



The Value of ATMP
how to assess, how
pay, how to finance

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Major characteristics of ATMPs

- ATMPs are potentially "curative" (many drugs - including cancer - slow the progression of a disease, but do not cure it) or are "transformative therapies"
- They concern 'limited' populations and are generally considered to be orphan or ultra-orphan drugs
- In many cases there are no comparators to set up non-inferiority studies or superiority
- Gene therapies require a single administration, while clinical and economic benefits spread over time (even decades)
- Innovativeness is judged mainly on the basis of the lack of alternatives;
- However, there is a potential for 'uncertainty' about how long the effects and outcomes will last

Key Questions?



Are ATMPs challenging healthcare system's sustainability?



A new value framework is needed to properly assess ATMPs?



If yes, why a new model is needed and how it should look like?



How to set prices? How to finance ATMPs?



Advanced therapy medicinal products and health technology assessment principles and practices for value-based and sustainable healthcare

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1. **Uncertainty** Although uncertainty in the evidence is an aspect of all HTA processes, the specific nature of the evidence that is available for newly approved ATMPs is considered likely to require special consideration.
2. **Discounting** The nature of the distribution of costs and benefits was felt likely to make the estimation of cost-effectiveness particularly sensitive to decisions about appropriate discount rates.
3. **Health outcomes and value** The way in which potentially curative treatments may be considered differently to treatments that create smaller incremental benefits for larger populations may raise questions regarding the existing methods of assigning value in HTA.



Value Assessment Methods and Pricing Recommendations for Potential Cures:

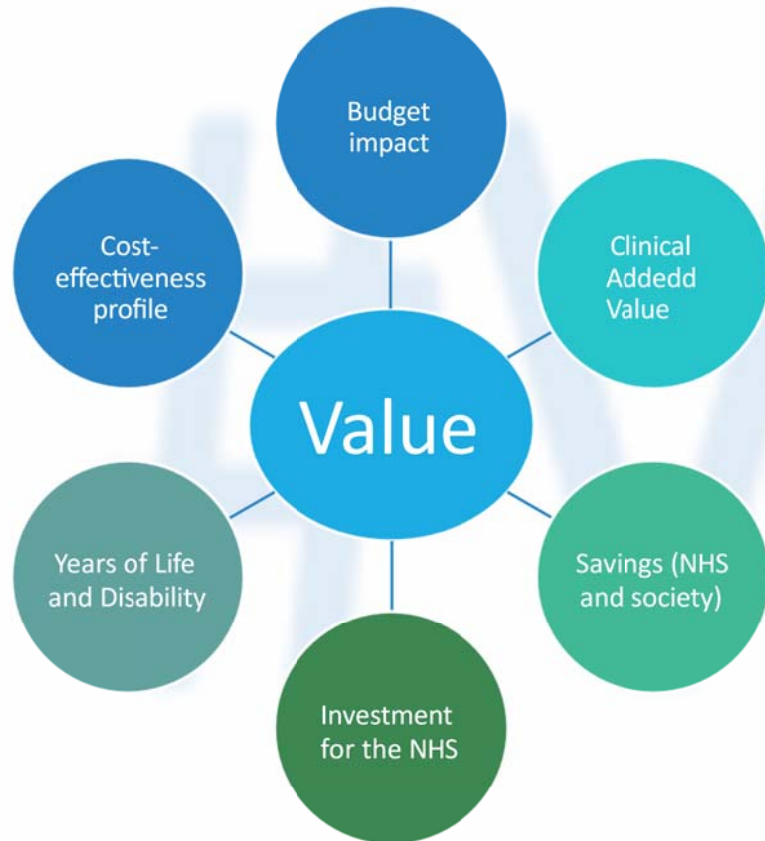
A Technical Brief

Draft Version

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1. Increased uncertainty v unrecoverable costs
2. Questions regarding ac dimensions of value
3. Time divergence betwe and benefits.
4. Affordability and fair sh economic surplus.

The ATMPs value framework



- Long-term budget impact models
- Long-term Clinical Benefit
- Economic savings from the perspective of society (direct and indirect)
- Value of the investment
- Recovery of years of life lost, or years lived in disability
- Uncertainty in clinical benefits vs. uncertainty in economic impact, and cost-effectiveness



The ATMPs value framework

Challenges for economic evaluations



- The decision on which therapy is worthy of funding should be based on an **estimate of the overall economic effects on the health system** and the health of citizens, not only considering the frontal and immediate costs, but also the long-term value to society.

- **The usual economic valuation**, based on the estimation of the costs of traditional therapies (drugs) and on the accrual basis of financial statements, is not very suitable for advanced therapies and their particular technological and industrial characteristics.

- They **require a new health and economic approach**



ATMP as investment 1/2

COVID-19 has highlighted that **healthcare is a fundamental investment** for any country and determines its economic and social sustainability

An increasing and significant proportion **of health expenditure has obvious investment** characteristics (multi- year benefits)

In principle, expenditure on ATMP can be similar to gross fixed capital formation, i.e. category of acquisition of movable property, machinery and equipment

But there may also be other possible solutions (production subsidies, intangibles, human capital R&D, etc.)



