Cerba Research

Transforming research, advancing health together.

Unlocking The Potential Of Rare Disease Drug Development

Discover the extraordinary world of rare diseases, where uniqueness defines the landscape. While these conditions may lack a singular definition, they encompass a myriad of fascinating conditions waiting to be understood and treated. With over 5,500 rare diseases identified by the World Health Organization, each one presents a unique challenge and opportunity for innovation. Unlock the potential of orphan drug designation, offering a beacon of hope for those affected by rare diseases. Join us in the pursuit of pioneering solutions and transforming the lives of those touched by rarity.

Our Rare Disease Highlights

36
Approved rare disease medicines across therapy areas

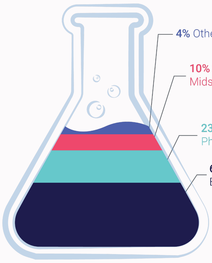
~80%
Trials including specialty testing

45,000+
Patients screened

190+
Rare disorder trials since 2018

39,900+
Patients randomized

Sponsor Type



Clinical Trial Phases Overview

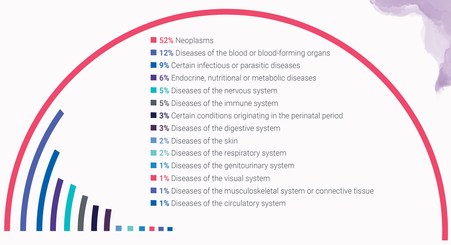
Phase I/NC **19%**

Phase II **41%**

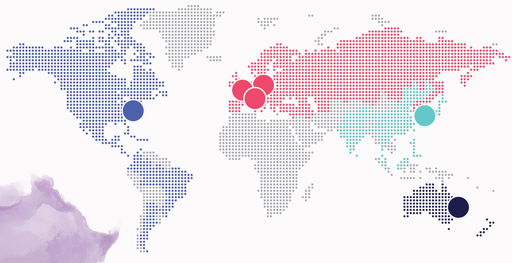
Phase III **38%**

Phase IV **2%**

Rare Diseases Trials By ICD 11 Classification

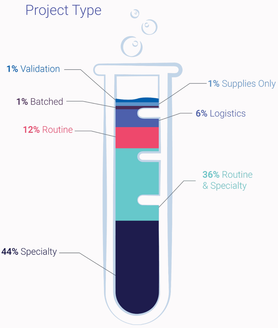


Countries Overview



A rare disease drug development company used Cerba Research's extensive laboratory network for their newly approved neurology drug.

Project Type



State-of-the-art Technologies

Cerba Research Is Uniquely Positioned With A Vast Array Of Laboratory Solutions For Your Rare Disorder Trial

1 ELISA-based Assays

Validated assays to detect antibodies against AAV vectors and target inserts using antibody-ELISA assays. Immune response by monitoring cytokines via MSD platform (CRS panel)... 50+ELISA-based assays.

2 Flow Cytometry

BD FACS Canto & Lyric, Cytek Aurora for MRD detection, enumeration, immunophenotyping, cell sorting & isolation, tumor microenvironment, intracellular cytokine (ICS) detection...

3 Cell Isolations

PBMC, BMMC, CD138+,...PBMC processing in 25+ countries with 45+ processing labs and growing...

4 Genetics & Genomics

Next-generation sequencing with FFPEs and liquid biopsies (e.g. ctDNA). Custom panels and already existing broad-panel assays for rare cancers / rare disorders.

5 Molecular Biology For CGTs

Detecting and quantifiying genes/viral genome expression (RCL, VCN).

6 Histopathology

FISH, NanoString® & IHC with 250+ IHC protocols available for analysis.

7 Safety / Routine

Cerba Research can perform a wide range of safety testings, such as, but not limited to coagulation, hematology, biochemistry, urinalysis, serology...

8 Immunogenicity

Cerba Research has the ability to support drug development from pre-clinical to post-approval with immunogenicity assays for your biologics, biosimilars & CGTs.

Acronyms

AAV: Adeno-associated viruses, **BMMC:** Bone marrow mononuclear cells, **CGT:** Cell and gene therapy, **CRS:** Cytokine release syndrome, **FFPE:** Formalin-fixed paraffin-embedded, **FISH:** Fluorescence *in situ* hybridization, **ICD:** International classification of disease, **IHC:** Immunohistochemistry, **MRD:** Minimal residual disease, **MSD:** Mesoscale discovery, **NPO:** Non-profit organization, **NC:** Not confirmed, **PBMC:** Peripheral blood mononuclear cells, **RCL:** Replication competent lentiviruses, **VCN:** Vector copy number.