A pilot study of ruxolitinib as a front-line therapy for 12 children with secondary hemophagocytic lymphohistiocytosis

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Table S1. Comparison of clinical parameters at diagnosis between the complete response (CR) and non-CR patients

Variables	Total (n=12)	CR group (n=8)	Non-CR group (n=4)	P
Temperature (℃)	39.31 ± 0.48	39.20 ± 0.35	39.50 ± 0.68	0.256
Soluble CD25 (pg/ml)	35466 (14129-218875)	35466 (14219-71345)	32066 (16978-218875)	0.933
Ferritin (ug/L)	3672 (1039-139139)	2631.50 (1039-74157)	8746 (2025-139139)	0.368
IFN-γ (pg/ml)	273 ± 170.30	274.1 ± 120.60	270.7 ± 269.00	0.976
Absolute neutrophil count ($\times 10^9$/L)	0.66 (0.38-17.34)	0.59 (0.38-0.96)	1.28 (0.59-17.34)	0.073
Hemoglobin (g/L)	89.50 ± 13.80	88.90 ± 12.00	90.80 ± 19.00	0.837
Absolute platelet count ($\times 10^9$/L)	79 (24-274)	60.50 (24-111)	110 (42-274)	0.154
Fibrinogen (g/L)	2.03 ± 1.30	1.86 ± 1.07	2.28 ± 1.83	0.626
Triglyceride (mmol/L)	3.95 ± 1.90	4.29 ± 1.92	3.66 ± 1.93	0.422
ALT (U/L)	55.25 (15.80-966)	41.10 (15.80-966)	79.3 (16.10-172)	1.000
EBV DNA at diagnosis, n (%)				
positive	8 (66.7)	6 (75)	2 (50)	0.547
negative	4 (33.3)	2 (25)	2 (50)	

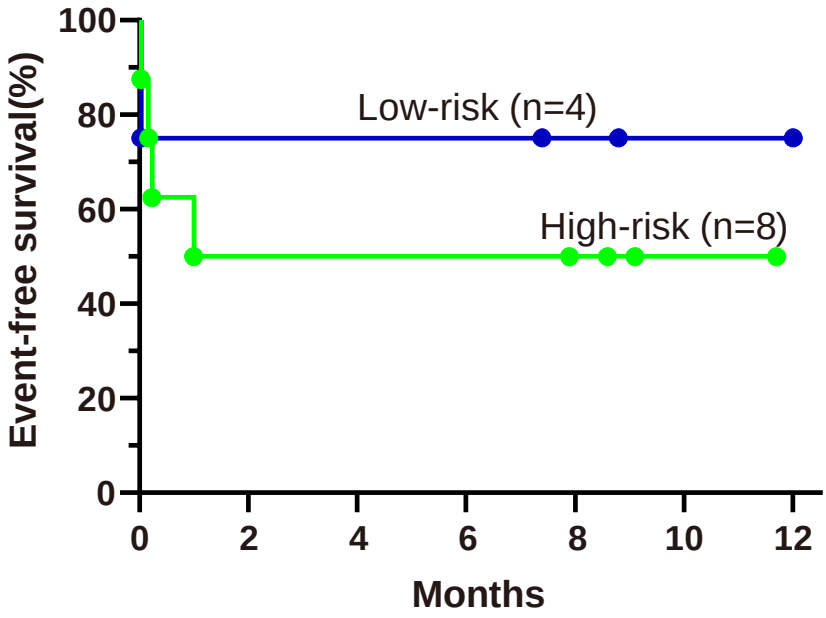

Figure.S1 Comparison of the event-free survival of patients in high and low risk group. Patients in high-risk group showed a tendency of worse EFS rate compared with patients in low-risk group, but there was no statistical significance in the small-scale analysis (50.0%±17.7 vs. 75.0%±21.7 %, p = 0.556).

