EXECUTIVE SUMMARY

Annual Report on *ACCESS 2023*

of Orphan Medicinal Products (OMPs) in Spain

aeLmhu

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

INDEX

01 AELMHU ACCESS REPORT

The Spanish Association of Orphan and Ultra-Orphan Drug Laboratories (AELMHU) is pleased to publish the key results and main **conclusions of its 2023 Annual Report on Access to Orphan Medicines in Spain.**

The Report analyses the status of products with a trade name and positive orphan designation by the European Medicines Agency (EMA) as of 31 December 2023, as well as the process these products follow to reach the Spanish market, from the assignment of the National Code by the Spanish Agency for Medicines and Health Products (AEMPS) to public reimbursement.

For more than 13 years, AELMHU has been working to offer more and better indicators that allow all interested stakeholders – patients, the scientific community, professionals, industry, public decision-makers and society in general – to periodically evaluate the availability of innovation in a field as complex and, at the same time, as hopeful, as rare diseases.

For our Association it is an honour and a responsibility to know that our reports have become the main reference for data on access to orphan and ultra-orphan medicines in Spain. To continue improving the available information, and similarly to last year, the Report provides, first, further detail and analysis on relevant aspects, such as timelines and therapeutic area for orphan medicines with an EC's marketing authorisation but yet to have a National Code in our country. And second, the Report updates our proposals to improve the access to orphan medicines in Spain.

During 2023, the approval of new laws and initiatives of vital importance for our industry were expected. In Spain, however, only the public consultation of the Royal Decree on health technology assessment was published, and the Strategic Plan for the Pharmaceutical Industry 2023-2025 still has not been released.

At the European level, the new Pharmaceutical Strategy for Europe will probably not be implemented until 2025, although the implementation of the European regulation on health technology assessment continues its progress.

The Association has participated in all relevant public consultations on initiatives related to orphan medicines and rare diseases at regional, national and international level. This Access Report reflects the Association willingness and determination to continue projecting more and better indicators to contribute to the common goal of improving the availability of pharmaceutical innovation in Spain. AELMHU is a not-for-profit organisation that brings together pharmaceutical and biotechnology companies with a strong commitment to discover, research, develop and commercialise innovative therapies capable of improving the situation of patients suffering from these so-called rare diseases.

The purpose of the organisation is to contribute to improving the situation of people affected by rare or low prevalent diseases, promoting knowledge of their pathologies and recognition of the therapeutic and social value that orphan medicines have in their daily lives and those of their families.

The current **members of AELMHU** are Alexion AstraZeneca Rare Diseases, Alnylam Pharmaceuticals, AOP Health, Argenx, Biocryst, Biogen, BioMarin, Chiesi, Csl Behring, Grupo Italfarmaco, Horizon, Immunocore, Insmed, Ipsen, Jazz Pharmaceuticals, Kyowa Kirin, Novartis, PTC Therapeutics, Sanofi, Sobi, Takeda, UCB, Ultragenyx Pharmaceutical and Vertex.

02 METHODOLOGY

The methodology for the 2023 Annual Report on Access to Orphan Medicines in Spain consists of four phases:

01 Identification of medicines with a valid orphan designation and trade name as of 31 December 2023.

An Orphan Designation (OD) is granted during the early stages of a medicine´s research programme. Of the more than 2,000 active ingredients that have received this OD from the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA), those with an active designation as of 31 December 2023 have been identified (more than 1,900). Of these, those with a trade name are identified (more than 250), excluding their new presentations or indications.

Overall, **199 orphan medicines with a commercial name and with an active orphan designation** as of 31 December 2023 are identified.

02 Orphan medicines with marketing authorisation but excluded from the European Register

Second, orphan medicines that have been withdrawn (or have not yet been included) in the European Commission's Community Register of Orphan Medicinal Products for marketing in the EU are excluded (n=42). This results in the number of orphan medicines **with a marketing authorisation (MA)** as of 31 December 2022, in EU countries **(n=147)**.

03 Orphan medicines with a National Code

The third step has been, for these 147 orphan medicines, to identify those which have been granted a **National Code** by the AEMPS in Spain **(n=123)**.

04 Orphan medicines reimbursed by the NHS

Finally, the **orphan medicines which are reimbursed by the NHS**, according to the information contained in the Nomenclator of the Ministry of Health, are identified **(n=78)**. The date of the first positive reimbursement decision for each orphan medicine is reported.

Note: Due to the fact that data cannot be obtained from any public source, this document does not reflect those medicines that are available in our country under specific situations and special authorisations within the framework of Royal Decree 1015/2009 (compassionate use, use under conditions other than those authorised, or access to foreign medicines).

03 CURRENT CONTEXT OF ORPHAN MEDICINES

Orphan medicines are innovative treatments, many of them of biotechnological origin, aimed at diagnosing, preventing or treating rare or low prevalent diseases, which are diseases defined as affecting fewer than 5 out of every 10,000 people and have no alternative treatment.

It is estimated that there are between 6,000 and 8,000 are diseases in the world, of which only 5% have some kind of diagnostic tool or treatment. Research and development, and innovation in this field represent an urgent need for society as a whole.

Aware of this situation, back in 2000 the European Parliament and the Council of the EU approved EU Regulation 141/2000, which will soon be updated, establishing, among other things, that "patients affected by rare diseases should be entitled to the same quality of treatment as other patients", incorporating incentives to improve knowledge and promote research, development and commercialisation of medicines in the field of rare diseases.

Since then, this legislation promoted by the EU and further activities in Spain have certainly had important positive effects. In fact, especially in recent years, the research efforts of pharmaceutical companies have been strengthened and the willingness of a growing number of companies capable of providing innovations in this field has increased.

