Supplemental Information

Recapitulating and correcting Marfan syndrome in a cellular model

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Table S1. Primers used in this study

Primer Name	**Sequence**
For detecting FBN1 mutation (Fig. 1E)	
mut-HphI-F	AATGTCAGCTTTTCTGCAA
mut-HphI-R	CCAGGGAAGCAACACAAACT
For verifying genome targeting (Fig. 2)	
F1	CCTATGCAATCGCAGAAACA
R1	GCTGCATATTTCTCCCTGTG
F2	TGCTCCTGCCGAGAAAGTAT
R2	TGGCATTCCAAAAGATAGCA
F3	GAACAGCCCAGGCTCTTTTA
R3	TGCAGCCCAATTCCGATCAT
F4	CTTCTGAGGCGGAAAGAACCA
R4	GAACCACAGCATGGGTTTCT
For conducting RT-(q)PCR (Figs. 4C and 6D)	
ACTA2-F	AGAATCCTGTGAAGCAGCTC
ACTA2-R	CTTTTCCATGTCGTCCCAGT
VE-CAD-F	ACTGGGGACCCAGGTTTAAG
VE-CAD-R	TGGAGTTTGCTATCCCAAGG
GAPDH-F	ACCACAGTCCATGCCATCAC
GAPDH-R	TCCACCACCCTGTTGCTGTA
MYH11-F	GACCCAGATGGAGGAGATGA
MYH11-R	GTAGCTGCTTGATGGCTTCC
SMMHC-F	GGAGGATGAGATCCTGGTCA

SMMHC-R	TTAGCCGCACTTCCAGTTCT
SMTN-F	GCTGAGGAGCTGATGACTAT
SMTN-R	CTCCTTCTCCAGCTTCTCAA
IL8-F	CAGTTTTGCCAAGGAGTGCT
IL8-R	ACTTCTCCACAACCCTCTGC
IL1B-F	GGAGAATGACCTGAGCACCT
IL1B-R	GGAGGTGGAGAGCTTTCAGT
COL12A1-F	ATCTCTGTTATACGCCGGGG
COL12A1-R	TGCAATTCCCTCTCTGCAGA
COL8A2-F	AGCACTCTTCCCTTTCTCCC
COL8A2-R	CCCACAGATGAACCCCTCTT
For verifying the transgene silencing (Fig S1)	
F1	AGTGAGAGGCAACCTGGAGA
R1	CACCTGCAAGTTTCAGCAAA
F2	TTTGCTGAAACTTGCAGGTG
R2	GCAGAGCGTCGCTAGCCAT
F3	ACCAGCTCGCAGACCTACAT
R3	ACATCCCCTGCTTGTTTCAA
F4	TTGAAACAAGCAGGGGATGT
R4	CAGCAGCTCGAATTTCTTCC
F5	GACAGGGGGAGGGGAGGAGCTAGG
R5	CTTCCCTCCAACCAGTTGCCCCAAAC
F6	GGGAAATGGGAGGGGTGCAAAAGAGG
R6	TTGCGTGAGTGTGGATGGGATTGGTG
For T7E1 assay to validate the site-specific cleavage in exon 24 of *FBN1* gene	
Forward	AGTCCATGCTGGGATGATCAAG
Reverse	CATGCACGCATCGGTGATAG
For confirmation of the FBN1 knockout	
P1-Forward	CGTGCACCCTATGCCAAGTT
P1-Reverse	GCATTCCTCAGTACCCCAGG
P2-Forward	TGAAGTGTTCCCAGGAGTGTG
P2-Reverse	AATCAAGAGCAAAGCCGCTG

Figure legend

Figure S1. Validation of MFS- and mcMFS-iPSCs. (a) RT-PCR analysis to determine the silencing of the transgenes using primers as shown in the schematic. (b) Pluritest. *Another MFS-iPSC line, different from Figure 1b. (c) Immunocytochemistry for pluripotency markers. Scale bars, 100 μM.

