Figure 1.

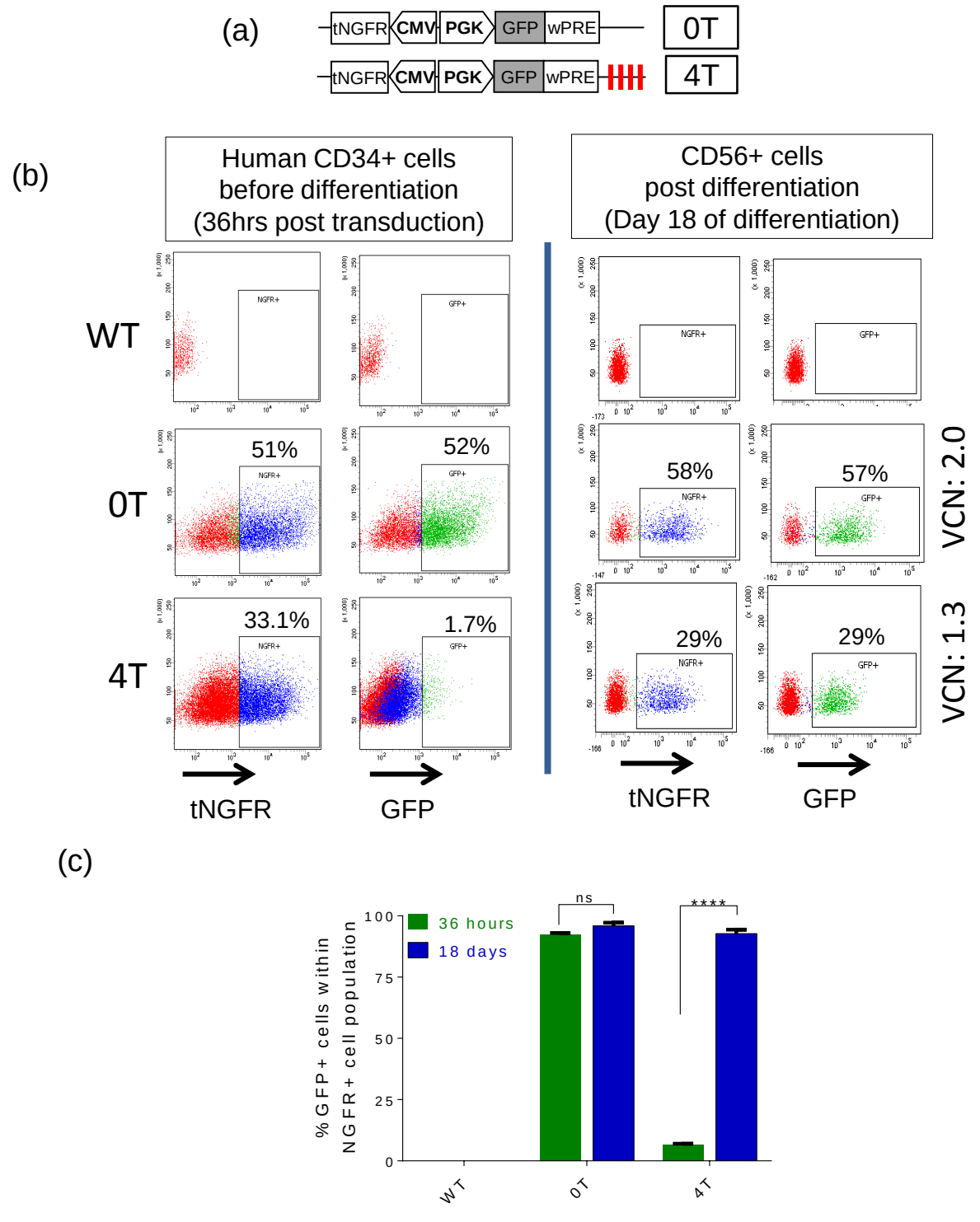

(a)
tNGFR
CMV
PGK
GFP
wPRE
0T
4T
(b)
Human CD34+ cells
before differentiation
(36hrs post transduction)
CD56+ cells
post differentiation
(Day 18 of differentiation)
WT
0T
4T
NGFR+
GFP+
51%
52%
33.1%
1.7%
58%
57%
29%
29%
VCN: 2.0
VCN: 1.3
tNGFR
GFP
(c)
% GFP+ cells within NGFR+ cell population
36 hours
18 days
ns
****
100
75
50
25
0
WT
0T
4T

