



2020 Annual Report

A Year of Accomplishments.



www.curevcp.org

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2020 ANNUAL REPORT

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.

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CEO LETTER

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.



Rob Long, CEO of Uplifting Athletes, Dr. Cheng Cheng, UC-Irvine, Nathan Peck, CEO of Cure VCP Disease

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

2021 STRATEGIC IMPERATIVE

INNOVATIVE & EXPEDITED UNDERSTANDING OF VCP DISEASE PROGRESSION

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.

\$120,000

FUNDING REQUIRED

FUNCTIONAL MEASURE STUDY



Cost: \$120,000

Data ownership: Nationwide & Patients

The Lowes Lab at Nationwide Children's is recognized among the neuromuscular disease community as experts in conducting these studies. Twenty VCP disease patients will participate in a 12 month study to measure physical performance. Patients will perform measures both in Columbus, OH and at home for a total of 6 sessions each.

CAPTURE MEDICAL RECORDS



Cost: \$0 to patients and researchers

Data ownership: Patients

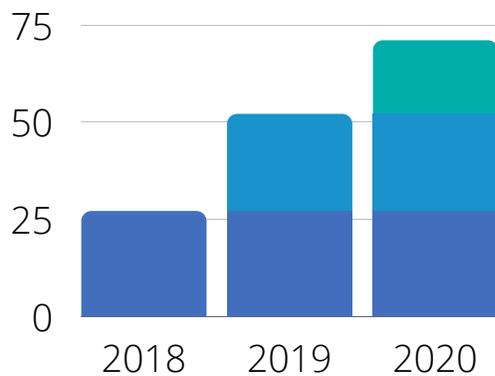
With a minimum of 40 VCP disease patients consenting to share their medical records, AllStripes will curate the data to understand trends and allow access to global researchers. Patients benefit by having a complete medical record archive for their use.

OUR PATIENT REGISTRY



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.

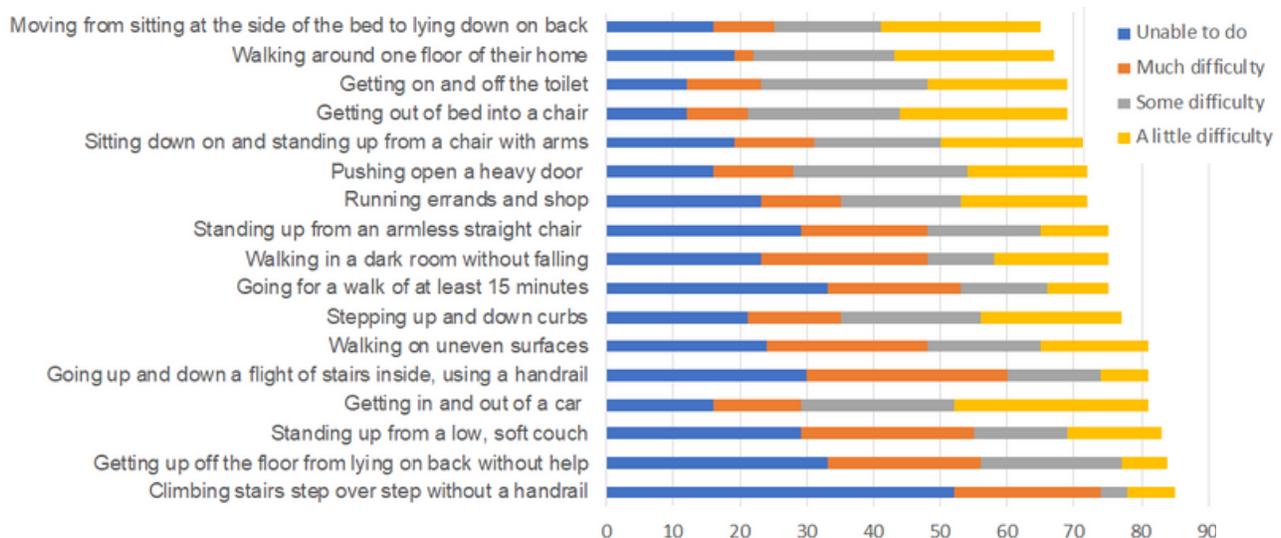
ENROLLED PARTICIPANTS BY YEAR



LOCATION OF ENROLLED PARTICIPANTS

Country	Number	%
Australia	5	7.4%
Brazil	1	1.5%
Canada	3	4.4%
Germany	2	2.9%
The Netherlands	1	1.5%
Thailand	1	1.5%
United Kingdom	5	7.4%
United States of America	50	73.5%

PATIENT REPORTED DIFFICULTIES OF DAILY LIFE



Source: Ikenaga C, et al. Orphanet J Rare Dis. 2020. 15(267).



