ANNUAL

REPORT
2025

aelmhu

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01. LETTER *from the* PRESIDENT

2025 has been a turning point in the field of rare diseases and orphan medicines. It has not only been a year of progress but also a moment of redefining frameworks and priorities.

The European Union and Spain are advancing in a process of profound review of their regulatory and legislative framework in pharmaceutical matters. The European Pharmaceutical Strategy, the European Action Plan on Rare Diseases, as well as the legislative and regulatory initiatives being promoted in our country, such as the Draft Law on Medicines and Medical Devices or the update of the Rare Diseases Strategy, represent a unique opportunity to build a more agile, predictable, and equitable system.

However, this transformation process is not without complexity. The unique characteristics of rare diseases—their low prevalence, clinical heterogeneity, or frequent absence of therapeutic alternatives—require specific responses.

Therefore, as a country and as a sector, we must ensure that future legislative texts rigorously reflect and align with the reality of the needs of the healthcare system and people with rare diseases.

The data continue to remind us of the magnitude of the challenge. To date, only a small portion of rare diseases have treatments, and access times to orphan medicines in Spain remain an area for improvement. Reducing these gaps is not only a matter of efficiency but also of equity.

In this context, at AELMHU we have strengthened our role as an active agent in the healthcare ecosystem. Throughout this year, we have intensified our work as knowledge generators, promoting reports on access to orphan medicines and clinical trials in rare diseases. We have also strengthened institutional dialogue, contributing with proposals and evidence to consultation and decision-making processes, and actively participating in the Joint Committee of the Pharmaceutical Industry Strategy, the PERTE for Cutting-Edge Health, or the European Alliance for Research on Rare Diseases (ERDERA).

All of this is driven by a firm conviction: only through shared knowledge and collaboration is it possible to advance. Administrations, healthcare professionals, researchers, patient associations, and the pharmaceutical industry are called upon to jointly build responses that meet the challenges we face.

Nothing we do would be possible without the commitment and work of our members. They are the engine that has allowed AELMHU to establish itself as the reference interlocutor in the field of rare diseases in Spain.

My gratitude also extends to the patient associations, whose voice and experience are essential to guide our work. They represent the ultimate purpose of every proposal and initiative we undertake.

I also want to acknowledge the work of our technical team, working groups, strategic alliances, and collaborators, whose rigor and dedication allow our Association to continue adding value in an increasingly demanding and dynamic environment.

At AELMHU we are excited about the future. A future in which regulatory and scientific advances translate into real improvements for patients; in which innovation arrives more swiftly and equitably; and in which the healthcare system is capable of responding, with anticipation and sensitivity, to the particularities of rare diseases.

We will continue working with determination, through dialogue and collaboration, to help build that future and to ensure that every step we take brings us a little closer to our common goal: improving the lives of people living with a rare disease.

Beatriz Perales
President of AELMHU

02

ORPHAN AND ULTRAORPHAN MEDICINES IN SPAIN

Annual Access Report 2025

Access data for orphan medicines in 2025 maintain the positive trend observed in the last two years, according to our latest Access Report, with data as of December 31, 2025.

The **20 treatments reimbursed in Spain last year represent the second highest figure since 2020**, only surpassed by the historic maximum recorded in 2023 (21). Thus, at the end of 2025, the National Health System (SNS) reimburses a total of 103 medicines with orphan designation, 66% of the 156 authorized for marketing in the European Union (EU), which is eight percentage points higher than the previous year (58%).

At the European Union level, **18 medicines obtained marketing authorization from the European Commission in 2025**, improving on the 17 treatments authorized the previous year.

However, this increase has not been reflected in the new medicines obtaining National Code (NC) in Spain, a necessary prior step to request their inclusion in public reimbursement. Last year, **only 9 medicines obtained the NC**, 64% less than the 25 registered in 2024.

On the other hand, the Report indicates that in Spain **there are still 31 orphan medicines with National Code not reimbursed**, 15 fewer than in 2024 (46), and that 42% of them have been waiting more than three years for a favorable reimbursement resolution, compared to 39% recorded at the end of 2024.

In 2025, the average waiting time from when an orphan medicine obtains the National Code until the approval of its reimbursement remained at **23 months**, the same as in the last two years, highlighting the need to move towards a more agile and efficient model that helps reduce access times to therapeutic innovation for patients with rare diseases.

Finally, the Report also covers the evolution of advanced therapies aimed at treating rare diseases. In this context, **the reimbursement of 2 of these therapies during 2025 stands out**, a milestone that reaffirms the trend change that began in 2024, after none were reimbursed in 2022 and 2023.

Currently, **15 advanced therapies have marketing authorization in Europe**, of which 9 are reimbursed by the SNS and 14 already have a national code in Spain.

→ Read more

Annual Access Report 2025

2025 DATA

Key figures: orphan medicines in the EU and Spain

Orphan Medicines in the EU and Spain

156 With MA in the EU

134 With NC in Spain

103 Reimbursed by the SNS

What's New in 2025

18 New Marketing Authorizations

9 New National Codes

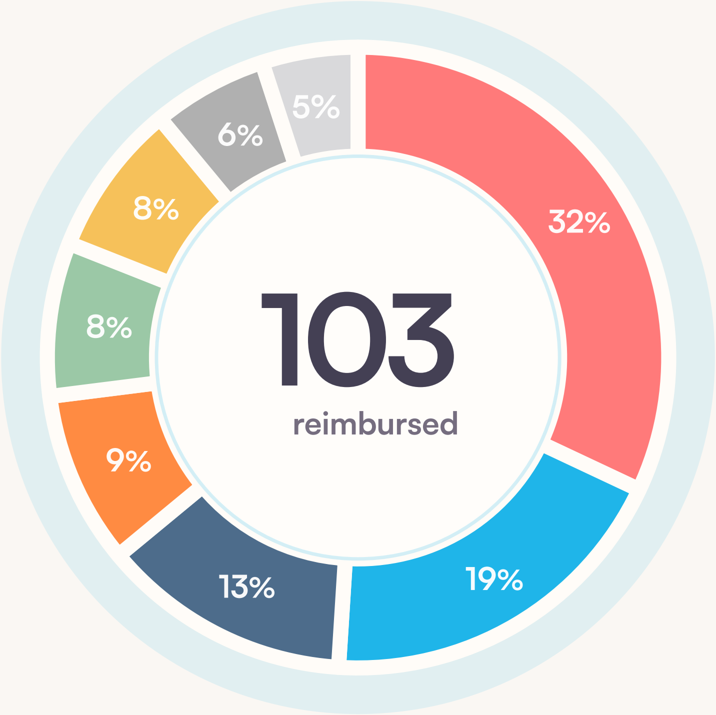
20 New Reimbursed Orphan Medicines

Source: Annual Access Report on Orphan Medicines – AELMHU. Data as of 12/31/2025.

THERAPEUTIC AREAS

Orphan medicines reimbursed by therapeutic area

103 orphan medicines reimbursed by the SNS by therapeutic area until December 31, 2025.



Oncology	32% of the total	33
Nervous system	19% of the total	20
Other	13% of the total	13
Metabolic	9% of the total	9
Hematology	8% of the total	8
Immunomodulators	8% of the total	8
Anti-infectives	6% of the total	6
Musculoskeletal system	5% of the total	5

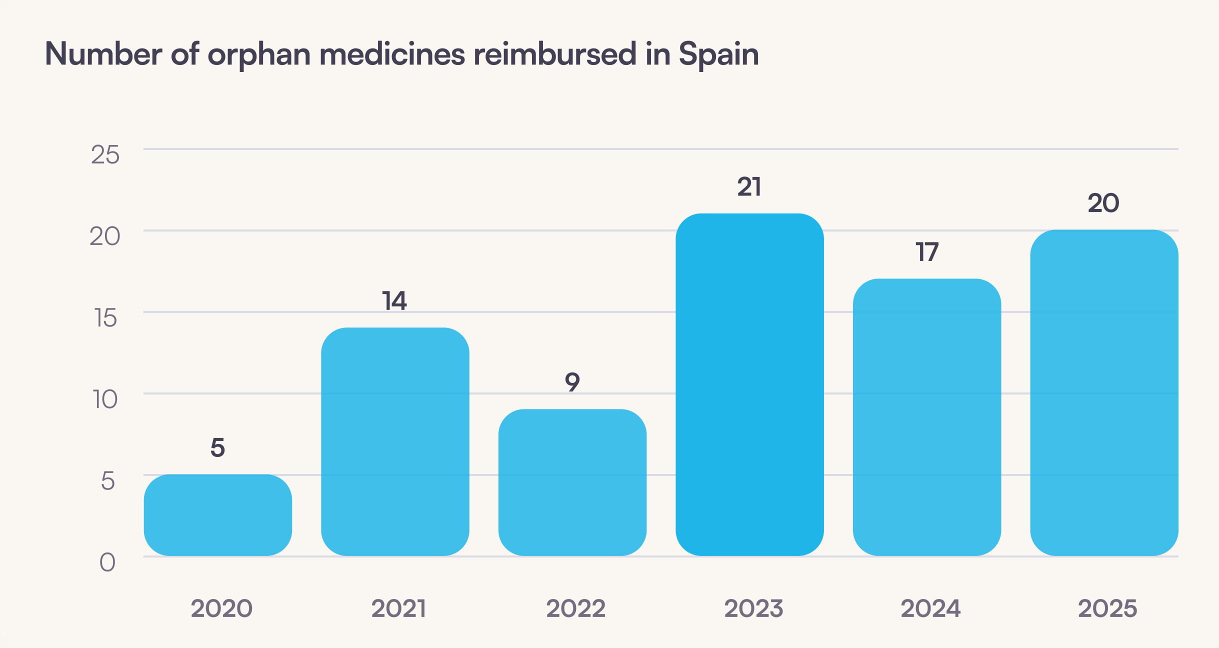
Source: Annual Access Report on Orphan Medicines – AELMHU. Data as of 12/31/2025.

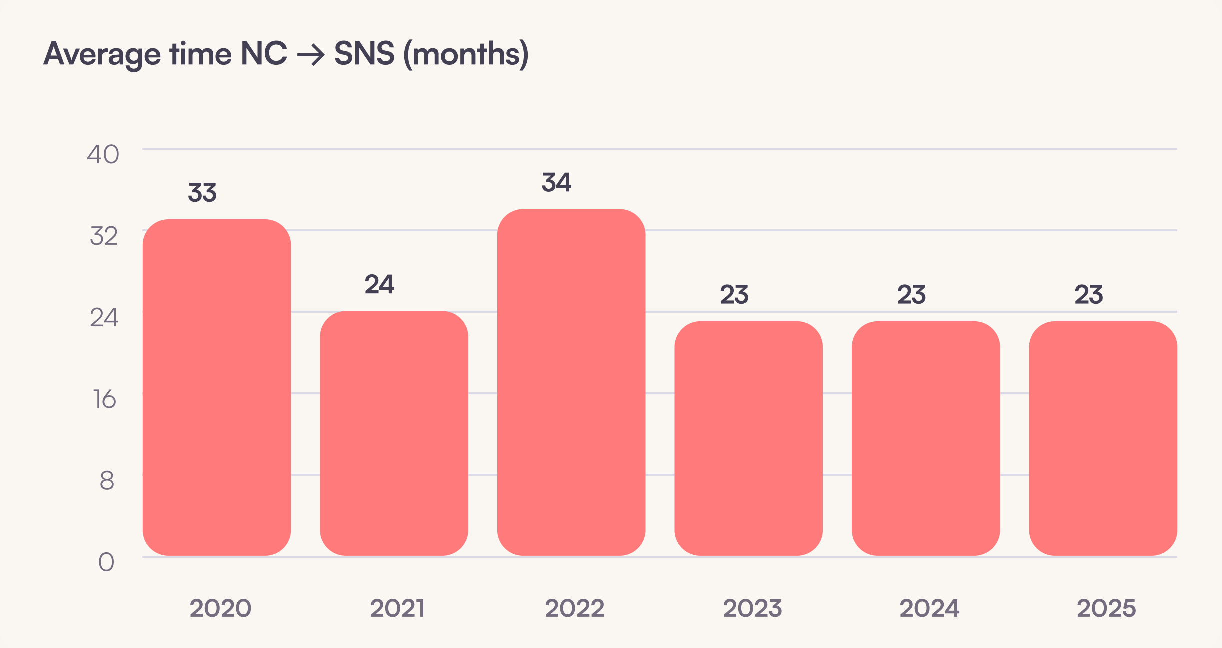
Annual Access Report 2025

ANALYSIS • 2020-2025

Reimbursement and access times over the last six years

Number of orphan medicines reimbursed per year and an average of months between the National Code and public reimbursement.





Source: Annual Report on Access to Orphan Medicines – AELMHU. Data as of 12/31/2025.

Annual Access Report 2025

ORPHAN MEDICINES WITHOUT APPROVED REIMBURSEMENT

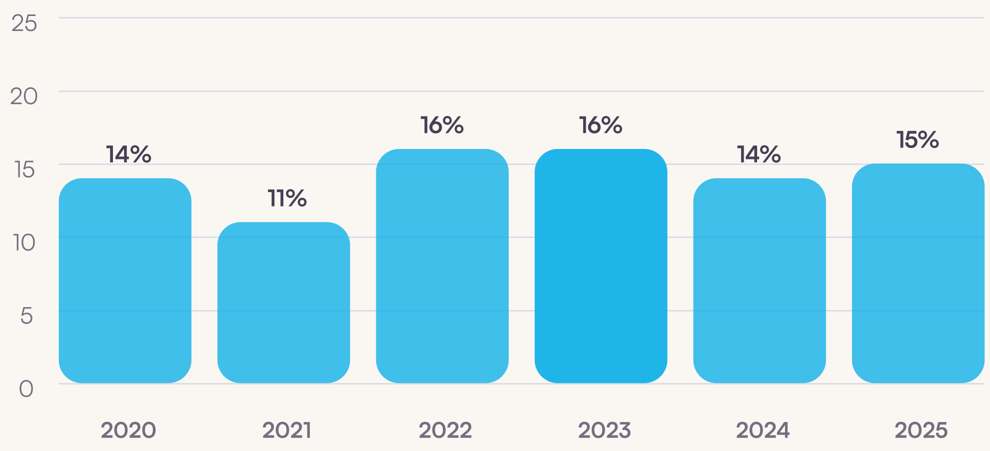
Orphan medicines not yet reimbursed and awaiting arrival to Spain

31 Orphan medicines with NC but not reimbursed

20 Not reimbursed by resolution

11 Under study or without reimbursement request

% Orphan medicines with MA but without NC · 2020-2025



22 Orphan medicines with MA but without NC

By years of waiting

50% Less than 1 year

9% Between 1 and 2 years

4.5% Between 2 and 3 years

36.5% More than 3 years

More than a third of the orphan medicines pending in Spain have already been waiting more than 3 years.

Source: Annual Report on Access to Orphan Medicines – AELMHU. Data as of 12/31/2025.

ADVANCED THERAPIES

Advanced therapies for rare diseases

46 Advanced therapies without MA

39 Included in the EMA's PRIME program

5 Academic

2 Under evaluation by the CHMP

15 Advanced therapies with orphan designation and MA

14 With NC

1 Without NC

OF THE 14 WITH NC

9 Reimbursed by the SNS

5 Not reimbursed by the SNS

2 New advanced therapies reimbursed in 2025 — consolidating the change in trend after 2022 and 2023, in which none were reimbursed.

Source: Annual Report on Access to Orphan Medicines – AELMHU. Data as of 12/31/2025.

03

WHO WE ARE

3.1 *WHAT it is AELMHU*

AELMHU is a non-profit association established in 2011 by a group of pharmaceutical and biotechnology companies, with a strong commitment to researching, developing, and commercializing innovative therapies to improve the quality of life of patients suffering from rare and ultra-rare diseases.

Our mission is to contribute to improving the health and quality of life of people affected by rare diseases, increasing the visibility and knowledge of these pathologies, highlighting research and its treatments, representing the associated companies, and defending their common interests.

