



Listening to the Voices that Matter: *Chronic and Recurrent Acute Pancreatitis Patient Perspectives*



A Survey of Patient
Experiences to Inform Future Decisions

Presented by:

Mission: Cure

Demonstrating a new model for curing disease

July 2018 • Z. Zhu, C. Kabelac, and G. Gil • ©Mission: Cure

Updated October 2019



A note from Mission: Cure:

Chronic pancreatitis is a painful, complex disease that impacts over 150,000 children and adults in the United States alone. Recurrent acute pancreatitis is a related diagnosis that impacts many more. There is a tremendous need to accelerate research to discover an effective treatment for this disabling disease. Mission: Cure was founded to do just that, and patient involvement is crucial to achieving this goal.

Mission: Cure is a growing nonprofit dedicated to measurably improving outcomes for chronic and recurrent acute pancreatitis patients. We believe that patients' voices need to be heard and should be placed at the center of directing drug development and evaluation. We want and need patient input to inform our conversations with the FDA, researchers, and drug developers. After all, only patients understand the facets of a complex disease that have the greatest impact on their quality of life.

However, for a community of patients who suffer from a disease that can be isolating and difficult to manage, getting their voices heard is challenging at best. Here, the patients suffering from chronic and recurrent acute pancreatitis get the chance to share their stories, and it is our job to pay attention.

By listening to their stories, Mission: Cure will be better equipped to prioritize the outcomes that matter most to patients themselves. The patients' impassioned answers will impact the direction of Mission: Cure's work and will drive us to find a cure.

Linda Martin & Megan Golden
Co-Directors
Mission: Cure

Chronic and Recurrent Acute Pancreatitis Patient Perspectives

A Survey of Patient Experience to Inform Future Decisions

Introduction

In drug development, the ultimate goal is to treat the many conditions patients experience and to improve the way patients feel, function, and survive. Therefore, patients who live with a disease should be placed at the center of the drug development process. However, the medical community has recognized that patient perspectives might differ significantly from what is expected by researchers, drug developers, FDA staff, and providers, and what patients care most about may not always be factored into clinical trials or approved labeling. This phenomenon is especially the case for rare and sometimes stigmatized diseases, like chronic and recurrent acute pancreatitis. Thus, under the direction provided by the FDA Patient-Focused Drug Development (PFDD) Initiative, we at **Mission: Cure** have begun an effort to understand patient experiences, needs, and priorities, starting from this patient survey launched in summer 2018. This report serves to update the preliminary findings released in July 2018 with the complete responses received up to December 2018.

This survey is an open-ended form that allows patients and caregivers to share insights into their experiences living with chronic and recurrent acute pancreatitis. It is intended to better inform **Mission: Cure**'s approach to curing pancreatitis by giving us a sense of the challenges faced by the patient population and laying the groundwork for further patient engagement and other PFDD efforts. With the findings of this report, **Mission: Cure** will be better equipped to make the critical decisions necessary to achieve our goals of patient advocacy and bringing better, more effective treatments to patients as quickly as possible.

Objectives and Methods

Throughout this survey, our primary objective is to better understand the perspectives of the pancreatitis patient community by collecting their stories and priorities.

Using an online, open-ended survey, we asked patients a series of eight questions (see Appendix 1). The first seven of these questions involved the collection of demographic information that will better equip us to advocate for patients and plan patient engagement strategies. The last question is of primary importance to this report, asking "If you had a chance to talk to the FDA, what would you want them to know about your experience with chronic or recurrent acute pancreatitis?" The responses have no structured requirements. Additionally, none of the questions were required for the completion of the survey, giving respondents the opportunity to report only the information they felt comfortable sharing.

We analyzed the results of the demographic questions, producing associated graphs in the process. For the last question regarding their personal experiences, we developed a codebook of recurring themes and coded each story individually using the software NVivo. The codebook and the consecutive coding was developed with collaboration by multiple individuals to minimize individual biases involved in understanding qualitative data. The same codebook was utilized in the data analysis of this report. The open-ended nature of this last question allowed respondents to spontaneously respond and did not direct their response. For this reason, the concordance of themes between narrative was important to examine. We identified major themes from the available set and chose quotations that best represented the overarching topics.

