

## ADOPT BBMRI-ERIC GRANT AGREEMENT NO. 676550

### DELIVERABLE REPORT

<b>Deliverable no</b>	7.4
<b>Deliverable Title</b>	Governance model for the sustainability of RD biobanks
<b>Contractual delivery month</b>	36
<b>Responsible Partner</b>	BBMRI-ERIC HQ
<b>Author(s)</b>	Erik Steinfelder, Anna-Liisa Bader, Luca Sangiorgi

## GOVERNANCE MODEL FOR THE SUSTAINABILITY OF RD BIOBANKS

### Executive Summary

As a service-oriented Research Infrastructure, BBMRI-ERIC develops several supporting tools to be made available for the entire biobank community, including the Rare Diseases community. The various services offered by BBMRI-ERIC will be continuously improved and expanded, while also creating additional awareness. The Common Service model seemed to be working for both IT and ELSI, whereas Quality Management had a more pragmatic approach.

At the start of the ADOPT BBMRI-ERIC project, the governance structure of Common Service ELSI and Common Service IT was considered a good model to use for Rare Diseases as well and was explored. New insights into the performance and effectiveness of the existing Common Services, however, showed that these make the straightforward need for support too complex and the administrative burden relatively high, thus wasting a significant amount of the already limited funding. Therefore it was decided to incorporate the RD activities into the overall program of BBMRI-ERIC and to secure a custodian construction for the already developed solutions and deliverables.

The possibility to collect and share where possible was already agreed upon in several European Rare Disease projects, all of which were funded during a limited time period. Adding the biobank activities of the Rare Disease community to the BBMRI-ERIC allowed us to increase the awareness, visibility and secure sustainability.



## Copyright notice



This work by Parties of the ADOPT BBMRI-ERIC Consortium is licensed under a Creative Commons Attribution 4.0 International License (<http://creativecommons.org/licenses/by/4.0/>).

## Document log

Issue	Date (yyyy-mm-dd)	Comment	Author/partner
D7.4	2019-03-01	Initial version of the report	Erik Steinfeld, Anna-Liisa Bader
D7.4_rev2	2019-11-06	Revised version of the report based on reviewer's comments	Erik Steinfeld, Caitlin Ahern

## 1. Background

Biological samples from rare disease (RD) patients are significant assets for research. They are important for usage in biomarker identification, preclinical studies, validation, and understanding disease pathology. In Europe, a disease is defined as “rare” when the incidence is fewer than 1 per 2,000 persons; currently there are more than 6,000 known RDs and more yet to be named. The availability of RD biological samples for research is paramount to the overarching goals of the International Rare Diseases Research Consortium (IRDiRC), in shortening the time to receive an accurate diagnosis and developing 1,000 new therapies for RD by 2027.

The development of a Common Service for Rare Diseases was foreseen under BBMRI-ERIC within the duration of ADOPT BBMRI-ERIC. Creating this would also have required a sustainable governance model, allowing RD biobanks that are currently operating well to continue their cooperation within BBMRI-ERIC.

The idea was to use the governance model of the existing Common Service model, including a Director(s) for the coordination of the activities, an executive board with representatives of biobanks, a network of biobanks and delegates of major RD projects responsible for setting annual objectives and policy in accordance with the BBMRI-ERIC work plan, and an advisory board including representatives of the different stakeholders (e.g., patients associations, industry, scientists).

To secure sustainability, several return-on-investment models were explored, using both core funding as well as project funding to maintain the services and research activities.



This project has received funding from the *European Union's Horizon 2020 research and innovation programme* under grant agreement No 676550.

## 2. Approach (methods)

BBMRI-ERIC had already developed a Common Service ELSI and Common Service IT to serve the needs of the community with specific questions and/or issues. The idea was to have the same approach for Rare Diseases and thus secure on one hand the opportunity to serve the community, but also ensure that that National Nodes are involved in the execution and management of the specific services.

Since the Quality services are organized in a different, more top-down approach, this model was also explored as a potential way forward.

Once a new governance structure was agreed on, the various deliverables from WP7 were intended to be integrated into the overall common services for Rare Diseases.

## 3. Results

By 2018, BBMRI-ERIC had established Common Service ELSI, Common Service IT and the Quality Management Service. The models of these services vary significantly, and BBMRI-ERIC has been seeking possibilities to make the services more “customer” oriented as well as more efficient. Therefore, an internal review of CS ELSI was performed in the summer of 2018. The review has also served as a basis to make a decision on the governance structure for the sustainability of Rare Diseases biobanks.

