GENE.CELL.TISSUE
ENGINEERING

KICK-OFF
8 OCTOBER 2021

GHENT ADVANCED THERAPIES & TISSUE ENGINEERING

Your gate to self-healing

PROGRAM BOOK

GATE IN NUMBERS

35+ Research groups

10+ Disciplines

4 Knowledge institutions:
- Ghent University
- Ghent University Hospital
- VIB
- IMEC

6 Ghent University departments

10 Business developers to support your research

4 Core facilities:
- cGMP unit
- small animal facility
- 3D printer facility
- Centre for Advanced Light Microscopy

2 Clinical research centers
- HIRUZ, Health Innovation and Research Center of Ghent University Hospital
- D.R.U.G., the drug research unit of Ghent University Hospital

WELCOME

GATE
GENE.CELL.TISSUE ENGINEERING

Welcome at the GATE Kick-off conference, the first GATE partnering event since its launch in 2020.

GATE is the new virtual institute of Ghent Advanced Therapies and Tissue Engineering, connecting researchers and clinicians from 4 knowledge institutes in the Gent ecosystem (Ghent University, Ghent University Hospital, VIB-UGent and imec-UGent), who work together with innovation professionals to develop faster, more efficient and more market-oriented solutions for regenerative medicine.

Regenerative medicine is by nature a multidisciplinary field, in which innovation management is becoming evermore complex. With experts from 35 research groups across more than 10 expertise domains, supported by a team of 10 business developers, GATE covers a broad scope of technological expertise, from designing genetic tools and cell products for therapy, to the development of new materials to guide stem cell behavior and construct tissues and organs for regenerative medicine, as well as artificial systems for drug screening and personalized medicine.

During the GATE kick-off, researchers, clinicians and industry professionals present the latest technological developments of gene, cell and tissue engineering for broad applications of organ-on-chip design, gene & cell therapy, and tissue regeneration. Next to technological innovation, the event will highlight successful partnerships between industry and Ghent academics to advance gene and cell therapy programs towards the clinic. During in-depth panel discussions, scientific and Industrial leaders will express and discuss their views on the current challenges preventing advanced therapies getting to the market and saving patients' lives.

The GATE kick-off offers a networking opportunity to meet researchers from different disciplines and to establish contacts with industry to develop new research collaborations. It is also an invitation to engage yourself in discussions on the needs and future perspectives of the field. Thus, welcome and enjoy the meeting!

GHENT UNIVERSITY UZ GENT imec VIB

PROGRAMMA GATE KICK-OFF

12:00 – 13:00	**Lunch**
13:00 – 13:10	**Welcome**
13:10 – 13:35	**Keynote lecture**
	Formation and running of the Austrian Cluster for Tissue Regeneration Heinz Redl — Director Ludwig Boltzmann Institute for Clinical and Experimental Traumatology, Austria
13:35 – 14:05	**Emerging technologies**
	Single-cell and multi-omics technologies at VIB-UGent — Niels Vandamme (VIB)
	Stretchable Circuits and their use in Tissue Engineering Applications — Jan Vanfleteren (imec)
	Delivering nucleic acids — Stefaan De Smedt (UGent)
	Industry pitches
14:05 – 15:00	**Clinical translation / gene delivery and therapy**
	Selective targeting of seizure networks in epilepsy with *in vivo* gene therapy — Robrecht Raedt (UZ Gent)
	Fiction to fact: ocular gene therapy for inherited blindness — Bart Leroy (UZ Gent)
	Lipid nanoparticle platform technology for nucleic acid-based immunotherapy — Bruno De Geest (UGent)
	Industry pitches
	Panel discussion
15:00 – 15:30	**Coffee Break**
15:30 – 15:50	**Clinical translation / advanced therapies and manufacturing**
	Gene therapy: dream or reality? — Linos Vandekerckhove (UZ Gent)
	GMP production of investigational ATMPs for early phase clinical trials — Bart Vandekerckhove (UZ Gent)
	Industry pitches
15:50 – 17:20	**Tissue engineering**
	Poly(2-oxazoline)s for advanced therapies and tissue engineering — Richard Hoogenboom (UGent)
	Versatile polymer toolbox: When chemical design meets 3D-printing to serve medical applications — Sandra Van Vlierberghe (UGent)
	Manufacturing of GMP grade human platelet lysate for biomaterials, cells and tissues — Hendrik Feys (Belgian Red Cross)
	Tissue reconstruction by tissue engineering — Phillip Blondeel (UZ Gent)
	Industry pitches
	Panel discussion
17:20	**Reception**

