

Table S1. COG samples: patient characteristics

| Sample | Age at dx (in years) | WBC (x10 ³ /μL) | Marrow Blast % | FAB | Cytogenetics | Treatment Protocol | Response to Course 1 | Follow-up (days) | Status | Cause of Death | Constit. pStat3 |
|--------|----------------------|----------------------------|----------------|-----|--|--------------------|----------------------|------------------|--------|----------------|-----------------|
| CBF 1 | 13.2 | 24.3 | 51 | M2 | 45,X,-Y,t(8:21)(q22:q22)[14]/46,XY[6] | CCG 2961 | W/D (NR) | 1569 | Alive | | - |
| CBF 2 | 3.8 | 19.8 | 32 | M2 | 45,X,-X,t(8:21)(q22:q22)[19]/46,XX[1] | CCG 2961 | CR | 3629 | Alive | | - |
| CBF 3 | 12.9 | 69.2 | 73 | M2 | 46,XX,der(7)t(7:9)(q22:q13),t(8:21)(q22:q22),add(9)(q13)[20] | CCG 2961 | CR | 485 | Dead | PD | - |
| CBF 4 | 15.1 | 7.9 | 7 | Unk | 45,X,-Y,t(8:21)(q22:q22)[20] | AAML03P1 | CR | 1634 | Alive | | + |
| CBF 5 | 4.0 | 5.7 | 77 | M2 | 46,XX,t(8:21)(q22:q22) | AAML03P1 | CR | 1197 | Alive | | - |
| CBF 6 | 11.1 | 19.7 | 85 | M2 | 45,X,-Y,t(8:21)(q22:q22)[18]/46,XY[2] | CCG 2961 | CR | 4013 | Alive | | - |
| CBF 7 | 4.2 | 38 | 59 | M2 | 46,XY,t(8:16:21)(q22:p11.2:q22)[8]/46, idem,der(9)t(1:9)(q25:q34)[20]/46,XY[2] | CCG 2961 | CR | 3754 | Alive | | - |
| CBF 8 | 11.5 | 31.8 | 28 | M2 | 46,XX,t(8:21)(q22:q22)[20] | CCG 2961 | CR | 451 | Dead | Infection | - |
| CBF 9 | 11.7 | 14 | 72 | M2 | 45,X,-X,t(8:21)(q22:q22)[20] | CCG 2961 | W/D (CR) | 2432 | Alive | | - |
| CBF 10 | 16.2 | 25.4 | 85 | M2 | 46,XY,t(8:21)(q22:q22)[20] | CCG 2961 | CR | 2319 | Alive | | - |
| NK 1 | 10.5 | 59.7 | 98 | M4 | 46,XY[20] | CCG 2891 | PR | 4631 | Alive | | + |
| NK 2 | 11.1 | 13.6 | 40 | M2 | 46,XX[21] | CCG 2891 | CR | 190 | Dead | Infection | + |
| NK 3 | 3.1 | 575 | 33 | M4 | 46,XY[20] | CCG 2891 | Fail | 321 | Dead | PD | + |
| NK 4 | 4.9 | 330.7 | 87 | M2 | 46,XY[20] | CCG 2891 | Fail | 463 | Dead | Other | + |
| NK 5 | 13.3 | 29.6 | 86 | M1 | 46,XX[20] | CCG 2891 | CR | 843 | Dead | PD | + |
| NK 6 | 9.7 | 21 | 68 | M1 | 46,XX[20] | CCG 2891 | CR | 3352 | Alive | | - |
| NK 7 | 10.8 | 63.3 | 66 | M4 | 46,XY[20] | CCG 2891 | CR | 2944 | Alive | | - |
| NK 8 | 15.0 | 91.3 | 61 | M1 | 46,XX[29]/NC[1] | CCG 2891 | CR | 3235 | Alive | | - |

Abbreviations:

FAB - French American British classification

W/D - Withdrawn from study

CR - Complete Response (<5% marrow blasts)

PR - Partial Response (5-25% marrow blasts)

