Backing visionary entrepreneurs

EIC Pathfinder and EIC Health portfolio

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Contents

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- EIC part of EISMEA
- EIC Pathfinder
- EIC Pathfinder 2021Challenge Calls in Health
- EIC Transition
- EIC Transition 2021 Challenge Call
- EIC Program Managers: Key roles and how they select thematic areas and topics
- Why Cell and Gene therapy (CGT) was selected for 2021 EIC Path Health Challenge
- EIC's response to the current global challenges faced by the CGT community: EIC Pathfinder Challenge call + EIC-ERC Workshop
- Selected examples of Pathfinder projects from the EIC Health and Biotech portfolio

EIC part of EISMEA

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EISMEA: The European Innovation Council and SME Executive Agency

EIC Board & President appointed by the EC

- -Pilot Advisory Board term just ended (May 2021)
- -Open call expired and selection for new Board Members is ongoing.
- -Open call for full time President is launched

EIC, Europe's most effective catalyst of breakthrough science into disruptive innovation

European Innovation Council

€10 bn programme to identify, develop and scale up breakthrough technologies and disruptive innovations in Europe

Unique in the world to combine research on emerging technologies with Accelerator for startups, SMEs and scaleups

EIC Fund largest VC deep-tech investor in Europe (over €3 bn)

Portfolio approach, Challenge Calls, Program Managers

EIC Pathfinder

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For advanced research to underpin breakthrough / game-changing technologies

Mainly open ("bottom up"), but also Pathfinder challenges (for emerging health, energy and digital technologies)	Mainly collaborative (3 or more partners)	Grants up to €3/4 Mt €3/4 million	Management of portfolios of projects by Programme Managers

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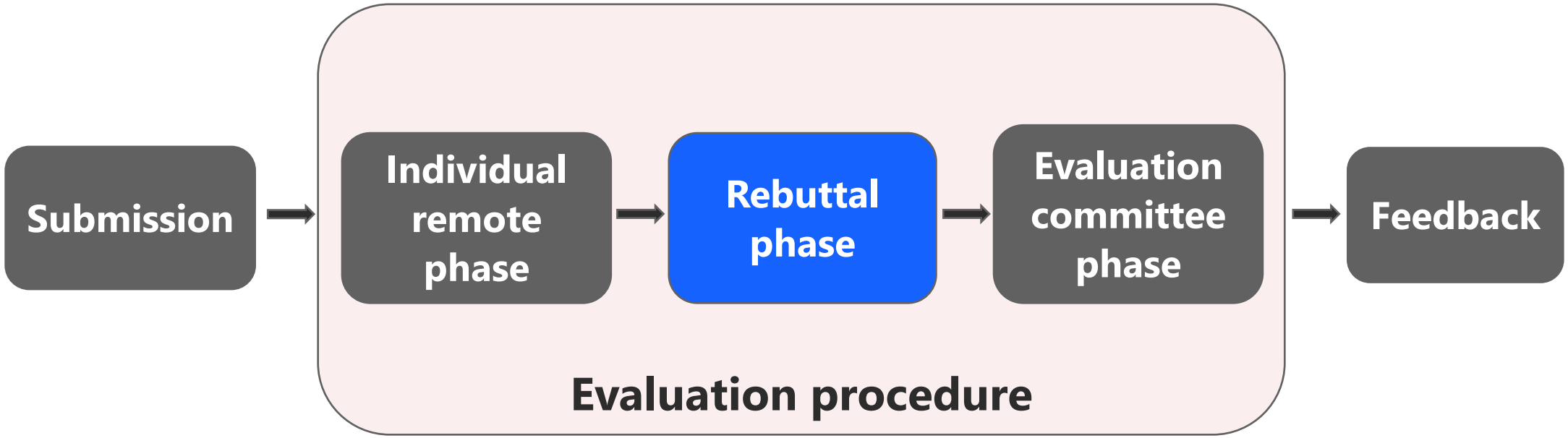
Pathfinder Open proposals evaluation scheme

