Assessing the economic burden of rare disease across Europe: A health economic study of financial and quality of life impacts



EE608

Kirsten Axelsena, Annie Kennedyb, Lisa Fengc, Charlotte Poond

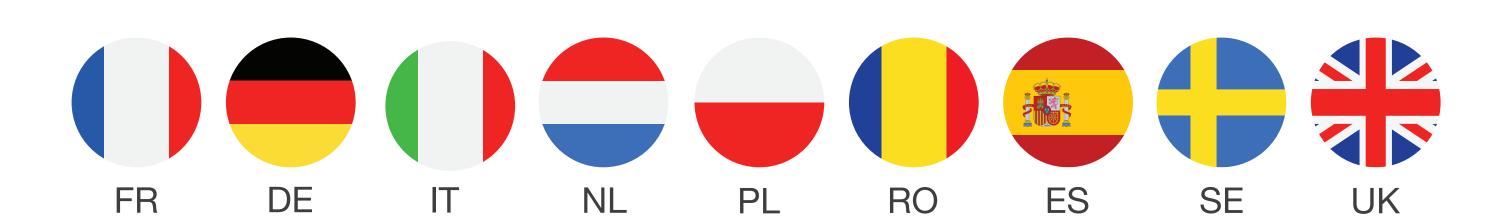
^aChales River Associates, New York, NY, USA ^bEveryLife Foundation for Rare Diseases, Washington, DC, USA ^cAlexion, AstraZeneca Rare Disease, Boston, MA, USA dCharles River Associates, London, United Kingdom

For more information, please contact Charlotte Poon at cpoon@crai.com



Introduction

- 30 million people in Europe live with an estimated 7,000 rare diseases (RDs), collectively representing a significant public health issue^{1,2}
- Even so, we **know little** of the impact of RDs experienced by patients, their families and the economy
- Objectives: To address this information gap, we estimate the economic and social impact for persons living with rare diseases (PLWRD) for 43 RDs across 9 European countries



Methods

Reviewed the **literature** on the economic cost of RDs

Developed a patient survey with the methodology informed by our literature review and **expert input** from 45 RD specialists

Disseminated the survey between February and May 2024 and collected **detailed** information about the diagnostic journey, healthcare utilisation, treatments and the wider impact associated with living with a RD from July to December 2023

Quantified the economic cost of 43 RDs using a prevalence-based approach



545 **PLWRD**

772

Reference group (without a RD)

Results

PLWRD incurred higher costs to society

Total excess costs

Direct medical costs

€184.5B

Includes physician and other HCP visits, procedures, Rx medications, inpatient days at hospitals, telehealth and home health appointments and durable medical equipment.

Direct non-medical costs

€37.4B

Includes paid caregivers, transportation and accommodation for health presenteeism, early care, disease-related daily living arrangments, long-term care facility days and visits to various therapists.

Indirect costs

€27.5B

Includes lost productivity costs from absenteeism, retirement and a forced shift to part-time work endured by the person living with the RD and their caregivers.

PLWRD experience inequitable care

Of PLWRD were misdiagnosed at least once in their diagnostic journeys

32%

Lower health-related quality of life reported by PLWRDs compared to reference group

3X

Longer time to diagnosis if misdiagnosed

compared to those without a misdiagnosis (36 vs 11 months)

78 days

Of lost productivity per year for each person with a rare disease across themselves and their caregivers

4X

Faster diagnosis when targeted EMA-approved treatments were available at symptom onset

year

Longer time to diagnosis for women compared to men, and +1.5 year longer time to start treatment

~ €249.3billion per year

Annual per-capita excess cost

€20,200

Reference group annual per-capita cost

€121,900

RD group annual per-capita cost



6X Higher

Conclusions

Our findings

- The economic impact of living with a RD extends beyond healthcare costs. A significant portion of the cost reflects the reduced earnings, productivity and career opportunities of PLWRD and their caregivers
- RD patients face a lengthy diagnostic journey, with each misdiagnosis adding costs and negatively impacting the quality of life for PLWRD and their caregivers
- Accessing specialist care and treatment are key drivers affecting non-medical and indirect costs and improving quality of life for patients and caregivers

Policy implications

- Public policies on RD innovation and treatment access should consider the full breadth of impact on PLWRD, caregivers and society
- Policies affecting newborn screening, genome sequencing, and other diagnostic tools need to be prioritised to reduce time to diagnoses
- Improving treatment equity and physician awareness can speed up access to treatment, helping reduce both medical and non-medical costs

References

- 1. Rare diseases European Commission. March 8, 2024. Accessed May 8, 2024. https://health.ec.europa.eu/european-reference-networks/rare-diseases_en
- 2. Addressing the challenges of persons living with a rare disease and their families: resolution /:adopted by the General Assembly. Published online December 16, 2021. Accessed May 14, 2024. https://digitallibrary.un.org/record/3953832

Disclosures