Session: Socioeconomic impact

# Comprehensive Approach to Measuring Rare Disease (RD) Outcomes and Impacts

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Cumming School of Medicine

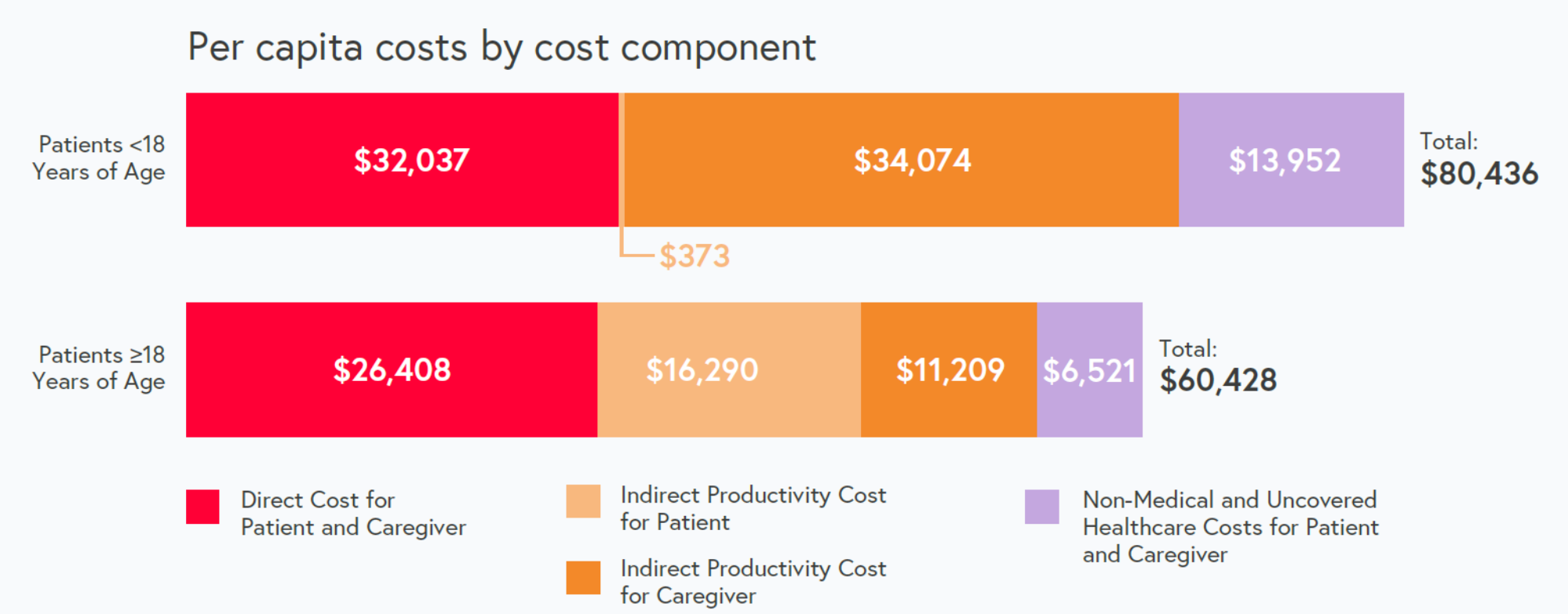
**CORD RQMO Meeting**
**Montreal, November 27 2024**

# Overview:
# Measuring Broader Impact on Patients and Families

- Going beyond the impact on the individual, and looking at the broader effects on the families and caregivers
- Work productivity and employment effects of caregiving
- Spillover effects
  - Caregiver and family member quality of life
  - Time spent on caregiving

**Measuring to Inform Decisions:**
**Planning resource allocation and health systems supports to address RD needs**

# Productivity Costs > Direct Medical Costs in Rare Diseases




- Total economic burden on 379 rare diseases in one year
- Derived from analysis of claims data and survey of ~1400 families (USA)

*The National Economic Burden of Rare Disease Study Infographic, February2021*

# Spillover Effects on Caregivers and Family Members

- Spillover effects – HRQoL effects for caregivers and family members – are rarely considered in cost-effectiveness analysis.
- Systematic review (n=80 studies); only 10 (8%) reported spillovers
- Most studies did not include a comparator, limiting ability to infer spillover effects
- Some national guidance bodies are now recommending inclusion of spillover effects
  - Research gaps remain to be addressed in estimation and incorporation methods to increase the adoption of inclusion of these measures

*- Wittenberg E, James LP and Prosser LA. Spillover Effects on Caregivers' and Family Members' Utility: A Systematic Review of the Literature. PharmacoEconomics 2019; 37:475–499.*

# Frameworks and Measurement of Costs in Rare Disease (RD)

To better advocate for supports: We need consistent standardized evidence to understand and communicate the full impacts of rare disease

PharmacoEconomics
https://doi.org/10.1007/s40273-023-01262-x

ORIGINAL RESEARCH ARTICLE

Check for updates

**Developing a Framework of Cost Elements of Socioeconomic Burden of Rare Disease: A Scoping Review**

Gillian R. Currie[1,2,3] · Brittany Gerber[2] · Diane Lorenzetti[2,4] · Karen MacDonald[2] · Susanne M. Benseler[1,3,5] · Francois P. Bernier[3,6] · Kym M. Boycott[7,8] · K. Vanessa Carlas[9] · Bettina Hamelin[10] · Robin Z. Hayeems[11,12] · Claire LeBlanc[13] · Marinka Twilt[14] · Gijs van Rooijen[9] · Durhane Wong-Rieger[15] · Rae S. M. Yeung[16] · Deborah A. Marshall[2,3,17,18]

To support researchers in consistently capturing the impacts of RD, we created a standardized list of cost elements

When applied to the literature we found few studies (<10%) reported productivity or education costs, travel or accommodation costs, government benefits or family impacts

