Roundtable on Centres of Expertise for Rare Diseases

Report of a multistakeholder discussion

11 October 2019

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1. Introduction

RaDiOrg – Rare Diseases Organisation Belgium is the Belgian umbrella organisation for 86 member organisations and around 400 individual members for whom no disease specific organisation exists. We were involved when the recommendations for the Belgian Plan Rare Diseases were developed. These recommendations led to the National Plan for Rare Diseases at the end of 2013.
Since then RaDiOrg has keenly been following up on the way the plan is being implemented. In the second domain of the plan (optimization of care) action 11 'New Centres of Expertise' has always been a prime focus of attention for us.
A lot has been achieved to improve attention and care for rare diseases since 2013, but no new Centres of Expertise have been formally identified or designated.

Because the relevance of concentrating expertise for rare diseases is commonly accepted by many stakeholders, but there is no plan to take steps towards the formal organisation of rare disease centres of expertise, RaDiOrg has taken the initiative to bring together relevant stakeholders to discuss the subject. The objectives of the afternoon were defined as:

- *Learning* about **Centres of Expertise (CoE) and the National Accreditation process for these CoE** from examples in other countries
- *Exploring* the **Belgian Rare Diseases Context -** strengths and weaknesses
- *Identifying* possible **actions** towards expertise centres in Belgium.

There was an immediate and enthousiastic willingness of all invited to participate. We sincerely thank each and every one of the people who contributed. The content and succes of the event and of this report are entirely thanks to their effort.

RaDiOrg hopes this report is a good reflection of the thoughts shared on Octobre 11th and that it can provide incentives to take next steps to formally identify and designate Centres of Expertise in Belgium.
One of the most common question the Belgian helpline for rare disease receives more than any other, is "where do I find the best expertise for my rare disease?". Establishing a national accreditation for the indentification and designation of Belgian CoE would address a clear and present need of patients and professionals. It would enable them to access the right expertise at the right time, which will undeniably improve the health outcome and quality of life of rare disease patients.

2. Brief introduction to the Belgian Context. The patient perspective.

This text reflects the content of the presentation made by Eva Schoeters, coordinator at RaDiOrg, introducing the patient perspective to the theme of the afternoon.

Let's start by looking into Belgium's **National Plan Rare Diseases**. This was published in december 2013. It contains 20 actions spread over 4 domains: *1. Diagnosis and information to the patient - 2. Optimization of care - 3. Information management - 4. Governance and sustainability.*
Expertise centres are mentioned in the plan 74 times in total, 4 of which in the title of one of the 20 actions.
Action 11 is titled 'New Centres of Expertise' and it describes how these will be created following an inventarisation of available expertise and the needs identified.
Despite all of this, no new centres of expertise were identified or designated since 2013. The reference centres that pre-existed the publication of the plan have not been transformed into centres of expertise.

For 6 rare diseases, or groups of rare diseases, Belgium has organised **reference centres.** These had been established long before the publication of the Belgian Plan Rare Diseases. The reference centres cover cystic fibrosis (7 centres), metabolic diseases (8 centres), neuro-muscular diseases (7 centres), refractory epilepsy (4 centres), hemophilia (5 centres, one of which has the lead) and Spina Bifida (3 centres). The centres work with a convention, which is an agreement with the National Institute for Health and Disability Insurance. Through this agreement an adapted package of care is financed which ensures patients get multidisciplinary care by dedicated teams of experts, automatically also including psychological care.
For the wide variety of diseases that are covered by the centres for metabolic and neuro-muscular diseases, no distinction in expertise is made between the centres. This means that for a disease like f.i. Hurler Syndrome (MPS1H), of which an estimate of 55 patients can be found in Belgium, these could be spread out over 8 centres, some having maybe as little as 5 patients.
The outcome for patients is not equal between the centres. A patient with a clearly neuro-muscular condition that was followed for over 7 years in centre A, finally got a definite diagnosis 3 months after once having visited centre B. Admittedly, the health outcome for the patient in question did not differ greatly, but her data were captured and will now be able to contribute to further evolutions in knowledge and hope for all affected by the same condition. And she has been given the possibility of finding and exchanging with fellow-sufferers.
Medical errors, specific to the disease for which the reference centres are designated, are regularly reported to us. These concern errors in prescribed medication, in timing of surgical interventions and in care coordination. The most worrying example being that of a patient that died due an epileptic seizure during dialysis. The patient in question suffered from a disease well known and extensively studied, that is characterised by epilepsy as well as nephrological manifestations.
The assessment of the functioning of the reference centres is done without involving patients or patient organisations.

Since march 2017, **24 European Reference Networks (ERNs)** for rare and complex diseases have been established. With 6100 rare diseases identified, this means an average of 254 per network, organised in sub-groups per network.
Belgium is represented in 23 out of 24 ERNs. In some ERNs, for instance Endo-ERN, up to 6 Belgian hospitals are member. Comments on this (over?)representation have been made before by other stakeholders than ourselves.
From the patient perspective what clearly stands out is that the ERNs do not help to identify the route to the best national expertise available for a given disease. This being said, RaDiOrg does not

deny the very valuable cause that the ERNs contribute to: equal quality of care for all throughout Europe, living with a rare disease.

