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# Achieving Holistic Person-Centred Care to Leave No One Behind

A contribution to improve the everyday lives  
of people living with a rare disease and their families

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May 2019

[eurordis.org/carepaper](http://eurordis.org/carepaper)



## About EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a **unique, non-profit alliance of over 800 rare disease patient organisations from more than 70 countries** that work together to improve the lives of the 30 million people living with a rare disease in Europe.

By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services.

**EURORDIS works across borders and diseases to improve the lives of people living with a rare disease.**

**The EURORDIS vision is to enable better lives and cures for people living with a rare disease.**

## About Rare diseases

**OVER  
6000**

distinct rare diseases

Each one affects fewer than  
**1 IN  
2000  
PEOPLE**



All together, an estimated

**30  
MILLION PEOPLE**

are living with a rare disease in Europe



**Expertise, knowledge, information on diseases and their consequences are scarce and difficult to access**



**Rare, complex, chronic, disabling, progressive, degenerative, often life-threatening**

**NO  
CURE**

for the vast majority of diseases and few treatments available

They are **geographically scattered** and often **isolated**

**Few experts, geographically scattered  
Research is fragmented**

In this paper, "people living with a rare disease" refers to people directly affected by the rare disease, people living with undiagnosed complex/disabling conditions and family members of both these groups. The use of the word "carers" in this paper refers to family members.

## Why this paper?

This paper presents **evidence on the unmet everyday needs of people living with a rare disease and their family members** (often the main carers), while also offering a **synthesis on policy and recommendations to achieve holistic care for rare diseases**.

Over the years, EURORDIS and its members have been taking part in discussions with all stakeholders to shape holistic care solutions. **Our wish is to continue being part of the solution.**

**The ambition of EURORDIS is to have holistic care provided to the 30 million people living with a rare disease in Europe, and their families, by 2030.**

**Our ambition is to see people living with a rare disease integrated in a society that indeed leaves no one behind.**

**Evidence demonstrates that people living with a rare disease and their families continue to face serious every day and social inclusion challenges. It is urgent to address these unmet needs. All stakeholders, working together, have the capacity to advance this change,** co-creating the necessary strategies, policies, knowledge, tools and sustainability frameworks to advance these priorities. Our objective with this position paper is to inspire that process, by highlighting and recommending part of those strategies.

Furthermore, we believe that the improvements to be achieved will not only benefit all people living with a rare disease and their families but also millions of people living with other chronic complex diseases/disabilities. These improvements will lead to more resilient and more efficient health and welfare systems, while bring about broad societal gains.

**With the proposals presented in this paper, we intend to support European countries in implementing the National Plans for rare diseases, the European Pillar of Social Rights, the United Nations Convention on the Rights of Persons with Disabilities and the Sustainable Development Goals set by the United Nations.**

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A version of this paper is available in French, Spanish,  
German, Italian, Portuguese and Russian via:

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# 1 Executive summary

## Our ambition for holistic care by 2030

Today, the **30 million Europeans living with a rare disease and their family members** (often the main carers) remain a marginalised and largely invisible population, with little information about their diseases and their rights, few treatments, and a high level of psychological, social and economic vulnerability.

**The ambition of EURORDIS is to have holistic care provided to the 30 million people living with a rare disease in Europe, and their families, by 2030.**

Holistic care covers the 360° spectrum of the health, social and everyday needs of people living with a rare disease and their families.

**Our ambition is to see people living with a rare disease and their families integrated in a society that provides holistic care, by:**

- **Being aware of their needs and effectively providing timely, high-quality care according to these needs;**
- **Breaking down barriers in access to care, treatment, education, employment, leisure, psychological support and all aspects of social inclusion;**
- **Enabling them to fully enjoy their fundamental human rights, on equal footing with other citizens.**

**People living with a rare disease should be integrated into a society that leaves no one behind.**

With this position paper we aim to inspire a process that ensures people living with a rare disease have access to holistic care.

We applaud all stakeholders for the progress achieved in the last decades. However, **evidence demonstrates that people living with a rare disease and their families continue to face serious every day and social inclusion challenges.**

For example, for **85% of people living with a rare disease** the disease impacts upon several aspects of their health and everyday life. Not surprisingly, a striking number of **7 in 10 people living with a rare disease and family carers have to reduce or stop their professional activity** and **69% also face an income decrease.**

## Why take action now?

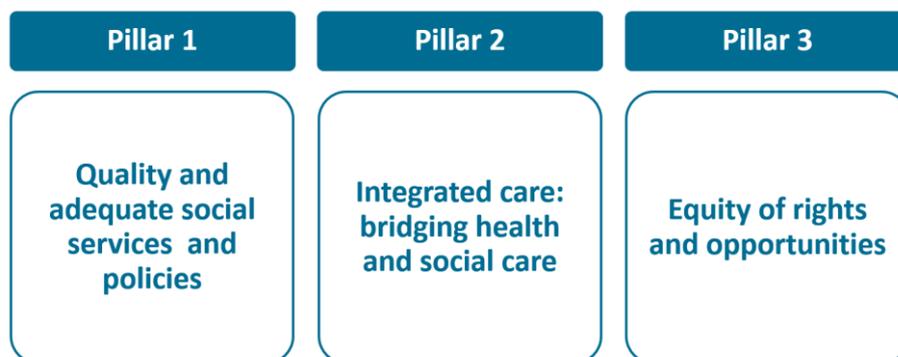
It is urgent to address the serious unmet needs of people living with a rare disease and their families. Doing so requires a multi-sector approach from research, to diagnosis, access to treatment, health care and social care, at both national and European levels. **All stakeholders have an instrumental role to play.**

The time to act is **now**, while:

- The **Sustainable Development Goals** are calling for effective global action to ensure that no one is left behind;
- The **European Pillar of Social Rights** is paving the way for the European Union and its Member States to deliver more effective social rights, equal opportunities and social inclusion;
- The **24 European Reference Networks** and the **European Network of Resource Centres for Rare Diseases** can act as platforms to gather and disseminate knowledge and good practice;
- **25 EU Member States have now adopted and are implementing a national plan** for rare diseases;
- **Organisations representing people living with a rare disease, public bodies, health and social care providers, and other stakeholders are ready to engage and to co-create policies and services.**

## Making holistic care a reality for people living with a rare disease

The strategy to ensure holistic and integrated care for rare diseases must be built on the following game-changers, explored in detail within this paper and which led to its recommendations:



Drawing from these pillars, we believe that significant improvements can be achieved if:

- **Care and support are organised within a holistic, person-centred, multidisciplinary, continuous and participative approach, considering both the person living with a rare disease and the family carers;**
- **Care providers across sectors are equipped with knowledge, good practice and care coordination strategies** allowing them to take into account the specificities of rare diseases;
- **Integrated care is effectively and timely delivered**, in coordination within and between health, social and community services and organisations representing people living with a rare disease;
- **Mechanisms to meaningfully engage people living with a rare disease and their representative organisations in the design, implementation and monitoring of policies and services are established;**
- **Social and disability policies effectively take into account the specificities of complex conditions and disabilities, such as rare diseases;**
- **People living with a rare disease and their families are informed and empowered to know and to manage their condition.**

## Our recommendations

To inspire the implementation of these improvements, **we call upon the EU, all European countries and all stakeholders** within the health and social sector, to **disseminate** this paper and to **take action** based on the recommendations put forward below.

**Only together, will we ensure that no one of the 30 million Europeans living with a rare disease is left behind. To ensure the improvements set out above are effectively achieved, we recommend:**

- 1. Making full use of EU instruments and European networks to implement holistic care for rare diseases**
- 2. Creating a supportive political environment at national level for holistic care for rare diseases**
- 3. Gathering and disseminating knowledge and good practices, to ensure that the needs of people living with a rare disease and their carers are adequately addressed by specialised and mainstream services**
- 4. Implementing specific mechanisms that ensure integrated care provision to rare diseases**
- 5. Guaranteeing meaningful engagement of organisations and representatives of people living with a rare disease in the design and implementation of policies and services**
- 6. Implementing specific measures that ensure access of people living with a rare disease and their carers to adequate social services and social protection**
- 7. Ensuring the recognition and adequate compensation of the disabilities experienced by people living with a rare disease**
- 8. Creating the conditions for people living with a rare disease and their carers to access adapted and sustainable employment**
- 9. Implementing specific mechanisms that empower people living with a rare disease and their carers, in co-creation and co-delivery with organisations representing people living with a rare disease**
- 10. Eliminating all types of discrimination, ensuring that people living with a rare disease have access to social, labour, educational, leisure inclusion on equal footing with other citizens**

## 2 Impact of rare diseases on daily life: understanding the specific challenges

Being confronted with a rare disease is a life changing and often challenging event for both the affected individuals as well as their families.

Health, defined by the World Health Organization as a state of complete physical, mental and social well-being<sup>1</sup>, is clearly compromised for 30 million Europeans living with a rare disease and their family members, often the main carers, whose daily lives, well-being and employment are affected.

The impact of rare diseases is no less considerable than that of other chronic diseases and disabilities. When compared to more prevalent chronic diseases, people living with a rare disease have a worse quality of life and experience higher losses in terms of medical care, social and economic activities<sup>2</sup>. Rare diseases are often serious, chronic, highly complex, severely disabling, degenerative and associated with comorbidities<sup>3,4</sup>. There are over 6,000 rare diseases and many people affected by rare and complex health conditions remain undiagnosed.

