

EVALUATING THE IMPACT OF PEER SUPPORT AND CONNECTION ON THE QUALITY OF LIFE OF PATIENTS WITH FAMILIAL CHYLOMICRONEMIA SYNDROME

¹Salvatore V, ¹Gilstrap A, ¹Williams K, ¹Hsieh A, ²Gwosdow AR, ¹Stevenson M, ³Davidson D.

¹Akcea Therapeutics, Cambridge, MA, USA; ²Gwosdow Associates Science Consultants, LLC, Arlington, MA; ³NorthShore University Health System, Bannockburn, IL.

INTRODUCTION

FCS:

• Familial Chylomicronemia Syndrome (FCS) is a rare metabolic disorder characterized by chylomicronemia due to reduced or absent lipoprotein lipase (LPL).¹

• The severely high triglyceride levels present in FCS carry an associated risk of recurrent, and potentially fatal, acute pancreatitis.²

• Patients also commonly experience extensive emotional, mental, and psychosocial challenges from the condition as well as anxiety, depression, and worry associated with maintaining the recommended diet.³

• There currently is no approved FDA-treatment for FCS. Most patients try to manage their disease through an extremely restrictive low-fat diet (<15-20 g of fat per day), but are still at risk of symptoms.

• IN-FOCUS, a recent self-reported study of the burden of illness of patients with FCS, highlights the impact of FCS on a range of measures of quality of life among patients.³

Connection:

• Patients with FCS experience feelings of isolation and depression due to lack of awareness of FCS and the limited availability of information and disease specific resources.⁴

• Individuals living with a rare disease have a significantly impacted quality of life relative to average population and those afflicted with common chronic disease.⁵

• Receiving support and information from someone who has had a similar experience has been shown to produce significantly more improvements in traditional quality of life assessments (SF-36) in female patients with breast cancer.⁶

• Case studies have demonstrated that patients with FCS report reduced psychological distress associated with their disorder when involved in FCS-specific support groups.⁴

METHODS

Questionnaire:

• Respondents were asked a series of questions, as detailed in the flow diagram below, about their experience with FCS, level of interaction with FCS-specific groups, and self-reported assessments of several measures of quality of life.

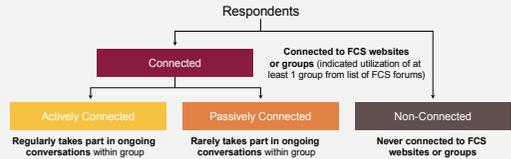
• Respondents assessed their current quality of life living with FCS or comparative assessments of quality of life before and after connection with FCS-focused support organizations.



What it Means to be Connected:

• In this study the level of connectedness was originally defined as 'passive' and 'active' and was later expanded to reflect non-connected patients.

• Post survey analysis showed noticeable differences in several quality of life measures in comparing passively, actively, and non-connected patient experiences.



Demographics:

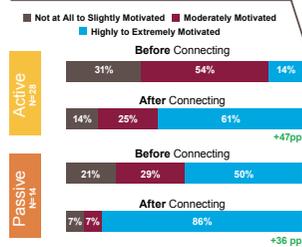
• Survey respondents could be either a patient or a care giver reporting on behalf of a patient.

• Survey was active in Canada and the United States.

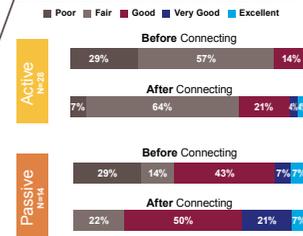
Demographics	Actively Connected	Passively Connected	Non-Connected
N	28	14	8
Gender (M:F), n (%)	13 (46) : 15 (54)	5 (36) : 9 (64)	3 (38) : 5 (62)
Age (years), Mean (range)	32 (15 – 57)	42 (24 – 68)	51 (19 – 64)
Location:			
United States, n (%)	28 (100)	11 (79)	7 (88)
Canada, n (%)	0 (0)	3 (21)	1 (12)

RESULTS

Motivation to Take Care of Health



Perception of Overall Health



SUMMARY:

Passively or Actively connected respondents reported significantly improved perceptions of overall health, disease outlook, and emotional well-being after connecting.

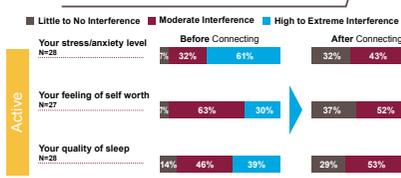
Connection increased respondents' motivation to take care of their own health, over 3 times as many actively connected respondents reported high or extremely high motivation after being connected.

As connection level increased, respondents reported higher levels of satisfaction with their primary treating physician.

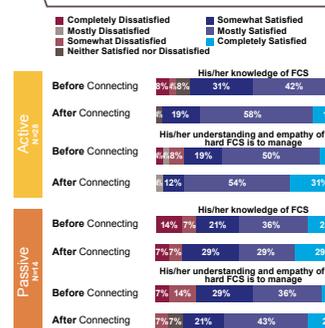
The effort to be actively connected resulted in a range of significant benefits compared to passive or non-connected.

57% of non-connected respondents reported their lack of connection was to not knowing about disease specific groups, their doctor not recommending a group, or not using online platforms to access the groups.

Reduction of Interference of FCS on Emotional and Mental Well-Being



Physician Satisfaction



Non-connected Barriers to Connecting

57% Non-connected patients reported reasons for not connecting were no knowledge of FCS specific websites or groups (29%), do not use platforms necessary to access (14%), or doctor did not recommend (14%).

BENEFITS OF BEING CONNECTED

Messages below represent the Top Benefits of Being Connected to an FCS organization as identified by respondents in the study.



"Doctors may understand the disease itself but patients with it understand the pain and struggles associated with it. It makes you feel like a human instead of a statistic."

"This group allows me a voice and freedom to know it's not just me, I'm not alone. This has incentivized me to take an active role in my health again."

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DISCLOSURES

• David Davidson: Speaker's Bureau for Amgen, Sanofi, Regeneron; Consulting for Akcea Therapeutics, Inc.
 • Valerie Salvatore, Alan Gilstrap, Karren Williams, Andrew Hsieh, and Michael Stevenson are employees of Akcea Therapeutics, Inc.
 • Andrea Gwosdow: Received payment from Akcea Therapeutics, Inc. for providing writing support.