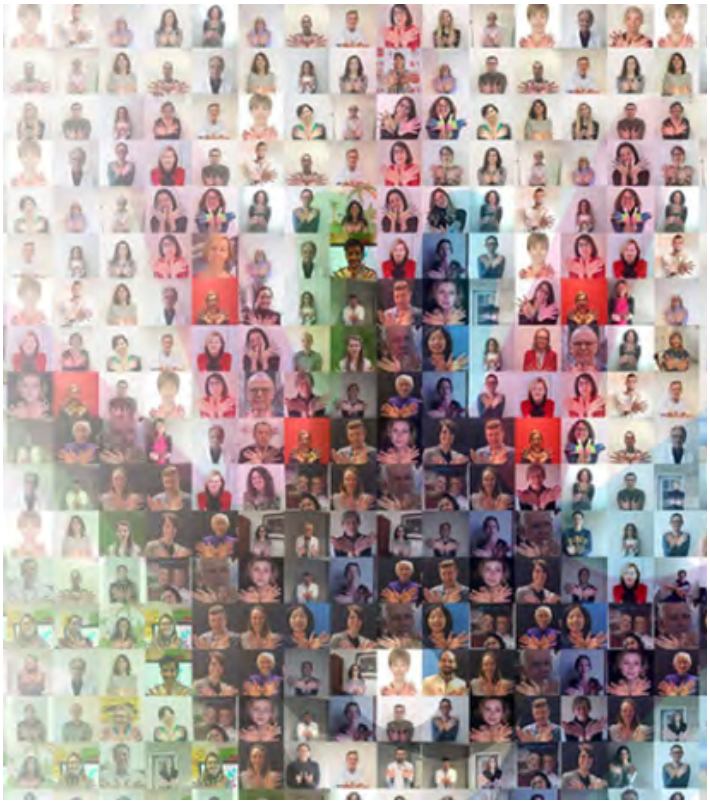
CVBF
Consorzio per Valutazioni Biologiche e Farmacologiche

CVBF ACTIVE INVOLVEMENT

IN THE EUROPEAN JOINT PROGRAMME RARE DISEASES

The **European Joint Programme on Rare Diseases** (**EJP RD**) is aiming to creating an **effective rare diseases research ecosystem** for progress, innovation, and benefit of everyone with a rare disease (RD).

To do so, the Programme supports RDs stakeholders by *funding research*, bringing together *data resources and tools*, providing dedicated *training courses*, and *translating high quality research into effective treatments*.



Since the beginning, *CVBF* has had an active role on several EJP RD work packages (WPs) focused on highly relevant issues around the RDs ecosystem.

CVBF is involved in many EJP RD Work Packages (WPs)

CVBF is involved in the **monitoring process** set within **WP1** aimed to evaluate the **quality of the EJP RD activities** and the achievement of the expected project goals during its whole duration. ***CVBF*** develops annual Monitoring reports based on the information gathered by the EJP RD partners.

CVBF worked on **Task 2.1 Prioritisation scheme** of the **WP2 *"Integrative research and innovation strategy"*** to develop Prioritization Guidelines based on criteria and procedures for decision-making processes on needs and actions directed to fulfil the EJP RD objectives.

CVBF is part of the **Advisory Regulatory Ethics Board (AREB)** within the **WP4 *"Ethical, regulatory, legal and Intellectual Property Rights (IPR) framework of the EJP RD"*** that oversees ethical issues and regulatory procedures foresaw by EJP RD and its partners. AREB ensures compliance with all international and European relevant rules as well as ethical norms, including but not limited to fundamental and patients' rights, data management, data protection and confidentiality, related to the projects founded within EJP RD and EJP RD itself.

WP16 - Massive Open Online Course (MOOC) on "Innovative Therapies and Personalized Medicine: New keys for the treatment of rare diseases"

CVBF is developing the course in collaboration with the *French Foundation for Rare Diseases* and *ERN Transplant Child*.

This **MOOC-2 course** provides an overview on the different innovative therapeutic approaches developed in the context of RDs as well as catches on the ethical, economic, and legal challenges related to their implementation.

CVBF is responsible for the preparation of the "*Personalised/precision medicine*" (week 4) and the *"Marketing and legal challenges related to the implementation of innovative and personalized therapies"* (week 5) sessions.

WP17 - "Modelling & Simulation (M&S): Research Methodologies for Small Populations in Rare Diseases"

This **M&S workshop** has been developed by ***CVBF*** in collaboration with the *University of Bari 'Aldo Moro'* and *University College of London*. It aimed at increasing the global scientific and technological capacity and it was held in **Bari, Italy on 4-5 July 2022**.

This workshop facilitated the knowledge discussion and exchange on the *M&S methodologies* as well as *strategies* to face complex and multifactorial conditions or RDs requiring highly specialised treatments and resources such as for small populations.

WP19 - "Modelling & Simulation (M&S): Research Methodologies for Small Populations in Rare Diseases"

CVBF has brought paediatric competences and expertise within the **WP19** to support the translation of *high quality research* into effective interventions that trigger valuable impacts on the RDs patient community. The aim is to create a bridge between *basic research* and *medical innovation*, also known as the "*bench to bed-side*" approach.

To this end, ***CVBF*** together with its WP19 Partners developed the ***Innovation Management Toolbox***, a *virtual library* that provides self-help resources in RD translational medicine *openely accessible* to the whole RD research community.