

**Sept 15th – Sept 18th, 2024
in Boppard am Rhein**

**“THE ERA OF HUMAN GENE THERAPY
– PROSPECTS AND CHALLENGES”**

Our aim is to train and educate a new generation of young scientists in the exciting and rapidly growing field of human gene therapy, and to foster their interaction by establishing world-wide networks and critical infrastructure. The 2024 HBIGS Summer school addresses this need by bringing together international leaders in relevant research fields and by enabling the participating PhD students to directly and actively interact with these experts as well as with each other. The invited speakers not only comprise leaders in biological sciences, such as the two organizers who have collaborated and contributed to gene therapy technologies for over a decade, but also experts in virotherapy, vaccine development, disease models, regulatory aspects & clinical translation.



Location:

Hotel „Das Ebertor“

Heerstraße 172

56154 Boppard

<https://www.das-ebertor.de/>



Dirk
Grimm

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Dominik
Niopek

Program overview

Sun. Sept 15th	Mo. Sept 16th	Tue. Sept 17th	Wed. Sept 18th
16:00 – 18:00 Registration (Hotel Ebertor)	09:00 – 10:30 Session 1	09:00 – 10:30 Session 5	09:00 – 11:15 Session 7
	10:30 Coffee break	10:30 Coffee break	11:15 Coffee break & farewell
	10:45 – 12:45 Session 2	10:45 – 12:45 Session 6	
	12:45 Lunch	12:45 Lunch	
	14:30 – 16:30 Session 3	16:00	
	16:30 Coffee break	Boat cruise on the Rhine	
	16:45 – 18:15 Session 4		
19:30 Dinner	19:30 Dinner	19:30 Dinner	

Scientific Program

Monday September 16th

Session 1:

Chair:

09:00 – 09:30 WELCOME & INTRODUCTION Prof. Dr. Dirk Grimm & Prof. Dr. Dominique Niopek

09:30 – 10:00 (A) Kühne, Patrizia
Dept. of Cardiology “Investigating the metabolic role of free PRAS40 in the Heart Failure with preserved Ejection Fraction (HFpEF) mouse model”

10:00 – 10:30 B) Hildebrand, Heinz-Georg
Dept. of Infectious Diseases “Engineering of myotropic AAV vectors for expression of broadly neutralizing anti-HIV-1 antibodies”

10:30 – 10:45 Coffee break

Session 2:

Chair:

10:45 – 11:15 (C) Holzleitner, Noah
TU Munich “Application of Deep Learning for Engineering of Miniature CRISPR-Cas Proteins”

11:15 – 11:45 (D) Fischer, Nico
Dept. of Infectious Diseases “Assessment and progression of AAV-based CRISPR/Cas Type VI-D or RNAi-based effector systems to combat CHB-triggered CD8+ T cell exhaustion”

11:45 – 12:15 (E) Lashkari, Ali
Biochemistry Center (BZH) “A Possible Role for Plasma Membrane Nano-domains in Unconventional Secretion of Fibroblast Growth Factor 2”

12:15 – 12:45 (F) Brenker, Luca
Institute of Pharmacy and Molecular Biotechnology “A multimodal anti-CRISPR effector regulation for opto- and chemogenetic control of CRISPR-Cas9 and Cas12 inhibition across a wide range of orthologs”

12:45 – 14:30 Lunch break incl. Poster Session

Session 3:		Chair:
14:30 – 15:00	(G) Bergamino, Mariano Clinical pharmacology and pharmacoepidemiology	“Study design of the LipOra Trial”
15:00 – 17:15	Poster Session incl. Coffee break	
	(1) Bhunia, Sayari Pharmacological Institute HD	“Optimisation of advanced gene editing techniques and their application in the treatment of cardiovascular diseases”
	(2) Southern, Nicholas Institute of Pharmacy and Molecular Biotechnology	“Phage-assisted evolution of chimeric chemogenetic and optogenetically switchable proteins”

Session 4:		Chair:
	(3) Lakkaraju, Sricharan Institute for Experimental Cardiology	“NT-HDAC4 Gene therapy : cardioprotection”
	(4) Gupta, Lavanya Dept. of Vascular Dysfunction Mannheim	“Identifying novel cysteinolytic regulators regulating stemness “
17:15 – 18:00	Blank, Antje Clinical pharmacology and pharmacoepidemiology	“From bench to bedside: clinical translation in practical terms”
19:30	Dinner	

Tuesday September 17th

Session 5:		Chair:
09:00 – 09:45	Kunz, Joachim Pediatric Oncology, Hematology and Immunology Heidelberg	“Gene therapy of hemoglobinopathies”
09:45 – 10:30	Grünewald, Julian Technical University of Munich	“Engineering and clinical application of CRISPR technologies in cardiovascular medicine”
10:30 – 10:45	Coffee break	
Session 6:		Chair:
10:30 – 11:15	VandenDriessche, Thierry	“Turbocharging gene therapy by boosting tissue-

	University of Brussels	targeted transcription”
11:15 – 12:00	Lächelt, Ulrich University of Vienna	“Non-viral delivery of nucleic acids”
12:00 – 12:45	Ungerechts, Guy NCT Heidelberg	“Immunovirotherapy – Clinical Translation”
12:45 – 14:00	Lunch break & Foto of all participants	
14:45 – 15:30	Haar, Janina – ONLINE Revvity Germany	“A scientific journey at the forefront of next-generation gene therapy vector development”
16:00	Boat cruise on the Rhine	
19:30	Dinner	

Wednesday September 18th

Session 7:

Chair:

09:00 – 09:45	Fakhiri, Fakhiri - ONLINE Hoffmann-La-Roche	“Mission: possible – How science and coffee bridged and fueled my journey to big biotech”
09:45 – 10:30	Vandenberghe, Luk Harvard Medical School	“Where the shoe fits (and doesn’t) – translating biomedical innovation in genetic medicine“
10:30 – 11:15	Backs, Johannes Cardiology Heidelberg	“Cardiometabolic Disease – from bench to bed”
11:15 – 12:00	Coffee break and farewell	