aelmhu
asociación española de laboratorios de medicamentos huérfanos y ultrahuérfanos

EXECUTIVE SUMMARY

REPORT ON ACCESS TO ORPHAN MEDICINAL PRODUCTS IN SPAIN 2020

INDEX

1. AELMHU ACCESS REPORT

Once again this year the Spanish Association of Orphan and Ultra-Orphan Medicines (AELMHU) will publish the main conclusions of its Report on the Analysis of Access to Orphan Medicinal Products (OMP) in Spain.

The study, which has become a benchmark standard in our country, offers an annual comparative analysis of the status of products possessing a trade name and positive orphan designation by the European Medicines Agency (EMA) in force as of the 31st of December, 2020. The report also assesses the procedure followed by these products upon their arrival in Spain, from assignment of a National Code (NC) by the Spanish Agency for Medicines and Health Products (AEMPS) to their public funding, over the course of the last year.

On the basis of this analysis the aim of the Association is to generate reference elements and indicators for use in the assessment of the approval and funding process as a whole, thereby contributing to the common goal of improving and expediting the availability of pharmaceutical innovation for all patients with rare diseases (RD) in Spain.

AELMHU is a non-profit organisation that, over the past decade, has brought together pharmaceutical and biotechnology companies with a strong commitment to discovering, researching, developing, and commercialising innovative therapies capable of improving the situation of patients suffering from rare diseases.

Current AELMHU member are: Alexion Pharmaceuticals, Alnylam, AMRYT Pharma, Novartis Gene Therapies, Biogen, Biomarin, Chiesi, CSL Behring, GW Pharmaceuticals, Intercept, Ipsen, Kyowa Kirin, PTC Therapeutics, Recordati Rare Diseases Spain, Sano, Genzyme, SOBI Swedish Orphan Biovitrum, Takeda, Ultragenyx and Vertex.

The Association's mission is to contribute to improving the situation of people affected by rare or infrequent diseases by promoting knowledge of their pathologies and recognition of the therapeutic and social value of OMP in their daily lives and those of their families.

This executive summary, prepared by Lasker at the request of AELMHU, analyses the principal indicators of access to OMP in the European Union (EU) and Spain, making these available to all interested parties - patient associations, public administrations, healthcare personnel, the scientific community and civil society - so that they may assess the availability of these treatments in our country.

2. METHODOLOGY

The 2020 Report on Access to Orphan Medicinal Products in Spain was prepared by Bioinnova Consulting in accordance with the following working methodology:

1. Of the more than 2000 active ingredients designated as orphan by the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA), those with orphan designation in force as of the 31st of December, 2020 (approx. 1700) and those with trade names (more than 200) have been identified. Among those with trade names there are products that appear more than once due to the fact that they have more than one indication. Removing these duplicities leaves us with a figure of 165 OMP.

2. Following this, we analysed which of these medicines have either been withdrawn from or have yet to be included in the European Commission's Community Register of Orphan Medicinal Products for marketing in the EU (n=49), resulting in the number of products that may be accessible for marketing as of the 31st of December, 2020 in European countries (n=116).

3. The third step was to identify which of these trade-name medicines with European marketing approval possess an AEMPS National Code authorisation for marketing in Spain (n=99).

4. Following this, those OMP that had been authorised for marketing as of the 31st of December, 2020, and subsequently marketed were identified according to the information collected in the AEMPS online medicine information centre: CIMA (n=51).

5. The commercialisation date of the marketed OMP was obtained from the Medication Database of the General Council of Pharmaceutical Associations (Bot PLUS) (not available for 5 OMP).

6. And finally, of those OMP that have obtained marketing authorisation from the AEMPS, we identified which of them have been funded by the Spanish National Health System (SNS) and since what date, according to the information contained in the Nomenclature of the Ministry of Health (n=45). The date of funding was taken as the initial date of approval.

3. OVERVIEW OF OMP IN THE EU AND SPAIN

Orphan medicinal products (OMP) are innovative medicines, many of them biotechnological in origin, destined for the diagnosis, prevention, or treatment of rare or infrequent diseases that affect fewer than 5 in 10,000 people and for which no alternative treatment currently exists.