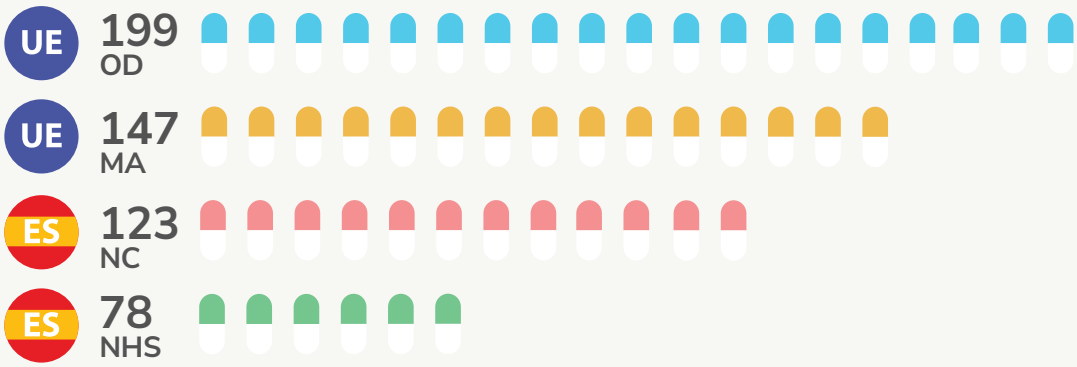
This is reflected in the data from the latest Annual Report on Clinical Trials in Rare Diseases in Spain, prepared by AELMHU. If in 2021 Spain reached a record number of clinical trials in the area of rare diseases, in 2022 this figure increased to 233 (3% increase from the previous year) and representing 25% of the total number of trials initiated in our country.

However, there are several areas for improvement so that the industry's efforts in delving into an area as complex as rare diseases have a direct reflection in better, and faster, access for patients.

In this sense, AELMHU will continue the dialogue and further analysis on numerous aspects, such as inequalities in the access to these treatments, or streamlining bureaucratic processes to facilitate an effective access to a unique opportunity for many people who suffer from this type of diseases and have yet to find a therapeutic alternative.

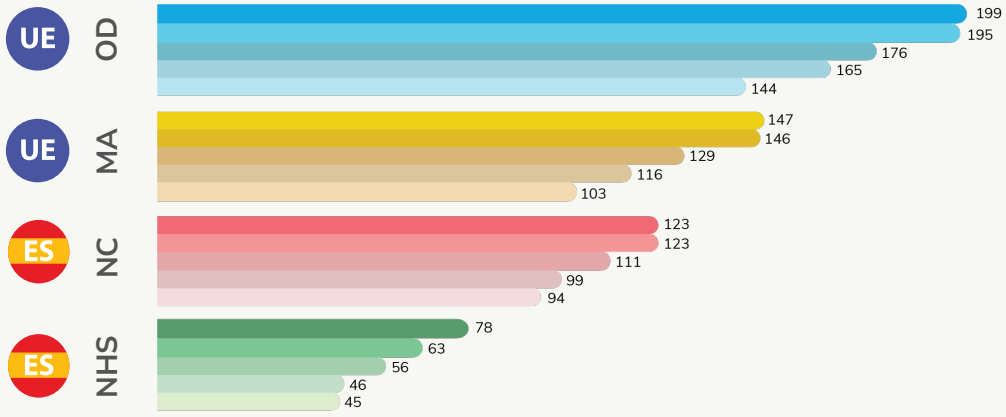
04 ORPHAN MEDICINES IN THE EU AND IN SPAIN 2023

Key indicators in the EU and in Spain



Year 2023 closed with 199 orphan medicines with a commercial name and with an active orphan designation as of 31 December 2023, 147 have an EU Marketing Authorisation, 123 received a National Code in Spain (84%), and 78 are reimbursed by the Spanish National Healthcare System (53%).

Evolution of the indicators in the EU and Spain 2019-2023



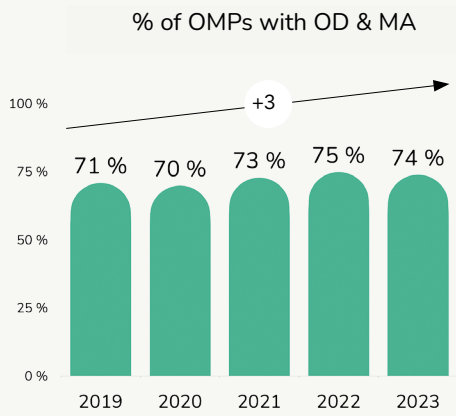
During 2023, almost all of the indicators reported here for the EU and Spain for orphan medicines improve, although importantly, with different magnitudes. The number of positive orphan designations **from the EMA and EU marketing authorisations have increased, albeit slightly**, while the number of orphan medicines that obtain the National Code in our country remains at 123.

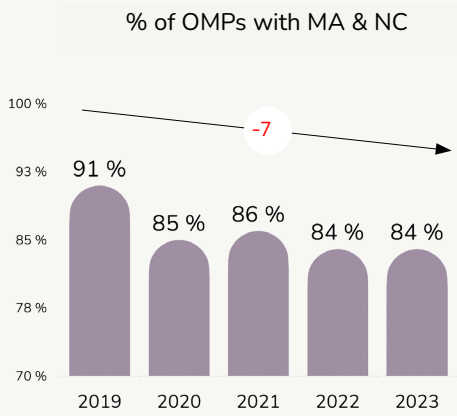
However, there is a **significant 24% increase in NHS-reimbursed orphan medicines**, with an additional 15 (from 63 to 78), and once orphan medicines that have lost their designation in 2023 (and therefore fall outside our relevant sample for analysis) are taken into account, as discussed below, even if they are still reimbursed.