Applicant has the right to address evaluators' comments



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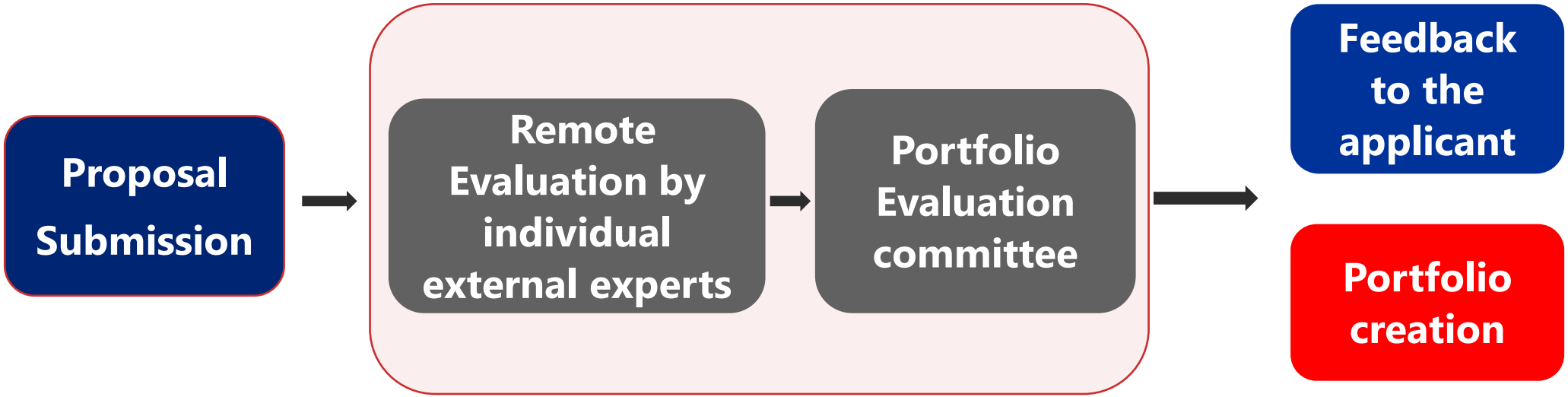
Pathfinder Challenge proposals evaluation scheme leading to the creation of portfolios



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EIC **Pathfinder** Challenge Call 2021 in Health & Biotechnology

EMERGING TECHNOLOGIES IN CELL AND GENE THERAPY

(Programme Manager for Health & Biotechnology: Iordanis Arzimanoglou)

EIC **Pathfinder** Challenge Call 2021 in Medical Devices

TOOLS TO MEASURE AND STIMULATE ACTIVITY IN BRAIN TISSUE

(Programme Manager for Medical Devices and Technologies; Enric Claverol)

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EIC **Pathfinder** Challenge Call 2021 in Living Materials

ENGINEERED LIVING MATERIALS

(Acting Programme Manager: Barbara Gerratana)

Integration of expertise in synthetic biology/morphogenesis, materials engineering, control engineering, artificial intelligence,

Additional opportunities for Pathfinder projects

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Projects or their beneficiaries funded through EIC Pathfinder are eligible

to receive additional **Ad hoc grants** (up to 3 per project or more if duly justified) with fixed amounts of up to €50,000 :

a) for complementary activities to explore potential pathways to commercialization

b) for portfolio activities.

to submit a proposal to the **EIC Transition** for transforming their research results into innovation opportunities;

to submit an **EIC Accelerator** proposal via the **Fast Track** scheme;

to receive free access to a wide range of **Business Acceleration Services**

EIC Transition

European Innovation Council

For transforming research results into innovation opportunities

New funding scheme to bridge gap between research phase (proof of concept) and innovation application	Mainly open ("bottom up"), but also Transition challenges (for medtech, energy storage)	Single applicants or small collaborations (max. 5 partners)	Grants up to 2.5M	In first phase, only for follow up to results from EIC Pathfinder and ERC PoC

EIC Transition

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Eligibility time wise :

- Pathfinder projects and ERC Proof of Concept. Start date of the grant is more than 12 months before the date of the Transition call deadline and end date of the grant for the eligible project is less than 24 months from the date of the Transition call deadline

IP status:

- You do not have to be the owner of the IP or one of the original beneficiaries, but have the right to use the IP or know-how generated in the initial project.

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EIC **Transition** Challenge Call 2021 in Health & Biotechnology

none

EIC **Transition** Challenge Call 2021 in Medical Devices

MEDICAL DEVICES: From Lab to patient

(Programme Manager Enric Claverol)

EIC Programme Managers (EIC PMs): three key roles

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- Oversees a thematic sector in a cross-cutting manner EIC (Pathfinder → Transition → Accelerator).
- **Identifies S/T critical thematic areas, pre-select topics within these areas and validates the topics for the purpose of developing Challenge Calls**
- Manages sector-specific portfolios (a set of projects with shared perspectives) created on the basis of the outcome of Challenge Calls

EIC Programme Managers: How they select areas

Guiding principles

- Strategic areas selected for Challenges must provide sufficient evidence of their disruptive and innovation potential
- Selection should not be overly influenced by the existing areas bearing most of the projects in the EIC portfolio, in order to ensure that EIC remains open and closely follows the constantly evolving trends
- There has to be even limited recognizable innovative industry activity in the selected area, so we do not start entirely for scratch
- Areas of intense focus attracting ample funding (COVID, epidemics monitoring), operated by other EC DGs or Agencies, should be avoided for obvious reasons

