

FIG. 1

String Analysis of Genes Identified in FXN Replacement Signature

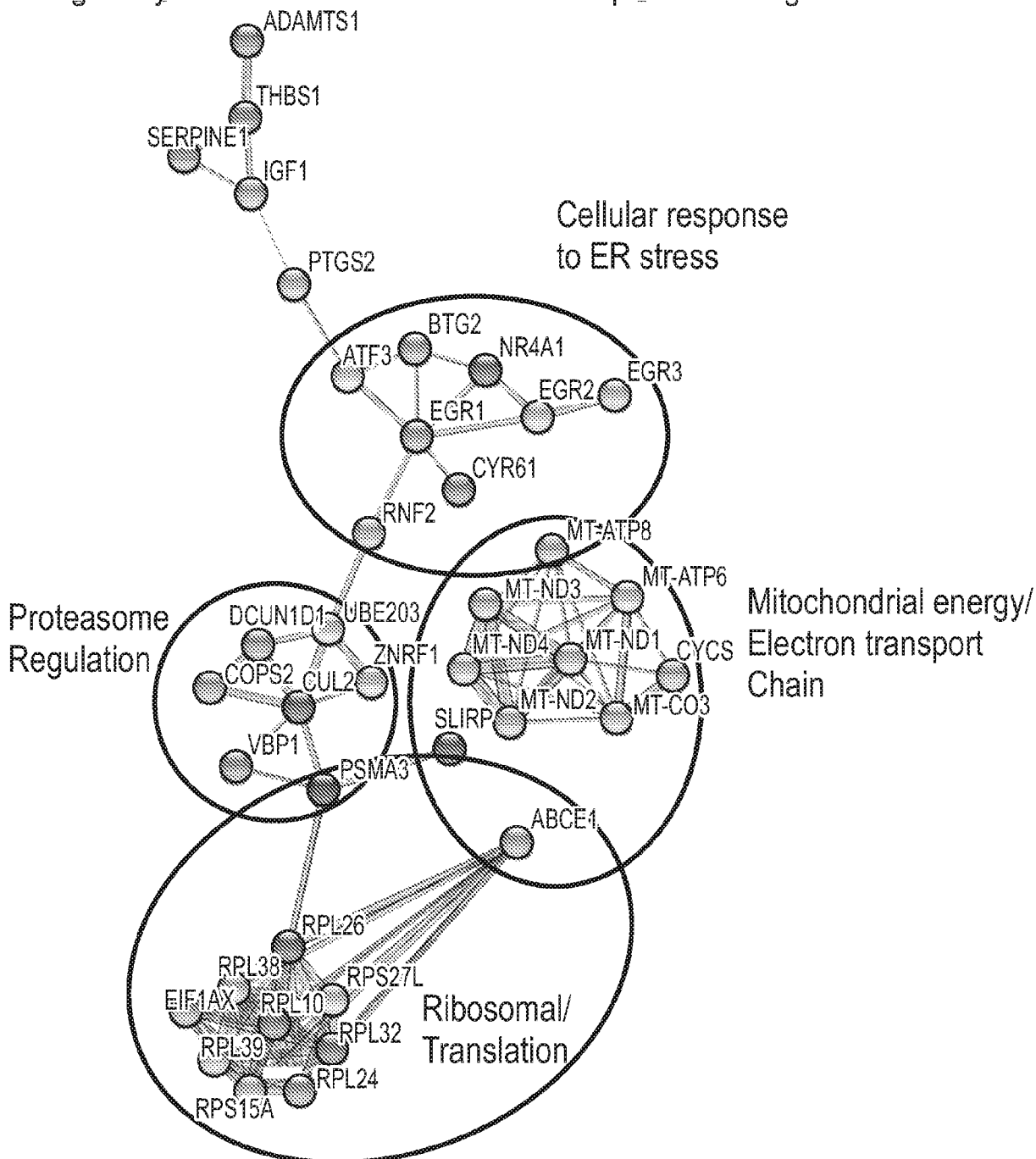
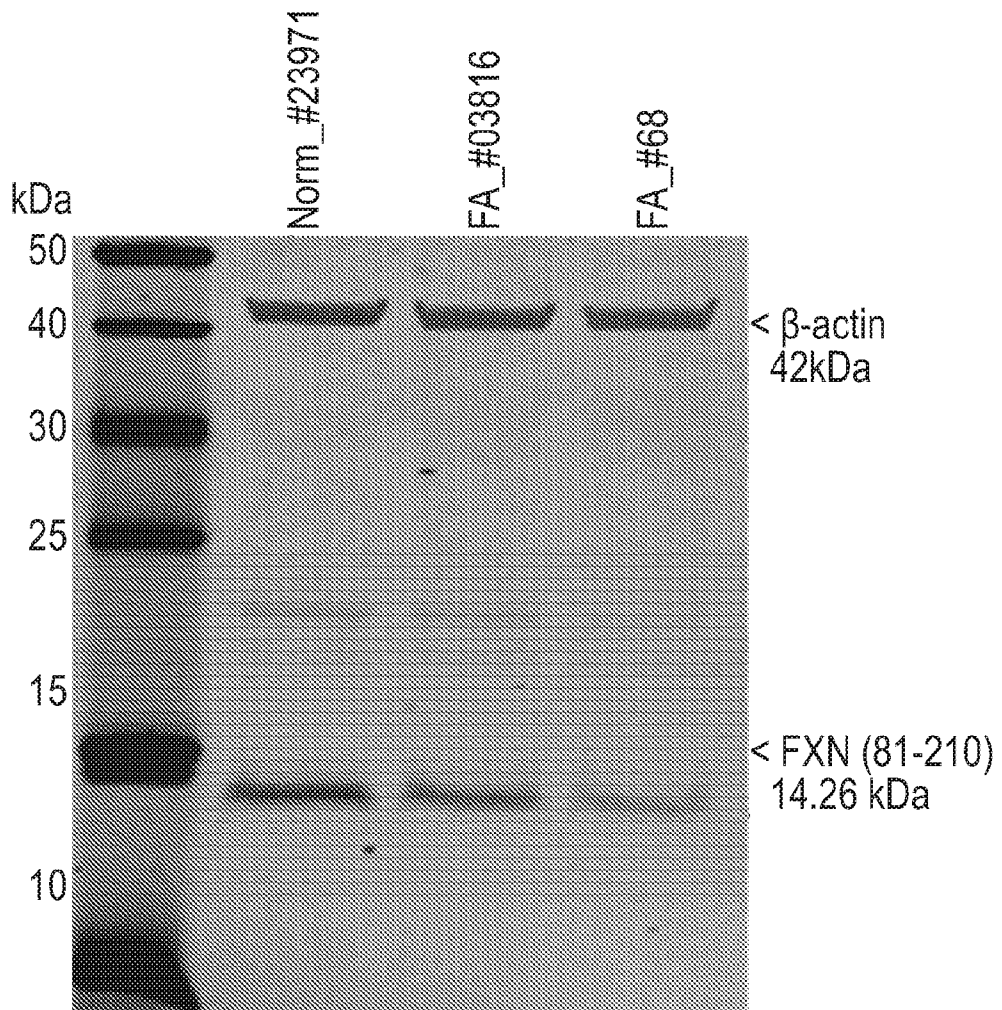


