Welcome to the pitching session on:

Strengthening the European ecosystem for Advanced Therapy Medicinal Products (ATMPs) and other innovative therapeutic modalities for rare diseases

Presentation order	First Name	Last Name	Job position	Organization	Country
1	Els	Henckaerts	Professor	KU Leuven	Belgium
2	Claudia	Lobato da Silva	Associate Professor	IBB - Institute for Bioengineering and Biosciences	Portugal
3	Volker	Morath	Scientist	Technical University of Munich	Germany
4	Tero	Järvinen	Professor & Chief Surgeon	Tampere University	Finland
5	Raymond	Schiffelers	professor of nanomedicine	University Medical Center Utrecht	Netherlands
6	Félix	Montero Julian	Global Scientific Director Pharma Quality Control	bioMérieux	France
7	Michael	Linden	Head Gene Therapy Research Platform	UCB	Belgium
8	Alperen	Acemoglu	CTO	GlaucoT Teknoloji AS	Turkey
9	Andras	Dinnyes	director	BioTalentum Ltd	Hungary
10	Lorenzo	Moroni	Professor	Maastricht University - MERLN Institute for Technology-Inspired Regenerative Medicine	Netherlands

If you want to interact with other participants please use the chat function on the top right corner

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Before we start…

- We are recording this session and it will be published on the IHI website and B2Match platform.
- We will also publish the presentation slides.

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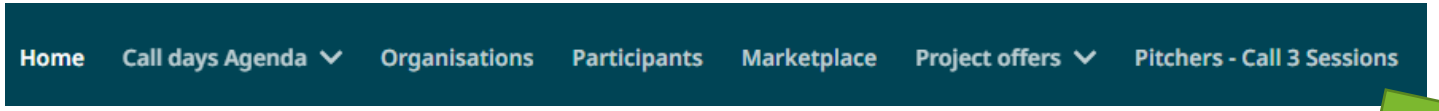
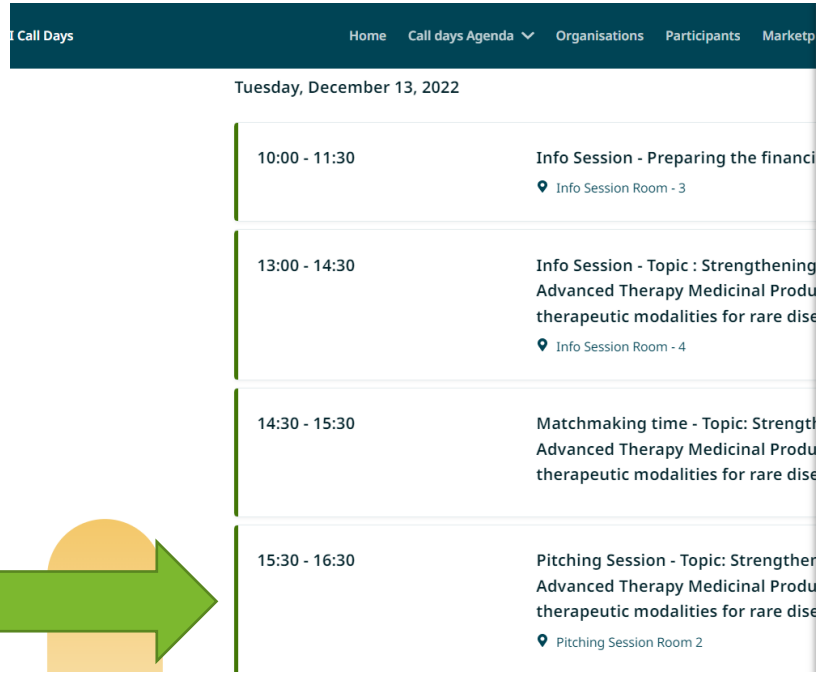
How to contact the presenters?





6	BioMérieux	Félix	Montero Julian	Director Pharma Quality Control	bioMérieux	France
7	ERD2: European Research and Development Ecosystem for Rare Diseases	Michael	Linden	Head Gene Therapy Research Platform	UCB	Belgium
8	Neuroprotective Glaucoma Treatment	Alperen	Acemoglu	CTO	GlaucoT Teknoloji AS	Turkey
9	Rare disease human in vitro preclinical testing with patient-specific iPSC line derived neuronal, neuroimmun and cardiac cell types	Andras	Dinnyes	director	BioTalentum Ltd	Hungary
10	A Biofabrication Toolkit to improve ATMPs for rare diseases	Lorenzo	Moroni	Professor	Maastricht University - MERLN Institute for Technology-Inspired Regenerative Medicine	Netherlands

SPEAKERS:

Alperen Acemoglu
CTO at GlaucoT Teknoloji AS

AD

andras Dinnyes
director at BioTalentum Ltd

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IHI Call Days | Call 3

- Strengthening the European ecosystem for Advanced Therapy Medicinal Products (ATMPs) and other innovative therapeutic modalities for rare diseases

AAV gene therapy development & manufacturing

Contact person name: **Els Henckaerts**

Organisation: KU Leuven, Laboratory of Viral Cell Biology & Therapeutics

E-mail: Els.Henckaerts@kuleuven.be

Link to:

- Marketplace opportunity: https://ihi-call days.ihi.b2match.io/marketplace/opportunities/UGFydGljaXBhdGlvbk9wcG9ydHVuaXR 5OjU0Mzg4
- Participant profile: https://ihi-call-days.ihi.b2match.io/components/25061?query=els%20hen

KU LEUVEN

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Challenges and objectives

9.1 million Euro invested by European Commission (RRF) in AAV GT facility	AAV discovery research Vector design & manufacturing
What is it? State-of-the-art **facility** enabling **preclinical development**, **base process development** and AAV **manufacture innovation**	**What is it?** Tackling off-target toxicity, longevity of transgene expression and manufacturing yield
Challenge? Full allignment with **industrial practise, end-to-end GT development**	**Challenge?** Access to regulatory, CMC and complex data analysis
Solution? Acces to network of biotech and pharma partners with relevant expertise	**Solution?** Acces to network of biotech and pharma partners with relevant expertise
Impact? Cost-effective, integrated GT development for rare diseases	**Impact?** Safer, cost-effective and long-lasting AAV gene therapy products

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Technical and scientific centre of excellence

Main activities

9.1 million Euro invested by European Commission (RRF) in AAV GT facility	AAV discovery research Vector design & manufacturing
AAV production (adherent & suspension) up to 50L	**Off-target delivery:** protein engineering, early CMC, in vitro & in vivo modelling, POC in disease models
Process development • base process • early CMC	**Viral epigenetics:** vector design, in vitro & in vivo modelling, HT sequencing & bio-informatics
Manufacturing innovation • plasmid/alternative DNA technology • additives • cell line development	**AAV manufacturing**: molecular virology, multi-omics, mathematical modelling and synthetic biology
Preclinical studies starting from solid POC	

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Technical and scientific centre of excellence

Expertise and resources offered

Expertise: AAV biology & vector design, process development and research-grade manufacturing, preclinical development, protein engineering

Infrastructure: newly built GT research facility (> 1000 m2) & core facilities

Team: skilled scientists, business development & IP

Partners: academia, clinicians, biotech and pharma

KU LEUVEN VIB Cima Universidad de Navarra UZ LEUVEN bicycle therapeutics TOUCHLIGHT ucb

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Expertise requested

- Pharma partners with experience in all aspects of AAV GT development
- Biotech companies providing complementary technologies
- Academic partners providing complementary expertise

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Strengthening the European ecosystem for Advanced Therapy Medicinal Products (ATMPs) and other innovative therapeutic modalities for rare diseases

BIO-INSPIRED CELL EXPANSION PLATFORMS TO PROMOTE THE FEASIBILITY OF AUTOLOGOUS TRANSPLANTATION FOR BONE MARROW FAILURE SYNDROMES (BMFS)

Contact person name: Cláudia Lobato da Silva / Sofia Martins

Organisation: iBB – Institute for Bioengineering and Biosciences, Instituto Superior Técnico, Lisboa, Portugal

E-mail: claudia_lobato@tecnico.ulisboa.pt / sofiamartins@tecnico.ulisboa.pt

Link to:

- https://ihi-call-days.ihi.b2match.io/marketplace/opportunities/UGFydGljaXBhdGlvbk9wcG9ydHVuaXR5OjU0NDkz
- https://ihi-call-days.ihi.b2match.io/participations/203006

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Challenges and objectives

- **THE PROBLEM**:
  - Allogeneic bone marrow (BM) transplantation (HCT) is unfeasible for patients lacking a suitable HLA-matched donor compromising BMFS treatment
  - Autologous HCT has been challenging due to reduced numbers of hematopoietic stem/progenitor cells (HSPC)

- **PROPOSED SOLUTION**:
  - ➢ Deciphering the molecular and cellular differences in the defective bone marrow microenvironment in a BMFS context towards the establishment of a **bio-inspired cell expansion device** able to recreate the elements of a functional niche to *ex-vivo* expand autologous HSPC

- **EXPECTED IMPACT**:
  - ✓ Improve the feasibility and widen the application of **autologous HCT as a first-line therapy** for patients in which allogeneic transplantation is unfeasible
  - ✓ Personalized *ex vivo* approach to identify new orphan drugs or optimize therapeutic regimens of currently administered drugs
  - ✓ Commercialization potential, also considering other potential applications *(i.e.* cell expansion device to surpass sub-optimal cell doses in autologous ex-vivo gene therapy settings, cord blood transplantation)

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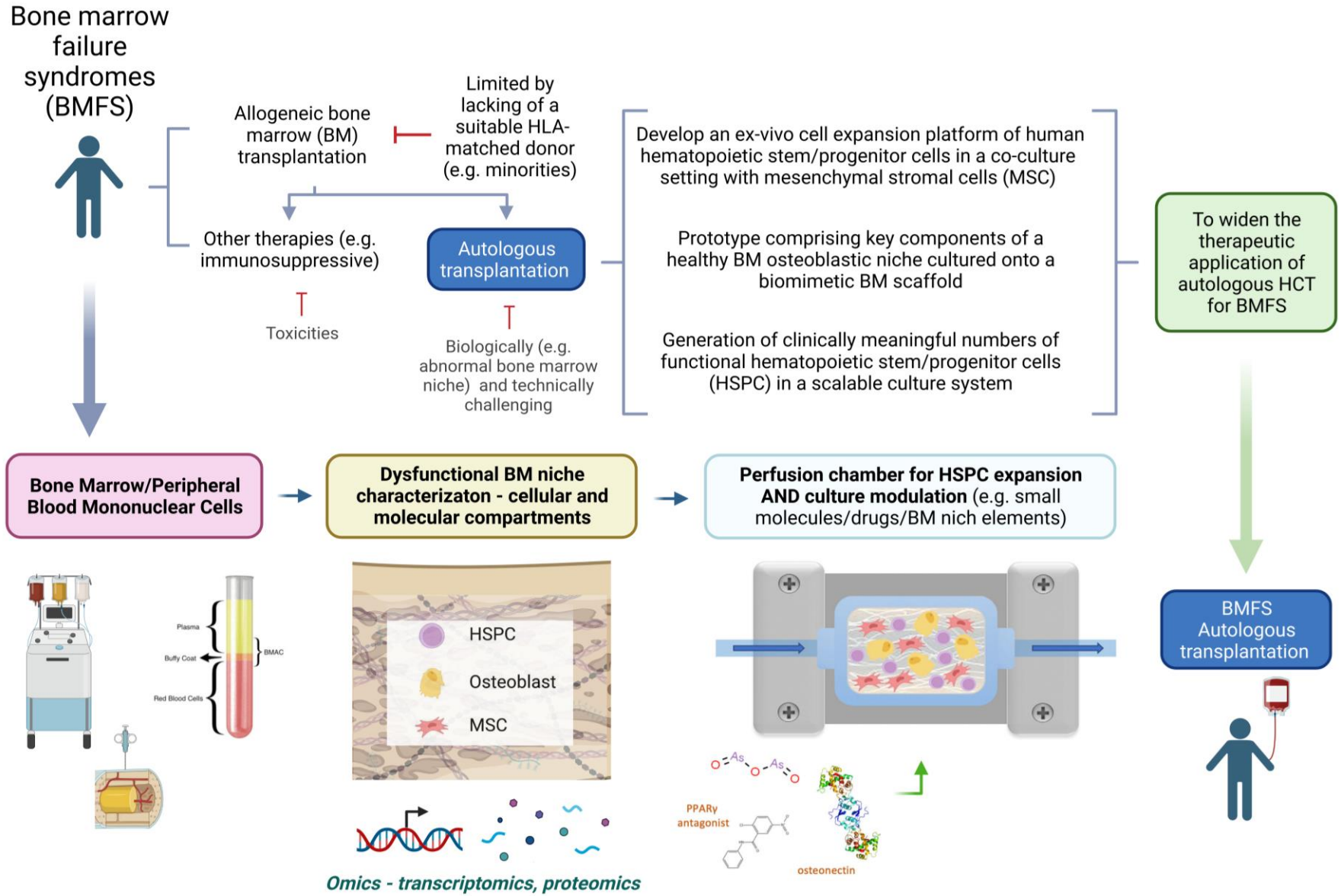
Main activities



