This annual report aims to be a comprehensive report of the activities for Cure VCP Disease in 2020. In spite of COVID-19, we have had an active and productive year towards driving collaborative research, increasing patient identification, and providing educational opportunities for the VCP disease community.

CURE VCP DISEASE MISSION

Cure VCP Disease, Inc. was formed in 2018 to drive efforts to cure diseases related to mutations of the Valosin Containing Protein gene. This includes the disease Inclusion Body Myopathy associated with Paget’s disease of bone and Frontotemporal Dementia (IBMPFD). The specific objectives of our organization are to:

- provide global education and awareness of VCP disease;
- develop and maintain a global patient registry of VCP disease patients;
- develop and maintain a fundraising vehicle;
- collaborate with other global organizations and groups advocating for cures and therapies for diseases related to VCP disease;
- sponsor, fund, host and participate in events and activities that promote efforts to advance treatments and cures for VCP disease.
BOARD OF DIRECTORS - OFFICERS

CEO
Nathan Peck
Americus, Georgia

Secretary
Sujata Patel, RPh, CFMP
Wadsworth, Ohio

Treasurer
Allison Peck
Americus, Georgia

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Ming Guo, MD, PhD
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Chris Weihl, MD, PhD
Washington University, St. Louis, MO

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On March 7, 2020, Cure VCP Disease celebrated the awarding of its first major research grant at the Uplifting Athletes Young Investigator Draft at Lincoln Financial Field in Philadelphia, Pennsylvania. At this event, I reflected how, in two short years, here we were awarding a significant research grant to a young investigator and how this award was bringing awareness of VCP disease to a large audience. The event was only four days before life would change for all Americans due to the "unprecedented" COVID-19 virus. I hope that you are able to see throughout this report how Cure VCP Disease refused to let COVID-19 slow us down from driving an "unprecedented" 2020 through innovative activities and events.

I had an even prouder moment in May when four VCP disease families and I presented to the U.S. Food and Drug Administration (FDA) during a Patient Listening Session. It took nine months to schedule this meeting, and when the Deputy Director of Division of Neurology 1 commented that she found the meeting "incredibly educational" and "it brought to life a greater understanding of the disease" it reaffirmed the "unprecedented" impact that Cure VCP Disease is making.

With tremendous support from our scientific community and volunteers, we have been incredibly resilient in adapting to the changes this year. We had to cancel our April 2020 Patient and Caregiver Conference scheduled in St. Louis, but we rescheduled our speakers to present during six monthly webinars. The webinars averaged about 35 participants from both the patient and scientific communities. We postponed our September 2020 VCP Scientific Conference, but we created a VCP Scientific Focus Group, which consists of 10-15 VCP focused global scientific researchers who present on their latest research. The meetings are helping to build momentum towards a rescheduled in-person scientific meeting in 2021 to be held in North America.

I am extremely proud of our rapid progress towards creating a remote functional measures study of VCP patients to understand the rate of degradation due to the disease. Instead of waiting years for government or academic funding to establish measurement locations, train personnel and incentivize patients to travel, we believe that COVID-19 has presented an opportunity for us to conduct all of these measures remotely using Zoom. We have already completed Phase 1 with our partner, Nationwide Children’s Hospital in Columbus, Ohio and intend to start Phase 2 with 20 patients in early 2021. Cure VCP Disease will fund this project to expedite the execution of this study. While it is vital to validate that there is no statistical difference between the measurement of patients in home versus in clinic, we are also excited that the data from this study will provide a greater understanding of the degradation of patients due to VCP disease and the potential to be used in future clinical trials.

Your participation and support have been vital towards aligning all of the pieces and reducing the complexity towards finding a cure for this awful disease. We appreciate and need your continued support to achieve even more "unprecedented" results in 2021!

Nathan Peck

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Quantitatively understanding the progression of VCP disease is vital towards developing future therapeutics and conducting clinical trials. Capturing patient medical records, measuring patients’ physical performance and archiving outcomes over time is the preferred method and a prerequisite for pharmaceutical organizations. These activities and measurements are considered a "natural history study." Some members of the VCP disease research community have conducted smaller natural history study activities, but not at the scale that will be required by pharmaceutical organizations and the FDA for clinical acceptance.

Many times these studies are funded by pharmaceutical companies or by the National Institutes of Health (NIH). Because VCP disease is an "ultra" rare disease, finding a well funded and interested pharmaceutical or biotech company could take years. Applying for NIH grants could also take years. Time is not a VCP disease patient’s friend.