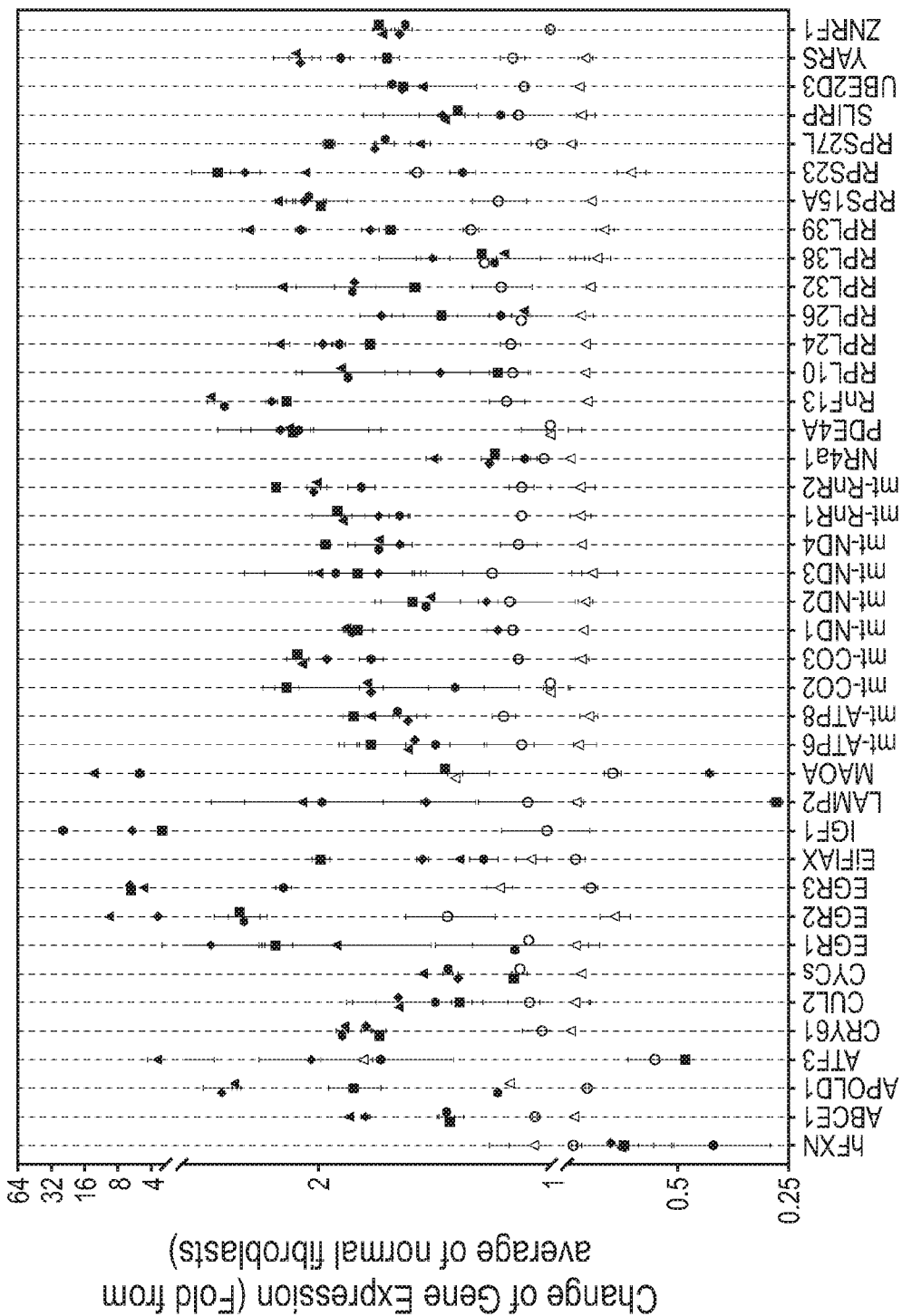
FIG. 2

Frataxin Expression Level in Normal and FRDA-derived Fibroblasts



Odysey Image #191_20Feb218

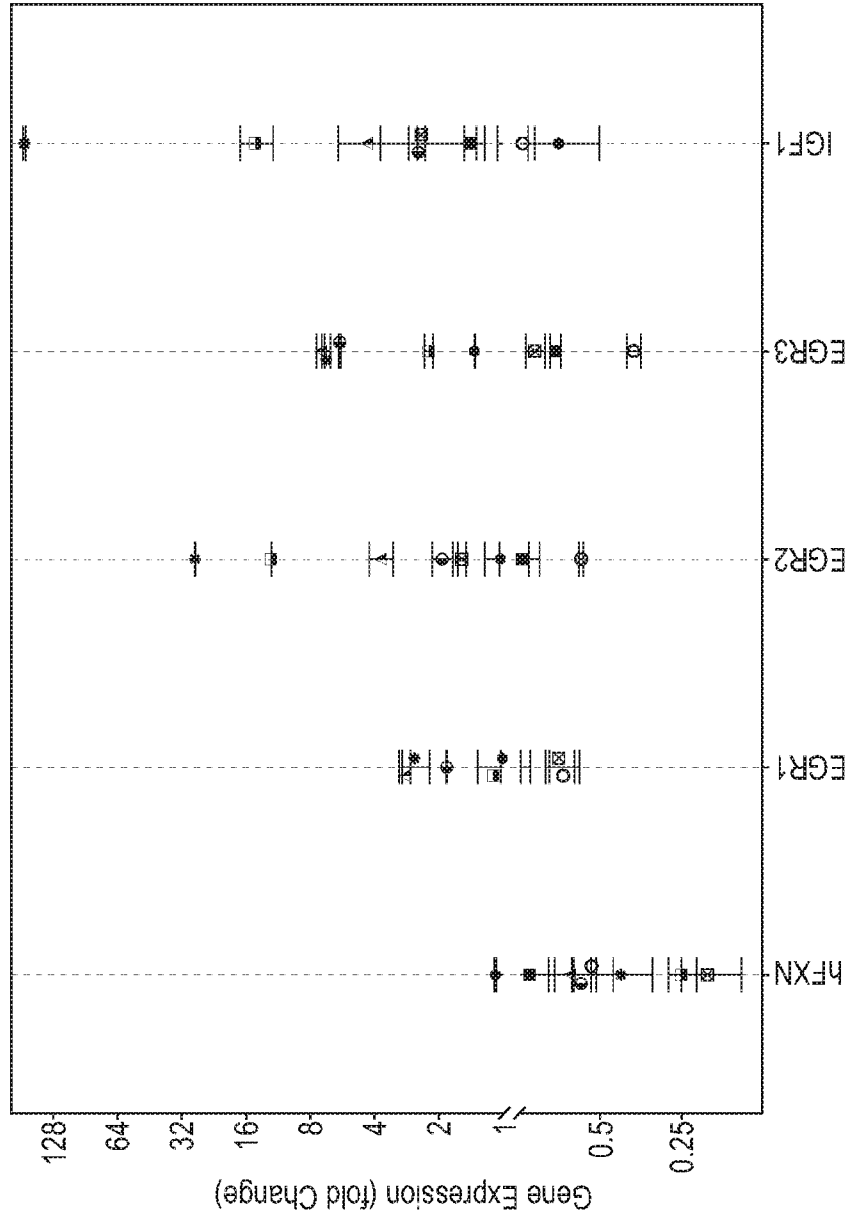
FIG. 3 Evaluation of FSGMs in patient-derived and normal fibroblasts



Baseline FXN(-) Expression Profile in FDRA-derived Fibroblasts and Normal Fibroblasts