We collaborate with patient associations, public administrations, and other sector agents, with the aim of contributing our knowledge and experience in the treatment of rare diseases.

As of the end of 2025, **AELMHU is made up of 27 associated companies**:

ALEXION AstraZeneca Rare Disease
Alnylam PHARMACEUTICALS
AMGEN
AOP HEALTH
argenx
ascendis pharma
avanzanite BIOSCIENCE
biocryst
Biogen
BIOMARIN
Chiesi
CSL
ESTEVE
IMMUNOCORE
insmed
IPSEN Innovation for patient care
ITALFARMACO
KYOWA KIRIN
NOVARTIS
pharma&
PTC THERAPEUTICS
sanofi
sobi
Takeda
ucb
ultragenyx
VERTEX

3.1.1 *New associates*

During 2025, **the Association has incorporated three new associated companies**, reaching the total of 27.

AVANZANITE BIOSCIENCE

Founded in 2022 and based in Amsterdam (Netherlands), the company partners with innovative biotechnology companies worldwide to drive the launch of medicines for rare diseases in Europe.

ESTEVE

Global pharmaceutical company founded in 1929 and headquartered in Barcelona. Focused on innovative and highly specialized solutions for unmet therapeutic needs.

PHARMA&

Global private company headquartered in Vienna (Austria) established in 2017, aiming to preserve access and promote the development of essential medicines worldwide, with a growing focus on hematology and oncology.

3.3 REPRESENTATIVE BODY

The Representative Body of the Association is the Board of Directors, responsible for its administration and representation. It is renewed every two years through a free vote of the members.

This body is composed of:

Beatriz Perales

PRESIDENT

Director of Market Access and Institutional Relations at Sobi Iberia.

María José Sánchez

VICE PRESIDENT

General Director of CSL Behring in Spain and Portugal.*

José Luis Moreno

SECRETARY

General Director in Italy, Spain, and Portugal of Ultragenyx.

Sergio Bullón

TREASURER

General Director in Spain of Alnylam Pharmaceuticals.

Iván Silva

BOARD MEMBER

Value & Access Director Southern Cluster & Country Manager at Kyowa Kirin in Spain.

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* María José Sánchez ceased to be a member of the Board of Directors on December 31, 2025, following her departure from CSL Behring.

04

WHAT WE DO

4.1 REPORTS

AELMHU aims to contribute to improving the health and quality of life of people living with rare diseases, **promoting their social visibility and highlighting the importance of research and equitable**, swift access to innovative treatments.

To achieve this goal, **the Association promotes the generation and dissemination of rigorous, reliable, and objective information**, enabling continuous monitoring of advances in research on new therapeutic alternatives and evaluating patients' access conditions to orphan medicines.

Throughout 2025, **AELMHU carried out intense and relevant activity in this area, resulting in the preparation of four specialized reports**. Three of them focused on access to orphan medicines (two quarterly reports and one annual report), which allow continuous analysis of the evolution of patient access to these treatments in Spain. The fourth report was dedicated to monitoring clinical trials in rare diseases, providing an updated view of the state of research and the development of new therapeutic options.

4.1.1 Access Reports

AELMHU publishes annual and quarterly reports on Access to Orphan Medicines in Spain, an analysis that has become a key document both in the pharmaceutical sector and in the field of rare diseases.

The reports analyze, among other aspects, **the number of available treatments, approval times, and the status of their reimbursement in Spain**, based on data obtained from public sources of the European Union and the Ministry of Health of the Government of Spain.

Thus, they offer a **comprehensive view of the journey that medicines follow from their designation as orphan medicines and their marketing authorization in the European Union**, to their incorporation into the Spanish market through the assignment of National Code and their reimbursement by the National Health System (SNS). The report examines the evolution between these milestones, the waiting times between each phase, and the regulatory and economic conditions applied.

Likewise, **the report includes information on medicines indicated for rare diseases that have lost their orphan designation but continue to be reimbursed**, as well as those that have received unfavorable reimbursement decisions. Another of the most relevant sections focuses on advanced therapies for rare diseases, analyzing their inclusion in European programs such as PRIME, their regulatory status, and the degree of access in our country.

INFORME
DE
ACCESO
2024

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asociación española de laboratorios de
medicamentos huérfanos y ultrahuérfanos

LOS MEDICAMENTOS
HUÉRFANOS EN ESPAÑA

Análisis de todos los MM.HH. con nombre comercial que tienen vigente la designación huérfana europea, a 31 de diciembre de 2024.

→ Read more

Ultimately, a thorough report on access to orphan medicines in Spain that is shared in full with AELMHU members, and in a more executive format, as a summary, with the rest of the stakeholders. **The publication of these reports generates great interest among experts, opinion leaders, the scientific community, associations, and institutions**, and achieves wide media coverage, reinforcing and consolidating AELMHU's role as the reference association of the pharmaceutical industry in the field of rare diseases and orphan medicines.

→ Read more

LOS MEDICAMENTOS HUÉRFANOS EN ESPAÑA
1er INFORME CUATRIMESTRAL DE ACCESO 2025

Situación de todos los Medicamentos Huérfanos (MM.HH.) con nombre comercial que tienen vigente la designación huérfana (OD), a 30 de abril de 2025

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LOS MEDICAMENTOS HUÉRFANOS EN ESPAÑA
2°INFORME CUATRIMESTRAL DE ACCESO 2025

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MM.HH. EN LA UE Y ESPAÑA

NUEVOS MM.HH. EN EL 2° CUATRIMESTRE DE 2025

9 Nuevas designaciones huérfanas con nombre comercial en la UE

8 Nuevas autorizaciones de comercialización en la UE

2 Nuevos códigos nacionales en España

14 Nuevos productos financiados por el SNS

LOS MEDICAMENTOS HUÉRFANOS EN ESPAÑA
2°INFORME CUATRIMESTRAL DE ACCESO 2025

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Situación de todos los Medicamentos Huérfanos (MM.HH.) con nombre comercial que tienen vigente la designación huérfana (OD), a 31 de agosto de 2025

4.1.2 *Clinical Trials Report*

The **Clinical Trials Report on Rare Diseases in Spain, published annually by AELMHU on the occasion of International Clinical Trials Day**, celebrated on May 20, highlights the importance of continuing to promote and drive innovation in Spain in the field of clinical trials and reaffirms the commitment of associated companies to the development of new therapies for rare diseases.

Likewise, **the report reflects the firm and sustained commitment of the pharmaceutical industry to research in rare diseases**, demonstrating a determined push towards innovation, knowledge generation, and the development of therapeutic solutions that address the unmet needs of patients.

This document, published by AELMHU since 2020, **has become a fundamental tool to promote debate and decision-making** aimed at ensuring that patients with rare diseases have access to more and better innovative treatments in the shortest possible time.

The report on 2024 data revealed that, during the past year, **9,294 clinical trials were authorized in Spain, of which 207 corresponded to rare diseases**, representing a 10% increase compared to 2023 data.

Additionally, **there was a 15% increase in the number of participants in clinical trials for rare diseases compared to 2023**. In total, in 2024, 3,659 people participated in this type of trial, of whom 602 were pediatric, representing 16% of the total.

The pharmaceutical industry established itself as the main driver of clinical trials for rare diseases in Spain, accounting for 95% of authorized studies.

INFORME
SOBRE
ENSAYOS
CLÍNICOS
PARA
ENFERMEDADES
RARAS
EN ESPAÑA
2024

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→ Read more

4.2 AELMHU MEETINGS

In 2025, **the Association continued to hold exclusive meetings for its members**, conceived as spaces for debate and reflection with key players in the healthcare and political sectors.

These forums **aimed to foster knowledge exchange, promote constructive debate, and share experiences, opinions, and ideas** around strategic issues of interest to the sector.

8 MEETINGS

+250 PARTICIPANTS

FEBRUARY

- **EUPATI Spain:** Roberto Saldaña, Innovation Director, and Mercedes Maderuelo, Head of Institutional Relations.

MARCH

- Carlos Martín Saborido, Director of the Health Technology Assessment Agency of the Carlos III Health Institute (ISCIII).

JUNE

- Javier Padilla, Secretary of State for Health, held at the headquarters of the Ministry of Health.
- Ana Rossignoli, Coordinator of the Health Technology and IPTs Assessment Group of the Spanish Agency of Medicines and Medical Devices (AEMPS).

SEPTEMBER

- Miguel Gómez-Pavón López, Deputy Director General of Strategy and Industrial Ecosystems of the Ministry of Industry and Tourism.
- Cristina Calatayud i Bassols and Jordi Pujadas, members of the Coordination and Strategy Unit of Medicines of the Catalan Health Institute (ICS).
- Carina Escobar, president of the Patient Organizations Platform (POP).

OCTOBER

- Lluís Alcover and Joan Carles Bailach, lawyers specializing in pharmaceutical law from the law firm Faus Moliner.

NOVEMBER

- César Hernández, general director of the Common Portfolio of Services of the National Health System and Pharmacy.


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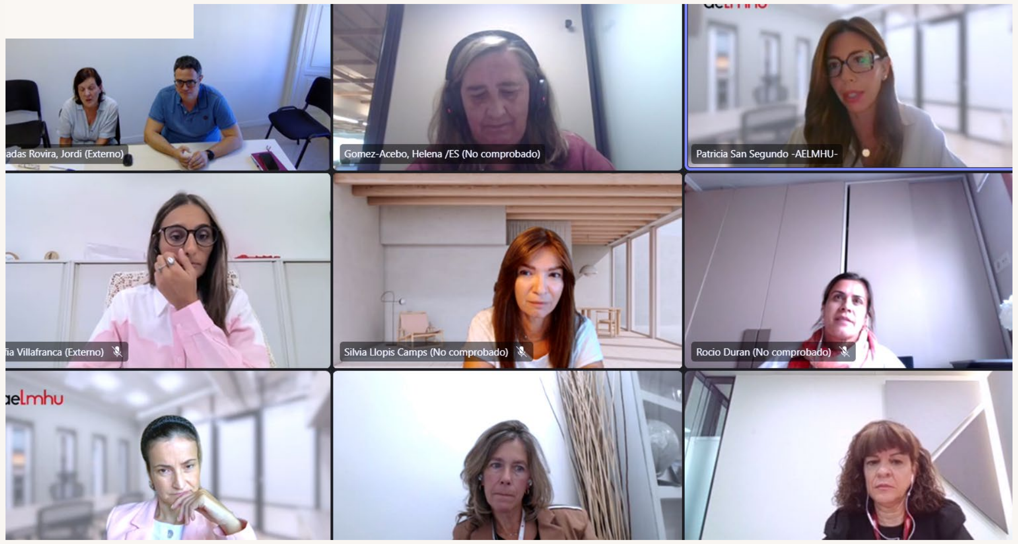
adas Rovira, Jordi (Externo)
Gomez-Acebo, Helena /ES (No comprobado)
Patricia San Segundo -AELMHU-
ia Villafranca (Externo)
Silvia Llopis Camps (No comprobado)
Rocio Duran (No comprobado)
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GOBIERNO DE ESPAÑA


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4.3 *TRAINING for MEMBERS*

In 2025, **AELMHU has launched a new training project aimed at its member companies**, with the goal of strengthening knowledge and updating in strategic areas for the sector. Thus, these initiatives respond to the Association's commitment to offer its members tools that facilitate decision-making and adaptation to an ever-evolving regulatory environment.

The first day, held on October 27 in Madrid, addressed **the challenges and opportunities of public procurement in the field of orphan and ultra-orphan medicines**, a key aspect to guarantee access to innovation within the National Health System. The session allowed for an analysis of how to apply current regulations and explore new formulas that promote more sustainable and value-oriented purchasing models.

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Zaragoza
1474

Leading experts in Administrative Law, such **as Carmen de Guerrero Manso and Gerardo García Álvarez**, both from the University of Zaragoza, offered a comprehensive view on issues such as the evolution towards strategic purchasing, the incorporation of environmental clauses, and the European perspectives that will shape the future in this field.

4.4 EVENTS

4.1.1 3rd Conference on Advanced Therapies for Rare Diseases

On March 25, AELMHU held its 3rd Conference on Advanced Therapies for Rare Diseases, **thanks to the sponsorship of Amgen, CSL Behring, PTC Therapeutics, Ultragenyx, and Vertex Pharmaceuticals**. The day brought together experts from the healthcare sector, such as patient associations, clinicians, managers, health economists, and representatives of the Public Administration, with the aim of fostering an open and constructive dialogue on advances in this type of treatment in Spain.

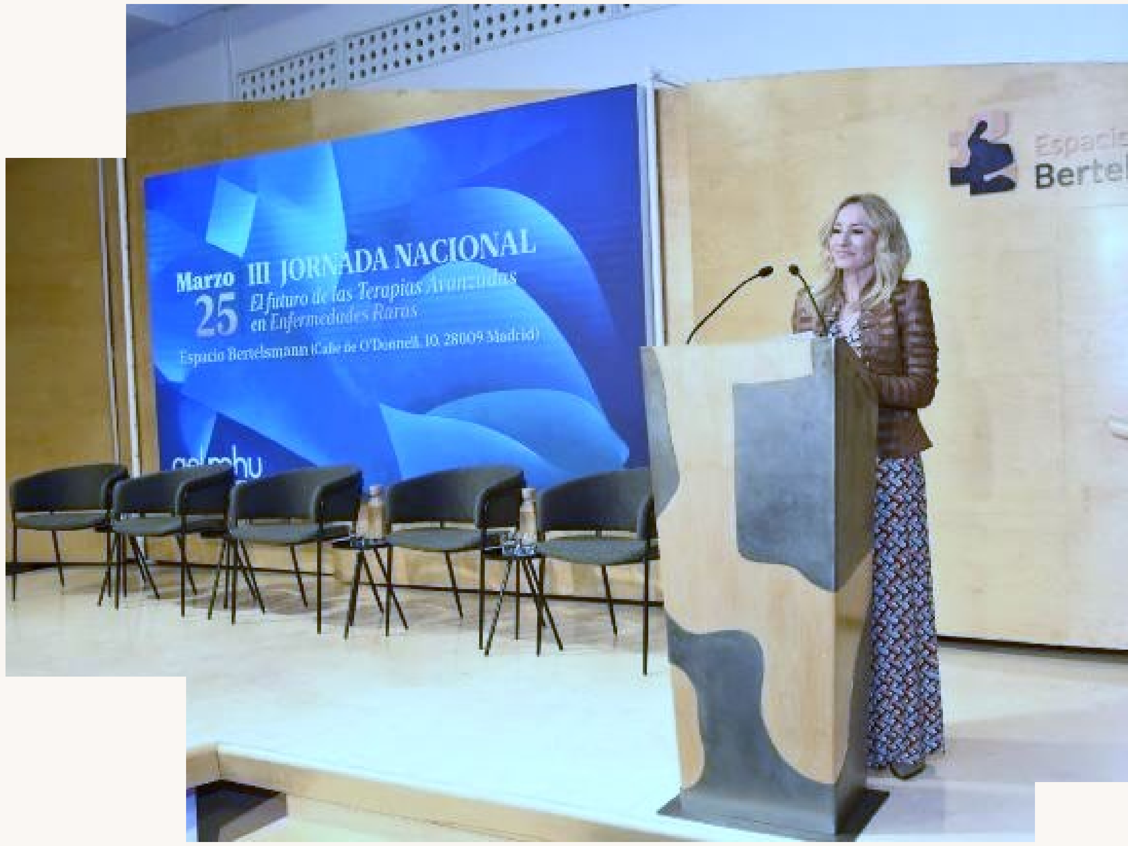
Beatriz Perales, president of AELMHU, was in charge of welcoming the event, whose institutional inauguration was carried out by **María Jesús Lamas**, director of the Spanish Agency of Medicines and Medical Devices (AEMPS).

Next, **Alejandro G. Solís, representative of the Health Technology Assessment and Therapeutic Positioning Reports Area of AEMPS**, gave the presentation 'Joint Clinical Evaluations: Keys for Innovation and Access to Advanced Therapies'.