Comparison across indicators

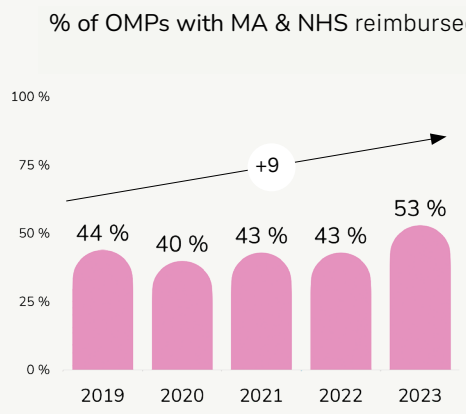
In total, as shown in the graphs below, **74% of all orphan medicines with a valid orphan designation have an EU marketing authorisation**, which represents a minimal reduction compared to the 2022 figure.

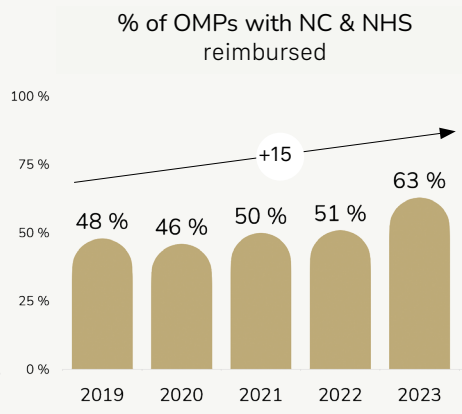
On the other hand, the proportion of applications **for the National Code in Spain** for those orphan medicines with a MA in 2023 remains identical to that of 2022, at **84%,** and seven percentage points lower than in 2019, when 91% of orphan treatments that had a marketing authorisation had requested a National Code.



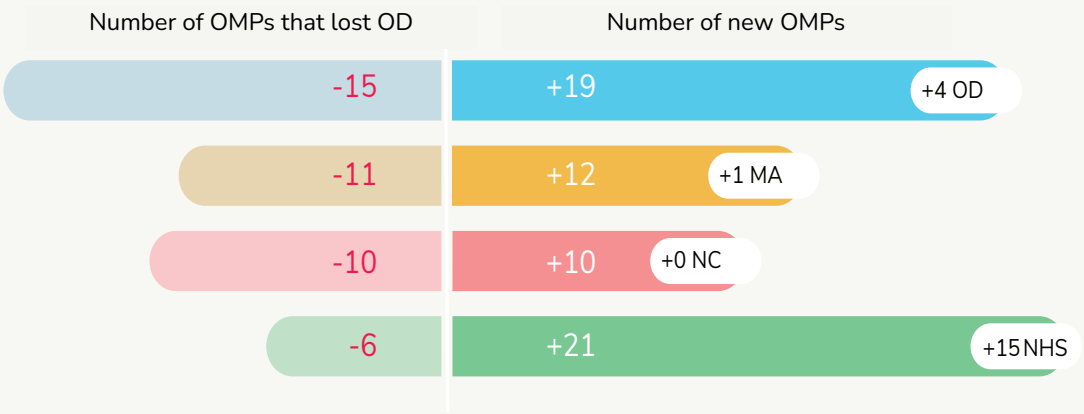


The significant increase can be seen in the orphan medicines reimbursed by the Spanish NHS, where **53% with an MA and 63% with a National Code, have been reimbursed in 2023**. These represent an increase of 10 and 12 percentage points respectively from 2022, the highest annual increase of any of the four indicators since 2019. Below we delve into orphan products that have been reimbursed in 2023.





Variation in the indicators 2022 – 2023

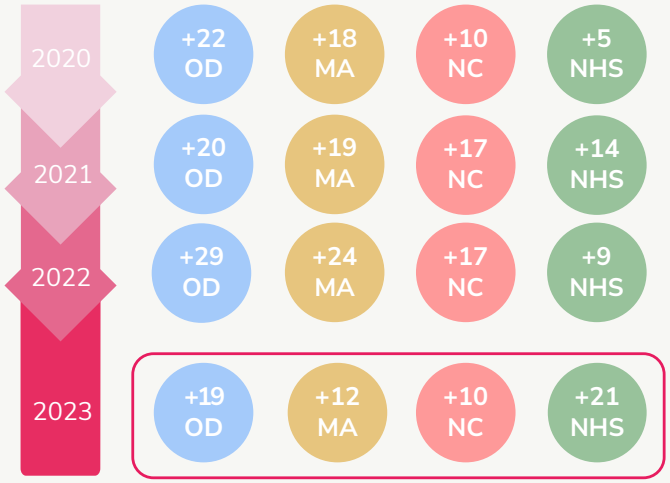


This Figure provides the number of **orphan medicines that have lost their orphan designation** during 2023 and would therefore be excluded from the analysis: 15 medicines with a trade name without an MA, 11 with an MA, 10 with a National Code, and 6 reimbursed. Thus, the main figures in **net terms for 2023** are as follows:

- An increase of 4 new positive designations.
- An increase of 1 marketing authorisation.
- Same number of National Codes.
- An increase of 15 new reimbursed orphan medicines.

Evolution of new orphan medicines and comparison over last years

The following figure shows the evolution of four key indicators included in the Report: new designations, marketing authorisations, national codes and products reimbursed in Spain, year by year since 2020.



