OIST OKINAWA INSTITUTE OF SCIENCE AND TECHNOLOGY
沖縄科学技術大学院大学

VISITING PROGRAM

TSVP TALK

Starting on a Low Note:
Playing with Kozak Sequences to Cure Dosage-Sensitive Gene Diseases

Rescheduled

2025 FRI. **Jul. 04**

14:00–15:00

HYBRID L5D23, ZOOM

For zoom and other details scan QR code or visit oist.jp/visiting-program

The age of genome editing—the recent breakthrough that enables rewriting genetic sequences in entire organisms—has opened a Pandora's box of therapeutic possibilities and potential misuses. For the seven thousand known single-gene disorders, most of which are currently incurable and many life-threatening, this rewriting could mean correcting the underlying mutations. Yet not all mutations are amenable to genome editing, and for many disorders, the number of distinct pathogenic variants can reach dozens or even hundreds, making direct correction unfeasible. The story we tell begins with a geneticist who, in the 1980s, identified a sequence present in all genes that is essential for protein production. It also begins with an evolutionary biologist who, in the same period, challenged the adaptationist dogma dominating evolutionary theory. We will show that in the age of genome editing, starting on the low note of a suboptimal evolutionary product may lead to an unexpected therapeutic strategy for a broad class of genetic diseases.

Alessandro Quattrone — University of Trento

Alessandro Quattrone is a full professor at the University of Trento, Italy, where he teaches Molecular Pathology. From 2007 to 2021, he served as the founding director of the Department for Cellular, Computational, and Integrative Biology at the same university. Prior to that, he was a visiting scientist at the US National Institutes of Health and held associate professorships at Johns Hopkins University and West Virginia University. Early in his scientific career, he pioneered the development of antisense oligonucleotide technology and discovered one of the first long noncoding RNAs implicated in cancer. His current research focuses on the preclinical development of therapeutic solutions for rare neural tumors and rare neurodegenerative diseases. He targets models of these conditions by modulating translational control and epitranscriptomic signaling with small molecules and gene editors. In addition to his academic pursuits, he is a science communicator and the founder of biotech startups.

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