At the end of 2017 a **Flemish Network for Rare Diseases** has been created that aims at translating the work of the ERNs to the Flemish level and at establishing a regional network around disease groups that reflect the categorization of the ERN groups. The Flemish networks for disease groups (5 pilot groups are presently operational) connect specialists from university hospitals, from local hospitals, from organisations for the first line care as well as patient organisations. They each set their own priorities and goals, identified as being important by the group.
The networks are facilitated by the Flemish administration for healthcare but they receive no funding.
On the French speaking part of the country a request to facilitate a similar network by AVIQ has not met approval. A request for a subsidy for a coordinator for a **Rare Disease Network Wallonia-Brussels** has not met with approval either. Due to this, no network has been created on the French speaking side of the country. The involved partners do not meet anymore.
The minister for Health of the Walloon Community has however chosen to invest in a 0800-helpline for rare diseases, neglecting the fact that a national helpline run by RaDiOrg and endorsed by Orphanet Belgium already exists.
From the rare disease patient point of view it is hard to understand that on the one hand international collaboration is presented as *a sine que non* raising care for rare disease patients to an optimal level, while on the other hand Belgian rare disease initiatives (be it networks or a helpline) are being conceptualised at a regional level.
Another concern is that the focus on networks, European or regional, cannot replace the concentration of real life experience in one team in one place. Therefore patients are still hoping to see centres of expertise established to stimulate optimal quality for their low prevalence disease.

Since 2016 the **seven university hospitals, all with a centre for human genetics, have been designated as a 'Function Rare Diseases'**. From mid 2018 onwards they also receive funding. These 7 'Functions Rare Diseases' are, however, <u>not</u> centres of expertise. As the law states, they are supposed to provide sufficient expertise in all rare diseases so that patients who are referred to them can be

(1) actively helped to receive the right diagnosis,
(2) that they are referred to 'real' Centres of Expertise in Belgium (not identified at present) or abroad
(3) that those who are managed abroad can continue to stay in Belgium while receiving quality management as instructed by the foreign Centre of Expertise.

RaDiOrg notices however that optimal referral is a problem. Following from the 'self-declared' expertise that these 7 university hospitals claim, they often do not point out to their patients that a different centre might have specialists that are more experienced and involved in their specific disease.

All of this leaves patients and patient organisations with a lot of concerns:

- How does a patient find his way to a well organised multidisciplinary team for his rare disease? And how does he know if his clinician has 2, 20 or 200 other patients with his disease?
- How can a clinician really judge his own competence? Doesn't this ask for objective assessment or benchmarking?
- Patient organisations have limited resources, if they wants to collaborate with a hospital, to contribute to care and communication, to create synergies with clinicians, how to decide which centre to attach themselves to?

- If there is clear expertise abroad, and in Belgium all or none are expert, how can a patient justify going abroad for follow-up and care?
- How can a passionate clinician-researcher build a sustainable team with shared expertise if the centre cannot be recognised for its expertise?
- How can a rare disease patient rely on chances to be included in a clinical trial if it is not signposted where the trials for his disease are likely to happen?

To our mind experience is key to optimal outcome of clinical interventions, in the handling of complex diseases and in addressing the psychological implications of a specific rare disease appropriately. And experience asks for relevant practice in numbers.
Patients and patient organisations should be involved as essential partners in identifying and in assessing expertise.

6100 rare disease expertise centres are not a realistic goal, but RaDiOrg believes we should identify and support the expertise we have in Belgium, stimulate what we need and collaborate with centres abroad for what we lack. We see that true excellence is rare, that it should be made visible and that it should be cherished, stimulated and supported.

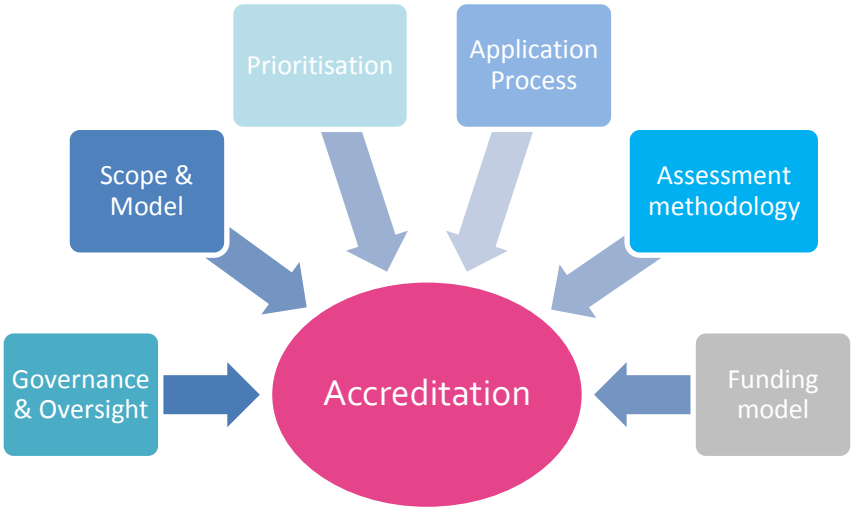
3. Rare Disease Centres of Expertise: National Examples of Accreditation Methods – Matt Bolz Johnson, ERN & Healthcare advisor EURORDIS

This text reflects the content of the presentation made by Matt Bolz-Johnson on October 11th as well as the comprehensive text he wrote on the subject, which you can access via this link. The text was written by Eva Schoeters and validated by Matt Bolz-Johnson.
Matt Bolz-Johnson has had firsthand experience as national commissioner in the U.K. responsible for the designation of National Centres of Expertise for ultra-rare diseases and highly specialised healthcare services. . He also lead the project team that developed the winning EC tender for the ERN assessment framework used in the first and second call for ERN and their Members applications.