PharmacoEconomics
https://doi.org/10.1007/s40273-023-01308-0

SCOPING REVIEW

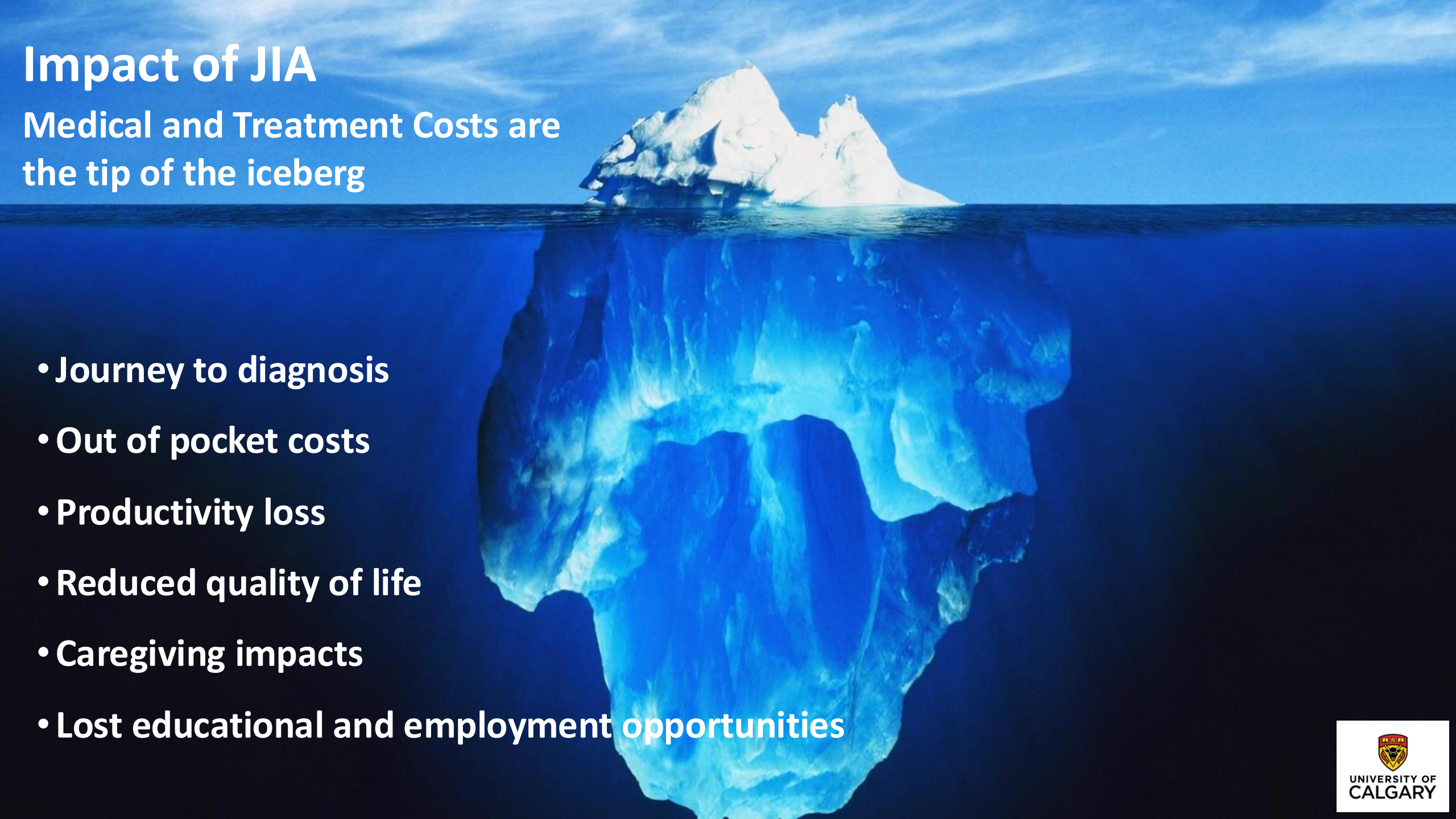
Check for updates

**Are We Capturing the Socioeconomic Burden of Rare Genetic Disease? A Scoping Review of Economic Evaluations and Cost-of-Illness Studies**

Deborah A. Marshall[1,2,3,4] · Brittany Gerber[1] · Diane L. Lorenzetti[1,3,5] · Karen V. MacDonald[1] · Riley Jewel Bohach[1] · Gillian R. Currie[1,4,6]

# Impact of JIA

## Medical and Treatment Costs are the tip of the iceberg



- Journey to diagnosis
- Out of pocket costs
- Productivity loss
- Reduced quality of life
- Caregiving impacts
- Lost educational and employment opportunities

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# Socioeconomic Impact of RD: Juvenile Idiopathis Arthritis (JIA)

- Literature reviews have highlighted a lack of evidence regarding both the humanistic and economic burden of JIA[1 - 3]
- Studies have shown costs are substantial and largely focused on medication and healthcare appointments[1 - 7]
- JIA has a significant impact on HRQoL for children living with JIA, [4, 8, 9] which improves following diagnosis[8, 9]
- Summary: Existing literature is **largely limited to costs to the health system** rather than the burden of JIA on children or their families[10]


