Join Us for the 2015 NACFC in Phoenix

We look forward to seeing you at the 29th annual North American Cystic Fibrosis Conference (NACFC) in Phoenix Oct. 8-10, with pre-conference sessions on Oct. 7.

As we prepare for the 2015 NACFC, we wanted to share some of this year’s highlights.

Plenary Speakers Will Address Personalized Medicine, CF Clinical Trials and Mental Health

We are entering an era of personalized medicine in which the CF care community will strive to provide the right treatments to the right patients at the right time. To achieve this goal, CF research and care will intersect as never before, encompassing efforts at the bench and at the bedside to move CF care into the 21st century.

John P. Clancy, M.D., will focus on personalized medicine in CF at the first plenary session, “CF Research and Care in the 21st Century – It’s Time to Get Personal,” at 4:30 p.m. Thursday, Oct. 8. Clancy, director of pulmonary medicine research at Cincinnati Children’s Medical Center, plays an instrumental role on several CF Foundation research committees, including the Biomarkers Consortium, the Translational Advisory Group, the Clinical Research Committee and the NACFC Planning Committee. He is also vice chairman of the Therapeutic Development Network’s Steering Committee.

On Friday Oct 9 at 9 a.m., the second plenary session, “Opening Doors to CF Clinical Research – Change is Coming,” will be presented by George Retsch-Bogart, M.D., professor of pediatrics in the division of pediatric pulmonology, at the University of North Carolina at Chapel Hill. As the faculty director of Network Operations for the CF TDN Coordinating Center and co-chair of the CF Clinical Research Executive Committee, Retsch-Bogart will highlight both the range of CF clinical trials across therapeutic categories coming in the next year as well as the tools being developed to help facilitate conversations between patients and their clinicians.
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Studies have identified anxiety and depression as co-morbidities that can affect multiple areas of clinical care, adherence and quality of life for individuals with CF and their caregivers. We are extremely fortunate that the following members of the International Committee on Mental Health in Cystic Fibrosis will provide information and guidance to care teams about screening and treatment at the third plenary session, “There is No Health Without Mental Health,” at 9 a.m. Saturday, Oct. 10:

Alexandra Quittner, Ph.D., Professor of Psychology and Pediatrics, University of Miami

Stuart Elborn, M.D., FRCP, Professor of Respiratory Medicine and Dean of Medicine, Dentistry and Biomedical Sciences, Queen’s University Belfast

Beth Smith, M.D., Associate Professor of Psychiatry and Pediatrics, University at Buffalo School of Medicine and Biomedical Sciences

Live Streaming

Let your patients, families and care team know that they can watch even more sessions this year through live streaming. Plenary sessions, select symposia, workshops, brown bags and One-on-One Live, a conversation between Dr. Anna Georgiopoulous and an adult with CF on the importance of screening for anxiety and depression, will be available for viewing. Access the live stream here.

Networking Opportunities

The 2015 NACFC will feature a wide variety of valuable networking opportunities:

- Poster Sessions — Daily poster sessions will offer opportunities to interact with colleagues about the latest CF research.
- Welcome Reception — Held on Thursday night, this reception will provide opportunities to connect with colleagues and broaden your professional network while experiencing the charm of Phoenix!
- Closing Event — This is the perfect atmosphere to bolster relationships with team members and strengthen professional connections during a relaxing dinner, topped off with rousing entertainment.

NACFC Mobile App

Be sure to download the 2015 mobile app when it becomes available in late September. Enhance your overall conference experience with CE credits and surveys on the go, venue maps, and full planning and scheduling features. A short, instructional webinar will be offered to demonstrate the app’s timesaving features.

Visit the CF Foundation booth in the exhibit hall to learn about basic and clinical research; education and clinical resources; the Patient Assistance Resource Center; public policy and access; and more.

For information about NACFC or to register, visit www.nacfconference.org.

2014 Mobile App Wins Plannie Award

The CF Foundation took home a Plannie Award for Best Use of a Mobile App at the recent Cvent CONNECT annual meeting June 14-18 in Las Vegas. Cvent, a leader in the field of conference registration and online solutions for events and surveys, awards “Plannies” to meeting planners in various categories.

