ANNUAL REPORT 2024

aelmhu

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01. LETTER *from the* PRESIDENT

During this year 2024, I have assumed the presidency of AELMHU with enormous enthusiasm and a sense of responsibility, grateful for the trust placed in the new Board of Directors by the companies that make up this Association.

It is truly an honour to continue the work carried out by the previous Boards of Directors of the Association, which has now established itself as a leading voice in the ecosystem of rare and ultra-rare diseases and orphan drugs nationwide. Our commitment is clear: to continue promoting awareness of rare diseases, raise awareness of patient needs, and work with all stakeholders to ensure that people suffering from these diseases have greater and faster access to the orphan treatments they so desperately need.

The year 2024 has been a period of significant challenges and opportunities for the sector. Legislatively, both in Spain and Europe, there has been a context of change and adaptation that could directly impact access to and availability of these innovative treatments. In Europe, the review of pharmaceutical policies and health technology assessment processes have been at the centre of the debate, seeking a balance between innovation, financial sustainability, and equitable access for patients.

In Spain, various initiatives and reforms have been implemented to improve regional coordination, streamline reimbursement, and promote transparency in decision-making related to orphan drugs. For this reason, at ALEMHU, we have emphasized the need to continue advancing in research for rare diseases and the development of new treatments, and we have called for the pharmaceutical industry to be involved in all legislative changes and processes. These advances must be translated into real actions for patients and within a favourable environment that allows us to promote the arrival of innovation in Spain.

In this regard, it is important to highlight that the main starting point for this change has been the approval of the Pharmaceutical Industry Strategy. This marked the beginning of a detailed review of pharmaceutical legislation, on which we have been working for the past few years. At AELMHU, we are aware that this transformation is not a quick process, as it requires time to ensure that the proposals, particularly the legislative ones, are appropriate to achieve a harmonized, coordinated, robust, and agile system, so that our country offers an attractive and favourable environment for the pharmaceutical sector.

With all this, AELMHU has continued working intensively to position itself as an active and rigorous interlocutor in this context. We have strengthened our role as data generators and facilitators of dialogue, publishing annual and quarterly updates of our Annual Report on Access to Orphan Drugs, as well as technical reports that provide data on the social value of orphan drugs, clinical trials for rare diseases, and technical positions on topics of special interest to the sector at both the regional and national levels. Furthermore, the involvement of our working groups in dialogue with stakeholders and participation in public consultations and specialized forums has grown significantly, allowing us to continue contributing with authority and strategic vision to the continuous improvement of the system.

These actions, of course, have consistently responded to AELMHU's fundamental mission: to promote the therapeutic and social value of orphan drugs and foster their equitable and early access for those most in need, focusing on two strategic lines. First, by facilitating the generation of reliable data that informs evidence-based decision-making through these reports; and second, by fostering ongoing dialogue among all stakeholders in the healthcare system: public administrations and regulatory agencies, the pharmaceutical industry, industry professionals, and patients.

Throughout the year, we have maintained constant contact with government agencies, scientific societies, patient associations, and other relevant stakeholders, convinced that only through collaboration can we move toward more effective and sustainable solutions.

I would like to take this opportunity to sincerely thank all AELMHU members, whose commitment and daily efforts drive our activity. Thanks to their involvement, the association has been able to consolidate initiatives and open new avenues for collaboration that enrich the ecosystem of rare and ultra-rare diseases.

I also thank the patient associations for their willingness, their constant support, and their trust in our work. They are the true reason for AELMHU's existence and the inspiration for us to continue working with enthusiasm and rigor.

I also extend my gratitude to our technical team, collaborators, and external experts, whose work, commitment, and involvement consolidate AELMHU as one of the leading organizations in both the orphan drug sector and the pharmaceutical industry.

Looking to the future, we are aware that the road ahead will continue to pose many challenges. The rapid pace of scientific advances, the need to adapt to evolving regulatory frameworks, and the imperative to ensure the equity and sustainability of the healthcare system require a coordinated, thoughtful, and consistent response from all stakeholders. At AELMHU, we are convinced that with dialogue, transparency, and a clear, patient-centered approach, we can transform expectations into tangible realities.

For this reason, and on behalf of the Association, with that future in mind, we express our commitment to continue leading and supporting these processes so that they become actions, always with the ultimate goal of improving the quality of life of people affected by rare and ultra-rare diseases in Spain.

Thank you all very much for joining us during this exciting time.

Beatriz Perales
President of AELMHU

02

GOVERNING AND REPRESENTATIVE BODY

02. GOVERNING *and* REPRESENTATIVE BODY

2.1 *GOVERNING BODY*

The **General Assembly** is the governing body of the Association, composed of each and every member (through a designated representative) in their own right and with absolute equality, which adopts its agreements by the majority principle or internal democracy.

2.1.1 *New partners*

During 2024, AELMHU added **two new associates**, bringing the total number of associated companies to 25. These additions were:

AOP HEALTH:

Global immunology company developing antibody-based medicines for patients suffering from serious autoimmune diseases and cancer.

ASCENDIS PHARMA:

A leading global biotechnology company dedicated to the research and development of products for the treatment of complex and devastating diseases. Its focus spans areas such as neurology, neuropsychiatry, specialized immunology, and rare diseases.

AOP HEALTH

ascendispharma

2.1.2 Meetings of the Governing Body

The Governing Body met five times throughout 2024, both in person and online, to discuss matters of interest and current relevance to the Association:

DAY	MONTH	CALL FOR MEETINGS	TOPICS DISCUSSED	FORMAT
8	February	Extraordinary Assembly	• Strategic Plan	On-line
18	March	Extraordinary Assembly	• Strategic Plan • Update of actions in recent months	On-line
31	May	Ordinary Assembly	• Approval of 2023 accounts • Elections to the Representative Body	Hybrid, in-person in Madrid
1	October	Update meeting	• Strategic Plan • Update of actions in recent months	On-line
28	November	Ordinary Assembly	• Strategic Plan Monitoring • 2025 Budget	Hybrid, in-person in Madrid

2.2 *REPRESENTATIVE BODY*

The Association’s representative body **is the Board of Directors,** which is responsible for its administration and representation.

The Board of Directors is elected by free and secret vote of the members. This representative body is responsible for making strategic decisions and managing the Association's affairs, acting on its behalf and safeguarding its interests.

2.2.1 *Elections to the Representative Body*

In May 2024, the Association held Board of Directors elections during its first Ordinary Assembly of the year, at which **Beatriz Perales,** Director of Market Access and Institutional Relations at Sobi Iberia, was appointed President of AELMHU for a two-year term, in accordance with its bylaws.

Beatriz assumed the position, replacing María José Sánchez, General Manager of CSL Behring in Spain and Portugal, who led the Association from 2021 to 2024. Following the May Assembly, María José became Vice President.

At the meeting, representatives and the technical team praised and thanked **María José Sánchez for her outstanding work** during her time at the helm of the Association.

The new Representative Body also incorporated **José Luis Moreno**, General Manager for Italy, Spain and Portugal of Ultragenyx, as secretary; **Sergio Bullón**, General Manager for Spain of Alnylam Pharmaceuticals, as treasurer for another term; and **Iván Silva**, Director of Market Access for the Southern Cluster and Executive Director for Spain at Kyowa Kirin, as a member.

With this new Board, the Association reaffirmed its commitment, and that of its member companies, to improving the situation of individuals and families affected by rare or infrequent diseases, promoting awareness of their pathologies, research, and recognition of the therapeutic and social value of orphan drugs.

BOARD OF DIRECTORS 2024-2026

Beatriz Perales

PRESIDENT

Director of Market Access, Institutional Relations and Communication at Sobi Iberia

Mª José Sánchez

VICE PRESIDENT

General Manager at CSL Behring for Spain and Portugal

José Luis Moreno

SECRETARY

VP & General Manager for Italy, Spain and Portugal **at Ultragenyx Pharmaceutical**

Sergio Bullón

TREASURER

VP, Country Manager Spain at Alnylam Pharmaceuticals

Iván Silva

BOARD MEMBER

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

03

ACTIVITIES

03. ACTIVITIES

3.1 *REPORTS*

In line with the Association's mission to contribute improving health and quality of life of people affected by rare diseases, AELMHU, since its inception, has promoted the development and access to reliable, accurate, and impartial information, in order to facilitate ongoing monitoring progress in the development of orphan therapies, as well as to identify obstacles that limit patient access to these types of treatments.

During 2024, the Association had a prominent and extensive activity in the field by promoting **five reports**:

- **Annual Report on Access** to Orphan Drugs in Spain 2023.
- **Two Four-Monthly Reports on Access** to Orphan Drugs 2024.
- **Annual Report on Clinical Trials** for Rare Diseases 2023.
- Report **"Differential Aspects of Orphan Drugs and Their Value from a Social Perspective",** by the Weber Foundation, with the support of the Association.

These sources of information have become fundamental documents for understanding the evolution of rare diseases and the situation of orphan drugs in our country.

3.1.1 *Access Reports*

AELMHU publishes annual and four-monthly reports on Access to Orphan Drugs in Spain, an analysis that has established itself as a benchmark in both the pharmaceutical sector and the field of rare diseases.

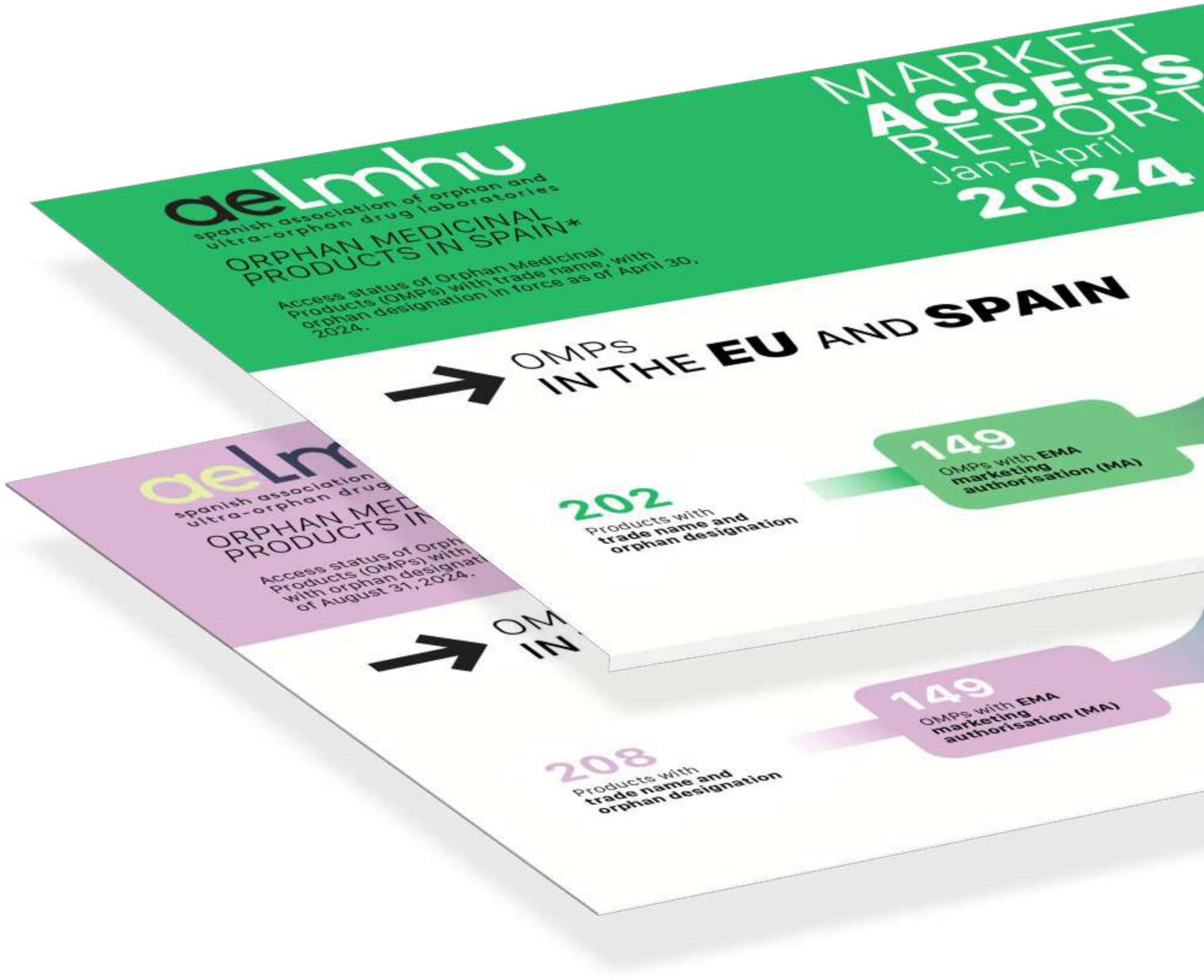
The reports focus on assessing the number of available treatments, approval times, and reimbursement status in Spain, based on data obtained from public sources within the European Union and the Spanish Ministry of Health.

