Cost Estimation of Managing Retinitis Pigmentosa in France in a Multi-Payer Environment from a Societal Perspective

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Introduction

Retinitis pigmentosa (RP) is a group of inherited genetic retinal diseases that can cause significant vision loss and potentially blindness[1] and affects approximately 30,000 people in France[1,2]. X-linked retinitis pigmentosa (XLRP) is one of the most aggressive forms of RP characterized by a progressive decrease of peripheral then central vision reaching legal blindness at a median of 45 years in affected males (vs median 70 years for RP)[3].

Currently, no treatment is available for XLRP; recommended management is as for RP, based on supportive care, and includes low-vision rehabilitation and use of optical aids[1].

XLRP is associated with significant clinical, humanistic, and economic burdens with difficulty undertaking daily tasks, psychosocial burden, barriers to work and decreased employment[4]. More precisely, the economic burden of XLRP is associated with substantial spending extending beyond the framework of "conventional" and medical healthcare expenditure (compensation services, benefits, absenteeism and productivity loss). In France, total annual costs for visually impaired persons were estimated at €10,749 million in 2006. The main cost components in the community were 'loss of income', 'paid assistance' and 'social allowances'[5].

Objective

To extensively explore the economic burden of XLRP by considering all stakeholders involved in its financing and to provide comprehensive findings on the management cost of RP in France by modeling the course of the disease from a societal perspective.

Methods

A systematic literature review (SLR) was conducted, which aimed to assess the economic burden of XLRP, RP and visual impairment in France. As a rare disease, paucity of evidence available on XLRP and RP in France was expected. Therefore, the research was extended to any chronic ocular disease associated with visual impairment. To ensure an exhaustive coverage of all existing data, other relevant sources including congress proceedings were also searched and targeted manual searches of the grey literature were performed. The details and results of the SLR have been described elsewhere[6].

Alongside the SLR, the real-world study EXPLORE-2 exploring the relationship between the clinical stages of XLRP and the associated clinical, individual, and societal level of burden in real-life settings, was used as a complementary data source[7]. The scope and objectives of the EXPLORE-2 study are presented in Table 1.

Table 1. Description of the EXPLORE-2 study.

Retrospective Chart Review Study Combined with Cross-sectional Patient and Caregiver Surveys to Document and Describe the Disease-related Clinical, Individual, Sociodemographic Characteristics of Patients with XLRP and the Impact of XLRP on Patients and Their Caregivers	
Population	• Patients (≥12 years) with XLRP diagnosis confirmed • Caregiver who has a personal relationship with and/or provides unpaid support or care to someone diagnosed with XLRP (e.g., partner, other relative, neighbor, friend)
Primary objective	To explore the relationship between the clinical stages of XLRP and the associated clinical, individual, and societal level of burden in a real-world setting.
Secondary objectives	• To describe the disease pathways, including diagnosis and management sequences in XLRP within routine clinical practice • To describe the quality of life, social and economic burden (e.g., limitations in independent life/work productivity/daily functioning) associated with XLRP • To describe the impact of the disease on the caregivers (the quality of life, economic impact) Exploratory objective: to evaluate country-specific characteristics and medical-resource utilization in the disease pathway
Geographical scope	23 sites in 10 countries (Austria, Belgium, Finland, France, Germany, Israel, Italy, the Netherlands, Spain, and England)

Data from the EXPLORE-2 study were used to inform model parameters (percentage of patients using technical aids to compensate visual impairment (VI) and associated average costs; proportion of patients receiving social benefits; proportion of patients suffering from secondary accidents (accidents and falls) and depression; employment rate and activity impairment). Only parameters for which sufficiently reliable data were available were considered for inclusion in the model.

Collected data were categorized according to a precise typology distinguishing data by their nature (health care resource utilization (HCRU) or cost) and the type of cost (medical or non-medical and direct or indirect). All retrieved costs and HCRU data were related to the payer financing it: national health insurance, private insurance, patient, society. The typology of costs by payer is shown in Figure 1.

