

ATMPs revolution & the respect of the patients' right to access to care:

Call to Action

Introduction

In the last decades, the developments achieved in molecular and cellular biology and the progress achieved in the study of DNA have radically transformed the biomedical field in addressing the treatment of various genome-based diseases. In particular, in recent **years new genic, cellular, and tissue engineering therapies** (the so-called **ATMPs**, Advanced Therapy Medicinal Products) have emerged, presenting new opportunities for the treatment and prevention of a variety of diseases or for restoring, correcting, or modifying compromised physiological functions in humans, including correcting mutations acquired on a genetic basis.

Their **characteristics** can be briefly summarized as follows, as they:

- **cure** (even otherwise fatal pathologies) **or** significantly **transform** the clinical history of the patient who has no therapeutic alternative, intervening directly on the causes of the disease;
- are **one-shot** therapies, that is, they are administered with a single treatment, unlike traditional drugs and protocols used for other pathologies, which provide for repeated and regular treatments, with an **evident temporal misalignment between current costs**, concentrated in the short term, **and future benefits**, spread over a longer time horizon;
- have **high investment costs**, because these are **personalized therapies with high complexity** (including production complexity), with a price that can exceed 2 million euros per administration, but which **present significant future** benefits in clinical, therapeutic, social and economic terms for the health systems and the health of patients (direct, indirect and social costs avoided, recovery in productivity, higher tax revenues, etc.), and above all, unlike other therapies, even the very expensive ones, ATMPs have a price that is paid only once as they are one-shot therapies;
- are **administered only in qualified and specialized centres** and arise from extremely innovative and complex platforms.

Advanced therapies are becoming not only a hope but a reality of cure for patients suffering from serious diseases who have not yet found a concrete therapeutic answer: by 2030 there could be as many as 60 advanced therapies that could change the life of 350 thousand patients worldwide.

The issue of ATMPs is related at least to the following patients' rights recognized by the European Charter of Patients' Rights¹, a milestone for those who work in advocacy for healthcare at the national and European level: the patients' right to access to care², the patients' right to personalized treatment³, the patients' right to Innovation⁴ and the patients' right to Information⁵.

The costs of this technology are high so that access by patients is often hindered by public health budget limits: how to combine the above-mentioned patients' rights in this new challenging context, characterized by health and humanitarian emergencies (fighting the pandemic and the war in Ukraine above all) with inevitable repercussions also on the financial resources of the countries of the European Union?

Call to Action

Across Europe, a patient who wants to access an ATMPs may face various obstacles of a social nature: lack of scientific knowledge, lack of regulatory uniformity and transparency, regional inequalities of access, and lack of public funds for this type of therapy.

For this reason, the following organizations have decided to launch this Call to Action which is aimed at national and European Institutions first, but also at the whole health ecosystem including civic & patient associations, and citizens.

We call on the European Institutions to:

- Promote a clear, transparent, and adequate regulation regarding ATMPs.
- Reducing health inequalities in access to ATMPs among the Member States also due to

¹ https://ec.europa.eu/health/ph_overview/co_operation/mobility/docs/health_services_co108_en.pdf

² www.activecitizenship.net/charter-of-rights/rights/351-2-right-of-access.html: *Every individual has the right of access to the health services that his or her health needs require. The health services must guarantee equal access to everyone, without discriminating on the basis of financial resources, place of residence, kind of illness, etc.*

³ www.activecitizenship.net/charter-of-rights/rights/341-12-right-to-personalized-treatment.html: *Each individual has the right to diagnostic or therapeutic programmes tailored as much as possible to his or her personal needs.*

⁴ www.activecitizenship.net/charter-of-rights/rights/343-10-right-to-innovation.html: *Each individual has the right of access to innovative procedures, including diagnostic procedures, according to international standards and independently of economic or financial considerations.*

⁵ www.activecitizenship.net/charter-of-rights/rights/350-3-right-to-information.html: *Every individual has the right to access to all kind of information regarding their state of health, the health services and how to use them, and all that scientific research and technological innovation makes available.*

financial constraints.