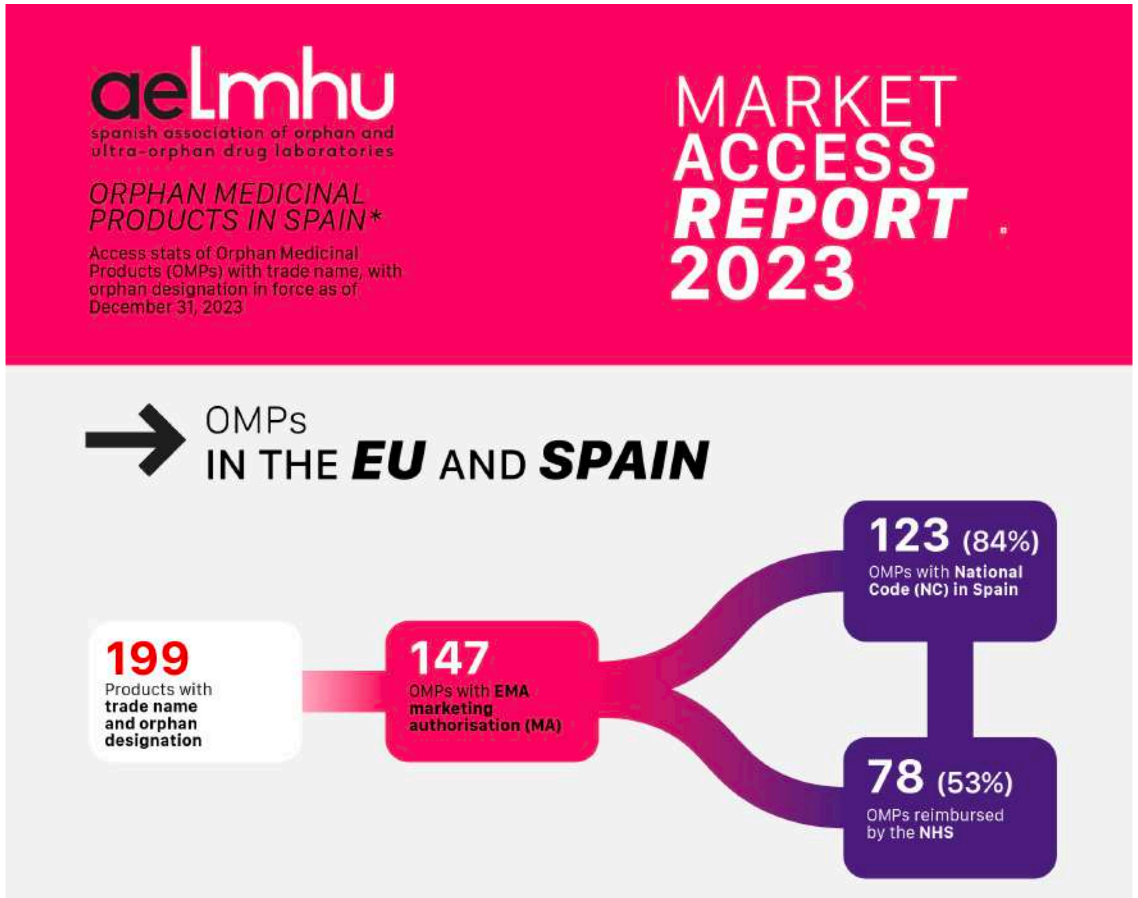
These updates provide a detailed overview of access to orphan therapies in our country.

Regarding the 2023 data, the Annual Access report counted 199 medicines with brand names and orphan designation by the European Medicines Agency (EMA), of which **147 had marketing authorization in the European Union.**

[You can access the Access Reports here.]()

At the national level, 10 new national codes (NC) were assigned, **bringing the total number of drugs with NC in Spain to 123**, and public reimbursement for 21 new orphan drugs**, bringing the total number of products reimbursed by the National Health System (NHS) to 78.**





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spanish association of orphan and ultra-orphan drug laboratories

*ORPHAN MEDICINAL PRODUCTS IN SPAIN**

Access stats of Orphan Medicinal Products (OMPs) with trade name, with orphan designation in force as of December 31, 2023

MARKET
ACCESS
REPORT
2023

OMPs
IN THE ***EU*** AND ***SPAIN***

199 Products with **trade name and orphan designation**

147 OMPs with **EMA marketing authorisation (MA)**

123 (84%) OMPs with **National Code (NC) in Spain**

78 (53%) OMPs reimbursed by the **NHS**

EXECUTIVE SUMMARY
Annual Report on
ACCESS
2023
of Orphan Medicinal Products (OMPs) in Spain

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Access to OMPs with existing EMA orphan designation and trade name as of 31 December 2023

These data revealed that 84% of orphan drugs approved in Europe reached our country, although only 53% of them were reimbursed by our NHS. Of these, almost half had restrictions on their approved indications or indications that were not reimbursed.

One of the most significant advances reflected in the report **was the reduction in the average time required to obtain a reimbursing decision, which fell from 34 months in 2022 to 23 months in 2023**, falling below the two-year threshold for the first time.

Despite this progress, the report revealed that **45 orphan drugs were still awaiting reimbursement in Spain**, and that 49% of them remained unresolved.

Furthermore, for the second consecutive year, **no advanced therapy received reimbursement from the NHS**, highlighting the need to review current criteria and procedures.

Regarding the first four-month update of 2024, between January and April, **9 new National Codes were assigned and 9 new medicines obtained orphan designation**, reaching **128 orphan products with National Codes, of which 85 were reimbursed in our country.**

On the other hand, the **waiting time** between obtaining the national code and its reimbursement remained at **23 months in the first four months of 2024, resulting in 43 orphan drugs with a National Code being unreimbursed** in our country.

For the second half of the year, the four-month report, covering the months between May and August, reflected the incorporation of **seven new orphan drugs, four treatments** obtained marketing authorization, **and seven more received the National Code**, while **three** were reimbursed.

132 orphan products had a national code, of which **85 were reimbursed, equivalent to 64% of the total.**

AELMHU's publications, in addition to being considered documents of interest to its *stakeholders*, have positioned the Association as an authoritative voice in the field of rare diseases and orphan drugs.

For the Association, it is a real responsibility to know that its reports have become the main reference for data on access to orphan and ultra-orphan drugs in Spain.

Market
ACCESS
REPORT
2024
Jan-April
on Orphan Medicinal
Products in Spain
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Analysis of trade-named OMPs with European Orphan Designation in force as of April 30, 2024

prepared by: bio innova Consulting

Market
ACCESS
REPORT
2024
May-August
on Orphan Medicinal
Products in Spain
aelmhu

Analysis of trade-named OMPs with European Orphan Designation in force as of August 31, 2024.

prepared by: bio innova Consulting

3.1.2 Report on Clinical Trials in Rare Diseases

The **Report on Clinical Trials in Rare Diseases in Spain, published annually on International Clinical Trials Day, celebrated on May 20,** highlights the importance of continuing to foster innovation in Spain in the field of clinical trials, and reaffirms the commitment of AELMHU members to research and development of new therapies.

The report, with 2023 data, revealed that **834 clinical trials were authorized in Spain that year, 190 of which were for rare diseases**, although this figure represented a 10% decrease compared to 2022 data.

On the other hand, on a positive note, it highlighted the significant increase in clinical trials in the early stages of development, which grew **by 19% compared to the previous year.**

The pharmaceutical industry has established itself as the main promoter of clinical trials in rare diseases in Spain, **accounting for 96% of the total, one point more than the previous year.**

As a summary of the data from the last five years, **between 2019 and 2023, 4,610 clinical trials were carried out in Spain, of which 1,032 (22%) focused on rare diseases.**

You can access the Clinical Trial Reports here.

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REPORT on
CLINICAL
TRIALS for
RARE
DISEASES
in SPAIN
2023

3.1.3 Report on the Social Value of Orphan Drugs

Developed by the Weber Foundation in collaboration with AELMHU, the report **"Differential aspects of Orphan Drugs and their Value from a Social Perspective“** analysed the specificities of these therapies and the value they generate in different areas of society, in addition to the substantial benefit they offer to the health and quality of life of patients with rare diseases and their communities.

DIFFERENTIAL
ASPECTS
OF ORPHAN
DRUGS
AND THEIR VALUE
FROM A
SOCIAL
PERSPECTIVE

Therefore, having effective treatments for rare diseases has a positive impact on areas such as employee productivity and personal care needs and burdens, and can lead to significant reductions in direct and indirect costs for both the healthcare system and society.

For example, the Report noted that introducing treatments for metabolic diseases can reduce direct costs by more than 80%, and that indirect costs for haematological and immunological diseases can be reduced by up to 88%.

Thanks to the collaboration agreement between the Weber Foundation and AELMHU, the Report was presented at the 5th **NewsRare Summer Course**, held on September 25 and 26, 2024.

You can access the Report on the Social Value of Orphan Drugs here.

The first day of the course, sponsored by the Association, featured an exceptional opening panel, with the participation of **Sergio Bullón,** member of the Board of Directors of AELMHU, **José Luis Poveda**, head of the Pharmacy Service at La Fe Hospital in Valencia, and **Álvaro Hidalgo**, President of the Weber Foundation.

Next, **Néboa Zozaya**, a health economist and one of the authors of the Report, spoke. She explained the document's content, which aims to provide an updated and comprehensive overview of Orphan Drugs, highlighting their clinical, healthcare and social value.

The challenges and advances in orphan drug research, access, and regulation were also addressed, as well as the lack of reimbursement and epidemiological data, regional inequalities in access to these drugs in Spain, and the urgent need to establish more agile and efficient regulatory processes.

Subsequently, two roundtable discussions took place, the first **on 'Research into orphan drugs'**, moderated by **Álvaro Hidalgo**, President of the Weber Foundation, with **Fernando Méndez**, from the EMA Orphan Drugs Committee, **Jorge Francisco Gómez,** coordinator of the Spanish Society of Internal Medicine (SEMI), and **Rocio Sánchez-Carpintero**, President of the Spanish Society of Pediatric Neurology (SENEP), to address the challenges and opportunities in orphan drug research, where the need for collaboration between agencies, scientific societies and clinicians to address these pathologies was highlighted.

The second, focused on **'Access and equity in orphan drugs',** featured the interventions of **Ana Rosa Rubio**, coordinator of the Pharmacy Area of the Health Service of Castilla La Mancha (SESCAM), **Elena Casaus**, head of the Division of the Advanced Therapies Unit of the Madrid Health Service (SERMAS), and **Juan Carrión**, President of the Spanish Federation of Rare Diseases (FEDER), where the inequalities in access to orphan drugs between autonomous communities were discussed, putting forward proposals to improve the equity and sustainability of the system and placing special emphasis on the need for a coordinated national strategy.

4th Rare Disease Protagonist of the Year Award was presented, in recognition of the 22q11 Syndrome Association 's significant contribution to improving the quality of life of patients with rare diseases.

The closing ceremony was led by **César Hernández**, General Director of the Common Portfolio of Services and Pharmacy of the Ministry of Health, who addressed the current challenges surrounding access to and financing of orphan drugs.

3.2 *AELMHU* *MEETINGS*

Given its strong reception in previous years, in 2024 the Association continued to hold specialized and exclusive meetings for its members. The goal was to create a space for debate with relevant third parties from the healthcare and political sectors, with the aim of promoting dialogue, sharing knowledge, and exchanging opinions, experiences, and ideas on an area or topic relevant to all stakeholders involved.

Proof of this was the more than **100 attendees at the eight meetings** held in 2024:

JUNE: 2 meetings

- **Elena Casaus,** Head of Division of the Advanced Therapies Unit of the Community of Madrid.
- **Rubén Moreno,** former Secretary General of Health and Consumer Affairs, Ministry of Health, Social Services and Equality.

JULY

- Visit to the **Red Únicas Building and the Sant Joan de Déu Hospital** (Barcelona).

SEPTEMBER

- Meeting with **Milena Peraita**, responsible for Compassionate Use at the Spanish Agency for Medicines and Health Products (AEMPS).

OCTOBER

- Meeting with **Raquel Yotti**, Commissioner of the Strategic Plan for Economic Recovery and Transformation (PERTE) for Cutting-Edge Health, of the Ministry of Science, Innovation and Universities.

NOVEMBER: 3 meetings

- **Eleni Pitta** and **Ana Rossignoli**, members of the HTA Coordination Group/Methodology Subgroup. IPT and ETS Area of the AEMPS.
- **Domingo Lamuño**, President of the Spanish Association of Reference in Inborn Errors of Metabolism (AECOM).
- **Lluís Alcover and Claudia Gozalo,** lawyers at the Faus & Moliner law firm.


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ENCUENTRO CON
ELENI PIT... ANA ROSSIGNOLI


aelmh
asociación española de laboratorios de
medicamentos huérfanos y ultrahu


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3.3 II NATIONAL CONFERENCE OF ADVANCED THERAPIES for RARE DISEASES

The event, held at the Camilo José Cela University Auditorium, featured speakers from various members of the public administration, national associations, employers' organizations, and various healthcare professionals.

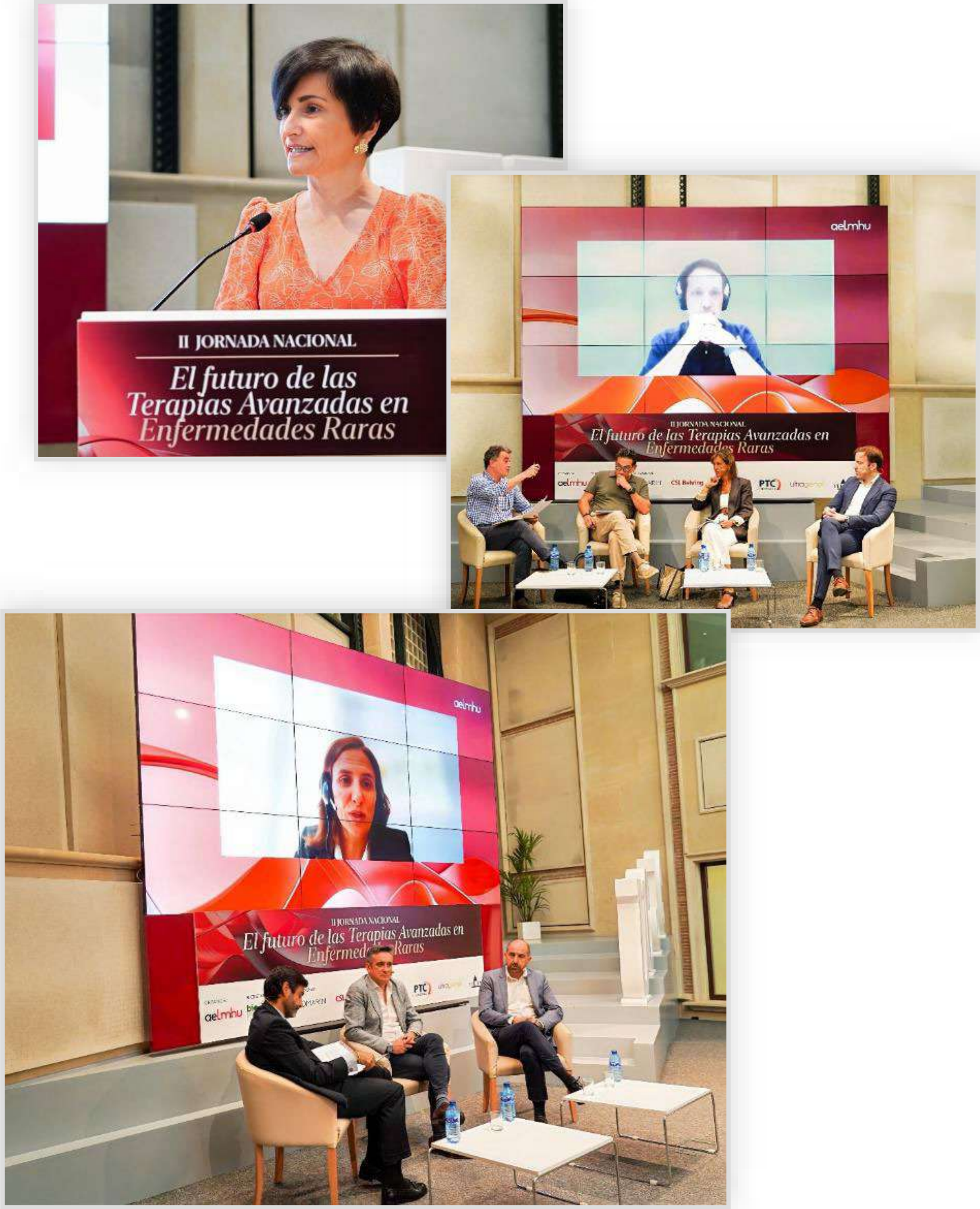
During the session, the importance of adopting a collaborative approach to overcome the regulatory and economic challenges that limit access to innovative treatments was emphasized.