Results

A total of 261 responses were collected through the survey. The first 145 of these made up the findings reported in the preliminary findings, while the last 116 responses have been incorporated into the findings reported hereafter. The people who participated were in some way involved in the patient community, either as patients themselves or as caregivers of patients. In the case of the latter, we requested that they respond to the survey from the perspective of the patient they care for.

Respondent Characteristics

Age

Reported by 229 respondents.

The mean age of survey participants was 41.5 years. The standard deviation of age distribution was 15.6. The age of patients ranged from 4 years old (where the caregiver responded to the survey) to 74 years old.

Gender

Reported by 259 respondents.

194 identified as female (74.9%), and 65 identified as male (25.1%).

Diagnoses

Reported by 257 respondents.

213 have been diagnosed with chronic pancreatitis (82.9%), 108 with recurrent acute pancreatitis (42.0%), and 52 with hereditary pancreatitis (20.2%).

Notably, most respondents report being diagnosed with more than one of the aforementioned conditions, leading to a greater number of diagnoses than respondents. Patients also had the option to self-report related diagnoses like diabetes or pancreatic divisum under the “Other” category on the question (see Figure 1).

Predominant Symptoms

Reported by 134 respondents.

In the first long-form question, survey respondents were asked to list three symptoms that have the most significant impact on their day-to-day life.

Pain is the most frequently mentioned as one of the three symptoms that has the greatest impact on their day-to-day life. It appears in the response of 92.5% of total respondents. Nausea and lack of appetite (62.8%) and fatigue (33.1%) were the second and third most prevalent symptoms reported.

Other symptoms mentioned by patients include abnormal bowel movements, depression or anxiety, and weight loss (see Figure 2).

Treatments

Reported by 246 respondents.

In the second long-form question, survey respondents were asked to describe their current treatment regimen, including medications, non-pharmacological therapies, or anything else that they may deem relevant.

The most common treatment approaches patients described were digestive enzymes (61.4%), diet modifications (37.0%) and unspecified pain medications (37.0%). If specific pain management techniques (celiac nerve blocks, cannabis, and opioids) are combined with the reports of unspecified pain management, then 61.8% of survey participants reported some form of chemical-based pain management, including 44 respondents who reported using opioids for this purpose. Other frequently cited treatments included unspecified medications (26.8%), nausea medications (14.6%), and surgical options (13.0%) (see Figure 3).

What do you want the FDA to know?

In the final question, survey participants were given an open-ended, narrative prompt that asked “If you had a chance to talk to the FDA, what would you want them to know about your experience with pancreatitis?”

The themes extracted from the responses to this question are on following pages. To note: Respondents that did not answer this open-ended question or provided invalid/irrelevant answers are excluded from data analysis (e.g. answered “Yes”, “N/A”). Because of this, 42 survey participants were not included, and we have 218 total valid answers for this question.

The percentage/number of respondents provided in this report correspond to participants that spontaneously mentioned the themes. These numbers may not be reflect the total percentage/number of participants that share these experiences.

Because of the open-ended nature of this question, the concordance between answers is particularly important to note. Similar themes were found both in the preliminary findings and those updated in this report, with slight differences in the prevalence of the sub-themes identified. The concordance of responses indicates that the major themes are shared across patient experiences and suggest trends across pancreatitis patients that merit further research.