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Expertise and resources offered

"***IST is well positioned to be an international leader in research and training programs in the field of biomanufacturing***"

(International Assessment of Research in Biological Engineering and Manufacturing reports (NSF, NIH))

- **Expertise:** (i) isolation and characterization of human HSPC and MSC; ii) GMP-compliant xeno(geneic)-free cultivation of human HSPC and MSC; iii) 2D and 3D co-cultures of human HSPC-MSC; iv) development of bioreactor platforms for stem cell cultivation; v) development of biomimetic ECM-based scaffolds.

- **Facilities:** two fully equipped cell culture labs, a bioengineering and analytical lab, a CRISPR – Cas9 lab

- **Clinical partner:** Pediatric Hematology Unit of Hospital D. Estefânia, Centro Hospitalar de Lisboa Central (CHLC) (Head: Paula Kjöllerström MD, National coordinator for the European Working Group of Myelodysplastic Syndrome (MDS) and Severe Aplastic Anemia (SAA) in children and adolescents)

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Expertise requested

- **Fundamental research centers** dedicated to Hematopoiesis and Hematopoiesis Stem Cell Biology (expertise with humanized mouse models for hematopoietic reconstitution, Advanced Bioimaging & Flow cytometry, …)
- **Multiomics Bioinformatics**
- **Clinical research centers**
- **Advanced cell therapy** SME
- **Scientific and Regulatory Affairs** consultant
- **Patient associations**

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Strengthening the European ecosystem for Advanced Therapy Medicinal Products (ATMPs) and other innovative therapeutic modalities for rare diseases

PET-Imaging of ATMPs using a novel Reporter Gene

Contact person name: Prof. Dr. med. Wolfgang A. Weber

Organisation: Technical University of Munich

E-mail: w.weber@tum.de

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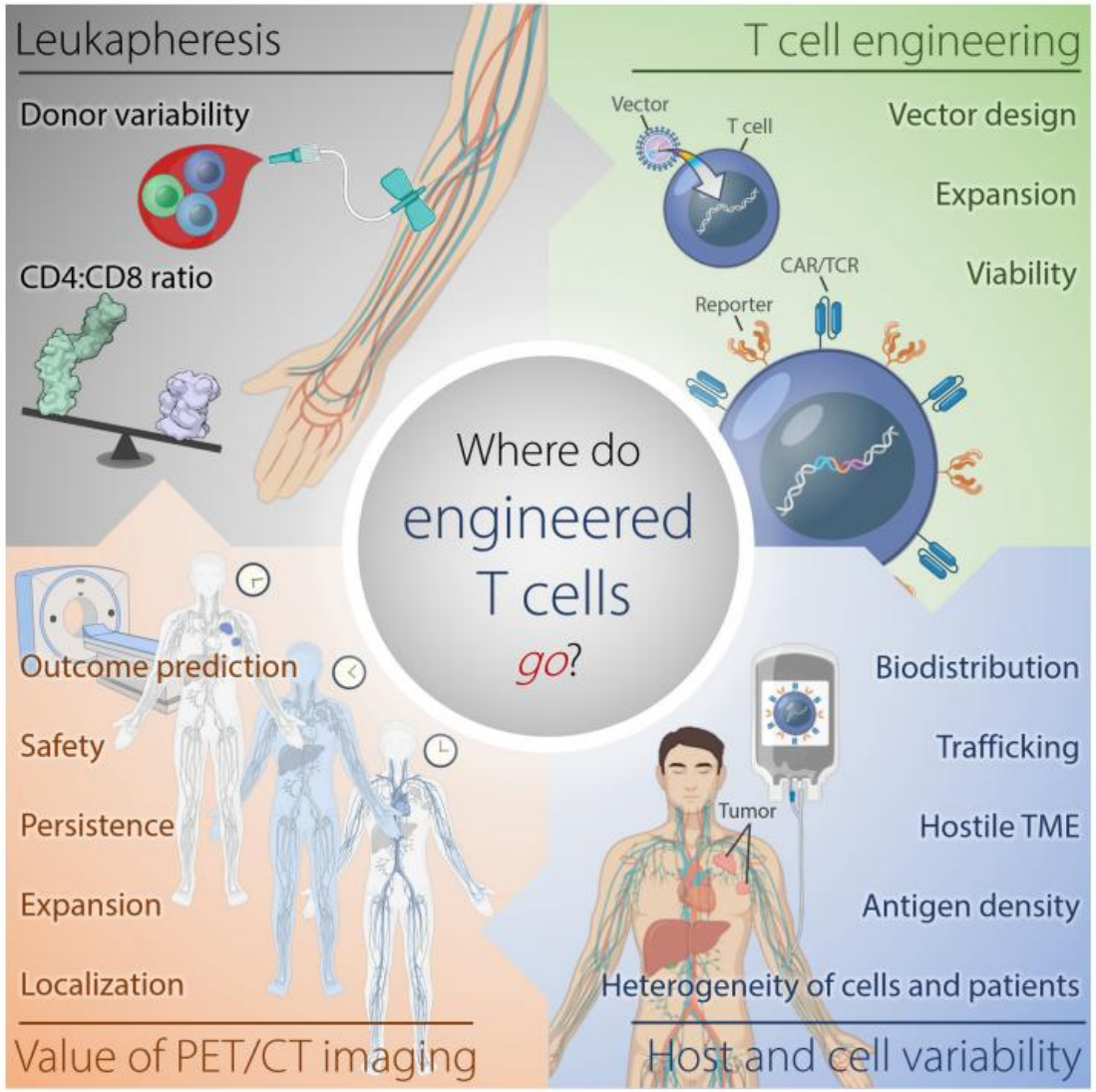
Challenges and objectives



Krebs et al., 2020 (10.3389/fonc.2020.577773)

- ATMPs (e.g. cell therapies) are non classical drugs that are difficult to monitor *in vivo* in animals and patients.
- We would like to contribute our expertise in Molecular Imaging and our services to analyze the ATMP, which is to be developed during the IHI project.

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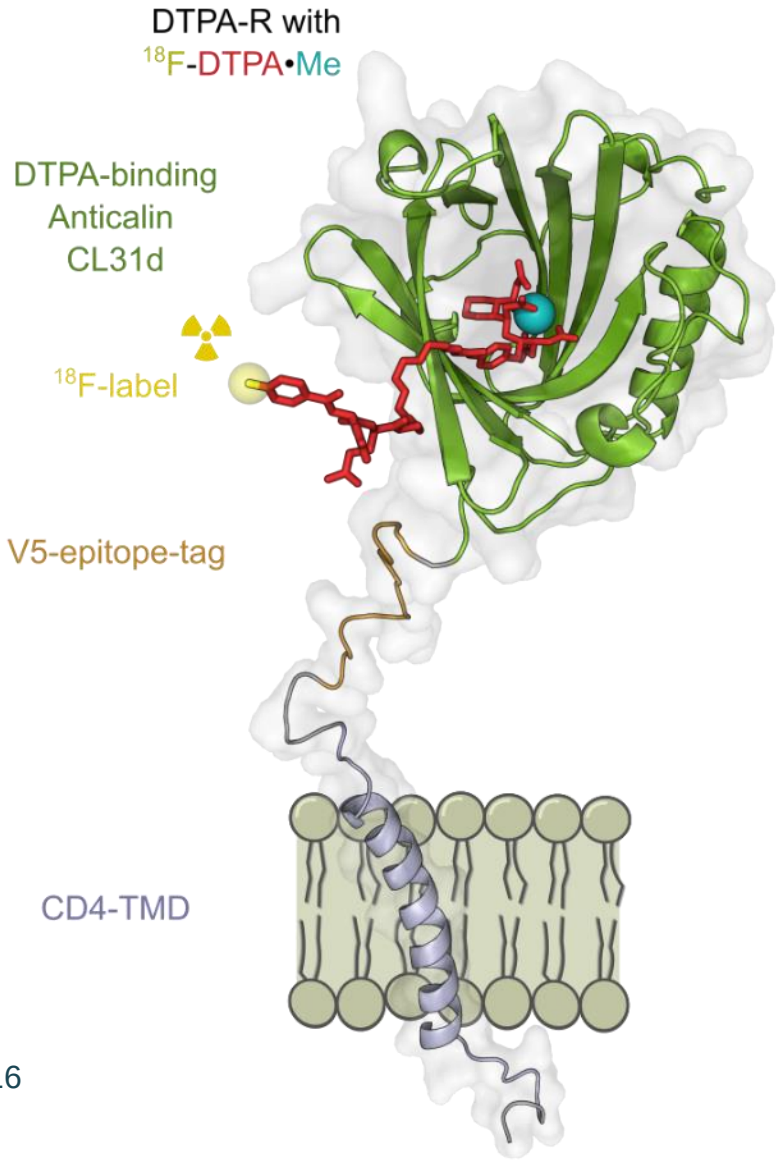
Reporter Gene Enables ATMP Imaging



- We have developed a novel and proprietary reporter gene technology. Please see WO2022101492A1 for further information.
- This reporter gene system is based on a membrane bound binding protein and an ^{18}F-labeled small molecule ligand.
- The reporter gene is called DTPA-R.
- DTPA-R offers highest sensitivity and specificity in class.