SPONSORS

PLATINUM

Janssen
PHARMACEUTICAL COMPANIES OF
Johnson & Johnson

SILVER

Lonza
Cell & Gene

proteintech®
Antibodies | ELISA kits | Proteins

Miltenyi Biotec

Rousselot
Biomedical

BRONZE

UNIVERCELLS
Technologies

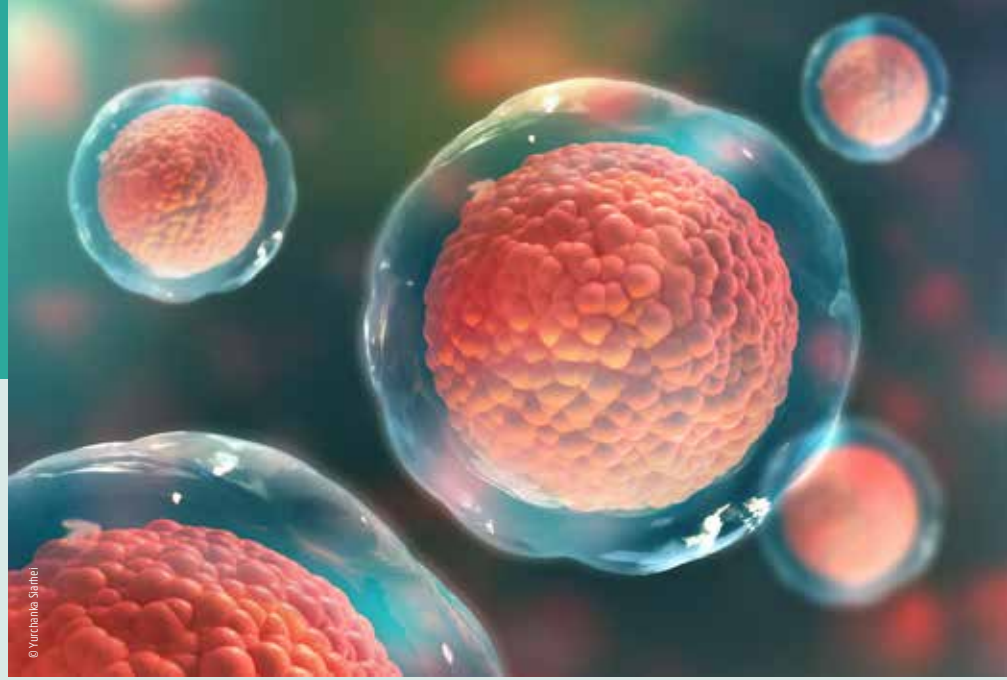
KEYNOTE LECTURE

‘Formation and Running of the Austrian Cluster for Tissue Regeneration’

Prof. Dr. Heinz REDL, Prof. Dr. Johannes GRILLARI

Ludwig Boltzmann Institute for Experimental & Clinical Traumatology, Vienna, Austria

The Austrian Cluster for Tissue Regeneration (ACTR) was founded in 2006 with 3 partners. To date it integrates the Ludwig Boltzmann Institute for Trauma (LBI Trauma) and 29 further research groups of 11 universities and 2 non-university institutions in Austria. The Cluster is no legal entity and has no budget. The Cluster offers state-of-the-art facilities for tissue engineering including biomaterials and cell/secretome (stem/endothelial) research as well as biophysical therapies. With its fully translational approach, an inter-disciplinary team including MD/VetMD, located partly in hospitals, and a GMP facility it provides an optimal research environment. ACTR covers - Neuroregeneration, Soft Tissue Repair, Cartilage/Tendon and Bone/Ligament Regeneration as well as competence centers for molecular biology, extracellular vesicles (EV), senescent cells, miRNA analysis, polymer synthesis, bioprinting, preclinical in vivo facilities incl. imaging, morphology.



© Yurchanka Siamei

In particular the LBI Trauma has a long tradition in bioadhesives and hemostats e.g. plasma derived fibrin matrix and its special use for growth factor and cell delivery as well as a gene activated matrix. Several experimental and clinical studies show efficacy for biophysical therapies, extracorporeal shock wave and light therapy as means to accelerate tissue repair and regeneration in various wounds and non-bone healing. However, the biomolecular mechanism by which this treatment modality exerts its therapeutic effects is only partially resolved, and such is a central research topic in the Cluster. Another goal of the Cluster is to use „medical garbage“ for regenerative purposes (fat, placenta).

For the orthopaedic field of particular interest are projects for the replacement of broken ligaments by degradable silk fibroin constructs which are replaced by endogenous ligament structures. The ACTR also provides special training opportunities (PhD program for bone and joints as well as support of Biomedical Engineering master programs). Since this year it is also supported by a society with academic and industrial partners.

To support the important translational efforts, the Cluster also currently includes 14 spin-off companies (of academic Cluster members) as associate members.

