

Impact of Maralixibat on Caregiver Burden for Patients With Alagille Syndrome and Progressive Familial Intrahepatic Cholestasis

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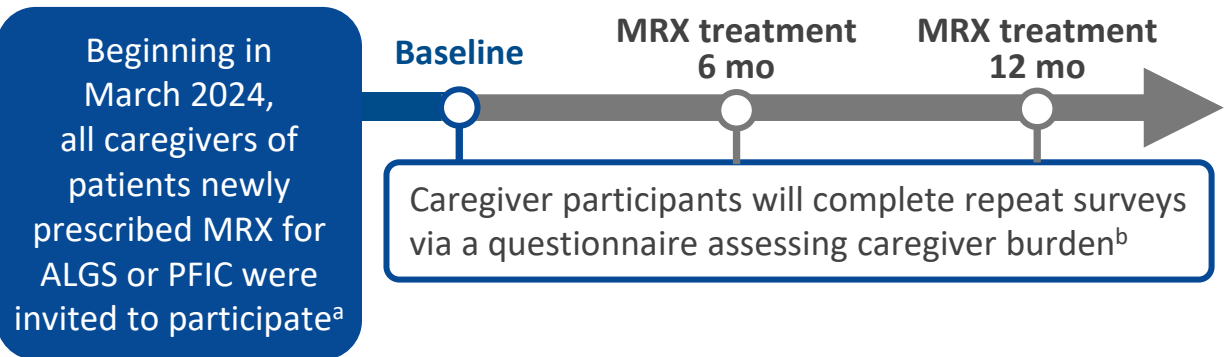
Introduction

- Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis (PFIC) are rare cholestatic liver diseases associated with severe pruritus, along with markedly reduced health-related quality of life (HRQoL).^{1,2}
- Maralixibat (MRX) is a minimally absorbed ileal bile acid transporter inhibitor that prevents enterohepatic bile acid recirculation and is approved for^{3,4}:
 - Treatment of cholestatic pruritus in patients with ALGS ≥3 months of age in the US and ≥2 months of age in the EU
 - Treatment of cholestatic pruritus in patients with PFIC ≥12 months of age in the US and treatment of PFIC in patients ≥3 months of age in the EU
- A recent multinational cross-sectional survey of caregivers of children with ALGS showed overall negative impacts on caregiver emotional health, sleep, daily activity, and work burden.⁵
- Clinical trials of maralixibat in participants with PFIC have shown that improvements in pruritus correlate with improvements in HRQoL and sleep^{6,7}; however, no studies have assessed the impact of maralixibat on caregiver burden in ALGS or PFIC.

Objectives

- To understand overall caregiver burden, HRQoL, and the impact of providing care for patients with ALGS/PFIC before and after administration of maralixibat.
- To report results of surveys at Baseline as surrogate for HRQoL at maralixibat treatment initiation.

Methods



^aAll patients prescribed maralixibat in the US receive the medication through a single pharmacy.
^bThe questionnaire included the validated HADS and ZBI-12 instruments.

- Data collected from the surveys at Baseline were analyzed using descriptive statistics, means, standard deviations, and frequencies/proportions.

Abbreviations

ALGS, Alagille syndrome; HADS, Hospital Anxiety and Depression Scale; HRQoL, health-related quality of life; MRX, maralixibat; PFIC, progressive familial intrahepatic cholestasis; ZBI-12; Zarit Burden Interview.