[1] Gidman W et al. Curr Rheumatol Rep. 2015 May;17(5):31.
[2] Angelis A, et al. Health Policy. 2015;119(7):964–79.
[3] García-Rodríguez F, et al. Pediatr Rheumatol Online J. 2021 Oct 9;19(1):152.
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[5] Angelis A, et al. BMC Musculoskelet Disord. 2016 Aug 2;17:321.
[6] Minden K, et al. Clin Exp Rheumatol. 2009 Oct;27(5):863–9.
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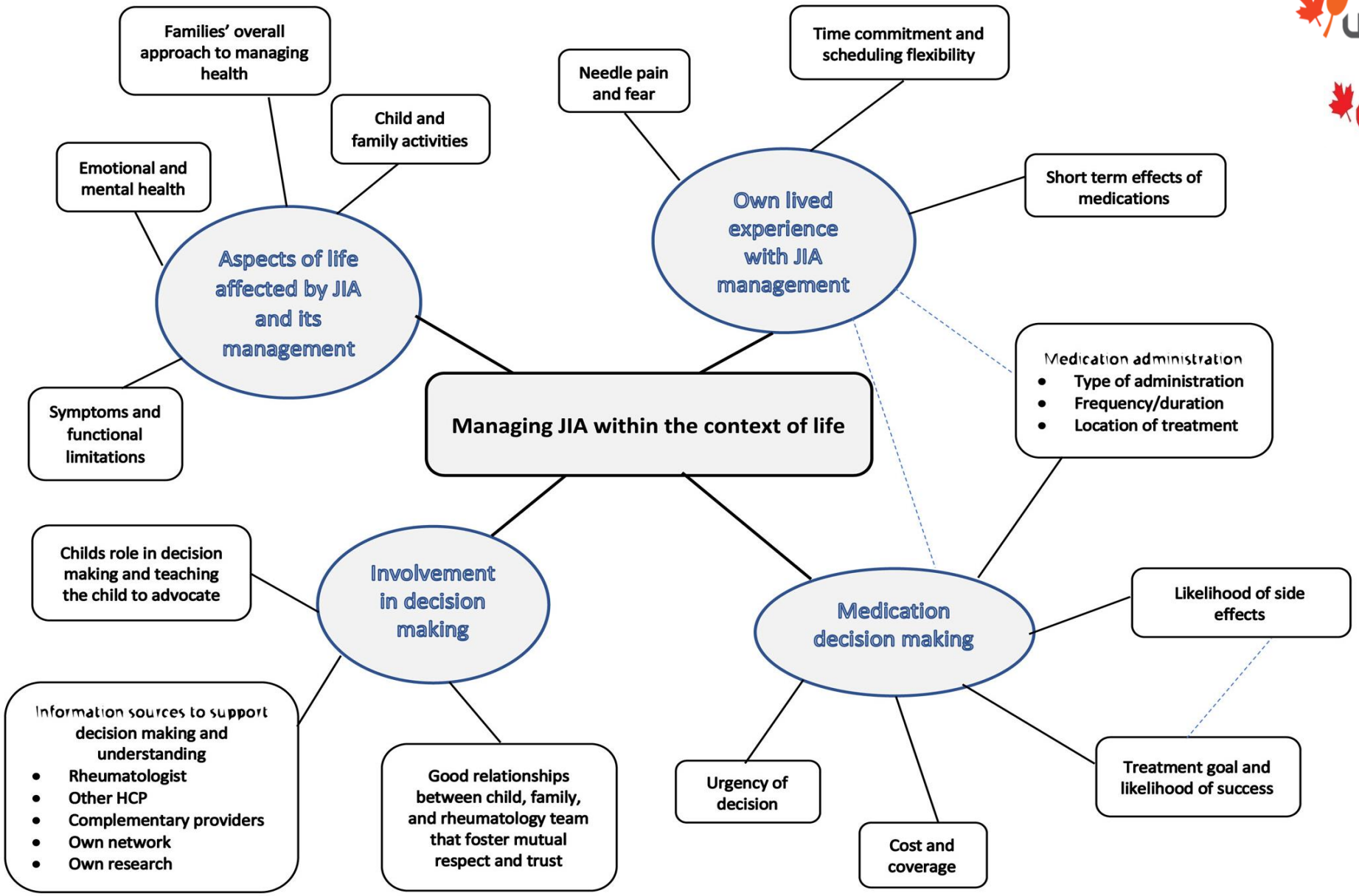
[8] Listing M, et al. Arthritis Res Ther. 2018 May 30;20(1):106.
[9] Smith AD, et al. Rheumatol Oxf Engl. 2023 Feb 1;62(2):794–803.
[10] Kip MMA, et al. Pediatr Rheumatol. 2019;17:20.

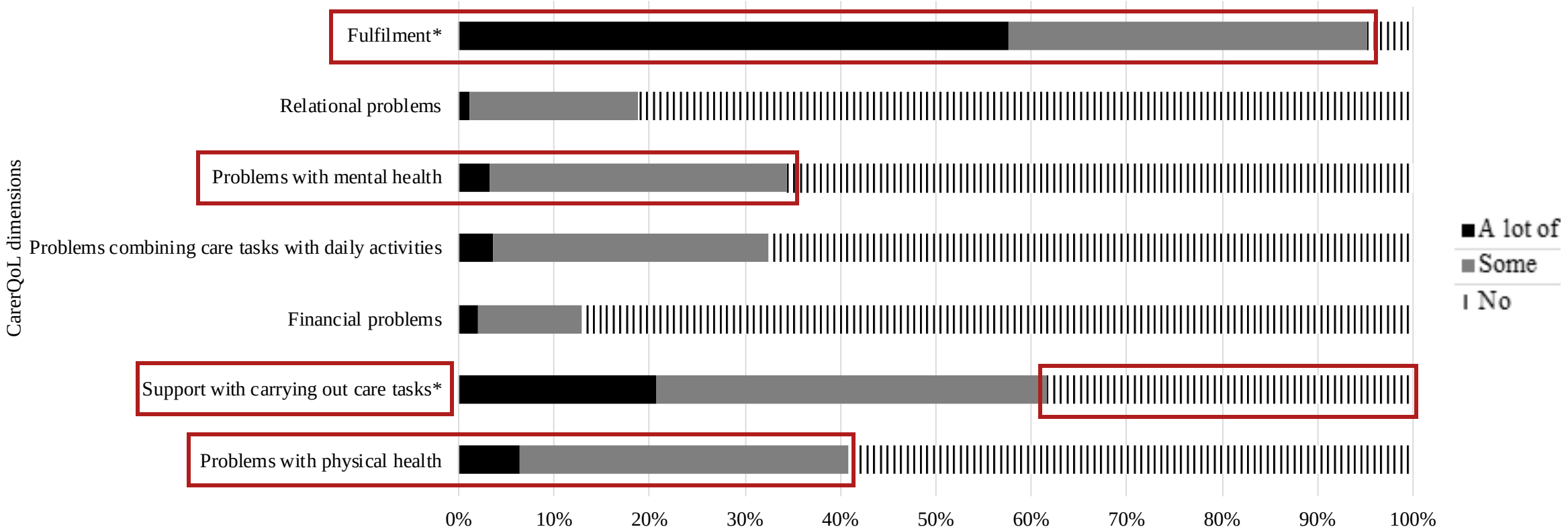



# Managing JIA: Family and patient perspectives

**Incorporating what matters most to children and their families in their life context**

Currie, G. R., et al. (2023). Managing juvenile idiopathic arthritis within the context of their life: What we learnt from children and youth living with juvenile idiopathic arthritis and their parents. Musculoskeletal care, 21(4), 1248–1260.

