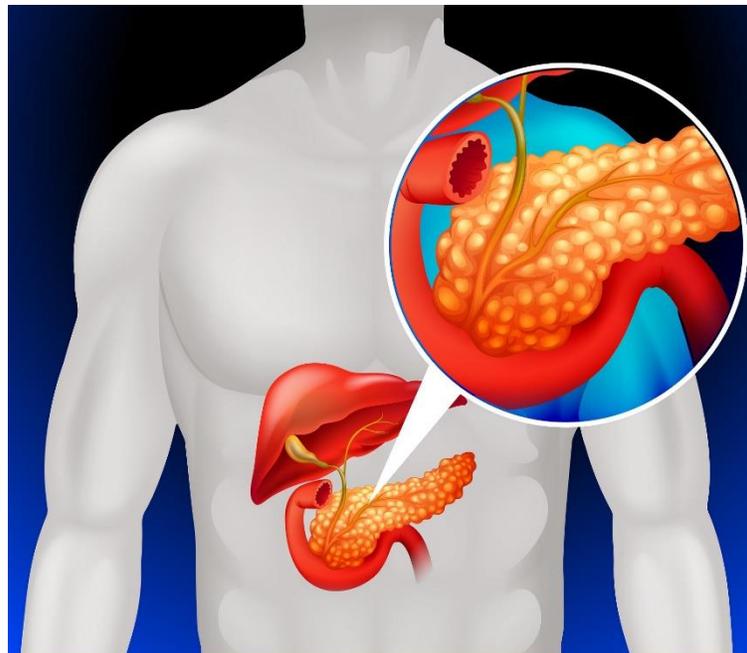




The  
**National Pancreas**  
Foundation

# **Externally-Led Patient- Focused Drug Development Meeting on Pancreatitis**

*March 3, 2020 | Silver Spring, MD*



## **WELCOME LETTER**

### **Dear Members of the Pancreatitis Community,**

On behalf of the National Pancreas Foundation, I want to welcome you to the Externally-Led Patient Focused Drug Development Meeting for Pancreatitis. We are so pleased that you are taking part in this event either in person or by webcast. We and our partners, Mission: Cure, Rebecca's Wish and the FCS Foundation, are eager to put a spotlight on the stories of patients and their caregivers who have been affected by acute and chronic pancreatitis.

We know that it will be impossible to hear the experiences of the hundreds of thousands of patients, including children, adolescents and adults, who have been the victims of these diseases each year in the United States. But representatives of the U.S. Food and Drug Administration are here today to hear a sample of the experiences that describe the burdens of these diseases, the problems in finding treatment, and the lasting effects of these conditions.

People affected by acute and chronic pancreatitis suffer from long-term complications of these diseases and are especially aware that there are no approved drug treatments to shorten the course or prevent ongoing problems due to these conditions. They are eager for cures or for treatments which will make real improvements in the outcomes of pancreatitis.

By being here today, you are providing valued input to the processes which can guide the clinical trials of new drugs and treatments for pancreatitis. Your testimony is invaluable to illuminate the full spectrum of the effects of these diseases. Thank you so much for helping to make a difference and for helping us to understand the real impact of pancreatitis.

Sincerely,

A handwritten signature in cursive script that reads "Dana K. Andersen" followed by a horizontal line.