EIC Program Managers: How they pre-select topics

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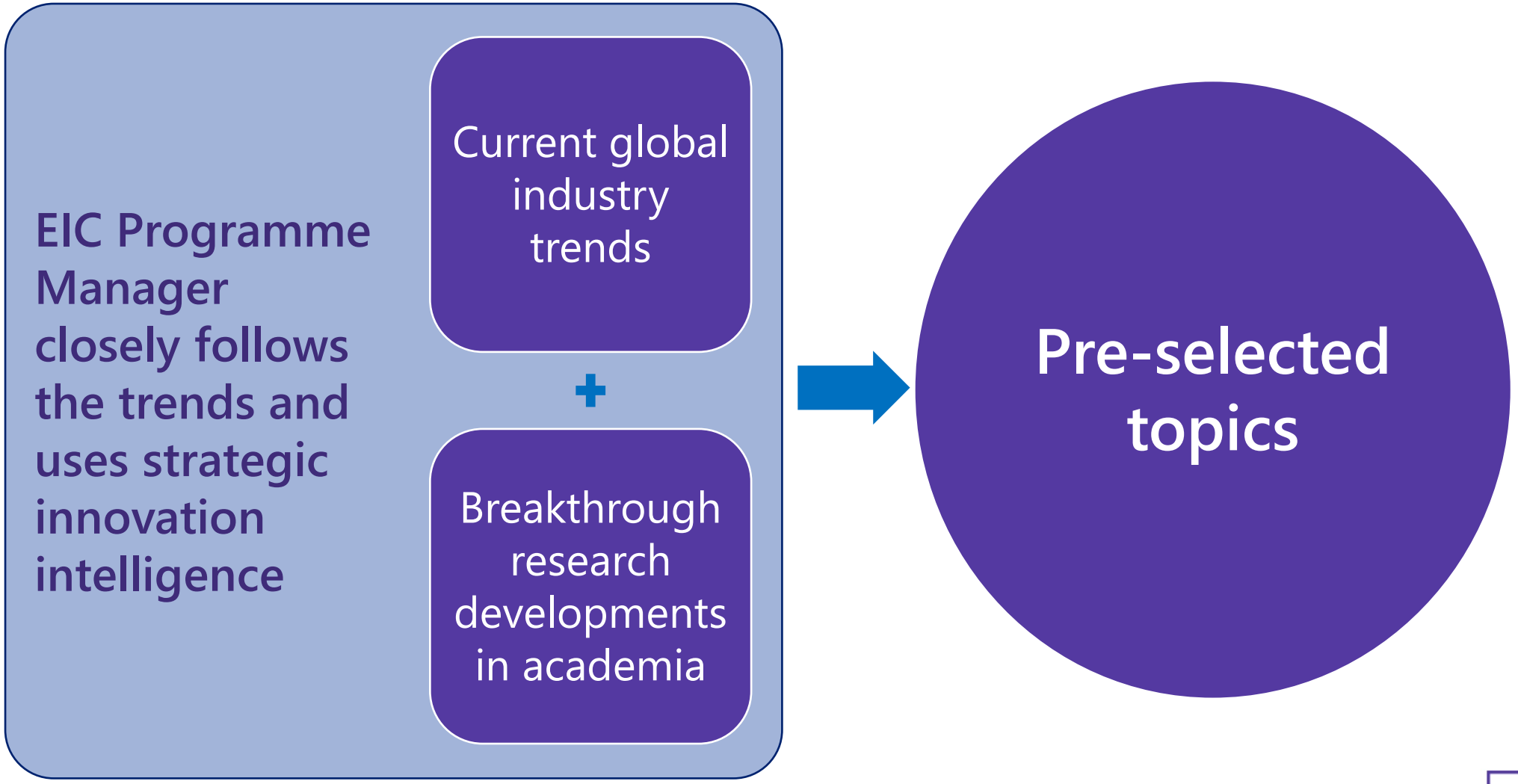
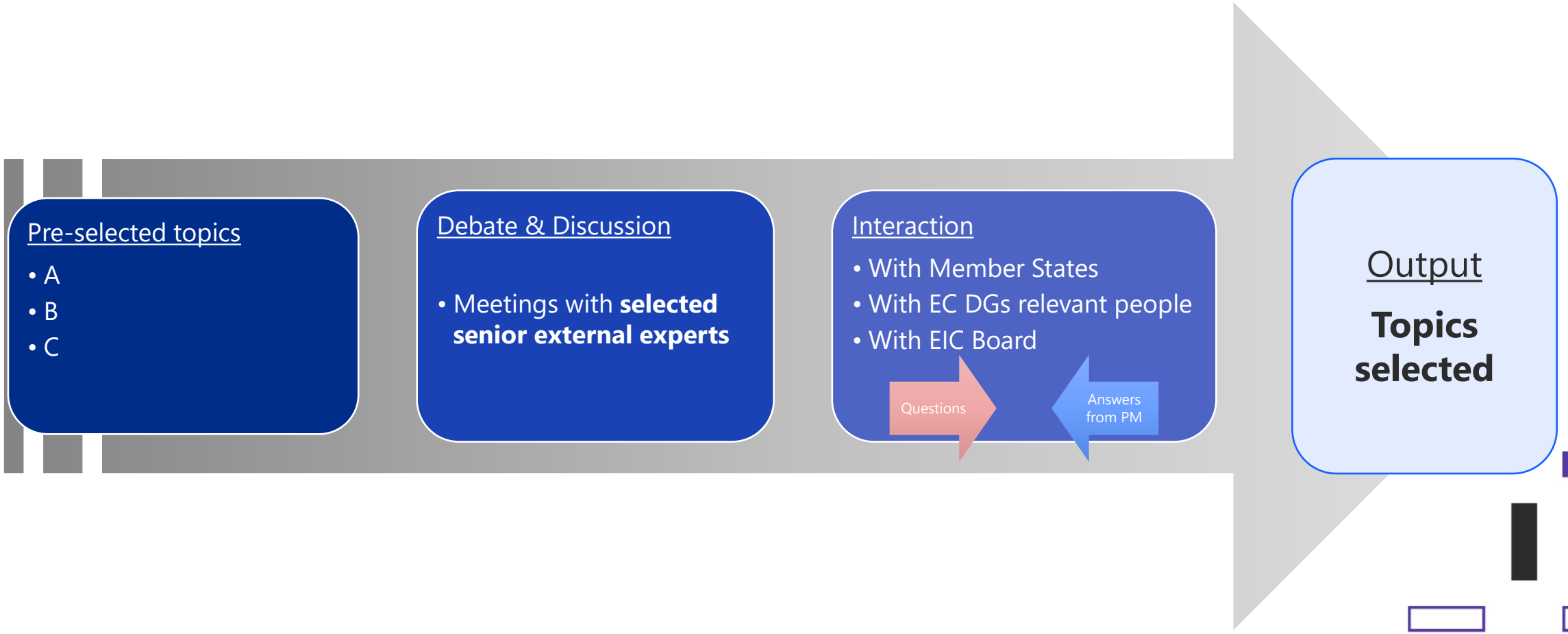
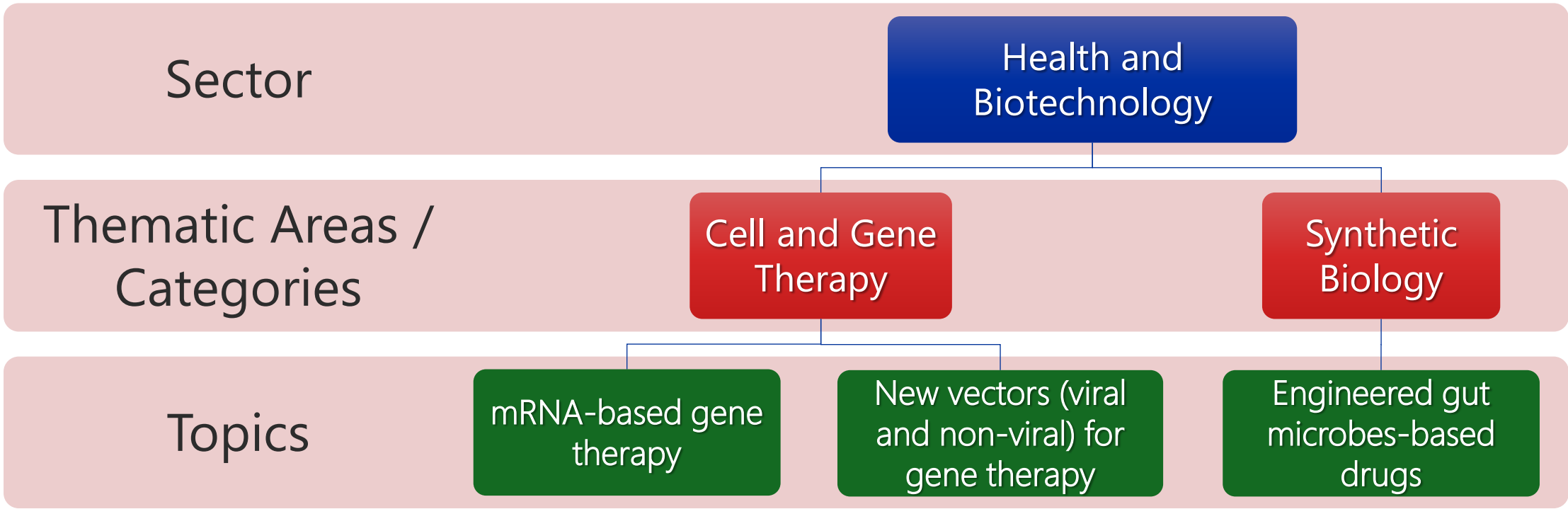