Figure S2. Analysis of the microarray data on MFS- and mcMFS-SMCs. (a-b) Scatter plots for comparison of probe intensities between the different samples to analyze the overall inter-sample variance. (c) A volcano plot demonstrating numbers of differentially expressed genes between the two groups. (d-f) Comparison between the two groups for transcript of FBN1 (d), collagens (e), and inflammation-related genes (f).

Figure S3. Characterization of ENVY-C1 hESCs that had *FBN1* knockout in both alleles. (a) Immunostaining for pluripotency markers in ENVY-C1 hESCs. Scale bars, 100 μM. (b) Schematic and analysis for detecting the *FBN1* transcripts near the targeted sites in ENVY-C1 hESCs using RT-PCR with primers as indicated.

Figure S1 Park et al.

Top

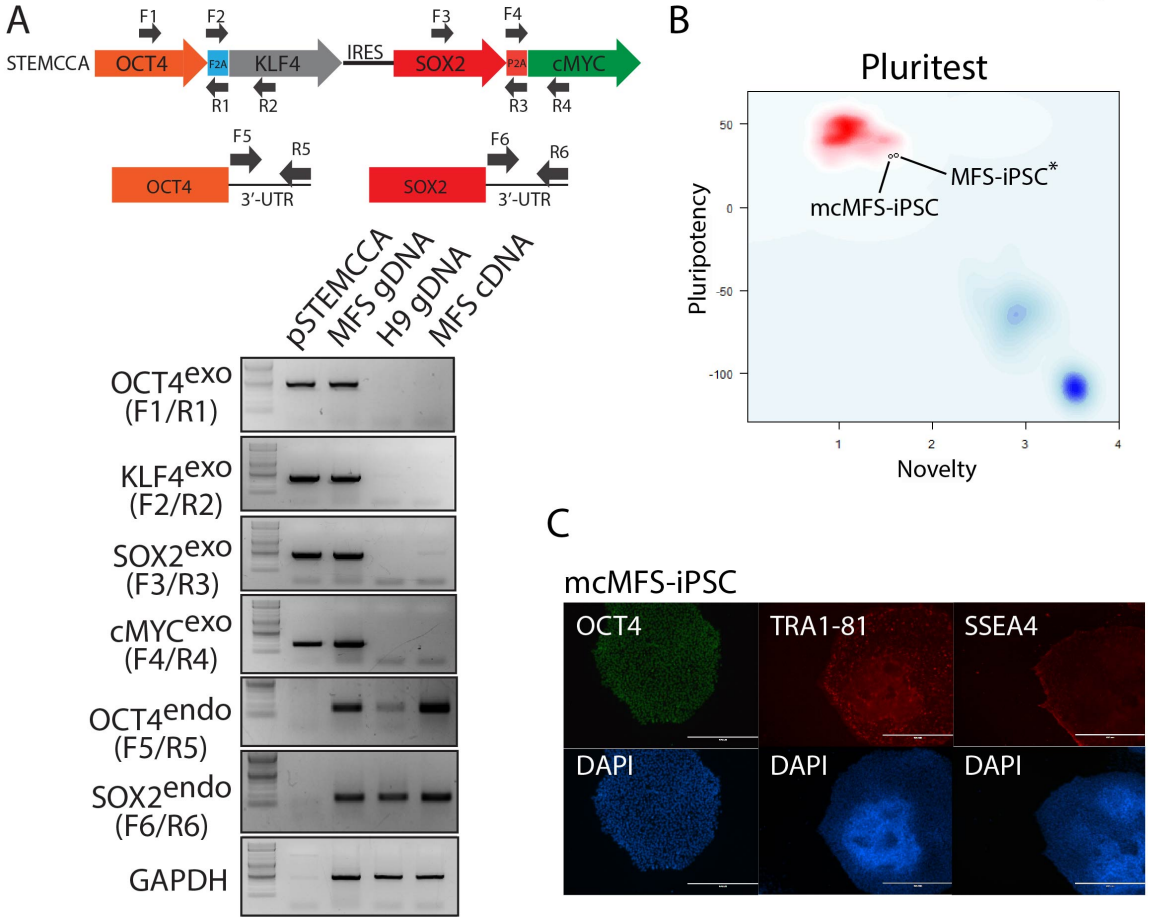

Figure S2 Park et al.