How do you create expert centres knowing there are 6100 rare diseases. It seems logical to try to follow the grouping as done in the ERN's but at the level of expert centres, it seems essential to also capitalize on the expertise of centres that focus on single, ultra rare diseases or small subgroups. How can you find the balance between general criterria and disease-specific criteria to really effectively identify existing expertise and create added value and better quality through an assessment process?

If we look to identify the core elements of existing national accrediation and designation process, we can see the same common elements – governance, scope/model, prioritisation, application process, assessment methodology and defining a funding model.

The core elements for accreditation are identified as these:



Healthcare accreditation[1] is often considered a hurdle to cross at a specific point in time, with little value for clinicians and day-to-day services delivery. However, published literature positions healthcare accreditation schemes as 'continuous quality improvement schemes'. Ideally the accreditation framework is continuously developed, with maturing quality standards to meet, stretch

[1] The EC published a literature review on the different models and their evidence base for healthcare accreditation / licensing.
https://ec.europa.eu/health/sites/health/files/ern/docs/mapping_ern_literature_en.pdf

targets etc which are relevant to the day to day running of services and are focused on the outcomes of patient care.

Ideally an accreditation system will mature over time, with the most advanced frameworks benchmarking outcomes of surgery and care, for centres to be awarded continued designation. The evidence is that a multiple model for accreditation should be adapted. All assessment methods have different positive and negative in different aspects, so it is the use of triangulation of a multiple assessment methodology which will qualify results across a service and increase the accuracy of the assessment.

For example, an approach using, i. self-assessment, ii. external validation using documentation review and iii. onsite visits would optimize the accuracy of the assessment and ultimately drive improvements to service quality. Both patient involvement and focusing the assessment on patient care improves the relevance of any assessment.

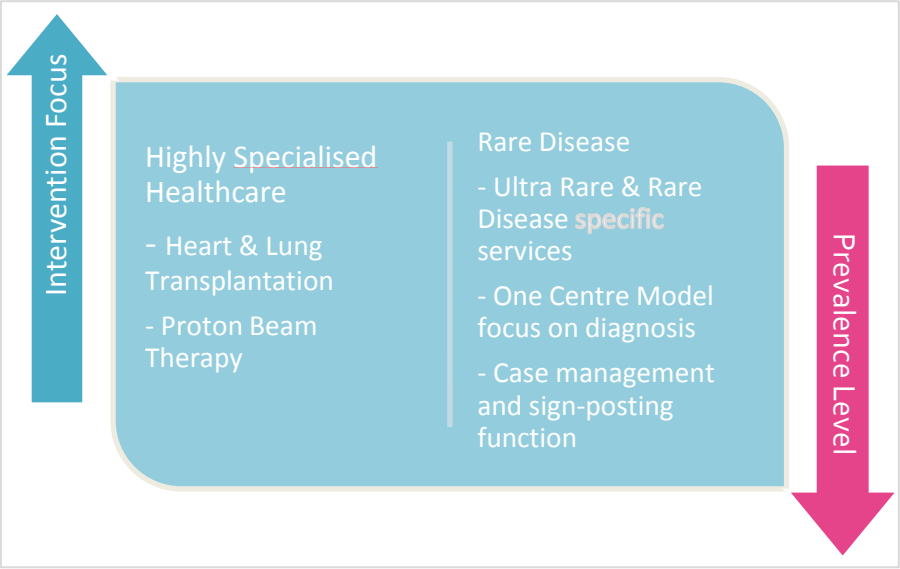
1. Governance & oversight

The governance of national accreditation / designation systems takes time and resources. Normally, MS have a national team who coordinate the designation / accreditation process and have an independent advisory group to make decisions e.g.: prioritisation and approval of applications. Most have representation from local 'health boards / regions' like in Spain, Sweden and clinical leadership from professional societies and patient representation (for transparency and good governance). Patient involvement in the governance and including their opinion about applications is always considered as part of the national application round e.g.: The Netherlands, UK, Sweden etc.

The capacity to process the volume of applications by the national team is inevitably the 'rate-limiting' factor to the number of the Centres of Expertise that can be designated each year.

2. Scope and model

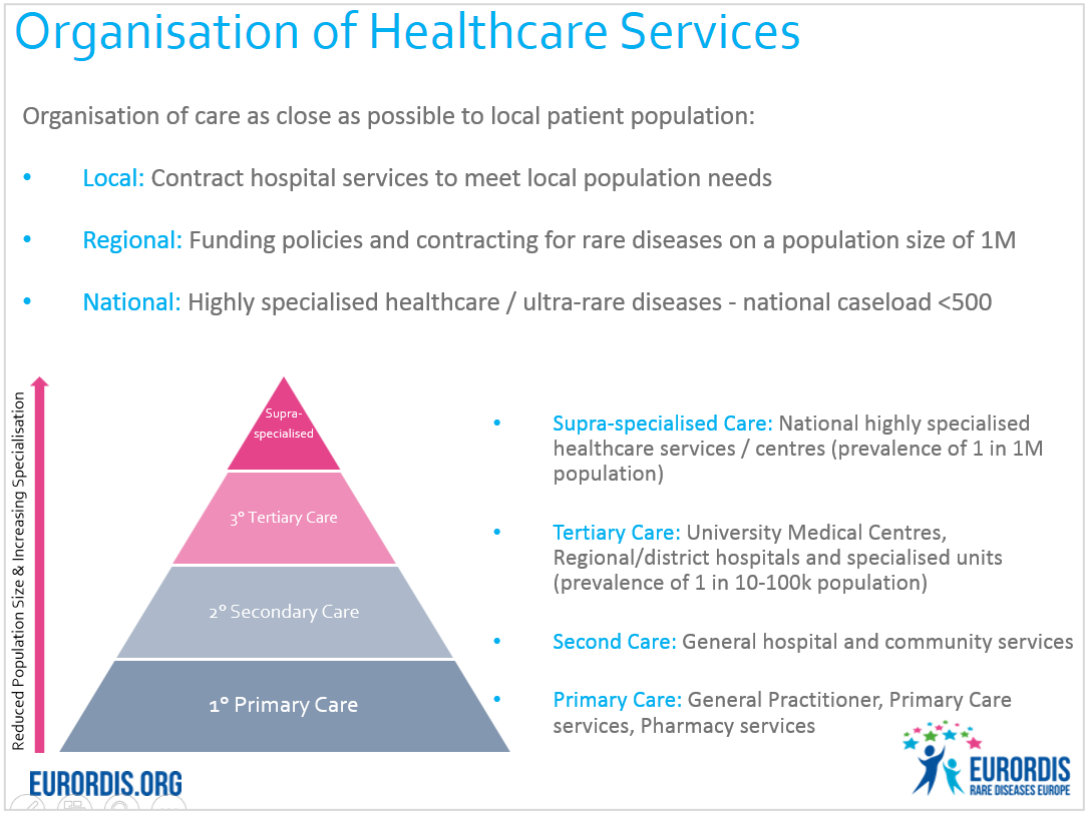
MS has adopted either an intervention focus or a disease focus to the model for Centres of Expertise.