EIC Programme Managers: How do they debate and validate topics



EIC Programme Managers: From sector to topic



Health & Biotechnology sector
Pathfinder Thematic Areas/Categories

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- Cancer with subcategories e.g. solid and blood cancers
- Neurological disorders
- Infectious diseases
- COVID-19
- Synthetic Biology-Synthetic Genomics
- Disease modeling/Regenerative medicine/Bioprinted tissues
- Inflammation/Autoimmunity
- RNA based therapies
- **Cell & Gene Therapy**
- Diagnostics
- Industrial biotechnology/non medical/Enzymes
- Biopharmaceutical processes/compounds/manufacturing

Why cell and gene Therapy?
Current Market outlook (I)

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- A single gene therapy SME acquisition/deal can exceed the annual budget of EIC (~1.5bn)
- Bayer acquired gene therapy company Asklepios BioPharma for $2 bn upfront and a potential further $2 bn in success-based milestones because of the company's gene therapy capabilities in AAV and manufacturing process (2021)
- Bristol Myers Squibb agreed to acquire cardiovascular company MyoKardia for $3.1 bn through an all-cash deal (2020): www.nature.com/articles/d43747-020-01182-1
- Roche acquired Spark Therapeutics Inc for $4.3 bn (2019)

Why cell and gene Therapy?
Current market outlook (II)

- Gene therapy is a market on the rise and significant growth should be expected
- New technology will increase the number of therapeutics (e.g. >$1bn was invested in gene editing-based start-ups by 2018 with the first human trials in 2019)
- However, the gene editing-based therapeutics/drugs are unlikely to have significant clinical impact before 2025

Why cell and gene Therapy?
Current market outlook (III)

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- According to the 2020 Pharma report "*Medicines in Development for Cell and Gene Therapy*" (https://www.phrma.org/en/Report/Medicines-in-Development-for-Cell-and-Gene-Therapy-2020), **362** cell or gene therapies were in the pipeline in 2019, 25% increase compared to the same figure in 2018. This number exceeded **800** trials in 2020
- However, currently approved gene therapies in the US market, are only a handful with RNA therapeutics being the first 2 therapies approved
- Despite the above figures, **5-10** CGT approvals per year are foreseen for the next 5 years

Why cell and gene Therapy?
Challenges

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Safety

In vivo efficacy

Manufacturing

Why cell and gene Therapy?
Challenges in manufacturing

European Innovation Council

Despite the undeniable ability of viral vectors to deliver pay and despite the remarkable potential of this approach to treat a wide range of diseases, gene therapy manufacturing is being faced with serious challenges such as:

- Scalability
- Advanced analytics to maintain quality and meet regulatory requirements
- Cost effectiveness
- Difficulty in maintaining productivity
- Accessibility

EIC's response to CGT challenges:
1: EIC Pathfinder Challenge call in the Work Program 2021:

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Expanding CAR-T cell therapies to solid tumors

Other than T cell Type effective Cell Therapies

Development and Manufacturing of autologous and allogeneic CAR-T cells

New gene therapeutic approaches

Improving gene delivery systems (viral and non-viral vectors)

Improving cell and gene therapy manufacturing

EIC's response to CGT challenges:
2: Cell and gene therapy: the first ever EIC-ERC workshop

29 June 2021

The e-report for the cell and gene therapy workshop held last June, is now available on the EISMEA's website:

https://eic.ec.europa.eu/news/cell-and-gene-therapy-first-eic-erc-workshop-recordings-and-presentations-available-now-2021_en

The e-report encompasses all presentations, 3 recordings, a Statement by the two Chairs and a summary Statement:

Statement from the EIC-ERC contact group co-chairs (europa.eu)

Statement from EIC Programme Manager Iordanis Arzimanoglou (europa.eu)

Selected examples of EIC-Pathfinder projects within the Health and Biotech portfolio

