



Health by Advanced Therapies

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**D5.6**  
**Publications and material for  
communication first report**  
Public

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Delivery date: 10/11/2019

Lead Beneficiary: INN-ACTA (Partner 8)



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

[www.restore-horizon.eu](http://www.restore-horizon.eu)

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## 1. Deliverable's description

This document describes the Communication and Dissemination activities of RESTORE – Health by Advanced Therapies, a Large Scale Research Initiative candidate funded by Horizon2020. The purpose of this document is to summarize all communication and dissemination actions implemented during the six months funding period, and including the various activities aiming at raise awareness of RESTORE Initiative, main topics, events and outputs. The activities addressed the citizen of Europe and beyond, public and scientific community, supporters' community and relevant stakeholders at different level, with the aim to increasing interest in RESTORE topics as well as to attract key opinion leader and stakeholders to contribute to the achievement of the goals of the mission.

## 2. Communication Tools and Material

Among the initially planned **communication tools** some of them are already in place from the very early stage of the project, and constantly updated:

### Press Releases

Up to now a couple of press releases have been prepared to publish announcement of funding and project start via Core Team Institutional Website and Official RESTORE website.

The last press release aiming to advertise the first Advanced Therapies has been published and distributed in Europe, Israel and United States. A copy is enclosed to this deliverable in Annex 1- Published Material.

### Messages to Community

Messages addressing RESTORE Community are regularly distributed via the RESTORE mail account to supporters signed up in the supporters mailing lists in accordance to GDPR Policy.

Messages in form as e-mails are sent to constantly keep the RESTORE Community engaged and informed about activities, events, access to website, working groups activity and share documents and informative material.

In addition, periodic **Newsletters** are distributed within RESTORE Community and available for download via supporter's reserved area and enclosed to the deliverable Annex 1 Published material.

### Project website

The **project website** was established during proposal preparation and improved in the new domain [www.restore-horizon.eu](http://www.restore-horizon.eu) during the first month of the project with an appropriate protocol for updating information on activities, techniques, and results of the project.

The website represents an important tool of communication and documents sharing with Public and Community. It includes a public facing section and two private sections, one for Supporters and one for Partners.

The public web pages provide the description of the project, its objectives, its mission, the events, the project beneficiaries and RESTORE supporters. It is mainly directed to the scientific and public community to raise awareness and interest regarding the research and to create a contact tool to facilitate collaborations. It also includes overall information of meetings, conferences, news, announcements and press-releases.

**Website for patients** A section of the website has been conceived as a communication tool with patients and their families and it is currently available in English, German, and Italian Languages.


In this section users (patients, patients relatives interested public, etc.) can address specific requests to RESTORE partners via a dedicated form.

## RESTORE FOR PATIENTS

SUPPORT RESTORE

### Information for Patients, Relatives and Patients Advocates

Select the available translations:



English version. More languages available soon.

▼ Chronic Diseases

A great number of people worldwide suffer from a broad array of chronic medical conditions ranging from cardiovascular diseases, cancer, diabetes, lung diseases, (auto) immune diseases, musculoskeletal and neurodegenerative diseases through to mental illnesses.

If you are one of those who are suffering, or know someone who is suffering with a chronic illness, you may have noticed that whilst for many of these medical conditions there are treatments available which help treat the symptoms, curative treatments appear to be a distant prospect. Since most of the currently prescribed and administered drugs often only alleviate the symptoms, they frequently have to be taken over long periods of time, or more often than not, for the rest of the person's life.

### Contact Us

Name \*

FirstLast

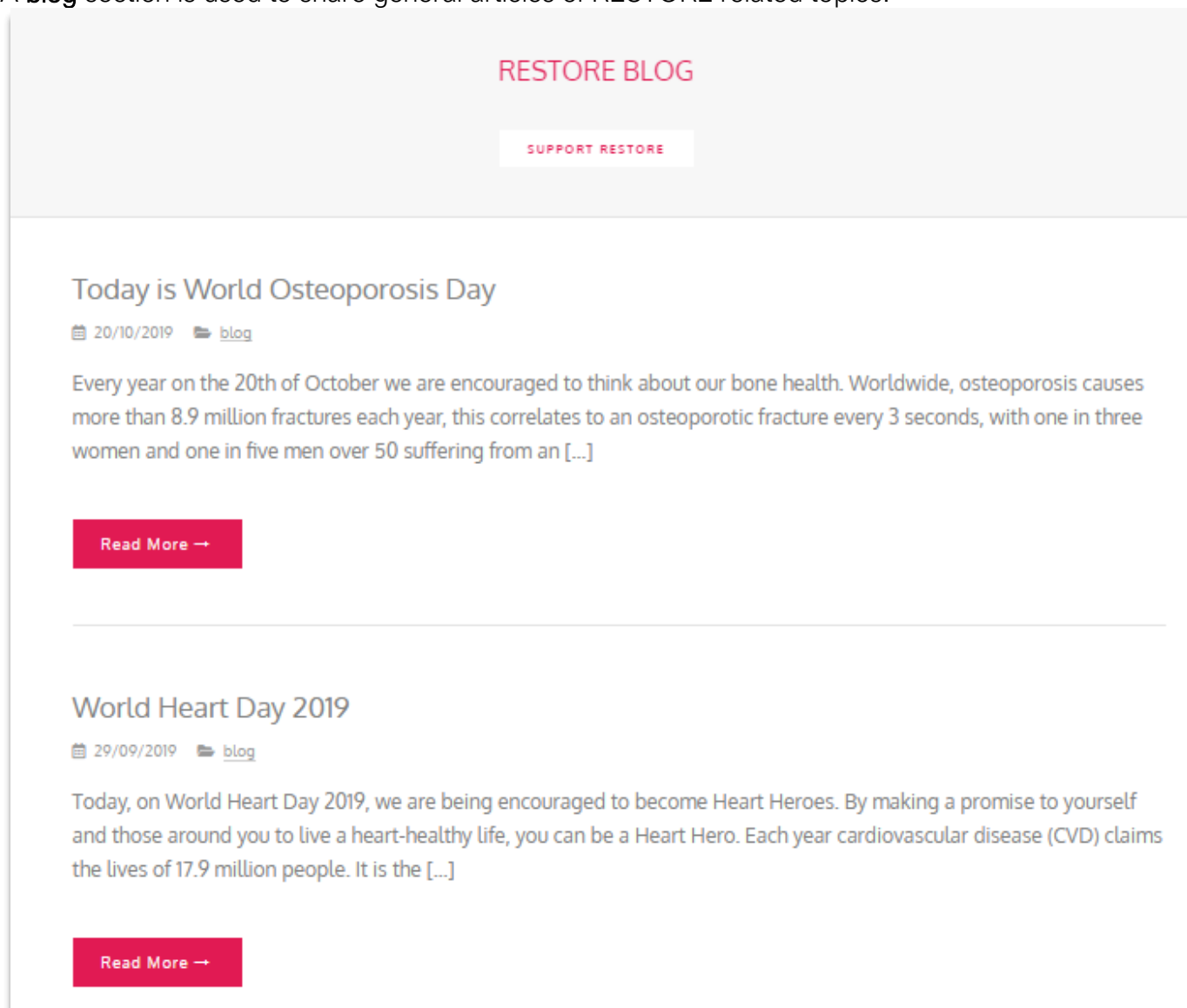
Email Address \*

Comment or Message \*

SUBMIT



A **blog** section is used to share general articles of RESTORE related topics.




**The Supporters private section**, password protected, currently hosts documents (as kick off meeting, working groups description, etc.) and a data base tool to facilitate collaboration on specific working groups activities. **The partners' reserved area** contains data base platforms for supporter's data collection and it is an important share point for internal communication and knowledge management, to collect project documents (meeting and TC minutes, presentations, partners full details contacts)


**Career corner** – getting young scientists for academic positions at research institutes and for positions in the industry can be problematic, especially in emerging fields such as Advanced Therapies. Even the FDA, a health authority, struggles with the issue and competes with industry on qualified personal ([on line article](#)). RESTORE provides, via its website, a job board for Advanced Therapies related positions. The job board is open to the public and members of the RESTORE team make sure that jobs fit to the purpose before clearing them for publishing on the website.

## Social media



RESTORE regularly communicates via [Twitter](#), [LinkedIn](#) and [Facebook](#) pages posting messages of RESTORE activities as participation to events, or news of topics related to

 Advanced Therapies to keep the community and followers updated. This tool allows reaching wide public audience, patients, their families and scientific community.





**RESTORE** @RESTOREhorizon · 22h

RESTORE's #1stATSM is now an EACCME accredited event (12 credits)! Join us in Berlin on 25-26 November to hear and discuss highlights and challenges in #advancedtherapies: from basic research to manufacturing and clinical implementation @fet\_eu



**RESTORE** @RESTOREhorizon · Oct 24

Finding the right people can be problematic, especially in emerging scientific fields. To assist the scientific community and industry in this very important task, we created the Career Corner specifically for #advancedtherapy related positions. @fet\_eu



**RESTORE** @RESTOREhorizon · Oct 22

Petra Reinke's welcome speech for the RegMed Forum - we are looking forward to an interesting discussion between patients, physicians and scientists about #advancedtherapies in general and for various clinical indications! @fet\_eu @BerlinPartner @ChariteBerlin @berlinnovation



**RESTORE** @RESTOREhorizon · Sep 30

Today! Hans-Dieter Volk presents @RESTOREhorizon at the final event of the Italian national IRMI project 'Italian Infrastructure for Regenerative Medicine' in #Bologna #advancedtherapies @FETFlagships



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6



**RESTORE** @RESTOREhorizon · Sep 18

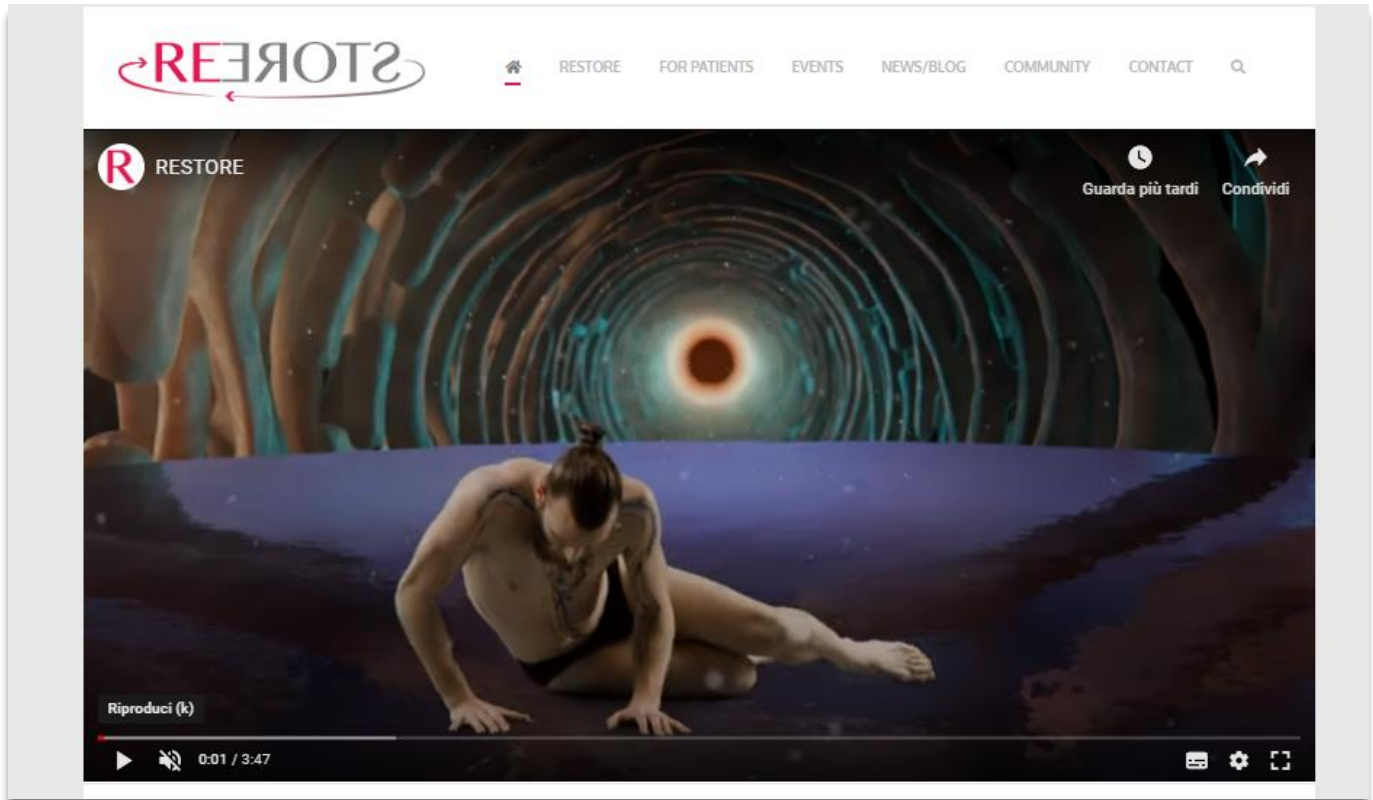
Zami Aberman of @Pluristem presents Pluristem, a @RESTOREhorizon partner, and RESTORE itself in a #manufacturing session at the #PLE2019 in #London #advancedtherapies @fet\_eu



## Advertisement Material

A first **video** aiming at advertising RESTORE has been produced and published in the home of RESTORE website. Moreover, the video has been shared through social media, YouTube channel and it has been projected during the Long Night of Science in Berlin (15 June 2019).

Currently, the video is available in English <https://youtu.be/f44xYZDPS30> and German language <https://www.youtube.com/watch?v=V8jm4--iXLI>



Communication and advertisement material are also available for download from the website

- [Slides for sharing](#)
- Two versions of RESTORE **fact sheet** have been prepared and shared with EC and public page of website.
- A RESTORE **leaflet** has been distributed during the Long Night of the Sciences in Berlin. The leaflet, enclosed in the annex is regularly updated with the new joining supporters.
- A **graphical recording** of the Kick off meeting has been also prepared and available for download at [https://www.restore-horizon.eu/wp-content/uploads/2019/05/Kick-off\\_meeting\\_graphical\\_day\\_12.jpg](https://www.restore-horizon.eu/wp-content/uploads/2019/05/Kick-off_meeting_graphical_day_12.jpg)
- **General road map** available for download at the [https://www.restore-horizon.eu/wp-content/uploads/2019/11/RESTORE\\_RoadMap\\_07.11.19.pdf](https://www.restore-horizon.eu/wp-content/uploads/2019/11/RESTORE_RoadMap_07.11.19.pdf)





## Meetings

During the first eight months of the CSA activities the RESTORE Consortium has organized meetings with European Commission, UK Embassy, Policymakers to present the objectives and strategy of RESTORE.

