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UPDATE OF ACCESS TO THERAPIES, INCLUDING ADVANCED AND INNOVATIVE ONES

OUTCOMES OF THE UNIAMO -
STAKEHOLDER WORK TABLES

THE EFFEMERIDS OF UNIAMO 15/2023

UNIAMO THE ITALIAN FEDERATION OF RARE DISEASES

Access to therapies, including advanced and innovative ones
Multi-stakeholder discussion table (05/12/2019, 12/01/2021,
19/01/2021, 15/09/2021,
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03/10/2023, 10/20/2023).

October 20, 2023 version

This notebook finalized on 20 October 2023, illustrates the results of the discussions, promoted by UNIAMO Italian Federation of Rare Diseases Onlus, within multi-stakeholder working tables regarding the problems of access to drugs, with a special focus on advanced and innovative therapies, experienced by patients during their treatment journey.

The opinions expressed by the participants are to be understood as personal and not representative of the official positions of the respective public or private bodies they belong to.

The document is a summary of what was discussed and aims to be a tool to support Italian policies, also with respect to Europe, highlighting points of convergence and also what does not yet have a unanimous opinion among the subjects involved, but on which we can work to find a concordance.

The Federation will continue to stimulate debate on these issues, involving all the actors involved and illustrating the positions of the community of people with rare diseases, collected through comparison processes internal and external to the Federation and in collaboration with Eurordis.

Cite this document as follows:

UNIAMO F.I.M.R. Access to therapies - Multi-stakeholder discussion table, 2023.

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This document has been translated using IA: please forgive any mistake!

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National Plan for Rare Diseases

30 million Europeans live with a rare disease, 72% of these pathologies are of genetic origin.

Pharmacological treatments Objectives

Promote timely access to etiological, pathogenetic, symptomatic and substitute drugs with proven effectiveness in positively modifying the natural history of the disease and improving the patient's quality of life;

Facilitate and simplify access to all the therapeutic options envisaged by the Individual Therapeutic Plans and drawn up by the clinicians of the Reference Centers for rare diseases, with a guarantee of systematic and timely availability of what is foreseen by the AIFA determinations;

Create an equitable system of access to treatments for rare diseases by breaking down disparities between different geographical areas (Regions/PPAA and ASL) or population subgroups. In particular, drugs in band C, off-label or not on the market in Italy, of proven effectiveness for the pathology in question must be dispensed without cost to the patient, when prescribed by the specialist of the reference center through the individual therapeutic plan (included in the PDTAP of Law 175/2021), based on criteria of essentiality and non-substitutability referring to the patient's condition and explicitly defined;

Integrate HTA evaluations for therapeutic strategies in rare diseases, with analyzes that concern the global therapeutic value and the impact on the expenditure incurred by other service sectors, as well as by families;

Guarantee, also through the alliance between Institutions and Associations, the generation and dissemination of correct information on the evidence supporting the different therapeutic strategies.

Actions

AIFA carries out an integrated evaluation of the existing regulatory tools in order to optimize their use for the different cases in a transparent and homogeneous way across all the Regions;

Establish a joint AIFA working group and technical table for rare diseases of the Regions to prepare a survey of drugs not in band A, H or included in 648 and other therapeutic strategies already guaranteed in the various Regions/PPAA and to draw up within 12 months from the publication of this plan a single list on a national basis, renewed annually. Identify the paths that allow the effective provision of these drugs by the NHS in a homogeneous way throughout the country.

Recognition, by a joint AIFA, Ministry of Health and technical table for rare diseases of the Regions, case studies of early access to treatments with adequate evidence of efficacy not yet authorized or available on the national territory. Analysis of possible tools to facilitate early access to these treatments; The working group undertakes to inform and consult the institutional and associative subjects interested in the specific topics covered.