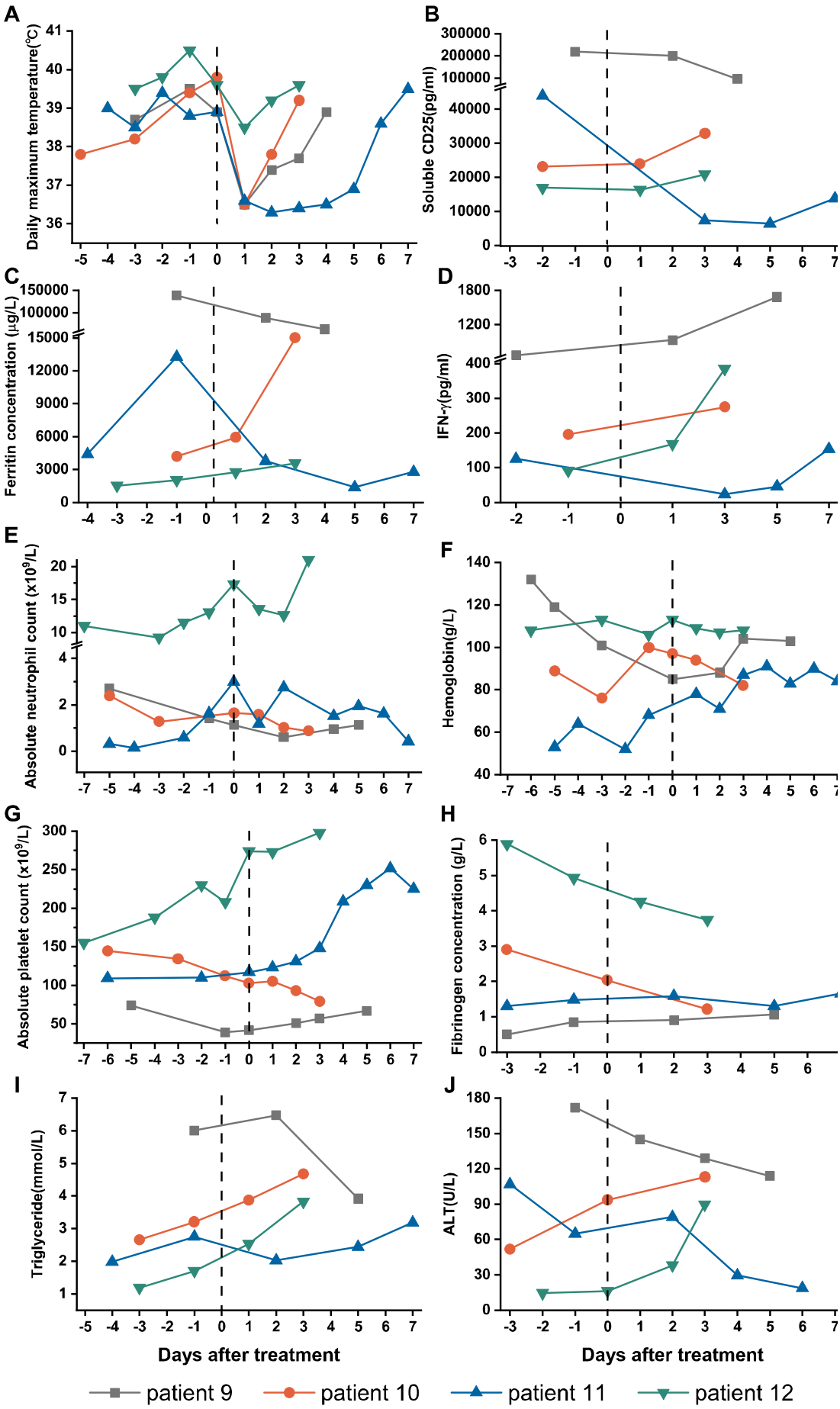
A
Daily maximum temperature(℃)
B
Soluble CD25(pg/ml)
C
Ferritin concentration (μg/L)
D
IFN-γ(pg/ml)
E
Absolute neutrophil count (x10⁹/L)
F
Hemoglobin(g/L)
G
Absolute platelet count (x10⁹/L)
H
Fibrinogen concentration (g/L)
I
Triglyceride(mmol/L)
J
ALT(U/L)
Days after treatment
Days after treatment
patient 9
patient 10
patient 11
patient 12

Figure.S2 Dynamics of HLH disease features during RUX treatment in 4 patients who had no response (patient 10, 12), progressed after partial response (patient 11), or still at high risk after partial response (patient 9). (A) daily maximum temperature; (B-D) The inflammatory markers soluble CD25 (sCD25), ferritin and IFN-γ cytokine level. Normal range values of sCD25, ferritin and IFN-γ are ≤6400pg/mL, ≤500 μg/L and ≤8pg/mL respectively by the clinical laboratory; (E-G) absolute neutrophil count, haemoglobin concentration, absolute platelet count; (H) fibrinogen concentration; (I) triglyceride; (J) alanine aminotransferase (ALT) concentration. The dotted line on the x-axis of each graph indicate the start of RUX treatment.