The organisation of healthcare is most effective when as close to the local population as possible. However for rarer conditions (for a prevalence of 1 in 5/10,000 population) then this is best

organised on a population size of 1 million people. This has been shown to improve the commissioning of specialised healthcare services and designing service specification and care pathways, as you have the critical mass of patients and the understanding of both the patients needs and the services needs.

For ultra rare diseases and highly specialised healthcare services that have a prevalence of 1 in 1 million population, these are best organised and contracted on a national basis, to get the economy of scale and critical mass of patients and service needs, to design optimal clinical pathways and services.



3. Prioritisation

In general a bottom-up expression of interest by hospitals or clinical teams is a good way to start identifying available expertise through an application process. The expressions of interest can then be reviewed with local health body representatives and the national team. Engagement with the patient community will help to identify potential new national services.

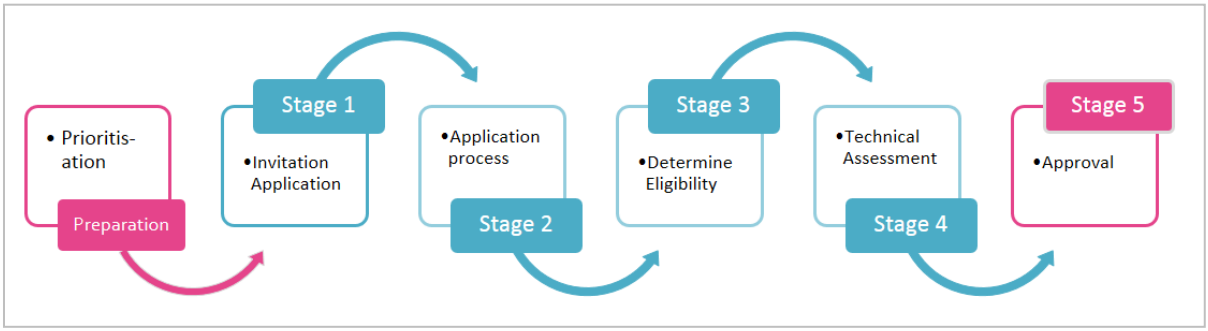
An outline for the application and the evidence base for the new service should be developed for potential national service with an outline of the service models and the identification of potential existing Centres of Expert. An advisory group, including patient representatives decides on a short list of services to be developed into full application in view of support.

The U.K. system of national commission was an extremely successful method of designating expertise and CoE, which was government-led and used a bottom-up approach to identified clinical areas where there was a need and societal added value for application for national accreditation and commissioning.

The UK National Specialised Commissioning Team (NSCT) had an advisory committee, of the 'great and the good' from the different Royal Societies and Patient Organisations. The Advisory Group for National Specialised Services (called AGNSS) used to review the list of potential applications and decide on the priority services for development each year. The clinical leads worked together for agreed priority services, to develop an application and the commissioners supported them and submitted the proposal/application.

Once initially approved by AGNSS, the NSCT developed a 'service specification'[2] for each national service which was then put in the contract with the successful Centre of Expertise.