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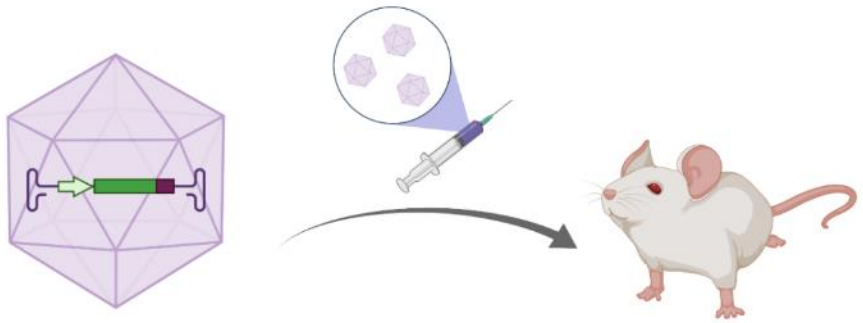
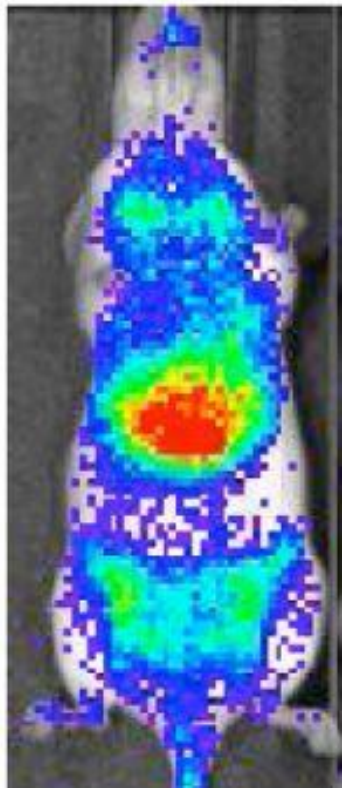
AAV9 Gene Therapy

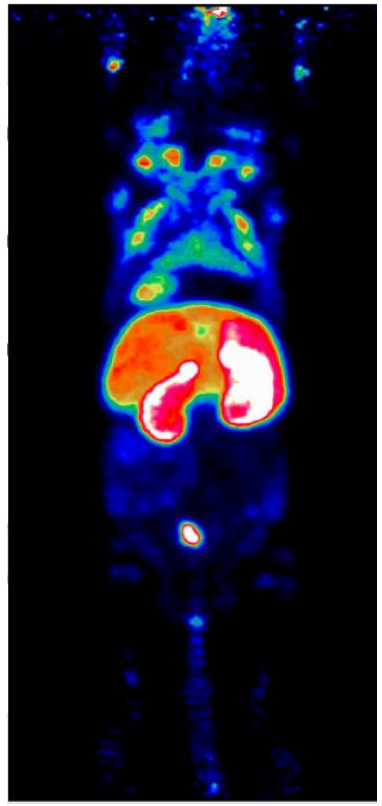
- AAV9 vectors encoding DTPA-R under the control of the CMV promoter were produced
- Nude mice were injected intravenously with up to $2.5x10^{12}$ vg)/mouse



BLI imaging of AAV9 transduction



PET imaging of AAV9 transduction



(Zincarelli et al., 2008)

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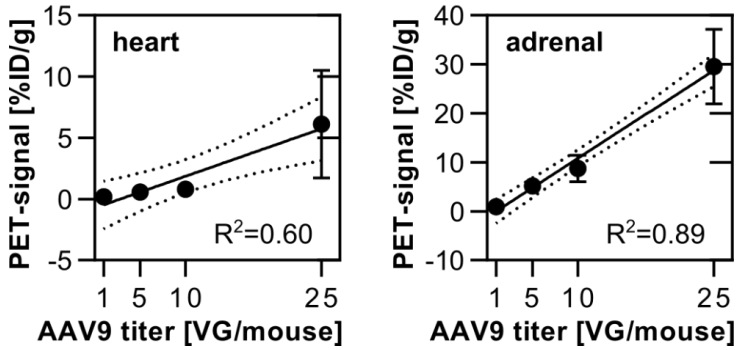
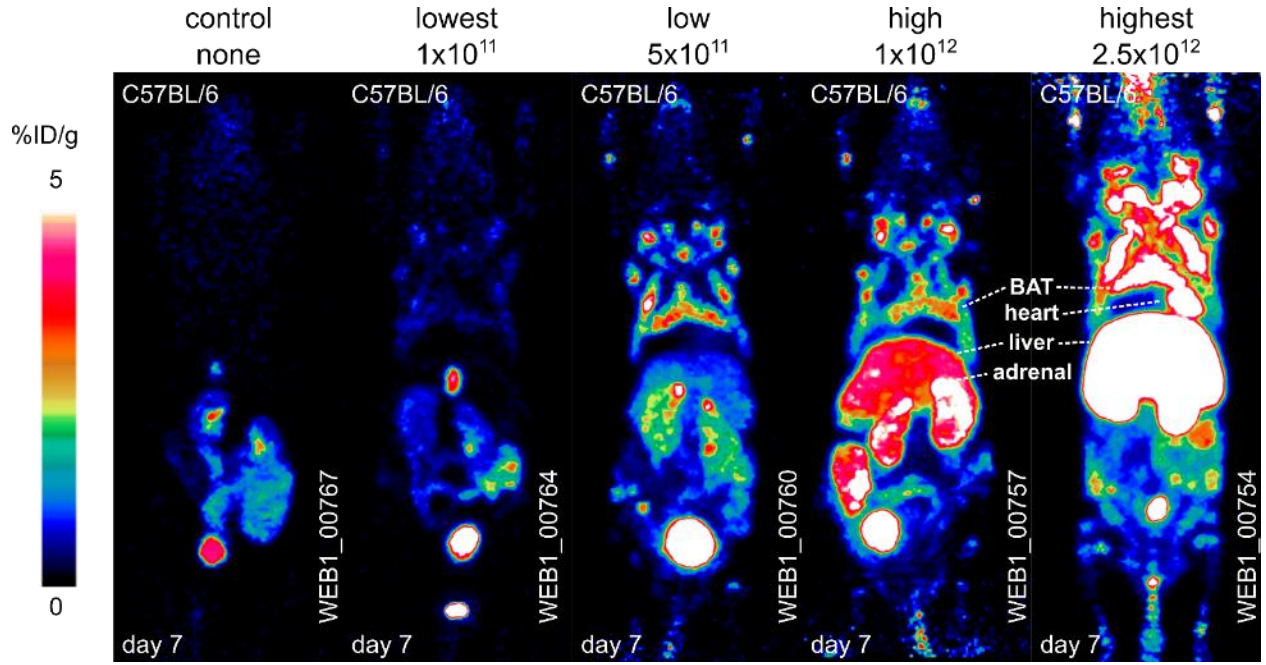
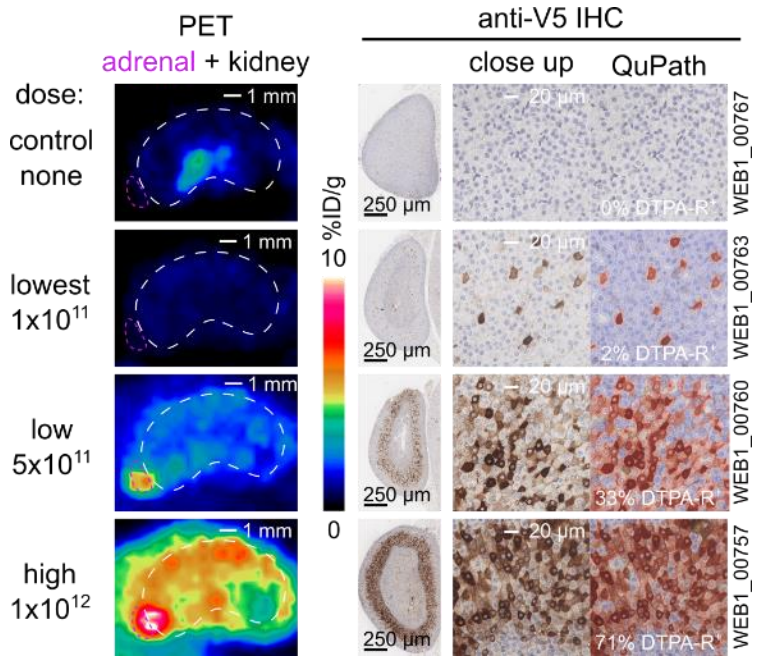
AAV9 Gene Therapy

- C57BL/6 mice were injected with $1x10^{11}$ to $2.5x10^{12}$ viral genomes / mouse.







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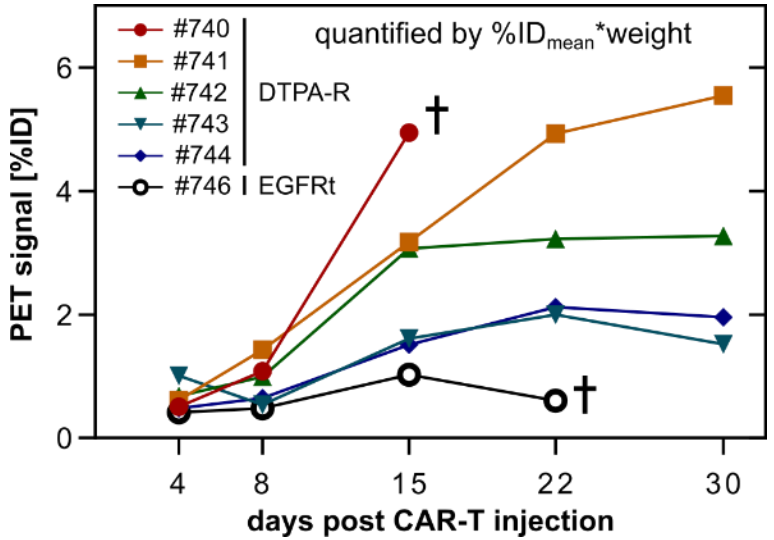
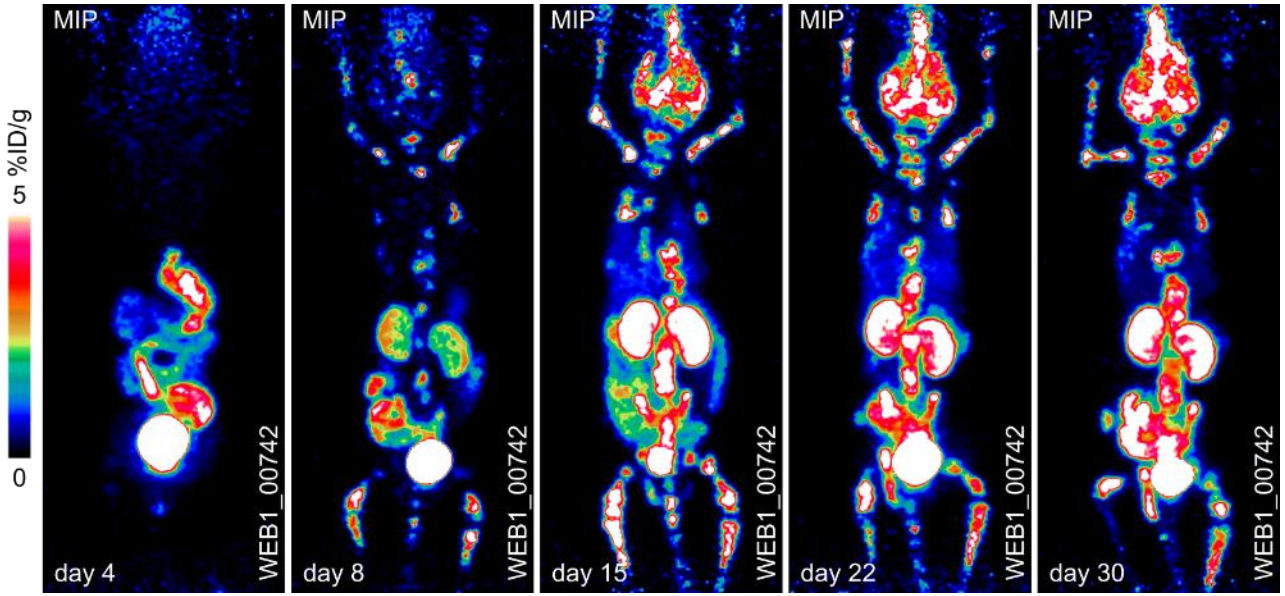
CD19-CAR-T Cell Therapy