The Best Use of a Mobile App award was based on the overall look and feel of the app, adoption metrics and marketing efforts, as well as the full usage of the suite of features and how those features are leveraged. The NACFC app was one of 13 apps up for the award.
Industry Group Names NACFC a Top Health Care Event

The CF Foundation's NACFC was acknowledged as a Top Healthcare Event at the Healthcare Convention and Exhibitors Association (HCEA) annual meeting June 20-23 in Denver. The acknowledgement came during a session focused on medical meeting industry trends presented by FreemanXP, a top meetings industry player that concentrates on meeting strategies and development.

NACFC was listed alongside industry giants, such as the American Diabetes Association, the American Heart Association and the American Association for Cancer Research. FreemanXP based its assessment on the caliber and quality of the event design, recent press interest and expected growth.

Quality Improvement Teams to Reconnect at NACFC

The second OneCF Center Learning and Leadership Collaborative (LLC2) teams will have a chance to reconnect at the NACFC after a June benchmarking site visit at the University of Minnesota.

Six teams and six quality coaches spent two days with the pediatric and adult programs.

An additional fifteen teams are participating in the Fundamentals LLC, which kicked off in May and continues through October 2016.

Quality coaches are currently visiting these sites. This group will convene in November 2016 for a face-to-face meeting and travel for a benchmarking site visit at Boston Children’s Hospital in April 2016. Twelve teams will participate in the Virtual Improvement Program, Fundamentals (VIP-F). This first of its kind program will kick off at NACFC and engage teams via asynchronous learning through March 2016.

Calling all CF Quality Coaches and OneCF “Pioneers”, LLC2 and FUN LLC participants!

Join quality improvement meetings at NACFC to learn, share and reconnect:

Tuesday, Oct. 6, 1-3:00 p.m., All CF Quality Coaches Session. This session is open to anyone who has participated as a CF Quality Coach - currently active or not. We’d love to connect with you to share what’s new and what’s on the horizon.

Tuesday, Oct. 6, 5:30-9 p.m., OneCF LLC “Pioneers”, OneCF LLC2, FUN LLC Participants and all CF Quality Coaches Session. An evening of celebration and learning, this session is open to any participant in the OneCF LLC “Pioneers”, OneCF LLC2 or FUN LLC, and any past or present CF Quality Coach. Our special dinner program speaker is Dr. Martin Wildman from Sheffield, UK, who will discuss how redesign and improvement of his CF Clinic created time for important adherence discussions with patients.

For information, contact Meghann Kinney at mkinney@cff.org.

Volunteer Engagement Conference Scheduled for NACFC

Approximately 150 West Coast chapter volunteer leaders, major gift donors and annual fund participants will gather in Phoenix on Oct. 8 for a Volunteer Engagement Conference, held in conjunction with NACFC.

With the theme of community and collaboration, session topics will range from CF Foundation patient resources and advocacy to panels on individual giving and adults with CF. After attending the NACFC opening plenary, attendees will end their day with an informal question and answer session with Robert J. Beall, Ph.D., Cystic Fibrosis Foundation president and CEO; Preston Campbell III, M.D., executive vice president of medical affairs; Michael Boyle, M.D., vice president of therapeutics development; and William Skach, M.D., vice president of research affairs.

For information, contact Meghann Kinney at mkinney@cff.org.

For information about QI activities and events at the NACFC, email Margie Godfrey, Ph.D., at margiegodfrey@gmail.com.
CF Care Model Design Project to Test Concepts in 2016

More than 60 clinicians, researchers, individuals with CF and family members met on May 26-27 at the CF Foundation to develop recommendations for testing a CF care model of the future in 2016. These participants in the Care Model Design Project brought their different perspectives together to inform a research plan where the findings would be implemented to produce optimal health for people living with the disease.

Cincinnati Children’s Hospital Medical Center and the Dartmouth Institute are leading the project, which is sponsored by the CF Foundation. Participants completed a prioritized concept roadmap, which features a number of concepts for testing, including:

• Enhancing the registry with more automated and open features
• Collaborative health tracking and home monitoring
• Customizable dashboards of personal clinical data
• Shared decision making tools, training and best practices
• An electronic exchange platform for sharing across the community
• Bundled payment reform pilot program
• Facilitation of transition to adult care
• Collaborative quality improvement network
• National Patient and Family Advisory Council Network

To learn more, join members of the design team during the NACFC at the CF Care Model Design Project Innovation Reception at 8 p.m. on Friday, Oct. 9, which is open to all attendees, or visit the CF Foundation booth. Representatives for each design concept will be available to talk about the concept and plans for testing. If your center is interested in participating in the testing phase, contact Sophia Thurmond at sophia.thurmond@cchmc.org.

