

Wednesday, 13<sup>th</sup> January, 9.30am, Online

Host: Dr. Aitziber L. Cortajarena

## **RNA-processing in health and disease: new experimental and therapeutic approaches**

*Lorea Blazquez García, PhD.*

*Ramon y Cajal and Ikerbasque Research Fellow*

*Neurosciences Area*

*IIS Biodonostia - OSI Donostialdea*

RNA processing, including splicing and polyadenylation, is a fundamental mechanism that strongly influences gene expression and contributes to disease. Emerging evidence indicates that mutations in non-coding sequences and alterations in trans-acting factors are involved in multiple human diseases by affecting the RNA-processing pattern of genes. Understanding misregulation of RNA-processing in human diseases is important to obtain a definitive molecular diagnosis but also to develop RNA-therapeutics strategies, which represent a powerful approach for several diseases. In the present talk, I will discuss current methodologies to study misregulation of RNA-processing and how we can employ that information to develop therapeutic strategies based on the use of antisense RNAs. These include synthetic antisense oligonucleotides (ASOs), which bind to complementary RNAs and modify their processing and more recently, RNA-targeting CRISPR-Cas systems which can ease the design of efficient antisense molecules and can overcome current limitations with ASO technology