- Promote new health and economic approach: the change in the accounting system to consider the expenses for ATMPs as investments instead of current expenditures. This paradigm shift should be registered by EUROSTAT.
- Support the development of ATMPs centres of excellence across the EU on the model of the European Reference Networks.
- Increase investment in research related to ATMPs.

We call on each EU Member State to:

- Introduce innovative payment models for ATMPs able to capture the “value proposition” of this innovation that guarantee, on the one hand, access to these highly innovative therapies to all potentially eligible patients and, on the other, sustainability for the NHS.
- Reduce health inequalities in access to ATMPs within the Country, with particular attention to the inner/internal areas.
- Ensure greater transparency between regulators, HTA bodies, and payers.
- Promote a multistakeholder approach to ATMPs governance.
- Guarantee civic participation in the ethics committees related to ATMPs.
- Promote wider knowledge about ATMPs among healthcare professionals.
- Increase investment in research related to ATMPs.

We call on Local Health Authorities, public & private providers, Health Care Professionals (HCPs), and Patients’ Advocacy Groups (PAGs) to better cooperate in the ATMPs field in order to:

- Strengthen patient education programs, communication campaigns and health literacy initiatives, ensuring major confidence among patients and citizens on the opportunities and challenges around the ATMPs.
- Building the public consent around ATMPs sharing best practices and successful case histories.
- Be actively involved in the development of new ATMPs as part of good clinical practice.

We call to citizens to:

- Be aware of the needs of patients with conditions that don’t respond to traditional treatment;

- Pay more attention to recognizing the official and renowned source of information related to healthcare.

Rationale

The relevance of these innovative therapies places them at the centre of the discussion on health and health policy choices for the future and the sustainability of each National Health System (NHS) across Europe. This will pose very delicate problems of choice and rationing in terms of access to treatment for patients, which could result in the treatment of fewer patients than eligible and therefore potentially treatable.

A solution in terms of identifying the resources necessary to finance them must therefore be tackled today so as not to arrive unprepared tomorrow. In fact, the volume of resources necessary to guarantee access to these therapies and the financing models are - in perspective - the decisive junctions, because they will determine the lesser or greater availability of health systems to bear the cost. The persistence of the current situation would inevitably lead, for example, to delay the access to ATMPs by potential patients and the limitation of their use to subgroups of patients, within the approved indication. It is, therefore, necessary to be prepared in time, to promptly overcome delicate and ethical choices for the health systems that may impact on the right for cure:

- which therapies can be reimbursed, given the resources available?
- Who and based on what arguments will explain to potential eligible patients that access to treatment is not possible for everyone?
- Who will decide the access thresholds and based on what criteria?
- What do we need to know in order not to be unprepared?
- And in what direction should we promote at the EU level a change to guarantee the right of access to as many potentially eligible patients as possible, avoiding forms of rationing or problems of financial sustainability that lead to inequity?

Proposal

Based on the above-mentioned scenario, it is clear that this type of therapies needs new and different payment and accounting methods, which take into account the high initial costs and the wide and lasting benefits over time, both for the patient and for the national health system.

These characteristics could be enhanced by providing a deferred payment model, with a risk-sharing mechanism outcome based that would enable the NHS to pay only for the “value” and to spread overtime the cost for therapy. This would allow the state to acquire more therapies in the short term and thus provide broader access to eligible patients while facing expenses over a longer period and only if the therapy has produced benefits. This payment scheme is not possible with the current accounting system and needs an institutional change of mindset that would allow to classify ATMP expenditure as an investment and not as a cost.