Figure 2

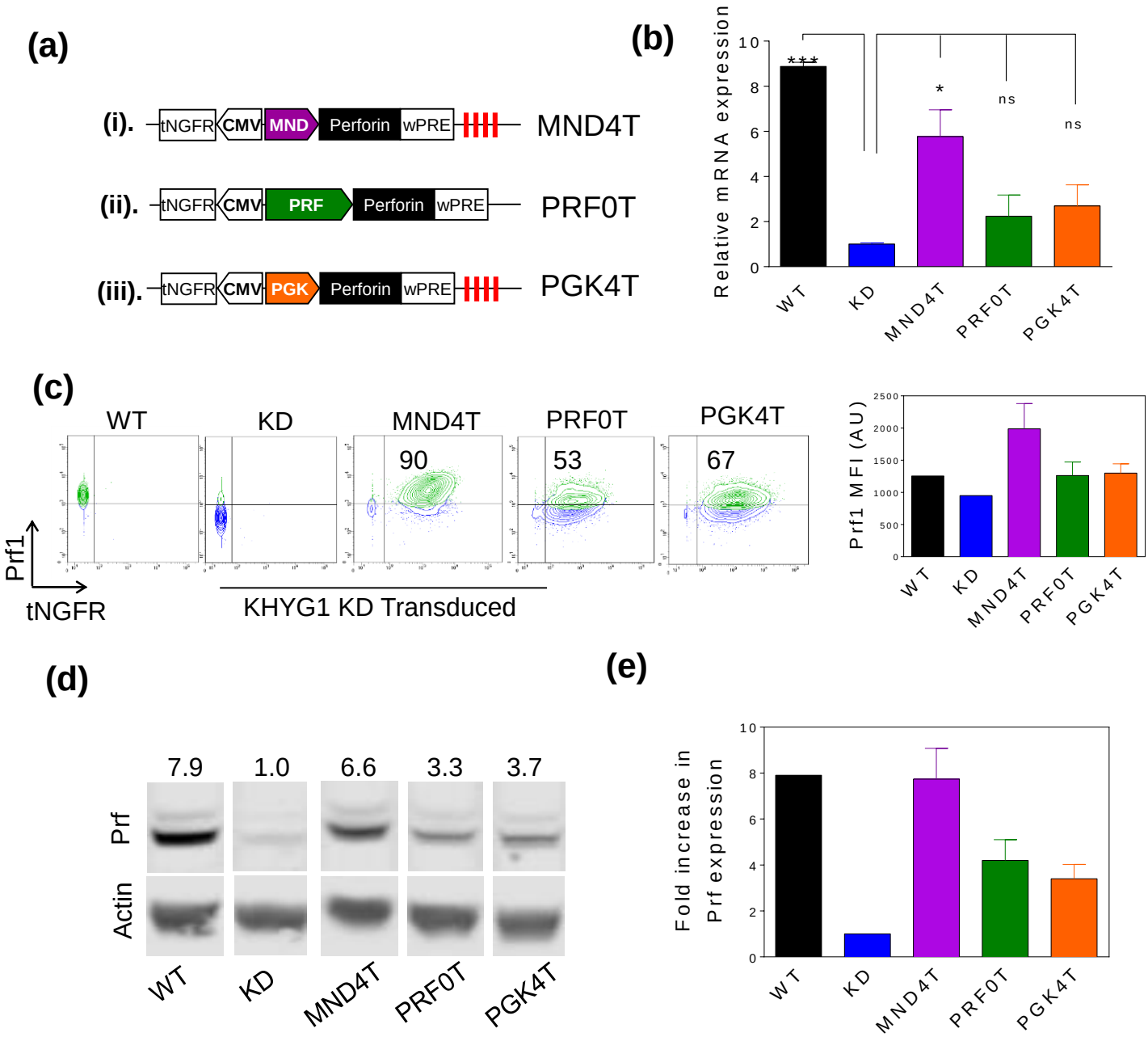

(a)
(i). tNGFR CMV MND Perforin wPRE MND4T
(ii). tNGFR CMV PRF Perforin wPRE PRF0T
(iii). tNGFR CMV PGK Perforin wPRE PGK4T
(b)
Relative mRNA expression
***
*
ns
ns
WT
KD
MND4T
PRF0T
PGK4T
(c)
WT
KD
MND4T
PRF0T
PGK4T
90
53
67
Prf1
tNGFR
KHYG1 KD Transduced
Prf1 MFI (AU)
(d)
7.9
1.0
6.6
3.3
3.7
Prf
Actin
WT
KD
MND4T
PRF0T
PGK4T
(e)
Fold increase in Prf expression
WT
KD
MND4T
PRF0T
PGK4T

Figure 3.