4. Application process



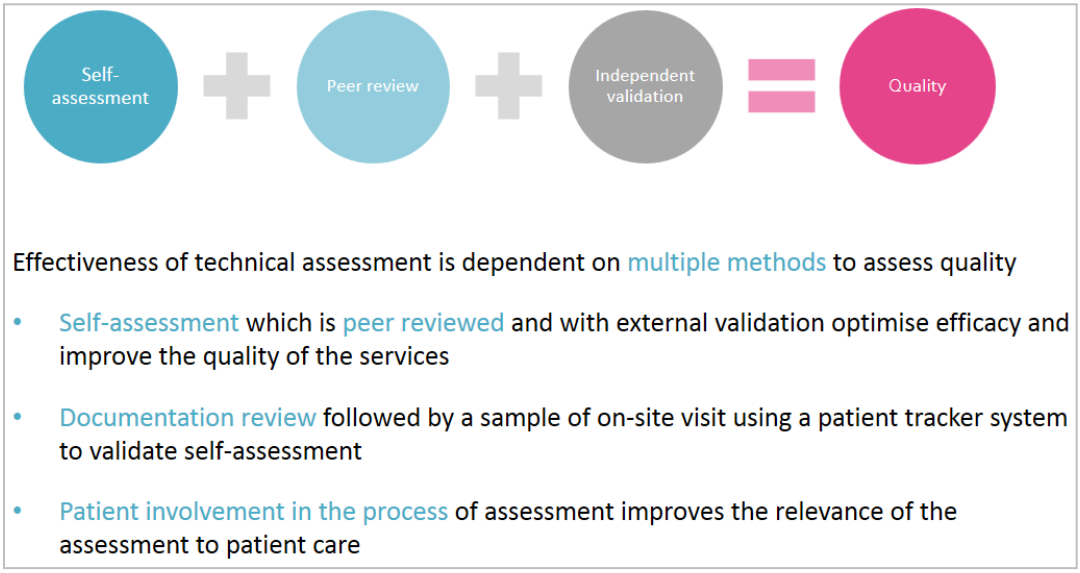
In the stage of the application process the initial expression of interest is reviewed by a public health lead and followed by a decision for the application to be developed in full. An endorsement by a relevant patient organization and professional society is required. Supporting evidence base is added on clinical care and efficacy. The application was normally developed by a number of existing expert centres collaborating together, to develop the application for national commissioning.

The applications include a needs assessment, information on the societal impact, expertise specific criteria and expert competency, referral pathways (inclusion and exclusion criteria), composition of the multidisciplinary team and the organisation of care, financing model and costing. Patient organisation and representatives' as well as the professional society gave their support in the form of a favourable or unfavourable opinion.

[2] See **list of service specifications** in the UK*:*
https://www.england.nhs.uk/specialised-commissioning-document-library/service-specifications/
See example of national **service specification for *Epidermolysis bullosa service*** *(All Ages)*
https://www.england.nhs.uk/wp-content/uploads/2018/08/Epidermolysis-bullosa-service-all-ages.pdf

Please see **list of highly specialised services in the U.K. (national services)** in the below link:
https://www.england.nhs.uk/wp-content/uploads/2017/10/prescribed-specialised-services-manual.pdf

5. Assessment methodology



Every 'model' for assessment or accreditation has its strengths and its weaknesses, so the literature highlighted that the best approach is to use 'multiple models' e.g.: three different models to triangulate the information being assessed.

Summary of the strengths and weaknesses of the different models for assessment and accreditation:

Table 1 - Summary of Assessment Methods			**Ability to assess**		
	Location	Effort/ intensity	Structure	Process	Outcome
Data					
Routine health system	Remote	+	+	+	+
Service specific	Remote	++	++	++	++
Service Based					
Visit	Local	++	+++	+++	+
Staff Interviews	Local	+++	+	+++	++
Patient Interviews	Local	+++	+	+++	++
Questionnaires					
Service Survey	Remote	+	++	++	+
Staff	Remote	+	++	++	+
Patient	Remote	+	+	+++	++

Case Review					
Medical Record Review	Local	+++	+	+++	+++
2nd Opinion	Remote	++	+	+	+++

Derived from the EC published a literature review on the different models and their evidence base for healthcare accreditation/licensing [1]

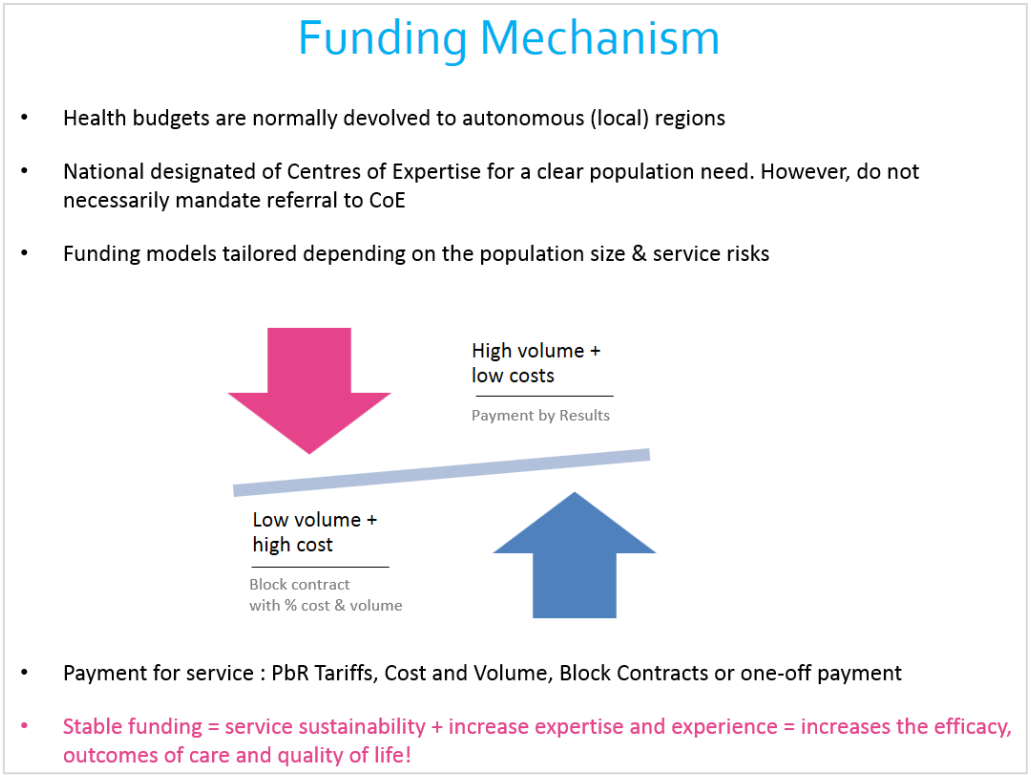
7. Funding model

Different contracting models balance the incentives of payment and exposure to risk, to create the optimum conditions for individual services, as each highly specialised service (surgery) and rare/ultra-rare disease have different needs.