If we want innovative therapies, we need to think about a new system for dealing with healthcare spending. We need to abandon the idea of "ceilings" and think over the long term: moving from spending on silos to revenue for citizens' health. In the last 30 years we have had many changes in the European accounting, made by EUROSTAT. In the 2008 NDA, the scope of investments was extended to include research and development (R&D) expenditure and expenditure on military defence systems. Particularly, expenditure on large military defence systems is capitalised. The new SNA also records large military weapons - warships, ballistic missiles and tanks, etc. - as fixed capital. - as fixed capital (interestingly, single-use products such as ammunition, missiles, rockets and hand grenades are treated as military stocks). As evidence of a continuous work of discussion and evaluation on possible updates in the System of National Accounts, several informal working groups promoted by the Eurostat Commission are active.

A concrete proposal is to focus on the need to change the accounting system at the EU level, and it needs to be addressed and discussed with the EU Institutions and relevant stakeholders: to transform the expenditure items relating to advanced therapies from current expenses to investment (or capital expenditure) so that innovative payment models for ATMPs can be implemented.

List of endorsers

ATMPs represent a new, yet another challenge for public health systems in all countries, and the following broad coalition of civil organization, Patients' Advocacy Groups (PAGs), and relevant stakeholders is engaged across Europe to promote at the European level the importance of access to advanced therapies by the highest possible number of patients.

International







<p><u>GaFPO Global Alliance for Patient Access</u></p>	
<p><u>IAPO Patients for Patient Safety Obseivatoiy</u></p>	
<p><u>Inteínational Alliance of Patients' Oírganizations (IAPO)</u></p>	
<p><u>Inteínational Patient Oírganisation foí Pírimaíy Immunodeficiencies</u></p>	

<p>Thalassaemia International Federation</p>	 <p>THALASSAEMIA INTERNATIONAL FEDERATION</p>
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European

<p>ASSOCIAÇÃO EUROPEIA DE SAÚDE EDUCATIVA E PREVENTIVA EM EPIGENÉTICA</p>	
<p>euPrevent</p>	
<p>European Liver Patients' Association – ELPA</p>	

National organizations

Albania	AMAD Centef	
Albania	togethef foí life	
Bulgaria	togethef with you	<i>e-здраве и култура</i>
Bulgaria	Association of Reproductive Health, Pregnancy and Childcafe "Smile"	 <p>Асоциация за репродуктивно здраве, бременност и грижа за децата "Усмихни се"</p>
Bulgaria	Alliance of Transplanted and Operated ATO "Future foí All"	 <p>АЛИАНС НА ТРАНСПЛАНТИРАНИТЕ И ОПЕРИРАНИТЕ АТО "БЪДЕЩЕ ЗА ВСИЧКИ"</p>
Bulgaria	Association of Patients with Cardiovascular Diseases	 <p>АСОЦИАЦИЯ НА ПАЦИЕНТИТЕ СЪС СЪРДЕЧНО-СЪДОВИ ЗАБОЛЯВАНИЯ</p>
Bulgaria	The Innovations Institute	 <p>INNOVATIONS INSTITUTE</p>

<p>Croatia</p>	<p>Centar za edukaciju i informiranje potrošača</p>	
<p>Cyprus</p>	<p>Cyprus Federation of Patients' Associations and Friends</p>	
<p>France</p>	<p>INDECOSA CGT</p>	
<p>Italy</p>	<p>AS.MA.RA</p>	
<p>Italy</p>	<p>CONSORZIO PER VALUTAZIONI BIOLOGICHE E FARMACOLOGICHE</p>	
<p>ITALY</p>	<p>FAIS</p>	
<p>Italy</p>	<p>Società Italiana Attività Regolatorie, Accesso, Farmacovigilanza</p>	

Kosovo	Patients' Rights Association in Kosovo	
Malta	Malta Health Netwoik	
Netherlands	Orphans Feeding Foundations	
Poland	The Institute for Patient Rights and Health Education	
Poitugal	MOG Movimento Oncológico Ginecológico	
Poitugal	Obseivatóio Saúde dos Povos	
Republic of Macedonia	Studioium	
Spain	Foio espanol de pacientes	
Spain	ASOCIACION GALEGA ASPERGER	

About the promoters

This “Call to Action” is part of a broader EU project⁶ developed at the EU level by Active Citizenship Network with the support of the #VITA coalition that allowed in the first half of 2022 to organize a EU training seminar titled “ATMPs revolution: empowering leaders of EU civic and patients’ advocacy groups”, with 96 participants from 17 Countries⁷.