The day continued with **two panel discussions featuring high-level speakers** who shared their experiences and perspectives on the future of advanced therapies.

The first, **titled 'Diagnosis of Rare Diseases: Neonatal Screening, Precision Medicine, and Data Management,'** moderated by Rubén Moreno, former Secretary General of Health and Consumption, included: Domingo González-Lamuño, former president of the Spanish Association for the Study of Inborn Errors of Metabolism (AECOM); Daniel de Vicente, board member of FEDER and EURORDIS and president of the patient association ASMD Spain; and María Luz Couce, head of the Neonatology Service and director of the Unit for Diagnosis and Treatment of Congenital Metabolic Diseases at the Clinical University Hospital of Santiago de Compostela.

The second panel, **titled 'Legislative Advances and Challenges in the Approach and Financing of Advanced Therapies for Rare Diseases,'** moderated by Jorge Mestre, health economist.



The speakers were **Carlos Martín Saborido**, director of the Health Technology Assessment Agency of ISCIII; **Nekane Murga**, coordinator of Advanced Therapies at Osakidetza; **José Luis Poveda**, coordinator of the Advanced Therapy Medicines Working Group of the Spanish Society of Hospital Pharmacy (SEFH); **Gerardo García-Álvarez**, principal investigator of the project 'Innovation for Cutting-Edge Health: Public Procurement, Technology, Environmental Sustainability, and Socioeconomic Factors'; and **Josep María Guiu**, director of the Pharmacy and Medication Area of the Consorci de Salut i Social de Catalunya (CSC).

As a closing remark, **Marian Corral, executive director of AELMHU**, highlighted the importance of updating the Advanced Therapies Approach Plan, involving autonomous communities, clinicians, researchers, and the pharmaceutical industry in this process.

The event was made possible thanks to the support of Amgen, CSL Behring, PTC Therapeutics, Ultragenyx, and Vertex Pharmaceuticals, partner companies of AELMHU.

→ Read more


Avances y retos en el diagnóstico de
con Terapias Avanzadas: cribado neonatal, medic
JORNADA NACIONAL
Moderador
Rubén Moreno
Daniel de Vicente
Vocal de la junta directiva de FEDER y de EURORDIS y presidente de la asociación de pacientes ASMD España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ultragenyx
VERTEX


III JORNADA NACIONAL
El futuro de las Terapias Avanzadas en Enfermedades Raras


Descanso


nzález-Lamuño
María Luz C
Daniel de Vicente
Vocal de la junta dire
FEDER y de EURORD
presidente de la asoc
de pacientes ASMD

Espacio
Bertelsmann


JORNADA
Moderador
Salud
Josep María Gu
Director del Área d
Farmacia de
Medicamento del
Consorci de Salut i
Social de Catalunya
(CSC)


Tecnologías Sanitarias
del Instituto de Salud
Carlos III
José Luis Poveda
Coordinador del Grupo
de Trabajo
Medicamentos de
Terapia Avanzada de la
Sociedad Española de
Farmacia Hospitalaria
(SEFH)
Gerardo García-
Álvarez
Investigador principal del
proyecto Innovación para
una Salud de Vanguardia:
Compra Pública,


Jorge Mestre
Economista de la
Salud
del Instituto de Salud
Carlos III
Josep María Guiu
Director del Área de
Farmacia de
Medicamento del
Consorci de Salut i
Social de Catalunya
(CSC)
José Luis Poveda
Coordinador del Grupo
de Trabajo
Medicamentos de

4.4.2 Training Workshop for Journalists on Rare Diseases

39 MEDIA MENTIONS

29 JOURNALISTS IN ATTENDANCE

Within the framework of the alliance signed with the National Association of Health Informers (ANIS), AELMHU promoted the organization of a new training workshop on rare diseases, **aimed at journalists specialized in health, with the goal of improving their knowledge and specialization on these pathologies and orphan medicines**.

Moderated by Susana Fernández, vice president of ANIS, the event was inaugurated by the vice president of AELMHU, María José Sánchez, who **provided an overview of the situation of rare diseases and their treatments in Spain** through the presentation of the main data from the Orphan Medicines Access Report for the first four months of 2025.

After her speech, the debate panel **'Challenges of rare diseases and their treatment from the regulatory, economic, research, and patient perspectives'** took place, featuring Isabel Motero, director of the Spanish Federation of Rare Diseases (FEDER); Eva Bermejo, director of the Rare Diseases Research Institute of the Carlos III Health Institute (ISCIII); Jorge Mestre, health economist; and Lluís Alcover, lawyer specializing in pharmaceutical law at Faus & Moliner.

Read more

4.4.3 AELMHU Awards 2025

The AELMHU Awards, held annually, have become **a benchmark in the rare diseases and orphan medicines sector**.

In 2025, during its seventh edition, **the Association once again recognized the work of professionals, organizations, associations, and institutions** that contribute to improving healthcare and the quality of life of people with rare diseases.

Participation was open to individuals and legal entities dedicated to research, dissemination, or awareness-raising about this type of disease with residence or activity in Spain, and **the categories distinguished can be seen on the right side of this page**.

The jury meeting took place online on September 15, and the winners were announced on September 18. After the winners were announced, **the Association began working on organizing the award ceremony, which was held on November 5 in Madrid**.

AWARD FOR THE BEST CARE PROJECT ON RARE DISEASES:

«Comprehensive care and rehabilitation program for people with hemophilia and congenital coagulopathies in Andalusia (PIAH)» by the Andalusian Hemophilia Association

AWARD FOR THE BEST PROFESSIONAL RESEARCH CAREER IN THE FIELD OF RARE DISEASES:

Dr. Pascual Sanz Bigorra

AWARD FOR THE BEST DISSEMINATION AND/OR AWARENESS PROJECT ON RARE DISEASES:

«Los Bufones de Velázquez» by the Association of People with Achondroplasia and Other Skeletal Dysplasias.

AWARD FOR THE BEST WORK IN COMMUNICATION, DISSEMINATION AND/OR AWARENESS ON RARE DISEASES:

Dr. Patricia Smeyers Durá

AELMHU HONORARY AWARD 2025:

Dr. Mª Luz Couce Pico – award granted by the members of AELMHU.

The 2025 edition featured, as in previous editions, **an external and independent jury, composed of the following professionals of recognized prestige in rare disease research, in the field of health and communication**:

MR. JESÚS AGUILAR

President of the General Council of Official Colleges of Pharmacists.

MR. DIEGO AYUSO

Secretary General of the General Council of Nursing (CGE).

MS. EVA BERMEJO

Director of the Rare Diseases Research Institute at the Carlos III Health Institute (ISCIII).

MS. SUSANA FERNÁNDEZ

Vice President of the National Association of Health Informers (ANIS).

MR. JORGE FRANCISCO GÓMEZ

Coordinator of the Rare Diseases Group of the Spanish Society of Internal Medicine (SEMI).

MR. DOMINGO GONZÁLEZ-LAMUÑO

Former president of the Spanish Association for the Study of Congenital Metabolic Disorders (AECOM).

MR. VICENTE MARTÍN

Member of the Board of Directors of the Spanish Society of Primary Care Physicians (SEMERGEN).

MR. JORGE MESTRE

Health economist.

MR. JOSÉ MARÍA MILLÁN

Scientific director of the Networked Biomedical Research Center for Rare Diseases (CIBERER).

MR. FRANCESC PALAU

Pediatrician and medical geneticist. Distinguished Researcher, Sant Joan de Déu Hospital and SJD Research Institute. Group Leader at CIBERER.

MS. BELÉN PÉREZ

Professor of Biochemistry and Molecular Biology in the Department of Molecular Biology at the Autonomous University of Madrid (UAM).

MS. RAQUEL SÁNCHEZ

Lawyer specializing in Health Law.

MS. NÉBOA ZOZAYA

First Vice President of the Health Economics Association (AES).

MS. MARIAN CORRAL

Director of AELMHU (with voice, but without vote).

AWARD CEREMONY

Journalist **José Ribagorda was in charge of hosting the award ceremony**, held at the Bertelsmann Space in Madrid.

Beatriz Perales, president of AELMHU, welcomed the event by thanking the awardees and the rest of the nominees for their **"years of work, effort, and commitment to science and to patients with rare diseases."**

Furthermore**, she called for dialogue among all sector agents so that these pathologies become a "priority area" in health policies**, as outlined in the Pharmaceutical Industry Strategy, aiming to position Spain as "a European benchmark in treatments for rare diseases, which will not only benefit patients but also drive scientific progress, the generation of quality employment, and sustainable growth throughout the country."

Subsequently, **Raquel Yotti, commissioner of the PERTE for Advanced Health of the Ministry of Science**, Innovation and Universities, gave the institutional opening of the event, recalling the importance of addressing the unmet needs of people with rare diseases, since “delays in diagnosis have very profound implications for each of these patients.” She also thanked AELMHU for its involvement with the PERTE and with each of its strategic priorities.

The evaluation and selection process of the awardees was presented by **Susana Fernández, vice president of ANIS, representing the jury**, who highlighted the high quality of the 70 applications submitted in this edition of the AELMHU Awards, and encouraged any organization, association, or person involved in the rare diseases sector to submit their proposal to participate in future editions.

The awardees of the 2025 edition were:

AWARD FOR THE BEST CARE PROJECT ON RARE DISEASES

It was awarded to the Andalusian Hemophilia Association (ASANHEMO) for its **"Comprehensive care and rehabilitation program for people with hemophilia and congenital coagulopathies in Andalusia (PIAH),"** which offers specialized physiotherapy, psychosocial care, therapeutic education, and support for patient autonomy, with a special focus on children, youth, and people in rural areas.

The award was received by **Moisés González, vice president of ASANHEMO, and Matilde Mora, social worker of the association**, and was presented by Eva Bermejo, director of the Rare Diseases Research Institute of ISCIII. The awardees emphasized that this recognition was "a validation of a way of understanding healthcare that does not wait for the patient to go to the hospital but goes out to meet them." "This award will drive us to reach those who still need us. And it is proof that healthcare with soul works," said Matilde Mora.

AWARD FOR THE BEST PROFESSIONAL RESEARCH CAREER IN THE FIELD OF RARE DISEASES

It was awarded **to Dr. Pascual Sanz Bigorra, Research Professor at the Spanish National Research Council (CSIC) in the Nutrient Signaling Unit of the Institute of Biomedicine of Valencia (IBV),** for his significant contribution in this field, especially in the study of rare diseases such as Lafora disease, an ultra-rare form of progressive myoclonic epilepsy.

After receiving the award from **Jorge Mestre, health economist, and Néboa Zozaya, first vice president of the AES,** Dr. Sanz Bigorra emphasized the importance of continuing to focus on research into rare and ultra-rare diseases like Lafora disease, an ultra-rare form of progressive myoclonic epilepsy in which his research group specializes.

AWARD FOR THE BEST PROJECT IN DISSEMINATION AND/OR AWARENESS-RAISING ABOUT RARE DISEASES

The winner was **“Los Bufones de Velázquez,” from the Association of People with Achondroplasia and other Skeletal Dysplasias in Spain (ADEE).** It is an audiovisual campaign aimed at combating the stigmas and prejudices that still persist in society towards people with achondroplasia, through the reinterpretation of the portraits of jesters painted by Diego Velázquez in the 17th century.

With **José María Millán, scientific director of CIBERER, and Belén Pérez, Professor of Biochemistry and Molecular Biology at the Department of Molecular Biology of UAM**, presenting the award, Carolina Puente, president of ADEE, and its treasurer, Sonia Sestelo, accepted it. “Our campaign wanted to show how, even after many centuries, people with achondroplasia and other skeletal dysplasias still carry certain prejudices and a social burden that is not fair at all,” explained the president of ADEE after receiving the award.

AWARD FOR THE BEST WORK IN COMMUNICATION, DISSEMINATION, AND/OR AWARENESS-RAISING ABOUT RARE DISEASES

It was awarded to **Dr. Patricia Smeyers Durá, a specialist in Neurology and Clinical Neurophysiology at the La Fe University and Polytechnic Hospital of Valencia**, for her project of children's stories about epilepsy, in which she uses accessible and educational language to help destigmatize the perception of this disease among society.

The doctor received the award from **Vicente Martín, a member of the SEMERGEN Board of Directors, and Susana Fernández, vice president of ANIS**, and dedicated her acceptance speech to highlighting the importance of incorporating children's perspectives in rare diseases: "Only from childhood innocence can we create and grow."

AELMHU HONORARY AWARD 2025

Chosen by all AELMHU Associates, it was awarded to **Dr. María Luz Couce Pico, scientific director of the Health Research Institute of Santiago de Compostela (IDIS),** for her leadership and long professional career in the approach, diagnosis, research, and training on rare diseases, especially in the areas of neonatal screening and genetic diagnosis.

Dr. Couce received the award from **members of the association's Board of Directors and the president of the Spanish Federation of Rare Diseases (FEDER), Juan Carrión.** She then gave an inspiring speech in which she addressed young doctors and researchers: “Believe in science, dream of transforming the natural history of rare diseases, and never forget that vocation and cooperation are the true engines of progress.”

The gala was closed by **Contrabandeando, a musical group that blends typical Latin American folk music with contemporary sounds** and includes several members with family affected by rare diseases. In their performance, they played ‘Amor,’ a song dedicated to families who, like them, live with these pathologies, and ‘Que nada borre tu risa.’

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4.5 *WORKING GROUPS*

The **working groups constitute one of the strategic pillars of action of AELMHU** and the main engine around which an essential part of the Association's activity is organized.

Their work is key to **consolidating AELMHU's positioning as a reference agent in the field of orphan and ultra-orphan medicines and rare diseases**, through the generation of knowledge, the promotion of multisectoral dialogue, and the development of initiatives and value proposals aimed at addressing the main challenges of the ecosystem. They also actively contribute to the monitoring and analysis of institutional and regulatory initiatives impacting the sector.

Through a transversal and collaborative approach, **the working groups address the priority areas for the Association**, fostering active participation of member companies and strengthening dialogue with the main institutional agents linked to the field of rare diseases.

During 2025, **the Association maintained its work structure organized into four strategic groups**:

70 MEMBERS

+30 MEETINGS

01 **ACCESS, PRICE, AND REIMBURSEMENT**

02 **ADVANCED THERAPIES FOR RARE DISEASES**

03 **POLITICAL AFFAIRS AND LEGISLATIVE AGENDA**

04 **CO-CREATION WITH PATIENT ASSOCIATIONS**

Composed of **representatives from the associated companies and coordinated by technical experts with the support of two spokespersons**, the groups meet monthly to share information, analyze the context, and discuss matters of common interest. Their activity focuses on identifying strategic areas of action, promoting relevant projects, preparing position papers, and designing and implementing initiatives aligned with the objectives set by the Strategic Plan approved by the General Assembly.

Additionally, **specific and ad hoc groups were organized, as well as mixed working groups**, which allow for broader participation of the Association and focused addressing of specific topics or strategic projects, integrating different stakeholders according to the nature of each initiative.

The AELMHU working group model constitutes a differentiating element by **addressing in a 360º manner all critical dimensions related to rare diseases**: regulatory, clinical, research, access, financing, public policies, and patient relations aspects.

Throughout 2025, **the high commitment of the associated companies to these groups was reaffirmed, exceeding 70 active members and holding more than 30 meetings**, demonstrating their central role in the strategy and daily operation of the Association.