Dana K. Andersen, MD  
Chair, Board of Directors, National Pancreas Foundation



## Externally-Led Patient-Focused Drug Development Meeting on Pancreatitis

### Silver Spring Civic Building

1 Veterans Pl, Silver Spring, MD 20910

8:30 AM – 12:30 PM | March 3, 2020

<b>8:00 – 8:30 AM Registration and Continental Breakfast</b>	
<b>Opening Remarks and Morning Overview</b>	
8:30 – 8:45 AM	<b>Welcome and Opening Remarks</b> <i>Dana K. Andersen, MD</i> <i>Chair, Board of Directors, National Pancreas Foundation</i>
8:35 – 8:45 AM	<b>Erica Lyons, MD – Overview of PFDD</b> <i>Medical Officer Team Leader</i> <i>Division of Gastroenterology and Inborn Errors Products</i> <i>Center for Drug Evaluation and Research</i> <i>U.S. Food and Drug Administration</i>
8:45 – 8:55 AM	<b>Overview of Pancreatitis</b> <i>Wahid Y. Wassef, MD</i> <i>Chief of Endoscopy</i> <i>Director, Pancreas Clinic, UMASS Memorial Health Care</i> <i>Professor, University of Massachusetts Medical School</i>
8:55 – 9:05 AM	<b>Introduction, Overview of Meeting, and Demographic Polling</b> <i>James Valentine, JD, MHS</i> <i>Meeting Moderator</i>
<b>Panel 1: Acute and Chronic Pancreatitis - Patient Perspective on the Burdens of the Disease and Current and Future Approaches to Treatments</b>	
9:05 – 10:25 AM	<b>Symptoms and Daily Impact   Current and Future Approaches to Treatments</b> <ul style="list-style-type: none"> <li>• Presentations by 4 Affected Individuals and Caregivers (20 minutes)</li> <li>• Moderated Audience Discussion Panel #1 (60 minutes)</li> </ul>
<b>10:25-10:40 AM Break</b>	
<b>Panel 2: Pediatric Pancreatitis - Patient Perspective on the Burdens of the Disease and Current and Future Approaches to Treatments</b>	
10:40 -10:50 AM	<b>Pediatric Pancreatitis – Patient Challenges and Obstacles to Care</b> <i>Melena D. Bellin, MD</i> <i>University of Minnesota Medical Center</i> <i>Schulze Diabetes Institute</i> <i>Associate Professor Pediatric Endocrinology</i> <i>Director of Research, Islet Autotransplant Program</i>
10:50 AM -12:10 PM	<b>Symptoms and Daily Impact   Current and Future Approaches to Treatments</b> <ul style="list-style-type: none"> <li>• Presentations by 4 Affected Individuals and Caregivers (20 minutes)</li> <li>• Moderated Audience Discussion Panel #2 (60 minutes)</li> </ul>
12:10 - 12:20 PM	<b>Summary Remarks</b> <i>Larry Bauer</i> <i>Sr. Regulatory Drug Expert, Hyman, Phelps, &amp; McNamara, P.C.</i>
12:20 PM –12:30 PM	<b>Next Steps, Summary, and Closing Remarks</b> <i>Jane M. Holt</i> <i>Co-Founder, National Pancreas Foundation</i>

# **ABOUT THE NATIONAL PANCREAS FOUNDATION**

[www.pancreasfoundation.org](http://www.pancreasfoundation.org)

## **Our Mission**

The National Pancreas Foundation (NPF) provides hope for those suffering from pancreatitis and pancreatic cancer through funding cutting edge research, advocating for new and better therapies, and providing support and education for patients, caregivers, and health care professionals.

## **Our History & Research Impact**

To fulfill its mission, the NPF raises and manages a charitable foundation from which grants are made directly to researchers seeking to resolve the challenging medical problems of pancreatic diseases. The NPF is the only foundation dedicated to patients who are suffering from all forms of pancreas disease – pediatric pancreatitis, acute pancreatitis, chronic pancreatitis and pancreatic cancer. A National Patient Registry is being developed for individuals suffering the isolating and debilitating conditions associated with pancreatic disease.

