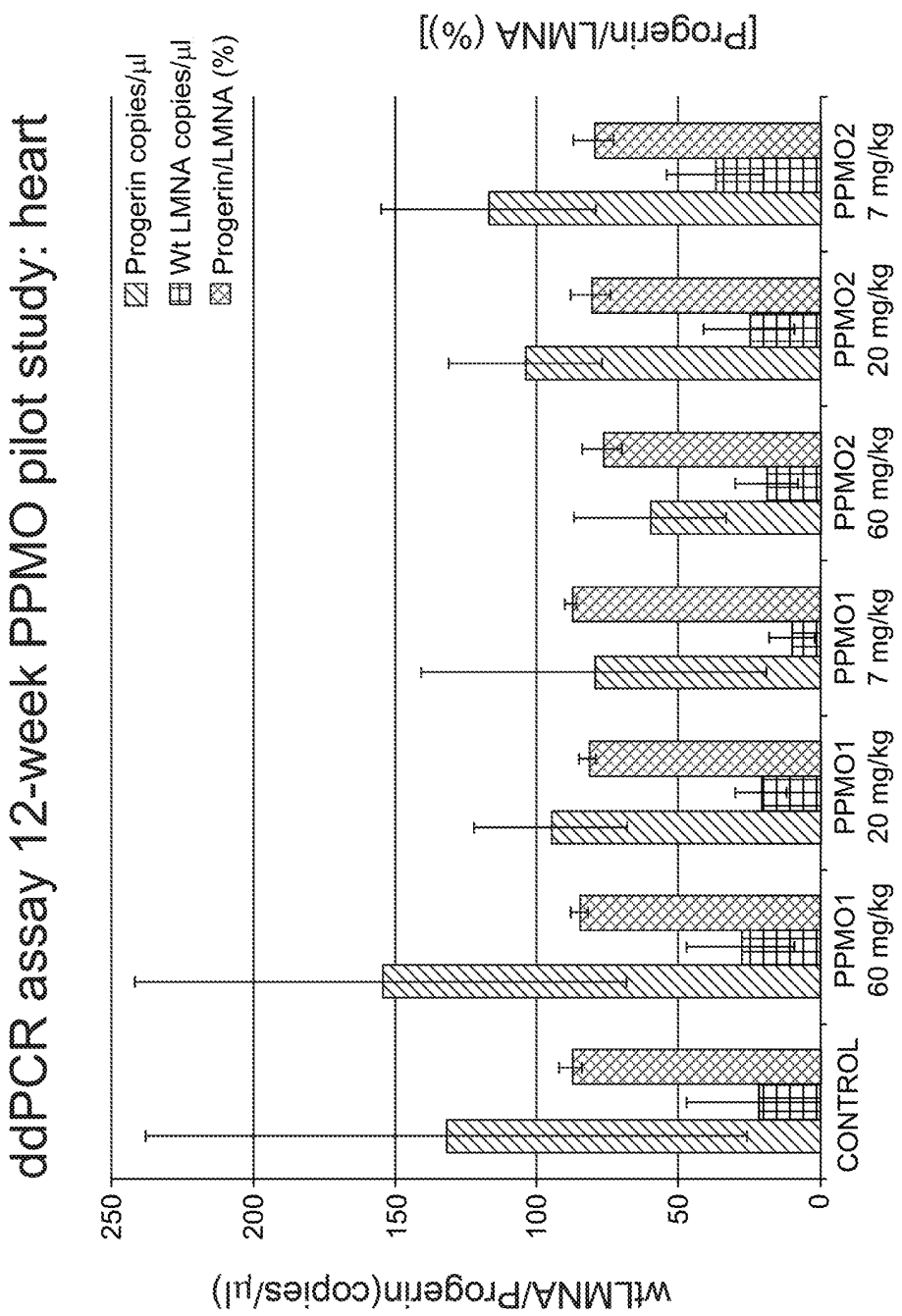

ddPCR assay 12-week PPMO pilot study: heart
Progerin copies/µl
Wt LMNA copies/µl
Progerin/LMNA (%)
wtLMNA/Progerin(copies/µl)
[Progerin/LMNA (%)]
250
200
150
100
50
0
CONTROL
PPMO1 60 mg/kg
PPMO1 20 mg/kg
PPMO1 7 mg/kg
PPMO2 60 mg/kg
PPMO2 20 mg/kg
PPMO2 7 mg/kg