The Kick Off meeting gathering over 200 participants was held on 6-7th May 2019 in Berlin. During the event breakout sessions for Working Groups have been organized allowing initial discussion and involvement of supporters in RESTORE activities.



The poster for the RESTORE KICK-OFF EVENT features a central image of a hand holding several hexagonal icons representing various scientific and medical fields. The text at the top includes the European Union flag, funding information from the Horizon 2020 programme, and the website [www.restore-horizon.eu](http://www.restore-horizon.eu). The event title 'RESTORE' is written in a large, stylized font, with 'KICK-OFF EVENT - 6th-7th May 2019 in Berlin' below it. The bottom section contains text about the event's purpose and contact information, followed by logos of partner organizations.

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

[www.restore-horizon.eu](http://www.restore-horizon.eu)

# RESTORE

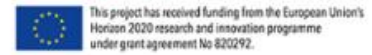
**KICK-OFF EVENT** - 6<sup>th</sup>-7<sup>th</sup> May 2019 in Berlin

**RESTORE Partners and Supporters** will meet aiming the sharpening of RESTORE roadmap and engagement of new supporters. If you are not yet supporter and you are interested to participate to RESTORE activities, contact us at [info@restore-h2020.eu](mailto:info@restore-h2020.eu)

Logos of partner organizations: CHARITÉ, University of Zurich, CATAPULT, TISSUSE, Pluristem, MACS, Inserm, Inn-Acta, FUNDRAISING, Telethon, and others.

A meeting at the EU Parliament was originally organized in October but due to overlapping commitment of EU parliament members the meeting has been postponed and it will take place on 5th December 2019. Details of the event and pre-registration are available on the website.





## Scientific Conference

The Scientific Conference “The First Advanced Therapies meeting” (1ATSM) accredited by EACCME® and entirely organized by RESTORE, will be held on 25-26th November 2019. The meeting will bring together experts from industry, patient organisations and academia to discuss the challenges within the field of Advanced Therapies and to drive forward a concerted interdisciplinary effort, making use of the excellent science, infrastructure and funding landscape within Europe, to make regenerative therapies a reality for all.

Around 400 participants are expected to attend the event.

Registrations and abstracts submission have been managed through the [1ATSM website](#) (hosted on the RESTORE website), that it is regularly updated with the most recent news and details about the programme.

The event has been advertised via social media, via website, via core partners’ network and via press releases published in Europe, Israel and United States

**EXPLORE**  
the latest highlights and discuss the most pressing challenges from the field of Advanced Therapies

**ENGAGE**  
with leaders in research, technology development and clinical application

**REGISTER NOW**  
[www.restore-horizon.eu/1atsm-home](http://www.restore-horizon.eu/1atsm-home)

**Selected Speakers:**  
 Ada Yonath - Director of Weizmann Institute of Science, Israel  
 Maksim Mamonkin - Baylor College of Medicine, USA  
 Michele De Luca - University of Modena, Italy  
 Manuela Gomes - University of Minho, Portugal  
 Tal Dvir - Tel Aviv University, Israel  
 Paolo Gasparini - University of Trieste, IRCCS, Italy  
 Timothy O'Brien - National University of Ireland Galway, Ireland  
 Alexandra Marques - University of Minho, Portugal

## RESTORE First Advanced Therapies Science Meeting

25 and 26 November 2019, Berlin

**REGISTER NOW on 1ATSM**

Career corner

**1ATSM**  
has been accredited by the  
**European Accreditation Council**  
**for Continuing Medical Education**  
**(EACCME®)**

The RESTORE Consortium (partners & supporters) participated to a numerous events (conferences, B2B meetings,...) that are summarized (in black font) in Tables 1 ( RESTORE Joint partner & supporter events) and Table 2 (RESTORE partners individual events) Some events (in magenta color) are planned in the upcoming months.

Table 1 RESTORE Joint events

Event Name	Location	When	RESTORE Partner	RESTORE Persons	Approached Audience	Content
	Brussels	22 February 2019	Charite, INSERM	A. Bennaceur-Griscelli, P. Reinke, A. Turhan, HD Volk		RESTORE in general, specific aspect pluripotent cells & their application in disease models & therapy, incl. haplobanking
	Brussels	25 March 2019	Charite, Telethon, Miltenyi	S Miltenyi, L Monaco, P Reinke, A. Turhan, HD Volk	EU officials and officers, policy makers	RESTORE specific aspects, manufacturing, rare disease
British-German Alliance for Advanced Therapies	Berlin	26 March 2019	Catapult, Charite, Miltenyi, TissUse	M Apel, U Marx, P Reinke, HD Volk	UK-DE SME and industry, policy makers	RESTORE in general and specific aspects, collaboration with UK-based scientific institutions and companies
Cell and Gene Meeting on the Med	Barcelona	23-25 April 19	Catapult, Charite, Pluristem	Z Aberman, C Feest	SME and industry	RESTORE general aspects, international awareness, industry/CDMO support
BioMed	Tel-Aviv	13-17 May 19	Charite, Pluristem	Y Yanay, G Goldsobel	SME and industry	RESTORE general aspects, international awareness, industry/CDMO support
CAAT Europe & Center of Entrepreneurship of TU Berlin Information Day on Microphysiological systems	Berlin	17 June 2019	TissUse & Charité	U.Marx; P.Reinke	SME, Industry; Academia	RESTORE general aspects, international awareness,

						industry/CDMO support
FOCIS	Boston	20 June 2019	TissUse, Miltenyi, Charité	H.-D. Volk; B. Atac-Wagegg; NN; S. Weber, A. Aiuti; P.Reinke	Academia, Industry	RESTORE general aspects, international awareness, industry/CDMO support
Phacilitate Learders Europe	London	17-18 September 2019	Pluristem, Charite	Zami Aberman, Petra Reinke, HD Volk, Mohamed Abou El-Enein	SME, Industry; Academia, Venture Capitals	
IMI2 proposal on 'Engineered T cells'	Germany	26 September 2019	Miltenyi, Charité	Michael Apel, HD Volk	European Research Consortium, 20 participants	Joint participation in IMI2 call on 'Engineered T cells', Acronym T2EVOLVE
Survey on regulatory and ethical issue of ATMP therapy		Start in November 2019	Miltenyi, Charité	Michael Apel, Mohamed Abou EL-Enein	RESTORE members	Joint survey on regulatory and ethical issues of ATMP therapies
The First Advanced Therapies meeting" (1ATSM)	Berlin	25-26 November 2019	RESTORE Core Team		Industry, academia and patients organizations	
Meeting at EU parliament	Bruxelles	5 December 2019	Charite + RESTORE Core team			Pursuing FET Flagship in Horizon Europe: Strategic Planning on Living Drugs by RESTORE
RESTORE Momentum workshop	Berlin	17 January 2020	RESTORE Core team			



Table 2 (RESTORE partners individual events)

Event Name	Location	When	RESTORE Partner	RESTORE Persons	Approached Audience	Content
ARM Directors meeting	San Francisco	5 January 2019	Pluristem	Zami Aberman	ARM members	RESTORE in general
Press release	Europe	14 February 2019	Miltenyi	Michael Apel	European researcher institutions, Journals, Press Agencies	The research initiative RESTORE Health – Therapies that cure
DECHEMA Board and Steering Committee	Frankfurt	25 February 2019	TissUse	Uwe Marx		RESTORE in general to attract the DECHEMA e.V. as a LOI partner
9th Orphan Drugs and Rare Diseases Global Congress 2019 Europe	London	5-6 March 2019	Telethon	Lucia Faccio	SME, Industry; Academia, Venture Capitals	RESTORE HEALTH BY ADVANCED THERAPIES – A EUROPEAN LARGE-SCALE RESEARCH INITIATIVE
CARAT consortium Meeting	Frankfurt, Germany	7 March 2019	Miltenyi	Michael Apel	European researchers	Presentation of collaboration joint opportunities between CARAT and RESTORE
CD20CAR-TIME consortium Meeting	Bergisch Gladbach, Germany	13 March 2019	Miltenyi	Patricia Marschall	German researchers	Presentation of collaboration opportunities with RESTORE
DECHEMA Meeting - Sections Cell Culture Technology and Medical Biotechnology	Hannover	20 March 2019	TissUse	Uwe Marx		RESTORE in general and proposal to advice during white paper writing "preclinical models"
European Society for Gene and Cell Therapy - Spring School	Naples (Italy)	3 - 5 April 2019	Telethon	Lucia Faccio	Academia	RESTORE HEALTH BY ADVANCED THERAPIES – A EUROPEAN LARGE-SCALE RESEARCH INITIATIVE
2019 Society for Biomaterials (SFB)	Seattle, US	3-6 April 2019	Minho	Rui Reis, Nuno Neves	SME, Industry; Academia, Venture Capitals	RESTORE in general

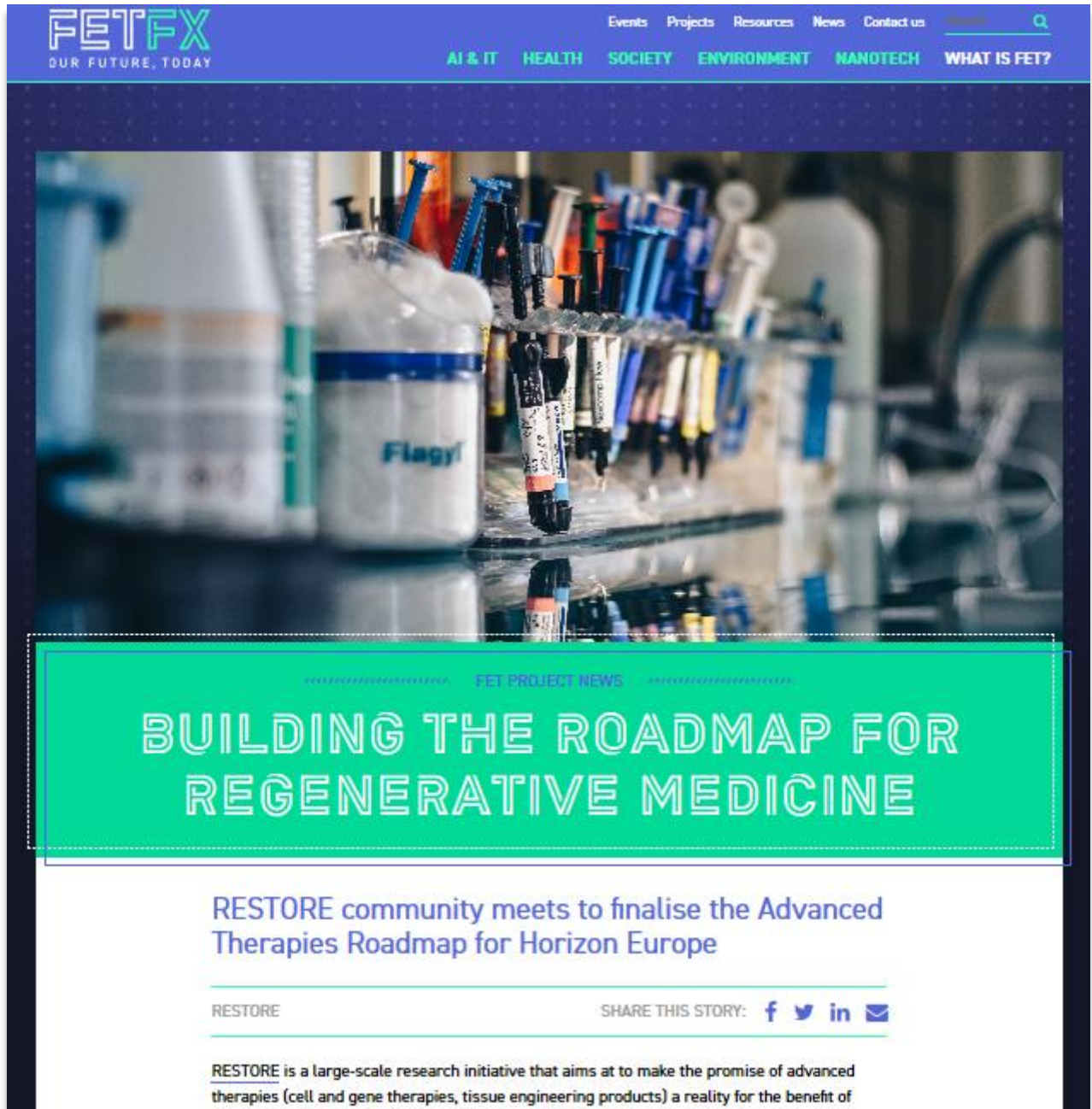
National Transfusion Medicine meeting	Salzburg	13 April 2019	Pluristem	Zami Aberman	Pharmaceutical, Medical	RESTORE in general
ARM Directors meeting	Barcelona	25 April 2019	Pluristem	Zami Aberman	ARM members	RESTORE in general
European Research Area Conference	Berlin	14 May 2019	Charite	C Feest	Policy Makers	RESTORE general aspects, national awareness, institutional support
World Advanced Therapies and Regenerative Medicine Congress 2019	London	16-17 May 2019	Telethon	nnamaria Merico	SME, Industry; Academia, Venture Capitals	RESTORE HEALTH BY ADVANCED THERAPIES – A EUROPEAN LARGE-SCALE RESEARCH INITIATIVE
13th International Symposium on Frontiers in Biomedical Polymers (FBPS 2019)	Tenerife, Spain	19-23 May 2019	Minho	Rui Reis, Nuno Neves	Academia, basic and applied researchers	RESTORE in general
TERMIS European Chapter Meeting 2019 (TERMIS-EU)	Rhodes, Greece	27-31 May 2019	Minho	Rui Reis, Nuno Neves	Academia, Industry	RESTORE in general
BIO	Philadelphia	3-6 June 19	Charite	C Feest	SME and industry	RESTORE general aspects, international awareness, industry/CDMO support
Advanced Cell & Tissue Culture Meeting 2019, University Cardiff, <a href="https://theactc.com/">https://theactc.com/</a>	Cardiff	04 June 2019	TissUse	Uwe Marx	Academia, SMEs	RESTORE in general and the content of the preclinical programme
Young life Scientists Forum 2019, EMBL <a href="https://www.life-science-forum-hd.de/">https://www.life-science-forum-hd.de/</a>	Heidelberg	06 June 2019	TissUse	Uwe Marx	Academia, basic researches	RESTORE in general and the content of the preclinical programme
Frontiers in Silk Sciences and Technologies (SILK 2019)	Trento, Italy	11-15 June 2019	Minho	Rui Reis, Nuno Neves	Academia, basic and applied researchers	RESTORE in general
“Long Night of the Sciences”	Berlin	15 June 2019	Charite	P. Reink,e H.-D. Volk	General Public	RESTORE general aspects,