The NPF and its founding members and volunteers are committed to maximum efficiency. The Foundation's goal is to direct as much of our revenue to programs. To achieve this we rely heavily on the invaluable donations of time, energy and financial support of the many friends and interested parties who have been touched by the suffering of so many people. Research grant proposals from a wide variety of researchers are solicited. With the help of the Foundation's Grant Review Council, the NPF makes grants to those whose proposals appear most likely to lead to scientific advancements in the field of pancreatic disease. Founded in 1997, the NPF has given over \$4 million to fund 121 groundbreaking research projects. The Foundation advocacy efforts have also been very effective, actively participating on the advisory council for National Institute of Diabetes and Digestive and Kidney Diseases, the National Cancer Institute Gastrointestinal Steering Committee, and as Department of Defense Consumer Reviewers. Furthermore, the NPF has supported initiatives that have led to millions of dollars of additional research funding by the National Institutes of Health, the Department of Defense, and other institutions.

## OUR PARTNERS



The Familial Chylomicronemia Syndrome (FCS) Foundation was established by Lindsey Sutton and Melissa Goetz, two disease advocates directly impacted by FCS. In addition, The FCS Foundation board of directors includes additional leaders from the FCS patient community.

FCS is a genetic condition that causes a buildup of fats in the blood that can increase the risk of severe abdominal pain and potentially fatal attacks of pancreatitis. It is also sometimes known as lipoprotein lipase deficiency (LPLD), Fredrickson Type 1 hyperlipoproteinemia and familial hypertriglyceridemia. There are currently no FDA approved therapies for the treatment of FCS. This disease frequently leads to symptoms such as pancreatitis and fatty deposits in the skin. Most patients take steps to reduce their risk of pancreatitis through lifestyle modifications including strictly limiting fat intake. FCS occurs in about 1 in 1 million people and the genetic disorder causes the body to not be able to break down certain fats correctly. The mission of The FCS Foundation is to make sure that all people have access to the information and support they need to get a proper diagnosis and manage FCS more effectively.

**Mission: Cure** Mission: Cure is a nonprofit organization founded and led by Chronic Pancreatitis patients and their families with a single mission- to find effective treatments and ultimately cures that will improve the lives of children and adults affected by chronic and recurrent acute pancreatitis. To achieve this urgent goal, Mission: Cure applies an innovative funding approach that leverages the best aspects of venture philanthropy, angel investing and monetary incentives to fund promising research and development aimed at delivering effective treatments to patients as quickly and efficiently as possible. Mission: Cure brings together patients and their families, researchers and industry leaders from a range of disciplines to align priorities and advance multiple areas of research in parallel, driving progress toward our goal to measurably improve patients' lives. This PFDD meeting is an important part of that effort. We have already made significant progress and leading scientists say chronic and recurrent acute pancreatitis treatments are within reach. With the support of the pancreatitis community and collaboration with all stakeholders, we are optimistic that we will achieve our goal to find a cure and put an end to pancreatitis pain and suffering!



### **Rebecca's Wish**

Rebecca's Wish is a nonprofit organization started by a 17- year old high school junior from San Antonio, Rebecca Taylor. Rebecca founded this 501(c)3 in 2018 as a result of her ten-year struggle with chronic pancreatitis and post TPAIT complications. Rebecca's Wish came to life after Rebecca was referred to Make-A-Wish and asked to

share her one, true wish. She replied that she wanted to meet with a medical philanthropist to help start a nonprofit that helps children just like her. Among our many accomplishments, Rebecca's Wish spearheaded an unprecedented agreement with local hospitals to help train the world's first pediatric-trained pancreatic endoscopists. We have funded the placement of two of these highly-specialized physicians in hospitals (Los Angeles and New Orleans). We are currently training a third pediatric-trained pancreatic specialist and intend to train one new physician every year. The mission of Rebecca's Wish is to promote the prevention and treatment of pancreatic diseases through education, partnership, and research while supporting the children and their families affected by these ailments.

