European Innovation Council

EIC PATHFINDER PORTFOLIO

CELL AND GENE THERAPY PORTFOLIO

Strategic Plan

BRUSSELS, NOVEMBER 2023

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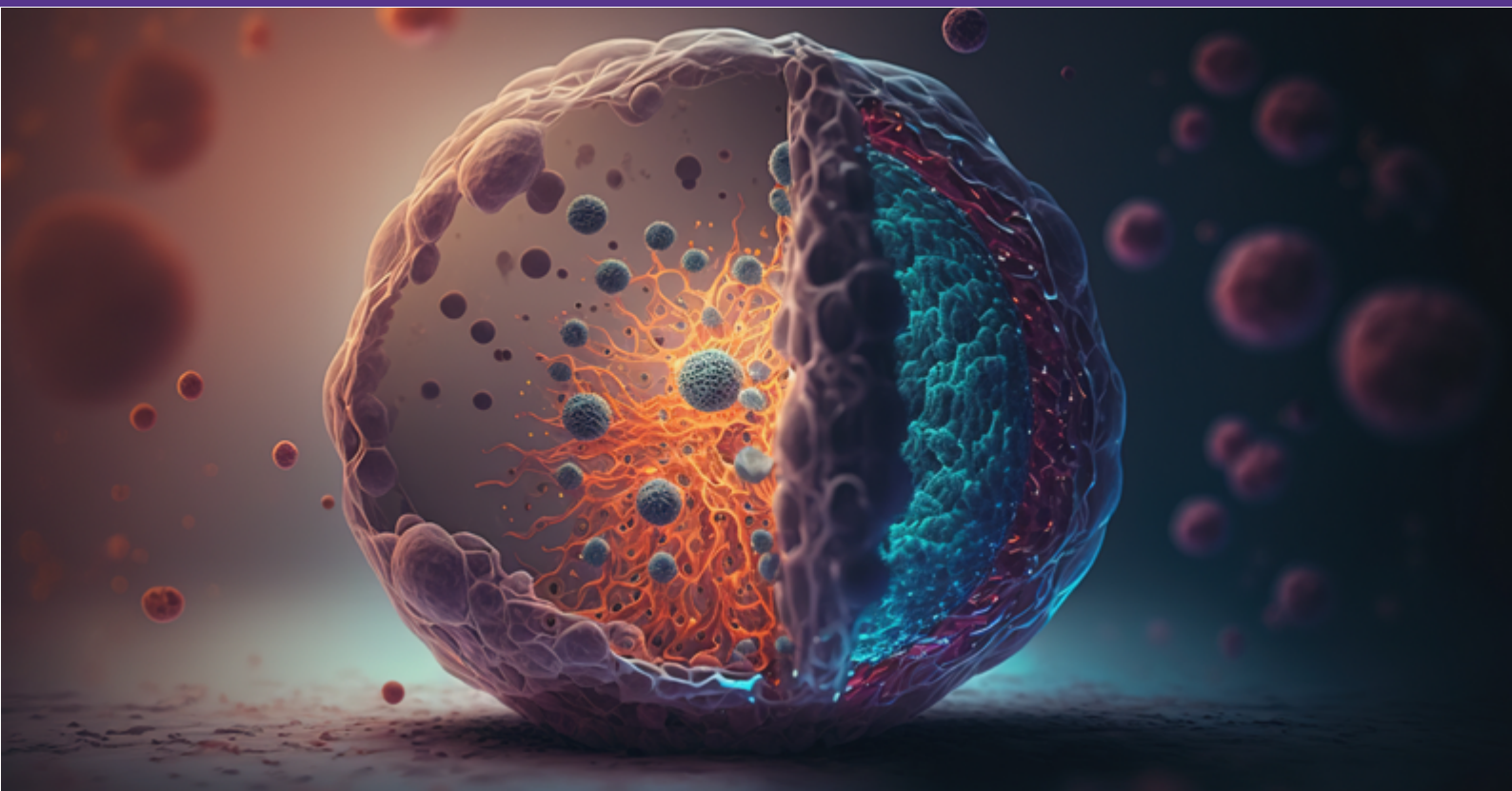

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1.
EIC CELL AND GENE THERAPY PORTFOLIO (CGT), PART OF THE EIC CGT STRATEGIC INITIATIVE

1.1
THE EIC CELL AND GENE THERAPY (CGT) STRATEGIC INITIATIVE

The EIC Program Manager (PM) for Health and Biotech introduced CGT as priority area to EIC in July 2020. Since then, he designed and brought forward a series of CGT strategic actions such as,

a. Targeted presentations to universities, biotech industry associations, policy-makers and biotech clusters on the current global CGT Trends and their revolutionary impact of medical treatment in a wide spectrum of diseases ;

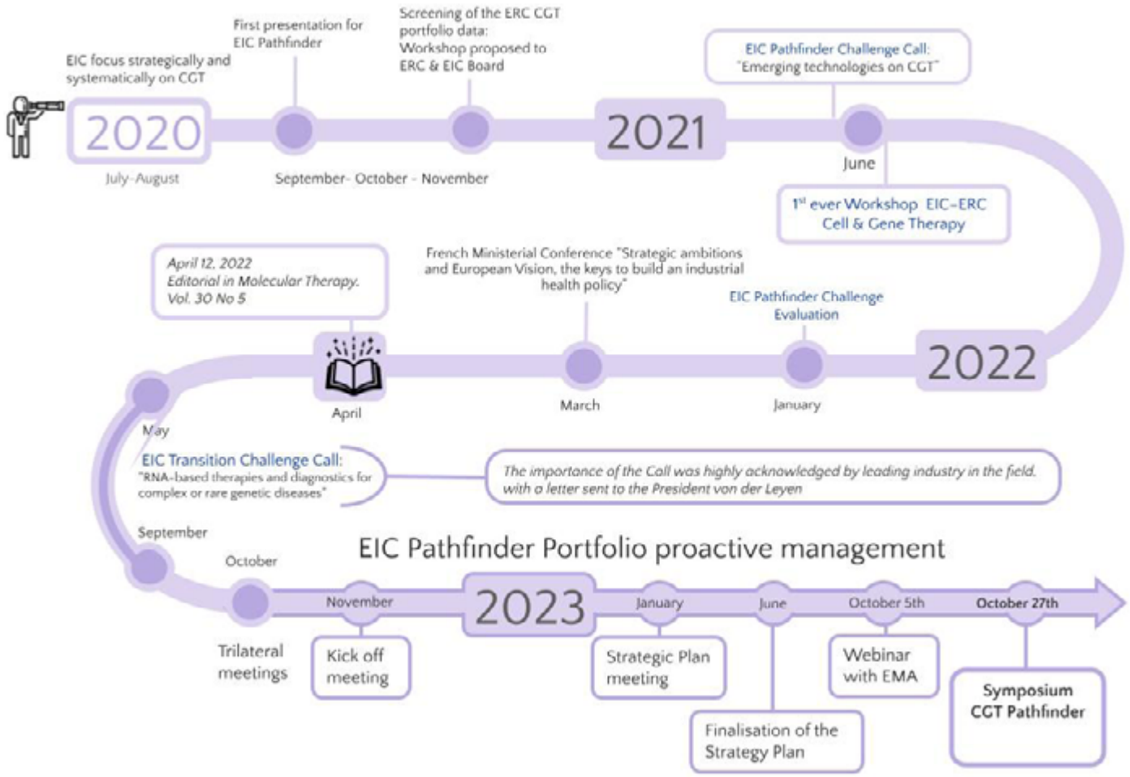
b. The 2021 CGT workshop, the first ever event jointly organised with ERC Cell and gene therapy: the first EIC-ERC workshop;

