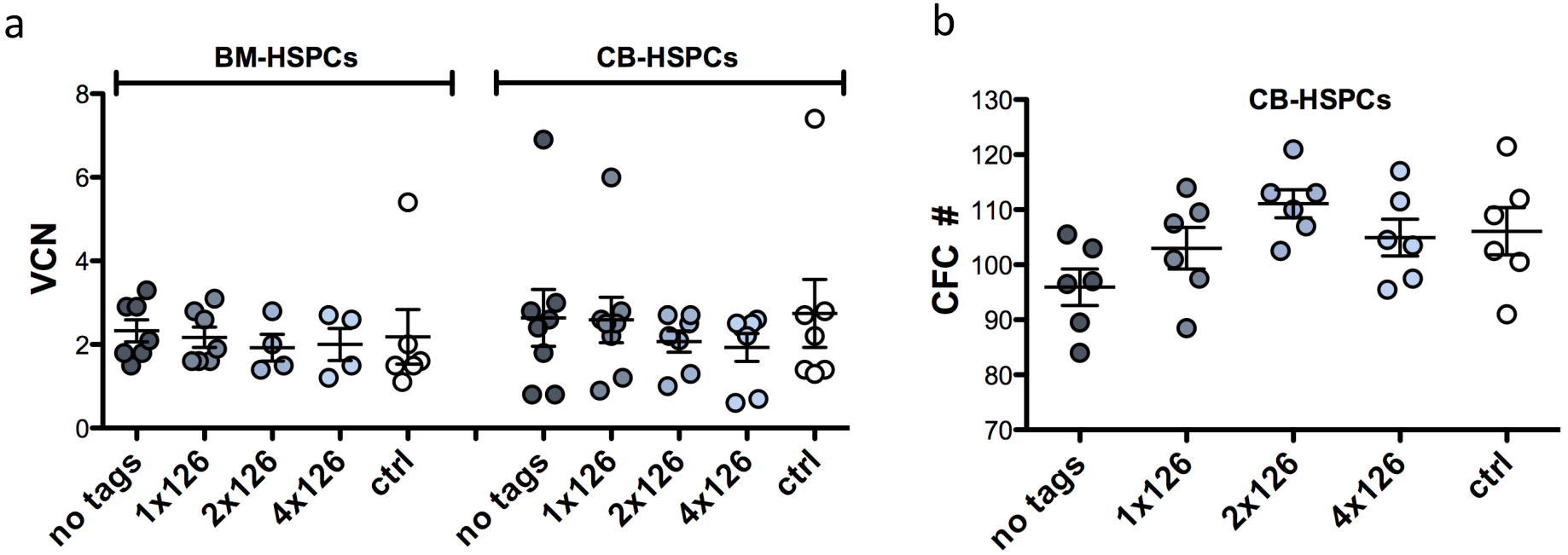
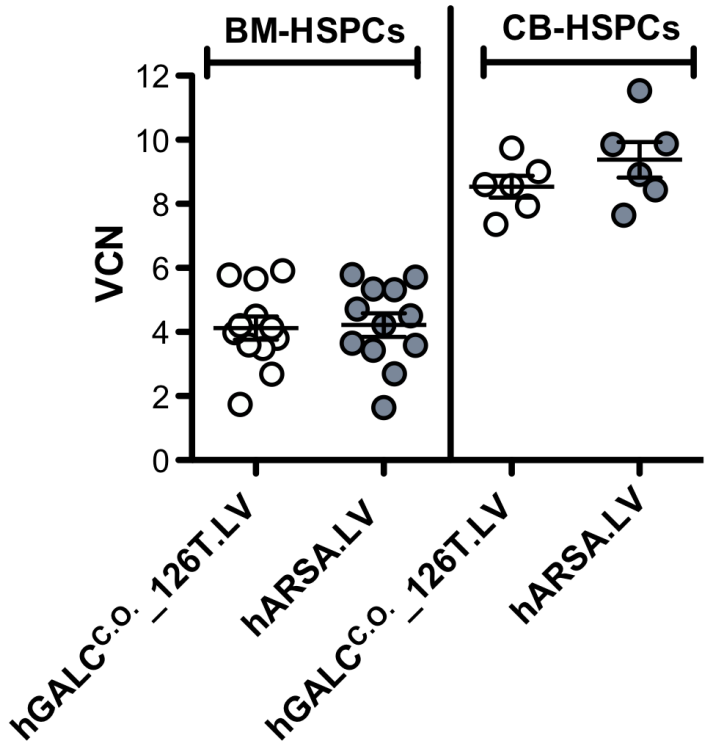
Supplementary Figure S1, Ungari et al. 2015



