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Supplemental Information

Toward Personalized Gene Therapy:

Characterizing the Host Genetic Control of

Lentiviral-Vector-Mediated Hepatic Gene Delivery

Thipparat Suwanmanee, Martin T. Ferris, Peirong Hu, Tong Gui, Stephanie A. Montgomery, Fernando Pardo-Manuel de Villena, and Tal Kafri

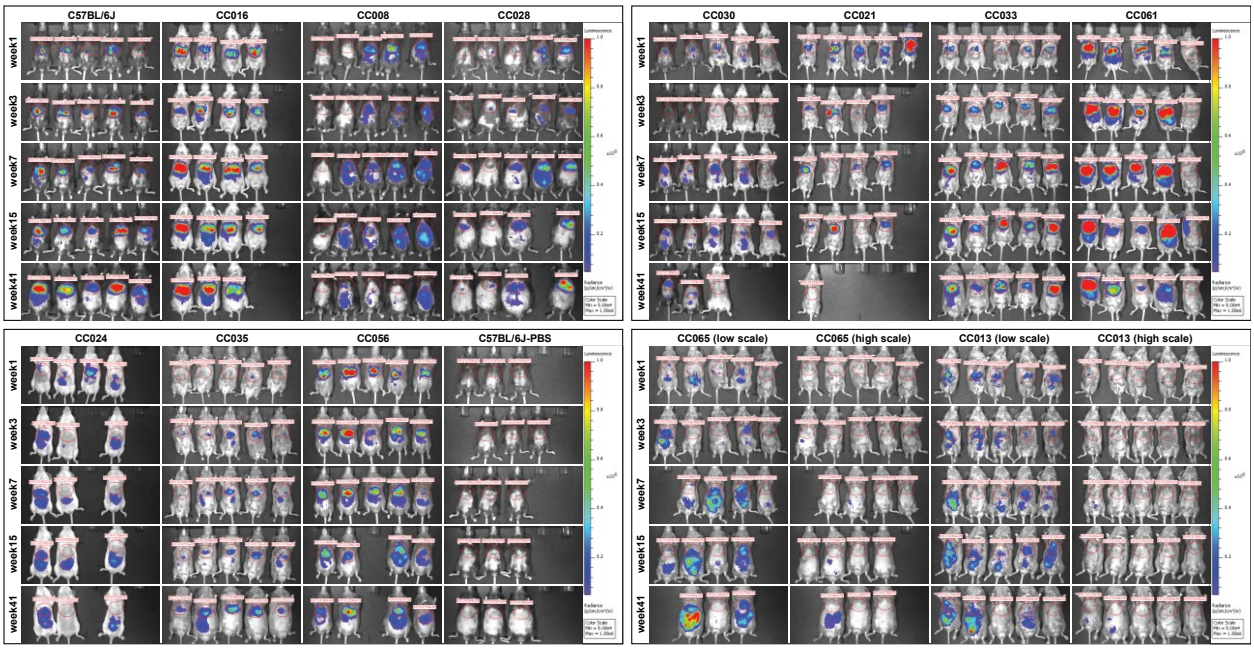

Figure S1. ***In vivo*** **Luciferase Expression**
Live imaging displaying luciferase activity was taken using IVIS Lumina optical imaging system at 1, 3, 7, 15, and 41 weeks after lentiviral vector injection. Images were displayed in luminescence surface radiance (p/sec/cm^2/sr) with the color scale set at 5x10^4-1x10^6 as minimum and maximum, respectively, with an exception in lower right panel where levels of luciferase expression in CC065 and CC013 were low and therefore low scale 1x10^4-1x10^5 was used alongside with high scale 5x10^4-1x10^6. Liver-specific luciferase activity in the marked (red ellipse) regions of interest (ROI) was quantified. Data was analyzed using Living Image Software and reported in photon/sec. Note that in the image of CC021 week 7, animals #1 and #2 were switched.