c. Pathfinder and Transition Challenge-based Calls in 2021 and 2022, respectively

d. An editorial article published in the high impact journal Molecular Therapy. All these actions are depicted in the schematic representation below

e. EIC CGT Symposium, in the context of the Annual Congress of the ESGCT which will take place on Oct 27, 2023

THE EIC CELL AND GENE THERAPY STORY



1.2
THE CREATION OF THE EIC PATHFINDER CGT PORTFOLIO

The Call HORIZON-EIC-2021-PATHFINDERCHALLENGES-01 Emerging Technologies in Cell & Gene Therapy (EIC Pathfinder challenge: Emerging technologies in cell and gene therapy (europa.eu) was published in the Work Programme 2021 and the accompanying Challenge Guide, targets the following areas:

I. Advancing cell therapy manufacturing and products to a clinical stage

II. Improving adoptive cell therapies (CAR-T, TCR, TIL)

III. Identifying next generation cell therapies for cancer

IV. Applying cell therapy to treat cancer patients in a personalised manner

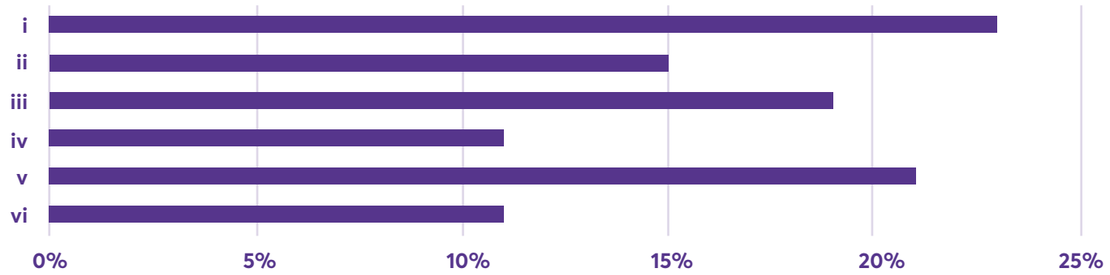
V. Improving the effectiveness and lowering the risks of gene delivery systems (vectors)

VI. Improving gene therapy manufacturing processes and production

The proposals received have covered all these areas (statistics based on the analysis performed by the Evaluation Committee/Panel members).

PROPOSALS RECEIVED PER TARGET AREA

(One proposal can tackle one or more areas)



Two stages of evaluation of the Call HORIZON-EIC-2021-PATHFINDERCHALLENGES-01 Emerging Technologies in Cell & Gene Therapy:

In the first stage, all applications were evaluated individually by external experts and scored against the evaluation criteria set out in the Work Program. All applications that pass the defined thresholds against the criteria were included in the second stage of the evaluation. At the second stage, all above threshold applications were considered collectively by an evaluation panel chaired by the Program Manager. For the creation of a coherent portfolio of projects to be funded, The EIC PM proposed to the evaluation panel members to follow the shared component logic/model which he detailed described to them. Detailed discussion among the Panel members followed and finally, the Panel endorsed the approach and the selection of proposals. According to this model, the evaluation panel applied the following portfolio considerations:

1. All proposals were mapped against well-defined categories/ building blocks;
2. The "Shared component" was identified based on one or more building blocks that are common to several proposals coming from different building blocks and
3. Starting from the most highly ranked proposal, a portfolio of proposals was selected based on maximising the shared component enabling the evaluation panel to group the projects and to identify a recognisable transversal pattern constituting the portfolio.

Soon after the selection of the nine CGT projects to establish the CGT portfolio and be funded under the EIC Pathfinder CGT Challenge, it became evident that two distinct sub portfolios instead of one portfolio, would better serve the purpose of proactive management following the logic of the "shared component". As a result, two subportfolios emerged:

CGT SUB PORTFOLIO 1:

is comprised of 6 projects. All projects in sub portfolio 1 share common aspects such as, scale up activities and cell therapy manufacturing platforms consistent with the area of "*Advancing cell therapy manufacturing and products to a clinical stage*". In addition, the vast majority of subportfolio 1 proposals (5*/6), addresses the area of "*Applying cell therapy to treat cancer patients in a personalized manner*".

PROJECT NUMBER	PROJECT ACRONYM	PROJECT TITLE
101071188	**AiPSC***	AI-powered platform for autologous iPSC manufacturing.
101071054	**SMARTER***	Smart manufacturing for autologous cell therapies enabled by innovative biomonitoring technologies and advanced process control.
101071140	**MUTAVAC***	Targeting cancer with mutanome based stem cell vaccine
101070740	**T-FITNESS***	Fine-Tuning T Cell Networks of Exhaustion by Synthetic Sensors
101070922	**PAT4CGT**	Automated online monitoring & control to improve processes and decision making in cell and gene therapy manufacturing.
101070950	**X-PAND***	Exploiting ex vivo expansion and deep multiomics profiling to bring novel, efficient and safer hematopoietic stem cell gene therapies to clinical application.