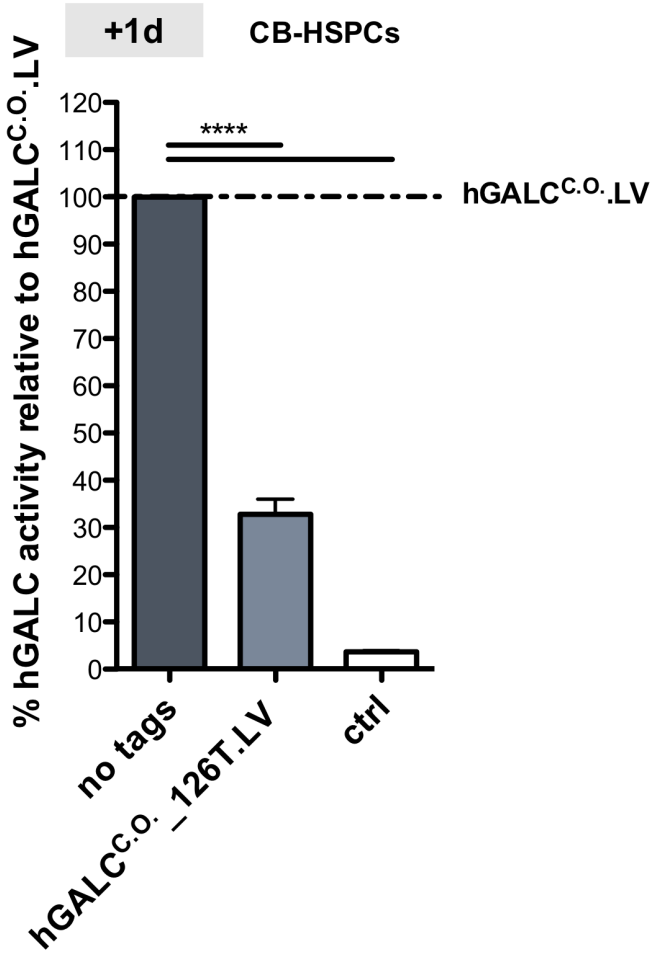
Supplementary Figure S1. VCN evaluation in BM- and CB-derived hHSPCs and clonogenic potential of BM-derived HSPCs. (a) VCN was measured on the *in vitro* culture progeny of BM-derived HSPCs (n≥4) and CB-derived HSPCs (n≥6) upon transduction with hGALC.LV (no tags), hGALC_126T.LV (1x126T), hGALC_2x126T.LV (2x126T), hGALC_4x126T.LV (4x126T) or hIDUA.LV (ctrl). **(b)** The number (#) of colonies derived from transduced CB-derived HSPCs (n=6) is reported. Average values ± SEM are reported.

