



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

Executive Summary

May 2019

eurordis.org/carepaper



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?

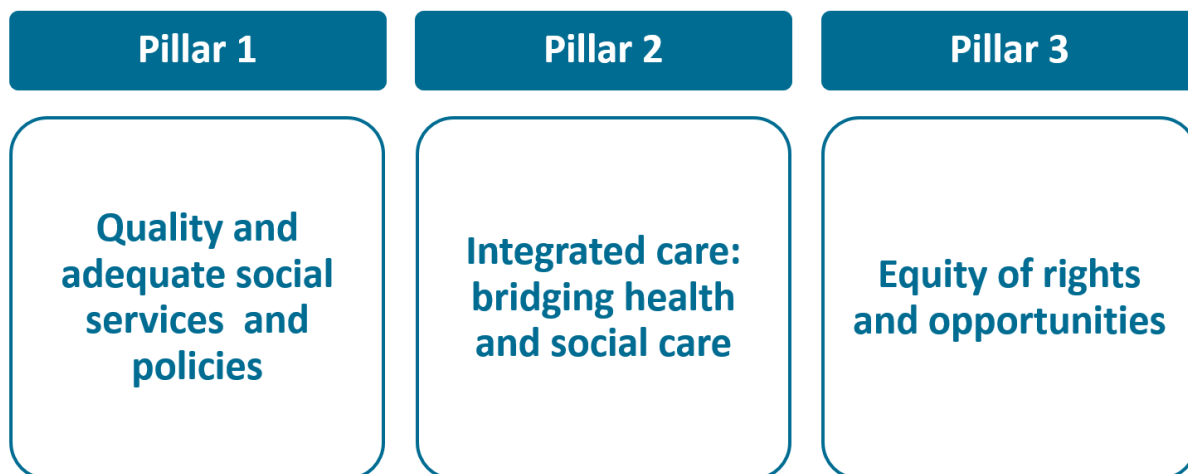
It is urgent to address the serious unmet needs of people living with a rare disease and their families. Doing so requires a multi-sector approach from research, to diagnosis, access to treatment, health care and social care, at both national and European levels. **All stakeholders have an instrumental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



Alec, Sanfilippo syndrome, Australia

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**



Philipp, osteogenesis imperfecta, Germany

Our recommendations in full

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.



Shelby and Taylor, Angelman syndrome, USA

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 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.

European countries must
improve national disability
assessment systems

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.



Louis and Matthieu, Becker muscular dystrophy, France

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.

To read the full version of this paper (also available in French, Spanish, German, Italian, Portuguese and Russian) please visit:

eurordis.org/carepaper

Notes

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of people living with a rare disease and their families

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.„



EURORDIS-Rare Diseases Europe

Plateforme Maladies Rares ♦ 96 rue Didot
75014 Paris ♦ France

EURORDIS Brussels Office

Fondation Universitaire ♦ Rue d'Egmont 11
1000 Brussels ♦ Belgium

EURORDIS Barcelona Office

Recinte Modernista Sant Pau ♦ Pabellón de Santa Apolonia
Calle Sant Antoni M^a Claret 167 ♦ 08025 Barcelona ♦ Spain

EURORDIS.ORG

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