RESPUESTA A LA CONSULTA PÚBLICA PREVIA

CONSULTA PÚBLICA PREVIA SOBRE EL PROYECTO DE ORDEN POR LA QUE SE ACTUALIZA LA CARTERA COMÚN DE SERVICIOS DE SALUD PÚBLICA

aelmhu

RESPUESTA al ANTEPROYECTO de LEY de ORGANIZACIONES de PACIENTES

aelmhu

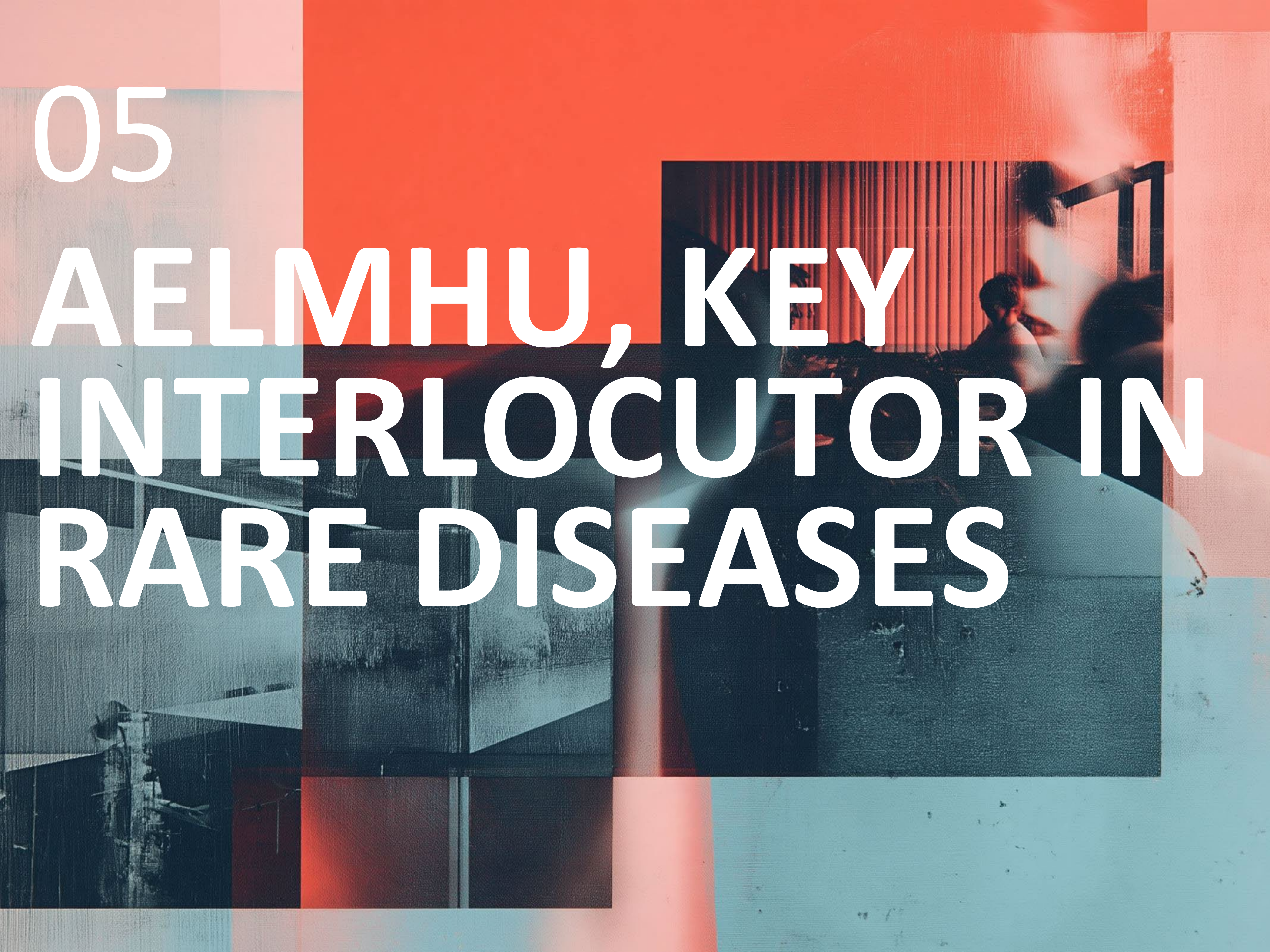
Nine strategic documents were promoted by the groups, addressing key topics such as: regional strategies for rare diseases, implementation of national neonatal screening, transition of patients from pediatric to adult age, the Profarma Plan, or the impact of United States commercial policies on the pharmaceutical sector.

These documents served **as a basis for dialogue with key decision-makers, allowing the Association's position on specific issues to be conveyed in a well-founded manner**, analyze specific situations, and propose alternatives to current challenges.

Additionally, **several groups worked transversally to develop and agree on documents with strategic recommendations**, facilitating AELMHU's active participation in consultations and public hearings at regional, national, and European levels, such as positions on the Draft Law on Medicines and Medical Devices or on the Advanced Therapies Approach Plan.

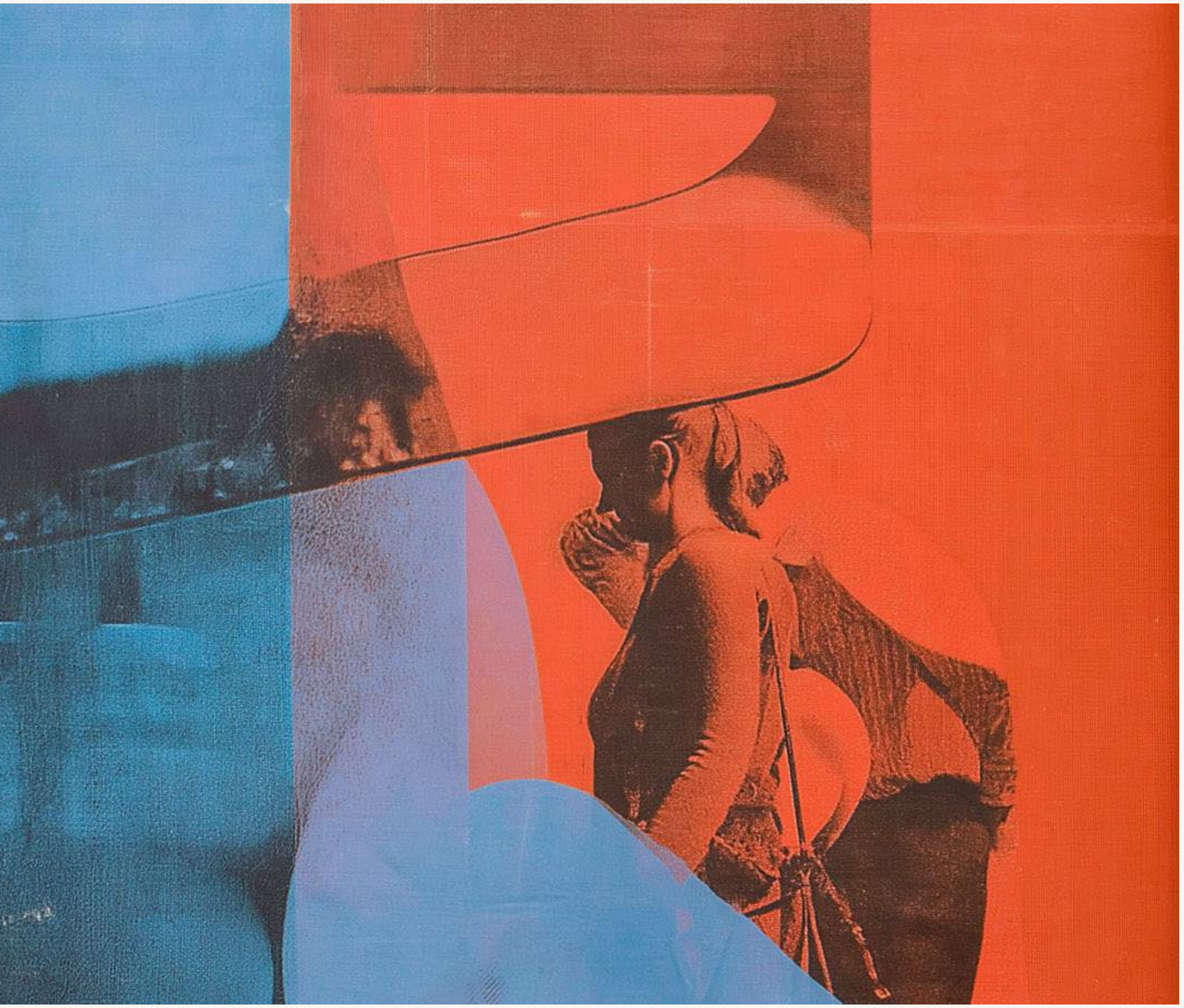
The working groups also **played a key role in the relationship with the Spanish Federation of Rare Diseases (FEDER) and other patient associations**, collaborating on joint initiatives such as interviews linked to anniversaries or quarterly meetings promoted by the Co-creation working group, as detailed in section 5.5 of this report.

The activity carried out within the framework of the working groups during 2025 **has strengthened AELMHU's capacity to anticipate challenges, build consensus, and actively contribute to driving key initiatives for the rare diseases ecosystem**. Likewise, these spaces continued to play an essential role in channeling expert knowledge, identifying shared priorities, and promoting proposals aligned with the sector's evolution and needs.



05

AELMHU, KEY INTERLOCUTOR IN RARE DISEASES



Throughout 2025**, AELMHU consolidated its position as the leading association of the pharmaceutical industry specialized in orphan medicines and other treatments for rare diseases**, strengthening its relationship with the main key players in the sector, both public and private.

In line with the Strategic Plan, **the Association intensified its political and institutional agenda, both at the national and regional levels, aimed at identifying and fostering new collaboration opportunities**. This strategic approach resulted in active participation in regulatory processes, public consultations, and strategic forums, strengthening its role as an expert and trusted interlocutor before administrations, patient associations, and sector entities.

Additionally, **AELMHU reinforced its presence in strategic European and national initiatives for the sector, contributing proposals that highlight research, innovation, and access to treatments for rare diseases**. Thanks to this approach, the Association has established itself as a technical and strategic reference, capable of generating constructive dialogue and offering effective solutions in a complex and constantly evolving environment.

5.1 *PARTICIPATION in CONSULTATIONS and PUBLIC HEARINGS*

In this regard, the Association, under the leadership of the technical team and in collaboration with working groups and representatives of member companies, **analyzed 19 consultations and public hearings at the regional, national, and European levels**.

Of these, **AELMHU participated in 12**, and for the remaining seven, it was decided not to make contributions because they were not directly applicable to the Association's objectives or because our position was already reflected.

Below are each of these contributions with their corresponding reference and direct access to the linked content.

19 CONSULTATIONS AND PUBLIC HEARINGS ANALYZED

12 SUBMITTED PROPOSALS

RESPUESTA A LA CONSULTA PÚBLICA PREVIA
PROYECTO DE REAL DECRETO POR EL QUE SE REGULAN LOS PROCEDIMIENTOS DE FINANCIACIÓN Y PRECIO DE LOS MEDICAMENTOS
aelmhu

Draft Royal Decree
regulating the financing and pricing procedures of medicines

→ Read more

RESPUESTA A LA AUDIENCIA PÚBLICA
PROYECTO DE DECRETO POR EL QUE SE REGULA LA ATENCIÓN TEMPRANA EN LA COMUNIDAD DE MADRID
aelmhu
asociación española de laboratorios de medicamentos huérfanos y ultrahuérfanos

Draft Royal Decree
regulating early care in the Community of Madrid

→ Read more

RESPUESTA A LA CONSULTA PÚBLICA PREVIA
CONSULTA PÚBLICA PREVIA SOBRE EL PROYECTO DE ORDEN POR LA QUE SE ACTUALIZA LA CARTERA COMÚN DE SERVICIOS DE SALUD PÚBLICA
aelmhu

Draft Ministerial Order
updating the Common Portfolio of Public Health Services related to information and epidemiological surveillance, population screening programs for neonatal and colorectal cancer, and other sections of the annexes of Royal Decree 1030/2006

→ Read more

ALEGACIONES al ANTEPROYECTO de LEY de los MEDICAMENTOS y PRODUCTOS SANITARIOS

Preliminary Draft Law
on medicines and medical devices

→ Read more

Public Consultation
on rare diseases by the Public Health Commission of the European Parliament

RESPUESTA a la
CONSULTA
PÚBLICA del
ANTEPROYECTO
DE LEY DE SALUD
PÚBLICA DE LA
COMUNIDAD DE
MADRID

Draft Bill
of public health of the Community of Madrid

→ Read more

Estrategia
de la Industria
Farmacéutica
2024-2028

Royal Decree
establishing the Interministerial Committee for monitoring the Pharmaceutical Industry Strategy

→ Read more

CONSULTA
PÚBLICA PREVIA
del
ANTEPROYECTO
de LEY de SALUD
DIGITAL

Draft Law
of digital health

→ Read more

RESPUESTA al TRÁMITE de AUDIENCIA e INFORMACIÓN PÚBLICA que **ACTUALIZA la CARTERA de CRIBADO NEONATAL y el COMITÉ ASESOR en GENÉTICA**

Draft Order
that updates the Neonatal Screening Portfolio and the Advisory Committee on Genetics

→ Read more

RESPUESTA al ANTEPROYECTO de LEY de **ORGANIZACIONES de PACIENTES**

Draft law
of patient organizations

→ Read more

PROYECTO de ORDEN por la que ACTUALIZAN LOS ANEXOS I, II Y III del **REAL DECRETO 1207/2006, de 20 de OCTUBRE, por el que se REGULA la GESTIÓN del FONDO de COHESIÓN SANITARIA**

Draft Order
updating annexes I, II and III of Royal Decree 1207/2006, of October 20, which regulates the management of the Health Cohesion Fund

→ Read more

RESPUESTA al TRÁMITE de AUDIENCIA e INFORMACIÓN PÚBLICA del **ANTEPROYECTO de LEY del PRINCIPADO de ASTURIAS de SALUD PÚBLICA**

Draft bill
of public health of the Principality of Asturias

→ Read more

5.2 *INSTITUTIONAL MEETINGS*

Throughout 2025, **AELMHU steadily strengthened its institutional relations through a series of meetings with senior representatives from the executive branch**, both at the national and regional levels. These strategic interactions were essential to highlight the work of the Association, convey its priorities, and reinforce its positioning as a technical and reference interlocutor in the field of orphan medicines and rare diseases.

These meetings **constituted strategic spaces to communicate AELMHU's messages in a structured and well-founded manner**, endorsing its voice as a technical and strategic actor in the healthcare ecosystem. The key projects, including the Reports on Access to Orphan Medicines, the Clinical Trials Report for Rare Diseases, the position papers, and contributions to public consultations, served as decisive tools to demonstrate the Association's technical knowledge and strengthen its capacity for dialogue with institutions.

Some of the meetings were:

CÉSAR HERNÁNDEZ

General Director of the Common Portfolio of SNS Services and Pharmacy

LAURA QUINTANILLA

Head of Division of the Advanced Therapies Unit, Health Department of the Community of Madrid

MARÍA FERNÁNDEZ

Deputy General Director of Healthcare Quality, Directorate General of Public Health

CRISTINA CALATAYUD AND JORDI PUJADAS

Members of the Coordination and Strategy Unit of Medicines, Catalan Institute of Health (ICS)

LOURDES GIL

Head of the Pharmaceutical Technologies Area, Ministry of Industry and Tourism

During these meetings, **proposals for improvement in the political, regulatory, and institutional fields were discussed**, deepening a constructive dialogue that facilitated cooperation and the identification of effective solutions. This interaction strengthened ties with the different agents of the healthcare system and enhanced AELMHU's capacity to influence the definition of policies, regulatory strategies, and programs that promote innovation and access to treatments for patients with rare diseases.

Thanks to this approach, **the Association strengthens its capacity to generate consensus and provide concrete solutions that directly contribute to improving the quality of life of patients**. Its participation in these spaces underscores its relevance in a complex and constantly evolving healthcare environment, reflecting its commitment to excellence, innovation, and sector advancement.

5.3 *SECTORAL REPRESENTATION BODIES*

5.3.1 *Pharmaceutical Industry Strategy*

In 2024, the Pharmaceutical Industry Strategy 2024–2028 was published, **an initiative of the Government of Spain aimed at reinforcing the role of the pharmaceutical sector as a key driver of public health, innovation, and economic development**.