There exist an estimated 6000 to 8000 different rare diseases in the world and diagnostic tools and treatments are only available for a very low percentage of these, around 5%. According to the Spanish Federation for Rare Diseases (FEDER), more than 3 million people in Spain are affected by this type of pathology.

In 2000, with the aim of improving the low level of scientific knowledge and boosting research in the field of rare diseases, the European Parliament and European Council adopted Regulation 141/2000, which states that "patients affected by rare diseases should be entitled to the same quality of treatment as other patients" and establishes incentives that promote research, development, and marketing of medicines by the pharmaceutical industry.

Since then, EU and Spanish legislation has sought to lay foundations that will facilitate the development of these medicines as well as their availability to patients with minority pathologies. However, continuing to improve equity in access to these treatments remains a necessity, as they represent a unique opportunity for many people who have not found therapeutic alternatives.

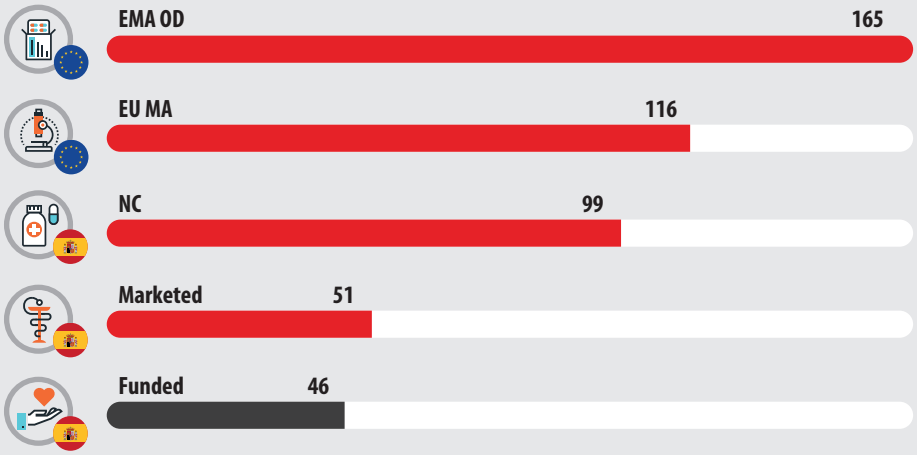
Fortunately, more and more laboratories have shown a willingness to innovate in this area and interest in OMP has grown steadily in the EU over the last twenty years.

In 2020 alone, the European Medicines Agency approved **22 new positive OMP designations, 73% more than in 2019**, the highest number of new designations in the last 5 years.

In all, as of 2020 **the EU currently lists 165 products** with a positive brand name and orphan designation, a **14.5% increase** on the previous year and the highest on record.

Community marketing authorisations in the area of OMP are also on the rise, **with 18 new authorisations, representing an increase of 13%** in a single year, bringing the total number of OMP authorised in all Member States to 116 by the end of 2020.

