

1st Joint Seminar of Center for Gene and Cell Therapy and Center for Stem Cell  
Biology Co-hosted by International Joint Usage/Research Center

Seminar Date and Time: 12/01/2020 (Tue) 17:00 ~ 18:15

Venue: online by Zoom

If you would like to attend this seminar, please contact the secretariat address on the poster.

Speaker (Name): Naoya Uchida

Affiliation, Title: Associate Professor, Division of Molecular and Medical Genetics, Center for Gene and Cell Therapy, IMSUT

Country: Japan

Subject: Hematopoietic stem cell-targeted gene therapy for sickle cell disease

Abstract: Sickle cell disease (SCD) is caused by a point mutation in the  $\beta$ -globin gene, resulting in anemia, pain, and organ damage. SCD can be cured by allogeneic hematopoietic stem cell (HSC) transplantation; however, a suitable donor is found for ~10% of patients. Therefore, we focused on development of HSC-based therapy with genetic modification to cure SCD. In HSC-targeted gene therapy, normal  $\beta$ -globin gene is added to patient HSCs with a lentiviral delivery, allowing for one-time cure of SCD. Recently, genome editing technologies were generated, and it allows us to develop gene correction of the SCD mutation in the  $\beta$ -globin gene for patient HSCs. In this seminar, HSC-targeted gene addition/gene correction therapy for SCD will be presented. (This seminar is co-hosted by Center for Gene and Cell Therapy and Center for Stem Cell Biology and Regenerative Medicine)

Language: Japanese

Organizer (Host Researcher): Naomi Okada, Hideki Taniguchi, Atsushi Iwama

[Poster](#)