

REPORT ON CLINICAL TRIALS ON RARE DISEASES IN SPAIN

Analysis of data on clinical trials in the field of rare diseases authorized in Spain in the period from January 1, 2016 to December 31, 2020.

Data obtained through the Spanish Registry of Clinical Trials of the Spanish Agency of Medicines and Health Products (AEMPS), analyzed by the Spanish Association of Orphan and Ultra-Orphan Drugs (AELMHU).

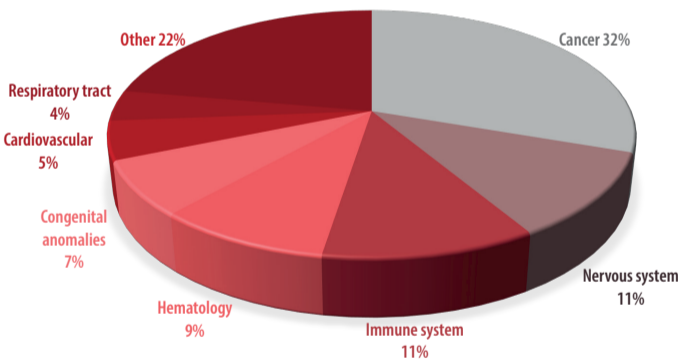


- There are more than **6,000 different Rare Diseases (RD)** in the world and only 5% have a diagnostic tool or treatment.
- **Clinical trials** are research mechanisms used to determine the safety and efficacy of drugs.
- In the field of RDs, they are particularly costly because of **the smaller number of patients, greater geographic dispersion and less specialization in the health centers.**
- The **pandemic** has posed an **added difficulty** for research, due to mobility restrictions and the special vulnerability of patients with RDs.
- Research in this field is **urgently needed.**
- **There is an increasing commitment on the part of patients,** researchers, industry and society as a whole to advance the study and treatment of RDs.

SPAIN REACHED AN ALL-TIME HIGH FOR CLINICAL TRIALS IN RARE DISEASES IN 2020

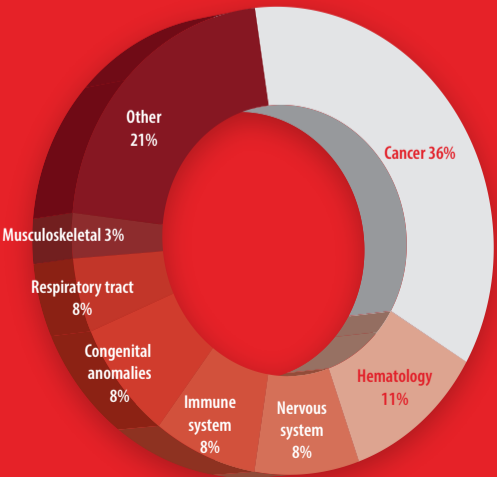
- 🇪🇸 In 2020, **1,018 clinical trials** involving more than **80,000 patients** were authorized in Spain.
- 🇪🇸 This represents a **23% increase** in the number of trials and an 87% increase in the number of patients compared to 2019.
- 🇪🇸 In the field of RDs Spain reached an all-time high of **194 trials** (5.5% more than in 2019), involving **3,191 patients.**

CLINICAL TRIALS IN RDs BY THERAPEUTIC AREA 2020



2 OUT OF 10 CLINICAL TRIALS CONDUCTED IN THE LAST 5 YEARS IN OUR COUNTRY INVESTIGATED MINORITY DISEASES

CLINICAL TRIALS IN RDs BY THERAPEUTIC AREA 2016-2020



In the period from 2016 to 2020, the following were carried out in Spain:

- 🔍 **4,216 clinical trials**, in which 264,577 patients have participated.
- 🔍 In the field of RDs, **827 trials, 20% of the total**, in which more than 16,253 patients participated, 6% of the total.