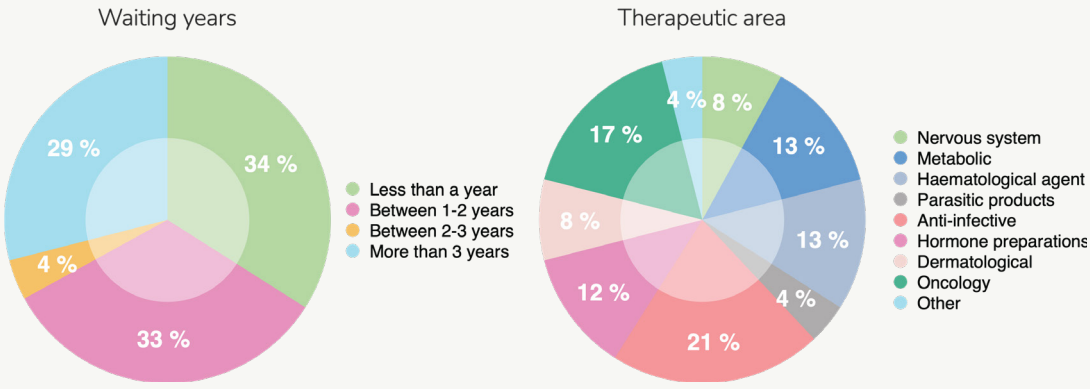
In absolute terms, all indicators improve in 2023, with double-digit growth, between 10 and 21 points. However, in relative terms compared to previous years, there are some worrying figures. In 2023, the first three indicators, designations, marketing authorisations and national codes, are at their lowest annual figures since 2020.

At European level, there are no more than 20 designations, with a more than 50% reduction compared to 2022. The number of marketing authorisations also suffered a similar decline, from 24 to 12. For Spain specifically, perhaps the most worrying figure is the 40% reduction in national codes, returning to 2020 figures. Without this authorisation, an orphan medicine cannot be available in Spain. Evaluating the performance of these indicators prior to obtaining NHS reimbursement is necessary to ascertain whether innovation is really reaching Europe and Spain. However, a considerable increase has been observed, in line with what has already been stated, in the number of new orphan medicines reimbursed in Spain (21), which represents a 133% increase compared to 2022.

05 ORPHAN MEDICINES WITH MARKETING AUTHORISATION WITHOUT NATIONAL CODE IN SPAIN

As of 31 December 2023, there were 24 orphan medicines with an EU marketing authorisation that had not yet arrived in Spain (without National Code), which represents 16% of those authorised at EU level, the same as in 2022, and the highest figure since 2019.

In terms of time elapsed since their marketing authorisation date, we can differentiate orphan medicines for which less than one year has passed (one third), between one and two years (another third) and more than two years (remaining third), but with a worrying almost 30% with a marketing authorisation date of more than 3 years ago. This figure is higher than in 2022.

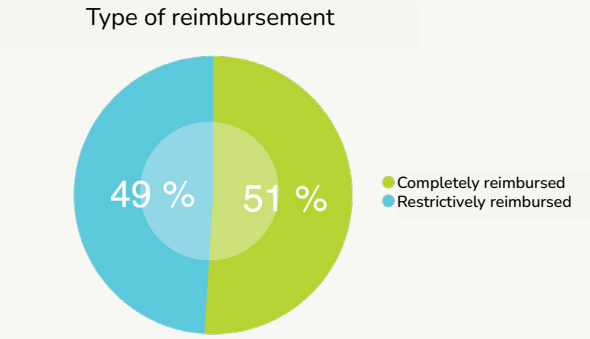


By therapeutic area, anti-infectives become the predominant area (21%), followed by oncological (17%), metabolic and haematological agents (13%), and hormonal preparations (12%).

06 ORPHAN MEDICINES REIMBURSED IN SPAIN

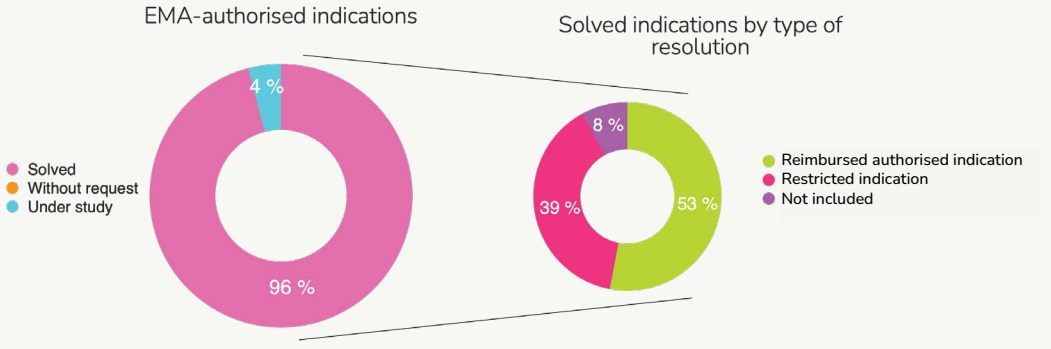
Reimbursed medicines in Spain

Of a total of 123 orphan medicines with a National Code, 78 are reimbursed, which represents 63%, 12 points higher than in 2022.



Of these, as indicated in the figure above, 49% (38) of the 78 orphan medicines that currently have a positive pricing and reimbursement resolution in the NHS, are reimbursed with restrictions, either due to restrictions in the authorised indication(s), or because they have non-reimbursed indications.

