**Course: Advance Bio Informatics**

**Module Title: Target Validation**

**Module No: 132**

**Target Validation**

* A potential target is identified in the context of a specific disease.
* A validated target is the one that can be manipulated with drugs to produce positive clinical effects in humans.

**Requirements for TV**

* **Genetic Approach**  
  Gene to disease correlation in animal model
* **Forward Genetics**
* **Reverse Genetics**

**Target Validation Tools**

Target Validation Tools help in assessing their genetic association with disease. The following tools are:

* Antisense agents
* Ribozymes
* Peptide nucleic acids
* Transcription factors
* Gene knockouts

**Antisense Technology**

Drugs as molecules acting on cellular proteins (enzymes, receptors).

**Downstream:** Block events at nuclear/ribosomal levels.

Prevent expression of aberrant proteins at earlier stage.

Oligonucleotide reagents

**Ribozymes**

Small RNA mol. cleaves other RNA mol. at sequence specific sites.

Hairpin Ribozyme: GUC

**HammerHead:** NUH, H is AUC

Mode of Delivery: Exogenously, Endogenously

**Peptide NAs:** block protein translation

Transcription Factors: Zinc finder proteins – high Affinity to bind to correct region of DNA.

**Gene Knockouts**