FIG. 4A

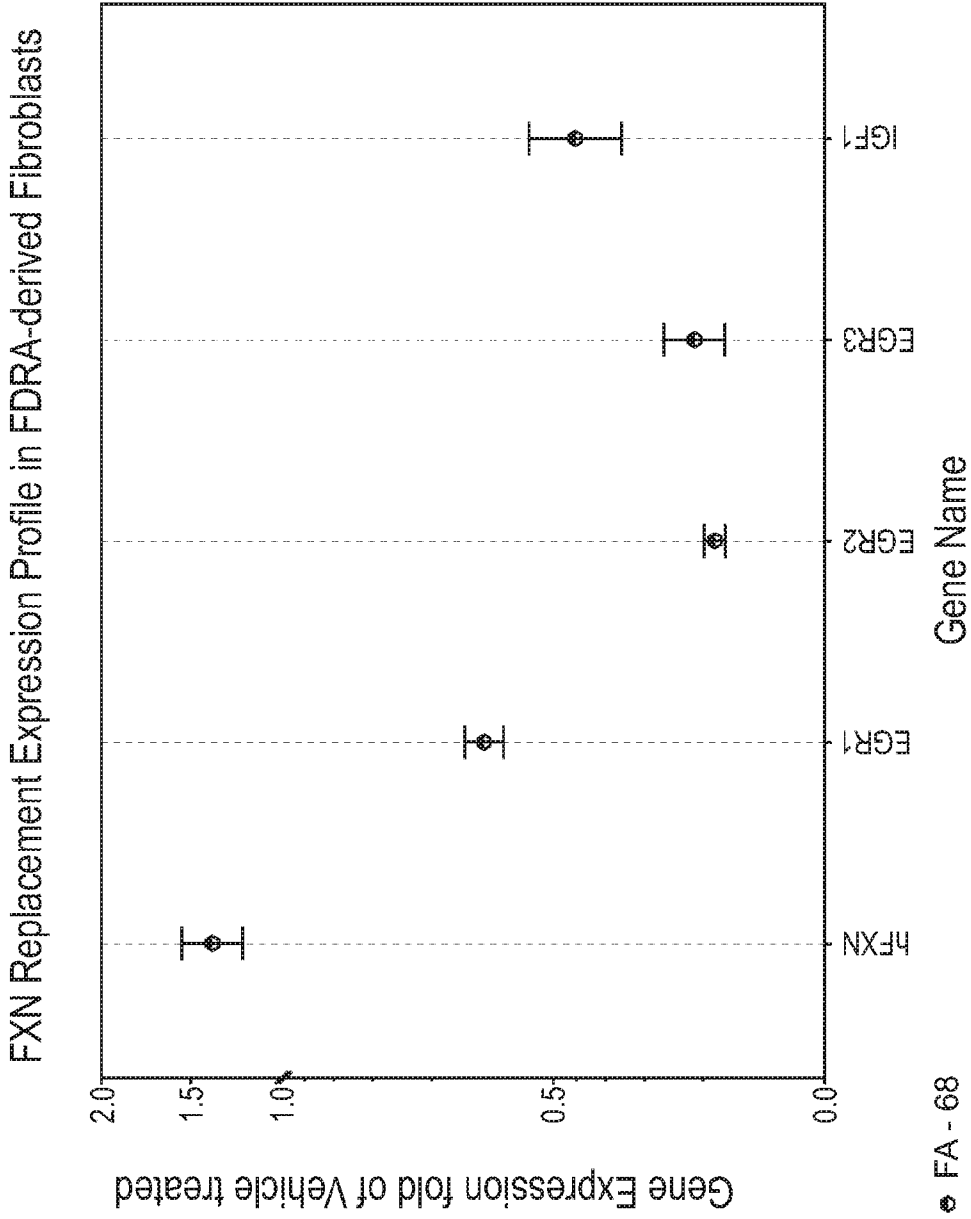
Baseline FXN(-) Expression Profile in FDRA-derived Fibroblasts and Normal Fibroblasts Grown in Hypoxia Conditions



● FA - GM03816 ▲ FA - GM04078 *FA - 4654 ■ FA - 68 □ FA - 4675 ○ FA - 4194 ● N - GM07522 ■ N - GM23971

EGR1, EGR2, and EGR3, and IGF1 Differentially Expressed in Six Frataxin-depleted Fibroblasts Compared to Two Control Normal Fibroblasts