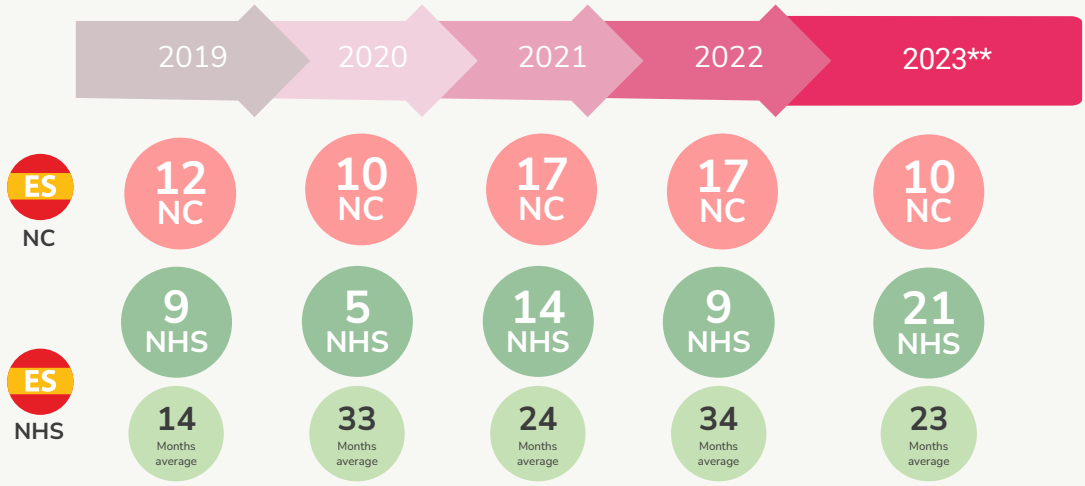
In addition, the 78 orphan medicines have a total of 113 indications authorised by the EMA, and of these, 60 are reimbursed without any restrictions, as detailed in the figure below:



Medicines reimbursed in 2023*

During 2023, 21 new orphan medicines were reimbursed in Spain: six during the first four months, nine in the second four months and six in the final four months. The 2023 figure is an all-time high in terms of orphan medicines reimbursed by the NHS, with an increase of 133% compared to 2022, and 50% compared to 2021.

However, the number of National Codes in 2023 decreases by more than 40% compared to 2022, matching the lowest figure since 2019.

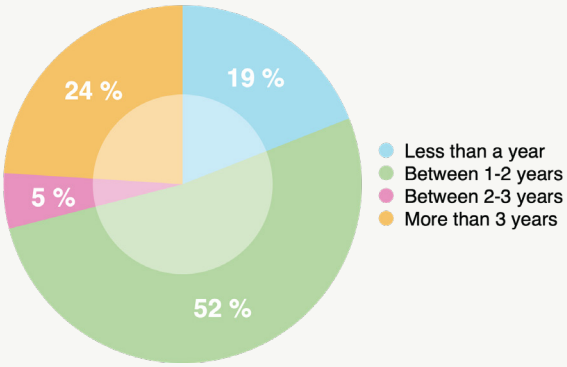


The average time elapsed for the 21 new medicines between the date of the EU marketing authorisation and National Code was six months (two months less than in 2022). The time between receiving the National Code and obtaining the positive pricing and reimbursement decision was 23 months, 11 months less than in 2022, which was the year with longest delay over the last five years. These times are similar to those of 2021, although they are still higher than in 2019, the year with the lowest figure in the series (14 months).

*Average waiting time between date of National Code and reimbursement date.
**As of 31 December 2023, EVRYSDI has not been included in this analysis, since although it obtained its positive reimbursement decision from the NHS on 1 January 2023, its orphan designation was withdrawn on 22 September 2023.

Of the 21 new medicines reimbursed throughout 2023, more than half of them have waited an average of 1-2 years to secure reimbursement, and almost 30% more than 2 years.

% of OMPs reimbursed during 2023 by waiting years:



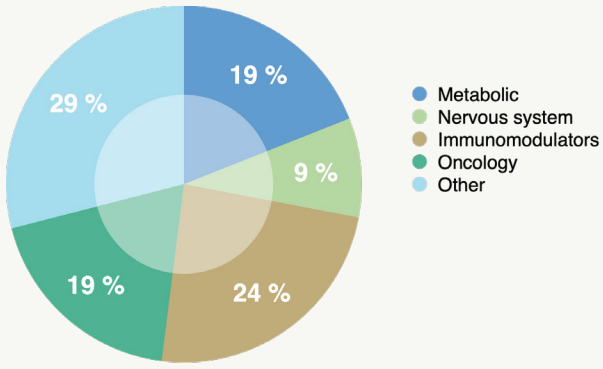
On the other hand, **38% of these 21 new medicines have restricted reimbursement** (based on the indications approved by EMA) and **100% of them have conditional reimbursement**.

In addition, all of them have follow-up conditions, **71% are subject to two or more reimbursement conditions**, 57% are subject to annual reviews of sales and prices, and 43% to an expenditure cap, among others.

As a novelty this year in the Executive Summary, 57% of the 21 orphan medicines have been considered at least twice by the Interministerial Commission on Medicines Prices (CIPM), and of these, five were considered three or more times, with a maximum of six times. On the other hand, all orphan medicines were reimbursed according to **criteria a and c**, as defined in current legislation, which refer to the severity, duration and sequelae of the pathology, and the therapeutic and social value of the medicine, respectively.

Finally, the three main areas of the 21 orphan medicines reimbursed this year are immunomodulators (24%), oncological diseases (19%) and metabolic diseases (19%).

