

PROGRAMME AND BOOK OF ABSTRACTS

30 – 31 January 2019
Frankfurt am Main · Germany

**Gene Therapy –
Ready for the Market?**

www.dechema.de/genetherapy2019



COMMITTEE

Ulf Bethke	Miltenyi Biotec GmbH, Bergisch Gladbach
Hansjörg Hauser	Helmholtz Centre for Infection Research, Braunschweig
Uwe Marx	TissUse GmbH, Berlin
Beate Müller-Tiemann	Sanofi-Aventis Deutschland GmbH, Frankfurt am Main
Bernd Schröder	Miltenyi Biotec GmbH, Teterow
Karin Tiemann	DECHEMA e.V. Frankfurt am Main
Andrea Traube	OPTIMA pharma GmbH, Schwäbisch Hall
Roland Wagner	Rentschler Biopharma SE, Laupheim

CONFERENCE VENUE

DECHEMA e.V.
Society for Chemical Engineering and Biotechnology
Theodor-Heuss-Allee 25
60486 Frankfurt am Main
Germany
www.dechema.de

The conference is an initiative of the DECHEMA working groups *Cell Culture Technology* and *Medical Biotechnology* and is organized by DECHEMA e.V.

Wednesday, 30 January 2019

10:00 Registration

10:45 Welcome

K. Rübberdt, DECHEMA e.V., Frankfurt am Main/D

Session 1 "Development and scalability of gene transfer vectors"

Chair: H. Hauser, Helmholtz Centre for Infection Research, Braunschweig/D

10:50 KEYNOTE LECTURE

From the Virus to the Vector: Novel Integrating Gene Therapy Vectors

A. Schambach¹; ¹ Hannover Medical School, Hannover/D

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11:35 KEYNOTE LECTURE

Next Generation AAV vectors for Cell and Gene Therapy

H. Büning¹; ¹ Hannover Medical School, Hannover/D

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12:20 **Novel stable producer cell lines: enabling scalable lentiviral vector manufacturing**

A. Coroadinha¹; H. Tomás¹; A. Formas-Oliveira¹; T. Vaz¹; A. Rodrigues¹; M. Carrondo¹
¹ iBET/ITQB-NOVA, Oeiras/P

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12:45 **Poster Talk: Development and evaluation of a serum-free manufacturing process for lentiviral vectors in industrial scale**

J. Dettmann¹; T. Schaser¹; M. Meyer¹
¹ Miltenyi Biotec GmbH, Bergisch Gladbach/D

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13:00 Lunch

Session 2 "Strategies for vector optimization"

Chair: B. Müller-Tiemann, Sanofi-Aventis Deutschland GmbH, Frankfurt am Main/D

14:00 KEYNOTE LECTURE

Receptor-targeted viral vectors: rational design for in vivo gene delivery

C. Buchholz¹; ¹ Paul-Ehrlich-Institut, Langen/D

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14:45 **Optimizing transduction for clinical manufacturing of CAR-T and genetically modified CD34+ cells using Lentiviral vectors**

C. Thirion¹; ¹ Sirion Biotech GmbH, Martinsried/D

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15:10 Coffee Break

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Session 3 "Challenges in GMP production of gene therapeutic products"

Chair: B. Müller-Tiemann, Sanofi-Aventis Deutschland GmbH, Frankfurt am Main/D

15:40	KEYNOTE LECTURE From CAR T cells to CAR NK cells for improvement of cancer retargeting <u>U. Köhl</u> ¹ ; ¹ Fraunhofer Institut für Zelltherapie und Immunologie, Leipzig/D	15
16:25	KEYNOTE LECTURE Manufacturing advances and scale-up for simple and complex ATMPs <u>J. Campbell</u> ¹ ; ¹ Scottish National Blood Transfusion Service, Edinburgh/UK	17
17:10	Analytical Preparedness to Support a Phase I Gene Therapy Product <u>M. Joubert</u> ¹ ; ¹ Sanofi, Framingham, MA/USA	18

17:35 - 20:00 Reception at the posters

18:00 - 19:00 Member Assembly of the DECHEMA Working Groups *Cell Culture Technology* and *Medical Biotechnology* (only for members)

Thursday, 31 January 2019

Session 4 "Advanced manufacturing of gene therapeutics"

