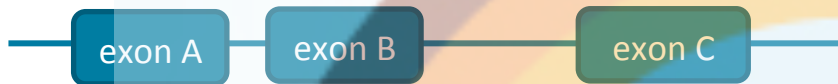


Normal

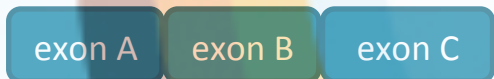
pre-mRNA



Splitting of exons and intron (= splicing)



mRNA



Translation into normal protein



protein



Mutation: pseudo-exon incorporation

Treatment option: exon-skipping

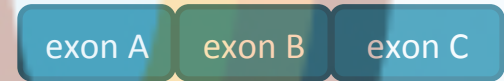
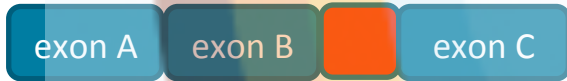
pre-mRNA



Splitting of exons and introns:
an additional exon is incorporated

Additional exon is masked

mRNA

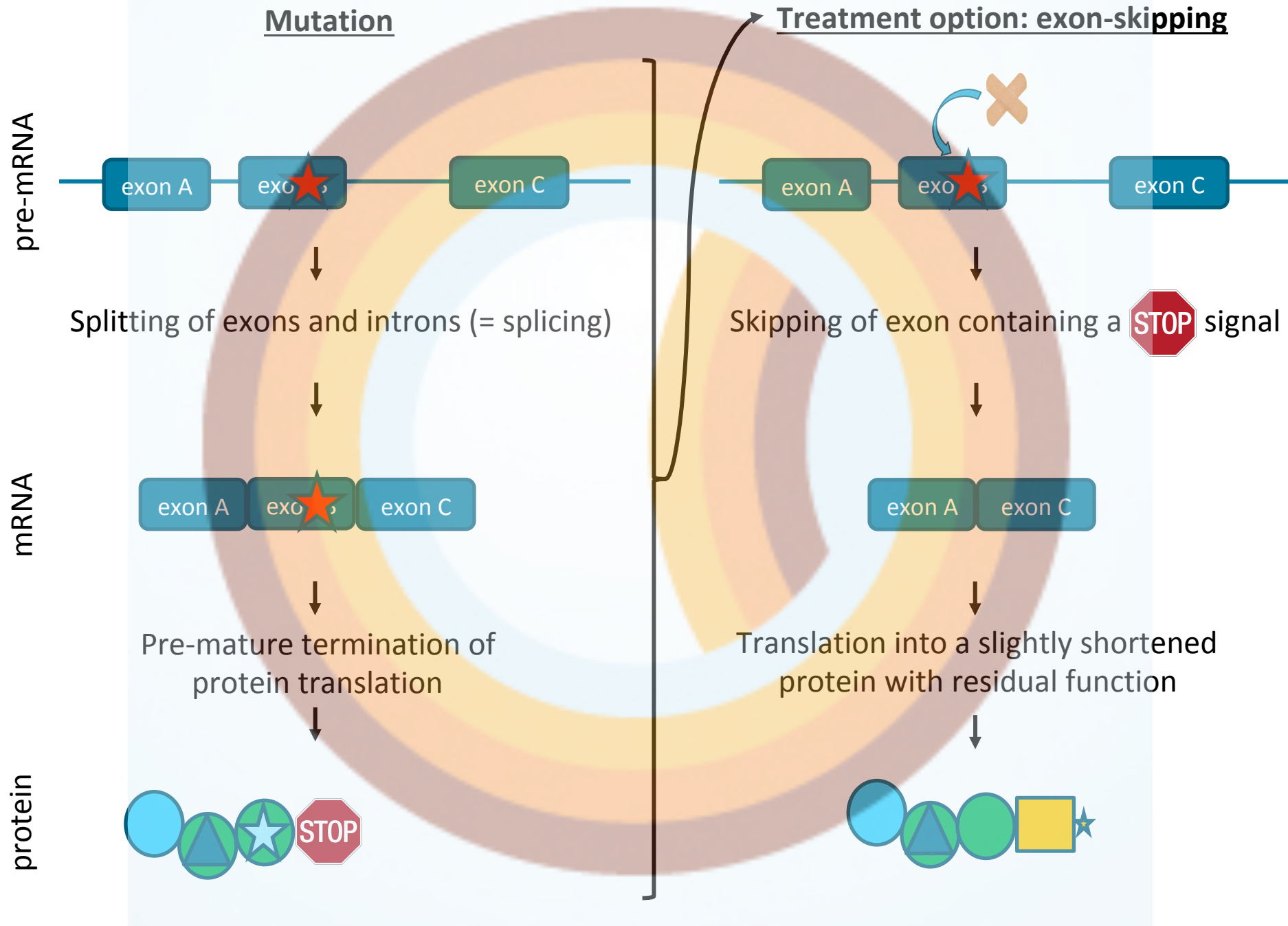


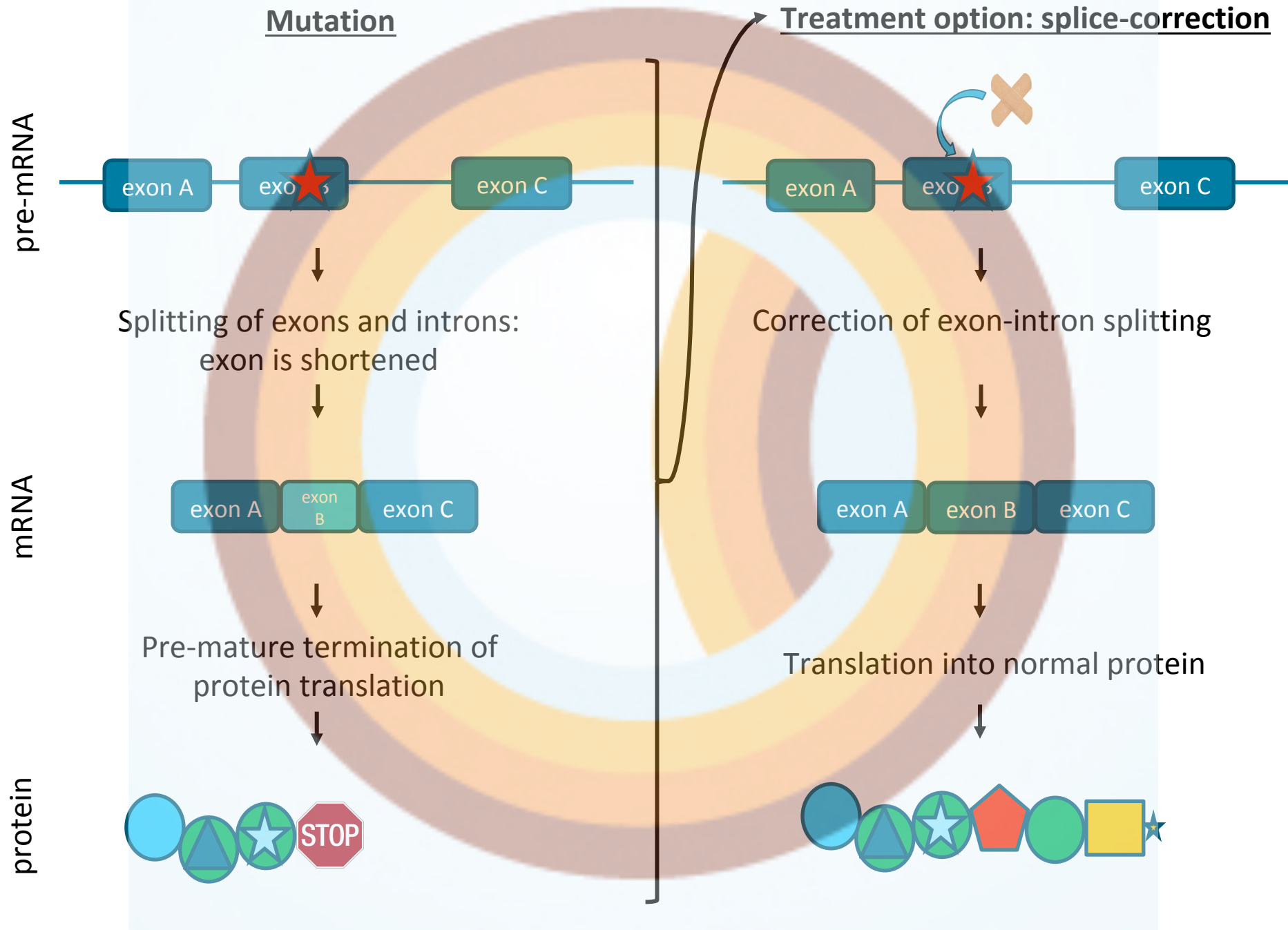
Pre-mature termination of
protein translation

Translation into normal protein

protein







Mutation

Treatment option: splice-correction

pre-mRNA

mRNA

protein

Splitting of exons and introns:
exon is shortened

Correction of exon-intron splitting

Pre-mature termination of
protein translation

Translation into normal protein



