



**Kick-off Meeting INEA Project
Hannover
2 December 2019 – 8.30-16.00**

Agenda		
8.30-8.45	Introduction of background and objectives of INEA Project	Moderators: Maurizio Scarpa
8.45 -9.15	Background presentation Activity 1: Implementation of new data sets for disease-specific adaptation of the ERN Clinical Patient Management System (CPMS) and implementation of measures to improve existing data set(s) quality and data validation	Klaus Mohnike
9.15-10.00	Background presentation Activity 2: To develop and implement case-oriented e-learning/e-training within MetabERN Including a short presentation on the choice of platforms	Terry Derks Fabian
10.00-10.30	Background presentation Activity 3: To generate interconnectivity in the entire care chain between IEM-patients, primary care physicians, local hospitals and the centres of expertise to prevent metabolic emergencies.	Terry Derks Bas Irene
10.30-10.40	Short coffee break	
10.40-12.30	Break-out sessions for each Activity and setting of milestones <i>Outcome: reach consensus on steps to take to achieve the end result</i>	All
12.30-13.30	Lunch break	
13.30-14.30	Continuation of break-out sessions	All
14.30-15.30	Presentation of conclusions per Activity	Terry and Klaus
15.30-16.00	Wrap-up of conclusions and consensus	Maurizio
End of the meeting		

Background documents		
Synopsis of the project and the 3 Activities		
Emergency protocol		
Survey results		
Evaluation manuscript for protocol		
Minimal data sets		

Annex I-

	5.3 To evaluate methods for digitalized management + follow up of IMDs via e-health tools
WP-6 Research, transitional activities and clinical trials	
Assessing scientific innovation for IMDs	6.1 Identify partners for calls /submission to grants 6.2 Identify strategies to deliver macro-molecules to the brain 6.3 Produce White Paper on innovative Therapies 6.4 Define a Roadmap for new clinical trials 6.5 Encourage use of innovative approaches in the development of medicines
WP-7 Capacity building and training	
Increasing knowledge and skilling up competencies of target groups; supporting Member States to provide highly specialised care to IMD patients	7.1 To develop and implement a Therapeutic Education Programme (TEP) for patients 7.2 To develop HCP training and teaching 7.3 Support to underequipped centres 7.4 Expanding the scope of MetabERN
WP-8 Continuity of Care	
Improving prevention, diagnosis and care in the disease areas covered by the Network	8.1 To define common strategy on quality assurance (QA) schemes (with ERNDIM and Orphanet) 8.2 To set up a framework for minimum requirements IMD new born screening 8.3 To define a comprehensive overview of measures for pre-implantation genetic diagnosis (PGD) 8.4 To create adult metabolic centres (transition)
WP-9 Patient Empowerment	
Improving patient-centred health-care, patients' involvement and integration of patients in our Network	9.1 Making recommendations for patient-centred healthcare including in clinical trials and broader industrial strategies 9.2 Implementation of patient involvement and integration in SNW and WP

Activity/Nr	Year 3				Year 4				Year 5				Linked deliverable(s)	PM
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4		
1. Coordination and Management														
1.1 Set up MetabERN communication touch points													MILESTONES Quarterly meetings (in person or on the phone)	1,5
1.2 Ensure active participation of MetabERN members													1.2.1 Develop and implement a project management strategy	0,5
1.3 Interact with other ERNs for overlapping diseases to create synergies and avoid duplications													MILESTONE Set-up meetings with coordinators of overlapping ERNs (TC or in person meeting)	0,5
1.4. Organisation of Boards Meetings													MILESTONE Organise Annual Board meeting	6
													MILESTONE Organise interim meeting at SSIEM	1,5
1.5 Track progress in Metab-ERN objectives													1.5.1 Develop quarterly monitoring and annual report (3 annual reports)	3,5
2. Dissemination														
2.1 Implementation of dissemination plan													2.1.1 Revision of the communication plan with implementation of the plans for dissemination for the next 3-years	1,5
													2.1.2 Content for website	3
													2.1.3 Publication of monthly	2,5