FIG. 1

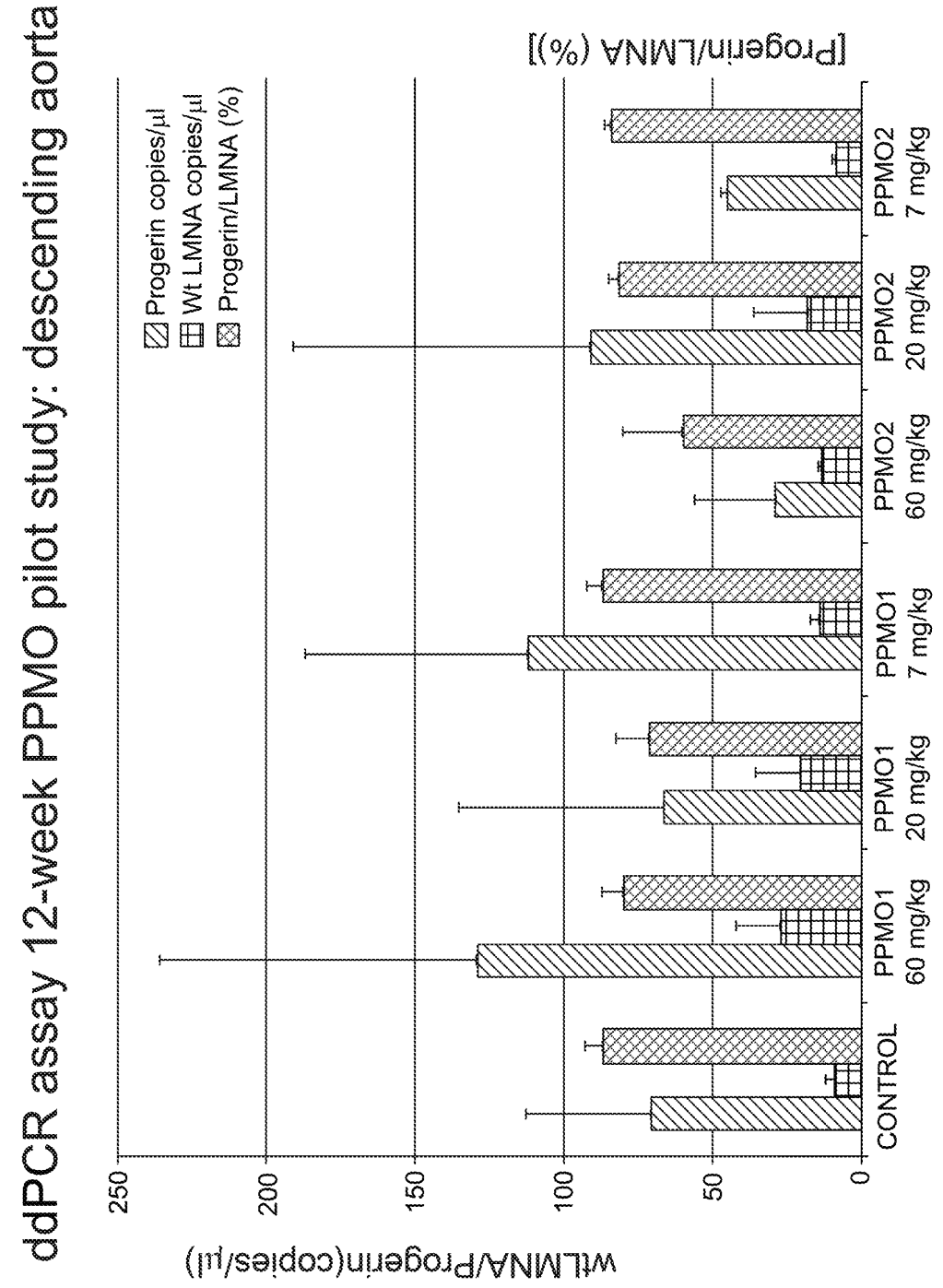

ddPCR assay 12-week PPMO pilot study: descending aorta
Progerin copies/µl
Wt LMNA copies/µl
Progerin/LMNA (%)
wtLMNA/Progerin(copies/µl)
[Progerin/LMNA (%)]
250
200
150
100
50
0
CONTROL
PPMO1 60 mg/kg
PPMO1 20 mg/kg
PPMO1 7 mg/kg
PPMO2 60 mg/kg
PPMO2 20 mg/kg
PPMO2 7 mg/kg

