

YMTHE, Volume 25

## **Supplemental Information**

**Survival Advantage of Both Human Hepatocyte**

**Xenografts and Genome-Edited Hepatocytes**

**for Treatment of  $\alpha$ -1 Antitrypsin Deficiency**

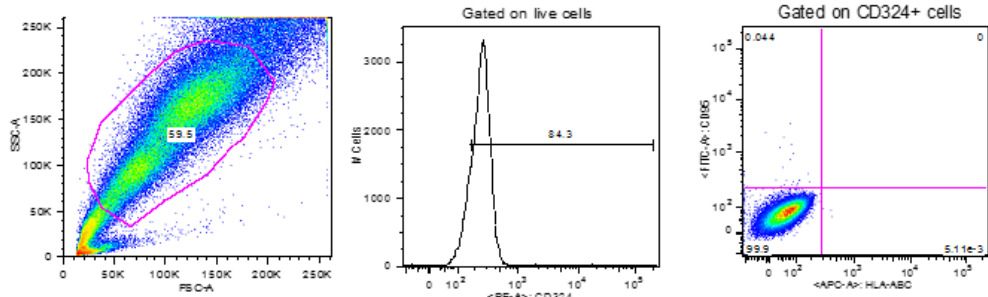
**Florie Borel, Qiushi Tang, Gwladys Gernoux, Cynthia Greer, Ziqiong Wang, Adi Barzel, Mark A. Kay, Leonard D. Shultz, Dale L. Greiner, Terence R. Flotte, Michael A. Brehm, and Christian Mueller**

**Table S1. Wild-type hepatocytes expand faster than hepatocytes expressing Z-AAT (PiZ).**

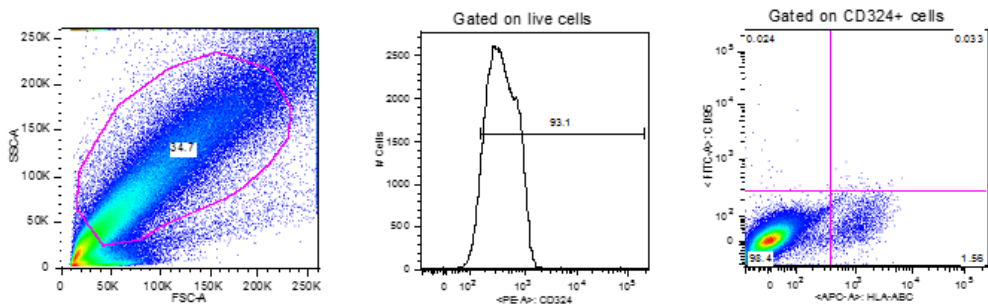
Genotype of host mouse	Genotype of implanted hepatocytes	Treatment	Animal #	% GFP (ddPCR)		% GFP (IHC)	
NSG-PiZ	PiZ-GFP	S+PHx	832	9.8	7.7	14	11.3
			848	2.7		12	
			849	10.7		7	
		S	833	4.5	5.4	16	11.7
			834	8.5		11	
			835	3.1		8	
	WT-GFP	S+PHx	838	42.2	31.9	43	44.5
			840	21.6		46	
		S	841	34.7	39.3	50	48.2
			842	39.4		47	
			843	45.3		47	
			844	37.7		48	
B6-GFP	None	None	NA	94.8	97.5	NA	NA
			NA	100.2		NA	

NSG: NOD-*scid*-gamma, B6: C57BL/6, PiZ: protease inhibitor Z, GFP: green fluorescent protein, WT: wild-type, S: splenic injection only, S+PHx: splenic injection and partial hepatectomy, ddPCR: droplet digital polymerase chain reaction, IHC: immunohistochemistry, NA: not available.

### Mouse Liver



### Humanized Mouse Liver



**Figure S1. Human specificity of anti-HLA antibody.** Mouse liver (upper panel) and humanized mouse liver engrafted with human hepatocytes (lower panel) were dissociated and stained with anti-CD324, anti-CD95 and anti-HLA-ABC antibodies. Hepatocytes were gated as CD324<sup>+</sup> cells and human hepatocytes as CD95<sup>-</sup>HLA-ABC<sup>+</sup> cells within the CD324<sup>+</sup> gate. Human hepatocytes (CD324<sup>+</sup>CD95<sup>-</sup>HLA-ABC<sup>+</sup>) are detected only in humanized mouse liver confirming the specificity of the anti-HLA-ABC antibody for human cells.