Figure 1. Diagnoses Reported by Survey Respondents

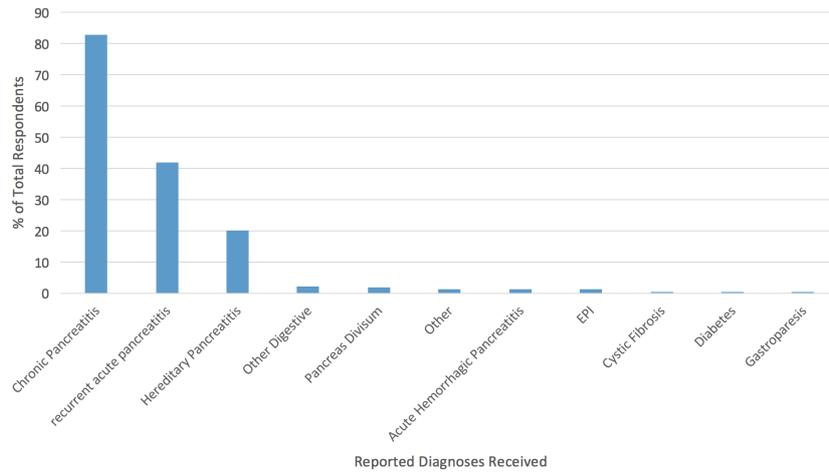


Figure 2. The Most Significant Symptoms as Reported by Survey Respondents

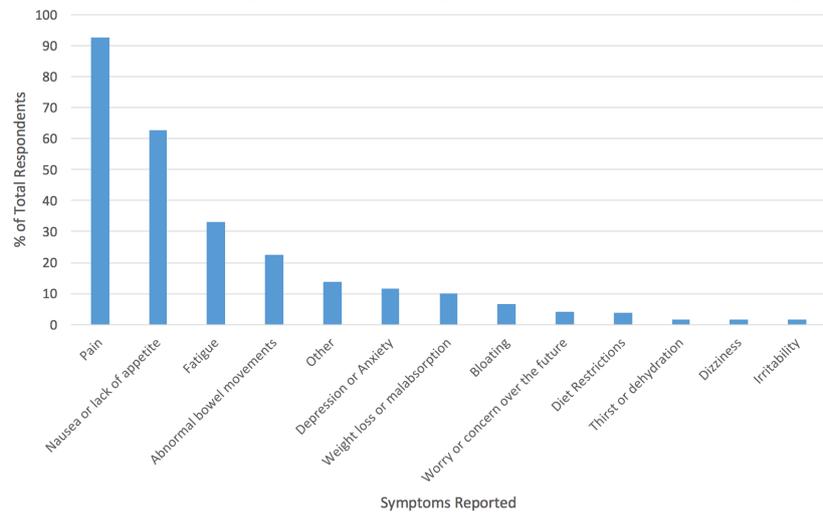
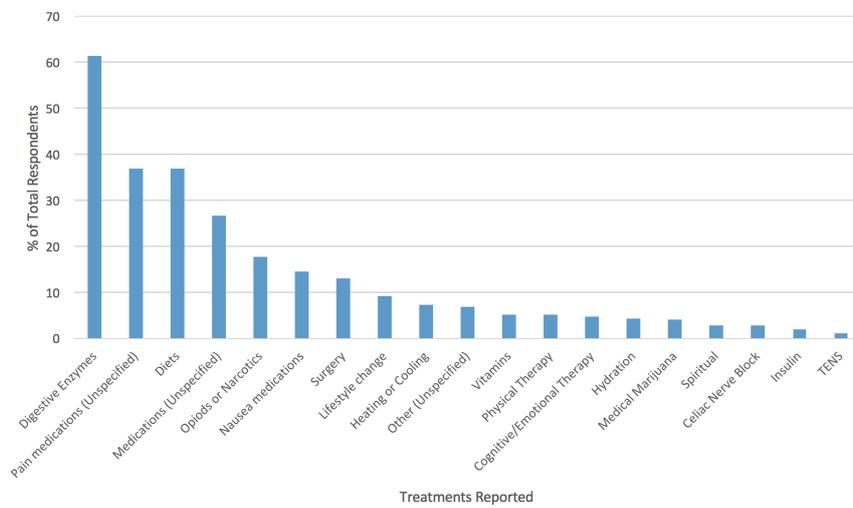


Figure 3. Treatment Regimes as Reported by Survey Respondents



Major Themes

1 Lack of Treatments

Reported by >70% of respondents

More than half of all respondents mentioned the lack of available treatments. Among those, patients overwhelmingly emphasized the limitations around pain medication and management (34.9%), especially under the current opioid restrictions, far surpassing mentions of other treatment inavailability.