The conference was opened by **Raquel Yotti**, PERTE commissioner for Cutting-Edge Health of the Ministry of Science, Innovation and Universities, and **Beatriz Perales, President of AELMHU**, and featured outstanding speakers such as **Sol Ruiz, head of the Division** of Biological Products, Advanced Therapies and Biotechnology of the AEMPS, and **Álvaro Hidalgo**, health economist, professor and Director of the Health Economics and Healthcare Management Research Group at the University of Castilla La Mancha (UCLM).

Marian Corral, Executive Director of AELMHU, then acted as master of ceremonies and introduced the two main discussion panels of the day:

The first, entitled "**Evaluation, Reimbursement and Public Procurement**", was moderated by health economist **Jorge Mestre. The following participated: José Luis Poveda**, manager of the La Fe University and Polytechnic Hospital; **Juan Manuel Fontanet**, representative of the Medicines Department of the Catalan Health Service; **Elena Casaus**, head of the Advanced Therapies Unit Division of the Community of Madrid; and **Lluís Alcover**, a lawyer specialized in pharmaceutical law at Faus & Moliner Abogados.

At the second table, **'Clinical Advances and Challenges,** moderated by ***Borja Smith***, CEO of BioInnova Consulting, featured speakers including **Víctor Jiménez**, Head of the Haematology Department at La Paz University Hospital; **Alessandra Magnani, Head of the Advanced Therapies and Immunotherapy Platform at Sant Joan de** Déu Hospital; and **Javier García**, Director of the Advanced Therapies Drug Development Department at ISCIII.



The speakers at both panels agreed on the need to increase investment in research for rare diseases, improve regulation to make it more efficient and flexible, implement purchasing policies that guarantee affordable prices, and consider non-economic factors when evaluating orphan therapies. They also highlighted the urgency of establishing mechanisms to ensure equitable and sustainable access to treatments for patients who require them, fostering collaboration among the various stakeholders in the healthcare system to achieve this common goal.

In closing remarks, **Isabel Motero,** Director of the Spanish Federation of Rare Diseases (FEDER), represented the patients, emphasizing the importance of streamlining access to these therapies, given their impact on the lives of those suffering from rare diseases.

Iván Silva, member of the AELMHU Board of Directors, closed the event by thanking all participants for their involvement, highlighting the success of the meeting, and the need to collaborate and work together to address rare diseases and their treatments.

The event was made possible thanks to the support of BioMarin, CSL Behring, Novartis, PTC Therapeutics, Ultragenyx and Vertex, companies associated to AELMHU.

3.4 *ACTIVITIES in COLLABORATION with the SPANISH FEDERATION of RARE DISEASES (FEDER)*

During 2024, various training activities were carried out together with FEDER, divided into two themes:

- **Training sessions on access to orphan drugs**

Offered **to help the association movement** understand the process of development and approval followed by rare disease medicines. During these sessions, an overview of the journey of these medicines was presented, covering their clinical development, orphan drug designation, marketing authorization (both at the European and national levels), pricing and reimbursement decisions, and finally market launch.

These training sessions **were delivered in segments:** first, participants with a beginner's level in the access topic were offered in person, followed by participants with intermediate and advanced levels, offered online.

Training for spokesperson

The spokesperson training session focused **on improving the communication skills of the Federation's new spokespersons.** The objective of this session was to provide the necessary tools to a group composed of members of the Board of Directors and the management of FEDER, so that they could develop their communication skills and effectively perform their role as spokespersons. To this end, it was carried out in person and included specialized media trainers, such as Elena Argandoña, a **journalist expert in Corporate Communication and communication skills trainer.**

These training sessions were part of the collaboration agreement between FEDER and AELMHU, and on this occasion were led by the **Co-creation with Patient Associations working group.** In this way, AELMHU reiterated its commitment to raising awareness and visibility of rare diseases, as well as access to their treatments, while also promoting close collaboration with patient associations.

SESIÓN FORMATIVA ACCESO A LOS MEDICAMENTOS HUÉRFANOS.

NIVEL AVANZADO

DR. JORGE MESTRE-FERRÁNDIZ
CONSULTOR ECONÓMICO INDEPENDIENTE
PROFESOR ASOCIADO, UNIVERSIDAD CARLOS III, MADRID
ACTIVIDAD REALIZADA EN EL MARCO DE LA **COLABORACIÓN ENTRE:**

feder aelmhu

25 NOVIEMBRE 2024

3.5 *INTERVIEWS with SECTOR STAKEHOLDERS*

Since September 2024, as an initiative proposed by the **Co-creation with Patient Associations working group,** a series of interviews have been published with various key stakeholders in the healthcare sector, including physicians, patients, industry associations, and experts, with the aim of raising the awareness of rare diseases and orphan drugs.

Through these conversations, we sought to raise awareness about the challenges faced by both patients and healthcare professionals, as well as highlight the importance of research and access to innovative therapies that improve the quality of life of those suffering from these rare diseases.

In total, from September to December 2024, a total of 11 interviews were published:

SEPTEMBER

- **Elena Arcega,** President of the Association for the Fight Against Inflammatory Biliary Diseases, Albi Spain.
- **Peter Löffelhard,** President of the Association of People Affected by Myeloproliferative Neoplasms, MPN Spain.
- **Cristina Pascual,** expert in haematological diseases and treasurer of the Spanish Apheresis Group (GEA) and President of the Spanish Immune Thrombocytopenia Group (GEPTI).

OCTOBER

- **Doctor Miguel Ángel Torralba** and **Marisol Prieto,** patient and President of the Spanish Association of Gaucher Disease Patients and Relatives (AEEFEG).
- **Dr. Begoña Polo,** from the Pediatric Gastroenterology and Hepatology department at La Fe Hospital in Valencia.
- **Dr. Montserrat Morales,** coordinator of the Adult Rare Diseases and Inborn Errors of Metabolism Unit at the 12 de Octubre University Hospital in Madrid.
- **Sonia Fernández,** President of the Spanish Patients' Association of Inherited Rickets and Osteomalacia (AERYOH).
- **Rosa Pérez,** President of the Spanish Association against Familial Chylomicronemia and a patient with this same rare disease.

NOVEMBER

- **Jordi Cruz,** Director of MPS Lysosomales, and **Dr. Álvaro Hermida,** clinician at the Congenital Metabolic Diseases Diagnosis and Treatment Unit at the University Clinical Hospital of Santiago.

DECEMBER

- **Dr. Jorge Francisco Gómez Cerezo,** head of the Internal Medicine Service at the Infanta Sofía University Hospital and Coordinator of the Minor Diseases Group of the Spanish Society of Internal Medicine (SEMI).

3.6 *AELMHU AWARDS*

The AELMHU Awards have established themselves as a **tradition and a benchmark** in the pharmaceutical and research sectors, as well as in associations for patients with rare diseases.

The objective of these awards is **to recognize the work of professionals and organizations and to distinguish the best projects in healthcare, dissemination, and awareness-raising regarding rare diseases.**

Participation was open to individuals and legal entities dedicated to research, dissemination, or raising awareness about these types of diseases, residing or operating in Spain. The following categories were distinguished:

AWARD FOR THE BEST CARE PROJECT ON RARE DISEASES

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

AWARD FOR THE BEST RARE DISEASE OUTREACH PROJECT

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

AELMHU HONORARY AWARD

The 2024 edition once again featured an external, independent jury composed of the following **renowned professionals** in the fields of health and communication, including research in these diseases:

- **Jesús Aguilar,** President of General Pharmaceutical Council of Spain (CGCOF)
- **Eva Bermejo,** Director of the Rare Diseases Research Institute at the Carlos III Health Institute (ISCIII).
- **Susana Fernández,** Vice President of the National Association of Health Informants (ANIS).
- **Carmen Fons,** Member of the Spanish Society of Pediatric Neurology (SENEP).
- **Javier García.** President of the Federation of Spanish Scientific Medical Associations (FACME).
- **Antoni Gilabert,** Director of Innovation and Partnership at the Health and Social Consortium of Catalonia.
- **Jorge Francisco Gómez.** Coordinator of the Minor Diseases Group of the Spanish Society of Internal Medicine (SEMI).
- **María Esperanza Marcos.** President of the Health Law Section of the Madrid Bar Association.
- **Jorge Mestre.** Health economist.
- **Jordi Nicolás,** Vice President of the Spanish Society of Hospital Pharmacy (SEFH).
- **Manuel Pérez,** Vice President of the Orphan Drugs and Rare Diseases Foundation (MEHUER).
- **Belén Pérez.** Vice President of the Spanish Association of Human Genetics (AEGH).
- **Raquel Sánchez.** Lawyer and spokesperson for the 2023 Honorary Award.
- **Marian Corral.** Director of AELMHU (without right to vote).

The jury meeting took place on September 29, and after the winners were announced, work began on organizing the awards ceremony, which was held in November.

AWARDS CEREMONY

Journalist **María Rey** was the master of ceremonies for the awards ceremony.

During the opening, **Beatriz Perales, President of AELMHU**, expressed her gratitude for the outstanding participation and the work done by the jury. She also praised the work of the members throughout the year **and offered a few words in memory of the victims of the DANA disaster in Valencia**, which also prevented one of the award winners from being able to attend the ceremony and receive the award.

The evaluation and selection process for the awardees were presented by **Raquel Sánchez,** lawyer and 2023 Honorary Award, representing the jury. In her remarks, she congratulated the winners for the excellence of their projects and encouraged them to continue their research efforts to achieve breakthroughs that benefit the lives of patients.

AWARD FOR THE BEST HEALTHCARE PROJECT ON RARE DISEASES FOR THE FIRST STAR MARÍA DE VILLOTA PROGRAM OF THE ANA CAROLINA DÍEZ MAHOU FOUNDATION

This program combines medical, psychological, and sports care to improve the quality of life of these young patients. The award was accepted **by Javier Pérez-Mínguez,** Director of the Ana Carolina Díez Mahou Foundation, who emphasized the importance of continuing to work to offer a better future to those affected by these pathologies. The award was presented by **Belén Pérez,** vice President of the Spanish Association of Human Genetics, and **Raquel Sánchez.**

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

It was for **Lluís Montoliu José,** a leading researcher in the field of albinism and a pioneer in applied biotechnology and gene editing, received the award from **María Esperanza Marcos,** President of the Health Law Section of the Madrid Bar Association, and **Jorge Mestre,** health economist.

AWARD FOR THE BEST RARE DISEASE OUTREACH PROJECT

'Run, stop Ataxia Telangiectasia', by the Spanish Association of Families Affected by Ataxia Telangiectasia (AEFAT), was the award-winning project. Rosa Casbas, the association's Communications Manager, and Álvaro de Arqués, one of the members and affected by this rare disease, collected the award

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

It was awarded **to José Luis Poveda Andrés,** manager of the La Fe University and Polytechnic Hospital, who could not attend the awards in person due to the situation that was being experienced at that time in Valencia with the DANA and participated via *streaming.*

In December, AELMHU President **Beatriz Perales** and Director **Marian Corral** visited La Fe Hospital in Valencia to present José Luis Poveda with his award and thank him for his work in the field of rare diseases.

AELMHU HONORARY AWARD 2024

Chosen by all AELMHU Associates, it was awarded (posthumously) **in recognition of the career and efforts of Dr. Josep Torrent- Farnell.** The award was presented by **César Hernández,** General Director of the Common Portfolio of Services and Pharmacy of the National Health System, and **the members of the AELMHU Board of Directors.**

Iolanda Arbiol, Director of the Dr. Torrent- Farnell Foundation, who was fortunate enough to share many years working alongside Dr. Torrent, wished to offer a few heartfelt words of recognition for his tireless work improving the lives of patients with rare diseases. **The award was then presented to his family, his sister Carme and his nephew,** who were deeply grateful for the tribute.

César Hernández closed the event by highlighting the social commitment of all those involved in supporting people with rare diseases. He emphasized that the Ministry of Health of Spain is working to accelerate the inclusion of new medications in the public portfolio, with the goal of facilitating their access for patients. However, he also acknowledged that there are still challenges to be addressed, such as the diagnosis and screening of these diseases, as well as improving healthcare for those affected.

The gala concluded with a presentation by **Cisco García,** a lawyer who, after a snowboarding accident in December 2015, decided to reinvent himself and dedicate entirely to wheelchair tennis, a sport in which he made his professional debut in 2018.

