



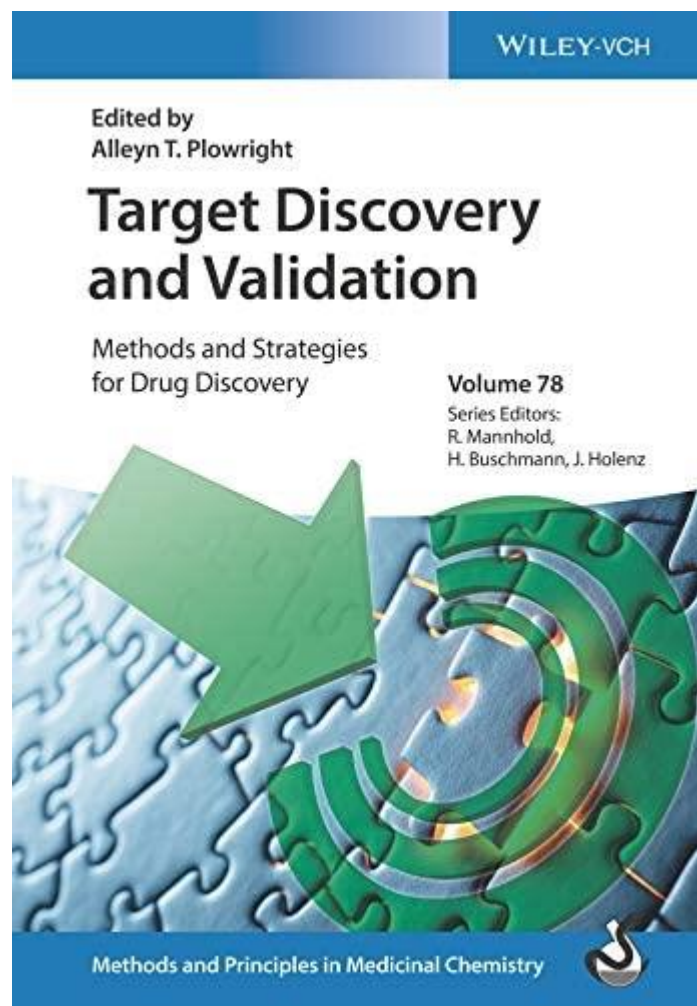
Methods in Drug Development

Target Validation

Step of «target validation» in drug development process



How are drug targets «validated»?



Approaches

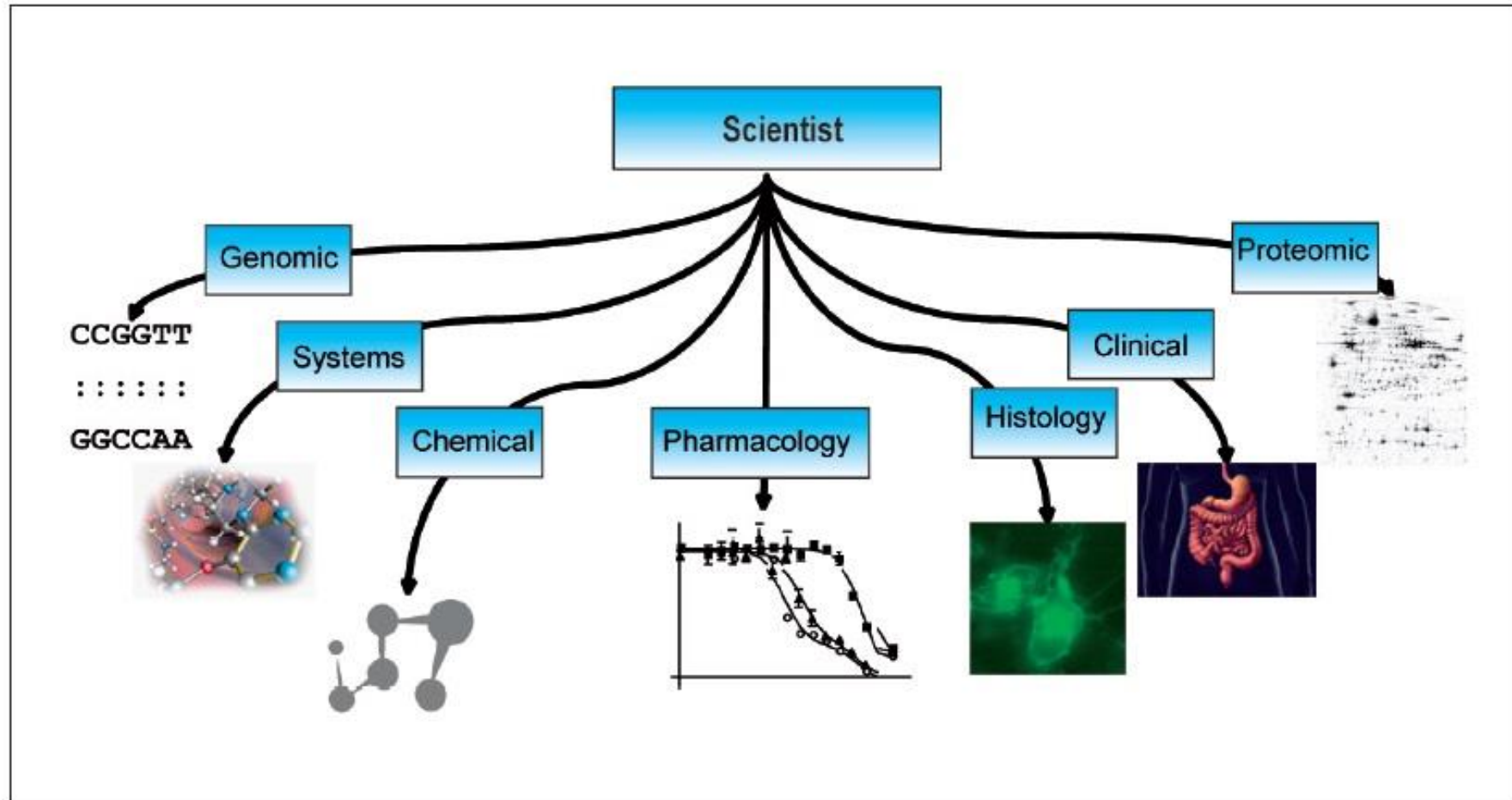
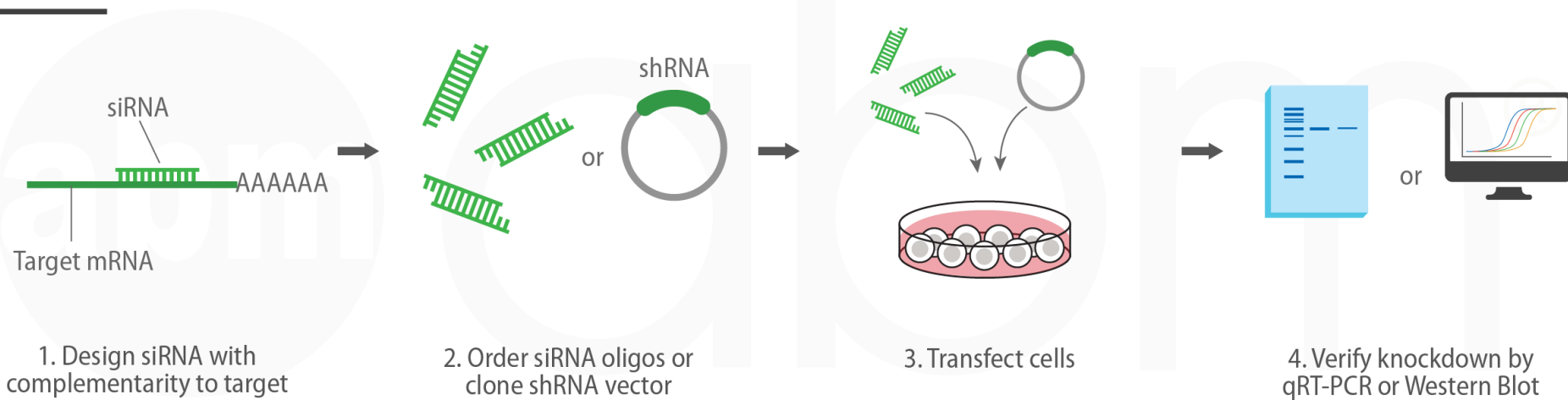