- Immunocompromised NSG mice received an i.v. Raji xenograft & $2x10^6$ αCD19-CAR-DPTA-R cells i.v.
- PET imaging was done on a weekly basis
- CAR T-cell expansion was quantified in upper body and hind legs





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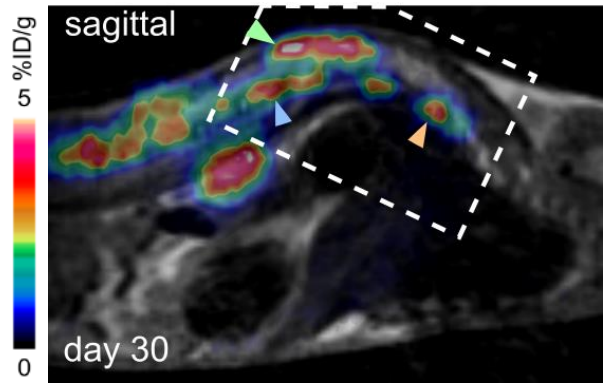
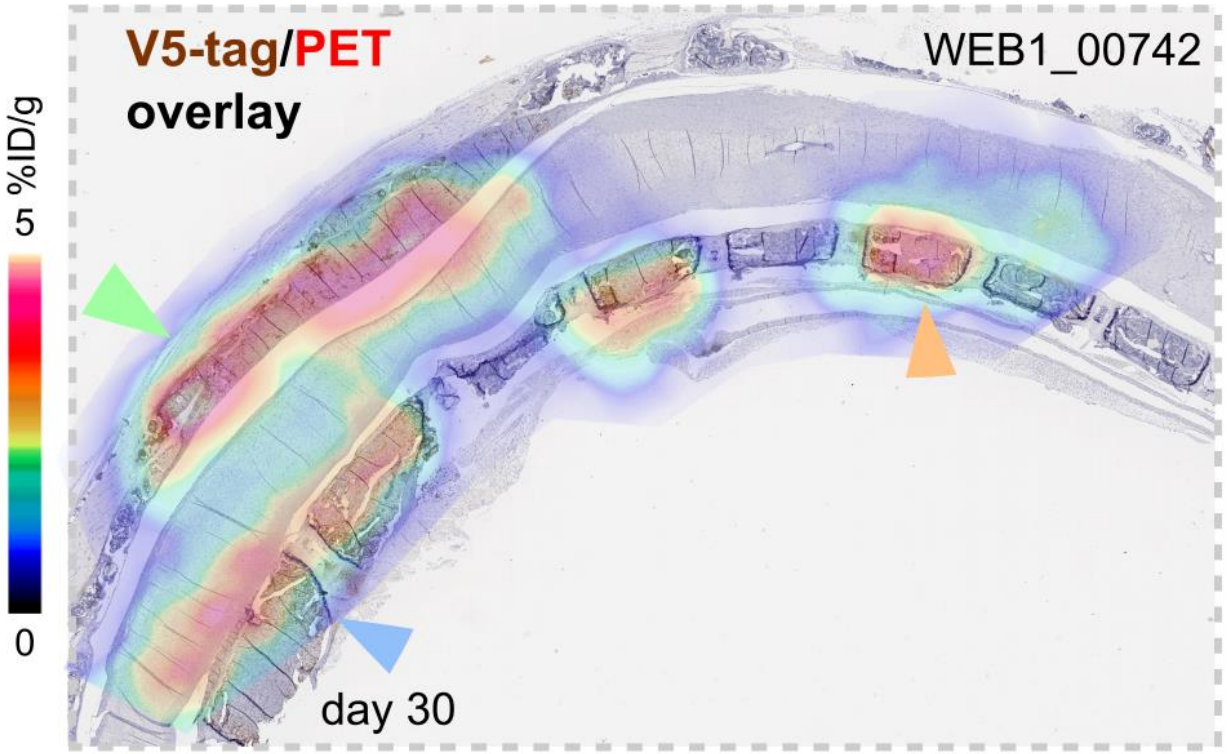
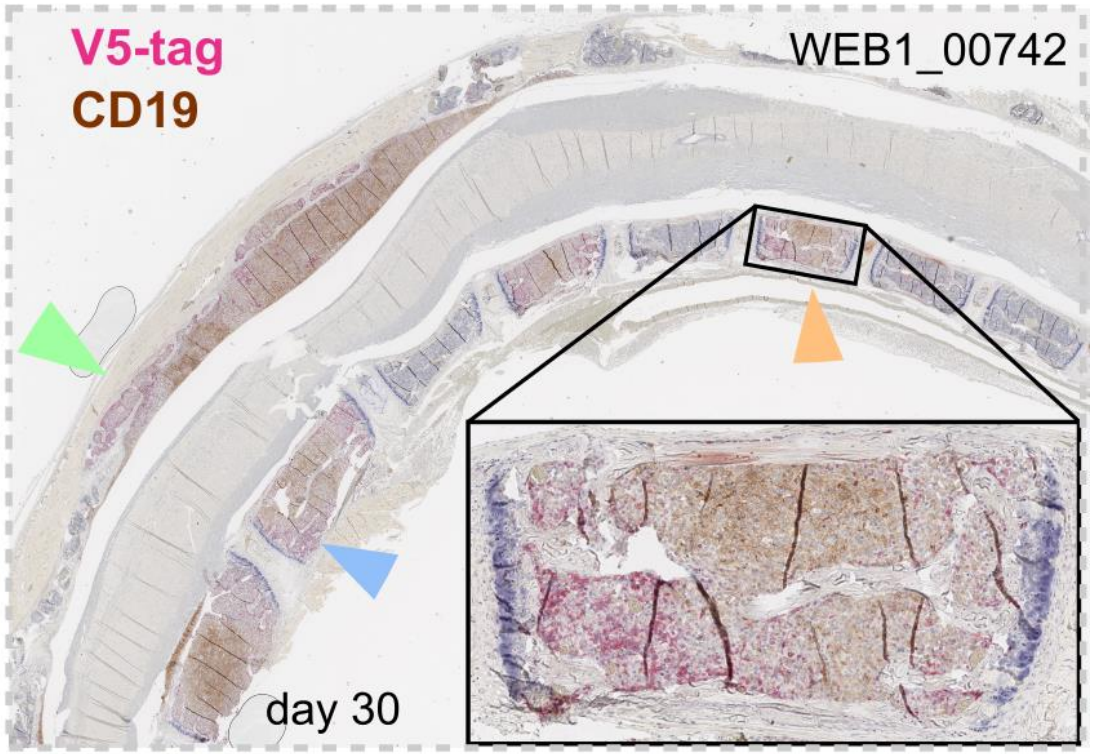
CD19-CAR-T Cell Therapy

- V5-IHC clearly identified vertebra with CAR-T infiltration
- Infiltration correlates with the PET-data





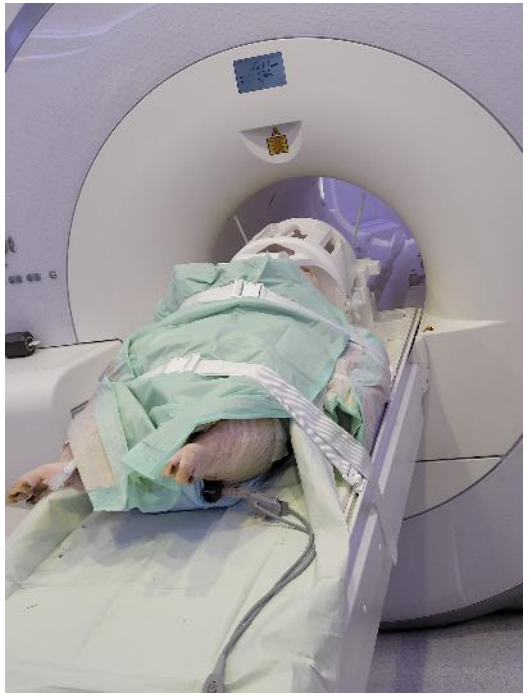


Animal models & 3Rs

- The 3 Rs stand for Replacement, Reduction and Refinement. Implementation of 3Rs is required by law.
- Molecular imaging (e.g. by PET) is one of the best ways to reduce number of animals used in experimentation and to refine the experiments by obtaining better quality data from every experiment.
- We are currently developing our reporter gene technology supported by the UK-based National Centre for the Replacement, Refinement and Reduction of Animals in Research (NC3Rs).

NC 3Rs Innovation Platform
Exploiting 3Rs technologies

- PET/MR & PET/CT scanners are available for different size animals, ranging from mice to minipigs.
- American *Association* for Accreditation of *Laboratory Animal* Care (AAALAC) certified facilities for small and large animals, up to BSL-2.



Minipig obtaining a PET/MR scan of the head

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Expertise and resources offered

- Our flexible reporter gene platform allows imaging of various ATMPs (Cell-, Viro-, or Gene-Therapy) in different animal models.
- Besides imaging of the ATMP (using a reporter gene) we can also image the disease by existing radioligands.

- Contact person: Prof. Dr. med. Wolfgang A. Weber
- Organisation: Technical University of Munich
- E-mail: w.weber@tum.de

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Thank you for your attention!

Prof. Dr. Wolfgang Weber
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Volker Morath
Katja Fritschle
Milica Zivanic
Prof. Susanne Kossatz
Philipp Bösl
Dr. Stephanie Robu
Luisa Krumwiede
Markus Mittelhäuser

Prof. Dr. Dirk Busch
Sarah Dötsch
Linda Warmuth

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PD Dr. Katja Steiger
Olga Seelbach
Marion Mielke

Prof. Dr. Christian Kupatt
Dr. Tarik Bozoglu

Matthias Ballhause (E&Z AG)

NC 3Rs Innovation Platform
Exploiting 3Rs technologies

MRI Klinikum rechts der Isar Technische Universität München

SFB 824

BayPAT

IHI Call Days | Call 3

Strengthening the European ecosystem for Advanced Therapy Medicinal Products (ATMPs) and other innovative therapeutic modalities for rare diseases

DecoDerma – Tranformative therapy for rare disease, epidermolysis bullosa

Contact person name: Tero Järvinen

Organisation: Tampere University

E-mail: tero.jarvinen@tuni.fi

Link to: https://www.tuni.fi/en/research/decoderma

- Marketplace opportunity: Therapeutic multi-functional recombinant protein for rare skin disease and beyond
- Participant profile: Interim CEO (Professor & Chief Surgeon)

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Challenges and objectives

- Aim is to develop a multi-functional recombinant protein as a drug for rare disease called epidermolysis bullosa
  - What problem are you trying to solve? Lethal Rare disease
  - Is your project suitable for IHI? YES
  - Introduction of potentially life-saving drug for lethal rare disease w. high unmet medical need

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Main activities

- Master cell bank (MCB) generation, recombinant protein expression & purification
- Pre-IND drug development
  - PK and Toxs studies in NHP
- Data package for IND approval
- Phase I & II clinical trials in EB

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Expertise and resources offered

- Final drug product identified
- Efficacy demonstrated in disease models
- Proof-of-concept (PoC) *in vivo*-studies performed in the most representative disease model
- Orphan drug designation (ODD) awarded
- CDMO confirmed the viability/feasibility of recombinant protein production
- Leading academic institute recruited for the phase I&II clinical trials