A high percentage of people living with a rare disease are affected by motor, sensorineural or intellectual impairments, which can occur simultaneously<sup>5</sup>. Even when they are not associated with a disability, rare diseases will in many cases influence the person's health and have a disabling impact on daily life.<sup>6</sup> **No specific treatment is available for most rare diseases** and existing treatments are not always able to minimise all of the complex impairments generated by a disease<sup>3</sup>. As a result, **rare diseases substantially affect life expectancy<sup>3</sup> and account for a considerable rate of the early-life deaths and life-long disabilities** in the European population<sup>7</sup>.

Evidence from the first European survey on the everyday impact of rare diseases<sup>a</sup> - 'Juggling care and daily life: The balancing act of the rare disease community'<sup>8</sup> - confirms that the consequences of living with a rare disease reach far and wide beyond the health niche. **85% of the respondents declared that the rare disease impacts upon several aspects of their health and everyday life.** The survey demonstrates that the consequences of rare diseases extend to the Activities of Daily Living<sup>b</sup>, socio-economic, family, education, employment and other social inclusion spheres:



Rare diseases have a serious impact on Activities of Daily Living



People living with a rare disease and their carers spend **significant time managing the disease and the care pathway**



The disease generates a **strong impact on employment and work-life balance** as well as **important economic burden**



Care pathways are complex and hard to manage



People living with a rare disease and their carers **lack information** and feel that **social services are badly prepared to support them**



There is a **serious impact on the mental health** of people living with a rare disease and their carers

<sup>a</sup> Survey of 3000 people living with a rare disease and carers, conducted through the EURORDIS survey initiative Rare Barometer Voices <https://www.eurordis.org/voices>, within the EU-funded project INNOVCare [www.innovcare.eu](http://www.innovcare.eu). Full results available here: <http://bit.ly/SurveyRD>.

<sup>b</sup> Activities of Daily Living (ADL) "refer to the basic tasks of everyday life, such as eating, bathing, dressing, toileting, and transferring and inability to execute these tasks indicate some degree of dependence and the need for assistance"<sup>37</sup>.

**The time spent managing the disease is significant:** 42% of the people living with a rare disease and family carers who responded to the survey spend more than 2 hours a day on illness-related tasks (e.g. hygiene, administration of treatments) and one third of the carers spend over 6 hours a day on these tasks.

Moreover, people living with a rare disease need follow-up care and support from different health professionals, often from several different medical specialists, as well as from social workers and other social and local service providers<sup>3</sup>. These may also include (re)habilitation, day-care, home care, personal assistants, respite services, adapted schools and work places, psychological support and social prescribing, among others<sup>9</sup>.

While care pathways remain very complex and fragmented across European countries, significant additional time is spent organising care outside of the home (e.g. arranging and attending appointments), which 64% of the respondents consider hard to manage.

Care pathways remain very complex and fragmented

Unsurprisingly, a striking number of 7 in 10 people living with a rare disease and their carers had to reduce or stop their professional activity, while 58% were absent from work over 15 days in the year prior to the survey.

The combined challenges with employment and with managing the various consequences of the disease aggravate the economic burden faced: 73% of the respondents state that the costs related with their disease are high while 69% also face an income decrease.

For many, if not most people living with a rare disease and their carers, plights like poverty, unemployment, stigmatisation and social exclusion are not distant concepts – they are a daily reality, and a direct consequence of their medical condition.

As a result of these circumstances, feelings of depression and unhappiness are three times more common amongst people living with a rare disease and their family carers compared with the general population<sup>8</sup>. The disease and the everyday challenges thus have a clear strong impact on mental health and wellbeing, leading to limited social integration prospects.

The combination of rarity, complexity and lack of effective treatment bring about additional challenges to the provision of holistic care as mainstream services are not flexible enough to take into consideration unprecedented needs<sup>10</sup>. In many cases, significant medical, psychological and social needs remain unmet.

Today, people living with a rare disease - children, adults, the elderly, and their family carers (who are primarily women<sup>8</sup>) - remain a marginalised and largely invisible population, with little information about their diseases and their rights, few treatments, and a high level of psychological, social and economic vulnerability.



Louis and Matthieu, Becker muscular dystrophy, France

<sup>8</sup> Survey sample compared to International Social Survey Programme 2011.

### 3 Taking action can significantly improve the lives of millions of people living with a rare disease in Europe

The ambition of EURORDIS is to have holistic care provided to the 30 million people living with a rare disease in Europe, and their families, by 2030.

Our ambition is to see people living with a rare disease and their families integrated in a society that is aware of their needs, that provides effective and timely, high quality, person-centred care, and that mitigates barriers in access to all aspects of social inclusion.

Our ambition is to see people living with a rare disease and their families integrated in a society that leaves no one behind, allowing them to fully embrace their fundamental human rights, on an equal footing with other citizens.

We applaud all stakeholders for the progress achieved in the last decades. With the important advances in rare disease care, from research and diagnosis to access to treatment and specialised health care, people living with a rare disease now have longer life expectancies.

However, it is still true today that depending on the Member State and region, treatment and (re)habilitation for rare diseases differ significantly on their availability and quality<sup>11</sup>. Furthermore, evidence demonstrates that people living with a rare disease and their families, who are often the main carers, face serious every day and social inclusion challenges across European countries.

The Commission Expert Group on Rare Diseases states that the failure to meet these serious unmet social needs affects their dignity, autonomy and other fundamental human rights expressed in the Universal Declaration of Human Rights and in the United Nations Convention on the Rights of Persons with Disabilities<sup>3</sup>.

It is urgent to address these unmet needs. The time to act is now, while:

- The Sustainable Development Goals are calling on an effective global action to combat societal challenges and to ensure that no one is left behind;
- The European Pillar of Social Rights is paving the way for the European Union and its Member States to deliver more effective social rights, equal opportunities and social inclusion for EU citizens;
- The 24 European Reference Networks for rare diseases and the European Network of Resource Centres for Rare Diseases are established and can act as platforms to gather knowledge and good practice on how to organise holistic care for rare diseases;
- 25 EU Member States have now adopted a national plan or strategy for rare diseases, and are focusing on implementing and monitoring them. The discussions on the unmet everyday needs remain on the agenda of most national rare disease conferences;
- Organisations representing people living with a rare disease<sup>a</sup>, public bodies, health and social care providers and other stakeholders are ready to engage and work together to co-create good practices that support the implementation of holistic care for people living with a rare disease;
- Organisations and representatives of people living with a rare disease are united in a strong movement, ready to engage in the co-creation of policies and services as equal partners.

Important frameworks have been put in place to support rare disease care. These must be sustained, but alone, they will not suffice. More action is required by all stakeholders to ensure that European countries effectively develop and implement policies and services that guarantee holistic care for rare diseases.

The rare disease community calls on all stakeholders to take action based on the recommendations put forward in this position paper. None of the millions of Europeans living with a rare disease should be left behind. The time to act is now.

All stakeholders must bear in mind that, as acknowledged by the European Commission, many “rare diseases are compatible with a normal life if diagnosed on time and properly managed”<sup>11</sup>. A timely and tailored diagnosis and care pathway, based on individual needs, will increase the levels of autonomy and participation in society of both the affected individuals as well as their carers.

Care models for people living with a rare disease also have the potential to serve a large number of people affected by other complex chronic conditions and disabilities. Improved care models for rare diseases will thus contribute to build more resilient, efficient health and welfare systems. These will also bring about broad societal gains, preventing millions of citizens from facing further health and social vulnerabilities, while also increasing social participation and employment prospects.

With the proposals presented in this paper, we intend to contribute to support European countries with the implementation of the National Plans for rare diseases, the European Pillar of Social Rights, the United Nations Convention on the Rights of Persons with Disabilities and the Sustainable Development Goals set by the United Nations.



*Shelby and Taylor, Angelman syndrome, USA*

## 4 Our recommendations

### EURORDIS and its members RECALL:

- ✓ The **Universal Declaration of Human Rights**;
- ✓ The **United Nations Convention on the Rights of Persons with Disabilities**;
- ✓ The **World Health Organisation Constitution**;
- ✓ The **Sustainable Development Goals**;
- ✓ The **United Nations Resolution on Global Health and Foreign Policy: Addressing the Health of the Most Vulnerable for an Inclusive Society**;
- ✓ The **European Charter of Fundamental Rights**;
- ✓ The **European Pillar of Social Rights**;
- ✓ The **Commission Expert Group on Rare Diseases Recommendations to Support the Incorporation of Rare Diseases into Social Services and Policies**;
- ✓ The **National Plans and Strategies for rare diseases**;
- ✓ **And EMBRACE** the forthcoming **United Nations Political Declaration on Universal Health Coverage**.

EURORDIS and its members **CALL UPON THE EU and all EUROPEAN COUNTRIES** to ensure that people living with a rare disease and their families are not left behind, in line with the provisions established in these important global, European and national frameworks.

We believe that significant improvements can be achieved if:

- ✓ Care and support are organised within a holistic, person-centred, multidisciplinary, continuous and participative approach, considering both the person living with a rare disease and the family carers;
- ✓ Care providers across sectors are equipped with knowledge, good practice and care coordination strategies, allowing them to take into account the specificities of rare diseases;
- ✓ Integrated, long-term care is delivered in an effective and timely manner, in coordination within and between health, social and community services and organisations representing people living with a rare disease;
- ✓ Mechanisms are established to meaningfully engage people living with a rare disease and their representative organisations in the design, implementation and monitoring of policies and services;
- ✓ Social and disability policies effectively take into account the specificities of complex conditions and disabilities, such as rare diseases;
- ✓ People living with a rare disease and their families are informed and empowered to understand and manage their condition.

