Cure Sanfilippo Foundation

https://curesanfilippofoundation.org/what-we-do/accelerating-research/

We read through the list of the current and past projects they have funded and found several dealing with gene therapies.

- ♦ One of the pre-clinical projects they're funding in 2023 deals with "treating mucopolysaccharidosis Type IIIC with hematopoietic stem cell gene therapy." They do not indicate which stem cells they use.
- ♦ 2020: "Preclinical drug production and dose finding for MPS IIIC gene therapy."
- ♦ 2020: "Development of gene-modified MPS IIIA & IIIB neural stem cell therapy."
- ◆ 2018: "Exploring gene-modified neural and mesenchymal stem cell therapy."

https://curesanfilippofoundation.org/what-is-sanfilippo/current-research-on-sanfilippo/therapeutic-avenues/

Cure Sanfilippo Foundation lists that they use stem-cell therapy and gene therapy. However, they do not indicate which stem-cells they use outside of Neural Stem Cells and Induced Pluripotent Stem Cells. They have also not responded to ALL's inquiry regarding if they use human embryonic stem cells or fetal tissue.

https://curesanfilippofoundation.org/2018/11/cure-sanfilippo-funds-crispr-geneediting/

As of 2018, Cure Sanfilippo Foundation funds LA BioMed's research using CRISPR gene editing, which is highly controversial in the bioethics field as it has led to scientists modifying DNA in human embryos and creating "designer babies."

Resources for Understanding CRISPR

"What is CRISPR" https://www.jp2mri.org/what-is-crispr

"Making Sense of Bioethics: Column 107: Editing Our Own Genes?"

https://www.ncbcenter.org/making-sense-of-bioethics-cms/column-107-editing-our-own-genes

"Crispr Gene Editing Can Cause Unwanted Changes in Human Embryos, Study Finds" https://www.nytimes.com/2020/10/31/health/crispr-genetics-embryos.html#:~:text=genetics%2Dembryos.html-, Crispr%20Gene%20Editing%20Can%20Cause%20Unwanted%20Changes%20in%20Human%20Embryos, cells%20to%20lose%20entire%20chromosomes.

"Hacking CRISPR: Patents, Gene Therapy & Embryos"

https://www.geneticsandsociety.org/biopolitical-times/hacking-crispr-patents-gene-therapy-embryos

"Making Sense of Bioethics: Column 174: The Foxes and the Henhouse" https://www.ncbcenter.org/making-sense-of-bioethics-cms/column-174-the-foxes-and-the-henhouse

"Inventors of CRISPR gene editing appointed to Pontifical Academy of Sciences" https://www.catholicnewsagency.com/news/248667/inventors-of-crispr-gene-editing-appointed-to-pontifical-academy-of-sciences

"House Committee Votes To Continue Ban On Genetically Modified Babies" https://www.geneticsandsociety.org/article/house-committee-votes-continue-ban-genetically-modified-babies