Supplementary Figure S2, Ungari et al. 2015



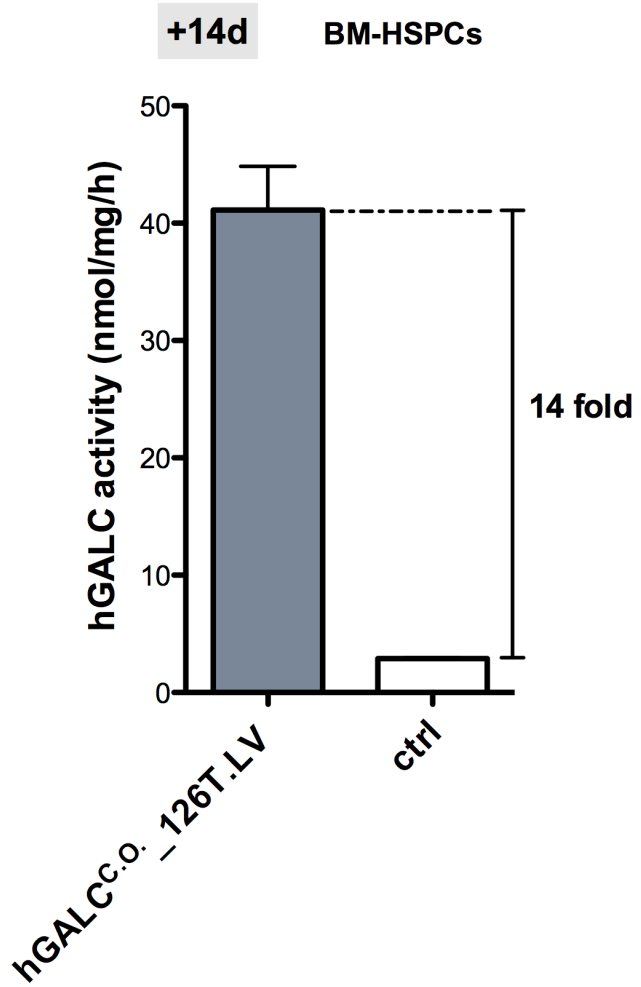
Supplementary Figure S2. VCN evaluation in BM- and CB-derived hHSPCs. VCN was measured on the *in vitro* culture progeny of BM-derived HSPCs (n=12 replicates coming from 6 donors) transduced either with hGALC $^{c.o.}$_ 126T.LV or hARSA.LV and CB-derived HSPCs (n=6 donors) transduced with either hGALC $^{c.o.}$_ LV or hGALC$^{c.o.}$_ 126T.LV or hARSA.LV. Average values ± SEM are reported.

Supplementary Figure S3, Ungari et al. 2015



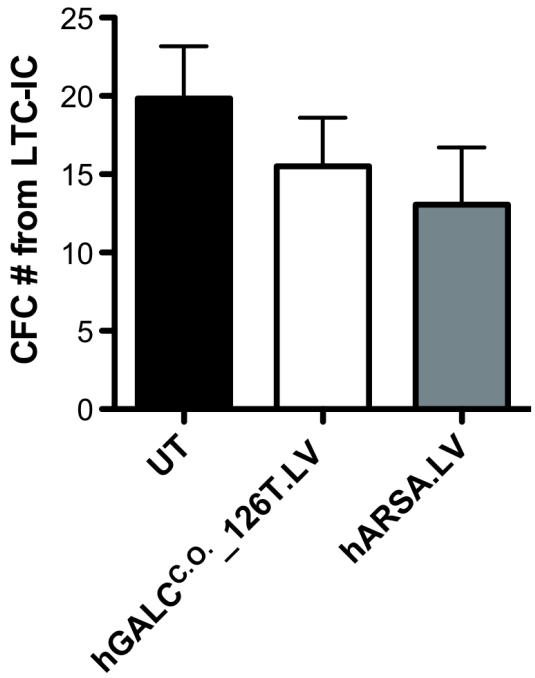
Supplementary Figure S3. Short-term hGALC activity upon transgene codon optimization. hGALC activity measured in CB-HSPCs (n=6 donors) upon transduction with hGALC$^{C.O.}$.LV, hGALC$^{C.O.}$_126T.LV or hARSA.LV at +1d is reported. Values are expressed as percentage relative to the activity achieved upon hGALC$^{C.O.}$.LV transduction (dotted line=100%) (mean ± SEM).