FIG. 2

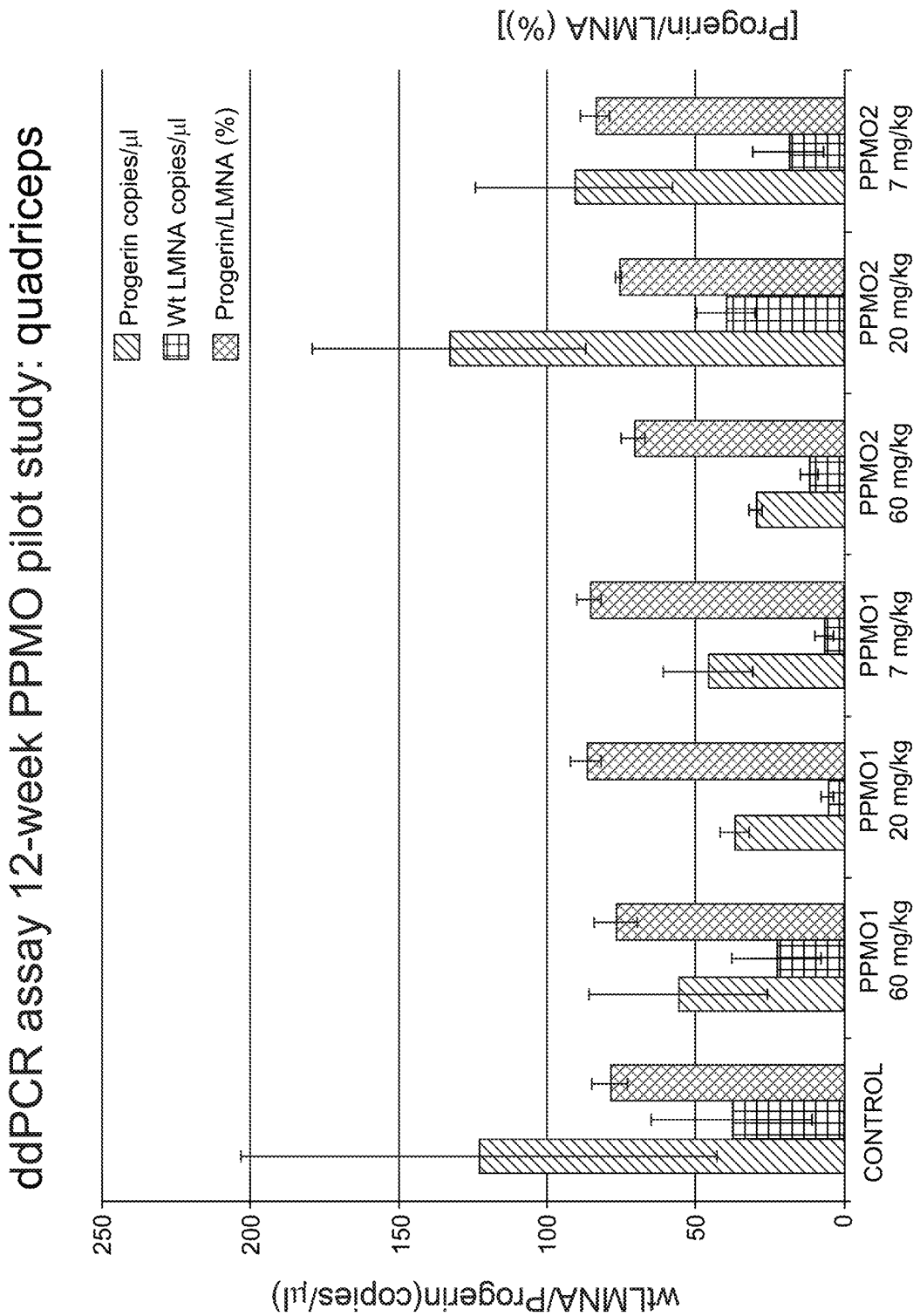

ddPCR assay 12-week PPMO pilot study: quadriceps
Progerin copies/µl
Wt LMNA copies/µl
Progerin/LMNA (%)
wtLMNA/Progerin(copies/µl)
[Progerin/LMNA (%)]
250
200
150
100
50
0
CONTROL
PPMO1 60 mg/kg
PPMO1 20 mg/kg
PPMO1 7 mg/kg
PPMO2 60 mg/kg
PPMO2 20 mg/kg
PPMO2 7 mg/kg