Communications to Patients and Families

Video Release for “Living Today, Adding Tomorrows”

The “Living Today, Adding Tomorrows” video series gives a glimpse into the everyday lives of adults with CF and the remarkable people in our community who help add more tomorrows for those with the disease. The latest video in the series features Dana Curry, a 31-year-old with CF, who uses fun and motivating techniques to fit in all her daily treatments and therapies. View the video here.

Postcards Announcing the New CFF.org to Patients and Families

The new website was relaunched on July 21 as part of the Foundation’s strategic plan to better address the needs and concerns of the CF community. The site has been re-envisioned to empower people living with CF by providing more information, tools and resources. We hope you received the postcards announcing the relaunch, and have been able to hand them out to your patients and families.

The site will continue to incorporate updates to existing information as new material is created. If you have questions about finding resources or feedback to help us improve the site for your patients and families, please contact Paula Lomas at plomas@cff.org.
Patient Registry Research Showcased at NACFC

We are excited by the opportunity at NACFC to showcase research conducted using data from the CF Foundation Patient Registry.

Data from the Standardized Treatment Of Pulmonary Exacerbations (STOP) Study, which used data from the standard CF Foundation Patient Registry along with additional study-specific elements, will be presented during the session “Toward Rational Management of Pulmonary Exacerbations” (Thursday, Oct. 8, 2-3:50 p.m.).

During the Epidemiology of CF session (Thursday, Oct. 8, 10-11:45 a.m.) the following two projects will be presented:

1. Continuity of Care during Transfer from Pediatric to Adult Cystic Fibrosis Care
2. Risk Factors Associated with Rapid FEV1 Decline in Adults with Early Stage CF Lung Disease: Cohort Study of the U.S. CF Foundation Patient Registry

In addition, the following projects will be presented as posters in the exhibit hall:

1. Treatment Response to Ivacaftor in Clinical Practice: Analysis of the U.S. CF Foundation Patient Registry
2. Associations between CF Center Practices and Recovery from Pulmonary Exacerbations
3. Estimating the Number of Individuals with Cystic Fibrosis in the United States
4. Prevalence and Characteristics of Patients Lost to Follow-Up in U.S. CF Foundation Patient Registry

The projects listed above were part of the CF Foundation's intramural research program in collaboration with external clinicians and researchers. The CF Foundation Patient Registry data is also used by external researchers. The figure below displays the number of requests from external researchers for registry data that have been received and the number of publications based on these requests by year.

Patient and Family Experience of Care Survey Gains Momentum

More than 1,500 people with CF and their families have completed a survey about their experience of care at a CF center since national data collection kicked off in May. Quality Data Management Inc., a HIPAA-compliant company, coordinates the data collection process on behalf of the CF Foundation. People with CF and their families are able to complete the survey using tablets at the point of care, a secure website, or a toll-free phone line. Individuals and families are surveyed every six months regarding their experience and all survey answers are anonymous.

For information about the survey, data collection at your center and results, contact Karen Homa, Ph.D., at homakaren@gmail.com or Kathy Sabadosa, MPH, at kathryn.a.sabadosa@dartmouth.edu or look for information at the CF Foundation booth at NACFC.
White House Honors CF Adult for Work in Precision Medicine

Emily Kramer-Golinkoff, an adult with CF, was honored at the White House in July as a “Champion of Change” in the field of precision medicine for her work to advance the development of treatments for cystic fibrosis caused by rare, nonsense CFTR mutations. Along with eight other Champions, Kramer-Golinkoff participated in a discussion to share more about the work she is doing to harness the power of data to better treat disease and improve health for those with CF. The program also featured remarks by Health and Human Services Secretary Sylvia Mathews Burwell, Senior Advisor to the President Brian Deese and Director of the National Institutes of Health Francis Collins.

Several members of the CF Foundation’s national staff attended the event, along with CF parent Erin Moore of Ohio and Michael Seid, Ph.D., director of Health Outcomes and Quality of Care Research in the Division of Pulmonary Medicine of Cincinnati Children’s Hospital Medical Center.

