

On behalf of the **RESTORE** consortium, I would like to invite you to attend the event: **"Pursuing FET in Horizon Europe: Strategic Planning on Living Drugs by RESTORE"** to give your views on how the EC will bridge the six short listed research teams from **Horizon 2020 to Horizon Europe**.

The event will take place at Dec 5th from 12:30 to 14:00 in the Member's Salon of the European Parliament. If you need any further details, please do not hesitate to contact us at hans-dieter.volk@charite.de

Please also find attached the **RESTORE** position paper on the Advanced Therapies Roadmap for Horizon Europe.

Kind Regards,

Hans-Dieter Volk

Draft Agenda

50 min short presentations – 40 min networking

MEP Mr. Ehler: introduction

- Why and What?

- Restore Health by “living drugs” – (*Hans-Dieter Volk, RESTORE coordinator*)

- Impact on Science and Technology Development

- Breakthrough in Stem Cell-based Therapies (*Marc Peschanski, Director I-Stem, INSERM, Paris*)
- The molecular ID card reveals the secret of each single cell (*Andreas Radbruch, Director DRFZ, Mir-Farzin Mashregi, DRFZ, Leibniz Association*)
- INSERM's input on FET Flagship and RESTORE (*Guillaume Fusai, Responsable Affaires Européennes, INSERM*)

- Impact on Patients

- Access to Advanced Therapies - Challenges and Solutions from People with Rare Diseases (*Karolina Hanslik, EURORDIS – patient advocates*)
- My life is better by reshaping immune system – a patient case report (*Petra Reinke, Director BeCAT, Charité Berlin and Ivana Slabu, patient*)

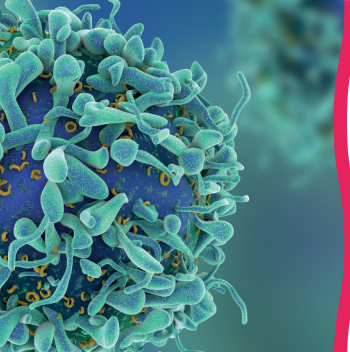
- Impact on Industry

- Opportunities of Advanced therapies (“living drugs”) for Big Pharma (*Ivana Cattaneo, Director Public Affairs Novartis Oncology Europe*)
- Emerging Technologies for living drug development – from engineering to artificial intelligence (*Zami Aberman, Executive Chairman, Pluristem, Haifa*)

- How?

- What do we recommend? (*Hans-Dieter Volk, RESTORE coordinator*)

Closing Remarks (DG RTD to be confirmed)



Health by Advanced Therapies

Pursuing FET in Horizon Europe: Strategic Planning on “Living Drugs” by **RESTORE**

WHY must Europe invest more in Advanced Therapies?

The increasing prevalence of chronic diseases and multi-morbidity due to demographic factors represents a high socio-economic burden for Europe. The direct health costs increased by 50% during the last decade and reached €1,526bn in 2017, a staggering 9.6 % of Europe's GDP (EU Health at a Glance 2018).

As current therapies rarely cure, but merely fight symptoms, life-long treatment is required. This results in potential adverse effects, limited quality-of-life, and increasing costs for society. Increasing health costs or restricting access to new therapies to a few wealthy patients are not solutions for a stable European society.

The **ultimate approach** therefore, is to tackle the problem of **affordable medicine by disruptive research and breakthrough innovations**. There is a high need to achieve sustainable improvements or even to cure chronic diseases. Advanced Therapies, in Europe categorized as ATMP, are often referred to as “living drugs” and could be the solution to sustainable improvements in chronic disease treatment. They can be specifically produced for individual patients and if feasible, they are also produced as off-the-shelf products.

Advanced Therapies are game changers that open up transforming therapeutic opportunities to “restore health” instead of just “treating symptoms”.

Advanced Therapeutics are no longer merely fiction but becoming a reality - cures exist for rare genetic diseases as well as for common immune diseases, cancer, and tissue injury. Some products are already on the market, mostly for rare diseases. However, **up until now only a few thousand patients worldwide have benefitted from this new category of precision medicine**.