FIG. 3

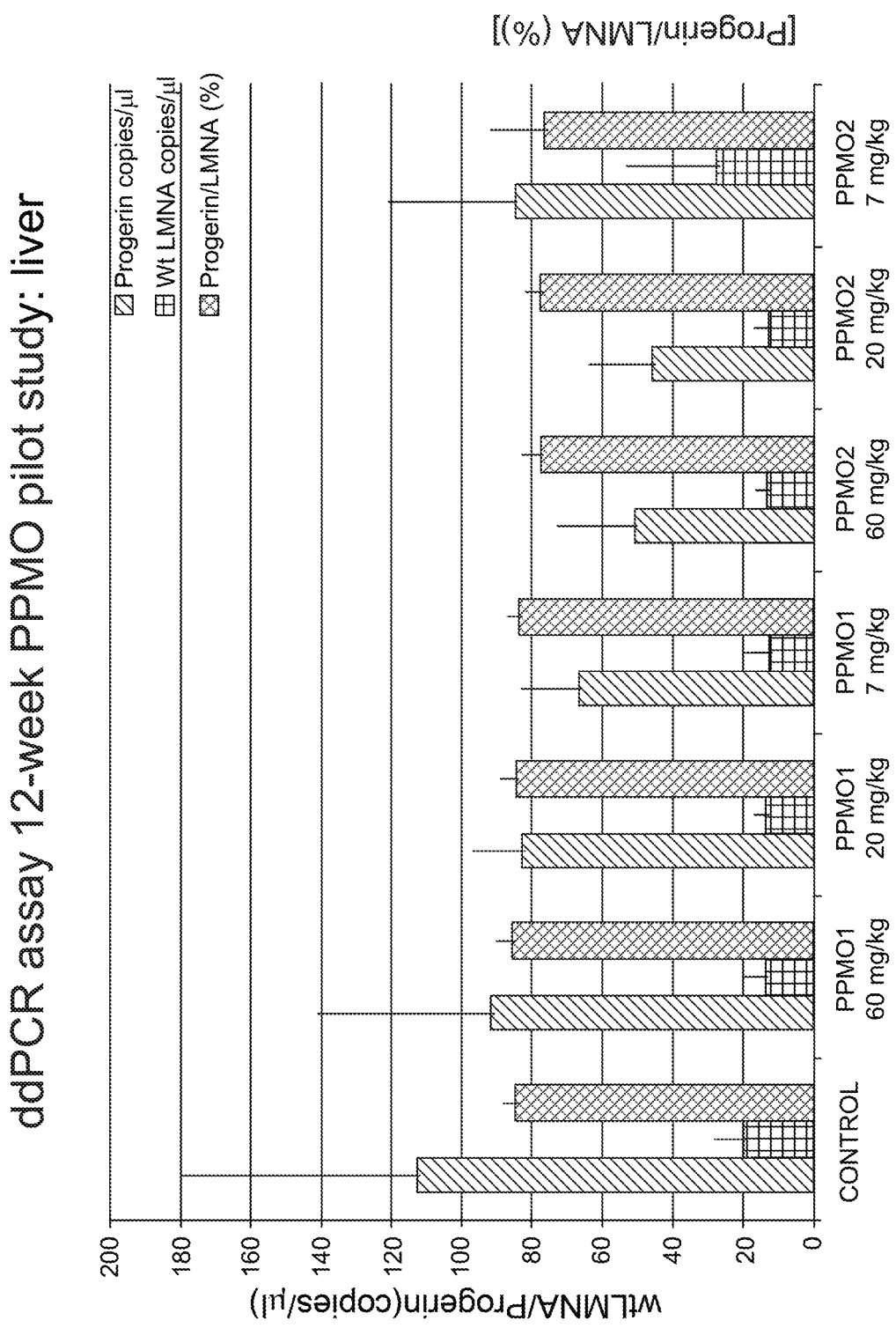

ddPCR assay 12-week PPMO pilot study: liver
Progerin copies/µl
Wt LMNA copies/µl
Progerin/LMNA (%)
wtLMNA/Progerin(copies/µl)
[Progerin/LMNA (%)]
0
20
40
60
80
100
120
140
160
180
200
CONTROL
PPMO1 60 mg/kg
PPMO1 20 mg/kg
PPMO1 7 mg/kg
PPMO2 60 mg/kg
PPMO2 20 mg/kg
PPMO2 7 mg/kg

FIG. 4