Action Area 1: Governance and Sustainable Financing



Governance & Sustainable Financing

FOCUS OF THIS SECTION:

Integration of rare diseases in relevant national policies, legislations and guidance in the health sector, education, social protection and employment

Adequate funding for diagnosis, treatment, care and services

Strategic policy frameworks with oversight and accountability

1.1 POLICY, PLANS AND LEGISLATION

Rare diseases require different sectors to work together to meet the needs of people living with a rare disease, their families and caregivers. This means ensuring they have fair access to effective healthcare and support services.

To meet the specific and complex needs of PLWRD in each country, we need comprehensive policies, plans and laws based on scientific evidence and international human rights standards

PLWRD and their families, caregivers and representative organizaations need to be involved in policy development and implementation.

PROPOSED ACTIONS

For Member States:

Develop and strengthen plans and laws for rare diseases (health and social care)

Establish oversight mechanisms to develop accessibile health and social services and oversee implementation of policies

Lead strategies to reduce stigma and discrimination

For the WHO:

Help countries develop and implement national rare disease policies and plans by providing evidence-based guidance, tools and technical support.

Ensure PLWRD have a voice in shaping policies, plans and services that affect them.

Raise the profile of rare diseases in global fora to ensure sustained collaboration and momentum

For International & National Partners

Help people with lived experience share and exchange knowledge, raise awareness, and advocate for responses to address challenges

Engage different stakeholders to inform the development and implementation of policies, plans and laws, especially those that reduce stigma and discrimination, raise awareness of rare diseases, and protect the human rights of PLWRD

Action Area 1: Governance and Sustainable Financing

1.2 FINANCING

National rare disease strategies need adequate, sustainable public financing and effective pooling of resources

Financing approaches should take into account the long-term, complex and potentially high-cost care needed for PLWRD and protect them and their families from financial hardship

PLWRD and their families must be part of decision-making processes for financing mechanisms

PROPOSED ACTIONS

For Member States:

Allocate adequate and sustainable funding for policies, plans and programmes for rare diseases

Support existing pooled funding schemes with dedicated taxes and private donations to fund existing pooled funding schemes, and ensure these schemes cover the costs of rare disease treatment and care

Make budget decisions based on the best available evidence on the impact of rare diseases and the cost-effectiveness of different solutions

Strengthen oversight of financing and resource allocation for rare diseases

Ensure that PLWRD and their families and caregivers are protected from financial hardship

For the WHO:

Provide technical support to Member States to help them plan how they budget for rare diseases, allocate resources and monitor spending.

Promote collaboration and knowledge exchange at international, regional, and national levels to strengthen understanding of the socioeconomic burden of rare diseases and the impact of investment in rare diseases.

For International & National Partners

Engage with countries in mobilizing sustainable financial resources and identifying functional gaps in resource allocation to support the implementation of national policies, programmes and services for rare diseases.

Support the development of funding models to cover the costs of diagnosis and care, and reduce associated costs, like travel for specialized services

Promote accountability to improve quality and efficient use of resources

Raise awareness of the social and economic impacts of rare diseases on health systems and society

AREA 1: GOVERNANCE AND SUSTAINABLE FINANCING

14. The integration of rare diseases in relevant national policies, legislation and guidance within and beyond the health sector, including in education, social protection, and employment, is important to meet the multifaceted needs of people living with a rare disease.

15. Adequately funded policies and programmes are required to ensure access to timely diagnosis, treatment, care and services across the life-course and continuum of care. Unaddressed rare diseases can lead to increased costs for governments, communities, families and individuals, as well as productivity losses for economies, many of which could be remedied by prevention, early detection, and timely treatment.

16. Strengthening governance for rare diseases also involves ensuring that strategic policy frameworks are established and supported by effective oversight, regulatory, and accountability mechanisms.

1.1 POLICY, PLANS AND LEGISLATION

17. Rare diseases require concerted intersectoral efforts to address the needs of people living with a rare disease, their families, and caregivers by providing them with equitable access to effective care and services. The development of comprehensive intersectoral policies, plans and legislation based on scientific evidence and aligned with international human rights standards strengthens governance for rare diseases and ensures that the complex needs of people living with a rare disease are addressed within the context of each country.

18. Stand-alone or integrated national rare disease policies and plans provide the framework for action, while establishing links with other relevant health and social policies, strategies and programmes. Legislation facilitates action by establishing working definitions, legal protections and accountability mechanisms to support accessible health and social services.

