

## Targeting mRNA translation in drug discovery without "shooting the messenger"

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Anima Biotech has developed a platform technology which visualizes and analyzes the kinetics of mRNA translation. The platform includes four components; Translationlight, Brightny, Cerebio and Compass, which identify small molecules that intervene in a novel target space regulating mRNA translation into protein.

mRNA, which is the molecule that relays instructions from DNA to the translation machinery, undergoes a wide range of processing, localization and stabilization, which have evolved to selectively and differentially regulate translation in response to intra-cellular and extra-cellular cues. Multiple layers of regulation are found after mRNA transcription, with nucleotide processing and protein binding occurring co-transcriptionally. Pre-RNA splicing, modification of ribonucleotides and modifications of 5'- and 3'- untranslated regions (UTR), dictate mRNA-protein association which determine mRNA nuclear transport dynamics and mRNA half-life. There are hundreds of RBPs which play an integral part in these regulatory events. mRNA selectivity and tissue specificity are conferred by tissue specific expression of RBPs and their regulatory enzymes, enabling differential regulation of the same mRNA in different tissues. Additionally, diverse families of enzymes and accessory proteins modify and bind to ribosomes, resulting in different populations of ribosomes that are responsible for translation of specific mRNA, or a group of mRNAs which are found in a shared pathway or complex.

Anima has identified novel MoAs and proteins that are targeted by its lead molecules across several disease indications, including fibrosis, cancer, neurodegeneration and viral diseases. These compounds do not directly bind to mRNA or degrade it, but rather intervene at the action of proteins which modulate mRNA fate in a tissue selective or disease-specific manner.