### Governance review of Common Service ELSI

It is clear that the BBMRI-ERIC CS ELSI has made considerable progress in a number of important areas since the CS was initially established. However, the current governance structure will need to be updated to maximize efficiency and effectiveness, or in other words, greater ‘professionalization’ of the CS ELSI governance structure.

It is suggested that the governance structure be streamlined considerably. The CS ELSI is currently governed by a team of 3 Directors and the Chief Policy Officer. The use of co-Directors might have served a particular purpose originally; however, it is not the most efficient and effective approach to governance and decision-making. A single director/manager, with expertise and experience in project management, should be identified and supported. While it is not essential that this individual be an expert in ELSI issues, some understanding of ELSI issues would also be important. The roles and responsibilities of everyone within the governance structure should be clearly articulated and reviewed to eliminate unnecessary overlap.

This clarification of structure and functioning will also be beneficial for the National Nodes that often perceive the management as lacking transparency. The governance structure and decision-making processes should be clearly communicated to the ELSI experts and leadership of each National Node.

At the other end of the spectrum, the structure and heterogeneity of contracts for experts in different countries should be addressed in a creative way to ensure both a fair remuneration and a clearer responsibility, also becoming a more professional structure.



The reviewers also advised how to progress forward in ensuring better sustainability.

a) Additional revenue:

Along with an improved planning of resources, the path toward better sustainability could include the generation of additional revenue through paid services. It is our understanding that developing a strategy to generate revenue is a key activity for 2019, including collaborating with industry, but how such a strategy would be developed is not clear. During our discussions with experts, it appeared that these kinds of activities might constitute a sensitive issue. It was also not clear to them that these ‘economic’ activities were even allowed for BBMRI. A clarification of this issue would therefore be beneficial.

b) Creation of ‘Quality check’ by CS ELSI:

Beyond the present activities, the evaluation of the readiness of projects seeking funding or regulatory approval regarding their ELSI component could become a standalone activity useful for the biobankers and scientists on one side, and the funding and regulatory agencies on the other side. The benefit for the former group would be better preparation prior to submission. The benefit for the latter group would be a faster review time, because this important step of reviewing the ELSI component would have been checked ahead of time.

The conclusions of the ELSI review have been used for further discussions about BBMRI-ERIC services in general. For the effective use of resources – economic as well as personnel – at this point of time creating a new governance structure for rare diseases will not meet the requirements of the community.

Given the fact that the number of samples and associated data is rather limited in the Rare Diseases field, causing challenges in clinical trials and development of new treatments, BBMRI-ERIC sees the value of supporting the RD Community through coordinated, sustainably funded services. However, the waste of resources through unnecessary bureaucratic processes inherent in a Common Service could not be justified. Adding the biobank activities of the Rare Disease community to the BBMRI-ERIC could increase the awareness, visibility and secure sustainability more effectively and efficiently than by creating a Common Service for Rare Diseases.

## 4. Next Steps

Given the thorough analysis and broad consensus within the stakeholders of BBMRI-ERIC on a different approach it was decided not to set up a specific governance model on Rare Diseases but rather to incorporate this in the existing structure of BBMRI-ERIC.

Since 2019 Rare Diseases is now a separate activity within the Annual Work Programme of BBMRI-ERIC to guarantee that work is scheduled, resources allocated and results monitored. The main action for 2019 is that the RD-Connect Registry and Biobank Finder were integrated to allow flow of information. A study is needed to see if the tool and current RD biobank area of the BBMRI-ERIC Directory can be merged or complemented – this is planned for late 2019. In preparation of these actions, discussions have been ongoing since September 2018 with leadership of longstanding RD biobank networks such as



EuroBioBank ([eurobiobank.org](http://eurobiobank.org)) and Telethon Network of Genetic Biobanks (TNGB; <http://biobanknetwork.telethon.it>) on the best way to serve the RD community together via the existing structures of BBMRI-ERIC. This Work Programme and the needed budget were approved during the AoM#12 meeting in Vienna. For the long term, a specific part of the strategy for the years ahead concerning Rare Diseases has been developed within our Vision Paper.

On a more practical level the coordination of RD activities is suggested in the diagram below and will be used for continued discussions to facilitate Access, Quality and Engagement under central coordination.

Figure 1: Central Coordination for a Rare Diseases chapter, similar as those of IT, QM and ELSI