Many respondents speak to the limitation of current treatment and the unmet medical needs, including misdiagnosis and late diagnosis, having to go the ER repeatedly when pain is unbearable, ineffective medications, affordability and insurance issues, particularly for experimental options and surgery, and the need to travel far for receiving treatments. 10.6% of respondents mentioned surgical treatment options. Of these, a few reported the positive impact of TP/IAT surgery on their disease condition and quality of life, but the difficulties of getting the surgery, including insurance coverage, were also highlighted, both by those seeking to get surgery in the future and those who had already undergone it. Furthermore, those in the former group generally spoke about the surgery fearfully and as a last resort measure in the absence of other options.

Some survey participants explicitly called for future action. Beyond improved pain management, some of the measures they ask for included:

- The development of more and new treatment options for pancreatitis specifically
Called for by 20 respondents
- Alternatives to frequent and expensive ER visits due to extreme pain and ineffective pain management
Highlighted by 20 respondents
- The development of new diagnostic tools that will prevent misdiagnosis or misattribution of the symptoms
Stressed by 16 respondents

Patient Statement

“This disease is horrible, I am in constant pain. I was referred to get the tp/ait surgery but because Medicare won’t cover the ait part I haven’t been able to. It is hard to get pain meds for this even with confirmed damage to the pancreas because of the opioid crack down.”

2 Burden of Symptoms and Impact on Life

Reported by >55% of respondents

95 respondents (43.6%) described their symptoms in their answers, including pain, nausea, fatigue, dehydration, and stomach/bowel symptoms. Pain dominates – out of these 95 respondents, 78 of them directly mentioned the debilitating pain.

121 respondents (55.5%) mentioned the impact of chronic pancreatitis on their life. 23 of these respondents highlighted a general decrease in their quality of life following the onset of their symptoms. In more specific terms, patients reported several other major ways chronic and recurrent acute pancreatitis have impacted their lives, including:

- Constraints on food and diet
Mentioned by 25 respondents
- Impact on life-changing decisions, such as quitting jobs because of chronic pancreatitis
Mentioned by 14 respondents
- Causing other health problems, such as malnutrition, diabetes, etc.
Mentioned by 12 respondents
- Influence on mental health, including suicidal ideation
Mentioned by 23 respondents
- Lack of predictability of symptoms, and thus, the possibility of planning things ahead
Mentioned by 10 respondents
- Impact on social life and relationships/friendships
Mentioned by 7 respondents

Patient Statements

“I would let them know how this truly affects one’s life. It isn’t a disease that you get over in a few days. It rearranges your whole lifestyle and plans. There are foods that can no longer be eaten, there is pain that doesn’t decline, and you can never make plans because you never know when this disease is going to rear its ugly head. I’ve been battling this for 15 years and it hasn’t gotten any easier.”

“This is a horrible disease. It takes over your life. Pain and nausea that comes on without warning. Effects sleep and everyday life and job. Not wanting to take a narcotic for fear of addiction. The cost of enzyme treatment is horrendous yet you need it. The up and down of not knowing how you will feel for the day affects your outlook on things which leads to being down.”

3 A Huge Lack of Understanding from Medical Professionals

Reported by >67% of respondents

Patients frequently feel a lack of understanding from medical professionals (especially ER doctors and general practitioners) who are poorly-informed of the various causes and painful symptoms of chronic pancreatitis. This can lead to dismissal, mistreatment, improper pain management, or lack of diagnosis.

Among the respondents that specifically mentioned their experience with improper treatment, common experiences included:

- Pain being dismissed or labeled as “faking it”; being labeled as “drug seeker,” especially at the ER and by medical professionals
Reported by 43 respondents
- Being made to feel that their condition was self-inflicted through alcohol use, particularly with non-alcohol etiologies
Reported by 21 respondents
- Struggling to afford or have their insurance cover the care they needed
Reported by 27 respondents

Patient Statement

“I am always in pain. Even on the morphine and hydrocodone. My amylase and lipase levels don’t raise anymore because my pancreas is so damaged, so I get blown off by doctors. The ones who take the time to scan my stomach see how swollen the pancreas is and know there’s a problem. My doctors where I live are uneducated about pancreas issues so I frequently get sent out of state. The doctors frequently dismiss me even when I have symptoms. I, as well as many others that I know with the disease often get dismissed and called drug seekers even though we really don’t want to be on the meds but we have to be to function.”