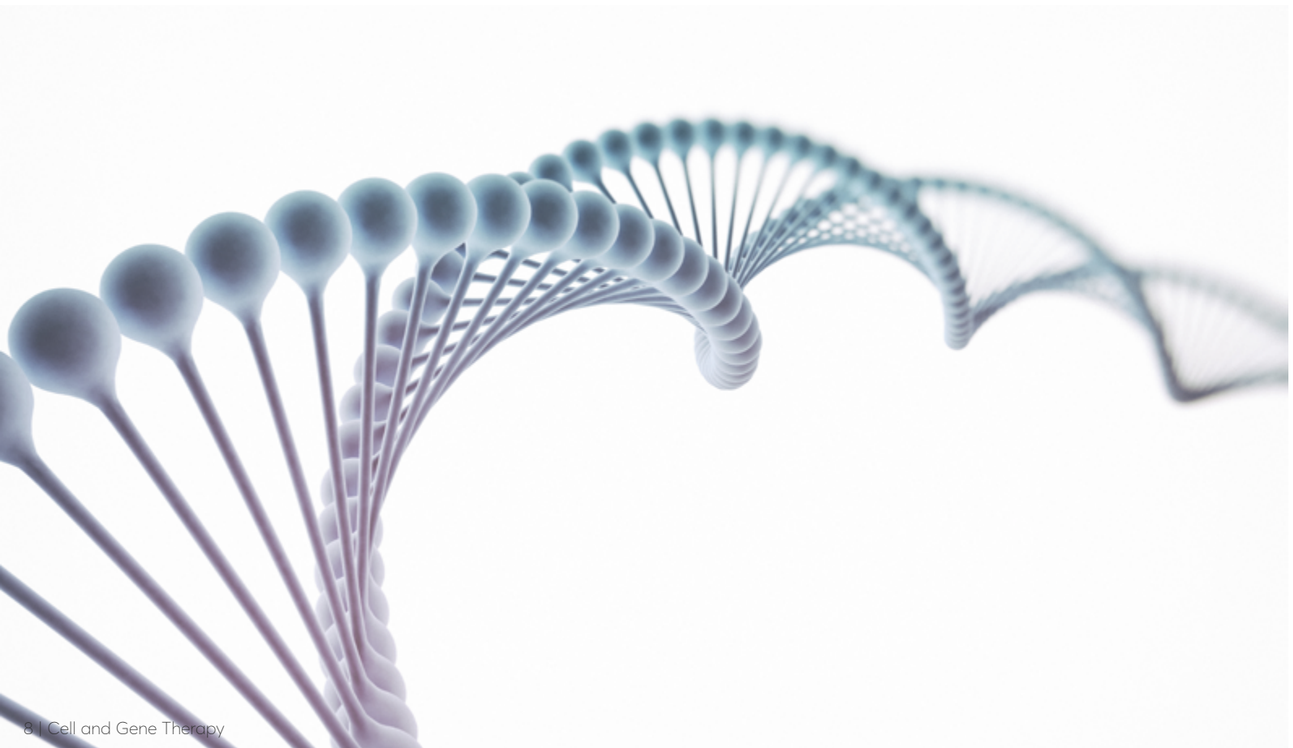
If the PM identifies project(s) funded under the EIC Pathfinder Open call with significant shared component with that of the CGT sub 1portfolio, he will ask that these projects will be moved in the subportfolio to benefit from the portfolio activities.

SUB PORTFOLIO 2

comprises 3 projects funded under the EIC Pathfinder CGT Challenge call. All projects in sub-portfolio 2 share common aspects such as gene delivery vectors (viral, non-viral, EVs) and "gene editing" consistent with the area: "Improving the effectiveness and lowering the risks of gene delivery systems (vectors)". In addition the majority of subportfolio 2 proposals (2*/3), addresses the area of "Improving gene therapy manufacturing processes and production".

PROJECT NUMBER	PROJECT ACRONYM	PROJECT TITLE
101070903	**EdiGenT**	New Prime Editing and non-viral delivery strategies for Gene Therapy.
101071041	**AAVolution***	Next-generation AAV vectors for liver-directed gene therapy
101071105	**NOVISTEM***	NOn-VIral gene modified STEM cell therapy.

If the PM identifies project(s) funded under the EIC Pathfinder Open call with significant shared component with that of the CGT sub portfolio 2, he will ask that these projects will be moved in the subportfolio to benefit from the portfolio activities.



1.3 TARGET AREAS OF ACTION AT PORTFOLIO LEVEL

1. Target Areas of action at Portfolio level

Three principal Target Areas of action are defined at portfolio level here and in the Strategic Plan: Technology, Regulatory and Exploitation.

Principal areas of Portfolio activities, as defined in the strategic plan, are:

2. Guiding principals behind portfolio activities

Portfolio vs project activities: Unlike individual project, portfolio activities are taking place between two individual or a subgroup of projects within the CGT portfolio and constitute a grant obligation. Main goals to be achieved with the portfolio activities include:

- Tackle challenges of common interest in any of the three areas shown above
- Leverage opportunities of common interest in any of the above three areas
- Amplify the impact of the portfolio and on the portfolio so that the impact of an activity is more than the sum of the activities performed independently by each individual project

1.4

GOVERNANCE OF THE EIC PATHFINDER CGT PORTFOLIO

The CGT portfolio is governed by the CGT steering committee and the PM. The CGT steering committee is formed by identified representatives of the CGT projects' (coordinators or deputies) who are responsible for the implementation of the portfolio activities. However, in certain cases, the PM may provide more than guidance, concrete ideas contributing to a more effective implementation of the portfolio activities. The CGT steering committee will be responsible to:

- Identify common needs and challenges among portfolio projects.
- Agree, on the concrete collaborations and accompanying activities identified and developed by individual portfolio projects, to be included in the strategic plan. This is done in cooperation with the PM.
- Ensure the proper implementation of the activities included in the strategic plan, always respecting individual portfolio project confidentiality issues).
- Ensure that the deliverables of the Portfolio work package are satisfactory and submitted to EIC in time.
- Report regularly to the PM and the assigned PO.

The steering committees of the two subportfolios will e-meet at least 4 times (preferably one on site and 3 online meetings) a year and, as needed putting the PM and the PO on copy. The PM and/or the PO may e-attend these meetings if their presence is asked by the coordinators or deemed necessary by the PM. The CGT steering committee composition is shown below. Each coordinator has identified a back-up to act on his/her behalf in the CGT Steering Committee.

CGT subportfolio 1 Steering Committee

Project Acronym	Coordinator	Contact persons for Portfolio activities and backups
AIPSC	Malin Stridh	Malin Stridh
SMARTER	Dorte Solle (named EU coordinator)	Vishal Sharma; Mariana Werner; Henrieta Fraser
MUTAVAC	Griscelli Frank	Annelise Bennaceur; Ali Turviv
T-FITNESS	Luca Gattinoni	Laura Maccari; Maka Malania
PAT4CGT	Anna Kamenskaya	Joanna Soares; Luc Henry
X-PAND	Luigi Naldini and Bernhard Gentner	Bernhard Gentner; Erika Zonari; Samuele Ferrari

CGT subportfolio 2 Steering Committee

Project Acronym	Coordinator	Contact persons for Portfolio activities and backups
EdiGenT	Filippo Del Bene	Claire Carapezzi
AAVolution	Alberto Auricchio	Valentina Bouché
NOVISTEM	Tom Taghon	Kevin Braeckmans

CGT EIC Pathfinder Portfolio Advisors

EIC PM invites senior experts with proven track record and vast experiences in the CGT field to advise/support portfolio project(s) respecting confidentiality issues from:

- **Academia/Research Institutions:** to provide scientific and technical expertise, to evaluate validity or viability of technology development strategies.
- **Biotech industry:** to provide guidance on commercialization and tech-to-market strategies and facilitate networking with key industrial players.
- **Regulatory Authorities:** to update on CGT regulatory framework.

