

Fragment Screening of RNA and Proteins: Exploring a new Target Space for Drug Discovery

Among the translated proteins are only few validated drug targets. A recent estimate based on currently approved drug substances enumerated 218 validated protein targets. The choice of RNA as drug target could be an alternative approach to target biomolecular pathways efficiently. Clinical studies are currently underway with oligonucleotides and small-molecule drug candidates targeting RNA, demonstrating the great potential and feasibility of choosing the RNA target area.

Advances in oligonucleotide drugs have been achieved screening the PAZ domain of human Ago2. The hits were then used to replace the 3'-terminal dinucleotide of a 21-mer siRNA. This had led to clinical candidates for siRNA drugs. Fragment screening has also been used to investigate the possibility of targeting non-coding RNA with small molecules. This study will be discussed and illustrated using several examples.

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