To ensure these improvements are effectively achieved, EURORDIS and its members **RECOMMEND:**

#### 1. Making full use of EU instruments and European Networks to implement holistic care for rare diseases

- ✓ All legislative proposals and recommendations deriving from the European Pillar of Social Rights must take into account the specific needs of people living with a rare disease, their carers and others with complex diseases/disabilities; the 'Social Scoreboard' should introduce clear indicators and monitoring tools to support effective policy changes;

- ✓ **Available EU mechanisms must support the implementation of social services and integrated, long-term care for people living with a rare disease, their carers and others with complex diseases/disabilities:**
  - **Funding lines and programmes should be secured under the Multiannual Financial Framework to support EU-wide networks and innovative projects** that allow Member States to co-create and transfer good practices and innovative care models;
  - **The European Structural and Investment Funds must act as an accessible and adequate mechanism to effectively support Member States to up-scale successful and innovative pilots** into mainstream services. **Cross-border activities - e.g. EU-wide platforms - should be supported by funding programmes, within the European Structural and Investment Funds, that support cooperation across countries;**
  - **The European Semester must be used as a vehicle to devise and implement adequate social and employment policies** at a national level;
  - **The European Commission Steering Group on Health Promotion, Disease Prevention and Management of Non-Communicable diseases must embrace and respond to the challenges of people living with rare diseases and their carers.**
- ✓ **National and European bodies must allocate financial and structural support to ensure the sustainability of Europe-wide platforms that gather and share knowledge and good practices on rare diseases, such as the European Reference Networks, the European Network of Resource Centres for Rare Diseases and Orphanet.** An enabling environment should be created to integrate these initiatives with national health and welfare systems.

## 2. **Creating a supportive political environment at a national level for holistic care for rare diseases**

- ✓ **All National Plans and Strategies for rare diseases must include provisions to enable people living with a rare disease and their carers to access timely and adequate integrated health-social care, social services and social protection.** These should include mechanisms to support the national organisations for rare diseases, as civil society organisations engaged in design, implementation and monitoring of policies/services. When updated and evaluated, National Plans must undergo a social check;
- ✓ **European countries must implement specific mechanisms to guarantee coordination between national policy sectors within a multidisciplinary approach, engaging health, social, work, education and research Ministries.** Inter-Ministerial working groups and shared budgets between Ministries should be implemented;
- ✓ **Sustainability mechanisms must be put in place and be made accessible to public bodies, civil society organisations and service providers** to ensure the implementation of holistic care.

## 3. **Gathering and disseminating knowledge and good practices to ensure that the needs of people living with a rare disease and their carers are adequately addressed by specialised and mainstream services**

- ✓ **European countries must recognise and support existing rare disease Centres of Expertise, national reference networks, Resource Centres, organisations representing people living with a rare disease and Orphanet teams,** capitalising on their robust expertise and knowledge to improve care provided by specialised and mainstream services. These services must be adequately staffed and resourced to be able to fulfil their mission;
- ✓ **European Reference Networks and their constituent health care providers must continue to function as a platform to collect and disseminate data, good practices and guidance on health care and integrated care** for rare diseases, in cooperation with organisations representing people living with a rare disease;

- ✓ **Training for health and social service providers must be developed and delivered**, building on the expertise of rare disease specialised services and organisations representing people living with a rare disease. They should increase professionals' capacity and knowledge on the diseases, the rights of the people living with a rare disease and their carers, available resources and good care practices;
- ✓ **The EU and European countries must continue to support pilot projects** as generators of good practice and innovative services. They should be allowed time for their design, implementation and long-term impact assessment in order to gather quality evidence on their direct and societal impact;
- ✓ **Socio-economic research in the field of rare diseases should be supported at national and European levels** in order to support decision making on health, social and integrated care reforms.

#### 4. Implementing specific mechanisms that ensure integrated care provision for rare diseases

- ✓ **European countries must promote coordination and interoperability between all parties involved in the care provision**, including health, social and community services, as well as organisations representing people living with a rare disease and affected individuals/carers. **Coordination protocols, procedures, IT and e-health tools can be used for this purpose**;
- ✓ **Rare diseases and undiagnosed complex health conditions must be considered by the risk stratification tools used by health care systems to make decisions on the provision of integrated care**, via the use of implemented codification systems;
- ✓ **All people living with a rare disease must be entitled to an individual, person-centred care plan to be delivered within a multidisciplinary, holistic approach**, coordinated between all care providers;
- ✓ **Descriptions of National care pathways for rare diseases and undiagnosed conditions should be developed**, indicating the process and care steps to follow, identifying the existing coordinating mechanisms and the different care providers' responsibilities;
- ✓ **Case management, as an effective care coordination mechanism, should be implemented across European countries** to support care for people living with a rare disease, their carers and others with complex diseases/disabilities. **Training on case management for rare diseases should be developed** and the **case manager profession should be recognised** within the national codes of occupations.



*Philipp, osteogenesis imperfecta, Germany*

## 5. **Guaranteeing meaningful engagement of organisations and representatives of people living with a rare disease in the design and implementation of policies and services**

- ✓ Beneficiary engagement in the design, implementation and delivery of services must become a requisite for health and social care provision to guarantee person-centred, participative care;
- ✓ Tools which provide information and training must be available for representatives of people living with a rare disease in order to build their knowledge and capacity to take part in decision making on care provision;
- ✓ Rare disease organisations, as civil society organisations, must be supported and considered as equal partners in the design, implementation and monitoring of policies and services.

## 6. **Implementing specific measures that ensure access of people living with a rare disease and their carers to adequate social services and social protection**

- ✓ European countries must guarantee that all people living with a rare disease and their carers are entitled to have access to a social worker and to adequate social protection and social inclusion provisions, adapted to their individual needs and to the cost of living. These provisions must aim to support independent living whilst also covering the needs of severely affected individuals;
- ✓ Those with complex conditions for whom a diagnosis and/or a disability assessment are not available must not be deprived from accessing social rights and social protection;
- ✓ Existing specialised social services for rare diseases, such as Resource Centres, must be recognised and supported to act as a hub of expertise and good practice as well as a direct service and training provider for professionals in the health and social sector;
- ✓ European countries should also see organisations representing people living with a rare disease as suitable stakeholders to deliver care, as complementary providers to public services. When they provide social and support services, these organisations must be financially supported to undertake this role.

## 7. **Ensuring recognition and adequate compensation for the disabilities experienced by people living with a rare disease**

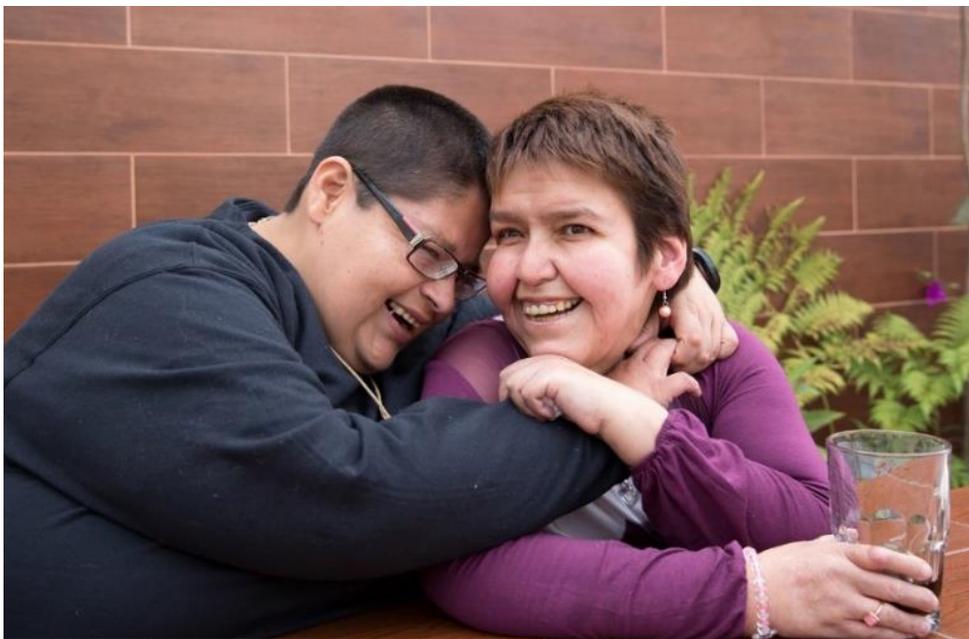
- ✓ The European Commission and European countries must implement the UN Convention on the Rights of Persons with Disabilities, taking into account the specific needs of people living with a rare disease;
- ✓ The European Commission must ensure that the needs of the rare disease community are taken into account within the EU Disability Strategy for 2020-2030;
- ✓ National and European stakeholders must strive to develop policies, procedures, services and technologies to build environments that aim at breaking barriers to participation in all areas of society, promoting autonomy and independent living;
- ✓ European countries must improve national disability assessment systems to ensure that there are no gaps in the integration of complex diseases causing disabilities. The functionality or incapacity of people living with a rare disease must be duly assessed and supported with adequate compensation measures;
- ✓ Existing tools and networks should be used to support the generation and dissemination of knowledge about the functional consequences of rare diseases. These include Orphanet's multi-lingual online tool and disability factsheets, European Reference Networks and the European Network of Resource Centres for Rare Diseases;
- ✓ National competent bodies should implement training for disability assessment teams on the functional consequences of rare diseases, in partnership with national alliances for rare diseases and with the networks mentioned above;
- ✓ To enable people living with a rare disease, and all those with disabilities, to live independently and participate fully in all aspects of life, European countries shall take appropriate measures to ensure

European countries must improve national disability assessment systems

their access, on an equal basis with others, to the physical environment, transportation, information, communications and to other facilities and services open or provided to the public.