AELMHU is an active part of the Strategy, along with other associations and sector employers' organizations, contributing its knowledge on the particularities of orphan and ultra-orphan medicines. Thus, the participation of the Association represents institutional recognition of the work it has been carrying out since its establishment to improve access to treatments for patients with rare diseases.

During 2025, **AELMHU has participated in five meetings of the Joint Committee of the Strategy** (3 of the Committee and 2 of the working group), collaborating in the development and implementation of the measures included in the text, through technical dialogue and joint work with public administrations, the private sector, and society.

The Strategy is structured around three fundamental axes: access and sustainability; the promotion of research and innovation; and competitiveness and industrial sustainability. Additionally, it identifies strategic therapeutic areas such as rare diseases, chronic conditions, therapeutic repositioning, antibiotic resistance and emerging infectious diseases, personalized precision medicine, and advanced therapies.

5.3.2 *PERTE for Cutting-Edge Health*

This year**, AELMHU has also joined as an active member of the PERTE Alliance for Cutting-Edge Health**, along with representatives from public administrations, scientific societies, business associations, and patient groups,

This PERTE is a strategic initiative of the Government of Spain that seeks to transform the healthcare system through science, innovation, and digitalization. Its goal is to improve prevention, diagnosis, treatment, and rehabilitation of patients in a personalized manner, incorporating technologies such as precision medicine, advanced therapies, and artificial intelligence.

It **includes 54 different actions carried out by five ministerial departments** and includes reimbursement through specific programs for clinical research projects aimed at medicines development in the academic field, or support for industrial innovation and sustainability projects in the pharmaceutical and medical products sector.

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Since our incorporation in April**, the Association has participated in a working session and a meeting of the Alliance**, in which the actions already carried out and in progress, the milestones achieved, the ongoing strategic objectives, and interministerial coordination were presented. In the working session, titled "The future of R&D&I in health as a lever for health, economic, and industrial transformation in Spain," the state of the R&D&I and digitalization ecosystem in Spain was defined, as well as proposals for future investment priorities were collected.

5.3.3 ERDERA

AELMHU joined in January the national working group of the European Alliance for Rare Diseases Research (ERDERA), which aims to advance research on prevention, diagnosis, and treatment of the millions of patients with rare diseases in Europe.

ERDERA brings together more than 170 organizations from 37 countries from the public and private sectors, including the main research institutes, patient associations, European research infrastructures, pharmaceutical and technology companies, public reimbursement agencies, non-profit foundations, regulatory institutions, health authorities, hospitals, universities, and medical societies.

With **an estimated total budget of 380 million euros until 2031**, ERDERA aims to have a significant impact on rare diseases by supporting research to develop new treatments and diagnostic pathways, leveraging the potential of health and research data, artificial intelligence (AI), and digital technologies.

AELMHU participates in ERDERA through the Spanish Working Group, which has met four times throughout 2025, advancing the objectives, priorities, and activities to be carried out within the framework of this European project.

5.3.4 *Intersectoral platform on urban wastewater*

During 2025, **AELMHU actively participated in the intersectoral platform created in response to the European Directive on the treatment of urban wastewater**. This platform, created and integrated by other sector associations, aims to promote coordination among the different stakeholders involved and facilitate a common approach to the technical, environmental, and regulatory challenges posed by the regulation.

The workspace **allows for structured dialogue between business associations, public administrations, and competent bodies**, fostering the exchange of information and joint analysis of the directive's implications.

Within this framework, **AELMHU has contributed its specific perspective on orphan and ultra-orphan medicines**, emphasizing the importance of considering their particularities in wastewater management.

The Association has **highlighted the possible direct and indirect costs that the implementation and development of the directive could entail at the national level**, both for the pharmaceutical industry and the healthcare system, including aspects related to waste management, adaptation of industrial processes, and ensuring the traceability of medicines.

Thanks to this participation, **AELMHU has contributed to ensuring that European and national policies reflect the reality and challenges of the sector**, promoting a balanced approach that allows environmental protection without compromising innovation, accessibility to treatments, and the sustainability of the healthcare system.

5.4 PARTNERSHIPS

Establishing partnerships and collaboration agreements is essential to strengthen the capacity for action in the field of rare diseases. Collaboration with other institutions and organizations, such as foundations, universities, and entities in the healthcare and social sectors, opens new opportunities to advance research, the development of innovative treatments, and the improvement of patients' quality of life.

Therefore**, the Association continued to carry out activities through collaboration agreements** signed in previous years, such as the case of the General Council of Pharmaceutical Colleges (CGCOF).

During 2025**, six video councils on "Orphan Medicines and Pharmacy" were launched** on the CGCOF YouTube channel and were later disseminated on the social networks of both entities, in order to offer educational and awareness content about these pathologies and orphan and ultra-orphan medicines.

In addition, **AELMHU signed two new collaboration agreements during 2025**.

→ Read more

5.4.1 *Alliance with the European University of Madrid (UEM)*

AELMHU signed a collaboration agreement with the European University of Madrid (UEM) to carry out joint activities in training, teaching, advising, and research.

The agreement **includes the possibility of jointly organizing training activities by both entities, such as courses, congresses, seminars, etc.**, as well as conducting studies and research projects, mutual advising, and exchange of information and documentation.

The first collaboration between the two organizations under this agreement took place with the **Official University Master's Degree in Medicines Discovery at UEM**, in which AELMHU collaborated in the preparation of the syllabus, the identification of professors, and the completion of the master's thesis (TFM).

Specifically, **this master's program, which aims to train professionals in the pharmaceutical, biotechnological, and biomedical industries**, will offer students the opportunity to carry out a TFM on a rare or ultra-rare disease, in which they will have to propose the development of a medicine considering all its phases, from molecular research to commercialization.

The Association participated in **the inauguration of the Master's program in October, which included the participation of our president, Beatriz Perales, as well as the president of FEDER, Juan Carrión**. In her speech during the event, held at the UEM Campus in Villaviciosa de Odón (Madrid), Beatriz Perales detailed the work AELMHU carries out to contribute through its actions to improving the health and quality of life of people affected by these pathologies. Actions focus on four pillars: collaboration, training, information, and visibility of the pathologies and their research.

→ Read more

5.4.2 *Alliance with the National Association of Health Informers (ANIS)*

The Association also signed a framework agreement with the National Association of Health Reporters (ANIS) to collaborate on joint activities that raise awareness and promote knowledge of rare diseases and orphan medicines.

Under this agreement, **AELMHU and ANIS will promote projects in areas of common interest to both organizations**, such as health, treatments, and the quality of life of people affected by rare diseases. For example, this includes participation in the congresses of both associations or the joint organization of training workshops.

Additionally, **both organizations commit to continuing to hold the Training Workshop for Journalists on Rare Diseases**, aimed at health-specialized journalists, which already has several editions. These types of actions, aimed at achieving a more informed, sensitive, and committed public opinion regarding this reality, also help establish bridges between information professionals, the pharmaceutical industry developing orphan medicines, and the community of people with rare diseases.

Read more

5.5

RELATIONSHIP with FEDER and PATIENT ASSOCIATIONS

As an essential part of its commitment to improving the quality of life for people with rare diseases, **AELMHU has maintained close collaboration throughout 2025 with the Spanish Federation of Rare Diseases (FEDER) and various patient associations.**

This relationship has materialized in **joint initiatives that strengthen the visibility of these pathologies, promote training, and foster dialogue spaces to advance shared solutions**. From participation in events to the involvement of the Association's working groups and training actions, the common goal has been to consolidate a relationship that allows responding to the needs of these organizations.

5.5.1 AELMHU and FEDER

As usual, **the Board of Directors and the technical team of the Association attended the presentation of the World Rare Disease Day campaign**, promoted by the Spanish Federation of Rare Diseases (FEDER), which this year took place in Oviedo.

On March 3, a meeting was held prior to the official event, in which FEDER gave prominence to 17 patient associations of rare diseases from Oviedo. **One of the most notable moments was the reading of the manifesto for World Rare Disease Day**, which outlined the main demands of the community.

Juan Carrión, president of FEDER, presented a recognition to each entity of the rare disease associative movement in Asturias, as a symbol of the effort and dedication of all those involved in the fight for patients' rights.

The following day, the official event was held, **a gathering that brought together all the agents involved and committed to raising awareness and addressing rare diseases**.

The event featured highly relevant institutional speeches, such as those by **Mónica García, Minister of Health, and Adrián Barbón, President of the Government of the Principality of Asturias**.

The event was closed by **Queen Letizia, who emphasized the importance of speeding up access times and reimbursement for treatments for rare diseases**. The queen expressed her solidarity with patients and their families, stressing that their voices must be heard and their demands must be translated into actions.

From AELMHU**, we feel deeply proud to have been part of this significant event**. The day not only served to raise awareness of the reality of these pathologies but also strengthened the spirit of collaboration among institutions, associations, the pharmaceutical industry, and civil society. This annual event is a clear example that when we work together, we can advance in the search for solutions to improve the lives of people suffering from rare diseases.

AELMHU also accompanied FEDER in other important events of the year, such as **the institutional act at the Assembly of Madrid on the occasion of Rare Disease Day**, held on March 18; the 'Leaving No One Behind' Conference, organized in Valencia on November 18; or the Andalusian Rare and Undiagnosed Diseases Regional Forum, hosted by the Virgen del Rocío University Hospital on December 16.

5.5.2 *Strategic meetings*

Likewise, **AELMHU has strengthened its collaboration with FEDER through the Co-creation Working Group with Patient Associations**, consolidating a stable space for dialogue and cooperation. Within this framework, three face-to-face meetings were held at FEDER's headquarters in Madrid (February, June, and November), designed to share reflections and identify collaborative actions.

During these meetings**, it was also possible to discuss and understand the problems faced by patients, identify their priority needs, and seek synergies to promote awareness projects and knowledge generation**.

In addition, **opportunities were shared to strengthen the participation of associations in decision-making**, with the common goal of advancing towards solutions that respond to the real demands of people living with rare diseases.

5.5.3 *Training sessions for patient associations*

On the other hand, **during 2025 seven training actions were carried out together with FEDER**, with the aim of providing knowledge and practical tools to strengthen the capacities of the associative movement, which were divided into three themes:

TRAINING SESSIONS ON ACCESS TO ORPHAN MEDICINES:

Three sessions were offered aimed at **helping organizations understand the approval and access process for medicines for rare disease**s. During these sessions, an overview of the journey of these medicines was presented, covering from their clinical development, designation as orphan medicines, marketing authorization at both European and national levels, to the decision on their price and reimbursement, concluding with their market launch. These trainings were held within the framework of the XIV FEDER Training School, held on October 24 in Burgos, and were taught by Jorge Mestre, health economist.

TRAINING SESSIONS ON STRATEGY:

Likewise**, two training sessions were also held for members of the FEDER Board of Directors and representatives of patient associations**, held on January 20 and June 12 in Madrid, on the importance of strategic reflection in these organizations, addressing key concepts and strategic principles, the importance of measuring and monitoring impact, the fundamentals that a professionalized entity should have, and the systems and tools for prioritizing objectives. Both sessions were taught by the consultancy Aleire Servicios Integrados, specialized in strategic and corporate development.

TRAINING SESSIONS ON SPOKESPERSONSHIP:

The two spokesperson training sessions, held on January 21 and September 30 in Madrid, focused on **improving the communication skills of the Federation's new spokespersons, as well as representatives of patient associations**. They were conducted in person and carried out with the strategy and communication consultancy Newlink.

5.5.4 *Collaboration with other patient associations*

AELMHU's commitment to people living with rare diseases also extends to other patient associations. During 2025, **the Association strengthened ties with other representative entities of the associative movement**, participating in their main events and contributing to creating spaces for dialogue that promote visibility, training, and the search for shared solutions.

Within this framework, **AELMHU organized a meeting for members with Carina Escobar, president of the Plataforma de Organizaciones de Pacientes (POP),** as detailed in point 4.2 of this report, and was present at several of their events, such as their IX Congress of Patient Organizations, held in Valencia on September 24-25; Carina Escobar's presentation at the Nueva Economía Fórum on October 20 in Madrid; and the presentation of their report ‘Integrated care for chronic conditions in Spain: Situation, tools, and challenges of health and social integration,’ on December 15 in Madrid.

Likewise, **the technical team of AELMHU strengthened the direct relationship with patient associations**, such as the Rare Diseases Association D'Genes, attending and participating in its XVIII International Congress on Rare Diseases, which took place on November 20 in Murcia; the ASEM Federation, which organized its events on the occasion of the National Day of Neuromuscular Diseases on November 14 at the Congress of Deputies; or the Spanish Federation of Turner Syndrome, which held its official presentation on September 12 at the FEDER headquarters in Madrid.

5.5.5 *Interviews*

These **interviews with patient associations have not only helped strengthen collaboration with the associative movement** but also generated content that expands the visibility and knowledge of rare diseases and orphan medicines.

Thus, following the initiative proposed by the AELMHU Patient Associations Co-creation working group, **during 2025 various key agents in the rare diseases sector were interviewed, including doctors, patients, and experts**.

Through these publications, both on the association's website and social networks, the aim was **to raise awareness about the challenges faced by both patients and health professionals**, as well as to highlight the importance of research and access to innovative therapies that improve the quality of life of those suffering from these pathologies.

28 INTERVIEWS IN 2025

In total, **28 interviews were published during 2025**:

JANUARY

- Isabel Motero, director of the Spanish Federation of Rare Diseases (FEDER), for World Depression Day.
- Isabel María Sarabia, social worker at the Rare Diseases Association D'Genes, for International Alagille Syndrome Awareness Day.

FEBRUARY

- Juan Carrión, president of FEDER, for World Rare Disease Day.

MARCH

- The spokespersons of the AELMHU working groups, on the occasion of International Women's Day.
- Naca Pérez de Tudela, president of the International Association of Relatives and Affected by Lipodystrophies (AELIP), and Dr. David Araújo-Vilar, for World Lipodystrophy Day.

APRIL

- Sandra Martín, member of the Board of Directors of MPS Lysosomal, Dr. Mónica López, head of the Internal Medicine Section at Ramón y Cajal Hospital in Madrid, and Dr. Javier Limeres, cardiologist at Vall d'Hebrón Hospital, for Fabry Disease Awareness Month.
- Cristina and Nacho, parents of a girl with Pompe disease.
- Daniel-Aníbal García, president of the Spanish Federation of Hemophilia (FEDHEMO), on the occasion of World Hemophilia Day.
- Patricia Marín, secretary of the Spanish Association of Progressive Osseous Fibrodysplasia (AEFOP); Sonia Las Heras, president of the Association; and Alejandro, her son; along with Dr. Javier Bachiller, rheumatologist at Ramón y Cajal Hospital, on the occasion of World Progressive Osseous Fibrodysplasia (FOP) Day.
- Estrella Mayoral, social worker and head of the direct care axis of FEDER, for World Undiagnosed Diseases Day.

MAY

- Enrique Carazo, president of the Foundation Against Pulmonary Hypertension (FCHP), on the occasion of the World Day of this pathology.
- Secundino Sacristán, president of the Spanish Association for the Fight Against Hemoglobinopathies and Thalassemias (ALHETA), for World Thalassemia Day.
- Miguel Ángel Tremiño, Internal Medicine nurse at the General Hospital of Elche, for World Nursing Day.
- Jordi Cruz, director of the MPS Lysosomal Association, and Chema, a patient, on the occasion of World Mucopolysaccharidosis Day.
- Aitana Aguilera, psychologist of the Neurofibromatosis Affected Association, for the World Day of this pathology.

JUNE

- Raquel Pardo, president of the Myasthenia Association of Spain (AMES), on the occasion of the International Day of this pathology.
- Rosalía Segura, secretary of the Spanish Sickle Cell Disease Association (ASAFE), coinciding with World Sickle Cell Day.

AUGUST

- Cuca Paulo, president of Stop FMF (Spanish Association of Familial Mediterranean Fever and Autoinflammatory Syndromes), for the Month of Autoinflammatory Diseases.