## **SPEAKERS**



### **Dana K. Andersen, MD, FACS**

Dr. Andersen received his undergraduate and medical school training at Duke University, where he was a member of Alpha Omega Alpha, the medical honorary society. He initially planned to become a Gastroenterologist, but a 6-month elective as a General Surgery resident convinced him that abdominal surgery was more interesting and more fun. He became Board Certified in both Internal Medicine and Surgery, and rose through the ranks of academic surgery to become Chief of General Surgery at Yale, Chair of Surgery at UMass, and Chief of Surgery and

Vice Chair of Surgery at Johns Hopkins Bayview Medical Center. In 2012 he joined the Digestive Disease Division at NIDDK.

In 2015, Jane Holt (co-founder) asked him to join the Registry Steering Committee, and in 2017 he joined the NPF Board of Directors. Dr. Andersen's research and clinical interests include both chronic pancreatitis and pancreatic cancer, and the mission of the NPF fits squarely with those interests. Before joining the NIH as a program officer in NIDDK, he cared for many patients with both forms of pancreatic disease and knows fully the challenges facing both patients and physicians. His transparent agenda at NIDDK is to increase funding programs related to pancreatic disease. The success of other disease-oriented foundations in driving awareness and research funding convinces him that the NPF has an important role in improving the care of patients with both benign and malignant pancreatic disease through its educational, support, and advocacy programs and is happy to contribute in any way to advance that mission.

Dr. Andersen currently serves as the Chair of the NPF Board of Directors and is an adjunct Professor of Surgery at Oregon Health and Science University in Portland, where he lives.



**Larry J. Bauer**

Larry provides counsel to drug manufacturers on a wide range of regulatory topics including Rare Pediatric Disease Designation requests, Orphan Drug Designation requests, Fast Track and Breakthrough Therapy Designation requests, drug development regulatory strategy, preparing regulatory submissions, and drafting regulatory policies and procedures. Mr. Bauer has extensive experience in patient advocacy and has expertise in the planning and execution of Patient Focused Drug Development meetings.



**Melena D. Bellin, MD**

Dr. Bellin is an Associate Professor of Pediatric Endocrinology and Diabetes and the Director of Research for Islet Autotransplantation at the University of Minnesota. She completed her medical school, pediatric residency, and endocrinology fellowship at the University of Minnesota, where she currently works in the Division of Endocrinology, Departments of Pediatrics with a joint appointment in the Department of Surgery (Schulze Diabetes Institute). Her research focuses include clinical islet transplantation for type 1 diabetes, chronic pancreatitis related diabetes mellitus, and total pancreatectomy and islet autotransplantation (TPIAT) for patients with severe chronic pancreatitis. In 2019 she received

a Presidential Early Career Award for Scientists and Engineers (PECASE) for her research on the treatment of chronic pancreatitis with TPIAT.



**Jane Holt**

Jane has spent the last 25 years committed to volunteer work in the Boston area. As a young mother, she became very involved in educational activities for her children. Having spent a decade surviving chronic pancreatitis, Mrs. Holt saw an acute need for greater awareness and education around diseases of the pancreas. And so, in 1997, she founded the National Pancreas Foundation with Co-Founder, Patter Birsic. She has been very involved in getting pancreatitis listed as one of the research topics for the Department of Defense Peer Review Medical Research Program and has served as a consumer reviewer for these programs. She also served as a member

of the Advisory Council of the National Institute of Diabetes and Digestive and Kidney Diseases. Ms. Holt is a graduate from Skidmore College with a B.A. in Biochemistry, and lives in the Back Bay of Boston.



**Erica Lyons, MD**

Dr. Lyons is a clinical team leader for the Gastroenterology team in the Division of Gastroenterology and Inborn Errors Products (DGIEP). Dr. Lyons was educated in Biology as an undergraduate and received her medical degree at the University of Louisville, where she subsequently completed a pediatric residency prior to joining the university faculty. During her time in Louisville, she served as the Medical Director for a pediatric practice focused on special needs populations and acted as the site's research coordinator, investigator, and PI for several clinical studies. She joined the Center for Drug

Evaluation and Research as a Medical Officer in DGIEP in January of 2017, where she evaluates numerous drug development programs for gastrointestinal indications. Her medical interests include pediatric rare diseases, eosinophilic gastrointestinal disorders, antiemetic therapies, and pancreatitis.



