



(C) / Patient (Pt) #	Age at Baseline (years)	INTEND: Baseline / Last Report	Δ From Baseline	H <sub>2</sub> O): Baseline / Last Report	Δ From Baseline	per diploid genome	expression vs. Normal
C1 / Pt1	0.8	29 / 56 (Wk 48)	+27 (93%)	33 / 89 (Wk 24)	+56 (170%)	6.2 (Wk 24)	~120%
C1 / Pt2	4.1	45 / 64 (Wk 48)	+19 (42%)	44 / 112 (Wk 48)	+68 (155%)	7.1 (Wk 24)	~250%
C1 / Pt3	2.6	34 / 34 (Wk 36)	0 (0%)	26 / 70 (Wk 24)	+44 (170%)	2.7 (Wk 24)	~80%
C1 / Pt4 (Control)	4.0	49 / 47 (Wk 36)	-2 (-4%)	58 / 46 (Wk 24)	-12 (-21%)	NA	NA
C1 / Pt5	1.0	36 / 53 (Wk 24)	+17 (47%)	14 / 78 (Wk 24)	+64 (457%)	2.2 (Wk 24)	~52%
C1 / Pt6	0.8	39 / 52 (Wk 16)	+13 (33%)	35 / 87 (Wk 12)	+52 (149%)	NA	NA
C1 / Pt7	0.9	43 / 53 (Wk 16)	+10 (23%)	29 / 68 (Wk 12)	+39 (134%)	NA	NA
C2 / Pt8	1.2	36 / 55 (Wk 4)	+19 (53%)	31 / 67 (Wk 4)	+36 (116%)	NA	NA