Supporting Information

Synthesis of the Anionic Polyrotaxane Hydroxypropyl-β-cyclodextrin:Poly(decamethylene phosphate) and Evaluation of its Cholesterol Efflux Potential in Niemann-Pick C1 Cells

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Figure S1A: Distribution of molar mass of poly(decamethylene phosphate) 1 in 66 mM phosphate buffer pH 10 with 5% ACN, calibration with pullulan.
Synthesis of FTIC-tagged CD 7

FTIC-tagged CD 7 was prepared according to Kräuter et al.\(^1\) starting from Mono-[6-deoxy-6-(2-aminoethylsulfanyl)]-\(\beta\)-CD which has been synthesized before according to Steffen et al.\(^2\)

![Chemical structure](image)

Mono-[6-deoxy-6-(2-aminoethylsulfanyl)]-\(\beta\)-CD (1.00 g, 8.1\(\times\)10\(^{-4}\) mol, 1.0 eq.) were dissolved in 18 mL anhydrous pyridine. A solution of fluorescein-5-isothiocyanate (Isomer I) (634 mg, 1.62\(\times\)10\(^{-3}\) mol, 2.0 eq.) in 18 mL pyridine was added over 20 min under \(\text{N}_2\). The reaction mixture was heated under stirring to 60 °C. After 3 h, the solvent was evaporated in vacuum at 60 °C. The residue was dissolved in 15 mL N, N-dimethylacetamide and precipitated in 200 mL cold acetone. The mixture was stirred overnight, then the precipitate was collected by centrifugation. The residue was redissolved in 100 ml water and stirred overnight. The crude product was purified by ultrafiltration using a 500 Da regenerate cellulose membrane against a 0.01 M \(\text{NH}_4\)OH solution and \(\text{H}_2\)O in sequence before freeze drying. A yellow, fluffy solid (816 mg, 5.15\(\times\)10\(^{-4}\) mol, 64 %) was recovered. \(^1\)H-NMR: \(\delta/\text{ppm}\) (DMSO-d\(_6\)): 9.24 (bs, 1H, COOH), 8.11 (bs, 1H, Ar-OH), 7.54-6.97 (m, 5H, H-11/12/15, NH), 6.83-6.46 (m, 6H, H-18/19/21/24/26/27), 6.36-5.25 (bs, 20 H, OH), 5.09-4.71 (m, 7H, H-1/1’), 4.10-3.45 (m, 26 H, H-3/3’/5/5’/6), 3.44-3.21 (m, 14H, H-2/2’/4/4’), 3.15-2.91 (m, 3H, H-6’a/H-8), 2.91-2.64 (m, 3H, H-6’b/H-7).


Figure S 3: NPC1-deficient cells treated with 25µM CD-equivalent FITC tagged polyrotaxane 6a in Opti-MEM media. (24 h 6a treatment). We proposed extra cellular matrix association as the fluorescent signal appears in the characteristic shape of fibronectin structure.