COVID-19 has presented an opportunity, with the increased acceptance of telehealth processes, to conduct the physical measurement of patients from the comfort of their homes. Cure VCP Disease has partnered with the Lowes Lab at Nationwide Children’s Hospital in Columbus, Ohio to pilot and conduct a remote functional measures study. Cure VCP Disease desires to self-fund the study in order to expedite its start and completion.

In parallel, Cure VCP Disease has been selected by a company called AllStripes to facilitate the collection and analysis of patients’ medical records. VCP disease patients can consent to allow AllStripes to collect their medical records, which will then be curated into reports and information that can be utilized for advancing treatments.

Cost: $0 to patients and researchers
Data ownership: Patients
With a minimum of 40 VCP disease patients consenting to share their medical records, AllStripes will curate the data to understand trends and allow access to global researchers. Patients benefit by having a complete medical record archive for their use.
Understanding the prevalence of VCP disease is critical for advancing research and drug development. The Cure VCP Disease CoRDS Patient Registry was created in June 2018 and is the only comprehensive, global database of VCP disease patients. Through our registry, patients have the opportunity to provide a real world understanding of their daily life, which can be used to shape future clinical trials.

<table>
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<tr>
<td>United States of America</td>
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**PATIENT REPORTED DIFFICULTIES OF DAILY LIFE**

SIGNIFICANT PUBLICATIONS & MEDIA

The Cure VCP Disease Patient Registry was highlighted in a paper published in the Orphanet Journal of Rare Diseases in Sept. 2020. Chiseko Ikenaga from Washington University in St. Louis analyzed the Cure VCP Disease Patient Registry and the functional measures performed by Dr. Chris Weihl at our 2019 Patient & Caregiver Conference. She concludes, "The Cure VCP Disease Patient Registry is useful for deepening the understanding of patient daily life." This publication helped to elevate the importance of patient involvement within rare disease research and disease characterization.

In January, Dr. Chris Weihl and Nathan Peck were guests on Episode 18 of the "CoRDS Cast" podcast. They discussed the role of patient advocacy organizations in working with researchers. The episode is available on Spotify, Apple Podcasts, or your favorite podcast service.

Patient Todd Warner created "VCP & Me" videos helping patients, care partners and friends understand how he has adapted to dealing with VCP disease. The videos can be found on the Cure VCP Disease YouTube channel.

In September, Nathan Peck participated on a panel at Global Genes Live! Summit entitled "Elevate Natural History Study Planning and Reach." The video is available at http://bit.ly/sept2020gg


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Q1 2020 ACCOMPLISHMENTS

YOUNG INVESTIGATOR TRAVEL SCHOLARSHIP

Cure VCP Disease provided a travel scholarship to a VCP researcher from Louisiana State University to participate in the AAA+ Keystone Symposium in Lake Tahoe, CA. The poster presentation was on "Understanding CRISPR knock-in flies to study the pathogenesis of IBMPFD related VCP mutations." The researcher also handed out Cure VCP Disease brochures.

RARE ENTREPRENEUR BOOTCAMP

Three officers from Cure VCP Disease were invited to participate in the Global Genes Rare Entrepreneur Bootcamp at Ultragenyx in Novato, California. The meeting was a fantastic opportunity to learn strategies for drug development from industry and other patient advocates.

UPLIFTING ATHLETES GRANT

Cure VCP Disease awarded its first ever research grant to Dr. Cheng Cheng at the University of California-Irvine as part of the Uplifting Athletes Young Investigator Draft matching grant program. Cure VCP Disease awarded a grant of $10,000, matched dollar for dollar by Uplifting Athletes. This grant is enabling Dr. Cheng to understand VCP inhibitors and antisense oligonucleotides (ASOs) in VCP mice models.

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**Q2 2020 ACCOMPLISHMENTS**

**FDA PATIENT LISTENING SESSION**
Cure VCP Disease conducted a Patient Listening Session with the FDA in May. This might have been one of our most important activities of 2020 as five patients and care partners shared their family and personal stories, which helped the FDA understand the burden of VCP disease. One of the directors at the FDA commented that they had recently heard of VCP disease but had no idea as to the extent of the burden of the disease. This meeting will have lasting impact as we continue to engage with the FDA on clinical trials.

**VIRTUAL MILLION DOLLAR BIKE RIDE**
Team Cure VCP Disease raised $22,000 for research while participating in the Million Dollar Bike Ride hosted by the University of Pennsylvania Orphan Disease Center. The ride was held virtually this year. Over the month of May, our eight bike riders completed over 576 miles, dedicating their miles and research dollars towards research for a cure.

**LAUNCH OF A WEBINAR SERIES**
COVID-19 necessitated the cancellation of our 2020 Patient & Caregiver Conference; however, our community was not deterred. All scheduled presenters participated in a series of 6 one-hour educational webinars, with up to 50 in attendance at each meeting. All presentations are available on our YouTube channel.