## 8. **Creating the conditions for people living with a rare disease and their carers to access adapted and sustainable employment**

- ✓ Access to high quality education must be guaranteed to all people living with rare diseases and complex conditions. When necessary, adapted schooling should be accessible and delivered in a way that supports all individuals to reach their maximum potential;
- ✓ European countries, via the Work-Life Balance Directive and other means, must ensure that people with complex conditions/disabilities and their carers have the right to specific mechanisms that support their access and retention in the labour market:
  - Flexible work arrangements, such as flexible working hours and remote work;
  - Reasonable leave of absence due to their health/disability condition or caring responsibilities;
  - Tailor-made assistance to improve their employment or self-employment, such as career counselling to explore fulfilling professional avenues;
  - Reasonable accommodation in the workplace.
- ✓ When leaving the labour market or having to work part-time due to the disease, people living with a rare disease, their carers and others with complex conditions must have access to social protection measures, pension rights and care support that allows them to live a dignified life;
- ✓ People living with a rare disease and with disability, who wish to study and/or to be active as volunteers for civil society organisations, must in no way be deprived from their rights, including disability and retirement benefits;
- ✓ All EU and national level legislation must guarantee that there is no form of discrimination based on health or disability status, concerning all forms of employment, including recruitment, hiring, employment, career advancement and safe and healthy working conditions.



*Jorge and Maria, Prader-Willi syndrome, Mexico*

**9. Implementing specific mechanisms that empower people living with a rare disease and their carers, in co-creation and co-delivery with organisations representing people living with a rare disease**

- ✓ **Care providers should be prepared to give non-directive assistance**, providing beneficiaries with relevant information, tools and counselling as well as allowing beneficiaries to express their wishes, to participate in decisions regarding their care and to direct their own services if they wish to;
- ✓ **Tool-kits to navigate national health and welfare systems should be developed at a national level, specifically for rare diseases;**
- ✓ **Rare disease helpline services, when existing, must be supported. All European countries should strive to implement a rare disease helpline;**
- ✓ **People living with a rare disease and their carers must be entitled to access psychological support and respite care services, should they wish to. An annual mental health assessment should be put in place** to ensure monitoring and adequate intervention to avoid burnout and depression;
- ✓ **Opportunities to foster peer-to-peer support between people living with a rare disease should be available and supported.** These can include 'learning from each other' seminars organised by organisations representing people living with a rare disease and/or health and social stakeholders.

**10. Eliminating all types of discrimination to ensure that people living with a rare disease have access to social, labour, educational and leisure opportunities on an equal footing with other citizens**

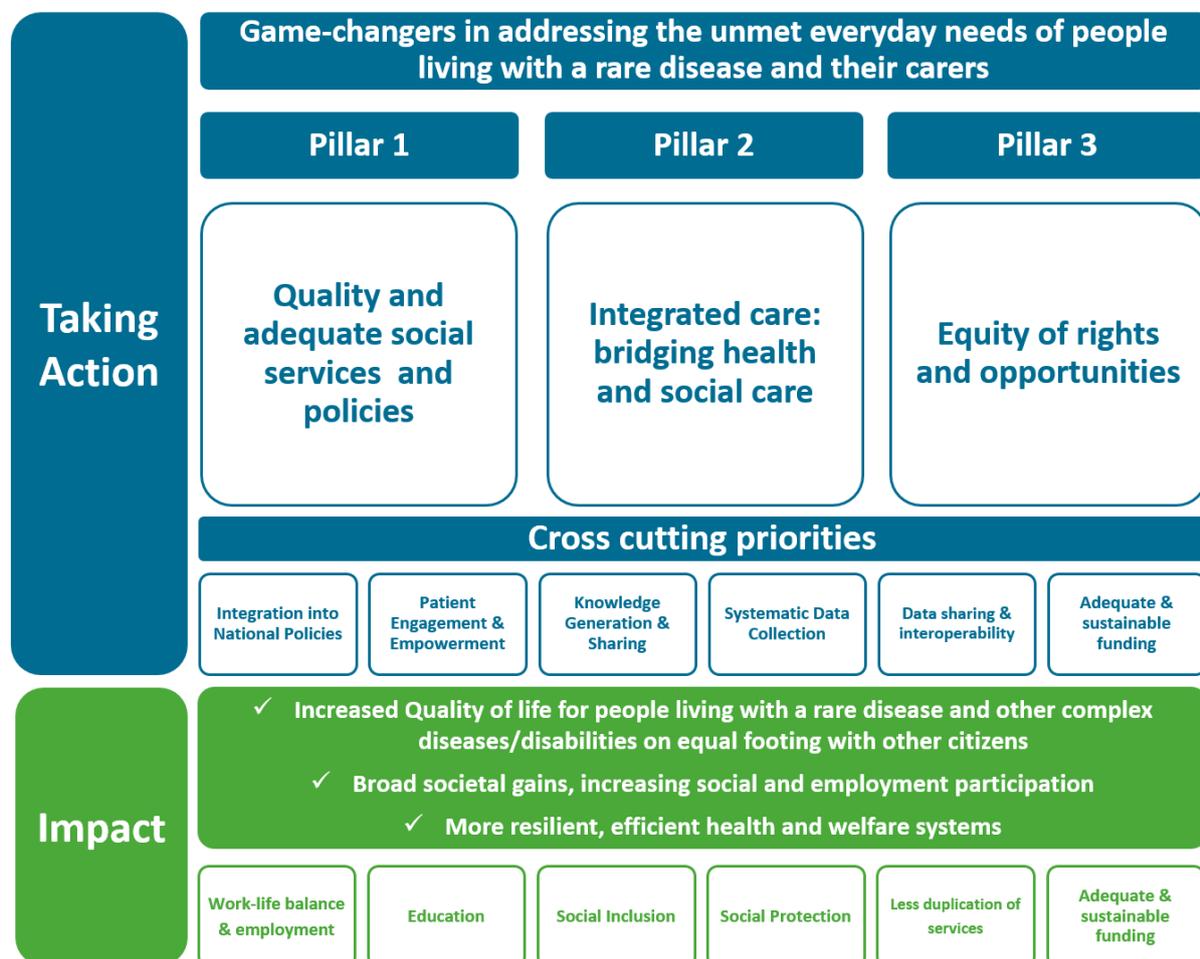
- ✓ **In line with the EU Charter of Fundamental Rights and the European Pillar of Social Rights (art 3), people living with a rare disease must not be discriminated against. They must have the right to equal treatment and opportunities regarding employment, social protection, education and access to all goods and services available to the public.**
- ✓ **All EU and national level legislation must guarantee that there is no form of discrimination based on health or disability status.**

## 5 A framework of strategies for a comprehensive approach to ensure holistic care for rare diseases

Addressing the unmet needs of people living with a rare disease and their carers requires a multi-sector approach from research, to diagnosis, to access to treatment, to health and social care, both at national and European levels. **All stakeholders have an instrumental role to play.**

This paper will focus on **three main pillars as the major game changers to guarantee holistic care for people living with a rare disease and their carers** (see image below). These were identified by EURORDIS through an extensive consultation process, involving people living with a rare disease and carers, their representative organisations, policy makers and service providers (see chapter "About this position paper", page 26).

A set crucial of crosscutting priorities has also been identified to enable the effective implementation of the pillars. These priorities, described briefly in this position paper (see chapter "Cross-cutting priorities", page 24), will often serve all areas of rare disease research, treatment and care.



## 5.1. Pillar 1: Quality and adequate social services and policies

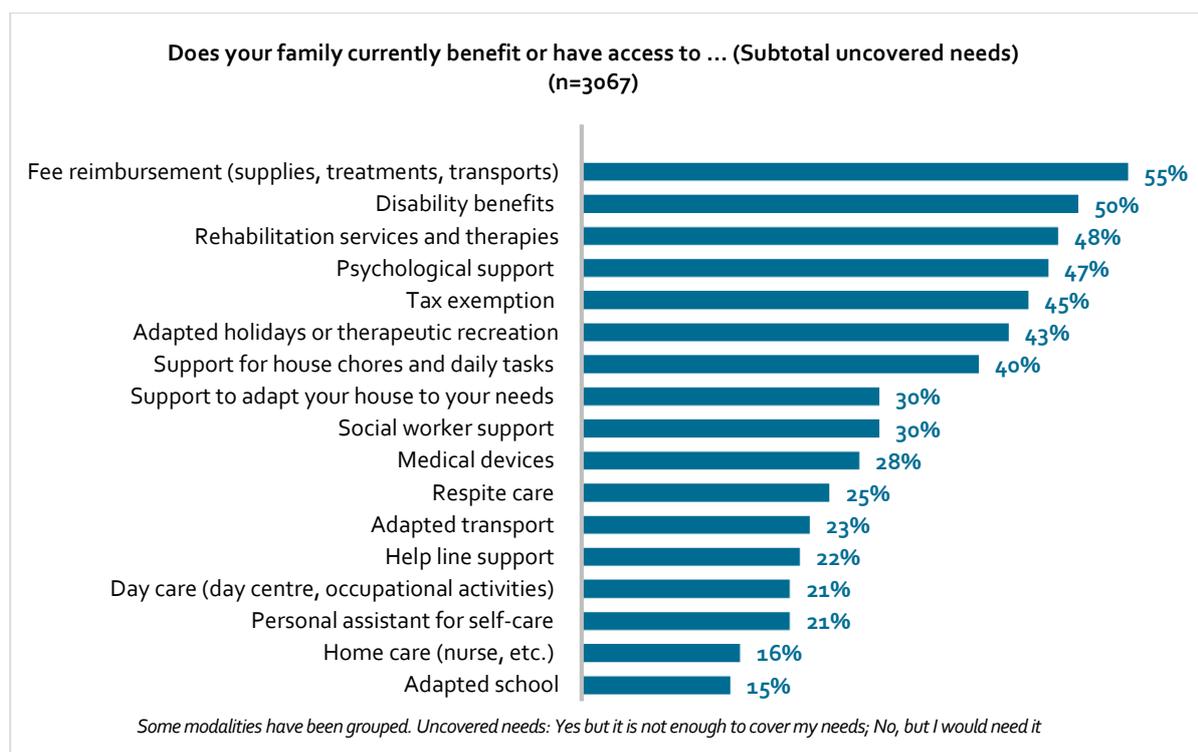
As stated in the Commission Expert Group on Rare Diseases Recommendations, **social services are vital to empower people with a rare disease and to improve their well-being and health**. Care for rare diseases should take into account social inclusion, psychological and educational development<sup>3</sup>.

