REPORT ON CLINICAL TRIALS IN RARE DISEASES IN SPAIN 2024

aelmhu
Spanish Association of Medical Humanities and Bioethics

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

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

AUTHORIZED CLINICAL TRIALS (2023-2024)

Number of clinical trials

22% Of clinical trials authorized are in rare diseases

Authorized clinical trials (2023-2024)

Participants included in **clinical trials**

Participants in clinical trials (2023-2024)

Promoters of clinical trials in rare diseases (2023-2024)

95% Of clinical trials in rare diseases by commercial promoters

Clinical trials in rare diseases by promoter (2023-2024)

Phases of **clinical trials** in rare diseases

Clinical trials in rare diseases by phase (2023 - 2024)

Percentage change in clinical trials by phase (2023-2024)

Participation of the autonomous communities in clinical trials in rare diseases

Catalonia, the Community of Madrid and **Andalusia** are the autonomous communities with the highest participation in clinical trials

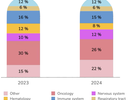
Center	Autonomous community	No. clinical trials
Hospital Universitario Vall d'Hebron	Catalonia	58
Hospital Universitario La Paz	Madrid, Community of	46
Hospital Universitario 12 de Octubre	Madrid, Community of	44
Hospital Clínico de Barcelona	Catalonia	40
Hospital Universitario La Fe	Community of Valencia	34
Hospital Universitario Virgen del Rocío	Andalusia	34
Clínica Universitaria de Navarra	Chartered Community of Navarra	34
Hospital Gregorio Marañón	Community of Madrid	33
Hospital de la Santa Creu i Sant Pau	Catalonia	32
Hospital Universitario de Móstoles	Community of Madrid	29
Hospital Universitario Ramón y Cajal	Community of Madrid	29
Complejo Hospitalario Universitario de Santiago	Galicia	29
Hospital Universitario Reyna de Mérida	Extremadura	28
Hospital Universitario Son Espases	Balearic Islands	28
Hospital de Sant Joan de Déu	Catalonia	26
Institut Català de Oncologia	Catalonia	25

Leading hospitals in clinical research in rare diseases (2023-2024)

Research on rare diseases by therapeutic area

Of the 202 clinical trials in rare diseases authorized in 2023, 56 have been in the area of oncology, 31 in the immune system and 25 for diseases of the nervous system, the same number of trials as for genetic phenomena

Clinical trials in rare diseases by therapeutic area (2023 -2024)



Clinical trials with **advanced therapies** authorized in Spain

52% Of the clinical trials with ATMPs authorized are for rare diseases

Authorized clinical trials with ATMPs (2023 -2024)

37% Of the participants included in CC trials with ATMPs have been for rare diseases

CLINICAL TRIALS IN RARE DISEASES 2020-2024

No. of **clinical trials**

In the **last five years**, 4,715 clinical trials have been authorized in Spain, **1,066 in rare diseases (23%)**

Authorized clinical trials (2020 -2024)

During the last 5 years, 260,198 people participated in a clinical trial, **18,972 in trials in rare diseases (7%)**

Participants in clinical trials (2020-2024)

Clinical trials by promoter

95% Of the clinical trials in rare diseases are commercial sponsored

Clinical trials in rare diseases by promoter (2020-2024)

Participation of research centers by autonomous communities

Clinical trials in rare diseases by autonomous community (2020-2024)

Leading hospitals in clinical research in rare diseases

Center	Autonomous community	No. clinical trials
Hospital Universitario Vall d'Hebron	Catalonia	572
Hospital Universitario 12 de Octubre	Community of Madrid	368
Hospital Universitario Virgen del Rocío	Andalusia	361
Hospital Universitario y Politécnico La Fe	Valencian Community	298
Hospital Clínico de Barcelona	Catalonia	294
Institute Catalan de Oncology	Catalonia	281
Hospital Universitario La Paz	Community of Madrid	253
Clínica Universidad de Navarra	Chartered Community of Navarra	219
Hospital Universitario Gregorio Marañón	Community of Madrid	217
Hospital Universitario Ramón y Cajal	Community of Madrid	183

Clinical trials by therapeutic area

Clinical trials in rare diseases have been mainly concentrated in the **oncology area**, followed by in rare diseases of the immune system, **nervous system, genetic phenomena and hematology**

Clinical trials with **advanced therapies** in rare diseases

Between 2020 and 2024, a total of **199** clinical trials with **advanced therapies** have been authorized in **Spain**, of which **101 (51%) have targeted in rare diseases**

Clinical trials with ATMPs (2020-2024)

Trials of advanced therapies for rare diseases have included **1,408 individuals** between 2020 and 2024, **36% of participants in clinical trials with advanced therapy drugs**

Participants in clinical trials with ATMPs (2020-2024)

"Source: Spanish Registry of Clinical Trials (REC - AEMPS), accessed April 2025. Data analysis prepared by Biomonitor Consulting.
Data from clinical trials with a start date in Spain between 01-01-2020 and 31-12-2024 inclusive. Only clinical trials with at least one site in Spain have been included. Multicentric clinical trials have been authorized in Spain.
Prices of the trials analyzed were harmonized, with each individual trial being counted in several autonomous communities. Some of the data in the report may differ from those in previous reports made after that year's report was published.

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