ISACB&ISVTE	Zurich	19-21 June 2019	UZH	Simon P. Hoerstrup	Academia	
First Achilles Conference	Porto, Portugal	8-10 July 2019	Minho	Rui Reis, Nuno Neves	Academia, basic and applied researchers	RESTORE in general
FoReCaST Second Workshop	Porto, Portugal	11-12 July 2019	Minho	Rui Reis, Nuno Neves	Academia, basic and applied researchers	RESTORE in general
Israel Medical Conference	Jerusalem	13 August 2019	Pluristem	Zami Aberman	Medical, Governmental	RESTORE in general
ESAO	Hannover	01 September 2019	Charité	H.-D. Volk		RESTORE HEALTH BY ADVANCED THERAPIES – A EUROPEAN LARGE-SCALE RESEARCH INITIATIVE
XLVI ESAO 2019 Meeting	Hannover, Germany	3-7 September 2019	Minho	Nuno Neves	SME, Industry; Academia, Venture Capitals	RESTORE in general
2nd International Conference on Biomaterials, Bio-Design and Manufacturing (BDMC 2019)	Tianjin, China	4-6 September 2019	Minho	Rui Reis	Academia, basic and applied researchers	RESTORE in general
Summer Reseach School "Biomaterials and Regenerative Medicine	Ulaan Baatar, Mongolia	10-13 September 2019	Minho	Rui Reis, Nuno Neves	Academia, basic and applied researchers	RESTORE in general
30th Symposium and Annual Meeting of the International Society for Ceramics in Medicine – Bioceramics 30	Nagoya, Japan	26-31 October 2019	Minho	Rui Reis	Academia, basic and applied researchers	RESTORE in general
Italian Infrastructure for Regenerative Medicine	Bologna	30 September 2019	Charite	HD Volk		
DCID & Next Generation Vaccines Days	Bergisch Gladbach, Germany	1 October 2019	Miltenyi	Andreas Dzionek	international researchers in cellular therapies	Presentation of collaboration opportunities with RESTORE
Wyss Zurich Annual Event	Zurich	3 October 2019	UZH	Simon P. Hoerstrup	Academia, basic and applied researchers	

Tissue Engineering & Regenerative Medicine International Society – AP Chapter and 7th Asian Biomaterials Congress - TERMIS-AP + ABMC7 2019 Congress	Brisbane, Australia	13-17 October 2019	Minho	Rui Reis, Nuno Neves	SME, Industry; Academia, Venture Capitals	RESTORE in general
Meet in Italy for Life Sciences 2019	Trieste, Italy	16-18 October 2019	Innovation Acta	Cannella, Dessole	SME, Academia, Industry	RESTORE in general
RegMed Forum	Berlin	22 October 2019	Charité	Reinke, HD Volk,	Patients, patients organizations	Zell- und Gentherapien – ein Dialog zwischen Patienten, Ärzten und Wissenschaftlern
MPNErare	Berlin	25-27 October 2019	Charite	H.D. Volk	Patients, patients organizations	RESTORE in general
Second Achilles Conference	Braga, Portugal	4-5 November, 2019	Minho	Rui Reis, Nuno Neves	Academia, basic and applied researchers	RESTORE in general
GDK Symposium/Biobanken: Ressource für Wissenschaft, Diagnostik und Therapie	Zurich	14-15 November 2019	UZH	Steffen Zeisberger	Academia	
ATMP workshop “Future in Medicine – Innovative Cell and Gene Therapies”	Bruxelles	3 December 2019	Miltenyi	Manal Hadenfeld	European stakeholders in science, health care and politics	Presentation of collaboration opportunities with RESTORE

## Collaboration with FETFX project

FETFX project ([www.fetfx.eu](http://www.fetfx.eu)) is an EC funded project that aims to enhance visibility and impact of FET research, using diverse communication and outreach activities. FETFX acts as a *communication hub* and it supports RESTORE communicating our activities through e-Magazines, events, workshops, newsletter and their social media channel ([Twitter](#), [Youtube](#), [LinkedIn](#)).



The screenshot shows the FETFX website with a blue header. The logo 'FETFX' is on the left, and navigation links 'Events', 'Projects', 'Resources', 'News', and 'Contact us' are on the right. Below the header, a navigation bar lists categories: 'AI & IT', 'HEALTH', 'SOCIETY', 'ENVIRONMENT', 'NANOTECH', and 'WHAT IS FET?'. The main content area features a large image of a laboratory with pipettes and a container labeled 'Flagyl'. Below the image is a green banner with the text 'BUILDING THE ROADMAP FOR REGENERATIVE MEDICINE'. Underneath the banner, the article title 'RESTORE community meets to finalise the Advanced Therapies Roadmap for Horizon Europe' is displayed. At the bottom, there is a 'RESTORE' tag, a 'SHARE THIS STORY:' section with social media icons for Facebook, Twitter, LinkedIn, and Email, and a short paragraph about the RESTORE initiative.

FETFX  
OUR FUTURE, TODAY

Events Projects Resources News Contact us

AI & IT HEALTH SOCIETY ENVIRONMENT NANOTECH WHAT IS FET?

FET PROJECT NEWS

# BUILDING THE ROADMAP FOR REGENERATIVE MEDICINE

## RESTORE community meets to finalise the Advanced Therapies Roadmap for Horizon Europe

RESTORE

SHARE THIS STORY: [f](#) [t](#) [in](#) [✉](#)

RESTORE is a large-scale research initiative that aims at to make the promise of advanced therapies (cell and gene therapies, tissue engineering products) a reality for the benefit of

## Events Conferences and Seminars

- **Major national and international Conferences** – RESTORE partners and supporters have participated and disseminated project activities through oral presentations, posters and discussions
  - i.e. Focis Conference in Boston on 20<sup>th</sup> June 2019, FutureTecEU in Berlin on 5<sup>th</sup> November 2019, Italian Infrastructure for Regenerative Medicine in Bologna on 30<sup>th</sup> September 2019.
- **Public participation to Open Event**
  - i.e. Long Night of the Sciences, 15<sup>th</sup> June 2019 in Berlin.
  - RegMed Forum 2019, patients and patient organizations event, 22<sup>nd</sup> October 2019 in Berlin
- **Organization of Conferences**
  - **Kick off meeting** gathering around 200 participants was held on 6-7<sup>th</sup> May 2019 in Berlin.  
During the event breakout sessions for Working Groups have been organized allowing initial discussion and involvement of supporters in RESTORE activities.
  - **The RegMed Forum** has met annually since 2014 to discuss the progress made and the highlights in Regenerative Medicine. This year the pan-European large-scale research initiative, RESTORE, joined the event as an organiser, thus the focus of the forum was shifted towards a more patient oriented perspective, concentrating on the exchange between patients, physicians and scientists about the developments, risks and opportunities of new cell and gene therapies (Advanced Therapies). Only joint efforts by all of these different groups can ensure the successful development and clinical implementation of this new class of therapies.  
The 2019 RegMed forum, which took place on October 22<sup>nd</sup>, offered an exciting programme where case studies from various indications in the following areas, immune diseases, cancer, musculoskeletal system, cardiovascular system and rare diseases, were presented by physicians, scientists, patient organisations and patients. This provided a broad perspective, including the lived experience of being on the receiving end of Advanced Therapies, and basis for discussion of the developments and risks involved in cell and gene therapy. In the final hour, a panel of experts discussed both with the audience and among themselves, the general themes and patient related aspects of Advanced Therapies, to round off an interesting and informative day.
  - **The RESTORE 1st Advanced Therapies Science Meeting (ATSM)** event is planned on 25-26<sup>th</sup> November 2019 in Berlin and the organization is currently ongoing.
  - Organization of “**RESTORE Momentum Workshop**” is planned on 17 February 2020
- **Seminars and events.** Hosting or attending seminars and scientific events is an efficient method of dissemination, especially to young audiences.
- **Meeting with EU parliament** planned on 5<sup>th</sup> December 2019

## Media

For reaching out to non-scientific people, the RESTORE message are advertised with press releases in appropriate and popular scientific journals and newspapers, and on [Youtube](#).

## Publications

Several publications will be done until the end of the CSA and afterwards and they will be based on the RESTORE roadmaps and working groups activities results.

## Newsletters

A **periodic newsletter** has been distributed to provide a means of reporting unfolding developments during the course of RESTORE. Considering the type of content, the newsletter is circulated within the RESTORE Community to inform supporters about events, activities and about any relevant updates.

Typical stories that are included are: suitably edited versions of project press releases; announcements of progress/milestones by single beneficiaries or workgroup collaborators; reports on conferences and meetings; news of publications; personnel announcements; information about forthcoming events.

The first 2 issues of Newsletter have been distributed to RESTORE Community and the PDF are enclosed to the current deliverable in Annex 1- Published Material.

## Position papers

The RESTORE Roadmap “Pursuing FET in Horizon Europe: Strategic Planning on “Living Drugs” by RESTORE” has been prepared and is public via the website. It will be distributed in occasion of the meeting at the EU parliament on 5th December 2019 in Bruxelles. A copy is enclosed to the current deliverable in Annex 1-Published Material.

## Survey

RESTORE prepares a survey addressing EU citizen with the aim to investigate the grade of knowledge, awareness and interest in the Advanced Therapies among general public. The survey will kick-off in November and results are expected at the beginning of 2020

## Requirements of dissemination and Communication material

Any dissemination of results (in any form, including electronic)

- i. display the EU emblem, or
- ii. include the acknowledgements of the EC for H2020 funding
- iii. almost all data generated during this phase will be reliable for gold/green level open access (dependent on the journal where the position papers will be published)

## Annex 1- Published Material



## RESTORE 1st Advanced Therapies Science Meeting

25th-26th November 2019, Berlin

RESTORE-Health by Advanced Therapies invites you to join the **First Advanced Therapies Science Meeting (ATSM)**, an EACCME<sup>®</sup> accredited event, which will be held at the Maritim Hotel, Stauffenbergstraße in Berlin from the 25<sup>th</sup> - 26<sup>th</sup> November 2019.

Advanced Therapies are one of the most recent and significant advances in medicine. They include gene and cell therapies and tissue-engineering approaches to not only treat disease symptoms, but to potentially provide a cure for otherwise incurable chronic diseases. **RESTORE** aims to ensure that these regenerative therapies are implemented as a standard of care across Europe and beyond. The **1<sup>st</sup> ATSM** will bring together experts from industry, patient organisations and academia to discuss the challenges within the field of Advanced Therapies and to drive forward a concerted interdisciplinary effort, making use of the excellent science, infrastructure and funding landscape within Europe, to make regenerative therapies a reality for all.

**RESTORE** is coordinated by Prof. Dr. Hans-Dieter Volk, Spokesperson for the BIH Center for Regenerative Therapies (BCRT) and his team, "Advanced Therapies are a potential game changer in health care, aiming to shift our focus from chronic treatment of disease to regeneration of health.", says Prof. Volk. He adds, "We are determined to translate promising research findings into safe therapies, and we are working across disciplines and national borders in order to achieve this goal. The **1<sup>st</sup> Advanced Therapies Science Meeting** provides the opportunity for participation in discussions on the still numerous obstacles in the way of implementing these promising therapies in routine clinical care."

Advanced Therapies, also known as "**living** drugs", are a disruptive innovation, i.e. an innovation that will change the paradigm of clinical drug development in the future. For some patients these regenerative therapies are already a reality, unfortunately for most patients this is not yet the case. The motivation behind **RESTORE** is to accelerate the availability of these "**living**" drugs to **all** those in need by supporting collaboration throughout the transdisciplinary network required during drug development and by helping to overcoming regulatory, scientific and technological roadblocks that stand in the way of such a trailblazing change.

"We are immensely grateful to all our supporters and to the EU for backing **RESTORE** and the **1<sup>st</sup> ATSM**, allowing us to share and evolve our vision for the future of Advanced Therapies." says Prof Volk. **RESTORE** is a Europe-wide large-scale research initiative, backed by an international and transdisciplinary community comprised of industrial partners, research institutions and hospitals.

The packed two-day **1<sup>st</sup> ATSM programme** will include talks from Nobel Prize winner **Ada Yonath** (Director of Weizmann Institute of Science, Israel), **Michele De Luca** (University of Modena, Italy), **Timothy O'Brien** (National University of Ireland, Galway, Ireland), **Maksim Mamonkin** (Baylor College of Medicine, USA), **Manuela Gomes** (University of Minho, Portugal) and many many more!