**James Valentine, JD, MHS**

James assists medical product industry and patient advocacy organization clients in a wide range of new drug and biologic development and approval matters. Mr. Valentine has been central to the transition of the FDA Patient-Focused Drug Development (PFDD) program to externally-led meetings, having helped plan and moderated two-thirds of those. Before joining his current firm in 2014, he worked at the FDA as a patient liaison where he facilitated patient input in benefit-risk decision-making and regulatory policy issues. There, Mr. Valentine

administered the FDA Patient Representative Program, helped launch the PFDD program, and developed the FDA Patient Network. He holds a law degree from University of Maryland and a masters from Johns Hopkins School of Public Health.



**Wahid Y. Wassef, MD, MPH, FACP**

Dr. Wassef is the Associate Chief Quality Officer Department of Medicine, Director of Advanced Endoscopy and Pancreatic Disease Clinic, and, Medical Director of Advanced Fellowship Program at the University of Massachusetts School of Medicine. His awards have included: Medical College of Virginia award for excellence in teaching, Governor's Award for excellence in clinical research by the American College of Gastroenterology for development of the PANQOLI (Pancreatitis Quality of Life Instrument) and Nobility in Science Award by the National Pancreas Foundation. His publications have included 65 original manuscripts in peer reviewed journals, multiple abstracts and chapters. Focus of research has been that of pancreatic disease.

The most significant study has been the development of the first quality of life instrument for the evaluation of patients with chronic pancreatitis: Pancreatitis Quality of Life Instrument (PANQOLI). This instrument was supported by

grants from the National Pancreas Foundation as well as the American College of Gastroenterology. It is currently available for use in the NPF website and since its publication in 2016 has been used in 6 original manuscripts, 4 ongoing clinical trials for the evaluation of various treatments in chronic pancreatitis patients, and 2 international studies in China and Denmark.

## **PANELISTS**

### **ADULT PANCREATITIS (PANEL 1)**



#### **Adriana Llamas Cowdin**

Adriana was first diagnosed with acute pancreatitis in 2015, with her first hospitalization lasting for 10 days. Between 2015 and 2016 she has undergone 17 procedures for her pancreatitis and was then diagnosed with chronic pancreatitis and pancreatic divisum and was living on and off feeding tubes. In November 2019, she had the TPIAT surgery and has regained parts of her life back.

In her personal time, Mrs. Cowdin is an avid reader, writer and published author who resides in Indianapolis, Indiana with her husband and fur baby, Henry Winston. She also enjoys traveling to IndyCar races where you can find her in the pits with an insider view as her husband is a Technical Director and Race Engineer in the IndyCar series.



#### **Eric Golden**

Eric is an investment banker, executive and attorney living in Santa Monica, California with his wife Rebecca and three children, Jonathan, Emma and Jackson. Mr. Golden was diagnosed with chronic pancreatitis in 2012 after years of worsening digestive issues. In an effort to learn as much as possible about the disease and help those suffering from it, he joined the board of the NPF and the Collaborative Alliance for Pancreatic Education and Research (CAPER) and co-founded Mission: Cure, a non-profit aimed at finding effective

treatments for the disease within 10 years. Professionally, Mr. Golden is the founder and Managing Director of Fluential Partners, an advisory firm focused on mergers and acquisitions, financing and other strategic transactions. He received a JD from Harvard Law School and an AB from Brown University.

**Jane Holt (please see bio above)**



### **Hillary Mendelsohn**

At the age of 36, Hillary was diagnosed with pancreatic cancer. Because of her dietary restrictions due to her streamlined digestive system, if she ate something that didn't work, acute pancreatitis would ensue. Over the last 16 years, she has had have had eight surgeries related to the pancreas. She is a pancreatic cancer survivor and pancreatitis patient that lives with the motto: Whatever it takes.