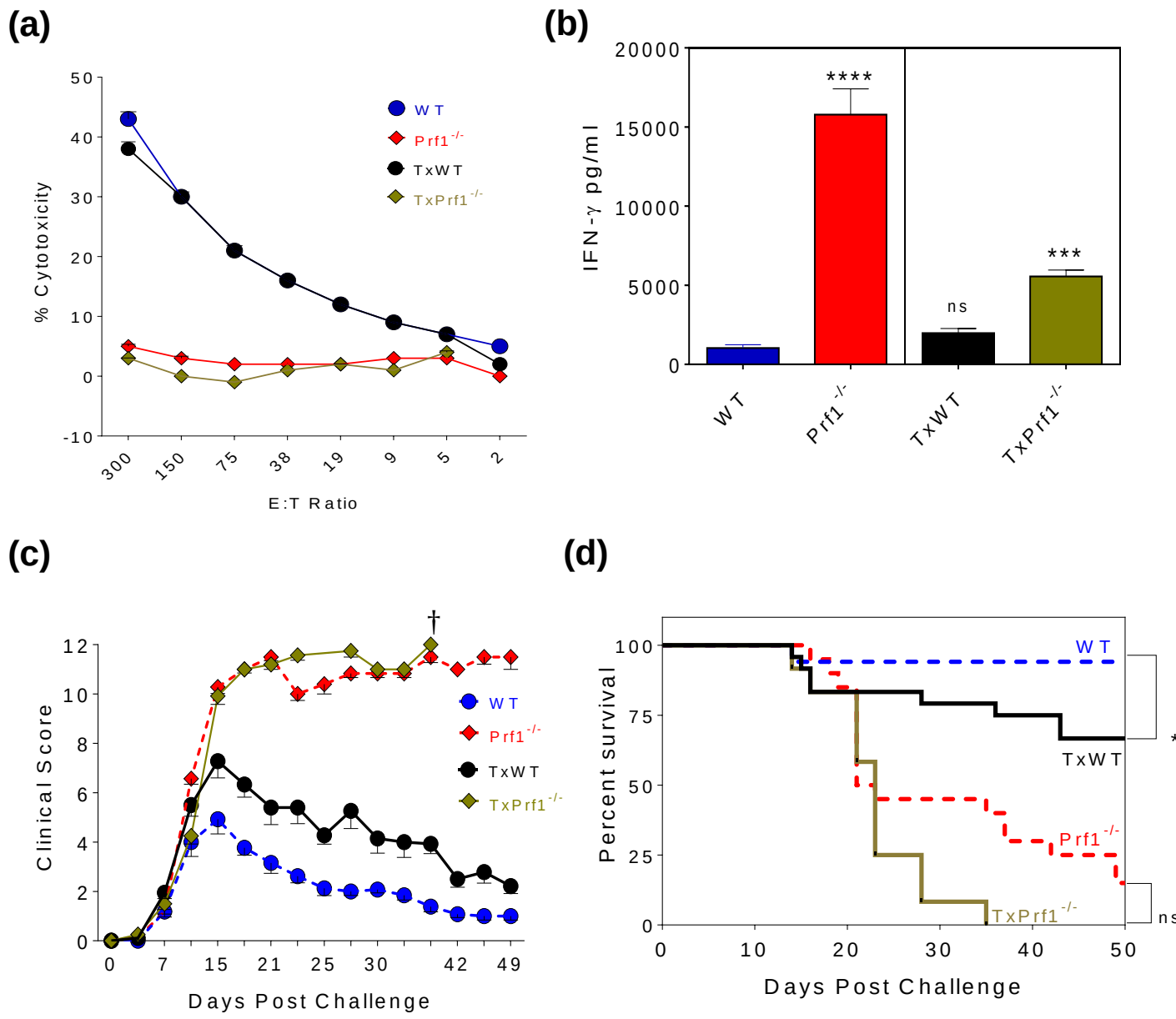

(a)
% Cytotoxicity
E:T Ratio
WT
Prf1 -/-
TxWT
TxPrf1 -/-
(b)
IFN-γ pg/ml
****
ns
***
WT
Prf1 -/-
TxWT
TxPrf1 -/-
(c)
Clinical Score
Days Post Challenge
†
(d)
Percent survival
Days Post Challenge
*
ns

Figure 4.