Chair: A. Traube, OPTIMA pharma GmbH, Schwäbisch Hall/D

09:00	KEYNOTE LECTURE Advancement of Viral Delivery Systems Manufacturing Processes Remains Critical to the Success of Cell and Gene Therapy <u>A. Kamen</u> ¹ ; ¹ McGill University, Montreal/CAN	19
09:45	An automated process for lentiviral transduction of CD34+ cells in a GMP-compliant closed system <u>E. Papanikolaou</u> ¹ ; U. Bissels ¹ ; A. Bosio ¹ ; S. Knöbel ¹ ¹ Miltenyi Biotec GmbH, Bergisch Gladbach/D	20
10:10	New level of integrity assurance at point of use and cryogenic robustness of single use systems for cell & gene therapy M. Neustetter ¹ ; <u>J. Kirchmair</u> ¹ ; T. Wurm ¹ ¹ Single Use Support GmbH, Kufstein/A	21
10:35	Downstream Purification Solutions for Viral Vectors: Enabling Platform Approaches to Advance Cell and Gene Therapy <u>F. Detmers</u> ¹ ; P. Hermans ¹ ; O. Terova ² ¹ ThermoFisher Scientific, Leiden/NL; ² ThermoFisher Scientific, Bedford, MA/USA	22
11:00	Poster Talk: Tentacle-based ion exchange chromatography for purification of viral vectors <u>M. Schulte</u> ¹ ; E. Wüstenhagen ¹ ; A. Aldinger ¹ ; T. Berger ¹ ; H. Marie ¹ ¹ Merck KGaA, Darmstadt, Darmstadt/D	37
11:15	Coffee Break	

Session 5 "Production of gene therapeutics for clinical application"

Chair: R. Wagner, Rentschler Biopharma GmbH, Laupheim/D

11:45	KEYNOTE LECTURE Towards intensified/optimized large scale production of viral vectors <u>O. Merten</u> ¹ ; ¹ Miltenyi Biotec Europe, Paris/F	23
12:30	KEYNOTE LECTURE Liver-based AAV Gene Therapy for Curative Treatments <u>M. Hörer</u> ¹ ; ¹ Freeline Therapeutics GmbH, Planegg-Steinkirchen/D	26
13:15	Lunch	

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Session 6 "Regulatory pathways to enable gene therapy"

Chair: B. Schröder, Miltenyi Biotec GmbH, Teterow/D

14:15	<p>KEYNOTE LECTURE Development and Regulation of Gene Therapy Medicinal Products <u>K. Cichutek</u>¹; M. Renner¹ ¹ Paul-Ehrlich-Institut, Langen/D</p>	28
15:00	<p>Gene Therapy in Point-of-Care Settings: from Adult Stem Cell Therapy to Germline Mitochondria Therapy: Missing Legal Therapy and Market Control? <u>T. Faltus</u>¹; ¹ Martin-Luther-Universität Halle-Wittenberg, Halle (S.)/D</p>	30
15:25	<p>Regulatory, Clinical and Logistics Challenges of Cell & Gene Therapy Trials – The CRO Perspective <u>A. Zobel</u>¹; ¹ PAREXEL International GmbH, Berlin/D</p>	32
15:50	Coffee and Networking	
16:15	End of Conference	

POSTER PROGRAMME

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Development and evaluation of a serum-free manufacturing process for lentiviral vectors in industrial scale	
<u>J. Dettmann</u> ¹ ; T. Schaser ¹ ; M. Meyer ¹	
¹ Miltenyi Biotec GmbH, Bergisch Gladbach/D	
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Tentacle-based ion exchange chromatography for purification of viral vectors	
<u>M. Schulte</u> ¹ ; E. Wüstenhagen ¹ ; A. Aldinger ¹ ; T. Berger ¹ ; H. Marie ¹	
¹ Merck KGaA, Darmstadt, Darmstadt/D	
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rAAV2 capsid protein modification, expression and stability	
<u>J. Teschner</u> ¹ ; R. Feiner ¹ ; K. Teschner ¹ ; M. Radukic ¹ ; Y. Hertle ¹ ; N. Biere ¹ ; D. Anselmetti ¹ ; K. Müller ¹	
¹ Bielefeld University, Bielefeld/D	
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Challenges of Developing a Process Control Strategy for Gene Therapy Manufacturing with a Focus on Process Analytics	
<u>C. Renzi</u> ¹ ; S. Godwin ¹	
¹ Sanofi, Framingham, MA/USA	
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High Quality Plasmid DNA supply for Gene Therapy	
<u>T. Hitchcock</u> ¹ ; H. Tharia ¹	
¹ Cobra Biologics, Keele/UK	