Activity/Nr	Year 3				Year 4				Year 5				Linked deliverable(s)	PM
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4		
													newsletter	
													2.1.4 Publication in journals/media	3
													MILESTONE Congress attendance	2
2.2 Policy-makers awareness													MILESTONE EMA/EP meeting on access to care	2
													2.2.1 European Parliamentary Meeting to present '5 Years of MetabERN' achievements'	3
2.3 Patient and family involvement													MILESTONE Organization of an European Metabolic Day	3
													MILESTONE Organise special sessions for patient organisations during MetabERN board meetings	1
													2.3.1 Organise expert training course for patient advocates in collaboration with European Patients' Academy (EUPATI)	1
2.4 Dissemination to medical audiences													2.4.1 Publication in relevant peer reviewed medical journals	3
2.5 Tailored information to patients													2.5.1 Publication of patient editorial articles in the MetabERN newsletters and content for the patient website	1,5
3. Evaluation														
3.1 Evaluate progress report on MetabERN													3.1.1 Evaluate three monthly progress reports	3

Activity/Nr	Year 3				Year 4				Year 5				Linked deliverable(s)	PM
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4		
activities, HCP activities and implement feedback														
3.2 Measure HCP satisfaction													3.2.1 Design and conduct semi-structured interview (guide) and online survey	3
3.3 Measure patient satisfaction													3.3.1 Design and run online survey	2
4. Guidelines, Care Pathways and Standardization for Medical Care and Transition Pathways														
4.1 Development a transition programme													4.1.1 Consensus paper on the status of the transition programmes in metabolic patients	3
4.2 Guidelines/ Care Pathways evaluation													4.2.1 Evaluate the pathways/GL by sending out a survey among users	3
4.3 To identify and disseminate best practices in overlapping ERN diseases through ERN-CG													4.3.1 To develop a consensus paper through multi-stakeholder consultations (ERNs/RD Connect/EC) on rare disease best practices. The focus of the paper on overlapping diseases	3
5. Virtual Counselling														
5.1 To foster and facilitate appropriate use of CPMS													5.1.1 Set-up an Operational Helpdesk for the organization and performance of CPMS consultation	2,5
5.2 Implement recommendations on cross-border multidisciplinary team													5.2.1 Organise e-Delphi process and online consensus meeting in order to establish recommendations → to be	3

Activity/Nr	Year 3				Year 4				Year 5				Linked deliverable(s)	PM
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4		
													included in consensus paper	
5.3 To evaluate methods for digitalized management + follow up of IMDs via e-health tools													5.3.1 Make an overview and evaluate existing E-health disease management tools and analyse the use for IMDs within MetabERN	3
6. Research, Translational Activities and Clinical Trials														
6.1 Identify partners for calls /submission to grants													6.1.1 Identification and submission of proposals for relevant grants programmes	3
6.2 Identify strategies to deliver macro-molecules to the brain													6.2.1 Position paper written by an expert group with recommendations for financial support	3
6.3 White Paper on innovative Therapies													6.3.1 Collect clinical research performed in the network	2
													6.3.2 Use of U-IMD for the description of disease natural history	2
													6.3.3 Economic models of innovative therapies for IMDs	3
													6.3.4 White Paper on state of the art of innovative therapies for IMDs	4,5
6.4 Roadmap for new clinical trials													6.4.1 Establishment of a working group on new clinical trial designs in collaboration with the EJP-RD project	2,5

Activity/Nr	Year 3				Year 4				Year 5				Linked deliverable(s)	PM
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4		
6.5 Encourage use of innovative approaches in medicines development													6.5.1 Joint FDA - EMA event to expand current strategic collaborative approach on Gaucher disease to other rare metabolic diseases	3
7. Education and Capacity-building														
7.1 Therapeutic Education Programme (TEP) for patients													7.1.1 Reach consensus on outcome domains for TEP by using Delphi method	2
													7.1.2 Identify providers of training (stakeholders, training institutions, HCPs etc.) and design of TEP	1
													7.1.3 Implement TEP	3
7.2 HCP training and teaching													7.2.1 Draft a White paper "Adequately meeting the needs of patients: best practices, recommendations, minimum requirements /parameters and outcome domains in setting up of the specialised teaching and training programmes"	3,5
													7.2.2 Develop strategy for an education programme on rare diseases in medical professionals CVs	2
													7.2.3 Identify providers of training (stakeholders, training institutions, HCPs etc.) and design certified training programme	1