SEPTEMBER

- Juan Da Silva, president of the Spanish Federation of Cystic Fibrosis (FEFQ), for the World Day of this pathology.
- Guillermo Yriarte, president of the Leber Optic Nerve Atrophy Association (ASANO), on the occasion of the World Day of this rare disease.
- Dr. Lluis Masana, emeritus professor of Medicine at the Universitat Rovira i Virgili, for the International Day of familial hypercholesterolemia.

OCTOBER

- Dr. María Mercadal, pediatric hepatologist at Vall d'Hebrón Hospital, on the occasion of World Progressive Familial Intrahepatic Cholestasis Day.

- Adriana Reyes, president of the HPN Spain Association, on the occasion of International Paroxysmal Nocturnal Hemoglobinuria Day.

- Natividad Romero Haro, coordinator of care and training activities at the DEBRA Association, for World Epidermolysis Bullosa Day, also known as “butterfly skin”.

NOVEMBER

- Dr. Ovidio Muñiz, attending physician of the Internal Medicine Service and head of the Lipids and Vascular Risk Unit at the Virgen del Rocío Hospital in Seville, for the World Day of familial chylomicronemia syndrome.

- Blanca Guarás, president of NET Spain, on the occasion of World Neuroendocrine Tumor Day.

- Manuel Rego and Begoña Martín, president and director of the ASEM Federation, on the occasion of National Neuromuscular Diseases Day.

5.6 *Sector events*

During 2025, **AELMHU also consolidated its role as a key player in the rare diseases ecosystem by participating as a speaker in 20 events and attending 46 more**, reaching a total of 66, which represented a 10% increase compared to the previous year.

This intense activity allowed the Association to **continue promoting knowledge, visibility, and access to treatments aimed at these pathologies**, strengthening its position as an authoritative voice before administrations, healthcare professionals, industry, and patient associations.

20 EVENTS AS SPEAKERS

46 EVENTS AS ATTENDEES

66 TOTAL EVENTS

5.6.1 *Participation in sector events*

LECTURE IN THE DEGREE IN PROTOCOL AND ORGANIZATION AT CAMILO JOSÉ CELA UNIVERSITY

08/01/2025

Marian Corral, executive director of AELMHU, gave a lecture within the Degree in Protocol and Organization in the Public Relations subject at Camilo José Cela University (UCJC), in which she presented our Association, detailed the activities we carry out and the main strategic objectives. Thus, she focused on the Association's commitment to patient access to orphan medicines and the improvement of knowledge about these pathologies.

→ Read more

1ST EDITION OF THE BRIDGES OF HOPE FORUM – INTEGRATED STRATEGIES FOR ADVANCEMENT IN RARE DISEASES

28/01/2025

Marian Corral, executive director of AELMHU, participated in this event in Valencia organized by the Pharmacy Service of the University and Polytechnic Hospital La Fe, where patients, healthcare professionals, and the pharmaceutical industry reflected on research and innovation in rare diseases, as well as the latest collaborative strategies in this field.

→ Read more

5TH MEETING OF EXPERTS IN THE DIARIOFARMA CYCLE ON ORPHAN MEDICINES

05/02/2025

Also in Valencia, Marian Corral was part of this meeting, where speakers addressed one of the main problems affecting patients with rare diseases: the lack of equity in access to orphan medicines. Our executive director was accompanied by Elena Gras Colomer, general director of Pharmacy of the Valencian Community; José Luis Poveda, manager of the University and Polytechnic Hospital La Fe-Valencia; Inmaculada Pitarch, from the Rare Neuromuscular Diseases Unit of Hospital La Fe and researcher at the Biomedical Research Networking Center on Rare Diseases (CIBERER); Fidela Mirón, vice president of the Spanish Federation of Rare Diseases (FEDER); and José Vicente Galindo, Pricing & Market Access Associate Director of Chiesi Spain.

→ Read more

SECOND EDITION OF ÚNICAS TALKS: 'PRIORITIES IN RARE DISEASES'

12/02/2025

Beatriz Perales, president of AELMHU, participated in the debate panel 'Development and access to new therapies', along with Dr. José María Moraleda, coordinator of the Spanish Network of Advanced Therapies, and Dr. Juan Bueren, director of the Biomedical Innovation Unit of CIEMAT, IIS Fundación Jiménez Díaz and CIBERER, and moderated by Dr. Alessandra Magnani, director of the Advanced Therapies and Immunotherapy Platform at Hospital Sant Joan de Déu Barcelona.

→ Read more

VIII SANITARIAS AWARDS FOR WOMEN'S LEADERSHIP IN HEALTHCARE BY REDACCIÓN MÉDICA

25/02/2025

The health-specialized media outlet Redacción Médica awarded our president, Beatriz Perales, one of its VIII Sanitarias Awards, which each year highlight women who have stood out for their leadership, innovation, and commitment to improving the healthcare system in Spain. She received the award from Lourdes Martínez-Berganza Asensio, vice president of the Spanish Society of Primary Care Physicians (Semergen), and Montserrat Chimeno Viñas, president of the Spanish Society of Internal Medicine (SEMI).

→ Read more

PRESENTATION OF THE CAMPAIGN 'UNMASK THE RARE ONES'

26/02/2025

Within the framework of the World Rare Disease Day, AELMHU, represented by its executive director, Marian Corral, participated in the closing of the presentation of the campaign 'Unmask the Rare Ones,' by the biopharmaceutical company Sobi in collaboration with the Spanish Federation of Rare Diseases (FEDER). This campaign aims to raise awareness in society about the situation of people suffering from these diseases, which affect fewer than 5 in every 10,000 people and face numerous obstacles in their daily lives, both in healthcare and in social, educational, and work environments.

→ Read more

MEETINGS WITH EXECUTIVES FROM RARE AND ULTRA-RARE DISEASE COMPANIES OF ADQUALIS

19/03/2025 – 08/05/2026

The executive director of the Association, Marian Corral, participated in these two meetings organized by the human resources consultancy AdQualis Human Results, which brought together executives from pharmaceutical companies that research, develop, or market orphan medicines or other treatments for rare and ultra-rare diseases.

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→ Read more

CONFERENCE 'TOWARDS A LONG-TERM STRATEGY FOR ONE-SHOT THERAPIES IN SPAIN'

28/04/2025

The executive director of the Association, Marian Corral, participated in the roundtable 'Theory and practice: balance between access and sustainability for one-shot therapies,' a panel moderated by Susana Álvarez, former deputy director general of Contracting of the Madrid Health Service, which also included Gerardo García-Álvarez, Professor of Administrative Law at the University of Zaragoza; Lourdes Gil, Head of Pharmaceutical Technologies Area at the Ministry of Industry and Tourism; and Jorge Mestre, Health Economics researcher and consultant.

→ Read more

CONFERENCE 'COMPASS OF EUROPEAN COMPETITIVENESS AND ITS IMPACT ON THE SPANISH HEALTH ECOSYSTEM'

12/05/2025

The president of AELMHU, Beatriz Perales, participated in this event, organized by LEVIN Public Health Affairs, in the 'Biopharmaceutical Competitiveness and Innovation Mission,' focused on promoting biopharmaceutical R&D in Spain, with special attention to orphan medicines, biosimilars, and advanced therapies. It featured the participation of César Hernández (Director General of the Common Portfolio of Services of the National Health System and Pharmacy of the Ministry of Health); Javier Urzay (Farmaindustria); Ion Arocena (ASEBIO), Encarnación Cruz (BIOSIM); Elena Casaus (AESEG) and Lidia Martín (ANEFP).

→ Read more

CONFERENCE 'FOR A COMPREHENSIVE APPROACH TO RARE DISEASES: CHALLENGES AND OPPORTUNITIES'

06/06/2025

AELMHU, through its executive director, Marian Corral, participated in this conference organized by the Spanish National Research Council (CSIC), in a new initiative of its CICERON Itinerary Program. The aim of the meeting was to promote interaction among researchers, sector companies, patients, public administrations, and media, as well as to showcase CSIC's contribution to the study of rare diseases.

→ Read more

CONFERENCE 'III EDITION OF RARE DISEASES. METABOLIC BONE PATHOLOGY'

25/09/2025

Sergio Bullón, treasurer and board member of AELMHU, participated in this meeting organized by the 12 de Octubre University Hospital, which has already established itself as a benchmark event in the sector, rare bone metabolism diseases, their learning through clinical cases, and the challenges that still exist to achieve a comprehensive approach. At the inauguration, Sergio was accompanied by the hospital's medical director, Dr. Julio Pascual; Dr. Montserrat Morales, head of Internal Medicine; and Dr. Guillermo Martínez Díaz-Guerra, head of the Endocrinology and Nutrition Section.

→ Read more

VI HEALTHCARE OBSERVATORY EL ESPAÑOL

30/09/2025

The VI Symposium of the Healthcare Observatory, organized by El Español and Invertia, featured the participation of prominent leaders from the healthcare sector and government representatives. Beatriz Perales, president of AELMHU, addressed the sector's position regarding the Draft Law on Medicines and Health Products. During her speech, she applauded the inclusion of some measures proposed by AELMHU during the public consultation period but also expressed the sector's concern about several aspects of the draft law.

→ Read more

INAUGURATION OF THE OFFICIAL UNIVERSITY MASTER'S DEGREE IN MEDICINES DISCOVERY

17/10/2025

After the agreement signed with the European University of Madrid (UEM), the Association, through its president, Beatriz Perales, participated in the inauguration this Tuesday at the UEM Campus in Villaviciosa de Odón (Madrid). In her speech, she explained the particularities of rare diseases and their treatments, and detailed the work carried out by the association to contribute with its actions to improving the health and quality of life of people affected by these pathologies.

Read more

SUMMIT OF LEADERS OF THE IBERO-AMERICAN ASSOCIATIVE MOVEMENT OF RARE DISEASES

21/10/2025

AELMHU participated in this summit, held at the Ministry of Health, which was inaugurated by the minister, Mónica García, and Juan Carrión, president of ALIBER and the Spanish Federation of Rare Diseases. It brought together representatives from Ibero-American countries with the aim of sharing the challenges and issues faced by the community, as well as advancing towards a global action plan on rare diseases. Our president, Beatriz Perales, accompanied by the executive director, Marian Corral, took part in a panel discussion about the challenges and opportunities posed by the implementation of the World Health Organization (WHO) resolution, promoted by Spain, which recognizes rare diseases as a global public health priority.

Read more

FEDER 'LEAVING NO ONE BEHIND' CONFERENCE

18/11/2025

The vice president of AELMHU, María José Sánchez, participated in the debate panel 'When health depends on access: a debate on inequity in access to treatments,' moderated by Daniel de Vicente, a member of the Board of Directors of FEDER. She was accompanied by César Hernández, general director of the Common Portfolio of Services of the National Health System and Pharmacy; Santiago de la Riva, vice president of the FEDER Foundation; and Víctor Rodríguez de Vera, lawyer from the Ramón y Cajal firm. The event, held at the La Fe University and Polytechnic Hospital in Valencia, focused on the challenges faced by people with rare diseases, such as diagnosis, access to treatments, and comprehensive care that guarantees equity across all autonomous communities.

Read more

XVIII INTERNATIONAL CONGRESS OF RARE DISEASES OF D'GENES

20/11/2025

Once again, the association, through its president, Beatriz Perales, played its role at the XVIII International Congress of Rare Diseases, organized by the D'Genes association at the Catholic University of Murcia. The president spoke to highlight the main challenges and obstacles to speeding up the arrival of orphan medicines to patients with rare diseases, such as accelerating and facilitating innovation and research; streamlining the evaluation and reimbursement processes for this type of treatment; or ensuring true territorial equity among all autonomous communities.

Read more

5.6.2 Events attendance

The Board of Directors and the technical team of AELMHU **attended 46 events with experts and health authorities throughout 2025** to closely follow legislative developments in the sector.

This strengthened the institutional positioning of the Association, consolidating its role as a key player in the field of rare diseases.

During the year**, AELMHU strengthened dialogue with Administration officials**: Javier Padilla (March 10), MEP Nicolás Fernández Casares (March 14), Juan José Pedreño (Health Counselor, Murcia, May 7), and Elvira Velasco (Popular Group spokesperson, November 11).

AELMHU also attended specialized forums such as the VI Summer Course newsRARE (Toledo, September 17, Fundación Weber); the sessions on the review of European pharmaceutical legislation by Diariofarma; and the Bioinnova sessions on advanced therapies.

Additionally, **it strengthened its collaboration with other pharmaceutical industry associations** by participating in the 10th anniversary event of Biosim, which brought together the main sector employers' associations.


DEL DATO AL IMPACTO SOCIAL
MESA 1 - Innovación con resultados: colaboración y nuevos modelos de financiación
newsRARE


CICLO DE COLOQUIOS
Revisión de la legislación farmacéutica europea
IMPACTO en ESPAÑA
12:20-13:40 Coloquio
Elena Nevado, diputada del Grupo Popular Europeo
Nicolás González Casares, diputado Grupo Socialistas y Demócratas
Margarita de la Pisa, diputada del Grupo Patriotas por Europa
Modera: José María López Alemany, director de Diariofarma
diariofarma
Lilly
MSD
NOVARTIS
Pfizer


ASOCIATIVO DE LAS EERR
EN IBEROAMÉRICA
Apoyan:
Red Iberoamericana
Patrocinan:
bridgebi


GOBIERNO DE ARAGON
JORNADA
Día Mundial Enfermedades Raras ARAGÓN


Harmonías


Sección de Derecho Farmacéutico
¿Está prohibida la publicidad de medicamentos antes de la decisión de financiación? De la STS de 4 de marzo de 2025 al APL de 8 de abril de 2025
ILUSTRE COLEGIO DE LA ABOGACÍA DE MADRID

06

COMMUNICATION

In 2025, **the Communication area has consolidated its role as a strategic and cross-cutting axis in AELMHU's activity**, evolving to become a positioning and value generation tool.

Throughout the year, **the Association has strengthened its communication strategy focused on impact, aiming to build a strong, recognizable, and distinctive voice within the field of rare diseases and orphan medicines**. This voice has sought not only to convey messages but also to contextualize advances, highlight innovation, and contribute to a better understanding of structural challenges.

The presence in the media has continued to be a fundamental pillar, not only in terms of visibility but also as a space for dialogue and agenda setting. Through a constant and proactive relationship with journalists and media outlets, AELMHU has managed to position its messages in key debates, providing an expert and rigorous perspective at moments of special relevance for the sector.

At the same time, **the digital ecosystem has continued to gain importance as a channel of connection with various stakeholders**. Content on the website and on LinkedIn, X, and YouTube profiles, as well as the creation of the Association's Instagram account, have allowed for broader reach and improved interaction capacity, fostering more direct, audiovisual, and dynamic communication.

Internally, **communication with members has been strengthened thanks to the renewal of the Weekly Bulletin as a channel for information and updates on sector and Association news**, as well as the sending of informational alerts on relevant milestones, especially regarding regulatory matters or those with high impact for associated companies. Both tools have contributed to strengthening the bond with AELMHU members, facilitating a continuous flow of information and knowledge.

6.1 AELMHU in the MEDIA

During 2025, **the Association increased its impact in both general and specialized media**, such as El Economista, El Español, La Vanguardia, Redacción Médica, iSanidad, and Europa Press.

This was **the result of the strategic dissemination of press releases**, the presentation of access and clinical trial reports, participation in events, and media inquiries on matters of direct relevance to the Association.