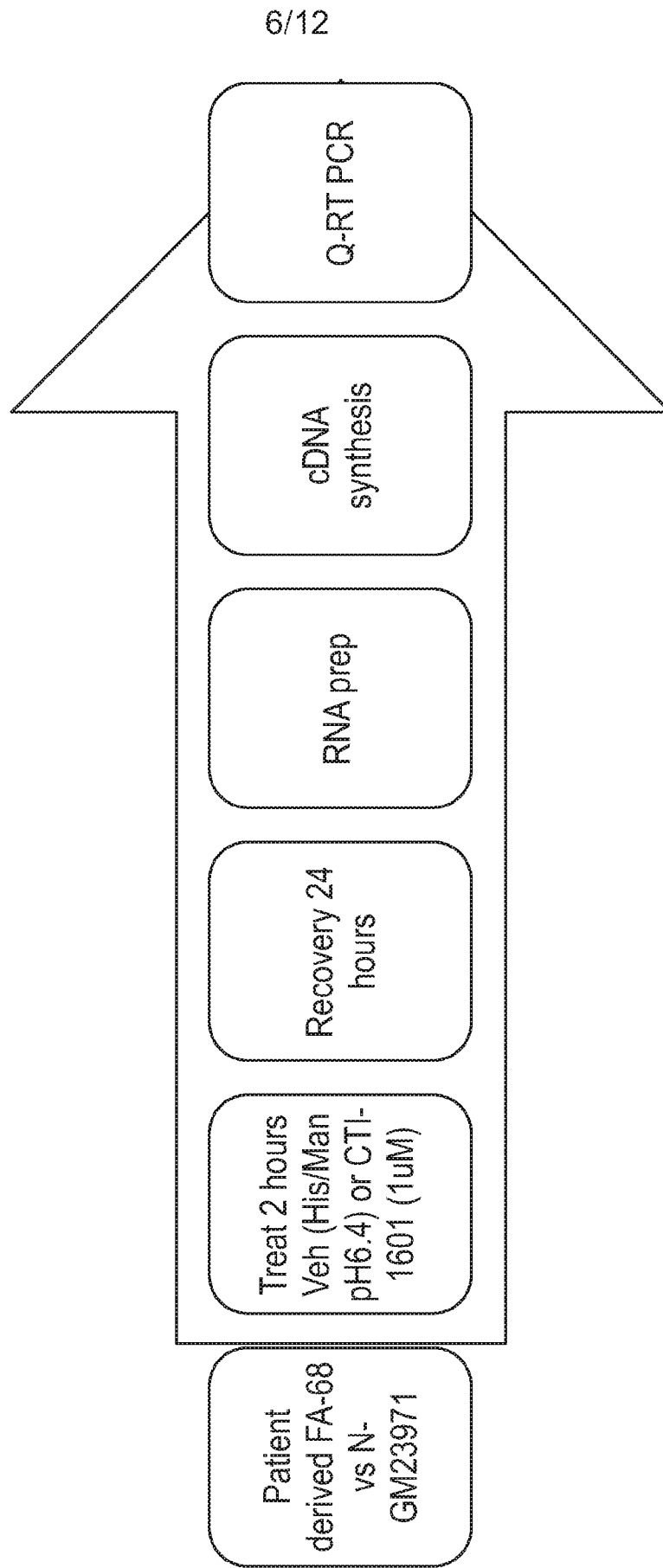
FIG. 4B



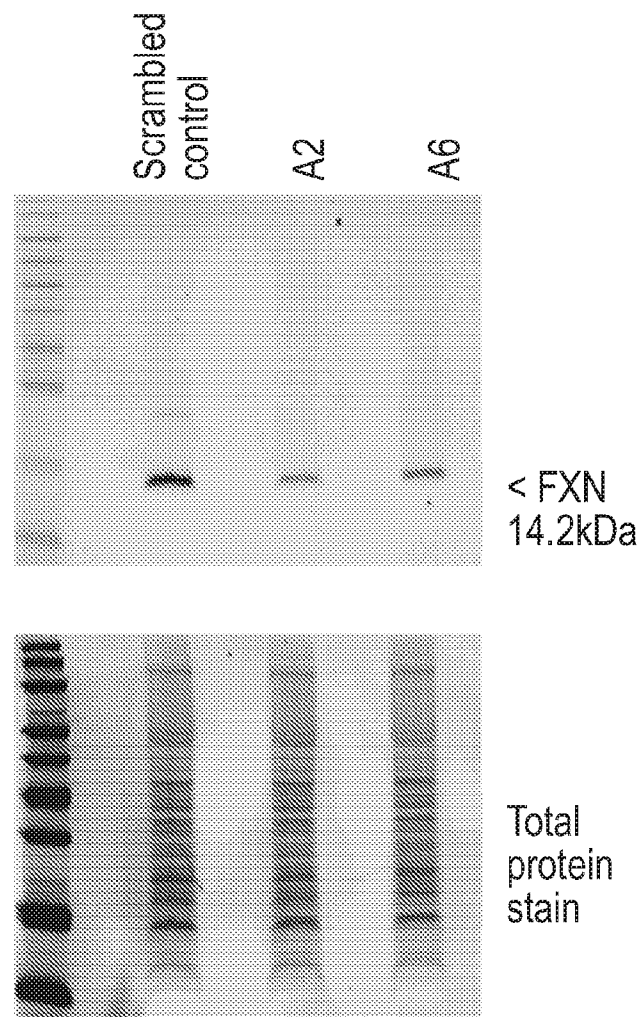
● FA - 68
EGR1, EGR2, EGR3, and IGF1 Differentially Expressed in One
Fratxin-depleted Fibroblast Cell Line (FA-68) Treated with
Fratxin-Replacement Drug Compared to Vehicle Treated

FIG. 5

Flow-chart for Generating FXN-Induced Signature



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	FXN	Total Protein	FXN/TP	FXN relative to scrambled control	% decrease relative to scrambled control
Scramble	860	17600	0.0489	100.00	
Clone A2	141	16000	0.0088	18.03	down 82%
Clone A6	213	15400	0.0138	28.31	down 72%