ATMP as investment 2/2

Sars-CoV-2 pandemic 'comes to our aid': the **health of EU citizens** and countries is crucial and crucial for **economic and sustainability and financial sustainability**

Trade-off between health and economy: in reality this is not the case at all, without good health conditions, there is economic damage, without re-establishing acceptable health conditions, economies will not start again

After Sars-CoV-2, the **value** of human life and **good health** for **growth potential is evident:** vaccines solve, more permanently, epidemic; in the same way, ATMP permanently treats different pathologies

Flow of benefits (direct and indirect) for many years (including effect on productivity) and **cost savings** (lower consumption of drugs and facilities, lower costs for care and family, etc.)

Can we deny the element of investment and **enrichment of the physical, human and technological** capital stock of a country that derives from ATMP? NO, **but then how can they be economically/accounted for?**



Are ATMP investments under an economic point of view?

1/2

- In terms of economic analysis, ATMP therapies have very special characteristics:
- they have a **high temporal asymmetry** between the emergence of costs – which are almost all upfront – and that of benefits. This is perhaps the main aspect that suggests an economic and accounting evaluation of these different and innovative therapies
- **produce direct and indirect benefits over time:**
 - In terms of savings: **healthcare savings**, **drug consumption** and **health services** of different kinds; the reduction of resources consumed directly in health centres and the reduction of family and **direct and indirect care resources**
 - In terms of value: the increase in life expectancy, the improvement of the qualities of human life, the treatment and stabilisation of different pathologies (with a clear impact **on the value of human life**); but also in terms of increasing the state of health of patients; **increased productivity**, **higher tax revenues**; the possibility, after the remission of the disease, to be able to continue to educate and participate in collective life; the **lengthening of the potential pension burdens**



Are ATMP investments under an economic point of view?

2/2

- **Net investments** are crucial for the growth of national income and the sustainability of debt
- Recent economic and technological developments have shown that there is a need for a **rethink concept of capital, both from an economic point of view, from the statistical point of view and from point of view of national accounts.**
- In the traditional accounting approach, the **notion of capital usually refers to economic capital**, which is clear that human, **social and natural capital** (linked to the environment and health conditions) are increasingly important for the future of countries.
- In order to do this, decisive **statistical and accounting innovation** is needed: expenditure on traditional and canonical investments must also be combined with other types of expenditure which cover new procedures and criteria **consider to be essentially current:**
 - to stay in the health case, how much of the expenses considered current today (for doctors, healthcare equipment or atmps) actually have an **investment nature**?
 - Without them, even the "physical" part of investment in health care (e.g. a hospital or treatment centre) would not in fact be usable?



Revisions of national accounts: recent experience

In the 1993 SNA, the scope of investment was expanded to include **software, mining exploration, artistic original valuables expenses**

In the **SNA 2008**, the scope of investment was **expanded to include R&D and military defense spending**

The **2010 ESA** expressly acknowledges that: "research and development expenditure" and "**military expenditure investment in nature**", so they are recorded as gross fixed capital formation and no longer as current expenditure. The identification and treatment of R&D as an investment is based on the "value in terms of future economic benefits" (see paragraph 216 of the SEC 2010). Similarly, expenditure on military systems is also investment in nature. The new accounting system realistically recognises their productive potential for a country's external security over several years.

In the Italian context, a crucial point is the criterion established in the **Manual on Public Deficit and Debt** (for R&D and the armaments sector) since the same logic adopted for the public good of security should apply to the **public good health**. It would be very embarrassing for the public budgetary authorities to declare that they were not prepared to follow this line, after the funds for the epidemic



"High road" to qualify ATMP as investment expenditure

Why?

The time has come to **review and update the current accounting conventions in a profound** considering that **some of the current expenditure** is necessary to **increase a nation's capital stock economic assets** – they are therefore decisive for the future of a country and its economic sustainability – and that for this reason they can be considered, at least in part, as investment expenditure

The **accounting criteria are the result of a compromise and are an accounting convention**

It can and must **be updated** in the course of technological conditions, the degree of economic and social development and the common feeling of the nations and peoples of the Community.



KEY MESSAGES

- ATMPs **are inherently innovative**: they treat diseases for which there are no clinically viable alternatives.
- ATMPs are characterised by a high effectiveness linked to their curative or transformative nature: the benefits are always measured in years, in some cases in ... **decades of life gained** (between 1 and 4) and in **lesser disability**
- **The impact on the annual budget is limited** (if we report it to the years in which the benefits are distributed and considering that the indications refer to limited populations), in the long term it is possible to record **savings in the perspective of the NHS**.
- They generate savings in "social costs" due to the high number of life years saved, productivity at work recovered, quality of life. The same intervention can be cost effective in a lifetime perspective and not cost effective in a 10-year analysis.
- ATMPs, having obvious elements of an **expense with benefits deferred over time, are in fact similar to an investment expense**
- The extended distribution of benefits over a longer period of time suggests a more harmonized **distribution of costs in economic accounting terms and in line with the realization of the benefits**.
- **ATMP expenses, similar to expenses relating to fixed investments**, are subject to the criterion that the impact on net indebtedness is represented by payments made on the basis of the state of progress (SAL) with the same impact on net indebtedness and needs, corresponding to the payments compared to the benefits of the treatment to which the patient is subjected.
- **That part of health expenditure** that presents a clear misalignment between upfront costs and life-time benefits should be requalified as investments under ESA rules





#VITA Innovative value of ATMPs

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