Definition of the methods that allow the treatment of the patient by the NHS with a plan drawn up by a reference center outside the region of residence, for all therapies/cures that respect the standards established according to the indications and evaluations of the AIFA, also at the ASL of residence, ensuring that the same minimum standards are respected in all Regions/PPAAs in the provision of treatments for rare diseases;

Promotion of training and information activities on the protection of patient safety as a principle underlying decisions;

Launch of a Horizon Scanning process and consequent economic impact assessments in the short-medium term (3-5 years) and plan national and regional strategies, in order to guarantee fair and sustainable access over time;

Definition and implementation of a path for reporting and managing shortages and/or unavailability in the national territory of drugs for the treatment of rare diseases, the acquisition of which can benefit from centralized interventions;

Ensuring the importation of drugs marketed in other countries with indications other than those used for rare diseases, for which there is scientific evidence of efficacy and safety, as long as they are included in the therapeutic plans drawn up by the Reference Centers for rare diseases.

ATools

Use the evidence obtained from "real world" data available through regional, national and European/international population monitoring in price negotiation and renegotiation procedures;

The same monitoring, with adequate integrations, can also be used for "real life" evaluation of the impact of treatments on care pathways and in the social sphere;

Strengthen and enhance the role of the Military Chemical Pharmaceutical Plant (SCFM), Production Unit of the Defense Industries Agency in implementing the action indicated in point 7;

Launch projects to monitor the effectiveness of off-label drugs at national and regional level, shared by AIFA, ISS and Regions/PPAA, with a view to rationalizing access and ensuring appropriate management of resources;

Definition of shared procedures on a national basis for accelerated paths for the activation of trials on drugs or integrated strategies which also include the use of particularly promising drugs;

Update the relevant legislation in order to make the actions indicated in this plan feasible, with particular reference to the Ministerial Decree on the therapeutic uses of experimental medicines of 7 September 2017 and the Ministerial Decree on the importation of medicines of 11 February 1997.

Law 175/2021

Article 4, paragraph 2

2. The National Health Service is responsible for health treatments, already foreseen by the LEAs or qualified as life-saving, included in the personalized diagnostic-therapeutic care plan and indicated as essential, belonging to the following categories:

t);

b);

c) pharmacological therapies, including innovative, class A or H, medicines to be provided pursuant to article 1, paragraph 4, of the legislative decree of 21 October 1996, n. 536, converted by law 23 December 1996, n. 648, dietary products and galenic and master formulations prepared in hospital pharmacies and public and private pharmacies affiliated with the National Health Service, as regards the latter in compliance with specific protocols adopted by the regions;

Art. 5 Pharmaceutical assistance and provisions to ensure the immediate availability of orphan drugs

1. Class A or H drugs prescribed for the care of patients suffering from a rare disease are dispensed by the following subjects:

a) the pharmacies of healthcare facilities, even in the case of outpatient administration of the drug;

b) the local health authorities to which the patient belongs, even if the rare disease has been diagnosed in a region other than that of residence;

c) public and private pharmacies affiliated with the National Health Service, in compliance with the provisions of the regional agreements stipulated pursuant to article 8, paragraph 1, letter a), of the legislative decree of 18 September 2001, n. 347, converted, with amendments, by law 16 November 2001, n. 405.

2. In derogation of the provisions regarding pharmaceutical prescriptions referred to in article 9 of law 23 December 1994, n. 724, for prescriptions relating to a rare disease the number of pieces that can be prescribed per prescription may be greater than three when provided for by the personalized diagnostic therapeutic care plan referred to in article 4, paragraph 1, of this law.

3. Pending periodic updates for their inclusion in hospital therapeutic handbooks or other similar lists prepared by the competent regional or local authorities pursuant to article 10, paragraph 5, of the legislative decree of 13 September 2012, n. 158, converted, with amendments, by law 8 November 2012, n. 189, the drugs referred to in paragraph 1 of this article are in any case made available by the regions.

4. In derogation of the provisions of the decree of the Minister of Health of 11 February 1997, published in the Official Gazette no. 72 of 27 March 1997, the importation of drugs on the market in other countries is permitted, even for uses not authorized in the countries of origin, provided they are included in the plans referred to in article 4, paragraph 1, of this law, as well as in the list referred to in article 1, paragraph 4, of the legislative decree of 21 October 1996, n. 536, converted by law 23 December 1996, n. 648. The drugs referred to in this paragraph must be requested from a hospital facility, even if used for home care, and are paid for by the National Health Service.

