

The impacts of olipudase alfa on adults with ASMD: The patient-reported experience



Solomon Mbu¹, Conan Donnelly¹, Justin Hopkins², Joslyn Crow², Toni Mathieson³

1) INPDR, 2) NNPfD, 3) NPUK



BACKGROUND

Acid Sphingomyelinase Deficiency (ASMD) is an ultra-rare autosomal recessive lysosomal storage disorder characterized by intracellular lipid accumulation in multiple organ systems resulting in significant impacts on morbidity and mortality. Olipudase alfa, an enzyme replacement therapy, was recently approved by several agencies for the treatment of the non-neurologic manifestations of ASMD. Studies demonstrate improvement in organomegaly, pulmonary function and lipid profiles with olipudase alfa. ASMD is a spectrum disorder with the variability in neurologic symptoms accounting for that spectrum. The effect olipudase alfa has on adult with ASMD symptoms and quality of life has not been documented.

OBJECTIVES

The study aims/objectives were to:

- Increase the understanding of the impacts of ASMD on adult patients,
- Explore the effects of olipudase alfa on adult patients,
- Gain insights from patients into the unmet need for treatment of ASMD

METHODS

The study consisted of an international online survey in English followed by semi-structured interviews. The survey was open to adults aged 18 years and over (or their parent/caregiver) who were fluent in English (including non-native English speakers), were able to give informed consent, had a confirmed diagnosis of ASMD and had received olipudase alfa as an experimental or approved therapy for ASMD. The semi-structured interviews were available to respondents who had consented to be contacted and had completed the online survey. Quantitative analysis of the results was undertaken. Interview transcripts were analyzed using an inductive thematic approach.

References

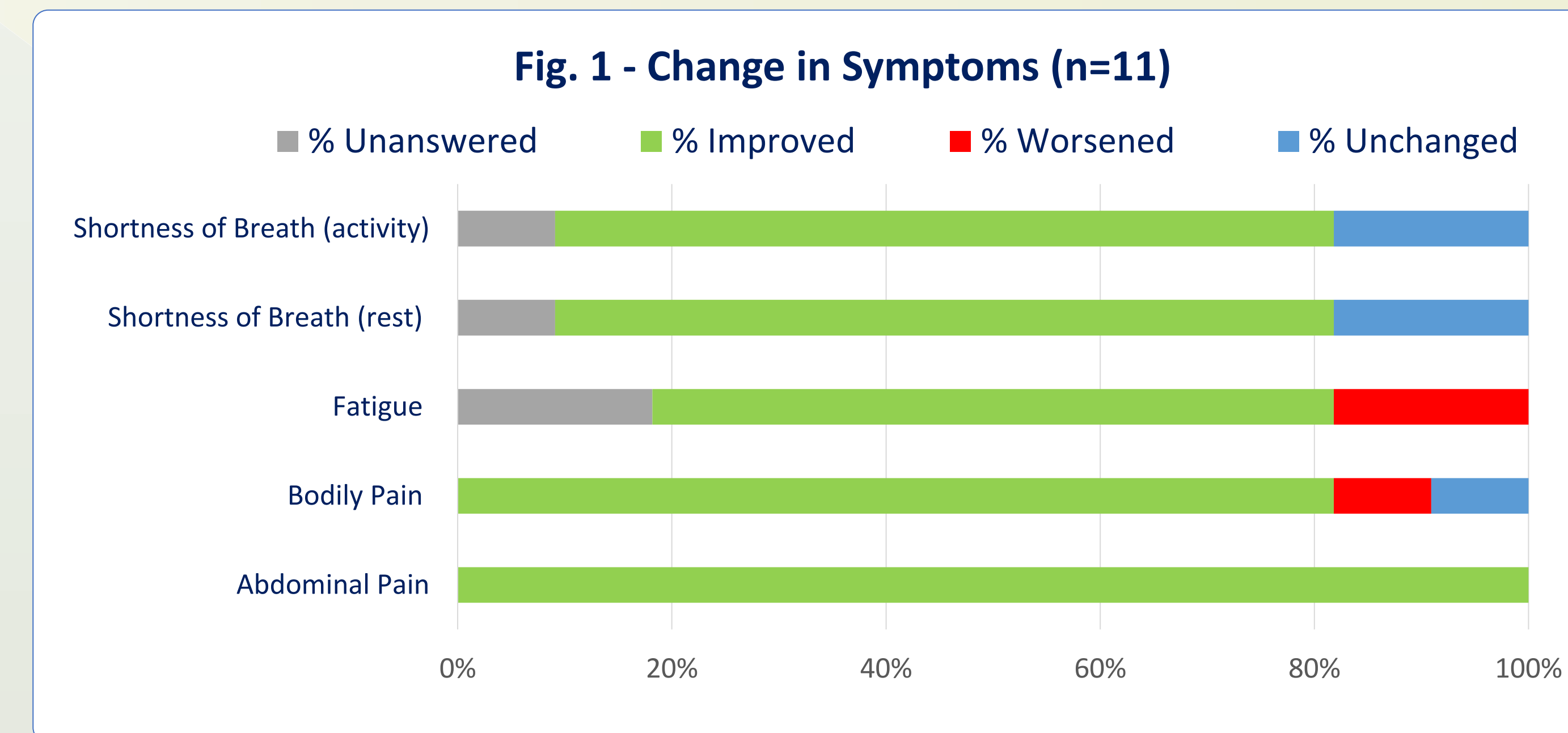
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".....Getting insurance approval was stressful. A requested change in the amount of saline required additional approval and delayed my enzyme shipment and infusion. I lost my career years earlier than expected retirement. Now with trying to arrange enzyme shipments while traveling, I feel like my life is not my own. I have many more skin problems like rashes since being on enzyme. I still am super thankful for the enzyme....."