PRINCIPAL ACCESS DATA TO OMP IN THE EU AND SPAIN, 2020

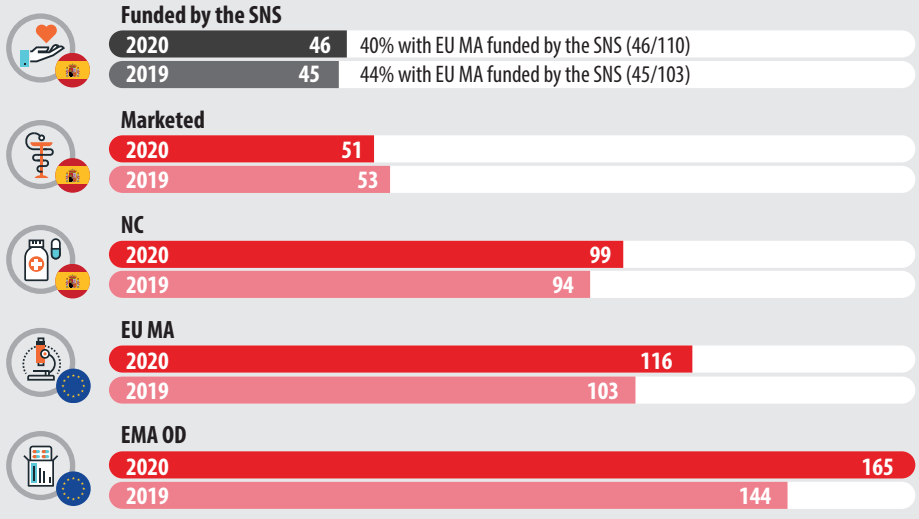


Of the 116 OMP currently in possession of EU marketing authorisation:

- **99** have obtained a **National Code** in Spain (85%).
- **51** have been authorised for marketing in our country (44%).
- and just **46** are funded by the SNS (40%).

While interest in orphan therapies is growing throughout Europe and the industry remains firmly committed to innovation in the field of rare diseases, access to innovation for patients is slowing down in all areas in Spain.

EU/SPAIN COMPARATIVE ACCESS DATA 2019_2020

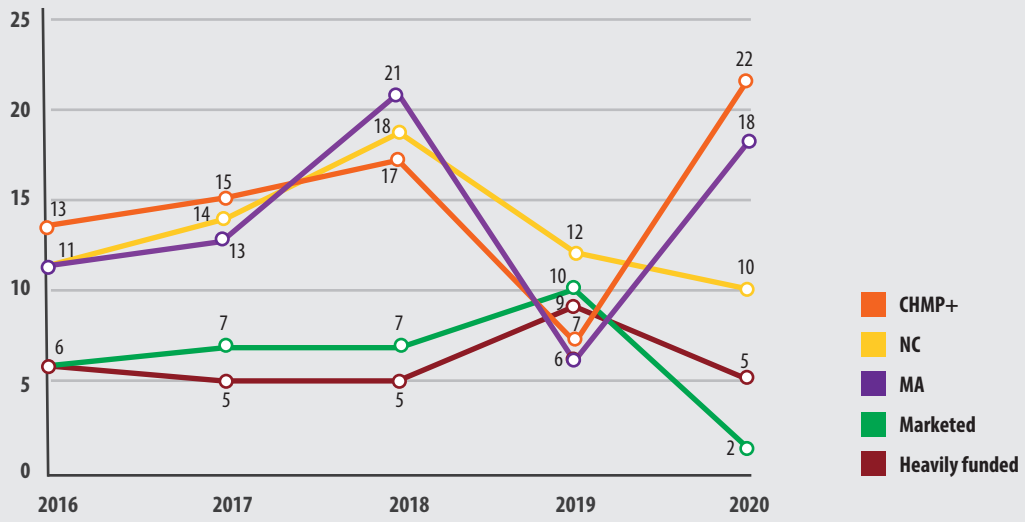


From the above we can deduce that between 2019 and 2020:

- **National Code applications fell by 6%** (from 91% of those authorised in the EU in 2019 to 85% in 2020).
- **National marketing authorisations decreased by 7%** (from 51% of EU MAs in 2019 to 44% in 2020).
- **the number of new orphan drugs funded by the SNS also fell, by 4%** (from 44% of those authorised in the EU in 2019 to 40% in 2020).

Moreover, if we analyse the period 2016-2020, we are looking at the worst data of the last five years. As can be seen in the graph below, the proportion of new orphan drugs funded per new drug marketed in Europe has fallen by 20% in five years.