PD - Progressive Disease

NR – No Response

Table S2. TXCCC samples: patient characteristics

| Sample | Age at dx (in years) | WBC (x10³ /μL) | Marrow Blast % | FAB | Cytogenetics | Assays Performed |
|---------------|-----------------------------|----------------------------------|-----------------------|------------|---|-------------------------|
| 1861 | 11 | 122,000 | 79 | M2 | 46,XY[20] | Stim; C188-9 |
| 1733 | 1 | 19,000 | 90 | M1 | Near tetraploid with t(2;12)(q33;p13) | C188-9; CFU |
| 2187 | 9 | 71,000 | 65 | M4 | 46,XY,t(1;6;11)(q21;q27;q23)[19],46,XY[1] | Stim; C188-9; CFU |
| 2053 | | | | | Data Not Available | Stim |
| 3190 | 10 | 400,000 | 95 | M4 | MLL-rearranged by FISH (relapse of #2187) 46,XY,der(10)inv(11)(q13q23)t(10;11)(p12;q13), | Stim; C188-9 |
| 3482 | 13 | 40,000 | 70 | M5 | der(11)t(10;11)(p12;q13)[20] | Stim; CFU |
| 1436 | 11 | 528,000 | 66 | Unk | Unknown | C188-9 |
| 2735 | 7 | 42,000 | 84 | M1 | 46,XY,t(8;21)(q22;q22)[11] | C188-9; CFU |
| 2742 | 3 | 53,000 | 79(PB) | Unk | 47,X,der(X)t(X;1)(q28;q21),+mar[20] | CFU |

Abbreviations:

FAB - French American British classification

PB – peripheral blood

Unk – unknown

FISH – fluorescence in situ hybridization

Stim – G-CSF stimulation

C188-9 – pStat3 and/or survival inhibition with C188-9

CFU – colony forming unit assay

Table S3. C188-9 IC₅₀s for inhibition of pStat3 in AML cell lines

| Cell Line | C188-9 IC₅₀ (μM) |
|------------------|---|
| Kasumi-1 | 4.5 |
| THP-1 | 4.8 |
| GDM-1 | 5.0 |
| NB-4 | 8.3 |
| HL-60 | 5.3 |
| KG-1 | 4.1 |

n=4 for Kasumi-1; n=3 for others

Table S4. C188-9 and JSI-124 EC₅₀s for induction of apoptosis in AML cell lines

| Cell Line | C188-9 EC₅₀ (μM) [n] | JSI-124 EC₅₀ (μM) [n] |
|------------------|---|--|
| Kasumi-1 | 8.4 [5] | 1.2 [3] |
| THP-1 | 15.2 [5] | 1.1 [3] |
| GDM-1 | 6.0 [4] | 9.5 [3] |
| NB-4 | 20.5 [6] | 13.0 [3] |
| HL-60 | 34.7 [6] | 6.1 [3] |
| KG-1 | 42.8 [3] | 6.5 [3] |
| K562 | 43.6 [5] | 44.9 [3] |

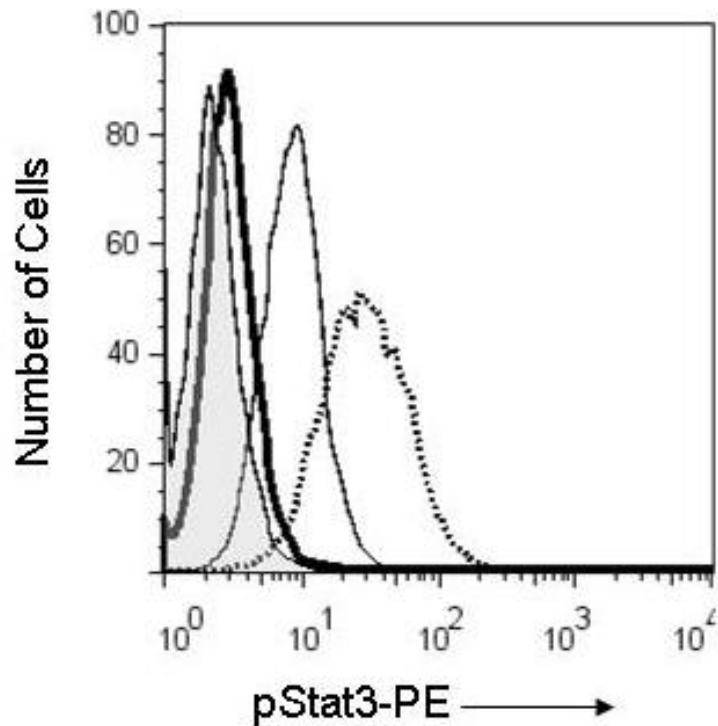


Figure S1. Immunoreactivity for pStat3 was blocked by the immunizing pY peptide. THP-1 cells were unstimulated or stimulated with G-CSF (100 ng/ml), then prepared and labeled for FACS analysis. One sample of G-CSF-stimulated cells was labeled with anti-pStat3 antibody that had been pre-incubated for one hour at 4° with the peptide used to develop the antibody (7.5 mg/100 ml reaction). Shaded: isotype control; solid line; unstimulated; dotted line: G-CSF-stimulated; Thick line: peptide-pretreated.