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Expertise requested

- Pharmaceutical companies as funding and research partner
- Research Institutes as research partners
- Experts on drug development to join the drug development team
- Patients advocates & organizations

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IHI Call Days | Call 3

ATMPs for rare diseases

Lipid-based nanomedicines

Contact person name: Raymond Schiffelers
Organisation: University Medical Center Utrecht
E-mail: R.Schiffelers@umcutrecht.nl

Link to:
- https://ihi-call-days.ihi.b2match.io/participations/192439/opportunities
- https://www.umcutrecht.nl/en/research/researchers/schiffelers-raymond-rm

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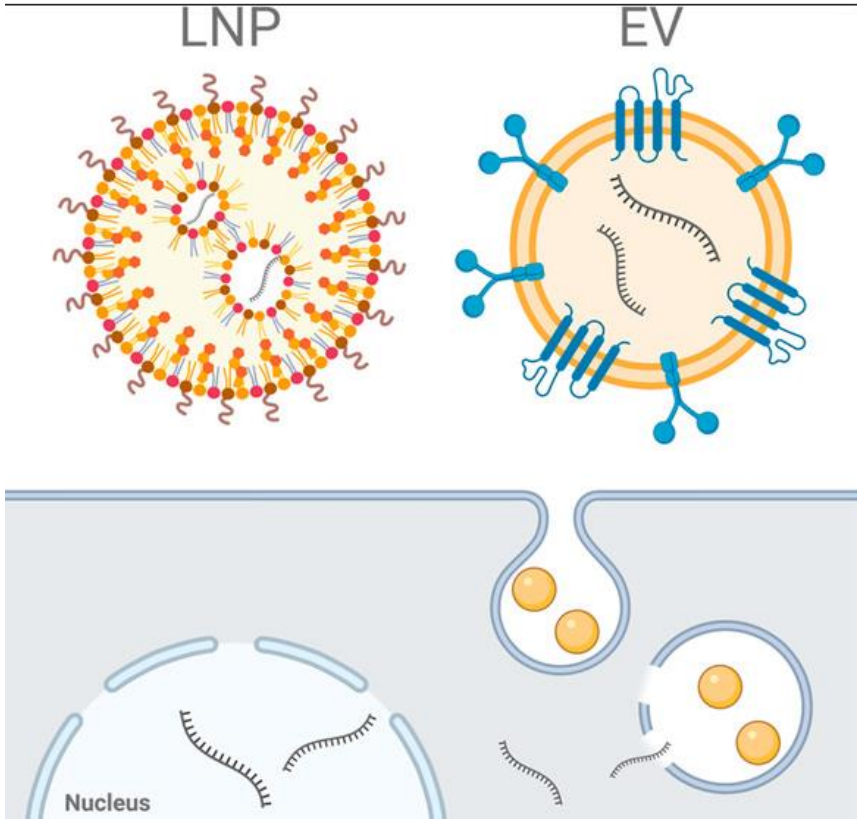
Challenges and objectives

- Nucleic acid therapeutics face difficulties reaching intracellular site of activity
- Design/Make/Evaluate Lipid-based nanomedicines that functionally deliver nucleic acids therapeutics
  - Natural (exosomes, extracellular vesicles)
  - Synthetic (LNPs, passive and actively targeted)
  - Hybrids



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Main activities

- Design, synthesis, characterization of lipid based nanomedicines
- Evaluate performance in sensitive in vitro/vivo reporter systems (CRISPR/Cas; Cre/Lox based)
- In vitro/vivo therapeutic testing (campus network)
  - Metabolic diseases (e.g. MMA-L-CHAD, patient organoids, zebrafish/mouse)
  - Cardiac (e.g. PLN, patient organoids, mouse)
  - Rare tumors (e.g. GBM, patient organoids, microsectioned tissue, mouse)

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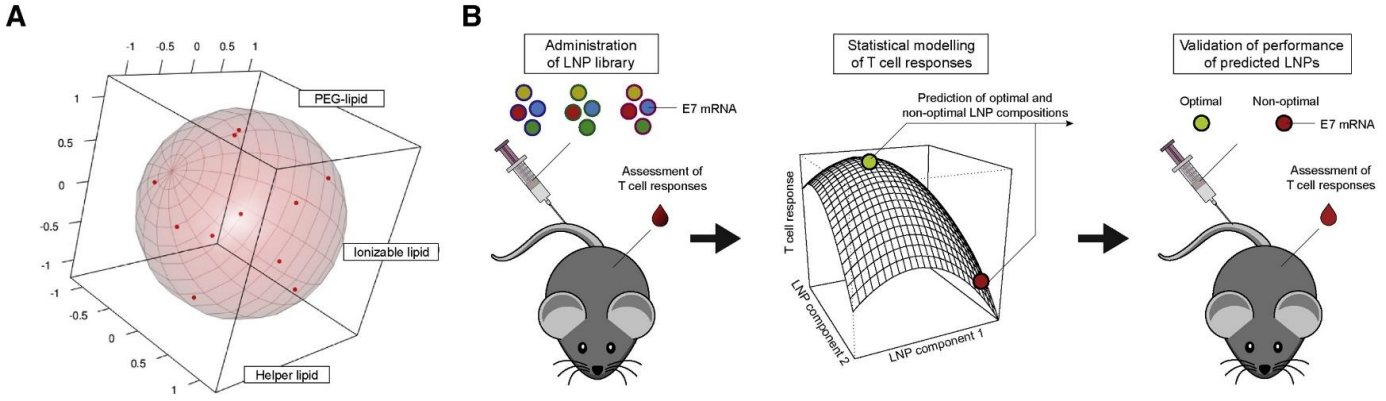
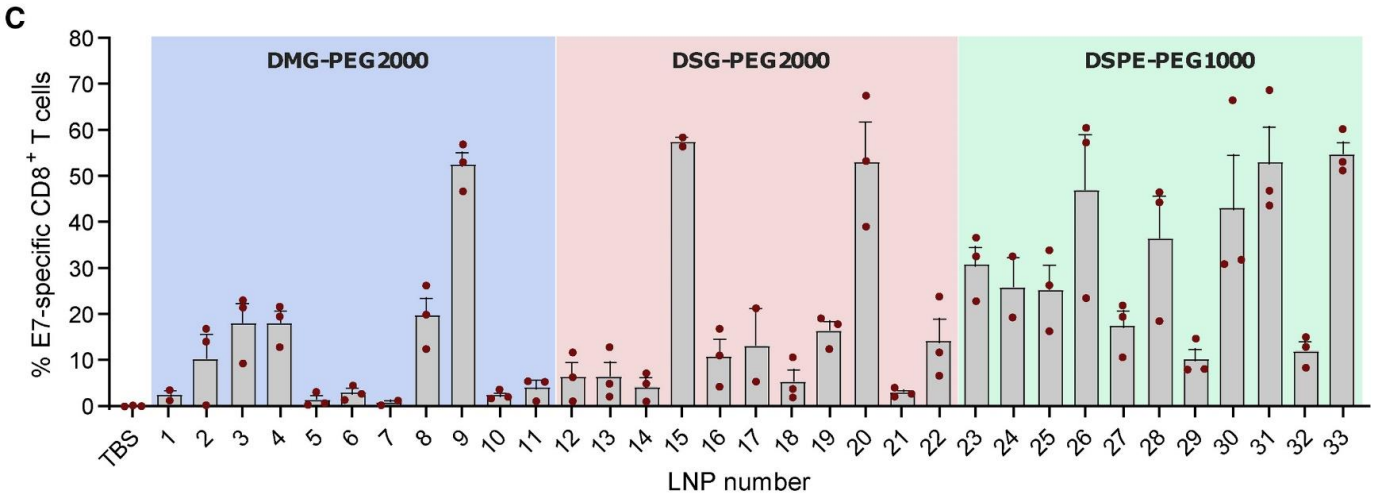
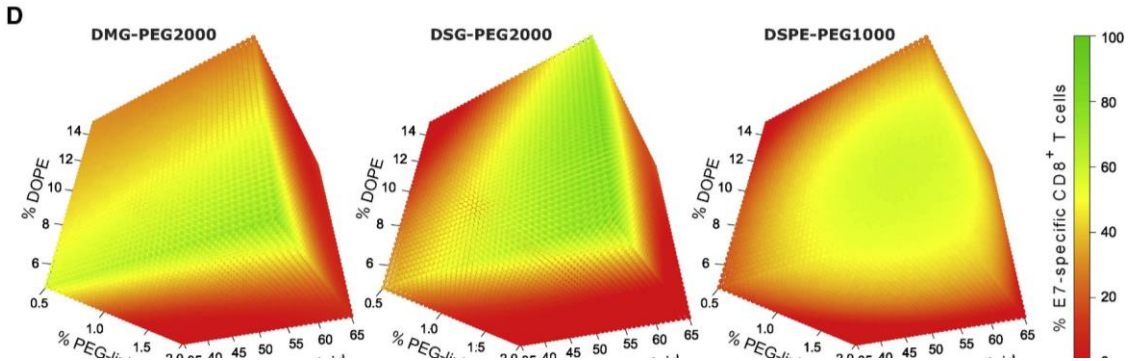
Library design of LNPs to generate immune reactive T-cells