In 2009, conclusions of a European survey revealed that **social security systems**, usually designed around common diseases, **are not flexible enough to take into consideration unprecedented health needs generated by rare diseases**<sup>10</sup>.

In the same year the **Council of the European Union recommended that European Member States (MS) gather national expertise on rare diseases** to support practices on diagnosis, medical care, education and social care in the field of rare diseases<sup>12</sup>.

Since then, **social policies and services have been a recurrent topic on the agenda of rare disease stakeholders, becoming a top priority for the majority of the national workshops** organised with the support of the European Joint-Action on Rare Diseases (RD-Action, 2015-2018) to aid the implementation of rare disease National Plans.

**Respondents to the first Europe-wide survey on the social impact of rare diseases have stated serious unmet needs regarding the access to a range of social and (re)habilitation services**<sup>8</sup>:



Access to the various social and support services and benefits may be hindered by the fact that **one third of people living with a rare disease have insufficient access to a social worker**: 22% of the respondents do not have access to a social worker and would need it, while 8% have access, but it is not enough to cover their needs.

In the absence of access to necessary social services and support, family members often have to take on the role of main carer attending to the social needs of their relative affected by a rare disease:

*I have had a full time job of supporting my children in daily tasks, accessing social educational and leisure activities, training them for many independent living tasks etc. Female carer, United Kingdom*

This evidence demonstrates that **efforts are yet to be made to ensure that**, as recommended by the Commission Expert Group on Rare Diseases, **the specificities of rare diseases are incorporated into mainstream social services within a holistic, person-centred approach with a human rights perspective**<sup>3</sup>.

**Specific provisions must be put in place to ensure that integration into mainstream social services and social protection happens effectively, guaranteeing that people living with rare diseases and their carers are effectively granted adequate standards of living, social protection and inclusion, as set by the European Pillar of Social Rights**<sup>d</sup>.

**Services such as adapted holidays, adapted housing, respite care, day care, personal assistance and adapted school are in no means superfluous.** By contributing to increase health and wellbeing, they **directly and substantially increase the quality of life and social inclusion** of people living with a rare disease, their carers and others with complex conditions/disabilities.

As a complement to mainstream services, in some European countries there are **specialised social services for people** living with a rare disease such as resource centres for rare diseases, respite services, therapeutic recreation programmes and adapted housing facilities. Wherever these exist, they **have proven to be an important support to people living with a rare disease, increasing their well-being as well as their social and employment integration.**



*Emily and Mia, biliary atresia, duodenal atresia, esophageal atresia (Long Gap/Type II), exocrine pancreatic insufficiency, Germany*

<sup>d</sup> The European Pillar of Social Rights, endorsed by the Heads of States, the European Commission and the European Parliament in 2017, is structured in three key priorities: equal opportunities and access to the labour market, fair working conditions, social protection and inclusion. This is now the reference framework to steer social and employment reforms at national level within the euro area. More information on the European Pillar of Social Rights is available here: [https://ec.europa.eu/commission/priorities/deeper-and-fairer-economic-and-monetary-union/european-pillar-social-rights\\_en](https://ec.europa.eu/commission/priorities/deeper-and-fairer-economic-and-monetary-union/european-pillar-social-rights_en).

## Our recommendations

### Implementing specific measures that ensure access of people living with a rare disease and their carers to adequate social services and social protection

- ✓ All legislative proposals and recommendations deriving from the European Pillar of Social Rights must take into account the specific needs of people living with a rare disease, their carers and others with complex diseases/disabilities; the 'Social Scoreboard' should introduce clear indicators and monitoring tools to support effective policy changes;
- ✓ European countries must guarantee that all people living with a rare disease and their carers are entitled to have access to a social worker and adequate social protection and social inclusion provisions, adapted to their individual needs and to the cost of living. These provisions must aim to support independent living whilst also covering the needs of severely affected individuals;
- ✓ Those with complex conditions for whom a diagnosis and/or a disability assessment are not available must not be deprived from accessing social rights and social protection;
- ✓ Existing specialised social services for rare diseases, such as Resource Centres, must be recognised and supported to act as a hub of expertise and good practice as well as a direct service provider and training provider for professionals in the health and social sector;
- ✓ European countries should also see organisations representing people living with a rare disease as suitable stakeholders to deliver care, as complementary providers to public services. When they do provide social and support services, these organisations must be financially supported to undertake this role.

## 5.2. Pillar 2: Integrated care: bridging health and social care

People living with a rare disease need follow-up care and support from different categories of health professionals, often from several different medical specialities, as well as from social workers and other social and local service providers<sup>3</sup>. These may also include (re)habilitation, day-care, home care, personal assistants, respite services, adapted schools and work places, psychological support and social prescribing, among others<sup>9</sup>.

The combination of rarity, complexity and lack of effective treatment brings about additional challenges in the provision of holistic care as mainstream services are not flexible enough to take into consideration unprecedented needs<sup>10</sup>. In many cases significant medical, psychological and social needs remain unmet.

Across European countries, care pathways are fragmented so obtaining the correct diagnosis, needed social care and support to manage the transitions between hospital and home and between childhood and adulthood remains a challenge<sup>13</sup>.

Evidence from the Europe-wide survey on the everyday impact of rare diseases<sup>8</sup> demonstrates how people living with a rare disease and their carers indeed face complex pathways:



The care pathway complexity is also confirmed by other studies, such as the study “Only Strong Survive”, carried out in Denmark, in which people living with a rare disease reported having been referred to between 10 and 30 social and healthcare professionals as contact points<sup>14</sup>. These affected individuals and carers also reported having to spend an average of 25 hours per month in contact with health and social professionals.

The lack of coordination between care providers is often described as one of the main challenges, forcing the affected person or their carer to take on the challenging and time-consuming role of care coordinator:

*Inter-professional communication works only through the good intentions and efforts of particular professional individuals. This is one of the main difficulties in the lives of families with disabilities. Today, departments communicate with each other primarily through patients themselves or their parents. However, this only works for very dedicated people. Not everyone is able or willing to carry this out. Male, Czech Republic<sup>8</sup>*

Integrated care, within the health system and between health, social and community services is essential to enable people living with a rare disease to overcome their care challenges and to secure the services and support that they need. This will thus allow them to achieve a quality of life on equal footing with other citizens and to increase their participation in society and in the job market<sup>15</sup>. Recent studies indeed demonstrate that integrated care is especially beneficial for people with complex needs<sup>16</sup>.

The Commission Expert Group on Rare Diseases recommends that Member States promote measures that facilitate multidisciplinary, holistic, continuous, person-centred and participative care provision to people living with a rare disease, supporting them in the full realisation of their fundamental human rights<sup>3</sup>.

The pilot of case management for rare diseases implemented within the EU-funded INNOVCare project (Romania, 2017-2018, with a total duration of 18 months) resulted in various positive outcomes within important daily life and care areas. People living with a rare disease and carers who benefited from the service increased their level of information about their disease, their rights and available services. Their capacity to manage their care also increased, while the service also reduced the burden faced by caregivers (assessed via the Zarit Caregiver Burden scale)<sup>37</sup>. Furthermore, the case management provision contributed to improvements for care providers and public bodies, as it enhanced the coordination amongst stakeholders involved in the care provision.

Additional research done within the INNOVCare project identified key priorities that European countries need to address to ensure integrated health and social care for rare diseases. These included collaboration and coordination at structural policy level, multidisciplinary care coordination and empowerment of people living with the disease and their carers.

In view of supporting the implementation of the Commission Expert Group Recommendations, the INNOVCare project (2015-2018) and the European Joint Action on Rare Diseases (RD-Action, 2015-2018) organised a workshop with representatives of all stakeholders in 2018. The role of various stakeholders in supporting integrated care for rare diseases was highlighted as an outcome of this workshop:

- **Centres of Expertise and Resources centres for rare diseases** have a critical role in facilitating integrated care provision at a national level as service providers and expert services connected to European networks. They are able to disseminate information on the diseases, available resources and good care practices, and can act as training providers for other professionals at national level;
- **European Reference Networks and the European Network of Resource Centres for Rare Diseases**, with their critical mass of people living with a rare disease and experts, represent an invaluable resource to gather information on the diseases and their consequences as well as on good practices for multidisciplinary care;
- **Other European-wide platforms** - e.g. the European Commission Steering Group on Health Promotion, Disease Prevention and Management of Non-Communicable Diseases – are of the utmost importance to ensure that services for rare diseases are aligned with mainstream health and social reforms. They should guarantee that relevant practices from the rare disease field are transferred to other care areas and across European countries.