Results

Table 1. Baseline Demographics of Caregivers

Parameter, n (%)	Caregivers (N=12)
Age, y	
17-29	2 (16.7)
30-39	6 (50.0)
40-49	4 (33.3)
Sex, female	9 (75.0)
Education	
12th grade or lower	2 (16.7)
High school diploma or equivalent	3 (25.0)
Bachelor's degree	3 (25.0)
Postgraduate degree	3 (25.0)
Associate's degree or technical/vocational program	1 (8.3)
Employment	
Full-time	8 (66.7)
Part-time	1 (8.3)
Unemployed or seeking work	3 (25.0)

Table 2. Baseline Demographics and Characteristics of Patients With ALGS or PFIC

Parameter, n (%)	Patients with ALGS or PFIC (N=12)
Age, y	
1-4	7 (58.3)
5-10	3 (25.0)
11-17	2 (16.7)
Sex, male	7 (58.3)
Disease state	
ALGS	11 (91.7)
PFIC	1 (8.3)
Itch severity ^a	
None	1 (8.3)
Mild	6 (50.0)
Moderate	3 (25.0)
Severe	2 (16.7)
Current medications for itch	
Ursodiol	10 (83.3)
Rifampin	3 (25.0)
Antihistamine ^b	1 (8.3)

^aItch severity was assessed and reported by the caregiver. ^bIncludes diphenhydramine or hydroxyzine.

Disclosures

ND has nothing to disclose. RHM and YK are consultants for Mirum Pharmaceuticals, Inc. RH and DBM are employees of and shareholders in Mirum Pharmaceuticals, Inc. WWK is a consultant for Mirum Pharmaceuticals, Inc., Travele Therapeutics, Inc, Ipsen Biopharmaceuticals, Inc, and Gilead Sciences, Inc.