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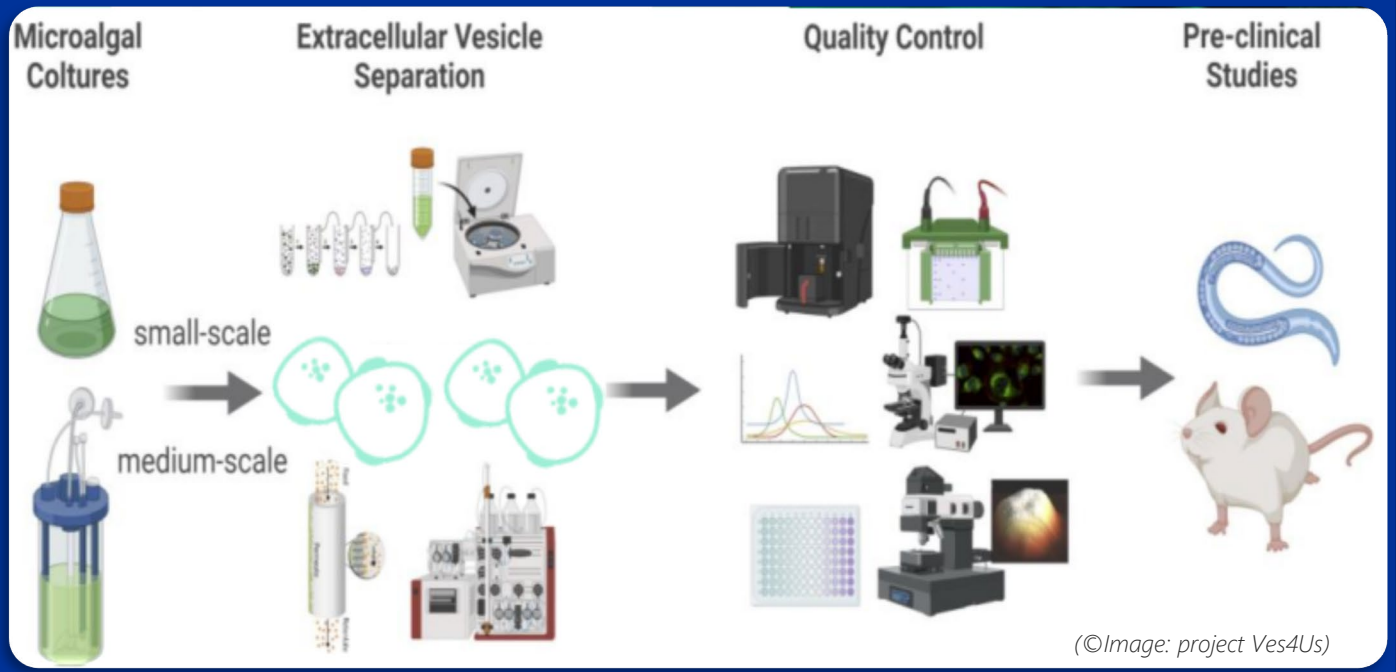
1. EVs for drug delivery in specific tissues (*project: Ves4Us*)

- Extracellular vesicles (EVs) are cell-derived, membranous particles that can transfer proteins and RNAs
- EVs as drug delivery vehicle are: i) well tolerated in the body, ii) have long circulating half-life, iii) are internalised by recipient cells and iv) are able of crossing the BBB

The aims of this project are to develop:

a) **EVs derived from natural source for drug delivery in a tissue-specific manner (brain, lung, skin, dendritic or tumor cells..) and**

b) **A new platform for the efficient production and functionalisation of EVs (current challenges), which would enable for their exploitation as tailor-made products**



(©Image: project Ves4Us)

2. Modelling pancreatic diseases with bioprinting (*project: Pan3DP*)

Developing therapies for pancreatic diseases, such as diabetes and pancreatic cancer, is hampered by a limited access to pancreatic tissue in vivo. Bioprinted (3D) tissue models, can mimic the native organ and, therefore, be used for modeling the pancreatic diseases in pharmaceutical testing.

The aims of this project are to:

a) Expand and unify the knowledge of 3D in vivo architecture of the developing pancreas

b) Develop technology that would effectively support vascularization in bioprinted pancreatic tissue units and

c) Establish conditions for in vitro differentiation and maturation of the bioprinted pancreatic tissue