Supplementary Figure S4, Ungari et al. 2015



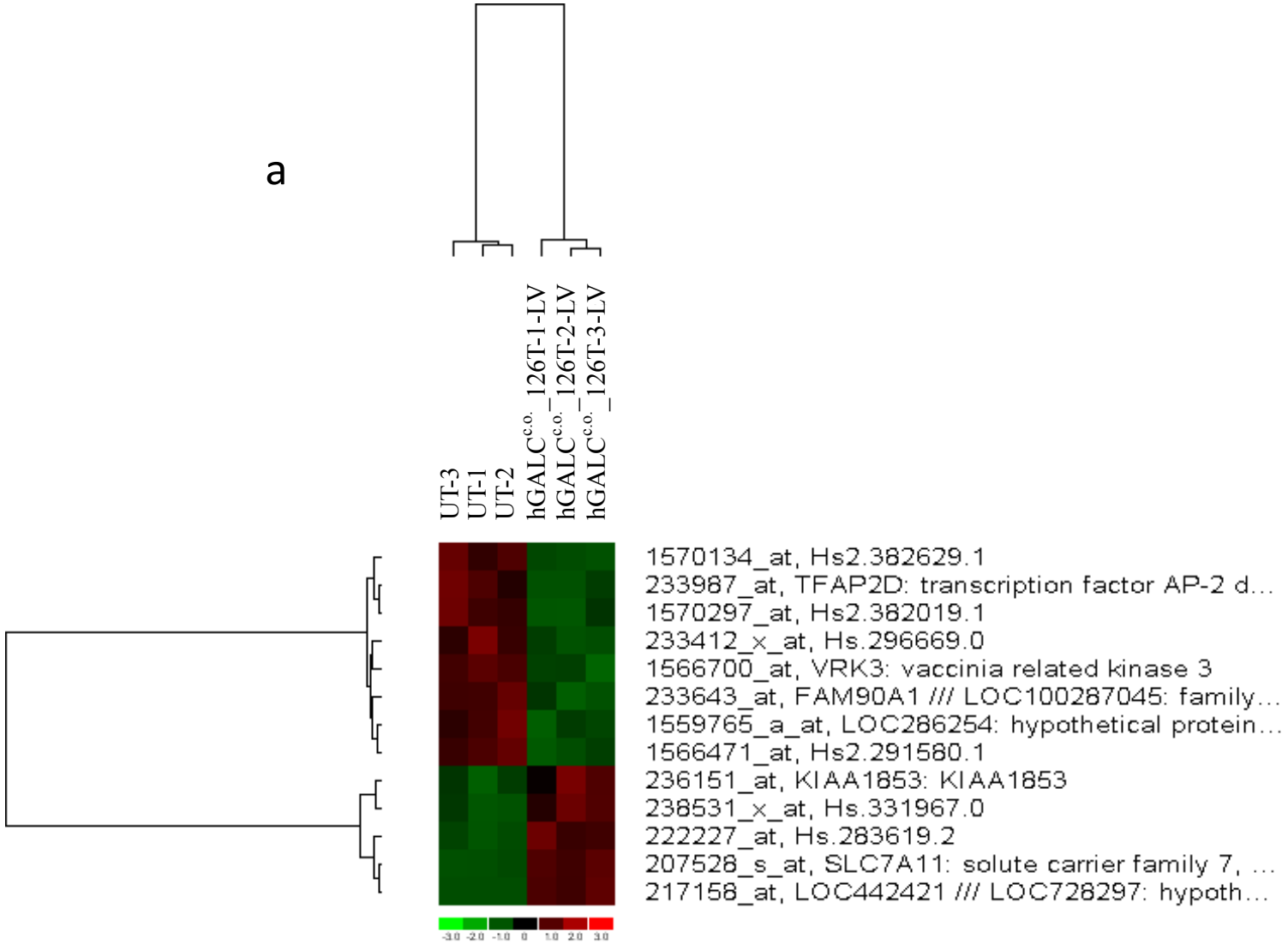
Supplementary Figure S4. Long-term hGALC activity upon transgene codon optimization. hGALC activity measured in BM-HSPCs (n=6 donors) upon transduction with hGALC$^{C.O.}$_126T.LV (1x126T) or hARSA.LV (ctrl) at +14 days is reported. Values are expressed as nmol/mg/h.