Activity/Nr	Year 3				Year 4				Year 5				Linked deliverable(s)	PM
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4		
7.3 Support to underequipped centres													7.3.1 Mapping of availability of training and teaching programmes in the underequipped centres	0,5
													7.3.2 Dissemination of best practice programmes to the under-equipped centres	1,5
7.4 Expanding scope of MetabERN													7.4.1 Develop exchange programme between MetabERN centres & non-member countries	1
													7.4.2 Implementation of the exchange programme	1,5
8. Continuity of Care														
8.1 Common strategy on quality assurance schemes													8.1.1 Find consensus on diagnostics Quality Assurance schemes in collaboration with ERNDIM and Orphanet	1,5
													MILESTONE Roll out of the schemes amongst the laboratories used by MetabERN HCPs	0,5
8.2 Framework for minimum requirements IMD new born screening													8.2.1 Define the minimum set of IMDs to be screened	1,5
													8.2.2 Create an overview of currently available and offered tests by country & numbers screened	1
8.3 Comprehensive overview of measures for prenatal													8.3.1 Mapping of currently available PND/PGD by surveys and internet searches	1,5

Activity/Nr	Year 3				Year 4				Year 5				Linked deliverable(s)	PM
	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4	Q1	Q2	Q3	Q4		
diagnostics														
8.4 Adult metabolic centres (transition)													8.4.1 Mapping of currently available expertise of diagnostics for adults by surveys & internet searches	2
9. Patient involvement														
9.1 Recommendations for patient-centred healthcare including in clinical trials and broader industrial strategies													MILESTONE Finding consensus on general recommendations for patient-centred healthcare in the field of IMDs by means of study of behaviour of HCPs and patients	2
													9.1.1 Writing a White Paper with recommendations on ensuring/ improving patient-centred healthcare (taking into account the environment, community, cultural differences and encourage patient participation (link with target 6.4.1 and 7.2.1)	3
9.2 Implementation of patient involvement and integration per WP and SNW													9.2.1 Identification of the areas per WP and SNW for patient integration in the network.	1
													9.2.2 Implementation and dissemination of models of patients engagement (recommendations) for patient involvement per WP and SNW	1
Total persons per month														121



European
Reference
Network

MetabERN

European Reference Network
for Hereditary Metabolic Disorders

Medical Executive Board - Rules of Procedure

Frequency of meetings

The Medical Executive Board meets every 2 months virtually via web to plan and follow up on the MetabERN activities. It also meets in person twice a year in Brussels (in the regional office of the Coordinators HCP (Udine)).

Membership

The Medical Executive Board (MEB) is part of the Governance structure of MetabERN and it consists of the network Coordinator, the two vice-Coordinators, one elected representative from the Subnetwork Coordinators and the Work Package leaders as well as the Chair and Chair of the Patient Board (or a representative from the Patient Steering Committee).

There is flexibility in which representatives is member of the MEB depending on availability. If there is a need for a formal election procedure, this can be set up at a later stage.

Chairing of the Medical Executive Board

The chairing of the Medical Executive Board will take place on the basis of rotation; the next chair is selected during the meetings. The chair will have the support of the Coordination Office for the organisation of the meeting.

Responsibilities

The MEB is responsible for:

- Pointing out and overseeing the strategic direction of the network;
- Setting of priorities for the coming years and overseeing the activities and progress of MetabERN as a whole in relation to the 3-5 year plan;
- Working closely with the General Board of Healthcare Providers to ensure any decisions made are implemented fully and subsequently promoted to ensure maximum effectiveness;
- The potential future establishment of contacts with third parties, industry, regulatory agencies and the development of a viable sustainability strategy for the network.
- The drawing up and approving of MetabERN governance documents such as the Non-compliance procedure etc.

