



Registration via the ENDOSCAPE website
<https://endoscape-2020.eu/registration.html>

ENDOSCAPE is a collaborative biotechnology project aimed at developing a beyond state of the art clinically applicable gene delivery technology. The **ENDOSCAPE** technology will have a major impact on the therapeutic opportunities for current and future drugs for a broad range of diseases and large patient groups.

The non-viral based **ENDOSCAPE** technology will enhance therapeutic efficacy in a more cost-effective manner thereby reducing costs of healthcare, improving the health of patients worldwide, and strengthening the competitive landscape of the EU in the worldwide quest for such an advanced technology.

ENDOSCAPE will overcome a longstanding and major bottleneck in the field of gene delivery, namely efficient, safe and cost-effective transfer of gene therapeutic products into the cytosol.

The **ENDOSCAPE** technology will be designed to allow targeting of any addressable cell type with all known genetic agents, thereby ensuring better patient therapy not limited to inherited disorders, but also for cancer therapy and therefore of importance for large patient groups.

The project is funded by the European Commission with about 7 million € under the Horizon 2020 initiative, with the grant agreement ID: 825730.

ENDOSCAPE consortium

- Charité – Universitätsmedizin Berlin
- Sapreme Technologies B.V.
- Max Planck Institute of Molecular Cell Biology and Genetics
- VIB-UGent Center for Plant Systems Biology
- Freie Universität Berlin
- University of Santiago de Compostela – Center for Research in Biological Chemistry and Molecular Materials
- The University of Rome Tor Vergata – Laboratory of Biosensors and Nanomachines
- Extrasynthese SAS
- Fraunhofer Institute for Molecular Biology and Applied Ecology IME
- University of Ferrara – Department of Life Sciences and Biotechnology
- The University of Basel – Institute of Pharmaceutical Medicine
- tp21 GmbH

International ENDOSCAPE Symposium

Harnack Haus
Ihnestr. 16-20
D-14195 Berlin

www.harnackhaus-berlin.mpg.de



International ENDOSCAPE Symposium

Berlin · 08 – 09 September 2022

SCIENTIFIC PROGRAM



This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 825730.

Day 1 11:30 – 19:45**Session 1:****Gene Therapy – Where We Stand Today**

- Non-viral gene therapy at a glance *Kazunori Kataoka, Tokyo*
- Gene therapy in monogenetic diseases *Company presentation*
- Suicide genes in cancer therapy *Company presentation*
- Participant presentations

Session 2:**Endosomal Escape – The Strategies**

- Strategies for intracellular delivery and endosomal escape
Vladimir P. Torchilin, Boston
- Photochemically-induced release *Pål Kristian Selbo, Oslo*
- Intracellular drug delivery *Company presentation*
- Glycosylated triterpenoids as endosomal escape enhancers
ENDOSCAPE-Partner Freie Universität Berlin
- Cell-penetrating peptides *Jean-Philippe Pellois, Texas*

Poster session

Day 2 08:15 – 18:30**Session 3:****Carriers of Endosomal Escape Enhancers and Genes**

- Design of efficient intracellular delivery vehicles
Jan van Hest, Eindhoven
- Scaffolds to carry endosomal escape enhancers and targeting ligands, and to polyplex effector genes: PAMAMs, peptides and DNA
ENDOSCAPE-Partners Charité - Universitätsmedizin Berlin, Freie Universität Berlin, University of Rome Tor Vergata, Universidade de Santiago de Compostela
- Nanocarriers *Company presentation*
- Chemical evolution refining intercellular mRNA, protein and Cas9/sgRNA delivery *Ernst Wagner, München*

Session 4:**Endosomal Escape – What Happens?**

- Intracellular routing of targeted payloads *Inge Zuhorn, Groningen*
- Mechanisms of endosomal trafficking and escape by SO1861
ENDOSCAPE-Partner Max Planck Institute of Molecular Cell Biology and Genetics
- Efficacy of endosomal escape enhancers in vitro and ex vivo
ENDOSCAPE-Partners University of Ferrara, Sapreme Technologies B.V., Charité - Universitätsmedizin Berlin
- Endosomal escape by photochemical internalization *Company presentation*
- Participant presentation

Poster session

Session 5:**GMP Production and Market**

- Controlled production of endosomal escape enhancers in plants
ENDOSCAPE-Partners Fraunhofer Institute for Molecular Biology and Applied Ecology IME, Extrasynthese SAS
- Gene discovery and synthetic biology for biosynthesis of endosomal escape enhancers in engineered plant cultures
ENDOSCAPE-Partner VIB-UGent Center for Plant Systems Biology
- Gene therapy from bench to bedside *Company presentation*
- Innovative solutions for GMP production of biopharmaceuticals
Company presentation
- Cost-of-goods analysis of the ENDOSCAPE technology
ENDOSCAPE-Partner University of Basel
- Cost-effectiveness analysis of potential gene therapies for hemophilia B based on ENDOSCAPE *ENDOSCAPE-Partner University of Basel*
- Participant presentations

Session 6:**Gene Therapy – Clinical Applications and Future Perspectives**

- Gene-based nanomedicines for cancer therapy and brain delivery
Christine Dufès, Glasgow
- Transplantation of genetically modified hematopoietic stem cells
Fulvio Mavilio, Modena
- Gene therapy of hemophilia *Antonia Follenzi, Novara*
- Current status of non-viral gene therapy: The ENDOSCAPE symposium at a glance *ENDOSCAPE-Partners*

Company presentations depending on registrations. If applicable, replacement by selected poster contributions as short presentations.