

# **RNA Therapeutics and snoRNA Biology**

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### **Applied Biology: RNA Therapeutics**

Treatment of monogenetic disorders by engineering natural small RNAs to specifically up- or downregulate disease-associated proteins (wet/dry lab projects). Collaboration with BioInnovation Institute, Copenhagen.

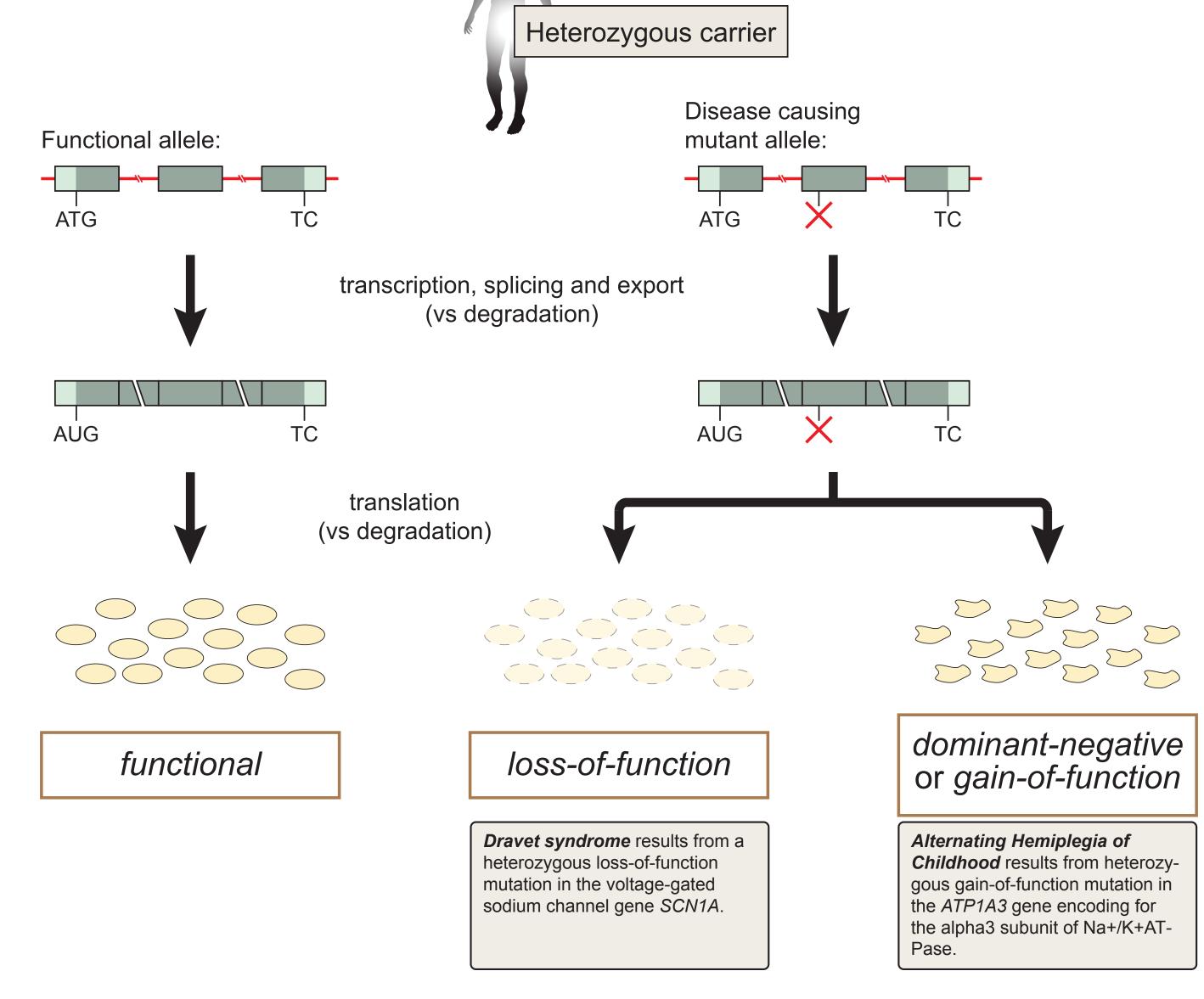
### Basic Biology: small nucleolar RNAs

Autoregulation and beyond: Conventional and non-conventional functions of box C/D small nucleolar RNAs in human cells (wet/dry lab projects).

