



**UNIVERSITÄTS
KLINIKUM** **FREIBURG**

XXIVth

ANNUAL MEETING DG-GT

GERMAN SOCIETY
FOR GENE THERAPY

FREIBURG
26.-28.09.2018



Institute
Transfusion Medicine
& Gene Therapy



DG-GT e.V.
Deutsche Gesellschaft
für Gentherapie e.V.



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Dear Friends and Colleagues,

on behalf of the Organizing Committee of the **German Society for Gene Therapy** (DG-GT), I cordially welcome you to the **XXIVth Annual Meeting** of our Society **in Freiburg**.

These are exciting times for gene and cell therapy. Several scientific breakthroughs in human gene therapy, genome editing and cancer immunotherapy have been successfully translated to the clinic in the last few years, and many more will follow for the benefit of severely diseased patients.

At this year's Annual Meeting, top researchers from all over the world will present their latest results and their cutting-edge developments in cell and gene therapy from the **26th to 28th of September 2018**. The conference will have a special focus on the technological advances made in iPS-derived cell therapies and genome editing as well as the preclinical and clinical implementation of therapeutic approaches to treat genetic disorders, infectious diseases and cancer.

In addition, we have set up a Public Day to inspire and motivate high school students for the exciting topic we are working on.

We all are looking forward to a stimulating conference and are pleased to see you soon in the beautiful city of Freiburg.

Best regards,

Toni Cathomen
(on behalf of the Organizing Committee)

Scientific Committee:

Prof. Dr. Hildegard Büning
Prof. Dr. Toni Cathomen
Dr. Tatjana Cornu
Prof. Dr. Boris Fehse
Dr. Zoltán Ivics
Prof. Dr. Christian Kupatt
Dr. Claudio Mussolino
Dr. Dirk Nettelbeck
Prof. Dr. Christof von Kalle

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Dr. Tatjana Cornu
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Dr. Zoltán Ivics
Prof. Dr. Christian Kupatt
Dr. Claudio Mussolino
Dr. Dirk Nettelbeck
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Meeting Venue:

Forum Merzhausen
Address: Marktplatz 4
79249 Merzhausen (Freiburg)

The Forum Merzhausen has an underground parking – fee required. Free parking spots are available next to the Bürgerbad (Friedhofweg 13) in 250 m distance from the venue.

Wi-Fi at the Venue:

Please choose the “FORUM Merzhausen” Network and accept. There is no password required.



Bus shuttle

Wednesday, Sept 26th, 2018

- 12:00 h Freiburg Central Bus Station - Stop No. 4
- 13:00 h Freiburg Central Bus Station - Stop No. 4
- 18:30 h Forum Merzhausen (Venue)
- 19:15 h Forum Merzhausen (Venue)

Thursday, Sept 27th, 2018

- 08:10 h Freiburg Central Bus Station - Stop No. 4
- 08:40 h Freiburg Central Bus Station - Stop No. 4
- 18:45 h Forum Merzhausen (Venue)
- 19:15 h Forum Merzhausen (Venue)

Friday, Sept 28th, 2018

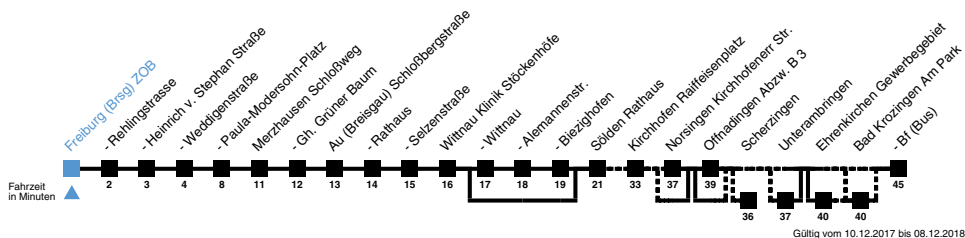
- 08:10 h Freiburg Central Bus Station - Stop No. 4
- 08:40 h Freiburg Central Bus Station - Stop No. 4
- 15:15 h Forum Merzhausen (Venue)
- 15:45 h Forum Merzhausen (Venue)

**In case you missed the shuttle service, please call a cab „Taxi Freiburg“
+49 761 55.55.55 or e-mail dispo@taxi-freiburg.de**

Alternatively, you can use the public bus service – see next page.
(Please note that DG-GT will not cover taxi costs).