For more information about **RESTORE** and the **1<sup>st</sup> ATSM**, to view the meeting programme or to register, please visit our website:

<https://www.restore-horizon.eu/1atsm-home/>

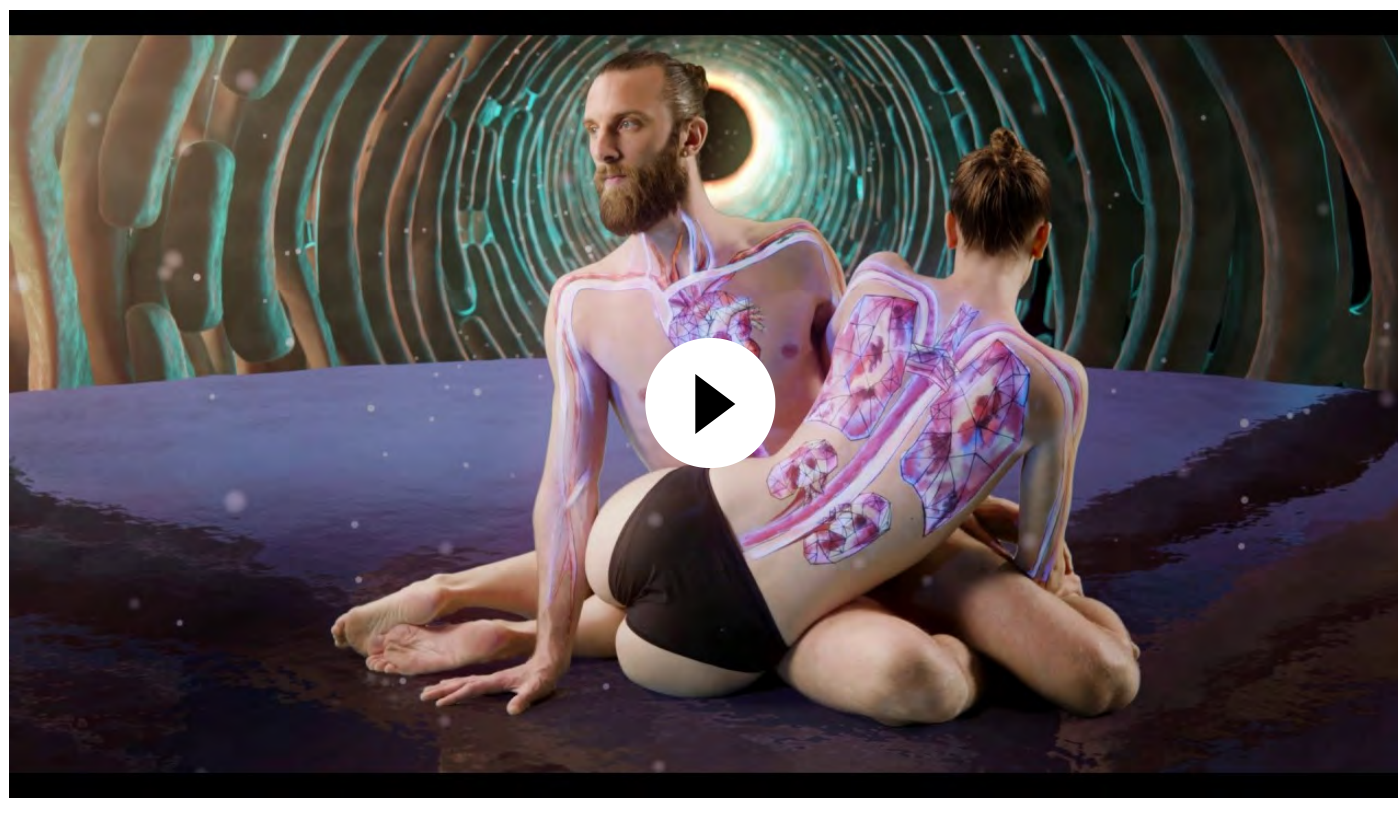
You can also follow us on [Twitter](#), [LinkedIn](#) and [Facebook](#) and tag us **@RESTOREhorizon**

For enquiries, you can contact us at: [info@restore-horizon.eu](mailto:info@restore-horizon.eu) or [contact@b-crt.de](mailto:contact@b-crt.de)

We welcome any journalist who is interested in attending the meeting and ask that you contact us at [contact@b-crt.de](mailto:contact@b-crt.de) to arrange your registration

**RESTORE** partners comprise the Charité Universitätsmedizin Berlin and Berlin Institute of Health (Germany), the University of Zurich (Switzerland), Cell and Gene Therapy Catapult (United Kingdom), TissUse GmbH (Germany), Pluristem Ltd (Israel), Miltenyi Biotec GmbH (Germany), INSERM – Institut National de la Santé et de la Recherche (France), Innovation Acta S.r.l. (Italy), Fondazione Telethon Milan (Italy), and the University of Minho (Portugal).





## Dear RESTORE partners and supporters,

We are delighted to share the first issue of our RESTORE newsletter, which will keep you up to date with all the latest news from the RESTORE community! You will find it filled with information regarding upcoming and previous events, calls for proposals in the field of Advanced Therapies, upcoming deadlines for our Working Groups and much more!

We always welcome your comments and ideas for the Newsletter - don't hesitate to send us a message and please feel free to share the RESTORE Newsletter with interested colleagues!

Sincerely,  
Mareen and Gady on behalf of the coordinator

## REVIEW



### RESTORE Kick-Off Event

May 6th-7th 2019, Mercure Hotel MOA, Stephanstraße 41, 10559 Berlin, Germany

RESTORE's preparatory phase was officially launched at the Kick-Off Event by discussions, presentations and networking between 200 representatives of supporters and partners. By further consolidating the specific Working Groups, we took first steps of formulating "The Advanced Therapies Roadmap for Horizon Europe". This strategy will include clear deliverables over the medium-term in pursuit of an ambitious long-term vision for 2030: A range of Advanced Therapy products on the market, having an impact in clinical routine, providing transformative patient benefit and being economically sustainable.



### Federation of Clinical Immunology Societies Annual Meeting

June 18th-21st, Boston (USA)

The Annual Meeting of the Federation of Clinical Immunology Societies (FOCIS)

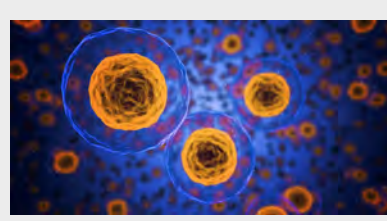
<http://www.focisnet.org/meetings/focis-2019/> is the definitive global meeting in translational immunology and brings together leading researchers and clinicians to present and discuss the latest breakthroughs in immune-mediated diseases. RESTORE Health by Advanced Therapies hosted a lunch session on 20 June 2019, where RESTORE partners presented the backbone of the Advanced Therapies Roadmap and discussed specific points like the preclinical evaluation of advanced therapies, innovative technologies for GMP, gene therapy of primary immunodeficiencies and next-generation regulatory T cell therapy.

## DEADLINES



### Working Groups

The leads and co-leads of the working groups are currently revising the Working Group Outlines, discussing open points and re-opening the revised drafts for further comments by supporters; see the [RESTORE website](#) for more detail. The drafts will be closed on July 3rd for final revision by leads and co-leads.



### Calls Innovative Medicines Initiative (IMI)

The two IMI2 calls „Supporting the development of engineered T cells“ and “Accelerating research & innovation for advanced therapy medicinal products” are open. Please find more information [here](#). RESTORE Partners and supporters are preparing to take part in both calls.

## EVENTS

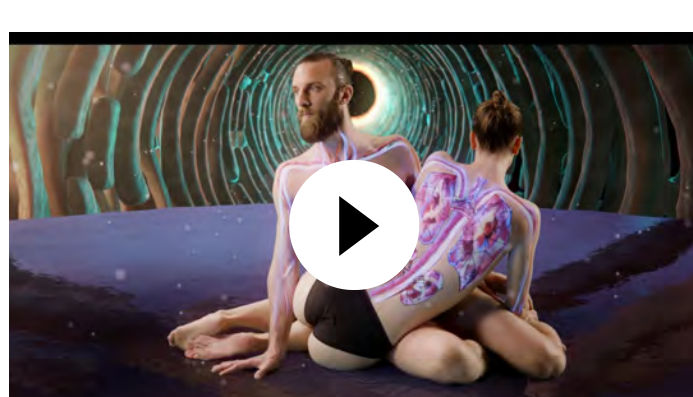


The 1st Advanced Therapies Science Meeting (<https://www.restore-horizon.eu/1atsm-home/#>) will be held in autumn this year.

What are the research challenges that will smash the current roadblocks and allow the development of curative treatments leading to the wider adoption of Advanced Therapies in clinical routine? Join the conversation on Foundational Research discussing new targets and indications, Preclinical Models and Technologies with a focus on human-on-a-chip, Manufacturing Technologies, Clinical Implementation, Regulatory Science and Clinical Trials. Selected Keynote Speakers include Tal Dvir (Tel Aviv University, Tissue Engineering and RegMed, Israel), Antonella Mott (University of Trento, Italy), Clinoa Rooney (Pediatrics-Hem-Onc Cell & Gene, Baylor College of Medicine, Houston, TX, USA), Eric Shifru (University of California, San Francisco, CA, USA) and Ada E. Yonath (Weizmann Institute of Science, Israel).

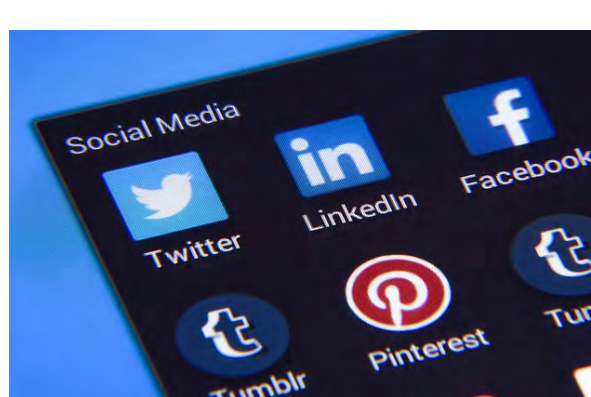
You are warmly invited to submit your abstract by 15 July 2019 [right here](#), meeting registration will open mid-July 2019.

## NEWS



### RESTORE Video

A first video about Advanced Therapies and RESTORE is now publicly available; it illustrates the achievements and potential of Advanced Therapy Medicinal Products for patients with severe chronic diseases in comparison to conventional drugs and also the transformational vision of RESTORE to cure diseases rather than just treating symptoms.



### Raising awareness

Raising awareness for Advanced Therapies is a key objective of RESTORE, we all are working hard to put and keep the topic on the agenda of policy makers and the public at large. Please do follow RESTORE on [Twitter](#) and [Facebook](#) for updates (and much more) and feel free to share.



### Patient information

We are currently preparing an information section on the RESTORE website for patients, families and patient advocacy groups. This section will include information about Advanced Therapies, RESTORE, a contact form and weblinks. We kindly ask for your support in providing relevant information with regard to national registries for clinical trials and patient organisations (Oncology and Hematology, Cardiovascular Diseases, Diseases of the central nervous system, Musculoskeletal Diseases, Endocrine, Metabolic and Genetic Disorders, Ophthalmology, Dermatology, Infectious Diseases, Gastroenterology, Genitourinary Disorders, Respiratory Diseases) in your region and country so we can support patients in their specific search for information and assistance. Please contact us [info@restore-horizon.eu](mailto:info@restore-horizon.eu).









# RESTORE

## Dear RESTORE partners and supporters,

Welcome to the second issue of the RESTORE newsletter. You will find information on the latest events, both past and upcoming, as well as news and calls for proposals. We hope you enjoy it, do not hesitate to send us a message and please feel free to share the RESTORE newsletter with interested colleagues!

You can unsubscribe by clicking the link at the bottom of the newsletter

Sincerely,  
Nicola, on behalf of the coordinator

[info@restore-horizon.eu](mailto:info@restore-horizon.eu)

## REVIEW



### RESTORE F2F

September 16th 2019, Catapult HQ, Guy's Hospital, London, UK

Members of the RESTORE core team met in London to discuss the progress, updates and future of RESTORE, including the Advanced Therapies Roadmap 2030.



### Phacilitate: Leaders Europe Event

17th -18th September 2019, London, UK

Members of RESTORE, including Zami Aberman, Petra Reinke and Hans-Dieter Volk, attended the Phacilitate: Leaders Europe Event, which brings together the entire ATMP ecosystem to promote opportunities provided by the EU market. RESTORE partners participated in round table and panel discussions.

### Italian Infrastructure for Regenerative Medicine (IRMI)

30th September 2019, Bologna, Italy

Prof. Hans Dieter Volk presented RESTORE during the final event of the national IRMI project, an Italian initiative, which aimed to create the infrastructure to facilitate exchange of knowledge between different disciplines. This kind of infrastructure is crucial for the advancement of regenerative therapies and medicinal products.

## DEADLINES



### Horizon 2020

#### CSA

H2020-SC1-HO-19-2020

Reliable and accessible information on cell and gene-based therapies

This Horizon 2020 coordination and support action (CSA) is open for applications. You can find more information [here](#). The deadline for applications is 7th April 2020.



### Future IMI topics

Calls planned for early 2020

To allow maximum time for applicants to form consortia and prepare proposals, the IMI regularly publishes information on potential future topics in advance of the official call launch.

The following relevant topics are under consideration:

**Facilitating the translation of advanced therapies to patients in Europe**

- Optimising patient access to new therapies for rare diseases.

**Big data, digital health, clinical trials and regulatory research**

- Data lakes

**Oncology**

- Prospective real-world clinical implementation of liquid biopsies
- Tumour plasticity

More details on these proposed future topics of interest can be found [here](#)

## 46th ESAO Congress: Smartificial devices for our future



September 3rd-7th 2019, Hannover, Germany

Prof. Hans Dieter Volk presented RESTORE during the tissue-engineering symposium at the annual European Society for Artificial Organs (ESAO) congress ([www.esao2019.org](http://www.esao2019.org)). This congress provides a unique opportunity to foster interdisciplinary collaborations in engineering, biological, medical and physical sciences, with the aim of promoting advances in science and technology for human health.

## EVENTS



The 1st ATSM is taking place from November 25th-26th 2019, at the Maritim Hotel, Stauffenbergstraße 26, 10785 Berlin, Germany.

If you want to take part in discussions highlighting the challenges facing Advanced Therapies from basic research and preclinical models to manufacturing processes, ethics, economics, regulatory issues and clinical implementation then [register now!](#)

During a packed two-day [programme](#), participants from science, industry and patient organisations will gather to consider the developments in Advanced Therapy research, the state of the art and future challenges for implementing Advanced Therapies as a standard of care in clinical settings. Sessions will range from foundational research, new targets and indications and preclinical models and technologies (especially organ-on-chip) to manufacturing technologies, clinical implementation, regulatory science and clinical trials, as well as big data and the ethical issues raised by emerging technologies and innovation. Selected keynote speakers include Michele De Luca (University of Modena, Italy), Tal Dvir (Tel Aviv University, Tissue Engineering and RegMed, Israel), Manuela Gomes (University of Minho, Portugal), Antonella Motta (University of Trento, Italy) Ada Yonath (Weizmann Institute of Science, Israel) and many, many more!

For the full programme and details of the keynote speakers visit our website: <https://www.restore-horizon.eu/1atsm-home/>

Registration closes on the 31st October 2019. Do not miss this opportunity take part in developing a new integrative, innovative and curative pan-European ecosystem for the future. Register [here!](#)

The 1st ATSM has been accredited by the Ärztekammer Berlin. German physicians can obtain up to 12 CME over 2 days.

An application has been made for EACCME® accreditation of this meeting.

