

#### OVERVIEW

Shifting from treating symptoms to curing chronic diseases by making the transformative promise of Advanced Therapies a reality for the benefit of patients and society and by making Europe a spearhead of Advanced Therapies in Science, Clinics and Biomedical Industry, that is the vision of the international project **RESTORE Health by Advanced Therapies**, coordinated by the Charité Berlin & Berlin Institute of Health.

#### WHY do we need RESTORE?



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, never-ending treatment is required, which means diminished quality-of-life, adverse effects and soaring cost for society. There is a high need to reach sustainable improvement for patients or even to cure them of chronic diseases – in other words, to disrupt the paradigm of "treating symptoms" with "restoring health". Advanced Therapies are the game changers that open up transforming therapeutic opportunities.

#### What are Advanced Therapies?

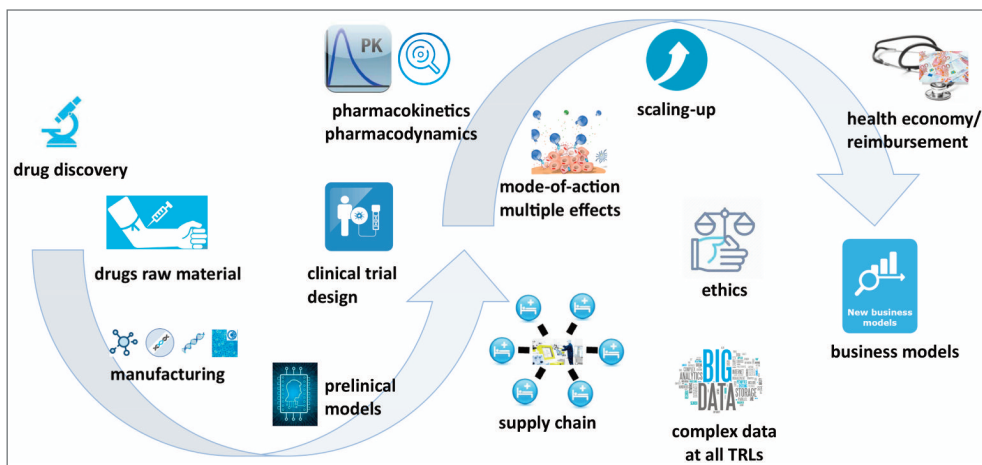
Advanced Therapies, in Europe categorized as Advanced Therapy Medicinal Products (ATMP), are often referred to as "living drugs" and include cell & gene therapy products and tissue engineered products, as well as the combination of these products with medical devices such as biomaterials. They are often produced specifically for individual patients (personalised therapies) but if feasible also as off-the-shelf products (a product derived from one donor for many patients). In their manufacturing, clinical use, and mode-of-action they are characterized by a high degree of complexity compared with conventional drugs or antibodies.

For genetic diseases, immune diseases, cancer and tissue injury potential cures through Advanced Therapies exist - it is reality, not fiction. Some products are already on the market, mostly for rare diseases which means only a few thousand patients worldwide have benefitted from Advanced Therapies until now.

#### Why are there so few Advanced Therapies implemented in clinical routine so far?

"Living drugs" are a **disruptive innovation** that challenges the "tried and tested" paradigm of drug development at almost all Technology Readiness Levels (Fig.1).

Fig. 1  
"Living Drugs" - a disruptive innovation shatters current para-digms on all levels and generates new high-value opportunities



At the advent of such a trailblazing change, obstacles and roadblocks abound. The recently approved Advanced Therapy products required a long and costly added-value chain (>20 years) and the complex manufacturing and clinical development results in high product prices.

Advanced Therapy development is driven by academia and small biotech (a new phenomenon in drug development), although Big Pharma steps in now. The pace of progress has accelerated over the last few years, notably in the US and Asia. Europe lags behind despite its high innovation potential and demonstrable successes in the past. Therefore, there is a need for a focused, determined and well-funded large-scale research initiative in Europe - that's the motivation to **found RESTORE**.

## WHAT is RESTORE aiming at?



### The **Vision**; “Living drugs” will RESTORE health to chronic disease patients

- Make the disruptive promise of Advanced Therapies to **cure** chronic diseases a reality
- Make Advanced Therapies accessible as **standard-of-care for every European 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 also in Europe



### The **Mission**

- Overcome the **technological and regulatory roadblocks** for Advanced Therapies in Europe
- Deliver a pipeline of **dozens of transformative ATMP products** “developed and made in Europe” by 2030
- Establish a **sustainable pan-European ecosystem** integrating research, clinics, industry and patients

## HOW to get there?

- Establish a well-funded consortium with a long-term perspective that is backed-up by the scientific community from Life, Social and Economic Sciences, relevant stakeholders, patient advocates and the public and is building on the **3T's: Transdisciplinarity, Technology, Translation** (to patient care).
- Devise the strategic **roadmap 2021-30** to remove obstacles in order to **ENABLE** make our vision come true
- Accelerate and de-risk the development of Advanced Therapies by implementing the **Research & Technology Innovation Platforms** (one tool - many applications), and the **Refined Translation Tool** (iterative improvement by learning from success and failure) at academic hubs.
- Form the RESTORE consortium as the docking point for Biotech/Medtech/Pharma **industry** and special interest **venture funds** and **embed RESTORE into international networks**.
- **Implement a governance** that **assures performance- and trend-conscious flexibility**
- Attract funding for dedicated **infrastructure** (academic hubs), open-calls for the **Research & Technology Innovation Platforms** (from early product development to pivotal clinical trials and implementation into clinical routine), **Cross-Topic Platforms** (smart manufacturing 4.0, health-economics) and the **Coordination & Support Activities** of RESTORE (coordination and communication, specialist training and workforce development, ethics, international networks, central data management, patient advocacies...).



## IMPACT of RESTORE

by disruptive **Innovations** and **Technologies** on:

### **Science and Technology**

- infrastructure and translational centers of excellence
- boosting Europe’s competitiveness in the ground-breaking area of “living drugs”
- fundamental knowledge and technology breakthroughs relevant for several fields
- next generation of translational scientists with a new mindset

### **Societal Benefits for Europe’s Society and Economy**

- boosting quality-of-life and survival rates for patients with chronic diseases
- decreased financial burden on the society by chronic diseases
- robust and innovative product pipeline for medtech/biotech/pharma industry and new players
- generation and retention of highly specialized workforces and expertise in Europe
- setting the highest ethical and quality standards for these new therapies

## WHO WE ARE - RESTORE COMMUNITY

The **RESTORE Core Team** are a formidable group of 10 partners from academic centers and biotechs with excellent international reputation and a strong track record (Charité/BIH, Univ. Zurich, Catapult CGT, TissUse, Pluristem, Miltenyi Biotec, INSERM/Univ. Paris Saclay, InnActa, Fondazione Telethon, Univ. Minho). The Core Team and their close to 300 committed supporters from 26 countries (EU-MS, EU-AC, USA, Singapore) form the **RESTORE community**.

Please visit the **RESTORE website** ([www.restore-horizon.eu](http://www.restore-horizon.eu)) for more details on the activities, follow us on **Twitter** and **Facebook**, and **JOIN US!**