7208

Freiburg (Brsg) ZOB → Bad Krozingen Bf (Bus)



Montag - Freitag	Samstag	Sonn- und Feiertag
1	1	1
2	2	2
3-4	3-4	3-4
5	5	5
6	6	6
7	7	7
8	8	8
9	9	9
10	10	10
11	11	11
12	12	12
13	13	13
14	14	14
15	15	15
16	16	16
17	17	17
18	18	18
19	19	19
20	20	20
21	21	21
22	22	22
23	23	23
0	0	0

● = bis Au (Breisgau) Selzenstraße
 ✕ = bis Sölden Rathaus
 ▼ = bis Kirchhofen Raiffeisenplatz
 * = bis Scherzungen
 ◆ = bis Unteranbringen
 + = bis Ehrenkirchen Gewerbegebiet
 a = bedient nicht von Witnau bis Witnau Biezighofen
 b = bedient nicht Norsingen Kirchhofen Str.

c = bedient nicht Offnadingen Abzw. B 3
 d = bedient Unteranbringen
 e = bedient auch von Ehrenkirchen Gewerbegebiet bis Bad Krozingen Am Park
 f = bedient Bad Krozingen Am Park
 g = ab 22.12.17, bis 2.11.18, Montag-Freitag (schulfrei), auch 12.2.18, 13.2.18
 h = Schulltag, nicht 12.2.18, 13.2
 i = Sonntag, nicht 24.12.17
 j = Behindertengerecht

7208

Merzhausen Gh. Grüner Baum → Freiburg (Brsg) ZOB



Montag - Freitag										Samstag		Sonn- und Feiertag	
5	22 _c	47 _b	47 _a							5		5	
6	18 _c	36 _b	36 _a	52 _a	52 _b					6	02 _c	6	12 _c
7	07 _a	08 _a	10 _b	24 _b	24 _{cd}	27 _a	43 _c	56 _b	57 _a	7	11 _c	7	11 _c
8	08 _c	23 _a	27 _b	38 _b	38 _a	51 _a				8	11 _c	8	11 _c
9	13 _c	43 _c								9	11 _c	9	11 _c
10	13 _c	43 _c								10	13 _c	10	13 _c
11	13 _c	43 _c								11	13 _c	11	13 _c
12	13 _c	34 _a	34 _b	43 _b	43 _a					12	13 _c	12	13 _c
13	13 _c	22 _a	53 _c							13	13 _c	13	13 _c
14	13 _c	43 _c								14	13 _c	14	13 _c
15	13 _c	43 _c								15	13 _c	15	13 _c
16	13 _c	23 _a	43 _c	57 _c						16	13 _c	16	13 _c
17	13 _c	27 _c	43 _c	57 _c						17	13 _c	17	13 _c
18	13 _c	27 _c	43 _c	57 _c						18	13 _c	18	13 _c
19	13 _c	43 _c								19	11 _c	19	11 _c
20	11 _c	41 _c								20	11 _c	20	11 _c
21	11 _c	41 _c								21	11 _c	21	11 _c
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● = bis Freiburg Paula-Modersohn-Platz b = ab 22.12.17, bis 2.11.18, Montag-Freitag (schulfrei), auch 12.2.18, 13.2.18 d = in der Regel Niederflurfahrzeug
a = Schultag, nicht 12.2.18, 13.2 c = Behindertengerecht



AWARDS & INITIATIVES

ESGCT awards acknowledge achievements at student level all the way to eminence in longer-standing careers.

Categories include:

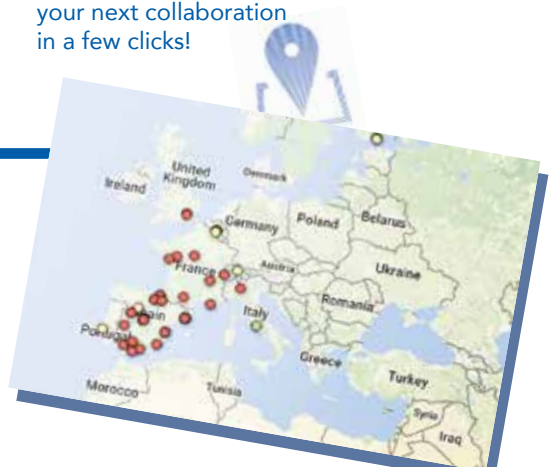
- ESGCT Outstanding Achievement -
- ESGCT Travel Grants for Best Scoring Abstract -
- ESGCT National Society Young Investigator -
(awarded at national society meetings)
- ESGCT Young Investigator -
- Public Engagement Award -
- Mentor -
- Vox Populi: Best Poster -



INTERACTIVE COMMUNITY HEATMAP

The interactive Gene & Cell Therapy Community Heatmap on our website shows where in Europe a growing number of academic institutions, biotech and pharmaceutical companies, non-profits and other organisations are

working to bring new treatments to patients. Learn who to ask for advice or find your next collaboration in a few clicks!