To learn more about the Champions of Change event at the White House, click [here](#).

Dartmouth Institute Selects Testing Sites for “Coproduction of Care” Concept

The Dartmouth Institute has selected testing sites for the “coproduction of care” concept that is a key tenet of the CF Care Model Design Project. The following alpha testing sites will work directly with the Dartmouth team:

- Ann & Robert H. Lurie Children’s Hospital of Chicago/Northwestern
- University of Minnesota
- Children’s Hospitals and Clinics of Minnesota

Meanwhile, Cincinnati Children’s Hospital Medical Center will continue testing a patient-facing platform for tracking patient-reported outcome measures (Orchestra) and share lessons and results with the alpha sites. Each site will be invited to join an international network of programs in Sweden and the U.K. that also are testing the coproduction concept.

With support from the Robert Wood Johnson Foundation and the CF Foundation, the Dartmouth Institute is leading a three-year project to test and evaluate technology that would enable individuals with CF and their care teams to coproduce or co-design care. Care teams would combine real-time, patient-reported outcome measures with clinical data to create a dashboard that they could use with patients to facilitate treatment decisions and support self-management. The dashboard data also could be used to enhance information in the CF Foundation Patient Registry.

Learn more about the concept of coproduction and the coproduction project at NACFC

Come join the symposium *CF with Fresh Eyes: A Partnership to Create the Future?* on Friday, October 9th at 10:30 am, featuring adults with CF, a parent, and adult and pediatric clinicians who will explore the concept of coproduction of care. To learn more about the coproduction project, contact Kathy Sabadosa, MPH, at kathryn.a.sabadosa@dartmouth.edu, or visit the CF Foundation booth at NACFC.
CF Advocates Contact their Elected Officials in Support of CF Research and Drug Review

As Congress considers funding levels for next year, volunteer advocates have been reaching out to their elected officials in support of increased funding for the Food and Drug Administration (FDA) and the National Institutes of Health (NIH). So far, nearly 9,000 messages have been sent to members of Congress, and many volunteers have met with their elected representatives, explaining the importance of robust funding of these agencies for cystic fibrosis research and drug development.

NIH-funded CF research supports scientists studying the disease from every angle, potentially leading to the cures of tomorrow. For example, NIH-funded research into innovative technologies was vital to Kalydeco’s development. The FDA also relies on adequate funding to swiftly review new treatments and quickly get them to patients. This is especially important as therapies that target the root cause of CF move through the pipeline.

Congress has yet to determine funding levels for the next fiscal year, which starts on Oct. 1. As Congress continues the process of setting funding levels, the CF community will continue its campaign to ensure sufficient funding for these agencies. You can find information about taking action here.

The Ensuring Access to Clinical Trials Act

The Ensuring Access to Clinical Trials Act (EACT) passed in the Senate by unanimous consent on July 17.

This piece of legislation was introduced in January of this year and makes the Improving Access to Clinical Trials Act (IACT) permanent. This will allow those with rare diseases, such as cystic fibrosis, to receive compensation for participating in clinical trials without fear of losing important health benefits through Social Security Supplemental Security Income and Medicaid.

This victory would not have been possible without the hard work of our volunteer advocates and the support of our care center network. More than 75 organizations, including patient groups, CF care centers and industry representatives signed a letter in support of the bill. Volunteers across the country sent more than 24,000 messages to their representatives asking them to support this legislation.

Click here to take action now! Tell your member of Congress to pass EACT before it expires on October 5, 2015.

For information, contact Stephanie Krenrich at skrenrich@cff.org.

Mutation Analysis Program

The Mutation Analysis Program (MAP) provides free genotyping and follow-up testing as needed for people with CF. Identifying which strand of the CFTR mutation a person has can help the person and his or her provider make informed decisions about their treatment options. Call 1-888-315-4154 or email MAP@cff.org for screening and to obtain the enrollment form.

Have Comments?

If you have comments or suggestions for Network News, please contact Paula Lomas at plomas@cff.org.