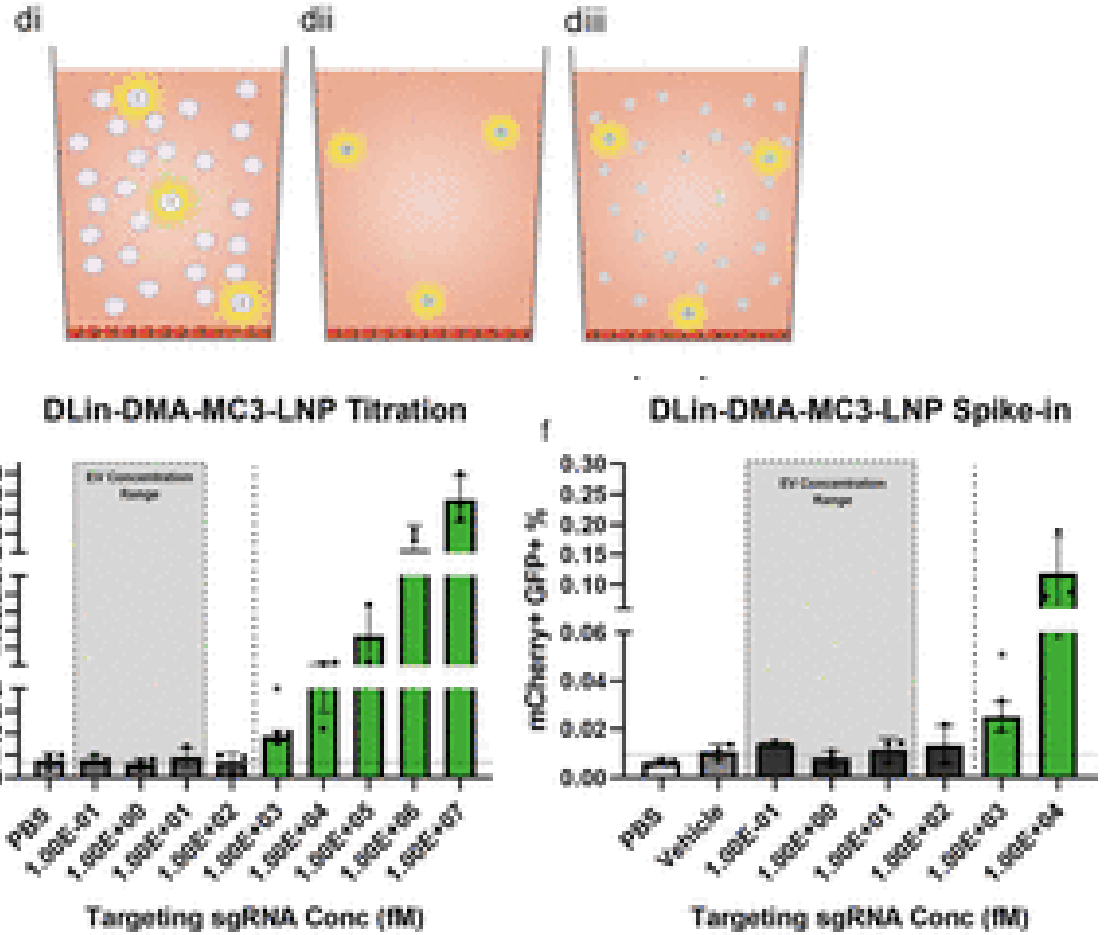


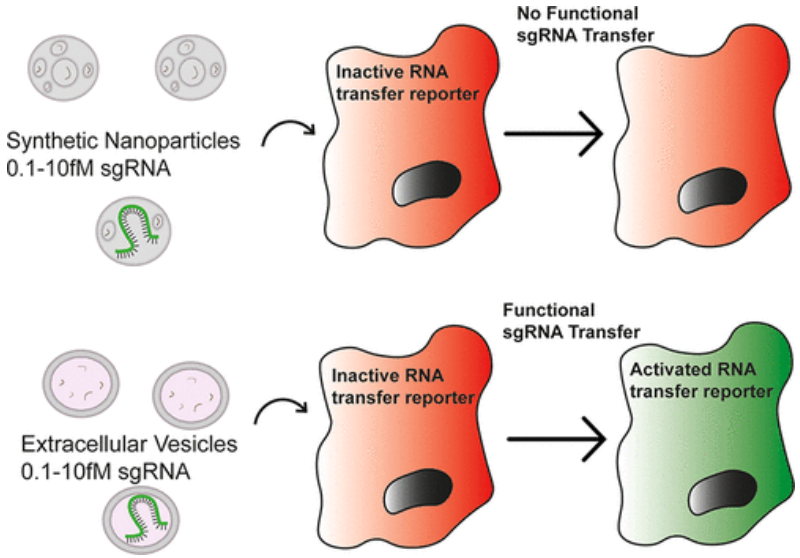




doi.org/10.1016/j.ymthe.2022.07.007

Head to head comparison in sensitive assays





doi.org/10.1021/acs.nanolett.1c00094

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Expertise and resources offered

- Local network, partners form EXPERT-consortium (https://www.expert-project.eu/), B-SMART consortium (https://b-smart-project.eu/)
- Fully equipped labs for chemistry, nanomedicine characterization, vitro/vivo testing, clinical partners,

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Expertise requested

- Large companies

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Topic 4: Strengthening the European ecosystem for Advanced Therapy Medicinal Products and other innovative therapeutic modalities for rare diseases

BioMérieux

Contact person name: Félix A. MONTERO JULIAN PhD

Organisation: bioMérieux

E-mail: felix.monterojulian@biomerieux.com

Link to:

- Marketplace opportunity
- Participant profile

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Challenges

The availability of relevant analytical tools is essential in ATMP manufacturing and process development, where they are used to control the product quality and safety, and to enable process improvement.

The development, manufacturing and commercialization of ATMPs products presents unique challenges to the whole ATMP ecosystem:

➢An evolving **regulatory landscape**, rapid advances in **manufacturing technologies**, products with **complex mechanisms** of action (MOA) and inherent **variability** in starting cellular material, are just a few examples of these challenges.

➢ **Lengthy and complex** manufacturing processes and in some cases, **extremely short shelf lives** (e.g., days), **low production volumes**, complex raw materials

➢ Requires high **traceability**, **data integrity** and manage the **scalability** depending on patient demands.

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bioMérieux' objectives and expected outputs

To develop and improve technologies/processes, analytic tools, methods and assays, needed for the development and production of ATMPs

These analytical methods, fully adapted to the ATMPs, must compensate the challenges described previously.

- **bioMérieux' contribution to the outcomes of the program shall encompass:**
  - Adding rapid and easy-to-use analytical technologies that bring overall efficiencies to the manufacturing process through simplified workflows -> to improve patient safety and access
  - Contributing to the making of a sustainable and accessible network of interconnected centres of excellence in ATMPs
  - Contributing to a legible regulatory landscape
  - Establishing standards

Expected outputs suitable for IHI

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Main activities as a IHI partner

- Identify the unmet needs in terms of analytical methods
- Select and test breakthrough technologies allowing their rapid implementation in ATMP’s cell-based process
- Contribute to the scouting and networking of centres of excellence
- Contribute to developing standards and feed into the European regulatory landscape

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Expertise and resources offered*

Experience in analytical methods design – MedTech and industrial applications

IMMUNOASSAYS

MOLECULAR BIOLOGY

MICROBIOLOGY

CELLULAR BIOLOGY

* IKOP - in-kind contributions to operational activities
* IKAA - in-kind contribution to additional activities

TBD

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Expertise and resources offered

- Improve technologies/processes, analytic tools, methods, assays
- Expertise in MedTech development - diagnostics
- Medical and pharmaceutical expertise
- Expertise in Immunomonitoring approaches
- Technologies for potency monitoring
- Expertise in regulatory aspects of analytical methods (ICH's Pharmacopeias, others)
- Experienced interactions with ecosystem stakeholders (ex, NIBRT, Catapult, NECSTGEN)
- Project management
- Project communications
- Contribute knowledge and expertise in legal, ethical and regulatory questions

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Expertise requested

- bioMérieux is interested in joining an existing consortium as a partner and as such, will be looking for the following profiles within the consortium:
  - ATMP developers
  - Pharma industry
  - Biotech and MedTech
  - CRO
  - CDMO
  - Patient organizations
  - Academic centers of excellence in Cell and Gene therapy

IHI Call Days | Call 3

Topic 4:
ERD²: European Research and Development Ecosystem for Rare Diseases

Contact person name: Michael Linden and Dirk Vander Mijnsbrugge

Organisation: UCB and Pfizer

E-mail: michael.linden@ucb.com; dirk.vandermijnsbrugge@pfizer.com

Link to:

- https://ihi-call-days.ihi.b2match.io/participations/202167/opportunities

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ERD^2 Project Proposal

Vision: To **establish strategically targeted networked centres of excellence that** are focused on ATMP technologies, ultimately benefiting patients through an increased availability of effective and safe treatments for rare diseases.

Expected Outcomes

- **Sustainable networks of excellence** accessible by all involved in the development of ATMPs & represents the most promising & impactful technologies
- **Agreed standards**
- A **streamlined and more transparent regulatory pathway** to optimise and speed up the development and delivery
- **Improved technologies/processes, analytic tools, methods, assays**

Expected Impacts

- Development will be **streamlined thanks to enhanced scientific & technological processes**.
- **Europe becomes more attractive for developing ATMPs**
- Ability of networks of excellence to improve the development of specialised rare disease treatments is **anticipated to benefit a broader range of diseases beyond rare diseases**.

Main activities of the ERD^2 project

- **Identifying the science and technology barriers** that are limiting the rapid and cost-effective development of in-vivo approaches for ATMPs and other innovative therapeutic modalities to underserved rare disease patients.

- **Build a successful Network of Excellence model** for the European ecosystem.

- **Regulatory (EMA/FDA) input and engagement**

- **Test the functionality** of the networks of excellence and the utility of the fit-for purpose processes developed both within the consortium and through use cases external to the consortium.

➢ ***Clinical trials are out of scope within the ERD^2 project. External use cases could potentially involve partners who are progressing their own clinical trials.***

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Possible examples of challenges* to focus on within The Networks of Excellence

Regulatory Activities

- **Align on technology-specific but program agnostic stepwise criteria** for development of novel technologies
- **Identify what can be leveraged** from real examples to progress new technologies and shape guidance on best practice from concept through to full validation.
- Maximisation of regulatory science by engagement in Agency pilots and enhanced interaction programs.

mRNA

- **Enable Targeted Delivery**

Methods developed to target distribution to specific tissue sub-types

- **Increase the stability of synthetic RNA**

To improve delivery into pre-clinical models which will also enable RNA Medicines to be used in a broader range of indications and could also reduce dosing frequency

NanoParticles (NP)

- **Lipid NP delivery of RNA**

To allow greater available/usable RNA (e.g., could reduce dosing frequency etc.) with data and results agreed with regulators to allow solutions to be incorporated in pre-clinical programs

Gene Therapy/Editing

- **Platform for Vector Production & Design**

Establish a pre-competitive infrastructure that is robust, reliable, scalable and high yield

- **Framework for standardisation** for assays and analytical tools
- **Research parameters** for vector potency

*These are proposed themes. Final selection will be made by the consortium partners

Anticipated to be in-vivo approaches only

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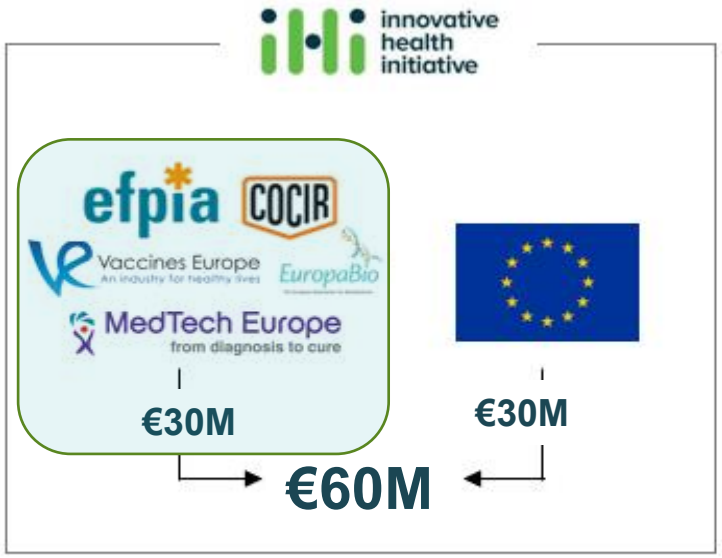
In-kind Expertise from Industry Partners (TBD)

Anticipated expertise contributions:

- Real-world expertise in RD therapeutic development
- Real-world evidence & expertise in regulatory aspects
- Experienced interactions with ecosystem stakeholders
- Medical expertise
- Digital medicine and diagnostics
- Project management
- Project communications
- Contribute knowledge and expertise in legal, ethical and regulatory issues



- 14 Medtech, EuropaBio and pharma companies in discussions, with 5 companies already providing indicative in-kind contributions.