The MEB also has insight in the budget of MetabERN and the grants that have been appointed to MetabERN.

Agenda

The Coordination Office shall draw up a provisional agenda which will be circulated two weeks before the meeting. Any proposal for an agenda item shall be sent to the Coordination Office at least 5 days before the meeting.

Decisions

Decisions are taken in the MEB by a majority of votes. The decisions taken are communicated to all MetabERN members.

Annex III- Non Compliance Procedure



MetabERN Governance Document

March 2019

Noncompliance of HealthCare Providers

1. In order to become, and remain, part of ERN ~~MetabERN~~, HealthCare Providers (HCPs) are expected to meet certain obligations.
2. These, in summary, are:
 - That they maintain relevant expertise in their Centre across those areas specified in the original application they made to become a member of the ~~MetabERN~~ Network.
 - That expertise is utilised for the benefit of the network by taking an active part in the work of at least one ~~Subnetworks~~ and one Work Packages, CPMS Panels, virtual and physical meetings, requests for information and general reporting.
3. Where the ERN ~~MetabERN~~ Management Team have reason to believe that an HCP within its network no longer complies with the obligations set out above, the procedures set out below will apply.

No evidence of engagement by the HealthCare Provider

4. As part of the monitoring process of the ERN, we shall be regularly checking that
 - a. Every HCP has at least one active CPMS User covering each of its nominated ~~Workstreams~~, where 'active' is defined as a registered user that has accessed the system within the last three months. Furthermore, even though there may be an active user for the Centre, we shall also be monitoring engagement with CPMS panel invites, CPMS meeting invites and attendance, and ~~MetabERN Workstream~~ meetings.
 - b. Every HCP is actively participating in the work of the SNWs and WPs and contributes to meetings and deliverables/projects, attending Teleconferences on a regular basis, replying to general requests of information (in the form of data collection on number of patients, surveys or reporting requests from either the Coordination Office or the respective SNW and/or WP leaders.
5. Where we find that an HCP is overall not an active and collaborative member of ~~MetabERN~~, the Coordinator shall contact the HCP in question via email, detailing their

concerns and trying to determine through this engagement whether there is an issue preventing the HCP from meeting the aforementioned minimum requirements. Where an issue is identified that has prevented the HCP from meeting the requirements, the Coordination Office shall work with the centre to determine how to overcome this, referring the issue to the appropriate colleagues within the network where necessary.

6. If no adequate response is received within three weeks of this initial contact, the issue will be escalated to the MetabERN centre manager, who will email the Centre and ask for an explanation for the current situation, whilst offering help and support of the MetabERN coordination team.
7. However, if no adequate response is received within three weeks of this contact, the issue will be escalated as set out in the next section.

Wider evidence of noncompliance or escalation of an existing issue

8. If an identified issue of noncompliance is escalated from the previous section, or if ERN MetabERN is officially informed in writing that one of the HCPs within its network no longer complies with the legal or operational criteria, as defined by the European Commission (e.g. there is strong evidence to suggest that expertise no longer exists within the HCP as was originally guaranteed in their application), the following procedures will apply:
 - ERN MetabERN Coordinator writes to the HCP Chief Executive to ask for clarification of the issue (offer of videoconference to discuss any issues is included in the letter).
 - If the issues cannot be resolved by the HCP providing the required information/evidence of compliance, the ERN Coordinator will write to the HCP CEO, copying the letter to DG SANTE, requesting an action plan within 8 weeks and a timetable to demonstrate how the compliance issues will be resolved within the next six months (although it is understood that if a particular expert moves employment to a different HCP or a clinician retires, that a reasonable amount of time might be needed to recruit and appoint a new expert).
 - If the HCP does not intend to maintain the levels of expertise in disease areas for which it was assessed during the 2016 application to join ERN MetabERN, the HCP must inform the Coordinator in writing, with a copy of the letter to DG SANTE and their member of the Board of Member States for ERNs.
 - The Coordinator and the Vice-Coordinators will then decide to terminate the membership agreement with the HCP.