POLICY FORUM

“Equal opportunities in healthcare, including for rare diseases” Patients, experts and policymakers in dialogue

07.11.2024

SUMMARY REPORT

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INTRODUCTION

This report offers a concise synthesis of the presentations and debates that took place as part of the policy forum "Equal opportunities in healthcare, including for rare diseases. Patients, experts and policymakers in dialogue", which took place on Thursday, November 7th 2024 at the Chamber of Representatives. Nearly one hundred participants were present, including Members of Parliament (MPs), patient representatives, civil servants of multiple relevant administrations (Sciensano, the Belgian Health Data Agency, the National Institute for Health and Disability Insurance – INAMI-RIZIV, the Belgian Healthcare Knowledge Centre – KCE, and the Federal Public Service Health), healthcare providers and pharmaceutical industry representatives. The policy forum was organized by University Hospitals Leuven (UZ Leuven), Rare Diseases Belgium (RaDiOrg) and the Rare Disease Diagnosis Alliance (RADDIAL), with the support of the seven other rare disease functions part of the following Belgian university hospitals: Universitair Ziekenhuis Antwerpen, Universitair Ziekenhuis Brussel, Universitair Ziekenhuis Gent, Cliniques Universitaires Saint-Luc, Centre hospitalier universitaire de Liège, Hôpital Universitaire de Bruxelles - H.U.B, and Grand Hôpital de Charleroi.

CONTEXT

In our country, more than half a million people live with a rare disease - being a disease of a life-threatening and/or chronically disabling nature with a prevalence of less than 5/10,000 inhabitants. Many spend too much time seeking diagnosis and appropriate treatment - in fact, it takes an average of 4.9 years for patients with a rare disease to receive an accurate diagnosis[1]. This leads to a significant health impact, with avoidable complications and many missed opportunities for a better quality of life.

Social awareness of rare diseases has undeniably increased in recent years. The Belgian Rare Diseases Plan, launched in 2013, has ensured progress in several key areas, but some key actions have not been implemented or have been incomplete to date. Nevertheless, rare diseases have remained on the political and societal agenda, thanks to the continuous efforts of various stakeholders.

There remain numerous challenges in the field of rare diseases. Key challenges were explained in a Memorandum for the attention of policymakers. Developed by RaDiOrg (Rare Diseases Belgium, the Belgian umbrella organisation for rare disease patients) and the Belgian College for Genetics and Rare Diseases, it was sent to Belgian party presidents shortly before the June 2024 elections. A specific memorandum on improving diagnosis for rare disease patients was published by the Rare Disease Diagnosis Alliance (RADDIAL) in June 2023 containing 12 policy recommendations validated and endorsed by several key opinion leaders in the field.

1 Eurordis, «Rare Barometer Survey», 2022

CONTEXT

The policy forum held on November 7, 2024, aimed to facilitate a dialogue between experts, patient representatives and politicians on the measures to be taken to meet these challenges and improve care for rare disease patients.

It was organized through the collaboration of the eight rare disease functions[2] - University Hospitals linked to a centre for human genetics competent for the interdisciplinary diagnosis, treatment and follow-up of rare disease patients - alongside RaDiOrg and the Rare Disease Diagnosis Alliance (RADDIAL).

The organisers stress that the announced new Belgian Rare Diseases Plan must not remain a dead letter but must be effectively implemented, taking into account the policy competences of the various authorities and their mutual coherence. This new Belgian Plan Rare Diseases is crucial for the realisation of a coordinated policy and an optimal health outcome for the more than 500,000 Belgians living with a rare disease and their relatives.

2 Universitair Ziekenhuis Leuven, Universitair Ziekenhuis Antwerpen, Universitair Ziekenhuis Brussel, Universitair Ziekenhuis Gent, Cliniques Universitaires Saint-Luc, Centre hospitalier universitaire de Liège, Hôpital Universitaire de Bruxelles - H.U.B, and Grand Hôpital de Charleroi

1ST THEME :

European Reference Networks (ERNs) and their integration into national networks (JARDIN: Joint Action on Integration of ERNs into National Healthcare Systems)

Introduction by: **Béatrice Gulbis (Hôpital Universitaire de Bruxelles - H.U.B.)**
Debate with: **Jan Bertels (Vooruit), Hervé Cornillie (MR), Jeremie Vaneeckhout (Groen) and Nawal Farih (CD&V)**

In her introduction, Prof. Béatrice Gulbis outlined the functioning of the European Reference Networks (ERNs), which aim to improve patient access to specialized care across the European Union by connecting over 1,600 healthcare units across 27 countries. She explained that Belgium faces challenges in fully integrating the ERNs into its national healthcare system, as there is no formal recognition or accreditation of ERNs at the national level. Additionally, there is an unworkable fragmentation of expertise and resources among the Belgian hospital functions involved.