The INNOVCare project and the European Joint-Action on Rare Diseases provided guidance and good practice to support the implementation of holistic and integrated care for rare diseases. **However, much more needs to be done to ensure that integrated health and social care for people living with a rare disease and their carers becomes a reality.**



*Benjamin, neurofibromatosis type 1, France*

## Our recommendations

### Creating a supportive political environment at a national level for holistic care for rare diseases

- ✓ **All National Plans and Strategies for rare diseases must include provisions to enable people living with a rare disease and their carers to access timely and adequate integrated health-social care, social services and social protection.** These should include mechanisms to support the national organisations for rare diseases, as civil society organisations engaged in design, implementation and monitoring of policies/services. When updated and evaluated, National Plans must undergo a social check;
- ✓ **European countries must implement specific mechanisms to guarantee coordination between national policy sectors within a multidisciplinary approach, engaging health, social, work, education and research Ministries. Inter-Ministerial working groups and shared budgets between Ministries should be implemented;**
- ✓ **Sustainability mechanisms must be put in place and be made accessible to public bodies, civil society organisations and service providers to ensure the implementation of holistic care.**

### Gathering and disseminating knowledge and good practices to ensure that the needs of people living with a rare disease and their carers are adequately addressed by specialised and mainstream services

- ✓ **European countries must recognise and support existing rare disease Centres of Expertise, national reference networks, Resource Centres, organisations representing people living with a rare disease and Orphanet teams,** capitalising on their robust expertise and knowledge to improve care provided by specialised and mainstream services. These services must be adequately staffed and resourced to be able to fulfil their mission;
- ✓ **European Reference Networks and their constituent health care providers must continue to function as a platform to collect and disseminate data, good practices and guidance on health care and integrated care** for rare diseases, in cooperation with organisations representing people living with a rare disease;
- ✓ **Training for health and social service providers must be developed and delivered,** building on the expertise of rare disease specialised services and organisations representing people living with a rare disease. They should increase professionals' capacity and knowledge on the diseases, the rights of the people living with a rare disease and their carers, available resources and good care practices;
- ✓ **The EU and European countries must continue to support pilot projects,** as generators of good practice and innovative services. They should be allowed time for their design, implementation and long-term impact assessment in order to gather quality evidence on their direct and societal impact;
- ✓ **Socio-economic research in the field of rare diseases should be supported at national and European levels** in order to support decision making on health, social and integrated care reforms.

### Implementing specific mechanisms that ensure integrated care provision for rare diseases

- ✓ **European countries must promote coordination and interoperability between all parties involved in the care provision,** including health, social and community services, as well as organisations representing people living with a rare disease and affected individuals/carers. **Coordination protocols, procedures, IT and e-health tools can be used for this purpose;**
- ✓ **Rare diseases and undiagnosed complex health conditions must be considered by the risk stratification tools used by health care systems to make decisions on the provision of integrated care,** via the use of implemented codification systems;
- ✓ **All people living with a rare disease must be entitled to an individual, person-centred care plan, to be delivered within a multidisciplinary, holistic approach** in coordination between all care providers;

- ✓ **National care pathways descriptions for rare diseases and for undiagnosed conditions should be developed**, indicating the process and care steps to follow, identifying the existing coordinating mechanisms and the different care providers' responsibilities;
- ✓ **Case management, as an effective care coordination mechanism, should be implemented across European countries** to support care for people living with a rare disease, their carers and others with complex diseases/disabilities. **Training on case management for rare diseases should be developed** and the **case manager profession should be recognised** within the national codes of occupations.



*Daniel and Ben, Tay-Sachs disease, B variant, infantile form*

## 5.3. Pillar 3: Equity of rights and opportunities

Equity of rights and opportunities span over a large range of social inclusion spheres including access to social services and social protection, education, work, leisure and built environments. Denial of equal access to these is a human rights breach that hinders social inclusion.

As stated by the Commission Expert Group on Rare Diseases, failure to meet the serious unmet social needs of people with a rare disease and their families affects their dignity, autonomy and other fundamental human rights expressed in the Universal Declaration of Human Rights and in the United Nations Convention on the Rights of Persons with Disabilities (UNCRPD)<sup>3</sup>.

As a Party to the UNCRPD, the EU has a legal obligation to promote, protect and ensure the full enjoyment of human rights by individuals with complex needs and disabilities. Other European and International binding instruments protect the right of access to health and social care, as well as the right of being protected from discrimination. These include the EU Treaty, the European Charter Fundamental Rights, the EU Disability Strategy and the UN Sustainable Development Goals (SDGs).

Regrettably, these dispositions are still far from being put in practice and responding to the situation. Today, a vast majority of people with a rare disease face challenges in social inclusion and are discriminated against.

### 5.3.1. Disability, autonomy, accessibility

The World Health Organisation defines disability as an umbrella term, covering impairments (problems in body function or structure), activity limitations (difficulties in executing a task or action), and participation restrictions (problems experienced by an individual in involvement in life situations)<sup>18</sup>. Everyone can be placed in a continuum of functioning and can be vulnerable to experiencing disability over the course of their lives<sup>19</sup>.

Most people living with a rare disease are indeed living with disability, which is presented in the form of impairments, activity limitations or participation restriction - and often as an accumulation of all these disabling factors.

A high percentage of people with a rare disease are affected by motor, sensorineural or intellectual impairments, which can occur simultaneously<sup>3 20</sup>. 72% of people living with a rare disease involved in EURORDIS' recent European survey on the impact of rare diseases on daily life, declared having difficulties with motor or sensorial functioning<sup>8</sup>.

According to the same survey, people living with a rare disease face serious limitations in their Activities of Daily Living (ADLs):



**These limitations and impairments, as well as other consequences of the disease on their health and wellbeing, are not always visible and symptoms can vary over time:**

*The difficulty lies in the impossibility of carrying a routine (...). The problem arises when one day you appear completely healthy, the next day you are sick, and two days later you appear completely normal again. Many people find it difficult to understand the disease and the process, and the absenteeism that entails<sup>8</sup>. Female, Spain*

As a consequence of their impairments and limitations in Activities of Daily Living, **people living with a rare disease find it difficult to have a routine and they face misunderstanding about the disease across all contexts of their everyday life and social inclusion**, including at school and at work. **This can seriously affect their autonomy.**

Recognition of their disability is the main challenge for people living with a rare disease: **34% of the respondents who have been submitted to a disability assessment find the percentage of disability assigned to them too low**, and **19% have not been submitted to a disability assessment despite feeling that they need to.**

*I am only 51 but able to do much less than many 75 year olds, but I would not qualify for any help from government benefits because the questions they asked don't capture the impact of my condition. I don't look ill but I am very ill, with a condition which no one understands or has heard of, so I get no sympathy<sup>8</sup>. Female, United Kingdom*

The challenge of obtaining a disability assessment affects timely access to tailored support services. People living with a rare disease are often prevented from accessing disability benefits. **50% of the survey respondents have uncovered needs regarding the access to disability benefits:** 28% of the respondents have access to disability benefits but find that they are not enough to cover their needs; while 22% do not have access to these benefits but consider that they would need to.

The **United Nations Special Rapporteur on the Rights of Persons with Disabilities**, Catalina Devandas Aguilar, in her Report to the United Nations General Assembly of 16 July 2018, **acknowledged that people living with rare diseases are living with disability and encouraged States to take action to address their needs:** "Health systems must respond to the needs of the diversity of persons with disabilities. States need to pay attention to the existing layers of identities within the disability community (...). **States should consider developing and implementing policies and practices targeting the most marginalized groups of persons with disabilities (e.g. persons with multiple or severe impairments, rare diseases or deaf-blindness) in order to accelerate or achieve de facto equality in access to health care.**"<sup>21</sup>.

The Commission Expert Group on Rare Diseases recommends to Member States that **rare diseases specificities should be integrated into national systems when assessing a person's level of functioning, in line with the United Nations Convention on the Rights of Persons with Disabilities<sup>3</sup>.**

The reference portal for rare diseases in Europe, Orphanet, has been developing important tools to support the assessment and recognition of the disabilities generated by rare diseases. These include an online multi-language tool listing all the disabling aspects of each rare disease, in line with the International Classification of Functioning and Disability, as well as a series of factsheets explaining the practical and functional consequences of each rare disease and how to best manage them. **These tools are crucial to support the integration of the specificities of rare diseases into national disability assessment procedures and to build the capacity of professionals to deliver high quality person-centred care.**

**Organisations representing people living with a rare disease have a key role to play in sharing information with disability assessment services. Where they have started to pro-actively engage and train professionals involved in disability assessments, improvements have been steadily achieved** (e.g. in Spain, with an initiative led by the national alliance for rare diseases – FEDER).