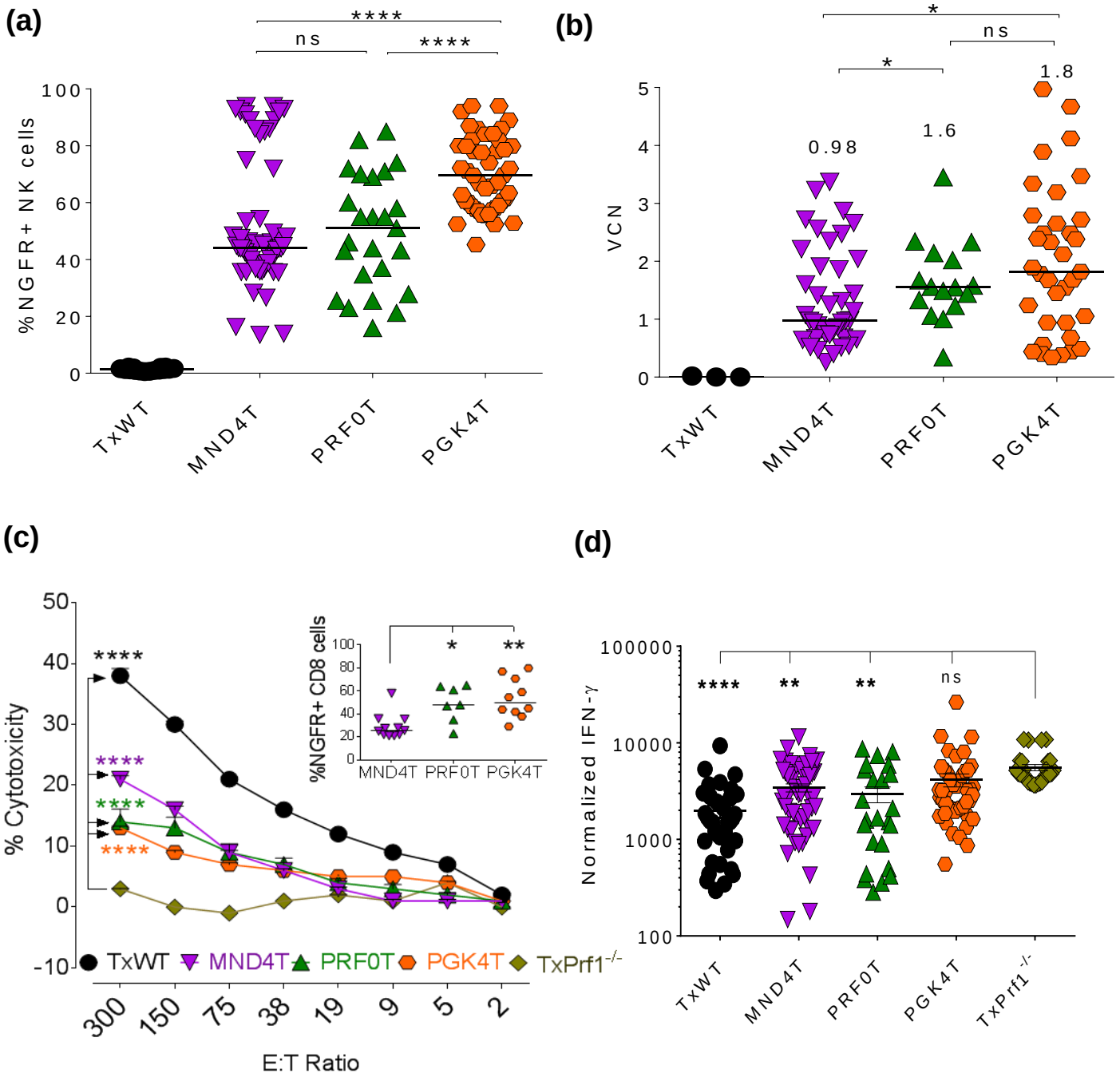
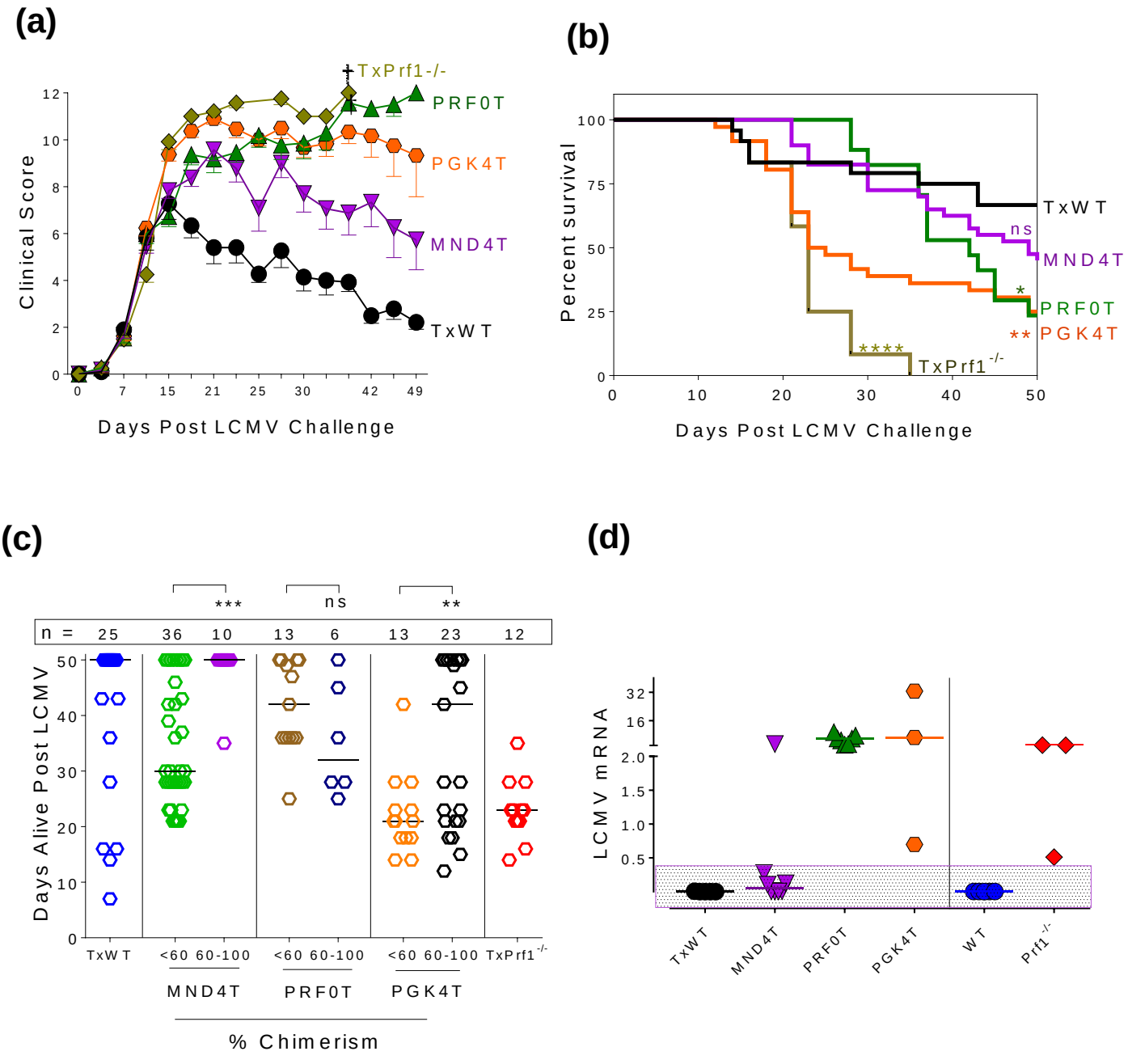

(a)
% NGFR+ NK cells
ns
****
****
TxWT
MND4T
PRF0T
PGK4T
(b)
VCN
*
ns
*
0.98
1.6
1.8
(c)
% Cytotoxicity
%NGFR+ CD8 cells
MND4T PRF0T PGK4T
TxWT MND4T PRF0T PGK4T TxPrf1-/-
E:T Ratio
(d)
Normalized IFN-γ
ns
TxPrf1-/-

Figure 5


(a)
Clinical Score
Days Post LCMV Challenge
TxPrf1-/-
PRF0T
PGK4T
MND4T
TxWT
(b)
Percent survival
Days Post LCMV Challenge
TxWT
ns
MND4T
*
PRF0T
** PGK4T
****
TxPrf1-/-
(c)
***
ns
**
n = 25 36 10 13 6 13 23 12
Days Alive Post LCMV
TxWT
<60 60-100
MND4T
<60 60-100
PRF0T
<60 60-100
PGK4T
TxPrf1-/-
% Chimerism
(d)
LCMV mRNA
TxWT
MND4T
PRF0T
PGK4T
WT
Prf1-/-