

MODALIS

MAY 16TH

E - DISEASE MODELS AND CLINICAL APPLICATIONS -> MUSCULO-SKELETAL DISEASES

30: Novel Single AAV Vector Treatment for Congenital Muscular Dystrophy Type 1A (MDC1A) Using CRISPR-GNDM® Technology

Yuanbo Qin, Talha Akbulut, Rajakumar Mandraju, Keith Connolly, Payal Pranami, Claudia Foster, Vandhana Chezhiyan, Alison Shottek, Tetsuya Yamagata

Type: Oral Abstract Session

Session Title: Musculo-skeletal Diseases

Location: Salon G

Time: 5/16/2022 10:30-10:45

H - CLINICAL TRANSLATION OF GENE AND CELL THERAPIES -> VECTOR PRODUCT ENGINEERING, DEVELOPMENT OR MANUFACTURING

49: NGS Based Evaluation of AAV Genome Integrity for Improved Production and Function

Keith Connolly, Vandhana Chezhiyan, Daniel Ferguson, Thomas Buttrick, Yuanbo Qin, Payal Pranami, Ahmad Alsomali, Tetsuya Yamagata

Type: Oral Abstract Session

Session Title: Vector Manufacturing and Engineering 1: Deciphering AAV Vector Genomes

Location: Ballroom A

Time: 5/16/2022 11:45 - 12:00

E - DISEASE MODELS AND CLINICAL APPLICATIONS -> NEUROLOGIC DISEASES

260: Robust Suppression of Tau by CRISPR-GNDM® System for Treatment of Tauopathies

Talha Akbulut, Claudia Foster, Iain Thompson, Christy Hoong, Prerana Pathak, Tetsuya Yamagata

MODALIS

Type: Poster Session

Poster Board Number: M-141

Session Title: Neurologic Diseases I

Location: Hall D

Time: 5/16/2022 17:30 - 18:30

MAY 17TH

E - DISEASE MODELS AND CLINICAL APPLICATIONS -> CARDIOVASCULAR AND PULMONARY DISEASES

619: Utilizing CRISPR-GNDM® Mediated Gene Activation of the Extra-Large Gene Titin for the Treatment of Dilated Cardiomyopathy and Other Titinopathies

Daniel Ferguson, Keith Connolly, Thomas Buttrick, Yuanbo Qin, Tetsuya Yamagata

Type: Poster Session

Poster Board Number: Tu-124

Session Title: Cardiovascular and Pulmonary Diseases

Location: Hall D

Time: 5/17/2022 17:30 -18:30

MAY 18TH

E - DISEASE MODELS AND CLINICAL APPLICATIONS -> NEUROLOGIC DISEASES

1027: Blocking SNHG14/UBE3A-ATS lncRNA Transcription with Dead Cas9 (CRISPR-GNDM®) Can Un-Silence Paternal UBE3A in an Angelman Syndrome Mouse Model

Barbara J. Mann, Talha Akbulut, Nicole Pandell, Claudia Foster, Elani Hillman, **Tetsuya Yamagata**

MODALIS

Type: Poster Session

Poster Board Number: W-153

Session Title: Neurologic Diseases III

Location: Hall D

Time: 5/18/2022 17:30-18:30

F - IMMUNOLOGICAL ASPECTS OF GENE THERAPY AND VACCINES -> IMMUNOLOGICAL ASPECTS OF GENE THERAPY AND VACCINES (INCLUDES HOST RESPONSES, THERAPY/PREVENTION OF INFECTIOUS DISEASES; EXCLUDES CANCER IMMUNOTHERAPY AND CANCER VACCINES)

1126: Evaluation of Cas9 Mediated Immune Response Effect on Long Term Transgene Expression in WT Mice and NHPs without Immune Suppressant

Rajakumar Mandraju, Talha Akbulut, Yuanbo Qin, Max Seidman, Gurudivija Thommandru, Claudia Foster, Payal Pranami, Tetsuya Yamagata

Type: Poster Session

Poster Board Number: W-252

Session Title: Immunological Aspects of Gene Therapy and Vaccines II

Location: Hall D

Start Time: 5/18/2022 17:30 - 18:30

Abstract



Poster (available after ASGCT presentation)