Cisco, in addition to moving the audience with his story of overcoming difficulties, brought the gala to a close, once again filled with sensitivity.

AELMHU's YouTube channel

AELMHUU Awards Gala


aelmhu
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Premios aelmhu 2024


aelmhu
mhu

04

WORKING GROUPS

04. *WORKING* **GROUPS**

With the goal of addressing specific areas in a targeted, agile, and efficient manner, the Association continued to rely on its working groups, carrying out **actions aligned with current political and institutional developments,** as well as with advances in the healthcare sector and other sectors relevant to its members.

During the 2024 financial year, AELMHU worked with four active groups:

- **Access**
- **Advanced Therapies**
- **Political Affairs and Legislative Agenda**
- **Co-creation with Rare Disease Patient Associations**

The groups, which each have **two spokespersons,** meet monthly to share information of interest and exchange opinions on current issues for companies.

All of this is done **with the aim of promoting projects, identifying areas of work, creating positioning documents, and designing and implementing activities of interest,** in accordance with the objectives set by the strategic plan approved by the Association's General Assembly.

Throughout 2024, companies' interest in joining these working groups grew stronger, **and they have been strengthened by the addition of new members, bringing the total to more than 60 across the four groups.**

CHALLENGES in the ADVANCED THERAPIES APPROACH for RARE DISEASES

Document made by the Advanced Therapies for Rare Diseases Working Group

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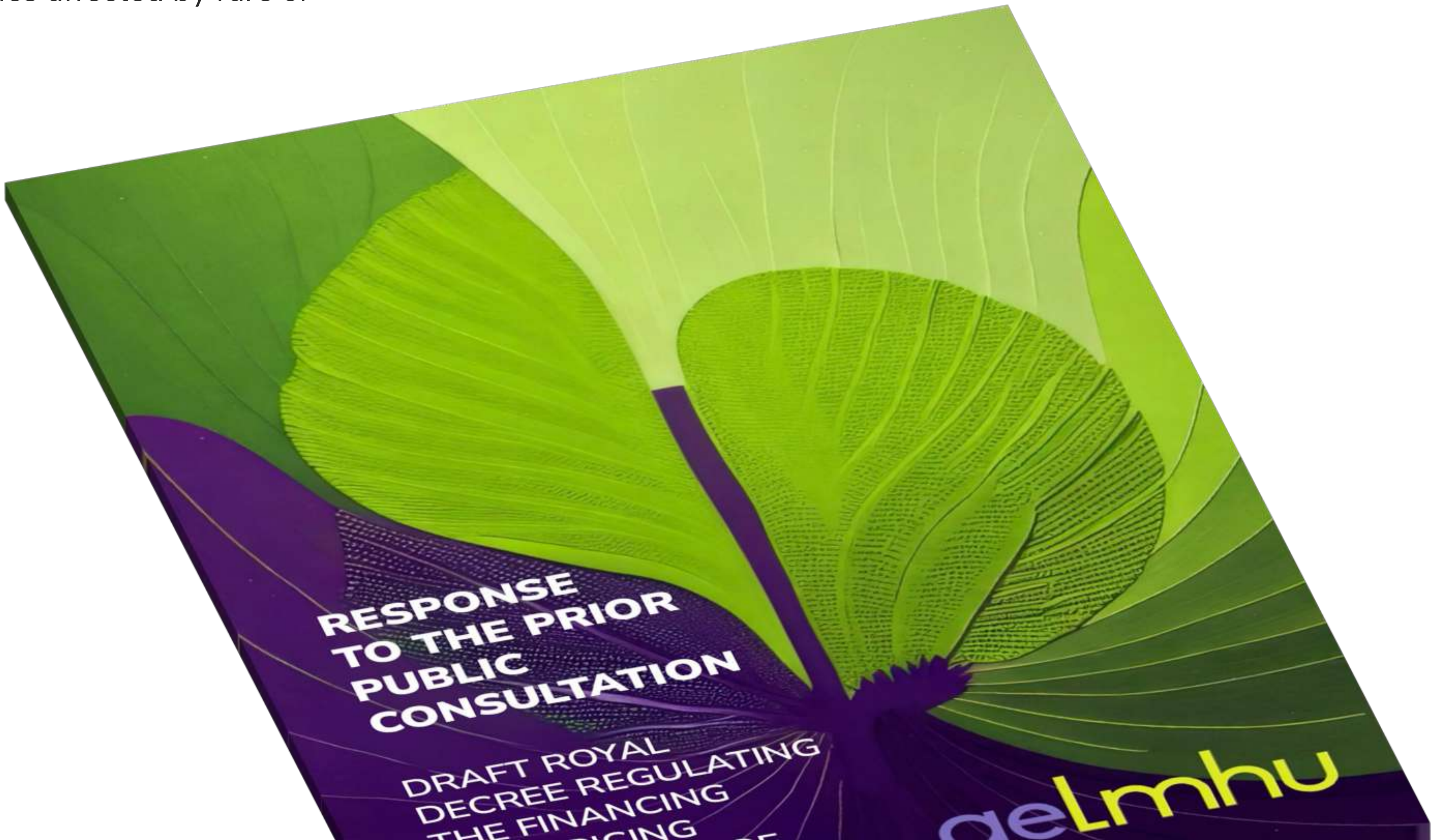
Various documents were developed through the working groups, such as the document on the Challenges of Advanced Therapies for Rare Diseases. These documents served as a platform for meetings with key decision-makers of interest to the groups, both at the regional and national levels, to demonstrate the Association's position on a specific issue or to analyse specific situations and offer alternatives to address current challenges.

Always with a common goal: to promote knowledge of pathologies and recognition of the therapeutic and social value of orphan drugs, and to improve the situation of individuals and families affected by rare or infrequent diseases.

With these documents, **the working groups had the opportunity to convey AELMHU's position** on issues of concern to key decision-makers in the field.

Along these same lines, **several groups worked together to develop and agree on documents** with recommendations that would facilitate participation in public consultations and hearings at the regional, national, and European levels, such as the **positioning of the Royal Decree on Pricing and Reimbursement or the Royal Decree on Health Technology Assessment (HTA).**



RESPONSE
TO THE PRIOR
PUBLIC
CONSULTATION

DRAFT ROYAL
DECREE REGULATING

Co-creation with Patient Associations working group, which has collaborated with FEDER for several years, implemented various training activities for its technical team, Board of Directors, and associated patient organizations.

Also, within this collaborative framework, three meetings were held between the two organizations at FEDER's offices in Madrid to share reflections, opinions, and other issues of interest to both entities.

In addition, the groups were able to collaborate and learn about institutions and their projects, such as the **Spanish Association for the Study of Inborn Errors of Metabolism (AECOM) or the Sant Joan de Déu** and its Únicas project to improve care for patients with rare diseases.

In this way, and thanks to the promotion and development of new initiatives, documents, events, and alliances within the various working groups, **AELMHU was able to continue its work raising awareness among public administrations about the need to continue researching in the field of rare diseases,** in order to accelerate accurate diagnosis and guarantee rapid and equitable access to orphan drugs.

05

INSTITUTIONAL RELATIONS

05. INSTITUTIONAL RELATIONS

Throughout 2024, AELMHU continued to play a key role in raising awareness about research into rare diseases, highlighting the importance of access to innovative treatments and early diagnosis in addressing these conditions.

The Association prioritized **building strong and well-founded ties with *stakeholders*** from key entities and institutions, both public and private, establishing itself as a qualified representative and leader in the field of orphan drugs and rare diseases.

Institutional work focused **on ensuring that AELMHU maintains significant influence in decision-making processes** and is considered an authoritative voice in the field of rare diseases and orphan drugs.

For this reason, and in line with its strategic plan, AELMHU intensified its political agenda to understand the new priority lines of work of government teams, both at the central and regional levels, and to seek new opportunities for collaboration that benefit people and families affected by rare diseases.

5.1 *PUBLIC PARTICIPATION in REGULATORY PROJECTS*

In this regard, the Association, through the collaborative work of its working groups, participated in various consultations and public hearings at the regional, national, and European levels.

PUBLIC CONSULTATION ON EARLY INTERVENTION IN THE COMMUNITY OF MADRID

In March, AELMHU presented its contributions to the preliminary public consultation on the draft *Royal Decree regulating early intervention in the Community of Madrid.* The following recommendations were made:

- **Ensuring rapid and equitable access to** disease diagnosis is a fundamental pillar in the universalization of early care.
- Including **as many rare diseases as possible in the newborn screening program** as a common and accessible tool for early detection.
- The **inclusion of neonatal screening enhancement measures** within the purview of the Madrid Early Care Technical Commission.

OBJECTIONS TO THE DRAFT OF ROYAL DECREE ON MAGISTRAL FORMULAS FOR CANNABIS PREPARATIONS

That same month, AELMHU prepared its contributions to the *prior public consultation of the draft of the Royal Decree establishing the conditions for the preparation and dispensing of magistral formulas based on standardized cannabis preparations*, suggesting that:

- The promotion of medicines prepared using magistral formulas must take into account **the investment made in R&D&I.**
- **A regulatory provision** must be established to prevent the substitution of industrially manufactured medicines for these products.
- The **proportion of active ingredient in master formulas should be limited.**

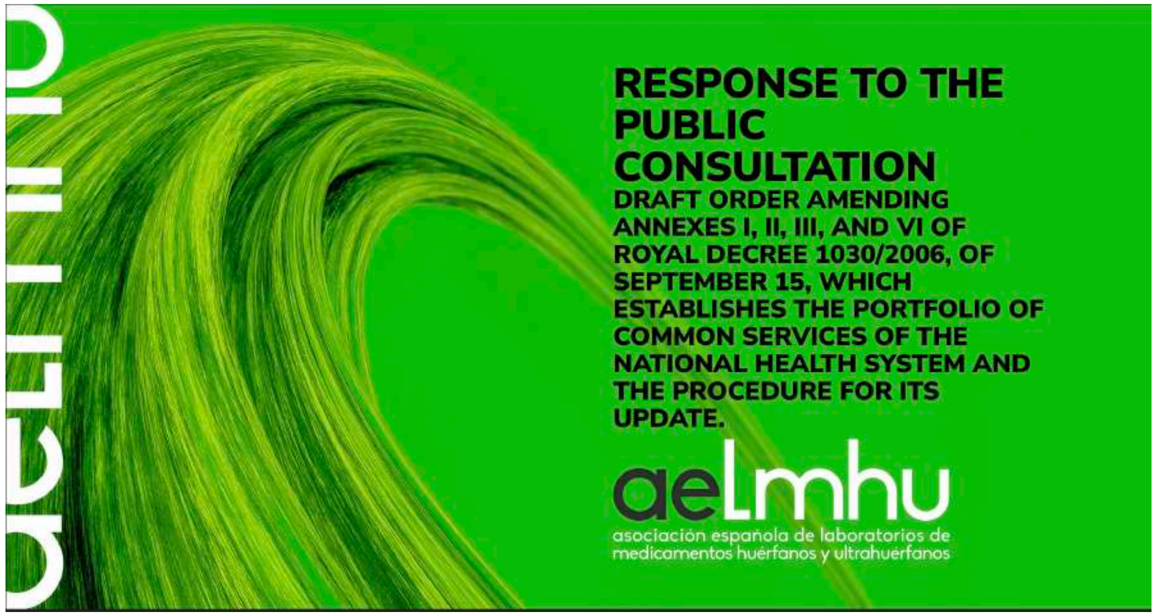
RESPONSE TO THE PUBLIC CONSULTATION

DRAFT DECREE REGULATING EARLY CARE IN THE COMMUNITY OF MADRID

PUBLIC HEARING ON SCREENING ANNEXES OF THE MINISTRY OF HEALTH

The Association participated in the *draft of the Order establishing the Portfolio of Common Services of the National Health System* and the procedure for updating it, submitting the following recommendations:

- Continue incorporating more diseases into the neonatal screening.
- Limit and **reduce the time required** for updating the common services portfolio.
- Take advantage **of regional assessments** to expedite the future inclusion of new pathologies in the neonatal screening list.
- Promote **collaboration with regional governments** in the research and development of analytical techniques and tools.
- Promote **the development by the Ministry of Health** of an information system that allows for proper monitoring and evaluation of these population programs at the regional and national levels.
- Promote **progress in the development of an annual technical** evaluation report on the screening program, which can be made available to citizens and government agencies.
- Promote the **development of lines of research in Rare Diseases.**



RESPONSE TO THE PUBLIC CONSULTATION

DRAFT ORDER AMENDING ANNEXES I, II, III, AND VI OF ROYAL DECREE 1030/2006, OF SEPTEMBER 15, WHICH ESTABLISHES THE PORTFOLIO OF COMMON SERVICES OF THE NATIONAL HEALTH SYSTEM AND THE PROCEDURE FOR ITS UPDATE.

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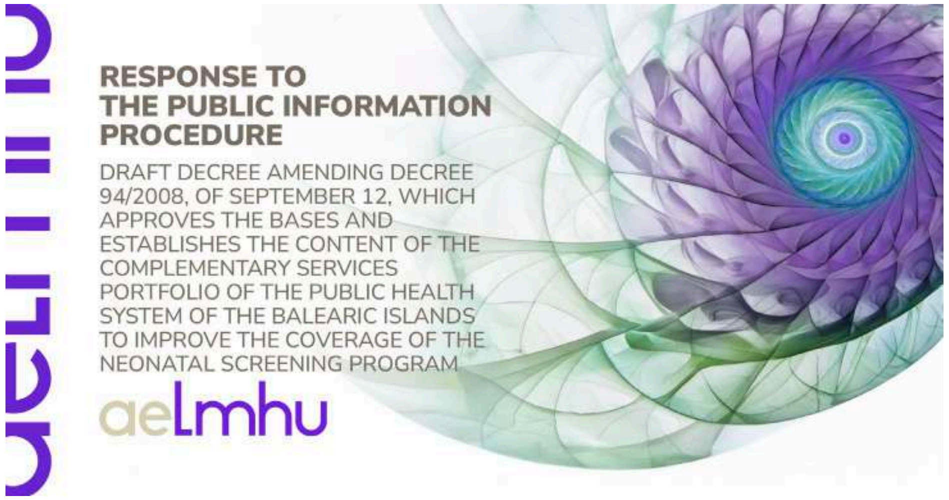
PUBLIC HEARING OF THE RARE DISEASES OBSERVATORY IN THE COMMUNITY OF MADRID

In April, AELMHU submitted its comments to the *public hearing and information process regarding the draft of the Order of the Ministry of Health establishing the Rare Disease Observatory of the Community of Madrid*, suggesting further exploration of new developments and proposals that raise awareness of less common diseases and their challenges. It also highlighted:

- The need to **address new proposals for improvement,** such as recognition of the economic and social value that pharmaceutical research brings to this field.
- Streamlining **bureaucratic processes and improving the** innovation evaluation process, among others.



