Docket #: S23-213

Detecting the Activity of RNA-modulating Drugs Using ADAR Editing

There is broad potential to modulate RNA using small molecules, replacing more costly and difficult-to-administer oligonucleotide therapies. However, methods for screening for such small molecules are lacking. Existing methods have a number of challenges including reporting only on binding (rather than the functional effects of the small molecule); being costly and time-consuming; and/or may require knocking-in a reporter cassette that may fail to recapitulate expression in the native context.

Stanford researchers have developed a fast, inexpensive, high-throughput method for screening small molecules for binding and functional activity in altering a target RNA in a cell using "adenosine deaminase acting on RNA" or "ADAR" editing.

Stage of Development
In vitro

Applications

- Screening small molecules in a high-throughput manner
- Determine PK/PD and biodistribution

Advantages

- Reports on binding to RNA as well as functional activity of the molecule
- Reports on RNA modulation in its native context
- Reports on Non-coding RNAs
- High-throughput
- Inexpensive

- Fast readout

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