The proposal of the parliamentary intergroup Sustainable Innovation in Healthcare

Try to introduce an experimental fund into the Budget Law that begins to interpret the investment in advanced therapy drugs as an investment, not as a cash expense.

Speech by Daniele Manca Co-President Intergroup Sustainable Innovation in Healthcare Senate of the Republic at the MonitoRare Convention, Rome 11 July 2023 Good morning everyone, first of all I would like to reiterate a concept; since the XVIII Legislature we have chosen to address the issue of economic sustainability, which is the biggest issue we face today, that is, making sustainable what is an extraordinary investment that research, innovation, has already produced, because the In our opinion, Advanced Therapies represent one of the biggest changes, one of the biggest transformations underway in the economic dynamics in our country. However, we do not have an adequate regulatory framework to make these great transformations universal, which the world of research and the pharmaceutical world has already activated.

The first question that we must therefore address is certainly this, the President rightly asked me for concreteness. I continue to think, we have done this since the XVIII Legislature in the transversality of political forces, we have built an intergroup, it is known that there is a need not only to weld health policies with social policies, but that these must be welded with the dynamics economies of this country. Because the Ministry of Economy and Finance represents the fundamental element from which to start, because we have seen that it is essential to carry out a reclassification of pharmaceutical spending. We are not going anywhere if we continue to have a drug purchasing policy that treats Advanced Therapies the same way as purchasing an aspirin.

Here we are faced with transformations that require a new legislative framework. If we want to have universal access as our objective, Advanced Therapies today do not have an economic-financial framework that guarantees universal access; we have seen all the data, especially in perspective, when the people who will be able to access these paths gradually increase, we will have the problem of how we treat an investment, because it is not a cash expense; the purchase of an advanced therapy for the Italian State cannot represent a current expense, because we have seen that here it is a question of treating a disease not the symptom, it is a question of restoring to people the dignity that rare diseases have taken away. The purchase of an advanced therapy produces savings in healthcare spending, because if we intervene correctly and monitor the ways in which these interventions are determined also at a regional level, we will finally be able to consider this as one of the first major investments in pharmaceutical policies, and in particular on the economic dynamics of this country. So the first concrete commitment is to try to introduce an experimental fund into the Budget Law that begins to interpret the investment in advanced therapy drugs as an investment, not as a cash expense. This is the biggest priority we face today if we want to aim to combine economic sustainability; I continue to consider it necessary to adequately finance the National Health Fund, there is no doubt about this, resources are also needed to close the Covid season; However, I continue to think that resources are not enough, if we do not interpret these transformations as an opportunity to reform public spending, the ways in which public spending acts in the local finance system, and in the Italian state budget system, then it is clear that we have constituted not just an intergroup.

I also thank UNIAMO for the training on 17 May because there is also a need to train Parliament, because we are in extraordinary dynamics of change, of research, of economic and social systems, of family dynamics, however it is clear that this change has need for new reforms. We will continue to work so that economic sustainability must have an initial concrete response in the Budget Law. Ensure that the Budget Law determines that experimentation on an effective Fund for Advanced Therapies, which is included in the State Budget as an investment, which also starts the path for a European reform, because we need to include this discussion within the reform of the European Stability Pact, which is currently suspended but which will come into force next year.

It will become a problem again, especially for a country with high public debt, that will be the obstacle to the development of advanced therapies towards universality, I would like this country to tell the families affected by these dynamics that it is no longer necessary to Abroad, you no longer need a large credit card to be guaranteed a fundamental right, universal access to these therapies.

This is the first element of concreteness that we must try to put together in the Budget Law, it is the only way we have to combine budget balance, i.e. economic sustainability, with universal access. Failure to resolve this issue risks putting two pillars of our Constitution in opposition, so let's hurry. The Government has activated an inter-ministerial table also at our request between the Ministry of Health and that of the Economy, I hope that it will be established soon, because the establishment of this table is the preliminary element to be able to have in the Budget Law a first response, even experimental, but here we are talking about an investment. I would like to prevent this country from continuing to consider the purchase of a drone and a cash expense the purchase of an advanced therapy that is decisive for transforming an illness and restoring quality of life to citizens and people who have unfortunately been less fortunate than us. . This for me is the fundamental battle: politics, regardless of the sense of belonging, must have as its objective, even challenging the Government, to ensure that an essential experimental phase can be started quickly to attract investments, enhance research and make so that this enormous change underway also has an adequate regulatory and legal framework to guarantee universal access to these therapies.

