Publications Using Data or Biomaterials from the CLN3 Natural History Study at NIH

<https://clinicaltrials.gov/study/NCT03307304>

**2023**

Dang Do AN, Sleat DE, Campbell K, Johnson NL, Zheng H, Wassif CA, Dale RK, Porter FD. Cerebrospinal Fluid Protein Biomarker Discovery in CLN3. *J Proteome Res*. 2023 Jul 7;22(7):2493-2508. doi: 10.1021/acs.jproteome.3c00199.

Dang Do AN, Baker EH, Farmer CA, Soldatos AG, Thurm AE, Porter FD. Brain proton MR spectroscopy measurements in CLN3 disease. *Mol Genet Metab*. 2023 May;139(1):107584. doi: 10.1016/j.ymgme.2023.107584.

Luckett A, Yousef M, Tifft C, Jenkins K, Smith A, Munoz A, Quimby R, Porter FD, Dang Do AN. Anesthesia outcomes in lysosomal disorders: CLN3 and GM1 gangliosidosis. *Am J Med Genet A*. 2023 Mar;191(3):711-717. doi: 10.1002/ajmg.a.63064.

**2022**

Laqtom NN, Dong W, Medoh UN, Cangelosi AL, Dharamdasani V, Chan SH, Kunchok T, Lewis CA, Heinze I, Tang R, Grimm C, Dang Do AN, Porter FD, Ori A, Sabatini DM, Abu-Remaileh M. CLN3 is required for the clearance of glycerophosphodiesters from lysosomes. *Nature*. 2022 Sep;609(7929):1005-1011. doi: 10.1038/s41586-022-05221-y.

Brudvig JJ, Swier VJ, Johnson TB, Cain JC, Pratt M, Rechtzigel M, Leppert H, Dang Do AN, Porter FD, Weimer JM. Glycerophosphoinositol is Elevated in Blood Samples From CLN3 Δex7-8 pigs, Cln3 Δex7-8 Mice, and CLN3-Affected Individuals. *Biomark Insights*. 2022 Jun 19;17:11772719221107765. doi: 10.1177/11772719221107765.

Dang Do AN, Thurm AE, Farmer CA, Soldatos AG, Chlebowski CE, O'Reilly JK, Porter FD. Use of the Vineland-3, a measure of adaptive functioning, in CLN3. *Am J Med Genet A*. 2022 Apr;188(4):1056-1064. doi: 10.1002/ajmg.a.62607

**2021**

Abdennadher M, Inati S, Soldatos A, Norato G, Baker EH, Thurm A, Bartolini L, Masvekar R, Theodore W, Bielekova B, Porter FD, Dang Do AN. Seizure phenotype in CLN3 disease and its relation to other neurologic outcome measures. *J Inherit Metab* Dis. 2021 Jul;44(4):1013-1020. doi: 10.1002/jimd.12366.

Hildenbrand H, Wickstrom J, Parks R, Zampieri C, Nguyen TT, Thurm A, Jenkins K, Alter KE, Matsubara J, Hammond D, Soldatos A, Porter FD, Dang Do AN. Characterizing upper limb function in the context of activities of daily living in CLN3 disease. *Am J Med Genet A*. 2021 May;185(5):1399-1413. doi: 10.1002/ajmg.a.62114.

Dang Do AN, Sinaii N, Masvekar RR, Baker EH, Thurm AE, Soldatos AG, Bianconi SE, Bielekova B, Porter FD. Neurofilament light chain levels correlate with clinical measures in CLN3 disease. *Genet Med*. 2021 Apr;23(4):751-757. doi: 10.1038/s41436-020-01035-3.

**2020**

Centa JL, Jodelka FM, Hinrich AJ, Johnson TB, Ochaba J, Jackson M, Duelli DM, Weimer JM, Rigo F, Hastings ML. Therapeutic efficacy of antisense oligonucleotides in mouse models of CLN3 Batten disease. *Nat Med*. 2020 Sep;26(9):1444-1451. doi: 10.1038/s41591-020-0986-1.