COMPARATIVE ACCESS DATA 2016_2020 IN THE EU AND SPAIN

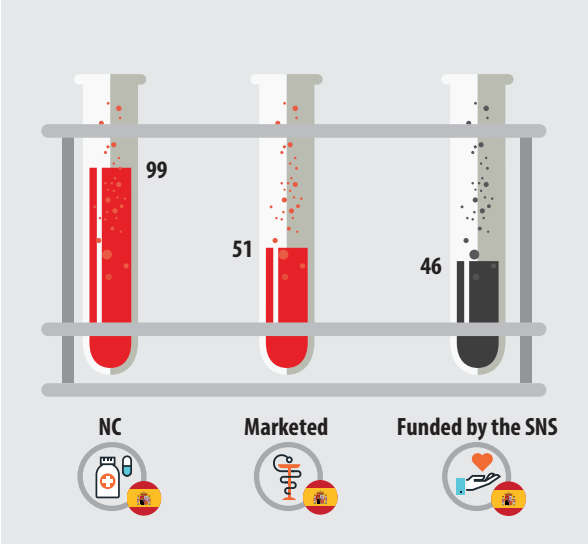


4. ACCESS TO OMP IN OUR COUNTRY

According to data from the 2020 Access Analysis Report, the upward trend reflected in the EU contrasts with access data in Spain, which shows a considerable decrease, especially in the funding of new treatments.

As the graph below shows, **only 46 OMP out of the 99 that arrived in our country last year have received SNS funding.**

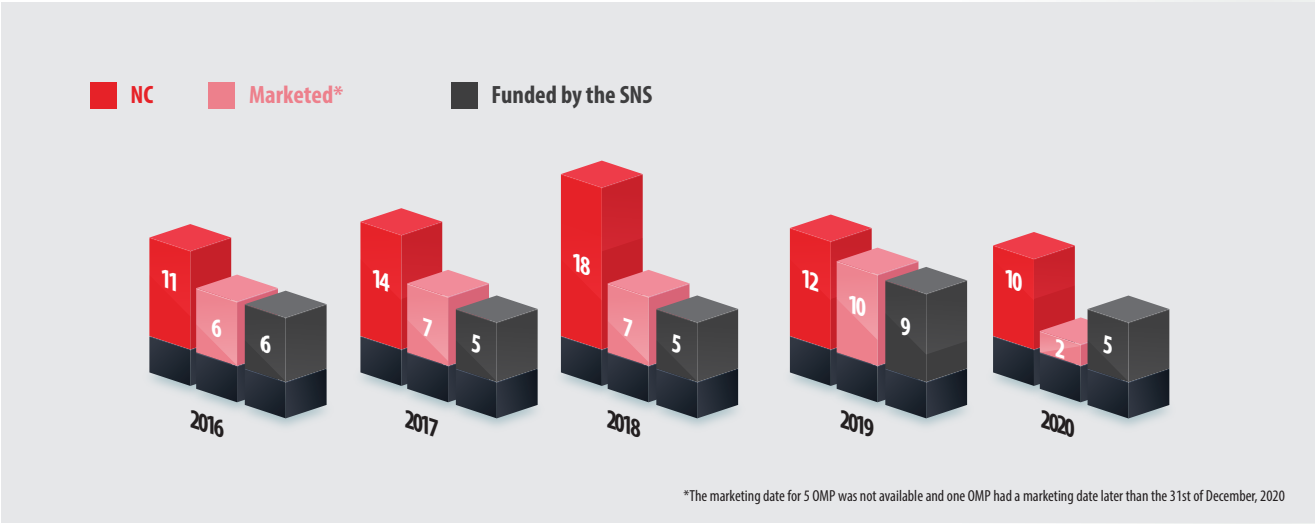
Nº OF OMP WITH NC AND FUNDING APPROVED BY THE SNS IN 2020



In total, only **5 new OMP were funded in Spain last year**, meaning 4 fewer funded products than in 2019, a decrease of 45% in a single year.

This reality could distance the availability of pharmaceutical innovations for Spanish patients with rare diseases with respect to other countries in our environment.