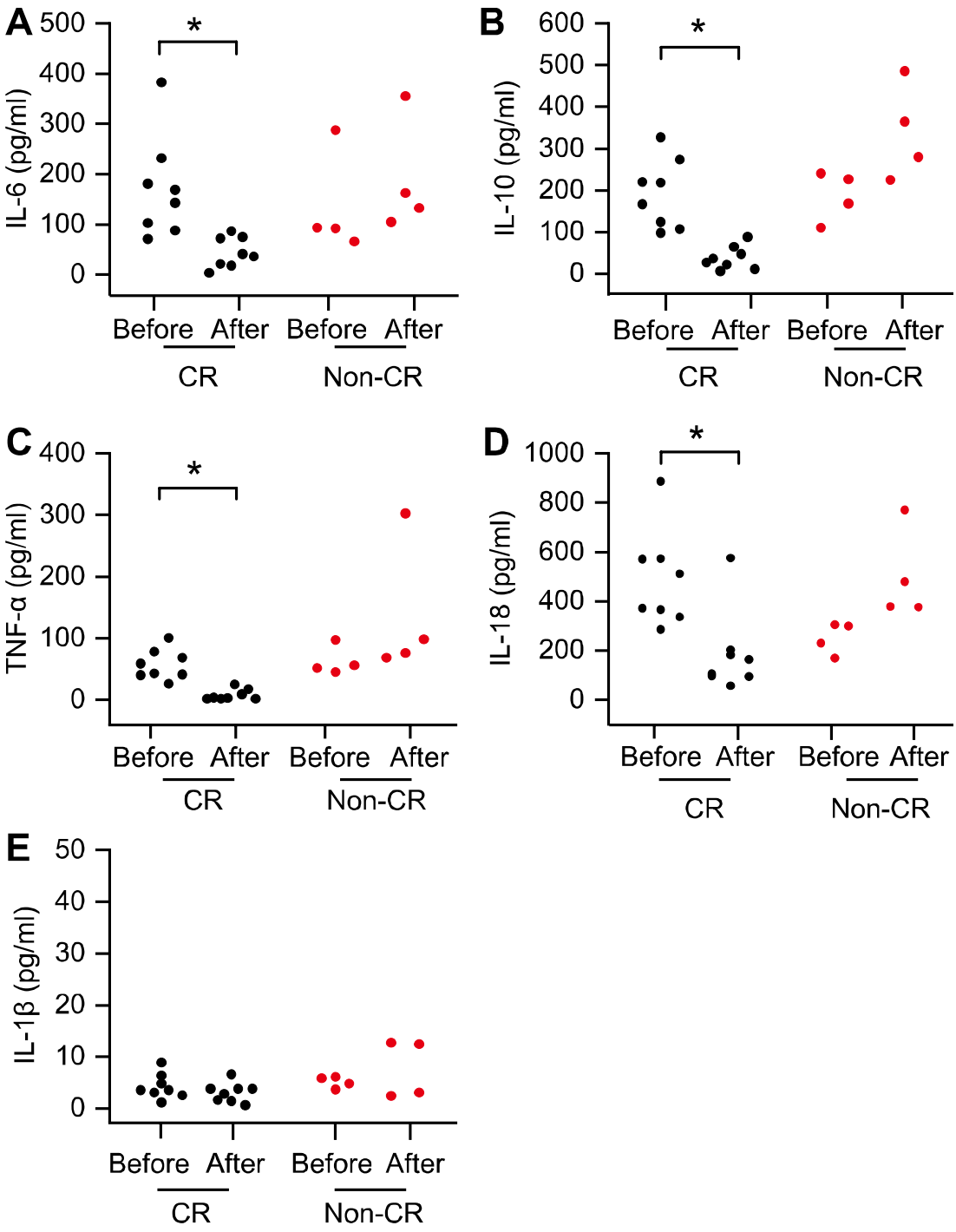

Figure.S3: The levels of IL-6, IL-10, TNF-α, IL-18 and IL-1β were measured before and 1 week after RUX treatment in complete response group (CR, n=8) and non-CR group (n=4) respectively. For patients who discontinued RUX within 7 days, the last known values were used as week 1 laboratory results. For statistical analysis, the paired sample Wilcoxon signed rank test was applied. *P<0.05, **P<0.01.