**RESPONSE TO
THE PUBLIC INFORMATION
PROCEDURE**

DRAFT DECREE AMENDING DECREE
94/2008, OF SEPTEMBER 12, WHICH
APPROVES THE BASES AND
ESTABLISHES THE CONTENT OF THE
COMPLEMENTARY SERVICES
PORTFOLIO OF THE PUBLIC HEALTH
SYSTEM OF THE BALEARIC ISLANDS
TO IMPROVE THE COVERAGE OF THE
NEONATAL SCREENING PROGRAM

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PUBLIC HEARING ON NEONATAL SCREENING IN THE ISLAS BALEARES

Also in that month, the association presented its objections to the draft of *the Decree of the Government of the Islas Baleares approving the bases and establishing the content of the complementary service portfolio of the autonomous public health system*, based on the following points:

- Promote the inclusion of **as many rare diseases as possible in the neonatal screening program,** prioritizing the inclusion of all pathologies with an approved treatment included in the Basic Common Portfolio of the National Health System.
- **Recognition of the specialty in clinical genetics,** both to align with Europe, as Spain is the only country without this specialty, and to accelerate access for patients.
- Strengthen **regional collaboration to expedite the future inclusion of new pathologies** in the screening list.

PUBLIC CONSULTATION ON THE PRELIMINARY DRAFT OF THE PUBLIC HEALTH LAW OF CANTABRIA

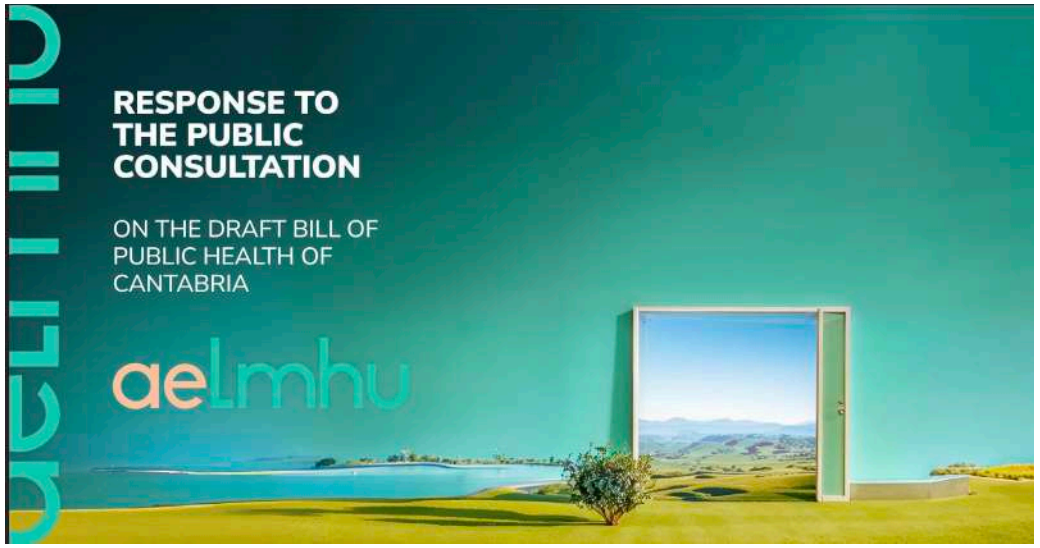
The Association also participated, in the month of April, in the prior consultation process for the preparation of the *Preliminary Draft of the Public Health Law of Cantabria*, sharing the following contributions and proposals:

- The request to **include as many rare diseases as possible in the screening program, streamlining** the process to reach 21 by 2026.
- Promoting and **fostering innovation to ensure the health and well-being of citizens throughout their lives,** through health policies that encourage therapeutic advances and research.
- **Public-private collaboration** in improving accessibility to medicines.

PUBLIC HEARING OF THE RARE DISEASES ADVISORY COUNCIL OF EXTREMADURA

In May, AELMHU submitted its contributions to the *public hearing that created the Advisory Council on Rare Diseases of the Extremadura Public Health System*, providing comments based on the following recommendations:

- The **participation of representatives from** not only regional but also national associations, who provide a holistic view of the situation regarding these types of pathologies.
- With the same objective as above, **include representatives of associations not limited exclusively to the field of patients.**



RESPONSE TO THE PUBLIC CONSULTATION

ON THE DRAFT BILL OF PUBLIC HEALTH OF CANTABRIA

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PUBLIC CONSULTATION NEONATAL SCREENING IN ARAGON

The Association submitted its comments on *the amendment to the Order of the Department of Health and Consumer Affairs regulating neonatal screening in the Autonomous Community of Aragon, based on the following recommendations:*

- Inclusion of **as many rare diseases as possible** in the neonatal screening program.
- Promote **progress in the development of an annual technical** evaluation report on the screening program.
- Limit and **reduce the time required for updating the** common services portfolio.
- Strengthen **coordination between healthcare levels** (Primary Care – Hospital) for the detection and monitoring of these patients with rare diseases.
- **Promote public-private collaboration** in the research and development of analytical techniques and tools.

RESPONSE TO THE PUBLIC CONSULTATION PROCEDURE

ORDER OF THE COUNSELOR OF HEALTH AMENDING THE ORDER OF JULY 13, 2007, OF THE DEPARTMENT OF HEALTH AND CONSUMPTION, WHICH REGULATES NEONATAL SCREENING IN THE AUTONOMOUS COMMUNITY OF ARAGON.

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PUBLIC HEARING ROYAL DECREE ON HEALTH TECHNOLOGY ASSESSMENT, MINISTRY OF HEALTH

The recommendations presented by AELMHU for this public hearing were structured in two parts. The first, with amendments, and the second, with contributions and suggestions, summarized in the following points:

- Industry participation: **greater presence of the pharmaceutical** and medical device industry in the bodies created by the Royal Decree, highlighting their knowledge and experience.
- Collaborative and consensual regulations: **methodological guides and guidelines** should be developed in collaboration with all stakeholders, including the industry.
- Developer rights and obligations: **removal of the obligation to provide R&D and production costs,** due to their complexity and lack of relevance to the evaluation; promotion of transparency without compromising patient access; adaptation of the system to the unique characteristics of each country and guaranteeing a transition period for developers.
- **Comparators must be authorized medications available in the NHS,** avoiding the use of unauthorized medications for the indication being evaluated.
- Specificity of Orphan Drugs: a specific process is requested **for the evaluation of orphan drugs,** considering the characteristics of rare diseases.
- Agile evaluation: **establish tight deadlines for completing the positioning report,** reducing access times.
- **Prioritization and non-duplication:** the goal is to avoid unnecessary re-evaluations at the regional level, except in exceptional and justified cases.
- Confidentiality and conflicts of interest: **allow the participation of experts involved in clinical trials or consulting,** especially in rare diseases, ensuring their objectivity; establish clear criteria on the impact of conflicts of interest.

PUBLIC CONSULTATION ON THE STRATEGIC PLAN FOR PERSONALIZED MEDICINE IN CASTILLA Y LEÓN

In November, the Association participated in the *public consultation of the Strategic Plan for Personalized Precision Medicine in Castilla y León* 2024-2030, through the following contributions:

- The recommendation to **include industry and patient associations within the governance framework integrated into the Plan,** so that they can provide a vision in this area.
- On the other hand, **allowing the participation of pharmaceutical industry experts within technical committees** would improve synergies and public-private collaboration.
- Limit and **reduce the time required to update and integrate new** personalized medicine services into the service portfolio.
- **Promote collaboration with regional** and commercial actors in the research and development of analytical techniques and tools.
- **Ensure rapid and equitable access** to disease diagnosis.
- **Strengthening the implementation of services included in the service portfolio** to ensure that there are no variations or inequalities in access to biomarker testing.

5.2 *INSTITUTIONAL* *MEETINGS*

Throughout 2024, the Association **held working meetings with various institutional representatives,** both with executive and parliamentary stakeholders at the national and regional levels.

The purpose of these meetings was to present AELMHU's work, as well **as to outline its position and the challenges it faces.** During these meetings, AELMHU's various projects served as a basis for disseminating key messages, such as the Access to Orphan Drugs Reports and the Report on Clinical Trials in Rare Diseases.

In addition, **proposals for improvements were discussed regarding issues of concern to the Association from a political, legislative, and institutional perspective.** This paved the way for dialogue focused on collaborative work with the various stakeholders within the healthcare ecosystem who are important to AELMHU, with the goal of continuing to improve the quality of life of patients suffering from rare diseases.

- **Isaac Túñez,** general secretary of Public Health and R&D&i of the Government of Andalucía.
- **María Isabel Priede Díaz,** general manager of Farmacia in Cantabria, and **Flora Pérez Hernández,** head of the Pharmaceutical Management Service of the Cantabrian Health System.
- **Eva Ortega Paíno,** General Secretary of Research of the Ministry of Science.
- **María Jesús Lamas Díaz,** Director of the Spanish Agency for Medicines and Health Products (AEMPS). Also present were **Antonio Blázquez,** head of the AEMPS Department of Medicines for Human Use, and **Antonio López Navas,** head of the Management Support Unit.
- **Marina Pollán,** Director of the Carlos III Health Institute (ISCIII).
- **Antonio Martínez Pastor,** President of the Health and Social Policy Commission of the Murcia Assembly and Member of the Popular Parliamentary Group.

- **Kilian Sánchez,** PSOE spokesperson for the Health Commission in the Senate and PSOE Health Secretary.
- **Ana Clopés,** Director of the Medicine Area of the Catalan Health Service (CatSalut), and **Manel Fontanet,** Technician of the Medicine Management of the Catalan Health Service.
- **César Hernández,** General Director of the Common Portfolio of Services and Pharmacy of the National Health System.
- **Jesús Aguilar,** President of the General Pharmaceutical Council of Spain (CGCOF), **Rita de la Plaza,** treasurer of the Council, and **Raquel Pérez,** Director of the cabinet of the Presidency of the Council.
- **Alejandro Vázquez Ramos,** Secretary of Health of Castilla y León.
- **Silvia Reboredo García,** Deputy General Director of Pharmacy of the General Directorate of Health Care of the Galician Health Service.
- **José Ramón Parada Jorgal,** manager of the Galician Health Service.

5.3 *PHARMACEUTICAL INDUSTRY STRATEGY 2024-2028*

In 2024, **the Pharmaceutical Industry Strategy 2024–2028 was published.** This is a Spanish government initiative aimed at strengthening the role of the pharmaceutical sector as a key driver of public health, innovation, and economic development. AELMHU, **along with other industry associations and employers' organizations, plays an active role** in this sector.

The Association's participation represents **institutional recognition** of the work it has been doing to improve access to treatments for patients with rare diseases since its inception. This new strategic framework will allow for active collaboration with all stakeholders, contributing its knowledge of the specifics of orphan and ultra-orphan drugs.

**Estrategia
de la Industria
Farmacéutica**
2024-2028

The strategy, which addresses health needs and the competitiveness and resilience of the pharmaceutical industry, is structured around **three key pillars:** access and sustainability, the promotion of research and innovation, and industrial competitiveness and sustainability. It also identifies the following **strategic therapeutic areas:**

- **Diseases.**
- **Chronicity.**
- **Repositioning.**
- **Antibiotic resistance and emerging infectious diseases.**
- **Personalized Precision Medicine.**
- **Advanced therapies.**

This Strategy is fully aligned with one of the organization's objectives: **to move toward a more equitable,** efficient, and patient-centered healthcare model.

This underscores the importance of fostering a competitive and sustainable environment that favours attracting innovation and strengthening the national pharmaceutical industry, promoting the availability of treatments for rare diseases.

Thus, AELMHU's participation in the 2024–2028 Strategy will consolidate its **commitment to transforming the pharmaceutical sector** toward a more resilient, sustainable model adapted to the specific needs of patients with rare diseases.

In this way, the parties involved will be able **to collaborate in the development and implementation of the measures outlined,** through technical dialogue and collaborative work with public administrations, the private sector, and society.

06

AELMHU

at EXTERNAL EVENTS

06. AELMHU at EXTERNAL EVENTS

During 2024, AELMHU consolidated its role as a key player in the rare disease ecosystem, **participating as a speaker in 28 events and attending 30 more, reaching a total of 58 events, which represented an increase of 23% compared to the previous year.**

This intense activity allowed the Association to continue promoting awareness, visibility, and access to treatments for rare diseases, strengthening its position as an authoritative voice among governments, healthcare professionals, industry, and patient associations.

6.1 *EVENTS with AELMHU PARTICIPATION*

EUROPEAN RARE DISEASE SUMMIT

02/14/2024

In Madrid, María José Sánchez Losada, **President of AELMHU at the time,** participated in the *European Rare Disease Summit 2024*, organized by Bamberg Health. The event, which brought together national and international experts, addressed issues such as legislative changes in Europe, access and reimbursement challenges, and the fundamental role of patients. AELMHU participated in the panel on "Innovation, financing and access to orphan drugs: Challenges and opportunities“, providing its expert perspective on the main challenges the sector is facing.

COORDINATES ON RARE DISEASES

27/02/2024

Sergio Bullón, member of the Board of Directors, represented AELMHU at the presentation of this report, organized by Sobi and Bioinnova Consulting in the Senate. The event highlighted the need to promote inter-territorial synergies and strengthen regional strategies. Bullón emphasized the importance of research in improving patients' quality of life.