About Active Citizenship Network

Active Citizenship Network (ACN) was initiated in 2001 as the European and international branch of the NGO Cittadinanzattiva (Active Citizenship), an organization, founded in Italy in 1978, which promotes citizens' activism for the protection of rights, the care of common goods, the support for people in conditions of weakness.

ACN is a flexible network of European civic organizations which are involved as partners in its different projects, addressed to encourage active participation of citizens in European policy-making. ACN mirrors Cittadinanzattiva's Italian policies, such as health, corporate social responsibility, education and training at the global level.

ACN's mission is to promote and support the construction of the European citizenship as an "active citizenship" which means the exercise of citizens' powers and responsibilities in policy-making.

ACN recognizes the prior role of national and local civic organizations in this process and advocates enrichment of the European Union subsidiarity concept by strengthening the relationship between institutions and citizens, so that institutions favour free initiative of citizens, both individuals and organizations, in carrying out activities directed towards the common good.

⁶ To know more: www.activecitizenship.net/patients-rights/projects/412-vita-value-and-innovation-of-advanced-therapies.html

⁷ To know more: www.activecitizenship.net/patients-rights/projects/416-atmps-revolution-empowering-leaders-of-eu-civic-and-patients-advocacy-groups.html

About #VITA coalition

The #VITA coalition

#VITA (Value and Innovation of **A**dvanced **T**herapies) is a group of pharmaceutical companies specialized in the Advanced Therapies industry, with the scope of promoting the dissemination and enhancement of Advanced Therapies with the following purposes:

- To disseminate knowledge among the various stakeholders about the innovative value and therapeutic benefits of Advanced Therapies for patients and citizens;
- To ensure that the stakeholders themselves acquire objective data and facts regarding the opportunities and criticalities of Advanced Therapies in order to initiate a constructive dialogue to ensure prompt access innovation by patients and health care professionals.

In 2022, **#VITA** members are: Bristol Myers Squibb, Gilead Sciences, Janssen, Pfizer, PTC Therapeutics, Roche, Vertex.

#VITA working group, coordinated by LS CUBE Studio Legale, is composed by Prof. Giorgio Alleva (Professor of Statistics at University of Rome, La Sapienza, Prof. Americo Cicchetti (Professor of Business organization at University Cattolica of Rome and Director of ALTEMS), Prof Mauro Marè (Professor of Financial Sciences at University of Tuscia and Luiss Business School), Prof. Eugenio Anessi Pessina (Professor of Business administration, University Cattolica del Sacro Cuore; Director of the Center for Research and Studies on Health Management – Cerismas) and by Lawyer Rosanna Sovani (partner of LS CUBE Studio Legale).

Repository & press articles

Below you can find the list of main documents, pillars of this Call to Action, as produced by a group of experts in Italy since 2019.

- [Rationale of the initiative](#)
- [An overall overview about ATMPs](#)
- Technical Policy Report from the #VITA Working Group *“The economic evaluation of advanced therapy medicinal products: characteristics and reasons for a new economic and accounting approach”* ([EN](#) – [IT](#))

Recent press articles (all in IT language)

- [Entro il 2030 in arrivo 60 nuove terapie geniche](#)
- [Sedimila terapie avanzate in sperimentazione, 60 in arrivo. La sfida alle malattie \(oggi\) incurabili](#)
- [Terapie avanzate, serve cambiare le politiche di bilancio europee](#)
- Le opportunità e le sfide delle nuove terapie avanzate ([1](#) – [2](#))