2.
ROADMAP LEADING TO THE DEVELOPMENT OF THE CGT STRATEGIC PLAN

2.1

1ST STEP:

THE TRILATERAL MEETINGS

[SEPT – OCT 2022]

EIC PM proactively managed a series of interactions/meetings (steps) leading to the development of the CGT strategic plan with first step the trilateral meeting. Online trilateral meetings took place between the assigned PO, the EIC PM and each portfolio project/member to: a) introduce each individual project's specific aims by the respective project coordinator and get a deeper knowledge of the project, b) present the concept of portfolio activities by the PM and PO and c) collect initial ideas for portfolio activities without influence from the other projects.

Each portfolio member selected two consortium members, one usually being the project Coordinator, to represent the entire consortium of the project in the kick-off meeting.

CGT PORTFOLIO PROACTIVE MANAGEMENT PERIOD

2.2

2ND STEP:
THE KICK-OFF MEETING

[26-27 OCT 2022]

The first "get to know" kick off meeting with representatives from all portfolio projects took place online on the 26-27 October 2022 for each subportfolio respectively. Each coordinator described their project in more details including major challenges they are expected to face at various stages of development. Also, they presented their initial ideas for potential collaboration with another portfolio project. At that time the portfolio projects did not know each other.

2.3

3RD STEP:
BRAINSTORMING WITHIN PROJECT CONSORTIA EXPLORING POTENTIAL COLLABORATIONS

[NOV-DEC 202]

First brainstorming between individual members of each consortium via online meetings to explore, discuss and agree on specific collaborations, leveraging all the expertise and experience in the consortium and feeding ideas to the coordinator along the entire process.

2.4

4TH STEP:
INTERACTIONS WITHIN SUBPORTFOLIO TOWARDS THE 1ST DRAFT OF THE STRATEGIC PLAN

[10-11 JAN 2023]

The third portfolio meeting with representatives from all members was held in Brussels (first onsite meeting) with aim to advance the work on the draft strategic plan (10th,11th January 2023, see Agenda in Annex 4.3, 4.4 for sub1 & sub2 respectively).

The EIC PM presented the scope of the portfolio, and the assigned PO moderated the discussion between the portfolio members per proposed specific collaboration. It should be noted, that the EIC PM invited two external senior experts for this meeting (one world-class scientist/physician with vast proven experiences in CGT (preclinical and clinical level) and a second expert, who is heading a biomanufacturing site/institute given that the focus of sub-portfolio 1 is precisely on cell therapy manufacturing) to be considered as potential source of support/Advisors to the portfolio members and the EIC-PM. The experts presented their proven experiences online and in one case, the expert asked questions posed by individual portfolio members.

The inter-portfolio member connections established during the subportfolio meetings led to inter-project collaborations at the technology level to be implemented at portfolio level (see 3.1.1 and 3.1.2 and Strategic Plan), and which were not included in the original proposals of the individual EIC-funded projects. The collaborations were identified in a stimulating and inspiring discussion covering the topics of the projects, also encouraged by the expert interventions.

2.5

5TH STEP:
PORTFOLIO PROJECTS TO ADVANCE AND CO-DRAFT THE PRE-AGREED COLLABORATIONS

[FEB-JUN 2023]

Online meetings between portfolio projects followed the onsite meeting, to advance the pre-agreed specific collaborations. Once agreement was achieved, the two parties (portfolio members/ projects) involved each time, co-drafted their proposed collaborative activity using Teams editing platform and collectively produced the first draft of the strategic plan.

2.6

6TH STEP:
PM+PO+PORTFOLIO STEERING COMMITTEE TO ADVANCE FURTHER THE STRATEGIC PLAN

[JUN 2023]

Early in June 2023, the EIC PM and PO critically discussed and edited on Teams all proposed collaborations in the draft strategic plan, one by one. Soon after, they called for a portfolio Steering Committee meeting (the fourth one with the entire portfolio) which took place online on June 7, with aim to discuss and advance further the portfolio activities towards finalisation of the strategic plan.

2.7

7TH STEP: THE FINALISATION OF THE SUBPORTFOLIO STRATEGIC PLANS

[SEE ANNEX 4.1, 4.2]

The work to finalize the CGT strategic plan was the outcome of a dynamic and demanding interaction between the CGT portfolio project representatives, the PO and the EIC PM and required several iterations also between the PM and the PO, to refine the objective of the collaborations and activities, edit text and finalise it (June-Sep, 2023). More specifically, each strategic plan is composed of:

a. The objectives of agreed collaborations (linked to unmet needs, challenges, or opportunities) in the target area of the CGT portfolio

b. The CGT portfolio activities to reach the set objectives and

c. he governance to guide the smooth implementation of the portfolio activities.

MAIN COMPONENTS OF THE STRATEGIC PLAN

- The objectives of agreed collaborations (linked to unmet needs, challenges, or opportunities) in the target area of the CGT portfolio
- The CGT portfolio activities to reach the set objectives
- The governance to guide the smooth implementation of the portfolio activities

The strategic plan is a dynamic document in the sense it can be updated. Request for an update can come either from the PM or the steering committee of the CGT sub-portfolio (see governance). The strategic plan can be updated biannually, as needed through provision of reports.

3. TARGET AREAS OF PORTFOLIO ACTIVITIES

The strategic plan describes the specific collaborations agreed between two or more portfolio projects each time. Within each collaboration, the plan describes the objective, the activities that need to be carried out to meet the objective, an estimate of time and resources/budget required for that.