## Our recommendations

### Ensuring recognition and adequate compensation for the disabilities experienced by people living with a rare disease

- ✓ The European Commission and European countries must implement the UN Convention on the Rights of Persons with Disabilities, taking into account the specific needs of people living with a rare disease;
- ✓ The European Commission must ensure that the needs of the rare disease community are taken into account within the EU Disability Strategy for 2020-2030;
- ✓ National and European stakeholders must strive to develop policies, procedures, services, technologies and build environments that aim at breaking barriers to participation in all areas of society, promoting autonomy and independent living;
- ✓ European countries must improve national disability assessment systems to ensure that there are no gaps in the integration of complex diseases causing disabilities. The functionality/incapacity of people living with a rare disease must be duly assessed and supported with adequate compensation measures;
- ✓ Existing tools and networks should be used to support the generation and dissemination of knowledge on the functional consequences of rare diseases. These include Orphanet's multi-lingual online tool and disability factsheets, European Reference Networks and the European Network of Resource Centres for Rare Diseases;
- ✓ National competent bodies should implement training for disability assessment teams on the functional consequences of rare diseases, in partnership with national alliances for rare diseases and with the networks mentioned above;
- ✓ To enable people living with a rare disease, and all people with disabilities, to live independently and to participate fully in all aspects of life, European countries shall take appropriate measures to ensure their access, on an equal basis with others, to the physical environment, transportation, information, communications and to other facilities and services open or provided to the public.



Alec, Sanfilippo syndrome, Australia

## 5.3.2. Adapted and sustainable employment

**Employment, as a vehicle for social inclusion and economic independence, is highly important for people living with a rare disease and their carers. Their integration and participation in the labour market is fully in line with the UNCRPD, the EU Charter of Fundamental Rights and the UN Sustainable Development Goals.**

Due to therapeutic and healthcare advances, people living with a rare disease have a longer life expectancy, higher functioning and greater expectations towards an autonomous and fulfilling life. They are **eager to work and often have the capacity to do so, provided the workplace and work schedule are adapted to the specific challenges generated by their condition, care and treatment pathway.** Equally, their carers need tailored support to allow them to fulfil their caring role whilst remaining in employment.

**Today, people living with a rare disease and their carers have serious challenges with access, retention and return to employment, bringing about devastating socio-economic consequences.**

Respondents to the European survey by EURORDIS<sup>8</sup> face significant employment challenges: **70% had to stop or reduce professional activity while 58% were absent from work 15 days in year prior to the survey.**

The consequences are aggravated by the fact that those who face unemployment may remain **out of the labour market for several years while awaiting a diagnosis or while taking on the role of care coordinator:**

*We were looking for a diagnosis (which took roughly 6 years). By asking a year "non paid break" - to pursue my diagnosis journey and to take care of my children, one of them also having similar symptoms - I received a refusal from the employer. So I decided to give up my job and stayed unemployed for 4 years. Feeling stronger after the diagnosis and the information about the disease (which my husband I and I sought by ourselves), I decided to pick up work again. One year: part-time and thus earning half as much as before. Later on and until now: working less than a part time and earning even less; but the job is my dream job and I still have a husband who is the main "breadwinner". That's how we manage to make ends meet<sup>8</sup>.*  
Female, Luxembourg

Furthermore, **having a rare disease limited the professional choices of 76% of the respondents, while 67% felt that it limited their opportunities to progress in their career (67%).**

Limited employment opportunities, coupled with the challenges in access to health and social care, leave people living with a rare disease and their carers in precarious situations as they struggle with **economic vulnerabilities: 69% of the survey respondents suffered a decrease in income due to their disease.**

**Access to adequate education and training is a pre-requisite for optimal integration in the labour market. Unfortunately, this can be limited for people living with a rare disease, whereby narrowing their chances to join the labour market, thus adding to the discrimination already faced in other areas. 24% of the survey respondents with school-age children declared that the schooling provided for their child was badly adapted. Additionally, 46% declared that their children were absent from school over 20 days per year due to their disease and care pathway.**

**70% of people living with a rare disease had to reduce or stop professional activity**

The European Commission's proposal for a **Work-Life Balance Directive** offers an opportunity to **meaningfully address the challenges of people living with a rare disease and their carers in the labour market, ensuring them the flexibility and protection needed to allow them to combine their care needs and responsibilities with an active professional life.**

**Supporting people living with a rare disease and their carers to access and retain employment will undoubtedly bring about many benefits for employees, employers, the economy and society at large.**

## Our recommendations

### Creating the conditions for people living with a rare disease and their carers to access adapted and sustainable employment

- ✓ Access to high quality education must be guaranteed to all people living with rare diseases and complex conditions. When necessary, adapted schooling should be accessible and delivered in a way that supports all individuals to reach their maximum potential;
- ✓ European countries, via the Work-Life Balance Directive and other means, must ensure that people with complex conditions/disabilities and their carers have the right to specific mechanisms that support their access and retention in the labour market:
  - Flexible work arrangements, such as flexible working hours and remote work;
  - Reasonable leave of absence due to their health/disability condition or caring responsibilities;
  - Tailor-made assistance to improve their employment or self-employment, such as career counselling to explore fulfilling professional avenues;
  - Reasonable accommodation in the workplace.
- ✓ When leaving the labour market or having to work part-time due to the disease, people living with a rare disease, their carers and others with complex conditions must have access to social protection measures, pension rights and care support that allows them to live a dignified life;
- ✓ People living with a rare disease and with disability, who wish to study and/or to be active as volunteers for civil society organisations, must in no way be deprived from their rights, including disability and retirement benefits;
- ✓ All EU and national level legislation must guarantee that there is no form of discrimination based on health or disability status, concerning all forms of employment, including recruitment, hiring, employment, career advancement and safe and healthy working conditions.



*Brian and Swen, congenital melanocytic nevus, The Netherlands*

### 5.3.3. Non-discrimination: access to equal opportunities

Although **people living with a rare disease** may have difficulties maintaining a routine or may experience episodes of disrupted activities due to the disease, most of them **have the capacity to fully participate in society, as long as a supportive environment is put in place.**

However, mainstream health services, social services, employers, education institutions and society at large still have a limited level of awareness in relation to rare diseases and their consequences:

*Many people find it difficult to understand the disease and the process, and the absenteeism that entails*<sup>8</sup>.  
Female, Spain

As a consequence, **people living with a rare disease often face discrimination and stigma based on their health status or chronic condition, experiencing unequal treatment in a number of areas** ranging from access to education, to employment, to leisure and to other essential support services. This is aggravated when the illness is invisible or changing<sup>22</sup>, which is the case for many people living with a rare disease.

The European survey conducted by EURORDIS has demonstrated, for example, that the rare **disease limits opportunities to get a job (67%) and the possibilities of being promoted (60%)**<sup>8</sup>. Respondents felt that employers were not willing to consider them during recruitment procedures

*I felt that employers are "afraid" to employ me, but this was officially never the reason. It took me 8 years to get a new part-time job.* Male, Poland

*Every time my disability got more obvious, the look of "oh, you are wasting my time" when I went to an interview got more obvious*<sup>8</sup>. Other gender, United Kingdom

**For 27% of respondents, the disease also prevented them from accessing higher education**<sup>8</sup>. Similarly, participants in a rare disease survey in Spain stated that they **felt discriminated in education (30%) and in the labour market (32%), either when searching for a job (17%) or in their current job (15%)**<sup>23</sup>.

Young people living with chronic conditions consulted within the scope of the European Patients' Forum EMPATHY project (2013)<sup>24</sup> identified **discrimination and stigma in education and the workplace as important obstacles**, and highlighted the broad stigma that they faced in everyday life. One participant said: "I think that this is an issue faced by many individuals with chronic conditions as they are constantly pitied, babied or made to feel bad about themselves because of their condition". Similar situations often occur to people living with a rare disease.

**Discrimination also takes place within healthcare services.** In a European survey to people living with Spina Bifida and Hydrocephalus, **32% of the respondents declared that they felt discriminated against when accessing general healthcare.** Attitudes experienced by the respondents included ignorance (42%), prejudice and stigma (31%) and disrespect (27%)<sup>25</sup>.

**It is crucial to raise awareness of rare diseases and to ensure that no form of discrimination based on health or disability status takes place in any setting.**



Lian, LMNA muscular dystrophy, South Africa

## Our recommendations

**Eliminating all types of discrimination, ensuring that people living with a rare disease have access to social, labour, educational and leisure inclusion on an equal footing with other citizens**

- ✓ In line with the EU Charter of Fundamental Rights and with the European Pillar of Social Rights (art 3), people living with a rare disease must not be discriminated against. They must have the right to equal treatment and opportunities regarding employment, social protection, education and access to goods and services available to the public.
- ✓ All EU and national level legislation must guarantee that there is no form of discrimination based on health or disability status.



*Laura, arthrogryposis multiplex congenital, Spain*

## 5.4. Crosscutting priorities

A set of a cross-cutting measures has been identified as necessary to achieve the effective improvement of care and support for people living with a rare disease across the 3 pillars. These are structural measures that, if effectively implemented, will benefit people living with a rare disease in all their challenges, from diagnosis to access to treatment, from health to social care and, very importantly, to holistic person-centred care.