Recognized and evaluated Belgian expertise networks that communicate with the ERNs are the way forward. Better cooperation between experts in hospital functions and other levels of care needs to be more strongly formalised, by reflecting ERNs in national expertise networks. Such national rare disease expert networks can significantly enhance patients' access to knowledge and expertise. They can also serve as partners for policymakers in developing optimal care for rare disease patients. One example is the implementation of efficient, structured patient data registers. This would also provide high-quality information for decisions on reimbursement and early access to innovative treatments, for example.

Moreover, to improve accessibility to centers of expertise linked to the ERN for patients in Europe, the EU has funded a three-year project called the Joint Action on Integration of European Reference Networks into National Healthcare Systems ('JARDIN'). The Federal Public Service Public Health and Sciensano, the Belgian institute for health, are participating in 'working group 8' dedicated to database management. The aim is to develop recommendations to ensure the interoperability of data structures at Member State level (local, regional, national) and European level. Professor Gulbis concluded that at national level sustainable support and a governance model are needed, but taking into account what is being put in place at the European Union level (ERN). This is to ensure that Belgian networks of expertise meet local needs and European standards, for example in terms of registers.

1ST THEME

During the debate, the importance of patient registration data for research advancements and health policy was emphasized. These are found to be currently suboptimal, although the establishment of the European Health Data Space (EHDS) is regarded as an important step in the right direction and as an impetus to improve patient registration. The Belgian Health Data Agency (HDA) will operationalize this registration. Ideally, this should lead to a single data flow.

A representative from Sciensano pointed out that the legal framework for collaborating with other healthcare actors on patient registrations data is currently missing. This framework should include the preferred coding language(s): the rare diseases-specific ORPHA-coding and/or the more generalized, soon-to-be-compulsory SNOMED-coding; and incentives for/obligation of coding by healthcare providers. An exchange of views took place about the desirability of an obligation to register patient data. If such an obligation were to be enforced, the means for concerned actors should increase in line with the added responsibility. An exchange of views also took place about the importance of uniform data registration by all concerned actors. While it is understood that such uniformity would be helpful, it is also pointed out that, in the past, no political consensus was found on such an obligation.

Next to that, Sciensano's financial means should also be increased in line with the higher number of registrations made. It was also noted that only two people at Sciensano handle registration efforts, which is insufficient compared to the 30 people working on the cancer registry. There appears to be a consensus that more training but above all more support is required for healthcare personnel if this registration effort is to be a success.

1ST THEME

POLICY RECOMMENDATIONS:

1.

INTEGRATION OF EUROPEAN REFERENCE NETWORKS (ERNS) INTO NATIONAL HEALTHCARE SYSTEMS

- Establish national rare disease expert networks that align with the structure and goals of ERNs.
- Develop a governance model to ensure these networks address local healthcare needs while remaining compatible with European standards.
- Facilitate cooperation between hospital experts and other levels of care to improve patient access to expertise and support optimal rare disease management.

2.

OPTIMIZED PATIENT DATA REGISTRATION

- Operationalize patient data registration under a single, standardized framework, potentially through the Belgian Health Data Agency (HAD), leveraging systems like the European Health Data Space (EHDS).
- Implement a legal framework to enable collaboration among healthcare actors, adopting rare disease-specific ORPHA coding and the general SNOMED coding.
- Allocate additional financial and personnel resources to institutions like Sciensano to handle expanded registration efforts effectively.

3.

SUPPORT AND TRAINING FOR HEALTHCARE PROFESSIONALS

- Increase support and training for healthcare professionals to ensure effective data registration and patient care within the ERN framework.

2ND THEME:

Transparent identification of expertise about rare diseases and the facilitation of access to this expertise

Introduction by:
Marion Delcroix (UZ Leuven)
Debate with:
Kathleen Depoorter (N-VA), Dirk Devroey (Open VLD), Jean-François Gatelier (Les Engagés), and Jeremie Vaneeckhout (Groen)

By way of introduction, Prof. Marion Delcroix discussed Belgium's current gaps in identifying and leveraging expertise for rare diseases. Expertise in rare diseases is not clearly identified, making it hard for patients to find. Barriers include a fragmented policy landscape and limited systemic funding. Prof. Delcroix proposed formalizing expertise recognition processes and encouraging political collaboration to improve the visibility and access to specialized care centres for rare disease patients.

