



Pilot Center

The project currently consists of a base of 7 pilot centres:

1. Netherlands – UMC Utrecht / SEIN – Boudewijn Gunning
2. Belgium – University Hospital Antwerp – Berten Ceulemans – Lieven Lagae
3. Denmark – Filadelfia – Rikke Steensbjerre Møller
4. UK – University College London – Institute of Child Health – Clinical Neurosciences – Helen Cross
5. France – Hôpital Necker-Enfants Malades – Service de neurologie – Epilepsies rares – CHU Paris – Rima Nabbout
6. UK – Royal Hospital for Children – Glasgow – Andraes Brunklaus
7. Italy – Coordination RESIDRAS – Renzo Guerrini and Francesca Darra

Other centres wishing to participate would be welcome.

Coordinating Comitee

PROJECT COORDINATOR

Prof. Bernardo Dalla Bernardina – CREP

SCIENTIFIC STEERING COMMITTEE

Prof. Renzo Guerrini and Prof. Francesca Darra on behalf of Residras Scientific Committee – Italy

Prof. Rima Nabbout – France

Prof. Helen Cross – UK

Dr. Andraes Brunklaus – UK

Dr. Rikke Steensbjerre Møller – Denmark

Prof. Lieven Lagae – Belgium

Dr. Boudewijn Gunning – Netherlands

DATA AND PRIVACY MANAGER

Fondazione Gabriele Monasterio – Luciano Ciucci

REGISTER MANAGER FOR PRIVACY AND DATA

Fondazione Toscana G. Monasterio: Dr. Fabrizio Bianchi – Manager for Tuscany Register of Rare Diseases and Tuscany Register for Congenital Defects

PROJECT CREATOR

DRAVET ITALIA ONLUS: Isabella Brambilla

REFERENTS DATA ANALYSIS

Dr. Gaetano Cantalupo and Dr. Federica Pieroni



Partners



Partners patient associations



Info

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In collaboration with



International Registry of Dravet Syndrome
and other Syndromes correlated
with genes mutations on SCN1A and PCDH19



www.platform-residras.com





What is the Platform-Residras Registry?

PLATFORM-RESIDRAS is a registry of Dravet Syndrome and other syndromes correlated with Gene Mutations SCN1A and PCDH19.

The main aim of the registry is to analyse the data under the following **10 headings**: Biographical Data; Genetic Investigations; Family History; Personal History; Onset of Epileptic seizures, Seizure Follow-up; Neurological and Cognitive Follow-up; Treatment; Adverse events; Gait Analysis. Each of these headings is composed of a number of variables, obligatory and optional, to be completed.



Why a Registry?

The **Registry** represents an important instrument to boost the development of national and international scientific collaboration on rare diseases and orphan drugs. To record the number of patients per pathology and their distribution, to consent to furnish precise data to organise the health services and, for researchers, it represents the possibility of conducting specific studies on pathogenesis and eventually on the development of new therapies.

A **Registry** can promote scientific research.

Only a complete and precise data set will generate new correlations and studies that could better define the parameters and the research projects needed for better patient care.

Developing a unique platform and creating a working partnership between European and non-European countries is fundamental in promoting coordinated research on an international level and to reach critical masses given the rarity of the diseases.



How to join the Registry?

IF YOU ARE A FAMILY

1. Find a doctor/specialist who would be willing to join the project, at www.platform-residras.com.
2. The family must sign a consensus with their own doctor.

IF YOU ARE AN ASSOCIATION

1. Find a doctor/specialist who would be willing to join the project, at www.platform-residras.com.
2. Work with the Association and your centre to support the project and provide information to the family.
3. The Association will support the cost of providing data or maintenance of software.

IF YOU ARE A DOCTOR

1. Request admission by submitting a request via the website on the following page: www.registry.platform-residras.com/registry/account_request/
2. Once admitted, send all documents for protocol to your own Ethical Committee (agreement, information, and consensus for the family translated into your own language)
3. Send the approval of your Ethical Committee to Platform-Residras to receive your login credentials.
4. Obtain the signed consent of the parents/legal guardians and then complete the Registry with the patient's personal information and medical history and commit to at least one follow up per year.



Access to the data

1. Each Centre will have access to solely their **own data**, and will on request be permitted to carry out research and analysis on the Registry's data set.
2. In the event that a Centre wishes to carry out analysis on the entire population of **Platform-Residras**, the request shall be made to the **Coordinating Committee** who will respond within a maximum of five weeks.
3. Further statistical analysis can be carried out by 'Fondazione Toscana Gabriele Monasterio', Pisa, subject to a commission.
4. External parties are permitted to request data from the Registry, the request shall be made to the Coordinating Committee who will respond within a maximum of five weeks.
info@platform-residras.com



The "history" of the Registry

The Registry is sponsored by Dravet Italia Onlus (www.dravetitalia.org) based on the experience of RESIDRAS, the corresponding Italian registry (www.residras.com).

The registry was created based on the work of the Scientific Medical Committee (www.residras.com/comitato-medico-scientifico.html) and of the 'Fondazione Toscana Gabriele Monasterio', Pisa, who deal with the technical management of the Registry.

PLATFORM-RESIDRAS was created following the success of the Italian experience, and in response to the interest in participation demonstrated by other European Centres.