Block contracts, cost & volume and payment by results (pay for activity) all have merits but a payment-by-results contract is more suitable to low cost, high volume disease areas and interventions (knee and hip replacement; A&E assessment and treatment) and is not suitable for a ultra-rare disease service where the activity levels can vary from 10 patients one year to 2 patients another. The stability of a block contract for an ultra rare disease or highly specialised healthcare services enable the hospital to maintain the clinical team (re budget) and build their clinical competency and safeguard patients access and max outcomes of care.

Funding models are a very important aspect of commissioning and national accreditation to reimburse and incentive quality of care and a stable workforce which can focus their energies in developing their knowledge and gain the experience which leading to improvement to the treatment they provide, which ultimately reduces costs to the health service.

Ultimately, the security of an tailored funding model is what hospitals and clinicians will look for in the discussions about national accreditation. It needs sufficient time to discuss and explore optimal mechanisms for Belgium.



Q&A following the presentation of Matt Bolz-Johnson:

- **On the advantages of standardised, evidence based care:**
  - Reported observation: clinicians with ***more experience*** in a given domain tend to do ***less examinations and tests for the follow-up*** in their patients. Clinicians with less patients with a given rare disease will tend to do more, preferring 'to be on the safe side'.
  - The ***use of standardised validated guidelines***, care according to validated recommendations, ***leads towards more effective and efficiënt health care*** and higher quality of life.
  - High volume centres and ***high volumes clinicians are more likely to adhere to evidence based guidelines***, because they are used to them. It is ***cost effective*** to get the right treatment at the right time. (MBJ)
  - Be aware that, as long as validated guidelines are used, **clinical variation in health care also benefits advances in disease management**. However you need to have clear currency to measure the outcomes. If guidelines are in place and outcome measures are clear, clinical variation is the **hotbed of innovation**. (MBJ)

- **Regarding the volume of rare disease patients per centre**
  - For a centre to be able to develop expertise in a given disease, ***a critical mass of patients*** in required. 400 of the 6.100 rare diseases identified account for 98% of all rare disease patients. It stands to reason that at least ***for the 2% of ultra-rare diseases, concentration of expertise is absolutely critical***.

- **On the advantage of age-related specialisation**.
  - Expertise is not only linked to diseases but also to age-groups, f.i. age-group newborns (importance for diagnosis), pediatric (different specialists are more important), adult care
  - In the Netherlands there are recognised experts for adults with intellectual disability, which is often a manifestation of a rare syndrome. In Belgium a lot of these patients get 'stuck' in pediatric care due to inexperience of adult clinicians with ID.

- **On the frequency of assessment of health care services**
  - Annual monitoring of hospitals with a designation is the basis in U.K. After five years the evaluation can lead to distinction between centres, and possibly to cutting down to fewer designations.

- **Regarding different expertise on the same disease in various centres**
  - For a given disease one centre might have expertise in diagnosis, another in disease management and yet another in a specific medical intervention for a specific diseases. How to align this with the concept of designation of expertise?
    MBJ: In the U.K. for ultra rare disease one national service has been designated but this might consist of several centres each specialised in a different disease aspect. This would be done by subcontracting the related services.
    So a clearly defined care pathway that identifies the specific services in the different centres, allows them to work together as a national expertise centre for a given rare disease.

- **Regarding the challenges of different languages in Belgium**
    - In view of a population of 11 million people, creating expertise centres for the two sides of the country equally, especially for ultra rare diseases, is not a good strategy to achieve and offer high quality expertise (MBJ)
    - Service specifications should be available in all national languages, this cannot be an obstacle (MBJ)
    - Interpreters should be a standard service in expertise centres; it is a logistic challenge and should not be regarded as more than that

- **Regarding the ideal composition of an advisory committee reviewing applications for the designation of specialised healthcare** (cfr. AGNSS in the U.K., see presentaion MBJ, 3. Prioritisation)?
    - All relevant stakeholders should be included, including policy makers at government level, people representing relevant commissions and patient representatives.
    - The committee should be respected as having the right authority; a gathering of 'the great and the good'
    - The chair of the committee should be carefully chosen to be someone who is well respected and has leadership qualities.

4. Identification of the strengths and weaknesses of the Belgian context

After discussion in smaller groups the participants presented their observations. The strengths were easily pinpointed. The enumeration of weaknesses developed into an identification of challenges.

The upside of the Belgian context and expertise centres:

- The Belgian healthcare system offers good quality of care, e.g. without long waiting lists for patients. There is quite a lot of expertise available in Belgium.
- Patients don't have to travel far for high expertise health care. Proximity of expertise.
- The models we are increasingly developing in Belgium (Flanders only up to now!) are collaborative instead of competitive.
- Clinical geneticists are being trained since a short while. Important evolution of great benefit to the rare diseases.