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Q3/4 2020 ACCOMPLISHMENTS

LAUNCH OF SCIENTIFIC FOCUS GROUP
Our network of VCP scientists and the Cure VCP Disease leadership team has been planning the first VCP Scientific Conference in North America, and the team was awarded a $60,000 grant from the NIH to conduct the conference at Caltech in September 2020. Due to COVID-19, we postponed the conference until 2021, but we maintained momentum by creating a monthly VCP scientific focus group, comprised of global VCP scientists. We have conducted four meetings that have encouraged networking, collaboration, and a safe environment for experts to share unpublished findings.

PARTICIPATION IN HAPPY HOURS
People need community, especially when faced with a rare disease and amplified by the pandemic. Led by patients and care partners, this monthly hour of casual conversation has provided a forum for patients and their family to discuss their symptoms and struggles. Throughout the last five months, we have conducted 14 "happy hours," where attendance and relationships have grown. One patient explained, "Even though many miles may separate us, we are not alone."

SUCCESSFUL PILOT STUDY CONDUCTED: REMOTE FUNCTIONAL MEASURES
Five VCP disease patients participated in a proof of concept study to evaluate the feasibility of launching a remote functional measures study. Using Zoom, a clinician from Nationwide Children's Hospital guided patients through tasks which measured each patient's physical abilities. In order to maintain standardization and consistency for the study, Cure VCP Disease purchased and provided equipment kits to each patient.
Thanks to our generous donors, Cure VCP Disease is financially positioned to make a tremendous global impact in the VCP disease community in the years to come. Our organization is led by a volunteer seven-member board of directors, and we have no staff. Our leadership prioritizes the use of cost-effective tools and maintaining low overhead so that we can maximize our investment in research, education and awareness.

**2020 EXPENDITURES**

- Research: 63%
- Printed Material & Resources: 11%
- Corporate Expense: 7%
- Software: 7%
- Insurance: 6%
- Conference Attendance: 3%
- Other: 3%

**Reporting as of November 7, 2020**

**$23K**

- Research: 63%
- Printed Material & Resources: 11%
- Corporate Expense: 7%
- Software: 7%
- Insurance: 6%
- Conference Attendance: 3%
- Other: 3%

**$23K**

- Research: 63%
- Printed Material & Resources: 11%
- Corporate Expense: 7%
- Software: 7%
- Insurance: 6%
- Conference Attendance: 3%
- Other: 3%

**$82K**

- Research: 63%
- Printed Material & Resources: 11%
- Corporate Expense: 7%
- Software: 7%
- Insurance: 6%
- Conference Attendance: 3%
- Other: 3%

**$71K**

- Research: 63%
- Printed Material & Resources: 11%
- Corporate Expense: 7%
- Software: 7%
- Insurance: 6%
- Conference Attendance: 3%
- Other: 3%

**2020 GRANTS RECEIVED**

- NORD® Capacity Building Grant: $5,000
- Global Genes® Global Impact Grant: $3,500
- MIDA® Patient Support Grant: $2,000
- 4imprint® Support Grant: $500

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ACCELERATING PROGRESS
TOGETHER WE CAN MAKE A DIFFERENCE

Accomplishments - 2020

- Participate @ World Lysosomal Conference
- Global Genes Rare Entrepreneur Bootcamp
- Participate @ Rare Disease Week on Capitol Hill
- CoRDS Cast podcast w/ Dr. Weihl
- Travel scholarship to researcher for AAA+ Protein Keystone Symposium
- Uplifting Athletes Young Investigator Draft

- FDA patient listening session
- 1st & 2nd online Happy Hours
- NIH funding for VCP Scientific Conference
- *Cancelled* Patient conference
- *Cancelled* MDA Scientific & Clinical Conference

- Dr. Nupur Ghoshal webinar
- Publication of paper in Orphanet Journal of Rare Disease
- 7th & 8th online Happy Hours
- Dr. Alyssa Johnson webinar
- Start of natural history remote study POC
- Speaker at Global Genes Live
- 9th & 10th online Happy Hours

- First VCP Scientific Focus Group
- First conference speaker webinar
- 3rd & 4th online Happy Hours
- Million Dollar Bike Ride
- Speaker at Rare Drug Development Symposium
- Connection with India neuromuscular researchers
- Dr. Tsui-Fen Chou webinar
- 5th & 6th online Happy Hours
- Completion - Phase 1 remote functional measures
- Lindsay Alfano webinar
- 11th & 12th online Happy Hours

- Atty Bryan Van Brunt webinar
- Selection to AllStripes
- 13th & 14th online Happy Hours