ECONOMIC BURDEN OR IMPACT OF CARING FOR INDIVIDUALS WITH AROMATIC L-AMINO ACID DECARBOXYLASE (AADC) DEFICIENCY - AN ANALYSIS FOR THE US

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1. Background

- Aromatic l-amino acid decarboxylase (AADC) deficiency is a rare neurometabolic disorder with 30 new cases estimated per year in the US
- Many individuals with AADC deficiency are severely delayed in reaching developmental milestones and suffer significant functional impairments that require highly specialized and time-intensive lifelong care[1]
- Other than palliative care, no treatment options currently exist for those with AADC deficiency[2]
- The impact of caring for patients with AADC deficiency was contextualized based on questionnaires administered to 12 caregivers3, and qualitative data from the same study highlighted the substantial burden of caring for an individual with AADC deficiency (unpublished)

"It's a big commitment and it's a lot and you do need to sacrifice a lot. Free time, socialisation, going out and doing things, um, you know, it just… it, it, it is what it is"
- 607 (caregiver to a 1-year-old)

"The impact has been tremendous. My other children, you know, lack sometimes getting attention from me, doing different things. The whole family has been, you know, affected, but it's been a balancing act"
- 601 (caregiver to a 6-year-old)

- Due to the rarity of AADC deficiency, current understanding and literature on the economic burden of this debilitating condition is limited and understudied

2. Objective

The objective of this study was to estimate the costs related to caring for individuals with AADC deficiency in the US

3. Methods

- A model was developed in Microsoft Excel to estimate the associated (weekly and annual) cost of caregiving for a patient with AADC deficiency in the US
- Primary model inputs were based on data from questionnaires completed by 7 US caregivers (i.e. time spent caring, amount of paid and/or unpaid help received, and the impact on employment)[3]
- Cost inputs were derived from public sources:
  - US employment rate came from statistics compiled by the Economic Co-operation and Development (OECD)[4]
  - Hourly wages from the US Bureau of Labor statistics[5]
- Population estimates of costs associated with providing care is calculated by aggregating all the time used for caregiving multiplied by the estimated costs of caregivers' time, based on employment rate and average hourly wages
- The aggregate of this estimate is subsequently used to derive a population-level estimate of costs in the US

4. Inputs

- Questionnaires capturing the time spent caring for individuals with AADC deficiency were completed by 7 primary caregivers of individuals with AADC deficiency[3]
  - Caregivers reported spending an average of 111 hours (range: 84-147) weekly on practical and emotional care for their child
  - In addition, a mean of 16 hours (range: 12-22) was spent weekly on administrative tasks and travelling/attending medical appointments related to AADC deficiency
  - In total, 14% (1/7) reported receiving unpaid support provided by the partner (12 hours/week)
  - And 43% (3/7) of caregivers reported productivity losses due to having to give up their work in order to provide care for their child with AADC deficiency.
  - Caregivers who had not changed their work but were already full-time homemakers were not included in the calculations and neither were direct medical and non-medical costs
- Country-specific indirect costs, specifically those associated with caregiver time (hourly employment wages and employment rate) were obtained from publicly available sources (Table 1)

Table 1. Key inputs

Parameters	US	Reference
Estimated number of AADC deficiency patients	30	Estimate
Employment rate	68.4%	OECD 2021
National average hourly wage, all employed	$27.07	US Bureau of Labour Statistics, May 2021
National average hourly wage, nursing assistants, orderlies, and psychiatric aides	$15.43	US Bureau of Labour Statistics, May 2021

5. Results

- Based on 30 new patients and their families in the country, the total number of hours needed for informal care was 200,741 hours annually, which corresponds to $5.4 million for informal care
- Annually, 173,160 hours on average were spent on practical and emotional care, representing an estimated total of $4,687,441 per annum
- Caregivers further spent 480 hours weekly on average (24,960 hours/year) on administrative tasks, representing an estimated total of $12,994 per week ($675,667 per annum)
- Caregivers (14%) received 50 hours weekly in unpaid support (2,621 hours/year), translating into an estimated total of £70,945 annually ($1,364 weekly)
- Hours of loss of productivity were estimated to be 17,206 annually, leading to an estimated $465,767 of family income loss

Table 2. Annual time and cost of caregiving for AADC deficiency in the US

Time for care	Hours/year	Cost/year
Caregiver time (i.e., direct efforts by caregivers)		
Time on practical and emotional care	173,160	$4,687,441
Time on administrative tasks	24,960	$675,667
Time of unpaid help used	2,621	$70,945
Lost productivity (i.e., impact on work)		
Time of caregivers giving up work	17,206	$465,767
Total estimated caregiving hours and costs, for all caregivers, per annum	**200,741**	**$5,434,053**
Total estimated lost productivity, for all caregivers, per annum	**17,206**	**$465,767**

6. CONCLUSION

- Primary caregivers spent almost every waking moment caring for the individual with AADC deficiency, requiring almost half to stop working and resulting in substantial annual indirect costs
- Caregivers who were already full-time homemakers were not included in the calculations, neither were direct medical and nonmedical costs, thereby potentially underestimating the overall economic impact of AADC deficiency
- This study provides an important insight into the substantial societal burden of AADC deficiency particularly from the perspective of caregivers and highlights the importance of considering the caregiver perspective when assessing the true burden of disease

References

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