We welcome your comments and ideas.
Increasing Need for Clinical Trial Participants

There are more CF clinical trials than ever before, with 15 studies recruiting patients and more planned to start in the coming months. This means that more than 1,400 new participants are needed to reach enrollment targets for these trials in the next six months. One way to help accomplish this ambitious target is to spread the word to patients and families about ongoing studies and increase patient referrals.

To help in this effort we have created an Ongoing and Upcoming Clinical Trial Spreadsheet that contains brief descriptions, eligibility information and links to further information for ongoing and soon-to-be enrolling clinical trials. The spreadsheet will be sent to clinic coordinators and center directors on a regular basis and can be accessed on CFF.org.

We also have a new educational resource that visually depicts the phases of clinical trials. This is available in print through resources@cff.org.

If you have any questions, email Christina Román at croman@cff.org.

A Virtual Connection: Care Center Town Halls

After the exciting announcement of the FDA approval of Orkambi, the CF Foundation hosted a webinar for the CF Care Center Network on July 9 that featured an interview with Mike Boyle, M.D., vice president of therapeutics development at the CF Foundation, and Mary Dwight, senior vice president for policy and CF community affairs at the CF Foundation. For those who were not able to attend, you can access the session here. Please provide us with your feedback using this link to help us improve webinars being offered.

The Town Hall on June 9, 2015 highlighted exciting projects underway at the CF Foundation to engage individuals with CF and their families. Cindy George, senior director of patient engagement, introduced the patient engagement program by addressing adherence, part of the CF Foundation strategic plan. “Re-envisioning of cff.org” was presented by Amy DeMaria, senior vice president of communications, who described the redesign of the Foundation website to the care center audience. Click here to view the June Town Hall.

If you have feedback for either of these sessions, or ideas for future sessions, contact Paula Lomas at plomas@cff.org.

Get the Word Out!

CF Adult and Family Advisors Group

The CF Foundation is still recruiting for volunteers to provide input and feedback on a variety of projects and topic areas. If you know a person within the CF community who may be interested in providing his or her unique perspective about CF care, research and quality of life, send them this application link: afasignup.cff.org. The members of the Adult and Family Advisors Group (AFA) have been a great source for feedback and knowledge on a variety of topics. We encourage you to utilize the AFA as a resource when you need input from the experts who are directly affected by CF and to spread the word to others who may be able to use this group.

For information about this recruitment process or the projects and topic areas we are recruiting for, contact Danielle Lowe or Kelsey Fredkin at communityaffairs@cff.org.
Publication Watch

This column highlights recently published work derived from the Foundation’s care center network or patient registry data. We congratulate the authors of these manuscripts and sincerely thank the many contributors who made this work possible.

Sustained Benefit from Ivacaftor Demonstrated by Combining Clinical Trial and CF Patient Registry Data
Gregory S. Sawicki, et al.

*American Journal of Respiratory and Critical Care Medicine*, published online July 1, 2015

This study compared ivacaftor-treated CF patients with a G551D mutation from the Phase 3 clinical trials and open-label extension to propensity-matched CF patients homozygous for the F508del mutation from the CF Foundation patient registry. Over the three-year time frame of the analysis, the rate of decline in FEV1 percent predicted for G551D patients receiving ivacaftor was slower than F508del homozygotes by nearly one half. This data suggests that ivacaftor may be a disease-modifying therapy. Read more here.

Assessing Differences in Mortality Rates and Risk Factors Between Hispanic and Non-Hispanic Patients with Cystic Fibrosis in California
MyMy C. Buu, et al.

*Chest*, published online June 18, 2015

This retrospective analysis of CF Foundation patient registry data for California residents with CF diagnosed from 1991-2010 showed that Hispanic patients have a higher mortality rate than non-Hispanic patients, even after adjusting for socioeconomic status and clinical severity. At 6 years of age, the mean FEV1 for Hispanic patients was also lower than for non-Hispanic patients (77 percent versus 89 percent-predicted). It will be important to understand the underlying mechanisms for this ethnic disparity in order to improve care for all CF patients. Read more here.

Efficacy and Safety of Ivacaftor in Patients With Cystic Fibrosis Who Have an Arg117His-CFTR Mutation: A Double-Blind, Randomized Controlled Trial
Richard B. Moss, et al.