SIGNIFICANT PUBLICATIONS & MEDIA

PUBLICATION

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 Wehl 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.



PODCAST

In January, Dr. Chris Wehl 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.



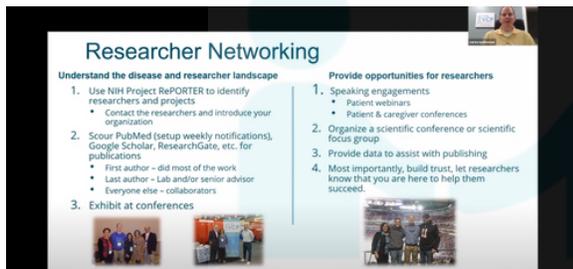
VIDEO



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

PRESENTATIONS

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>

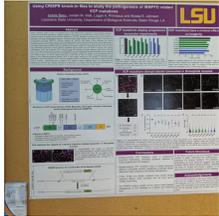


In June, Nathan Peck participated at the Global Genes Rare Drug Development Symposium entitled "Exploring the World of Funding Research." The video is available at <http://bit.ly/june2020gg>



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.

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.



Q3/4 2020 ACCOMPLISHMENTS

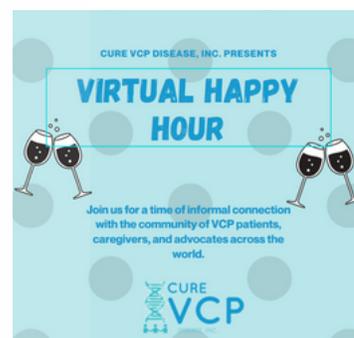
LAUNCH OF SCIENTIFIC FOCUS GROUP



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.



FINANCIAL UPDATE

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.

AMOUNT RAISED BY YEAR

\$23K

2018

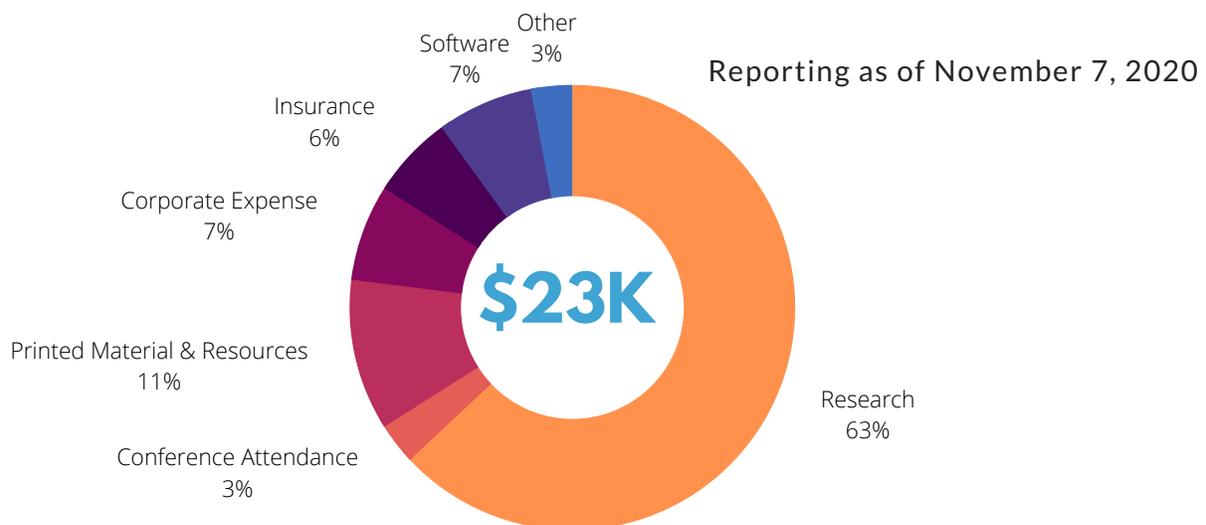
\$82K

2019

\$71K

2020 TO DATE

2020 EXPENDITURES



2020 GRANTS RECEIVED



NORD
National Organization
for Rare Disorders

\$5,000

Capacity Building Grant



Global Genes
RARE Foundation Alliance
Member

\$3,500

Global Impact Grant



MDA Muscular
Dystrophy
Association

\$2,000

Patient Support Grant



4imprint

\$500

Support Grant



ACCELERATING PROGRESS

TOGETHER WE CAN MAKE A DIFFERENCE

Accomplishments - 2020