Figure 1 Evaluating a target typically involves searching many different data types

Genomic approaches

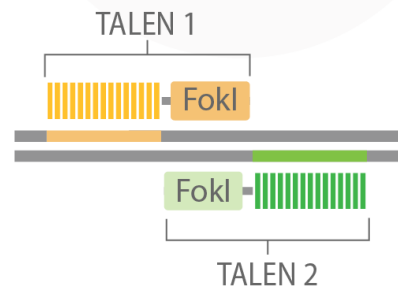
Gene Knockdown Workflow

RNAi

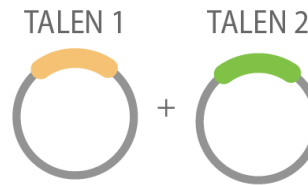


Genomic approaches

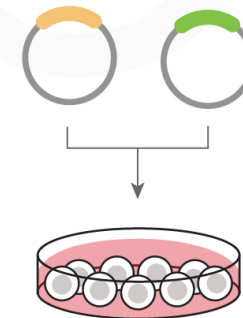
TALEN



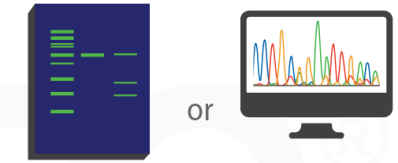
1. Design TALEN DNA-binding domains - each 33 amino-acid repeat corresponds to 1 target nucleotide



2. Order or clone TALEN vectors



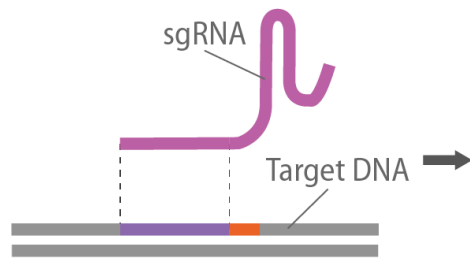
3. Transfect cells



4. Screen & validate for biallelic knockout using Mismatch Cleavage Detection Assay and Sanger Sequencing

Genomic approaches

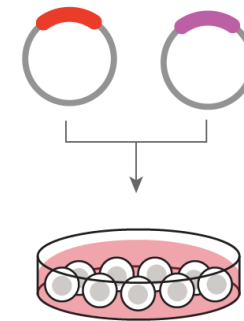
CRISPR



1. Design sgRNA with complementarity to target site upstream of PAM site



2. Order or clone sgRNA and Cas9 vector(s)



3. Transfect cells



4. Screen & validate for biallelic knockout using Mismatch Cleavage Detection Assay and Sanger Sequencing