This 24-week double-blind, placebo-controlled RCT enrolled 69 patients 6 years and older with an R117H mutation. Ivacaftor did not result in a significant improvement in FEV1 (treatment difference in mean absolute change in FEV1 percent predicted of 2.1 percent, p=0.20) in the overall study population, but did significantly improve FEV1 in the adult subgroup (treatment difference in mean absolute change in FEV1 percent predicted of 5.0 percent, p=0.01). Also of note, ivacaftor resulted in significant treatment differences in sweat chloride (-24.0 mmol/L, p<0.0001) and CFQ-R respiratory domain scores (8.4, p=0.009). Collectively, the results suggest that the drug might benefit patients with the R117H mutation who have established disease. Read more here.

Impact of Sustained Eradication of New *Pseudomonas aeruginosa* Infection on Long-Term Outcomes in Cystic Fibrosis
Nicole Mayer-Hamblett, et al.

*Clinical Infectious Diseases*, published online May 13, 2015

This cohort study used observational follow-up data on children participating in the Early Pseudomonas Infection Control (EPIC) trial who received standardized therapy for newly acquired Pa. Of the 249 participants, 172 (69 percent) achieved sustained eradication of Pa during the trial (sustained eradicators). Over the median 5-year follow-up, sustained eradicators had a 74 percent reduced risk of developing chronic Pa and a 57 percent reduced risk of mucoidy compared with nonsustained eradicators. Of note, there was no association between eradication status and clinical outcomes including rate of exacerbation and lung function decline. Read more here.

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Outcome of Infants with Indeterminate Diagnosis Detected by Cystic Fibrosis Newborn Screening

Clement L. Ren, et al.


This retrospective analysis of the CF Foundation Patient Registry data from 2010 to 2012 compared demographic, diagnostic, anthropometric, health care utilization, microbiology, and treatment characteristics between infants with CF and infants with CRMS diagnosed via newborn screening. By using CF Foundation guideline definitions, 1540 and 309 infants met the criteria for CF and CRMS, respectively. Infants with CRMS tended to have normal nutritional indices. However, 11 percent of infants with CRMS had a positive *Pseudomonas aeruginosa* respiratory tract culture in the first year of life. Of note, 40.8 percent of infants with CRMS were entered into the registry with a clinical diagnosis of CF, which may reflect misclassification or clinical features not collected in the registry. Read more here.

Behavioral and Nutritional Treatment for Preschool-Aged Children With Cystic Fibrosis: A Randomized Clinical Trial

Scott W. Powers, et al.


This RCT included 78 children aged 2-to-6 years old with CF and pancreatic insufficiency. The behavioral intervention (individualized nutritional counseling targeting increased energy intake and training in behavioral child management skills) was compared to a control group provided education. Sessions occurred weekly for eight weeks then monthly for four months (the treatment period). Participants then returned to standard care with 12-month follow-up thereafter. From pretreatment to post-treatment, the intervention increased daily energy intake by 485 calories vs 58 calories for the control group (P < .001) and increased the weight-for-age Z score by 0.12 units vs 0.06 for the control (P = .25). From pretreatment to the 12 month follow-up, the intervention increased the height-for-age Z score by 0.09 units vs −0.02 for the control (P = .049). The results provide evidence that behavioral and nutritional treatment may be efficacious for preschoolers aged 2-to-6 years old with pancreatic insufficiency. Read more here.

Clinical Care Guidelines Update

**Nontuberculosis Mycobacteria (NTM)**

The Nontuberculosis Mycobacteria Consensus Statements are being prepared for submission to *Thorax.*

**Screening and Treatment of Depression and Anxiety**

The Depression and Anxiety Consensus Statement manuscript has been submitted to *Thorax.*

**Clinical Care for Preschool Aged (2-5 years old) Children**

The committee is preparing the manuscript for re-submission to *Pediatrics.*

**Enteral Nutrition Consensus Statements**

Sarah Jane Schwarzenberg and Amanda Leonard are chairing this committee. These statements are currently under development.

**Colorectal Cancer Screening Consensus Statements**

Albert Lowenfels and Alexander Khoruts are chairing the colorectal cancer screening consensus statements. These statements are currently under development.

**Diagnosis Consensus Conference**

Philip Farrell, Patrick Sosnay, and Clement Ren, will be chairing the diagnosis consensus conference. The conference will take place in October.

For information about the clinical practice guidelines contact Sarah Hempstead at shempstead@cff.org.