLICATIONS

- **Improved engraftment and therapeutic efficacy by human genome-edited hematopoietic stem cells with Busulfan-based myeloablation.** *Molecular therapy. Methods & clinical development*
Poletto, E., Colella, P., Pimentel Vera, L. N., Khan, S., Tomatsu, S., Baldo, G., Gomez-Ospina, N.
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- **Genome Editing for Mucopolysaccharidoses.** *International journal of molecular sciences*
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2020; 21 (2)
- **Engineering monocyte/macrophage-specific glucocerebrosidase expression in human hematopoietic stem cells using genome editing** *Nature Communications*
Scharenberg, S. G., Poletto, E., Lucot, K. L., Colella, P., Sheikali, A., Montine, T. J., Porteus, M. H., Gomez-Ospina, N.
2020; 11: 1-14
- **Genome-edited autologous stem cell transplantation with enhanced brain conditioning to correct progranulin deficiency**
Colella, P., Suarez-Nieto, M. V., Tejeda-Polanco, E., Vera, L., Poletto, E., Basurto, J., Gomez-Ospina, N.
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- **Hematopoietic stem cell transplantation for lysosomal disorders: Unraveling the mechanisms for CNS repopulation**
Arozqueta-Basurto, J., Poletto, E., Vera, L., Colella, P., Gomez-Ospina, N.
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- **Basic and translational research in rare diseases in low- and middle-income countries: challenges and solutions.** *Journal of community genetics*
Rodrigues, G., Poletto, E., E Vairo, F. P., Baldo, G.
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- **Laronidase-loaded liposomes reach the brain and other hard-to-treat organs after noninvasive nasal administration.** *International journal of pharmaceuticals*
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2024: 124355
- **Clinical development of autologous genome-edited hematopoietic stem cells to treat mucopolysaccharidosis type I**
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ACADEMIC PRESS INC ELSEVIER SCIENCE.2024
- **Genome-edited hematopoietic stem cells as a curative approach for Gaucher disease type 1**
Vera, L., Gastou, M., Arozqueta-Basurto, J., Colella, P., Feng, A., Poletto, E., Gomez-Ospina, N.
ACADEMIC PRESS INC ELSEVIER SCIENCE.2024
- **Editorial: Genome editing in stem cells.** *Frontiers in genome editing*
Bayarsaikhan, D., Poletto, E.
2024; 6: 1357369
- **Pilot study of newborn screening for six lysosomal diseases in Brazil** *MOLECULAR GENETICS AND METABOLISM*

- Kubaski, F., Sousa, I., Amorim, T., Pereira, D., Silva, C., Chaves, V., Brusius-Facchin, A., Netto, A. B. O., Soares, J., Vairo, F., Poletto, E., Trometer, J., Souza, et al
2023; 140 (1-2): 107654
- **Microglia Replacement with Bone Marrow-Derived Cells after Transient Inhibition of the Colony-Stimulating Factor 1 Receptor (CSF1R) is Superior to Standard Myeloablative Conditioning in Neuropathic Lysosomal Storage Diseases**
Colella, P., Suarez-Nieto, M., Sayana, R., Poletto, E., Vera, L., Basurto, J., Gomez-Ospina, N.
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 - **Engineering Human Hematopoietic Stem and Progenitor Cells for Lineage-Specific Expression of Galactocerebrosidase Using Genome Editing**
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 - **Ex vivo gene therapy for lysosomal storage disorders: future perspectives** *EXPERT OPINION ON BIOLOGICAL THERAPY*
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 - **Diagnosis and Emerging Treatment Strategies for Mucopolysaccharidosis VII (Sly Syndrome).** *Therapeutics and clinical risk management*
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 - **Experience of the NPC Brazil Network with a Comprehensive Program for the Screening and Diagnosis of Niemann-Pick Disease Type C** *INTERNATIONAL JOURNAL OF NEONATAL SCREENING*
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 - **Biochemical diagnosis of aromatic-L-amino acid decarboxylase deficiency (AADCD) by assay of AADC activity in plasma using liquid chromatography/tandem mass spectrometry** *MOLECULAR GENETICS AND METABOLISM REPORTS*
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