The legal framework for the recognition of expertise is unclear, with ambiguities about which political level has precise powers on this matter. A methodology for identifying expertise needs to be developed and implemented – this has been awaited since 2013. Managing national accreditation/ designation systems takes time and resources. In other Member States, a national team coordinates the designation/accreditation process and an independent advisory group decides on e.g. prioritisation and approval of applications. The capacity to process the volume of applications from the national team is inevitably the 'speed limiting' factor for the number of centres of excellence that can be designated each year. Since the European Reference Networks (ERNs) apply the above methodology for the inclusion of national centres, their criteria and documents could identify and recognize Belgian centers of expertise. 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. Since many aspects of the establishment of centers of expertise fall within the realm of the regions, inter-ministerial agreements will also have to be made as part of this exercise to ensure the same criteria apply across regions.

2ND THEME

During the debate, the willingness was expressed by multiple policymakers to develop an updated Belgian Rare Disease Plan. Raising awareness among policymakers about the importance of advancing rare disease policy is key in achieving this. That said, stakeholders present voiced the concern that an updated plan alone would not suffice in properly and quickly addressing the existing challenges. There is a need for a true commitment for change by means of an action plan that contains clear and concrete goals, a timeline and the necessary means.

It was also pointed out that the balance between increased financial means and an efficient allocation of financial means will also have to be considered. The matter of regionalization vs. federalization was also raised, and most policymakers agreed that the federal level should be in charge when it comes to concentrating expertise and recognizing expertise.

Professor Delcroix also discussed the need to create incentives for hospitals to collaborate and ensure healthcare providers are equipped to refer patients to the appropriate excellence centers. Improving the practical possibilities for referrals, rather than imposing a referral obligation, was emphasized. There were differing views among the panel members about the desirability of such a referral obligation, and it was suggested that policymakers should collaborate closely with academics as well as first-line and second-line professionals about this question. Aside from the updated Belgian Rare Disease Plan, it was pointed out that a model for closer cooperation across healthcare actors should be developed. Among other things, interventions from the audience emphasized that the current Belgian Rare Disease Plan does not adequately facilitate the proper functioning of all healthcare actors - this should be improved in the updated Plan.

2ND THEME

POLICY RECOMMENDATIONS:

1.

UNIFIED FRAMEWORK FOR IDENTIFYING AND RECOGNIZING EXPERTISE

- Formalize the process for recognizing expertise in rare diseases by implementing a clear, national methodology.
- Use the European Reference Networks (ERNs) criteria as a foundation for accrediting Belgian centers of expertise.
- Establish a national team to coordinate the designation process, supported by an independent advisory group to prioritize and approve applications.

2.

CREATE AND IMPLEMENT AN UPDATED BELGIAN RARE DISEASE PLAN

- Update the Belgian Rare Disease Plan, and include a detailed action plan with clear goals, timelines, and allocated resources.
- Ensure that the federal government takes the lead in coordinating expertise recognition, with inter-ministerial agreements to address regional responsibilities effectively.
- Balance increased funding with efficient allocation to maximize impact.

3.

IMPROVE ACCESS AND REFERRAL SYSTEMS FOR RARE DISEASE PATIENTS

- Develop incentives for cross-hospital cooperation and equip healthcare providers with the tools to make effective referrals to specialized centers.
- Enhance the referral systems, in collaboration with academics and first-line and second-line healthcare professionals.

3RD THEME:

Multidisciplinary and integrated care with case management for each person suffering from a rare and complex disease

Introduction by:
Eva Schoeters
(Rare Diseases Belgium – RaDiOrg)
Debate with:
Jan Bertels (Vooruit), Kathleen Depoorter (N-VA), Caroline Désir (PS), and Jean-François Gatelier (Les Engagés)

In her introduction, Eva Schoeters addressed the importance of a multidisciplinary and integrated care approach for all complex and rare disease cases. As a minimum, this approach should provide for individual care plans, for regular multidisciplinary consultation – inside medical centers as well as transmurally – and effective case management.

She added the importance of coverage of transportation costs and the cost of treatment abroad when necessary. She pointed out the inequity in the current healthcare system, where the quality of care depends on a disease's visibility and the attention it receives from policymakers. This situation puts less prevalent and lesser-known diseases, including most rare diseases, at a disadvantage. Ms. Schoeters further questioned whether the current system of conventions, including the proposed generic rare disease convention, is the right tool to solve this problem given the difficult and slow nature of the process. Integrated and specialized healthcare, she argued, would significantly improve outcomes for rare disease patients by addressing their unique challenges.