RESULTS

78.2% of patients reported improvement in **symptoms** since starting treatment with olipudase alfa, with all patients reporting improvement in abdominal pain and approximately 80% reporting improvements in shortness of breath, bodily pain and fatigue. Few respondents reported worsening symptoms in fatigue and bodily pain (Fig. 1)

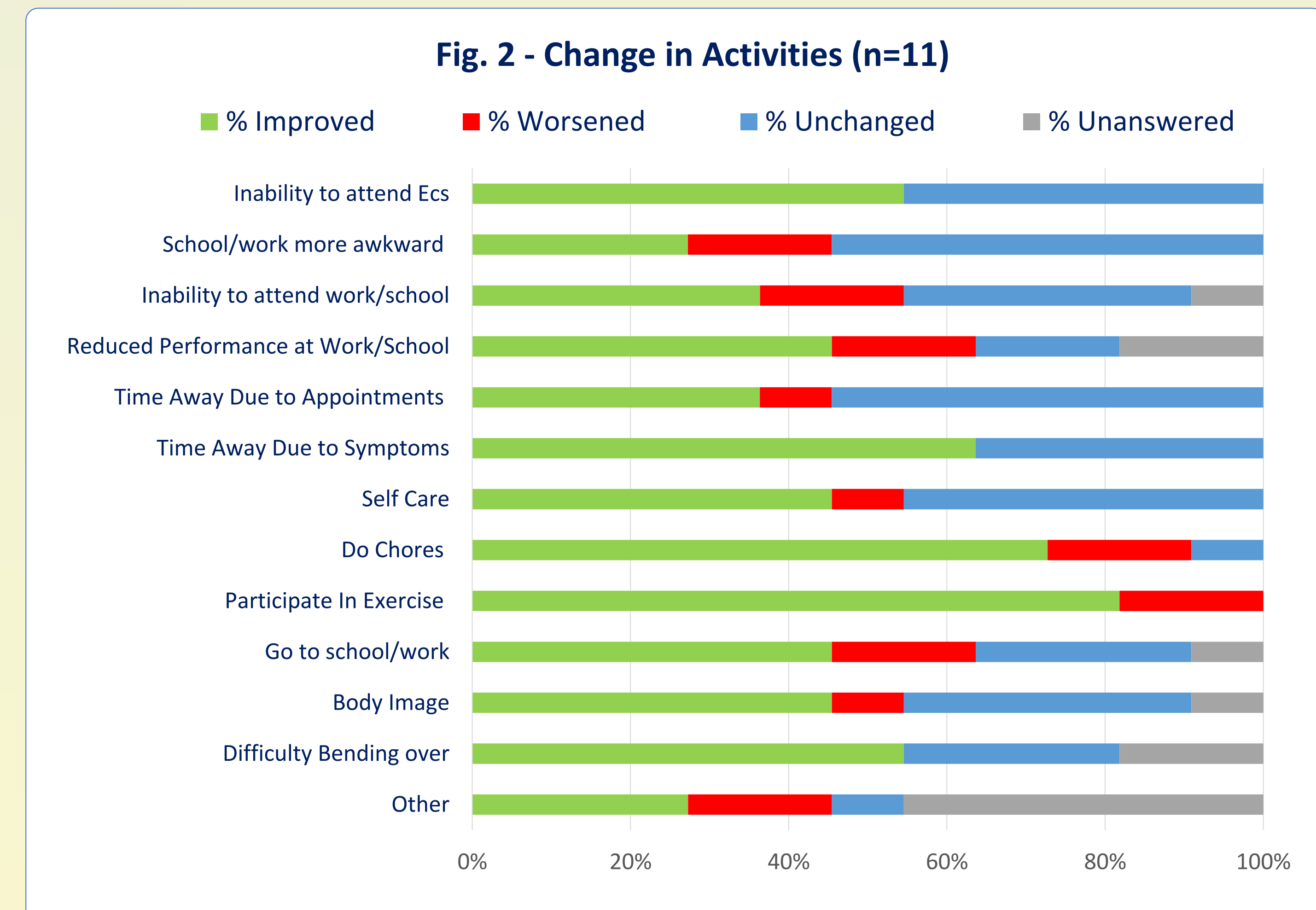
49% of patients reported improvement in their ability to perform **activities** since starting treatment with olipudase alfa, with a majority of respondents reporting significant improvement in symptoms such as ability to participate in exercise (82%), ability to do chores (73%), time away due to symptoms. In contrast, most respondents reported an "unchanged" status in symptoms such as school/work more awkward (55%) and time away due to appointments (55%). Few respondents reported worsening of ability to perform activities such as exercise, chores, and performance at work/school (Fig. 2)



"Well, I definitely missed out a lot, as far as socialising. On both those last two halves of years at high school, I missed a lot there, but I was missing a lot anyway, because I was having to stay home or go to doctor's appointments."