2016¬2020 ACCESS DATA IN SPAIN



*The marketing date for 5 OMP was not available and one OMP had a marketing date later than the 31st of December, 2020

5. ACCESS TIMES

Research into rare diseases is urgent as many patients still lack specific treatment for their disease. While many pharmaceutical companies are committed to investing in and developing drugs for the treatment of rare diseases, **access to OMP by Spanish patients is hampered by significant barriers.**

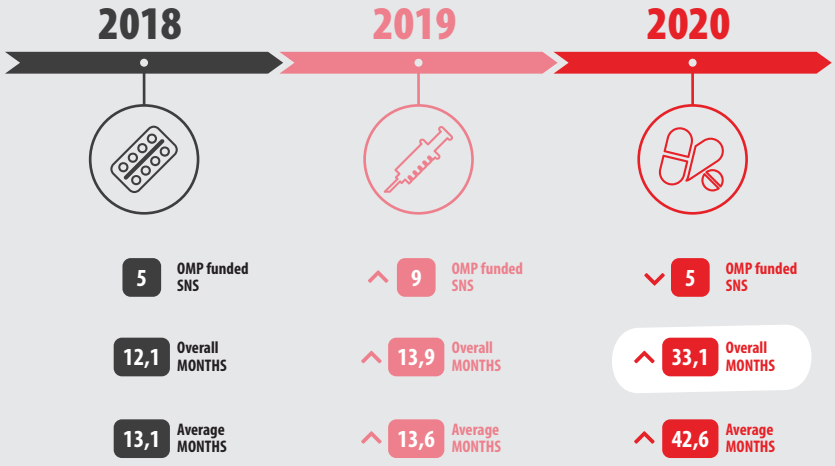
According to data from the AELMHU 2020 access report, one of the most important barriers is undoubtedly the waiting period for funding, which is increasing as the years go by. **At present in Spain 46 OMP receive funding while for 53 funding remains pending.**

FUNDED MEDICINES

With regard to the 46 funded medicines, while the report identifies that during 2018 and 2019 the waiting time for public funding for an orphan drug reached approximately one year on average (12.1 months and 13.9 months, respectively), **by the 31st of December, 2020 the average waiting time in our country had increased by 19 months to 33.1 months on average.**

Of the five orphan drugs that finally obtained public funding in 2020, three had to wait more than four years for decisions regarding pricing and reimbursement.

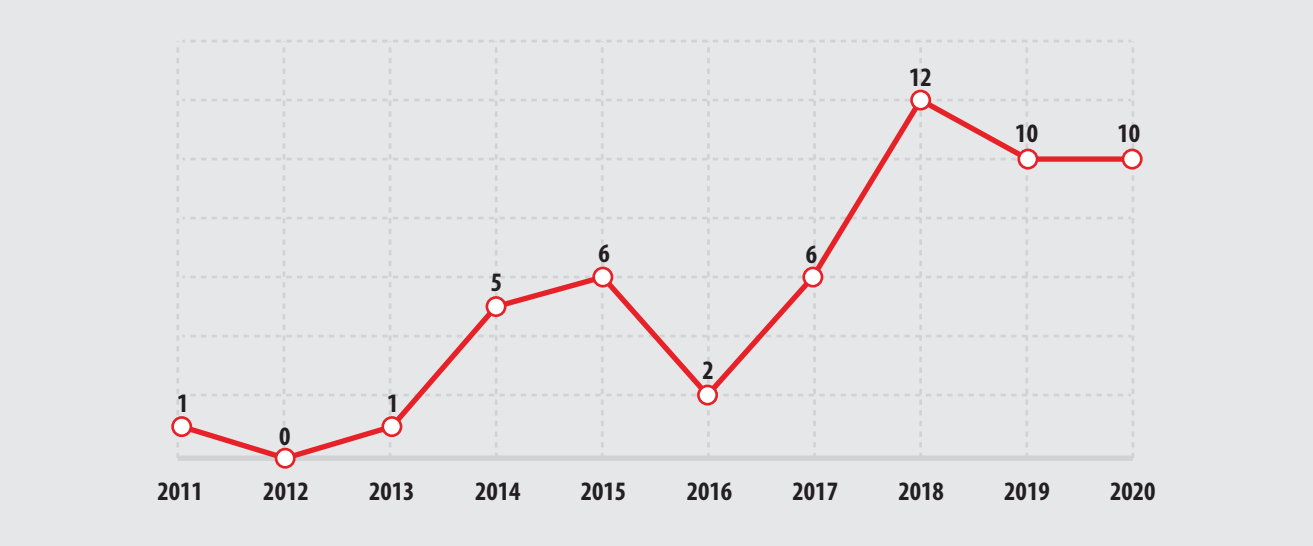
INCREASE IN FUNDING WAITING TIMES 2018_2020