# Caregiving Quality of Life in Parents (n=250)




- 95% report some or a lot of fulfilment from carrying out care tasks
- 38% report having no support for carrying out care tasks
- 39% and 34% report some or a lot of problems with physical health and mental health respectively

*- Grazziotin et al. Factors Associated with Care- and Health-Related Quality of Life of Caregivers of Children with Juvenile Idiopathic Arthritis. Pediatric Rheumatology 2022;20:51*

# Socioeconomic Impact of JIA: Parent Productivity and Usual Activities

Caring for a child with JIA has **a large impact** on workplace productivity and usual activities for caregivers.

These effects are related to disease activity (higher disease activity, more impairment)

## Work Changes

- 9.3% report changed work commitment in the last year

## Absenteeism

- Accounting for up to 17% of work time

## Presenteeism

- Up to 27% of work was impaired

## Usual Activities

- Up to 27% impairment in regular daily activities

# Socioeconomic Impact of JIA: YOUTH productivity and Usual activities

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**Absenteeism**

- Accounting for up to 17% of work time

**Presenteeism**

- Up to 47% of work was impaired

**Usual Activities**

- Up to 67% impairment in regular daily activities

Youth are missing work due to their JIA.

**Larger impact on presenteeism** than for parents (17%).

**Effect on usual activities** than for parents (27%).



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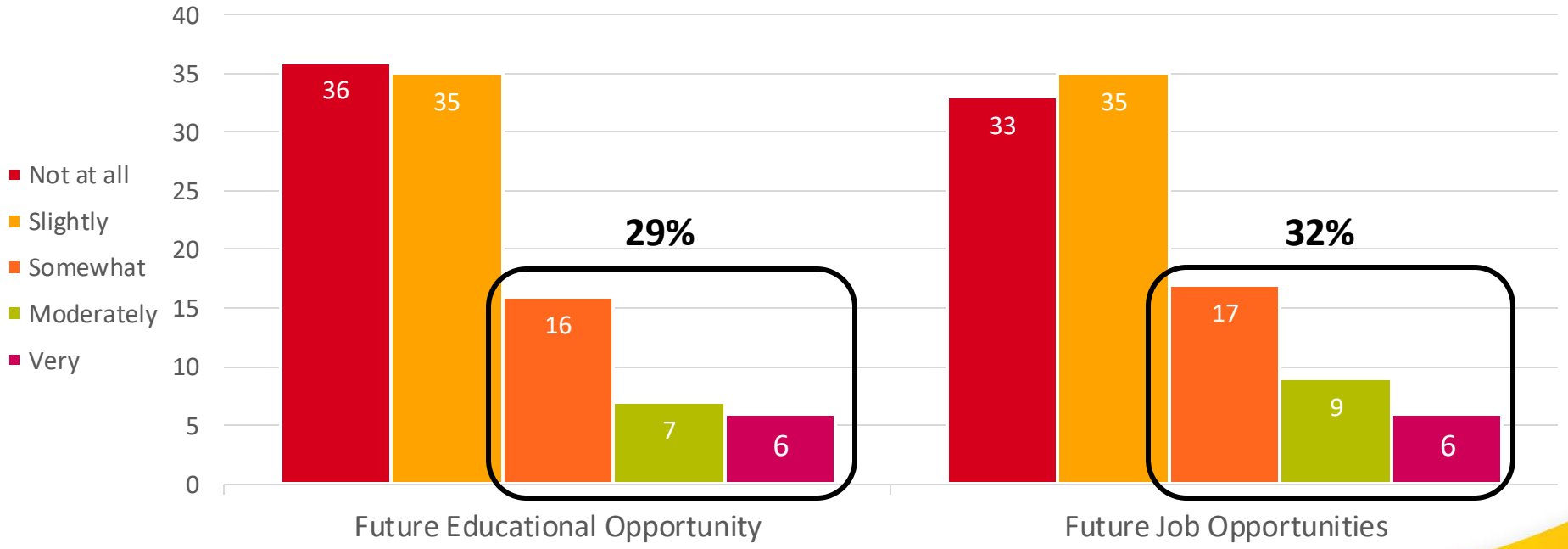
# Socioeconomic Impact of JIA: YOUTH effects on school and future career

Missing school -

- 44% miss days (median 5 days in three month period) due to child's arthritis
- 89% miss days (median 3 days) due to appointments

38% of parents think their child's school performance affected by their arthritis

Parent concerns about child's future educational and job opportunities:



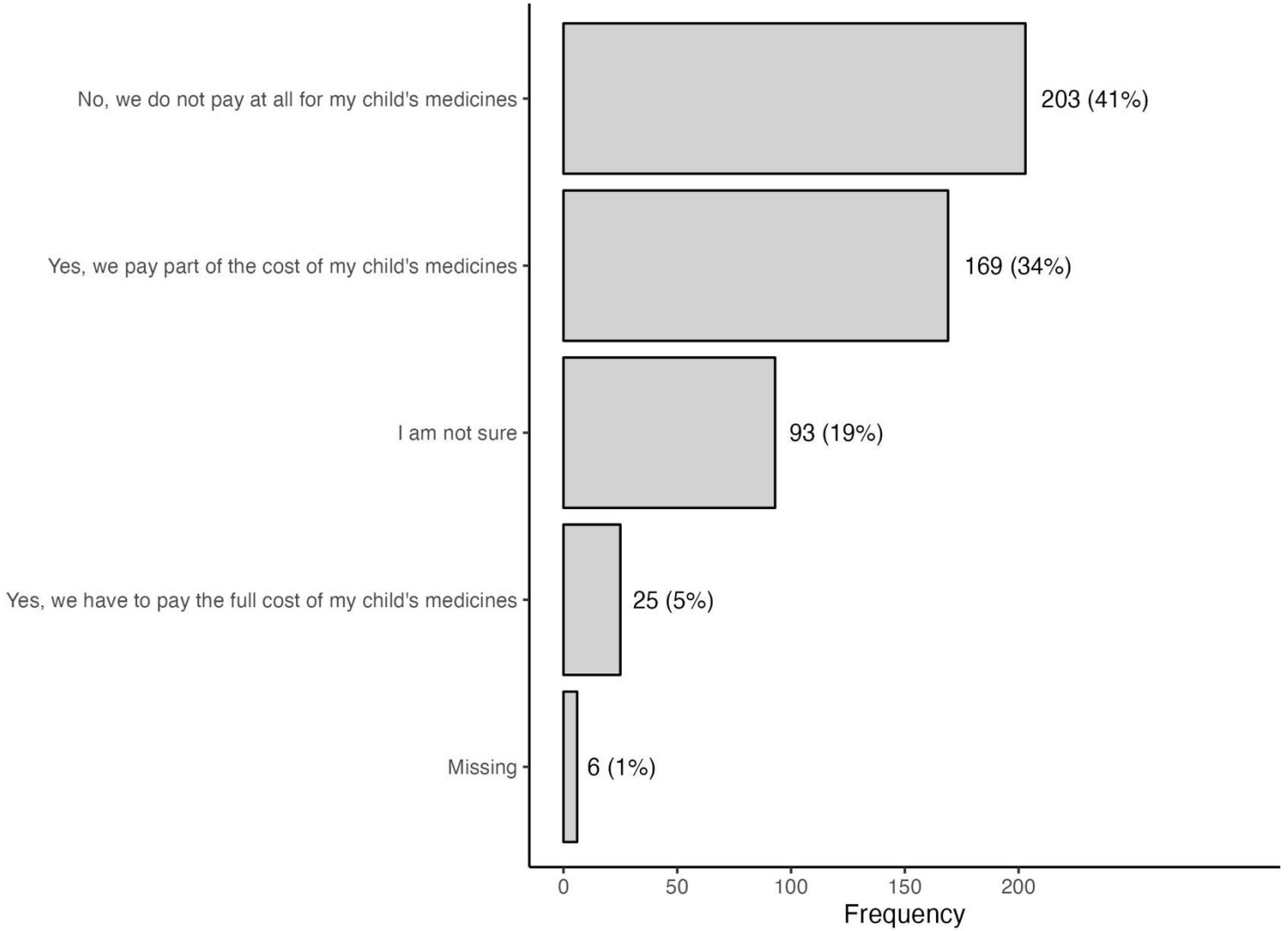
# JIA impacts both parents and youth

| | Impacts For Parents | Impacts for Youth with JIA |
|---|---|---|
| Work Impacts | **Absenteeism**: Accounting for up to **17%** of work time | **Absenteeism**: Accounting for up to **17%** of work time |
| | **Presenteeism**: Up to **27%** of work was impaired | **Presenteeism**: Up to **47%** of work was impaired |
| | **Cost of Lost Work Hours**: **$4324** (Inactive Disease) to **$5786** (Moderate/High Disease) | |
| Usual Activities | Up to **27%** impairment in regular daily activities | Up to **67%** impairment in regular daily activities |
| Education | | **44%** miss days due to arthritis (median: 5 days in a 3-month period) |
| | | **89%** miss days due to appointments (median: 3 days in a 3-month period) |

EJP RD

D.Marshall, CORD, 2024

# Out of pocket costs of medicines



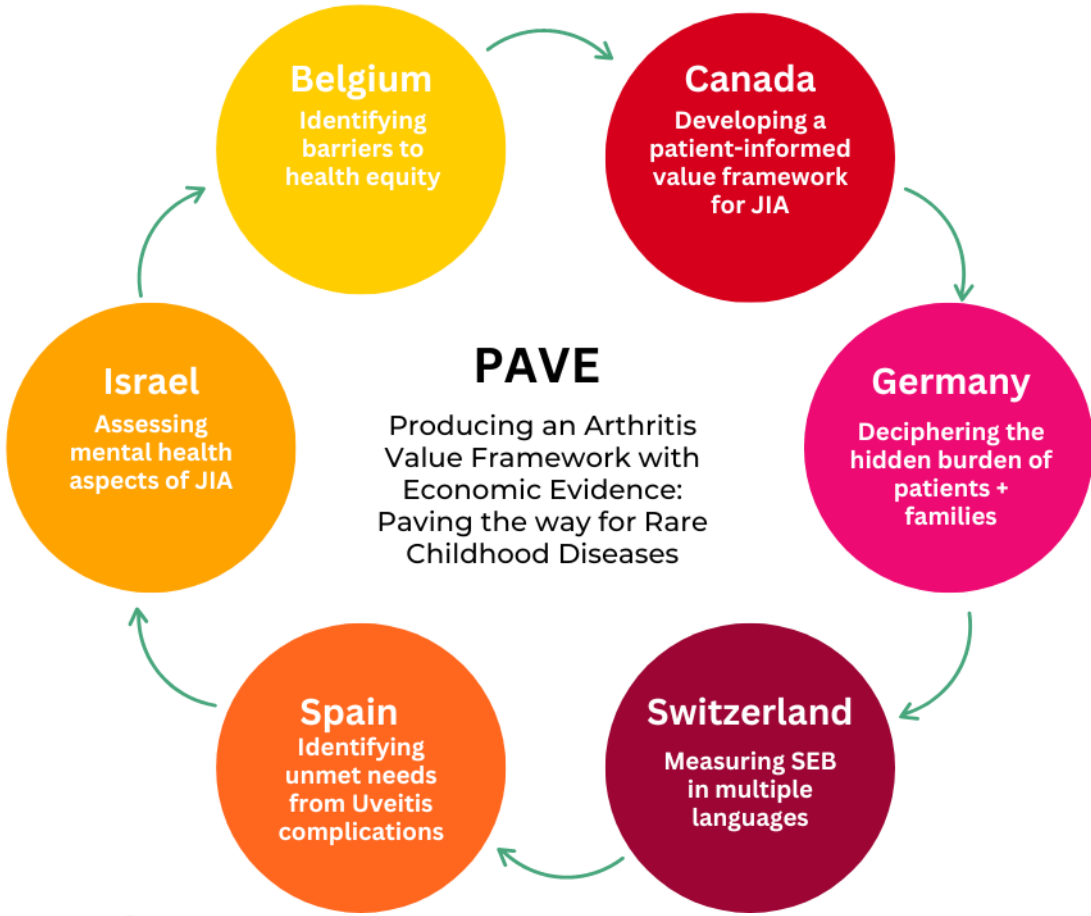

- Up to 59% of families pay some or all of the costs of medications

# Applying these learnings

- Though studies have demonstrated that costs to patients, families and society are high, our scoping review (and others) highlight that these costs are not routinely included
  - These studies undercount the hidden burden of rare disease borne by patients and families leading to a gap in our global understanding of the full impact of rare diseases on families.