4 Information and Research Needed

Reported by 14% of respondents

Patients called for more research on the causes and treatments of chronic pancreatitis. Respondents reported feeling “alone” and “scared” in treating the disease because of the huge lack of available information, with multiple patients saying that the “patient usually knows more than the doctors.” Pediatric conditions, as mentioned by some patients and caregivers, is an area that specifically requires research.

Patient Statements

“This disease needs much research and doctors need to be up to date on all advancements. I feel like everything I’ve learned has been from support groups, not doctors!”

“Doctors all seem to be giving very different treatment advice. Some will tell you to eat whatever you want, while others tell you to eat a very specific diet, and this kind of mixed information can be very confusing and disheartening to a young patient.”



The report update was conducted by Gabriela Gil on behalf of the Mission: Cure team. The preliminary report was authored by **Mission: Cure**'s 2018 summer interns and the rest of the Mission: Cure team. From the bottom of our hearts, we would like to thank all of the patients who openly shared their stories with us. This report would not have been possible otherwise.
- Zixi (Jessica) Zhu, Carly Kabelac, & Gabriela Gil
2018 Summer Interns, **Mission: Cure**

Appendix 1:

Survey Instrument

Dear Pancreatitis Patient or Caregiver,

You probably know that chronic pancreatitis is a painful, complex disease that impacts over 150,000 children and adults in the United States alone, and there are also people who have had multiple acute episodes. There is a tremendous need to accelerate research to discover an effective treatment for this disabling disease. Mission: Cure was founded to do just that, and today you can help us achieve this goal.

Mission: Cure is a growing nonprofit dedicated to measurably improving outcomes for chronic/recurrent acute pancreatitis patients. We believe that patients' voices need to be heard and should be placed at the center of directing drug development and evaluation. We want and need your input to inform our conversations with the FDA, researchers, and drug developers. Please take a few moments to complete this short survey in regards to your patient perspective—your input is extremely important and will truly make a difference.

Thank you for your time and contribution!

Sincerely,

Linda Martin and Megan Golden

Co-directors, Mission: Cure



Demographics

Note: If you are a caregiver, please answer the following questions from the perspective of the pancreatitis patient you care for.

Age:

Gender:

Where are you from?



Please share your experience

Have you been diagnosed with the following condition(s)? Please select all that apply.

- Chronic Pancreatitis
- Recurrent Acute Pancreatitis
- Hereditary Pancreatitis
- Other _____

Please list three symptoms that have the most significant impact on your day-to-day life.

What is your current treatment regimen? You can include medications, non-pharmacological therapies, or anything you think could be relevant.

If you had a chance to talk to the FDA, what would you want them to know about your experience with pancreatitis?

Future Information

If you would like to further contribute to our relentless mission to cure chronic pancreatitis by providing your patient perspective, or to receive news and information from Mission: Cure, please leave your contact information below. If you have any questions or comments, please email info@mission-cure.org

Email:

Cell Phone:

How did you find out about this survey?

- Patient Facebook groups
- Mission: Cure’s website
- Mission: Cure’s social media
- My medical professional
- Other people
- INSPIRE
- Other _____



About Mission: Cure:

We are a coalition of doctors, researchers, patients, and entrepreneurs pioneering a new approach to curing disease, especially pancreatitis, through innovative outcome-based financing.

We aim to demonstrate that innovative financing can be harnessed in the field of medical research and development for diseases. Our first target is chronic pancreatitis, and our approach involves Pay-for-Success financing. This model brings together payers, patients, researchers, and impact investors to streamline the process of getting treatments into the hands of the people that need them the most. **Mission: Cure** serves as an intermediary for a model that benefits everyone. If a set of measurable, pre-determined outcomes are reached, impact investors get positive financial returns, traditional payers, benefit from lowered long-term healthcare costs, and patients' health outcomes improve.

*To find out more about our organization or how to get involved, visit <https://mission-cure.org>.
If you have any further questions regarding this project,*

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