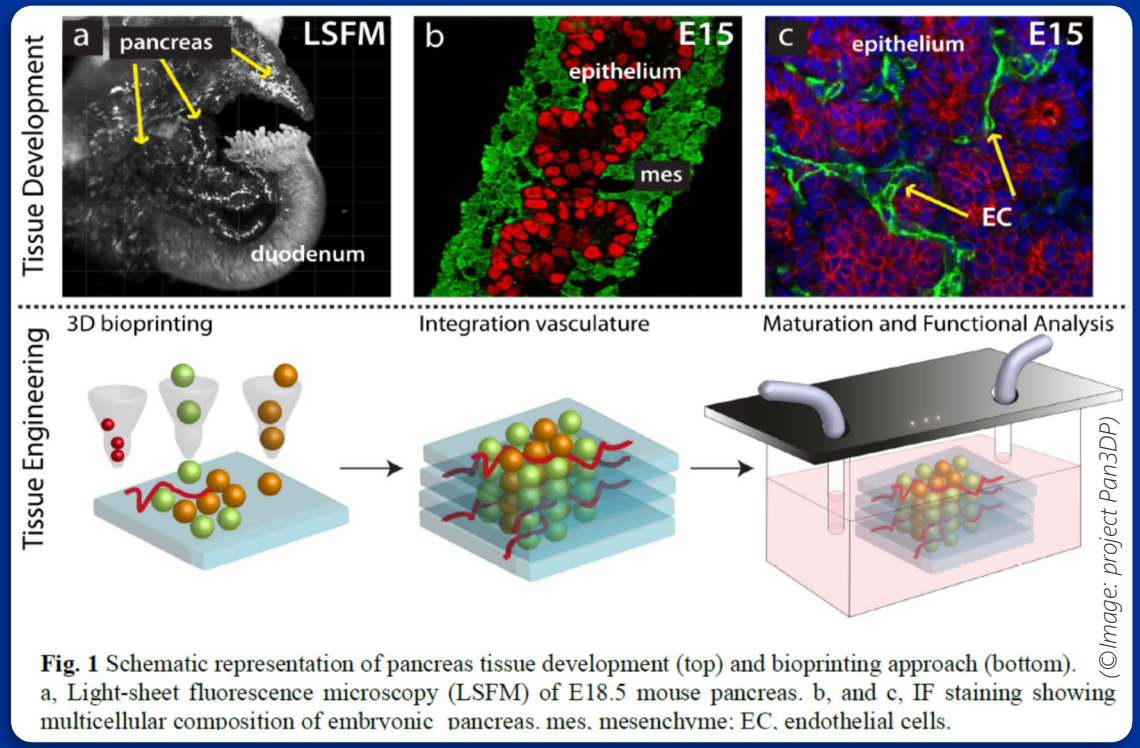


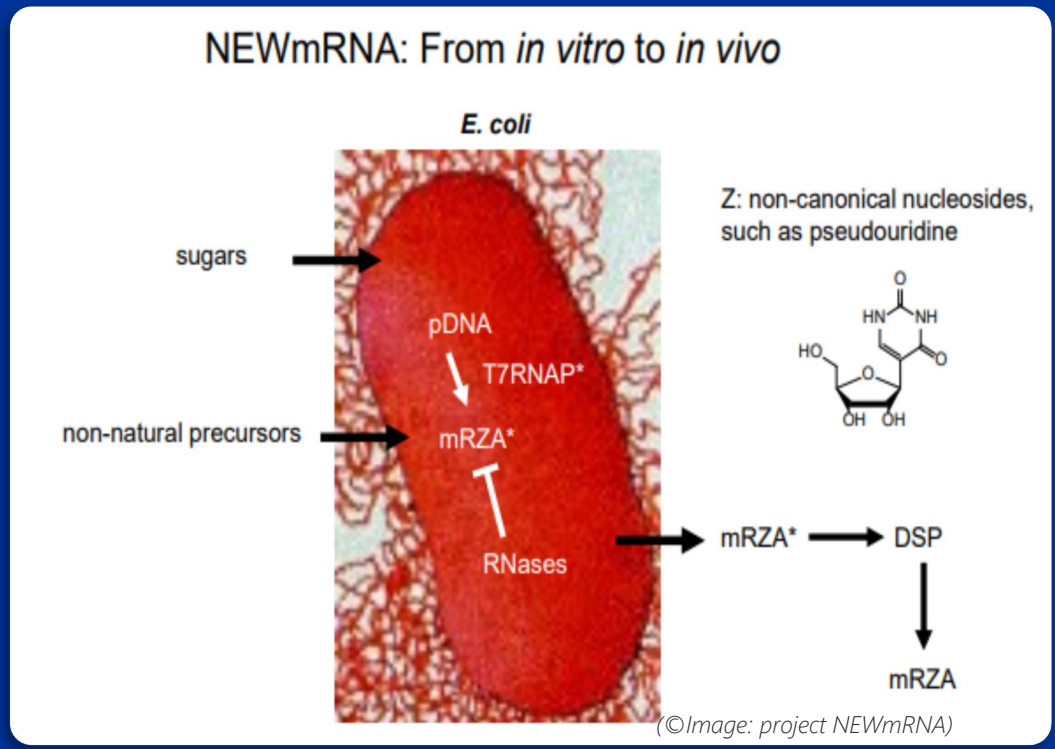
Fig. 1 Schematic representation of pancreas tissue development (top) and bioprinting approach (bottom). a, Light-sheet fluorescence microscopy (LSFM) of E18.5 mouse pancreas. b, and c, IF staining showing multicellular composition of embryonic pancreas. mes, mesenchyme; EC, endothelial cells.