Top

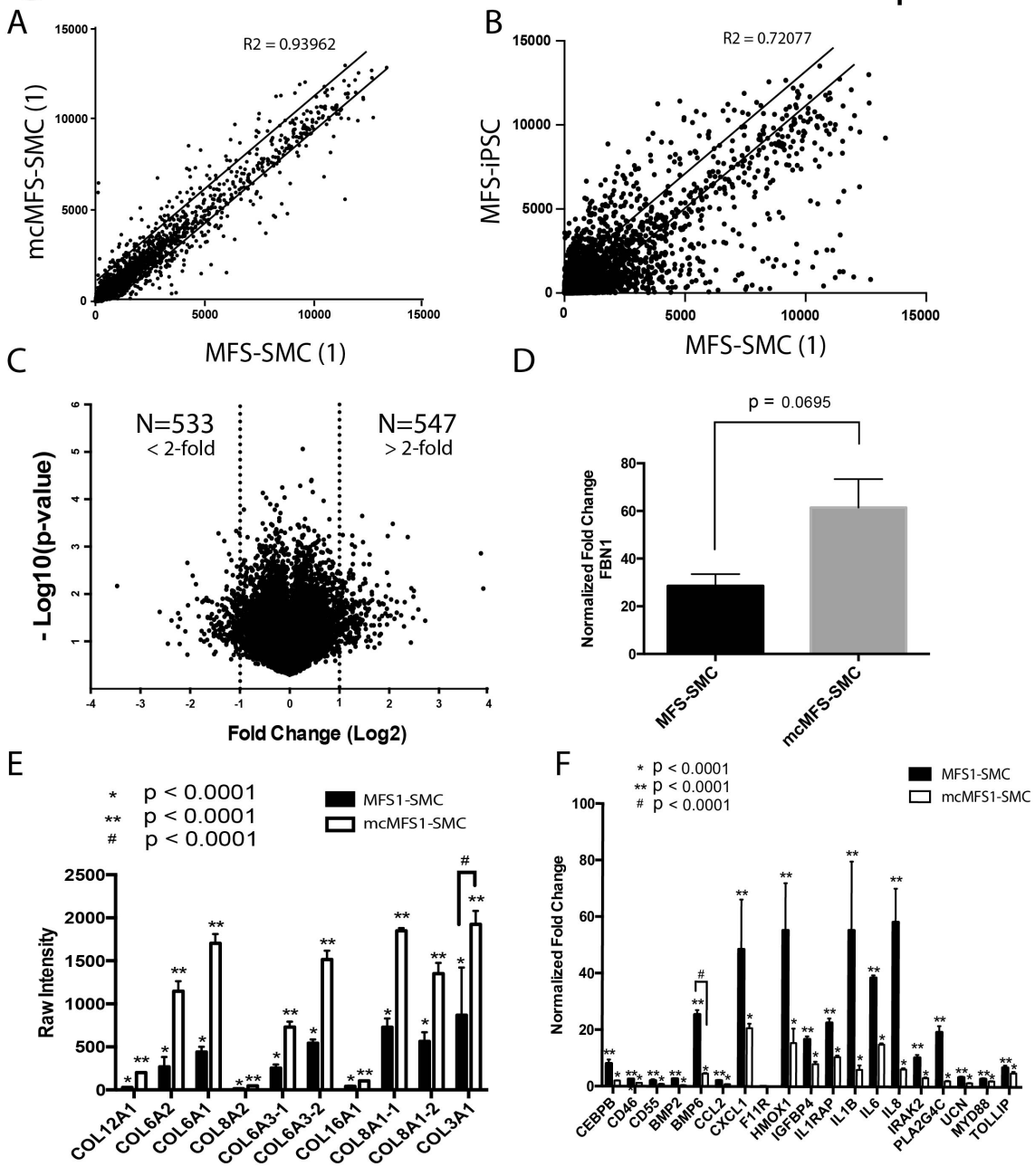

Figure S3 Park et al. Top