FIG. 6

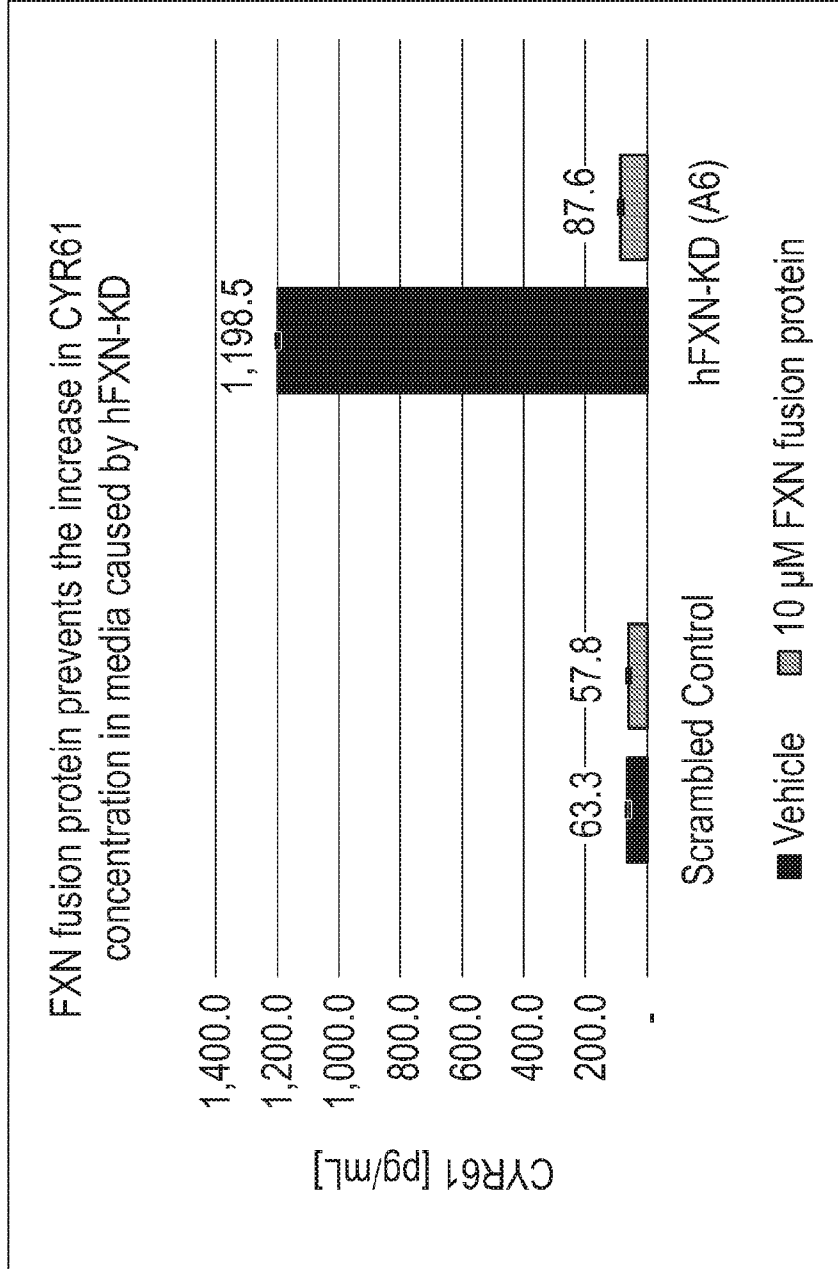


FIG. 7

Effect of mitochondrial impairment and hFXN on
CYR61 expression in cell model