Supplementary Figure S5, Ungari et al. 2015



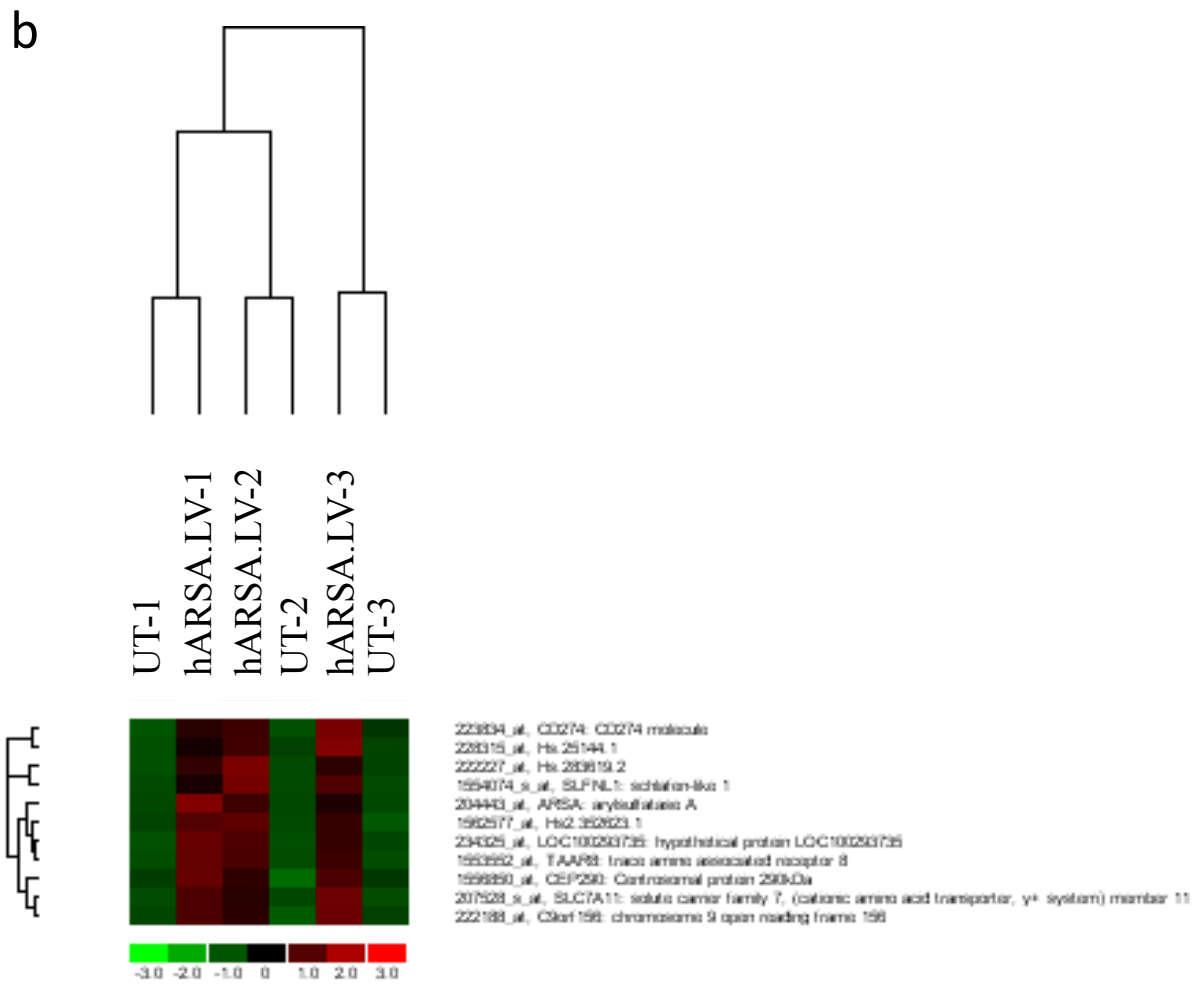
Supplementary Figure S5. Clonogenic potential of long-term BM-derived HSPCs upon hGALC$^{c.o.}$_ 126T.LV trasduction. BM-derived HSPCs (n=9 replicates coming from 3 donors for transduced cells and n=3 replicates coming from 3 donors for UT cells) were transduced with hGALC$^{c.o.}$_1xmiR126T.LV or hARSA.LV and kept in culture on a feeder cell layer for 5 weeks and then were plated for the clonogenic assay. The number (#) of CFC are reported (mean±SEM).