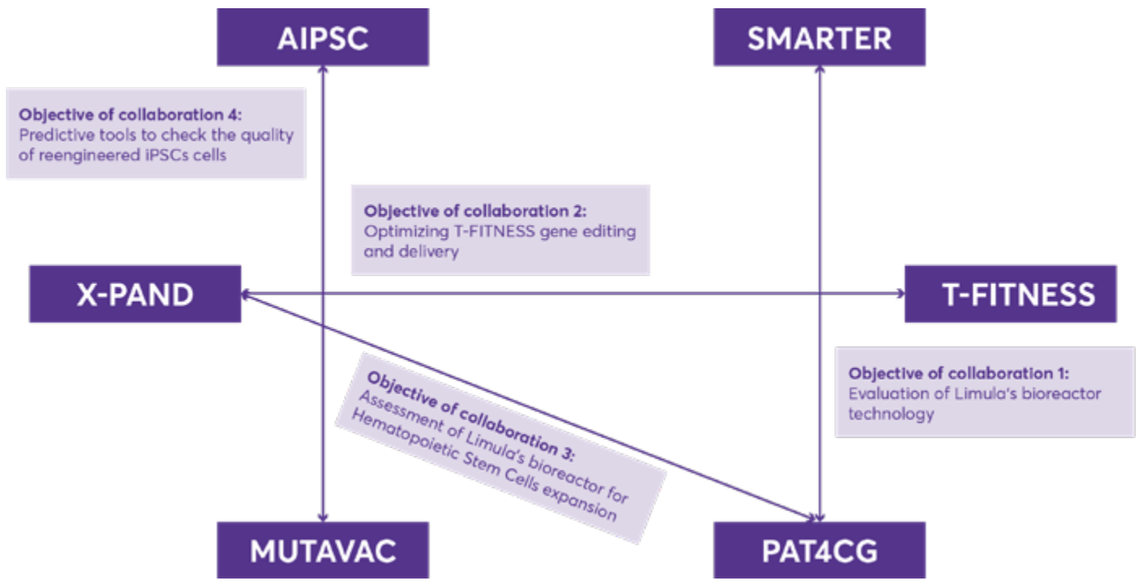
3.1

PORTFOLIO ACTIVITIES AT TECHNOLOGY LEVEL

3.1.1

PROJECT INTERACTIONS IN SUBPORTFOLIO1 TO IMPLEMENT THE ACTIVITIES AT TECHNOLOGY LEVEL.

PROJECT INTERACTIONS IN SUBPORTFOLIO1 TO IMPLEMENT THE ACTIVITIES. OBJECTIVES OF COLLABORATION



3.1.2

DESCRIPTION OF THE SUBPORTFOLIO 1 COLLABORATIONS AND ACTIVITIES

1. SMARTER and PAT4CGT

Objective of collaboration 1: Evaluation of Limula's bioreactor technology to produce Achilles' cell therapy (TILs), which could be applicable to other autologous cell therapies.

Portfolio Activities

- Limula will build a bioreactor prototype adapted to Achilles use case, including automation systems and single-use plastic kits. This system will then be transferred to Achilles to enable process development runs with healthy donor T cells, followed by test runs with the TIL-like model cells.
- **Estimated Costs:** 75K€ (25K€ from PAT4CGT & 50 K€ from SMARTER)
- **Estimated Time:** 6M (Starting in Q4)

2. T-FITNESS and XPAND

Objective of collaboration 2: Optimize T-FITNESS gene editing in T cells and hematopoietic cells

Portfolio Activities

- T-FITNESS will evaluate a lipid nanoparticle platform (LNP) for delivery of CRISPR/Cas9 components developed by XPAND to reduce electroporation-induced cytotoxicities in T cells.
- X-PAND will evaluate off-target genome editing toxicities in small numbers of HSCs using the next-generation DISCOVER-seq platform developed by T-FITNESS
- Both projects will screen diverse small molecules (e.g., CDC7 and P53 inhibitors) to enhance genome editing efficiency.
- T-FITNESS and X-PAND will share protocols and assist each other with troubleshooting. T-FITNESS will run X-PAND samples on the next-generation DISCOVER-seq platform.
- **Estimated Costs:** Estimated Costs: 50K for consumables, sequencing, labor, and dissemination activities (T-FITNESS); 25K for research activities (X-PAND).
- **Estimated Time: Task 1:** Q1 2024-Q2 2025; Task2: Q1 2025-Q4 2025; Task3: Q1 2024-Q2 2025

3. PAT4CGT and X-PAND

Objective of collaboration 3: Assess the application of Limula's novel bioreactor technology with in-situ centrifugation capabilities for washing, concentrating, and expanding cell cultures across scales to the upstream processing (concerning the starting material) and downstream processing (concerning cell expansion in the bioreactor) of HSC-based gene therapies developed by San

Raffaele Telethon Institute for Gene Therapy (Ospedale San Raffaele).

Portfolio Activities

- Upstream process evaluation (before genetic engineering): Frozen bulk apheresis samples will be procured. The frozen apheresis product will be thawed, washed and concentrated with the Limula device. Successively, CD34+ cells will be enriched by CliniMACS and CD34+ cell recovery/ purity will be assessed and compared to aphereses processed with a manual washing procedure.
- Downstream bioreactor process evaluation (after genetic engineering): Bulk CD34+ cells, engineered by lentiviral vectors or gene-edited, will be expanded in the Limula bioreactor. Cell yield, numbers and viability, as well as immunophenotype will be assessed and compared to a manual expansion process.
- **Estimated Costs:** 60K€ (Personel & Material)
- **Estimated Time:** 3M (Starting in Q1/2024)

4. AiPSC and MUTAVAC

Objective of collaboration 4: Correlate the morphological pattern of iPSC clones with their genomic instability profile potentially leading to the development of tool(s) for checking the quality of of engineered iPSC cells.

Portfolio Activities

- AiPSC will perform morphological analysis with their AI-pipeline.
- MUTAVAC will evaluate the genome integrity of DNA samples obtained from several iPSC clones provided by AiPSC. The study will allow to detect hot spot chromosomal aberrations and mutations with digital precision according to international standards and guidelines.
- AiPSC and MUTAVAC will combine their data which were originally produced in blinded fashion, to establish associations or correlations between morphology and genomic profile of iPSC clones.
- If successful, this portfolio activity will serve as a proof of concept for testing genomic instability based on morphological features through AI. Once proven feasible this capability could be applied to AiPSC and MUTAVAC projects in a more general manner which could be the bases for seeking additional funding for this purpose.
- **Estimated Costs:** 100K€ (MUTAVAC 50K€ consumables, sequencing, technician & AiPSC 50K€ cell culture consumables, stem cell technician)
- **Estimated Time:** (Q4 2024- Q2 2025)