% of OMPs reimbursed during 2023 by therapeutical area:

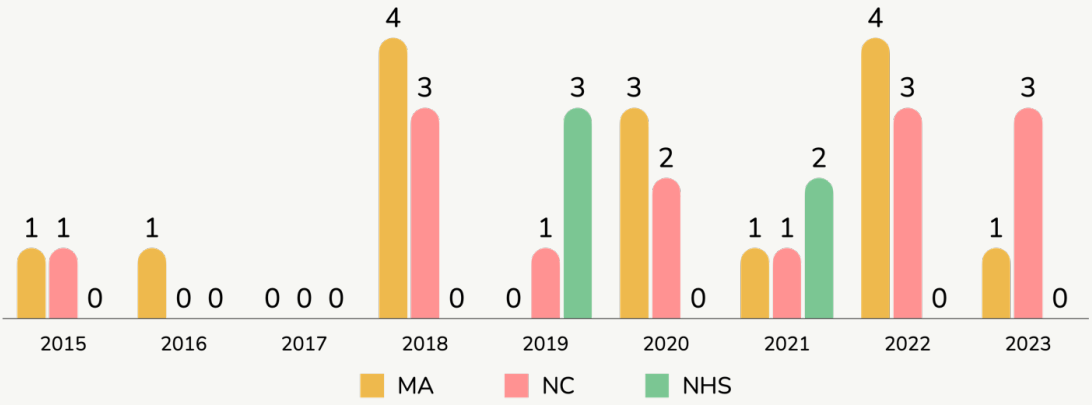


07 ADVANCED THERAPIES WITH ORPHAN DESIGNATION

During 2023, one therapy has received a marketing authorisation in Europe (four in 2022), three have obtained a national code, but none have been reimbursed by the NHS. The last two indicators are the same as in 2022.

With the new therapy, there would currently be 15 advanced therapies with an EU orphan designation and marketing authorisation, of which 14 have a National Code, 27% more than in 2022. However, since no therapies have been reimbursed in 2023, there are still five reimbursed (33%). Two of these therapies are oncological, and the rest are one ophthalmological, one immunomodulatory and one for the nervous system, respectively. In addition, all five therapies are included in Seguimed and Valtermed and have at least two additional reimbursement conditions, including payment-by-results (4) and price reviews (3).

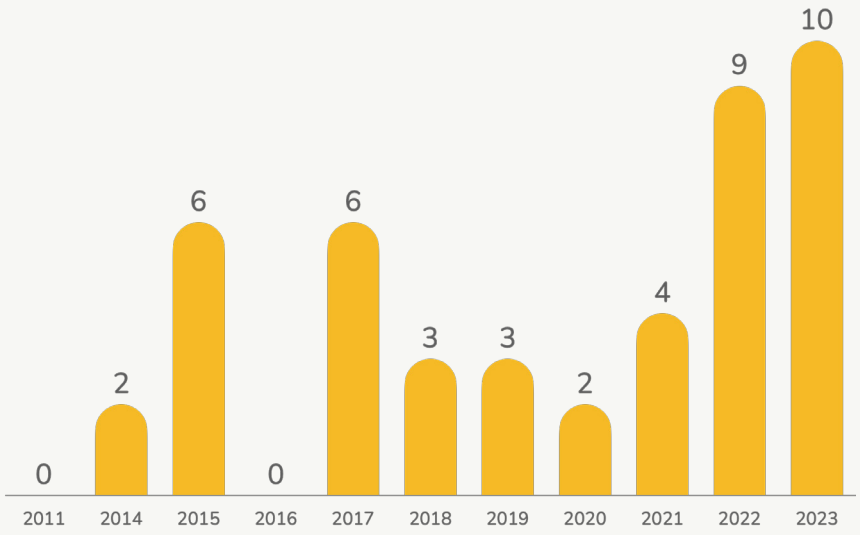
Of the remaining nine therapies not reimbursed by the NHS, four have an unfavourable pricing and reimbursement resolution, and five are under evaluation or have not asked for reimbursement.



Finally, **33 advanced therapies with an orphan designation** are included in the EMA's **PRIME programme**, which supports the development of medicines targeting unmet medical needs, and a further **three are under evaluation by the CHMP**.

08 ORPHAN MEDICINES NOT REIMBURSED

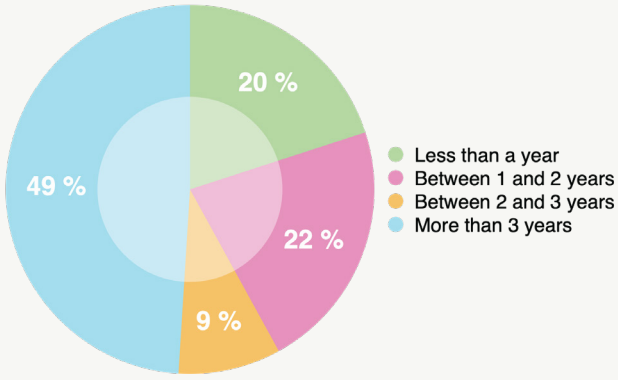
There are currently **45 orphan medicines not reimbursed** in Spain, 15 less than in 2022. Of these, 60% (27) have received a negative pricing and reimbursement resolution, while the remaining 40% (18) are under evaluation or their reimbursement has not yet been requested.



As shown in the figure, of these 45 medicines:

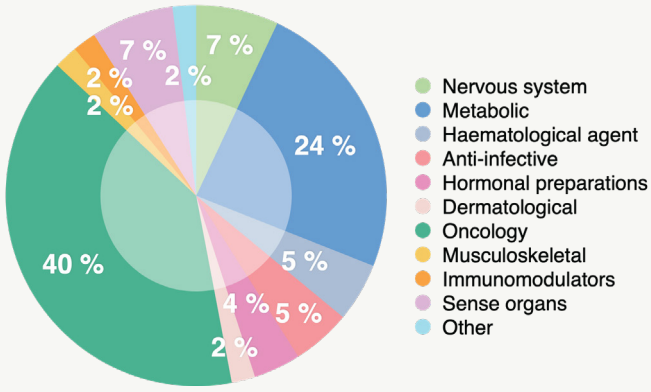
- 18% (8 orphan medicines) have been waiting for public reimbursement since receiving their national code between 2011-2015.
- 60% (27) of the non-reimbursed medicines received their national code during 2017-2022.
- 22% (10 orphan medicines) received their national code in 2023.