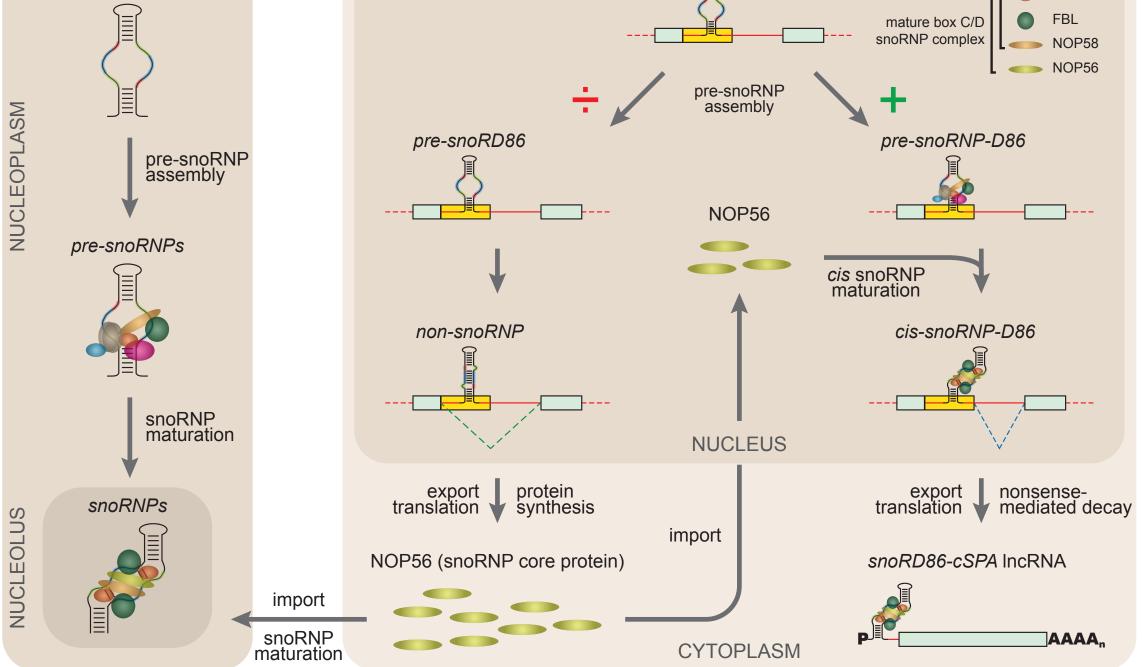
 other box C/D
 NOP56 gene

 snoRNA (host) genes
 box C/D snoRNP

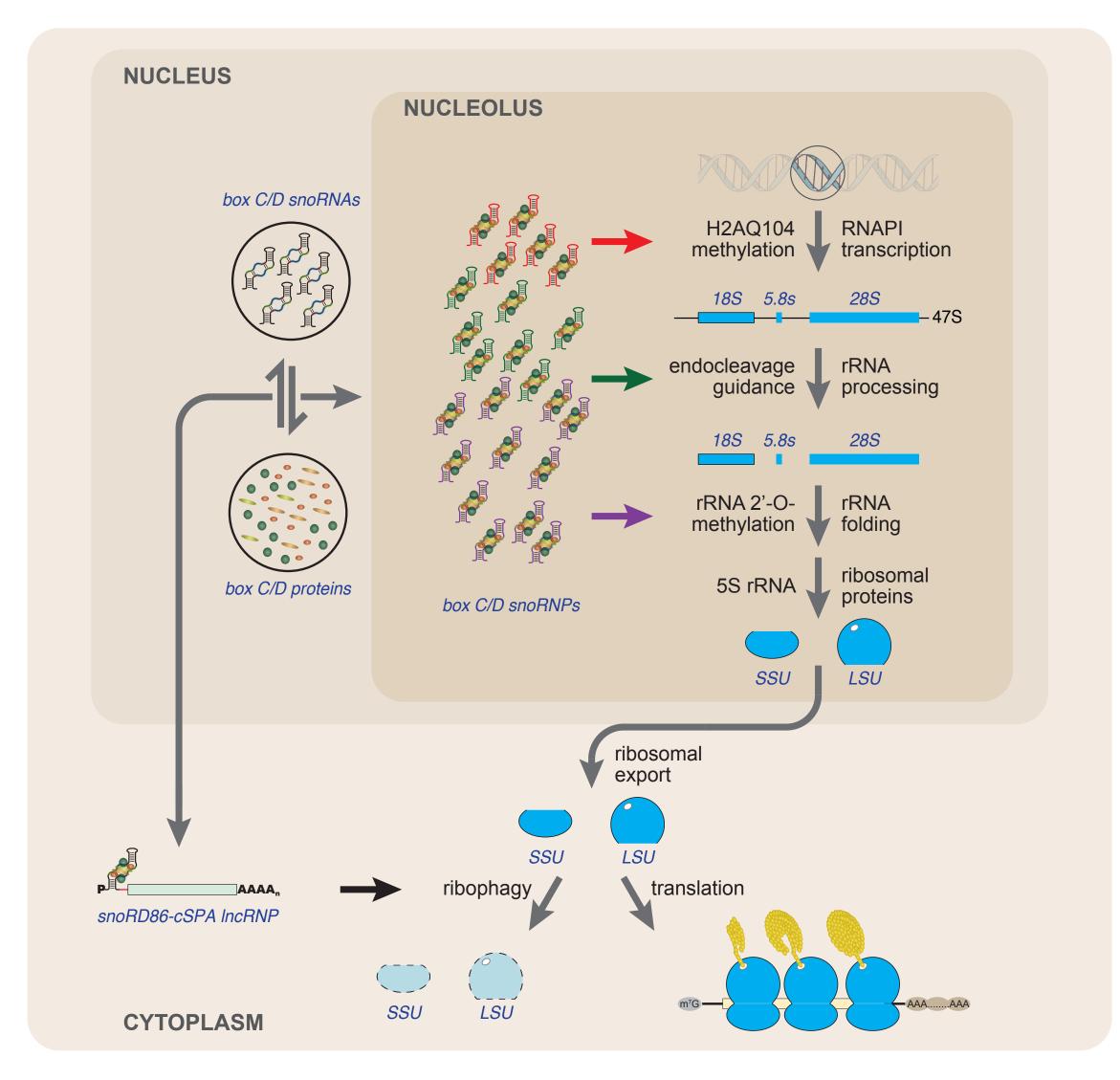
 pre-snoRDs
 image: snoRDs



#### Figure 1 | Disease caused by a heterozygous dominant mutation in



## Figure 3 | Box C/D snoRNP Autoregulation by a cis-Acting snoRNA in the NOP56 Pre-mRNA.



one allele of a gene.

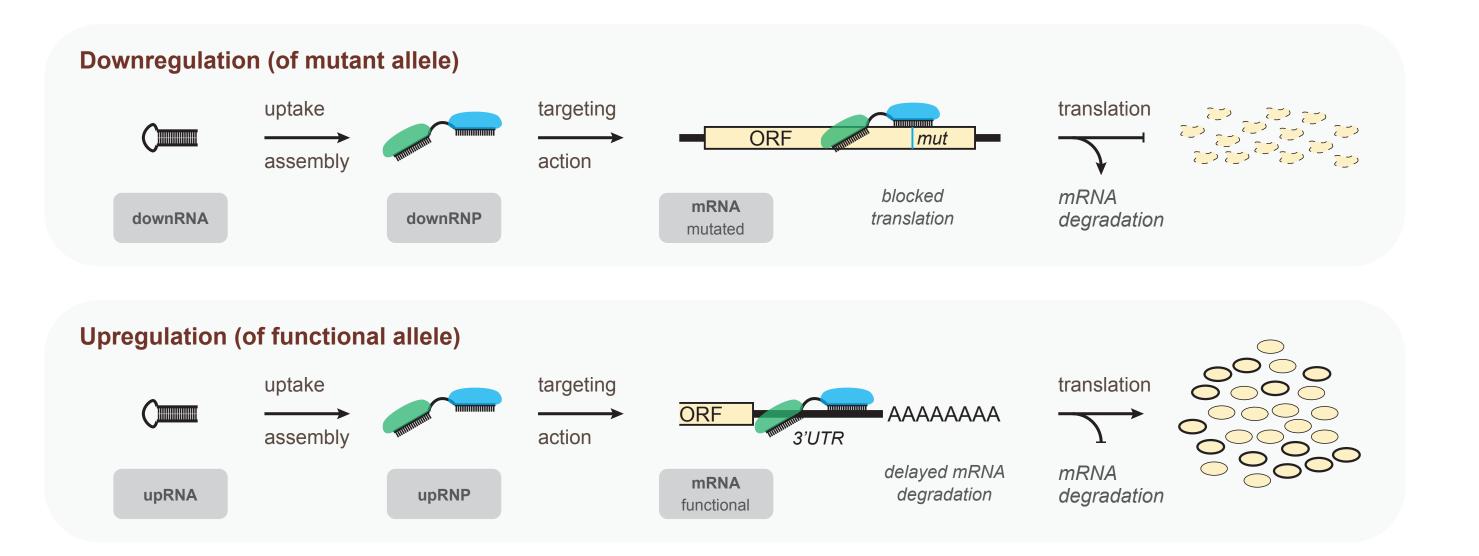


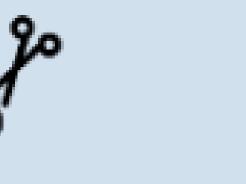
Figure 2 | RNA-based Methods For Targeted Down- or Upregulation of Protein Levels.

Figure 4 | Deciphering novel critical functions of box C/D small nucleolar RNAs in controlling ribosomal metabolism.

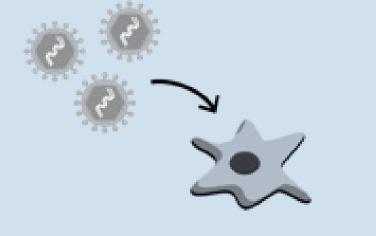
### **Selected Methods**

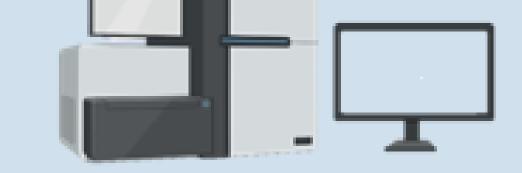












Classic molecular & cellular biology

Fluorescence microscopy CRISPR-based genome editing & screening

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Immuno-precipitation & proximity labeling

Lentiviral screening

Next-generation sequencing

### What snoRD to like?













smartRNA Bio Studio @ BII