The talking points

Talking about accessibility and fair distribution of pharmacological treatments means addressing a very broad world, which brings with it a series of facets that each need specific in-depth analysis.

As part of the development and marketing process of a drug, we start with research, which must also be appropriately directed through discussion and participation, from the early stages of conception, of patient representatives and with the patients themselves. Good research, with well-structured and directed subsequent phases of experimentation, which collect all the necessary data, are the fundamental premises for compiling a dossier that may not encounter obstacles along the way.

Next, discussions are needed between regulatory bodies and pharmaceutical companies, which can clarify all prior, concurrent and subsequent doubts.

A comparison would also be appropriate between AIFA and the Regions, which through the registers can contribute to implementing knowledge of the number of patients potentially interested in therapy, despite the limitations resulting from missed diagnoses. In fact, often when a therapy arrives on the market, diagnoses increase in a sometimes substantial manner.

From the regulatory agency, the next step is to actual availability at a regional level, in the administration centers, sometimes passing through the purchasing centers and also taking into account the company budgets and the forecasts made in the previous year. The need was therefore felt for some form of horizon scanning that could simplify these steps and bring them into planning rather than emergency management.

In the post-approval process, there are no shortage of further obstacles: the prescription centers can also be outside the Region: the prescriptions must also be verified in the Region of residence, with further waiting steps; any shortages and unavailability make supply difficult (think for example of the chronic shortage of immunoglobulins). The management of shortages also includes imports from abroad, which today have tested but time-consuming procedures.

Sometimes drugs are marketed for pathologies other than the one for which they are used: off-label and repurposing must be carefully evaluated and "reconversion" procedures facilitated.

In all this, some big themes:

economic sustainability, both at the level of individual countries and at the level of healthcare companies, which many high-cost therapies pose as a challenge to healthcare systems;

the need for access at European level to be fair and possibly contemporary treatments for ultra-rare diseases and their overall management: price negotiations for each individual state are highly impactful for smaller pharmaceutical companies

the abandonment of some therapies by the pharmaceutical industry, for economic reasons, and the consequent need to make them available to patients, if the only possibility of treatment and cure, with analysis also of the causes of abandonment to understand if something in the system needs to be changed

the lack of truly transformative treatments for most rare diseases indicating the need for greater incentives for targeted research

The need for all these paths and processes to be participated by patients and their representatives is now well established: the effectiveness and efficiency of the entire system is recovered.

The discussions in outline

ATMP: Acceleration of therapy approval process

Harmonious distribution of delivery centers in the Regions

- Invest in training and infrastructure in some centers to avoid patient movements WHO:

The individual Regions; the Ministry for regional coordination

ATMP

Economic sustainability

- Creation of a specific fund WHO Parliament TOOLS Budget Law DOCUMENTATION/LAWS See proposal Parliamentary Intergroup innovation in Healthcare - Proceedings of the MonitoRare Convention

- Allocation to multi-year costs (investment) CHI European Community

For ultra-rare pathologies, central price determination commission avoiding the cost of submitting dossiers in each individual member state WHO European Community and Member States

LITERATURE/LEGISLATION UNIAMO ephemeris 6/2022

ATMP: Acceleration of therapy approval process

Submission of better dossiers to AIFA, through:

- early dialogue between Pharma and AIFA even before the submission of the CHI: AIFA dossier
- compilation of a better dossier, with better scientific evidence, setting the necessary WHO from the beginning:
PHARMA; testing centers; Patient associations and their representatives.
INSTRUMENTS:
 - use of training developed during the ERICA - WP4 project for the centers developing the trials;
 - use of models for statistics on small numbers
 - support from the European Advisory Board (see Annamaria De Luca) at the start of the trials.
- enrich the dossier with proms and prems and other data CHI Pharma, Patient Associations, Institutions TOOLS
 - specific, qualitative and quantitative investigations; systematized collection of data LEGISLATION
 - PNMR Pharmacological treatments ob. 4
 - Proposal of law Loizzo, Panizzut, Lazzarini, Matone
- make greater use of pre-clinical data, with particular attention to those concerning efficacy and not just safety CHI Pharma, Universities, research centres, patient associations, institutions TOOLS
 - data collection tools and specific setup LEGISLATION PNMR Research ob. 8