5. Transfer of Open Call projects to Challenge-based CGT portfolio.

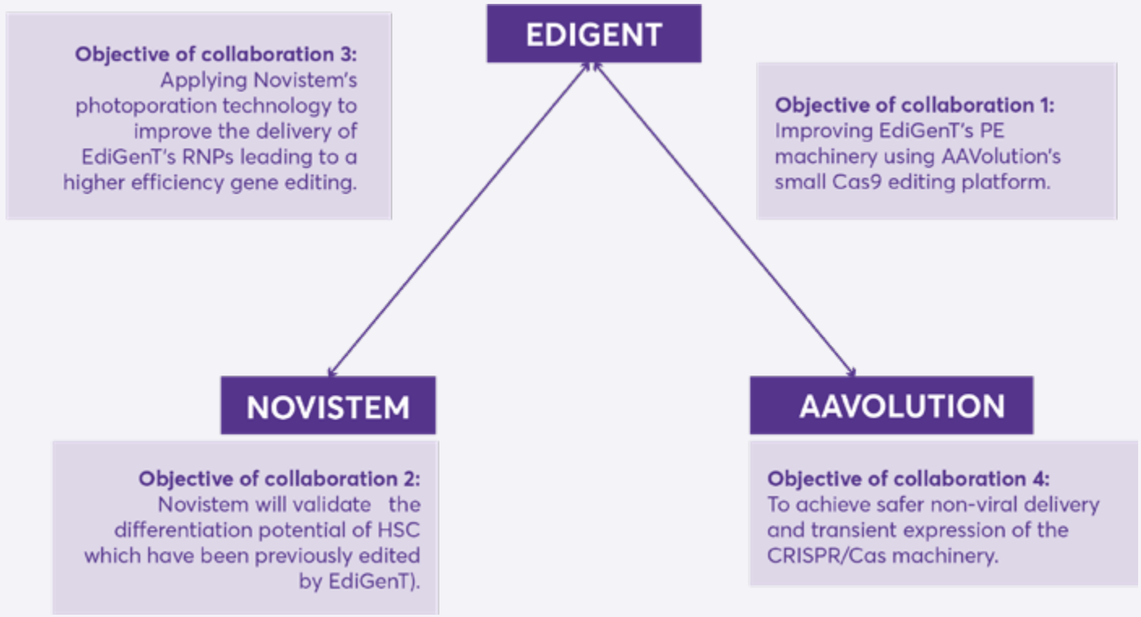
Objective: Identify projects funded under the EIC Pathfinder Open call with adequate amount of S&T shared with the Challenge-based CGT portfolio (shared component), for the purpose to interact with the CGT portfolio projects. This action is initiated by the PM.

Portfolio Activities

- Presentation of the projects deriving from the open call to the existing portfolio members, followed by discussion.
- Meetings between individual projects from the open and challenge-based calls, to identify points of common interest potentially leading to establish portfolio activities.
- Update the strategy plan with all the newly defined concrete collaborations between open and challenge-based projects.

3.1.3

PROJECT INTERACTIONS IN SUBPORTFOLIO2 TO IMPLEMENT THE ACTIVITIES AT TECHNOLOGY LEVEL



3.1.4

DESCRIPTION OF THE SUBPORTFOLIO 2 COLLABORATIONS AND ACTIVITIES.

1. EdiGenT and AAVolution

Objective of collaboration 1: Improving EdiGenT's PE machinery using AAVolution's small Cas9 editing platform

Portfolio Activities

- EdiGenT will use AAVolutions small Cas9 platform expected to be more efficient than that previously used (currently under development in Cereseto's lab).
- Estimated Costs: 20K€
- Estimated Time: 24M

2. NOVISTEM and EdiGenT

Objective of collaboration 2: Novistem will validate the differentiation potential of HSC which have been previously edited by EdiGenT).

Portfolio Activities

- Novistem will test the HSCs cells for their potential to differentiate, after they will have been edited by EdiGenT.
- Estimated Costs: 30K€
- Estimated Time: 24M

3. EdiGenT and NOVISTEM

Objective of collaboration 3: Applying Novistem's photoporation technology to improve the delivery of EdiGenT's RNPs leading to a higher efficiency gene editing.

Portfolio Activities

- NOVISTEM's photoporation technology will be used to deliver RNP nanoparticles to sickle-cell patient-derived HSCs and iPSCs, as well as, SCID-derived iPSCs.
- Estimated Costs: 30K€
- Estimated Time: 18M

4. EdiGenT and AAVolution

Objective of collaboration 4: To achieve safer non-viral delivery and transient expression of the CRISPR/Cas machinery.

Portfolio Activities

- Analysis of the pre-existing immune response to RNP nanoparticles in mice
- Estimated Costs: 50K€
- Estimated Time: 24M

5. Transfer of Open Call projects to Challenge-based CGT portfolio

Objective: Identify projects funded under the EIC Pathfinder Open call with adequate amount of S&T shared with the Challenge-based CGT portfolio projects (shared component logic), which would allow for the participation of the former in the portfolio activities of the latter.

Portfolio Activities

- Presentation of the projects deriving from the open call to the existing portfolio projects, followed by discussion. This action is initiated by the PM
- Meetings between individual projects from the open call with that from the CGT portfolio, to explore points of common interest potentially leading to establish collaborations and portfolio activities.
- Update the strategy plan with all the newly defined collaborations between open and challenge-based projects.

3.2

PORTFOLIO ACTIVITIES AT REGULATORY LEVEL

OBJECTIVE :

Familiarizing portfolio projects with CGT regulatory framework and practises

Portfolio Activities

- Each portfolio project to inform the EIC PM and PO of challenges/bottlenecks in their S/T work that are worth communicating to the EMA, for potential future regulatory action.
- EIC PM and PO have invited regulatory experts from EMA specializing in the CGT area, who will present the current CGT regulatory framework to the portfolio projects on Oct 5, 2023, and by doing so will initiate a dialog at portfolio level with the CGT projects. More specifically, the dialog with EMA is aimed at contributing new knowledge pertinent to regulate in cell therapy manufacturing (subportfolio 1) and gene delivery systems (subportfolio 2).

3.3

PORTFOLIO ACTIVITIES AT EXPLOITATION LEVEL

OBJECTIVE 1:

Increase individual portfolio member's knowledge on the CGT global market.

Portfolio Activities

- Portfolio members to exchange results of their individual market research analysis to identify key players of common interest with which, partnership(s) can be explored at portfolio level.
- PM and PO to catalyse the participation of portfolio projects in different EIC Business Acceleration Services (BAS), especially those providing Tech to market knowledge throughout the duration of the EIC funding.

- A webinar with EPO experts aimed to enhance the knowledge and skills of portfolio projects to build a robust IP strategy, is foreseen for later this year.
- PM and PO to share with portfolio projects CGT-related market reports for the benefit of the portfolio projects

OBJECTIVE 2:

Offer the possibility to portfolio projects to engage with investors at portfolio level.

Portfolio Activities

PM and PO to catalyse targeted meetings between various sort of investors (Investment companies, VCs, Business Angels) and CGT projects at portfolio level

OBJECTIVE 3:

Increase the exposure of the CGT portfolio to the industry and academia.

