FIRST REPORT ON THERAPEUTIC POSITIONING REPORTS OF ODs

SPAIN TAKES A YEAR TO EVALUATE ORPHAN DRUGS APPROVED BY EUROPE

- 40% of the TRPs of ODs initiated in the period 2013-2022 have not yet been published.
- In 38 of 52 financed ODs, the TPR was published after the favorable resolution of price and reimbursement
- The implementation of REvalMed has not improved evaluation times or the transparency of the evaluation process.

Madrid, July 4, 2022- The evaluation of Orphan Drugs (ODs) is delaying access to innovation for Spanish patients with Rare Diseases (RDs). This is the main conclusion drawn from the first Analysis of the Therapeutic Positioning Reports (TPRs) of Orphan Drugs carried out in our country. A pioneer project developed by the Spanish Association of Orphan and Ultra-Orphan Drug Laboratories (AELMHU) in its aim to provide the greatest amount of data and access indicators in Spain.

Even though all the ODs pass an exhaustive analysis by the European Medicines Agency (EMA), in which the agencies of the different Member States participate, when they arrive in our country, they begin a long process of re-evaluation in which the Spanish institutions re-examine their efficacy, as well as the budgetary impact they cause.

This evaluation is articulated through the so-called "Therapeutic Positioning Reports" (TPR), an essential tool for obtaining public funding for treatments. Despite the efforts made by the Ministry of Health to speed up the process in 2020, with the creation of the Drug Evaluation Network (REvalMed), the data show a delay in access to treatments of almost a year.

According to the main results yielded by this AELMHU study*, a total of 415 TPRs have been initiated in our country since 2013, of which 29% (122) correspond to ODs that develop therapies in a wide variety of areas, among which oncology (30%), metabolic products (21%) and antineoplastics and immunomodulators (10%) stand out.
Of these 122 TPRs, 49 (40%) are pending publication, while only 43% of them are publicly funded. Furthermore, in 38 of the 52 financed ODs, the TPR was published after the favorable price and reimbursement resolution.

On the other hand, each year the processing time for the TPRs of ODs is longer than for all other drugs, which shows that the information is not as swift as it should be. This makes access to therapeutic innovation in the field of RDs an increasingly slower and more unpredictable process.

Currently, our country takes 70% longer to evaluate orphan products than when TPRs were created, going from an average of 214 days between the start of the dossier and its dispatch to the Common Portfolio in 2013, to the current 363 days for the ODs evaluated by the REvalMed pilot program.

For AELMHU, the ODs should have economic evaluation criteria that consider the particularities of these diseases with low prevalence, and it is important to emphasize that the unmet needs that these drugs treat require an early approach in access.

The acceleration in the research and development of new ODs represents a great opportunity that should stimulate the sum of efforts of all the actors involved to place Spain at the forefront in terms of efficiency, response capacity and attractiveness in the scientific-healthcare field.

However, REvalMed is not producing the expected results. Since the prioritization criteria are not clear, there is a lack of transparency in the follow-up and coordination of the work of the Coordinating Group, which is delaying access for Spanish patients. Furthermore, as the Spanish Agency of Medicines and Health Products (AEMPS) itself has been demanding, it is necessary to provide this body with sufficient economic and human resources to deal with a massive volume of dossiers that is growing every year.

Therefore, AELMHU advocates the creation of an evaluation platform based on a homogeneous model of transparency, governance and adequate publicity that facilitates access to the ODs, to improve the quality of life of people with RDs.

* Analysis of Therapeutic Positioning Reports initiated between 2013 and March 2022, and with a publication date prior to May 13, 2022.

About AELMHU

AELMHU is a non-profit association that 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 drugs.

Currently, AELMHU's partners are Alexion AstraZeneca Rare Disease, Alnylam, AMRYT Pharma, Biomarin, Chiesi, CSL Behring, Ferrer, GenSight Biologics, Insmed, Ipsen, Jazz Pharmaceuticals, Kyowa Kirin, Novartis, PTC Therapeutics, Recordati Rare Diseases, Sanofi, SOBI, Takeda, UCB, Ultragenyx and Vertex Pharmaceuticals.