- **make AIFA approval processes more effective, through greater transparency of the evaluation elements WHO: AIFA TOOLS:**
- **procedures available on the website, "open AIFA" type meetings for discussion**
- **parallel assessment at European level LEGISLATION**
- **Balduzzi Law (100 days)**

- **use the European joint assessment also in the individual member states WHO Parliament, AIFA TOOLS**
- **specific, qualitative and quantitative investigations; systematized collection of data LEGISLATION/LITERATURE**
- **Effemeride UNIAMO 6/2022 - Access to therapies**

- **more precise identification of the number of people potentially interested in the therapy WHO AIFA, Regions, Patient Associations TOOLS**
- **updated and complete registers with the possibility of identifying subgroups;**
- **neonatal screening where possible LEGISLATION/LITERATURE Uniamo Ephemeris 6/2022 - Access to therapies**

- **adoption of risk sharing agreements when setting the reimbursement price WHO AIFA, Pharma, Parliament INSTRUMENTS**
- **specific, qualitative and quantitative investigations; systematized collection of data LEGISLATION/LITERATURE**
- **French and/or German model**

Set up the search correctly

- **increase interactions between academic research and pharmaceutical research WHO:**

Pharma, Universities, research centres

- **listen to patients' requests regarding the expected results, involving them from the beginning of the research planning CHI Pharma, Universities, research centers, patient associations**

LEGISLATION/LITERATURE UNIAMO Ephemeris 6/2022 - Access to PNMR Therapies Research ob. 8



ATMP and Orphan Drugs

Acceleration of making therapy available to the patient

- activation of horizon scanning procedures that allow the Regions to prepare the administrative documents necessary for the prompt availability of CHI therapy:

Regions, AIFA, TOOLS:

- specific site on active clinical trials
- creation of a specific task force in each Region, with a national comparison where necessary LEGISLATION/LITERATURE PNMR Pharmacological Treatments objective 6 Good practice of the Veneto Region

- corporate planning consequently adequate in budgets, compared to the number of patients followed with the pathology WHO Regions, Health Directors, INSTRUMENTS Purchasing Centers

- activation of a communication method from the Task Force to individual healthcare companies
- registers for identifying patients, with the specification of the treatment center

Orphan Drugs

Deficiency management

- verify the possibility of production by the CHI Military Chemical Pharmaceutical Plant:
Institutions, IFM, Parliament LEGISLATION/LITERATURE AIFA register
PNMR Pharmacological treatments ob. 7

- verify the possibility of galenic formulations WHO Institutions, Hospital Pharmacists LEGISLATION/LITERATURE
-Law 175/2021

Unavailability

- monitoring of situations of unavailability, with verification of specific emergency situations and coverage with transfers between hospitals.

WHO:

AIFA, Hospital Pharmacists, Regions LEGISLATION/LITERATURE PNMR
Pharmacological treatments ob. 7 DruGhost developed with PI AIFA-SIFO

- import from abroad (see below) see another paragraph

Long times and a lot of bureaucracy for importing from abroad; repeated practices for each individual patient

- the procedures for importing from abroad could be centralized in the IFM avoiding the duplication of procedures for each hospital pharmacy and for each patient WHO:

Parliament, IFM, AIFA, Hospital Pharmacists and Health Directorates

LEGISLATION/LITERATURE Centralized import made by IFM recently

Ministerial Decree 1997 PNMR Pharmacological treatments actions point 8 and Tools n. 3 and 6

Farmaci in repurposing

Procedures and models to be developed

- Develop a useful platform for research centers that want to try to test drugs in repurposing CHI:

