



KEMIJSKI INŠTITUT

Vabilo na Forum40 / Invitation to the Forum40

**Dr. Petra Sušjan Leite**

D12, Department of Synthetic Biology and Immunology

Četrtek / Thursday, 30. 11. 2023 ob / at 13:00

Velika predavalnica Kemijskega inštituta / Great Lecture Hall

**WEBEX**

<https://ki-ljubljana.webex.com/ki-ljubljana/j.php?MTID=m38b214f071022d40752670d7fbf1b881>

Join by meeting number (access code): 2784 449 4411

Meeting password: isW42xu2Bf9

## Therapeutically relevant strategies for CTNNB1 gene upregulation

CTNNB1 gene encodes  $\beta$ -catenin, a multifunctional protein regulating the Wnt target gene transcription that is critically important during early embryonic development. Insufficient levels of  $\beta$ -catenin due to mutations in one copy of the gene lead to CTNNB1 syndrome, a rare neurodevelopmental disorder which manifests in a host of cognitive and motoric impairments of various severity. We will discuss some of the strategies we are employing and optimizing in an attempt to increase CTNNB1 expression and activity, including antisense oligonucleotides and spliceosome-mediated RNA trans-splicing, which could serve as the basis for the development of antisense therapy to ameliorate  $\beta$ -catenin deficiency.



Vljudno vabljeni / Kindly invited