8 PRESS RELEASES

18 INTERVIEWS AND MEDIA COLLABORATIONS


elEconomista.es
20 Años

Beatriz Perales: "Es fundamental tener un proceso de precio y financiación flexible de los medicamentos huérfanos"

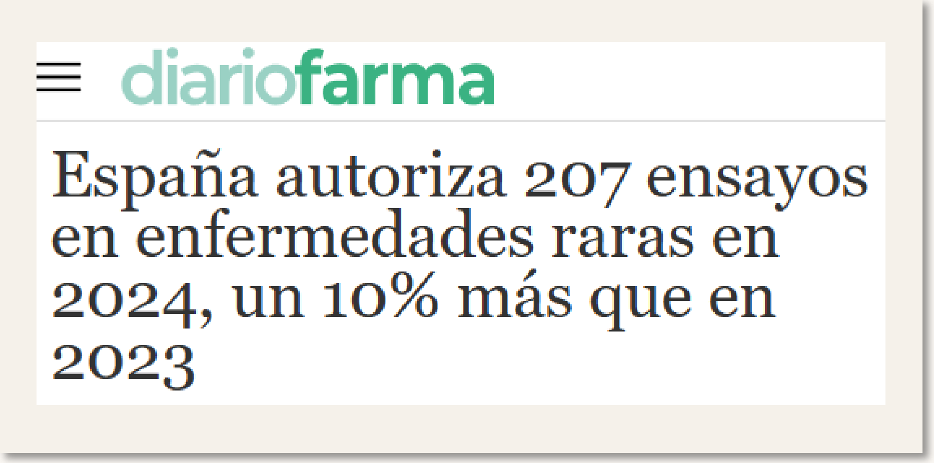
- *La presidenta de AELMHU reclama "la creación de un programa de acceso temprano para este tipo de tratamientos"*


AA EL ESPAÑOL invertia

OBSERVATORIO DE LA SANIDAD

España financió 17 medicamentos para tratar enfermedades raras en 2024, más del triple que en 2020

- La cifra del pasado año es la segunda más alta de los últimos 5 años.


diariofarma

España autoriza 207 ensayos en enfermedades raras en 2024, un 10% más que en 2023

06 COMMUNICATION

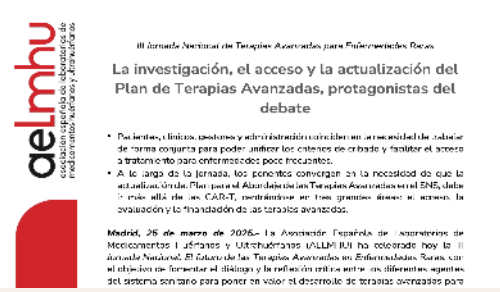
From AELMHU, **eight press releases were issued about milestones and own activities**:

AELMHU PRESENTA SU INFORME ANUAL DE ACCESO A LOS MEDICAMENTOS HUÉRFANOS EN ESPAÑA EN 2024

ESPAÑA FINANCIA EL 58% DE LOS MEDICAMENTOS HUÉRFANOS AUTORIZADOS EN LA UNIÓN EUROPEA

Annual Report on Access to Orphan Medicines 2024

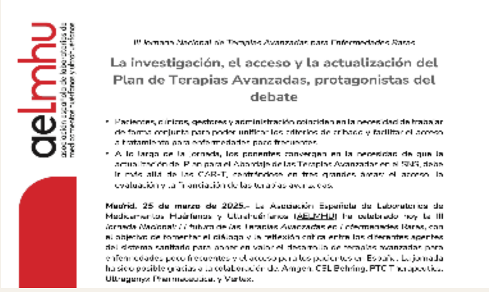
Read more



La investigación, el acceso y la actualización del Plan de Terapias Avanzadas, protagonistas del debate

Collaboration agreement with the General Council of Pharmaceutical Colleges

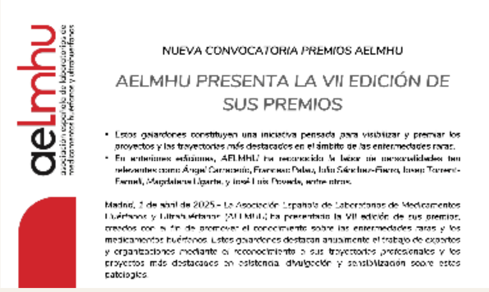
Read more



La investigación, el acceso y la actualización del Plan de Terapias Avanzadas, protagonistas del debate

III National Conference on Advanced Therapies for Rare Diseases

Read more

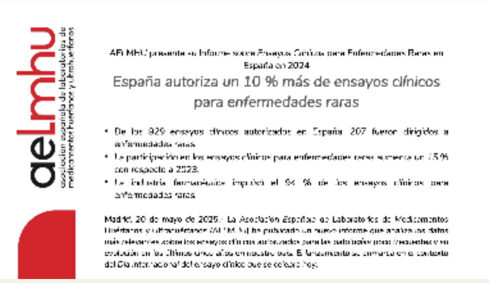


NUEVA CONVOCATORIA PREMIOS AELMHU

AELMHU PRESENTA LA VII EDICIÓN DE SUS PREMIOS

Launch of the VII edition of the AELMHU Awards

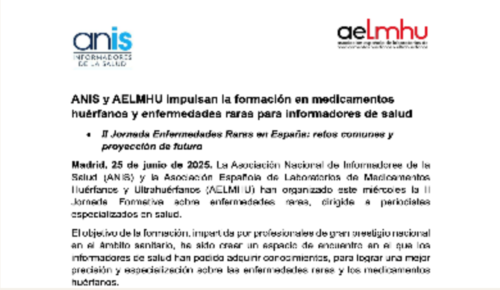
Read more



España autoriza un 10 % más de ensayos clínicos para enfermedades raras

Clinical trial report on rare diseases 2024

Read more



ANIS y AELMHU impulsan la formación en medicamentos huérfanos y enfermedades raras para informadores de salud

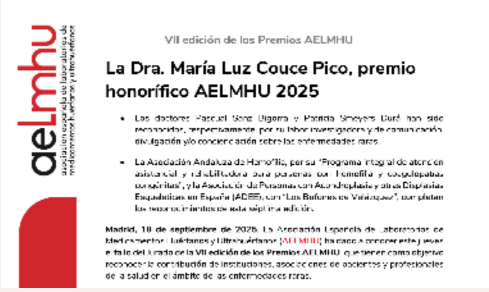
- ***II Jornada Enfermedades Raras en España: retos comunes y proyección de futuro***

Madrid, 25 de junio de 2025. La Asociación Nacional de Informadores de la Salud (ANIS) y la Asociación Española de Laboratorios de Medicamentos Huérfanos y Ultrahuérfanos (AELMHU) han organizado este miércoles la II Jornada Formativa sobre enfermedades raras, dirigida a periodistas especializados en salud.

El objetivo de la formación, impartida por profesionales de gran prestigio nacional en el ámbito sanitario, ha sido crear un espacio de encuentro en el que los informadores de salud han podido adquirir conocimientos, para lograr una mejor precisión y especialización sobre las enfermedades raras y los medicamentos huérfanos.

Training day for journalists on rare diseases

Read more

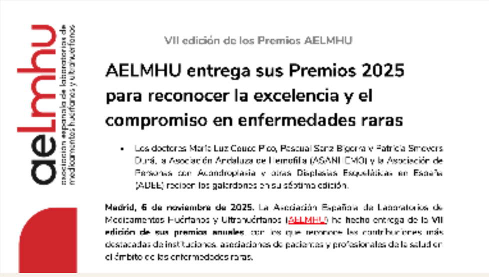


VII edición de los Premios AELMHU

La Dra. María Luz Couce Pico, premio honorífico AELMHU 2025

Jury decision of the VII edition of the AELMHU Awards

Read more



VII edición de los Premios AELMHU

AELMHU entrega sus Premios 2025 para reconocer la excelencia y el compromiso en enfermedades raras

Award ceremony of the VII edition of the AELMHU Awards

Read more

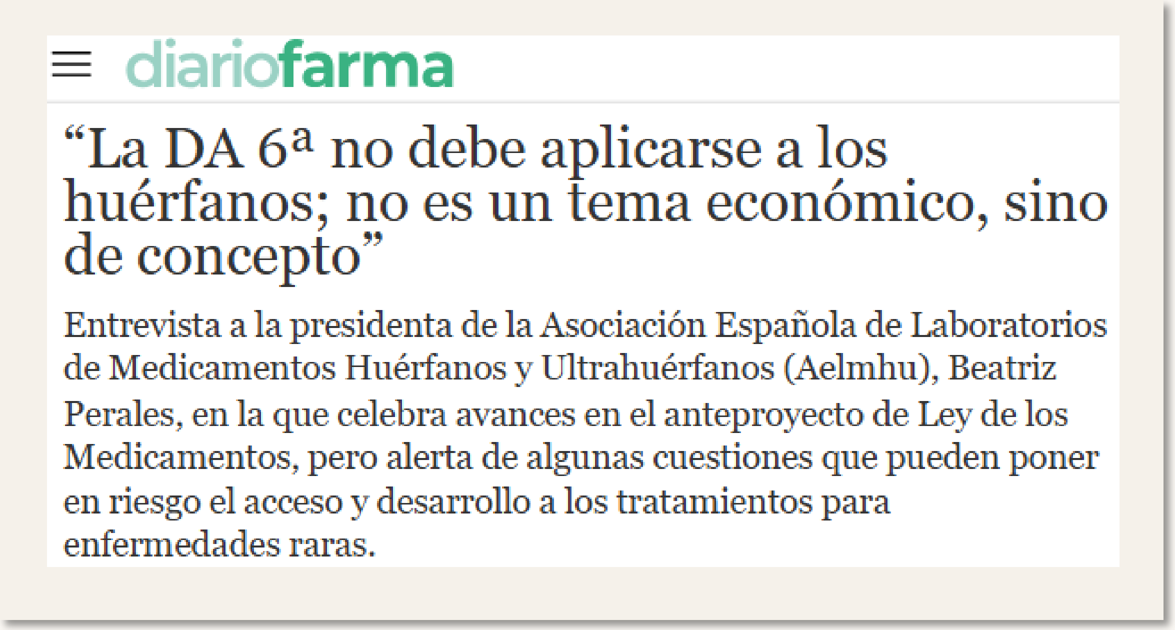
Additionally, through its spokespersons and members of the Representative Body, **the Association also participated in 18 interviews and information requests throughout 2025**, conveying the key messages and strategic proposals of AELMHU to media outlets such as Cadena Ser, Diariofarma, El Economista, iSanidad, Diario Médico, and Demócrata, among others.

Perales: "Mi carrera como farmacéutica empezó en mi casa, con mi madre"

Beatriz Perales, galardonada en los Premios Sanitarias, resalta la importancia de la investigación en enfermedades raras

El lento acceso a los medicamentos huérfanos: "La paciencia para los pacientes no es infinita"

Los fármacos destinados a enfermedades raras tardan de media más de dos años en llegar a España desde que los aprueba la Unión Europea



"La DA 6ª no debe aplicarse a los huérfanos; no es un tema económico, sino de concepto"

Entrevista a la presidenta de la Asociación Española de Laboratorios de Medicamentos Huérfanos y Ultrahuérfanos (Aelmhu), Beatriz Perales, en la que celebra avances en el anteproyecto de Ley de los Medicamentos, pero alerta de algunas cuestiones que pueden poner en riesgo el acceso y desarrollo a los tratamientos para enfermedades raras.

El sector del medicamento reivindica el diálogo con Sanidad como oportunidad para actualizar la legislación

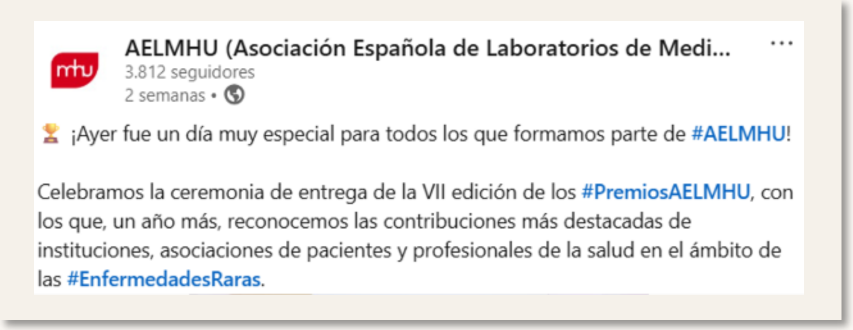
- Las principales patronales exponen su 'lista de reyes magos' en el VI Simposio del Observatorio de la Sanidad.

6.2 DIGITAL STRATEGY and ONLINE CHANNELS

AELMHU also continued growing on its main corporate digital channels. The Association kept increasing its online presence and community: it surpassed 12,000 followers on its LinkedIn, Instagram, and Twitter social media profiles, and reached nearly 200 posts throughout 2025.

The AELMHU website received over 30,000 visits and had more than 16,000 active users, thanks to the constant updating of content, such as news about the most relevant current events of the Association, the anniversary interviews mentioned in section 5.5 of this Report, as well as the publication of our reports and main projects, such as the AELMHU Awards.

Our presence on YouTube was also strengthened thanks to the publication of educational videos about rare diseases and orphan medicines in collaboration with the General Council of Pharmaceutical Colleges (CGCOF), which generated 580,000 views. Likewise, both the training workshop for journalists and the 3rd National Conference on Advanced Therapies for Rare Diseases were broadcast live, and the full ceremony of the VII AELMHU Awards was published.



AELMHU (Asociación Española de Laboratorios de Medi...
3.812 seguidores
2 semanas •

🏆 ¡Ayer fue un día muy especial para todos los que formamos parte de #AELMHU!

Celebramos la ceremonia de entrega de la VII edición de los #PremiosAELMHU, con los que, un año más, reconocemos las contribuciones más destacadas de instituciones, asociaciones de pacientes y profesionales de la salud en el ámbito de las #EnfermedadesRaras.

aelmhu_
AELMHU
7 publicaciones 114 seguidores 350 seguidos
Página oficial de la Asociación Española de Laboratorios de Medicamentos Huérfanos, Ultrahuérfanos y otros tratamientos para Enfermedades Raras

30-09-2025
AELMHU en el VI Simposio del Observatorio de la Sanidad de El Español

La presidenta de la Asociación Española de Medicamentos Huérfanos y Ultrahuérfanos (AELMHU), Beatriz Perales, ha participado este martes en el VI Simposio del Observatorio de la Sanidad de El Español, para abordar la posición del ...

DIGITAL PRESENCE · ONLINE COMMUNITY

Digital Audience and Community Growth

Sustained growth on social media and the web. The Instagram account was added in 2025 as a new strategic channel.

SOCIAL MEDIA

12,000+

Total Followers

Across LinkedIn, Instagram, and X. Sustained year-over-year growth.

POSTS

≈ 200

Social Media Posts

Active online strategy across all channels.

WEBSITE

30,000+

Visits to aelmhu.es

With over 16,000 active users throughout the year.

YOUTUBE

580,000

Views

AELMHU videos + CGCOF channel.

Distribution by Channel · Online Community

Three active channels: LinkedIn (professional), X (institutional), and the new Instagram account launched in 2025.

LinkedIn Professional community in the health and pharmaceutical sector	+3,000	25% of followers
X (Twitter) Institutional communication, press and media	+9,000	74% followers
Instagram New account launched in 2025 · informative content	+200	2% followers

Audiovisual content on YouTube

EDUCATIONAL VIDEOS · CGCOF

580,000

Cumulative Views

Results of the 6 video tips on Orphan Medicines and Pharmacy published with the General Council of Pharmaceutical Colleges.

THE 6 VIDEO TIPS

● **01** Do you know what rare diseases are? And orphan medicines?

● **02** Research, diagnosis, and treatment of rare diseases

● **03** What is the development and approval process in Europe?

● **04** How is access to orphan medicines?

● **05** Do you know the social value of orphan medicines?

● **06** Main myths about orphan medicines

6.3 COMMUNICATION with ASSOCIATES

Regarding internal communication with associates, **during 2025 the style and the sending platform of the Weekly Bulletin were renewed to create a more visual format adapted to the communication needs of the Association**.

Throughout the year, **41 weekly bulletins were sent to more than 140 people from the 27 associated companies of AELMHU**, with the aim of keeping them informed about news related to rare diseases, orphan medicines, the pharmaceutical industry, and our Association.

RENEWED IN 2025

Weekly Bulletin

Every Friday with updates on rare diseases, orphan medicines, the pharmaceutical industry, and our Association.