Institutions, Regions, ERN, AIFA etc. LEGISLATION/LITERATURE

- European project Remedy4All

- PNMR Pharmacological Treatments Actions point 8

Costs

- Pharma companies should not dramatically increase costs for repurposing treatments that have already been in use for years. CHI: Pharma, Parliament

Reconversion times

- the reconversion times of the drugs are however very long; simplifications would be appropriate with respect to some procedures already carried out for the first placement on the market of the drug in question (development of specific pathways) WHO: Parliament, EU

ATMP and orphan drugs

Equity

- encourage research especially in pathologies that have never had a treatment
- incentivize and support research for transformative treatments
- make treatments available at least in all member states
- study models for the availability of treatments for ultra-rare diseases

WHO:

European Parliament through the review of the pharmaceutical strategy;
Min Sal Italy LEGISLATION/LITERATURE Art. 168 of the treaty (guaranteeing an adequate level of health for all EU citizens)

Class C drugs, off label

Uniform availability in all Italian regions;

- establishment of a specific AIFA - TT MR Regions / PPAA working group for the recognition of drugs and drafting of a single list on a national basis

WHO:

AIFA - Regions - Patient Representatives LEGISLATION/LITERATURE

PNMR Pharmacological Treatments, Objective 2

Medicines not on the market in Italy

possibility of import with simplified procedures

- Update the reference legislation DM 7 September 2017 and DM 11 February 1997

WHO:

AIFA - Regions - Patient RepresentativesParliament all stakeholders

LEGISLATION/LITERATURE Ministerial Decree 11/2/97 Ministerial Decree

7/9/2017 PNMR Pharmacological treatments instrument 6

The participants in the work of the Tables

The participants at the tables were chosen for their expertise on the topics covered, trying to give a global representation of the main system stakeholders, from European to Italian institutions to the pharmaceutical industry.

Simona Aliprandi - Pipeline Partner, Roche Anna Ambrosini - AriSLA Delegate Giacomo Baruchello - Vice President and General Manager Region Europe South · Blueprint Medicines Simona Bellagambi - EURORDIS Delegate, UNIAMO Foreign Representative Stefano Benvenuti - Head of Public Affairs Telethon Barbara Bonamassa - Committee for Advanced Therapies (CAT) alternate member Italian Medicines Agency (AIFA) - Innovation and Pharmaceutical Strategy Division (Ex European Assessment Unit) Simone Boselli - Public Affairs Director - EURORDIS Rare Diseases Europe Loris Brunetta - President of the Thalassemic Liguri Association Agnese Cangini - Health economist - Member of the Executive Board at EunetHTA - AIFA Francesca Caprari - Market Access Alexion, AstraZeneca Rare Disease Rita Cataldo - Delegate ASSOBIOTEC FEDERCHIMICA Americo Cicchetti - Director of the High School of Economics and Management of Health Systems Maria Elena Congiu - MinSal - DG Health Planning Chiara Cordova - Conference of the Regional Councils Filippo Cristoferi - Chief of Staff & External Affairs AIP Erica Daina - Coordination of Rare Diseases Lombardy Region Francesco De Lorenzo - President F.A.V.O.

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Vincenzo Giustozzi - Market Access Lead - Medac Farma Angela Iano -
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of the Giambone Foundation Roberta Joppi - Regional Technical
Commission on Medicines, Pharmaceutical-Prosthetics-Devices
Directorate Doctors, Veneto Region Yllka Kodra - Medical Director - Office
5 - Essential levels of assistance, territorial and socio-health assistance -
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ATMP Forum Beppe Lanzillotta - Director Government Affairs Italy &
International Government Affairs and Policy at Alexion Pharmaceuticals
Giovanni Leonardi - General Director of Innovation and Research in
Healthcare - Ministry of Health Armando Magrelli - Vice Chair of the
Committee for Orphan Drugs (COMP), European Medicines Agency,
London Cristiana Marchese - ASS Delegate. RETINA Marco Marchetti -
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Pallara - President of SMA FAMILIES Riccardo Palmisano - President of
Assobiotec Federchimica Francesca Pasinelli - General Director - Telethon
Foundation Sandra Petraglia - Pre-Authorization Area Manager - AIFA
Paolo Pietrangelo - Conference of Regional Councils Lara Pippo - Head of
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Board of Directors - ASAMSI Elena Pompeo - Patient Partnership
Manager, Medical Affairs and Clinical Operations Department · Roche
Italia Concetta Quintarelli European Medicines Agency (EMA). Scientific
Committee members and experts