Professionally, Mrs. Mendelsohn's life is very busy as the Executive Vice President of The Carlyle Group, Ltd., a real estate company, that specializes in the manufactured housing industry. The company founded 40 years ago, owns and operates one of the largest privately held portfolios in the United States with properties in 10 states. In 1999, she created an internet company and is Founder and CEO of thepurplebook: The Definitive Guide to Exceptional Online Shopping, producing and publishing nine resource guides for online shopping in book form and launching the online version ([www.thepurplebook.com](http://www.thepurplebook.com)), in addition to consulting in the field of e-commerce and go-to media expert on online shopping trends.

Ms. Mendelsohn is the proud mother of Max (23) and Gabriella (21), and has expanded her family to include her new husband Mike and his children, Jonathan (21) and Brooke (20). They live in Los Angeles.

### **PEDIATRIC PANCREATITIS (PANEL 2)**



### **Logan Williams** *(As told my his Mother, Erica Williams)*

Logan is an amazing 7-year old boy on an epic journey with his pancreas. He is in the second grade, playing soccer and soon to start baseball. He is caring, insightful, feels emotions to the fullest, and has a great sense of humor. Mr. Williams was first diagnosed with pancreatitis at the age of four. It was not until he was five that his parents learned it was caused by a genetic mutation. As his journey continues, Mr. Williams continues to leave his family in awe of his resiliency. Hereditary pancreatitis is progressive and in this last year they are beginning to see it express itself more on a daily basis. Mr. Williams has been approved for the TPIAT – his family is grateful to have it as an option, knowing it is a huge leap, and they are very hopeful to help carve out steps in between.



### **Attie Jamison**

Diagnosed with pancreatitis at the age of 4, Attie spent time in the hospital each year as she was growing up. Eventually, the diseased pancreas began to affect her health in ways that caused her quality of life to suffer. As a dancer, she was extremely active and disciplined, but her body was beginning to let her down. After missing auditions and dress rehearsals, the frustration and pain were becoming more than she could handle. In December of 2018, Ms. Jamison underwent surgery at Cincinnati Children's Medical Center to remove her pancreas. Recovering from a TPIAT and adapting to a new normal has been challenging and rewarding. Ms. Jamison is a freshman at Muskogee High School in Muskogee, Oklahoma. She is a member of the Varsity Dance Team and participates in many AP classes. She is also an active leader in her youth group at Timothy Baptist Church. She enjoys teaching children's church and excels at organizing and interacting with small children. She loves to dance ballet, tap, jazz and hip hop, and she is a master "TikTok" performer.



### **Gracie Jamison**

On Gracie's 16th birthday, she sat shocked in the emergency room as she realized she was facing the same pancreatic disease she helped nurture her younger sister through for years. Fearful, she faced a journey of medical inquiries, blood work, and recording attacks and pain values ("on a scale of one to ten"). Ms. Jamison is a leader in her youth group at Timothy Baptist Church, where she also teaches children's church and is adored by the young children she works with. She is a member of the Muskogee High Video Productions Crew where she assists in photographing, filming, and live streaming sporting events each week. After three summers of mowing yards to purchase her first car, Ms. Jamison now enjoys working in the gifts section and customer service at a local pharmacy after school.



### **Rebecca Taylor**

Rebecca is 17 years old and lives in San Antonio, Texas. Her first attack of pancreatitis occurred at the age of 7. After years of hospitalizations and further pancreatitis attacks, Ms. Taylor underwent a TPAIT at the age of 12. Rebecca still suffers complications from her transplant that continues to require long-term hospitalizations. She is a constant advocate for better pediatric pancreas care and founded a nation-wide charity named Rebecca's Wish that has raised over a million dollars to help research, patient care and charitable services for children in need.



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