41
Weekly bulletins in 2025

+140
People who receive it

27
Member companies

SECTIONS

- About AELMHU
- News
- Documents of interest
- Recommendations
- Events

Thus, **the bulletin gathers every Friday the main news, documents of interest, reading recommendations, and sector events, as well as AELMHU's activities throughout the week**.

Boletín semanal de aelmhu

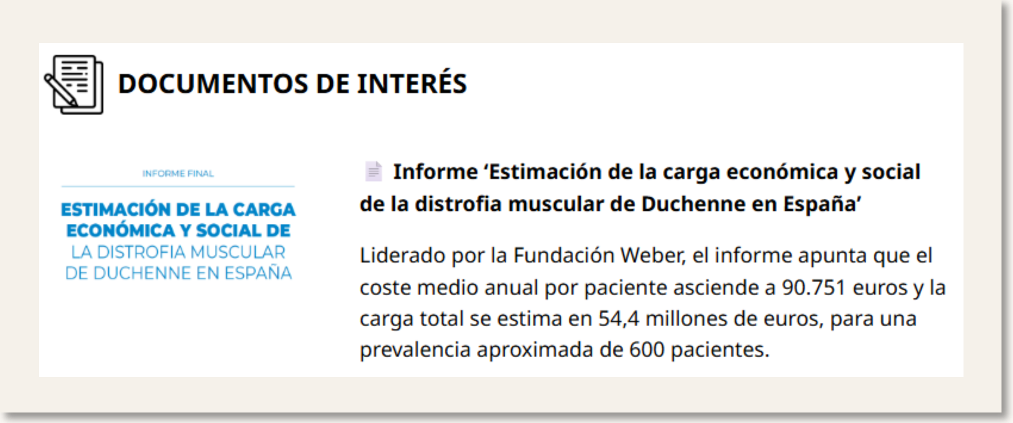
17 - 21 noviembre

SOBRE AELMHU

Eventos con participación de AELMHU

XVIII Congreso Internacional de Enfermedades Raras de D'Genes

La presidenta de AELMHU, **Beatriz Perales**, intervino este jueves en el congreso de D'Genes, en la mesa sobre acceso a medicamentos huérfanos, para reivindicar que la innovación llegue lo antes posible a los pacientes con enfermedades raras.



DOCUMENTOS DE INTERÉS

INFORME FINAL

ESTIMACIÓN DE LA CARGA ECONÓMICA Y SOCIAL DE LA DISTROFIA MUSCULAR DE DUCHENNE EN ESPAÑA

Informe 'Estimación de la carga económica y social de la distrofia muscular de Duchenne en España'

Liderado por la Fundación Weber, el informe apunta que el coste medio anual por paciente asciende a 90.751 euros y la carga total se estima en 54,4 millones de euros, para una prevalencia aproximada de 600 pacientes.

NOTICIAS

César Hernández avanza que la Ley del Medicamento volverá al Consejo de Ministros antes de final de año

15 países europeos advierten de que la directiva de aguas residuales puede encarecer y limitar medicamentos

6.3 COMMUNICATION with MEMBERS

Within the internal communication with associates, **the real-time informative alert service was also strengthened**, which reaches the representatives of the 27 companies associated with AELMHU and the more than 70 members of the four working groups of the Association.

In total, **the Association's technical team sent 29 alerts throughout the year**, aiming to keep them updated on legislative and regulatory developments relevant to the sector, especially regarding orphan medicines and rare diseases.

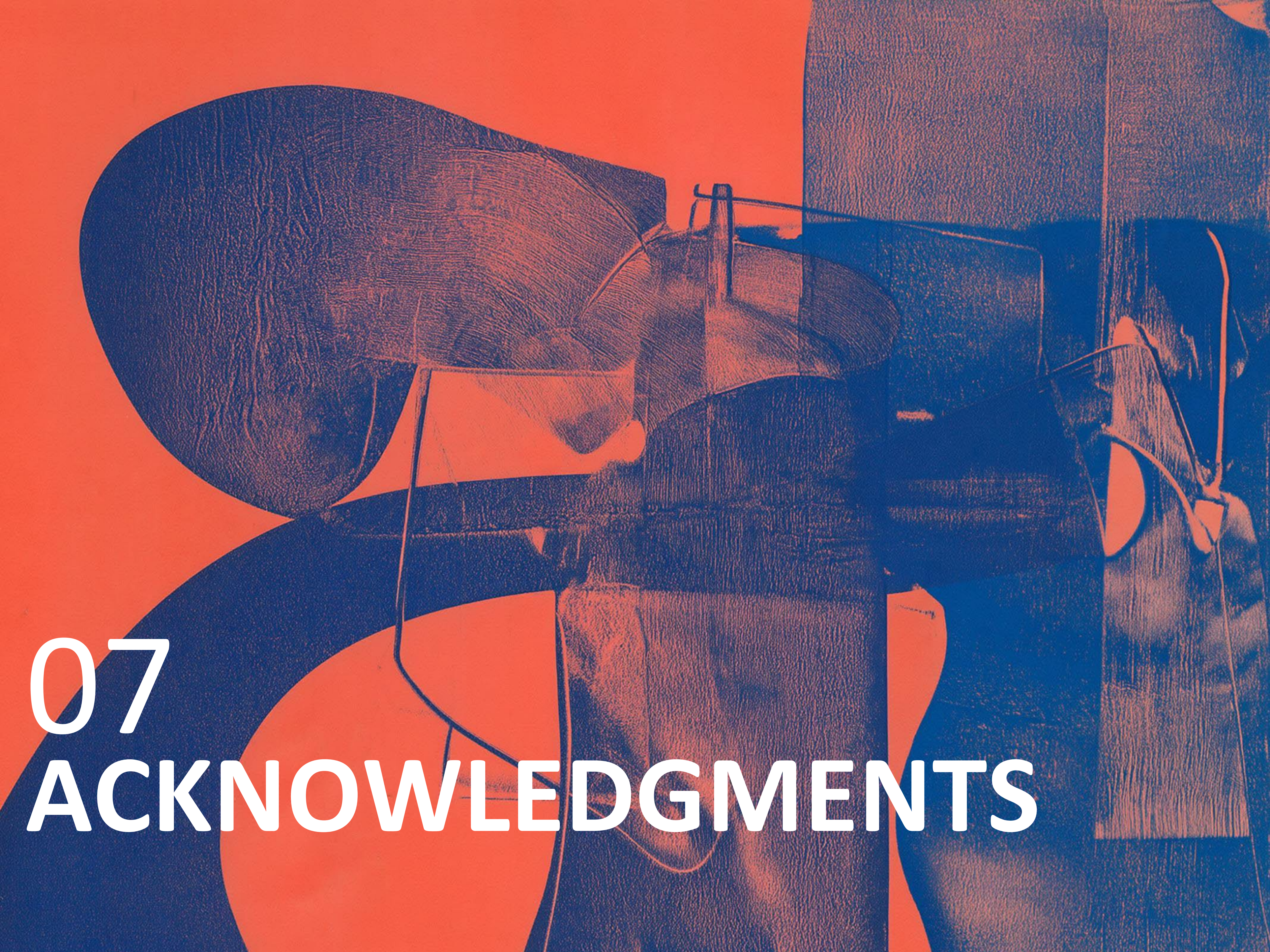
Among the most relevant topics were initiatives promoted by the Ministry of Health and European institutions, such as the European Critical Medicines Law or the SNS 2025-2028 Advanced Therapies Plan. Additionally, monitoring was carried out on various regulatory projects and public consultations related to digital health, neonatal screening, sustainability in public procurement processes, and the organization of the health system, including the creation and approval of the State Public Health Agency (AESAP) and the development of the Draft Law on Patient Organizations.

IN REAL TIME

Informative alerts

Specific communications regarding legislative and regulatory updates relevant to members, especially concerning orphan medicines and rare diseases.

+29	+100
Alerts sent during 2025	Members receive them



07

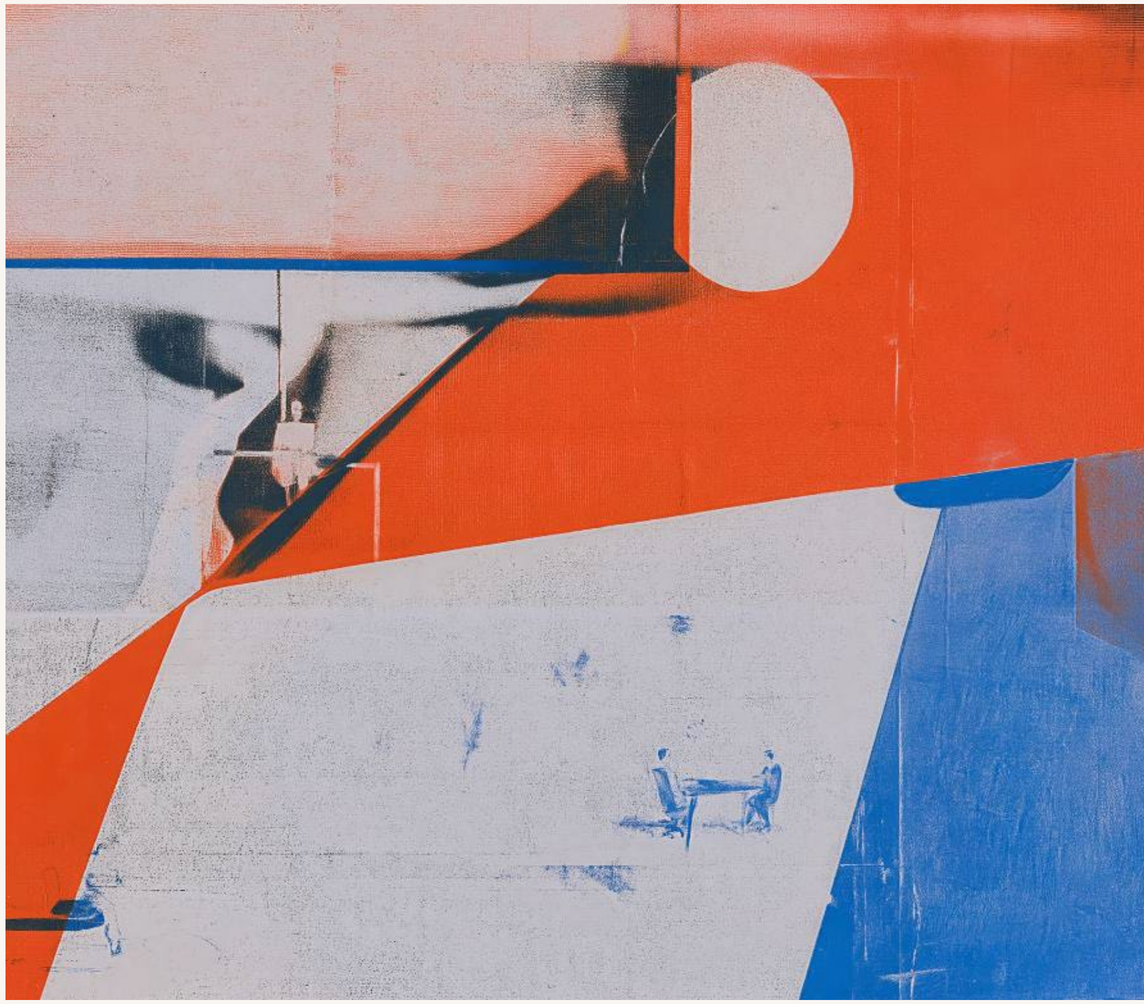
ACKNOWLEDGMENTS

All the actions and achievements recorded in this report have been possible thanks to the commitment and collaboration of our 27 associated companies and those who share our mission: to improve the health and quality of life of people living with rare diseases and their families. Healthcare professionals, medical and scientific societies, media, patient associations, universities, consultancies, public and private institutions, etc., to all of them we extend our sincerest thanks for their support and dedication throughout this year.

Thanks to this cooperation, **we have joined efforts to advance the visibility of these pathologies, promote research, and facilitate access to innovative treatments**. Each initiative developed reflects the strength of joint work and the shared commitment to building a healthcare system more sensitive to the needs of patients.

A special recognition goes to the Spanish Federation of Rare Diseases (FEDER), spokesperson and representative of patients living with these pathologies, who are the true reason for AELMHU's existence.

Thank you all for another year of collaboration, dialogue, and projects that bring us closer to the common goal: that no person with a rare disease is left behind!



adee ADEE.ES

AdQualis Human Results

AECOM Asociación Española

AEEPOMPE

aefop

AELIP

agencia española de medicamentos y productos sanitarios

ALHETA

Alianza Iberoamericana de Enfermedades Raras

AMES Asociación Miastenia de España

ASAFE

asanol

ASMD

asanhemo

d´genes Asociación de Enfermedades Raras

Asociación de Afectados de Neurofibromatosis

AES Asociación de Economía de la Salud

HPN

anis INFORMADORES DE LA SALUD

bio innova CONSULTING

CADENA SER

ciber ER

Comunidad de Madrid CONSEJERÍA DE SANIDAD

ConSalud.es

Farmacéuticos

Consejo General de Enfermería

CSC Consorci de Salut i Social de Catalunya

CONTRA BANDE ANDO

CORREO FARMACÉUTICO

CREMADES & CALVO-SOTELO ABOGADOS

CSIC

d´genes Asociación de Enfermedades Raras

debra Piel de Mariposa

Demócrata

DIARIO MEDICO

diariofarma

EL ESPAÑOL

EL GLOBAL

elEconomista.es

ERDERA European Rare Diseases Research Alliance

Espacio Bertelsmann

eupati

Faus Moliner

feder

Federación asem

FEDE

fq Federación Española Fibrosis Quística

fedhemo Federación Española de Hemofilia

Fegerec

FESTURNER

FNETH

FEP foro español de pacientes

Fundación Almar

Fundación Contra la Hipertensión Pulmonar

FUNDACIÓN GASPAR CASAL

HUMANS

frA FUNDACIÓN RAMÓN ARECES

FMF

GACETA MÉDICA

GENERALITAT VALENCIANA Conselleria de Sanitat Universal i Salut Pública

GOBIERNO DE ARAGON

SJD Sant Joan de Déu Barcelona · Hospital

H

Hospital Universitario 12 de Octubre

La Fe Hospital Universitari i Politècnic

ibv INSTITUTO DE BIOMEDICINA DE VALENCIA CSIC

ILUSTRE COLEGIO DE LA ABOGACÍA DE MADRID

IM MÉDICO

Institut Català de la Salut

iier Instituto de Investigación de Enfermedades Raras

idis INSTITUTO DE INVESTIGACIÓN SANITARIA SANTIAGO DE COMPOSTELA

isc Instituto de Salud Carlos III

Interempresas

iSanidad

ISPOR Spain Chapter

justonavas | Fotografía

LA RAZÓN

LEVIN Public Health Affairs

LIDER IT·Consulting

magallanes consultores de dirección

GOBIERNO DE ESPAÑA MINISTERIO DE CIENCIA E INNOVACIÓN

GOBIERNO DE ESPAÑA MINISTERIO DE INDUSTRIA Y TURISMO

GOBIERNO DE ESPAÑA MINISTERIO DE SANIDAD

MPS LISOSOMALES

NET ESPAÑA

ME New Medical Economics

Newlink

newsRARE

nextep IN HEALTH

NEF Nueva Economía Fórum

Osakidetza

PERTE Para la Salud de Vanguardia

PLATAFORMA DE ORGANIZACIONES DE PACIENTES

RERCSIC Biomed

rm

RIBERSER

RPP IMPACT ADVOCACY

SEMERGEN AP

SEMI

sefh Sociedad Española de Farmacia Hospitalaria

TORNOS ABOGADOS

UCLM Universidad de Castilla-La Mancha

Únicas Raras pero no invisibles

Universidad Camilo José Cela

Universidad Zaragoza

ue Universidad Europea

Weber

ANNUAL

REPORT
2025

mhu aelmhu

spanish association of orphan and ultra-orphan drug laboratories