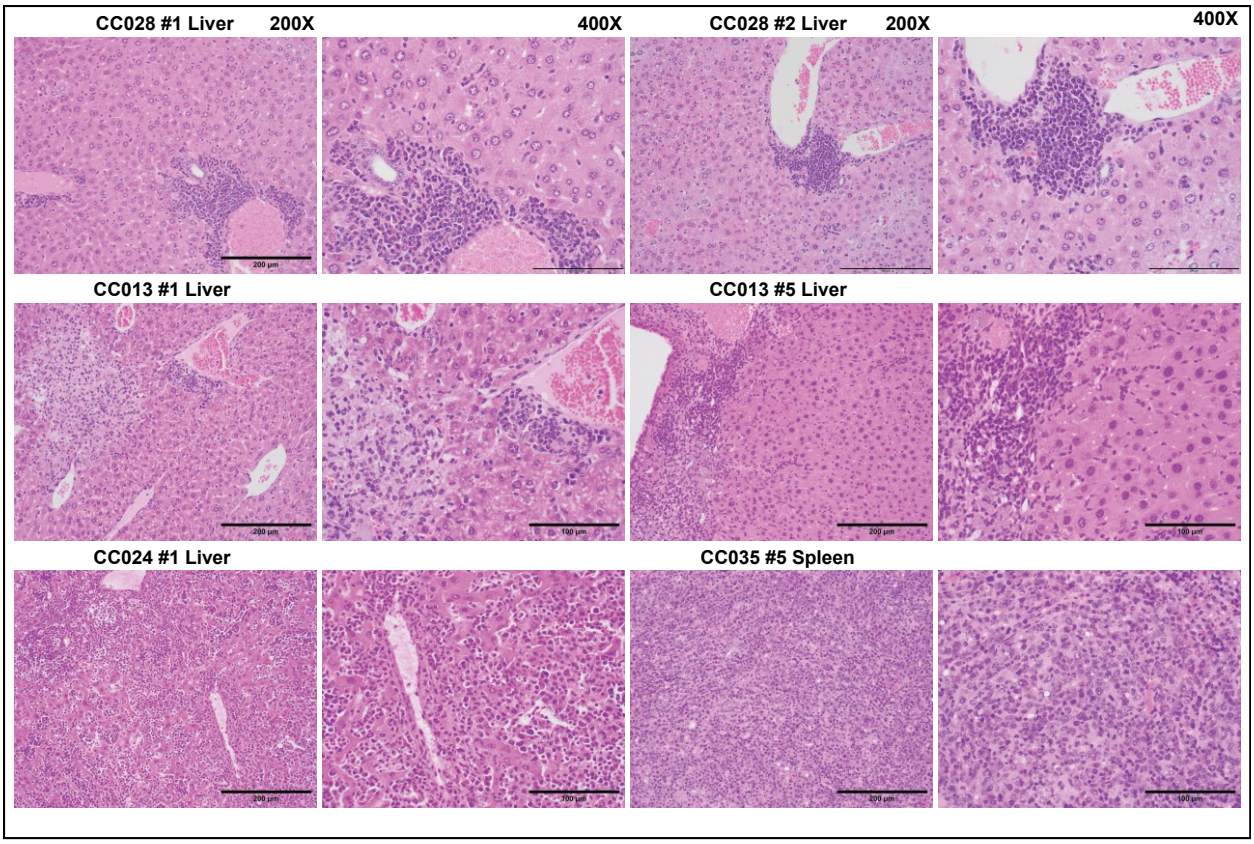

Figure S2. Pathology Assessment of Liver and Spleen
H&E stained sections of liver and spleen tissues of six animals from four strains (CC028/GeniUnc, liver tissues from animals #1, and #2; CC013/GeniUnc, liver tissues from animals #1 and #5; CC024/GeniUnc, liver tissue from animal #1; CC035/Unc, spleen tissue from animal #5). Microscopic histopathological analysis revealed: CC028/GeniUnc#1, mononuclear chronic periportal infiltrate in the liver tissues; CC028/GeniUnc#2, mononuclear chronic periportal infiltrate in the liver tissues; CC013/GeniUnc#1, mononuclear chronic periportal infiltrate and granuloma with central hepatocellular death & area with eosinophilic droplets; CC013/GeniUnc#5, mononuclear chronic periportal infiltrate and presumed granuloma with central hepatocellular death; CC024/GeniUnc#1, mononuclear neoplasia—likely lymphoma or leukemia; CC035/Unc#5, likely mononuclear neoplasia (lymphoma) in spleen tissue. Sectioned images were taken using 200X and 400X magnifications, and displayed with scale bar in micron.

Table S1. Mortality through Experiment

Mouse Strain	Mice Dying (#/total)	Last Timepoint (number)
CC016/GeniUnc	1/4	Week015 (1)
CC028/GeniUnc	1/5	Week007 (1)
CC021/Unc	4/5	Week001 (1), Week015 (3)
CC030/GeniUnc	2/5	Week015 (2)
CC065/Unc	2/5	Week003 (1), Week015 (1)
CC024/GeniUnc	1/4	Week001 (1)
CC056/GeniUnc	1/5	Week007 (1)

Table S2. Pathological Examination of Abnormal Tissues

Animal ID	Pathology Assessment
CC013/GeniUnc_1	Liver: mononuclear chronic periportal infiltrate and granuloma with central hepatocellular death & area with eosinophilic droplets.
CC013/GeniUnc_5[a]	Liver: mononuclear chronic periportal infiltrate and presumed granuloma with central hepatocellular death.
CC024/GeniUnc_1	Liver: mononuclear neoplasia—likely lymphoma or leukemia.
CC028/GeniUnc_1[a]	Liver: mononuclear chronic periportal infiltrate in the liver tissues.
CC028/GeniUnc_2[a]	Liver: mononuclear chronic periportal infiltrate in the liver tissues.
CC035/Unc_5	Spleen: likely mononuclear neoplasia (lymphoma) in spleen tissue.

[a]Common in aged mice.