The downside of the Belgian context and expertise centres:

- **On the validation of expertise**
  - There is ***no validated definition of what expertise is***.
    We also need to consider different levels and types of expertise for rare diseases (f.i. expertise in early diagnosis, expertise in medical interventions, in management of specific complex diseases, …). This is all perfectly acceptable as long as it is clearly defined what these consist of.
  - There is a lack clear **outcome measures and indicators** to be able to do long term assessment and provide a basis for continuous improvement. This would turn the hurdle of accreditation into a tool for long term (self)evaluation and improvement.
    However we have to **be careful not to add to the administrative burden**. There is a limit to the amount of evaluations a team can do.
  - Identification and long-term evaluation of expertise can also be a challenge at the level of the healthcare administration.
  - There is no data management that enables objectified benchmarking. For Cystic Fibrosis such a register does exist and is used to this purpose. The PO encourages exchange of best practices to get the quality of care up everywhere. It is fundamental to gather data to be able measure clinical outcomes.
    MBJ: be weary of adding registers as a criterium, this is a massively complex issue; better to concentrate on criteria for healthcare. The issues on registers and research will follow automatically.

- **On the framework for the identifcation of expertise**
  - **Need for a clear and dynamic framework for rare disease expertise that inspires and stimulates**. Concern that accreditation as we know it turns the whole system into something static that discourages young, high potential clinicians. There have to be dynamic opportunities and clear goals for young clinicians, in order to get them motivated and inspired to commit to rare diseases.
    This is important as a strategic consideration for the long term.

- **Networks** potentially offer a more dynamic framework than the designation of centres of expertise.
- **Need to use clear indicators and collect outcome measures in registers to validate expertise**; allows for benchmarking.
- Important **criteria for expertise: research, concentration of knowledge/excellence and the accumulation of experience.** 7 centres for one disease(group) for instance does not allow to do this optimally.

- **On the dissemination and sharing of knowledge and expertise**
  - **Outreach by experts is an essential and mandatory task**. Experts need to train and educate on their expertise. Otherwise patients not managing to access the best expertise immediately will be confronted with local centres that are entirely ignorant. There is a big healthcare risk in that.
  - There should be a mandatory framework for HCPs outside of the centres of expertsie to liaise with the centres.
  - Networks can enable knowledge and expertise to be shared.
  - Collaboration can double the impact of expertise. Sharing of knowledge and expertise should be mandatory.
  - There are massive sets of service specifications available in other countries (f.i. U.K. [3] and the Netherlands). They are accessible to all online. We don't have to invent everything from scratch.

- **On the importance of intervention capability**
  - This requires the right equipment and adequate numbers of interventions to create expert experience
  - Not comparable to knowledge capability which can be more easily shared.

- **On the visibility of expertise**
  - At present ***visibility of expertise is poor and non-validated.***
    We have a situation where there is a lot of *perceived* expertise. The media become a force that contribute to this perceived expertise that is not objectively validated.
  - The **developing networks are not visible** at all yet. Their existence needs to be made explicit.
  - Patients with sufficient personal competences will find their way to the best expertise no matter how, but a large group of patients does not. Clear identification and referral would enable ***all*** to access optimal expertise.

- **On the importance of dedicated individual clinicians**
  - A lot of the best expertise for rare diseases is to be found in **dedicated individual expert clinicians at present**, who are passionate, develop experience, do research and are active

[3] See **list of service specifications** in the UK*:*
https://www.england.nhs.uk/specialised-commissioning-document-library/service-specifications/
See example of national **service specification for *Epidermolysis bullosa service*** *(All Ages)*
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in international networks. It concerns high quality expertise that is not anchored and supported in a sustainable way.
    - We should be **alert to expertise in people, not necessarily in centres**. Expertise is often situated in specific young team members who are hidden behind the names of superiors in publications.
      Dynamic frameworks like networks might offer more potential to leverage this expertise than designated expertise centres.
    - We need to have a framework that stimulates young clinicians towards expertise. The older generation of dedicated individual clinicians will not be there for ever.
    - There is no culture for hierarchy in Belgium. Expertise is easily claimed by all. There is insufficient differentiation.

- **On the challenge of funding:**
    - **In general funding is lacking**. Important aspect of the framework that needs to be developed is the consideration of how to allocate resources.
    - **Existing reimbursement systems** are coded in a way that they are **often not applicable** to the care set up in a multidisciplinary diagnostic and management centre. Therefore high quality multidisciplinary care becomes a difficult challenge instead of an asset for a medical centre from a financial perspective.
    - Pay for performance funding is hard. The funding of the reference centres should be the basis for all.

5. Barriers towards creating expertise centres in Belgium

Group discussion and presentation of the findings led to the identification of the following barriers:

- **Implementation incapability – tendency to status quo:**
  - lack of know-how in how to find opportunities and overcome hurdles to introduce change, a science in itself (implementation science).
  - **Systems like to self-preserve:** there is a methodology for system change, the **two-loop model for a paradigm shift**. You need to connect people in the process to move forwards. You have to develop a new system while the old is still in place, and in time switch from old to new.
  - **Lack of leadership towards change and lack of willingness to change,** not only on the political level but also on the level of hospital management. Hospitals are afraid to loose ground; the political level doesn't want to rock the boat. We stay locked in a status quo.
  - **Lack of patient involvement**: patient are valuable potential drivers towards change.

- **Political complexity and lack of governance**
  - **responsability for different aspects of healthcare is very fragmented**.
    Hard to come to agreements and decisions overarching all the political levels.
  - **Governance of the allocation of resources is very difficult** because of this political fragmentation.
  - **As a result healthcare for rare diseases is currently not based on validated knowledge,** science and clear criteria but on agreements between centres and experts. Centres come to a sort of exchange of services and referrals for which there is no objectified set of criteria.
    This is symptomatic of a lack of governance on how resources are allocated.