E-MAIL heinz.redl@trauma.lbg.ac.at

WEBSITE http://www.tissue-regeneration.at/

SESSION I EMERGING TECHNOLOGIES

Single-cell and multi-omics technologies at VIB-UGent

Niels VANDAMME

VIB Single Cell Core Manager

Stretchable Circuits and their use in Tissue Engineering Applications

Jan VANFLETEREN

Professor / Senior Scientist (UGent / IMEC)

Delivering Nucleic Acids

Stefaan DE SMEDT

Dir Lab Gen Biochem & Pharm, Ghent Research Group on Nanomedicines, Faculty of Pharmacy, UGhent

Industry pitches

Niels VANDAMME

VIB Single Cell Core Manager

Single-cell and multi-omics technologies at VIB-UGent

Single-cell technologies are revolutionizing all life sciences fields, ranging from disease areas to plant science. Working at the cutting edge of this field, VIB wants to combine single-cell multi-omic strategies, advanced imaging, artificial intelligence/machine learning and personalized disease models to fundamentally impact the current medical practice. This pitch talk will provide an overview of how VIB acts as an ecosystem where top-notch single-cell research, technology-testing, innovation programs and service nodes are intertwined.

Jan VANFLETEREN

Professor / Senior Scientist (UGent / IMEC)

Stretchable Circuits and their use in Tissue Engineering Applications

A core competence of the UGent-IMEC Centre for Microsystems Technologies (CMST) to which J. Vanfleteren belongs is the development and application of novel technologies for fabrication and assembly of electronics and sensor circuits. In the course of the years technology platforms for ultrathin chip packaging, stretchable circuits, integration of circuits in soft polymers and textiles, microfluidics, etc. have been developed. This broad technology platform also has potential for applications in the domain of tissue engineering. As an example stretchable electronic circuits and electrode matrices allow for the construction of stretchable MEA's (Micro Electrode Arrays), which allow for simultaneous mechanical and electrical stimulation of cells and tissues. This multi-cue stimulation could possibly improve the quality of differentiation of stem cells towards cell types like cardiomyocytes, smooth muscle cells and brain cells, which all have electrical and mechanical components in their operation. The presentation will provide a brief overview of the developed technologies.

Stefaan DE SMEDT

Dir Lab Gen Biochem & Pharm – Ghent Research Group on Nanomedicines – Faculty of Pharmacy - UGhent

Delivering Nucleic Acids

Therapeutic strategies based on nucleic acids (NAs) have been introduced. As years ago, when recombinant proteins began to be developed as drugs, with NAs we are again on the brink of a revolution in drug development. The lack of delivery technologies is a major bottleneck for the breakthrough of NA bio-therapeutics. Our research group represents a rather exceptional team of intensively collaborating scientists at Ghent University with a deep focus on barriers that hamper efficient (*in vivo / ex vivo*) delivery of nucleic acids. In this presentation I will briefly overview the technologies we are working on for the purpose of nucleic acid delivery, with a special focus on nanomedicines and photoporation.

Industry pitches

SESSION II

CLINICAL TRANSLATION / GENE DELIVERY AND THERAPY

Selective targeting of seizure networks in epilepsy with in vivo gene therapy

Robrecht RAEDT

Associate Professor, Department of Head and Skin

Fiction to Fact: Ocular Gene Therapy for Inherited Blindness

Bart P LEROY

Head of Department & Professor of Ophthalmology & Ophthalmic Genetics, Ghent University Hospital & Ghent University
Attending Physician, Children's Hospital of Philadelphia

Lipid nanoparticle platform technology for nucleic acid based immunotherapy'

Bruno DE GEEST

Full Professor at UGhent

Industry pitches
Panel discussion moderated by Prof. Dr. Bart Leroy

Robrecht RAEDT

Associate Professor, Department of Head and Skin

Selective targeting of seizure networks in epilepsy with in vivo gene therapy

Standard treatment for epilepsy consists of repeated systemic administration of anti-epileptic drugs (AEDs) which inhibits neuronal function. These AEDs have effects throughout the whole body, also at times when there is no risks for seizures and therefore often result in severe side effects. Research at 4BRAIN focuses on developing new therapies for focal epilepsy where interventions are specifically targeted towards the seizure focus. During my talk I will introduce the concepts of chemogenetics and optogenetics as potential therapies for focal epilepsy. Both gene therapy approaches rely on local injection of viral vectors in the seizure focus to induce expression of inhibitory receptors and/or ion channels in neurons causing epileptic seizures. The inhibitory action of these newly introduced proteins can be triggered with light (optogenetics) or selective chemical compounds (chemogenetics). Both opto- and chemogenetic approaches do not affect normal functioning of non-targeted, healthy neuronal tissue and can be limited to periods when the risk for seizures is high. I will shortly highlight some of the promising results we obtained with opto- and chemogenetics in rodent models for temporal lobe epilepsy, demonstrating that these gene therapy approaches have potential to become new, patient-tailored therapies for focal epilepsy.