(©Image: project Pan3DP)

3. A Synthetic Biology approach for mRNA (*project: NEWmRNA*)

- mRNA is a major actor in the expression of cellular information. Its diverse role encompasses major life science applications including vaccination via mRNA-based gene therapy and diagnostics to control pests in agriculture.
- A novel cellular assay is proposed to rapidly assess the impact of non-canonical forms of mRNA on translation and cellular signaling, by reengineering enzymatic mRNA synthesis via T7 RNA polymerase

- **Two specific aims of this project, which clearly constitutes a synthetic biology approach, are to:**
  a) Redraw the chemical map of the translation of mRNAs in human cells, and
  b) Transform biological mass production of mRNAs by introducing crucial steps to its industrial-scale production of mRNA



(©Image: project NEWmRNA)

Selected examples of EIC Pathfinder projects within the Health and Biotech portfolio

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4. Changing current industrial biotransformations catalyzed by enzymes (*project: Hotzymes*)

- Enzymes catalyze complex biotransformations. This project proposes a new concept to exert functional control over different enzymes using magnetic heating
- To accomplish this, there is a need to design and fabricate a new generation of magnetic bioreactors suitable to Biocatalysis
- From high-cost to low-cost biopharmaceuticals

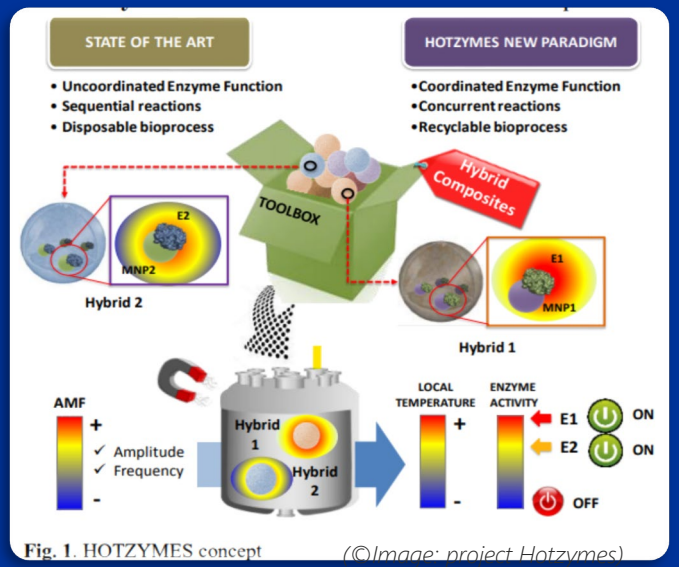

Fig. 1. HOTZYMES concept (©Image: project Hotzymes)

The main aim of this project is to contribute to the change of the current industrial biotransformation process with uncoordinated enzyme function, sequential reactions and disposable bioprocesses into a coordinated enzyme function, concurrent reactions and recyclable bioprocesses (game-changing?)

5. A platform for selection, expansion and training of T cells for solid Tumor adaptive cell therapy (*project: INCITE*)

Adoptive Cell Therapy with engineered T cells (TCR-transgenic and CAR-T cells) has indeed demonstrated success in the treatment of patients affected by leukemias, but is much less effective against lymphomas and solid tumors. One likely explanation is that we do not educate the right type of T cells. The T cells considered to be the gold standard for tumor therapy, but the proper and safe way to generate these fit T cells for clinical purposes is still an unresolved matter

The aim of this project is to engineer a tailored micro-environment (using a novel 3D microfabrication technology), that will be used for T cell education, in order to generate the fittest anti-tumor T cells for advanced adoptive T cell therapy

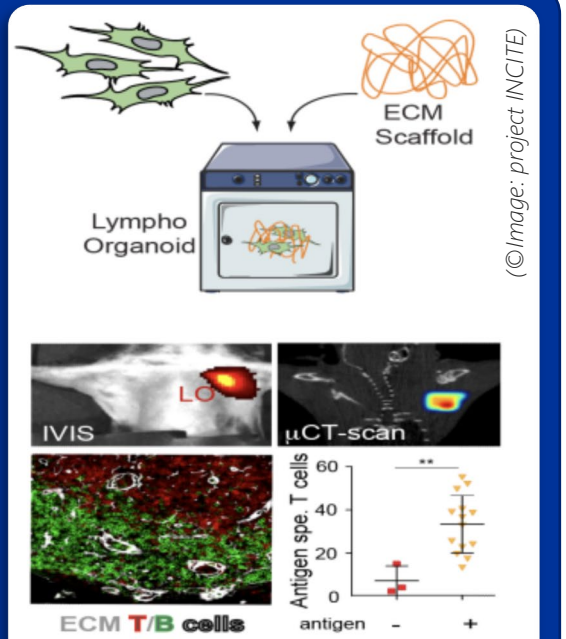

Figure 1.1c Proof-of-principle approach for generating functional lymphorganoids for in vivo applications showing accumulation of antigen-specific T cells into newly developed immune niches