BLOG

Newly launched, the ESGCT blog is written by members and guest bloggers and covers all themes related to gene and cell therapy; from fundamental science to patient and public engagement. Read it on our website and get in touch if you would like to contribute!

FIND OUT MORE: [ESGCT.EU](https://www.esgct.eu)

ESGCT SPRING SCHOOL 2019

Naples, Italy, 3-5 April

- Intensive three-day training course
- Leading researchers
- Debates
- Networking with Europe's current and future leaders in the field
- Support for young researchers
- Free for students
- Very limited spaces for industry and academia

Book from 1st December 2018 to secure one of 150 seats.

www.esgct.eu/Congress/Spring-School.aspx

EUROPEAN SOCIETY OF
GENE & CELL THERAPY



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Social Program

Historix Sightseeing Tour “City, gates and storytellers”

Follow the trusty horsegroom Lambert of Fryeburg (or his charming wife Hiltrud) on a walk through narrow alleyways and secret courtyards. Find out how the old ringwall, the gates and our famous “Bächle” came to be built. What happened when Freiburg came under siege? Who killed Master Konrad? Hear of Butcher Hauri’s ruthless deed and see for yourself what’s left from the Middle Ages.

Meeting Point: Schwabentor, at the stairs leading up to Schwabentorsteg
(see map below)

When: Wednesday, September 26th, 2018 at 8:00 pm.





