ANNUAL REPORT ON ACCESS TO ORPHAN PRODUCTS IN SPAIN2023

The Spanish Association of Orphan and Ultra Orphan Drug Laboratories (AELMHU) publishes the results of its Access Report 2023.

SPAIN FINANCES MORE THAN HALF OF THE ORPHAN PRODUCTS AUTHORIZED BY THE EU FOR THE FIRST TIME IN 5 YEARS

- *Spain finances 53% of orphan products authorized in Europe*
- *In 2023, the average time from National Code to public funding of orphan products isalmost two years.*
- *Orphan products are innovative treatments that are exclusively aimed at diagnosing,preventing or treating patients with rare diseases.*

Madrid, January 24, 2024 - *A few weeks before the celebration of World Day for Rare Diseases, the Spanish Association of Orphan and Ultra-Orphan Drug Laboratories (AELMHU) publishes the results of its Annual Access Report, which analyzes all orphan medicinal products (OMPs) with a commercial name, which have the European orphan designation in force as of December 31, 2023. In it, the Association analyzes in detail the situation of orphan products in Europe and their availability for patients with rare diseases (RD) in Spain.*

According to the data available for 2023, at the European level there has been a decrease both in the number of new orphan designations, from 29 in 2022 to 19, and in the number of new commercial authorizations, from 24 in 2022 to 12. However, in this same period, Spain has improved its access data, increasing the number of orphan products financed and reducing the times for their financing, thus contributingto improving the decrease that has occurred in recent years.

In 2023, 21 new orphan products were financed, 12 more than in the previous year, reaching a total of 78 orphan products financed by the National Health System (NHS), of the 147 that have marketing authorization from the European Medicines Agency (EMA), i.e. 53%. Compared to previous years, this is the first time in five years that Spain has financed more than half of the orphan products authorized by the European Union. Despite this, it should be borne in mind that almost half of these new products are financed with restrictions. In addition, 100% obtained a conditional price: 5 have monitoring conditions, 12 are subject to a sales and price review, 1 is subject to a maximum cost per patient and 9 have an expenditure ceiling, among other conditions. In contrast, there are still a total of 45 orphan products that are not funded, corresponding to therapeutic areas such as oncology at 40% and metabolic at 24%,

linkedin © 2023

the main unfunded areas. In addition, 49% of these products have been awaiting funding for more than 3 years. The criteria for not financing them are mainly two: for reasons of rationalization of public spending in pharmaceutical provision and for their therapeutic and social value and incremental clinical benefit, taking into account their cost-effectiveness ratio.
In this regard, no advanced orphan therapy was approved for funding last year, something that already happened in 2022.

At ending, Spain only had 123 orphan products with a National Code out of the 147 that were authorized for marketing in the EU. This means that 24 authorized orphan products did not reach our country (16% of those authorized). This is the same percentage aslast year, which is the highest figure in the last 5 years.

One of the determining factors that could cause innovation not to reach EU Member States, once a marketing authorization has been obtained, are delays in financing times. In 2022, funding for orphan products reached an average waiting time of 3 years, which could partly explain why 16% of the products authorized in Europe have not arrived by 2023.

However, the average time to funding in the last year has been reduced to 23 months, 11 months less than in 2022. This undoubtedly represents significant progress compared to the delays that have occurred in recent years. However, it is necessary to continue this momentum in order to reach the financing times of 2019, whose average waiting time was 14 months, or even to reduce them..

According to María José Sánchez Losada, president of AELMHU, "during 2023 and despite the intensity of the elections, AELMHU has intensified the dialogue with the different public administrations to raise awareness of the slowdown and decline in access to orphan products in recent years. The improvements in both funding and waiting times during the last year have brought about a significant change which, from our Association, we value positively and we hope that this will become a trend in the coming years".

In this sense, the Association highlights the joint effort of all the actors in these changes led by the Ministry of Health and reinforces the organization's commitment to "continue working and providing recommendations on the evaluation, access and financing of orphan products with the agents involved in improving the quality of life of patients with rare diseases", concludes the president.

About AELMHU

AELMHU is a non-profit association, which brings together pharmaceutical and biotechnology companies with a clear commitment to invest in discovering and developing innovative therapies capable of improving the health and quality of life of patients suffering from rare and ultra-rare diseases. AELMHU wants to serve as an interlocutor with society, the scientific community and political and health institutions on issues related to orphan and ultra-orphan products.

© 2023

Currently, AELMHU's partners 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.

linkedin

© 2023