- **Lack of strategic framework**
  - **Lack of an adaptive and a reinforcing framework**.
  - **Lack of involvement and engagement of the framework** leads to insufficient sustainability.
  - **Lack of quantitative and qualitative indicators.**
  - **Lack of dynamic funding** tailored to the needs, and that stimulates.
  - **Lack of clear criteria and methodology for identification and for long term assessment** of expertise centres
  - **No signposting towards expertise for HCPs and patients**; lack of information to navigate the rare disease field

- **Perception of expertise – image-building**
  - **Label of centre of expertise is starting to become insignificant, is missing it's goal.**
    Becoming member of a European Reference Network is being claimed as a mark of expertise.
  - Being a centre adressing certain pathologies reflects on the image of a centre, on the way it is perceived. This results in a **reluctance to abandon certain pathologies**, at a hospital level as well as at the level of individual clinicians.

Hospitals are afraid that narrowing down their services (specialisation), will reflect negatively on public perception. Important to identify the drivers for this.

- **Lack of systemic funding mechanism**
  - **Competitiveness for resources** leads to self-declared expertise.
  - Defensive attitude of hospitals about designation of expertise because of the fear of the financial implications. All hospitals are struggling with funding and finances. But none want to let go of any of their services.
  - Lot of **unclear and intertwined systems of money allocation**
  - Unclear earmarking of subsidy for hospital centres, attributing it clearly to specific services; but then earmarking it for particular services requires indicators to measure their performance (the obstacles are often related)

- **Culture**
  - **Fatalism**: 'This is Belgium!'-attitude: it is too difficult; there are too many rare diseases, the political system is too difficult, there is no real will, we are stuck in defensive attitude.
  - **Lack of confidence in collaboration**; everybody is locked in a competitive attitude without confidence that collaboration will pay off and be rewarded. Competition in itself is not an obstacle, it is a positive incentive towards progress and quality.
  - **Lack of involvement and engagement** of the actors and the stakeholders on the issue of rare diseases.
  - **Lack of involvement of (and investment) in patient expertise**; involvement of patient expertise can be a driver towards change

- **Language and inequalities**
  - Complexity of the different languages used in the national healthcare organisations.
  - Flemish people tend to look for expertise in the Netherlands. French speaking people tend to look towards France.
  - **Inequal access to high quality healthcare;** financial and social status is a barrier for people to reach this expertise abroad. We need everybody to be able to access the same quality of care.

6. Possible next steps

What is necessary to move the situation forward? After a short group discussion the following thoughts are shared:

- **On formalising current informal processes**
  - Look at current best practice, see what works best, make it explicit, formalise and then expand to other rare disease areas. Institutionalise the models developed by mutual agreement in an informal set-up at present.
  - Political leadership is key. Without it, clinicians can only continue to collaborate with informal agreements, but we should aim for structural improvements for all.
  - Structure the Flemish network groups that are already operational, make them explicit, and formalise them.
    A formal board could be set up to evaluate the models developed by these networks. The federal level needs to validate them and create legislation to anchor them. Through legislation these can become mechanisms (models) to expand towards other disease areas, that are not being served yet.

- **On getting the political level to address the challenge**
  - Write down what we want, bring it to the table of negotiations at the federal level and make a note for the next governmental agreement on how to develop this.
  - Use political connections and influence to get our proposal into the federal government agreement.
  - Clinical experts can prepare proposals for the political level to act on. Elements for a framework can be offered to the political level.

- **On the characteristics of the strategic framework**
  - The framework should stimulate and reward sharing of knowledge and resources. This has the potential to leverage expertise in the field. It will counter the lack of confidence to collaborate, and the defensive attitude of competition.
  - we need both individual performance and team performance, this dilemma has to be managed to generate maximum advantages.
  - Learn from and use the models that have been used in other countries; examples of succesful procedures are plenty.
  - A multistakeholder advisory board should be involved, including patient representatives.

- **On the allocation of resources**
  - Adapt the allocation of the resources so that it is aligned to diagnostic procedures, follow-up and treatment that adhere to best practice guidelines. At the moment, some techniques are reimbursed thought they are obsolete.
  - Make funding dependent on outcome measures.
  - Make funding dependent on adherence to validated guidelines.

- **On creating a basis of involvement and engagement of all stakeholders**
    - **policy makers need to be sensibilised** on the necessity of designating expertise centres; lobby work towards the political level is needed
    - **raise awareness to shift towards ownership of the problem by all**; rare diseases concern us all. Initiative towards improvements and change should not be dependent on the coincidence of a policy maker being touched by a rare disease personally.
    - **We need a switch in the perception that one disease is more important or a bigger societal challenge than another.**
      Bigger awareness and perceived severity of particular diseases in comparision with others is not evidence based, but results from communication campaigns and emotional response to individual cases.

- **On involving the periferal hospitals and the first line**
    - we need to involve and train physicians in the peripheral healthcare centres and the first line; educate on the red flags for rare diseases in order for patients to be able to be referred to the right expertise
    - outreach and training need to be put in place as mandatory commitments of expertise centres.
- **Other**
    - Boost the interministerial conference to generate more coherent structures for rare disease management
    - Install standard service of interpreters (or a technical device for simultaneous translation) to facilitate access of expertise for patients, regardless of their language. All clinicians would be bilingual ideally.

We should not accept the status quo. All participants acknowledge the problem and the importance of moving forward. Other countries have done it, why shouldn't we? **Foster optimism! Yes, we can!**

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