MEDICINES PENDING FUNDING

Regarding the 53 products pending positive funding, the report also shows that average waiting times are soaring: **54% of having waited more than 3 years for decisions relating to pricing and reimbursement.**

NUMBER OF OMP WITH NC PENDING FUNDING IN SPAIN (2011_2020)



On the basis of the above it is clear that:

- 13 OMP have been awaiting public funding since the 2011-2015 period.
- **Since 2016, 40 OMP still await SNS funding:**
  - **18%** of OMP that received NC in **2016**.
  - **43%** of OMP that received NC in **2017**.
  - **67%** of OMP that received NC in **2018**.
  - **83%** of OMP that received NC in **2019**.
  - And **100%** of OMP that received NC in **2020**.

6. AELMHU ASSESSMENT OF 2020 ACCESS DATA

1 Despite the positive progress of innovation in the field of rare diseases in the EU, our country shows a downward trend in all parameters and indicators relating to assessment of access to orphan drugs by Spanish patients.

2 The ratio of public funding for orphan treatments in our country in 2020 is 45% lower than in 2019. Only 4 out of 10 medicines authorised in Europe are funded in Spain.

3 The pace of this funding is also slowing. The waiting time for an orphan drug to obtain decisions regarding pricing and reimbursement has increased by 19 months to more than 33 months on average.

4 There are still 53 National Code treatments pending favourable price and reimbursement decisions: 2 out of 5 have been waiting for more than 3 years.

5 This situation may act as a disincentive to the arrival of new therapies in Spain, thereby impairing patient access to new medicines available on the market.

6 In order to improve this situation AELMHU considers it essential to ensure equity in diagnosis and access to treatment for Spanish patients, to develop a clear, streamlined process to reduce funding delays, and to aim for greater certainty and consensus in assessments.

7 To this end, the Association wishes to continue to show its support for all those living with rare diseases via new outreach and awareness-raising projects and by maintaining an unwavering commitment to research into and development of new orphan and ultra-orphan treatments

8 Aware as we are of the difficult situation that our country is undergoing as a result of the pandemic caused by COVID-19, and with the firm conviction that improving access to orphan drugs in Spain is a task that challenges us all, AELMHU also wishes to reiterate its offer to the Administration to seek a joint solution capable of ensuring that all orphan therapies reach patients suffering from a minority disease in our country as soon as possible.

7. BIBLIOGRAPHY AND ACRONYMS

BIBLIOGRAPHY

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https://www.ema.europa.eu/en/medicines/download-medicine-data#rare-disease-(orphan)-designations-section

2 Community register of orphan medicinal products:
http://ec.europa.eu/health/documents/community-register/html/reg_od_act.htm?sort=a

3 Orphan medicinal products authorised by AEMPS:
https://cima.aemps.es//cima/publico/home.html

4 Healthcare information database, Bot PLUS:
https://botplusweb.portalfarma.com/

5 Orphan medicinal products funded by SNS:
https://www.mscbs.gob.es/profesionales/farmacia/home.htm

6 Interministerial Commission agreements on medical product prices:
https://www.mscbs.gob.es/profesionales/farmacia/CIPMyPS.htm

ACRONYMS

EMA:	European Medicines Agency
COMP:	Committee for Orphan Medicinal Products
OD:	Orphan Designation
MA:	European Marketing Authorisation by centralised procedure
UE:	European Union
NC:	National Code
SNS:	National Health System (Spain)
AEMPS:	Spanish Agency of Medicines and Medical Devices
OMP:	Orphan Medicinal Products
CHMP:	Committee for Medicinal Products for Human Use

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