REPORT ON CLINICAL TRIALS FOR RARE DISEASES IN SPAIN 2025

Clinical trials **are research mechanisms used to determine the safety and efficacy of medicines**

Currently **94%** of rare diseases do not have a **diagnostic tool or treatment**

AUTHORIZED CLINICAL TRIALS (2024-2025)

Number of clinical trials

Of the authorized clinical trials are for rare diseases

Participants in clinical trials

Sponsors of clinical trials for rare diseases

Of clinical trials for rare diseases are sponsored by **commercial sponsors**

Phase of clinical trials for rare diseases

Participation of autonomous communities in clinical trials for rare diseases

Catalonia, the Community of Madrid, Andalusia and the **Valencian Community** are the regions with the most participation in clinical trials for rare diseases.

119 centres were involved in the 216 clinical trials for rare diseases in 2025

Research on rare diseases by therapeutic area

Of the 216 clinical trials for rare diseases authorized in 2025, 44 were in the field of oncology, 44 in the field of the immune system and 33 for diseases of the nervous system

Clinical trials with advanced therapy medicinal products in Spain

Of the authorized clinical trials involving ATMPs are for rare diseases

Of participants in clinical trials of advanced therapy medicinal products have been for rare diseases

CLINICAL TRIALS for RARE DISEASES (2020-2025)

Number of clinical trials

Over the past six years, 5,638 clinical trials have been authorized in Spain. **1,216 of which were for rare diseases (21%)**

Over the past six years, 340,377 participants have taken part in clinical trials, of which **34,310 participating in trials for rare diseases (7%)**

Clinical trials by sponsor

Clinical trials for rare diseases are driven by commercial sponsors

Participation of autonomous communities in clinical trials for rare diseases

Leading hospitals in clinical research for rare diseases

Clinical trials by therapeutic area

Oncology remains the most prominent area in clinical trials for rare diseases, but there **has been a shift towards other therapeutic areas**, particularly the immune system and the nervous system

Clinical trials with advanced therapy medicinal products for rare diseases

Between 2020 and 2025, a total of **245 clinical trials involving advanced therapy medicinal products were authorized in Spain, of which 144 (47%) focused on rare diseases**

Trials of advanced therapy medicinal products for rare diseases have involved **1,828 participants** in 2024 and 2025, accounting for **30% of participants in clinical trials of advanced therapy medicinal products**

Source: Spanish Register of Clinical Trials (REC - AEMPS), accessed in February 2026. Data analysis carried out by Biomedical Scientific.

* Figures for the period 2024-2025 are not valid for previous years, as the criteria were not being updated from the REC (AEMPS) before 2024.

** Clinical trials for rare diseases are those for rare diseases identified in ORPHA-KEM and GARD-KEM and which have been actually used in the Spanish Register of Participants Involved in Clinical Research on Rare Diseases.

*** Oncology includes all oncological diseases, regardless of the specific tissue affected. Thus, it includes, for example, all tumors of the nervous system and hematologic neoplasms (which, under other criteria, may not be considered "strictly" oncology in a given case).

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