Social Event

Peterhofkeller

Thursday, September 27th, 2018

7:30 pm Admission and purchase of drink tickets

8:00 pm Dinner | Poster Award | Party with Maddis'son Brass Band und DJane Conny

Peterhofkeller | Niemensstraße 10 | 79098 Freiburg

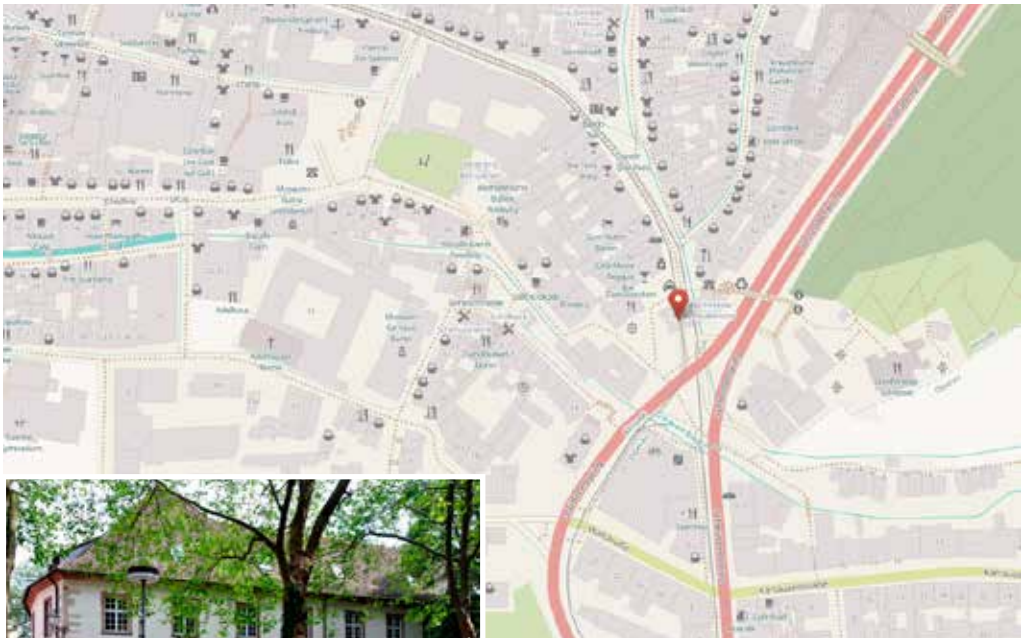


Bild: Andreas Praefcke



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Program Forum Merzhausen

DAY 1 – WEDNESDAY, SEPTEMBER 26, 2018

12:00	14:00	Registration XXIV th Annual Meeting DG-GT
12:30	14:00	Buffet Lunch
Session I - Opening Session Chairs: Christof von Kalle & Toni Cathomen		
14:00	14:15	Introduction: Christof von Kalle, Toni Cathomen
14:15	15:00	Keynote Lecture 1 J. Keith Joung, Boston <i>In vivo CRISPR gene editing with no detectable genome-wide off-target mutations</i>
15:00	15:30	Fulvio Mavilio, Audentes <i>Advancing AAV Gene Therapy for Neuromuscular Diseases: the Case of X-Linked Myotubular Myopathy</i>
15:30	15:45	Giandomenico Turchiano, Freiburg <i>A novel preclinical genotoxicity assay, CAST-Seq, enables new insights in DNA repair dynamics and chromosomal aberrations in CRISPR-Cas edited human hematopoietic stem cells</i>
15:45	16:15	Coffee break Industry Exhibition
Session II - Platform Technologies Chairs: Zoltan Ivics & Oliver Müller		
16:15	16:45	Christian Buchholz, Langen <i>Receptor-targeted viral vectors in immunotherapy</i>
16:45	17:15	Zoltan Ivics, Langen <i>Non-viral Genome Engineering with the Sleeping Beauty transposon system</i>
17:15	17:30	Axel Rossi, Hannover <i>Vector uncoating limits adeno-associated viral vector-mediated transduction of human dendritic cells and vector immunogenicity</i>
17:30	17:45	Jasmin Kuhn, Munich <i>Delivery of Cas9/sgRNA ribonucleoprotein complexes with multifunctional oligomer nanoparticles</i>
17:45	19:00	Poster Session I
19:30	-----	Speakers Dinner Hotel Oberkirch, Münsterplatz (<i>invited speakers only</i>)
20:00	-----	Historix Sightseeing Freiburg



DAY 2 – THURSDAY, SEPTEMBER 27, 2018

Session III - iPS-based Cell Therapies Chairs: Ute Modlich & Jan Pruszk

9:00	9:30	Thomas Moritz, Hannover <i>Cell and Gene Therapy employing pluripotent stem cell derived myeloid cells</i>
9:30	10:00	Georges Lacaud, Manchester <i>Direct Reprogramming of Fibroblasts to Hematopoietic Progenitor Cells</i>
10:00	10:15	Mania Ackermann, Hannover <i>Human iPSC-derived phagocytes as a cellular therapeutic to treat bacterial infections</i>
10:15	10:30	Van Trung Chu, Berlin <i>CRISPR/Cas9-mediated gene insertion in mouse and human HSCs: an approach for gene therapy</i>
10:30	11:00	Coffee break Industry Exhibition

Session IV - Infectious Disease Chairs: Axel Schambach & Robert Thimme

11:00	11:30	Hans-Peter Kiem, Seattle <i>Cell and Gene Therapy for HIV Cure</i>
11:30	12:00	Patrick Arbuthnot, Johannesburg <i>Single stranded AAVs encoding engineered dimeric TALENs inhibit hepatitis B virus replication in vivo more efficiently than similar vectors that produce antiviral CRISPR/Cas9 derived from Staphylococcus aureus</i>
12:00	12:15	Tatjana Cornu, Freiburg <i>A highly efficient and GMP-compliant protocol to manufacture CCR5-edited cells to treat HIV infection</i>
12:15	12:30	Henning Olbrich, Hannover <i>Potency of high affinity gB-CAR-T cells designed against cytomegalovirus in vitro and in infected humanized mice</i>
12:30	14:00	Lunch break Industry Exhibition



Session V - Preclinical Modeling Chairs: Anja Ehrhardt & Christian Kupatt

14:00	14:30	Christian Kupatt, Munich <i>Preclinical evaluation of cardiovascular gene therapy</i>
14:30	15:00	Alberto Auricchio, Naples <i>Expanding AAV transfer capacity in the retina</i>
15:00	15:15	Judith Montag, Hannover <i>Successful knock-in of hypertrophic cardiomyopathy-mutation R723G into the MYH7 gene leads to HCM pathology in pigs</i>
15:15	15:30	Eric Ehrke-Schulz, Witten/Herdecke <i>Delivery of a multiplex CRISPR/Cas9 machinery for the treatment of Duchenne muscular dystrophy with one single gene deleted adenoviral vector</i>
15:30	16:00	Coffee break Industry Exhibition

Session VI - Clinical Translation - in vivo Chairs: Stefan Kochanek & Manfred Schmidt