Bart P LEROY

Head of Department & Professor of Ophthalmology & Ophthalmic Genetics,
Ghent University Hospital & Ghent University
Attending Physician, Children's Hospital of Philadelphia

Fiction to Fact: Ocular Gene Therapy for Inherited Blindness

Inherited retinal dystrophies (IRDs) represent a large group of blinding disorders. Efforts to slow evolution, stabilize and improve retinal function for IRD patients have emerged in the last 15 years. The eye is a privileged target for such remedies, whether it be at the level of retinal pigment epithelium, photoreceptor cells or retinal ganglion cells. Indeed, with better gene identification in the individual patient, improved insight into mechanisms of disease has led to the development of different innovative treatments.

As such, gene augmentation therapy for one form of inherited, congenital, retinal blindness, using adeno-associated virus, has recently been introduced to market as the first reimbursed ocular gene therapy in Europe and the USA. After assisting development of this treatment in the USA, we have now successfully started treating Belgian patients with subretinal injections of voretigene neparvovec (Luxturna®) for *RPE65*-related IRD.

With gene augmentation therapy through an intravitreal injection of AAV2 (lenadogene nolparvovec - Lumevoq®), we have also been able to improve visual function in patients with Leber Hereditary Optic Neuropathy, a retinal ganglion cell disease due to the frequent 11778G>A mutation in the mitochondrial gene *ND4* within a clinical trial setting.

Other approaches tested in clinical trials include antisense oligonucleotides (AONs) for the treatment of *CEP290*-related IRD. A 17-mer AON, sepofarsen, is injected intravitreally, with measurable effect on visual function. An overview of the effects of several ocular gene therapies will be presented, within the framework of inherited retinal blindness.

Bruno DE GEEST

Full Professor at UGhent

Lipid nanoparticle platform technology for nucleic acid based immunotherapy

The recent COVID-19 pandemic has demonstrated the huge potential of mRNA technology to generate efficacious vaccines at tremendous speed and has resulted in the first-time approval of two mRNA-based vaccines (BioNTech/Pfizer and Moderna). This success would however have been impossible without two decades of preceding lipid-based nanoparticle (LNP) development. LNPs are highly efficient nanocarriers that protect the fragile mRNA from ubiquitous RNAses and enable it to cross the endosomal membrane. LNPs are multicomponent systems with the **ionizable lipid** being considered as the main driver of LNP activity and safety. The expertise of the De Geest lab overarch chemistry, immunology and nanotechnology and together with Dr. Stefaan De Koker from eTheRNA Immunotherapies, libraries of ionizable lipids were developed that combine improved potency and safety of prophylactic and therapeutic mRNA vaccines and mRNA-based immunotherapies. In this presentation will cover the roadmap of this successful academia-industry collaboration and how this has led to successful valorization.

Industry pitches

Panel discussion moderated by Prof. Dr. Bart Leroy

SESSION III

CLINICAL TRANSLATION / ADVANCED THERAPIES AND MANUFACTURING

Gene therapy: dream or reality

Linos VANDEKERCKHOVE

Professor, Infectious Diseases Specialist UZGhent,
coordinator HIV Reference centre and HIV Cure Research Centre

GMP production of investigational ATMPs for early phase clinical trials

Bart VANDEKERCKHOVE

BVDK Lab, Ghent University; Cell Therapy Unit 3, UZ Gent

Industry pitches

Prof. Linos VANDEKERCKHOVE

Professor, Infectious Diseases Specialist UZGhent,
coordinator HIV Reference centre and HIV Cure Research Centre

Gene therapy: dream or reality

The administration of gene therapy to cure a fatal disease was introduced in our health system over the last decade. These therapies are complex to develop, and the production process requires an extensive quality control system that goes far beyond the classical control of a medicinal product. The development and production process requires a transfer gene that is using the specific carrier to be deliver as a treatment at the right spot at the right time. A collaboration with a multitude of stakeholders is necessary to make this process possible. I will explain the different steps to come to this process and highlight some recent advances in the field. Moreover, I will show how new gene-therapy breakthrough technology can be explored to treat complex infectious diseases. In addition, I will highlight the potential impact of university translational research towards implementation strategies of newly developed gene therapies.

Bart VANDEKERCKHOVE

BVDK Lab, Ghent University; Cell Therapy Unit 3, UZ Gent

GMP production of investigational ATMPs for early phase clinical trials

The Cell therapy Unit facilitates Phase I/II clinical trials investigating safety and efficacy of Gene Therapy and Somatic Cell Therapy medicinal products by setting up the manufacture of these medicinal products according to the principles of Good Manufacturing Practices. In close interaction with the Principle Investigator, we define the specifications of the incoming goods, design the production process, define the proper quality controls and finally perform the validation runs. This is described in the investigational medicinal product dossier and filed to the Federal Agency for Medicines and Health Products. After approval, the ATMP is produced in quantities required for the clinical study. At the moment, we are producing ATMP for 3 clinical trials and setting up the production process of two additional ATMPs. During my presentation, these projects will be discussed.