# Together we can PAVE the way forward to better understand Rare Childhood Diseases

- ✓ Understand all the ways that childhood arthritis affects young people and families
- ✓ Co-create with families a framework to understand and measure these impacts
- ✓ PAVE the way forward to help develop solutions to improve the lives of children and families




**Producing an Arthritis Value-Framework with Economic Evidence**
**Paving the Way for Rare Childhood Diseases**

D.Marshall, CORD, 2024

# RAre Disease Administrative Data Research (RADAR) Team: putting the health system impact of rare diseases on the radar (DA Marshall, D Baribeau, F Bernier, I Stedman)

- Administrative health data are critical in measuring outcomes (mortality/ morbidity), health system monitoring, quality improvement, health research (prevalence, burden of disease, costs) and policy decision-making (planning and resource allocation).
- Rare genetic diseases (RGDs) cannot be identified reliably in these databases because the ICD-10 coding system used in Canada includes only a small fraction of known RGDs.
- Consequently, most RGDs are hidden or underrepresented, and their prevalence, impact and burden, and variability by RGDs and jurisdiction across Canada is not well understood.
- Having dedicated codes to enable the identification of RGDs would allow us to determine how many patients in Canada are affected by RGD, and quantify the impact and burden of these diseases and related disparities in access to care. This evidence is needed to inform resource allocation and planning to support health systems and address the needs of this equity-deserving patient population.
- Aim to address existing evidence gaps to guide next steps regarding new coding systems (such as adopting ICD-11) in Canada.

# Thank you!
# On behalf of our UCAN, C4R SOLVE and PAVE teams

**Questions and Discussion**

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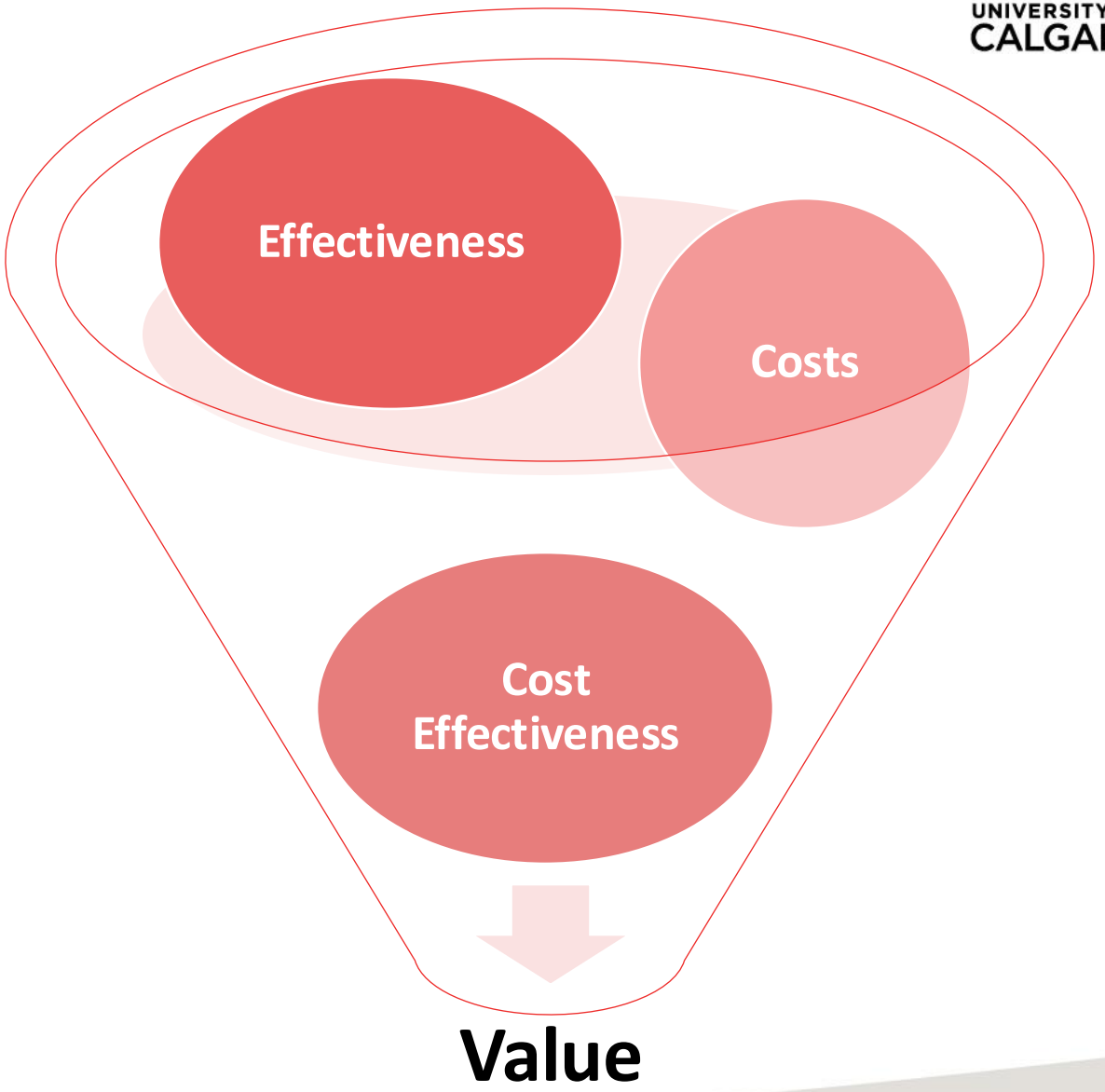
# Measuring Value in Personalised Medicine

## Measure What Matters to Patients, Families and Their Communities

## Evaluate Complex Clinical Pathways Reflecting PM Testing and Treatment Trajectories

# Perspective on Value: Typically Cost-Effectiveness from the Payer Perspective

- Effectiveness: Outcomes associated with the intervention (e.g. life years, quality-adjusted life years)
- Costs: Monetary expenditures associated with direct costs of health services
- Cost-effectiveness: Incremental cost-effectiveness ratio (ICER) measures efficiency as marginal cost per unit of effectiveness (PM vs standard of care)