II CONFERENCE ON MINORITY DISEASES

29/02/2024

Coinciding with **World Rare Disease Day,** AELMHU, represented by its then President, **Maria José Sánchez Losada,** participated at an event organized by the **12 de Octubre Hospital** in Madrid. The event focused on the use of artificial intelligence and advances in the treatment of rare diseases of cardiological, neurological, and oncological origin.

The event also included recognition of the work of various patient associations for their outstanding contributions to improving the quality of life of people with rare diseases: the Spanish Federation of Hereditary Metabolic Diseases, the Association of Neurotransmitter Diseases, and the Spanish Association for SATB2.

CYCLE OF MEETINGS OF EXPERTS ON ORPHAN DRUGS

April, May and June

With the aim of analysing the challenges of orphan drugs and delving into the key issues related to this type of treatment from different perspectives, the specialized health media **Diariofarma,** in collaboration with several pharmaceutical companies, **wanted to include several representatives of AELMHU on the panel of participants in the Cycle of Meetings of Experts on Orphan Drugs.**

Throughout the sessions, topics such as the current status of treatments, changes in pharmaceutical legislation that will affect these products, their social value, and access challenges, among others, were explored. The events took place during April, May, and June in Madrid and Seville.

CHALLENGES OF ECONOMIC EVALUATION IN THE NEXT DECADE

04/29/2024

The Director of the Association, Marian Corral, participated in the round table "Importance of research for early diagnosis of rare diseases", organized by the Ibercaja Foundation and the Spanish Federation of Rare Diseases (FEDER), within the framework of the World Day of People Without Diagnosis.

During this meeting, rare disease experts highlighted the crucial importance of research and early access to diagnosis to ensure equitable access as fundamental pillars for addressing the challenges faced by these patients and their families.

IMPORTANCE OF RESEARCH FOR EARLY DIAGNOSIS OF RARE DISEASES

10/04/2024

Beatriz Perales represented the Association at the event "Challenges of Economic Evaluation in the Next Decade“, organized by BioInnova Consulting in collaboration with several pharmaceutical companies. Throughout the event, the need for open dialogue among all stakeholders, including scientific societies, regulators, patients, and pharmaceutical companies, was highlighted in order to achieve high-quality and widely accepted economic evaluations.

The importance of ensuring equity and quality in the economic evaluation of medicines to ensure the sustainability of the health system was also emphasized. The urgency of allocating greater investments in health and implementing structural changes to address future challenges was also highlighted.

4TH EDITION OF SINGULARES – UNITED FOR RARE DISEASES

11/06/2024

For the fourth consecutive year, Singulares organized its annual "United for Rare Diseases" event. **Marian Corral, Executive Director of AELMHU,** participated in one of the panels to address such relevant topics in the approach to rare diseases as diagnostic delays, advances in screening, and unmet patient needs.

His intervention also highlighted the need for a specific and/or differentiated model for evaluating and reimbursing orphan drugs.

THE MANAGEMENT OF DISCRETION IN ACCESS AND REGULATION OF ORPHAN DRUGS

June 25, 2024

During the 116th FUINSA Conference, **Beatriz Perales, President of AELMHU,** underscored the importance of developing a robust regulatory framework that guarantees equitable and sustainable access to orphan drugs. She also emphasized that discretion in the regulation and reimbursement of these drugs must be carefully managed to avoid arbitrary decisions that harm both patients and the industry.

Furthermore, he emphasized the need to establish clear and transparent criteria that allow for informed decision-making, ensuring legal certainty and facilitating constructive dialogue between the administration and all stakeholders involved.

DIALOGUES ON HEALTH POLICY

During the month of July, AELMHU, **through its Director, Marian Corral,** was able to participate in events with two of its main *stakeholders* in the field of health regulation, thanks to the cycle of meetings "Dialogues on health policy", organized by Bioinnova,

The first of these, held behind closed doors, was with **Javier Padilla, Secretary of State for Health,** where a small group was able to chat with him and openly share his views on the healthcare system. The format facilitated a rich exchange between various stakeholders in the sector.

Marian Corral, Executive Director, also attended the second meeting of this series, this time with **Pedro Gullón, General Director of Public Health and Health Equity,** who shared the Ministry's priority strategic lines and discussed different perspectives and points of view with attendees.

5TH WEBER SUMMER COURSE

26-27/09/2024

Once again, the Association participated this year in the **NewsRare Summer Course,** organized by the University of Castilla–La Mancha in collaboration with the Weber Foundation.

During the first day, sponsored by AELMHU, Sergio Bullón, member of the Board of Directors, participated in the opening ceremony of the event, where the report "Differential Aspects of Orphan Drugs and Their Value from a Social Perspective" was presented, prepared by both entities. The day included panel discussions on research and access, and **was closed by César Hernández, General Director of the Common Portfolio of Services and Pharmacy of the NHS.**

The second day, **Beatriz Perales, President of AELMHU,** participated in a panel of experts, positively assessing the publication of the draft of hte Royal Decree on Health Technology Assessment, which implied advances towards greater transparency and efficiency in the evaluation processes and underlining the need to guarantee clear deadlines, more active participation of patients, and greater consideration of the particularities of orphan drugs.

SPANISH HEALTH OBSERVATORY

3/10/2024

The 5th Symposium of the Health Observatory, organized by El Español and Invertia, featured prominent healthcare leaders and government representatives. **Beatriz Perales, President of AELMHU,** highlighted the importance of rare diseases and orphan drugs.

During his speech, he addressed key topics such as the need for European legislative reform to guarantee access to incentives for innovation in orphan drugs and the adaptation of health assessments to the characteristics of rare diseases.

D'GENES ASSOCIATION CONGRESS

11/22/2024

For another year, the association, through **Marian Corral,** also participated in the 17th International Congress on Rare Diseases, organized by the **D'Genes association at the Catholic University of Murcia.** The Director spoke about rare diseases, their research, orphan drugs, and access to these treatments.

In his speech, she emphasized the importance of ensuring equity in diagnosis and access to treatment, streamlining funding processes, and recognizing the specificity of orphan drugs in future legislation. She also stressed the need to incentivize the development of new therapies and foster collaboration among all stakeholders.

NATIONAL CONFERENCE ON HEALTH MANAGEMENT AND RARE DISEASES

11/12/2024

At the National Conference on Healthcare Management and Rare Diseases, organized by SEDISA and Amgen, **Beatriz Perales, President of AELMHU,** highlighted the importance of patient associations in raising awareness and researching rare diseases.

She also highlighted the need for public-private collaboration and the importance of a care model that includes social and emotional needs. To improve patients' quality of life, it is essential to work together with all stakeholders.

IMPROVED CARE IN RARE NEUROMUSCULAR DISEASES

11/26/2024

Marian Corral, Executive Director of AELMHU, moderated the panel discussion **"The Management of Neuromuscular Rehabilitation in Institutions: Perspectives and Advances“**, creating a space for reflection and debate on the challenges and opportunities in rare disease care from a hospital perspective.

During the meeting, topics such as the need to promote more flexible organizational models, the value of collaboration between referral centres, and the importance of ensuring equitable and rapid access to orphan drugs were discussed. The discussion, enriched by the insights of executives from referral hospitals, highlighted the commitment to continuous improvement in the care of these patients.

RARE DISEASES AND ACCESS TO ORPHAN DRUGS

10/12/2024

Biomarin, one of AELMHU's associated companies, organized a roundtable discussion in conjunction with RPP Consultant,.

Through Marian Corral, who spoke on the panel "**Adapting Legislative Reforms to Better Address Patient Needs and Improve Treatment Availability"**, the debate focused on key topics such as updating drug evaluation and reimbursement processes, promoting equity in access to innovative therapies, and the importance of incorporating the patient perspective into decision-making.

The dialogue included contributions from public administration, healthcare assessment, associations, and the pharmaceutical sector, with a collaborative approach aimed at strengthening the National Health System.

OTHER EVENTS WITH AELMHU PARTICIPATION:

At the same time, several entities in the educational sector wanted to meet with AELMHU as an authoritative voice to promote awareness of rare diseases and orphan drugs. The Association gave keynote sessions at **the Camilo José Cela University (UCJC),** at the **CESIF (Center for Advanced Studies of the Pharmaceutical Industry), at the Riberser** Master's in Rare Diseases, **and** at the Master's in Rare Diseases at **Talent Up.**

Also, throughout 2024, the Association was invited to participate in closed-door meetings:

- Meeting with CEOs of companies specialized in orphan drugs, organized by **Adqualis** in March.

- ThinkThank Evidenze, also in October, organized **by Evidenze Health.**

- In recognition of its commitment and experience in the field of rare diseases and orphan drugs, AELMHU, represented by its Director, participated as a member of the jury for **the Merco Awards – Leadership and Reputation in Health.**

6.2 *OTHER SECTOR EVENTS*

Throughout 2024, AELMHU attended several meetings with experts and health authorities to closely monitor the legislative developments that shaped the sector's agenda, as well as other current and interesting issues in the healthcare field.

The AELMHU Board of Directors and Management attended the following events during the first two months of 2024: on January 26, a breakfast organized by Kreab with the **Secretary of Health of Cantabria, César Pascual,** attended by Marian Corral; on January 31, the **ISPOR Spanish Chapter Conference: HTA:** Towards Excellence in Care Processes in Rare Diseases; on February 5, the conference **"Rare Diseases in Spain: Current Situation and Future",** organized by Bioinnova and UCB; and on February 12, the **Nextep Conference on the legislative reform of the pharmaceutical sector.** The Association was represented at all of them by Marian Corral, Executive Director.

Furthermore, in February, the Association's technical team attended the presentation of the **World Rare Disease Day campaign,** organized by the Spanish Federation of Rare Diseases (FEDER); ***Epiforward 2024, organized by the*** Epiforward Patient Association; the Sanitarias Awards ***for Medical Writing,*** which were attended by the Minister of Health; the 13th Conference organized by CIBERER, under the slogan **"Research is Progress";** and the presentation of the Únicas Project at the **Sant Joan de Déu Hospital** in Barcelona.

During the month of March, the Association also attended several key events. It was invited to the **Sanitarias Awards** organized by Redacción Médica, and on March 5th, it attended the **annual celebration of World Rare Disease Day,** organized by FEDER, which took place in Seville. The following day, March 6th, AELMHU attended the presentation of the **"Innovative Women"** Report, organized by the Ministry of Science, Innovation and Universities.

Furthermore, on March 18, the Executive Director attended a meeting organized by KREAB with the **Secretary of State for Health, Javier Padilla.** Marian Corral, Executive Director of AELMHU, also attended **the FEDER institutional event at the Madrid Assembly.**

In April, Diariofarma organized a **meeting on orphan drugs and rare diseases**, which was also attended by AELMHU; on the 22nd, **Sant Joan de Déu and the LaCaixa Foundation held the 1st *Unique Talks:*** *Rare Diseases in Barcelona;* Bioinnova organized a ***series of debates on advanced therapies;*** while on the 23rd, the **Ramón Areces Foundation,** with the support of ***FEDER,*** held the conference **Current Situation of Rare Diseases in Spain.**

In May, AELMHU attended two events: the **inauguration of the MPS Association headquarters in Barcelona on May 16; and the** ***Impact of Changes in HTA Regulation*** in Madrid on May 28, organized by ISPOR, both through its Director, Marian Corral.

The Neonatal Screening Days* organized by Más Visibles,** the event to commemorate ***CSUR's 15th anniversary: Present and Future, and the presentation of the RPP Consulting Guide: *Guide to the new EU term 2024-2029* **took place in June,** on the 4th, 10th and 25th respectively.

The Association also wanted to be present, as a demonstration of its commitment to patients and the sector's agenda, through its technical team and its Director, Marian Corral.

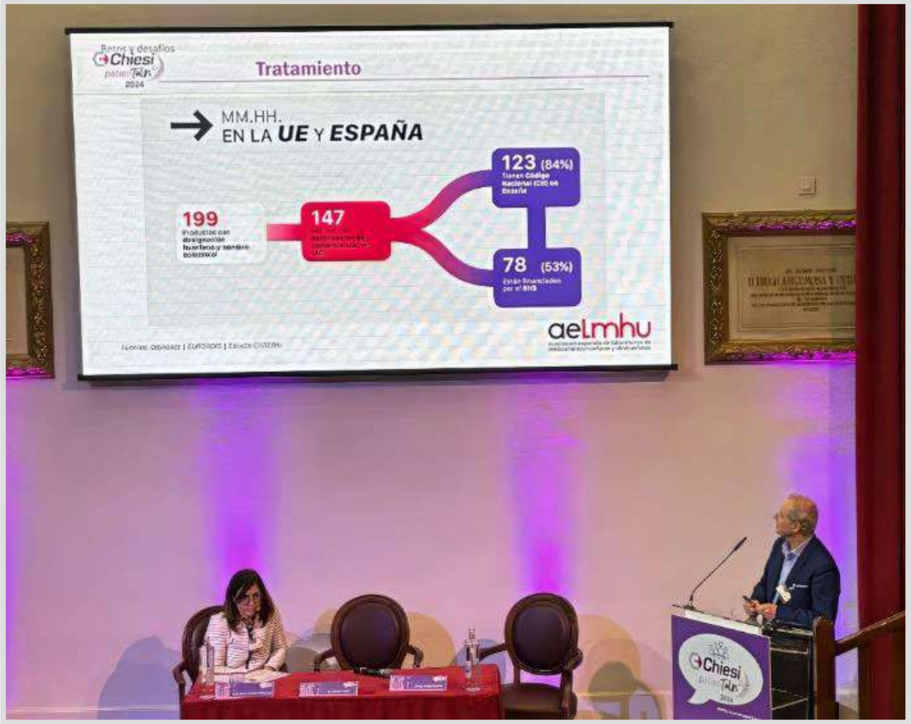
Marian Corral, Executive Director of AELMHU, was able to attend, **during the month of September,** the conference organized by the Ministry of Health under the title '***Conference Information on Health Technology Assessment',*** *as well as* the event '***Challenges and Opportunities in Gene Therapy Reimbursement',*** organized by Bioinnova and CSL Behring.