## Italian Infrastructure for Regenerative Medicine (IRMI)

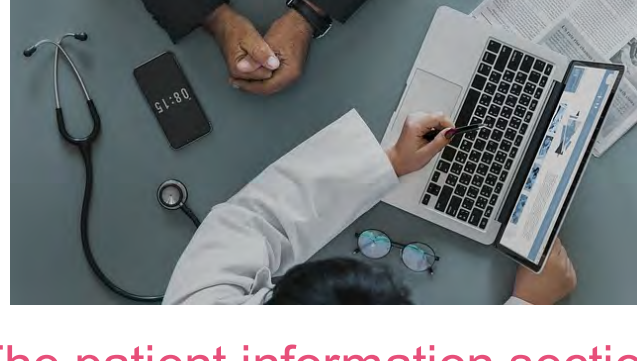


October 22nd 2019, Berlin

The annual RegMed forum (<https://regmed-forum-2019-zell.b2match.io/home>) will be held this year on October 22nd in Berlin. The RegMed forum meets yearly to discuss the most recent developments in regenerative medicine. This year the focus on the exchanges between patients, physicians and scientists on the development and risks of, as well as the opportunities afforded by, novel cell and gene therapies. Such topics will be discussed in the areas of rare diseases, immunotherapy cancer, immune diseases, tissue engineering and endogenous regeneration. Speakers include Petra Reinke (BeCAT, Berlin), Jörn-Sven Kühl (University Hospital Leipzig), Sybille Landwehr-Kenzel (Charité, Berlin), Nathalie Maureen Otto (Charité, Berlin), Boris Schmitt (German Heart Center, Berlin), Joachim Sproß (National Society of Muscular Diseases, Freiburg), Tobias Winkler (BCRT, Berlin) and Ditta Zobor (University Hospital Tübingen)

The meeting is jointly organised by Cluster Health Capital, RESTORE, the BIH Center for Regenerative Therapies (BCRT) and the Berlin Centre for Advanced Therapies (BeCAT). You can register [here](#) until October 21st 2019. The conference language is German and participation is free.

## NEWS



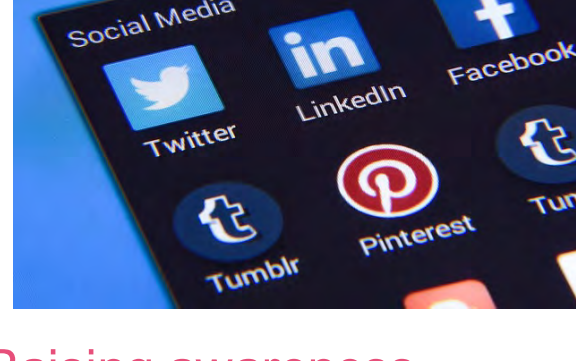
### The patient information section on the RESTORE website is now available!

This section provides information for patients, families and patient advocacy groups about RESTORE and Advanced Therapies. It contains various web links to other useful websites related to Advanced Therapies and a contact form for those wishing to contact RESTORE directly. Click [here](#) to visit the patient area.



### EBISC survey

European Bank for induced pluripotent Stem Cells (EBISC) wants your opinion! This IMI supported project provides a non-for-profit human induced pluripotent stem cell (iPSC) bank for use by researchers across academia and industry. They are running a survey until the end of October 2019 to ensure they continue to offer relevant products and services to the scientific community. If you would like to take part click [here](#)



### Raising awareness

Please continue to raise awareness about Advanced Therapies. This is a key RESTORE objective. We are working hard to maintain an awareness of Advanced Therapies in the public sphere, as well as on the radar of policy makers. Please follow RESTORE on [Twitter](#) and [Facebook](#), as well as via [LinkedIn](#), for updates and information. Tag us @RESTOREhorizon and feel free to share!



## 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 making Europe a spearhead of Advanced Therapies in Science, Clinics and Biomedical Industry - that is the aspiration of the international project RESTORE, coordinated by the Charité Berlin & Berlin Institute of Health.

The initiative recently secured the support of the European Commission and receives initial funding totaling €1m for the 1-year preparatory phase of the large-scale research initiative. Having started officially on 1 March 2019, the kick off meeting of the preparatory phase will be held in Berlin on 6 and 7 May 2019.

## WHY do we need RESTORE?



The increasing prevalence of chronic diseases and multi-morbidity due to demographic factors, health care, and life conditions 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. This results in adverse effects, limited quality-of-life, and increasing costs for society. There is a high need to reach

sustainable improvement or even to cure of chronic diseases - in other words, to disrupt the paradigm of "treating symptoms" with "restoring health". Advanced Therapies are game changers that open up ground-breaking therapeutic Opportunities and can transform the life of patients.

## What are Advanced Therapies?

Advanced Therapies, in Europe categorized as Advanced Therapy Medicinal Products (ATMP), are also often referred to as 'living drugs', and include somatic cell therapy products, ex vivo and in vivo gene therapy products and tissue engineered products, as well as the combination of these products with medical devices, such as biomaterials. (Fig. 1).

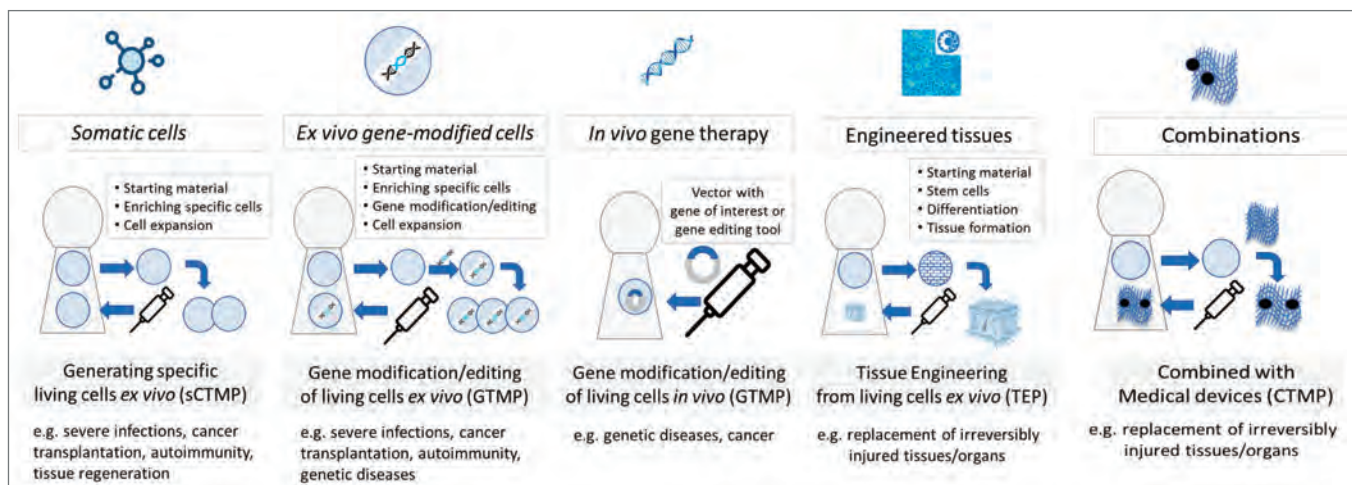


Fig. 1  
Categories of  
Advanced  
Therapies

They are often produced specifically for individual patients (personalised therapies) but if feasible also as off-the-shelf products (one donor - 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.

There are potential cures for genetic diseases, immune diseases, cancer, tissue injury by Advanced Therapies – it is not fiction but reality. Some products are already on the market, mostly for rare diseases (Fig. 2). This means that only a few thousand patients worldwide have benefitted from Advanced Therapies until today.

**YESCARTA®**  
(axicabtagene ciloleucel) Suspension for IV infusion

**KYMRIAH®**  
(tisagenlecleucel) Suspension for IV infusion

**IMLYGIC™**  
(talimogene laherparepvec)

**ALFISEL**

**ZALMOXIS**

**Strimvelis**

**spherox**

**LUXTURNA**

**HOLOCLAR®**

Fig. 2  
Advanced Therapy Products approved in Europe for treatment of cancer, genetic diseases, immune diseases, tissue injury.

## Why are only 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. 3).

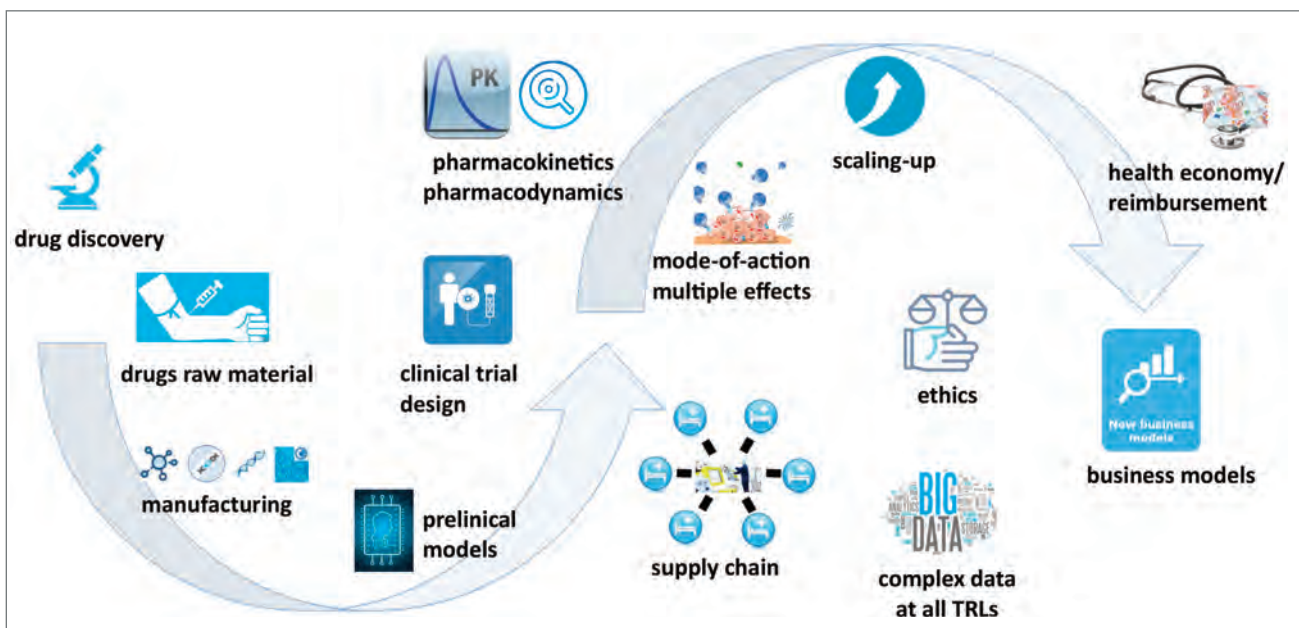


Fig. 3  
„Living“ Drugs - a disruptive innovation shatters current paradigms but generates new high-value developments

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, development and clinical benefit 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 US and Asia. Europe lags behind despite its high innovation potential and demonstrable success in the past. Therefore, there is a need for 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 diseases

- Make the disruptive promise of Advanced Therapies a reality to **cure** chronic diseases
- 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 revolutionary AT products** “developed and made in Europe” by 2030
- Establish a **sustainable pan-European ecosystem** integrating research, clinics, industry and patients

The overarching goal of Advanced Therapies/Regenerative Medicine is to restore the function of impaired cells, tissues, or organs by addressing two basic principles that depend on the kind of injury and tissue involved:

- Stimulating endogenous regeneration and tissue repair mechanisms (angiogenesis, stem cell activation, immune balance, immune restitution) for combating many chronic diseases, such as cancer, tissue injury, immune, cardiovascular, musculoskeletal, and neurological diseases etc.
- Providing biological replacement strategies if the targeted cells/tissues/organs are irreversibly injured or do not possess regenerative capacity. Biological replacement strategy approaches require also additional support of engraftment and sustained function of those products after delivery to the patient for combating genetic diseases and end-stage tissue/organ injury.

## HOW to get there?

- Establish a well-funded consortium with long-term perspective that is backed-up by the transdisciplinary scientific community, relevant stakeholders, patient advocates and the public and that is embedded into international networks (Fig.4)
- Devise the strategic roadmap 2021-30 to overcome roadblocks in order to make our vision come true
- Accelerate and de-risk the development of Advanced Therapies by implementing the Research & Technology Innovation Platforms and Refined Translation Tool (Fig 4+5)
- Form the RESTORE consortium as the docking point for biotech/medtech/pharma industry and special interest venture funds
- Implement a governance that allows flexibility
  - to respond to new trends and to achieve defined key performance indicators
  - to engage at any time more scientists and further stakeholders who can contribute significantly to the success of Advanced Therapies



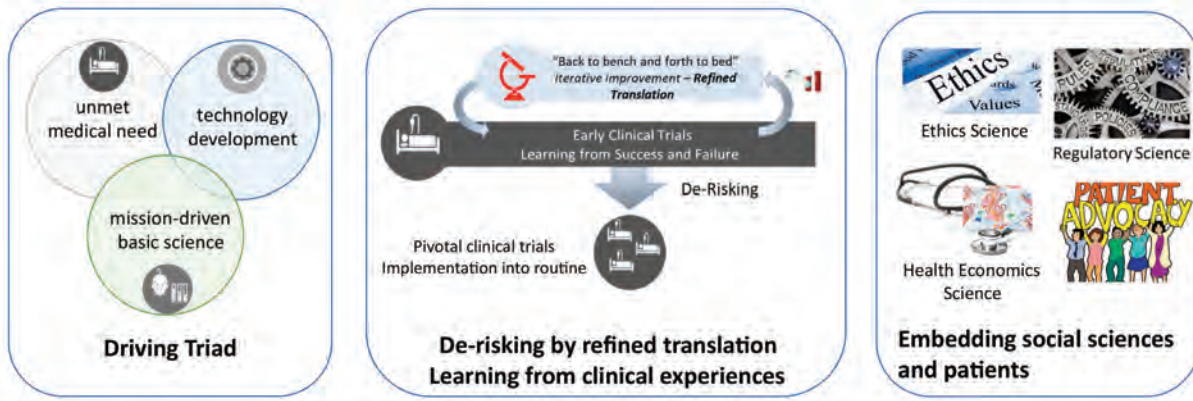


Fig. 4  
Building on the 3T's:  
T Transdisciplinarity,  
T Technology,  
T Translation