# Productivity Loss Among Parents of Children with Arthritis: Work Productivity and Activity Impairment Questionnaire (WPAI)

- 12% of parents had made changes in their work commitment due to their child's JIA

**73% reduced working hours**

**13% stopped working altogether**

**Absenteeism**

- Parents missed an average 3.2 hours of work

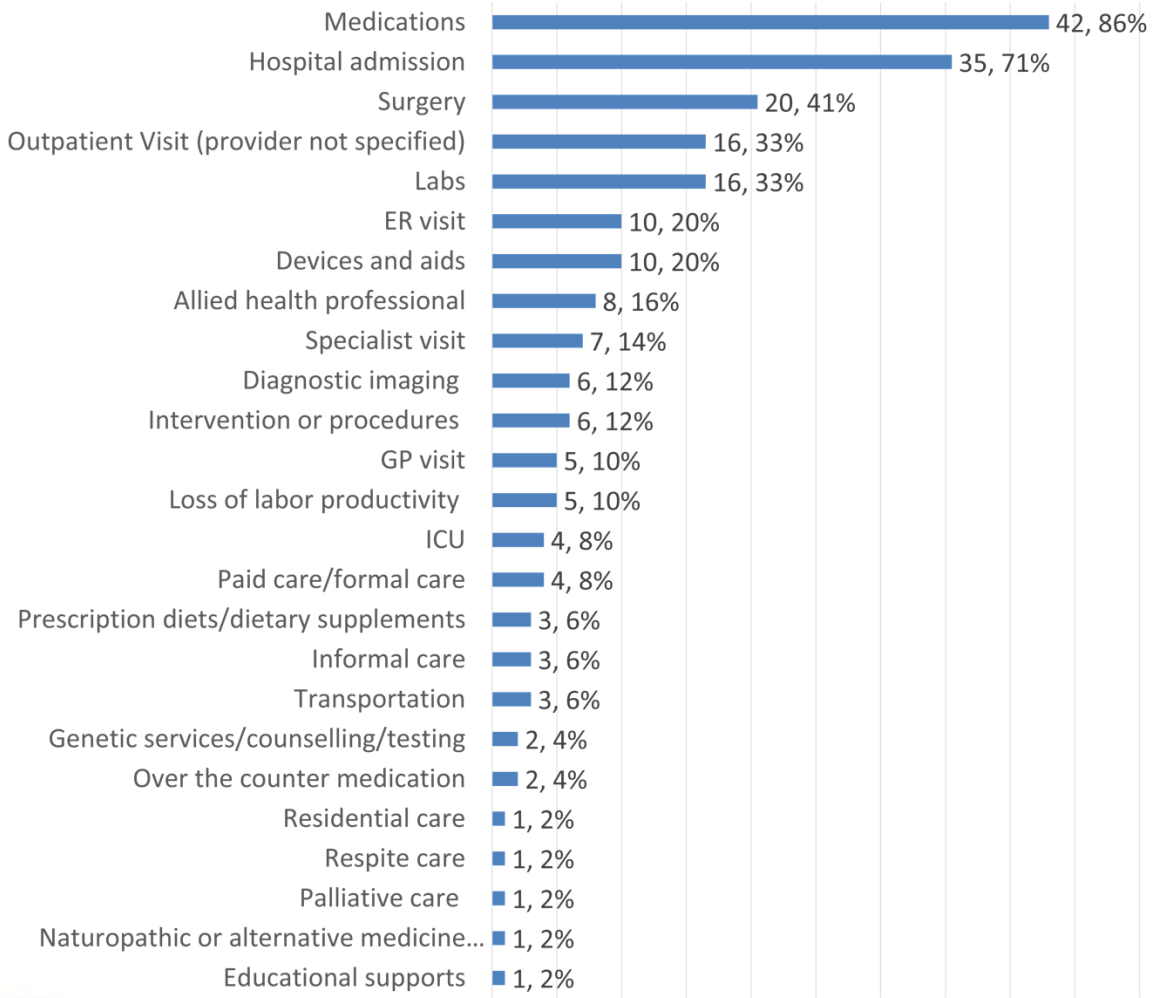
**Presenteeism**

- Mean Impairment to productivity: 20%

- For those working, overall work impairment = 26%
- Mean impairment to usual activities: 20%

*- Grazziotin et al. What is the impact on workplace productivity and usual activities for caregivers of children with juvenile idiopathic arthritis (JIA)? Under review*