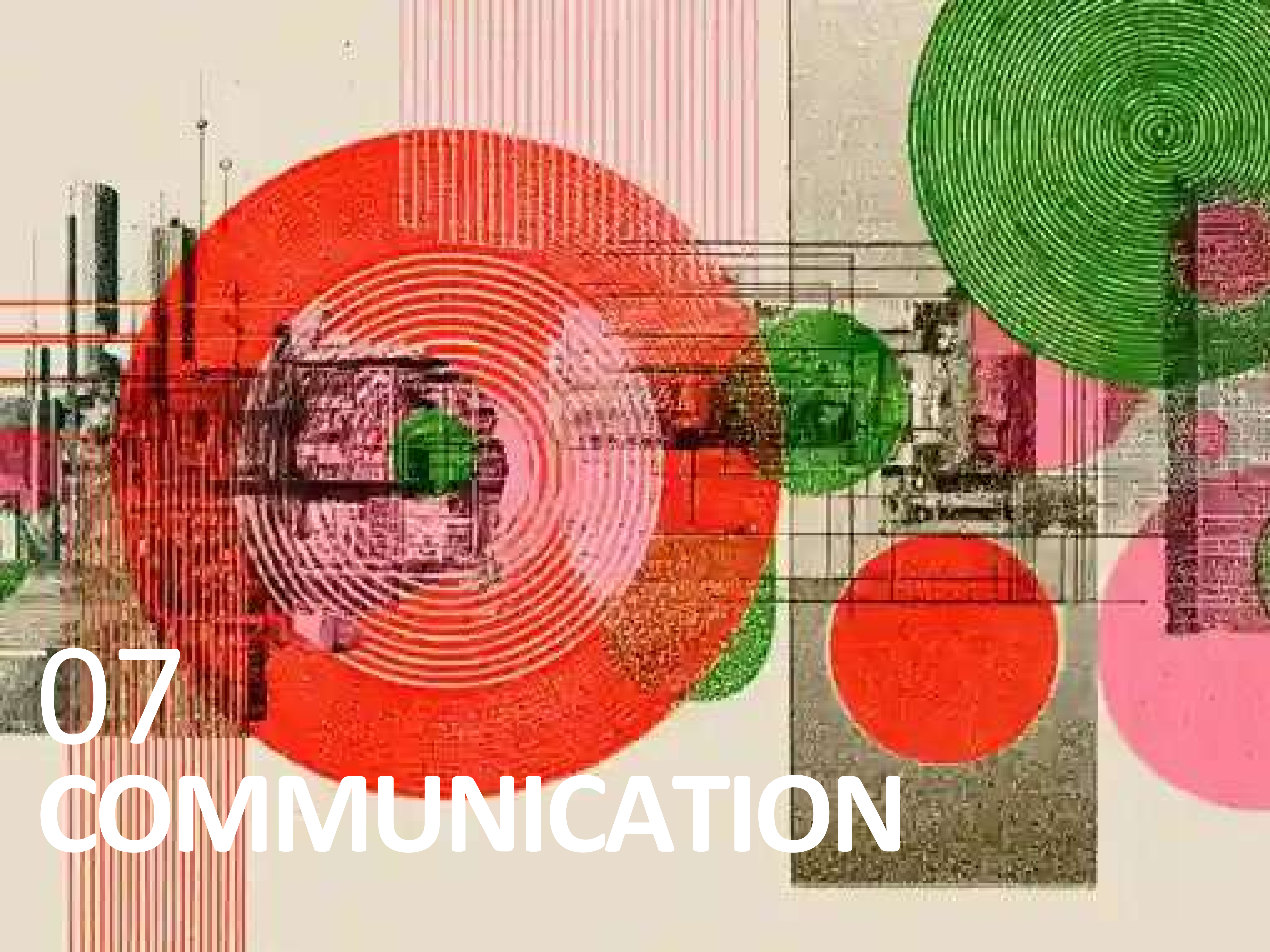
The General Council of Pharmaceutical Associations Awards were also held, a gala attended by the Director, representing the Association.

Bioinnova and IPSEN also invited AELMHU to their ***Regional Forum on Rare Diseases event*** in Madrid; and FEDER, for its part, invited the Association to the event on '***Research: The Key to Science'*** in Barcelona, *in* October and November, respectively.

As a result of collaboration with the Association of Institutional Relations Professionals (APRI), **Marian Corral, Executive Director of AELMHU,** attended one of the meetings organized by the Association, this time with **Javier Padilla, Secretary of State for Health,** in October.

During November, the Association also attended two more events: the presentation of the documentary ‘***La vida en una gota***’**, by Mas Visibles,** and **the Chiesi Patient Talks, organized by Chiesi,** which was the last event attended by the Association during 2024.





07

COMMUNICATION

07. COMMUNICATION

Communication has become a fundamental pillar for the Association, enabling effective interaction with various stakeholders in the sector. In a context where visibility and media play a key role, **communication has become a strategic tool for disseminating information and raising awareness about rare and** ultra-rare diseases.
Through clear and proactive communication, the Association strengthened its public presence in 2024, raising awareness and promoting support for those living with these rare conditions.

Dissemination through various channels allowed the message to reach a wide audience, facilitating collaboration with institutions, professionals, and society in general.

Having a prominent media presence not only allowed AELMHU to publicize its actions and commitments, but also **helped consolidate the trust of key industry representatives, strengthen its reputation, and position it as an authoritative voice** in the field of orphan drugs and rare diseases.

In an increasingly digital world, where information spreads quickly and immediately, maintaining good media image is crucial to building credibility and ensuring that messages reach the public in a clear and understandable manner.

AELMHU has developed content creation initiatives across the media landscape, strengthening its commitment to research and development of innovative treatments that improve the lives of patients with rare diseases and their families.

Throughout 2024, the Association maintained an **active media presence** through press releases, social media participation, and constant website content updates. Furthermore, the Board of Directors spokespersons responded to the Association's requests and offered to answer interviews and media requests.

7.1 *AELMHU IN THE MEDIA*

During 2024, the Association **recorded a total of 299 media impacts, resulting from the strategic dissemination of press releases,** the presentation of access reports, its active participation in events, and media inquiries on matters of direct relevance to the Association, both in general and specialized media.

The Association **issued eight press releases** on its own milestones, which, in addition to being well-received, gave the organization visibility and opened the door for the media to expand on the information they initially received:

- Annual Report on Access to Orphan Drugs 2023.
- New edition of the AELMHU Awards.
- Report of Clinical Trials in Rare Diseases 2023.
- Renewal of the Board of Directors.
- Report: 'Differential Aspects of Orphan Drugs and Their Value from a Social Perspective'.
- II National Conference on Advanced Therapies for Rare Diseases.
- Jury's decision on the 2024 AELMHU Awards.
- AELMHU 2024 Awards Ceremony.

AELMHU's impacts **were divided into four different media types,** depending on the nature of their content: agencies, health specialists, economics, and general media. The vast majority of appearances were national and in digital format.

In this regard, regarding media types, the most notable impact was on *El Economista*, a financial news outlet; general publications such as *La Razón* and *El Mundo;* health-focused publications such as *Diario Médico* and *iSanidad;* and the news agencies *EFE* and *Servimedia*, among others.

All of this demonstrated that AELMHU continued to generate media interest at various levels and positioned itself as an authoritative voice for discussing rare diseases and orphan drugs.

7.2 *INTERVIEWS with AELMHU*

Through its authoritative voices, **the Association also appeared in various media interviews throughout 2024.**

Among the most relevant appearances, **the main headlines in the health/healthcare sector stood out, which wanted to count on the valuable contributions of AELMHU,** consolidating their messages as authorized influencers, among which are *Redacción Médica* and *IM Médico*, with multiple mentions, in addition to periodic appearances in *Diariofarma*, *ConSalud*, *Sanifax* or *iSanidad*, among others.

El fármaco huérfano fija un precio de financiación temporal para su impulso

Definir la patología y registrar a los pacientes que beneficiarán son otras recomendaciones que dan desde Aelmhu

El fármaco huérfano suma toda la cadena sanitaria al fondo de financiación

Los laboratorios de estos medicamentos apuestan por la participación "constructiva" de toda los agentes implicados

Triple evaluador para la financiación temporal del fármaco huérfano

La propuesta de Aelmhu requiere que la compañía farmacéutica ofrez

Beatriz Perales, presidenta de Aelmhu.

María José Sánchez (Aelmhu): «Financiar medicamentos huérfanos supone todavía un promedio de dos años»

El fármaco huérfano 'español' celebra el nuevo rol del paciente en Europa

La patronal aplaude el proyecto piloto para dar certezas a este sector y que se extenderá todo 2025

España financia el 53% de los medicamentos huérfanos autorizados en Europa

Los ensayos clínicos sobre enfermedades raras en 2023 descienden un 10 %

España aprueba 21 medicamentos para enfermedades raras, un 42,8% más

Esperanza en enfermedades raras: España financia más de la mitad de los medicamentos huérfanos autorizados en Europa

- Por primera vez en cinco años, en 2023 se sufragaron 21 nuevos fármacos, 12 más que en el ejercicio anterior y se alcanzó un total de 78 costeados por el Sistema Nacional de Salud
- Pese a las buenas noticias, todavía hay 45 fármacos que no se financian y corresponden a áreas terapéuticas como las oncológicas -en un 40%- y las metabólicas, en un 24%

Aelmhu renueva su Junta Directiva, con Beatriz Perales como nueva presidenta

La nueva presidenta sustituye a María José Sánchez se será vicepresidenta; Iván Silva director de Acceso al Mercado para el clúster del Sur se incorpora a la Junta Directiva como vocal y Sergio Bullón continuará como tesorero

«España necesita un proceso específico de financiación para medicamentos huérfanos»

Beatriz Perales, presidenta de la Asociación Española de Laboratorios de Medicamentos Huérfanos y Ultrahuérfanos, analiza los retos que enfrentan estos tratamientos en España

7.3 *DIGITAL*

AELMHU also continued to grow on its main corporate social media and web, adapting the messages and content on each of them.

In parallel with its press coverage, the Association strengthened its presence on LinkedIn, establishing itself as an authoritative and relevant source in the digital world by disseminating information about its activities and initiatives. This achievement was possible thanks to the growing **awareness of the importance of communicating its actions effectively and appropriately,** adapting to the channels where its target audiences are present.

In this context, the Association **continued to strengthen its positioning, with the goal of increasing its recognition** and expanding its opportunities for dialogue with various stakeholders, whether companies or actors in the legislative and pharmaceutical sectors.


AELMHU (Asociación Española de Laboratorios de Medi...
3.571 seguidores
3 meses •

Nuestra presidenta Beatriz Perales Zamorano ha participado hoy en la Jornada Nacional sobre Gestión Sanitaria y Enfermedades Raras, organizada por SEDISA - Sociedad Española de Directivos de la Salud y Amgen España que ha ... más


AELMHU (Asociación Española de Laboratorios de Medi...
3.571 seguidores
4 meses • Editado •

Hoy hemos acompañado a Federación Española de Enfermedades Raras en su jornada sobre Investigación en Enfermedades Raras bajo el lema "El movimiento asociativo, la llave de la ciencia" en el Museo de la Ciencia CosmoCaixa de ... más


AELMHU (Asociación Española de Laboratorios de Medi...
3.571 seguidores
6 meses •

Ayer se celebró la primera jornada de la V edición del Curso de Verano de 2024, de NewsRARE, gracias a la colaboración de la Fundación Weber y el co-patrocinio de #AELMHU. ... más


AELMHU (Asociación Española de Laboratorios de Medi...
3.571 seguidores
4 meses • Editado •

Un año más, hemos acompañado a la asociación D´Genes en el XVII Congreso Internacional de Enfermedades Raras bajo el lema "Diagnóstico temprano y avances en investigación: abriendo puertas a la esperanza, en la sede de la ... más

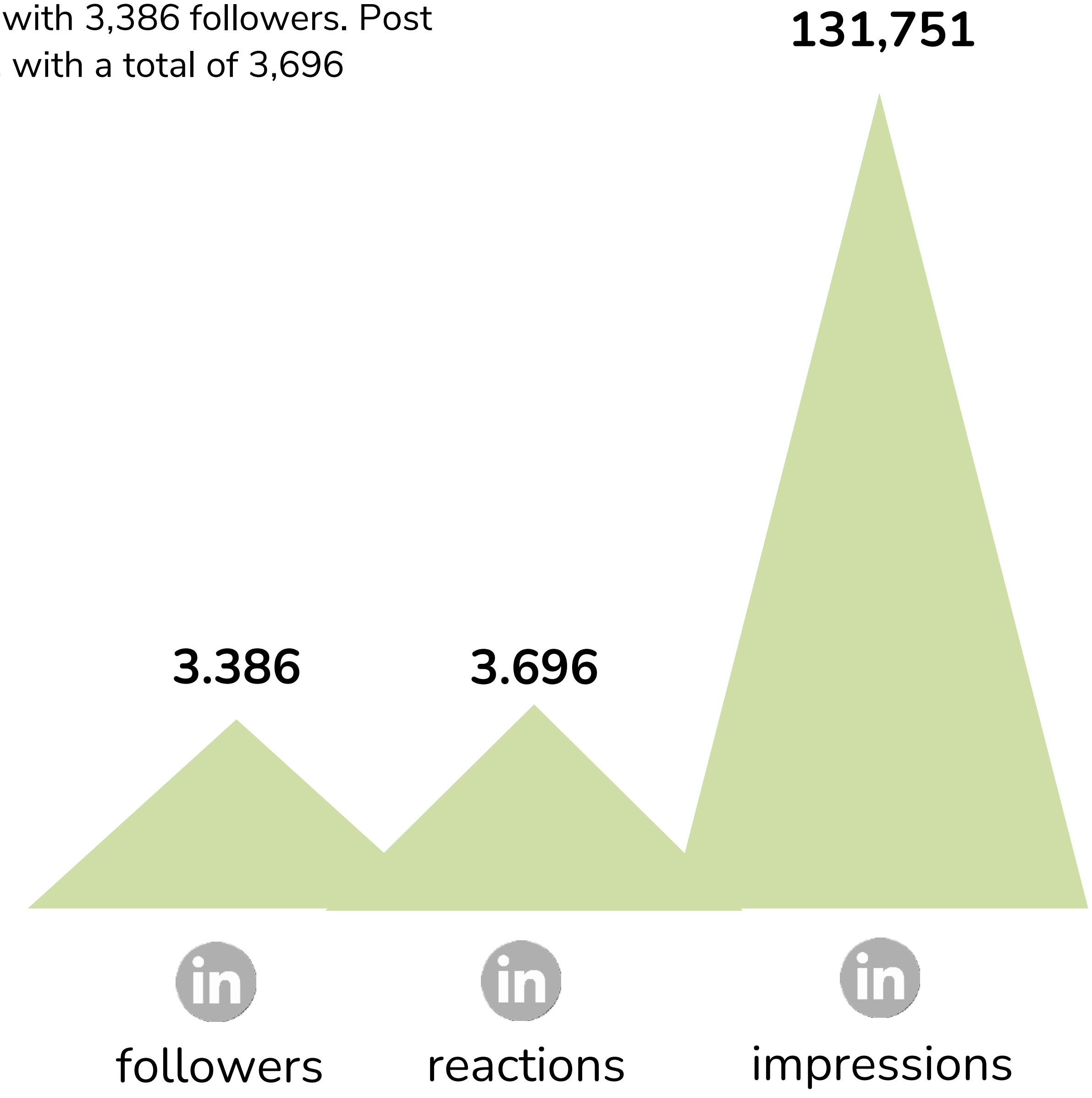

AELMHU (Asociación Española de Laboratorios de Medi...
3.571 seguidores
5 meses • Editado •