16:00	16:45	Keynote Lecture 2 Katherine High, Spark Therapeutics <i>Turning genes into medicines: AAV-mediated gene therapy for genetic disorders</i>
16:45	17:15	Janbernd Kirschner, Freiburg <i>In vivo AAVg gene therapy to treat spinal muscular atrophy</i>
17:15	17:30	M. Dominik Fischer, Tübingen <i>First Clinical Gene Therapy for CNGA3-linked Achromatopsia</i>
17:30	17:45	Regine Mühlfriedel, Tübingen <i>A novel approach to facilitate assessment of therapeutic strategies in extremely rapid degenerating preclinical models of retinal disease</i>
17:45	18:00	Regine Heilbronn, Berlin <i>Neuropeptide-Based Gene Therapy for Treatment of Focal Epilepsy</i>
18:00	19:00	Poster Session II
19:30	-----	Social Event Peterhofkeller 19:30 - Dinner 21:30 - Poster Awards



DAY 3 – FRIDAY, SEPTEMBER 28, 2018

Session VII - Cancer Immune Cell Therapy Chairs: Renata Stripecke & Justus Duyster

9:00	9:30	Andrew Kaiser, Miltenyi Biotec <i>How CAR T-cells will change the way we treat cancer – the German perspective</i>
9:30	10:00	Margherita Norelli, Milan <i>Monocyte-derived IL-1 and IL-6 are differentially required for cytokine release syndrome and neurotoxicity by CAR-T cells</i>
10:00	10:15	Razieh Monjezi, Würzburg <i>CRISPR/Cas9 meets Sleeping Beauty: generation of CAR T-cells with enhanced therapeutic index using single-step, fully non-viral gene-transfer and genome-editing</i>
10:15	10:30	Jamal Alzubi, Freiburg <i>A novel chimeric antigen receptor (CAR) T cell approach eliminates prostate cancer in a mouse tumor model</i>
10:30	11:00	Coffee break Industry Exhibition

Session VIII - Cancer Gene Therapy Chair: Florian Kreppel

11:00	11:30	Monika Lusky, Transgene <i>Poxvirus – based oncolytic immunotherapy: clinical development and preclinical studies</i>
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Session IX - Wolfram Ostertag Lecture Chairs: Manuel Grez & Boris Fehse

11:30	12:00	Christopher Baum, Hannover <i>The team science of translation: Wolfram Ostertag in memoriam</i>
12:00	13:30	Lunch General Meeting Members DG-GT

Session X - Clinical Translation - ex vivo Chairs: Hildegard Büning & Charlotte Niemeyer

13:30	14:00	Alessandra Magnani, Paris <i>Gene therapy for primary immunodeficiencies: Where do we stand?</i>
14:00	14:15	Thomas Nerreter <i>Super-resolution microscopy reveals CD19dim expression on a subset of myeloma cells that can be targeted with CD19-CAR T cells</i>
14:15	14:30	Richard Paul Harbottle, Heidelberg <i>A Novel Non-Viral, Non-Integrative DNA Vector System for T-Cell Engineering</i>
14:30	15:00	Chiara Magnani, Milan <i>Non-viral engineering of CAR1K cells with Sleeping Beauty transposons for paediatric acute lymphoblastic leukemia</i>
15:00	-----	Farewell
15:15	-----	Board Meeting DG-GT



Programm Hörsaal Kinderklinik

MITTWOCH, 26. SEPTEMBER 2018 | PUBLIKUMSVERANSTALTUNG FÜR OBERSTUFENSCHÜLER | 9:00 – 12:15

9:00	9:15	Einleitung: Christof von Kalle, Toni Cathomen
9:15	9:45	Hildegard Büning, Hannover <i>Gentherapie: Gestern - Heute – Morgen</i>
9:45	10:15	Axel Schambach, Hannover <i>Neue virale Vektoren für den sicheren Gentransfer in Zellen des Blut- und Immunsystems</i>
10:15	10:45	Toni Cathomen, Freiburg <i>Mit CRISPR-Cas und anderen Genschere zur Medizin von morgen</i>
10:45	11:15	Pause
11:15	11:45	Anja Ehrhardt, Witten/Herdecke <i>Gen-basierte Therapien mit Viren - auf dem Weg zur direkten Anwendung im Patienten</i>
11:45	12:15	Christof von Kalle, Heidelberg <i>Personalisierte Medizin – Krebstherapie 2.0</i>