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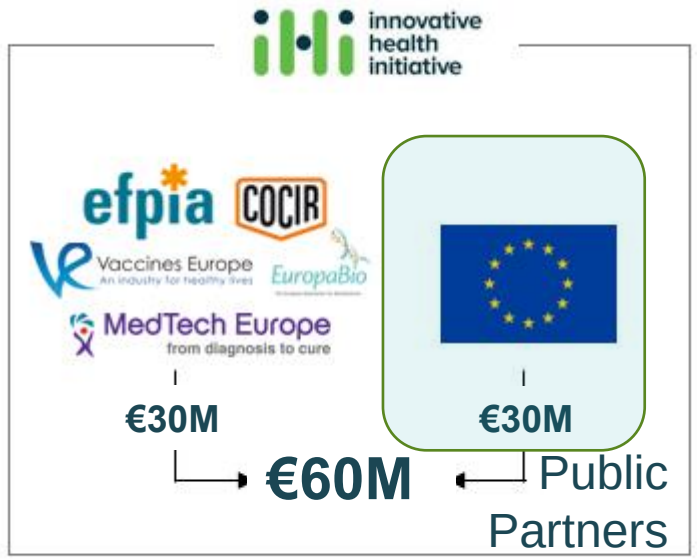
Expertise Needed from EU Public-Sector Partners (TBD)

Clinical Trials activities are NOT the Focus

- **Expertise in Gene Therapy, mRNA, NP/LNPs**
  - Understand & ability to solve science and technology challenges
- **Expertise in Real World Evidence and Natural History (non-interventional / pre-interventional)**
  - Understand patient & unmet needs
- **European Rare Disease Ecosystem Expertise**
  - Stakeholders, networks & knowledge sharing
- **Regulatory Expertise**
  - Agency practical expertise in the development through to registration space, especially within ATMPS/other innovative therapeutic modalities for RD.
- **Project co-ordination, management and communication**
  - Experience with coordinating and managing multi-year, multi-million-Euro projects with many contributors; previous IMI experience essential
  - Proven leadership in project communication and dissemination



- Academic organisations
- Regulatory authorities
- EU Patient organisations
- Non-Profit Research organisations
- Health Care Providers

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Note: EU public sector partners must be located within the EU or associated countries e.g. Norway, Israel or Turkey. Institutions in the UK, Switzerland and US are not eligible for IHI funding.

IHI Call Days | Call 3

Neuroprotective Glaucoma Treatment

GlaucoT

Contact person name: Alperen Acemoglu
Organisation: GlaucoT Teknoloji AS
E-mail: alperen@glaucot.com

Link to:

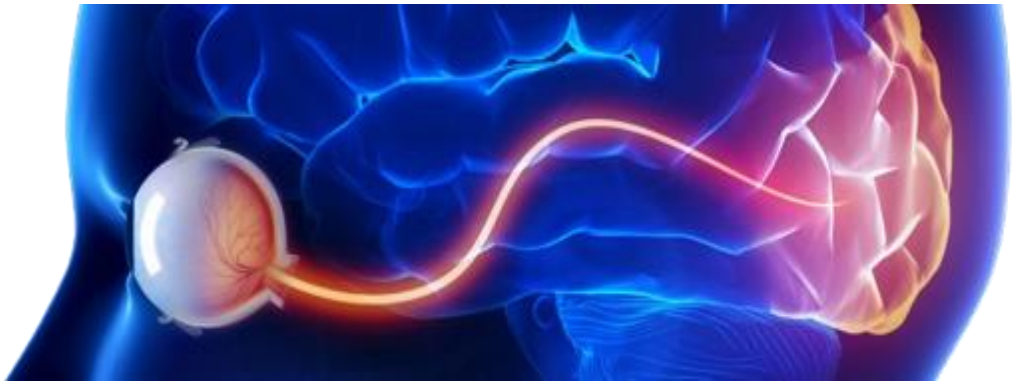
- Marketplace opportunity:
https://ihi-call-days.ihi.b2match.io/participations/196862/opportunities
- Participant profile:
https://ihi-call-days.ihi.b2match.io/participations/196862

GlaucoT
Innovation For Sight

innovative health initiative

Challenges and objectives

- Glaucoma is a Neurodegenerative disease!
  - Even if the patients use today's gold standard treatment, 15% of the patients go blind. This blindness is **irreversible**.
  - IHI Call 3 Topic 1 or Topic 5
  - Our noninvasive neuroprotective treatment method provides increased electrophysiological activity in the entire visual path.



GlaucoT
Innovation For Sight

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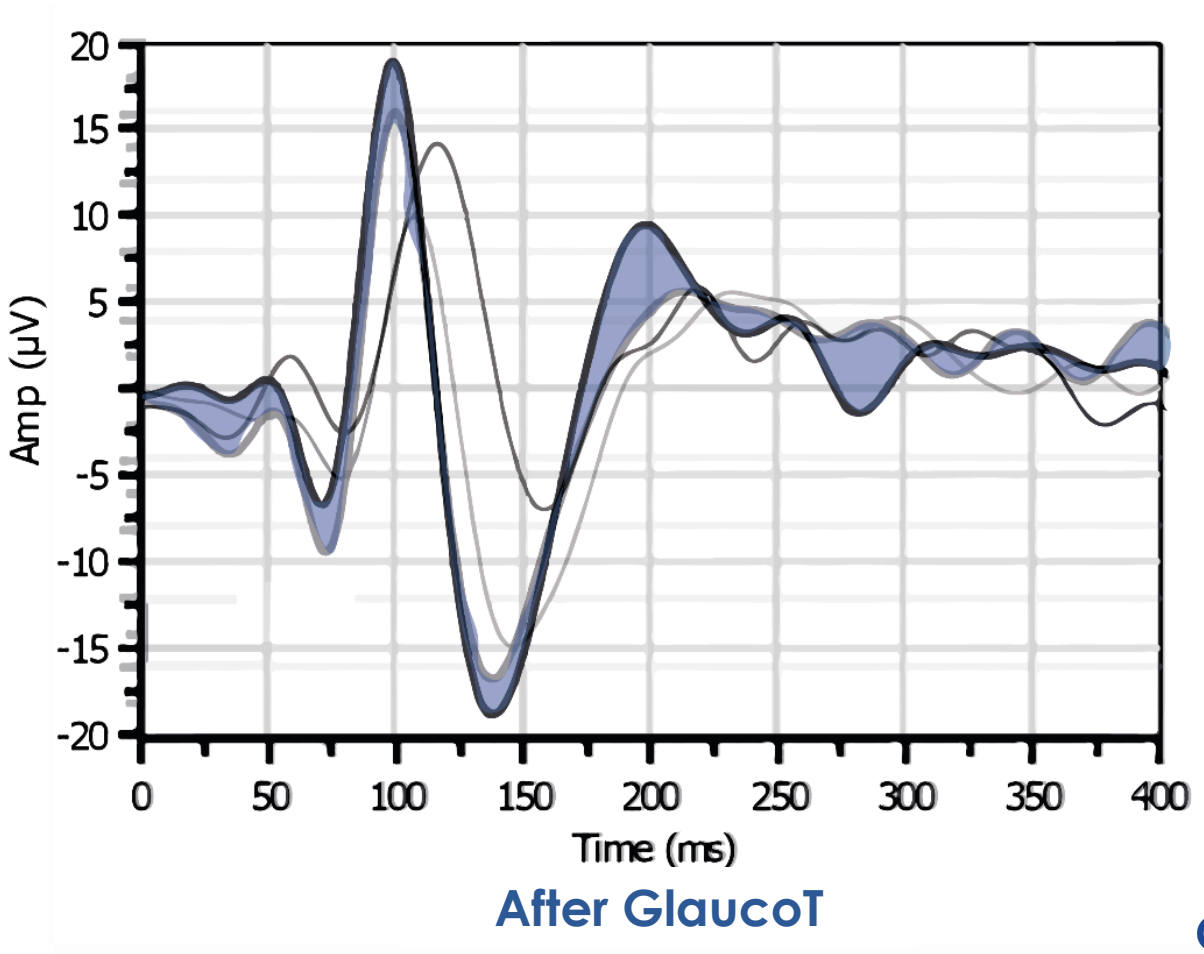
Clinical Data

Increased
Electrophysiological activity



GlaucoT
Innovation For Sight

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*https://clinicaltrials.gov/ct2/show/NCT05253534?term=glaucot&draw=2&rank=1

Main activities

GlaucoT

The World's First Neuroprotective
Wearable Glaucoma Treatment Device

- Our objective is to decrease the number of permanent blindness cases due to Glaucoma disease.
- In this project, our main activities will be
  - **Performing clinical trials** with the key opinion leaders for creating statistically significant data on larger patient populations.
  - **Wearable device developments** and improvements based on the feedback coming from the end users.
  - **Usability trials** of wearable glaucoma treatment device and the mobile application environment.

GlaucoT
Innovation For Sight

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Expertise and resources offered

We have an expertise in

- medical device *development* and
- *manufacturing* capabilities with a cleanroom and a sterilization center.
- commercial activity in ophthalmology market

At the moment, we perform clinical trials in Ege University and Manisa Celal Bayar University in Turkey.

GlaucoT
Innovation For Sight

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Expertise requested

- List profiles for desired partners, by category (SME, large companies, research institutes, other)
- Clinical centers
- Hospitals
- Ophthalmology departments
- KOL’s in Glaucoma and/or neurodegenerative diseases.

GlaucoT
Innovation For Sight
Redefining Glaucoma Treatment
GlaucoT
Innovation For Sight
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IHI Call Days | Call 3

Advanced Therapy Medicinal Products (ATMPs) for rare diseases

Rare disease human in vitro preclinical testing with patient-specific iPSC line derived neuronal, neuroimmun and cardiac cell types

Contact person name: **Prof Dr Andras Dinnyes**

Organisation: **BioTalentum Ltd**

E-mail: **andras.dinnyes@biotalentum.hu**

Link to:

- https://ihi-call-days.ihi.b2match.io/marketplace/opportunities/UGFydGljaXBhdGlvbk9wcG9ydHVuaXR5OjU0MDk0
- Participant profile: **https://biotalentum.eu**

Challenges and objectives

- Goal: providing an advanced tool for human in vitro testing of lead molecules or gene therapy prior to large animal preclinical or clinical studies
  - New innovative therapies might lead to costly failures in late preclinical and clinical stages.
  - Reliable small animal models often lacking for rare human diseases.
  - In the IHI risk mitigation for translational failures is an important issue.
  - The patient-derived iPSC-based in vitro platform, providing several organotypic models is a relevant proxy for the target patient population.

Main activities

- Using de novo or banked rare-disease patient hiPSC lines to generate the **relevant organotypic in vitro models** for the advanced therapy application.

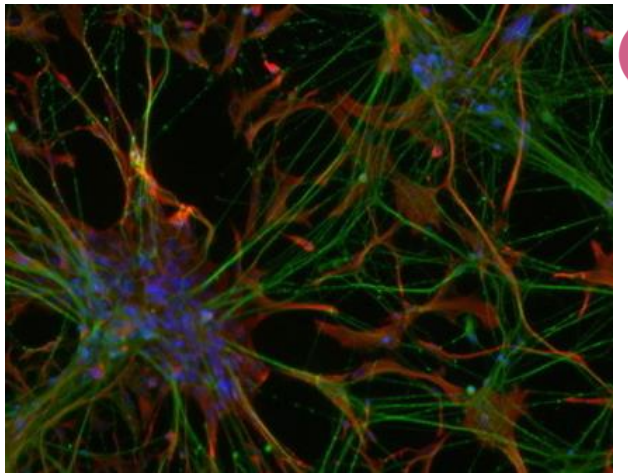
- Characterize and analyze in depth the models and test the ATMP candidates on the models, providing feed back to the development process in preclinical stage, derisking the clinical translational steps.

Expertise and resources offered

Human in vitro 3D models

- BioTalentum Ltd (BIOT) is generating human iPSC lines and models since 2009.
- We have **coordinated** 13 EU FP6/FP7/H2020 projects scientifically. https://biotalentum.eu/international-projects/
- We have generated and validated complex **2D and 3D models for neurodevelopmental, neurodegenerative diseases**, including several rare diseases with CNS phenotype.
- We are experienced in **microglia-like cell** generation and their incorporation in the 3D CNS models.
- Furthermore, **2D and 3D cardiomyocyte models** are produced at BIOT.
- We have an ongoing hiPSC-derived **beta-cell/Langerhans islet** program for regenerative medicine also suitable for in vitro modelling.