Ayer entregamos los galardones de la VI 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 al ámbito de las ... más


AELMHU (Asociación Española de Laboratorios de Medi...
3.571 seguidores
9 meses • Editado •

Varios miembros de AELMHU hemos podido visitar las instalaciones del Hospital Sant Joan de Déu Barcelona Déu y la Casa de Sofía. Durante el encuentro también hemos podido conocer mejor el proyecto Únicas SJD, una red integrada por ... más

With this, the Association's LinkedIn profile **grew by 18% during 2024,** closing the year with 3,386 followers. Post impressions reached 131,751, with a total of 3,696 reactions.



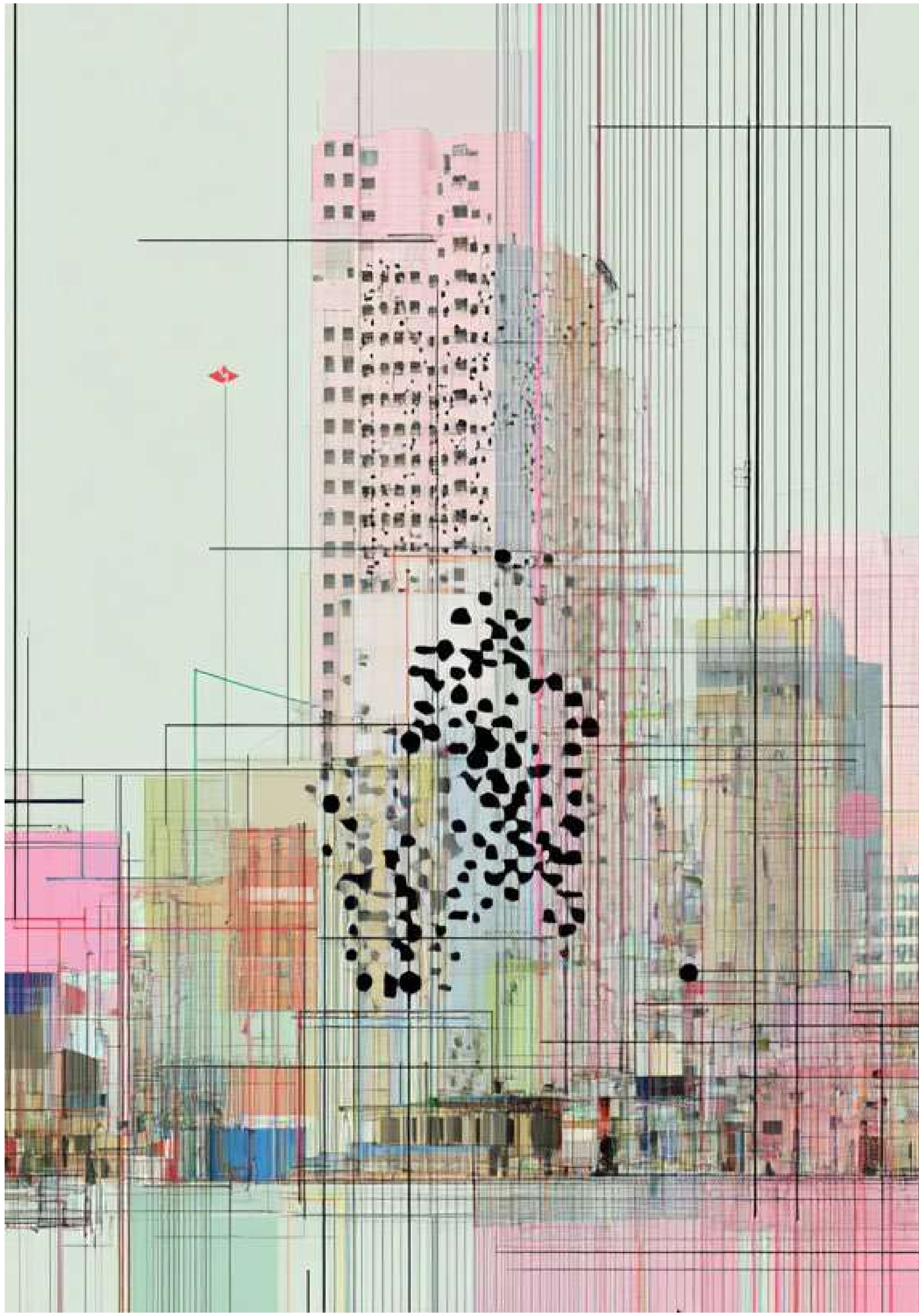
08
PARTNERSHIPS

08. PARTNERSHIPS

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

Furthermore, these agreements facilitate the dissemination of knowledge and documents, raise social awareness, and promote more inclusive public policies, fostering a more accessible environment for people living with these rare diseases. **Strategic cooperation is key to advancing the visibility and resolution of the challenges posed by rare diseases.**

For this reason, AELMHU signed **two** collaboration agreements during 2024.



8.1 *PARTNERSHIP with the WEBER FOUNDATION*

The collaboration agreement **between the WEBER Foundation and AELMHU** established a permanent framework for joint work to define and position both organizations within their respective areas of activity. The agreement was signed by **Álvaro Hidalgo,** President of the Weber Foundation, and **Beatriz Perales, President of AELMHU**.

Within the framework of this agreement, the report "Differential Aspects of Orphan Drugs and Their Value from a Social Perspective" was jointly prepared by both entities. It analyses the specificities of orphan drugs and highlights their value from a social, clinical, and healthcare perspective. It also addresses challenges in research, access, financing, and equity.

As detailed in point 3 of this 2024 Annual Report, the report was presented at the **5th NewsRare Summer Course,** held on September 25 and 26, 2024, and sponsored by AELMHU.

The first panel discussion of the day, on ***Orphan Drug Research,*** highlighted the need for greater collaboration between regulatory agencies, scientific societies, and clinical professionals, addressing barriers such as limited reimbursement and difficulties in patient recruitment. Experts such as **Fernando Méndez (EMA**), **Jorge Francisco Gómez (SEMI)** and **Rocío Sánchez-Carpintero (SENEP)** participated**.**

The second panel, focused on ***Access and Equity,*** highlighted the marked inequalities between autonomous communities and proposed moving towards a coordinated national strategy. **Pharmacy officials from SESCAM and SERMAS spoke,** along with **Juan Carrión,** President of FEDER.

The event was closed by ***César Hernández*, General Director of the Common Portfolio of Services and Pharmacy of the National Health System,** who called for stronger collaboration between administrations, industry, and the scientific community to ensure that patients with rare diseases can access innovative therapies in a fair and timely manner.

As part of this collaboration, a special issue on orphan drugs was published in *NewsRare* magazine, which addressed the challenges and advances in this field in depth.

In addition, **an interview was broadcast in this same format and, later, digitally, with the President of AELMHU, Beatriz Perales,** in which she explained the strategic role of the association, the evolution of the sector, and the priorities on the agenda for the coming years.

In order to broaden the report's reach and facilitate international knowledge sharing, **a full translation into English was also completed.** This initiative sought to ensure that the content and results were accessible and understandable to a wider audience, thus promoting greater global dissemination and understanding of the reality of orphan drugs.

8.2 *PARTNERSHIP with the GENERAL PHARMACEUTICAL COUNCIL*

AELMHU also formalized a collaboration agreement with the **General Pharmaceutical Council (CGCOF)**, with the aim of improving pharmacists' knowledge of orphan and ultra-orphan drugs, those intended to treat rare diseases. This agreement includes the joint organization of training and informational activities, as well as health programs and campaigns.

The first action under this agreement will be the launch of the "Pharmacy and Orphan Drugs" **video advice channel on the General Pharmaceutical Council' YouTube channel in 2025,** with the aim of offering educational and awareness-raising content on orphan and ultra-orphan drugs.

The agreement for this alliance was signed by **Jesús Aguilar,** President of CGCOF, and **Beatriz Perales, President of AELMHU**.

09
ACKNOWLEDGMENTS

09. ACKNOWLEDGMENTS

All the activities included in this report would not have been possible without the **commitment, help and involvement of the AELMHU associates**, whose Collaboration and effort are key to achieving the organization's objectives.

ALEXION AstraZeneca Rare Disease
Alnylam PHARMACEUTICALS
AMGEN
AOP HEALTH
argenx
ascendis pharma
biocryst
Biogen
BIOMARIN
Chiesi
CSL Behring Biotherapies for Life™
IMMUNOCORE
insmed
IPSEN Innovation for patient care
ITALFARMACO
Jazz Pharmaceuticals
KYOWA KIRIN
NOVARTIS
PTC THERAPEUTICS
sanofi
sobi
Takeda
ucb
ultragenyx
VERTEX

Thank you so much!

for your support and involvement

AELMHU also wishes to express its **gratitude to the healthcare professionals, medical and scientific societies, media outlets, patient associations and foundations, universities, consulting firms, institutions, and others** who have collaborated with the association in 2024. This collaboration has resulted in combined efforts to improve the health and quality of life of people affected by rare diseases and their families, and to raise awareness of their conditions and treatments.

Once again, the Association would like to give **special mention to the Spanish Federation of Rare Diseases (FEDER),** the representative and spokesperson for patients and the true motivation and reason for AELMHU's existence. Thank you for another year of work and collaboration!

AdQualis Human Results
AECOM Asociación Española Para el Estudio de los Errores Congénitos del Metabolismo
Asociación Española de Enfermos y Familiares de la Enfermedad de Gaucher
aefat
Asociación Española de Genética Humana – Actividad considerada de interés divulgativo por la AEGH
AERyOH
aemps agencia española de medicamentos y productos sanitarios
Albi España
APRI
ASEDEF
Asociación Conquistando Escalones
d'genes
Duchenne Parent Project España
AER ELX
AEGH Asociación Española de Genética Humana
+visibles
anis Informadores de la Salud
Retina Murcia
Bamberg Health
bio innova consulting
Castilla-La Mancha Consejería de Sanidad
CESIF
ciber ER
Comunidad de Madrid Consejería de Sanidad
Farmacéuticos Consejo General de Colegios Farmacéuticos
CSC Consorci de Salut i Social de Catalunya
CTO
diariofarma
efpia
EL ESPAÑOL
Epiforward
EURORDIS Rare Diseases Europe
Evidenze Knowledge in Health
Executive Forum España
facme Federación de Asociaciones Científico Médicas Españolas
Faus Moliner
feder
FUINSA Fundación para la Investigación en Salud
Fundación Ibercaja
Fundación Dr. Torrent-Farnell
mehuer Fundación Medicamentos Huérfanos y Enfermedades Raras
frA Fundación Ramón Areces
Síndrome de Dravet Fundación
Fundación Teófilo Hernando I+D del Medicamento / Drug Discovery
Generalitat Valenciana Conselleria de Sanitat Universal i Salut Pública
GEPTI Grupo Español de Trombocitopenia Inmune
Gobierno de Aragón
GEA Grupo Español de Aféresis
SJD Sant Joan de Déu Barcelona · Hospital
SaludMadrid Hospital Universitario 12 de Octubre
La Fe Hospital Universitari i Politècnic
Ilustre Colegio de la Abogacía de Madrid
IM Médico
iier Instituto de Investigación de Enfermedades Raras
idis
isciii Instituto de Salud Carlos III
iSanidad
Conselleria de Salut Servei de Salut
ISPOR Spain Chapter
KREAB Worldwide
LASKER
Legado María de Villota
merco Empresas
Gobierno de España Ministerio de Ciencia e Innovación
Gobierno de España Ministerio de Sanidad
MPN España
MPS Lisosomales
Newlin
nextep in health
PERTE para la salud de vanguardia
rm
Región de Murcia Consejería de Sanidad
RIBERSER
RPP
SJD Sant Joan de Déu Barcelona · Hospital
sedisa
SEMI
S.E.N.P.
Servizo Galego de Saúde
singulares Unidos por las EERR o poco frecuentes
sefh Sociedad Española de Farmacia Hospitalaria
Síndrome Quilomicronemia Familiar
Sacyl
Tornos Abogados
UCLM Universidad de Castilla-La Mancha
Universidad Camilo José Cela
VINCES Building Legitimacy
Weber

Thank you so much
for your support and involvement!

ANNUAL

REPORT
2024

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