Portfolio Activities

- EIC CGT Symposium: The EIC CGT Symposium to be held in Brussels on Oct 27, 2023, in the context of the Annual Congress of the 30th European Society of Gene and Cell Therapy, is a milestone event for the EIC CGT portfolio. EIC PM conceived and brought forward this Symposium, to demonstrate the importance and effectiveness of the EIC Pathfinder portfolio as a vehicle to contribute achieving critical mass in the CGT area, potentially leading to European strategic autonomy in the same field in the medium run
- PM and PO to organize meetings at portfolio level with relevant industry people of potential interest to the entire portfolio and/or individual project level. In this context, EIC PM invited the company Eurekare, which presented their new cell therapy manufacturing initiative and related services. Meetings with other relevant companies will follow, for the same purpose.
- PM and PO to introduce Pathfinder portfolio projects to EIC non-Pathfinder CGT companies, in order to explore potential collaboration/partnerships on a mutual benefit basis.

3.4

ASSESSMENT OF PORTFOLIO ACTIVITIES WORK-IN-PROGRESS

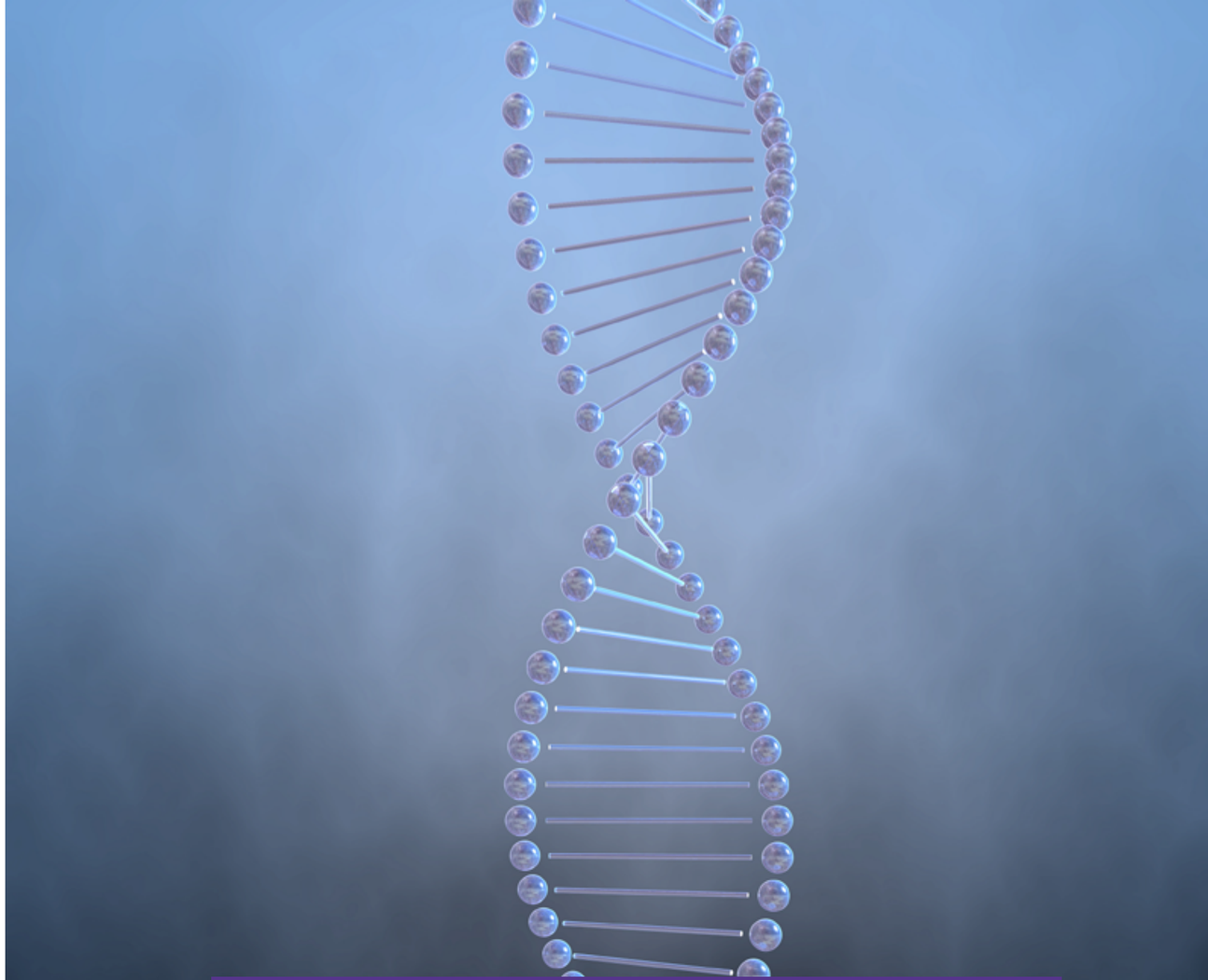
Assessment of portfolio activities work-in-progress will take place during the scheduled regular individual or combined project Reviews, after the end of the corresponding reporting periods as shown below:

SUB PORTFOLIO 1:

PROJECT ACRONYM	REPORTING PERIODS
AiPSC	**12M, 18M, 18M**
SMARTER	**12M, 24M**
MUTAVAC	**12M, 12M**
T-FITNESS	**12M, 18M, 18M**
PAT4CGT	**12M, 24M**
X-PAND	**12M, 18M, 18M**

SUB PORTFOLIO 2:

PROJECT ACRONYM	REPORTING PERIODS
EdiGenT	**12M, 16M, 16M, 16M**
AAVolution	**12M, 18M, 18M**
NOVISTEM	**12M, 18M, 18M**



4. ANNEXES

4.1

Strategic Plan subportfolio 1: Advancing cell therapy manufacturing and products to a clinical stage

Contents

4.2

Strategic Plan subportfolio 2: Improving effectiveness and lowering the risks of gene delivery systems

Contents

4.3

Agenda of onsite sub-portfolio 1 Meeting Jan 10, 2023

European Innovation Council

EIC Pathfinder Challenge: Emerging Technologies in Cell & Gene Therapy

Subportfolio 1: ***Advancing cell therapy manufacturing and products to a clinical stage***

10 January 2023

09:00 **Arrival**

09:30 Welcome and **Introductory remarks by I. Arzimanoglou** (Programme Manager for Health and Biotechnology)

09:50 **Foreword 1 on-line by Mark Ferguson** (Chair, EIC Board) (TBC).

09:55 **Foreword 2 by Timo Hallantie** (Head of the Unit Pathfinder)

10:00 **Roundtable introduction of all projects (2'-3' min each)**

10:15 **Presentation of the sub-portfolio individual projects** on specific strategic collaborations between members leading to portfolio activities.

