Opening Remarks

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Good afternoon and welcome also from me to the 17th edition of the European Patients' Rights Day. The choice of dedicating this event to the theme of advanced therapies was an easy one for us, as it is consistent with the fervour, but also the concerns, that this very topical issue is facing.

In outlining the contours of this meeting, we had three characteristic elements in mind:

1) The desire to bring together, as much as possible, the interlocutors who have put forward concrete proposals on the subject of ATMPs in recent months, including through the organisation of events in the European Parliament (e.g. A - B - C), in a very timely but perhaps also isolated manner.

   Our intention is also encouraged by the fact that for the first time this conference is being hosted not by one but by two informal Interest Groups: the MEPs Interest Group "European Patients' Rights and Cross-border Healthcare" and the TRANSFORM MEP Interest Group, whose members I am sure will convey what emerges today to the new-born Public Health permanent subcommittee, established on February at the EU Parliament to better address public health matters.

2) Receiving a signal of attention at the broadest levels of the European Commission, for which I thank the Health Commissioner Stella Kyriakides for the video message that we will shortly hear and which will inevitably serve as a food for thought and encouragement in today's debate.

3) To go beyond the current narrative related to the European pharmaceutical legislation review that seems to be limited to the following statement: "the fate of the pharmaceutical sector is at stake as well as the future of national health systems". This is what is stated in the introductory article published by a well-known media, regarding an event held just yesterday here in Brussels on ATMPs, not by chance in the total absence - in the panel - of the patients' point of view. So, talking about the future scenarios of ATMPs that the review may outline, what about the justified expectations of patients? What is holding us back from including and considering in the debate also the point of view of the so-called 'final users' of the health service, the ultimate recipients of the decisions whose effects will be experienced in practice?

Travelling at different speeds, how can innovation and access be combined? To try to answer these questions, the 17th European Patients' Rights Day will gather inputs from the European Institutions, the World Health Organization, Patient Advocacy Groups from different countries, independent experts and relevant stakeholders in the field of Advanced Therapy Medicinal Products whom I thank in advance for accepting our invitation.

As far as we are concerned, to the question "how can innovation and access in the field of ATMPs be combined?" we do not have an answer, but in October 2022 we presented a proposal right here in Parliament, supported by 43 associations, to try to make up ground for patients' right to access advanced therapies, calling for a change in institutional mentality to classify spending on ATMPs as an investment and not as a cost. The cost of these therapies could be amortised over the years in relation to the savings generated over time. In particular, they could be shown in the state budget over several years and not all in the year of expenditure. This would significantly increase the financial sustainability of these costs by the public sector over time and promote greater and more equitable use of ATMP medicines in the population, without putting public finances at risk.

This proposal also received an encouraging endorsement from the new Italian health minister in January 2023[1].

[1] [1] ***FARMACI: SCHILLACI, 'NUOVO QUADRO NORMATIVO PER RIMBORSO TERAPIEINNOVATIVE'** = 'Con un apposito tavolo'. Roma, 17 gen 2023. (Adnkronos Salute) - Il tema delle terapie avanzate "daun lato presenta costi assoluti estremamente elevati e dall'altro la capacità di incidere in modo decisivo e in tempo rapidissimi sulla storia naturale di patologie ad elevata mortalità e di impatto sulla salute evitando cure prolungate nel tempo. Questi aspetti rendono i costi di queste terapie più un investimento, una spesa in conto capitale che una spesa corrente. Si tratta quindi di definire un nuovo quadro normativo specifico per il rimborso di queste terapie da parte del Ssn che preveda da un lato la possibilità di un periodo di ammortamento del costo e dall'altra uno schema a rimborso a risultato 'pay for performance'". Lo ha annunciato il ministro della Salute, Orazio Schillaci, nel suo intervento in Commissione Sanità e Lavoro al Senato nel seguito delle comunicazioni sulle politiche del suo dicastero. "Per definire una ipotesi di questo nuovo quadro ribadisco l'opportunità di attivare un apposito tavolo che includa oltre alla competenti direzioni generali*

Above all, we believe that the two aspects can only be better combined and find a point of connection with each other if in the field of ATMPs, we also succeed in strengthening and guaranteeing spaces for civic participation and for PAGs in the decision-making process. This is also why, in Italy, we warmly welcomed the invitation from the national institutions to join the National Ethics Committee for clinical trials on advanced therapies ('ATMPs'), set up in February 2022 at the Italian Medicines Agency, as the only actor to represent the civic and patient point of view on the issue. For the same reason, we urge to go beyond the extreme polarisation of the public/private debate that is accompanying the pharma legislation review, which, despite its inevitable complexity, needs to be integrated from a civic and patient perspective.

The patients' rights to innovation and to access, both of which are proclaimed in the European Charter of Patients' Rights, whose principles and message underpin this Day, should be inextricably linked in every area of healthcare. Unfortunately, in many contexts this does not happen, and even in the field of ATMPs where innovation is advancing, there are even cases of withdrawal or threatened withdrawal of existing and accessible therapies, and today we will also report on this.

And so, in the presence of innovative therapies solutions, endorsed by the competent regulatory authorities, available on the market and already being used by categories of eligible patients, having already proven their effectiveness, we should have the courage to affirm the principle of irreversibility of access to treatment. Those who are using them cannot be continually exposed to this situation of uncertainty. The credibility of the healthcare system as a whole and of all the players involved, public and private, depends on it, especially the medical history of so many patients who see in these innovative treatments a hope that was not even imaginable a few years ago, as well as the dreams of those who live next to them, and the ultimate essence of ATMPs is that of being 'dream savers'. This is confirmed by what was recently published in the New England Journal of Medicine: according to the researchers, never before has a study on the use of CAR-Ts against solid tumours shown such encouraging results as those achieved by a trial, which was, by the way, all developed in Italy.

Such news is what gives us encouragement to move forward.

I end by thanking in advance Mariam Zaidi who will moderate our event today.

I wish you a fruitful meeting.

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del ministero della Salute, i rappresentanti esperti della nostra Aifa, del Mef, i tecnici della Ragioneria dello Stato, ed altri competenti esterni e in particolaredi enti e istituti di ricerca che si sono interessati alla materia", ha concluso Schillaci. (Frm/Adnkronos Salute) ISSN 2465 – 1222. 17-GEN-23 18:21NNNN