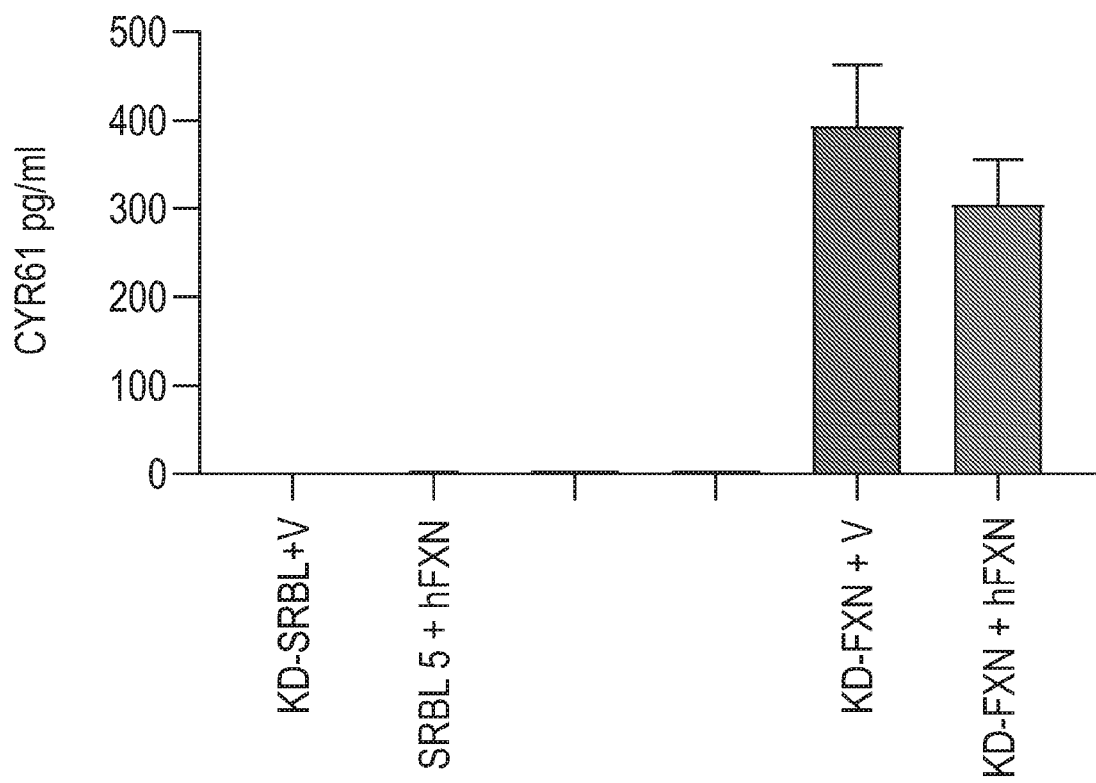


FIG. 8

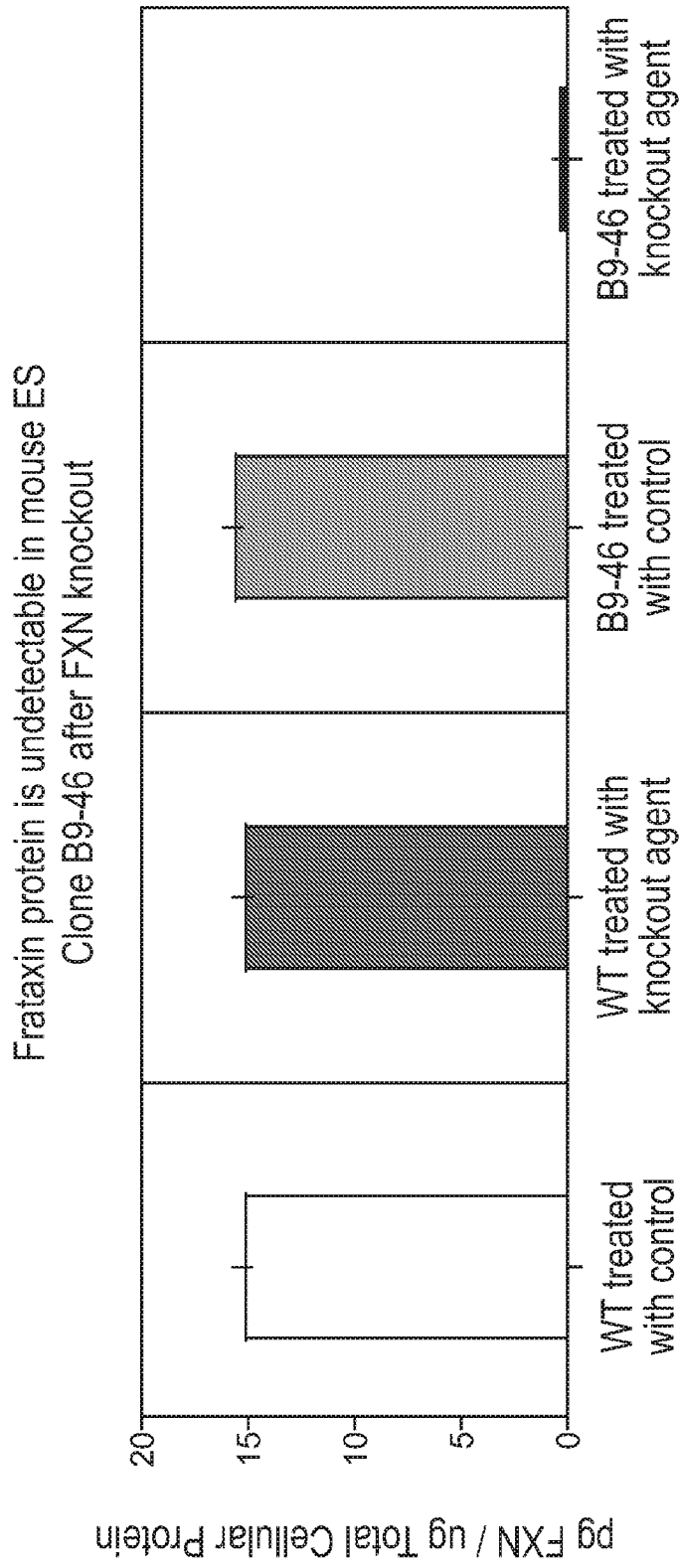
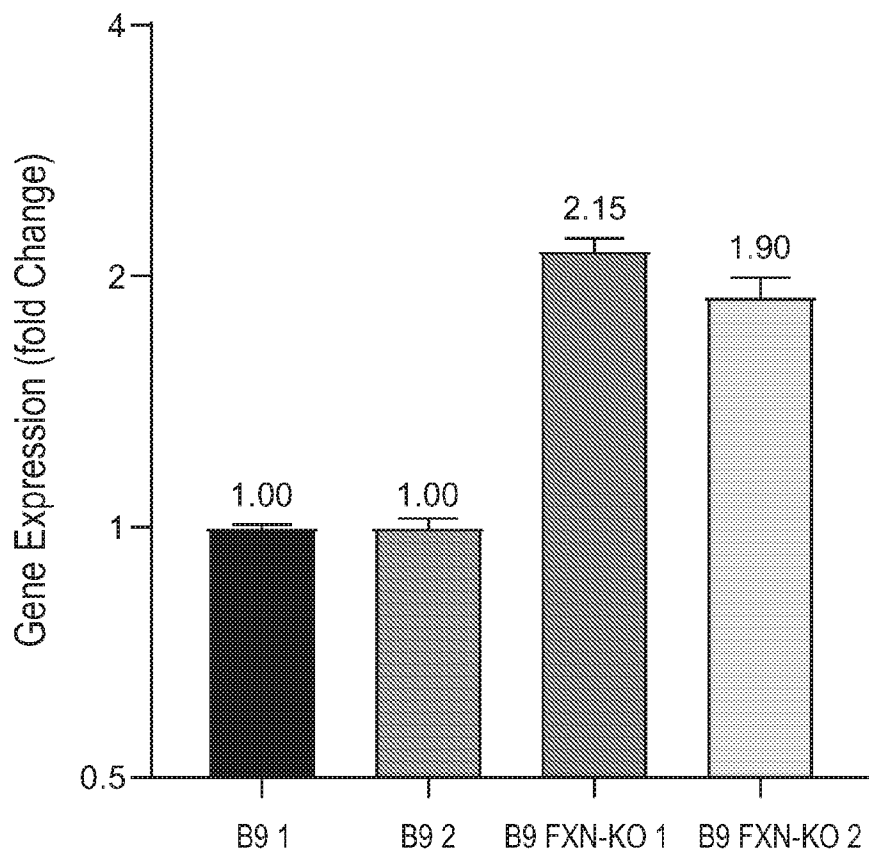