19. Policy development and implementation requires involvement of all relevant stakeholders, including people living with a rare disease, their families, caregivers and representative organizations.

PROPOSED ACTIONS

20. *Member States*
    a. Develop, review, update, strengthen and implement national and/or subnational policies, plans and legislation that cover both health and social care for rare diseases, and define their scope of coverage, as appropriate to local need and context, to facilitate interpretation and implementation of service coverage, reimbursement of therapies, data collection and monitoring, among others.

b. Establish oversight mechanisms, such as dedicated task forces or coordination bodies, to promote development of accessible health and social services and oversee the implementation of policies related to rare diseases, enhancing accountability and effective management.
c. Regularly assess, where applicable, the implementation of policies and plans for rare diseases and evaluate their impact on health outcomes and lived experience.
d. Lead and coordinate intersectoral strategies for reducing stigma and discrimination and promoting the human rights of people living with a rare disease across the life-course. Integrate these within broader health promotion and other strategies such as flexible educational and work environments, in efforts to increase the independence and autonomy of people living with a rare disease.

21. *Secretariat*
    a. Support the implementation of the global action plan through the development and application of normative, evidence-based technical guidance including definition of rare diseases and scope of coverage, and strategies to inform the development, strengthening, and roll-out of national and/or subnational policies, plans and legislation.
    b. Provide assistance in outlining mechanisms that proactively encourage and support the active participation of people living with a rare disease, their families and caregivers in all aspects of policymaking and service planning.
    c. Bring high-level attention to rare diseases within multilateral forums, as appropriate, to help ensure sustained and concrete visibility, momentum, and collaboration.

22. *International and national partners*
    a. Facilitate knowledge translation and exchange among national, regional, and international stakeholders, including people with lived experience and the organizations that represent them, to raise awareness and advocate for specific responses to address the challenges of people living with a rare disease, their families, and caregivers.
    b. Actively engage stakeholders across sectors to inform the development and implementation of evidence-based policies, plans and legislation, paying explicit attention to preventing stigma and discrimination, increasing the visibility of rare diseases in the global agenda, and protecting the human rights of people living with a rare disease, their families and caregivers.

1.2 FINANCING

23. National rare disease strategies require adequate, sustainable public financing and effective pooling of resources through national health financing arrangements. Rare disease services and products should be considered in benefit-package design and priority-setting processes, with financing approaches that account for the long-term, complex and potentially high-cost nature of care while protecting persons living with a rare disease, their families and caregivers from financial hardship and reducing out-of-pocket payments. Complementary contributions from philanthropic and other partners

may support system strengthening and innovation when aligned with national priorities and integrated into existing financing and accountability arrangements.

24. Collaboration with people living with a rare disease, their families and caregivers is essential in decision-making processes related to financing mechanisms.

PROPOSED ACTIONS

25. *Member States*
    a. Allocate adequate and sustainable funding for policies, plans and programmes for rare diseases using dedicated domestic budgetary allocations.
    b. Channel earmarked taxes and private philanthropic funding through existing pooled arrangements and include rare diseases treatment and care costs in the benefit packages of these arrangements.
    c. Use the best available evidence on the epidemiological, clinical, social and economic burden of rare diseases, including impacts on families and caregivers, as well as effectiveness and cost-effectiveness of interventions to inform sustainable budgets and resource allocation.
    d. Strengthen oversight of financing and resource allocation for rare diseases by establishing monitoring and accountability mechanisms, including designated focal points, units or functional divisions within the ministry of health or equivalent body.
    e. Ensure that social protection mechanisms and benefits, protect people living with a rare disease, their families and caregivers from financial hardship under the principles of UHC.

26. *Secretariat*
    a. Provide technical support, tools and guidance to Member States in strengthening their national capacity to engage in resource planning, budgeting and expenditure monitoring on rare diseases.
    b. Promote collaboration and knowledge exchange at international, regional, and national levels to strengthen understanding on the socioeconomic burden and impact of investment in rare diseases.

27. *International and national partners*
    a. Actively engage with countries in mobilizing sustainable financial resources and identifying functional gaps in resource allocation to support the implementation of national and/or subnational policies, programmes and services for rare diseases.
    b. Support the development of funding models to cover the costs of diagnostics and therapeutics, and to offset the costs associated with referral, for example, for travel and specialist services and interventions.
    c. Promote accountability to improve quality and efficient use of resources.
    d. In partnership with relevant stakeholders, raise awareness of the social and economic impacts of rare diseases on health systems and society.