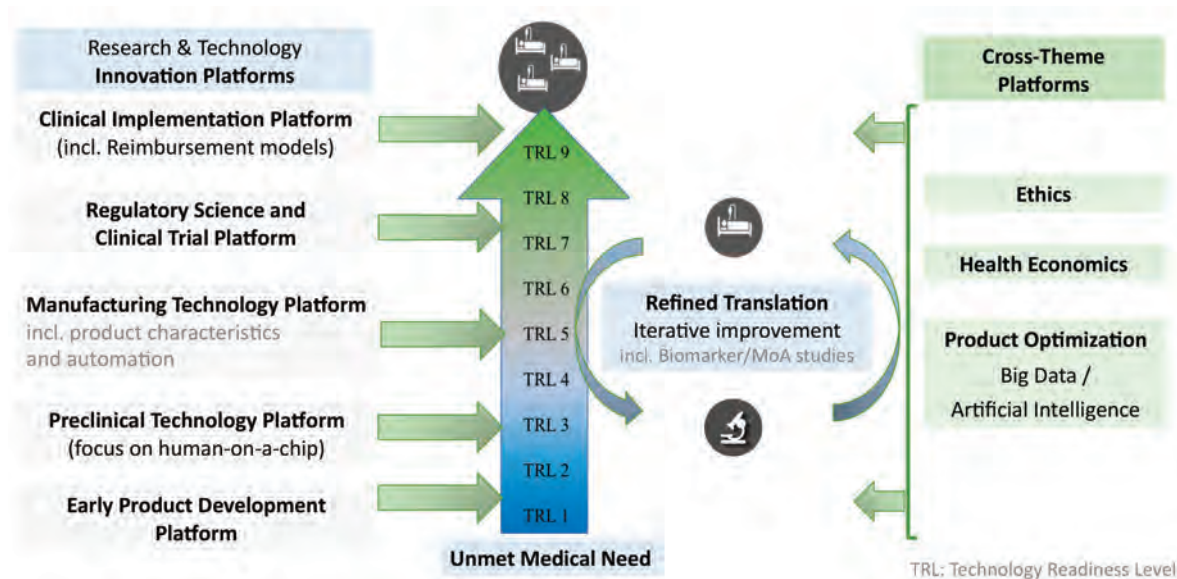


Fig. 5  
Accelerating and  
De-Risking by  
Research & Technology  
Innovation Platforms

One solution  
⇒ Many applications

## IMPACT of RESTORE

### by Disruptive Innovations and Technologies on:



#### Science and Technology

- ⇒ infrastructure and **translational centers of excellence**
- ⇒ boosting Europe's competitiveness in the groundbreaking area of **"living" drugs**
- ⇒ fundamental knowledge and technology **breakthroughs** relevant for many areas
- ⇒ next generation of **translational scientists** with new mindset



#### Societal Benefits for European's Society and Economy

- ⇒ improved survival and quality-of-life for patients with **chronic diseases**
- ⇒ decreasing financial burden of the society by **chronic diseases**
- ⇒ innovative **product pipeline** for medtech/biotech/pharma industry and new players
- ⇒ generation and retention of highly **specialized workforces** and expertise in Europe
- ⇒ setting **ethical and quality standards** for these new therapies

Fig. 6  
Scientific and societal  
Impact of RESTORE

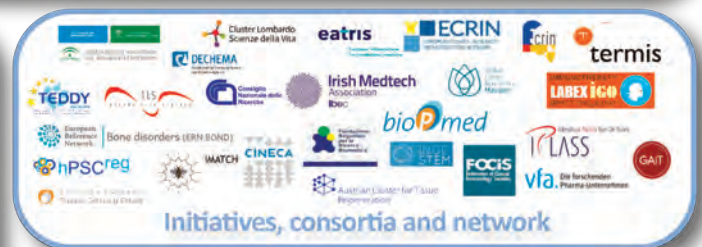


## WHO WE ARE - RESTORE COMMUNITY

The RESTORE Core Team is a formidable groups of 10 partners from academic centers and biotechs with excellent international reputation and a strong track record (Fig. 7)

Fig. 7  
RESTORE Core Team from Academia and Biotech

The Core Team and close to 300 committed supporters (Fig. 8) from 26 countries (EU-MS, EU-AC, USA, Singapore) form the RESTORE community.

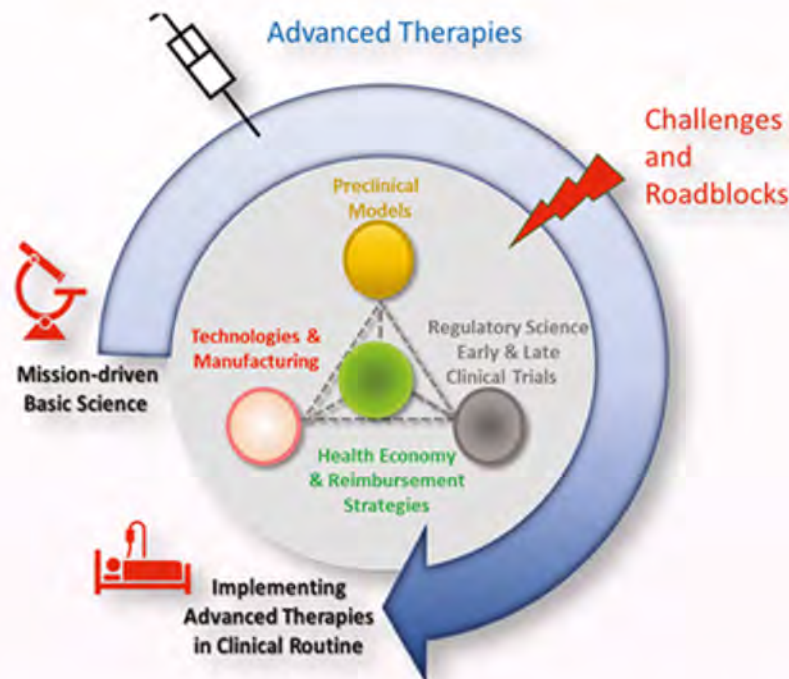


Please visit the **RESTORE** website ([www.restore-horizon.eu](http://www.restore-horizon.eu)) for more details on the activities of the initiative and **JOIN US!**



## RESTORE - Health by Advanced Therapies

(Advanced Therapy Medicinal Products and Biologised Medical Devices)

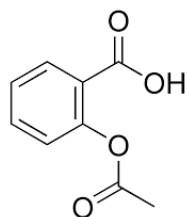


ALL for Advanced Therapies. With Passion. For Patients.



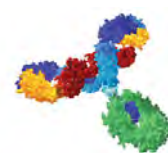
# Current Therapeutic Tools

since >120 years



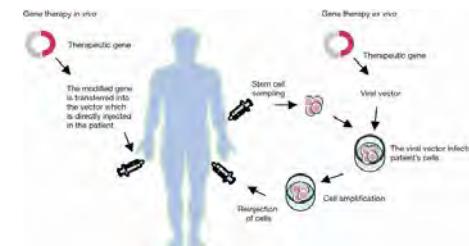
Defined, chemically produced  
**small molecules**

since >30 years

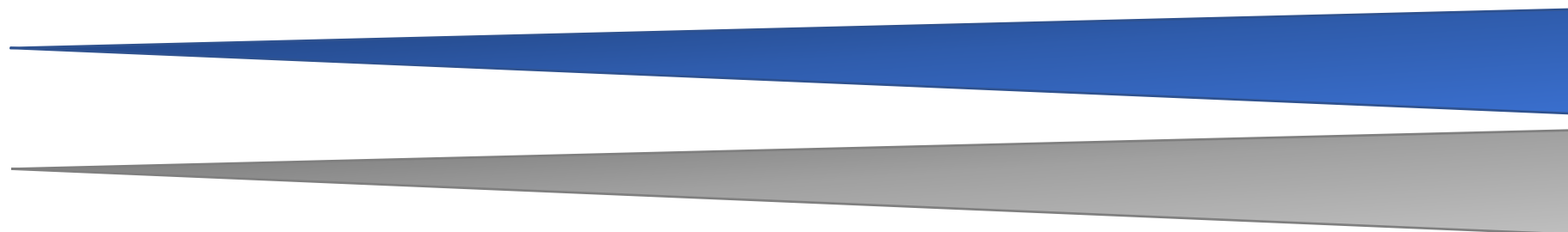


Protein-based drugs  
produced in living cells  
**Biologics**

since >10 years



“Living” medications  
(living cells, tissue, gene therapies)  
**Advanced Therapies**



chance to **cure**  
(game changer)

complexity

# The Vision

- Making the transforming promise of Advanced Therapies a reality for patients' benefit
- Making Europe the spearhead of Advanced Therapies R&D and application

# The Mission

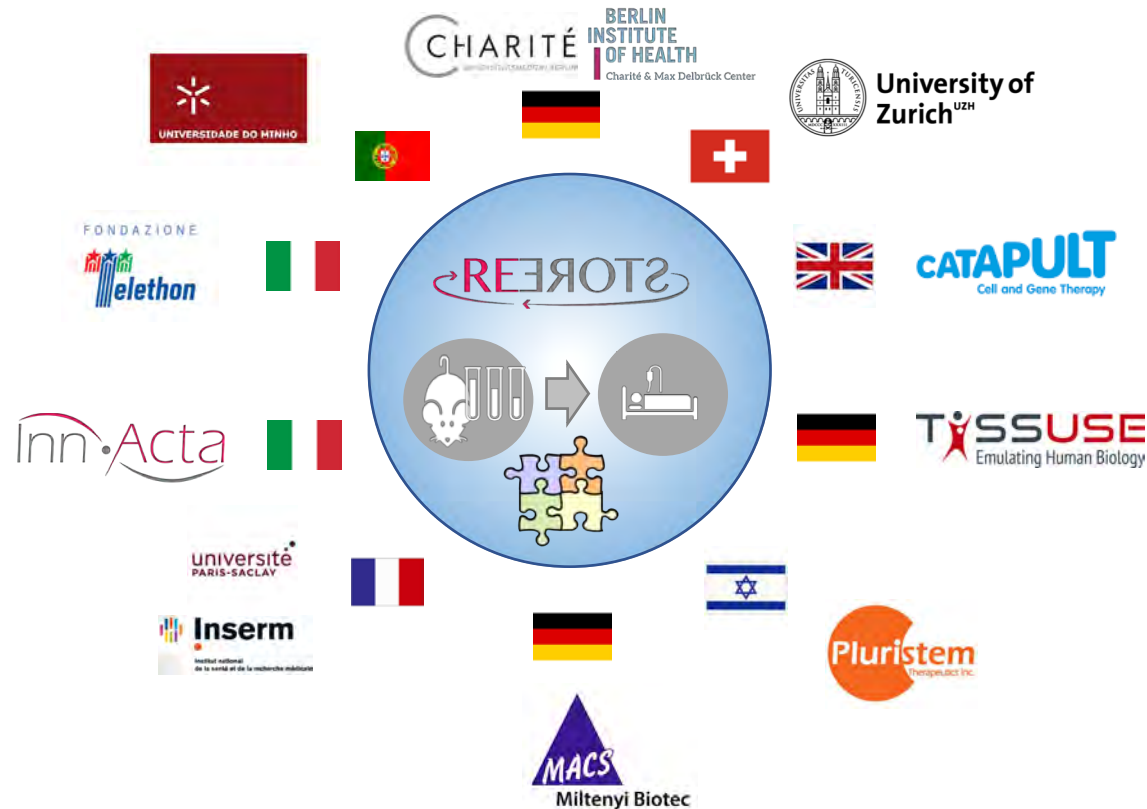
- Creating a pipeline of dozens of Advanced Therapy products developed and made in Europe by 2030
- Establishing a European ecosystem that will carry the effort further into the future

# How to **get** there

- Establishing a well-funded consortium, which is backed-up by relevant stakeholders, the scientific community and the public
- Devising a strategic roadmap to allocate funds in order to make our vision come true

# Who **we** are – RESTORE community

## Core Team



## Supporters



RESTORE - Health by Advanced Therapies





# Where are we now?

- After a competitive procedure the European Commission (EC) selected **RESTORE** as a candidate for the new „Large-Scale Research Initiatives“ European program
- We received €1 Million and one year time to prepare a detailed proposal how to best use up to €1 Billion over ten years to reach our goals

# **What** can you do?

**Reach out to us**  
**Engage with our joint activities**  
**Help Europe get there**

# The Pain

- Society became accustomed to being treated for chronic diseases but rarely healed by conventional drugs resulting in high burden for patients and society
- Only a few treatments that cure from chronic disease have reached the market
- Progress is starting in the US and Asia – Europe lags behind despite its high innovation potential

# The **S**olution

- Get everyone with a proven track record together
- Let them identify the bottlenecks and make a plan to release it
- Give them the means to do that

# Our Plan

- We are identifying the challenges that get in the way of Advanced Therapies
- We are devising the strategy where to put the money to overcome these challenges
- We will execute the plan, with the European community, between 2021-2030

# Join us!

<https://www.restore-h2020.eu/>

[info@restore-h2020.eu](mailto:info@restore-h2020.eu)

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



RESTORE - Health by Advanced Therapies





### RESTORE Consortium - CURING CHRONIC DISEASES BY "LIVING DRUGS"

#### 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.

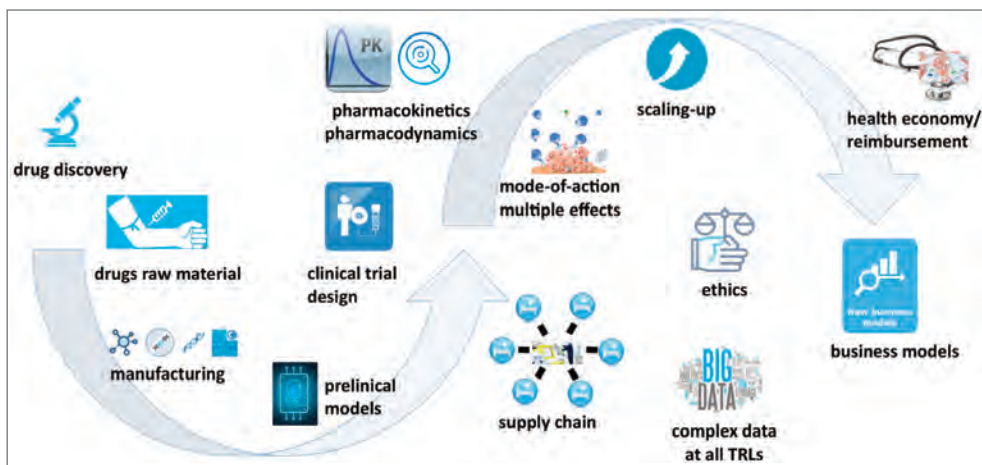
#### What are Advanced Therapies?