Industry pitches

SESSION IV TISSUE ENGINEERING

Poly(2-oxazoline)s for advanced therapies and tissue engineering

Richard HOOGENBOOM

Full Professor (UGhent)

Versatile polymer toolbox: When chemical design meets 3D-printing to serve medical applications

Sandra VAN VLIERBERGHE

Professor / Group Leader, UGent

Manufacturing of GMP grade human platelet lysate for biomaterials, cells and tissues

Hendrik FEYS

Principal Investigator, Rode Kruis Vlaanderen

Tissue reconstruction by tissue engineering

Phillip BLONDEEL

Chairman of the Department of Plastic Surgery

Industry pitches

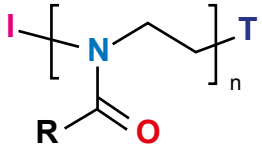
Panel discussion moderated by Prof. Dr. Catherine Van Der Straeten

Richard HOOGENBOOM

Full Professor (UGhent)

Poly(2-oxazoline)s for advanced therapies and tissue engineering

Polymers have revolutionized the world and are also omnipresent in biomedical applications, such as polymer excipients in oral tablets, PEGylation of proteins and carriers as well as a wide range of biomedical devices and as scaffolds for tissue engineering.



Within our research group we are developing poly(2-oxazoline)s as the next generation of polymers for biomedical applications. Poly(2-oxazoline)s are tertiary amide containing polypeptide mimics, as shown on the right, that have been shown to have good biocompatibility and can suppress interactions with proteins and cells. Importantly, the synthesis of poly(2-oxazoline)s provides ample opportunities to tailor the polymer properties for specific applications by modulating the hydrophilic/ hydrophobic balance through the side chain R-group, introducing specific functional and/ or reactive groups in the side chains, enabling crosslinking to form hydrogels, as well as orthogonal functionalization with multiple functional groups in the side chains and the chain ends, such as drugs, targeting ligands and labels. Moreover, poly(2-oxazoline)s are precursors for linear polyethyleneimine and provide access to defined cationic polymers for gene therapy.

Within this lecture, the poly(2-oxazoline) platform will be presented and specific research examples will be show-cased for polymer-drug conjugates, cationic polymers for gene therapy as well as crosslinked polymer hydrogels for tissue engineering.

Sandra VAN VLIERBERGHE

Professor / Group Leader, UGent

Versatile polymer toolbox: When chemical design meets 3D-printing to serve medical applications

Biofabrication is a specific area within the field of tissue engineering which takes advantage of rapid manufacturing (RM) techniques to generate 3D structures which mimic the natural extracellular matrix (ECM). A popular material in this respect is gelatin, as it is a cost-effective collagen derivative, which is the major constituent of the natural ECM. The material is characterized by an upper critical solution temperature making the material soluble at physiological conditions. To tackle this problem, our group focusses on different gelatin functionalization strategies which enable covalent stabilization of 3D gelatin structures.

In a second part, synthetic acrylate-endcapped, urethane-based precursors (AUP) based on polyethers and polyesters, will be discussed with exceptional crosslinking behaviour and CAD-CAM mimicry compared to conventional materials. Within this synthetic material class, also insight will be provided on the shape memory properties of polyester-based AUPs (two filed patents).

Several polymer processing techniques will be covered including conventional 3D printing using the Bioscaffolder 3.1 and two-photon polymerization. A number of biomedical applications will be tackled including adipose tissue engineering, vascularization, ocular applications, etc. The results show that chemistry is a valuable tool to tailor the properties of (bio)polymers towards processing while preserving the material biocompatibility.

REFERENCES

1. Van Hoorick, J.; et al. *Acta Biomaterialia 2019*, 97, 46-73.
2. Tytgat, L.; et al. *Acta Biomaterialia 2019*, 94, 340-350.
3. Arslan, A.; et al. *Materials Today 2020*, 44, 25-39.
4. Delaey, J.; et al. *Advanced Functional Materials 2020*, 30, 1909047.
5. Van Hoorick, J.; et al. *Biofabrication 2021*, 13 (1), 015017.

Hendrik B. FEYS

Principal Investigator, Rode Kruis Vlaanderen

Manufacturing of GMP grade human platelet lysate for biomaterials, cells and tissues

Blood establishments prepare, store and distribute blood components for transfusion including red cell concentrates, plasma and platelet concentrates. Platelet concentrate inventory management is particularly challenging because blood legislation restricts platelet transfusion to a 5 day window following donation. In Flanders, 3%-5% of platelet concentrates is consequently lost for transfusion. Yet, platelets beyond 5 days of storage is still a valuable resource of over 300 different bioactive molecules including cytokines and growth factors, stored in organelles. These factors can be released in the surrounding liquid medium and subsequently harvested. The resulting *human platelet lysate* (hPL) is then used as tissue culture supplement, for instance to replace serum from bovine origin. Our research team has developed a novel method to produce hPL. Advantages of our method are (i) production in a closed system, (ii) efficient release of factors and (iii) high standard clinical grade (GMP). Production in a closed system allows to keep maximal sterility avoiding exposure to ambient air. To this purpose a novel bag set was designed to transfer platelets and initiate factor release. Efficient release of factors was demonstrated using ELISA. Biochemical composition was compared to hPL from other manufacturers. The resulting hPL was furthermore tested in tissue culture of six different cell lines, *in vitro*. Finally, the team is implementing regulations and procedures to assure GMP grade material in the near future. In conclusion, we have developed a novel method for GMP grade hPL production. The hPL is intended to assist cell and tissue manufacturing in Flanders and beyond.