Figure 1. Sleep Quality Experienced by Patients With ALGS or PFIC

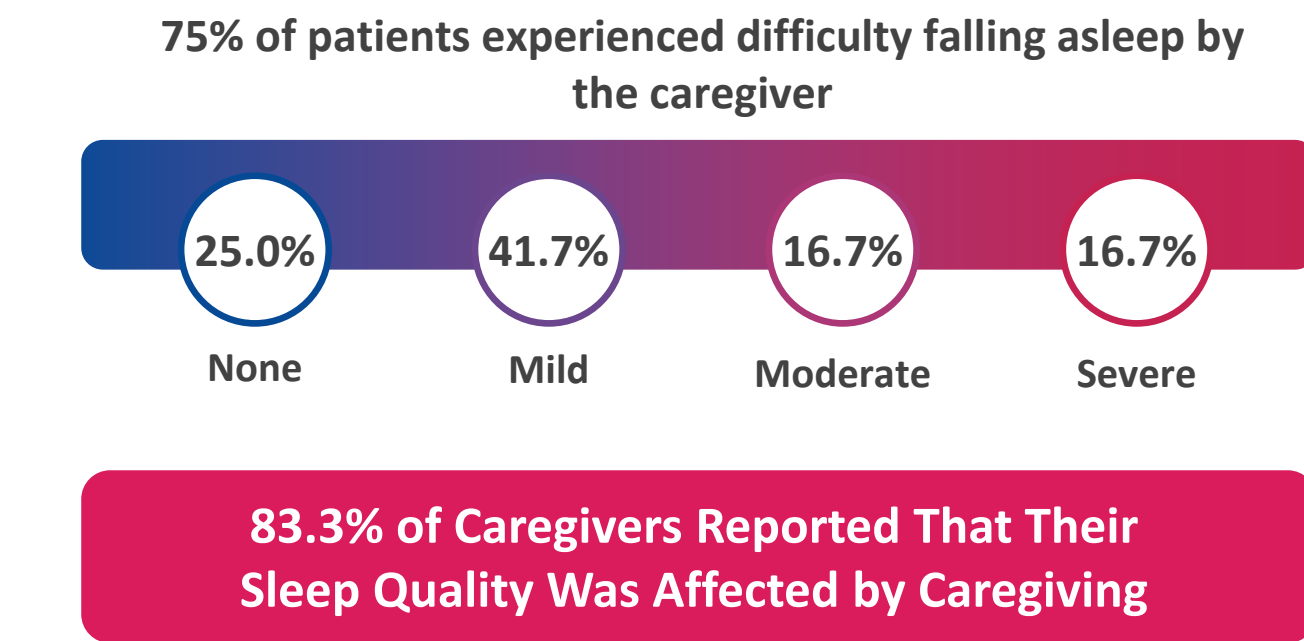
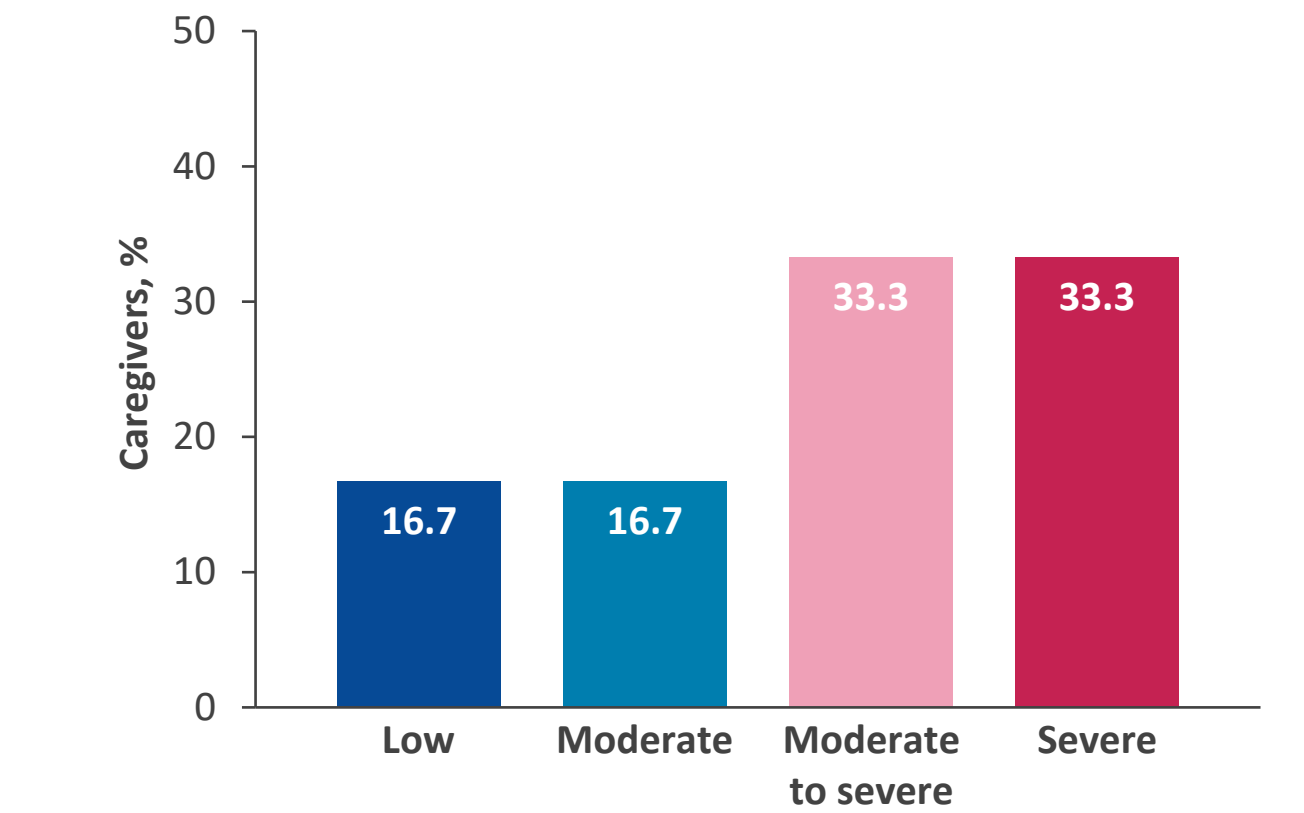


Figure 2. Impact of Caregiving on Caregiver Sleep Quality^a



Overall, only 25% of caregivers reported good sleep quality in a usual week, 58.3% reported fair sleep quality, and 16.7% reported poor sleep quality.