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**: **T**ransdisciplinarity, **T**echnology, **T**ranslation (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 **In**novations and **T**echnologies 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!**





## 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, coordinated by the Charité Berlin & Berlin Institute of Health.

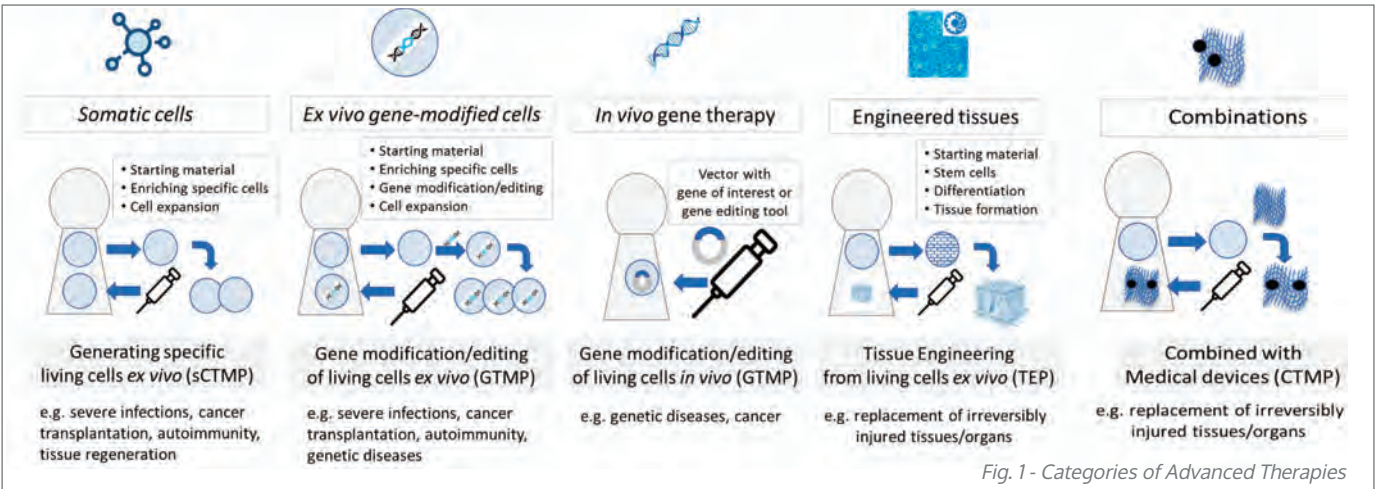
The initiative recently secured the support of the European Commission and receives initial funding totaling €1m for the 1-year preparatory phase of the large-scale research initiative. Having started officially on 1 March 2019, the kick off meeting of the preparatory phase takes place in Berlin on 6 and 7 May 2019.

## WHY do we need RESTORE?

The increasing prevalence of chronic diseases and multi-morbidity due to demographic factors, health care, and life conditions 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 somatic cell therapy products, ex vivo and in vivo gene therapy products and tissue engineered products, as well as the combination of these products with medical devices, such as biomaterials. (Fig. 1).



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 (Fig. 2) which means only a few thousand patients worldwide have benefitted from Advanced Therapies until now.

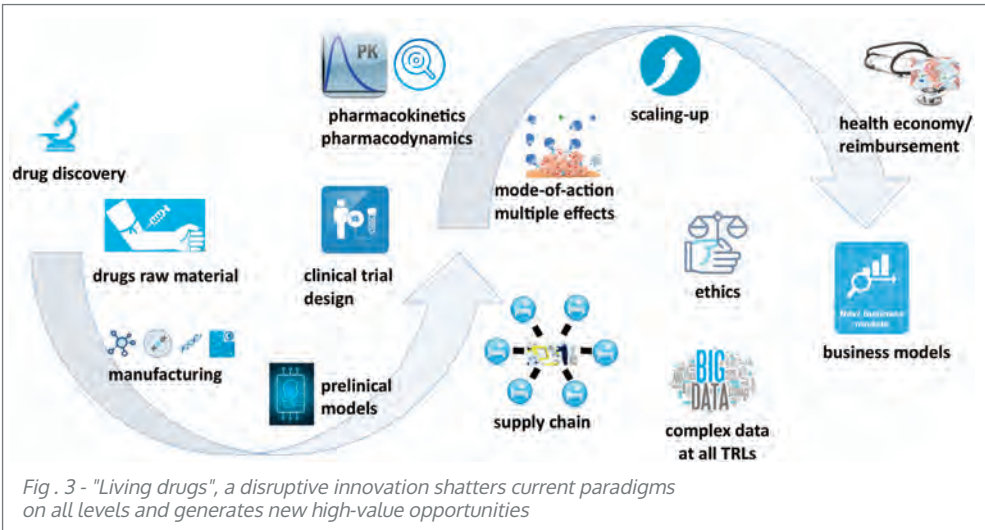


Fig. 2  
Advanced Therapy Products approved in Europe for treatment of cancer, genetic diseases, immune diseases, tissue injury.

## 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. 3).

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

The overarching goal of Advanced Therapies/Regenerative Medicine is to restore the function of impaired cells, tissues, or organs by addressing two basic principles that depend on the kind of injury and tissue involved:

- Stimulating endogenous regeneration and tissue repair mechanisms (angiogenesis, stem cell activation, immune balance, immune restitution) for combating many chronic diseases, such as cancer, tissue injury, immune, cardiovascular, musculoskeletal, and neurological diseases etc.
- Providing biological replacement strategies if the targeted cells/tissues/organs are irreversibly injured or do not possess regenerative capacity. Biological replacement strategy approaches require also additional support of engraftment and sustained function of those products after delivery to the patient for combating genetic diseases and end-stage tissue/organ injury.

## HOW do we 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. (Fig 4+5)
- 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...).

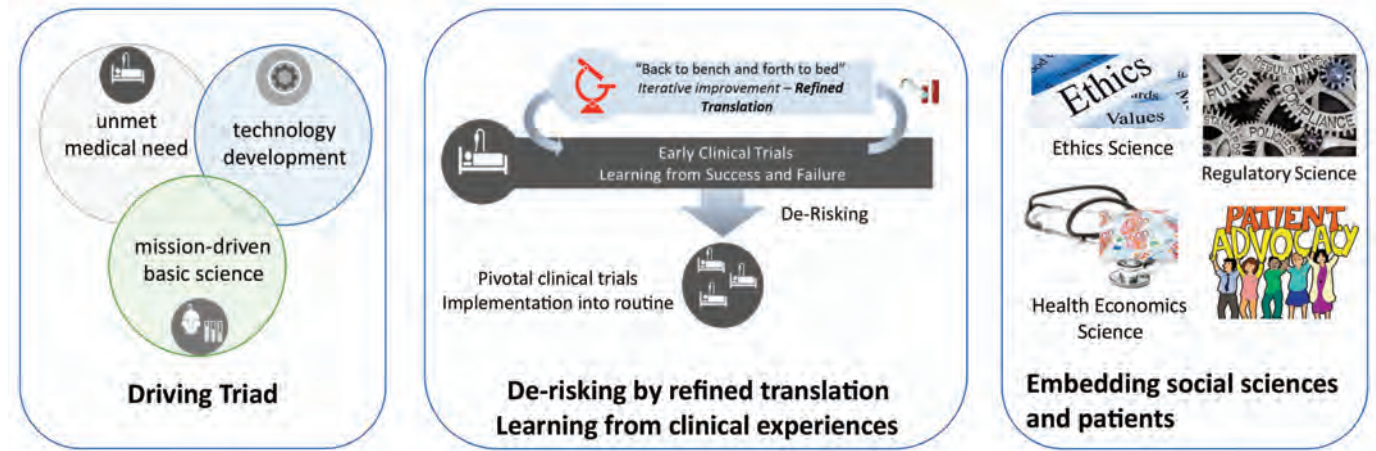


Fig. 4 - Building on the 3T's: **Transdisciplinarity, Technology, Translation**

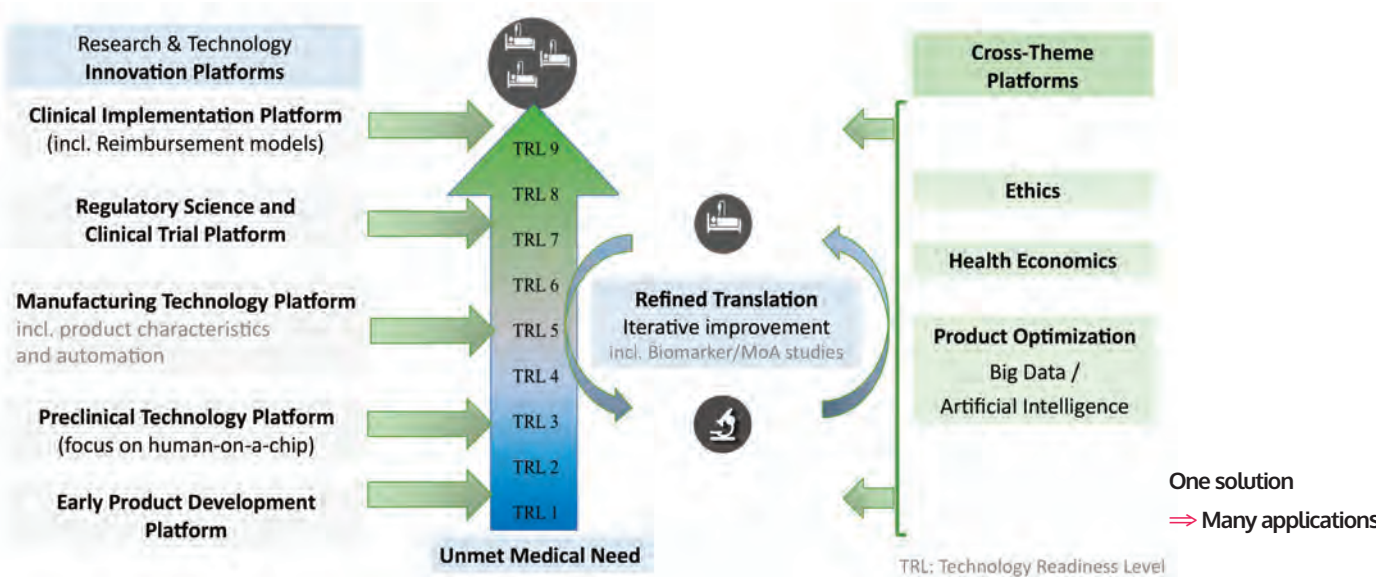


Fig. 5 - Accelerating and De-Risking by Research & Technology Innovation Platforms



## IMPACT of RESTORE

by disruptive **In**novations and **Tech**nologies 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

Fig. 6 - Scientific and societal Impact of RESTORE

## WHO WE ARE - RESTORE CORE TEAM

The RESTORE **Core Team** is a formidable groups of 10 partners from academic centers and biotechs with excellent international reputation and a strong track record (Fig. 7)

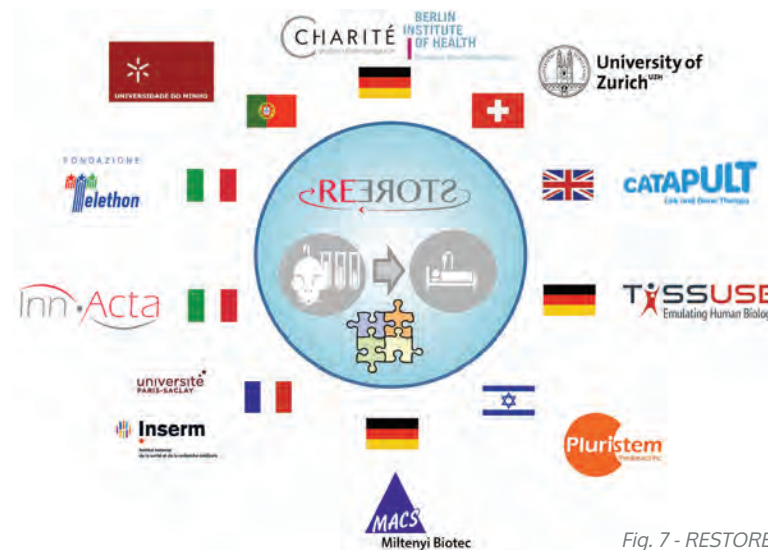


Fig. 7 - RESTORE Core Team from Academia and Biotech



Health by Advanced Therapies

JOIN US!

[www.restore-horizon.eu](http://www.restore-horizon.eu)



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



Health by Advanced Therapies



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## WHO WE ARE - RESTORE COMMUNITY

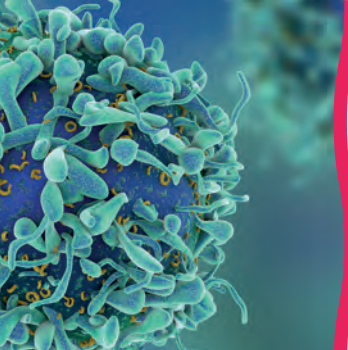
The Core Team and close to 300 committed supporters (Fig. 8) from 26 countries (EU-MS, EU-AC, USA, Singapore) form the RESTORE **community**.