Philip BLONDEEL

Chairman of the Department of Plastic Surgery

Tissue reconstruction by tissue engineering

In our quest to avoid the present disadvantages of autologous tissue transplantation, reconstructive surgeons are looking for ways to build large volume tissues outside the human body.

We therefore count on the theory of self-assembly. This is also called 'bottom-up' or 'histio-induction' tissue engineering, just like an embryo develops out of two cells that join and afterwards are able to proliferate and differentiate into a human body. Human cells are seen as building blocks held together with a biological liquid environment (also called bio-ink), in which hormones, antibodies and messenger proteins move around as inter-cellular communicators. Cells stimulate or inhibit each other by this sophisticated type of communication.

We now are able isolate human stem cells donated by patients, let them grow in clusters or spheroids and have them differentiate into different types of cells. Triple cultures or a complex of a multitude of cells form the corner stones for building critical masses.

We also understand now how to create and develop blood vessels with the use of 3D bioprinting both at a microscopic level (capillaries) as at a macroscopic level. Larger vessels can then be sutured to recipient vessels in the human body. This part of our research is essential as tissue growth always follows the sprouting of blood vessels.

Human cells are embedded in an environment called the SVF. It is a gelatinous environment filled with mostly collagen that serves to maintain support for the cells and blood vessels that bring along oxygen, nutrients and fluids.

Industry pitches

Panel discussion moderated by Prof. Dr. Catherine Van Der Straeten

INDUSTRY PITCHES

Scale matters: European Spatial Biology Center (ESBC) for new biological discoveries and therapies

Nachiket KASHIKAR Co-founder (European Spatial Biology Center)

Antisense oligonucleotides for Inherited Retinal Diseases

Aniz GIRACH

Chief Medical Officer, ProQR Therapeutics

Insights on the partnership between Novartis and UZGent as Ocular Gene Therapy Treatment Center for Belgium and Luxembourg

Quentin SPILLIAERT

Cluster Medical Lead - Ocular Gene Therapy (Novartis)

UCB's emerging ambition in Gene Therapy

Michael LINDEN

UCB GT Research Leadership (UBC)

A breakthrough polymer platform for the emerging gene delivery era

Victor R. DE LA ROSA Managing director at Avroxa BV

Technology innovation a step further towards optimized gene therapy manufacture

Tania PEREIRA CHILIMA

CTO of Univercells Technologies

BIO INX – The Future of Healthcare

Jasper VAN HOORICK

Project Lead (BIO INX)

Unlocking clinical translation for gelatin-based biomaterials

Jeff DAELMAN

Business Development Manager Innovation (Rousselot BV)

A lattice and stem cell sandwich

Pieter HEYVAERT

Medical 3D planning & printing Ambassador (Materialise Leuven)

From idea to patient

Dominiek ROSSILLION

Business Development Manager (QbD)

Together through the Gate towards a brighter future for patients with Inherited Retinal Diseases.

Dagmar HOEBEN

EVT Lead Ophthalmology Benelux (Janssen Pharmaceuticals)

Nachiket KASHIKAR

Co-founder (European Spatial Biology Center)

Scale matters: European Spatial Biology Center (ESBC) for new biological discoveries and therapies

Spatial biology has emerged as the "next big technology" for life sciences and promises to usher in a new era in both basic and translational research. Spatial biology maps the molecular inventory (e.g., proteins, mRNA, DNA, and metabolites) of any biological tissue in a quantitative manner at a single-cell or even subcellular level. It provides the why, what, how, and importantly the where of the molecular logic behind tissue development, disease initiation and progression. Such an understanding is indispensable for identifying new disease biomarkers, drug targets, and designing future clinical trials.

As unrelenting technological developments in spatial biology take place all over the world – both academically and commercially – Europe must be at the forefront of this revolution. The European Spatial Biology Center (ESBC) aims to be the key enabler for next-generation innovations in areas of high unmet need, viz. neurology, cancer, infectious diseases, developmental biology, and agricultural biology. ESBC's mission is to create the largest infrastructure in Europe (and possibly in the world) to process samples from clinical trials, biobanks, academic labs, and biopharma such that exquisite spatial biology data is generated at scale with advanced data analytics. For instance, working closely together with the clinicians and researchers, we can gain novel insights from the samples in the biobanks to generate "big spatial biology data" such that in combination with advanced machine learning tools, we can understand the diseases better. We trust that through ESBC, Europe will lead the way in advancing disease therapeutics, tissue engineering and diagnostics.