“Living drugs” challenge the “tried and tested” paradigm of drug development at almost all Technology Readiness Levels from drug discovery to reimbursement. At the dawn of such a trailblazing change, obstacles and roadblocks abound. The recently approved ATMPs required a long and costly added-value chain and the complex manufacturing and development processes resulted in high product prices. There is a high demand for many more Advanced Therapy products that can be developed cost-effectively and in a timely manner to enable the use of Advanced Therapies by millions of EU citizens.

The pace of progress has accelerated over the last few years, notably in the US and Asia **but much less so in Europe**, despite its high innovation potential and demonstrable success in the past. The EU can decide whether to be merely a **payer** for revolutionary (and expensive) drugs developed elsewhere that might not be affordable for all patients in need, or to become a **player** by launching a virtuous circle where drug expenditures by the healthcare systems stimulate economic growth in a research and innovation intensive sector such as red biotechnology.

Therefore, there is a need for a focused, determined and well-funded **large-scale research initiative** in Europe to create a true **European Research Area on Advanced Therapies**, combining the power of small, scattered teams for disruptive science and technology with larger structures for developing recent successes into innovation with impact (Wu L et al. Nature 2019, <https://doi.org/10.1038/s41586-019-0941-9>). That was the motivation to found **RESTORE**, an EU Horizon 2020-funded network from the former **FET-flagship competition**, consisting of a core team and >300 supporters from academia, industry, patient advocates and other stakeholders (<https://restore-horizon.eu>).

RESTORE aims to establish a sustainable European ecosystem integrating transdisciplinary research, clinical centers, pharma and tech industry, regulatory bodies, patients, and public society to overcome technological and regulatory roadblocks in Europe for the broad implementation of Advanced Therapies.



This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 820292.



info@restore-horizon.eu
www.restore-horizon.eu

The Vision of RESTORE

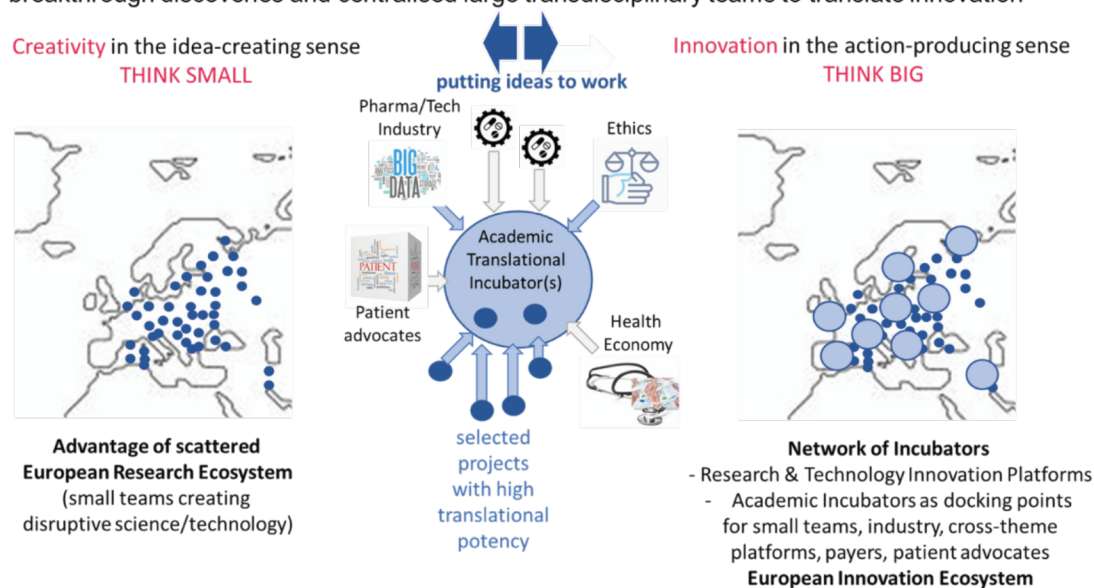
- Deliver a pipeline of revolutionary “living drug” products “**developed and made in Europe**” and transform the disruptive promise of Advanced Therapies into a reality to cure chronic diseases
- Make them **accessible/affordable** as “**standard-of-care**” for every patient in need
- Make **Europe a spearhead** of Advanced Therapies in Science, Clinics and Biomedical Industry
- Make use of the **enormous socio-economic potential** of novel breakthrough-therapies in Europe

Key elements to achieve these aims are (Fig.1):