Supplementary Figure S6, Ungari et al. 2015



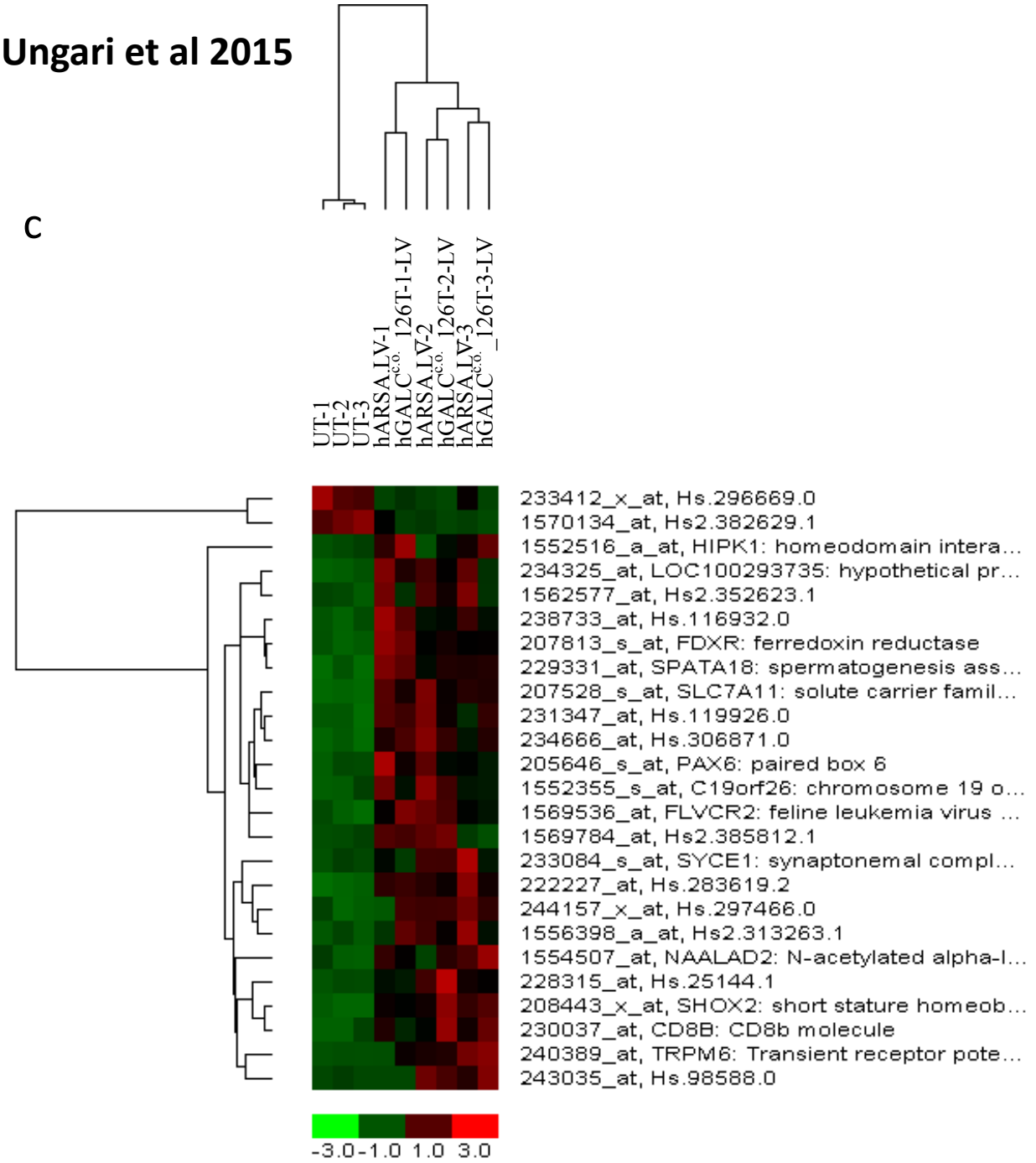
Supplementary Figure S6. Gene expression analysis on BM-derived HSPCs upon hGALC$^{c.o.}$_126T.LV trasduction. Supervised Affymetrix analysis of hGALC$^{c.o.}$_126T.LV (**a**), hARSA.LV (**b**), LV-transduced (**c**) and untransduced (UT) HSPCs. Comparison of the expression pattern identifies a set of deregulated genes according to fold change >2 and a p value of <0.05.