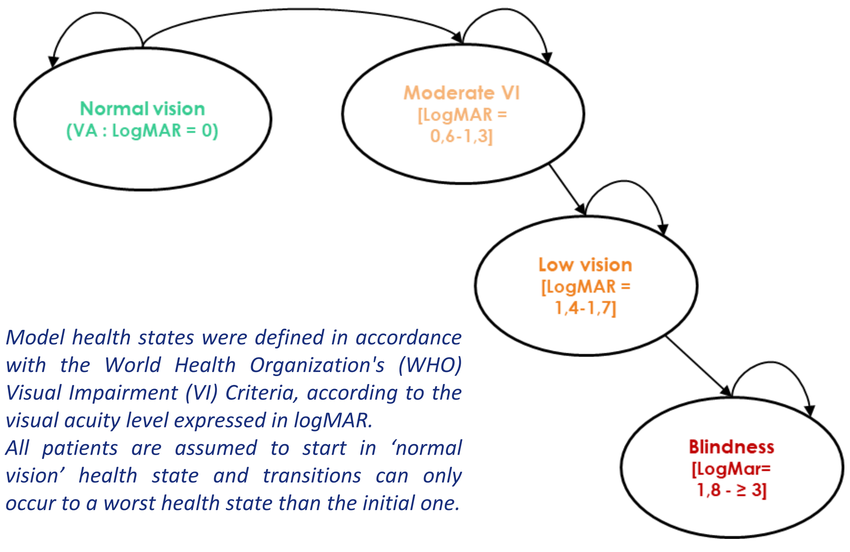
Figure 1. Typology of costs by payer.

National health insurance	Private insurance	Patient	Society
Direct medical costs Dedicated fraction costs of the diagnosis, follow-up and low vison rehab. **Direct non-medical costs** Dedicated fraction of transportation costs **Indirect medical costs** Dedicated fraction of hospitalisation costs due to secondary injuries & depression	**Direct medical costs** Dedicated fraction costs of the diagnosis, follow-up and low vison rehab. **Direct non-medical costs** Dedicated fraction of transportation costs **Indirect medical costs** Dedicated fraction of hospitalisation costs due to secondary injuries & depression	**Direct medical costs** Flat-rate contribution on medical visits and transportation costs. **Direct non-medical costs** Dedicated fraction of technical aid costs (optical assistance) **Indirect medical costs** Flat-rate contribution on hospitalisation costs due to secondary injuries & depression	**Direct medical costs** - Disability compensation benefit (*prestation de compensation du handicap)* - Allowance for disabled adults **Indirect medical costs** Patients and caregivers' productivity losses

The model consisted in a four-health state lifetime horizon cost of illness (COI) model using visual acuity (VA) stages of disease natural progression: normal vision, moderate visual impairment, low vision, blindness. Transition probabilities simulating disease progression were estimated using the SLR published by Lam et al. 2024[8] synthesizing data from 14 studies documenting XLRP-related rate of VA deterioration over time

The analysis conducted considered a model entry age at age 8 and a rapid deterioration of VA (scenario 1) over a lifetime time horizon (~70 years).

Figure 2. Economic model structure.



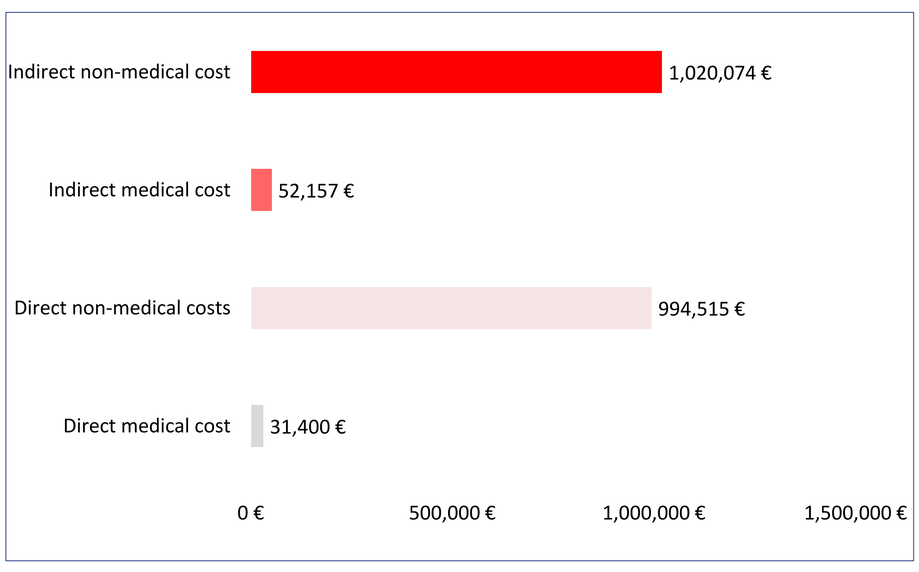
Model health states were defined in accordance with the World Health Organization's (WHO) Visual Impairment (VI) Criteria, according to the visual acuity level expressed in logMAR.
All patients are assumed to start in 'normal vision' health state and transitions can only occur to a worst health state than the initial one.