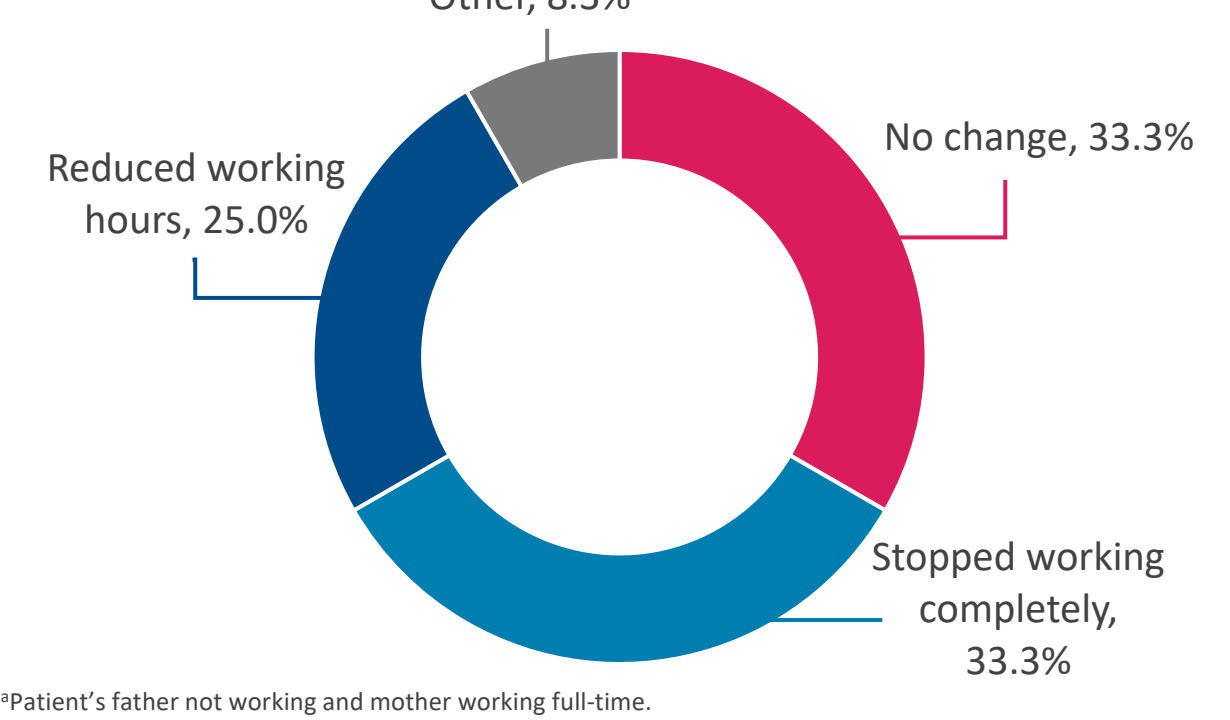
Most (75%) caregivers reported that their sleep may be somewhat to very much affected by their child's itch.

Acknowledgments

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~60% of Caregivers Reduced Work or Stopped Working Completely as a Result of Caregiving

Figure 3. Change in Employment Status Among Caregivers



Of 75% of caregivers employed outside of the home, 66.7% missed at least 8 hours worth of work, and 22.2% missed at least 40 hours or more of work per month.

Half of caregivers (50%) responded that their caregiving experience with patients with ALGS or PFIC had moderate to severe impact on their productivity.

Most (75%) caregivers reported that caregiving affected their finances.

Conclusions

- Early results from this study show a negative impact of caregiving on sleep, productivity, employment, and mental health of caregivers who care for patients with ALGS or PFIC prior to the initiation of maralixibat treatment.
- Further longitudinal data are needed to quantify more clearly the HRQoL burden on caregivers associated with caring for a patient with ALGS or PFIC and to determine whether maralixibat can lead to improvements in caregiver HRQoL.

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