WORLD RARE DISEASE DAY

According to the Report of Access to Orphan Drugs in Spain 2021

THE NHS FINANCES ONLY 50% OF THE ORPHAN DRUGS THAT ARRIVE IN SPAIN

- In the past 3 years, Spain has rejected the financing of more than half of the orphan drugs that have arrived in our country, with only 48% financed in 2019, 46% in 2020 and 50% in 2021.
- Of the 129 products that have orphan designation and marketing approval in the European Union as of December 31, 2021, 111 have arrived in Spain and only 56 are financed by the National Health System (NHS).
- The average financing process time has been 2 years -- 11 months less than in 2020, but 10 months longer than in 2019.
- Orphan drugs are innovative treatments that are exclusively aimed at diagnosing, preventing, or treating minority diseases.

Madrid, February 21, 2022.- In honor of World Rare Disease Day, The Spanish Association of Orphan and Ultra-Orphan Drug Laboratories (AELMHU) presented its Report of Access to Orphan Drugs in Spain, which analyzes the evolution and current situation of orphan drugs in Spain.
The report reveals that in 2021 there was an improvement in access, approval times and in the financing of orphan drugs compared to the previous year, which was affected by the impact of the Covid-19 pandemic.
In the past year, 14 new orphan drugs have been included in the National Health System (NHS), compared to the 5 approved in 2020, bringing the current number of orphan drugs currently financed to 56.
Despite these ratios, only 50% of the orphan drugs that have arrived in our country are financed by the NHS, which is a very similar proportion to that which has been maintained in recent years (48% in 2019 and 46% in 2020).
The average time to obtain such financing was 24 months -- 11 months less than in 2020, although the average is still far from the 14 months in 2019.
According to María José Sánchez, president of AELMHU, "the indicators of access for patients with rare diseases to their treatments improved in the past year, resulting from effort made by the Administration and the flexibility shown by the pharmaceutical companies, but we must remember

that this improvement only shows we've made up for the delays accumulated during the pandemic.

The data reveals that of the 55 orphan treatments awaiting funding, 51% have been on hold for more than 3 years, showing a delay in access for patients. This time barrier may increase if the current situation ends up discouraging the arrival of the growing number of innovations in the field of rare diseases to Spain. Currently, 18 orphan drugs with marketing approval have not arrived in Spain, of which 61% have had this authorization for more than a year.

For the first time, the report includes a specific vision of advanced therapies, considered to be the treatments with the best prospects for the future. In 2021, of the 14 drugs financed by the NHS, 2 were advanced therapies, and the average time for approval was 21 months. Advanced therapies are, therefore, in a similar situation to other orphan drugs, both in terms of financing and waiting times.

In this regard, the president of the Association calls for "progress towards a new model that allows our country better access to innovation in the treatment of rare diseases, especially considering that pharmaceutical research has accelerated and will offer a greater number of therapeutic solutions for rare diseases in the coming years. These therapies are capable of improving patients' quality of life and that of their families".

For AELMHU, it is essential to review and improve the current system to develop a new model that provides more adaptability and clarity in the processes, by shortening timeframes and generating greater certainty and consensus in the evaluations. These measures would increase Spain's attractiveness in this area and encourage the greatest possible number of therapies to reach people affected by a rare disease.

With this objective on the horizon, the Association reiterates its full willingness to contribute its knowledge and effort, convinced that only the union of all the agents involved in the process will contribute to improve the quality of life of patients with rare pathologies that, in most cases, lack treatment.

About AELMHU

AELMHU is a non-profit association that brings together pharmaceutical and biotechnology companies with a clear commitment to invest in the discovery and development of innovative therapies, capable of improving the health and quality of life of patients who suffer from rare and ultra-rare diseases.

AELMHU would like to serve as an interlocutor between society, the scientific community, and the political and health institutions on the topic of orphan and ultra-orphan drugs.
Currently, AELMHU's members are Alexion Astrazeneca Rare Diseases, Alnylam, AMRYT Pharma, Novartis Gene Therapies, Biomarin, Chiesi, CSL Behring, Jazz Pharmaceutical, Ipsen, Insmed, Kyowa Kirin, PTC Therapeutics, Recordati Rare Diseases Spain, Sanofi Genzyme, SOBI, Takeda, Ultragenyx y Vertex.

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