

Stanford

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Publications

PUBLICATIONS

- **Gene regulation in inborn errors of immunity: Implications for gene therapy design and efficacy.** *Immunological reviews*
Ghanim, H. Y., Porteus, M. H.
2024
- **Genetically Corrected RAG2-SCID Human Hematopoietic Stem Cells Restore V(D)J-Recombinase and Rescue Lymphoid Deficiency.** *Blood advances*
Pavel-Dinu, M., Gardner, C. L., Nakauchi, Y., Kawai, T., Delmonte, O. M., Palterer, B., Bosticardo, M., Pala, F., Viel, S., Malech, H. L., Ghanim, H. Y., Bode, N. M., Kurgan, et al
2023
- **Engineering a Potential Curative Treatment for Hemophilia A Using an AAV Dual Targeting Strategy**
Johnston, N., Amorin, A., Nguyen, L., Ghanim, H., Porteus, M. H.
CELL PRESS.2023: 267
- **Gibbin mesodermal regulation patterns epithelial development.** *Nature*
Collier, A., Liu, A., Torkelson, J., Pattison, J., Gaddam, S., Zhen, H., Patel, T., McCarthy, K., Ghanim, H., Oro, A. E.
2022
- **Allele-Specific Gene Editing Rescues Pathology in a Human Model of Charcot-Marie-Tooth Disease Type 2E** *FRONTIERS IN CELL AND DEVELOPMENTAL BIOLOGY*
Feliciano, C. M., Wu, K., Watry, H. L., Marley, C. E., Ramadoss, G. N., Ghanim, H. Y., Liu, A. Z., Zholuteva, L., McDevitt, T. C., Saporta, M. A., Conklin, B. R., Judge, L. M.
2021; 9: 723023
- **Blood-brain barrier dysfunction in aging induces hyperactivation of TGF beta signaling and chronic yet reversible neural dysfunction** *SCIENCE TRANSLATIONAL MEDICINE*
Senatorov, V. V., Friedman, A. R., Milikovsky, D. Z., Ofer, J., Saar-Ashkenazy, R., Charbash, A., Jahan, N., Chin, G., Mihaly, E., Lin, J. M., Ramsay, H. J., Moghbel, A., Preininger, et al
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