Supplementary Figure S6, Ungari et al. 2015

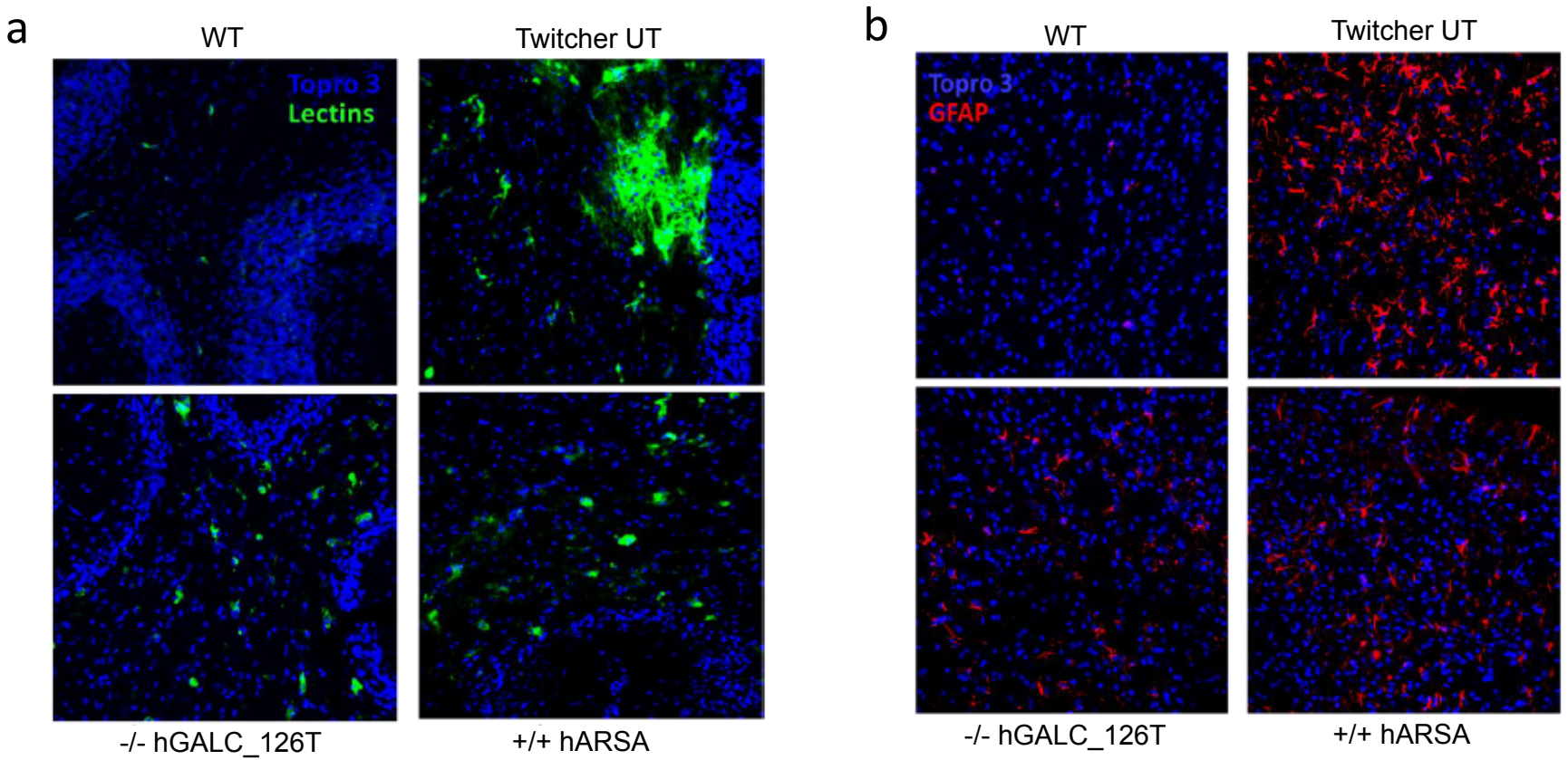


Supplementary Figure S6, Ungari et al 2015

C



Supplementary Figure S7, Ungari et al. 2015



Supplementary Figure S7. Evaluation of storages accumulation and astrogliosis. Representative pictures of lectin staining in the cerebellum (a) and GFAP staining in the pons (b) of gene therapy treated mice and controls are reported.

Supplementary Table S1, Ungari et al. 2015

	hGALC$^{c.o.}$_126T.LV	hARSA.LV
Bone Marrow	**6.87±3.22**	**4.66±2.61**
Brain	**0.14±0.12**	**0.02±0.01**
Spinal Cord	**0.79±0.62**	**0.19±0.20**
Liver	**0.05±0.02**	**0.03±0.01**

Supplementary Table S1. VCN measured in indicated tissues of -/- hGALC_126T (n≥6) and +/+ hARSA (n≥3) mice are reported (mean±SD).