- ✓ **Integration of specific measures to address the everyday needs of people living with a rare disease and their carers into National Plans for rare diseases and national mainstream policies;**
- ✓ **Engagement of people living with a rare disease and their carers in the design, implementation and evaluation of services and policies;**
- ✓ **Establishment of specific measures to support the empowerment of people living with a rare disease and their carers;**
- ✓ **Knowledge generation and sharing across European countries and within national contexts, across stakeholders and sectors, in cooperation with people living with a rare disease, their carers and their representative organisations;**
- ✓ **Training of health and social care professionals, as part of their university courses as well as within their professional continuous training, to increase their capacity and knowledge on the diseases, the rights of the people living with a rare disease and their carers, available resources and good care practices;**
- ✓ **Training people living with a rare disease, building their capacities and knowledge to manage their own care and their self-confidence to increase their participation in society;**
- ✓ **Systematic data collection, via social research, registries, portals and other data collection methods, in order to build a repertoire of both the unmet needs of people living with a rare disease as well as of successful good practices and services;**
- ✓ **Data sharing and interoperability, across sectors and professionals, and allowing the person living with a rare disease to access and manage their own care record;**
- ✓ **Adequate and sustainable funding at a national and EU-level to guarantee that essential policies, services, structures and networks are effectively implemented and sustainable.**



*Raife and Eddison, Xeroderma pigmentosum, United Kingdom*

## 6 Conclusion: the time to act is now

The ambition of EURORDIS is to have holistic care provided to the 30 million people living with a rare disease in Europe, and their families, by 2030.

Our ambition is to see people living with a rare disease and their carers integrated in a society that leaves no one behind.

To achieve this, it is urgent to address the serious unmet every day and social needs of people living with a rare disease and their carers.

All stakeholders, working together, have the capacity to advance this change, co-creating the necessary strategies, policies, knowledge, tools and sustainability frameworks to guarantee holistic care for rare diseases.

EURORDIS and its members thus call upon the EU and all European Countries to ensure that these recommendations are incorporated into and effectively implemented via important frameworks and means at European and national levels:

- All legislative proposals deriving from the European Pillar of Social Rights must take into account the specific needs of people living with a rare disease, their carers and others with complex diseases/disabilities;
- Available EU mechanisms must support the implementation of social services and integrated care for people living with a rare disease, their carers and others with complex diseases/disabilities:
  - Funding lines and programmes should be secured under the Multiannual Financial Framework to support EU-wide networks and innovative projects that allow Member States to co-create and transfer good practices and innovative care models;
  - The European Structural and Investment Funds must act as an accessible and adequate mechanism to effectively support Member States to up-scale successful and innovative pilots into mainstream services. Cross-border activities - e.g. EU-wide platforms - should be supported by funding programmes, within the European Structural and Investment Funds, that support cooperation across countries;
  - The Participatory Foresight in Rare Disease Policy (Rare 2030) must take into account this paper's recommendations when building scenarios to support future policy decisions on rare diseases.
- European Reference Networks, and their constituent health care providers, must continue to function as a platform to collect and disseminate data, good practices and guidance on health care and on integrated care for rare diseases, in cooperation with organisations representing people living with a rare disease;
- All National Plans and Strategies for rare diseases must include provisions to enable people living with a rare disease and their carers to access timely and adequate integrated health-social care, social services and social protection.

These important frameworks are crucial. But alone, they will not suffice. More action is required by all stakeholders to ensure that European countries effectively develop and implement policies and services that guarantee holistic care for rare diseases.

EURORDIS and its members call upon all stakeholders, within the health and social sector, to disseminate this paper and to take action based on the recommendations put forward in this paper.

Only together will we ensure that no one of the millions of Europeans living with a rare disease is left behind. The time to act is now.

## 7 About this position paper

This position paper results from the consultation of people living with a rare disease and their carers, EURORDIS members, volunteers and team through a series of initiatives aimed at identifying and addressing the everyday unmet needs of people living with a rare disease. These initiatives included:

- European-wide surveys conducted by EURORDIS: "The Voice of 12,000 Patients. Experiences and expectations of rare disease patients on diagnosis and care in Europe" (2009); "Juggling care and daily life: The balancing act of the rare disease community" (3000 respondents, conducted via EURORDIS survey initiative Rare Barometer Voices) (2017);
- Consultations involving EURORDIS volunteers and members: Workshops at EURORDIS Membership Meetings 2014 Berlin and 2017 Budapest; Meetings of the Councils of National Alliances and of European Federations (2017-2018); Webinars with EURORDIS members and volunteers (2017-2018); Consultations with EURORDIS Social Policy Advisory Group, EURORDIS Board of Directors and EURORDIS European Public Affairs Committee (2017-2018);
- EURORDIS' support in the elaboration and implementation of policy documents: Commission Expert Group Recommendations to Support the Incorporation of rare diseases into Social Policies and Services (2016); EURORDIS' contribution to European Commission consultation on the European Pillar of Social Rights (2016);
- EURORDIS' coordination of scientific publications: "Rare diseases", integrated in the Handbook Integrated care (2017); "Bridging the gap between health and social care for rare diseases: key issues and innovative solutions (2017);
- EURORDIS' participation in EU-funded projects: European Joint-Actions on Rare Diseases: EUCERD Joint-Action (2012-2015) and RD-Action (2015-2018); INNOVCare, on integrated care for rare diseases and complex conditions (2015-2018).

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- 3,000 people living with a rare disease and carers who responded to the first EU-wide survey on the everyday impact of rare diseases, conducted via EURORDIS survey initiative Rare Barometer Voices;
- EURORDIS [Social Policy Advisory Group](#);
- EURORDIS Board of Directors;
- National Alliances and European Federations for Rare Diseases;
- EURORDIS members at large;
- Partners and stakeholders involved in the EU-funded INNOVCare project and in the European Network of Resource Centres for Rare Diseases (RareResourceNet);
- Partners and stakeholders involved in the European Joint-Actions for Rare Diseases, in the Commission Expert Group on Rare Diseases and in the European Reference Networks;
- The European Commission, AFM Téléthon, as well as all the public and private funders who supported EURORDIS activities in the social field and EURORDIS surveys over the last 10 years.

## 8 Glossary

**Case management:** person who assists the planning and coordination of care. The role of case managers per rare diseases includes: being a central point of contact on care issues; planning and coordinating holistic, person-centred care by supporting the design and implementation of individual care plans together with the beneficiary and his/her family and in coordination with the different care providers; informing, supporting and empowering beneficiaries and professionals. <https://innovcare.eu/case-managers-rare-diseases-roles-training-outlines/>

**Centres of expertise:** healthcare units highly-specialised in the management and care of people living with a rare disease, which aim at providing the highest standards of care to deliver timely diagnosis, appropriate treatments, and follow up. Each Centre of Expertise is specialised in a single rare disease or in a group of rare diseases. [https://www.eurordis.org/sites/default/files/publications/factsheet\\_Centres\\_Expertise.pdf](https://www.eurordis.org/sites/default/files/publications/factsheet_Centres_Expertise.pdf)

**EU Committee of Experts on Rare Diseases (EUCERD, 2010-2013) and Commission Expert Group on Rare Diseases (2013-2016):** expert groups established to support EU policy on rare diseases, notably to provide policy guidance on the effective implementation of the 2008 EU Commission Communication on Rare Diseases: Europe's challenges and the 2009 Council Recommendation on action in the field of rare diseases. The work of these expert groups was supported by the two EU Joint Actions for rare diseases, the EUCERD Joint Action (2012- 015) and RD-ACTION (2015-2018) and brought together representatives from: all EU Member States, Iceland, Norway, Switzerland, European Commission, Committee for Orphan Medicinal Products of the EMA, industry, academia, individual experts and representatives of people living with a rare disease. <https://www.eurordis.org/content/new-eu-committee-experts-rare-diseases>

**European Reference Networks (ERNs):** virtual networks involving healthcare providers across Europe. ERNs aim at facilitating discussion on complex or rare diseases and conditions that require highly specialised treatment, concentrated knowledge and resources. The first ERNs, launched in March 2017, involve more than 900 highly-specialised healthcare units from over 300 hospitals in 26 EU countries. These 24 ERNs are working on a range of thematic issues including bone disorders, childhood cancer and immunodeficiency. The process and criteria for establishing an ERN and for selecting its members are set in EU legislation. [https://ec.europa.eu/health/ern\\_en](https://ec.europa.eu/health/ern_en)

**Integrated care:** set of coherent methods and models on the funding, administrative, organizational, service delivery and clinical levels designed to create connectivity, alignment and collaboration within and between the cure and care sectors. The goal of these methods and models is to enhance the quality of life, consumer satisfaction and system efficiency. <https://ijic.org/articles/10.5334/ijic.2530/print/>. Integrated care pathways are based on a person-centred, multidisciplinary, holistic, continuous and participative care provision, enabling the exchange of information/expertise and the coordination between health, social and other support services located at national, regional and local levels.

**Resource centres for rare diseases:** resource centres for rare diseases are a one-stop shop service, complementary to health and social care services, specifically designed for people living with a rare disease and their carers. Resource centres provide holistic services and support, while also creating a bridge between people living with a rare disease and their carers and various stakeholders, services and professionals providing health care, social care, and social support – including (re)habilitation, education and employment. Examples of resource centres for rare diseases: Ågrenska, Sweden; Frambu, Norway; NoRo, Romania. <https://innovcare.eu/social-services/resource-centres-for-rare-diseases/>

**RareResourceNet - European Network of Resource Centres for rare diseases:** aims at accelerating the development and the implementation of holistic high quality care pathways for people living with a rare disease across Europe. The network's objectives focus on networking and mutual learning, gathering and disseminating good practices and expertise, developing and delivering training for people living with a rare disease and professionals. <https://innovcare.eu/social-services/rarerresourcenet/>

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# Achieving Holistic Person-Centred Care to Leave No One Behind

A contribution to improve the everyday lives  
of people living with a rare disease and their families

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A Position Paper by EURORDIS and its Members

“The ambition of **EURORDIS** is to have holistic care provided to the 30 million people living with a rare disease in Europe, and their families, by 2030.”



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