3RD THEME

During the debate, panel members shared the view that progress can be made with regards to offering an integrated care trajectory for rare disease patients. In this regard, it was suggested that it must first be determined which diseases could qualify for this trajectory, and that adequate care budgets must be made available. Panel members agree that determining which diseases would be eligible for such a trajectory should be a delicate, well-considered exercise. In this regard, the point was also raised that diseases with a very small number of patients should not be overlooked.

Audience interventions emphasized the need for decisive action, moving beyond just expressing the will to change. The emerging ecosystem of innovative technology players should also be considered as potentially important actors in integrated care trajectories where the patient can be central. Panel members agreed that the patient should at all times be central and tended to agree that technology companies could be involved in this exercise.

3RD THEME

POLICY RECOMMENDATIONS:

1.

IMPLEMENTATION OF INTEGRATED CARE TRAJECTORIES

- Develop and implement integrated care plans for rare and complex diseases, ensuring regular multidisciplinary consultations across medical centers and care settings.
- This approach should include case management and equitable access to necessary services such as transportation and treatment abroad.

2.

INCLUSIVE CRITERIA FOR ELIGIBILITY IN CARE TRAJECTORIES

- Establish well-considered criteria for determining which rare diseases qualify for integrated care trajectories.
- Ensure that even diseases with very small patient populations are not excluded, promoting equity across all rare conditions.

3.

INVOLVEMENT OF INNOVATIVE TECHNOLOGY

- Consider the involvement of innovative technology providers in integrated care, ensuring that the patient remains central to the trajectory.

Q&A
SESSION WITH THE AUDIENCE

During the Q&A session, both audience members and expert speakers emphasized that an actionable, updated Belgian Plan for Rare Diseases should be an important objective in the short term. Aside from the plan itself, there should also be clarity about the timeline and the allocated budget. Other points made include the importance to overcome taboos to come to decisive action, and to make sure that policymakers remain consistently aware about the importance of making progress in rare disease policy. It was also mentioned that patient associations play a crucial role in informing patients about important new developments, and that they need more government support to fulfil their essential role as stakeholders and representatives of patients within the health landscape.

WRAP-UP BY MODERATOR CHRISTOPHE DEBORSU & CALL-TO-ACTION BY EVA SCHOETERS

By means of wrap-up, the moderator thanked the speakers and the audience members for a very lively and stimulating debate. Following this, Eva Schoeters held a final plea towards the policymakers present to maintain awareness about rare disease policies. She pointed out that rare disease patients are very numerous, as opposed to what the word 'rare' would seem to imply, because there are so many different rare diseases. Hence the importance of systemic changes to allow for better healthcare for all low-prevalence diseases. She urged the policymakers to participate in RaDiOrg's actions on the occasion of the upcoming Rare Disease Day on February 28th 2025.

MORE INFORMATION AVAILABLE HERE:

ABOUT THE CO-ORGANISERS

University Hospitals Leuven (UZ Leuven), in collaboration with the seven other rare disease functions - i.e. recognised centres for human genetics responsible for the interdisciplinary diagnosis, treatment and follow-up of rare disease patients.

More information on uzleuven.be:

RaDiOrg (Rare Diseases Belgium), the Belgian umbrella association for people with rare diseases. RaDiOrg is the national alliance of Eurordis, the European federation for rare diseases.

More information on radiorg.be:

The RaDiOrg memorandum can be found here:

Rare Disease Diagnosis Alliance (RADDIAL), an initiative of five pharmaceutical companies, aiming to initiate a broad dialogue between stakeholders aimed at accelerating the diagnosis pathway for rare disease patients.

The RADDIAL Memorandum and more information can be found on raddial.be/:

This report synthesizes the discussions and recommendations from the policy forum “Equal opportunities in healthcare, including for rare diseases. Patients, experts and policymakers in dialogue", which took place on Thursday, November 7th, 2024, at the Chamber of Representatives. The main themes discussed include the integration of European Reference Networks (ERNs) into national healthcare systems, the identification of and access to expertise on rare diseases, and the implementation of multidisciplinary and integrated care. Participants stressed the need for a new Belgian Rare Disease Plan, better coordination between levels of government, and more efficient patient registration systems. Concrete recommendations and calls for action were formulated to improve the care of rare disease patients in Belgium.

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