



# Cystic Fibrosis Research News

## **Title:**

Utilization of Electronic Patient-Reported Outcome Measures in Cystic Fibrosis Research: Application to the GALAXY Study

## **Lay Title:**

Utilization of Electronic Patient-Reported Outcome Measures in Cystic Fibrosis Research

## **Authors:**

Meghana Sathe, MD<sup>\*a</sup>; Baha Moshiree, MD<sup>\*b</sup>; Phuong T. Vu, PhD<sup>c</sup>; Umer Khan, MS<sup>c</sup>; Sonya L. Heltshe, PhD<sup>c, d</sup>; Melita Romasco<sup>c</sup>, Steven D. Freedman, MD, PhD<sup>e</sup>; Sarah Jane Schwarzenberg, MD<sup>f</sup>; Christopher H. Goss, MD MSc<sup>c, d, g</sup>; and A. Jay Freeman, MD<sup>h</sup>

\*Joint First Authors

## **Affiliations:**

<sup>a</sup>Division of Pediatric Gastroenterology, Hepatology and Nutrition, University of Texas Southwestern/Children's Health, Dallas, TX

<sup>b</sup>Division of Gastroenterology, Atrium Health, Charlotte, NC

<sup>c</sup>Cystic Fibrosis Foundation Therapeutics Development Network Coordinating Center, Seattle Children's Research Institute, Seattle WA

<sup>d</sup>Department of Pediatrics, University of Washington School of Medicine, Seattle, WA

<sup>e</sup>Division of Gastroenterology, Beth Israel Deaconess Medical Center, Boston, MA

<sup>f</sup>University of Minnesota Masonic Children's Hospital, Minneapolis, MN

<sup>g</sup>Department of Medicine, University of Washington School of Medicine, Seattle, WA

<sup>h</sup>Division of Gastroenterology, Hepatology and Nutrition, Children's Healthcare of Atlanta, Emory University, Atlanta, GA

## **What was your research question?**

GALAXY focuses on the development and utilization of electronic questionnaires that could be used in the out-of-clinic (OOC) environment to understand existing GI symptomatology in persons with cystic fibrosis (PwCF) ages 2 and up.

## **Why is this important?**

With CFTR modulators significantly improving both the quantity and quality of life of persons with CF, it is important to consider how to involve an active and overall healthy population of children and adults in ongoing clinical research to continue to make improvement in the care we provide PwCF. In addition, the need to innovate ways in which to successfully conduct

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[cfresearchnews@gmail.com](mailto:cfresearchnews@gmail.com)



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clinical research in the OOC is timely amidst of the Coronavirus Disease 2019 (COVID-19) pandemic.

## **What did you do?**

We created the GALAXY ePROM which consists of three validated GI ePROMs (PAC-SYM, PAGI-SYM and PAC-QOL) were combined with a Stool-Specific questionnaire to make up the GALAXY ePROMs and administered prospectively across 26 CF centers in the United States. The ePROMs were completed at the enrollment visit and then electronically at weeks 1, 2 and 4. PwCF at least 2 years and older were eligible for the study. Reminders were only provided by the mobile application during the study window.

## **What did you find?**

There were 402 participants enrolled in GALAXY. Of those, 169 (42%) were under 18 years old and 193 (48%) were female. Eighty percent of participants had at least 1 ePROM fully completed, slightly higher in those  $\geq 18$  years of age (82.5%) compared to those  $< 18$  years of age (76.5%). When assessing the completion for all 4 ePROMs, the percentage was 77.6%, also higher among those  $\geq 18$  year of age (81.5% versus 72.2% for  $< 18$  years of age). Responses to all questionnaires were consistent over the 1-month study period, showing stability and accuracy of the GALAXY ePROMs.

## **What does this mean and reasons for caution?**

Our study demonstrated that GI symptoms can be feasibly collected with good reproducibility using the GALAXY ePROMs. This mechanism of symptom collection may provide an efficient tool for future CF trials. Our work provides a means to successfully gather data on gastrointestinal symptoms in the OOC environment in a wide range of patients.

## **What's next?**

The GALAXY ePROMs are currently being used as part of the PROMISE study, an observational of PwCF on elexacaftor/tezacaftor/ivacaftor (Trikafta™) and will be used in future GI interventional therapeutic trials, both involved drugs and treatment algorithms.

## **Original manuscript citation in PubMed**

<https://pubmed.ncbi.nlm.nih.gov/34305007/>