Fig. 8 - RESTORE Community



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





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](mailto:info@restore-horizon.eu)  
[www.restore-horizon.eu](http://www.restore-horizon.eu)

## The Vision of RESTORE

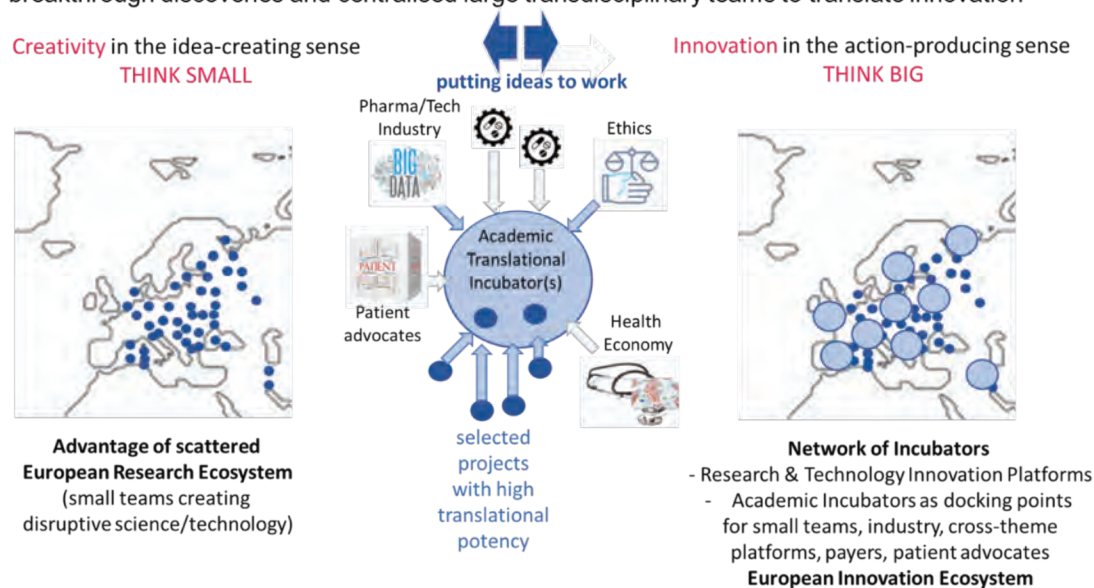
- Deliver a pipeline of revolutionary “living drug” products “**developed and made in Europe**” and transform the disruptive promise of Advanced Therapies into a reality to cure chronic diseases
- Make them **accessible/affordable** as “**standard-of-care**” for every patient in need
- Make **Europe 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)**

## DAY I - Monday, 6<sup>th</sup> May 2019

**09.30-10.30 Registration and coffee**

Atrium | All participants

### Welcome

Main Seminar Room MOA 5 | All participants

**10.30-10.35 Welcome and objectives of the kick-off meeting**

Hans-Dieter Volk, Petra Reinke - Charité - Universitätsmedizin Berlin, Germany

**10.35-10.45 Welcome Address**

Axel Pries - Dean of the Medical Faculty of the Charité and Chairman of the BIH Executive Board (interim)

### Introduction

Main Seminar Room MOA 5 | All participants

**10.45-11.20 RESTORE - Health by Advanced Therapies - Why, what, how, who?**

Hans-Dieter Volk - Charité - Universitätsmedizin Berlin, Germany (20 min presentation + 15 min panel discussion)

**11.20-11.50 Introduction of RESTORE core team**

Charité/BIH, Univ Zurich, Catapult, TissUse, Pluristem, Miltenyi Biotech, Inserm/Univ Paris Saclay, InnActa, Telethon, Univ. Minho  
(3 min/each partner, total 30 min)

**11.50-12.15 Introduction into roadmap and working groups**

Hans-Dieter Volk - Charité - Universitätsmedizin Berlin, Germany (20 min)

### Break

Atrium | All participants

**12.15-13.10 Lunch**

### I. Overarching Elements

Main Seminar Room MOA 5 | All participants

**13.15-13.40 Ethics**

**13.40-14.10 Big Data / Artificial Intelligence to handle product characteristics, biomarker, (pre)clinical outcome**

Lead: Pluristem, Co-Lead: Catapult

**14.10-14.30 Economic aspects: Health technology assessment, reimbursement**

## II. RIA Module "Research Platform Manufacturing" - Working groups 1-3

Main Seminar Room MOA 5 | All participants

### 14.30-15.00 **WG 1: Manufacturing: somatic and gene-modified cells**

Lead: Pluristem, Co-Lead: Miltenyi, Inserm

### 15.00-15.30 **WG 2: Manufacturing: tissue engineering & composite products**

Lead: University Zurich, Co-Lead: University Minho

### 15.30-16.00 **WG 3: Manufacturing: *in vivo* gene therapy and gene editing**

Lead: Telethon, Co-Lead: Charité

### Coffee break

Atrium | All participants

### 16.00-16.20 *Coffee break*

### Break-out sessions (WG 1+2+3 in parallel):

#### 16.25-17.00 Seminar Room MOA 3 | Interested participants **WG 1: Manufacturing: somatic and gene-modified cells (30 min)**

#### 16.25-17.00 Seminar Room MOA 4 | Interested participants **WG 2: Manufacturing: tissue engineering & composite products (30 min)**

#### 16.25-17.00 Seminar Room MOA 1 | Interested participants **WG 3: Manufacturing: *in vivo* gene therapy and editing (30 min)**

## III. RIA Module "Research Platform Manufacturing" - Working groups 4-5

Main Seminar Room MOA 5 | All participants

### 17.00-17.30 **WG 4: Manufacturing: pluripotent stem cells and adult stem cells**

Lead: Inserm, Co-Lead: Telethon

### 17.30-18.00 **WG 5: Manufacturing: *ex vivo* gene delivery / gene editing**

Lead: Catapult, Co-Lead: Miltenyi

### Break-out sessions (WG 4+5 in parallel):

#### 18.10-18.40 Seminar Room MOA 3 | Interested participants **WG 4: Manufacturing: pluripotent stem cells and adult stem cells (30 min)**

#### 18.10-18.40 Seminar Room MOA 4 | Interested participants **WG 5: Manufacturing: *ex vivo* gene delivery / gene editing (30 min)**



# Kick-Off Meeting

06<sup>th</sup>-07<sup>th</sup> May 2019

Mercure Hotel MOA - Stephanstraße 41, 10559 Berlin - Germany

## Sum-up day I

Main Seminar Room MOA 5 | All participants

18.45-19.15

### Summary break-out sessions day I

Leaders of break-out sessions WG 1-5

19.15-19.20

### Closing remarks day I

Hans-Dieter Volk - Charité - Universitätsmedizin Berlin, Germany (5 min)

## Networking get-together

Atrium | All participants

19.20-20.45

*Cheese & Wine*



## DAY II - Tuesday, 7<sup>th</sup> May 2019

**08.00-08.25 Registration day II**

Atrium | All participants

**Welcome**

Main Seminar Room MOA 5 | All participants

**08.25-08.30 Welcome day 2**

Hans-Dieter Volk - Charité - Universitätsmedizin Berlin, Germany (5 min)

**IV. RIA Modules "Preclinical Models" and "New Clinical Applications" - Working groups 6-9**

Main Seminar Room MOA 5 | All participants

**08.30-09.00 WG 6: Pre-clinical model systems: *in vitro* and *in vivo***

Lead: TissUse, Co-Lead: Inserm

**09.00-09.30 WG 7: New clinical applications: support of endogenous regeneration**

Lead: Charité, Co-Lead: Pluristem

**09.30-10.00 WG 8: New clinical applications: tissue and organ replacement**

Lead: Telethon

Co-Lead: University Zurich

**10.00-10.30 WG 9: New clinical applications: cancer**

Lead: Miltenyi, Co-Lead: Inserm

**Coffee break**

Atrium | All participants

**10.30-10.55 Coffee break**

**Break-out sessions (WG 6+7 and 8+9 in parallel):**

Seminar Room MOA 3 | Interested participants

**11.00-11.30 WG 6: Pre-clinical model systems: *in vitro* and *in vivo* (30 min)**

Seminar Room MOA 4 | Interested participants

**11.00-11.30 WG 7: New clinical applications: support of endogenous regeneration (30 min)**

Seminar Room MOA 3 | Interested participants

**11.35-12.05 WG 8: New clinical applications: tissue and organ replacement (30 min)**

Seminar Room MOA 4 | Interested participants

**11.35-12.05 WG 9: New clinical applications: cancer (30 min)**

## V. RIA Modules "Regulatory Science & Health-Technology Assessment", "Early Clinical Trials" and "Late Clinical Trials" - Working groups 10-12

Main Seminar Room MOA 5 | All participants

**12.10-12.40**    **WG 10: Clinical research: regulatory science, refined translation, early HTA & early clinical trials**  
Lead: Charité, Co-Lead: Catapult

**12.40-13.10**    **WG 11: Clinical research: pivotal clinical trials and marketing authorization**  
Lead: Telethon, Co-Lead: Pluristem

**13.10-13.40**    **WG 12: Post-Trial and long-term follow-up, data warehouse and patient registry**  
Lead: Telethon, Co-lead: Pluristem

### Break

Atrium | All participants

**13.40-14.35**    *Lunch*

### Break-out sessions (WG 10+11 in parallel, WG 12):

**14.40-15.10**    Seminar Room MOA 3 | Interested participants  
**WG 10: Clinical research: regulatory science, refined translation, early HTA & early clinical trials (30 min)**

**14.40-15.10**    Seminar Room MOA 4 | Interested participants  
**WG 11: Clinical research: pivotal clinical trials and marketing authorization (30 min)**

**15.15-15.45**    Seminar Room MOA 3 | Interested participants  
**WG 12: Post-Trial and long-term follow-up, data warehouse and patient registry (30 min)**

## VI. RIA Module "Implementation & Exploitation" - Working Groups 13-14

Main Seminar Room MOA 5 | All participants

**15.50-16.20**    **WG 14: Valuation and innovative reimbursement models for new Advanced Therapies**  
Lead: Charité, Co-lead: Catapult

**16.20-16.50**    **WG 13: Implementation of new Advanced Therapies into clinical routine**  
Lead: Miltenyi, Co-lead: Telethon

### Coffee break

Atrium | All participants

**16.50-17.10**    *Coffee break*



## Summary break-out sessions day II

Main Seminar Room MOA 5 | All participants

17.15-18.00

### Summary break-out sessions day 2

Leaders of break-out sessions WGs 6-12 (5 min each, total 35 min)

## Next Steps

Main Seminar Room MOA 5 | All participants

18.00-18.15

### Dissemination during preparatory phase

Charité - Universitätsmedizin Berlin, Germany (15 min)

18.15-18.20

### Science meeting Nov 2019 and patient advocacy group meeting(s)

Hans-Dieter Volk, Petra Reinke - Charité - Universitätsmedizin Berlin, Germany (5 min)

18.20-18.25

### Closing remarks and farewell

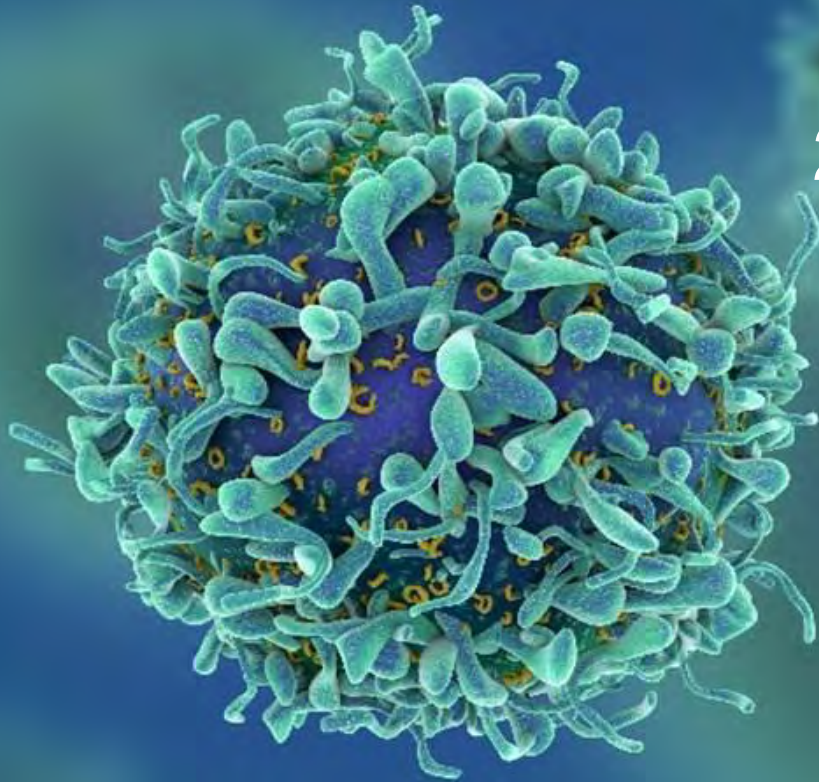
Hans-Dieter Volk - Charité - Universitätsmedizin Berlin, Germany (5 min)

18.30

### End of day II

## End of programme

<https://www.restore-horizon.eu/1at-sm-home/>



25-26 November 2019

Hotel Maritim, Berlin

[Register now!](#)

1st Advanced Therapies Science Meeting



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



Health by Advanced Therapies

# 1<sup>st</sup> Advanced Therapies Science Meeting

25<sup>th</sup>-26<sup>th</sup> November 2019 | Berlin

## EXPLORE

the latest highlights and discuss the most pressing challenges from the field of Advanced Therapies

## ENGAGE

with leaders in research, technology development and clinical application

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### Selected Speakers:

**Ada Yonath** - Director of Weizmann Institute of Science, Israel

**Maksim Mamonkin** - Baylor College of Medicine, USA

**Michele De Luca** - University of Modena, Italy

**Manuela Gomes** - University of Minho, Portugal

**Tal Dvir** - Tel Aviv University, Israel

**Paolo Gasparini** - University of Trieste, IRCCS, Italy

**Timothy O'Brien** - National University of Ireland Galway, Ireland

**Alexandra Marques** - University of Minho, Portugal



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Health by Advanced Therapies

JOIN US!



Ready, steady... go!

Today marks the official start of **RESTORE - Health by Advanced Therapies**, entering the preparatory phase to become a Large-Scale Research Initiative. Advanced Therapies are new treatment modalities (e.g. cell and gene therapy, tissue engineering) that aim to consign the never-ending treatment of chronic ailments to the past and instead offer sustained improvement and even cures. To enable Europe to become a competitive leader in this field, **RESTORE** is defining an Advanced Therapies roadmap 2021-2030 for Europe.

At its core the transformational aspiration of **RESTORE** is addressing pressing healthcare challenges for patients, hospitals and society, spanning disciplines, leveraging sound science and communicating transparently. The European Commission funds **RESTORE** for one year to fully develop the roadmap as the basis for a Large-Scale Research Initiative.

The **RESTORE** core team and its supporters will hold their kick-off meeting on 6/7 May 2019 in Berlin, Germany, where they will be analysing bottlenecks, proposing solutions and ways to implement them. It is a team effort that is carried by **RESTORE**'s many supporters who have given endorsements and are recognising that the timing is propitious, that concerted action is needed and that collaboration is the way forward for success in Europe and beyond.

Let's make the promise of Advanced Therapies become a reality: The future begins today.



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[www.restore-h2020.eu](http://www.restore-h2020.eu)