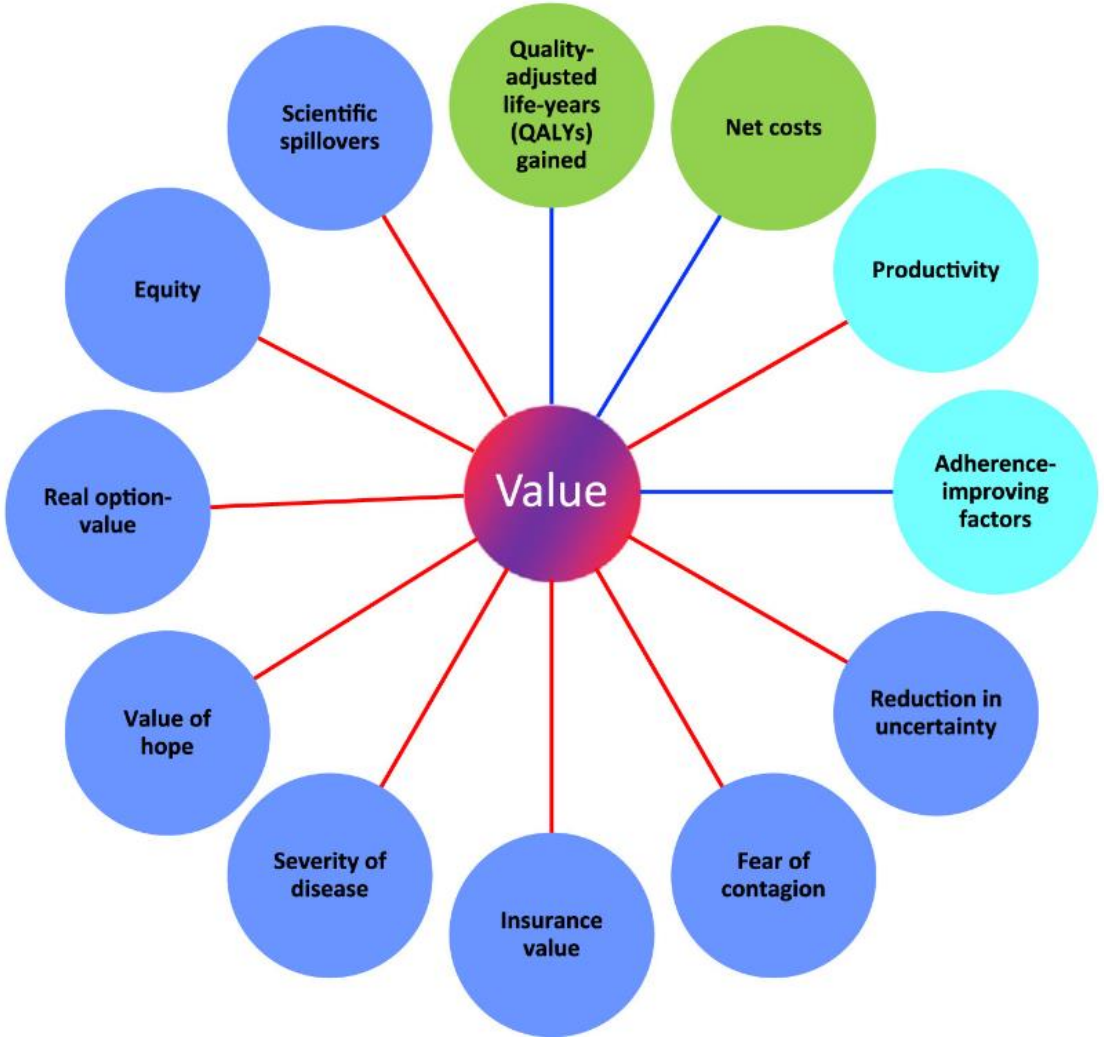
# Systematic Review: Costs Reported in Economic Evaluations for Rare Diseases




- Only 7/49 studies used a societal perspective; others used healthcare system and payer perspectives
- Medical costs (e.g., medications, hospitalizations, outpatient visits, laboratory tests and surgery) were the most commonly reported costs.
- Few studies reported costs to patients and families such as productivity, transportation, informal care, over the counter medication or educational supports
- **Unique aspects to PM: advanced genetic testing, use of private labs or out of country travel for testing, participation in research, physician advocacy time**

*- Currie GR et al. Developing a Framework of Cost Elements of Socioeconomic Burden of Rare Disease: a Scoping Review. CIHR Knowledge Synthesis, Under Review*

# Broadening our View of Value: Extended Cost-Effectiveness




- Going beyond the impact on the individual, and looking at the broader effects on the families and caregivers
- Work productivity and employment effects of caregiving
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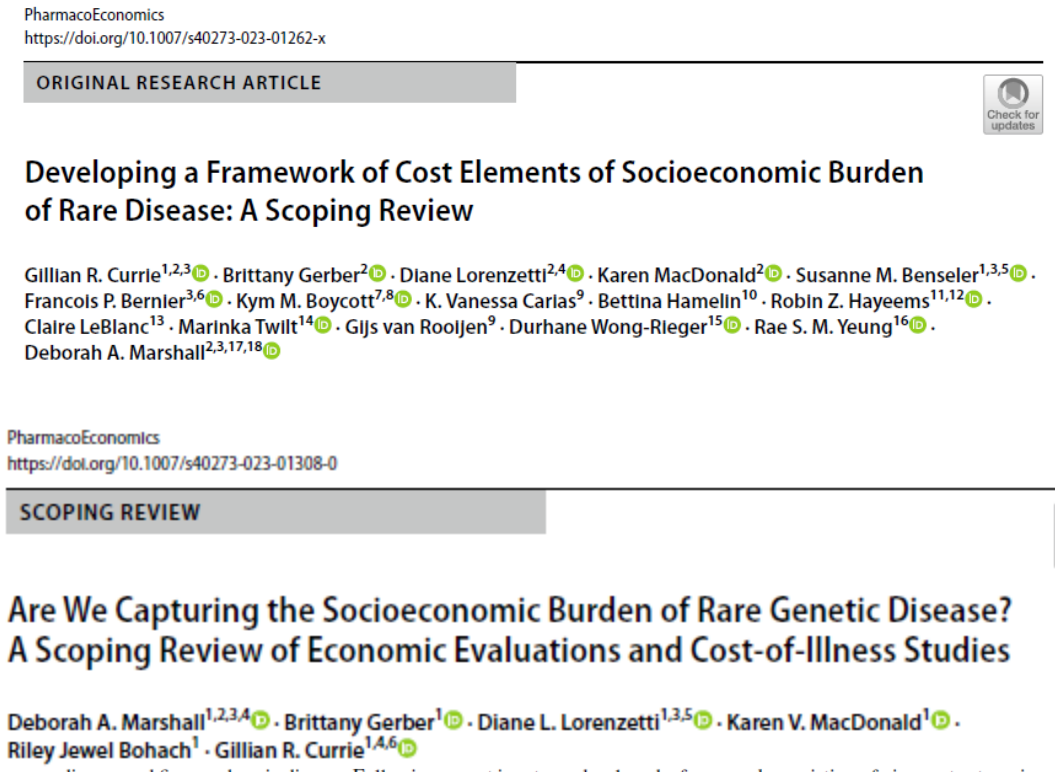
*- Lakdawalla D, Doshi J, Garrison L et al. Defining elements of value in healthcare-a health economics approach: an ISPOR Special Task Force Report [3]. Value Health. 2018;21(2):131-13*

# Frameworks and Measurement of Costs in Rare Disease (RD)

1. Identify existing frameworks for measuring cost elements of SEB for chronic or rare diseases + draw on experts to develop a standardized list of cost elements

2. Apply the framework to examine what costs are included in the existing literature: Few studies (<10%) reported productivity or education costs, travel or accommodation costs, government benefits or family impacts



PharmacoEconomics
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ORIGINAL RESEARCH ARTICLE

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SCOPING REVIEW

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