FIG. 9

qPCT analysis of CYR61 expression in
FXN-KO MES cells



Pre- and post-FXN-KO

FIG. 10A

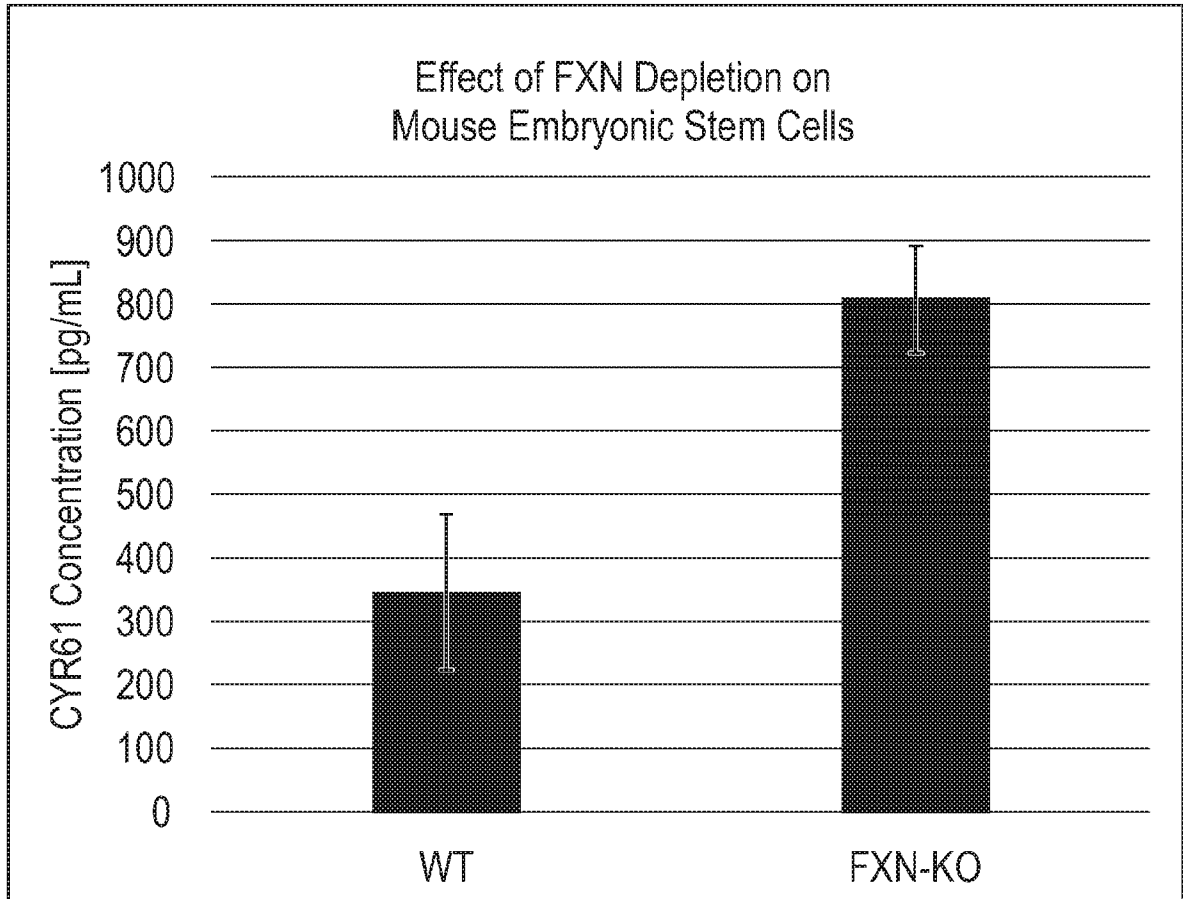


FIG. 10B