A Genome Editing Lead Finding Platform for Therapeutics


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Background

- Editas Medicine is a leading genome editing company that is translating new genome editing technologies into therapeutics. To this end, we have developed a flexible lead finding platform to identify and characterize highly active and specific genome editing agents. Hundreds of RNA – protein complexes (RNPs) are assayed directly in primary cells at any locus. Genome editing rates are measured with next-gen sequencing using targeted PCR amplification. This process is equally applicable to all RNA guided nucleases including Cas9 and Cpf1 orthologues and variants.

- Targeted amplification and sequencing, while a broadly used tool in the editing field, has critical limitations due to being anchored by two PCR primers. This includes a size bias making large insertions and deletions poorly detected and unexpected translocation events undetectable. To eliminate these challenges we have developed a uni-directional targeted screening methodology, UDiTaS, that is rapid, quantitative, removes bias associated with variable length PCR amplification, and is capable of measuring large deletions and translocations as well as more typical indels.

Conclusions

- Screening and optimization platform developed for unbiased investigation of RNA-Protein complex (RNPs) in cell lines and primary cell models

UDiTaS is a uni-directional targeted screening method useful for simultaneous measurement of small genome edits and the junctions of larger chromosomal rearrangements.