ABSTRACT

The present invention relates to a small activating nucleic acid molecules and uses thereoffor treating diseases and conditions, such as thrombocytopenia, related to THPO protein deficiency or insufficiency. As described herein, small activating nucleic acid molecules can be double-stranded or single-stranded RNA molecules targeting the promoter region of the *Thpo/THPO* gene through an RNA activation mechanism and comprise a first nucleic acid strand and a second nucleic acid strand. The double-stranded RNA molecule targeting the promoter region of the *Thpo/THPO* gene comprises two nucleic acid strands of 16 to 35 nucleotides in length, wherein one nucleic acid strand has at least 75% homology or complementarity to a target selected from the promoter region of the *Thpo/THPO* gene. The present invention also relates to pharmaceutical compositions and formulations comprising the small activating nucleic acid molecules and methods for up-regulating the expression of the *Thpo/THPO* gene in a cell and treating diseases and conditions, related to THPO protein deficiency or insufficiency , by administering small activating nucleic acid molecules, pharmaceutical compositions, and formulations thereof.