



**French Foundation for rare diseases**

**A national coordinating Hub for RD  
research in France**

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# A new cooperation model to boost research on rare diseases

**Flagship initiative** of the 2nd French National Rare Diseases Plan

**5 Founding members:** unique cooperation of patients' organisations, research institutes and clinical centres



A **non-profit private** structure **to coordinate, federate and fund rare diseases research** in order to accelerate scientific, medical and social innovations

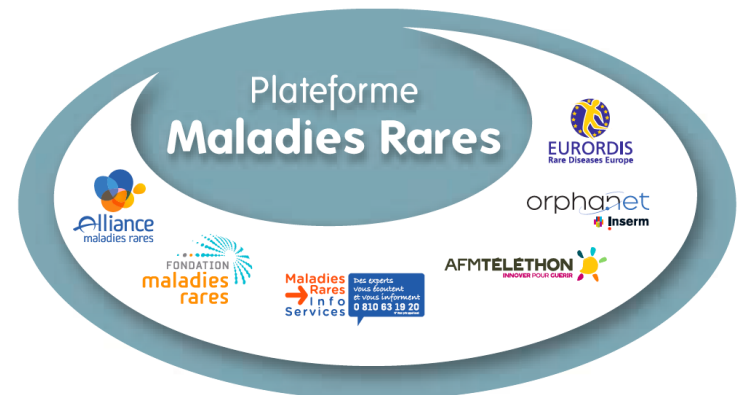
Encompassing research **on all rare diseases, from life sciences to social sciences and humanities**

**Board of Directors:**

5 Founding members + 10 Experts on RD

Scientific Strategy endorsed by a **Scientific Board** made of 30 highly renowned international scientists

**Operational team** located both at **Headquarters in Paris**, at the heart of the National RD Platform and all over France with **7 regional field coordinators** in direct contact with academic and clinical teams



# Our objective and activities

## BRIDGING ALL RESEARCH STAKEHOLDERS

### UNDERSTANDING rare diseases

- To Facilitate researchers' access to:
- Cutting-edge technology
  - Funding resources
  - Rare diseases R&D expertise



### DEVELOPING New treatments

- To speed up clinical innovation with:
- Early detection of drug candidates
  - Experts consulting
  - Public private partnerships

### SHARING At the international level

- Empower EU cooperation
- Support international developments (such as IRDiRC)
- Pilot project in developing regions



### IMPROVING Patients' care and lives

- To support societal advances through:
- Funding of dedicated research
  - National think tanks on key issues
  - Contribution to acquainted policies

# Results of a 3-year action

**106 projects funded** to unravel the molecular bases of rare diseases

## UNDERSTANDING Rare Diseases



**35 model organisms developed** to decipher pathological pathways and identify therapeutic options

**Good-practice guidelines and data sharing promoted**

A **R&D training** on rare diseases research developed with 5 partnering universities

**9** Calls for Proposals already handled



## DEVELOPING

### New treatments

Research funding programs to **identify new drug candidates** and to **improve therapeutic strategies**

**Detection and anticipation of the R&D needs** of innovative diagnostic and therapeutic approaches to **accelerate clinical development**

Thousands of molecules already tested for **6** diseases

**3** Calls for Proposals already closed

**+100** drug candidates identified, 58 of them supported towards clinical development

# Results of a 3-year action

**10** European projects supported in answering to the main funding research programme

Participation and alignment to **international developments**

Participation to international research projects as full partner bringing experience built in France

Support to an **international pilot project** with TWAS (research symposium gathering 8 Mediterranean bordering countries) in Sept. 2014



**IMPROVING  
Patients' care &  
lives**



**SHARING at  
international level**



Promote and fund research projects on the **impact of RD**

**National working groups** on key issues such as economic evaluation, information and protection of patients

**17 research projects funded** teaming up clinicians, patients organisations and experts of social sciences

**168** patients organisations involved

**3** calls for proposals completed



FRENCH FOUNDATION FOR  
**rare diseases**

# Our Sponsors and Partners



# PROMOTE RESEARCH ON RARE DISEASES