OMPs not reimbursed by waiting years



Moreover, almost half of the non-reimbursed orphan medicines with a national code **have been waiting more than three years**, and only 20% have been in this situation for less than a year.

OMPs without reimbursed by therapeutic area



Of the non-reimbursed orphan medicines, 40% are for oncological diseases, 24% for metabolic diseases and 7% for immunomodulators, among others.

The Annual Report also includes information on CIPM meetings that have considered these medicines and the agreed criteria for denying their reimbursement (information reflected in the minutes published as of 31 December 2023).

In summary, of the 21 orphan medicines that have been considered by the CIPM during the period 2018-2023, and according to the information available on the Ministry of Health's website on the **agreed criteria for denying their reimbursement**, in all but 2 the **criterion d** - rationalisation of expenditure - was selected. On the other hand, **criterion c** – therapeutic and social value – was included in 16 decisions refusing reimbursement, and, finally, **criterion e** – existence of other alternatives at a lower price – in three decisions.

Of the 10 medicines not reimbursed receiving a national code in 2023, only two have been considered by the CIPM. For both, the reason included for rejection was **criterion d**, while for one, **criterion e** was also included.

09 KEY RESULTS

01 For AELMHU, the data on access to orphan medicines in 2023 reflect that the future of orphan treatments in the EU continues to be positive, which represents **extraordinary news for all those patients and families** who have not yet found a diagnosis or treatment for their pathologies and look to pharmaceutical innovation with great hope.

02 In the last year, **almost all parameters at EU level have improved, but with differences**. At the national level, an all-time high has been achieved in terms of orphan medicines reimbursed by the NHS (21), and the time elapsed between the national code and a positive pricing and reimbursement decision has been reduced to 23 months. However, there has been a reduction in the number of national codes granted in 2023, so the percentage of national code applications with respect to the total number of marketing authorisations at EU level remains the same.

03 The figures in the EU are positive, in terms of new orphan designations (19) and new marketing authorisations (12). However, **these numbers are much lower than those observed during 2020-2022** and would need to be reversed if there is no improvement during 2024. Therefore, these two indicators deserve to be monitored in the short term.

04 In terms of reimbursement, the percentage of orphan medicines reimbursed by the NHS has managed to exceed 50% of the total number of European marketing authorisations, implying that **Spain now reimburses 5 out of every ten orphan medicines authorised by the EU**. This increase includes the reimbursement of orphan medicines that had already been waiting for several years, which were reflected by the lower numbers of reimbursed medicines in recent years as well as the increase in those not reimbursed. However, **none of the 10 treatments that have obtained a national code during 2023 has been reimbursed** (the most recent date for the 21 medicines is November 2022).

05 The percentage of new products reimbursed during 2023 with restrictions (based on EMA-approved indications) has been reduced, but **all of them are still subject to conditional reimbursement and follow-up conditions**, and now 71% are subject to two or more reimbursement conditions, 57% to annual reviews of sales and prices, and 43% to expenditure caps.

06 On the other hand, as of 31 December 2023, there were **24 orphan medicines with an EU marketing authorisation but yet to receive a national code in Spain**. This represents 16% of the total authorised orphan medicines in the EU, same percentage as in 2022, and the highest figure in the last four years. Of these, 67% have been authorised for more than a year without having yet entered our market, which demonstrates a lower availability of relevant innovations for Spanish patients.

07 Likewise, despite these developments are the future of healthcare, particularly in the field of rare diseases, in 2023 Spain did not reimburse any advanced therapies with orphan designation. And of the 15 therapies with marketing authorisation in the EU, 14 have a National Code, 27% more than in 2022. Therefore, only 33% are now reimbursed by the NHS.

08 One of the most positive results compared to 2022 is the reduction in waiting times between receiving the national code and the reimbursement decision. Although the 11-month reduction is considerable, the current 23 months are **still far from meeting the timelines included in current legislation**.

09 Currently, **45 orphan medicines are not reimbursed in our country**, 15 fewer than in 2022, although almost half (49%) have been waiting more than three years. The main reasons for not reimbursing them have been the rationalization of public expenditure and the therapeutic and social value of the medicine.

10 ASSESSMENT AND RECOMMENDATIONS

01 This new edition of the Annual Report on Access to Orphan Medicines in Spain aims to continue providing information on the current, and historical, situation of the access to these treatments in Spain. The objective is to **continue the dialogue with all agents in the sector, in order to improve patients' access to the treatments** that, unfortunately, so desperately need.

02 The 2023 market access data shows positive and encouraging results, especially in terms of medicines reimbursed by the NHS, since it is the first time, at least since 2019, that **Spain reimburses five out of 10 orphan medicines with a European marketing authorisation**.

03 To a lesser extent, but also important and encouraging, is the **reduction in waiting times** between the National Code and the positive decision on pricing and reimbursement, especially if the trend of recent years, with delays of more than 30 months, is reversed. It is imperative to continue working on shortening these timelines to be able to provide treatment to patients in a timelier manner. To this end, AELMHU expresses the **commitment** of all its members to work collaboratively with all agents, in a trusted environment.

04 It is important to **note that none of the orphan medicines receiving a national code in 2023 is currently reimbursed** – related to waiting times, this aspect requires an effort from all parties to ensure better access to the recently authorised treatments.

05 There is also an indicator for Spain with no improvement, **since still 16% of orphan medicines with a marketing authorisation are without a national code**, and in fact, the upward trend in the number of medicines in this situation is worrying if it continues. While it is true that the reasons are complex, a short-term goal should be to reduce this percentage to at least 2019 levels (9%), with a view to reducing it further in the medium term.

06 This context leads us to demand, once again, that Spain should be able to **improve and speed up the approval processes of orphan medicines**, especially as their safety and efficacy have already been accredited by the European Medicines Agency (EMA).

