Stanford

Edina Poletto

Contingent OTP 1, Pediatrics - Genetics

Publications

PUBLICATIONS

- **Improved engraftment and therapeutic efficacy by human genome-edited hematopoietic stem cells with Busulfan-based myeloablation.** *Molecular therapy. Methods & clinical development*
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- **Engineering monocyte/macrophage−specific glucocerebrosidase expression in human hematopoietic stem cells using genome editing** *Nature Communications*
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- **Newborn Intravenous Injection of Liposomal CRISPR/Cas9 Complex Has No Incidence of Off-Targets or Tumors in Mice.** *Pharmaceutics*
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- **Laronidase-loaded liposomes reach the brain and other hard-to-treat organs after noninvasive nasal administration.** *International journal of pharmaceutics*
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- **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
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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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