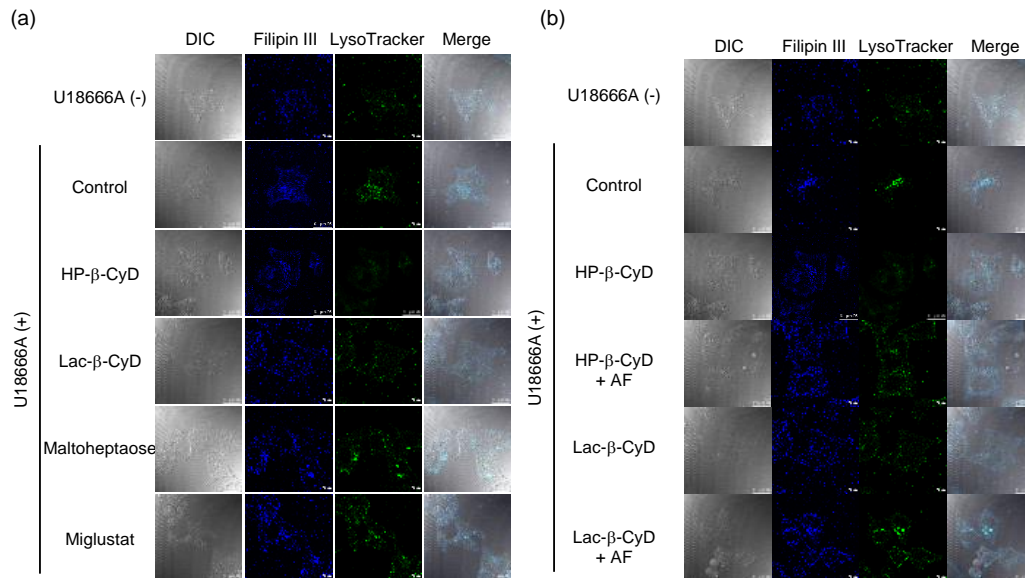


## Supplementary material

# In vivo Efficacy and Safety Evaluation of Lactosyl- $\beta$ -cyclodextrin as a Therapeutic Agent for Hepatomegaly in Niemann-Pick Type C Disease



**Figure S1.** Free cholesterol-lowering effect of Lac- $\beta$ -CyD in NPC-like liver cells. U18666A-treated HepG2 cells were incubated with culture medium containing Lac- $\beta$ -CyD, HP- $\beta$ -CyD, miglustat, or maltoheptaose (1 mM, 24 h) at 37°C, and fixed in 4% PFA. (a) Fluorescence of cells stained with Filipin III (cholesterol) and LysoTracker® Green DND-26 (endolysosomes). Effect of AF on the Lac- $\beta$ -CyD free cholesterol lowering effect in endolysosomes of U18666A-treated HepG2 cells. Cells were exposed to culture medium containing Lac- $\beta$ -CyD or HP- $\beta$ -CyD (1 mM)  $\pm$  AF (1.0 mg/mL) for 24 h at 37 °C. (b) Fluorescence of cells stained with Filipin III (cholesterol) and LysoTracker® Green DND-26 (endolysosomes).