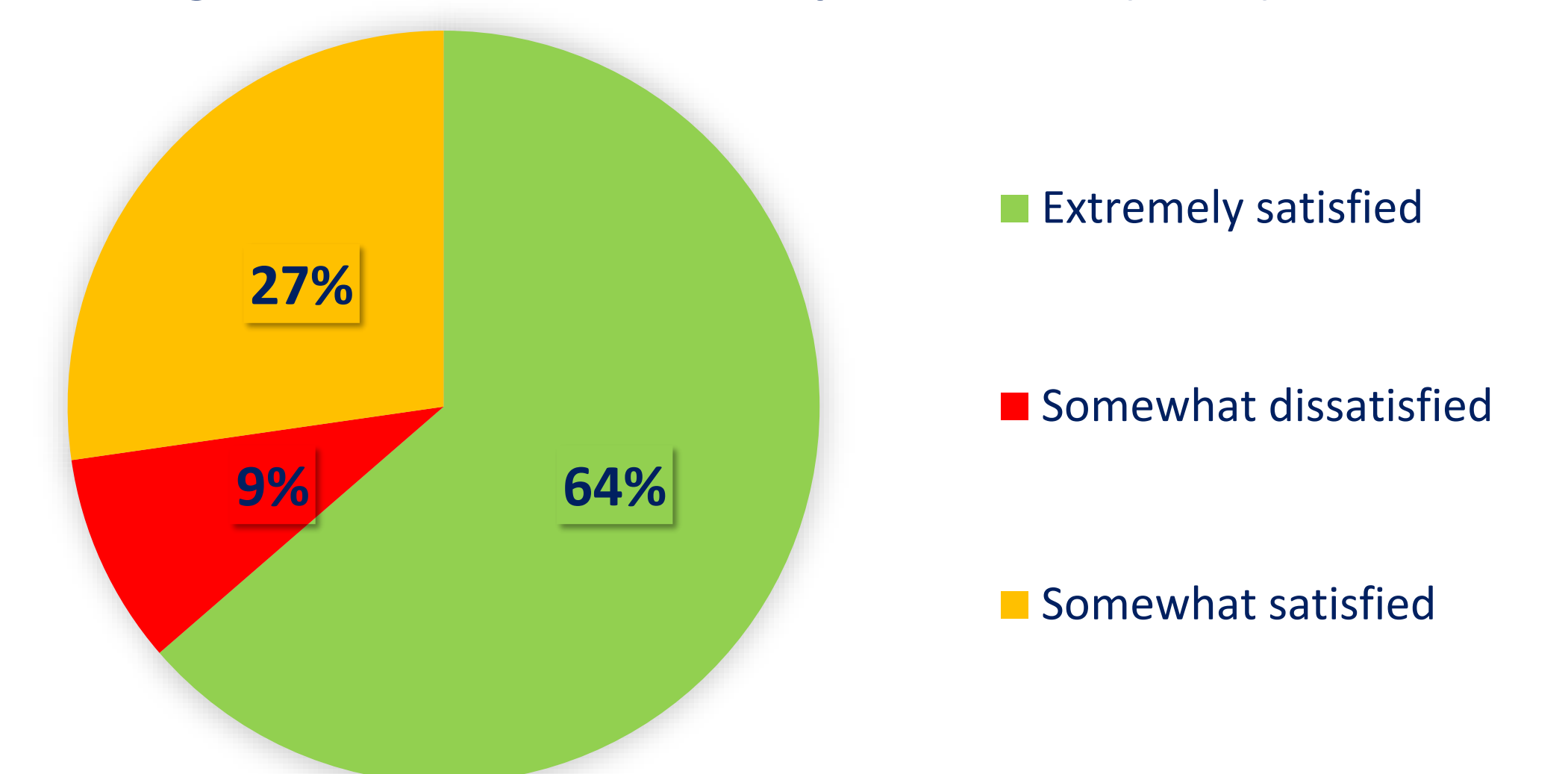
64% (7/11) of respondents were extremely satisfied with olipudase alfa to manage their symptoms of ASMD. 27% (3/11) were somewhat satisfied, while 9% (1/11) were somewhat dissatisfied with olipudase alfa in managing their symptoms of ASMD (Fig. 3).

Respondents were also asked about their global impression of disease burden since starting treatment with olipudase alfa. 46% (5/11) of respondents reported their condition improved, 36% (4/11) reported their condition stabilized, 9% (1/11) reported their condition was progressing slower than without treatment, and 9% (1/11) reported their condition was progressing faster than expected (Fig. 4)



"It has reversed years of damage and given me hope and a future...."

Fig. 3 - Satisfaction with Olipudase Alfa (n=11)



CONCLUSIONS

Olipudase alfa was associated with improvement of many of the non-neurologic manifestations of ASMD for the majority of adults over a prolonged period. Respondents perceived improvement or stabilization of disease and were generally satisfied with olipudase alfa as treatment for ASMD.

Fig. 4 - Global impression of disease burden since starting olipudase alfa (n=11)