Aniz GIRACH

MD – Chief Medical Officer, ProQR Therapeutics

Antisense oligonucleotides for Inherited Retinal Diseases

ProQR Therapeutics is a clinical stage biopharmaceutical company established in 2012 and based in the Netherlands. ProQR Therapeutics develops intravitreal RNA therapies for genetic ophthalmic diseases, as well as Axiomer® RNA editing and TRIDENT® RNA pseudouridylation platforms to potentially address the unmet medical need diseases for non-ophthalmic diseases. RNA therapies are designed to correct genetic mutations, without directly impacting the DNA. ProQR's pipeline consists of 3 clinical staged assets for inherited retinal diseases (IRD): sepofarsen for a disease called Leber Congenital Amaurosis type 10, QR-421a for a disease called Usher's syndrome/non-syndromic retinitis pigmentosa, and QR-1123 for a disease called autosomal dominant retinitis pigmentosa. The Department of Ophthalmology & Center for Medical Genetics of Ghent University Hospital & Ghent University is involved in 2 of these programs involving sepofarsen and QR-421a. Prof Dr Bart P. Leroy being Principal Investigator in both programs. The objective of this talk will be to understand the characteristics of RNA therapies for IRDs and other genetic ophthalmic diseases, discuss sepofarsen and QR-421a clinical trial data to date and look at the future of intravitreal RNA therapies in the wider ophthalmology landscape.

Quentin SPILLIAERT

Cluster Medical Lead - Ocular Gene Therapy (Novartis)

Insights on the partnership between Novartis and UZGent as Ocular Gene Therapy Treatment Center for Belgium and Luxembourg

Michael LINDEN

UCB GT Research Leadership

UCB's emerging ambition in Gene Therapy

UCB's therapeutic ambition is to move from symptom control towards etiology-based treatments, achieving disease modification and cure.
In that respect, UCB's recent investments in advanced therapeutic modalities, and more specifically its AAV gene therapy technology platform, will help us deliver on that ambition by further driving the development of differentiated solutions with unique outcomes for patients.
Allowing to leverage our strong and growing heritage in neurological diseases, and well aware of remaining unmet need for our patients, UCB prioritized the central nervous system (CNS) therapeutic area for its early GT projects.
Building on our strong relationship with UGent and UZGent through historical collaborations, as well as expanding on numerous existing academic partnerships in the gene therapy space (KULeuven, CIMA University of Navarra, University of Chile, King's College London), UCB is interested to explore synergies with the GATE community to strengthen its gene therapy platform activities.

Victor R. DE LA ROSA

Managing director at Avroxa BV

A breakthrough polymer platform for the emerging gene delivery era

The Covid pandemic has catalyzed the emergence of RNA as a key element in human development in the 21st century. RNA provides unique advantages that anticipate its future central role in prophylactic and therapeutic applications, Agri-tech and beyond.
To extract the full potential of these delicate molecules, a formulation and delivery vehicle is needed. While the current solutions, mostly based on lipid nanoparticles (LNPs), have been invaluable to enable fast vaccine development, they suffer from many pitfalls. These include non-biodegradability, low stability, limited scope, undesired immunogenicity, and a prohibitive price tag.

At Avroxa, we have developed a unique polymer platform that offers substantially superior gene delivery performance over the current state-of-the-art, while tackling crucial aspects of the ideal delivery vehicle.
Our polymer platform is composed by two families, Ultroxa® and UltraPEI®, both sharing an unparalleled level of versatility and structural modularity.

Ultroxa® polymers exhibit enhanced properties over the widespread polyethylene glycol (PEG), potentially providing safer and more effective LNPs.

UltraPEI® is our orthogonal solution to gene formulation, providing a complete alternative to LNPs and offering exceptional performance, broad applicability throughout all RNA types, high stability of the formulation, and biodegradability. Moreover, UltraPEI® has been designed to have a low production cost, enabling the development of RNA therapies for all and the realization of RNA's full potential across the board. Avroxa: where there's chemistry, we make it happen.

Tania PEREIRA CHILIMA

CTO of Univercells Technologies

Technology innovation a step further towards optimized gene therapy manufacture

Advanced therapies provide a significant improvement in quality of life to patients with indications with no current treatment; The commercialization of these breakthrough therapies present several challenges some of which are attribute to technology availability and process performance. Given the nascent nature of the gene therapy market, technologies used for product manufacture are either manual (e.g. cell factories) or inherited from other applications (e.g. mAbs & vaccines). Both approaches pose limitations relating to scalability, automation, footprint etc.