- **AiPSC (#101071188)**
- **SMARTER (#101071054)**
- **MUTAVAC (#101071140)**

11:00 **coffee break**

11:15 **Presentation by the rest of individual projects of the same sub-portfolio**

- **T-FITNESS (#101070740)**
- **PAT4CGT (#101070922)**
- **X-PAND (#101070950)**

12:00 **Q&A session & Discussion (moderated by I Amarantos)**

12:30 ***lunch break***

13:45 **Recorded Presentation by Darrin Morrissey**; NIBRT Chief Executive Officer (National Institute for Bioprocessing Research and Training, Ireland)

14:15 **Feedback from portfolio members on the relevance of the expert to the portfolio activities and overall potential.**

14:30 *Coffee break*

14:45 **"Strategic Intelligence at EIC: The case of CGT" by Keith Sequeira** (Head of Unit, EIC Board, Strategy and Feedback to Policy).

15:00 **On-line Presentation by Prof Hans-Peter Kiem**; MD, PhD (Stephanus Family Endowed Chair for Cell and Gene Therapy; Deputy Director, Translational Science and Therapeutics Division; Director, Stem Cell and Gene Therapy Program; Professor, Fred Hutchinson Cancer Center (FHCC); Professor of Medicine and Pathology, University of Washington (UW); Associate Head, Heme Malignancy Program, Cancer Consortium (UW/FHCC); Chief, Gene Therapy and Regenerative Medicine Division, WaNPRC, UW; President, American Society of Gene and Cell Therapy)

15:45 **Q&A from projects** to **Prof Hans-Peter Kiem**

16:00 **Feedback from portfolio members on the relevance of the expert to the portfolio strategic actions and overall potential**

16:15 **Portfolio members to elaborate further on the proposed collaboration schemes (from the morning sessions). Coordinated by Ioannis Amarantos and Daniel Martinez Linares.**

16:45 **Next steps leading to finalisation of the portfolio strategy plan by Iordanis Arzimanoglou**

17:15 **end**

4.4

Agenda of onsite sub-portfolio 2 Meeting Jan 11, 2023

European Innovation Council

EIC Pathfinder Challenge: Emerging Technologies in Cell & Gene Therapy

Subportfolio 2: Improving the effectiveness and lowering the risks of gene delivery systems (vectors)

11 January 2023

09:00 **Arrival**

09:30 Welcome and **Introductory remarks by I. Arzimanoglou** (Programme Manager for Health and Biotechnology)

09:45 **Foreword 1 on-line by Mark Ferguson** (Chair, EIC Board) (TBC).

09:50 **Foreword 1: Anne-Marie Sassen** (Head of Programme Manager's Office)

09:55 **Foreword 2: Timo Hallantie** (Head of Unit Pathfinder)

10:00 **Roundtable introduction of all projects (5' min each)**

10:15 **Presentation of the sub-portfolio individual projects** on specific strategic collaborations between members leading to portfolio activities.

- **EdiGenT (#101070903)**
- **AAVolution (#101071041)**
- **NOVISTEM (#101071105)**

11:00 **coffee break**

11:15 **Q&A session & Discussion (moderated by I Amarantos)**

11:30 **Recorded Presentation by Darrin Morrissey**; NIBRT Chief Executive Officer (National Institute for Bioprocessing Research and Training, Ireland).

12:00 **Feedback from portfolio members on the relevance of the expert to the portfolio activities and overall potential.**

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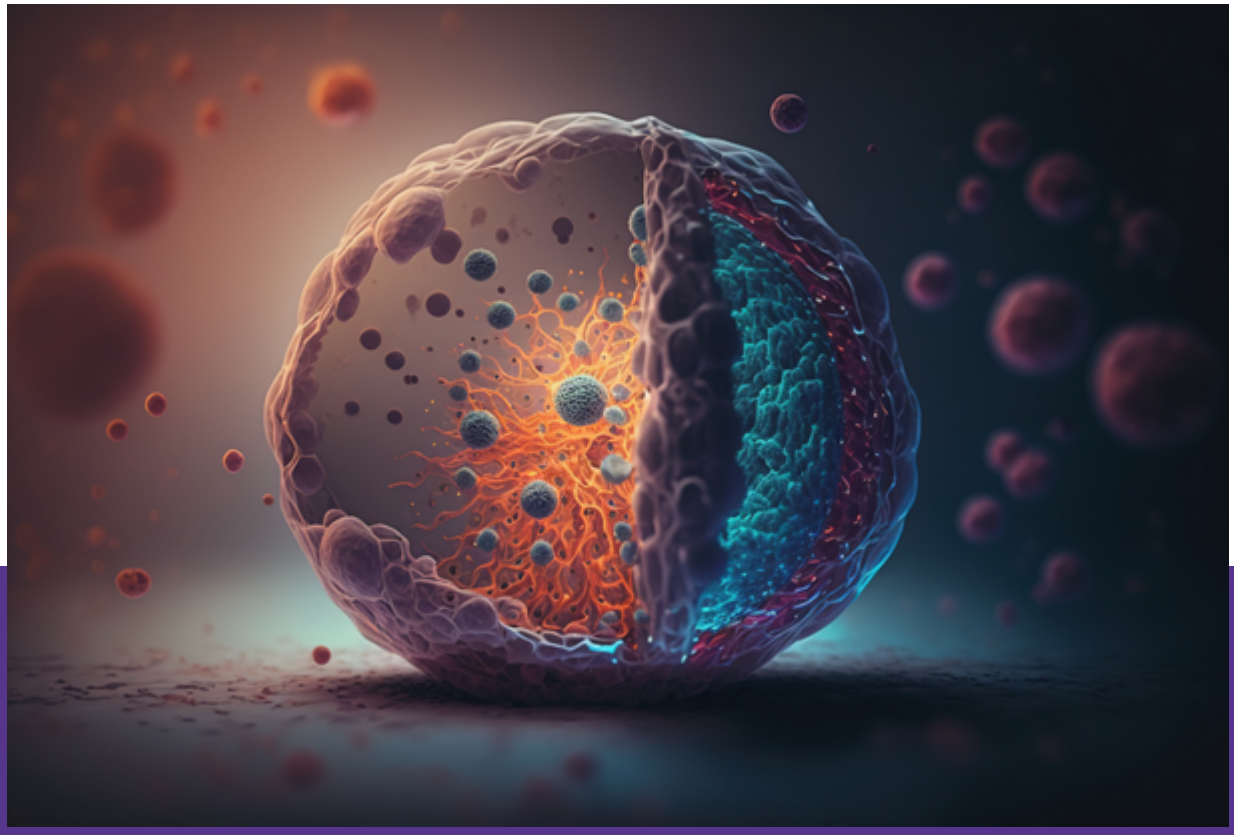
16:00 **end**



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