Results

Over a lifetime time horizon in the base case analysis, the estimated cost to society of a patient diagnosed with XLRP at the age of 8 is **2,098,146€.**

RP's current standard of care generates substantial spending on all the cost categories considered. The highest-cost categories refer to direct and indirect non-medical costs, accounting for 96% of disease-related expenditure.

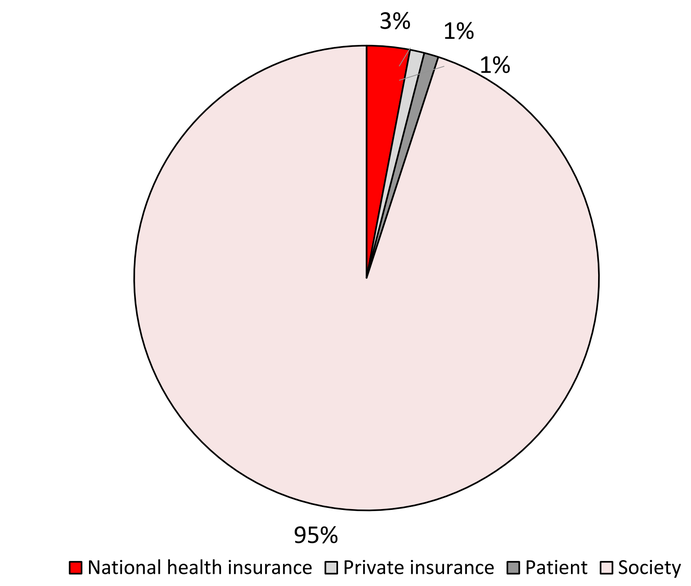
Figure 3. Summary of costs by cost category in the base case analysis.



Several payers contribute to this cost with only 3% of total costs borne directly by the French health insurance system, while the remainder is borne by society including:

- Costs borne by other payers, such as departmental councils, whose funds are used to finance the maisons départementales des personnes handicapées (MDPH).
- Costs borne by other social security branches (Autonomy branch, managed by the Caisse nationale de solidarité pour l'Autonomie, and the Family branch, managed by Allocations familiales).
- Costs borne directly by patients.

Figure 4. Breakdown of costs by payer in the base case analysis.



Conclusions

The COI model developed was used to estimate the economic burden associated with RP within a societal perspective, in an environment where no disease-specific treatment is currently available.

The distribution of the estimated economic burden by payer shows the multiplicity of payers involved in the disease management. Amongst those payers, it is interesting to note that the National health insurance only contributes to 3% of the XLRP management costs. Main expenses are supported (95%) by the other branches of Social Security and local allowances.

So far, the perspective taken for the assessment of innovative treatment is often restricted to the healthcare system, which means only costs borne by the national health insurance are considered.

The arrival of a new treatment could increase the expense of the health insurance but could however decrease the overall expenses related to the XLRP management.

Therefore, it could be interesting to consider a broader perspective, considering organizational and financial impact integrating all disease management costs or savings.

Main findings

- **Restricting the economic impact of potential RP-indicated innovative treatments to a healthcare system perspective leads to disregard most of the costs incurred in managing the disease.**
- **Since formally only the healthcare perspective is considered in HTA, the demonstration of the economic value of a RP innovative treatment could be biased.**
- **Adequate value assessment of these treatments should consider all payers involved in its financing in the framework of a societal perspective.**

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