- Combine the benefits of small science teams scattered across Europe (MS/AC) and their breakthrough ideas with centralized large transdisciplinary teams to translate innovation into measurable benefits for patients and society.
- Broadly accessible **Research and Technology Innovation (RTI) platforms** (mission-driven basic science for discovering new targets and indications, manufacturing, product characterization, advanced preclinical models, biomarkers, early and late clinical development/regulatory affair, scaling-up/automation, and reimbursement), and **Cross-theme platforms** (ethics, artificial intelligence, health economy).
- **Academic translation incubators** as docking points for mission-driven basic research, technology developers, biotech/pharma industry, and special interest venture funds
- Coordination and support action for all efforts.

Fig.1 How can we cost-effectively accelerate and de-risk Clinical Development of Advanced Therapies ?

Combine the benefits of small science teams scattered across Europe (MS/AC) with their breakthrough discoveries and centralised large transdisciplinary teams to translate innovation



IMPACT and RETURN of INVESTMENT

- Boosting Europe’s competitiveness in the ground-breaking area of “living drugs”
- Improved survival and quality-of-life for patients with chronic diseases, such as cancer, rare diseases, immune diseases, metabolic and cardiovascular diseases etc.
- Prevention of chronic diseases by pre-emptive curative treatment of acute events, such as trauma, ischemia/reperfusion injury, infection etc.
- Decreased financial burden on society by chronic diseases
- Innovative product pipeline for existing industry and new players inducing high return of investment
- Generation and retention of highly specialized workforces in Europe
- Setting ethical and quality standards for these new therapies

PROBLEM

Recent funding through research and innovation actions (RIAs) and support for SME's as well as some single ERC grants under FP7 and Horizon 2020 programmes (around €150-200 million in total) has been beneficial in driving the new field forward, but it is **structurally and budget-wise not sufficient and competitive for the future**. The originally planned new FET flagship programme would have provided a good opportunity to mobilise the various existing potentials and make Europe competitive for the future in this field - the question therefore arises, how to make alternative resources and organisational models available after the cancellation of FET-Flagships.

RECOMMENDATIONS

- Tackle the rising problem of chronic diseases for patients and society by **providing affordable medicine** taking advantage of the disruptive research and breakthrough innovations in Advanced Therapies.
- **Switch from the current focus on scattered, time limited, unconnected EU-funding to a concerted funding program for Advanced Therapies**, integrating innovative elements supporting a creative and productive ecosystem and to build a true European Research Area on Advanced Therapies.
- **Take the momentum gained from the FET-Flagship initiative in bringing together stakeholders in the field and go in new directions** by supporting sustainable structural elements with sufficient budget according to the **achievement of key performance indicators** over the next seven years of the Horizon Europe programme, as an adequate structural and financial alternative to the cancelled FET-flagship programme:
 - **Coordination and Support Action (CSA)**: consortium with transparent and dynamic governance that provides support, guidance, education, advice, networking, and expertise for the large-scale research initiative on Advanced Therapies
 - **Infrastructure**: Network of Academic Translational Incubators (Hubs) with adequate infrastructure (competitively selected, co-financing only, basic budget by national/regional funding organisations) as docking points for small teams with breakthrough projects, patients advocates, biotech, pharma and technology providing industry, health insurances and special interest venture funds to accelerate and de-risk the development of Advanced Therapies by:
 - i) implementing broadly accessible Research and Technology Innovation platforms,
 - ii) performing high-quality clinical trials,
 - iii) applying the Refined Translation Tool for de-risking late and costly clinical development
 - iv) implementing incubators as docking point for industry and special interest venture funds
- **Research and Innovation Actions (RIAs)**: Feed the small teams to generate mission-driven disrupting science and technology interlinked in thematically structured consortia. Keep the RIA projects flexible such that they can be halted or extended based on key performance indicators, for getting new pipeline projects and building-up European Research & Technology Innovation platforms (incl. smart manufacturing 4.0, health-economics, ethics).
- **Private Public Partnership and SME support**: **To bring innovation to the market and patients** we need to strengthen the relationships between academia with industry and investors to support private funding and sustainability for EU-funded projects on Advanced Therapies, incl. support of SME/industry docked to the programme notably through the **European Innovation Council (EIC)**