07 Orphan medicines provide significant health benefits for patients with rare diseases, but also economic and social value for Spain, as AELMHU has been defending for many years, from the conviction that **innovation is a tangible asset for society as a whole and a project for the future and the country**.

08 In terms of legislative matters related to access, the 2023 elections disrupted the initial previsions that we already echoed in the 2022 Access Report, and the development of laws and regulations.

09 After a year with many novelties for Spain, the acceleration in the research and development of new orphan treatments represents a great opportunity that should **stimulate the sum of efforts** to place us at the forefront in terms of efficiency, responsiveness and attractiveness.

10 The factors that ultimately determine whether patients suffering from a rare disease have access to an orphan medicine are multiple and complex. The improvement in several indicators included in the Report is undeniable, but it is necessary to continue analysing many aspects, such as the **recognition of the economic and social value of pharmaceutical research on new products and indications, equity in access to these treatments, the streamlining of bureaucratic processes, the improvement of the evaluation process, and providing greater certainty, transparency and participation throughout the approval process, among others**.

11 To this end, **AELMHU shared** two documents with the **Ministry of Health during 2023**, one with recommendations about the evaluation and access process of orphan medicines, and another on an "Early Access" program for medicines with orphan designation or medicines for rare diseases.

A **summary** of these is given here, in the following aspects:

- In terms of evaluation, **set up a commission between the competent bodies** (and, in their absence, working groups) of the Administration and the sectoral representatives of the pharmaceutical industry, for the development of evaluation models for innovation specific to rare diseases. This commission **should collaborate with the existing initiatives** already developing more general methodological guidelines, with the aim of establishing a transparent and predictable process for the submission, resolution and reporting of allegations, and increasing patients' and clinicians' participation.

- The criteria for reimbursement set out in the current legislation would be appropriate at a general level, but it is necessary to continue working on the relevant Royal Decrees on the subject. The aim is to **be able to include other attributes beyond the evaluation of their cost-effectiveness and efficiency criteria**, such as medical need, quality of life and perceptions and experience of patients and their caregivers, the family burden of the disease, and aspects of equality, equity or solidarity. One possibility would be to use a multi-criteria decision analysis to determine the value of the orphan medicine.

- Linked to the evaluation of orphan medicines is their pricing and reimbursement decision. In the short to medium term, the goal should be to **improve on the good results of 2023** and ensure we do not return to the figures of previous years. To this end, at AELMHU we believe in the **need to provide certainty, agility, and greater transparency in the processes of evaluation, pricing and reimbursement**.

- There are controls and restrictions on the reimbursement and use of orphan medicines, which in part come from the uncertainty associated with their use due to the characteristics of rare diseases. These controls are often linked to clinical and/or financial results, stimulating greater transparency and governance, which allows a more homogenous access than the existing territorial disparity, and facilitates data collection. Still, **further work is needed on how to optimise the use of real-life data during the evaluation, pricing and reimbursement processes of orphan medicines**. A key element is to improve the State Registry of Rare Diseases (REER), ensuring that the collection of real-life outcome data is adapted to routine clinical practice in the Centres, Services and Reference Units of the National Health System (CSUR)/hospitals, supporting the digital transformation of the sector.

- **'Early access' models**, referring to situations where the patient has access to the medicine even if there is no pricing and reimbursement decision ("medicines in a temporary reimbursement situation"), were one of the most relevant topics for orphan medicines in Spain. It is important to note the significant reduction in treatments not reimbursed by the NHS. The time elapsed between issuing the National Code and the reimbursement decision depends on multiple factors, and at **AELMHU we are hopeful with the Ministry of Health's willingness to continue working towards our common goal of ensuring that patients have appropriate access to existing and future treatments**.

- The recommendations offered in 2023 by AELMHU on a possible accelerated evaluation model for "**medicines in a temporary reimbursement situation" are based on the commitment by companies to communicate their marketing decision in Spain to the AEMPS to initiate the pricing and reimbursement process**. Thus, for treatments that meet certain eligibility criteria, a process could be started, lasting up to 15 months and with a quality-register database, where the information available while it is temporarily reimbursed is collected and assessed. The pharmaceutical company would not be able to exceed the prices of relevant comparator countries where the medicine is already available. In addition, once the pricing and reimbursement process has been completed with a positive resolution, a rebate would be made based on the price differential; in the event of a negative resolution, its reimbursement would be determined according to need.

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As a final comment, and beyond the figures included in the Report, it is imperative to ensure equity in patients' access to orphan medicines between the different Autonomous Communities.

AELMHU would like to reiterate its invitation for all of us to join forces together – industry, patients, the scientific community, professionals and public decision-makers – in order to find solutions that improve the quality of life of all those people who suffer from a rare disease.

We hope that in our 2024 quarterly reports we can continue to report good news.

11 SOURCES OF INFORMATION

01 Medicines with positive orphan designation from the European Medicines Agency

02 Community Registry of Orphan Medicines

03 Orphan medicines authorised by the AEMPS

04 Orphan medicines reimbursed by the NHS (Nomenclátor)

05 Agreements Interministerial Commission on Medicines Prices (CIPM)

12 ACRONYMS

AEMPS: Spanish Agency for Medicines and Health Products

CHMP: The Committee for Medicinal Products for Human Use

COMP: Committee for Orphan Medicinal Products

EMA: European Medicines Agency

EU: European Union

MA: Marketing Authorisation

NC: National Code

NHS: National Healthcare System

OD: Orphan Designation

OMPs: Orphan Medicinal Products

REER: State Registry of Rare Diseases

aelmhu

spanish association of orphan and ultra-orphan drug laboratories

informacion@aelmhu.com