- **Read outs: high-content imaging, functional Ca2+ signaling, multi-omics, electron microscopy etc.**



Expertise requested

- We are looking for a consortium which would be interested to incorporate the in vitro models and assays into the preclinical pipeline for rare-disease ATMP development.
- If needed, we are also offering project preparation support via BioTalentum Consulting branch, based on our extensive experience in EU project management. https://biotalentum.eu/consulting/

IHI Call Days | Call 3

Strengthening the European translational research ecosystem for advanced therapy medicinal products (ATMPs) for rare diseases

A Biofabrication Toolkit to improve ATMPs for rare diseases

Contact person name: Lorenzo Moroni

Organisation: Maastricht University, MERLN Institute for Technology-Inspired Regenerative Medicine, Complex Tissue Regeneration department

E-mail: l.moroni@maastrichtuniversity.nl

Link to:

- Marketplace opportunity: https://ihi-call-days.ihi.b2match.io/participations/203450/opportunities
- Participant profile: https://ihi-call-days.ihi.b2match.io/participations/203450

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Challenges and objectives

- Describe the main objectives of your proposed project / proposal and how they address the outcomes and impacts of the topic.
  - Delivery of multiple biological factors in a controlled/targeted manner in 3D
  - Examples of successful use of biofabrication in ATMPs:
    - Skeletal (both bone and cartilage)
    - Kidney
    - Lung
    - Thryoid
    - Cardiovascular
    - Intestine
    - Metabolic disease
    - Neural (both CNS & PNS)

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Main activities

- Technology-centered
- Develop and characterize biological constructs able to deliver biological factors (e.g. mRNA, nucleotides, small molecules) in a control/targeted manner via controlling multi-material composition, architecture of final implant, for ATMPs.

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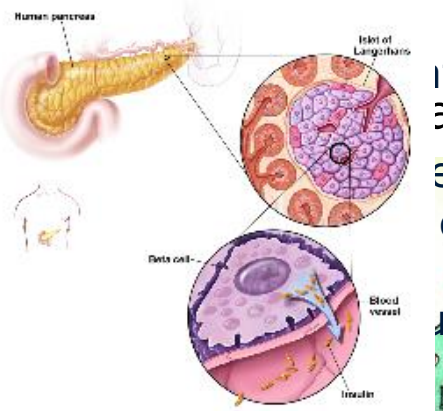
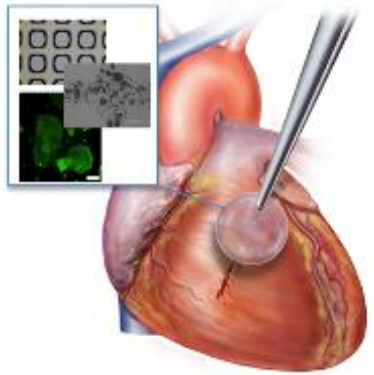
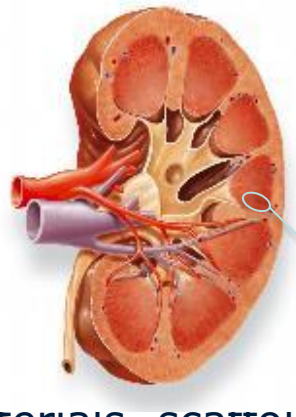
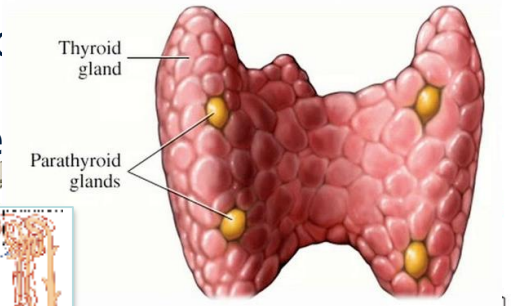
Expertise and resources offered

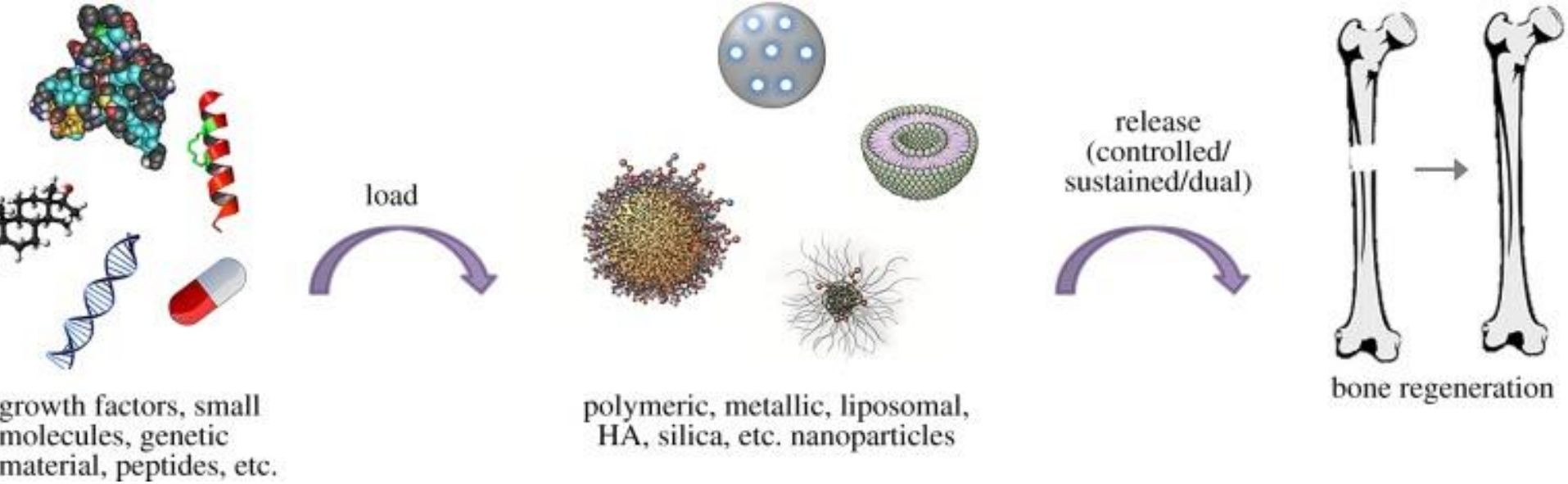
Islets transplant Cardiac tissue model *In vitro* nephron Thyroid 3D Screening











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- Immune response of biomaterials, scaffolds, and biomedical devices
- Apply biofabrication technologies to study regenerative and degenerative

Marchioli G et al., Biofabrication 2015
Marchioli G et al., Adv Healthcare Mater 2016

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Expertise requested

- Biological Factors;
- ATMP application;
- Preclinical & clinical;
- Regulatory;
- Health Technology Assessment;
- GMP production;

Welcome to the pitching session on:

Strengthening the European ecosystem for Advanced Therapy Medicinal Products (ATMPs) and other innovative therapeutic modalities for rare diseases

Presentation order	First Name	Last Name	Job position	Organization	Country
1	Els	Henckaerts	Professor	KU Leuven	Belgium
2	Claudia	Lobato da Silva	Associate Professor	IBB - Institute for Bioengineering and Biosciences	Portugal
3	Volker	Morath	Scientist	Technical University of Munich	Germany
4	Tero	Järvinen	Professor & Chief Surgeon	Tampere University	Finland
5	Raymond	Schiffelers	professor of nanomedicine	University Medical Center Utrecht	Netherlands
6	Félix	Montero Julian	Global Scientific Director Pharma Quality Control	bioMérieux	France
7	Michael	Linden	Head Gene Therapy Research Platform	UCB	Belgium
8	Alperen	Acemoglu	CTO	GlaucoT Teknoloji AS	Turkey
9	Andras	Dinnyes	director	BioTalentum Ltd	Hungary
10	Lorenzo	Moroni	Professor	Maastricht University - MERLN Institute for Technology-Inspired Regenerative Medicine	Netherlands

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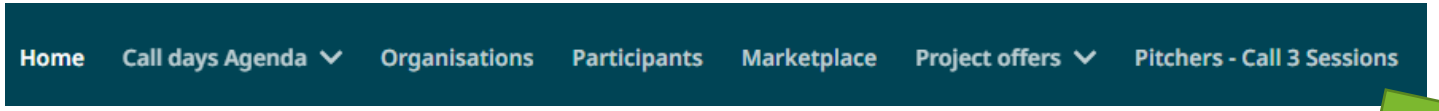
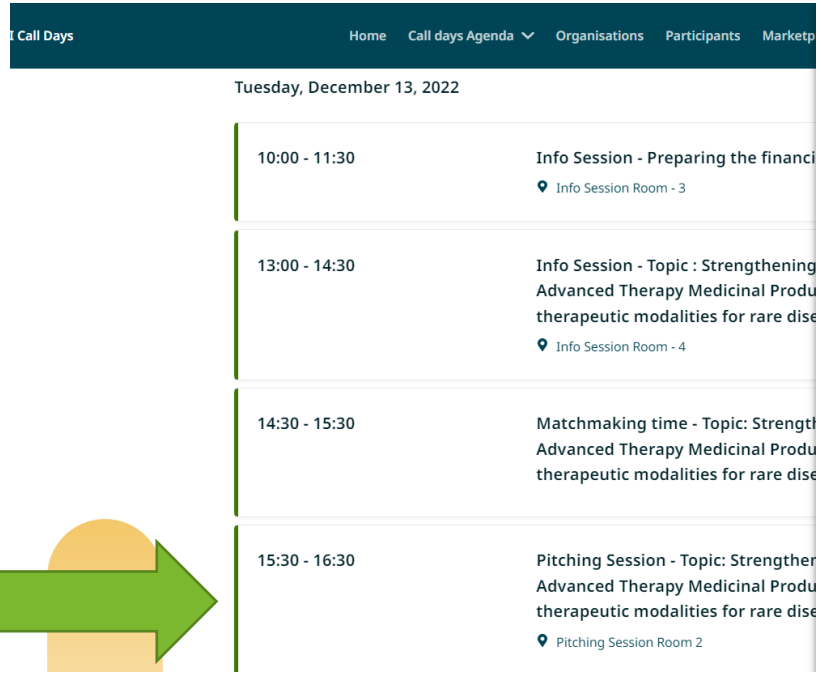
How to contact the presenters?





6	BioMérieux	Félix	Montero Julian	Director Pharma Quality Control	bioMérieux	France
7	ERD2: European Research and Development Ecosystem for Rare Diseases	Michael	Linden	Head Gene Therapy Research Platform	UCB	Belgium
8	Neuroprotective Glaucoma Treatment	Alperen	Acemoglu	CTO	GlaucoT Teknoloji AS	Turkey
9	Rare disease human in vitro preclinical testing with patient-specific iPSC line derived neuronal, neuroimmun and cardiac cell types	Andras	Dinnyes	director	BioTalentum Ltd	Hungary
10	A Biofabrication Toolkit to improve ATMPs for rare diseases	Lorenzo	Moroni	Professor	Maastricht University - MERLN Institute for Technology-Inspired Regenerative Medicine	Netherlands

SPEAKERS:

Alperen Acemoglu
CTO at GlaucoT Teknoloji AS

AD

andras Dinnyes
director at BioTalentum Ltd

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Thank you for your attention

ihi.europa.eu

COCIR Advancing Healthcare

efpia

EuropaBio The European Association for Bioindustries

MedTech Europe from diagnosis to cure

Vaccines Europe

Co-funded by the European Union