Technology Offer

Small molecules as RNA-binding protein inhibitors

Innovative Azetidine-Based Small Molecules

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RNA plays a vital role in gene expression regulation, and its dysregulation is linked to diseases such as cancer, neurodegeneration, and viral infections. Targeting RNA-binding proteins or RNAs, such as microRNAs (miRNAs) and mRNAs, represents an emerging area in drug discovery. However, traditional RNA-binding small molecules often rely on linear hydrophobic aromatic scaffolds, which limit chemical diversity and therapeutic potential. This invention introduces azetidine-based small molecules, which take advantage of the unique steric rigidity and structural properties of a four-membered azetidine core. This scaffold provides a foundation for novel RNA-binding compounds by enabling the attachment of functional groups that improve binding efficiency and selectivity.

Technology

Researchers from the Chemical Genomics Center of the Max-Planck Society have developed a platform for constructing RNA-binding molecules centered on the azetidine core scaffold. By attaching specific aromatic RNA-binding fragments to the azetidine core, these compounds expand the chemical space, enabling customizable libraries and the development of therapeutically relevant molecules.

The key advantages of this method include:

- **Innovative Chemical Space**: Expands beyond conventional scaffolds with the azetidine core, unlocking novel RNA-binding possibilities.
- **High Customizability**: Flexible attachment of RNA-binding fragments tailored for specific applications.
- **Enhanced Efficacy**: Demonstrated potential to improve RNA-binding selectivity and potency.
- **Broad Applicability**: Suitable for drug development in oncology, virology, and neurodegeneration.

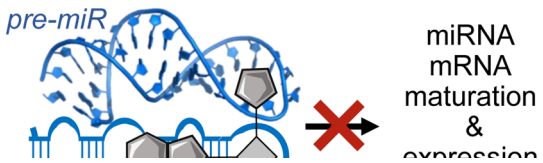

Figure 1 Schematic illustration of a small molecule with an azetidine core binding the RNA and inhibition RNA maturation and expression

Patent Information

Patent Application PCT/EP2023078937, filed on 18th of October, 2023

Opportunity

We welcome **research partnerships** and **licensing** to advance this method toward clinical application and are interested in developing novel RNA-targeting therapies that offer improved safety profiles and efficacy.

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