### 2023 年度

# 基盤医学特論 開講通知

Information on Special Lecture Tokuron 2023.4-2024.3

## Title: Engineered CRISPR systems for disease treatment & diagnostics

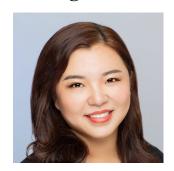
Teaching Staff: Xue (Sherry) Gao, PhD

T.N. Law Assistant Professor

Chemical and Biomolecular Engineering, Rice University

Date: November 28 (Tue.), 2023 17: 00~(90 minutes)

日時:令和5年11月28日(火) 17時00分より(90分)



Room: Meeting Room 2, (Basic Medical Research Building, 1st floor)

場所:基礎研究棟1階 会議室2

Language: English 言語: 英語 (事前の申込みは不要です。No Registration Required.)

### Abstract:

The recent discovery of the CRISPR genome editing systems has been revolutionizing both basic biological research and the treatment of human genetic disorders. However, there are remaining challenges in improving the precision and multiplexity of the current CRISPR systems for genome manipulation. In this seminar, I will overview our recent development of highly specific and powerful genome-editing tools for the treatment and diagnostics of diseases and the discovery of new potential small-molecule drugs. First, I will introduce the development of high-precision and multiplex CRISPR genome-editing strategies for safe and effective molecular therapy to treat genetic disorders; Second, I will describe the application of these advanced CRISPR tools for fungi genome engineering to enable the production of novel small molecules with therapeutic potential; Finally, I will present the improved ultrasensitive viral detection by using the engineered CRISPR systems to advance timely and accurate diagnostics for the current and future pandemics and epidemics.

#### References:

- 1. Yang, J †, Song, Y †,..., Gao, Y\*, Zhang, Y\*, and Gao, X\*. Engineered LwaCas13a with enhanced collateral activity for nucleic acid detection. *Nature Chemical Biology*. 2023.
- 2. Yuan Q, Gao X\*. Multiplex base- and prime-editing with drive-and-process CRISPR arrays. *Nature Communications.* 2022,13(1): 2771.
- 3. Lee S, Ding N, Sun Y, ..., Zuo E\*, Gao X\*. Single C-to-T substitution using engineered APOBEC3G-nCas9 base editors withminimum genome- and transcriptome-wide off-target effects.

**Science Advances.** 2020, 6(29):

4. Gao X, Tao Y, Lamas V, et al. Treatment of autosomal dominant hearing loss by in vivo delivery of genome editing agents. *Nature*. 2018, 553(7687): 217-221.

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