Angelo Ricci - President FIAGOP
Claudia Russo Caia - Patient Value & Access Head Takeda Italia S.p.A.
Zeno Righetti - Product Manager - Roche
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Massimo Scaccabarozzi - President Farmindustria
Annalisa Scopinaro - President of UNIAMO
Giovanna Scroccaro - President of the Prices and Reimbursement Committee, AIFA
Rossana Sovani - Head of Legal Public Affairs - LS CUBE Law Firm, VYTA delegate
Luisa Strani - Patient Advocacy Lead Alexion, AstraZeneca Rare Disease
Paola Torreri - Istituto Superiore di Sanità
Filippo Urso - SIFO Regional Secretary



UNIAMO Italian Federation of Rare Diseases

UNIAMO Italian Federation of Rare Diseases is the body representing the community of people with rare diseases.

It has been operating since 1999 for the protection and defense of the rights of people with rare diseases and their families, and has over 160 affiliated associations which are constantly growing.

Develop a constant dialogue with representatives of the institutions (Ministries, AIFA, Istituto Superiore di Sanità, Agenas, Regions, clinical reference centres, ERN network, GPs and PLS, scientific societies, etc.), researchers, private players representing the requests of people with rare disease and possible solutions.

It gives a voice to all the people who find themselves affected by a rare or ultra-rare disease, as well as those who are still looking for a diagnosis.

The sense of disorientation, uncertainty, loneliness, the pain felt when receiving a diagnosis of a rare disease are alleviated by the awareness that the Federation, together with all the Associations, makes every possible effort to improve the quality of life of the person and their his family members and caregivers.

Concrete support is given with the SAIO service (listening, information and orientation service) - aimed at individuals and associations -, with other support projects and with awareness-raising, promotion and protection of rights, advocacy in all the sectors, from research to bioethics, from health approaches to social supports.

You can support our action in many ways:

- making your professionalism available
- offering us pro-bono services
- with your 5x1000 (tax code 92067090495)
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The Ephemèrides of UNIAMO

The idea of a Uniamo editorial series is not new. However, a series of conditions had to be met for it to become reality.

In the search for a name that would characterize our publications we came across "effemèride".

The Treccani dictionary reports the following definition:

effemèride (or efemèride) s. f. [from lat. ephemeris -īdis, gr. ἔφημερίς -ίδος «diary», comp. of ἐπί «above» and ἡμέρα «day»]. -

1. a. Anticam., the books in which the king's actions were recorded were called ephemerides, first day by day (hence the name), then according to a broader chronological scheme. b. In full, diary, daily chronicle of events: but what more do I spend in giving you an e. of my life? (D. Bartoli).

2. In more recent times, the term has been used as the title of periodical publications, especially of a literary or scientific nature (never of political newspapers); for example, the literary Ephemèrides, which were printed in Rome from 1772 to 1795 and contained reviews of new books; the scientific and literary Ephemèrides for Sicily, which were published from 1832 to 1840.

3. Table or group of numerical tables, called e. astronomical (or even nautical, as they mainly serve the needs of navigation), which provide the coordinates of the stars (or other astronomical data variable over time) at pre-established and equal intervals, for example. from day to day or from hour to hour. By extension, also the books, generally published annually, which contain such collections.

Each of the three definitions contains an element that we felt close to us: the daily recording of documents, which reminds us of an ideal journey into pathology; the periodic publication, which responds to our wishes; the table that provides the coordinates, our aspiration and intent in the publication of these brochures.

The relative rarity of the use of this term, its feminine connotation, its originality given that the last person who used it dates back to 1840 for literary or scientific publications, further convinced us that we were made for each other for the other: Federation and effemèride, community of people with rare diseases and periodic publication that recounts a journey and tries to guide its route.

Here is therefore the beginning of a series that will follow the federation's activity by giving an account of the meetings and working groups set up on specific themes, and the fruit of their work.



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