As a manufacturing technology provider Univercells Technologies is continuously analyzing the challenges experienced by gene therapy developers to offer solutions that would fit their specific needs. In collaboration with developers, CDMOs and Academics Univercells Technologies looking to apply the principles of intensification and chaining to gene therapy manufacture to address the key challenges facing gene therapy producers. These technology principles are embodied in a structured fixed-bed bioreactor and a multi-step automated platform for integrated continuous bioprocessing with low footprints.
The innovative structured fixed-bed enables a low shear environment and homogeneous nutrient for cell growth across scales. This results in a reliable intensified and scalable viral production reaching high-throughput at low footprint.

Jasper VAN HOORICK

Project Lead (BIO INX)

BIO INX – The Future of Healthcare

At BIO INX, we are focusing on the development and commercialization of 'bioinks' for biofabrication applications. Biofabrication is the scientific field which uses 3D printing for the generation of 3D tissues or tissue substitutes for a whole range of applications. These applications consist of both near future market applications like the development of 3D tissue models for (personalized) drug screening or to replace animal testing via so called "organ-on-chip" technology. To this end, animal trials can significantly be reduced as already human cells can be incorporated, thereby limiting issues with interspecies differences. Other applications can also consist of the generation of semi-permanent prosthesis, requiring proper biointegration. In the longer term, the technology of biofabrication can be used in the field of organ regeneration, by 3D printing patient specific tissue using autologous cells, thereby overcoming typical issues related to organ transplants.

However, what all these strategies require is a combination of cells, a 3D design and so called 'bioinks' which enable printing of cells but also ensure high viability after printing. And development of these bioinks is what BIO INX is focusing on, with a clear focus on high resolution technologies.

Jeff DAELMAN

Business Development Manager Innovation (Rousselot BV)

Unlocking clinical translation for gelatin-based biomaterials

Modified gelatins (e.g. GelMA) have been around for over 2 decades, but despite a growing body of scientific literature and patent applications, no clinical application has (yet) made it to the patient. We believe this is due to a lack of material suitable for use in clinical trials: materials that are consistent in their physical and chemical properties, are GMP certified and have the required level of purity.

Rousselot intends to bridge that gap, using a case study we will demonstrate the importance of considering upscaling and material consistency as early in the process as possible.

Pieter HEYVAERT

Medical 3D planning & printing Ambassador – Materialise Leuven

A lattice and stem cell sandwich

3D printed bioresorbable implants, printed tissues and even 3D printed organs are the future of personalized healthcare. Nonetheless, many challenges remain to make these innovations commercially available on a large scale.

Today, "in-between technologies" that bridge the gap between 3D printing and engineering living tissue, exist and can be used in vivo.

One of these examples is the use of skeletal stem cells in combination with 3D printed titanium lattice structures.

Dominiek ROSSILLION

Business Development Manager

From idea to patient

Dominiek is business development manager for QbD. He has a rich consultancy background in Quality within the lifesciences, within big pharma, but also mid-size and small start-ups.
Within QbD he is the sales lead of the ATMP core team. The core team focusses on trends, developments and solutions for customers. And this in the field of development methodologies, Quality, Regulatory and clinical. This always from a practical and applicable point of view.

Quality by Design is a knowledge organization that supports life science companies in bringing products and therapies to the market. Qbd has extensive expertise in clinical trials, regulatory affairs, quality assurance and validation within the life science sector, more specifically the classic pharma, biotech, healthcare and medical devices. The latter, together with ATMPs (cell and tissue therapies), is one of QbD's two focal points. Qbd was founded by Bart Van Acker, a Bio engineer himself, and recently celebrated its 10th anniversary. QbD is active in 8 countries with over 400 employees worldwide. Moreover, our services are growing rapidly worldwide thanks to our software product (eQMS) Scilife. QbD also contributes to the Persomed story where we actively contribute to innovative personalized medicine.

Dagmar HOEBEN

EVT Lead Ophthalmology Benelux (Janssen Pharmaceuticals)

Together through the Gate towards a brighter future for patients with Inherited Retinal Diseases

INTERESTED IN COLLABORATING WITH GATE?

CONNECT TO THE GATE OFFICE

Organizing committee

Prof. Philip Blondeel

Prof. Robrecht Raedt

Dr. Bernard Depypere

Prof. Linos Vandekerckhove

Prof. Bart Leroy

Prof. Sandra Van Vlierberghe

An Van Den Bulcke - Chemtech

Tim Desmet – ACT-T

Gudrun Antoons - GATE Coordinator

Nele Pien - GATE Communication Manager

Karen Vervisch - Finances

GENE.CELL.TISSUE
ENGINEERING

Contact us

Gudrun Antoons
Gudrun.Antoons@UGent.be
T +32 471 4497341

info@gatehealth.be
gatehealth.be
https://www.linkedin.com/company/gatehealth

GHENT UNIVERSITY

UZ GENT

imec

VIB
science meets life